HEARING ON EXAMINING POLICIES THAT INHIBIT INNOVATION AND PATIENT ACCESS

HEARING

BEFORE THE

COMMITTEE ON WAYS AND MEANS
HOUSE OF REPRESENTATIVES

ONE HUNDRED EIGHTEENTH CONGRESS

FIRST SESSION

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United States House Committee on
Ways & Means
CHAIRMAN JASON SMITH

FOR IMMEDIATE RELEASE
May 3, 2023
No. HL-02

CONTACT: 202-225-3625

Chairman Smith and Health Subcommittee Chairman Buchanan Announce Subcommittee Hearing on Examining Policies that Inhibit Innovation and Patient Access

House Committee on Ways and Means Chairman Jason Smith (MO-08) and Health Subcommittee Chairman Vern Buchanan (FL-16) announced today that the Subcommittee on Health will hold a hearing on examining policies that will have negative effects on medical innovation and reduce patient access to therapies. The hearing will take place on **Wednesday, May 10, 2023, at 2:00pm in 1100 Longworth House Office Building**.

Members of the public may view the hearing via live webcast available at https://waysandmeans.house.gov. The webcast will not be available until the hearing starts.

In view of the limited time available to hear the witnesses, oral testimony at this hearing will be from invited witnesses only. However, any individual or organization not scheduled for an oral appearance may submit a written statement for consideration by the Committee and for inclusion in the printed record of the hearing.

DETAILS FOR SUBMISSION OF WRITTEN COMMENTS:

Please Note: Any person(s) and/or organization(s) wishing to submit written comments for the hearing record can do so here: WMSubmission@mail.house.gov.

Please ATTACH your submission as a Microsoft Word document in compliance with the formatting requirements listed below, **by the close of business on Wednesday, May 24, 2023**. For questions, or if you encounter technical problems, please call (202) 225-3625.

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All submissions and supplementary materials must be submitted in a single document via email, provided in Word format and must not exceed a total of 10 pages. Please indicate the title of the hearing as the subject line in your submission. Witnesses and submitters are advised that the Committee relies on electronic submissions for printing the official hearing record.
All submissions must include a list of all clients, persons and/or organizations on whose behalf the witness appears. The name, company, address, telephone, and fax numbers of each witness must be included in the body of the email. Please exclude any personal identifiable information in the attached submission.

Failure to follow the formatting requirements may result in the exclusion of a submission. All submissions for the record are final.

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The Committee seeks to make its facilities accessible to persons with disabilities. If you require accommodations, please call 202-225-3625 or request via email to WMSubmission@mail.house.gov in advance of the event (four business days' notice is requested). Questions regarding accommodation needs in general (including availability of Committee materials in alternative formats) may be directed to the Committee as noted above.

Note: All Committee advisories and news releases are available on the Committee website at http://www.waysandmeans.house.gov/.

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EXAMINING POLICIES THAT INHIBIT INNOVATION AND PATIENT ACCESS

WEDNESDAY, MAY 10, 2023

HOUSE OF REPRESENTATIVES,
SUBCOMMITTEE ON HEALTH,
COMMITTEE ON WAYS AND MEANS,
Washington, DC.

The subcommittee met, pursuant to call, at 2:11 p.m., in Room 1100, Longworth House Office Building, Hon. Vern Buchanan [chairman of the subcommittee] presiding.

Chairman BUCHANAN. The committee will come to order.

Thank you for being with us today for the hearing focused on innovation and some of the biggest roadblocks.

We can all agree that America is a global leader of innovation, and the government should do everything it can to foster an environment that promotes greater innovation and patient access to innovative care.

Unfortunately, we have all seen the news about recent examples of government getting in the way: CMS' restrictive coverage mandate for new, promising Alzheimer's treatment, repealing the Trump admin rule with no replacement still, CMMI considering changes to cover for part B drugs that receive FDA accelerated approval, USTR's TRIPS waiver of critical IP protection for COVID vaccine, and the so-called government negotiation of drug prices implemented under the Inflation Reduction Act.

In fact, just last week data was released on a third promising Alzheimer's drug, showing it significantly slows the progression of the disease. But it will still be a subject of recurrent restrictive CMA mandates.

The landscape has changed since June 2021. The Aduhelm was approved, but CMS refuses to consider it, the coverage, despite evidence showing they are very effective in treating Alzheimer's in its early stages.

This is progressive, and 6.7 million Americans living with it don't have time to wait on CMS to come to its senses. This delay means many things to a lot of the different patients, and it has been a big challenge. In fact, in April, 26 bipartisan attorney general across—26 attorney generals across the country sent a letter to Secretary Becerra and the administration on CMS to consider a requirement for covering these drugs.

And I am submitting this letter for the record today.

[The information follows:]

GENTNER DRUMMOND
ATTORNEY GENERAL

April 26, 2023

The Honorable Xavier Becerra
Secretary
U.S. Department of Health & Human Services
200 Independence Avenue, S.W.
Washington, DC 20201

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

Dear Secretary Becerra and Administrator Brooks-LaSure:

As Democratic and Republican Attorneys General representing nearly half of the states and territories of the United States, we ask the Centers for Medicare & Medicaid Services ("CMS") to reconsider the requirements for Coverage with Evidence Development ("CED") for Food and Drug Administration ("FDA")-approved monoclonal antibodies ("mAbs") directed against amyloid for the treatment of Alzheimer's disease. Specifically, we ask that CMS provide full and unrestricted Medicare coverage for FDA-approved Alzheimer's treatments, consistent with its decades-long practice of covering FDA-approved prescription drugs for Medicare beneficiaries. This coverage will ensure that all Americans benefit from treatments that the FDA has concluded are "important advancement[s] in the ongoing fight to effectively treat Alzheimer's disease."[1]

Today, more than six million Americans are living with Alzheimer's disease, adversely impacting their families, our medical communities, and our economy. Given the progressive nature of this terminal disease, we ask you to ensure all patients have appropriate access to FDA-approved treatments.

As you likely know, Alzheimer's disease is a progressive brain disorder that damages and eventually destroys brain cells, leading to a loss of memory, thinking, and other brain

[1] Press Release, Federal Drug Administration, *FDA Grants Accelerated Approval for Alzheimer's Disease Treatment* (Jan. 6, 2023), https://www.fda.gov/news-events/press-announcements/fda-grants-accelerated-approval-alzheimers-disease-treatment.

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functions.[2] By 2050, nearly 13 million Americans are projected to live with Alzheimer's disease. In 2022 alone, Alzheimer's and other dementia cost the nation $321 billion; 64 percent, or $206 billion, was covered by the taxpayer-funded Medicare and Medicaid programs.[3] Unless a treatment to slow, stop, or prevent the disease is approved and accessible to people, by 2050, Alzheimer's is projected to reach a total cost of $1 trillion (in 2022 dollars).[4]

In November 2022, positive results from the Phase 3 trial of lecanemab, an mAb for the treatment of mild cognitive impairment due to early-stage Alzheimer's disease, were reported by the trial's sponsors Eisai and Biogen.[5] The data demonstrated that this mAb therapy slows cognitive and functional decline over 18 months and reduced amyloid, a biological marker of Alzheimer's disease. In a study of nearly 1,800 individuals in the early stages of Alzheimer's, patients who received lecanemab had a reduced rate of cognitive decline, which suggests that lecanemab will give people with Alzheimer's more time to participate in daily life and live independently. While lecanamab was also associated with certain adverse effects, patients and their providers can assess these risks against the potential benefit of many more months of being able to recognize and interact with loved ones. Lecanemab received accelerated approval by the FDA on January 6, 2023,[6] and other mAbs directed against amyloid for the treatment of Alzheimer's disease are currently under FDA review.

As you know, last year CMS evaluated a different mAb treatment and issued a national coverage determination ("NCD") for not just that product, but all future mAb therapies directed at amyloid for the treatment of Alzheimer's disease.[7] Under the current NCD, CMS will only cover mAbs when they are administered through clinical trials or other studies. This decision creates a barrier to care for older Americans, especially individuals living in rural and underserved areas that are unlikely to be served by institutions administering clinical trials. It is an enormous physical and financial burden for Medicare beneficiaries to travel to the few research institutions that host the trials. Patients, families, and caregivers living in rural and underserved areas should have the same opportunity for access to treatment. Unless CMS reconsiders the April 2022 NCD, access to this important therapy for Alzheimer's disease will be extremely limited or nearly nonexistent. Given how quickly Alzheimer's can progress, ensuring that patients across the United States have fair access to potential life-changing treatment is extremely important. Otherwise, only patients wealthy enough to pay for the treatment entirely out-of-pocket, or those who happen to live someplace with an ongoing clinical trial, will have access to these new therapies.

We encourage CMS to reconsider the CED requirements for FDA-approved monoclonal antibodies targeting amyloid for the treatment of Alzheimer's disease. This would provide

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[2] *Id.*
[3] ALZHEIMER'S ASSOCIATION FACTS AND FIGURES, https://www.alz.org/alzheimers-dementia/facts-figures (last visited March 3, 2023).
[4] *Id.*
[5] Van Dyck, Christopher H. et al., *Lecanemab in Early Alzheimer's Disease*, NEW ENGLAND J. MED (Jan. 5, 2023), https://www.nejm.org/doi/10.1056/NEJMoa2212948.
[6] *See* Press Release, Federal Drug Administration, *supra* note 1.
[7] Press Release, Centers for Medicare and Medicaid Services, *CMS Proposes Medicare Policy for Monoclonal Antibodies*.... (Jan. 11, 2023), https://www.cms.gov/newsroom/press-releases/cms-proposes-medicare-coverage-policy-monoclonal-antibodies-directed-against-amyloid-treatment.

Medicare beneficiaries living with mild cognitive impairment due to Alzheimer's disease and early-stage Alzheimer's disease with immediate access to an FDA-approved treatment if the patient and clinician weigh the benefits and risks and decide it is the right treatment plan. Our request reflects that of the patient community and is consistent with a request the Alzheimer's Association submitted to CMS on December 19, 2022,[8] and letters submitted by members of Congress on January 30, 2023,[9] and February 17, 2023.[10] The Alzheimer's Association's request included a letter signed by more than 200 Alzheimer's disease researchers and experts expressing their confidence in the lecanemab data, saying there should be "no barriers" to accessing the drug if it is approved.[11] Perhaps even more importantly, CMS's reconsideration would also ensure that the federal government is addressing this important class of drugs in a consistent and fair manner. Unlike CMS, the Veterans Health Administration is covering the cost of lecanemab when patients meet certain criteria.[12]

We thank you for your leadership on issues important to Americans living with Alzheimer's and other dementia, their caregivers and our Nation. Directing CMS to reconsider its requirements for CED for FDA-approved mAbs may be the most impactful decision of your careers—and certainly the lives of millions of Americans.

Sincerely,

Gentner Drummond
Oklahoma Attorney General

Keith Ellison
Minnesota Attorney General

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[8] Letter from Joanne Pike, President, Alzheimer's Association, to Chiquita Brooks-LaSure, Administrator, Centers for Medicare & Medicaid Services (Dec. 19, 2022), https://alz.org/media/Documents/final-NCD-reconsideration-request.pdf.

[9] Letter from Darin LaHood et. al, Members of United States Congress, to Xavier Becerra, Secretary of U.S. Department of Health and Human Services, and Chiquita Brooks-LaSure, Administrator of Centers for Medicare and Medicaid Services (Jan. 30, 2023), https://portal.alzimpact.org/media/serve/id/63f5385de2f42.

[10] Letter from Susan M. Collins et. al., Senators, United States Senate to Xavier Becerra, Secretary of U.S. Department of Health and Human Services, and Chiquita Brooks-LaSure, Administrator of Centers for Medicare and Medicaid Services (Feb.17, 2023), https://portal.alzimpact.org/media/serve/id/63e18cbda47a2.

[11] Letter from Paul Aisen, MD., et al., to Xavier Becerra, Secretary of U.S. Department of Health and Human Services, and Chiquita Brooks-LaSure, Administrator of Centers for Medicare and Medicaid Services (Dec. 20, 2022), https://www.alz.org/media/Documents/joint-letter-alzheimers-scientists-lecanemab.pdf.

[12] Department of Veterans Affairs, Lecanemab-irmb Criteria for Use, Feb. 2023, https://www.va.gov/formularyadvisor/DOC_PDF/CFU_Lecanemab-irmb_LEQEMBI_CFU.pdf & Spencer Kimball, *Veterans Health Administration Will Cover Alzheimer's Treatment Lequembi, Eisai Says,* CNBC, March 13, 2023, https://www.msn.com/en-us/health/medical/veterans-health-will-cover-alzheimers-treatment-leqembi-for-some-patients/ar-AA18A3Bq.

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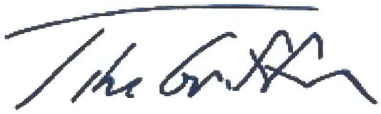

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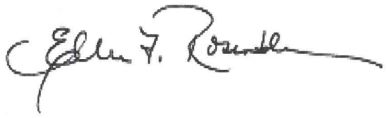

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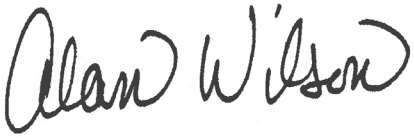

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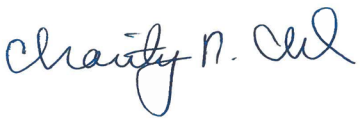

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Chairman BUCHANAN. As someone who has firsthand been devastated in terms of the impact, in terms of Alzheimer's, my own father, and because I am one of the oldest districts in the country, this issue is personal and important to me. In fact, in the effort to push CMS to do their job the right way, I introduced the bipartisan MERIT Act earlier this year to require CMS to consider each new drug on its own rather than as a class.

FDA approval, whether traditional or accelerated, is a full approval and CMS should not be second-guessing the scientists at FDA who granted the approval in the first place.

Additionally, as a former ranking member of the Trade Subcommittee, the TRIPS waiver for COVID vaccine is of particular concern to me, given it is directly undermines the mission of the USTR to vigorously protect Americans' interests abroad, including protecting intellectual property rights.

I have led multiple letters signed by the House—my House colleagues, opposing the TRIPS waiver, because there no reason to continue pursuing such a waiver. It will be only our adversaries to access critical IP that they have no other possession.

The pandemic is over. The public health emergency ends tomorrow. And we have an abundance of vaccine doses that are available for people in the furthest, farthest reaches of the Earth. Unfortunately, the physical infrastructure doesn't exist to get the dosage to those people. Instead of giving away our IP to other countries, we should be helping to teach them how best to update their outdated infrastructure.

If we continue down this path of working against innovators, we will start falling behind countries like China that are willing to do whatever they can to pass us by.

Finally, I want to mention CMMI because, despite having innovation, it is one of the greatest barriers to actual innovation and healthcare. Since 2010, CMMI has released many demonstration projects, some of which were mandatory. But it has not realized savings greater than the amount of the money Congress has spent on the agency.

We all want Medicare and Medicaid to run efficiently, but it is time that Congress reasserts its control over these decisions that works to truly help promote American innovation. There is bipartisan interest in many of these topics we are going to talk about today, and we should find bipartisan solutions to them.

I am in the business personally of trying to get to "yes" with my colleagues. So, I would like to challenge my friends on the other side of the aisle to work with us on a way to unleash American innovation.

We all want America to lead the world in medical innovation. And we want America to have access to the newest, best groundbreaking treatments as soon as possible. I hope we can leave this hearing today with a renewed sense of bipartisanship and willingness to work together on policies that protect and enhance innovation.

I am pleased to recognize the gentleman from Texas, Mr. Doggett, for his opening statement.

Mr. DOGGETT. Well, thank you very much, Mr. Chairman.

And I certainly share those objectives with you, and I want to sincerely thank you personally for reaching out to me last week regarding the CMS demonstration project. I think that you are known for seeking bipartisan action, and I hope that we can do that as much as possible in this committee.

Unfortunately, on this first hearing regarding payment for drugs subject to accelerated approval and related issues, I do have some significant policy differences with you regarding how to assure access to innovative new drugs without paying monopoly prices.

Twenty years ago, in this room the Medicare prescription drug program was narrowly forced through the Ways and Means Committee, and then it took an almost all-night session and a lot of arm twisting to get enough Republicans to vote for it to pass it in the House and make it law.

With one notable line in that very lengthy bill, Big Pharma ensured it would retain monopoly power and the ability to charge the very highest prices in the world through a complete prohibition against any negotiation over drug prices by Medicare.

Finally, last year Democrats provided a very narrow carve-out to eventually allow negotiation on a very small number of drugs that offers no hope of lower prices to most Americans. So extremely narrow and restrictive was that carve-out that the financial services firm Raymond James said, quote, Pharma's CEOs are likely popping champagne and smoking cigars, end quote.

Yet unwilling to yield even this smallest sliver of monopoly power, Big Pharma promotes scare tactics that insist we cannot have both reasonable prices and essential innovation.

All of us want to encourage cures and treatments for dreaded diseases long before we or a loved one face a troubling diagnosis. Despite its overly generous tax incentive, its taxpayer-funded research, its monopoly profits, Big Pharma, I believe, has actually been doing far, far too little to secure the type of new medications which we all would like to see.

Worried about a competitor with a better idea, monopolies and oligopolies are not known in any industry for being particularly innovative. Over a decade, 78 percent of new drug patents were not for new cures that we need but were small modifications to existing drugs designed simply to extend monopoly power and monopoly prices.

Among the ten 6 top-selling drugs in this country, 66 percent of the patent applications were filed after FDA approval and an average of 74 patents were granted on each drug. And while there are pathways intended to get innovative drugs and devices to market quickly, the FDA's accelerated approval program and the Medical Device Breakthrough Program, I believe, are deeply flawed. In fact, the data is out there. In about 40 percent of all drugs that are granted accelerated approval fail to complete their confirmatory clinical trials after coming to market as is required by law. Those trials are critical to ensuring drugs have a clinical benefit and meet all safety requirements.

Similarly, in its first 3 years, the FDA granted a remarkable 222 devices as breakthrough designations, despite poorly designed studies that did not demonstrate real benefit on many of these devices and some safety studies that showed substantial risk to patients.

I have long been concerned with medical device safety, and it is apparent that the FDA has increasingly become a captive of those that it is charged with regulating. It has not been forceful enough or creative enough, early enough to protect patients' safety.

At a bare minimum, physicians ought to be required to report device safety issues and the FDA ought to provide unique device identification numbers, as I have urged it to do in the past, so we can remove faulty devices from the market very quickly.

Despite these many significant concerns, our Republican colleagues would have taxpayers pay monopoly prices for questionable drugs and devices. Such thinking has fueled our flawed patent system and reimbursement system which actually disincentivizes innovation. With a government-granted monopoly and guaranteed Medicare coverage, it is much easier to tweak and repackage existing drugs rather than to develop the new cures that we need.

While Big Pharma may claim the billions that they earn on these drugs are devoted to research and development and new cures, the reality is that manufacturers are spending more on marketing and propaganda than R&D, more on stock buybacks and dividends than R&D.

The real angel investors in research and development for new cures in in America are none other than American taxpayers. Over the last decade every single newly approved drug relied on taxpayer-funded research, and taxpayers funded the majority of total research and development spending.

U.S. taxpayers remain the largest source of R&D funding in the entire world. Yet American patients continue to face the very highest prices, forcing them to ration or skip necessary medications all together.

Finally, Mr. Chairman, we have a responsibility to ensure that patients come first and that it is their health and their livelihoods, not drug prices, which must be non-negotiable. Unaffordability and inaccessibility are not the unavoidable side effects of innovation. They are the result of unrestrained monopoly power.

I thank each of our witnesses with differing views for joining us today to examine that monopoly power. And I hope that moving forward we will not once again yield to the power of Big Pharma, instead, move to advance reasonable solutions that promote competition and achieve lower prices.

Thank you so much.

Chairman BUCHANAN. Thank you, Mr. Doggett.

I am pleased to recognize the chairman of Ways and Means Committee, Chairman Smith, for his opening statement.

Chairman SMITH. Chairman Buchanan, Ranking Member Doggett, it is pleasure to be with you once again.

And I want to thank all the witnesses for being here.

I want to thank you for the opportunity to share a few thoughts on how current White House policies are threatening medical innovation and patient access to care.

Across America, millions of patients are anxiously hoping for new breakthrough cures and devices that will improve their quality of life or even give them more years with their loved once.

The scientists that research these cures, they rely on Congress to craft policies that support innovation. Patients deserve peace of

mind that these therapies will be available to them when approved. Poor policymaking through both Congress and executive action, however, could have a chilling effect on the development of and access to the next drug, next device, or treatment. Unfortunately, that is what we are seeing today with decisions made by agencies such as CMS.

Broadly restricting coverage for Alzheimer's treatments, the first approved in nearly 20 years, is a devastating blow to the patients and caregivers relying on new innovations. Importantly, these restrictions are disproportionately felt by those living in rural America who don't have access to qualifying clinical trials.

I applaud subcommittee Chairman Buchanan's work on this issue, and I hope that in the light of continued positive data, such as the study released last week, CMS will reconsider this decision.

I also have concerns that the CMS Innovation Center's proposed policy to devalue accelerated approved drugs will slow access to breakthrough innovation such as many cancer therapies.

Congress shares much of the blame, too. The Inflation Reduction Act established a new drug price control scheme. We all want to make medications more affordable, but making Washington the price setter will only lead to fewer cures and less access to them. Experts warn that price controls will lead to 135 fewer cures and discourage the development of generic and biosimilar competition, a far more patient friendly approach for lowering drug prices.

Patients relying on breakthrough medical devices are also facing uncertainty after a Trump-era innovative coverage rule was repealed by the administration. I know members on both sides of the aisle will be closely watching for a meaningful replacement.

Lastly, the Biden administration's decision to waive IP protections for vaccines and potentially expand the therapeutics and diagnostics is setting a very dangerous precedent and opening the door for countries like China to steal our innovation.

Right now, there are 322 different medicines being developed to treat cancer, 192 for rare genetic diseases, 83 for Alzheimer's disease, and hundreds of others. Patients cannot afford Washington's anti-innovation policies. I look forward to working with all my Ways and Means colleagues, both Republican and Democrat, to promote access to these future cures.

And I yield back, Mr. Chairman.

Chairman BUCHANAN. Thank you.

I now want to introduce the witnesses.

Mr. Gonzales, who is a National Early-Stage Advisor for the Alzheimer's Association, I personally want to thank you for your courage and taking the time to be with us today.

Okon is the Executive Director of Community Oncology Alliance.

Dr. La—whatever—I am sorry—is the Professor of the Pharmaceutical Economic and Public Policy at the University of South—Southern California.

Mr. Makower, Dr. Makower, is the Director of Stanford University Byers Center for Biodesign.

Mr. Kesselheim is a Professor of Medicine at Harvard Medical School.

The committee has received your written statements and will be a part of the formal record.

Mr. Gonzales, you are recognized. For 5 minutes.

STATEMENT OF TONY GONZALES, NATIONAL EARLY-STAGE ADVISOR, ALZHEIMERS ASSOCIATION, ACTING PROGRAM EXECUTIVE DIRECTOR, U.S. DEPARTMENT OF VETERANS AFFAIRS

Mr. GONZALES. Chairman Buchanan, Ranking Member Doggett, and members of the subcommittee, thank you for the opportunity to testify before you today and share my story about what access to innovation means to me.

It means more time with my wife, my kids, and my grandson. My name is Tony Gonzales. I am 48 years old, from Santa Maria, California. And last year I was diagnosed with mild cognitive impairment. I know this disease can destroy careers, relationships, and every day it robs me more and more of my memories.

A few years ago, my family and I noticed the first few signs that something was wrong. Then one day I got lost coming home from work. I was in my hometown. I was in my car, on a road that I had driven thousands of times. And I had no idea where I was. I had no idea where I had been or where I was going. All I knew was I needed to call my wife for help.

I spent the next couple of years, couple of years searching for an answer. Two years after my initial symptoms, I finally received a diagnosis: Mild cognitive impairment. When I was diagnosed, it would have given me so much hope to have the opportunity to access treatments that can give me more time. I would like to have the chance to make the decision if the treatments are right for me and my family instead of Medicare making that decision for me.

I became a member of the Alzheimer's Association Early-Stage Advisory Group to help raise awareness of this disease, especially for those people who are under 65 and not typically the face of the Alzheimer's disease.

When I am in a meeting and someone tells me they have never met someone with dementia, I say to them, well, now you have. You see, I want people to see the impact of this disease that it has on real people and real families in America.

The incredible bipartisan support for increases in Alzheimer's research funding at the NIH over the years are starting to pay off. In the last year and a half, we have seen two treatments get FDA approval and another one that we will submit to the FDA soon. These treatments have the able to change the course of this disease. The fact that they exist and are approved by the FDA and yet people like me cannot access them because of Medicare is frustrating and humiliating.

As many of you know, CMS is restricting access to these breakthrough therapies by creating additional hoops to jump through. This creates even more of a barrier to care for people living in rural and underserved communities like those in my hometown.

Medicare is treating people with MCI and Alzheimer's differently when they apply this restriction to an entire class of drugs, current and future. This action has a ripple effect as well. Private insurance and health systems follow Medicare's lead. If Medicare won't cover, chances are that other insurances won't either and health

systems won't make it available, thus, taking more time away from people including for me and others who aren't on Medicare.

This is an urgent issue. The Alzheimer's Association estimates more than 2,000 individuals aged 65 or older transition per day outside eligibility for these treatments. As of today, that number is approximately 248,000 people who have progressed past the point of eligibility since approval in January. Keep in mind this number doesn't even include people like me who are under 65.

Earlier this year nearly 100 bipartisan members of Congress, including many on this subcommittee, sent letters to the administration, raising concerns with CMS' coverage policies around these FDA-approved Alzheimer's treatment. Thank you.

As recently as last week, another company announced positive top-line results for their new Alzheimer's treatment. This innovation will mean nothing without access. CMS must immediately reconsider. They must look at the clear evidence now before them. And when they do, I trust they will acknowledge that these treatments are absolutely reasonable and necessary for people like me with a terrible progressive disease and no other treatment options.

Refusing to take another look at NCD further expands the divide between CMS and the Alzheimer's community. We are losing time, and this is unacceptable. More time is more than just a number of months or years that I may gain from such treatments.

I wake up every day, hoping to know who I am, who my wife is, who my kids are. When I wake up and I realize that it is a win. So, I live for today. I want more time to be with my grandson, Sandy, take him to the park, and to be able to do that on my own. I don't drive anymore, but I can still hang out with him and spend time with him.

You see, when you get a death diagnosis, it becomes very clear to you having more time means everything to me. It would allow me to walk my daughter down the aisle, meet another grandchild. It gives me another chance at living my best every single day, time to live again, time to hope again.

It truly is an honor to speak with you today and share my story. I hope it inspires all of you to continue your work, urging CMS to treat those with Alzheimer's fairly.

And, lastly, I hope you remember to live for today. Love those around you. I wish you all good brain health, and I look forward to answering any questions you may have.

Thank you.

[The statement of Mr. Gonzales follows:]

Tony Gonzales
Alzheimer's Association Early-Stage Advisor

Written Testimony
United States House Committee on Ways and Means, Health Subcommittee Hearing on "Examining Policies that Inhibit Innovation and Patient Access"

May 10, 2023

Chairman Buchanan, Ranking Member Doggett and members of the Subcommittee, thank you for the opportunity to testify before you today to share my story about what access to innovative breakthroughs means to me: more time with my wife, kids and grandkids. My name is Tony Gonzales. I am from Santa Maria, California, I am 48 years old, and last year I was diagnosed with mild cognitive impairment. I know this disease can destroy careers, relationships, and every day it robs me of more and more of my memories. But I stand here today to tell you, this disease does not define who I am. I am living my best life with MCI, and using every opportunity to share my story as my way of fighting back.

A few years ago, my family and I noticed the first few signs that something was wrong. I found myself having difficulty with my work as a development manager for the American Cancer Society. Others were noticing too. My family, friends, coworkers, clients, all were making excuses for my actions. Then one day I got lost coming home from work. I was in my hometown, I was in my car, on a road I had driven thousands of times - and I had no idea where I was, where I had been or where I was going. All I knew was that I needed to call my wife for help.

Another turning point was when I was at home with my family. I came in from outside and saw an open package on the kitchen table addressed to me. I was excited, because I had been waiting for some special American Flag socks that I was going to wear for my son's wedding. I reached in and pulled out my new pair of fun socks, then I excitedly showed them to my family. They looked at me like my head had just blown off – apparently just five minutes before, I had done the same exact thing. At the time, we thought my forgetfulness was due to being stressed and tired.

I spent the next couple of years bouncing from my general practitioner to UCLA, then finally to UCSF. I realize now that my experience was much more complicated due to my age: I was fighting a stigma that dementia only affects people over 65. Despite numerous MRIs, a spinal tap, sleep studies, two neuro psych exams, and a CT scan, I was told by medical professionals that I was too young to have dementia, and instead my symptoms were attributed to sleep apnea.

Following that incorrect diagnosis, I made an effort to get healthier and to eliminate what I had been told was the problem, I lost over 180 pounds. Unfortunately, my issues persisted after the

weight loss, and I had to go back to seeking an accurate diagnosis. Two years after my initial symptoms, I finally received one: mild cognitive impairment (MCI).

I sometimes think about how this process could have been smoother, or gotten me to the right information without losing so much time. For example, I didn't know at the time of my early symptoms that my Grandmother had passed from complications due to dementia and there were other family members who showed signs but never were addressed. The Hispanic culture I was raised in is not one that understands this disease and it was always dealt with shame and secrecy.

I live in a beautiful part of California. My hometown of Santa Maria sits in between Los Angeles and San Francisco. But in order for me to get the quality of care needed, the newest tests and be a part of the latest study we have to travel to one of these two cities. Living with this disease makes it very difficult to travel and then you throw the COVID years on top of it…I can tell you…it has been a very difficult couple of years for my wife and me.

I became a member of the Alzheimer's Association Early Stage Advisory Group to help raise awareness of this disease - especially for people who are under the age of 65 and not typically the "face" of Alzheimer's disease. When I'm in a meeting and someone tells me they've never met someone living with Alzheimer's disease, I say to them "now you have." I want people to see the impact this disease has on real people and families.

I, like many others who are diagnosed with MCI or dementia, was offered little direction about what to do, other than get our affairs in order and to pretty much go live a life of forgetting. It would give us so much hope to have the opportunity to access treatments that can give us more time - I'd like the chance to make the decision if the treatments are right for me and my family instead of the Centers for Medicare & Medicaid Services (CMS) just saying no.

Over the last few years, there has been incredible progress in the Alzheimer's research space thanks to the bipartisan support in Congress. The historic increases in funding at the NIH are starting to pay off: over the last year and a half, we've seen two treatments get approved by the Food and Drug Administration (FDA) and another one that will submit to the FDA later this year! These treatments have the ability to change the course of this disease. The fact that these treatments exist and are approved by the FDA and yet people like me cannot access them because Medicare refuses to cover them is frustrating and humiliating.

The benefits of these treatments will only be realized if patients have access. The treatments in this class give people more time at or near their full abilities to participate in daily life, remain independent and make future health care decisions. Treatments that deliver these benefits are as valuable as treatments that extend the lives of those with other terminal diseases.

As many of you know, under the national coverage determination (NCD) currently in place, CMS would only cover monoclonal antibodies directed against amyloid for the treatment of Alzheimer's disease (mAbs) through Coverage with Evidence Development (CED). This means

for the accelerated approval pathway treatments individuals must be enrolled in randomized clinical trials (none of which exist because they have been completed) and for treatments approved through the traditional approval pathway treatments patients must be enrolled in prospective comparative studies. This decision creates even more of a barrier to care for Americans, especially individuals living in rural and underserved areas - just like my hometown. Restricting access as CMS is doing - makes it nearly impossible for many people to even have the opportunity to talk with their doctors about these treatment options.

Additionally, this NCD applies to drugs approved as of April 2022 AND all future drugs in this class. Medicare has never done that before - they've never pre-judged treatments by restricting access before anything is even approved by the FDA. CMS has repeatedly pledged to move quickly to modify the NCD if warranted by new evidence — which they have failed to do to date. They've been presented with a myriad of new evidence including peer reviewed journal articles discussing the treatments and a letter from over 200 clinicians and researchers about the efficacy of the treatment. Medicare refusing to cover FDA-approved Alzheimer's treatments has a ripple effect as well - private insurance follows Medicare's lead. If Medicare won't cover, chances are that other insurance won't cover either. Thus taking more time away from people including those who aren't on Medicare.

Based on projections from the Alzheimer's Association, more than 2,000 individuals aged 65 or older transition per day from mild dementia due to AD to moderate dementia due to AD, and therefore outside the anticipated indicated population of these treatments. Given the progressive nature of this terminal disease and the absence of treatment alternatives, delays would deny these Medicare beneficiaries the opportunity to benefit from this treatment. As of May 10, that number is approximately 248,000 people who have progressed past the point of eligibility for Leqembi since it was first approved on January 6. Keep in mind, this number doesn't include people like me who are under 65 and eligible for this treatment.

In March 2023, the U.S. Veterans Health Administration announced that it would cover the FDA-approved Leqembi (le-kem-bee). This decision will allow veterans living with mild cognitive impairment and early stage Alzheimer's disease to access medically necessary and beneficial treatments. Each day matters when you're living with Alzheimer's, this is a great opportunity for our veterans. The coverage decision made by the VHA is in sharp contrast to CMS which continues to block medically necessary and beneficial treatments to Medicare beneficiaries.

Earlier this year nearly 100 bipartisan members of Congress - in both the House and Senate - including many on this Committee - sent letters to CMS and HHS, raising concerns with CMS's coverage policies around these FDA-approved Alzheimer's treatments. As recently as last week, another company announced positive top line results for their anti-amyloid treatment, donanemab. Thankfully they and others are continuing to research this area even though Medicare refuses to give access to people like me.

This moment, in addition to the new data announced this month regarding donanemab, will provide CMS with a new opportunity to initiate a reconsideration. I am not asking for any

commitments to the outcome of such a reconsideration process. It is the initiation of the process itself that is crucial. Declining to reopen the NCD upon traditional approval would further escalate the stark and expanding divide between CMS on one hand and the FDA and VA on the other, as well as between CMS and the Alzheimer's community.

Americans living with Alzheimer's disease are entitled to FDA-approved therapies, just as are people with conditions like cancer, heart disease and HIV/AIDS. They deserve the opportunity to assess in partnership with their health care provider if an FDA-approved treatment is right for them. People are losing the opportunity, they're losing days, weeks, months, and memories. They're losing time. And it is unacceptable.

More time is more than just the number of months or years. I wake up every day hoping to know who I am, who my wife is, who my kids are. If I do that, it's a win. I live for today. I want more time to be with my grandson. Take him to the park - and be able to do that on my own. I can't drive anymore, but I can still hang out with him and spend time with him. When you get a death diagnosis, life really becomes clear to you. Having more time means everything to me - it would allow me to walk my daughter down the aisle, meet another grandchild, it gives me another chance at living my best every single day. Time to live again. Time to hope again.

I am honored to speak with you today and share my story. I hope it inspires you to continue your work in urging CMS to treat those with Alzheimer's fairly. And lastly, I hope you remember to live for today, love those around you, and I wish you good brain health. Thank you!

Chairman BUCHANAN. Thank you. That was very inspiring.
Mr. Okon, you are recognized.

STATEMENT OF TED OKON, EXECUTIVE DIRECTOR, COMMUNITY ONCOLOGY ALLIANCE

Mr. OKON. Chairman Buchanan, Ranking Member Doggett, and members of the Health Subcommittee, I am the Executive Director of the Community Oncology Alliance, a nonprofit organization dedicated to cancer patients and their independent oncology providers.

My wife, Susan, practiced as an oncology nurse for 10 years. And we have had family and friends with cancer living with it and dying from the disease. I want to make it very clear that my overriding goal is to ensure that every American with cancer, regardless of the demographic, financial, or other status, has access to the highest quality, most affordable cancer care close to home.

I also add that both my wife and I are Medicare beneficiaries. I am alarmed at the rising cost of cancer drugs. Clearly, drug companies have primary responsibility because they determine the launch and subsequent list prices of prescription drugs.

However, our country has a bizarre, convoluted health system where the price of drugs and the cost of patients are two very different and often disjointed things with drug costs to Americans fueled by intermediaries like PBMs and so-called nonprofit 340B hospitals.

As Dr. Mark Fendrick of the University of Michigan and creator of Value-Based Insurance Design has often lectured me, when Americans talk about the high price of drugs, they are really referring to the high cost to them of what they pay out of pocket.

Our Nation has made great strides in cancer treatment, especially with the increasing availability of immunotherapies. As I was preparing my testimony, an oncologist called me about a 35-year-old woman who had recurring gastrointestinal, esophageal, and brain cancer since she was 18 years old. Six months ago, she developed a cancer in the small bowel that spread to her other organs. She was put on a treatment regimen including immunotherapy. After 4 months, she is in complete remission.

My wife calls these immunotherapies nothing short of revolutionary, as she has seen firsthand in administering them.

We must not only ensure that all Americans with cancer have access to these innovative, cutting-edge therapies but also that we foster their development. That is why I am concerned that our already overregulated Medicare system is getting even more regulated by the government.

As I explain in more depth in my written testimony, there is a fundamental lack of understanding of the life cycle of cancer drugs, how uses in different types of cancer and sub-cancers are researched and developed over time after a drug is first approved by the FDA, sometimes for a single indication.

Certainly, drug companies won't stop researching new innovative drugs due to the IRA because that is their lifeblood. However, the threat of government negotiations will be a huge obstacle to research and developing new using in different types of cancer over time.

Ask yourself if you would invest research funds in new uses of cancer drugs with the looming threat of price cutting by government negotiation. How CMS figures out how to negotiate the single price for a drug with multiple indications, values, and therapeutic competition is nearly impossible.

This also is truly alarming, especially since I believe that the threat of drug price negotiations will simply fuel drug launch prices higher. The unintended consequence of the law meant to lower drug prices may actually increase them.

Additionally, both the IRA and the President's recent executive order using the CMS Innovation Center to lower drug prices in certain situations of accelerated drug approvals uses physicians as variable hostages between the government and drug companies. Physicians will feel the brunt of lower drug reimbursement and an operational nightmare of dual reimbursement systems in the case of the IRA. Poor public policy, dare I utter the word sequestration, and regulation have already caused massive consolidation of independent physicians and expensive mega-health systems, costing patients, Medicare, employees, and taxpayers more for drugs and medical care.

And let me explain that the CMS Innovation Center, rather than being a testing center to innovate payment reform, has become a vehicle for now three administrations to attempt to lower drug prices by end-running the Congress in existing law. This was not the intent of Congress in creating the CMS Innovation Center.

I fear we are heading down a dark path in this country where innovation is stifled, consolidation fuels increasing healthcare costs, and America have less access to the medical providers of their choice.

Like with cancer treatment, we just can't treat the symptoms of our healthcare system by Band-Aiding it with regulation upon regulation. We need to treat the underlying disease, which includes runaway hospital consolidation, profiteering middlemen, and obstacles to fostering true drugs competition. Every American with cancer and other serious diseases is counting on us.

Thank you for the opportunity to testify, and I will answer any questions.

[The statement of Mr. Okon follows:]

Written Testimony on Hearing:

Examining Policies that Inhibit Innovation and Patient Access

May 10, 2023

United States House of Representatives

Health Subcommittee on Ways & Means

Ted Okon

Executive Director, Community Oncology Alliance

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Submitted May 8, 2023

The opinions expressed in this testimony are mine and were prepared by me.

Chairman Buchanan, Ranking Member Doggett, and members of the Health Subcommittee of the House Committee on Ways & Means, I appreciate the opportunity to submit this written testimony and to be asked to appear as a witness at this extremely important hearing. I frame this written testimony, opening statement, and answers to questions from the perspective of cancer treatment.

I am the Executive Director of the Community Oncology Alliance (COA), an organization dedicated to advocating for the complex care and access needs of patients with cancer and the community oncology practices that serve them. COA is the only non-profit organization in the United States dedicated solely to independent community oncology practices, which serve the majority of Americans receiving treatment for cancer. Since its grassroots founding 20 years ago, COA's mission has been to ensure that patients with cancer receive quality, affordable, and accessible cancer care in their own communities where they live and work, regardless of their racial, ethnic, demographic, or socioeconomic status.

My wife Susan practiced as a certified oncology nurse for 10 years, administering cancer therapies to patients with solid tumors. We have had family and friends with cancer, living with it and dying from the disease. **I want to make it very clear that my overriding goal is to ensure that every American with cancer, regardless of demographic, financial, or any other status, has access to the highest quality, most affordable cancer care close to home.** I should also add that my wife and I are both Medicare beneficiaries.

I am alarmed at what is happening right now with cancer care and will happen in years to come as a result of misguided and even destructive public policy. As I write this, oncology practices are dealing with a shortage of mainstay generic cancer drugs. Treatments are being delayed and oncologists are having to make decisions as to how to treat their patients with alternative and, typically, lesser therapies. Unfortunately, delays and denials of cancer drugs are something oncologists deal with daily at the hands of pharmacy benefit managers (PBMs). The top PBMs are an oligopoly with a stranglehold on the nation's prescription drug market. They fuel drug costs for Americans and are pushing independent pharmacists out of business, creating pharmacy "deserts" in rural areas. These PBMs have also merged with the top health insurers to throw "prior authorization" roadblocks, "fail first" step therapy, and other so-called "utilization management" tactics at oncologists to dictate how to treat their patients. Then, the PBMs mandate how, when, and where cancer patients will get their potentially life-saving drugs, often via PBM-owned mail order pharmacies.

I am equally alarmed by the rising cost of cancer drugs. Nothing I write or say defends pharmaceutical companies or lets them off the hook. They have primary responsibility for drug costs because they set the launch and subsequent list prices of prescription drugs. However, as I will explain, our country has a bizarre, convoluted health system where the "price" of drugs and the "cost" to patients are two very different and disjointed things. As Dr. Mark Fendrick of the University of Michigan and creator of value-based insurance design has often lectured me, when Americans talk about the high "price" of drugs, they are really referring to the high "cost" to them – namely, what they pay out-of-pocket. I believe that the pharmaceutical industry has to get more

proactive and creative in how companies approach pricing drugs, especially tying prices to drug effectiveness. At the same time, those in Congress and elsewhere who want to eviscerate the industry are basically signing death warrants for those Americans with cancer and other serious diseases. We all need to work together to arrive at meaningful, real solutions.

Our nation has made great strides in cancer treatment, especially with the increasing availability of immuno-oncology drugs (immunotherapies). As I was preparing this testimony, an oncologist called me about a 35-year-old woman who had recurring gastrointestinal, esophageal, and brain cancers since she was 18 years old. Six months ago, she developed a cancer in the small bowel that was metastatic (i.e., spread to other organs). She was put on a treatment regimen including immunotherapy. After four months, she is in complete remission. My wife calls these therapies nothing short of "revolutionary" as she witnessed over her 10 years as an oncology nurse as immunotherapies became available for treating cancer..

The good news in cancer treatment is that we have more precise diagnostic tools and drugs. Mortality from cancer is decreasing and Americans with the disease are living longer.[1] The bad news is the cost of treating cancer is increasing, especially as new, innovative drugs available have much higher prices. Unfortunately, both underlying drug prices and out-of-pocket costs are the result in part due to misguided public policy that has piled bad policy and regulation upon bad policy and regulation. Rather than having a healthy economic market for drugs that fosters competition, which in turn motivates innovation and controls prices, we have a highly regulated market with forced price controls and mandatory discounts that leads to shortages, stifles innovation, and actually increases costs for patients and taxpayers.

Let me now discuss the impact that new public policy and regulation will have on cancer treatment, as well as patient cost and access.

Inflation Reduction Act (IRA)

There are certain provisions of the IRA that are positive in helping lower the out-of-pocket costs of prescription drugs for Medicare beneficiaries like me and my wife. However, the provision empowering the government (Medicare) to "negotiate" drug prices is fraught with numerous perils. It is clear that the reality of how groundbreaking new pharmaceuticals are developed, especially cancer drugs, is simply not understood.

A cancer drug is typically indicated for not just one cancer type (e.g., breast cancer) but for a variety of cancers, even subsets within cancers (metastatic breast cancer, HER2-, etc.). For a variety of reasons, what typically happens is a new cancer drug is introduced with one or maybe two indications. Then, as the drug is approved and used to treat cancer, a pharmaceutical company

[1] "Risk of Dying from Cancer Continues to Drop at an Accelerated Pace", American Cancer Society, January 12, 2022.

will conduct research and development on other indications (e.g., other cancers, expanded indications within a cancer, different drug combinations, etc.) for the drug.

As one example, the Food & Drug Administration (FDA) approved a small-molecule oral targeted therapy, Imbruvica (ibrutinib), for mantle cell lymphoma on November 13, 2013. Subsequent to that, the FDA approved the drug 11 times since 2013 for other indications and drug combinations, with the most recent approval on August 24, 2022, for pediatric patients with chronic graft-versus-host disease. It is the first and only drug in its class to be approved for the treatment of children with this disease.

Imbruvica is likely to be one of the initial Medicare Part D drugs targeted for "negotiation." I call your attention to a current article in Health Affairs[2] examining the complexity of "negotiating" the price of Imbruvica for just one of its indications. Understand that there will only be one "negotiated" price of the drug, yet the drug, as with most other cancer drugs, has varying relative value in different types of cancer. How will this reasonably be accounted for in one "negotiated" price of the drug?

Additionally, with a limit on the years of exclusivity before a drug with no generic or biosimilar competition gets "negotiated," there will likely be two consequences. First, drug manufacturers will increase the launch prices of their drugs knowing that in time, if a drug has no competition, its price will be "negotiated" downward. This means that under the IRA, all patients, not just Medicare beneficiaries benefiting from the lower "negotiated" price, will be paying more for their drugs because, ironically, *launch prices will increase*. Second, and especially disconcerting, is that drug companies will not invest in expanded indication research anytime near when a drug will be the target of government "negotiation." This will most certainly limit research and development for new indications and uses of a drug. For example, in the case of Imbruvica, it is highly unlikely that the pediatric breakthrough use of the drug would have been invested in and investigated. And it's important to note that many indications in pediatric cancers are developed after cancer drugs are first approved for adult cancers.

Please understand, I am not justifying or defending any pricing decisions relative to Imbruvica. I am simply using it as a case study of how a drug's lifecycle is complicated and as a convenient example to consider given the just-released Health Affairs article.

With Medicare Part B drugs subject to drug price "negotiations," it is even more complex than with Part D. These are drugs that have to be administered (infused or otherwise) under direct physician supervision. As such, the "negotiated" price of the targeted Medicare Part B drug will create a second Medicare reimbursement rate – the "negotiated" "maximum fair price" (MFP) rate for providers, in addition to the current reimbursement rate based on "average sales price" (ASP). Not only will this create an operational nightmare of having two reimbursement rates for community oncology practices and other independent physician Part B providers, but it will

[2] "Medicare Price Negotiation: The Example Of Ibrutinib", Health Affairs Forefront, May 2, 2023.

drastically reduce the variable component of Medicare reimbursement above the fixed "negotiated" MFP. According to an analysis by Avalere Health[3] this variable add-on reimbursement would be reduced by 49.5 percent.

As crafted, the IRA puts providers in the middle of the "negotiations" between the government and drug companies. Rather than a rebate provided by the drug company to Medicare for the "negotiated" amount, the IRA creates this second reimbursement rate based on MFP in addition to the ASP-based rate. This is not only an operational challenge to administer but is a drastic payment cut to the "negotiated" drugs. Additionally, the MFP will be included in the calculation of ASP, thus further driving down total reimbursement.

History has clearly documented that repeated and misguided cancer care payment cuts cause independent cancer care providers to close or merge with expensive hospital systems.[4] When independent practices close, medical care almost always shifts to much more expensive hospitals. Furthermore, access to care is threatened as cancer clinics and other specialty facilities simply close, especially in rural areas, due to financial pressures. Ironically, this results in higher out-of-pocket costs for patients and in access issues, especially in rural areas.

As with Part D drugs, given the lifecycle development of indications of Part B drugs over time, it is especially concerning how innovation in new cancer drugs and new indications will be impacted by misguided and poorly implemented federal public policies, such as the IRA. In effect, this is a grand experiment on the nation's cancer care system but without any safeguards or small-scale demonstrations, or pilots to guard against unintended consequences. Think about this as akin to developing a drug. Before any widescale clinical research is conducted, limited trials are conducted to assess the safety and basic effectiveness of the drug. That is not happening here.

I now call the Committee's attention to examples of how new, innovative cancer therapies are producing remarkable results.

A 75-year-old female developed stage IV breast cancer in 2018 that was ER/PR negative and HER2+. She received appropriate indicated treatment for her type of cancer and achieved near complete remission in 2019. She did well until the summer of 2022 when she developed a right-sided neck lesion. Her biopsy confirmed recurrent disease with metastases to bone, lungs, and lymph nodes. She received the same treatment as before, and her follow-up scans in April revealed disease progression. She had a large node (the size of a golf ball) on the right side of her neck. She was depressed and planning to give up. Her oncology team decided to switch her over to an innovative new therapy. After just one dose, she had a significant reduction in the size of the mass in her neck.

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[3] "IRA Medicare Part B Negotiation Shifts Financial Risk to Physicians", Avalere Insights & Analysis, November 29, 2022.

[4] "2020 Community Oncology Alliance Practice Impact Report", Community Oncology Alliance, April 24, 2020.

A 40-year-old teacher with metastatic breast cancer is on immunotherapy (in conjunction with chemotherapy) and now has no evidence of disease for more than two years. She continues to teach, which is remarkable because her triple-negative breast cancer has an average survival rate of just one year.

A 91-year-old with melanoma that had spread to his liver and brain underwent surgery, radiation therapy, and an immunotherapy that was granted accelerated approval for exactly his disease scenario less than a year after FDA approval. That patient is former United States President Jimmy Carter.

I can go on and on about the 40-year-old who presented with a near terminal diagnosis at age 31 but, after a year of immunotherapy nine years ago is alive and working. Or the 55-year-old now eight years from an initial presentation of stage IV melanoma with metastatic disease to the liver and brain, who is in remission after two years of immunotherapy. This is the innovation we must protect and foster.

In relation to biosimilars, the IRA does increase Medicare reimbursement for biosimilars. However, it is important to understand that according to a recent research paper in Health Affairs,[5] biosimilar adoption in 340B hospitals is lower and use of more expensive biologic drugs is higher. According to the investigators, *"Our findings suggest that the* [340B] *program inhibited biosimilar uptake, possibly as a result of financial incentives making reference drugs more profitable than biosimilar medications."* This certainly jeopardizes the continued development of a healthy biosimilar market, which has so much promise in bringing down the prices of expensive biologics.

The Accelerating Clinical Evidence Model

Following President Biden's *Executive Order on Lowering Prescription Drug Costs for Americans*[6], the Health and Human Services (HHS) Secretary issued a report[7] directing the CMS Innovation Center (CMMI) to test models relating to the Executive Order. One such model is the *Accelerating Clinical Evidence Model*, which *"would adjust Medicare Part B payment amounts for Accelerated Approval Program (AAP) drugs to give manufacturers an incentive to expedite and complete confirmatory clinical trials"* per the HHS report. However, the report goes on to say that, *"Although drugs with multiple indications make up a large portion of accelerated approvals, CMS Part B fee-for-service drug payments are not tied to specific indications, making a variable, indication-based pricing scheme difficult to implement."*

As background, the AAP is extremely important to producing new, innovative treatments for many types of cancer and to expediting their availability to cancer patients. However, as with the IRA,

[5] "The Role Of Financial Incentives In Biosimilar Uptake In Medicare: Evidence From The 340B Program", Health Affairs, May 2023.
[6] "Executive Order on Lowering Prescription Drug Costs for Americans", White House, October 14, 2022.
[7] "A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans", HHS, February 14, 2023.

there are two problematic themes in this proposed *Accelerating Clinical Evidence Model*. First, HHS acknowledges that drugs with multiple indications, which is the case with most cancer therapies, make up a large percentage of accelerated approvals. What this means is that because Part B drug reimbursement is not tied to specific indications, HHS could lower reimbursement for a drug because clinical trials are lagging *for just one indication*. Second, as with the IRA, this model puts providers in the middle of the government and drug manufacturers. In this case, providers are "hostages" of sort, being used as an "incentive" for drug manufacturers to timely proceed with clinical trials related to accelerated approval drugs. This is yet another pressure point that will simply drive independent oncology practices into the arms of more expensive health systems, typically those making money off of 340B drugs. Additionally, this will likely force drug manufacturers to rethink and limit research into expanding a drug's indications. Coupled with the IRA, this is a chilling prognosis for the impact on new cancer therapies and indications.

I also want to comment on the use of CMMI to essentially change Medicare drug reimbursement without new laws from Congress. The prior two administrations attempted to use CMMI three times to lower Medicare drug reimbursement for the vast majority of the country. Let me state for the record that I was a big supporter of CMMI as a means of bringing innovation to CMS in "testing" models in a contained phase one pilot before rolling the model out in phase two nationally or at least to a larger population after models have demonstrated success without negatively impacting patients. That was certainly the intent of Congress and the letter of the law in crafting CMMI. However, unfortunately, CMMI has become a vehicle for the executive branch to end-run Congress in order to change reimbursement on a large scale without any phase one limited testing. That is not the law and not the intent of Congress in crafting CMMI.

Generic Drug Shortages

The current shortages of key generic mainstay cancer drugs include cisplatin, carboplatin, methotrexate, BCG, and even sterile water. Drug shortages are nothing new as I testified to Congress over 11 years ago.[8] The current shortages can be blamed in part on being exacerbated by COVID-related supply chain issues and historic inflation, but the root cause is the same: financial. Why is there less financial incentive for drug makers to manufacture generic drugs? Simply because the mandatory discounts (340B) and rebates (PBMs), where applicable, make these products a financial loser. This ties right back to the concept of government intervention in driving down prices – essentially, price fixing – that will certainly make drug manufacturers more selective in what drugs and indications they research and develop. And as I write this testimony, I am hearing from community oncology practices across the country that are running out of these essential drugs and will be forced to make very difficult treatment decisions. *This is a true crisis!*

[8] "Testimony on: Drug Shortages Crisis to the United States House of Representatives Committee on Oversight and Government Reform Subcommittee on Health Care, District of Columbia, Census, and the National Archives", Ted Okon, Community Oncology Alliance, November 27, 2011.

Conclusion

The United States has way too much bad public policy that is negatively impacting and distorting the prescription drug market and, unfortunately, is now adding more. If Congress is not careful, it will end up putting even greater pressure on independent physicians, forcing them to continue to merge into ever consolidating expensive large hospitals and health systems, most with 340B drug discounts. The result will be that, regardless of how low drug prices are "negotiated" down, Americans will end up with higher out-of-pocket costs for drugs and their overall care. And access to innovative oncology and other specialty providers will be limited, especially in rural areas and made more severe due to health care workforce shortages.

As I have repeatedly stated, drug companies have a major role to play in the underlying pricing of drugs. They need to be more proactive and creative in how drugs are priced. However, that requires regulations and bad public policies impeding value-based and other creative pricing mechanisms are removed. Additionally, to ignore that there are many forces at work – notably, PBMs and 340B hospitals – fueling drug costs higher for Americans is sticking our collective heads in the sand. Fortunately, it is encouraging to see both the Senate and House coming together in a bipartisan spirit to address PBM issues.

It is extremely important that we increase access to mainstay generic drugs and, most importantly, to innovative new cancer therapies. I just heard from an oncologist as I was preparing this testimony about a 74-year-old woman whose lung cancer was so extensive it eroded into her heart, and she almost died. Now, after two years of immunotherapy, with no harsh chemotherapy, she has no sign of cancer and has lived a normal life for over four years, the last two requiring no treatment. Or the case of a 58-year-old man with such extensive lung cancer that he required emergency abdominal surgery because the cancer had spread and caused his bowel to perforate. After immunotherapy alone, he has been in remission for over two years and would never have survived more than a few weeks or months at best without this treatment. This was previously unheard of in cancer treatment.

It is unsettling that the *Coverage with Evidence Development* (CED) program, which is intended to allow drugs approved by the FDA to reach patients sooner, may be turning the corner and being used to limit payments for drugs approved. This is just more regulatory action by the payment agency (CMS) limiting access to new drugs approved by the safety and effectiveness (FDA) agency.

I will conclude by offering some specific summary recommendations for Congress related to innovation, access, and drug costs.

- Congress needs to ensure that new regulation and law does not hinder drug innovation, especially with cancer and other types of therapies that have long lifecycles of new indication approvals.
- Congress needs to remove providers from the middle of government and drug company "negotiations." Rather than create a new reimbursement (MFP) in addition to the current

rate (ASP), the "negotiated" amount should be rebated from the drug maker to Medicare. Rebate mechanisms are already in place in government programs *and the IRA* to do just that.

- Congress needs to put "guardrails" on CMMI so that it tests smaller pilot models which show clear success before larger demonstrations or rollouts. CMMI should be a testing agency, not a vehicle for the executive branch to bypass Congress.
- Congress needs to ensure that CMS does not overstep the boundaries of its mandate versus that of the FDA in using CED to block Medicare beneficiaries like me and my wife from having access to innovative, potentially lifesaving drugs. Congress needs to legislate guardrails to keep CMMI true to is mission and congressional intent.
- Congress needs to understand the complexities of differences between drug "prices" and "costs" and address the intermediaries who profit off of drugs, resulting in the widening of the gap between drug "list" prices and "net" prices.

COA stands ready to work with Congress on these recommendations and others. We want to provide meaningful input on ensuring that drug costs come down for Americans with cancer and other serious diseases, as well as fostering research and availability of innovative new cancer therapies and incentivizing the manufacturing of essential generic drugs.

I appreciate the opportunity to provide this testimony.

Ted Okon
Executive Director
Community Oncology Alliance

Chairman BUCHANAN. Thank you.

Dr. Lakdawalla, you are recognized.

I tried. I tried get a little bit better.

STATEMENT OF DARIUS LAKDAWALLA, PROFESSOR OF PHARMACEUTICAL ECONOMICS AND PUBLIC POLICY, USC LEONARD D. SCHAEFFER CENTER FOR HEALTH POLICY & ECONOMICS

Mr. LAKDAWALLA. I appreciate it. Thank you.

Chairman Buchanan, Ranking Member Doggett, and honorable members of the subcommittee, thank you for the opportunity to testify today about the impact of Federal policy on medical innovation.

My name is Darius Lakdawalla. And I am an economist, a professor at the USC Mann School of Pharmacy and Pharmaceutical Sciences and Price School of Public Policy, and the Director of Research at the Schaeffer Center for Health Policy and Economics.

As background, I have been studying medical innovation for nearly three decades. And I co-wrote the chapter on biomedical research in the Handbook of Health Economics. The opinions I offer today are my own and don't represent those of the University of Southern California or the USC Schaeffer Center.

Over the last 50 years, medical breakthroughs have lessened the scourge of cardiovascular disease, cancer, and many other conditions. Researchers have estimated that longer lives provided annual value equal to half of GDP. The question is: How can we sustain the pace of technological innovation, while ensuring patients have access to new technologies?

Medical innovation is costly to pursue. 90 percent of medicines that undergo human trials will fail to launch. Firms will pursue risky innovations only if they expect commensurate financial rewards which are ultimately paid by American consumers.

This tradeoff between innovation incentives and patient access is often framed as an either/or proposition. Either we reward innovators with high prices, or we restrict prices to make new therapies more accessible.

For example, in the early days of part D, our research estimated Medicare price negotiation could lower drug prices by 20 to 25 percent. But the resulting innovation slowdown would cost future Americans about half a year of life expectancy. Though it sounds modest, this is equivalent to every surgeon in American forgetting how to perform heart bypass surgery.

Fortunately, there are solutions. Our study also demonstrated that expanding prescription drug coverage is worth the cost because it simultaneously rewards innovators and makes innovation more accessible.

Today's drug prices determine tomorrow's drug launches. Research suggests that every $21/2 billion of revenue removed from a drug class costs society one new drug approval. For every legislated reduction in Medicare drug prices, as the Inflation Reduction Act promises, we will lose future treatments.

To lessen this risk, we should pursue a more surgical approach to restraining prices. Rewards should be lower for technologies producing less value to patients but higher for those producing more.

Measuring the value of new medicines is hard, but we have the tools to do it properly. Old-fashioned methods like quality-adjusted life years, or QALYs, fail to measure value to patients properly. A new method called Generalized Risk-Adjusted Cost-Effectiveness, or GRACE, corrects these errors and does not discriminate against patients with disability or terminal illness as older methods do.

The IRA provides an opportunity to better align price and value for individual drugs but only if CMS employs evidence-based and scientifically validated measures for measuring value to patients. To align prices and value, USC Schaeffer Center researchers have proposed a different model, starting with lower drug prices at launch and encouraging uptake for clinically eligible patients and accelerating real-world evidence collection.

Subsequently, drug prices would change according to evidence-based real-world benefit. Finally, robust generic or biosimilar competition would drive down prices when the drugs' exclusivity period ends.

Innovative drug pricing policies like these require careful implementation. CMMI's efforts to develop new payment mechanisms for drugs launched under accelerated approval are a potential path forward, but success depends on payments that accurately reflect value to patients.

Policy precedence exists for the controlled launch of new technologies such as CMS' Coverage with Evidence Development Paradigm. However, under CED, as currently implemented, many technologies still languish, even after years of restricted access. While CMS has a legitimate interest in evaluating real-world evidence on medical necessity, restricting access undermines CED's original evidence-gathering goal.

Other reforms are needed. IRA inflation rebates and several other part D program features encourage higher, not lower, launch prices. And by reducing prices for established branded drugs, IRA discourages generic entrants by lower their rewards from challenging patents.

By ensuring generous prescription drug insurance, drug prices that reflect the value they deliver to patients and effective competition throughout the pharmaceutical supply chain, we can achieve improved health for Americans today and also for Americans tomorrow.

Thank you, and I look forward to your questions.

[The statement of Mr. Lakdawalla follows:]

USC Schaeffer
Leonard D. Schaeffer Center
for Health Policy & Economics

Testimony of Darius N. Lakdawalla, Ph.D.

Quintiles Chair in Pharmaceutical Development and Regulatory Innovation,
Mann School of Pharmacy
Professor, Sol Price School of Public Policy
Director of Research, Leonard D. Schaeffer Center for Health Policy & Economics
University of Southern California

Before the

U.S. House Ways & Means Subcommittee on Health

Examining Policies that Inhibit Innovation and Patient Access

May 10, 2023

Key Points:

- Americans have benefited substantially from new medical treatments, and the United States serves as the engine of medical innovation for the world.
- Important public policies have helped stimulate medical innovation. However, significant regulatory and legislative barriers remain that impede the pursuit of valuable innovation.
- Drug price policy debates often hinge on the tradeoff between innovation and access. Our research confirms that Medicare price negotiation lowers prices in the short term but poses significant risks over the long-term to both medical innovation and the health of Americans.
- There are policy solutions, however, that can simultaneously ensure access and encourage innovation.
- A balanced policy approach includes: generous and widely available prescription drug coverage, drug prices that align with the value delivered to patients, and robust competition within the pharmaceutical supply chain.
- The Inflation Reduction Act of 2022, as currently implemented by CMS, does not align with such an approach. Targeted reforms, including reforms to CMS' policies around coverage with evidence development, could help promote innovation and long-term health for all Americans.

Chairman Smith, Chairman Buchanan, Ranking Members Neal and Doggett, and Honorable Members of the Subcommittee, thank you for the opportunity to testify today about the impact of federal policy on medical innovation.

My name is Darius Lakdawalla, and I am an economist, a professor at the USC Mann School of Pharmacy & Pharmaceutical Sciences and USC Price School of Public Policy, and the Director of Research at the USC Schaeffer Center for Health Policy & Economics. By way of background, I have been studying innovation in the health care sector for nearly three decades, and I co-wrote the chapter in the *Handbook of Health Economics* on intellectual property and biomedical research. The opinions I offer today are my own and do not represent the views of the University of Southern California or the USC Schaeffer Center.

The Value of Innovation – an Economist's Perspective

On August 7, 1963, Patrick Bouvier Kennedy was born six weeks premature to President Kennedy and the First Lady. Despite the best care available at the time, Patrick survived less than 48 hours, struggling to breathe and ultimately succumbing to neonatal respiratory distress syndrome. In the decades since, a raft of innovative treatments for premature infants, including effective treatments for respiratory distress, have been studied, developed, launched, and disseminated to neonatal intensive care units around the country. As a result, the survival rate for infants born six weeks premature has now reached 98%. The average American family today enjoys a standard of care that, 60 years ago, was unthinkable even for the First Family of the United States.

Valuable innovation in neonatal medicine is not an isolated example. Medical breakthroughs have extended lives that would previously have been lost to cardiovascular disease, cancer, infectious diseases, and a host of other conditions. Researchers have estimated that longevity improvements have provided the same value as half of all the other goods and services produced in the economy. Economists at the Bureau of Economic Analysis found that even expensive medical technologies can produce benefits to patients that exceed their costs. Medical innovation has transformed society over the past century, because new ideas from basic scientific research have spawned breakthrough treatments, which have in turn improved the lives of patients in need.

This background highlights the twin challenges for policies affecting biomedicine: How can we sustain the pace of technological innovation while ensuring patients have access to the new technologies that emerge?

Complicating these challenges, medical innovation is costly to pursue. Among investigational medicines that undergo human trials, 90% will fail to launch. Pharmaceutical and medical device firms will undertake the costs of innovation only if they expect commensurate financial rewards. However, these rewards must ultimately be paid by all Americans, through out-of-pocket payments, health insurance premiums, or taxes. Indeed, USC Schaeffer Center research quantifies the extent to which American consumers still remain the engine of global medical innovation.

The tradeoff between incentives for innovation and healthcare access for patients is typically framed as an either/or proposition: Either we reward innovators with high prices, and deny many patients access to therapies they desperately need, or we make new therapies broadly accessible by limiting their prices, starving innovators of rewards for developing new drugs.

From the inception of the Medicare Part D program nearly two decades ago, this stark tradeoff has animated debates about whether Medicare should directly negotiate drug prices. For this reason, in the early days of the Part D program, my colleagues at the USC Schaeffer Center and I conducted and published the first academic study estimating the costs and benefits of Medicare price negotiation.

Our research shed important light on this fundamental tradeoff: We estimated that Medicare price negotiation could lower drug prices by about 20-25%, but that the resulting slowdown in medical innovation would ultimately cost future Americans about half a year of life expectancy. That may not sound like a lot, but it is equivalent to what would happen if every surgeon in America suddenly stopped performing heart bypass surgery.

The point is that this tradeoff between innovation and access is real, and it has consequences for future generations.

But there are solutions. Our study of price negotiation also demonstrated that generous prescription drug coverage can serve as the knife that cuts through this knotty tradeoff. Expanding the availability and the generosity of drug coverage is worth the cost because it simultaneously rewards innovators *and* makes new drugs broadly accessible.

This research highlights a path forward, in the spirit of the grand bargain struck by Senator Hatch and Representative Waxman in 1984. The bipartisan Hatch-Waxman Act, despite its complexities and challenges, ensured access for existing drugs while preserving incentives to develop better drugs for future generations.

The 'Right' Price

Better lives for patients and their families is the goal. Simply paying more to encourage any and all innovation is not the means to achieving it. Rather, paying more only for innovations that improve lives will encourage industry to seek out and develop new medicines that help us achieve healthier outcomes.

The way we set prices for medicines today affects both the number and the nature of drugs launched tomorrow. Empirical research has established that drug development activity responds to expected future revenues: The most recent evidence in economics suggests that every $2.5 billion of revenue removed from a drug class costs society one new drug approval in that class.

The implication is that for every legislated reduction in Medicare drug prices—as the Inflation Reduction Act (IRA) promises—we will lose future treatments. The risk is that some or many of those lost future treatments could have substantially improved or lengthened

patients' lives. To lessen this risk, we should pursue a more surgical approach rather than blanket policies to cut prices. Rewards should be higher for technologies that produce more net benefit, or "value," to patients, and they should be lower for technologies that produce less value.

Measuring the value of new medicines is hard, but decades of steady research progress have yielded the tools we need to do it properly. Old-fashioned methods of economic analysis—for instance traditional cost-effectiveness and quality-adjusted life-years (QALYs)—fail to measure value correctly. While many have justifiably observed the ethical challenges posed by QALYs, our research demonstrates that traditional QALYs also get the mathematics of value assessment wrong.

A new value assessment method, called Generalized Risk-Adjusted Cost-Effectiveness (GRACE) corrects these errors by recognizing the long-established principle that goods are more valuable to people who have less of them. Analogously, health improvements are more valuable for people with disabilities, terminal illness, or other severe disease. As such, GRACE also comports with federal law by avoiding value assessments that discriminate against vulnerable patients with disabilities or terminal illness.

The IRA provides an opportunity to better align price and value for individual drugs, but only if CMS employs credible, evidence-based, and scientifically validated methods for measuring value to patients, like GRACE.

Another challenge arises when attempting to align drug prices with value. A drug's value changes over its lifecycle, and its price should change over time to reflect that. At launch there is great uncertainty about how the drug will perform outside of strictly controlled clinical trials. This uncertainty reduces the drug's value at launch, but it can be resolved with data collected from early real-world users.

Researchers at the USC Schaeffer Center have proposed a three-part-pricing framework. In this model, drugs would first undergo an initial "evaluation phase" in which manufacturers launch with a lower price in exchange for early access to Medicare coverage and the possibility of exemption from IRA inflation rebates if the drug meets prespecified effectiveness benchmarks. These benchmarks would be jointly determined by CMS, FDA, and the manufacturer. Using Alzheimer's treatments as an example, benchmarks could involve cognitive performance measures and/or rates of adverse events like brain bleeding. A lower launch price would increase early uptake for patients the FDA deemed clinically eligible, thereby accelerating the collection of real-world evidence on the drug's effectiveness.

The second part of the three-part pricing model is the "reward phase," during which the drug's price changes in response to the real-world benefit demonstrated by new evidence collected in the evaluation phase. If the drug fails to demonstrate value, the price would be set accordingly. Likewise, if the drug achieves its targets, innovators would be rewarded with a high price. Finally, the "access phase" would utilize robust generic or biosimilar

competition to drive down prices upon the drug's loss of exclusivity, improving patient access in the long term.

Innovative drug pricing policies such as these need extensive study and gradual implementation. CMMI's efforts to develop new payment mechanisms for drugs launched under accelerated approval could provide a means to pilot this approach, provided that payments under these mechanisms reflect proper and accurate assessments of value to patients.

There are policy precedents for the "controlled launch" of a new drug or device. CMS's coverage with evidence development (CED) paradigm was designed to provide new technology with the opportunity to demonstrate benefit, in cases where evidence was deemed insufficient to meet the standard of reasonable and necessary care. Similarly, CMS's Medicare Coverage of Innovative Technology (MCIT) policy, had it not been subsequently rescinded, would have provided the coverage and payment mechanisms for such an experiment in the context of "breakthrough" devices.

However, these policy solutions require follow-through: innovators need to be rewarded when they deliver on their commitments. Unfortunately, in the case of CED, it appears that many technologies still languish under years of restricted market access without any certainty of a future of expanded access. And without an existing replacement for MCIT, innovators of breakthrough technologies face uncertain reimbursement opportunities.

Moreover a "controlled launch" should not mean a "scuttled launch." In the recent case of Alzheimer's treatments, CMS has chosen to severely limit access for new medicines that have been or may be approved through the FDA's accelerated pathway, citing concerns about safety, efficacy, and even the appropriateness of amyloid plaque as a surrogate endpoint for Alzheimer's treatment. CMS does have a legitimate interest in evaluating real-world evidence on medical necessity. However, restricting access among patients the FDA deemed clinically eligible limits our ability to gather the real-world evidence that was the original goal of CED.

Policy Reforms Can Help Align Coverage and Payment with Value

Prices must be aligned with value. Unfortunately, the regulatory hurdles to doing so are becoming steeper. Recent policy changes encourage companies to launch at higher, not lower, prices. Once limited to the Medicaid program, inflation rebates have now been introduced into Medicare by the IRA. Inflation rebates that cap price growth—even for drugs that accumulate better-than-expected evidence of real-world effectiveness—limit the ability of prices to rise in response to compelling real-world evidence. The incentives thus created move manufacturers to launch at the highest possible price and to hope their drug works according to the most optimistic real-world clinical scenario. Otherwise, if drugs are launched at lower prices, manufacturers cannot raise their prices later, even if their real-world performance warrants it.

Medicare Part D's benefit design also implicitly encourages high list prices. Part D insurers favor high list prices in part because they move patients more rapidly to the catastrophic phase of coverage, where federal reinsurance payments await. While the IRA's Part D benefit redesign provisions may moderate these reinsurance-related incentives somewhat, other program features (such as an intense focus on premiums and the structure of the risk corridors program) suggest the upward pressure on list prices will continue absent other market changes.

The arrival of new treatments is one of many steps in creating value for patients and society. Patients then need access to these new medicines. For drugs with market exclusivity, USC Schaeffer Center research shows that generous prescription drug insurance ensures access. In general, the introduction of Medicare Part D succeeded in expanding access to pharmaceuticals for American seniors while limiting their financial burden. Yet there is room for improvement.

The link between increasing out-of-pocket costs and patient adherence is well-established. USC Schaeffer Center research found that higher out-of-pocket burden corresponds with lower patient utilization of insulin, while other studies have found similar relationships between patient costs and adherence in rheumatoid arthritis, breast cancer, and chronic kidney disease. In addition, USC Schaeffer Center research demonstrated in the context of novel oral anticoagulants (NOACs) that prior authorization and step therapy restrictions in Part D plans harmed patient health. Patients in plans with more restrictions were less likely to use NOACs, had worse adherence when they *did* use NOACs, took longer to fill their initial NOAC prescription, and faced higher risk of mortality/stroke/transient ischemic attack. This research does not imply that every access restriction harms patient health. Rather, it highlights the need to evaluate the risks and benefits of access policies, just as we evaluate the risks and benefits of new medicines. Access rules underlie the negotiating leverage that health insurers retain in private markets. However, access rules need not shorten lives or harm health. Evidence-based access restrictions would steer patients to lower-cost but therapeutically similar alternatives, providing negotiating leverage without compromising patient health outcomes.

Supporting robust competition is another powerful way to promote access. Once innovative drugs have exhausted the patent protections provided by law, generic or biosimilar entry can dramatically reduce prices. Policies that facilitate timely generic or biosimilar entry will help, although opaque practices in the pharmaceutical supply chain that inflate generic prices and limit biosimilar competition must be addressed as well.

Unfortunately, some IRA provisions will discourage and delay generic entry. Under the Hatch-Waxman Act, generic drug companies that successfully challenge a branded drug patent receive 180 days of generic exclusivity, enabling them to earn a high price until other generic manufacturers enter. The prospect of this reward motivates generic firms to undertake costly legal challenges that might allow them to enter the market first.

The IRA indirectly reduces incentives for generic entry. By reducing prices for branded drugs, the IRA correspondingly lowers the prices that the first generic entrant can charge. Lower rewards to generic manufacturers inevitably will lead to reduced generic entry. This slowdown inflicts harm on uninsured or under-insured patients in the commercial market who will see fewer opportunities to benefit from low-cost generic drugs.

Conclusion: A Grand Bargain - Balancing Innovation and Access

Federal policy is among the most powerful levers available to influence both healthcare costs and innovation incentives. Indeed, this is why the patent clause is enshrined in our Constitution. The challenge in biomedicine is to regulate in a way that creates the most value for both current and future generations of Americans. While there is value in reducing healthcare costs and improving patients' access to existing drugs in the short-term, there is also value in ensuring a continuing stream of innovative therapies for future generations. Both are important, and our research at the USC Schaeffer Center demonstrates that we do not have to choose between them.

A policy solution that strikes a balanced approach, in the spirit of the bipartisan bargain struck by the Hatch-Waxman Act, is required. By ensuring generous prescription drug insurance, drug prices that reflect the value they deliver, and effective competition throughout the pharmaceutical supply chain, we can achieve improved health for Americans today and tomorrow.

Chairman BUCHANAN. Thank you.

Dr. Makower, you are now recognized.

STATEMENT OF JOSHUA MAKOWER, M.D., DIRECTOR, STANFORD BYERS CENTER FOR BIODESIGN, STANFORD UNIVERSITY

Dr. MAKOWER. Thank you, Chairman Buchanan and Ranking Member Doggett, for the opportunity to testify today.

My name is Josh Makower, and I have dedicated the past 34 years of my life to developing therapies and technologies to improve patient care.

Over this time I have founded ten independent medical device companies which collectively have improved the lives of millions and created thousands of jobs in the United States.

In addition to being a physician, inventor, and entrepreneur at Stanford University where I am a professor of Medicine and Bioengineering, I am also the cofounder and director of the Stanford Byers Center for Biodesign.

For 22 years, we have been teaching students, fellows, and faculty the process of medical innovation. And innovations our students have created have touched the lives of 8 million patients to date.

My opinions and my testimony today are my own and do not represent the opinions of the organizations I am affiliated with.

I am here today because of a growing concern that threatens our ability to continue to deliver the improvements to health outcomes innovators like myself have worked so hard to achieve over the years. Increasingly, medical technology innovators are confronting a valley of death where their technologies have received FDA authorization, but no CMS or insurance coverage is in place to allow patients to gain access to them. Simply put, America's seniors and patients across the country are all too often not getting timely access to critical medical technologies for many years, if ever.

Being science and data-driven, my colleagues and I at Stanford Biodesign Policy program have taken some time to just study how difficult the environment has become. In work we published last January, we surveyed 336 healthcare innovators and investors to ask how long based on their own experience it took for breakthrough technologies to achieve Medicare coverage, coding, and payment.

Our research found that Medicare patients often wait many years to get access to FDA-authorized technologies. Surveys respondents reported that nationwide Medicare coverage for breakthrough medical technologies takes an average of 4.7 years following FDA authorization.

While the survey of opinions of the innovators was a place to start, our group followed up this work and used publicly available data to determine what the actual reality is. And it is much worse than we thought.

In the second study, we discovered of novel medical technologies authorized by the FDA between 2016 and 2019, only 44 percent achieved nominal Medicare coverage by the end of 2022 and the median time to achieve this nominal coverage was actually 5.7 years, a whole year longer than our initial survey indicated. We are

working towards publishing the results of the second study in the near future.

In our original study, over half the innovators said that they were unlikely to take on a breakthrough medical technology project without some form of accelerated reimbursement pathway. The reimbursement pathways are so challenging right now that 69 percent of respondents who made investments in companies developing breakthrough medical technologies said they would be less likely to do so again unless there was an expedited reimbursement pathway.

While we have not studied the impact of these delays and decisions on actual patient morbidity and mortality, given that these diseases address—I am sorry—these technologies address diseases such as diabetes, stroke, cancer, heart disease, spine, and orthopedic disorders, we are confident that, when we do this further analysis, we are likely to find the impact on patients will be significant.

We are eagerly awaiting the release of a new proposed rule from CMS and hopeful that it is a meaningful and impactful proposal that will accelerate patient access to critical medical technologies.

The tragic truth is, while this valley of death remains, patients throughout the United States in each of your congressional districts are being impacted, unable to access breakthrough medical technologies that have been proven safe and effective by the FDA.

In addition to CMS' rulemaking, Congress has introduced legislation to address these serious concerns for the past three sessions with strong bipartisan support including in the last session Cures 2.0. At root, the concept that would be ideal is to obtain coverage very shortly after FDA authorization, allowing for any continued evidence collection to be obtained as the process of adoption begins.

As a physician and innovator, I encourage all of you to continue the strong bipartisan work towards achieving this—towards addressing this growing concern. The work that we have invested in inventing and developing cures, therapies, and diagnostics are only beneficial when patients and providers can access them.

Thank you, Chairman Buchanan and Ranking Member Doggett, for the opportunity to testify today.

I also want to thank the entire committee for their support of the science that has led to these important breakthroughs. And I look forward to working with you and all the members of this body to achieve our common goal of improving patient care.

And I look forward to answering your questions.

Thank you very much.

[The statement of Dr. Makower follows:]

Thank you Chairman Buchanan and Ranking Member Doggett for the opportunity to testify today.

My name is Josh Makower and I have dedicated the past 34 years of my life to developing therapies and technologies to improve patient care. Over this time I've founded 10 independent medical device companies which collectively have improved the lives of millions and created thousands of jobs in the United States. In addition to being a physician-inventor and entrepreneur, at Stanford University where I am a Professor of Medicine and Bioengineering, I am also the co-founder and Director of the Stanford Byers Center for Biodesign. Our organization is focused on improving health outcomes and health equity through innovation education, translation and innovation policy. For 22 years we've been teaching students, fellows and faculty the process of medical innovation and the innovations our students have created have touched the lives of over eight million patients to date. I am also on the board of nine medical device companies and an advisor to New Enterprise Associates. The opinions in my testimony today are my own and do not represent the opinions of any of the organizations I am affiliated with.

Throughout my time working in the medical technology innovation ecosystem, I, along with my fellow innovators, have encountered many hurdles. We work with all of the stakeholders involved in delivering patient care to overcome them, because our shared and common goal is to save and improve the quality of life for patients. I am here today because of a growing concern that threatens our ability to continue to deliver the improvements to health outcomes innovators like myself have worked so hard to achieve over the years. Increasingly, medical technology innovators are confronting a "valley of death" where their technologies have received FDA authorization, but often no CMS or insurance coverage is in place to allow patients to gain access to them. Simply put, America's seniors and patients across the country are all too often not getting timely access to critical medical technologies for many years, if ever.

Being science and data driven, my colleagues and I at Stanford Biodesign Policy Program have taken some time to study just how difficult the environment has become. In work which we published last January, we surveyed 336 healthcare innovators and investors to ask how long, based on their own experience, it took for breakthrough new technologies to achieve Medicare coverage, coding and payment. The survey also asked questions to determine whether a clear path to reimbursement would affect innovation and investment in clinical areas of particular importance to Medicare patients. I have included the full survey as a part of my submitted testimony, but I did want to highlight some of the deeply concerning results that we found.

Our research found that Medicare patients often wait many years to get access to FDA-authorized technologies. Survey respondents reported that nationwide Medicare coverage for breakthrough medical products takes an average of 4.7 years following FDA authorization. While a survey of innovator opinions was a place to start, our group followed up on this further and using publicly available data – assessing a cohort of novel technologies approved or cleared by the FDA between 2016 and 2019, we discovered the results were much worse than initially presented. In this second study, we discovered only 44% of that cohort achieved nominal Medicare coverage by December 2022, and the median time to achieve this nominal coverage was actually 5.7 years. One whole year longer than our initial survey indicated. We are working towards publishing the results of this second study in the near future.

A swift, predictable pathway for coverage of breakthrough medical technologies would encourage innovators and investors to take on high-impact projects in fields that are important to Medicare beneficiaries, such as cardiovascular disease, stroke, and cancers. Achieving appropriate reimbursement is one of the greatest risks that innovators, and the investors who fund them, must consider in deciding whether to undertake new projects to improve patient care.

In our original survey, 84% of innovators said they would likely take on a novel or breakthrough product as their next project if there was an accelerated reimbursement pathway in place, while 53% said that they were unlikely to do so without such a pathway. While it is increasingly difficult for small start-ups with novel technologies to attract investment, it is all the more notable that investors agreed with our findings. The reimbursement pathways are so challenging right now that 69% of respondents who made investments in companies developing breakthrough medical devices said they would be less likely to do so again without an expedited reimbursement pathway.

While we have not yet studied the impact of these delays on actual patient morbidity and mortality, given that these technologies address diseases such as diabetes, stroke, cancer, heart disease, spine and orthopedic disorders, we are confident that when we do this further analysis we are likely to find the impact on patients will be significant.

Based upon our extensive research and findings, we do believe that a well-designed program that enables coverage while continuing to collect evidence could benefit patients by accelerating access to important health advances and encouraging invention, innovation and investment in critically important areas of unmet clinical needs.

For the past three presidential administrations, CMS has examined creating a new dedicated accelerated coverage pathway for novel medical technologies that addresses unmet needs for America's seniors. We are eagerly awaiting the release of a proposed rule and hope that it is a meaningful and impactful proposal that will accelerate patient access to critical medical technologies. The tragic truth is while this "valley of death" remains, patients throughout the United States – in each of your Congressional districts – are being impacted, unable to access breakthrough medical technologies that have been proven to be safe and effective by the FDA. In addition to CMS's rulemaking, Congress has introduced legislation to address these serious concerns for the past three sessions with strong, bipartisan support, including in the last session: "CURES 2.0". At root, the concept that would be ideal is to obtain coverage very shortly after FDA authorization, allowing any continued evidence collection needed to be obtained as the process of adoption begins. As a physician and innovator, and honestly as a potential Medicare patient someday sooner than I'd like to admit, I encourage all of you to continue the strong bipartisan work towards addressing this growing concern.

The work that we have invested in inventing and developing cures, therapies and diagnostics are only beneficial when patients and providers can access them. Our work in this area has clearly identified a serious challenge that is confronting innovation and patient care, and I remain hopeful that a policy solution can be provided which would help bridge this gap, and put an end to the "valley of death" for many innovations created to help Americas seniors.

Thank you again Chairman Buchanan and Ranking Member Doggett for the opportunity to testify today. I also want to thank the entire committee for their support of the science that has led to these important breakthroughs and I look forward to working with you and all the members of this body to achieve our common goal of improving patient care. I look forward to answering any of your questions.

Chairman BUCHANAN. Thank you.
Dr. Kesselheim, you are recognized.

STATEMENT OF AARON S. KESSELHEIM MD, JD, MPH, PROFESSOR OF MEDICINE, HARVARD MEDICAL SCHOOL

Dr. KESSELHEIM. Chairman Buchanan, Ranking Member Doggett, members of the subcommittee, my name is Aaron Kesselheim. I am a primary care doctor and Professor of Medicine at Harvard Medical School where I run the program on Regulations, Therapeutics, and Law, or PORTAL, at Brigham's and Women's Hospital.

I want to focus my comments today on meaningful drug innovation because not all innovation is the same. Meaningful drug innovation provides useful benefits to patients with diseases that don't have effective therapies or measurably improves upon existing treatments.

One way the government generates meaningful innovation is through funding by the NIH. While individual manufacturers certainly contribute to drug development, NIH funding provides extensive contributions, usually at the earliest stages when the risk is greatest and private companies are not willing to get involved.

One highly visible recent example that occurred—occurred with the mNRA COVID vaccines. Here, the U.S. Government invested about 432 billion to develop, produce, and purchase vaccines and provided a guaranteed market for the final stages of development, almost completely derisking the investment for manufacturers.

In my written comments, I review the substantial and essential role played by public funding in transformative drugs like sofosbuvir for hepatitis C, TDF–FTC for HIV prep, buprenorphine for opioid use disorder, as well as every cell and gene therapy available in the U.S.

But meaningful drug innovation is unfortunately quite rare. In the last decade, fewer than one third of new drugs demonstrated meaningful added therapeutic benefits. Yet these drugs, like all brand name drugs in the U.S., are invariably expensive, costing far more than patients spend for the same drugs in other industrialized countries.

Drug launch prices have increased exponentially, such that about half of new drugs are now initially priced above $150,000 a year. Low additional-value drugs are also widely advertised, as anybody who has watched a football game can tell you, making up about three quarters of top advertised drugs.

As a result, a large number of U.S. patients use low-added value drugs at substantial cost to them and the U.S. healthcare system. We found that over half of the 50 top-selling drug in Medicare had low-added clinical benefits, accounting for $20 billion in annual net spending.

Since the government through Medicare, Medicaid, and other programs is also the single biggest purchaser of drugs in the U.S., it must distinguish between meaningful drug innovation and innovation that doesn't add to patients' outcomes. In the case of aducanumab for Alzheimer's disease, the FDA approved the drug based on no clear evidence that it worked. And despite it causing potentially dangerous brain swelling and bleeding in up to 40 percent of the patients who took it, the manufacturer still price it at

initially $56,000 a year which could have led the government to pay for this one drug more than the entire budgets of NASA.

So, the CMS issued a national coverage determination to restrict payment to the context of a clinical trial, which is exactly what was needed to determine whether or not the drug actually worked.

As a second example, CMMI recently announced a project to pay less for accelerated approval drugs which are FDA approved based on unvalidated surrogate measures only. Yet they are just as exceedingly expensive as traditional approvals. Why should taxpayers pay whatever excessively high prices the manufacturer wants to set for a drug without evidence that it affects clinical outcomes that patients care about, how patients feel, function, or survive?

CMMI's plan also provides incentives manufacturers need to complete confirmatory studies in a timely fashion and get evidence for these drugs' actual clinical benefits. The price can then be adjusted if the drug is actually meaningfully innovative. In the past 2 year alone, about two dozen accelerated drug approval indications have been withdrawn after negative confirmatory study.

As a final example, CMS under the Trump administration issued a problematic rule to require CMS to pay for every medical device labeled as a breakthrough by the FDA. But the FDA's criteria for this designation were so lax that, as Representative Doggett pointed out, over 200 device qualified in the first 3 years of the program. And some of those didn't actually show any useful benefits for patients and had important safety risks. Smartly, CMS has since walked back from this rule to avoid the government wasting taxpayer dollars.

Congress can help further support meaningful drug innovation. I have three ideas for you today. First, the NIH's budget should be doubled. But shockingly, a bill passed by the House instead cut NIH's funding by $10 billion. This would devastate future transformative drug development and doom the prospects of the patients getting useful treatments in many areas of unmet medical need.

Second, Congress should give the government more authority to reduce unnecessary spending on excessively priced drugs that do not provide meaningful clinical benefits to patients. For example, the Inflation Reduction Act vested in CMS the authority to negotiate prices for certain drugs based on their clinical value and other important factors. But the bill has numerous exclusions including having to wait at least 9 to 13 years before negotiated prices take effect. Congress should build on the IRA to negotiate fair prices for all new drugs shortly after approval, as is done in all other industrialized countries.

Finally, the U.S. should look for new ways to ensure patients and taxpayers only pay for meaningful innovation by establishing a new expert organization to provide evidence-based reports on new drugs' added clinical value, pricing, and any disparities in access. This body can help patients better distinguish meaningful and less useful innovation and make important clinical decisions about them.

All of these steps will better help ensure that meaningful innovation is incentivized and that patients aren't going bankrupt or putting their health at risk, spending money on low-value drugs or medical devices.

Thank you very much.
[The statement of Dr. Kesselheim follows:]

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HOW THE US GOVERNMENT SUPPORTS MEANINGFUL DRUG AND DEVICE INNOVATION: FUNDING DEVELOPMENT OF TRANSFORMATIVE THERAPIES AND AVOIDING EXCESSIVE PRICES FOR NEW PRODUCTS WITH LIMITED BENEFITS

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United States House of Representatives
Health Subcommittee of the Ways & Means Committee
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Washington, D.C.

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Summary of major points

- The US government supports drug innovation in two important ways. First, through the **National Institutes of Health (NIH),** it supports the development of transformative drugs.
    - Much NIH support is focused on drug discovery and the early stages of development, but public funding can also include substantial impact in later stages of drug development, including proof of concept testing and even the pivotal clinical trials leading to FDA approval.
    - The essential role of the government in supporting drug innovation is particularly notable in the development of **transformative drugs**—those that are both innovative and have had a groundbreaking effect on patient care.

- Second, the government is the largest single purchaser of prescription drugs through Medicare and Medicaid, among other programs, and it can take steps to ensure that taxpayer funds are used to preferentially provide access to patients for meaningful pharmaceutical innovation.
    - Truly transformative drugs are unfortunately rare, and many new drugs **do not** offer important advances in efficacy or safety for patients despite generally being sold at high prices that make them quite profitable for manufacturers.
    - It is therefore crucial for the government to distinguish between meaningful pharmaceutical innovation and low-value innovation in purchasing expensive prescription drugs and medical devices.
    - In recent years, the government has tried to do that by (a) issuing a **National Coverage Determination to pay for the Alzheimer's disease drug aducanumab**, which was initially priced at $56,000 per year despite no clear evidence that it works; (b) proposing **special payment models through the Centers for Medicare and Medicaid Innovation** that would limit payment for drugs without clear evidence of patient benefits and ensure that cell and gene therapies are reimbursed according to the clinical benefits they provide; and (c) **withdrawing a rule that would have forced the government to pay for medical devices authorized by the FDA without clear evidence of important patient benefits**.

- Congress should take several steps to ensure the continued discovery of transformative drugs and to ensure that patient and taxpayer funds are not wasted on excessively priced drugs and medical devices that offer little additional meaningful benefits to patients.
    - Congress should **double the NIH's budget**, not reduce it like was recently proposed in a bill passed by the House of Representatives.
    - Congress should **expand Medicare's ability to negotiate fair drug prices** initiated under the Inflation Reduction Act.
    - Congress should create a national body tasked with providing **public reports that can help patients and their physicians better distinguish meaningful from low-value pharmaceutical innovation**.

Chairman Buchanan, Ranking Member Doggett, and Members of the Committee:

My name is Aaron Kesselheim. I am an internal medicine physician, lawyer, and a Professor of Medicine at Harvard Medical School, in the Division of Pharmacoepidemiology and Pharmacoeconomics of the Department of Medicine at Brigham and Women's Hospital in Boston, one of the main Harvard teaching hospitals. I lead its Program On Regulation, Therapeutics, And Law (PORTAL), an interdisciplinary research center that studies the intersections between prescription drug affordability and use, laws and regulations related to medications, and the development and cost of drugs. PORTAL is one of the largest non-industry-funded research centers in the country that focuses on pharmaceutical use, law, and economics. In 2020, I was elected to the National Academy of Medicine. I am honored to have been invited today to talk to you about the essential role that the US government plays in pharmaceutical innovation. I will then discuss some recent steps that the government has taken to incentivize *meaningful* drug innovation, while trying to ensure that vulnerable patients are not exposed to ineffective new drugs or devices sold for high prices.

I. Role of US government in supporting patients' access to transformative drug innovation

The greatest source of pharmaceutical innovation in the world is the National Institutes of Health (NIH). A new medication or biotechnology drug usually emerges from a long course of research that starts with pivotal basic science discoveries, followed by translational and applied studies, product development research, and clinical testing. While the contribution of industry-based research to drug development remains vital, NIH funding to academic medical centers and discoveries made in government laboratories provide extensive contributions to drug development. According to one review, every single drug approved by the FDA from 2010 to 2016 could be traced back to funding from NIH in some way.[1] In another review of 356 drugs FDA-approved from 2010 to 2019, investigators linked NIH funding to 354 (99.4%), calculating that on average public funding of basic or applied research contributed about $1.44 billion per approval.[2]

Much NIH support is focused on drug discovery and the early stages of development, which is when private funding is the least available because it is when the greatest risk lies. Activities at these stages include describing the pathophysiology of diseases, charting biochemical pathways that could be modulated, isolating druggable targets on proteins, and developing systems to allow for *in vitro* testing of potential lead compounds that could serve as therapeutics. For example, in the case of direct-acting antivirals that offer a nearly fully effective cure for chronic hepatitis C virus infection, a research study led by Rachel Barenie at PORTAL identified $60.9 million in NIH funding closely related to the development of sofosbuvir (Sovaldi), including developing hepatitis C virus cell culture systems and growing the virus *in vitro*.[3] Many large pharmaceutical companies have actively moved away from this sort of work in recent years, making the contributions of the NIH in this area even more essential to the identification of new treatments.

In addition, public funding can also include substantial impact in later stages of drug development, including proof of concept testing and even the pivotal clinical trials leading to FDA approval. In work led by Rahul Nayak, we published a review of drugs approved from 2008-2017, which found that 25% (62/248) were based on patents or other late-stage intellectual contributions from publicly-supported research

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[1] Galkina Cleary E, Beierlein JM, Khanuja NS, McNamee LM, Ledley FD. Contribution of NIH funding to new drug approvals 2010-2016. Proceedings of the National Academy of Sciences of the USA 2018;115(10):2329-2334

[2] Galkina Cleary E, Jackson MJ, Zhou EW, Ledley FD. Comparison of Research Spending on New Drug Approvals by the National Institutes of Health vs the Pharmaceutical Industry, 2010-2019. JAMA Health Forum 2023;4(4):e230511.

[3] Barenie RE, Tessema FA, Avorn J, Kesselheim AS. Public funding for transformative drugs: the case of sofosbuvir. Drug Discovery Today 2021;26(1):273-281.

institutions.[4] Among 69 new biologic agents approved by the FDA during the same time period, 29 drugs (42%) had late-stage contributions from public-sector institutions or originated from a public-sector spin-off company.[5] Drugs with links to late-stage public funding were more likely to receive expedited FDA approval or be designated first-in-class, two markers that often indicate therapeutic importance. In a recent review of NIH records connected to use of the drug tenofovir disoproxil fumarate-emtricitabine (TDF-FTC, or Truvada) as HIV pre-exposure prophylaxis (PrEP), we found that the idea originated at the Centers for Disease Control and Prevention (CDC), and pivotal research evaluating use of the combination was supported by an estimated $143 million of highly-related direct NIH funding, for example covering the key trials helped establish TDF-FTC's clinical efficacy for PrEP.[6]

The essential role of the government in supporting drug innovation is particularly notable in the development of transformative drugs—those that are both innovative and have had a groundbreaking effect on patient care.[7] I led a survey of clinical leaders in over a dozen different medical specialties from the top 30 academic medical centers in the US to determine what they thought were the most transformative drugs in their specialties to have been approved by the FDA from 1984-2009. Among the final list of 26 drugs and drug classes, we then examined the developmental history, based on primary sources, such as the patents, articles published in the peer-reviewed literature, and interviews with key innovators.[8] One key finding among these transformative products was the centrality of publicly-funded government- and academic-based innovators and discoveries made by academic researchers supported by federal government funding, while others were jointly developed in both publicly funded and commercial institutions.[9] Over the course of nearly four decades, the active ingredient in buprenorphine was synthesized by a pharmaceutical manufacturer, but it was developed for opioid use disorder primarily by investigators in government and academic centers, including a formal government-industry partnership for commercialization. Nearly $40 million in highly-related NIH went to institutions and investigators supported the development of buprenorphine as a treatment for opioid use disorder.[10]

Perhaps the most highly visible example of public funding supporting drug development occurred with the development of the transformative mRNA COVID-19 vaccines. According to a recently-published PORTAL research study led by my colleague Hussain Lalani, the US government invested at least $31.9 *billion* to develop, produce, and purchase mRNA covid-19 vaccines, including sizeable investments in the three decades before the pandemic through March 2022 relating to development of lipid nanoparticles as a drug delivery system, synthesis and modification of mRNA and small interfering ribonucleic nucleic acid, definition of the prefusion "spike" protein structure of SARS-CoV-2, and development of RNA vaccine biotechnology for use in humans.[11] In this case, not only did the NIH and US government provide substantial

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[4] Nayak RK, Avorn J, Kesselheim AS. Public section financial support for late stage discovery of new drugs in the United States: cohort study. BMJ 2019;367:15766.
[5] Nayak R, Lee CC, Avorn J, Kesselheim AS. Public-sector contributions to novel biologic drugs. JAMA Internal Medicine 2021;181(11):1522-1525.
[6] Tessema FA, Barenie RE, Avorn J, Kesselheim AS. Federal funding for discovery and development of costly HIV drugs was far more than previously estimated. Health Affairs 2023;42(5):642-649.
[7] Sampat BN. Academic patents and access to medicines in developing countries. American Journal of Public Health 2009;99:9-17.
[8] Kesselheim AS, Avorn J. The most transformative drugs of the past 25 years: a survey of physicians. Nature Reviews: Drug Discovery 2013;12(6):425-431.
[9] Kesselheim AS, Tan YT, Avorn J. The roles of academia, rare diseases, and repurposing in the development of the most transformative drugs. Health Affairs 2015;34:286-294.
[10] Barenie RE, Kesselheim AS. Buprenorphine for opioid use disorder: the role of public funding in its development. Drug and Alcohol Dependence 2021;219:108491.
[11] Lalani HS, Nagar S, Sarpatwari A, Barenie RE, Avorn J, Rome BN, Kesselheim AS. US Public investment in the development of mRNA COVID-19 vaccines: retrospective cohort study. BMJ 2023;380:e073747.

support the key discoveries and development of the mRNA vaccine technology, but it also provided a guaranteed market for the final stages of development. These highly effective vaccines have helped protect millions of people from the complications of COVID-19, and they would not have been discovered or disseminated as quickly in the first years of the pandemic without the key participation of the government.

Sofosbuvir, TDF-FTC as PrEP, buprenorphine for opioid use disorder, and COVID-19 vaccines are just a small number of the extremely important pharmaceutical innovations that have arisen directly from substantial government investment in the past few decades. For example, imatinib (Gleevec), developed in large part by researchers at the Dana-Farber Cancer Center in Boston, was approved in 1998 for chronic myelogenous leukemia. It helped turn a rare disease with few effective treatments into one that many patients can now live with for years. More recently, gene therapies like voretigene neparvovec (Luxturna) now offer substantial improvements for patients with a congenital form of blindness. Gene therapies like these approved in the US thusfar all have their origins in NIH funding to academic institutions or in spinoffs from such institutions that developed indispensable know-how and underlying forms of technology.[12]

II. Role of US government in steering patients away from ineffective or dangerous innovation

While the government has had a substantial, consistent, and undeniable role in supporting the development of useful pharmaceutical innovation, it is also important to recognize that truly transformative drugs are unfortunately rare. Indeed, by several metrics, pharmaceutical innovation as a whole in the US has been disappointing, especially in recent years. Although the overall number of new drugs approved by the FDA has increased in the last few years, many new drugs do not offer important advances in efficacy or safety for patients despite generally being sold at high prices that make them quite profitable for manufacturers. In a recent review of FDA-approved drugs from 2007-2017 led by my PORTAL colleague Thomas Hwang, we found that among 267 new drugs rated by 5 key international independent drug evaluation groups, *fewer than one-third* (31%) were rated as having high added therapeutic value by at least one organization.[13] Although these therapeutic benefit assessments are made without reference to the drugs' prices, all newly approved drugs are invariably expensive, particularly in the US. Indeed, Ben Rome in our PORTAL group recently showed that from 2008 to 2021, launch prices for new drugs increased exponentially by 20% per year, such that by 2020-2021, 47% of new drugs were initially priced above $150,000 per year.[14] Thus, while some important new drugs are developed and marketed every year, many newly marketed drugs are very costly and may offer little clinical advantage over medications that are already available.[15] Not only are low-additional-value drugs commonly approved by the FDA, they are also widely advertised. In a study led by my PORTAL colleague Neeraj Patel, we found that among 81 top-advertised drugs, 73 drugs had at least one therapeutic benefit rating and were associated with advertising spending of $22.3 billion from 2015 to 2021—but only 20 of these commonly marketed drugs (27%) were rated by any agency as having high added therapeutic value.[16] It is therefore not surprising that a large number of US patients use low-value

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[12] Vokinger KN, Avorn J, Kesselheim AS. Sources of innovation in gene therapies—approaches to achieving affordable prices. New England Journal of Medicine 2023;388(4):292-295; Newham M, Vokinger KN. Adverse effects of acquisitions in the pharmaceutical industry. Nature Medicine 2022;28(7):1342-1344.

[13] Hwang TJ, Ross JS, Vokinger KN, Kesselheim AS. Association between FDA and EMA expedited approval programs and therapeutic value of new medicines: retrospective cohort study. BMJ. 2020;371:m3434

[14] Rome BN, Egilman A, Kesselheim AS. Trends in prescription drug launch prices, 2008-2021. JAMA 2022;327(21):2145-2147.

[15] Some of these new drugs are effective, but are just second- or later-in-class products. Such products may offer some utility to patients, such as those who cannot tolerate the first-in-class product, and so we could offer more tailored incentives for their production.

[16] Patel N, Hwang T, Woloshin S, Kesselheim AS. Therapeutic value of drugs frequently marketed using direct-to-consumer television advertising, 2015-2021. JAMA Network Open 2023;6(1):e2250991.

drugs, at substantial costs to patients and the US health care system. A recent study led by my PORTAL colleague Alex Egilman reviewed the 50 top-selling drugs in Medicare in 2020 and their therapeutic value assessments by 3 of the same organizations. We found that *over half of the drugs* (27, or 55%) had a low added therapeutic benefit rating, accounting for $19.3 billion in annual estimated net spending, or 11% of total Medicare net prescription drug spending that year.[17]

It is widely recognized that the US spends more per capita on brand-name prescription drugs than any other industrialized nation. The federal and state governments are also the largest single purchaser of prescription drugs; indeed, Medicare alone accounts for more than one-third of the country's total drug spending. Since too many of these products offer limited added therapeutic benefits over other existing products, it is essential for the solvency of the US health care system that the government ensure it does not pay extremely high prices for new drugs that do not actually offer meaningful added clinical benefits.

In recent years, various government agencies have taken steps intended to ensure that there is fair reimbursement for meaningful innovation, but that the government does not pay excessively for drugs offering unclear or limited additional benefits. Next, I will focus on a few of these steps, which are the focus of today's hearing.

A. CMS' national coverage decision for aducanumab (Aduhelm)

Alzheimer's disease is the most common cause of memory impairment and dementia in older adults, and it is a progressive and often debilitating medical condition. It can have a major impact on quality of life and independence, and is the 6th leading cause of death in the US. Patients with Alzheimer's disease lack effective treatments that have meaningful long-term effects on thinking, behavior, or maintaining independent living.

Aducanumab (Aduhelm) was designed to reduce protein deposits called amyloid plaque in the brain. Excessive amyloid plaque is a main feature of Alzheimer's disease, but not everyone with amyloid plaque has or will get Alzheimer's disease. Unfortunately, the key trials studying aducanumab provided no clear evidence that it worked. The drug was evaluated in two identical 18-month randomized trials involving over 3000 patients with early Alzheimer's disease. These trials were stopped before completion because they were found to be futile in a pre-specified analysis of the full dataset, even though aducanumab substantially reduced amyloid plaque in both trials. When reviewed individually, the key measure of the effect of the drug on the things that matter most to people with Alzheimer's and their families—remembering, learning, reasoning, and functioning[18]—was no different than placebo in one trial and only slightly better than placebo in the other, with people in the high-dose aducanumab group declining only slightly less than people randomized to placebo.[19] The absolute difference was small, 0.39 points on a 19-point scale, which is lower than the 1-2 point change cited as the smallest difference likely to be noticeable

[17] Egilman AC, Rome BN, Kesselheim AS. Added therapeutic benefit of top-selling brand-name drugs in Medicare. JAMA 2023;329(15):1283-1289.
[18] Woloshin S, Kesselheim AS. What to know about the Alzheimer drug aducanumab (Aduhelm). JAMA Internal Medicine 2022;182(8):892.
[19] In the low-dose aducanumab arm in Study 302, the effect was not statistically significant, again precluding the ability to assess efficacy with respect to secondary outcomes among both the high- and low-dose treatment arms. Alexander GC, Knopman DS, Emerson SS, Ovbiagele B, Kryscio RJ, Perlmutter JS, Kesselheim AS. Revisiting FDA approval of aducanumab. New England Journal of Medicine 2021;385(9):769-771.

by physicians.[20] In addition, patients across both trials randomized to high-dose aducanumab frequently experienced problems including brain swelling (35% with the drug vs. 3% with placebo) and bleeding. An advisory committee of 11 experts outside the FDA (including myself) reviewed the available evidence and nearly unanimously concluded (10 votes no, one abstention) that these data did not support a conclusion that aducanumab slows cognitive decline but were concerned about the substantial safety risks.

However, the FDA approved the drug anyway, under its accelerated approval program, agreeing with the manufacturer that the amyloid lowering was reasonably likely to lead to actual clinical benefits at some undetermined point in the future. This decision was made despite a "council of senior agency officials" concluding that "there wasn't enough evidence it worked" and one even noting that approval could "result in millions of patients taking aducanumab without any indication of actually receiving any benefit, or worse, cause harm."[21] There were numerous related flaws in the decision. The FDA initially approved the drug for all patients with Alzheimer's disease, even though it was only tested in patients with mild disease (that approval language was later amended). The manufacturer-written and FDA-approved labeling also called for less frequent monitoring than was performed in clinical trials,[22] which could heighten the risk for severe complications of the brain swelling and bleeding commonly associated with the drug, and did not include contraindications for drugs that could further increase that risk. Although drugs approved via accelerated approval must conduct post-approval studies because they lack evidence that they affect real clinical outcomes, the manufacturer of aducanumab was given 9 years for its trial. The FDA said that based on the results, "If the drug does not work as intended, we can take steps to remove it from the market."[23]

The decision met with widespread disapproval by the medical community. Large academic centers like Cleveland Clinic, Mt Sinai, my own Mass General Brigham, and the Veterans Administration declined to put the drug on formularies, while regulators in Europe and Japan rejected it outright. Wanting to "establish aducanumab as one of the top pharmaceutical launches of all time,"[24] aducanumab's manufacturer initially listed the drug at an average price of about $56,000 per year (it was much later reduced by half). At that price, if only one-tenth of patients with Alzheimer's disease were prescribed it, Medicare's total annual spending would exceed $28 billion (more than six times as much as Medicare spent to cover any other drug in 2019.)[25] There would be substantial additional costs: considering charges for infusion services, repeated imaging and medical management (including hospitalization for severe symptoms), treatment costs could have exceeded $100,000 per patient per year, of which Medicare covers a substantial portion but still leaves patients with large out-of-pocket costs.[26] In this way, US taxpayers were poised to spend as much as $6-$29 billion per year (more than the total budgets of NASA or the CDC)[27] on a drug with unclear and likely

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[20] Andrews JS, et al. Disease severity and minimal clinically important differences in clinical outcome assessments for Alzheimer's disease clinical trials. Alzheimer's & Dementia: Translational Research & Clinical Interventions 2019;5:354-363.
[21] Belluck P, Kaplan S, Robbins R. How an unproven Alzheimer's drug got approved. July 19 2021. Available from: https://www.nytimes.com/2021/07/19/health/alzheimers-drug-aduhelm-fda.html
[22] Chiong W, et al. Decisions with Patients and Families Regarding Aducanumab in Alzheimer Disease, with Recommendations for Consent: AAN Position Statement. Neurology 2021;17:10.1212/WNL.0000000000013053.
[23] https://www.fda.gov/drugs/news-events-human-drugs/fdas-decision-approve-new-treatment-alzheimers-disease
[24] US House of Representatives. The high price of Aduhelm's approval: an investigation into FDA's atypical review process and Biogen's aggressive launch plans. December 2022.
[25] Cubanski J. Relatively few drugs account for a large share of Medicare prescription drug Spending. Kaiser Family Foundation. April 19, 2021. Available from: https://www.kff.org/medicare/issue-brief/relatively-few-drugs-account-for-a-large-share-of-medicare-prescription-drug-spending/
[26] Chiong W, et al. Decisions with Patients and Families Regarding Aducanumab in Alzheimer Disease, with Recommendations for Consent: AAN Position Statement. Neurology 2021;17:10.1212/WNL.0000000000013053.
[27] Katz J, Kliff S, Sanger-Katz M. Drug could cost the government as much as it spends on NASA. New York Times. June 22, 2021. Available from: https://www.nytimes.com/2021/06/22/upshot/alzheimers-aduhelmmedicare-cost.html.

unnoticeable benefits that could have put thousands of patients' lives at risk. Reflecting this projection, the Centers for Medicare and Medicaid Services (CMS) announced largest-ever annual increase in Medicare premiums due to anticipated aducanumab spending with monthly Medicare Part B premiums increasing from $148.50 to $170.10 and Part B deductible increasing 15%, from $203 to $233.

In this context, CMS made the reasonable decision to issue a national coverage determination—something it rarely does for FDA-approved drugs—to limit coverage of aducanumab and other potential anti-amyloid monoclonal antibodies approved under accelerated approval for patients enrolled in clinical trials only. Medicare is prohibited by law from paying for any medical products that are not "reasonable and necessary." Since aducanumab was approved by the FDA despite a lack of any clear clinical benefit, CMS' proposal to restrict coverage of the drug to its use in clinical trials was the most scientific pathway forward to help understand whether the drug actually works and whether any benefits it had outweighed its substantial risks. This decision was actually quite generous of CMS, since it is usually the financial responsibility of the manufacturer to supply the drug in the context of enrolling of patients in post-approval trials for patients receiving accelerated approval drugs. Ultimately, the manufacturer made the business decision to stop distribution of the drug rather than subject it to further clinical testing to tell if it actually worked to help patients.

CMS's aducanumab decision to live up to its Congressional mandate (even if the FDA did not, in this case) to support effective, necessary care wisely avoided wasting the nation's health care resources on a drug with no proven efficacy and substantial risks. CMS' decision also served as a major incentive for any other manufacturer with anti-amyloid monoclonal antibodies targeting Alzheimer's disease to complete trials of the drug's clinical effects as expeditiously as possible. Patients with Alzheimer's disease deserve new treatments that have reliable evidence that their benefits outweigh their risks, and the CMS decision supported this goal by rejecting paying for a drug with no clear evidence of benefit unless patients were enrolled in trials designed to determine whether that benefit existed.

B. CMMI's Demonstration Projects

The Center for Medicare and Medicaid Innovation (CMMI), situated within CMS, was created by the Affordable Care Act for numerous reasons, including the testing of innovative payment and service delivery models for Medicare and Medicaid beneficiaries. CMMI has launched numerous novel payment models in the last decade,[28] some of which have covered Medicare drug spending. CMMI's most recent drug pricing-related pilot project was a set of 3 proposals affecting the way Medicare patients pay for certain generic drugs, expensive cell and gene therapies, and accelerated approval drugs lacking proven clinical benefit to patients. In these potential pilot projects, CMMI sought to ensure that CMS paid for treatments in ways that are related to the benefits they provide to patients.

For example, one model involves paying less for drugs that receive accelerated approval from the FDA than for drugs granted traditional approvals. Accelerated approval, as described in the aducanumab case, is a special pathway through which the FDA can approve drugs based on changes to surrogate measures—laboratory testing, radiologic studies, or biomarkers like amyloid level—rather than changes to clinical outcomes that are of actual importance to patients (how they feel, function, or survive). Some surrogate measures can accurately predict clinical endpoints, but the accelerated approval program is designed for promising drugs based on changes to surrogates only reasonably likely to predict actual clinical benefits

[28] CMS. Innovation models. Available from: https://innovation.cms.gov/innovation-models#views=models.

with the requirement that they conduct post-approval studies to show an effect on those clinical measures. Because it is difficult for the FDA to follow up on its requirement for post-approval trials, these trials can be delayed.[29] In many cases, post-approval studies continue to test surrogate measures, providing unclear insight into the usefulness of the drug for patients.[30] In some cases, those post-approval studies have been negative—in the last 2 years alone, about 2 dozen accelerated approval-based indications of approved drugs have been withdrawn based on negative confirmatory studies.[31]

Thus, accelerated approval drugs are, by definition, approved based on having uncertain clinical effects and without a clear pathway for if or when any clinical benefits will be demonstrated. They are also invariably expensive, costing hundreds of thousands of dollars per year or more, because in the US we allow manufacturers to set their own prices for newly-approved drugs. Yet, aducanumab aside, nearly all FDA-approved drugs have been covered by Medicare Part B at the average sales price (plus a small additional amount), and accelerated approval drugs distributed through retail pharmacies generally must be covered by Medicare Part D plans, particularly if they fall in one of 6 protected classes, which includes cancer. For Medicare and Medicaid, accelerated approval therefore often becomes a pathway for a new product to enter the market, but also a mandate for government payers to cover high prices for unproven therapies.[32]

In this context, CMMI's demonstration project makes logical sense. If a drug is not yet shown to have clinical benefit, payment for it should be consistent with that state of the evidence. If new data come out, a fair pricing level can be reconsidered. But while the drug is FDA-approved based on limited evidence, patients and taxpayers should not be expected to pay whatever excessively high price the manufacturer decides it wants to set. As a secondary benefit, CMMI's model pricing structure could provide incentives for manufacturers to complete their post-approval studies in a timely fashion, helping garner needed evidence of the drug's actual clinical benefits to help better inform clinical decisionmaking.

CMMI's proposal to pay for cell and gene therapies involves helping coordinate and administer multi-state agreements that would be dependent on outcomes. This model is useful because multiple cell and gene therapy treatments have been approved in recent years and priced at eye-popping levels. Most recently, etranacogene dezaparvovec (Hemgenix) for hemophilia B (factor IX deficiency) was made available at $3.5 million. In addition, not all cell and gene therapies are fully curative; rather, some still require additional expensive treatments, and the effects may wane over time. Since evidence for the efficacy and durability of response is unknown at the time of approval, for gene therapies, payers are faced with the risk of paying too much upfront for unrealized benefits. For example, some patients initially respond to CAR T-cell therapy but then rapidly progress, requiring stem cell transplants or leading to death. Current payment approaches in the US for these products largely do not take outcomes into account, which is why CMMI's proposal is useful. It can help ensure that patients receive the potentially life-changing benefits of gene therapies when those benefits are meaningful, and try to ensure that payments for them are more closely linked to the clinical benefits they provide.

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[29] Deshmukh AD, Kesselheim AS, Rome BN. Timing of Confirmatory Trials for Drugs Granted Accelerated Approval Based on Surrogate Measures From 2012 to 2021. JAMA Health Forum 2023;4(3):e230217.
[30] Gyawali B, Hey SP, Kesselheim AS. Assessment of the Clinical Benefit of Cancer Drugs Receiving Accelerated Approval. JAMA Internal Medicine 2019;179(7):906-913.
[31] Perrone M. The FDA's speedy approval of experimental new drugs came to a screeching halt this year. Fortune December 7, 2022.
[32] Gellad WF and Kesselheim AS. Accelerated approval and expensive drugs — a challenging combination. New England Journal of Medicine 2017;376(21):2001-2004.

Finally, CMMI's third proposal to encourage Medicare prescription drug insurers to offer certain key generic drugs for a flat $2 copay can help promote medication adherence to essential medications for common, chronic conditions such as high blood pressure and diabetes. Medication non-adherence is common among patients with high out-of-pocket costs, and well-designed studies have shown that reducing patient out-of-pocket costs can improve adherence and important clinical outcomes.[33] Unfortunately, in recent years, some generic drugs have been subject to price increases for a variety of reasons, which can lead to changes in out-of-pocket costs.[34] Here again, as with the other two proposals, CMMI attempted to ensure that patients have access to meaningful innovation—in this case, essential generic medicines.

C. Ending the CMS MCIT Pathway Rule

In January 2021, CMS finalized a rule called Medicare Coverage of Innovative Technology (MCIT) that would guarantee up to 4 years of federal coverage for devices authorized by FDA under the Breakthrough Devices Program. The breakthrough program for medical devices has been available in pilot form since 2014 to expedite development and approval of certain high-risk medical devices for serious or life threatening conditions.[35] As codified in 2016, the FDA was directed to grant breakthrough device designation for devices (1) that provide for more effective treatment or diagnosis of life-threatening conditions and (2) which are either in the best interest of the patient, for which no alternatives exist, or that offer substantial advantages over alternatives. But in its subsequent guidance, the FDA announced its intention to apply these criteria broadly, for example, defining providing "for more effective treatment" as covering the manufacturer's "*reasonable expectation* that the device *could* provide for more effective treatment or diagnosis of the disease or condition" (emphasis added).[36] Guidance for other criteria also set low bars.

Perhaps not surprisingly given these lax criteria, large numbers of medical devices have qualified for this designation (222 in the program's first three years alone), with some that do not actually offer real clinical benefits to patients. In one review of breakthrough devices first made available from 2016-2019, investigators found breakthrough-designated devices FDA-authorized primarily via studies that used short-term, surrogate measures of effectiveness—which may not translate into clinical benefits, as with aducanumab—using safety data alone (without supporting evidence of effectiveness), and despite well-described serious safety risks.[37] The MCIT rule also included no requirement that additional post-approval studies of these devices be conducted as a condition of Medicare coverage.[38]

Ending the implementation of this rule was therefore consistent with the other moves described in these comments, albeit in the context of medical devices. The MCIT rule was a wrongly-conceived approach that would have forced Medicare to pay for ineffective or potentially dangerous device "innovation." By stepping back from the rule, CMS returned to its baseline requirement of covering new technologies that are

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[33] Choudhry NK, et al. Full coverage for preventive medications after myocardial infarction. New England Journal of Medicine 2011;365(22):2088-2097.
[34] Patel AN, Kesselheim AS, Rome BN. Frequency of generic drug price spikes and impact on Medicaid spending. Health Affairs 2021;40(5):779-785.
[35] Kesselheim AS and Hwang TJ. Breakthrough medical devices and the 21st Century Cures Act. Annals of Internal Medicine 2016;164(7):500-502.
[36] FDA. Breakthrough devices program: guidance of industry and Food and Drug Administration staff. December 18, 2018.
[37] Johnston JL, Dhruva SS, Ross JS, Rathi VK. Early experience with the FDA's Breakthrough Devices program. Nature Biotechnology 2020;38:933-938.
[38] Rathi VK, Johnston JL, Ross JS, Dhruva SS. Medicare's New Device-Coverage Pathway—Breakthrough or Breakdown? New England Journal of Medicine 2021;384(12):e43.

reasonable and necessary, rather than being forced to cover potentially non-useful new medical devices merely because they were given the FDA breakthrough device designation, which is not a consistent indicator of truly meaningful innovation for patients.

III. Future Steps

As these examples show, not only does the government fund some of the most transformative drugs we have, but it can also take steps to ensure that patients and taxpayers avoid wasting resources on drugs that are not meaningful innovation. This latter role is extremely important in providing the necessary incentives for the private market to also invest its resources in generating optimally useful innovation that offers the greatest benefit to patients. The current system in which Medicare and Medicaid—as the largest single payers in the market—too often end up reimbursing at unnecessarily high prices for low-value new products is one reason why there are so many unimpressive new prescription drugs and medical devices and so few truly transformative therapies.

There is also more than the government should be doing in this area to support the development of and payment for meaningful drug (and device) innovation for patients' benefit. First, under no circumstances should Congress be looking to reduce the NIH's budget. A bill that recently passed the House of Representatives reportedly cut the NIH's funding by $10 billion in fiscal year 2024, or about 20% of its annual budget.[39] This would devastate the prospect of future transformative drug development and doom prospects of future useful treatments in many areas of unmet medical need. Instead, the NIH budget should be expanded considerably—even doubled in size—and more funding dedicated to supporting pivotal clinical trials of NIH funded products that could be used to bring more such products through the final stages of the development process, as well as to post-approval comparative effectiveness studies in which drugs are tested against each other to determine which drugs are better for which patients.

Second, Congress should give the government more authority and leverage to reduce unnecessary spending on excessively priced pharmaceutical products that do not provide meaningful benefits to patients. For example, the Inflation Reduction Act (IRA) of 2022 for the first time vested in CMS the authority to negotiate prices for certain drugs based on their clinical value and other important factors. This is an important step to ensuring that the government pays fair prices for these products, but the bill is limited in that it only applies to a small number of products and has numerous exclusions, including drugs for which Medicare spends less than $200 million per year, drugs approved within the last 9 years (13 years for biologics), and drugs with one rare disease approval. Congress should build on this legislation to give CMS the authority to negotiate fair prices for all new drugs shortly after approval, as is done in all other industrialized countries.

Finally, the US should look for more ways to help ensure that patients and taxpayers only pay for meaningful innovation. For example, there is no national body right now in the US designed to help patients identify drugs with limited clinical value so that they can make informed clinical decisions about them. Congress should establish and fund a new expert panel to provide rapid-turnaround evidence-based reports on new drugs' added clinical value, pricing, and any potential disparities in access. Its recommendations could be non-binding, but the body would be tasked with issuing high-profile data-driven pronouncements on these issues regularly. Everyone who believes that marketplaces function best with more information should support such an organization.

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[39] Firth S. Democratic Senators rebuke cuts to NIH in House-passed bill. MedPage Today. May 5, 2023.

Chairman BUCHANAN. Thank you for—all of you for your testimony. We will now proceed to the questions-and-answer session, and I will begin.

Mr. Gonzales, what an incredible story. I just appreciate your courage and you being here today to speak on behalf of 6.7 million people.

Mr. GONZALES. Yes, sir.

Chairman BUCHANAN. You know, I have been impacted, you know, family member myself. So, I took care of my dad for almost 10 years. So, I know what that process of not just you are going through but your family and community and friends and everybody else.

I guess: What more can we do to help you? What would be the top priorities? I want to give you a little bit more time to talk about where, you know, kind of where we go from here. I know in terms of the drugs there is a third one out that has possibilities, and it seems like it is getting a little bit better.

But as someone said, you know, just taking the drugs, even if you get another 6 months of your life, if you start now, is a gigantic difference and there is a lot to be said for that.

But I just want to turn it over to you and give you a little time to talk us through that.

Mr. GONZALES. Sure. Thank you very much.

Yeah, 8 months. I wake up with one day. I wake up with one day. And so, you are looking at me and saying what does 8 months mean. Well, if you ever spent some time with me, you would see that in that one day I pack a lot. I pack time for family, friends, my community, my government, my religion in one day.

You give me 8 months and see what I will do. See what many of these people will do that are not getting access to these drugs, and they are slipping away every day.

So, for me, it means more time to be me again. I am already losing the memories. I am already losing who I was in my community. That is okay. Things have changed. But help me have a new beginning. This disease has changed. We are now in the era of treatment, and I need your help to take us the rest of the way.

Thank you.

Chairman BUCHANAN. Thank you very much.

Mr. Okon, you talked about a lot of different things. I wanted to get your ability just to expand on some of the thoughts that you had in terms of the patents and innovation and stuff.

Mr. OKON. Well, first of all, Mr. Chairman, I mean, you talk about the last decade, 10 years in cancer, it is remarkable. I have heard story after story. As I said, my wife, who was an oncology nurse for 10 years until 2019, when I asked her what was the breakthrough and she talked about IO drugs, she said it is just absolutely remarkable.

And we live in an era now where the understanding of the genetic background's access to biomarker testings are really allowing to us do more precision medicine.

So, my biggest fear is that when we talk about negotiating drug prices, in my world of cancer you are talking about a life cycle of drugs, that a drug being launched and introduced for one, maybe even two indications, and subsequent. In my written testimony, I

refer to a drug, Imbruvica, that was, over 9 years, had 11 different indications.

So, my biggest concern is that we keep the innovation going in cancer care and not stop the innovation—not on a particular drug—on the indications after it is launched.

Chairman BUCHANAN. Well, thank you.

I now recognize the ranking member, Mr. Doggett, for any questions that he might have.

Mr. DOGGETT. Thank you so much.

I would say, first, I appreciate all the witness testimony but certainly the courage that you show, Mr. Gonzales, in confronting this cruel disease and in being here as very forceful advocate today. I have a long relationship with the Alzheimer's Association, and I recognize the true desperation that many families feel about this.

I think that much of the research shows that a good way to get new cures is to invest in the NIH, the taxpayer-financed disease-specific research. There is some indications that a 10 percent increase in NIH disease-specific research yields a 4 to 5 percent increase in new drugs, and so I am particularly concerned that under what we have termed the "Republican Default on America" legislation that was approved a week guy that in a 22 percent cut in NIH funding is going deny us the very kind of cures that all of us today seek.

And as far as Big Pharma is concerned, my concern is that it often intimidates patients and disease advocacy groups, that anything that touches their bottom line, that prevents them from charging all that those who seek a little more time with their families, that they can charge whatever the market will bear. I think paying outrageous drug prices hasn't resulted in innovation. In fact, I think it has had just the opposite effect.

Dr. Kesselheim, you have cited the enormous contribution that NIH funding research has had. Can you just speak to the differences in research conducted by manufacturers who purely have a profit motive and the research that is being funded by the American taxpayer who has a strong interest in meeting public health needs?

Dr. KESSELHEIM. Sure. So, the NIH tends to fund a lot of the early stages in drug development. As you pointed out, every single drug can ultimately trace its origins back to NIH funding and basic and translational science.

But what we have found actually in some of the research done at PORTAL is that a lot of the most transformative, most important drugs are also, can also be linked to public funding in their later stages of development, as well, from the—from testing the original—from the original testing on the product, even to some of the clinical trials, as well. That tends to be where—where manufacturer funding of new drugs tends to predominate in those later stages.

The risk is less. And actually as the trials get larger and larger, the risk gets smaller and smaller. And a lot of industry funding also goes into making small changes to drugs after they have already been approved to extend their market exclusivity on the underlying active ingredient as long as possible.

Mr. DOGGETT. You know, as we have heard today, whether it is Alzheimer's or ALS or cancer, there is a desperation to get these new cures. I think that patients deserve a system that generates innovative research.

But I would just ask you about the accelerated approval process and whether that is providing false hope in many cases or is providing real hope for cures?

Dr. KESSELHEIM. I think that the accelerated approval pathway is a useful pathway when used correctly. It provides, you know, early access to very promising treatments on the promise that they will eventually do meaningful clinical testing.

I mean, you mention the word "cures." I think we all wants cures or meaningful innovation. The problem with accelerated approval drugs is that a lot of them are, when they aren't given accelerated approval, we don't actually know what they do. There is some suggestion there. There is some promise there. They need additional testing. They are not the same as drugs approved on the basis of showing changes to actual clinical endpoints like many traditional approval drugs are.

Mr. DOGGETT. While my focus has been principally on drug pricing today, this hearing also, of course, deals with the question of medical devices. Through the years we have had some bipartisan concern about medical device safety. I have worked with colleagues from this committee, like Bill Pascrell and Brian Fitzpatrick. Senator Warren and Senator Grassley have sought greater accountability on post market surveillance of safety concerns.

Dr. Kesselheim, knowing of the negative repercussions of misaligned incentives and Medicare reimbursement, as well as safety and efficacy concerns that arise from these devices, do you think it is appropriate for Medicare to guarantee 100 percent coverage of so-called breakthrough devices?

Dr. KESSELHEIM. It is not. Coverage in Medicare should be what is reasonable and necessary. That is not the same thing as the breakthrough therapy designation which is given by the FDA at extreme—at sometimes extremely early stages of device development when we don't actually know what effect the device will have on patient outcomes.

Mr. DOGGETT. Thank you very much.

Thank you, Mr. Chairman.

Chairman BUCHANAN. Mr. Smith of Nebraska.

Mr. SMITH of Nebraska. Thank you, Mr. Chairman.

Thank you to all of our witnesses.

Mr. Gonzales, thank you for sharing your story here today.

I do want to associate myself with the concerns that Chairman Buchanan expressed regarding the TRIPS waiver. As chairman of the Trade Subcommittee, this is obviously an important issue that is of great concern to me, as well.

However, in the interest of time, I would like to focus on my concerns with the CMS Innovation Center, known as CMMI. Tasked with testing payment and care delivery models in order to saved Medicare money and improve patient care quality, CMS has tested more than 50 models since its creation.

Despite billions of taxpayer dollars spent setting up and evaluating these models, only six of these were found to have delivered

statistically significant savings, actually a less than 12 percent success rate. Instead, these models have been used to make major, often controversial changes to fundamental parts to the Medicare benefit such as part B drugs, kidney care, oncology, and more, often generating bipartisan concern.

For example, I do have a copy of a June 2021 letter which Congresswoman Terri Sewell and I sent to CMS, along with 247 bipartisan co-signors, expressing concerns with the lack of transparency and stakeholder participation in CMMI's model development process.

Mr. Chairman, I would request this letter be inserted into the hearing record.

Chairman BUCHANAN. So, moved.

Mr. SMITH of Nebraska. Thank you.

Congress of the United States
Washington, DC 20515

June 2, 2021

Elizabeth Fowler
Deputy Administrator and Director, CMS Innovation Center
Centers for Medicare & Medicaid Services
2810 Lord Baltimore Drive
Baltimore, MD 21244

Dear Deputy Administrator and Director Fowler:

With this letter, we are reasserting and clarifying our commitment to the Center for Medicare and Medicaid Innovation (CMMI) which is intended to test different innovative delivery system and payment models to improve quality in providing care to Medicare and Medicaid beneficiaries. We also believe there is room for improvement regarding its authority and obligations, particularly pertaining to the scope and duration of demonstration projects and the transparency of its actions and decision-making processes. In this regard, there are bipartisan concerns.

We note that the authorizing statute requires the gathering of "input from interested parties." However, adequate consultation and transparency in the processes used to develop these experiments are rarely observed and CMMI demonstrations are less effective than they could be for the lack of this external expertise. We believe that CMMI could strengthen its model development by allowing more stakeholder engagement. Further, Congress and the public need to know how results will be sampled and evaluated and which beneficiaries stand to be affected. The Department of Health and Human Services needs to reveal the modeling which produces estimates of savings and how quality will be affected. Consistently, modeling has been biased toward savings rather than improving beneficiary health or addressing health disparities. Stakeholders need to know what analytics and standards are used to define a successful demonstration.

As we look toward the future of CMMI, we believe it will be stronger with greater transparency and increased participation from stakeholders. We believe in greater use of real-time data to immediately understand the impact of models on healthcare providers and patients so that decisions can be made quickly about the value of a demonstration. And we insist CMMI's actions reflect its intended mission, to carry out demonstration of projects of limited scope and duration to test new payment and delivery concepts.

We kindly request that you share your plans for making CMMI a more transparent and we would like to learn more about how you will develop models that focus on measurable cost savings, address beneficiary health, and reduce health disparities through models that are both appropriately scoped and can be adopted or abandoned based on their impact. We look forward to working with you to ensure CMMI is effective in designing and assessing innovative delivery system models which will improve quality for Medicare and Medicaid beneficiaries and reduce health system costs.

Sincerely,

Congress of the United States
Washington, DC 20515

Terri A. Sewell
Member of Congress

Adrian Smith
Member of Congress

/s/
Colin Z. Allred
Member of Congress

/s/
Steven Horsford
Member of Congress

/s/
Gus M. Bilirakis
Member of Congress

/s/
Sheila Jackson Lee
Member of Congress

/s/
Vern Buchanan
Member of Congress

/s/
Bill Johnson
Member of Congress

/s/
Salud Carbajal
Member of Congress

/s/
John Joyce, M.D.
Member of Congress

/s/
Tony Cárdenas
Member of Congress

/s/
Ron Kind
Member of Congress

/s/
Ed Case
Member of Congress

/s/
Stephanie Murphy
Member of Congress

/s/
Yvette D. Clarke
Member of Congress

/s/
Tom Rice
Member of Congress

Congress of the United States
Washington, DC 20515

/s/
Brian Fitzpatrick
Member of Congress

/s/
John H. Rutherford
Member of Congress

/s/
Jan Schakowsky
Member of Congress

/s/
Jason Smith
Member of Congress

/s/
Kurt Schrader
Member of Congress

/s/
Darren Soto
Member of Congress

/s/
Debbie Wasserman Schultz
Member of Congress

/s/
Paul Tonko
Member of Congress

/s/
David Schweikert
Member of Congress

/s/
Brad Wenstrup, D.P.M.
Member of Congress

I have also worked on legislation which would create some commonsense guardrails for CMMI to ensure the design, testing, and expansion of these models is in line with congressional intent. This legislation has been bipartisan in the past.

I introduced the first version of this bill during the Trump administration, proof that these longstanding concerns are not tied to a specific President or one particular model. I hope we can continue working on that legislation in a bipartisan fashion.

Mr. Okon, your organization works with patients who have been impacted by CMMI models in the past. Based on your experiences, what do you feel are the most necessary guardrails to ensure the integrity of model testing without unnecessarily hurting beneficiaries or providers?

Mr. OKON. Yeah, Mr. Smith, this is, as I said in my opening statement, we have now three administrations that have basically have gone over to what I call the little toy box called CMMI and pulled out and basically tried to basically end-run all of you in Congress to change drug reimbursement. And I think that is extremely, extremely dangerous.

And I think that—so when you talk about guardrails specifically, if you go back and read the law, the ACA that created CMMI, the whole concept was that you would do a limited phase 1 model, that then if it worked and saved the money and didn't hurt patients' enhanced care, that you would do an expanded phase 2 model. That is not happening. This is, if you look at the President's recent executive order, it is let's go use CMMI to change drug pricing.

So, I think there are a lot of guardrails. I think what you did, and Ms. Sewell, it just this should be duplicated again. And the entire Congress should put guardrails on CMMI so that it is not a vehicle to end-run the Congress. It is a true vehicle to test innovation.

And one more thing. I was the biggest proponent of CMMI when it was created, the idea of having an Innovation Center in CMS. But I don't think it has upheld that charter.

Mr. SMITH of Nebraska. Thank you. I appreciate your insight.

Moving along here, I apologize for the brevity of our time. One of the healthcare sectors which stands to benefit the most from new and emerging technologies is care delivery at home. We know the care in the home allows patients to receive necessary care close to their families and caregivers without needing to worry about transportation, whether it is dialysis, other innovative approaches.

So, ultimately, Dr. Lakdawalla, can you walk us through how innovators would factor potential Medicare coverage of a breakthrough product into their research and investment calculation and how that could be applied in home care, as well?

Mr. LAKDAWALLA. Sure. Thank you for the question.

I think part of what—part of the uncertainty that we face right now regarding the incentives for innovation is how CMS is going to think about setting maximum fair prices. And we don't know very much about it. My hope is that CMS will employ modern economic methods, to include value to patients, as part of their maximum fair price assessment. And, if it does so, then the kinds of issues that you raise, Congressman, would absolutely be part of the calculus regarding value to patients.

Alzheimer's disease is a salient example here. It imposes considerable burdens on patients and families outside of what you might consider traditional healthcare spending. Those kinds of impacts in terms of caregiver burden, transportation, disruption to lives, are all part of value. And it goes to the question of paying more for more valuable technologies than paying less for less valuable technologies.

Mr. SMITH of Nebraska. Okay. Thank you.

I yield back.

Chairman BUCHANAN. Mr. Thompson, California.

Mr. THOMPSON. Thank you, Mr. Chairman.

And thank you to all of our witnesses for being here.

And Mr. Gonzales, thank you very much for your very compelling testimony. And I think everyone would agree that we need to do everything we can to make sure patients get the medications that they need and that will make them healthy.

I would like to start by reminding folks that the Inflation Reduction Act, which we passed in the last Congress with no help from our Republican colleagues, reduced the deficit by $300 billion over 10 years. And the drug price negotiation provisions in that bill saved taxpayers $288 billion. We also capped the price of insulin at $35 a month and capped seniors' out-of-pocket costs at $2,000 per year.

I am not sure what my colleagues who voted against this bill hear from their constituents, but I can tell you the seniors I hear from at home are pretty darn happy with these changes.

I would also like to just make a couple of very obvious facts known: One, you can have the most exciting innovative drug in the world, but, if no one can afford to buy it, it doesn't help a single person; and, two, Americans pay more for the same drugs than people in other countries do; and three, in every other industrialized country, the government negotiates drug prices with manufacturers. They do not just take whatever price the manufacturer wants.

And that is where I would like to begin my questioning.

Mr. Kesselheim, or Dr. Kesselheim, you talked about the importance of funding the National Institutes of Health. We have heard a lot today about how high drug prices are apparently necessary to fund research and development.

Can you talk a little more about how the research taxpayers fund at NIH has helped pharmaceutical companies develop their products?

Dr. KESSELHEIM. Sure. The research that goes on at NIH is fundamental to drug development and manufacture—it helps identify targets. It helps identify the origins of disease. It helps identify the systems and create testing systems in which drugs can be tested. All of that information is then used by manufacturers when—you know, in developing particular products or moving particular products forward.

Sometimes NIH funding even supports clinical trials and proof of concept. So, the NIH funding does a lot of work in developing and leading to drug development, particularly the most important drugs that we have.

Mr. THOMPSON. Thank you.

You also, in your testimony, stated that we should expand, not repeal the negotiation provisions that we passed in the Inflation Reduction Act?

Dr. KESSELHEIM. That is right. We—right now, the negotiations in the Inflation Reduction Act occur at about 9 to—or implemented at about 9 to 13 years after the drug is approved. And, you know, as you mentioned, in every other industrialized country, the prices are negotiated at the time of—near the time of drug approval.

Those prices can be negotiated fairly, such that important, meaningful—clinically meaningful drugs are given a substantial reimbursement. But, most importantly, the—a lot of drugs out there do not offer added clinical benefits. Those drugs can be—the prices of those drugs can be restrained to where the—and negotiated to a point where they are more reflective of the actual value that they provide.

Mr. THOMPSON. So just a little more on criteria. If we were to expand drugs eligible for the negotiations, what sort of criteria should we use?

Dr. KESSELHEIM. I think that all new drugs should be eligible for negotiation within a year of their being first approved by the FDA. That would be the most fair way of going about it.

Mr. THOMPSON. And, if we have to do them piece by piece, are there drugs that are unfairly priced or drugs that are transformative for patients—should they be moved to the head of the line? How do you work all of that out?

Dr. KESSELHEIM. Well, right now—right now, the way that it is done in Germany, for example, is that all drugs—the price for the drug is set by the manufacturer, and that is the price for the first year. And, during that first year, all drugs are—go through an evaluation process to determine how clinically meaningful they are. And then, at the 1-year point, the drug is negotiated in line with that—with that clinical meaningfulness.

So, I think that that is—that is a model where you are not blocking drugs from getting on the market. Drugs can get on the market. Patients can get access to them. And then what we eventually do is, very soon thereafter, figure out what the fair price of those drugs should be.

And I think that in taking into account that fair price, you definitely need to account if the National Institutes of Health or some other public entity was a substantial contributor to the funding of those products and de-risked the investment that the subsequent manufacturers made in them.

Mr. THOMPSON. Thank you very much.

Yield back.

Chairman BUCHANAN. Mr. Kelly of Pennsylvania.

Mr. KELLY. I thank the chairman. And thank you all for being here today.

It is interesting, because we ask you all to give up a day of your life to come in and talk to us. And then you have 5 minutes to try to get out what you have already presented to us in writing. And then we try to hurry up and ask you a question.

So, Mr. Okon, what you all do and the doctors you work with are incredible.

Dr. Wenstrup and I were talking. It would be good to get some actual operators, doctors who work through this every single day.

As a Hyundai dealer, I am involved in something called Hope on Wheels. This is an effort between Hyundai Motor America and Hyundai Motor dealers. For every single Hyundai that is sold, there is a contribution that is made towards the development of or the eradication of childhood cancer, with the goal being that no parent, no family ever has to hear that your child has cancer.

So far, we have raised about $225 million, which is significant, but not near enough. And so, I look at what it is that we are trying to do. Well, President Biden shares the same goal we all have. We don't want anybody to have to suffer.

Now, his Cancer Moonshot Initiative is really admirable. And that is why I am so concerned that, at the same time he was relaunching the Moonshot, the administration was taking major steps to devalue the accelerated approval pathway for new drugs coming onto the market.

Now, CMS did this first with Alzheimer's drugs. Then CMS Administrator Brooks-LaSure said she viewed accelerated approval as a—as being separate from traditional approval. About 85 percent of all drugs that go through the accelerated approval program are cancer drugs. So, if CMS is successful in expanding the Alzheimer's precedent to other categories of drugs like cancer drugs, patients will see their success to these innovative, new, lifesaving cures severely restricted or even cut off altogether.

So, Mr. Okon, what effect will this have on cancer patients? I am—specifically, the children that I have seen.

Mr. OKON. It is so important that cancer patients, because of the nature of this disease—it is not cancer, singular. It is over 200 cancers. And Mr. Kelly, when you look at certain cancers like breast cancer, there is HER2-positive, HER2-negative, there is adjuvant, there is metastatic. And so, we have got to get away from the notion that this is a cookbook.

We know now more about the genetic profile. We have biomarkers that allow us to do more precision medicine. And what may work on one individual who looks and talks like another individual, the drug may work on one and not the other.

And I am particularly concerned about pediatric cancers that treatments typically get developed after, in the lifecycle of the drug, adult cancers. And, again, I go back to saying that, if you are facing and you are a manufacturer—if you are—you are a businessman. You are facing putting more money into research and you know the drug is going to get negotiated downwards, it is a problem.

And I think one of the fundamental problems that you hear here is that there are very different drugs that we are talking about. When you talk about Alzheimer's, when you talk about cancer, that is very different than other areas of medicine, and that is what is so important.

So, it is—it is alarming.

Mr. KELLY. It is alarming. And, you know, the size and scope of the government is incredible. And trying to work your way through it is almost impossible. I admire all of you for what you do and the frustration that you must face every single day when

you are trying to help people and cure people and knowing that the process you are going to go through is oftentimes more difficult than the answer you are trying to find.

I think, too often, we concentrate on the cost of things and not on the effect of things. I wish we could get this reversed, but I don't know. I think it would be wonderful if, not just in this committee, but in all of the Congress, we could concentrate more on policy and less on politics. I think the answers and the developments would be incredible.

I want to thank you all for being here.

And you have given—Mr. Gonzales, you give a very inspiring time—the best time I have spent is with my grandchildren. I am hoping that, sometime in the future, they look back and say the best time they spent was with their grandfather.

God bless you. Good luck with everything.

And with the rest of you, thanks so much for what you are doing. We appreciate you being here today.

I yield back.

Chairman BUCHANAN. Mr. Blumenauer of Oregon.

Mr. BLUMENAUER. Thank you, Mr. Chairman.

I think we are looking at different aspects of this challenge. One of the things that hasn't been focused on here is that we are forcing American consumers to pay the highest drug prices in the world, assuming that this filters out in terms of innovation.

Dr. Kesselheim, you point out that the majority of the innovations are more engineering patents, not new medicines. They are repackaging so that they can expect to gain more value over time.

This high cost of medicine is driving this showdown that we have got over the deficit that is encouraging my Republican friends to vote for a 22 percent reduction in the National Institute of Health.

We have got to get a handle on exploding costs, and it just seems to me—Dr. Kesselheim, you highlight some of the problems associated with rushed approval without showing benefits. You talked about the brain—you want to talk a little bit about the danger of giving people medicine that hasn't been fully vetted and shown that it provides benefits for people?

We don't want to give false hope to folks if their brain is going to swell or something like that.

Can you elaborate on part of what you put in your testimony?

Dr. KESSELHEIM. Sure. So, as I think that that is a fundamental role that the FDA plays in this process, is to try to make sure that, when drugs are approved, that they are—that there is clear effectiveness that those drugs will have, and that those benefits outweigh the risks of those drugs.

I think that the FDA, unfortunately, did not do its job in the case of Aducanumab because of the lack of clear evidence of benefits and the substantial risks that were associated with those drugs, including the risk of brain swelling and bleeding in up to 40 percent of patients who received that drug.

And so, I think that, in that context, CMS did the best that it could by saying, Look, we are only going to pay for this drug if it is being tested to show if the drug actually works in the first place. I thought it was a totally reasonable approach given the fact that the FDA made a bad decision in approving that particular drug.

And that, again, is why we have a process in which we want—we need to gather evidence about drugs and new devices, because they can be so dangerous. They can be so effective and so useful and transformative, but they can also be very dangerous. And that is why we need adequate testing of them.

And, you know, the FDA—when the FDA is given flexibility, as in the accelerated approval pathway, to approve drugs before they are shown to have benefits, then we need a clear pathway to generate those after approval. And, you know, I think that that is what the CMMI proposal is intended to do, is intended to limit—limit spending on those drugs until we actually know whether or not they work, and then, of course, the price can be, you know, raised to the appropriate level.

Mr. BLUMENAUER. In your testimony, you talked about 81 top advertised drugs, that only 27 percent of them were demonstrated of having high therapeutic value.

Dr. KESSELHEIM. That is right. So, we are—we are deluged. So, as doctors, you know, doctors receive a lot of promotion of drugs. But the consumers are also deluged with drug advertising. And, in a study that we recently did and published in the JAMA Network, we looked at all of the top advertised drugs and found that only 20 percent of them were shown to have added clinical value to patients.

And so, really good important drugs sell themselves. Doctors will prescribe them and use them, and patients will ask for them. And so, that is—I think is why we see a lot of direct-to-consumer advertising that involves drugs that don't have a lot of added clinical value.

Mr. BLUMENAUER. I think this is very important. The pharmaceutical industry spends more money on advertising than they do on research. We need to be sure that we are getting high value.

We are in the process of having this battle over the deficit. What we are talking about here is an opportunity to be able to rein in some of these extreme costs, to be able to give more value to the taxpayer, and not give them medicine that will give them false hope, or worse, even be dangerous.

The American public is paying for the research for around the world, and it is doing so in a very inefficient fashion.

And I appreciate, Doctor—it is not just because you are wearing a bowtie, but I appreciate what you put in your testimony talking about the downsides of rushing, undercutting a process to make sure that it actually has value and holding the industry accountable. We have all got experiences in our family of people who have suffered, for example, with Alzheimer's.

I don't want to give false hope. Worse than that, I don't want to give medicine that will do damage, or that we are going to end up cutting services and research because we haven't been able to do our job right.

I yield back.

Chairman BUCHANAN. Dr. Wenstrup, Ohio.

Mr. WENSTRUP. Thank you, Mr. Chairman.

Thank you all for being here today.

You know, it is discouraging to see at any time if we are doing things that inhibit innovation and limit patient access to the latest

treatments or discourage investment in new technologies and cures. As a physician, I know how—I know firsthand how dangerous it is to delay or deny access of proper treatment to a patient. And I think Americans deserve better than that. That is the system we are living in.

Dr. Kesselheim, you mentioned NIH. We have a Doctors Caucus here. We have been out to NIH. They do some wonderful things. There is no doubt about it. We have been very supportive of NIH in many, many ways. But it doesn't mean we shouldn't have oversight over everything that they do and where the money is being spent. And no one is that Godly, okay, that they can't be questioned on the type of research they do.

And you mentioned the difference. We have had great things come out of the commercial industry, great things come out of NIH. But I will tell you what commercial industry has not done. They haven't funded research to create viruses that become more infectious to human beings and kill millions of people, okay? So, there is a difference there sometimes, and we have to watch over that as a country.

But I will tell you, we as a Doctors Caucus, were meeting with CMS about 8 years ago, and they are telling us how great everything is working now, the things that they are implementing.

I looked her in the eye, and I said, do you know why we are here? Do you know why we are in Congress? Because you have taken the joy out of taking care of people.

And that is what has happened over time. You know, I hear my colleagues saying, I don't want to give anybody a medicine. You don't give anybody a medicine, my friend. Doctors do, and patients decide. They do this together.

And so, what is really missing with some of the things that you are talking about, it takes away the power of hope. And I don't mean false hope. When you have good bedside manner, you talk about all the odds, and maybe this will help, and maybe it won't. And that is fair. And it is called bedside manner.

But that is what is missing in these discussions up here. It is totally missing. We don't talk about the value of prevention up here. We don't talk about the value of cures and the savings. We don't talk about the value of someone's life. It is all dollars and cents. That is all it is.

We don't talk about how someone may live longer and continue to go to work and pay taxes, right? We don't talk about the value of productive life because this medicine, even though you might have a chronic disease, is allowing you to live a life. And I know you understand this, Mr. Gonzales.

And doctors that sit there every day with patients in front of them and look them in the eye and talk to them, they understand it. The people that wear the white coats, not the ones writing the white papers. I am sorry.

So here we are. You know, it is—that we—look at MCIT, the MCIT rule. It has been more than 2 years. The Biden administration has not proposed a replacement rule for this. If you didn't like it and you canceled it, fine, but tell us why. And tell us what data you used that said you needed to cancel it. And there has been no discussion on this since.

So, I am proud to work with Representatives DelBene, Blake Moore, Terri Sewell, to introduce the Ensuring Patient Access to Critical Breakthrough Products Act. It is bipartisan, a bill that would codify the MCIT rule and give millions of seniors a chance to live longer, healthier lives while supporting the companies and innovators who are investing in these critical medical technologies and devices.

Mr. Okon, one of the most important reasons for providing this pathway is to give patients access to technologies that will improve their health and extend their lives. Mr. Okon, what does further delay in the MCIT rule mean for patients who are waiting for the next novel treatment?

Mr. OKON. Well, again, I can say, Dr. Wenstrup, is that cancer patients, because of the knowledge that we have, as you know, about so much more of their makeup, they depend on innovation. They depend on new drugs. And so—and especially when you look at, you know, some of the rare cancer, pediatric cancer. So, I applaud all of you. I applaud anything bipartisan. And the idea that—that this would push the MCIT, I think, is absolutely key and certainly important in cancer care.

Mr. WENSTRUP. And it is upon us to make sure that something like this——

Mr. OKON. Yeah.

Mr. WENSTRUP [continuing]. Is working and doing what it is intended to do, not just say, it is okay, let it go.

And, Dr. Makower, how are the current challenges around Medicare coverage impacting investment into breakthrough technologies, and what impact could a bill like the Ensuring Patient Access to Critical Breakthrough Products Act or a new CMS rule have on the development of new technologies?

Dr. MAKOWER. Thank you for the question.

I think that it is often lost on people that most of the new and novel medical technologies are actually created by very small venture-backed companies that rely on investors to support their work.

During the time that there was a belief that MCIT actually was going to be put into place, there was an amazing wave of enthusiasm and investment that went into breakthrough therapies that could really make a difference in people's lives, areas like diabetes, heart disease, very challenging and very difficult problems to solve. But given the encouragement that there would be a bridge to somewhere, an opportunity to bring their products to patients on the other side, that investment was spurred.

When the MCIT was cast aside, there was definitely an impact in the industry and in the innovative community. And I would say, as our survey indicated, the impact of not having a clear pathway to coverage and reimbursement on the other side of all the work that goes into demonstrating that a product is safe and effective with the FDA, is a real depressing factor for further investment in very important therapies for patients.

Mr. WENSTRUP. Thank you. Yield back.

Chairman BUCHANAN. Mr. Higgins, New York.

Mr. HIGGINS. Thank you, Mr. Chairman.

Drug development, you know, firstly, is a long, drawn-out process. You know, on average, it takes 10 to 15 years to develop fully

a drug. It is a public-private partnership. The Federal Government is typically involved in the front end, which is much less profitable. And then, when those drugs reach a point of going into clinical trial to test both safety and efficacy, the pharmaceutical industry becomes involved, and that is the profit-earning phase of drug development.

You know, for example, the messenger RNA, which is the genetic material that tells or instructs a cell to make a protein, which was the active ingredient in the mRNA vaccine, was a result of decades of drug development financed by the Federal Government. So, the Federal Government isn't in the way. It is really leading the way. And that has to be acknowledged.

You know, you think about it, in the first 7 months of COVID, the best thing that our healthcare system could do to somebody that was stuck with COVID is to give them Tylenol to reduce pain and fever.

These drugs were developed, and they accrued to the great benefit of the private sector because of Federal Government-financed basic research.

For example, Moderna, which developed one of the messenger RNA vaccines, pre-COVID, was $20 a share. At the peak of COVID, it was $497 a share. So, it is always a public-private partnership. And virtually, every drug that came to market in the last 10 years, the Federal Government had a major financial role in bringing that drug to market. It doesn't really get any profit from it. It just does it because it is the right thing to do.

So, you know, the whole idea of, you know, being critical of the Federal Government, I can see, Mr. Gonzales—you provided very compelling and thoughtful testimony. I thank you for that. But I think we need to understand the role that each has. And, you know, it is—you know, all these horrible chronic diseases, the pharmaceutical companies spend billions of dollars in advertising. And you watch those commercials. Everybody is happy. It is sunny. It is great news, and everybody is good looking.

But the idea is to get consumers to say, Yeah, I want that because I want to look like that, I want to feel like that. And sometimes it is not the best treatment for an individual.

The other thing is, you know, innovation, by its very definition is inefficient. Ninety percent of clinical trials fail. So, the only failure in drug development research is when you quit, or you are forced to quit because of lack of funding. So, let's recognize the important role of both the Federal Government and the private sector.

Dr. Kesselheim, you noted in your testimony that the Republican bill just passed the House on the debt ceiling would result in significant cuts to the National Institutes of Health. That is the—exactly the opposite of what we should be doing right now. We should be increasing our investment in medical research, not slashing it.

Do you care to offer some thoughts?

Dr. KESSELHEIM. Yes. I think—I completely agree. I feel like, no, we should not be cutting the NIH budget, we should be doubling the NIH budget, because there is a long track record of success of the NIH investing in transformative drugs. And so, I think that, if we provide more opportunities for that, then we will get

more drugs for unmet medical need, and—you know, and we will be able to help patients better that way.

Mr. HIGGINS. Anybody else?

Mr. GONZALES. For me, I heard a few things today, and I will try to explain this as best as I can.

Meaningful and necessary. What is meaningful and necessary continually comes over and over in the data and the things that I see in the press. And I think I have demonstrated and talked to you about what is meaningful and necessary in my life.

But let me ask you: What is meaningful and necessary in your life? What if this were you? I am not much older than you, and you are older than me.

The government should not be having this conversation with me. This should be between myself and my physician. The fact that I have to travel across this country of ours, this great country of ours, and deal with this disease, not knowing where I am at, having my wife near me every single time, being cold, shaking, shivering, this needs to be between a patient and their doctor, period.

That is what I need. That is what I want.

Well, it was my understanding the accelerated approval was created to give people with unmet needs—it helps the innovation that they deserve. So, what the heck are we doing?

I am here, yes, to tell you, my story. But think about those—while you are thinking about cost, and you are thinking about finance, let me let you in on what to think about. I don't get to have a checkbook anymore, sir. I don't get to have money with me anymore, because I can't be trusted with it, because I can't do the math anymore. I have a first-grade-level math, and I served as a CEO. I served well in business, real estate. And now—you can look at me. A first-grade-level math.

What is reasonable and necessary is that we need this to go between the patient and the doctor.

Thank you.

Mr. HIGGINS. Yield back.

Chairman BUCHANAN. Dr. Murphy of North Carolina.

Mr. MURPHY. Thank you, Mr. Chair.

I will just reiterate, Mr. Gonzales, patient-doctor, right?

Mr. GONZALES. Yes, sir.

Mr. MURPHY. Because what the last administration did with the whole damn vaccine fiasco was made it between a government and a citizen. I will just—I will go back to that. We were pro-vaccine, but dammit, everybody didn't need it. And it took the power of doctors of prescribing away.

All right. I will get back to this issue because I think we have just kind of gone on a little bit disarray here.

I will join every single Republican and Democrat here. We need to cut the cost of medicine. And, if we can pass a bill that gets rid of direct-to-consumer advertising, I hope we can make a unanimous vote, because I will tell you I have never, in my 30 years of prescribing, to this day, ever prescribed anything because of seeing people on the television.

And patients will come in every so often and say, What about this drug?

I said, this drug is nice, but you don't have that disease, okay?

So, it provides no benefit. We are one of two countries in the world—New Zealand is the other one that provides direct-to-consumer advertising. So, let's get rid of that.

Let's attack PBMs, which have become an absolute parasite and extorted moneys from patients and pharmaceutical companies just to the expense of bottom line of insurance companies. Let's have some meaningful legislation on that. Let's really get to the problem of this in the United States, because no other country does that. They don't have direct-to-consumer advertising or PBMs.

So, guys, you know, it just—it kills me here. I think our Democratic colleagues started out with well-intentions, but they did not think about the secondary, tertiary, quaternary consequences.

Yeah, Moonshot Cancer is great. But, if you ain't got no fuel for the rocket, where is it going to go? If you can't do anything with that, it is not going to work.

You know, Dr. Kesselheim, let me ask you a question: Do you know how much it costs to bring one drug to market—one molecule?

Dr. KESSELHEIM. There are a lot of varying estimates of that.

Mr. MURPHY. On average.

Dr. KESSELHEIM. The estimates in the literature range from anywhere, on average, from a few hundred million dollars to the pharmaceutical industry estimates of a few billion dollars.

Mr. MURPHY. I say mostly 2 to 2.5 is the most common thing I see quoted.

For every molecule that comes to market, how many molecules are—go into that—go into the development of that drug, do you think?

Dr. KESSELHEIM. Well, so there—again, it happens at different stages.

Mr. MURPHY. Sure.

Dr. KESSELHEIM. In terms of the beginning of clinical trials, there are about—there is about 10 molecules for everyone. But, for the most expensive, later-stage clinical trials, about half of drugs tested are approved. So, two to one.

Mr. MURPHY. So, I have seen numbers higher than that, but I am not going to argue with that.

So, we have a portion here where so much money and so many scientists are working on molecules with the hope of therapy. So many of our drugs do not start out doing what we think they are going to do.

You know, you look at actinomycin for Wilms tumor. It was an antibiotic, by God. Same thing with all the other things. This is how we developed penicillin. Nobody knew that. It is accident.

So, walk me through—Keytruda was started in 2000, I think, 2014, right? Came out for the indication of melanoma. What happens now, because it is—now has an indication for small cell, the lung, melanoma, lymphoma, rectal tumors, GI tumors, other tumors, walk on and on and on.

So, when we have these new indications for a drug—and I can walk you through about 10 of these drugs for that—I deal with prostate cancer, and Xtandi has done the same thing. I have seen such a miraculous change in 10 years from people I normally said,

you had to go get your affairs in order, to say, Hey, you are going to see your grandkids live.

So, as we walk through these indications, all of a sudden, we hit a wall—an artificial wall that has been put up because the IRA says, Nope, you can't explore this anymore, even though they could possibly cure one other thing and one other thing.

And you know where it is going to hurt the most? It is in pediatric diseases, pediatric cancers, because you know what? We have to experiment on adults first. And, if we are not allowed to do indication upon indication to try to push things forward because of some artificial barrier, we are going to kill kids in the future.

You look at what has happened with Wilms tumor, you look at what happens with childhood leukemias—things that are absolutely curable today. But they would not have happened if we had not been able to march forward.

Yes, I want to cut drug costs as much as anybody. You know the $35 insulin? It is just like a balloon. You push in it here; it is going to push out somewhere else. That is the fallacy that is being told to the public.

And, yes, I know drugs that don't work. Tell me about the 27 percent of drugs that you don't think—excuse me—the other 67 or 63 percent that don't work. Where are the—how do you define limited clinical value?

Dr. KESSELHEIM. So, I said limited added clinical value. A lot of those drugs are drugs that do the same thing as drugs that are already on the market, or drugs that are generic that are already on the market.

Mr. MURPHY. And you know that some people react different to every medicine.

Dr. KESSELHEIM. Of course.

Mr. MURPHY. And I will use epilepsy for an example. Somebody walks in your clinic, you can throw 50 drugs up on the wall, and if—50 different people will react different one every time. But, if you have added clinical benefit, you are going to pull away about 90 percent of those and say, well, if you fail this, you fail this, you fail this, good luck, you will never drive again.

Dr. KESSELHEIM. But I wasn't saying you shouldn't approve those drugs. I was just saying you shouldn't pay more for them than the other drugs that are already on the market that work the same way.

Mr. MURPHY. Yeah, but if you say that, for example, like Germany, they see one year and say which one they are going to ratchet that down. You can't determine data in one year whether something works or not. That doesn't—that just doesn't give you nearly enough time to determine clinical value.

So, yes, there are things we can do to try to cut drug costs in this country, absolutely. But this is an asinine plan to do it, and I think it is going to hurt patients. We have already had drug lines taken off—clinical lines taken off because the pharmaceutical companies won't expend interest or won't expend money because they know they won't be able to recoup it.

Thank you.

With that, Mr. Chairman, I will yield back.

Chairman BUCHANAN. Mr. Hern, Oklahoma.

Mr. HERN. Thank you, Mr. Chairman, for hosting this hearing on innovation in healthcare.

I thank our witnesses.

Mr. Gonzales, thank you so much for being here. There is not a member up here or probably most in this room haven't been touched by Alzheimer's and the other disorders that really affect the livelihoods of our friends, our loved ones. So, thank you for being here.

As an engineer, I am always fascinated by the incredible science and technology people have used to create lifesaving medical devices and drugs.

I want to thank my two colleagues, two doctors up here, that are expert witnesses in their own right, expert questioners, who—who know what they are talking about, have been on the receiving end of this, have seen it. And I believe that, as I have said with many instances in Congress, we have a lot of people up here who talk about things they know nothing about. And, when you have people who know what they are talking about, it is refreshing.

You know, in recent years, innovative software technology, known as prescription digital therapeutics, PDTs for short, have come to the market. These healthcare phone applications are studied in the clinical trials and reviewed by the FDA for safety and efficacy before they can be prescribed to patients in the healthcare and healthcare providers.

You know, PDTs have been put to use in treating opioid addiction, veterans with PTSD, children with ADHD, and a host of other illnesses, including diabetes and mental health disorders. While the FDA has approved these digital health solutions, there are still roadblocks to widespread access. The current number of senior citizens on Medicare is estimated to reach nearly 61 million by the end of this year, and many of them have no access to these treatments due to lack of coverage from CMS.

If America is to remain the leader in the healthcare technology innovation, we must ensure that the FDA and CMS approval process for DPTs are in sync. That is why I, along with Congressman Mike Thompson, along with a couple of Senators, introduced H.R. 1458, the Access to PDT Act, to allow CMS to cover PDTs.

While I am on the topic of FDA and CMS synchronization, I want to echo the bipartisan support we heard today for the MCIT rule and Congressman Wenstrup's legislation to codify it. Our seniors deserve timely access to these lifesaving breakthrough treatments.

Today, I also want to discuss rare disease drugs and treatments. Earlier this year in a hearing, I shared my personal connection to the rare disease community, and my concern about recent legislation discouraging innovation in this space. The IRA includes an exemption from the negotiation process for orphan drugs; however, the exemption is limited to orphan drugs that are already—they are only for one rare disease or condition.

As many of you know, many rare disease drugs are often discovered as a second or third indication for a drug, as my colleague just indicated. It is clear the authors of this bill wanted to protect innovation in the rare disease space, but this provision falls short.

I am calling on my Democrat colleagues to work with me on a technical fix to the bill to make sure that more rare disease drugs and treatments are protected from the negotiation process. I was really encouraged in our HHS budget hearing when Secretary Becerra committed to work with me to ensure the rare disease drug pipeline is not damaged. And, again, I really hope we can work on a bipartisan fix to this issue. I believe there is a middle ground we can find that prevents abuse of orphan drug exclusion but also protects innovation.

My questions—I have got two short ones. Mr. Makower, or Dr. Makower, can you comment on the impact PDTs are having on patients and the need for a clear path to reimbursement?

Dr. MAKOWER. Absolutely.

Digital therapeutics have a tremendous opportunity to improve patient outcomes in care, and it is very, very important that we find a way to cover these technologies for patients. The fact that they are being currently, sort of in a—in a box, unable to be reimbursed because of a technicality, is really a problem and needs to be solved, and we really need some modernization around the entire benefit category process.

Mr. HERN. Thank you.

Dr. Lakdawalla, can you comment on the recent changes to rare disease drug policy, and what impact do you anticipate?

Mr. LAKDAWALLA. Sure. Thank you, Congressman.

It is likely that there will be reduction in innovation in rare disease, because follow-on indications are now potentially penalized under the IRA. It is also the case, more generally, there will be reductions in incentive to innovate.

It is notable, though, that rare disease often features very high unmet need for patients. And, as an economist, I can tell you that that means the value of any given health improvement is greater because patients have so little health that even a given relatively modest improvement of health can be quite valuable. That needs to be accounted for in the way CMS sets maximum fair prices to at least mitigate some of these issues for rare disease, where value is at a premium.

Mr. HERN. Thank you for your responses.

And, again, thank each of you for being with us today.

Mr. Chairman, I yield back.

Chairman BUCHANAN. Ms. Sewell, Alabama.

Ms. SEWELL. Thank you, Mr. Chairman.

I want to thank our witnesses for your testimony today, especially Mr. Gonzales, whose testimony and life's journey is both powerful and inspiring. God bless, sir.

Since the 116th Congress, I have been one of the leading champions of the Medicare Multi-Cancer Early Detection Screening Act, along with Representative Jodey Arrington.

As a daughter that knows firsthand what it is like to lose a parent to pancreatic cancer, it has become my mission to ensure that every American has access to lifesaving, early-detection tests, and all the treatments to help them get well.

Last Congress, this legislation garnered support from 258 bipartisan House cosponsors, and more than 400 leading advocacy groups across all 50 States. And in the 117th Congress, we hope

to have more. This bill creates the authority for CMS to cover blood-based, multi-cancer early detection tests, and future test methods once approved by the FDA.

With innovation increasing in the space of cancer treatment, it is imperative that our legislation promotes an agile, evidence-based process that prioritize safety and cost effectiveness.

Mr. Kesselheim, my question is to you. The bill that I am talking about, my multi-cancer early detection bill, does not establish a coverage mandate for multi-cancer early detection tests, but rather, it gives CMS the authority to create coverage parameters through the national coverage determination process.

In your opinion, how can we better ensure that our coverage policies are keeping at pace with medical innovation?

Dr. KESSELHEIM. It is a great question, and it sounds like—and I think that it is a really important bill, because early detection of cancer is so important, and that is the time when we might be able to best intervene on—particularly on very—you know, very dangerous cancers like pancreatic cancer.

So I think that if there were new early detection tests that were proven to actually reduce mortality from cancers, that it would be a no-brainer for CMS to cover them, and it would be important to—and I think that this is where collaboration between FDA and CMS can be very important in helping ensure that the information that is transmitted to FDA in getting a diagnostic test authorized can then be quickly evaluated and given the green light by CMS. And so, it should be able to be done efficiently.

And I think what—hopefully what your bill can do is provide more resources and more guidance for it to allow FDA and CMS to do this in this context.

Ms. SEWELL. It does, sir.

I think that we should—we, the public, especially since we put so much money towards NIH and research and development of drugs, we should make sure that everyone has access to these amazing medical innovations.

The reality is that there are ways that you can test blood and be able to screen for over 40 different cancers. And so, when that is actually approved by FDA, I don't want it to wait. I want Medicare to cover it, especially since we know that for cancer, age is a determinant in the diagnosis of cancer.

So, look, I think that it is important, and I know for me, it is cancer. For you, sir, it may be Alzheimer's. The point is we have, as a Nation, really developed amazing medical innovation. The fact that we could come up with a vaccine in 10 months for a global pandemic means that if we want to put our resources, our time, and our energy behind the best and brightest researchers and doctors, we can find cures for some of these diseases.

And I believe that our job on Ways and Means, especially around Medicare, is to help facilitate that. And one of the things that I had hoped was the Center for Innovation with CMS would do that.

Mr. Okon, can you talk a little bit more—elaborate more about the guardrails that we really need for CMMI?

Mr. OKON. Yes. First of all, I want to say, whatever we can do, Congresswoman, in terms of pushing that and promoting that bill—I know how cancer has affected you. I know how it has af-

fected me. We will do whatever, because the idea of catching these things earlier and screening through blood tests literally will not only save lives, it will save money as well, too.

Ms. SEWELL. Yes. Absolutely.

Mr. OKON. Let me just say briefly that you would never approve a drug without clinical trials, which demands informed consent by a patient.

The same thing has to happen at CMMI. You can't conduct an experiment when the patient isn't aware and hasn't signed informed consent, and you can't do that without—as I said before, without a smaller test.

So, the guardrails need to be that you can't just use this as an end-run game of a mandatory, big model that is going to do whatever CMS wants to do by using its Innovation Center. We need to put guardrails. So, the same way we approach a clinical trial and safety is the same thing at CMMI.

And, once again, I will say, not just to be agreeable, but anything we can do to support that legislation and put guardrails on CMMI and return it to what it really should be as an innovation center, we are behind it.

Ms. SEWELL. Thank you, sir.

Thank you, Mr. Chairman.

Chairman BUCHANAN. Yeah. Mrs. Miller, West Virginia.

Mrs. MILLER. Mr. Chairman, I yield 45 seconds to Dr. Murphy.

Mr. MURPHY. Thank you, Mrs. Miller.

Thank you, Mr. Chairman.

I just want to bring up one other point. We have talked a lot about cancer. We have talked about a lot of neurologic drugs. The great untold story right now is antibiotic-resistant bacteria. In 2019, more people died from antimicrobial-resistant infections than HIV, AIDS, or malaria.

So, if we are going to continue down this pathway of stifling research, we are going to—where we used to thought penicillin or a quinolone or anything like that, a sulfur drug would take care of things, more and more and more and more people are going to die.

So again, a bad consequence of a bad bill.

Mrs. MILLER. Thank you.

Thank you, Mr. Chairman.

From a State of 1.7 million people, West Virginia has 39,000 people living with Alzheimer's disease. Patients that live in the mountainous rural communities of West Virginia face significant barriers in accessing primary healthcare, let alone specialized clinical trials.

In a 2019 study of one of their clinical trials, WVU's Neurology Department found that more than 25 percent of patients had to travel more than 100 miles one way to participate in the trial.

Furthermore, patients with neurodegenerative disorders, such as Alzheimer's, depend on family members or a caretaker to bring them to their clinical trial visits, which only increases the barriers for patients.

That is why CMS' national coverage determination for Medicare coverage of the Alzheimer's drug Aduhelm, and all future drugs in its class, is so worrisome. I am concerned that rural patients won't be able to access an entire class of drugs just because of where they live.

Mr. Gonzales, you touched on the difficulty of having to travel long distances to access tests and studies. Can you tell a little bit—some more of the difficulties that patients do face in their ability to participate in these trials?

Mr. GONZALES. Sure. Thank you very much for asking the question.

It is extremely difficult, and I am going to go on a different—kind of different route.

Mrs. MILLER. Just do it quickly.

Mr. GONZALES. When I have to go somewhere, my wife is with me. So, there is two of us each time we are going somewhere. You have costs in travel. You have costs in hotels. Then you have costs in meals. You have costs in what you are going to wear. And then, at the end of the day, there is the cost in me. There is what is going to happen to me. Every time I travel, it is about 2 days to get myself back to normal.

Mrs. MILLER. It makes you tired. I——

Mr. GONZALES. It does.

Mrs. MILLER. I understand that.

Mr. GONZALES. Tired and cold and shaky.

Mrs. MILLER. Yes. Continuing with the theme of policies which are—disproportionately affect rural patients, I am also concerned about CMMI's accelerating clinical evidence model. This model slashes Medicare payments for drugs approved through the FDA's highly successful accelerated approval pathways until they complete traditional approval. This is entirely nonsensical, and I can't really understand why they do that.

Mr. Okon, smaller medical providers, including oncology centers in rural areas, typically operate on a very slim margin. Don't you think that this attempt to slow reimbursements of accelerated approved drugs will have a disproportionate impact on these rural providers and, therefore, their patients?

Mr. OKON. Absolutely. It is amazing, Congresswoman Miller, that the consolidation, that impact of Federal policy already has had in independent providers, and that consolidation, especially in rural areas. So what happens? They close their doors. And then patients don't have access.

And the problem is specifically with this one model is that, again, it puts—as I have said, it puts providers—us as hostages in the middle between the government and the drug company, whether it be the IRA in the negotiation, or here, where, if a product doesn't have one clinical trial for one indication, they haven't done it properly, they are going to knock down reimbursement.

And you knock down reimbursement—we have seen this, history has demonstrated it, you not only have cancer clinics, but other providers that close. And, as a result, who suffers the most? Patients in rural areas——

Mrs. MILLER. You are right.

Mr. OKON [continuing]. And also, patients who have—who we——

Mrs. MILLER. I have one more question.

Mr. OKON [continuing]. Targeted health disparities.

Mrs. MILLER. One more question——

Mr. OKON. Yes.

Mrs. MILLER [continuing]. Okay? Because you have explained that, due to the IRA, drug companies probably won't invest in expanded indication research when a drug will be target on the government's negotiation. Therefore, this law will certainly limit research and development for new indications.

What impact does this have on patients; specifically, cancer patients?

Mr. OKON. Well, that is the problem, is we talk about drugs as if they are the same, whereas in cancer drugs, you are talking about different indications developed over the lifecycle of the drug. I am not apologizing for the pharmaceutical industry. We have a problem, as Dr. Murphy said, with high cost of drugs.

But the problem is, as you get closer to this negotiation, you would not put funds forward to study a new indication, especially—especially indications that deal with pediatric cancers.

So you have a real problem in terms of the nature of drugs and lifecycle development, especially when you talk about cancer drugs, as opposed to drugs that may have just one indication always.

Mrs. MILLER. Thank you. I yield back.

Chairman BUCHANAN. Mr. Fitzpatrick, Pennsylvania.

Mr. FITZPATRICK. Thank you, Mr. Chairman.

Thank you all for being here. As co-chair of the Bipartisan Cancer Caucus in Congress and also having lost a brother to cancer, there is an issue of particular interest to me, which involves the impact that the Inflation Reduction Act will have on the development of future cancer medicines, which oftentimes are small-molecule drugs.

As you all know, under existing law, small-molecule drugs are now subject to price negotiations after 9 years. In contrast, biologics will have 13 years.

This small molecule penalty, as it has come to be known, will have devastating impacts on the development of new cancer medicines and on other small-molecule drugs. To avoid this crisis, I believe we all must commit to working across the aisle to ensure that small molecules are afforded the same 13 years as biologics are. And I would encourage all of my colleagues on both sides of the aisle to support this effort today.

My question is for you, Mr. Okon. Are you concerned that, if the small molecule penalty remains in place, that it could lead to a halt of new cancer medicines and lead to developers investing more heavily in biologic products instead?

Mr. OKON. I think, Mr. Fitzpatrick, that logic would dictate that you have a longer time before the negotiation takes place, that you are going to be tilting your investment in biologics, right? Biologics are great. They have done a remarkable, wonderful job in cancer.

But the issue with these small-molecule drugs where they—and I am not the oncologist here, but where they basically penetrate the blood-brain barrier, they are absolutely essential.

So, in my written testimony, I gave the example of Imbruvica, a small molecule that has basically been developed over the course of 11 indications over 9 years. Nine years is the magic mark. So I think that you are going to have a tilt to the process, and I think, at the very least, you should have 13 years for small molecules.

Mr. FITZPATRICK. And what do you believe the intent in the IRA was of this disparity between biologics to small molecules?

Mr. OKON. I just that there is a basic misunderstanding. Again, I live in the world of cancer. I think there is a basic misunderstanding of looking at a drug is a drug is a drug. And a small molecule is not as new, innovative as pronounced as a biologic. But, in cancer, they are essential.

I mean, right now, we are even dealing with cancer drugs that are—that are in short supply that are generic drugs that have been used for over 20 years. We are at a crisis point. Literally today, we are at a crisis point. So, I think that is part of the reason why.

Mr. FITZPATRICK. And do you believe that this will undermine President Biden's Cancer Moonshot goal of reducing cancer death—the cancer death rate by half in 25 years?

Mr. OKON. Well, I think, until the Cancer Moonshot—and I have told this to the Cancer Moonshot people—become aware of what is happening in not just research and development, but what is happening in terms of provider reimbursement, access, consolidation, PBMs—you name it—until they realize that, you can't just have something that sounds great and innovative as a Moonshot without dealing with the reality of our total cancer care system.

Mr. FITZPATRICK. Thank you, Mr. Okon.

Mr. Chairman, I am going to be working hopefully in a bipartisan manner with this committee to address this very issue. And I also will be submitting a question for the record on another priority of mine related to the HELP Copays Act.

But I am out of time, so I yield back.

Chairman BUCHANAN. Mr. Evans, Pennsylvania.

Mr. EVANS. Thank you, Mr. Chairman.

Dr. Kesselheim, last year, Congress and the Biden administration passed the Inflation Reduction Act, which really will help the American people, including constituents of Philadelphia. This law is making healthcare more affordable and accessible to all people, especially in middle-income neighborhoods, which I care deeply about.

For too long, the American people have been witness to government officials talking about the need to reduce the costs of prescription drugs, but little action has taken place.

Through the Inflation Reduction Act, we are taking steps to actually reduce the cost of prescription drugs. Two examples. IRA caps Medicare part D spending at $2,000 per year. Over 57,000 of my constituents are enrolled in this Medicare part D program. This provision potentially helps thousands in my district save money on needed medication next year.

The IRA caps spending on insulin under part B and D at $35 per month, is essentially over $140,000 in Medicaid benefits in my home State of Pennsylvania.

This is one question—will you discuss the affordability of these drugs along with the impact on the health premium of out-of-pocket costs?

Dr. KESSELHEIM. Yeah. I mean, I think that this is another very important part of the Inflation Reduction Act, along with the price negotiation part of it. The parts of the Inflation Reduction Act that lowered out-of-pocket costs for Medicare patients, which, be-

cause of the extremely high prices for these drugs set by manufacturers in the U.S., because we allow manufacturers to set whatever price they want, we get extremely high prices for drugs.

And Medicare beneficiaries were paying, especially those—some of those with cancer were paying enormous amounts out-of-pocket for those products. And so, I think it will be a very important step to reduce those out-of-pocket costs.

But I think it is also important that the IRA, in addition to that, included a process for negotiating drug prices, because that can also help reduce the overall spending on drugs that is not just out of pocket, but also the spending of—that, you know—through the—from taxpayers in general on the costs of Medicare and Medicaid.

Mr. EVANS. Do you have recommendations to address this affordability challenge?

Dr. KESSELHEIM. Sure. I mean, I think that there are a lot of ways to try to address the affordability challenge for prescription drugs. I think the—so, first of all, we need to be making sure that we are prescribing the right drugs. There are many cases where patients are given prescriptions for brand-name drugs when a lower cost brand-name drug or a generic drug might be good—just as good for those patients.

I think we also need to be thinking about what reasons that there are that why these drugs are so expensive, and one of the reasons that they can be so expensive is, as Congressman Doggett mentioned in his opening remarks, the fact that we allow drug companies to obtain dozens and dozens of patents on these products to extend their market exclusivity and prevent timely competition from other products as well.

Dr. KESSELHEIM. So, I think we need to be thinking about those kinds of issues to try to understand why drugs are expensive and to try to figure out how we can ensure that patients are prescribed drugs that are—that are reasonably priced and the correct drugs for them.

And then, if they are, you know, in cases where there are—it is an expensive drug and there are no other opportunities—options for patients, that is where insurance is supposed to come in and cover those costs. And that is why I think it is useful that we have the out-of-pocket caps as part of the Inflation Reduction Act, as well.

Mr. EVANS. Thank you, Mr. Chairman.

I yield back the balance of my time.

Chairman BUCHANAN. Ms. Tenney of New York.

Ms. TENNEY. Thank you, Mr. Chairman and Ranking Member.

And thank you to our witnesses today.

I really appreciate your testimony and your expertise and the time that you spend researching and trying to find cures and also having Mr. Gonzales here.

And I—your family must be so grateful for every minute they have you. So, we—we wish you the best and we hope that some of these gentlemen here may be able to help you with research to get you a longer life and a longer life with your family.

Mr. GONZALES. Thank you, ma'am.

Ms. TENNEY. So, we appreciate you being here, because I know it is not easy to do this and sit in front of Congress.

But, I want—you know, I want to make just a couple comments and I just, you know, the United States has proven to be the most important health innovation ecosystem in the world. We spend more than any other country by more than 28 percent. I think Germany is the next closest, Canada, Switzerland. And we put, we invest a lot of money into trying to find cures. And a lot of these groundbreaking cures, it is incredible.

However, the success, we see these headwinds due to policies enacted under the Biden administration—I know the IRA has been brought up many times. But it takes on average right now 3.3 years for treatments approved by the FDA to be covered by Medicare.

So, Mr. Makower, your "valley of death" discourages innovation, denies new treatment for seniors. While the Trump administration did some work to close the gap, the Biden administration quickly repealed role, which we have heard a little controversy over today, and has yet to replace it with another alternative in 2 years.

And one of the things that I really picked up on that I wanted to go back and say we have gone back and forth.

And, Mr. Okon, you made some comments that was exactly my experience. I took care of both manufacture parents, both with very serious illnesses. My dad was a survivor of a dissecting aortal aneurism. Ended up paralyzed, blind, and with multiple organ failure for the last 7 years of his life but was able to serve. So, he had a strong life force for sure.

But you described something that just made me realize, had he not had a daughter who was a lawyer and someone who could advocate for him, you describe a bizarre, convoluted system, PBMs, nonprofit 340B hospitals, high cost of what it is to be out of pocket. This is what I see as, like, when we overregulate—and I think we should appropriately regulate.

Tell us what—about this overregulation and why it is preventing us from having innovation, why this is—this sort of one-size-fits-all formula? I understand the need to regulate. I think our—you know, Big Pharma is being said in a negative connotation. But how can we make this, so we regulate appropriately, while maximizing innovation and understanding the protection for patients?

Mr. OKON. Well, I will tell you, Congresswoman Tenney, to mention two things that you talked about, is that—and it is good to see that there is bipartisan now awareness that PBMs are a problem. PBMs are fueling drug prices in my world of cancer care. I can't tell you how many times a patient is denied or delayed a drug, a cancer drug, because of a PBM. And so we have got to do something about PBMs.

But I will tell you—and I know it is a sore topic for some. But we did a study using the hospital's own data. These large nonprofit 340B hospitals, do you realize that they are marking up cancer drugs, the top cancer drugs on average five times? Our report is right on our website. It is the hospital's own data.

So, again, to go back to what I said in my written testimony, in my oral that Dr. Mark Fendrick always says there is a difference between the price and the cost. I am not apologizing for the pharmaceutical industry. They do dumb things. But the fact of the matter is it is also the cost. And you walk into some of these big health

systems, and you are literally in a cancer drug, having them marked up five times, even a lot greater than that.

So, you—and the other thing we talk about other countries. You don't see other countries with this convoluted system that we have. You don't see PBMs that basically have merged with insurers, that are now hiring doctors. United Healthcare owns 70,000 physicians. You don't see hospitals marking up——

Ms. TENNEY. Could I—if I can just reclaim my time for a moment, I want to tell you I have a friend who is a veterinarian, owns multiple veterinary clinics. And he said it is lot cheaper to get an MRI for a dog than it is for a human being because there is so much intervention from government, insurance companies, and other nonphysician-based groups that have really kind of undermined our system.

So I—although they all have a role, I just get very concerned about how they have taken control of our healthcare system and we have lost control. And the innovators and the people that we need to solve the problems that we have to come up with the cures, that we need for the future are being lost in this PBM, you name it, every type of bureaucracy that is preventing us from innovating.

But I really—I had a bunch of other questions but I have run out of time.

But thank you so much to all of you, and I am sorry I didn't get to everyone.

Thanks again, and I yield back.

Chairman BUCHANAN. Mr. Moore, Utah.

Mr. MOORE. Thank you, Chair.

Thanks for being here today, witnesses.

Our capacity for innovation is among one of our Nation's greatest assets. Right? We have seen this play out time and time again as we have led the world in so many different circumstances. And, you know, I am primarily speaking of the healthcare sector, obviously, especially today.

Disruptors, entrepreneurs, innovators continually enhance patient care and outcomes with new discoveries and developments. Utah, the State where I represent, is home to some of the most creative innovators in the industry. And I really do appreciate the chairman for assembling this group to ensure that America remains at the forefront of medical innovation.

A couple of questions for Dr. Makower.

In your testimony you mentioned that investors may be hesitant to support innovative medical devices because they may be trapped in the "valley of death" that my colleague from New York discussed, you know, that period where without Medicare coverage following an FDA approval. It is hard for folks to really wrap their heads around that, given that, you know, they are both government entities and the lapse that exists there. Right?

So how has the situation affected patients who could benefit from potentially lifesaving or life-altering novel medicines? Give us some—your thoughts on that.

Dr. MAKOWER. It is incredibly impactful.

Many of these therapies have undergone rigorous clinical trials, substantial evidence, ultimately resulting in a rendering by the

FDA that their technologies are safe and effective, diseases like heart failure, like cancer, diabetes.

And in the sense that—let's just take one example, continuous glucose monitoring. We all know how tightened control of glucose can ultimately prevent very expensive and very devastating side effects, the loss of limbs, heart attacks, stroke. Those types of delays are very significant for patients as they wait for technologies like that to be available.

Mr. MOORE. Thank you.

It is. It is hard for folks—I mean, they think of these organizations as one and the same and they are derived from the same area. We have got to be more in sync with being able to deliver this care to these patients.

We have also witnessed the consequences of MCITs which are all in Utah. Again, I bring up Utah. There is a local company with an FDA breakthrough device designation for Parkinson's patients, and it lost a significant amount of funding following the repeal of MCIT. This company has struggled to replace the lost funds. And as a result, Parkinson's patients may never have access to a product that could improve their motor function.

My colleagues and I firmly believe that a pathway to MCIT is essential for Medicare coverage of innovative technology. So, I co-led a bipartisan bill with Dr. Wenstrup and Congresswoman DelBene and Sewell to reinstate an MCIT-like pathway.

Can you share why a modification of the current coverage pathway is insufficient and why a pathway similar to MCIT is needed?

Dr. MAKOWER. Absolutely. I think there is a misunderstanding that when a technology is deemed a breakthrough, it really means that that product has the potential to have a major impact on a debilitating or life-threatening disease.

After that designation, then there is a substantial amount of evidence that is necessary to clear FDA. Very few companies actually make it, or technologies actually make it to the FDA approval. Once they have finally crossed that gauntlet, to have proven themselves safe and effective with the FDA, especially for breakthrough technologies, that is where the opportunity is to give patients access to it. And I think as evidenced by our survey, innovators are very, very open to continuing to generate evidence development while it is available to patients.

And I think that the proposal that has been put forward, which I think is a very supportable one, would allow that and give patients access, early access, to these therapies as soon as they are available by FDA, while continuing to collect any necessary evidence that CMS may require.

Mr. MOORE. Thank you so much. I appreciate it.

Lastly, Dr. Lakdawalla, the Biden administration has chosen to weaken intellectual property protections for vaccines and is contemplating a similar action for diagnostics and treatments in addition to that. How might this decision adversely affect our longstanding atmosphere of innovation and the future accessibility for the cure of patients?

Mr. LAKDAWALLA. Thank you, Congressman Moore.

In general, weaker IP protection we know lowers incentives to innovate simply because it also weakens the rewards for innovation.

There is a narrow path for the usefulness of intellectual property waivers, but the problem is there is risk on several sides. We know if waivers are granted solely for very low-income countries, it doesn't have much impact on innovation and it can have significant impacts on people's health.

The problem is that if you waive IP rights in one country, there is the possibility and the expectation that it may happen for other countries that are not low-income countries. And it is that expectation that then can dampen innovation, even absent the actual waiver.

So, it is opening Pandora's box. It is a possible strategy, if there are very tight guardrails, that we can do this successfully, but I worry that there are considerable risks when we go down this path.

Mr. MOORE. Thank you for your perspective.

I yield back.

Chairman BUCHANAN. Mr. Davis, Illinois.

Mr. DAVIS. Thank you, Chairman Buchanan, and thank you, Mr. Doggett.

I want to thank all of the witnesses because this has been a very profound discussion that we have had this afternoon.

Mr. Gonzales, I want to simply associate myself with what all of my colleagues have said about your courage, your determination, your advocacy, and the fact that you are with us this afternoon. Your testimony will linger with me for a long time.

Mr. GONZALES. Thank you, sir.

Mr. DAVIS. You know, when I think of healthcare, I really think of the evolution and development that has brought us to where we are.

I grew up in the rural south, and most of the people that I knew when I was a kid had no access to real healthcare at all. There was one physician in the county where I lived. There was no Medicaid and Medicare which means that most of the people that I interacted with had no way to pay for healthcare.

I also remember that there were no hospitals, and so Hill-Burton got passed. Then we were fortunate that the war on poverty got going, the march and the demonstrations. And we got passage of what we called community health centers. And now we have a network of federally qualified health centers all over the Nation. And so, I call all of these great movements towards where we are.

Then we got the Affordable Care Act that—the Obama bill, to some people, Obama medicine, and just recently the Inflation Reduction. Now we are at a level where we are talking about not just the reduction of cost but also the continuation of therapies and continuation of medications that can be helpful, and we subscribe that all of these have been very helpful.

Dr. Kesselheim, I was interested in your testimony where you—and I agree with you that we ought to double the amount of money that we put into the National Institutes of Health. I believe that you can define the greatness of a society by how well it treats its old, how well it treats its young, how well you treat those who are infirmed, suffer with disabilities, are described as being disadvantaged. And I was wondering about that.

And so, if you are not willing to put in the resources that are needed, you know, if you give tax cuts to the wealthy, if you dis-

avow needs movement, does that move us towards where we need to be going?

Dr. KESSELHEIM. It definitely doesn't.

And I would say that I agree that if you double the NIH's budget that you would be able to put a lot more money into doing things that the NIH doesn't invest as much money in right now including trying to ensure that approved drugs are tested in populations like elderly patients or children, in doing comparative effectiveness studies to test drugs against each other, because drug companies refuse to do that, because there is a risk that their particular drug may not win.

And so you don't get a lot of those essential tests that would inform physician-patient decision-making around—around those kinds of products.

So I think you could move a long way towards the kind of society that you are talking about by providing additional resources that the NIH could then—could then use to invest in those kinds of testing to be able to, you know, to be able to understand how drugs works in these—in these kinds of populations, to improve disparities and access to them and in disparities and availability in the kinds of payment who are enrolled in clinical trials.

All of those things are things that would morph funding, that the public infrastructure could better do.

Mr. DAVIS. Thank you very much, Mr. Chairman.

And I must say to you this has been a great hearing.

Chairman BUCHANAN. Thank you.

Mr. DAVIS. Thank you.

And I yield back.

Chairman BUCHANAN. Mrs. Steel of California.

Mrs. STEEL. Thank you, Mr. Chairman.

And thank you all the witnesses for long hours.

And thank you, Mr. Gonzales, that your testimony was just really touching. And I try to understand that what you are going through, but I understand that what your families are going through because my mom was really sick before she passed away with cancer. So, I totally get that.

This hearing is extremely important for the constituents I serve and for California's economy. The Medicare coverage of innovative technology pathway to accelerate Medicare coverage for breakthrough devices was supposed to be effective on March 15, 2021. President Biden delayed the effective dates numerous times, first to May 15 and then until December 15. And he only rescinded this rule which would speed up safe and effective new medical devices to Medicare beneficiaries.

I am disappointed in President Biden's decision to rescind MCIT, and I am frustrated that it has been taking over 2 years for the Biden administration to issue a proposed rule for his version traditional coverage of emerging technologies.

As our witness, Dr. Makower, said in the recent paper Medicare beneficiaries are more likely to have life-threatening or irreversibly debilitating diseases or conditions. They are more likely to benefit from access to breakthrough technologies that promise more effective treatment or diagnosis.

With this in mind, Dr. Makower, can you share with us the breakthrough example and how it would directly improve patient care? And, secondly, could you comment on how rescinding MCIT without a replacement has made it difficult for California's life science community to innovate?

Dr. MAKOWER. Absolutely.

As I mentioned, we have just recently done a study with actual data from publicly available sources and in that data set are numerous technologies. Many of them do, I would say the vast majority of them apply to seniors. There are many examples of this. One other example, I have given the CGM example.

Another example is technology, let's say, to prevent the likelihood of stroke during certain interventions. I think we can all say that to have a stroke is a devastating thing, and the cost of managing someone who has had a stroke is very expensive to the healthcare system.

So, there is a long list of these, but I will go to the next part of your question to answer that one next. Certainly, patient impact is significant.

In California, we have seen a retraction of dollars from investors willing to back these important new therapies. Valuations are down. Jobs are being lost. And, most importantly, patients are being impacted.

I think that there is a reticence—and I teach students and, you know, encourage them to go out into the world and invent important solutions. And when they go out and they talk to their colleagues who are actually in the business of doing it and understand how difficult it is and how unlikely it is that they can be successful, they look elsewhere. That is a tragic phenomenon that we must reverse.

And I think the key here is to be able to bring your product to market after rigorous testing, once it is validated to be safe and effective, to find a way to make it available to patients as soon as possible.

Mrs. STEEL. Thank you.

Dr. Lakdawalla, if I pronounce it right, before I begin, I would love to recognize you that you are a professor at the University of Southern California. I am happy that you are here as one of our witnesses. Fight on.

Doctor, we have been seeing a 13 percent of in bioscience industry employment, nearing 335,000 jobs in 2021 and roughly $7 million in R&D expenditure alone in fiscal year 2020. There are roughly 13,000 life sciences establishments in California with $90 billion in output contributions alone in the Los Angeles area, Long Beach, Anaheim.

Could you elaborate on how the U.S. International Trade Commission's assessment of a proposed waiver of intellectual property rights for COVID–19 diagnostics and therapeutics at the World Trade Organization could damage California's life science's ecosystem's proposed national security risk of IP theft by CCP and how expanding this waiver would threaten investments, research, and development work in our State?

Mr. LAKDAWALLA. Thank you, Congresswoman Steel.

I think there are two issues here. One is what actually happens, and two is what could happen.

So, what actually happens here is the extent to which IP rights are waived will have a chilling effect on investment immediately or the technologies that are impacted. That is a direct effect that we can see.

But the other kind of potentially more uncertain and in some ways more insidious risk is that if there is a waiver or if there are, as we see, waivers, then that has to be built into everybody's risk benefit calculus and investment. And there has to be an understanding that there is a chance of IP waivers and other disease areas, and that has a chilling effect, more broadly, outside of the areas where the waivers take place.

So that is, I think, probably the bigger risk because that potentially spreads across a wider swath of the life sciences industry, impacting employment and innovation spending in our home State, as well as in firms all over the world.

Mrs. STEEL. Thank you very much.

I yield back.

Chairman BUCHANAN. Mr. Smucker, Pennsylvania.

Mr. SMUCKER. Thank you, Mr. Chairman.

I would like to thank the chairman for allowing me to participate. I am not a member of this subcommittee, but it has been fascinating and I think a very important discussion.

I would like to thank each of the witnesses for being here, particularly Mr. Gonzales. Thank you for your courage in sharing your story. That is, I think, really helpful for us to hear your story.

I can tell you that I have heard from people all across my district who have been in similar circumstances with Alzheimer's and have seen the devastating impact but also other diseases. We have been touched in my family by cancer and heart failure I think someone mentioned. And so all of us, I think, have been impacted.

And so, I am not an expert in this area. I am not a doctor. I don't have a background in healthcare. But knowing the value of your system here where we are known across the world as developing some of the best treatments and then seeing some of the potentials that we have, I am from Pennsylvania. And particularly the southeast part of Pennsylvania we have a really great biotech industry and a pharma industry. We have talked to a lot of companies that are developing things that could be absolutely life-changing and transformational going forward, I think someone else mentioned that, you know, to people but also could save a lot of money.

And so, I support—I have supported, after hearing some of these stories, government funding to help drive some of this. So, NIH, I have always supported it, and research.

But I think the really important thing—and I was a business owner. So, I understand a regulatory system that works well to encourage innovation, and I understand the risk and reward. You have to get that right. And So, I get very concerned when we do things that drive down that innovation. It is one of the concerns I had with the IRA. Our CBO said that there would be—they estimated 13 less new drugs developed. I think I have that number right. But other outside—13 fewer cures is what they said and

other outside experts indicated that number could be as high as 135 different cures.

Mr. Lakdawalla, do you agree with those estimates? And then I have a few other questions that I think are going to be follow-ups to some of the things that are discussed. But what do you think of those estimates? And tell me about the impact that will have on people if they are true.

Mr. LAKDAWALLA. Thank you, Congressman Smucker.

The best evidence and the peer-reviewed economics literature suggests that every 2-½ billion-dollar reduction in pharmaceutical revenues leads to one less drug approval.

In principle, there are estimated to be hundreds of billions of dollars of lost revenue due to the Inflation Reduction Act, according to the CBO. So, one might do the multiplication and see that it is way more than what the CBO forecasts.

Now in fairness, the effect might not be linear. So maybe it is not fair to just multiply in that way. And it is also just difficult to predict exactly what will happen because we are now embarking on a grand new experiment that no country has ever performed. The U.S. is the biggest global engine of innovation. So, what we do here has bigger impacts.

Mr. SMUCKER. Yeah, so it will have an impact. I am concerned. I am running out of time.

I do want to get to—there has been—other questioners have brought up this difference between traditional accelerated FDA approval. And I just want to understand that.

In her testimony before the Energy and Commerce Committee back in April 26, CMS Administrator Brooks-LaSure said that CMS views FDA-accelerated approval in a different category that is different than full approval.

What does that mean? And in your view what effect will that have on innovation?

Mr. LAKDAWALLA. Well, I think it is a depressing effect on innovation. I think it is important to think about the rationale for breakthrough approvals in the first place, that it exists because there are situations of very high unmet need where the benefit risk calculus is different.

When patients have very few alternatives, then it may make more sense to use a technology with more uncertainty.

Mr. SMUCKER. That is why I support The Right to Try so much.

Mr. LAKDAWALLA. Exactly.

Mr. SMUCKER. But should we be thinking in Congress of taking steps to clarify that reasonable and necessary standard? Is that something we should be thinking about?

Mr. LAKDAWALLA. Well, I think that standard is very ambiguous, and I think the more that can be done to clarify it for innovators, the better it is for incentives to innovate.

Mr. SMUCKER. All right. I am out of time.

Again, thank you so much for being here.

Chairman BUCHANAN. Ms. DelBene, California.

Ms. DELBENE. Washington.

Chairman BUCHANAN. Washington.

Ms. DELBENE. Thank you, Mr. Chairman and Ranking Member, for allowing me to join this hearing, focused on this important topic on ways we can boost medical innovation.

I want to thank all of our witnesses for taking the time and joining us. Your feedback has been incredibly helpful.

My home State of Washington is home to many leading medical device companies and promising startups that are developing cutting-edge therapeutics and diagnostics.

Americans battling diabetes, cancer, heart disease, and so many other challenges depend on these innovators to develop the next breakthrough technology to improve and save lives. But if we want these medical advances to make a difference, people need to have access to them. And, unfortunately, even after the FDA has determined that a breakthrough medical device is safe and effective, it can take over 5 years for Medicare to cover it. And so, we have got to do better for Medicare beneficiaries.

That is why I have championed the bipartisan legislation, along with my colleagues, Representatives Wenstrup, Sewell, and Moore, on this subcommittee called the Ensuring Patient Access to Critical Breakthrough Products Act. This bill would create a speedy and predictable pathway for Medicare coverage so that seniors have faster access to the newest cures and therapies, while ensuring that these technologies remain safe, effective, and relevant to the Medicare population.

Dr. Makower, you talked about this a little bit earlier. But I wondered if you could discuss how speeding up Medicare approval for breakthrough technologies could actually lead to major cost savings for Medicare and any examples you might have on that.

Dr. MAKOWER. Absolutely. Absolutely. And thank you for your support of that bill.

The way that you can save money with devices is by avoiding complications, complications of the disease itself. I gave the example of diabetes. Heart disease, very expensive. Stroke, extremely expensive. The impact of losing a limb, also tremendously expensive. That, in exchange for a device which would allow someone to track their blood glucose and with more regularity and control to avoid those complications, is a very small price to pay for these savings and just an example of the types of savings that a device could provide to the system if it was able to be covered.

Ms. DELBENE. And, you know, we are talking about the savings, the financial savings. But, clearly, in terms of the impact on patients and quality of life and better outcomes, that also may be more qualitative but incredibly important, too.

Dr. MAKOWER. Absolutely right.

Ms. DELBENE. What types of companies are developing breakthrough technologies from your point of view, and what are the barriers that they face right now?

Dr. MAKOWER. I mean, to be a medical device innovator, you face tremendous barriers at every step of the process.

Many of these inventions have never been accomplished before. It requires tremendous courage, the ability to convince other investors to join you on that journey, employees to join you on that journey. And every study, every clinical study, every advance, every iteration of the technology is always fraught with risk.

Then the regulatory process begins and that is—that can be very long. And it can be very difficult. And the FDA has a fantastic safety record. It is not an easy process to navigate, even if you have a de novo 510(k) or a PMA, which are categorizations for usually breakthrough technologies.

Upon the other side of it, you now have a fully staffed company with the, per regulations, a full-on team with salaries and jobs, all making sure that that product is going to be produced exactly the same every time and that the data will continue to be monitored and all the reporting requirements to the government.

What happens next really matters. If you cannot sell your product, if you cannot get that into patients' hands, obviously, we have talked about the patient impact. But who funds that? Investors have to fund that. And as time goes on, they lose patience. And those companies may go out of business, depriving those patients of that therapy, ultimately.

That is why timely, predictable, and, you know, speedy access to technology is so important, not only for patients but really for the innovation ecosystem.

Ms. DELBENE. Yep. And making sure that we look at the data and the science behind it to get us there.

Dr. MAKOWER. Absolutely.

Ms. DELBENE. Thank you so much, everyone, for being here.

I yield back, Mr. Chairman.

Chairman BUCHANAN. Thank you.

Last but not least, Mr. Arrington from Texas.

Mr. ARRINGTON. I was worried, Mr. Chairman, you would introduce me as being from California, as well.

Now that my greatest fear has been allayed.

Chairman BUCHANAN. Should I say West Texas?

Mr. ARRINGTON. I don't need anymore primary opponents. Thank you.

Honored by your presence and your contribution to our discovery and our problem-solving exercise.

Let me frame this up a little bit. In my opinion, America is the laboratory of innovation for maybe many reasons, but two key features are market economy and patent system: Market economy, creating value for consumers primarily through competition but other elements, and the patent system promoting innovation through protecting intellectual property.

Would you nod your head if you agree so far?

Okay.

Well, the problem is government intervention, at least too much, the wrong kind can hurt the market economy side. And the private sector can—they are very resourceful—can exploit or game the patent side. And that is what I want to focus on for the purpose of my question.

So, the patent system is set up to develop or encourage the development of novel products. But you will have drug companies that will create an original novel product. And then they will apply for a new application, if you will, of the original product.

Now the question is: Is it truly a new product? But what they will do is build a wall or what they call a patent thicket of patents around we'll call it duplicative patents, or another name is ter-

minal disclaimers. But it becomes this dense wall around this new patent application derivative of the original product.

Well, any competitive group with a competitive product to challenge whether or not that innovation is, in fact, novel would have to go through layers of litigation of each of the duplicative patent. And it is onerous, and it is expensive.

And if you are the original patent with the new application, you just spend $25,000 on all those patents. Maybe it is a million dollars to litigate each of them. And so maybe you are $10 million, $15 million to protect an extension of what I would call monopoly forces or an exclusive market. We will never know if that was a legitimate novel development because the competitors don't have the resources to litigate through that packet thicket.

Now I did the best I could to explain what I believe is a major barrier to innovation, because the incentive system in too many cases is for the—for the company, the branded company, if you will, is to spend the money to protect all these patents as opposed to invest the hundreds of millions, if not billions, into creating novel value.

So, the incentive is to do one, which is to prevent competition, anticompetitive monopoly forces, versus the money it takes to develop truly novel innovation.

Now, Dr. Lakdawalla, I have done my best to explain that. Do you believe that system exists today? And does that system, in fact, inhibit innovation and ultimately as a result, limit patient access to new treatment and cures?

I yield for your answer.

Mr. LAKDAWALLA. Thank you, Congressman Arrington.

I think that there are some foundational problems in the way that we price and sell drugs that come to a head in various different ways.

One of the foundational problems is that prices often don't reflect value. And so, drugs are rewarded long past when they are actually producing incremental value and that then creates these kinds of distortions. So, I think the causality is a couple of layers beneath that.

I think rewarding drugs for value would also stimulate new entry that results in more creative destruction via the entry of new drugs that can compete.

I do think that there are a number of issues with biosimilar entry and generic entry that are problematic. We overpay for generics and the Schaeffer research center shows, for instance, that Medicare pays more than what consumers pay in cash at Costco.

So, some of these are quick wins in the way generics and biosimilars function, but I think getting prices right would go a long way towards addressing a number of different symptoms of our various economic diseases in this market.

Mr. ARRINGTON. Mr. Chairman, my time has expired.

Chairman BUCHANAN. You are from Texas. You get an extra 10 minutes.

I would like to thank our witnesses for appearing before us today. I think it has been very productive.

We have received several statements of support for this hearing in ensuring patients have access to innovative therapies.

Without objection, I submit those for the record.

Please be advised members have 2 weeks to submit written questions to be answered in writing later. Those questions and your answers will be made part of formal hearing record.

With that, the meeting stands adjourned.

[Whereupon, at 4:50 p.m., the subcommittee was adjourned.]

MEMBER QUESTIONS FOR THE RECORD

Questions for the Record on Hearing:

Examining Policies that Inhibit Innovation and Patient Access

May 10, 2023

United States House of Representatives

Health Subcommittee on Ways & Means

Ted Okon

Executive Director, Community Oncology Alliance

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Submitted September 5, 2023

The subject of our innovation hearing was on policies that inhibit innovation and access. But, it is worth pointing out that a lack of policy can be a contributing factor. This is the case for copay accumulators and similar programs implemented by insurers and PBMs. These programs prohibit any form of copay assistance a patient uses from counting towards their deductible. This practice has been shown to have a negative impact on patients financially and from a health and treatment perspective. A growing number of States over the past few years are taking a proactive approach and enacting their own laws prohibiting this practice. Unfortunately, this has not spurred on any action from HHS or a big Congressional push outside of the HELP Copays Act.

Could you please respond to the below questions regarding this issue:

1. Copay accumulators are not a new issue, so why has it taken so long for them to be addressed legislatively?
2. Why are the only bills being passed on this issue happening at the State level?
3. Is there a way to put a dollar total on the amount of money patients would save if a Federal ban were to happen? Or, are these programs too opaque for that to be possible?

Thank you for your full and fair consideration of this request.

Sincerely,

Brian Fitzpatrick
Member of Congress

Congressman Fitzpatrick, here are my responses to your three questions for the record.

First, it is important to understand that dealing with the top health insurers and their pharmacy benefit managers ("PBMs") can best be described as playing the arcade game of "whack a mole." Not to trivialize this in any way, but the insurers and their PBMs have an amazing ability to find new sources of revenue from patients, physician providers, and pharmacists when any existing sources of revenue are threatened. As such, as rebates and direct and indirect renumeration fees ("DIR fees") come under scrutiny and legislative restraints at the state and federal levels it becomes clear how the insurers and their PBMs have found a way to literally take money out of patients' pockets with copay accumulators. Unfortunately, until this year, Congress has sat back and allowed these programs to be implemented without any restraints. The result is that money intended to help patients in need is being pocketed under copay accumulator programs. Congress must act to ban these programs. Sitting back as they proliferate is not an option.

Second, states are way ahead of the United States Congress. Simply put, it's only really this year that Congress has come together on a bipartisan basis to investigate destructive practices of insurers and their PBMs. Now, Congress must turn all the hearings and talk into legislative action to catch up to State legislation.

Third, unfortunately, it is difficult, if not impossible, to put a dollar estimate on copay accumulator programs. That is because of the opaqueness of these programs. The top insurers and their PBMs love operating in the dark because they have been allowed to do so. Lack of transparency really benefits them.

I appreciate the opportunity to provide these brief answers and certainly am available to answer any other questions and work with you and your office on stopping destructive programs such as copay accumulator programs.

Ted Okon
Executive Director
Community Oncology Alliance

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Submission Date: May 24, 2013

Alliance for Aging Research Comments to Ways and Means Health Subcommittee on Patient Access and Inhibiting Innovation

Dear Chairman Smith, Subcommittee Chairman Buchanan, and Ranking Member Doggett,

The Alliance for Aging Research (the Alliance) is pleased to submit comments in response to the Ways and Means Health Subcommittee's May 3, 2023 (No. HL-02) request for more information on policies that inhibit innovation and patients' access to new drugs.

The Alliance remains deeply concerned with the Center for Medicare & Medicaid Services' (CMS) coverage with evidence deployment (CED) paradigm, which restricts access to lifesaving therapies and deters research into potential cures. Its use and unique presence in the Medicare program restricts access to life-extending therapies and is increasingly serving as a deterring factor for investment in treatments for diseases that disproportionately impact older Americans.[1]

When CMS deploys CED, coverage is limited only to beneficiaries that participate in a follow up clinical study with parameters defined by CMS. CMS dictates whether this study takes the form

[1] Peschin, Susan. Prepared Comment on Medicare's Coverage with Evidence Development (CED) for February 13-14, 2023 Meeting of the Medicare Evidence Development and Coverage Advisory Committee (MEDCAC). 13 Feb 2023. https://www.agingresearch.org/document/prepared-comment-on-medicares-coverage-with-evidence-development-ced/

of A) a confirmatory clinical trial where Medicare beneficiaries may receive, and could be required to pay for, a placebo for an FDA-approved treatment, or B) a clinical data registry. Under both options, beneficiary access is limited – sometimes only to hundreds or a few thousand individuals.

It is hard to find anyone opposed to the idea of continued development of the evidence base for therapeutics to evaluate efficacy and safety signals in real world settings. However, CMS's use of participation in a clinical data registry as a condition of coverage under CED has served to severely restrict access. CMS often includes site restrictions in CED criteria that only permit larger hospitals (including academic medical centers) and provider groups to provide covered services. Effectively, this restricts access for populations not typically in close proximity to these facilities, including communities of color and rural populations.

Last year's decision to implement CED for the entire class of monoclonal antibodies that target amyloid in treating Alzheimer's disease was precedent setting, as CMS had never before finalized a CED for an on-label use of a FDA-approved drug or biologic. CMS' use of CED has historically been limited to situations where there was uncertainty about the effectiveness of medical intervention, especially in the device and diagnostic space, where clinical trials with smaller enrollment are more commonplace.[2] However, recent statements by CMS officials suggest the agency may begin using CED more broadly, even in situations where there is no significant uncertainty.[3] In a January 2023 forum at the UCSF-Stanford Center of Excellence in Regulatory Science and Innovation (UCSF-Stanford CERSI), panelists including CMS's Chief Medical Officer, Dr. Lee Fleisher, discussed the application of CED to other clinical areas. During the same discussion, Dr. Rena Conti – an adviser to CMS – suggested the paradigm would be well suited to use for future therapeutics in the areas of infectious disease, oncology, and cell and gene therapies.[4]

More recently, Administrator Chiquita Brooks-LaSure's testimony before the House Energy & Commerce Committee indicated that current CMS leadership does not view therapeutics approved through the FDA's accelerated approval pathway (AAP) to be "in the same category" as those approved through the traditional pathway. The Administrator's comments raise concerns that future AAP approvals may be at high risk of being subject to CED coverage restrictions. The Administrator's comments came despite the clear intent of Congress that the AAP designation is intended to accelerate patient access to therapeutics in areas with high unmet clinical need.

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[2] Grogan, Joseph. *Medicare's 'Coverage With Evidence Development': A Barrier To Patient Access And Innovation*. 1 May 2023. https://healthpolicy.usc.edu/article/medicares-coverage-with-evidence-development-a-barrier-to-patient-access-and-innovation/

[3] Alliance for Aging Research. *Façade of Evidence: How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity*. 13 Feb 2023. https://www.agingresearch.org/wp-content/uploads/2023/02/Facade-of-Evidence-CED-2-13-2023.pdf.

[4] 2023 CERSI Summit - Panel 2: Cross-Agency Synergy to Accelerate Access to Medical Products. 8 Jan 2023. https://www.youtube.com/watch?v=2acW0KMYCII&t=1398s

CMS also has not provided clear standards for the type of data that would merit retirement of CED coverage limitations. In some cases, such as for the use of positron emission tomography (PET) scans for the detection of beta-amyloid related to Alzheimer's disease,[5] a robust CED trial of over 18,000 individuals was completed, but CMS then subsequently required a second study focused on communities of color.[6, 7] As a result, amyloid PET remains under CED restrictions and is not broadly available ten years after the original determination despite robust data illustrating the diagnostic's value in both confirming and ruling out Alzheimer's disease.

Peer-reviewed meta-studies back up the claim that CMS imposes CED restrictions unilaterally and without a time limit.[8] "An August 2022 systematic review of CED program history, published in The American Journal of Managed Care identified that between 2005–2022, CMS issued a total of 27 NCDs requiring CED. Only four have been retired by the Agency, which has taken an average of 8 years to do so. Under its current paradigm, CMS has enabled 22 CEDs to continue in perpetuity, including several that have been ongoing for more than 15 years."[9]

CMS states that they are unable to consider cost considerations in the NCD and CED processes. However, therapeutics subject to CED are consistently more expensive than the prior standard of care. The dynamics of increased costs for items under CED in combination with CMS's track record of extended CED periods, restrictive use criteria, and evolving data thresholds required in order to grant greater access raises questions about CMS's potential use of CED as a utilization management technique.

In brief, CMS's use of CED has been problematic. In addition to access restrictions, the now-established precedent for CMS to use CED for drugs and biologics could have a chilling effect on investment in areas of unmet clinical need. Companies and investors may be less likely to fund research and development efforts in areas where CMS is likely to use CED, as the potential patient population available is greatly reduced. This could ultimately result in fewer treatment options for older Americans, who are more likely to suffer from chronic diseases and other conditions where there is an urgent need of the development of new therapeutics.

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[5] Centers for Medicare and Medicaid Services. CAG-0031N: Beta Amyloid Positron Emission Tomography in Dementia and Neurodegenerative Disease. 27 Sept 2013. https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=N&NCAId=265

[6] New Ideas: Imaging Dementia-Evidence for Amyloid Scanning. *Original IDEAS Study*. Accessed 24 May 2023. https://www.ideas-study.org/Original-Study

[7] New Ideas: Imaging Dementia-Evidence for Amyloid Scanning. About Us. Access 24 May 2023. https://www.ideas-study.org/About-Us

[8] Alliance for Aging Research. *Façade of Evidence: How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity*. 13 Feb 2023. https://www.agingresearch.org/wp-content/uploads/2023/02/Facade-of-Evidence-CED-2-13-2023.pdf.

[9] Alliance for Aging Research. *Façade of Evidence: How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity*. 13 Feb 2023. https://www.agingresearch.org/wp-content/uploads/2023/02/Facade-of-Evidence-CED-2-13-2023.pdf.

The Alliance published a report highlighting the history of Medicare's application of CED and relevant considerations in February 2023. We have included the executive summary as an addendum to this letter, and the full report is available at: https://www.agingresearch.org/wp-content/uploads/2023/02/Facade-of-Evidence-CED-2-13-2023.pdf.

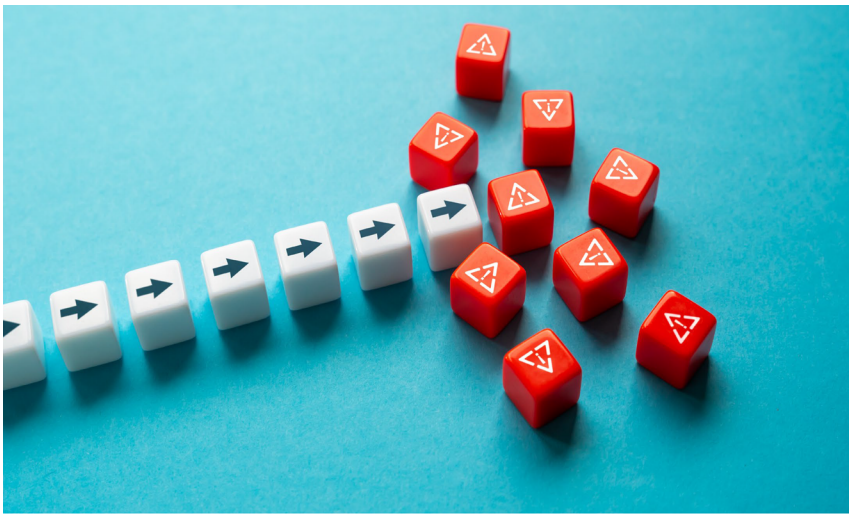
The Alliance urges Congress to prevent CMS from misusing CED to restrict patient access to new therapies, particularly for diseases that impact older Americans disproportionately. Legislation to provide a "presumption of coverage" for FDA-approved drugs and biologics that are clinically indicated for use for the Medicare population would restore the pre-April 2022 status quo, protect beneficiary access, and ensure appropriate use of the NCD process.

Sincerely,

Susan Peschin
President and CEO
Alliance for Aging Research

Michael Ward
VP, Public Policy and Government Relations
Alliance for Aging Research

Addendum: Executive Summary of Façade of Evidence How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity



Façade of Evidence:

How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity

February 13, 2023

Executive Summary

The Medicare program is the predominant insurer for the over 65 and disabled populations and provides medical coverage for 64 million Americans. By statute, the Centers for Medicare & Medicaid Services ("CMS" or "the Agency") provides coverage for items and services that are deemed "reasonable and necessary" under the Social Security Act (the "Act"). By comparison, the U.S. Food & Drug Administration ("FDA") generally approves a drug or biological product based on a finding that it is "safe and effective" based on the Federal Food, Drug, and Cosmetic Act. Separately, medical devices are approved based on a "reasonable assurance" of safety and efficacy to receive FDA approval.

Given the FDA's rigorous, evidence-based approval process, CMS has largely considered FDA-approved drugs and biologics as "reasonable and necessary." Medicare can formally

establish national coverage policy for Medicare Part B physician-administered services or therapeutics through a National Coverage Determination ("NCD") or allow a Medicare contractor to establish regional coverage guidelines. More commonly, the need for therapeutics and services are considered on a claim-by-claim basis.

CMS has the option to issue a NCD to set a single coverage standard on how an FDA-approved product or service is covered nationally in the Medicare Part B program. Between 2012 and 2022, CMS issued 336 NCDs, primarily for medical devices and services. CMS utilizes a range of potential coverage outcomes for NCDs, from full coverage to a prohibition on coverage. Given the size of the Medicare population, NCDs represent a high-stakes decision by the Agency that can either procure coverage for a new therapeutic or result in nearly 20% of the U.S. population being unable to potentially access a treatment for a given condition. Unfortunately, it is not uncommon for the CMS coverage decision process to become highly politicized due to its economic impacts on public and private payers, industry, specialty providers, and national and regional medical systems and hospitals.

In recent years, CMS has escalated its focus on the prices of drugs, biological products, and medical devices at a time when growth in U.S. healthcare cost increases have outpaced economic growth, notwithstanding the fact that such considerations fall outside of Medicare's legal mandate. Drug pricing and payment policies are statutorily distinct from coverage considerations. CMS has repeatedly insisted that it does not consider the price of medical products and services when determining coverage policy; however, former HHS assistant secretary Dr. Richard Frank has characterized NCDs as "the most powerful coverage tool that Medicare has and have generally been reserved for Medicare services that are costly …"[i]

Since 2005, CMS has turned to using an extralegal paradigm known as coverage with evidence development ("CED" or an "NCD requiring CED"). Initially, CED was utilized to accelerate access to medical devices, which have fewer clinical trial requirements in comparison to drugs and biologics. As time passed, CMS expanded its use of CED to other therapeutic types and diagnostics. Under CED, the Agency denies Medicare coverage for an FDA-approved item or service *except* when it is provided to beneficiaries within a population-limited clinical study, such as a CMS-approved clinical trial or data registry. Beneficiaries who are ineligible under the

strict CED requirements, cannot access the clinical study sites, or are reluctant to be required to enroll in a clinical study to receive access are left without coverage.

Once CMS places a treatment in CED, it is extraordinarily difficult for the coverage restriction to be lifted. An August 2022 systematic review of CED program history, published in *The American Journal of Managed Care* identified that, between 2005–2022, CMS issued a total of 27 NCDs requiring CED. Only four have been retired[ii] by the Agency, which has taken an average of 8 years to do so.[iii] Under its current paradigm, CMS has enabled 22 CEDs to continue in perpetuity, including several that have been ongoing for more than 15 years.[iv]

CMS sets "conditions of coverage" ... for health facilities participating in CED studies that often prohibit access for beneficiaries in rural communities and in communities of color. In some cases, the lack of enrollment from these populations has provided the Agency justification to continue a CED determination.

Additionally, CMS sets "conditions of coverage" (e.g., the treatment is only provided for beneficiaries in certain settings of care and overseen by designated specialists) for health facilities participating in CED studies that often prohibit access for beneficiaries in rural communities and in communities of color. In some cases, the lack of enrollment from these populations has provided the Agency justification to continue a CED determination. In practice, NCDs requiring CED have been operationalized and evolved to restrict access to potentially life-saving therapies for millions of Medicare beneficiaries.

CMS's unchecked use of CED has led to harmful consequences including:

- The continuation of restrictive coverage requirements for an indefinite period of time;
- Barriers to beneficiary access for potentially clinically meaningful items and services and the deterioration of health outcomes;
- Perpetuation and exacerbation of access to care barriers for beneficiaries of color and those from rural communities;

- The regulatory repudiation of the FDA's statutorily-authorized accelerated approval program;
- The imposition of unnecessary costs and burdens on sponsors and healthcare providers; and
- The failure to advance the Congressional intent of Medicare.

As a result of these harmful outcomes, it is imperative that CMS cease its use of CED. However, CMS has indicated its intent to instead deploy additional NCDs requiring CED by commissioning a November 2022 report from the Agency for Healthcare Research and Quality (AHRQ) on recommendations to refine CED study design requirements.[v] On February 13-14, 2023, the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC) will use the final AHRQ report as a basis for its discussion and provide its recommendations to CMS.[vi] Other potential expansions of CED by CMS include a proposed rule that conditions coverage of technologies (and potentially including drugs) on the collection of additional evidence in CMS-approved studies[vii]; and recommendations from the Medicaid and CHIP Access Commission (MACPAC) for Congress to grant states outright authority to limit Medicaid formularies based upon Medicare NCD determinations.[viii]

To read the full report, visit: www.agingresearch.org/facadeofevidence

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[ii] Sean Dickson et al., *Limiting Coverage Based On Efficacy And Safety: A Path Forward For Medicare Regarding The Alzheimer's Treatment Aducanumab*, Health Affairs (June 7, 2021), https://www.healthaffairs.org/do/10.1377/forefront.20210604.489197.

[ii] There were two CEDs, artificial hearts and home oxygen for cluster headaches, that resulted in revocation of the NCD and deferral of coverage decisions to local contractors.

[iii] See Emily P. Zeitler et al., Coverage with Evidence Development: Where Are We Now? 28 AM. J. MANAGED CARE 382, 382 (Aug. 2022), https://www.ajmc.com/view/coverage-with-evidence-development-where-are-we-now-. Dr. Zeitler's et al conclusions are not new. Other studies have similarly concluded that "CED schemes . . . are often costly, complex, and challenging." Carlo Federici et al., Coverage with evidence development schemes for medical devices in Europe: characteristics and challenges, 22 EUR. J. HEALTH ECON. 1253, 1253–73 (Nov. 2021).

[iv] *See* Zeitler, *supra* note xii, at 385–87.

[v] Agency for Healthcare Res. & Quality, *Analysis of Requirements for Coverage With Evidence Development (CED) – Topic Refinement* (Nov. 2022), https://effectivehealthcare.ahrq.gov/products/coverage-evidence-development/research-report.

[vi] MEDCAC Meeting: Analysis of Coverage with Evidence Development (CED) Criteria: https://www.cms.gov/medicare-coverage-database/view/medcac-meeting.aspx?medcacid=79.

[vii] Fleisher LA, Blum JD. A Vision of Medicare Coverage for New and Emerging Technologies—A Consistent Process to Foster Innovation and Promote Value. JAMA Intern Med. 2022;182(12):1241–1242.

[viii] Medicaid Coverage based on Medicare National Coverage Determination: Review of Draft Chapter and Recommendations for March Report, MACPAC, January 26, 2023, https://www.macpac.gov/wp-content/uploads/2023/01/04_Medicaid-coverage-based-on-Medicare-national-coverage-determination-NCD_-Review-of-recommendations-and-draft-chapter-for-March-report.pdf.

May 24, 2023

The Honorable Jason Smith
House of Representatives
Washington, DC 20515

The Honorable Richard Neal
House of Representatives
Washington, DC 20515

The Honorable Vern Buchanan
House of Representatives
Washington, DC 20515

The Honorable Lloyd Doggett
House of Representatives
Washington, DC 20515

RE: House Ways and Means Subcommittee on Health Hearing on Examining Policies that Inhibit Innovation and Patient Access

Dear Chairman Smith, Ranking Member Neal, Subcommittee Chair Buchanan and Subcommittee Ranking Member Doggett:

The Alliance of Community Health Plans (ACHP) thanks the Ways and Means Health Subcommittee for its May 10 hearing, "Examining Policies that Inhibit Innovation and Patient Access." We are submitting the following comments for the record on patient access to innovative and affordable drugs, particularly new classes of emerging drugs intended to treat Alzheimer's disease and obesity.

ACHP is the only national organization promoting the unique payer-provider aligned model in health care, delivering affordable, coordinated and comprehensive coverage options. ACHP member companies collaborate with their provider partners to deliver higher-quality coverage and care to tens of millions of Americans in 38 states and D.C. Deeply rooted in their communities, ACHP member companies understand the value of an integrated system of care, in which providers, payers and community leaders work together to enhance access to services and improve health outcomes.

The Centers for Medicare & Medicaid Services' (CMS) released a National Coverage Determination (NCD) in 2022 for Aduhelm and other anti-amyloid drugs approved by the Food and Drug Administration (FDA) with an indication to treat Alzheimer's disease. The NCD limits coverage to patients participating in clinical trials or those meeting specific clinical criteria.

ACHP supports the NCD because of the serious documented risk and unanswered questions about current anti-amyloid drugs. The current policy allows time for additional research through clinical trials and studies to determine this drug class's efficacy and safety. Patients and families deserve more evidence to clearly understand if anti-amyloid drugs really work and what are the tradeoffs in using them.

During the hearing, lawmakers called for the reversal of CMS' NCD and discussed requiring the agency to consider each FDA-approved drug individually, rather than as part of a class. ACHP strenuously

supports clear standards set at the class level to ensure consistency in coverage determinations and predictability in planning for benefit design purposes.

As new drugs and therapies emerge, ACHP stresses the importance of basing coverage decisions on science, patient outcomes and safety. Many emerging drugs offer hope and potential benefit for patients yet carry health risks and staggering price tags that weigh heavily on consumers, taxpayers and purchasers. In recent months, ACHP member companies have reported a rise in prescriptions of GLP-1 agonists that treat diabetes and obesity including the drugs Ozempic and Mounjaro. While effective in specific applications, the drugs are extremely expensive and require lifetime use to remain successful. It is important to assess the promise of new drugs and therapies for coverage determinations on balance with the excessive cost drug manufacturers charge.

ACHP looks forward to a thoughtful conversation in support of innovation, patient access and the financial sustainability of our nation's health care system.

ACHP's full comment on the 2022 NCD for the anti-amyloid class of drugs is attached. Please contact Josh Jorgensen, ACHP Associate Director of Legislative Affairs, at jjorgensen@achp.org with any questions.

Sincerely,

Ceci Connolly

Ceci Connolly
President and CEO, ACHP

February 10, 2022

Administrator Chiquita Brooks-LaSure
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244

RE: NCD Determination for Aduhelm

Dear Administrator Brooks-LaSure:

The Alliance of Community Health Plans (ACHP) applauds the Centers for Medicare & Medicaid Services (CMS) on its balanced decision regarding the proposed National Coverage Determination (NCD) for monoclonal antibodies targeting amyloid for the treatment of Alzheimer's disease. The proposed NCD would include coverage for Aduhelm (Aducanumab), the latest treatment developed for Alzheimer's disease, as well as similar treatments currently under development. The coverage with evidence determination is sound and will allow the opportunity to assess the medical necessity of these types of drugs based upon the collection of additional safety and efficacy data.

As you know, ACHP represents the nation's top-performing, provider-aligned, community-based health plans for more than 24 million Americans nationwide. We support the development and coverage of novel drugs that improve health outcomes, but monoclonal antibodies targeting amyloid have not demonstrated effectiveness, safety or value. We strongly support the agency's goal to ensure that access to this treatment is made based upon strong clinical and scientific evidence that will be gathered through further studies and clinical trials. This proposed coverage decision balances that admirable goal while not giving individuals and families facing the everyday challenges Alzheimer's disease presents a false hope.

ACHP offers this detailed input after consulting with top clinical leaders (such as medical directors and pharmacy leaders) from our member plans. This letter is based on their extensive expertise and understanding based on years of experience in the field. It represents our membership's collective understanding of the available scientific literature and the desire to ensure the best health outcomes for the patients our members are trusted to care for.

Background
Aduhelm was evaluated in two identically designed phase 3 randomized, placebo-controlled clinical trials named Study 301 (ENGAGE) and Study 302 (EMERGE), which had primary objectives to demonstrate efficacy and safety in early Alzheimer's disease.

In 2019, both studies[1] were stopped by Biogen for futility following a planned interim analysis. In 2020, Biogen performed numerous post hoc analyses on the trial data and suggested that in one of the two identical trials

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there was a small statistical difference in the rate of cognitive decline as measured by one of the clinical scales used in the trials in the highest dosage arm. Importantly, this difference was not identified in the highest dosage arm of the other identical trial. Additionally, there were no differences in the rate of cognitive decline identified in the lower dosage arms of either trial.

Many scientists and biostatisticians have observed that such a finding during post hoc analysis should be hypothesis-generating and requires confirmation in prospectively designed clinical trials. This is of particular importance since one trial had a positive finding and the other did not. It is not an appropriate assumption to label one of the trials as "true" and the other as "false."

The FDA Advisory Committee charged with reviewing the clinical trial data for Aduhelm all voted against approval due to lack of sufficient evidence of effectiveness on patients. Despite the Advisory Committee's strenuous objections, the FDA ultimately approved[2] Aduhelm under the Accelerated Approval pathway. The agency's decision was based on a reduction in beta-amyloid plaques in the brain. The FDA cited beta-amyloid plaques as a surrogate endpoint, a reduction of which "is reasonably likely to result in clinical benefit."

Since its approval, numerous major health plans – including ACHP member Point32Health – announced they will not cover the drug outside of any national requirement to do so given concerns about the effectiveness and safety of the drug. They are joining a list of major health systems and insurers such as the Cleveland Clinic, Mount Sinai and the Department of Veterans Affairs who are rightfully questioning whether prescribing this treatment is in the best interest of patients.

Lack of Efficacy & Significant Safety Concerns
While Aduhelm has not definitively demonstrated benefit in clinical trials, a significant number of participants in both studies suffered from numerous concerning adverse effects. About 41% of patients in the Phase 3 trials suffered from amyloid related imaging abnormalities (ARIA) including brain swelling (ARIA-E) and small bleeds or microhemorrhages of the brain (ARIA-H). Indeed, one patient treated with Aduhelm in the Phase 1b trial died of an intracranial hemorrhage believed to be related to study treatment.

Of note, the phase 3 clinical trials excluded patients who were on anticoagulant drugs (blood thinners) due to the known risk of ARIA-H (brain bleeds) with Aduhelm. The current FDA label does not list concurrent use of blood thinners as a contraindication, precaution or warning.

There are grave concerns that patients may receive Aduhelm and anticoagulants together with disastrous outcomes, including death from bleeding into the brain. ACHP is encouraged that the Centers for Medicare and Medicaid Services made safety and efficacy a priority in the proposed NCD.

The Concern with Beta-Amyloid Plaques as a Surrogate Endpoint
The full role of beta-amyloid plaques in the pathophysiology of Alzheimer's Disease is not completely understood. While presence of amyloid plaques in patients with cognitive impairment are a hallmark of Alzheimer's disease, their causative role in development of the disease and whether they are an effective therapeutic target remains in doubt.

[1] Alexander GC, *et.al.* Revisiting FDA Approval of Aducanumab. July 28, 2021; DOI: 10.1056/NEJMp2110468
[2] https://www.fda.gov/drugs/news-events-human-drugs/fdas-decision-approve-new-treatment-alzheimers-disease
[3] https://www.cdc.gov/media/releases/2018/p0920-alzheimers-burden-double-2060.html
[4] ICER: Aducanumab for Alzheimer's Disease: Effectiveness and Value. Evidence Report. June 30, 2021.

Additional Resources: 2
https://www.washingtonpost.com/health/2021/07/05/aduhelm-new-alzheimers-drug-amyloid/
https://www.citizen.org/wp-content/uploads/2575.pdf?eType=EmailBlastContent&eId=76f2ebab-44d9-4b5c-a6a1-2ab30f66c229
https://energycommerce.house.gov/newsroom/press-releases/pallone-and-maloney-command-cms-s-proposed-medicare-coverage-decision-on
https://www.washingtonpost.com/health/2021/07/09/aduhelm-new-alzheimers-drug-hhs-inspector-general/

Some experts question the validity of using beta-amyloid plaques as a surrogate endpoint to predict likelihood of clinical benefit. This is largely based on the fact that numerous investigational drugs targeting amyloid plaques have failed to demonstrate any improvement in cognitive function decline, despite reducing amyloid plaques.

In addition, it is hypothesized that other markers, including nerve inflammation and tau protein tangles may also play important roles in the disease. Unfortunately, since the controversial FDA approval of Aduhelm was based on beta-amyloid plaque reduction as a surrogate marker, several pharmaceutical companies whose previous anti-amyloid drugs failed to demonstrate any clinical benefit now aim to file for FDA approval of similar drugs. Doubling down on anti-amyloid therapies at this time would likely discourage research for other Alzheimer's treatment targets, which could provide more useful and proven therapeutic modalities in this devastating disease.

As for the randomized controlled trials, there are measures that must be undertaken by Medicare in order to gather wholesome data. Among people ages 65 and older, African Americans have the highest prevalence of Alzheimer's disease and related dementias (13.8 percent), followed by Hispanics (12.2 percent), and non-Hispanic whites (10.3 percent)[4], American Indian and Alaska Natives (9.1 percent), and Asian and Pacific Islanders (8.4 percent). The final coverage with evidence development must ensure that the enrolled patients reflect the nation's diverse population diagnosed with Alzheimer's disease. Patients and families need a clearer understanding of whether a reduction of beta-amyloid plaques provides a clinical benefit that is reflective upon a diverse population and limiting coverage to generate additional evidence is a step in the right direction.

Affordability for the U.S. Healthcare System

There is a well-established prescription drug affordability crisis in the United States, which acutely impacts our nation's public insurance programs and the populations they serve. We applaud the Biden Administration for its continued focus on the problem of exorbitant drugs. Coverage for high-priced, unproven therapeutics will only exasperate existing cost concerns – and do little to improve the health of the nation.

Even with Aduhelm's recently announced price cut, an annual price of $28,200 per patient set by Biogen will have staggering effects on patient access, insurance premiums and taxpayers. The Institute for Clinical and Economic Review's (ICER)[4] panel (which included numerous experts in Alzheimer's disease) unanimously voted against Aduhelm with respect to providing any additional benefits over standard care.

During a cost-effectiveness analysis, ICER's model generously included an assumption that the post hoc analysis finding of delayed cognition decline in the high dose group in the single trial was true. The analysis identified an appropriate value-based annual price range of $3,000 - $8,400, far from the current Aduhelm annual list price and Biogen's staggering $56,000 price at release.

With the aforementioned issues in mind, if Congress acts to allow Medicare to directly negotiate drug prices and those prices cannot be accessed by commercial payers, there will be a massive cost shift to the commercial market, which would undoubtedly result in double digit premium increases for employers and working Americans. In addition, due to the significant safety concerns regarding ARIA-E and ARIA-H, numerous brain

[1] Alexander GC, *et al.* Revisiting FDA Approval of Aducanumab. July 28, 2021; DOI: 10.1056/NEJMp2110468
[2] https://www.fda.gov/drugs/news-events-human-drugs/fdas-decision-approve-new-treatment-alzheimers-disease
[3] https://www.cdc.gov/media/releases/2018/p0920-alzheimers-burden-double-2060.html
[4] ICER: Aducanumab for Alzheimer's Disease: Effectiveness and Value. Evidence Report. June 30, 2021.

Additional Resources:
https://www.washingtonpost.com/health/2021/07/05/aduhelm-new-alzheimers-drug-amyloid/
https://www.citizen.org/wp-content/uploads/2575.pdf?eType=EmailBlastContent&eId=76f2ebab-44d9-4b5c-a6a1-2ab30f66c229
https://energycommerce.house.gov/newsroom/press-releases/pallone-and-maloney-command-cms-s-proposed-medicare-coverage-decision-on
https://www.washingtonpost.com/health/2021/07/09/aduhelm-new-alzheimers-drug-hhs-inspector-general/

imaging studies are recommended to monitor for signs of these common adverse effects, which adds to overall costs and patient affordability.

Also, there are concerns that, despite coverage only being available through clinical trials, some costs may be inappropriately pushed onto Medicare. To safeguard the integrity of the trials, CMS should provide guidance to ensure all precisely delineate the conditions under which health care related items and services must be considered as part of the trial, and thus reimbursed by Medicare.

Dr. Lee Fleisher, CMS Chief Medical Officer and Director of the Center for Clinical Standards and Quality noted in the draft decision that due to "the potential for harm, and important questions that remain, we have determined that coverage with evidence development through clinical trials is the right decision for Medicare patients, clinicians, and caregivers." We applaud the proposed national coverage determination for ensuring that the health care system is paying for a drug that is effective and safe.

We appreciate the continued engagement with you and members of your team. ACHP strongly supports this balanced policy, and we encourage CMS to proceed with finalizing the NCD decision for monoclonal antibodies targeting amyloid for the treatment of Alzheimer's disease. Please contact Michael Bagel, ACHP Director of Public Policy, at mbagel@achp.org or (202) 897-6121 with any questions.

Sincerely,

Ceci Connolly
President and CEO
ACHP

Cc: The Honorable Xavier Becerra
Secretary
Department of Health and Human Services

Mr. Paul Spitalnic
Director and Chief Actuary
Office of the Actuary
Centers for Medicare & Medicaid Services

[1] Alexander GC, *et.al.* Revisiting FDA Approval of Aducanumab. July 28, 2021; DOI: 10.1056/NEJMp2110468
[2] https://www.fda.gov/drugs/news-events-human-drugs/fdas-decision-approve-new-treatment-alzheimers-disease
[3] https://www.cdc.gov/media/releases/2018/p0920-alzheimers-burden-double-2060.html
[4] ICER: Aducanumab for Alzheimer's Disease: Effectiveness and Value. Evidence Report. June 30, 2021.

Additional Resources:
https://www.washingtonpost.com/health/2021/07/05/aduhelm-new-alzheimers-drug-amyloid/
https://www.citizen.org/wp-content/uploads/2575.pdf?sType=EmailBlastContent&eId=76f2ebab-44d9-4b5c-a6a1-2ab30f66c229
https://energycommerce.house.gov/newsroom/press-releases/pallone-and-maloney-command-cms-s-proposed-medicare-coverage-decision-on
https://www.washingtonpost.com/health/2021/07/09/aduhelm-new-alzheimers-drug-hhs-inspector-general/

The Alliance to Fight for Health Care
Advancing employer-provided coverage

May 17, 2023

The Honorable Vern Buchanan
Chairman
Committee on Ways and Means
Subcommittee on Health
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Lloyd Doggett
Ranking Member
Committee on Ways and Means
Subcommittee on Health
1139 Longworth House Office Building
Washington, DC 20515

Dear Chairman Buchanan and Ranking Member Doggett,

Thank you for the opportunity to submit the following comments for the hearing record in connection with the May 17, 2023, hearing, "Health Subcommittee on Why Health Care is Unaffordable: Anticompetitive and Consolidated Markets." We applaud the committee for working to address health care costs and for the opportunity to share ways in which the subcommittee can build on existing policy to lower health care costs for workers, employers, and the federal government.

The **Alliance to Fight for Health Care** is a diverse coalition comprised of businesses, patient advocates, employer organizations, unions, health care companies, consumer groups, and other stakeholders that support employer-provided health coverage. Together, we are working to ensure that employer-provided coverage remains an available and affordable option for working Americans and their families. The Alliance is dedicated to pursuing policies that increase competition and transparency to bring meaningful change — and cost savings — to our health care system and patients everywhere.

Employers want to address policies that, first and foremost, are driving up costs for patients. Between 2015-2019, prices for individuals with employer-sponsored insurance grew close to 18.3% while utilization grew just 3.6%. Growth in health care prices, and particularly inpatient hospital prices — which grew 24.6% — remains a persistent challenge to access and affordability. If we're going to help patients, we have to look at the problem.

A key variable in this equation is intensified market concentration and increasing consolidation. Many studies suggest that some versions of consolidation increases prices in the markets for both hospitals and physicians, as do certain forms of vertical integration among hospitals and physicians' groups. There is also a well-documented correlation between concentration in the provider market and prices, suggesting that some of the difference in prices in different areas is attributable to providers' market power. Unfortunately, due in part to perverse incentives that exist in the market today, the percentage of high- or very-highly concentrated markets has continued to grow in recent years. In 2010, the Congressional Budget Office (CBO) found that 63 percent of the 124 metropolitan statistical areas studied had highly or very highly concentrated hospital markets. By 2017, that share had risen to 70 percent, and the concentration of those already in the "highly concentrated" range intensified. The Alliance believes that we must address these perverse incentives through common-sense, bipartisan policies.

Further, the Alliance believes a hospital that is truly providing the highest quality care at the best prices should welcome additional transparency. Increased access to pricing and quality data will enable the

market to work more effectively and efficiently, and support employer efforts to innovate, ultimately leading to better costs and quality outcomes for patients.

A recent Morning Consult poll on health care issues conducted on behalf of the Alliance found **health care costs are the No. 1 concern among insured Americans**. What's more, 57% of insured adults said **reducing health care costs should be Congress' top priority**. But insured adults do not want to start over. Nearly 70% of insured adults, across the political spectrum, prefer to **strengthen the existing system.** Further, a majority of adults want Congress to work to lower the cost of health care for ALL Americans, not just those who receive coverage on the exchanges or in federal health care programs like Medicare and Medicaid.

The Alliance to Fight for Health care agrees. We want to work with the Congress to improve the U.S. health care system and reduce health care costs for ALL Americans by advancing policies to reduce health insurance premiums and increase affordability. And we come to the table with bipartisan ideas. We encourage Congress to continue the work of this committee to reduce costs, increase competition, and ultimately improve health outcomes for millions of American workers and their families by enacting polices to:

- **Remove restrictions preventing pro-patient competition in health care markets**
- **Protect patients from paying hospital prices for doctors' office visits**
- **Align value-based care incentives to benefit patients across all health care markets**
- **Give employers the flexibility to design programs to address chronic conditions and improve health outcomes**

Policy goal: Remove restrictions preventing pro-patient competition in health care markets

Employers want to create health plan designs that provide extra help to people with chronic or costly health conditions to improve health outcomes. Currently, "anti-tiering" and "anti-steering" clauses in contracts between providers and health plans restrict plans from creating innovative, high-value programs such as high-performance networks. Passing legislation like the Healthy Competition for Better Care Act (118th H.R. 3120) would enable more group health plans and health insurance issuers to enter into agreements with providers that guide enrollees to high-value providers and provide incentives to encourage enrollees to seek higher-quality, lower cost care. This legislation also aims to allow for positive forms of integrated provider and payor functions to allow these models to continue delivering efficient, high-quality care. There is significant support for such proposals. Recent polling by the Alliance indicates that 85% of insured adults feel employers should be able to give employees who have enrolled in their company's health plan a discount for seeing a high-quality provider.

Policy goal: Protect patients from paying hospital prices for doctors' office visits

The Alliance supports lowering the cost of health care services through policy proposals such as site-neutral payment reform. Current Medicare and private health insurance payment policies pay more for services provided in hospital outpatient departments (HOPDs) – usually provider offices owned by but not located in the hospital. According to the Medicare Payment Advisory Commission (MedPAC), this disparity is incentivizing health care consolidation and higher-health care costs. As shown in an AMA survey, currently fewer than half of physicians now work in physician-owned practices, a trend that has sharply risen since 2012.

MedPAC discussed the payment disparity in their June 2022 report to Congress, "[I]n 2022, Medicare pays 141 percent more in a hospital outpatient department than in a freestanding office for the first hour of chemotherapy infusion." As noted by MedPAC, "partly in response to these incentives, in recent years hospitals have acquired more physician practices, and hospital employment of physicians has increased." MedPAC also notes that the resulting increased reimbursements are not linked to clear benefits, such as improved quality of care for beneficiaries, but they are linked to increased costs for patients.

Congress can build on site-neutral payment reform by requiring Medicare to align payment rates for certain services across the three main sites where patients receive outpatient care — HOPDs, ambulatory surgical centers (ASCs), and freestanding physician offices. MedPAC, in its June 2022 report, estimated expanding site-neutral payment policies in Medicare could generate $6.6 billion in annual savings for Medicare and taxpayers and lower cost-sharing for Medicare beneficiaries by $1.7 billion.

The savings if voluntarily adopted by the commercial market are likely even greater. New research by University of Minnesota economist Steve Parente conducted on behalf of the Alliance estimates that expanding site-neutral payment reform in Medicare and encouraging adoption in the commercial market could result in nearly $60 billion in savings annually in the commercial market.

Requiring transparency in reporting where care is provided (i.e., a hospital or a physician's office) is another commonsense step that can help improve clarity for all consumers. These policies can all be designed to protect vulnerable rural or safety net hospitals, while protecting patients from climbing costs and consolidation. There is significant support for site-neutral payment reform. The Alliance's recent Morning Consult poll found 86% of insured adults, across political parties, believe health care costs should remain the same regardless of where the service is received.

Policy goal: Align value-based care incentives to benefit patients across all health care markets

The Alliance believes that federal cost reduction and quality improvement efforts should seek to improve the health care market for *all* beneficiaries. Encouraging collaboration between public and private providers and payors could accelerate beneficial changes for all participants. Creating pathways to engage the group health market in CMS Innovation Center (CMMI) models more meaningfully will promote multi-payer collaboration and encourage public-private partnerships that improve quality, reduce costs, and advance the system as a whole.

All patients should have a seat at the table in advance of future model development and be part of an open dialogue to promote coordination and learning to help improve the system together.

Policy goal: Give employers the flexibility to design programs to address chronic conditions and improve health outcomes

The Alliance also supports policies that reduce barriers to high value care, including enabling plans and employers to offer more high-value care pre-deductible. Laws and rules limiting pre-deductible coverage for chronic disease prevention, onsite medical clinics and telehealth inhibit employers' ability to offer high-value and potentially life-saving care to their employees on an equitable basis. Because of this, the Alliance supports legislation, including:

- The Chronic Disease Management Act (S. 655) (which allows greater flexibility to offer pre-deductible coverage for chronic disease prevention.
- The Telehealth Expansion Act (H.R. 1843/S.1001), which makes permanent the flexibility for plans to offer telehealth pre-deductible.
- Legislation that allows employers to provide more robust services (like chronic disease management and primary care) at onsite medical clinics pre-deductible without charging cost-sharing.

The Alliance supports meaningful steps toward introducing the necessary transparency, accountability, and consumer protections into our health care system to meaningfully reduce costs, improve outcomes, and drive towards value.

You can find a longer list of our recommended policies — including the barriers they aim to address — on our website at www.fightforhealthcare.com.

We look forward to working together on a bipartisan basis to increase competition and transparency that makes health care more affordable, supports continued innovation, improves job-based coverage, and advances the health care system for all patients.

Respectfully,

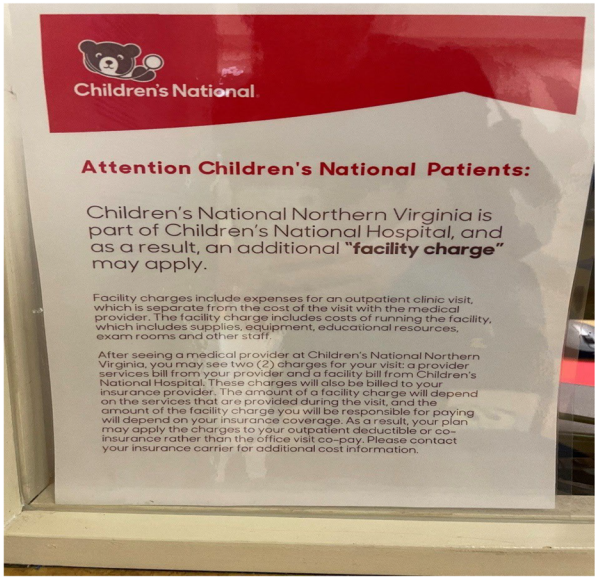
The Alliance to Fight for Health Care

APPENDIX

Same doctor. Same office. Should baby April pay more when they change the sign on the door?

When a physician's practice is bought by a larger hospital and the sign on the door changes, patients should not be forced to pay more. While the **Alliance to Fight for Health Care** appreciates the critical work hospitals do to care for patients and recognizes the challenges all sectors are facing given record-level inflation, patients should not be forced to pay hospital prices and hospital add-on fees for care that can be safely provided in doctors' offices. Site-neutral payment policies would reduce the incentives for hospitals to buy up physician practices, which will lower costs for patients.

This is an example of what happens to patients when a hospital buys their doctor's office. It shows a recent notice that baby April and her mom saw posted while checking in for their usual office visit last month. The office is over 11 miles from the hospital.



In case you missed it!
The News & Observer: "The health care didn't change. The office hasn't moved. Why is UNC now charging more?"

Sneaky fees are driving up health care costs for patients. A recent News & Observer article, "The health care didn't change. The office hasn't moved. Why is UNC now charging more?" highlights a growing trend of hospitals purchasing independent physician practices and clinics and charging patients more by adding so-called "facility fees."

The article describes how some UNC patients received a letter informing them that their dermatology clinic would be converted into a hospital-based clinic: "Almost everything about the health care at those clinics would stay the same, the letter assured patients. The location of the clinics, the doctors working there and the care they provided would not change." In fact, the only clear change, according to the letter, was an "additional 'facility fee' from UNC hospitals."

The article explains, "Health policy experts say this is an increasingly popular way for hospitals to get more money for providing the same care. By declaring free-standing clinics to be part of the hospital, they are able to tack on a facility fee, boosting their revenue."

The article quotes Ge Bai, a health policy researcher at the Johns Hopkins Bloomberg School of Public Health, who said, "It squeezes dollars from the pockets of patients and payers and channels them to the hospital's bank account."

The ***Alliance to Fight for Health Care*** **opposes hospital tactics that increase the financial burden on the patient and encourages Congress to expand site-neutral payment policy, which aims to align payment rates for certain services that are commonly and safely provided in lower-cost care settings.**

- The News&Observer

The health care didn't change. The office hasn't moved. Why is UNC now charging more?

By Teddy Rosenbluth

Published online March 13, 2023

Last month, some UNC Health patients received a letter informing them that three outpatient dermatology clinics would be converted into "hospital-based clinics."

Almost everything about the health care at those clinics would stay the same, the letter assured patients. The location of the clinics, the doctors working there and the care they provided would not change.

What will change, the letter pointed out, is how patients are charged for that care.

Beginning on March 6, patients of the clinics have been charged an additional "facility fee" from UNC Hospitals.

This fee, which one health policy expert researcher called a "revenue-generating gimmick," will almost always result in a more expensive bill for the patient and their insurance provider, said several experts interviewed by the N&O.

Health policy experts say this is an increasingly popular way for hospitals to get more money for providing the same care. By declaring free-standing clinics to be part of the hospital, they are able to tack on a facility fee, boosting their revenue.

“It squeezes dollars from the pockets of patients and payers and channels them to the hospital’s bank account,” said Ge Bai, a health policy researcher at the Johns Hopkins Bloomberg School of Public Health.

A NATIONAL TREND

In North Carolina, hospital-based clinics are common.

UNC Health operates 75, Duke Health 35 and WakeMed 24, according to spokespeople from the health systems. All charge facility fees.

Hospitals argue that facility fees are necessary to afford running large medical facilities at all hours of the day and night.

But critics question whether that facility fee is necessary for some of these clinics, like UNC’s dermatology offices, that keep regular hours and are miles away from a hospital. They point out that the health systems have many clinics that are not “hospital-based” and are able to operate without an added facility fee.

Hospitals have been purchasing and re-labeling independent physician clinics to boost revenues for the last decade or so, said Matthew Fielder, a health policy researcher at the USC-Brookings Schaeffer Initiative for Health Policy.

There is no statewide or national data on how many clinics have been “converted” into hospital departments in recent years.

However, a recent report to Congress found that people are increasingly seen by their doctors at places billed as hospital outpatient departments. The percentage of appointments at that type of facility rose from 9.6% in 2012 to 13.1% in 2019, the analysis found. That’s a 27% increase.

For patients, the change can result in hundreds or thousands of dollars added to their bills. One Ohio woman saw her portion of the bill for her arthritis injections increase from $30 to $354 after the clinic providing the injections was converted into a hospital department, Kaiser Health News reported.

Facility fees create a strong incentive for hospitals to buy up independent clinics and flip them into hospital clinics, said Barak Richman, a researcher at the Duke-Margolis Center for Health Policy.

This is particularly problematic in North Carolina, which has one of the most consolidated health care markets in the country.

“It’s a widespread phenomenon,” Richman said. “It has fueled consolidation for nothing but bad reasons.”

Alan Wolf, a spokesperson for UNC Health, said the billing changes were necessary to keep up with wage and pharmaceutical inflation, which he said has “far exceeded reimbursement for dermatology services.”

He said the change will allow the clinics to hire more staff and cut appointment wait times.

Fielder said he’s unaware of any evidence that shows this type of reclassification meaningfully improves access to care.

“There is, on the other hand, abundant evidence showing that changes like these increase providers’ revenues,” he said. “UNC has delivered these services in a physician office setting until now, and many other providers are continuing to do so.”

On the federal level, insurance companies have pushed for “site-neutral” Medicare billing, which would make clinic reimbursement rates the same regardless of whether they are independent or hospital-affiliated.

A report published last month by the Blue Cross Blue Shield Association found that adopting these policies could save the federal government, private health insurance companies and consumers a combined $471 billion over 10 years.

Bai said the best way to avoid facility fees at outpatient clinics is to call ahead and ask the billing department whether there will be a facility fee. If there is, she said patients could potentially find another provider.

However, she said this advice comes with an important caveat:

“The billing department might not be able to give a clear answer and patients might not have the time and energy to check when under stress.”

UNC HEALTH

Feb. 6, 2023

Dear Patient,

We are writing to let you know that UNC Dermatology and Skin Cancer Center's clinics will be converting to hospital-based clinics March 6, 2023.

We would like to let you know what this transition means for your future care. You will continue to see your same provider at the same location, and your provider will participate in the same insurance plans. You also will continue to have access to our highly skilled and compassionate care team. In addition, this transition allows our clinics to offer additional hospital-based resources and care that can only be obtained at an academic medical, teaching, and research facility such as UNC Hospitals. We look forward to providing our services to you and your family.

The names of our clinics will change to:
UNC Hospitals Dermatology & Skin Cancer Center at Southern Village
UNC Hospitals Dermatology & Skin Cancer Center at Raleigh
UNC Hospitals Dermatology & Skin Cancer Center at Hillsborough

Like our other hospital-based clinics, you (or your insurance provider) will be billed by both your provider and by the hospital. UNC Faculty Physicians will bill you for medical provider services such as those performed by a medical doctor, nurse practitioner or physician assistant. UNC Hospitals will bill you a facility fee, as well as for other services such as drugs or tests you receive during your visit. As a result of this change, your financial responsibility could differ from your copay amount/previous visits.

Your liabilities (charges) will be based on how your insurance processes claims based on the new hospital-based setting including deductibles, coinsurance and co-pays.

Our patient financial **representatives** at UNC Hospitals are available to assist you with understanding these billing changes. Please call our **Patient Accounts Department** at (984) 974-2222 or toll free at (800) 594-8624 if you need to speak with them.

Mohs surgery will now only be available at our Southern Village location. This service is not converting to a hospital-based clinic, and you will only be billed by UNC Faculty Physicians for Mohs surgical services. In addition, dermatopathology also is not converting to a hospital-based clinic, and you will only be billed by UNC Faculty Physicians for dermatopathology services.

Our providers and staff hope to make this transition as smooth as possible for you. You have a choice in medical providers, and we hope you will continue to rely on our practice for your healthcare needs. If you choose another healthcare provider, you will have full access to your medical records.

Thank you for trusting us with your care.

Teddy Rosenbluth covers science and health care for The News & Observer in a position funded by Duke Health and the Burroughs Wellcome Fund. The N&O maintains full editorial control of the work. This story was originally published March 13, 2023, 7:45 AM.

*The **Alliance to Fight for Health Care** is a diverse coalition comprised of businesses, patient advocates, employer organizations, unions, health care companies, consumer groups and other stakeholders that support employer-provided health coverage. Together, we are working to ensure that employer-provided coverage remains an effective and affordable option for working Americans and their families. The coalition (previously working as the **Alliance to Fight the 40**), led the successful effort to repeal the so-called 40% "Cadillac Tax" on health care coverage.*

@HealthCareFight | www.fightforhealthcare.com

# # #

Alzheimer's Association and Alzheimer's Impact Movement Statement for the Record

United States House Committee on Ways and Means, Health Subcommittee Hearing on "Examining Policies that Inhibit Innovation and Patient Access"

May 10, 2023

The Alzheimer's Association and Alzheimer's Impact Movement (AIM) appreciate the opportunity to submit this statement for the record for the United States House Committee on Ways and Means, Health Subcommittee hearing on "Examining Policies that Inhibit Innovation and Patient Access." The Association and AIM thank the Subcommittee for its continued leadership on issues important to the millions of people living with Alzheimer's and other dementia and their caregivers.

This statement highlights the urgency of addressing a harmful decision made by the Centers for Medicare and Medicaid Services (CMS) that continues to block access to Food and Drug Administration (FDA)-approved Alzheimer's therapies. Specifically, CMS' National Coverage Determination (NCD) on "Monoclonal Antibodies Directed Against Amyloid (mAbs) for the Treatment of Alzheimer's Disease" is imposing severe restrictions on access to the first class of treatments to change the course of Alzheimer's disease. We appreciate the growing bipartisan support in Congress for CMS to immediately open a reconsideration of this unprecedented decision and provide access to these breakthrough treatments, if patients with their clinicians decide such a treatment is right for them.

Founded in 1980, the Alzheimer's Association is the world's leading voluntary health organization in Alzheimer's care, support, and research. Our mission is to eliminate Alzheimer's and other dementia through the advancement of research; to provide and enhance care and support for all affected, and to reduce the risk of dementia through the promotion of brain health. AIM is the Association's advocacy affiliate, working in a strategic partnership to make Alzheimer's a national priority. Together, the Alzheimer's Association and AIM advocate for policies to fight Alzheimer's disease, including increased investment in research, improved care and support, and the development of approaches to reduce the risk of developing dementia.

Innovation and Breakthrough Treatments

Alzheimer's is one of the most significant health issues facing Medicare beneficiaries and their families, and now, for the first time, treatments have been approved by the FDA that change the course of the disease. Aducanumab (marketed as Aduhelm) received FDA accelerated approval on June 7, 2021 and lecanemab (marketed as Leqembi) received FDA accelerated approval on January 6, 2023. As with the first drugs in any class, additional therapies build upon initial breakthroughs to deliver more efficacious treatments. Lecanemab is proven to slow cognitive and functional decline over 18 months and significantly positively affect biological markers of Alzheimer's disease. In a study of 1,800 individuals in the early stages of Alzheimer's, lecanemab reduced the rate of cognitive decline by 27 percent. On well-established measures to assess the

quality of life for dementia patients and caregivers, it slowed decline by half. The peer-reviewed, published results show lecanemab will provide patients with more time to participate in daily life and live independently. This will mean patients have more months of recognizing their spouse, children and grandchildren. This will also mean more time for people to drive safely, promptly take care of family finances, and participate fully in hobbies and interests.

Adding to the strength of evidence around mAbs, on May 3, 2023, positive top-line results of the Phase 3 trial of donanemab were released and marked the strongest such results reported to date. The results showed donanemab met all of its primary and secondary endpoints, and slowed clinical decline by 35 percent compared to placebo on the primary outcome measure. According to the company, we anticipate the FDA issuing a traditional approval decision on donanemab as soon as the end of the year. Additional clinical trials are underway and offer the hope of additional treatments.

This is just the beginning of meaningful treatment advances. History has shown that approvals of the first drugs in a new category invigorates the field, increases investments in new treatments, and encourages greater innovation. The progress we've seen in this class of treatments and in the diversification of treatment types and targets over the past few years provides hope to those impacted by this devastating disease.

CMS Continues to Restrict Patient Access

While these breakthroughs are exciting and offer hope to those with Alzheimer's disease and their families, without Medicare coverage of this class of treatments, access for those who could benefit from these newly-approved treatments will only be available to those who can afford to pay out-of-pocket and find a health system willing to administer such treatments. Without coverage, people simply are not able to access treatments.

Unfortunately, in 2022, CMS implemented an unprecedented and restrictive NCD that not only applies to the two currently approved FDA-approved Alzheimer's therapies but also applies to all future treatments in the same class. Using coverage with evidence development (CED) requirements, CMS will only cover mAbs treating Alzheimer's approved through the accelerated approval pathway for individuals enrolled in randomized clinical trials, and treatments approved through the traditional approval pathway when patients are enrolled in "prospective comparative studies." This decision creates an immediate barrier to care for older Americans, especially individuals living in rural and underserved areas. Unless CMS immediately reconsiders the NCD, access to these Alzheimer's treatments will continue to be extremely limited, and for some nonexistent, by the agency's CED requirements even after traditional approval by the FDA.

Americans living with Alzheimer's disease are entitled to FDA-approved therapies, just as are people with conditions like cancer, heart disease and HIV/AIDS. And, they deserve the opportunity to assess if an FDA-approved treatment is right for them.

The Veterans Health Administration (VHA) now offers lecanemab for U.S. veterans. Medicare beneficiaries with early Alzheimer's deserve this same access, not delays. Treatments taken in the early stages of Alzheimer's would allow people more time to participate in daily life, remain independent and make health care decisions for their future.

Despite unequivocal evidence confirmed by the scientific community, CMS continues to state it is not "reasonable and necessary" for people living with Alzheimer's to access FDA-approved treatments without barriers. CMS has stated that it is not covering FDA-approved anti-amyloid treatments for Alzheimer's because it has a different standard than FDA. The CMS standard is defined in statute as "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member." Using that statutory definition, CMS has decided these treatments are unreasonable and unnecessary for the Medicare population, even though the treatments have been definitively shown to slow the progression of the disease and improve the quality of life for patients and their caregivers. This is unprecedented. CMS has never before determined an FDA-approved drug to not be reasonable and necessary.

CMS has said it views therapies approved under FDA's accelerated approval pathway differently than those approved under traditional approval. However, there is no scientific or medical justification for CMS to restrict access to a product that has demonstrated a clinical benefit in peer-reviewed randomized controlled clinical trials solely because it received approval from FDA under a pathway other than traditional approval. The accelerated approval pathway at the FDA is full approval. This fact continues to be reaffirmed by bipartisan members of Congress who established the accelerated approval pathway and by the FDA itself. Prior to these Alzheimer's therapies, CMS has provided Medicare coverage for every single FDA-approved drug under the accelerated approval pathway.

This decision sets a dangerous precedent that could stifle innovation for Americans who have no other options. If CMS continues to treat the accelerated approval pathway differently, it won't just be people living with MCI and early-stage Alzheimer's who are unable to access treatments that change the course of the disease, it will ripple down to rare diseases, cancer, and others. If Medicare will not cover new treatments under accelerated approval, it discourages the research industry from pursuing crucial treatments for populations with unmet needs. This delay could mean fewer therapies on a slower timeline when days, weeks, and months matter.

CMS has stated that it plans to cover these treatments the day they are approved under traditional approval at FDA. However the CED will still require patients to be enrolled in a prospective comparative study, referred to as a "claims-based registry" during CMS Administrator Brooks-LaSure's recent testimony before Congress. However, CMS has confirmed the fact that it has never before used a registry for a drug treatment, further raising concerns about the state of CMS' preparations to date. It is unclear how CMS plans to ensure equitable access, particularly for those living in rural and underserved communities, to the treatment via the claims-based registry. It is also not clear how CMS plans to collect the scientific data the agency states has not already yet been collected, or what that data is.

These new FDA-approved treatments taken in the early stages of Alzheimer's could mean a better quality of life. They allow people more time to participate in daily life, remain independent and make future health care decisions. These benefits will only be realized if patients have access to the treatments. Any barrier — whether cost, coverage, logistics, or knowledge — to accessing FDA-approved treatments is unacceptable and is not patient-focused.

Alzheimer's Community Losing Meaningful Time

Because of these unprecedented and unnecessary CMS coverage obstacles, people are losing the opportunity to discuss with their health care providers and their families if these treatments are right for them. They are losing days, weeks, months — memories, skills, and independence. They are losing time. And it is unacceptable.

Underscoring this urgency, based on Alzheimer's Association projections, **more than 2,000 individuals aged 65 or older transition per day from mild dementia due to Alzheimer's to moderate dementia due to Alzheimer's, and therefore outside the anticipated indicated population of these treatments.** Given the progressive nature of this terminal disease and the absence of treatment alternatives, delays are denying these Medicare beneficiaries the opportunity to benefit from this treatment. **As of May 10, that number is approximately 248,000 people who have progressed past the point of eligibility for lecanemab since it was first approved on January 6, 2023.**

All individuals, families, and caregivers facing a devastating, fatal disease deserve the opportunity to access FDA-approved treatments. As the Subcommittee will hear from Alzheimer's Association National Early-Stage Advisor Tony Gonzales, more time in the early stages of the disease is more than just the number of months or years. He wakes up every single day hoping to know who he is, who his wife is and who his kids are. If he is able to do that, it is a win. More time means one more day taking his grandson to the park, it means walking his daughter down the aisle, it means getting to meet his next grandchild. We need to listen to people living with the disease. They deserve the right to access these FDA-approved therapies now, while they still can, if they and their clinician decide it is right for them.

Growing Bipartisan Calls for Access to Treatments

The consequences of CMS' decisions are devastating for those with early symptomatic Alzheimer's disease — a progressive, terminal disease — who are currently denied access to FDA-approved treatments within their limited window of clinical eligibility. This is causing real harm to Medicare beneficiaries, leading to growing confusion and anger throughout the Alzheimer's and other dementia community.

Because of this impact on constituents across the country, there is growing momentum and political pressure on CMS to change its policy and stop blocking access to FDA-approved Alzheimer's treatments. In February, Representatives LaHood (R-IL) and Tonko (D-NY) led 72 bipartisan members in sending a letter to HHS and CMS emphasizing the importance of access

to FDA-approved Alzheimer's treatments. Senators Collins (R-ME) and Capito (R-WV) led a similar letter in the Senate, signed by 20 bipartisan leaders. During the numerous budget and legislative hearings in March and April, over 40 bipartisan members in the House and Senate sharply and repeatedly questioned HHS Secretary Becerra and Administrator Brooks-LaSure on why CMS continues to hold Alzheimer's treatments to a different standard than other diseases.

Adding to the nationwide support, in April, a bipartisan group of attorneys general from 26 states and territories sent letters urging HHS and CMS to reverse the unprecedented decision to block access to FDA-approved Alzheimer's therapies.

Despite this growing momentum and the urgency of the issue, CMS rejected the Alzheimer's Association's request for reconsideration even though it is obligated to do so when provided with, "additional scientific evidence that was not considered during the most recent review along with a sound premise by the requester that new evidence may change the NCD decision." That reconsideration request, submitted to CMS on December 19, 2022, included a letter signed by more than 200 Alzheimer's researchers and experts expressing their confidence in the lecanemab data, saying there should be "no barriers" to accessing the drug if it is approved.

The Alzheimer's Association and AIM thank the Chairman for reintroducing bipartisan legislation to ensure timely Medicare coverage of FDA-approved therapies. As no two treatments are the same, it is important that CMS evaluate them individually and based on their own scientific evidence, rather than one broad category. The Mandating Exclusive Review of Individual Treatments (MERIT) Act (H.R. 133) would require CMS to evaluate treatments and cures individually and based on their own merits, rather than as a broad class of drugs. We also support the bipartisan Access to Innovative Treatments Act (H.R. 2408) which would create a transparent process for ensuring that CMS responds and reconsiders drugs for Medicare coverage when sufficient data is collected on the drug's effectiveness.

Conclusion

With the recent lecanemab coverage announcement by the VHA and the likelihood of FDA traditional approval, in addition to the growing bipartisan support in Congress and the new donanemab data, CMS must immediately initiate a reconsideration of the harmful NCD. The initiation of the process itself is crucial. Declining to reopen the NCD upon traditional approval would further escalate the stark and expanding divide between CMS on one hand and the FDA and VHA on the other, as well as between CMS and the Alzheimer's community.

The Alzheimer's Association and AIM appreciate the steadfast support of the Subcommittee and its continued commitment to issues important to the millions of families affected by Alzheimer's and other dementia. We ask that the Subcommittee continue to stress the urgency to HHS and CMS of immediately opening a reconsideration of the NCD to remove the CED requirements for FDA-approved mAbs for the treatment of Alzheimer's, based on substantial new evidence published since the finalization of the NCD. We look forward to working with the Subcommittee and other members of Congress in a bipartisan way to ensure Medicare beneficiaries living with

MCI and early-stage Alzheimer's have immediate access to FDA-approved treatments, if the patient and clinician decide it is right for them.

U.S. House of Representatives
Ways & Means Committee
Subcommittee on Health

Hearing:
Examining Policies that Inhibit Innovation and Patient Access

May 11, 2023

Statement for the Record
American Academy of Dermatology Association

Chairman Buchanan and Ranking Member Doggett, on behalf of the more than 17,000 U.S. members of the American Academy of Dermatology Association (Academy or AADA), thank you for the opportunity to submit a statement for the record regarding your hearing, *Examining Policies that Inhibit Innovation and Patient Access.* The Academy applauds Congress for its actions to recognize policies that limit patients' ability to receive innovative and timely treatments. In dermatology, drugs and other therapies are frequently delayed or denied due to unnecessary prior authorization and step therapy policies. While we recognize there has been bipartisan support for prior authorization and step therapy reforms and appreciate the Centers for Medicare and Medicaid Services (CMS) recent action to address these burdens, further steps are needed to ensure patients' access to medically necessary and innovative treatments.

The Academy believes:

- Congress must direct the CMS to provide increased oversight of Medicare Advantage (MA) plans to ensure that they are not unnecessarily delaying or denying patients access to innovative therapies.

- Congress should direct CMS to extend its recent prior authorization policies as outlined in its proposed rule, "Advancing Interoperability and Improving Prior Authorization Processes," to include drugs to safeguard timely access to

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innovative treatments.

- Congress should require MA plans to develop a gold-carding policy for frequent treatments as it would alleviate administrative burdens placed on providers and, more importantly, protect beneficiaries' access to innovative care.

- Committee members should support bipartisan bill, the *Safe Step Act* (H.R. 2630), as it would ensure physicians remain the clinical authority over a patient's care.

Detailed recommendations can be found below.

Utilization Management Policies and the Impact on Patient Access to Innovative Therapies

Emerging therapies and technology to treat skin diseases continue to change the field of dermatology; however, patients face significant barriers to accessing these innovative treatments when MA and Part D plans implement unnecessary utilization management policies such as prior authorization and step therapy. The Academy has long advocated for solutions that remove prior authorization and step therapy policies that adversely impact patient care. For many skin diseases, new technologies for drugs and devices offer patients safer and more effective treatment options. These therapies, especially for chronic and complex skin conditions, are highly specialized and nuanced, and their efficacy is dependent on several patient factors. Prior authorization and step therapy policies that place a third party in a decision-making position, with no knowledge of the complexity or full history of a patient's condition, are not only inappropriate; they also impede a patient's access to the most effective treatment, and a delay can cause irreparable harm.

The Academy maintains that the clinically indicated choice of therapy should be respected and should rest on the patient-physician relationship where all critical factors—including efficacy and safety of all the treatment options, co-morbidities, and support system—are considered, fully discussed, vetted, and prescribed. Thus, prior authorization and step therapy policies must not be misused nor based solely on cost savings at the expense of clinical efficacy to ensure patient access to innovative treatments, especially those that offer less risk and better outcomes. We urge Congress to request that CMS increase its oversight of MA

plans so that they do not unnecessarily delay or deny treatment with unwarranted utilization management policies.

Timely Access to Innovative Treatments Through Prior Authorization Reforms

Following strong bipartisan support in the previous Congress, especially from those on the Committee, for the *Improving Seniors Timely Access to Care Act*, CMS released proposed rules on prior authorization reforms to ensure timely access for patient care. While we recognize and appreciate recent CMS action, including its proposed rule, "Advancing Interoperability and Improving Prior Authorization Processes," CMS stopped short of increasing patient access to innovative treatments by excluding drugs from the proposed policies.

Dermatology is disproportionately impacted by prior authorizations for both generic and brand drugs. We appreciate Congress working to address prior authorizations in the *SUPPORT for Patients & Communities Act* (Public Law No: 115-271), which was enacted into law in October 2018. Congress included language, that the Academy advocated for, to create a standardized electronic prior authorization form for Medicare prescription drugs intended to streamline and reduce prior authorization delays. While these policies have increased traditional Medicare beneficiaries' timely access to drugs, problems continue in Medicare Advantage and Part D.

Prescription drugs account for the majority of prior authorization requests in dermatology. The Academy's 2020 Prior Authorization Survey found that approximately 25% of patients that come to a dermatology practice require prior authorization.[1] On average, dermatology offices have spent $40,000 on additional staff to help manage the prior authorization process, which takes 3.5 hours each day. In fact, dermatologists could see an additional 5 to 8 patients daily if no prior authorization was required. Needless to say, unwarranted prior authorization policies, especially those implemented for high-volume treatments, are a tactic used to exhaust providers, particularly those in small or solo practices who may not be able to devote the time and energy to the prior authorization process. Patients are ultimately deprived of access to medically necessary and innovative treatments due to unnecessary prior authorization policies.

To address timely access to innovative therapies, CMS needs to expand its

[1] https://www.aad.org/dw/monthly/2020/october/facts-at-your-fingertips-prior-auth-practices

electronic prior authorization and payer policies in its proposed rule to include drugs. AADA calls on Congress to direct CMS to extend its recent prior authorization and payer policies in its proposed rule, "Advancing Interoperability and Improving Prior Authorization Processes," to include drugs to safeguard patients timely access to innovative treatments.

Gold-Carding Could Increase Timely Access to Innovative Care

The Academy recommends that Congress direct CMS to implement a gold-carding policy similar to the *Getting Over Lengthy Delays in Care as Required by Doctors (GOLD CARD) Act of 2022* (H.R. 7995, 117th Congress) to increase timely access to innovative care for patients. "Gold-carding" is a type of program to improve efficiency and reduce burden on practices by exempting providers from prior authorization requirements if they have demonstrated a consistent pattern of approvals. AADA would be supportive of legislation that would exempt physicians from prior authorization requirements for the plan year if at least 90% of prior authorization requests were approved the preceding year.

In the CMS proposed rule, "Advancing Interoperability and Improving Prior Authorization Processes," CMS states that "gold-carding programs could help alleviate the burden associated with prior authorization and that such programs could facilitate more efficient and timely delivery of health care services to enrollees." In fact, CMS notes the success they have seen with similar programs they have implemented, such as the one they use in the Medicare Fee-for-Service Review Choice Demonstration for Home Health Services.

Gold-carding is a common-sense reform that will help reduce barriers to care, allow physicians to spend more time with patients, and put treatment decisions back where they belong – in the hands of physicians and patients. The AADA urges Congress to direct CMS to develop a gold-carding policy that would protect beneficiaries' access to receiving innovative services and medications in a punctual manner.

Step Therapy Policies Delay Patient Access to Innovative Therapies

Step therapy or "fail first" policies have been shown to inhibit patient access to life-changing therapies and adversely impact patient outcomes. Step therapy is often used as a cost containment tool by health insurance plans, requiring patients to try one or more prescription drugs before coverage is provided for a drug selected

by the patient's health care provider. Requiring patients to try and fail treatments jeopardizes the health of patients, potentially resulting in dangerous consequences. Step therapy incorrectly assumes that all patients start care at the same point in their disease process, and that the trajectory of their condition will be the same. It must also therefore make exceptions for stage and extent of disease, patient characteristics and current treatment, including if the provider believes the recommended course of action by the carrier could cause harm to the patient. In general, patients must be able to have access to alternative treatments if the first line option is not optimal or contraindicated.

While the Academy understands the need to contain health care costs, we are concerned that step therapy policies often do not take into account: a patient's medical history; whether or not the patient has already tried a certain drug and failed; if a patient has a medical condition that would interfere with the efficacy of the drug; if a drug's side effects would interfere with the patient's ability to perform their job, and; if the drug best for the patient is one with a different ingestion method or dosage form.

Due to this dangerous and burdensome practice, AADA urges members of the Committee to support bipartisan bill H.R. 2630, the *Safe Step Act*, which would ensure physicians remain the clinical authority over a patient's care, and to lessen the burden on patients required to go through step therapy protocols instituted by insurance companies. Modeled after state legislation, which the Academy is on record supporting through the State Access to Innovative Medicines (SAIM) Coalition, the bill provides a process for patients to easily access a request for an exception to step therapy protocol. The bill applies to insurance plans regulated by the federal Employee Retirement Income Security Act (ERISA). The bill would also require insurance companies to approve an exception request within three days, or 24 hours in the event of an emergency when the patient's life or health is in danger. To date, 35 states have enacted step therapy reform laws.

Conclusion

On behalf of the Academy and its member dermatologists, I thank you for holding this hearing, allowing the opportunity for stakeholders to submit a statement for the record, and for your commitment to ensuring patient access to innovative and life-changing treatments. The Academy looks forward to working with you and asks that you continue to consider including physician stakeholders' opinions in your ongoing hearings. As the Committee considers the challenges facing patient access to

innovative therapies, we look forward to being a reference for this issue and others in the future.

The Academy appreciates your leadership on these issues and asks that the Subcommittee please consider the impact of these policies on the welfare of patients and unnecessary increased cost to the health care system.

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May 3rd, 2023

Secretary Lisa Barton
U.S. International Trade Commission
500 E Street, SW
Washington, D.C. 20436

Re: Comment on COVID-19 Diagnostics and Therapeutics: Supply, Demand, and TRIPS Agreement Flexibilities, Investigation No. 332-596

Dear Secretary Barton,

On behalf of Americans for Tax Reform, we appreciate the opportunity to provide input regarding Investigation No. 332-596, *COVID-19 Diagnostics and Therapeutics: Supply, Demand, and TRIPS Agreement Flexibilities*[1]. We urge the U.S. International Trade Commission (USITC), in its resulting report, to recognize the vital importance of intellectual property (IP) protections and recommend against any expansion of the TRIPS waiver.

Americans for Tax Reform (ATR) is a taxpayer advocacy organization that opposes all tax increases and supports limited government, free-market policies. Strong IP rights are key feature of the free market, particularly in healthcare – they ensure manufacturers are incentivized to innovate, ensure medicines are safe and effective, and have the resources to invest in the next generation of cures.

IP rights are so important that they are explicitly protected in the Constitution. The Founding Fathers recognized the importance of intellectual property rights in Article 1, Section 8 of the Constitution: "To promote the Progress of Science and useful Arts, by securing for limited times to Authors and Inventors the exclusive Right to their respective Writings and Discoveries."

Last year, the Biden administration, with the World Trade Organization (WTO), made the mistake of agreeing to waive IP protections under the TRIPS Agreement for COVID-19 vaccines. [2] During negotiations regarding this agreement, the United States Trade Representative (USTR) failed to consult with Congress, though it is the USTR's duty to provide substantial briefings on negotiations to and share all negotiating texts with Congress. This failure prompted a letter from a bipartisan group of Senators calling on the USTR to be more transparent.[3]

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[1] COVID-19 Diagnostics and Therapeutics: Supply, Demand, and TRIPS Agreement Flexibilities; Notice of Investigation and Scheduling of a Public Hearing," 88 Fed. Reg. 7757 (Feb. 1, 2023). https://www.usitc.gov/secretary/fed_reg_notices/337/332_596_notice_02012023sgl.pdf

[2] World Trade Organization, Ministerial Decision on the TRIPS Agreement, WT/MIN(22)/30, WT/L/1141 (Jun. 17, 2022), https://docs.wto.org/dol2fe/Pages/SS/directdoc.aspx?filename=q:/WT/MIN22/W15R2.pdf&Open=True

[3] "Bipartisan Senate Finance Committee Members Call for Improved Transparency from U.S. Trade Representative; Cite Failure to Consult with Congress on Recent Trade Negotiations: The United States Senate Committee on Finance." United States Senate Committee On Finance, 10 May 2022, https://www.finance.senate.gov/ranking-members-news/bipartisan-senate-finance-committee-members-call-for-

As the WTO is considering the expansion of the waiver to include COVID-19 diagnostics and therapeutics, it is the USITC's job to examine the short- and long-term consequences of this policy. According to the USTR, this report will help inform whether the United States will support an expansion of the TRIPS waiver.

Further undermining IP protections will weaken manufacturers' incentives to innovate new cures and treatments, will reduce investment in medical innovation, and will threaten the strength of the U.S. biopharmaceutical sector.

Developing new medicines is a costly, risky, and time-consuming process. Without IP protections, there is no guarantee that manufacturers will recoup the time and money they invested in the project. During an average drug development process, a manufacturer must invest an average of $2.6 billion[4] and spend 11.5 to 15 years in research and development.[5]

Even so, most drug development programs fail.

As detailed by Stephen Ezell of the Information Technology & Innovation Foundation (ITIF), for 5,000 to 10,000 compounds screened during basic drug discovery phases, 250 molecular compounds (2.5 to 5 percent) make it to preclinical testing. Of the 250 molecular compounds, 5 make it to clinical testing. Thus, as little as 0.05 percent of drugs make it from drug discovery to clinical trials.[6]

Of the few medicines that make it to clinical testing, only about 12 percent of medicines that begin clinical trials are approved for introduction by the FDA.[7]

Even if a drug is approved, it is likely that the profits from said drug will not recoup its R&D costs. One study in the Health Economics journal found that 80 percent of new drugs made less than their capitalized R&D costs.[8]

Certainly, drug development is a high-risk business. The last thing this industry and its investors need are more disincentives to innovate; for example, the looming threat of their IP rights being stripped from them by an expanded TRIPS waiver.

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improved-transparency-from-us-trade-representative-cite-failure-to-consult-with-congress-on-recent-trade-negotiations.

[4] Sullivan, Thomas. "A Tough Road: Cost to Develop One New Drug Is $2.6 Billion; Approval Rate for Drugs Entering Clinical Development Is Less than 12%." Policy & Medicine, 21 Mar. 2019, www.policymed.com/2014/12/a-tough-road-cost-to-develop-one-new-drug-is-26-billion-approval-rate-for-drugs-entering-clinical-de.html.

[5] Stephen J. Ezell, "The Bayh-Dole Act's Vital Importance to the U.S. Life-Sciences Innovation System" (ITIF, March 2019), 24–25, https://itif.org/publications/2019/03/04/bayh-dole-acts-vitalimportance-us-life-sciences-innovation-system.

[6] Stephen J. Ezell, "Ensuring U.S. Biopharmaceutical Competitiveness" (ITIF, July 2020), https://itif.org/publications/2020/07/16/ensuring-us-biopharmaceutical-competitiveness.

[7] "Research and Development in the Pharmaceutical Industry." Congressional Budget Office, Apr. 2021, www.cbo.gov/publication/57126.

[8] Vernon JA, Golec JH, Dimasi JA. Drug development costs when financial risk is measured using the Fama-French three-factor model. Health Econ. 2010 Aug;19(8):1002-5. doi: 10.1002/hec.1538. PMID: 19655335.

Most importantly, in healthcare, the consequences of a lack of medical innovation are a matter of life and death. Reduced investments mean less research into cures and/or treatments for cancer, Alzheimer's, heart disease, brain disorders, HIV/AIDS, and more.

Importantly, without IP protections, COVID-19 vaccines would not have been completed or distributed as quickly as they were. Allowing the seizure of IP through an expanded TRIPS waiver would undermine this system of medical innovation which, ironically, paved the way for the products the WTO seeks to strip IP rights from.

Further undermining IP rights will also harm American workers and industry strength. IP supports millions of high-paying jobs across the country.

According to the United States Patent and Trademark Office (USPTO), IP-intensive industries accounted for $7.8 trillion in GDP in 2019, or 41 percent of the economy. These industries accounted for 47.2 million jobs, or 33 percent of total U.S. employment.[9]

Pharmaceutical manufacturers are no exception – these businesses invest over $100 billion in the U.S. economy every year, directly supporting over 903,000 jobs.[10] When indirect jobs are included, pharmaceutical innovation supports 4.4 million jobs and $1.4 trillion in total economic impact.[11] These jobs are high paying – the average compensation is over $145,000 – nearly $60,000 more than other industry averages in the U.S.[12]

Expanding the TRIPS waiver to cover COVID-19 diagnostics and therapeutics would further undermine IP rights, thus threatening medical innovation, vital investment, and American jobs. It is imperative that the USITC, in its report, correctly recognize the dangerous, long-term consequences of waiving IP rights.

Additionally, unilateral decisions by the Executive Branch to waive IP rights under the TRIPS Agreement inappropriately circumvent Congress's constitutional authority over trade policy. Certainly, any conclusion the USTR comes to does not override Congress's Constitutional authority to regulate commerce with foreign nations, or the constitutional mandate to protect IP rights.

Onward,

Grover Norquist
President, Americans for Tax Reform

Isabelle Morales
Federal Affairs Manager, Americans for Tax Reform

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[9] "Intellectual Property and the U.S. Economy: Third Edition." United States Patent and Trademark Office, Department of Commerce, 17 Mar. 2022, https://www.uspto.gov/ip-policy/economic-research/intellectual-property-and-us-economy.
[10] "The Economic Impact of the U.S. Biopharmaceutical Industry: 2020 National and State Estimates." PhRMA, Mar. 2022, https://phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/0-9/2020-Biopharma-Jobs-ImpactsMarch-2022-Release.pdf.
[11] Ibid.
[12] Ibid.

Bayh-Dole COALITION

Celebrating the past. Protecting the future.

May 10, 2023

The Honorable Vern Buchanan
Chairman
Subcommittee on Health
House Ways and Means Committee
1139 Longworth House Office Building
Washington, D.C. 20515

The Honorable Lloyd Doggett
Ranking Member
Subcommittee on Health
House Ways and Means Committee
1139 Longworth House Office Building
Washington, D.C. 20515

Dear Chairman Buchanan and Ranking Member Doggett:

On behalf of the Bayh-Dole Coalition, I appreciate having the opportunity to provide comments in advance of your May 10 hearing "Examining Policies that Inhibit Innovation and Patient Access." Specifically, I'd like to draw your attention to past policies and present proposals that deter companies from commercializing medical technologies that originate at federally-funded academic research centers and federal laboratories which are at the cutting edge of life science research.

The coalition is a diverse group of innovation-oriented organizations committed to celebrating and protecting the Bayh-Dole Act, which jumpstarted American innovation by allowing federally-funded research universities, small companies, and nonprofit labs to retain and license the patents on the discoveries they made. Nowhere has this impact been greater than in the creation of badly-needed drugs, vaccines, and other medical therapies.

Prior to that 1980 law, the federal government retained the patent rights for the research discoveries it fully or partially funded. Bureaucrats licensed less than 5% of those patents to companies that could turn good ideas into real-world products for consumers. Even worse, not a single new drug was developed despite the billions of taxpayer dollars invested in National Institutes of Health R&D under those policies, policies which destroyed incentives for the private sector to assume the tremendous risk and expense necessary to turn federally-funded inventions into useful products. And nowhere are these risks and costs greater than in drug development which can easily cost companies $2.6 billion on average with little chance the drug will make it through the development pipeline.

Thanks to our system of public-private sector R&D alliances made possible by Bayh-Dole, today, the United States leads the world in the life sciences. We are particularly unique in that half of our new drugs originate in small companies. While no drugs were developed under prior government patent policies, under Bayh-Dole, at least 300 new drugs and vaccines are now fighting disease here and abroad.

By allowing universities to own and manage their federally-funded inventions, we launch three new companies and nearly three new products based on academic patents every day of the year. No other country comes close to this success. Currently, more than 15,000 startup companies spun out of campuses help drive our economy, keeping us at the forefront of innovation.

Despite these successes, there are those who want to return us to the pre-Bayh-Dole era of stagnation. We have seen firsthand that such efforts do not work. For example, the federal government found out the hard way that undermining patent licenses would cause companies to pull back from public-private partnerships. In the late 1980s, the National Institutes of Health began inserting a "reasonable pricing" clause in licensing deals known as cooperation research and development agreements, or CRADAs. The clause essentially gave the NIH the ability to relicense a patent if it objected to the price of any commercialized product. Proponents predicted that this provision would lead to reducing drug prices. That never happened. What did happen was companies walked away from NIH partnerships.

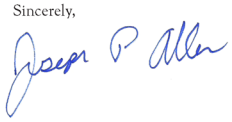
The policy change caused the number of CRADAs signed to plummet, and the NIH reversed course in 1995 after concluding that "the pricing clause has driven industry away from potentially beneficial scientific collaborations with PHS (public health service) scientists without providing an offsetting benefit to the public. Eliminating the clause will promote research that can enhance the health of the American people."

There are also those who urged the Biden administration to abuse the Bayh-Dole Act's "march-in" rights and invoke them to relicense patents on drugs they deem too expensive. Because the law provides no such authority, every petition filed on this basis has been correctly dismissed by every administration, Democratic or Republican, which considered them. The Biden administration was the most recent, dismissing a petition to march in on the prostate cancer drug, Xtandi. That was the fourth time this particular petition has been appropriately dismissed.

Unfortunately, such attempts to misuse the law make many industry partners, particularly small companies, wonder if the government can be trusted to enforce the law as written. If this confidence is lost, we face great peril as evidenced by the unprecedented rallying of our public and private sectors to combat the Covid-19 pandemic. That effort will not be replicated if we ever allow the Bayh-Dole Act to be misused by its opponents.

Our patent-based Bayh-Dole system works. It is a recognized international best practice. For these reasons, I urge the committee to recognize that strong and predictable intellectual property protections are necessary for public-private partnerships to succeed and produce new lifesaving therapies for patients.

Sincerely,



Joseph P. Allen
Executive Director
Bayh-Dole Coalition

CC: Chairman Jason Smith

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Biotechnology Innovation Organization
Statement for the Record
U.S. House Committee on Ways & Means Subcommittee on Health
Examining Policies that Inhibit Innovation and Patient Access
May 10, 2023

The Biotechnology Innovation Organization (BIO) appreciates this opportunity to present these comments to the Committee as it examines policies that will have negative effects on medical innovation and reduce patient access to therapies. Our comments focus on two areas that threat patient access to innovative medicines in Medicare: (1) the anti-innovation policies enacted as part of the Inflation Reduction Act (IRA) (2) government policies that that are blocking patient access to new FDA-approved treatments.

BIO is the world's largest trade association representing nearly 1,000 biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than thirty other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, delay the onset of such diseases, or prevent them in the first place. As a result, our members' novel therapeutics, vaccines, and diagnostics not only have improved health outcomes but also have reduced health care expenditures due to fewer physician office visits, hospitalizations, and surgical interventions.

IRA Impacts and Recommended Actions

The IRA authorizes the Secretary of Health and Human Services to "negotiate" the price Medicare pays for certain medicines. With stiff penalties for companies that don't comply, these are not so much negotiations but more aptly named, "price controls". These price caps will be imposed on 100 medicines in the Medicare program by 2031. These government price controls will hurt innovators – particularly small biotech – and patients desperate for new treatments.

New medicines are extremely costly to develop, requiring enormous amounts of private investment – but the IRA threatens these investments. Health consulting firm Avalere estimates that the IRA will cost biotech companies $450 billion over the next decade. Such a staggering reduction in revenue will obviously lead to cuts in R&D spending. According to estimates by University of Chicago economist Tomas J. Philipson, the IRA's price controls could result in 135 fewer new drug approvals for patients and the consequent loss of 331 million life years by 2039.

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Certain areas of research will feel the impact more than others because the IRA's price controls apply differently to different kinds of medicine. So-called "small molecule" drugs are subject to price controls just nine years after earning FDA approval. By contrast, biologics – complex medicines derived from natural sources – are subject to price controls after 13 years. Most pharmaceuticals on the market today, including, for example, 89 anti-tumor drugs for treating cancer, are small molecule. But the IRA disincentivizes and penalizes this critical research and robs patients of life-changing new treatments. Further research on oncology medicines continues after FDA approval. That's when scientists perform additional tests to determine whether a medicine developed to treat one cancer is effective at treating another. But the threat of near-term price controls makes companies much less likely to invest in additional post-approval research.

We're already seeing companies move away from small-molecule research. For instance, Eli Lilly said it would stop work on a small-molecule treatment for blood cancer that was already in clinical trials.[1] Novartis and GSK have also cancelled or suspended cancer-drug projects.[2] Cancer isn't the only research area that will suffer. For example, for neurological diseases like Alzheimer's, small-molecule medicines offer some of our best prospects for breakthroughs. Meanwhile, Alnylam recently ended plans to test its drug Amvuttra to treat the rare Stargardt eye disease, citing the potential impact of the IRA.[3]

This unfortunate trend is likely to worsen as long as IRA price controls remain in place. To address it, Congress should repeal this price control mechanism. Absent repeal, critical steps should be taken to help mitigate the IRA's damaging effects. An important first step would be to apply the same 13-year window to both small-molecule drugs and biologics.

Other steps should be taken as well. The orphan drug exemption from price controls is too limited and will stifle research and development into rare and hard-to-treat diseases. Specifically, orphan drugs designated for *only one* disease or condition and approved for only that one disease or condition are exempt from negotiation. Any subsequent designations – even for another orphan condition – would result in the elimination of the exemption for all orphan conditions. This exemption should be modified to allow for multiple orphan indications to be exempt from price controls. According to IQVIA, of the 564 drugs

[1] https://www.reuters.com/business/healthcare-pharmaceuticals/drug-companies-favor-biotech-meds-over-pills-citing-new-us-law-2023-01-13/
[2] https://endpts.com/eli-lilly-rolls-snake-eyes-as-it-axes-two-early-stage-drugs-including-a-40m-cancer-therapy-from-fosun/#:~:text=Senior%20Editor,a%20%2440%20million%20cancer%20drug.
[3] https://www.bloomberg.com/news/articles/2022-10-27/alnylam-halts-work-on-eye-drug--citing-new-us-law-over-pricing

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with orphan approvals, 104 of these drugs are approved for two or more indications – most of which are for rare cancers and blood disorders.[4]

In addition, the time-limited exemption from price controls for small biotech drugs – which currently expires in 2029 – should be made permanent. Biotech companies generally focus on early- and mid-stage research, and they typically lack the resources to conduct late-stage, hugely expensive clinical trials or build out a worldwide sales and distribution network. That's why they often partner with larger companies that have more production and distribution experience. Vital Transformations recently analyzed a cohort of 363 new medicines approved by the FDA between 2011 and 2020 and found that 55 percent were developed by small firms with less than $500 million in annual revenue. But it was large companies who managed post-FDA approval development, marketing, and scale for many of these medicines. The success of this diversified ecosystem has led to a 152 percent increase in U.S. external R&D partnerships and investments since 2011, per Vital Transformations estimates.

CMS Implementation – Recommended Improvements

Congress should also increase its oversight of the Centers for Medicare & Medicaid Services (CMS) as the Agency moves forward in implementing the IRA's price negotiation program. It is critical that CMS implement this program in a fair, predictable, and transparent manner with the ultimate goal of maintaining patient access to all necessary therapies.

To that end, we note our strong disappointment that key aspects of CMS' draft initial guidance were issued as final without allowing for comments from stakeholders, which is a concerning step backward from CMS's stated commitment to transparency. The need for such a fulsome process is especially acute here, given the novelty and complexity of the Negotiation Program; the vast ramifications that the program will have for patients, providers, pharmacies, manufacturers, and countless other stakeholders; and the potentially profound negative repercussions for patient access to needed therapies that could follow from errors, misunderstandings, or gaps in understanding. In these circumstances, the Agency should maximize transparency and engagement in its decision-making process, including by both affording a full opportunity for comment and meaningfully responding to stakeholder feedback.

A critical policy that CMS finalized without opportunity for comment was its decision that, in determining which drugs are eligible for negotiation, it would not treat drugs approved under unique New Drug Applications (NDAs) or Biologics License Applications (BLAs) as distinct drugs but, rather, would combine NDAs and BLAs with the same active

[4] See: https://www.iqvia.com/insights/the-iqvia-institute/reports/orphan-drugs-in-the-united-states-rare-disease-innovation-and-cost-trends-through-2019

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moiety/active ingredient together for negotiation purposes. CMS must reverse this policy as it is bad for innovation, bad for patients, and not supported by the statute. CMS's approach leaves no incentive for therapeutic advancement and will have significant, negative impacts on innovation for years to come. Biopharmaceutical innovation is incremental, relying on sustained and continuous improvements to molecules, pathways, and modes of administration to achieve maximum clinical benefit for patients. Researchers cannot take significant leaps and develop new active moieties with each generation of treatment. By combining drugs at the active moiety or active ingredient level, CMS is harming investments into new therapies, including for orphan and hard to treat diseases. For the sake of pharmaceutical and biotechnology innovation, and patient access to needed therapies, CMS's current framework cannot stand.

CMS also needs to take a number of steps to ensure that its negotiation process is fair, predictable, and transparent. The statute mandates that CMS "develop and use a consistent methodology and process" for MFP negotiation. Although no two negotiations will ever be identical—because the circumstances of each selected drug are unique—all negotiations should be subject to a clear and reasonable framework. A consistent process not only is statutorily required but also helps to ensure that CMS complies with its obligation to treat similarly situated entities in a similar manner, absent a reasoned basis for distinction. CMS's proposed process falls far short of these principles.

We recommend a number of actions CMS should take to enable a negotiation process that allows for meaningful engagement and dialogue between CMS and manufacturers, including providing for in-person meetings throughout the process. Manufacturers should also be permitted to supplement initial submissions to CMS. Permitting supplemental submissions is well warranted. Under the statute, manufacturers are given only one month to prepare a voluminous submission of complex information, including information regarding Non-Federal average manufacturer price (non-FAMP); research and development costs; production and distribution costs; federal financial support for discovery and development; pending and approved patent applications, FDA exclusivities, NDAs or BLAs and approvals thereof, market data; and revenue and sales volume data. In some cases, requested data may also not exist in a format required by CMS, such that the manufacturer will need to painstakingly convert raw data from multiple sources into such a format. Manufacturers will assuredly work with utmost diligence to comply with CMS's submission requirements. Still, they may need the flexibility of a supplement to their timely submission for legitimate reasons.

In addition, CMS should provide a meaningful justification of its initial offer to a manufacturer, as well as any response to a manufacturer's counteroffer and afford the manufacturer a meaningful opportunity to comment on the response the MFP is set. As with any good faith negotiation, open dialogue will be vital to the success of the MFP negotiation. To this end, CMS should specify that its initial offers and its responses to any counteroffers include meaningful explanations of how the Agency arrived at the offer or response,

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including how the offer or response is supported by the statutorily enumerated negotiation factors and any other information upon which the Agency relied, and how the Agency considered and weighted such factors and information.

CMS must also clarify how its review of the evidence will inform its setting of the MFP. CMS's approach remains unclear and presents untenable levels of uncertainty. Essentially, CMS has said it will use the net price of the "therapeutic alternatives" of drugs selected for negotiation as a starting point and then adjust this starting point based on its review of the clinical evidence. In addition, CMS has said it may make further adjustments based on other data manufacturers are required to submit, such as "recoupment" of research and development costs. But CMS has not provided a framework for how it will review all this evidence. Nor has the agency indicated how certain evidence or factors will be weighed. This lack of clarity and uncertainty is of great concern and our position is that CMS should clarify its standards for evidence review and be transparent and accountable about what evidence drove its decisions in setting the MFP and why. Further, CMS's review of the evidence should focus on factors that are critical for patients, specifically factors related to clinical benefit and unmet medical need and de-emphasize manufacturer specific data elements such as cost of production and research and development costs.

Finally, CMS should eliminate its proposed, one-sided requirement that manufacturers destroy all records related to the negotiation process and submit a Certificate of Data Destruction to CMS certifying that all information received from CMS during the negotiation period and potential renegotiation period(s) was destroyed. Basic due process mandates that manufacturers be given the ability to maintain records related to negotiation proceedings. Moreover, BIO opposes the blanket prohibition on manufacturers from disclosing or otherwise publicizing information "in the initial offer, including the ceiling price, or the concise justification from the Secretary or any subsequent offer of concise justification, nor information derived from those justifications or offers…". This one-sided information control heightens the ultimate public complaint that the entirety of the "negotiation" process is anything but actual "negotiation." BIO disagrees with this approach – which essentially allows CMS to operate in secret with no accountability – and recommends CMS abandon it.

What is more, CMS appears to be making a more general affront to the protected speech of affected manufacturers. As has been reaffirmed many times before, prior restraints on speech are presumptively unconstitutional.[5] The government faces a heavy burden in showing a compelling interest in keeping negotiation discussions private, and we fail to see a legitimate reason why the government's interests are so advanced by muzzling private

[5] *See, e.g., Near* v. *Minnesota* 283 U.S. 697 (1931).

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companies in the context of Medicare price negotiation discussions.[6] In fact, in this instance, any potential disclosure by a manufacturer would likely relate to truthful information that is, at a minimum, of significance to at least a portion of the public involved in the transaction of health insurance and health consumption. As such, we recommend CMS abandon these burdensome and unnecessary confidentiality and anti-disclosure provisions.

Government Action that Harms Access: Limiting Coverage for Drugs Approved under FDA's Accelerated Approval Pathway

Originally conceived to address one of the world's most daunting public health challenges—the AIDS epidemic—and reinforced for use in cancer and rare diseases, the FDA's accelerated approval pathway has yielded more than 270 treatments over its 30 years.[7] These treatments give patients with life-threatening diseases therapeutic options where minimal or none previously existed. Yet, this approval pathway has come under attack by both public and private payers, claiming accelerated approval drugs are improperly driving spending and questioning the FDA's approval decisions.

Under the accelerated approval pathway, the FDA may approve a drug intended to ameliorate serious unmet medical need that demonstrates safety and efficacy in well-controlled clinical trials where efficacy is based on a surrogate or an intermediate clinical endpoint that is reasonably likely to predict clinical benefit, rather than the primary clinical outcome that may take years to measurably manifest.

Yet, critics of the pathway mistakenly claim that accelerated approval drugs do not meet the FDA's "full" standard for safety and efficacy. These concerns are the basis for current policy proposals proposed by several state Medicaid programs, the Medicaid and CHIP Payment and Access Commission (MACPAC) and recent comments by the Medicare payment Advisory Commission (MedPAC) These off-base pronouncements come, even as the FDA has been clear that approval through its accelerated approval program is no half measure or anything less than "full" approval.[8]

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[6] As has been reaffirmed in many instances by the US Supreme Court, the government must articulate a compelling government need for the negotiation to remain out of the public discourse and must simultaneously introduce a narrowly tailored method for so restricting this discussion. In the context of this guidance, we see no such articulation of either a compelling need nor a narrow restriction. In fact, we see just the opposite. *See, e.g., New York Times Co.* v. *United States,* 403 U.S. 713 (1971).

[7] Kenneth E. Thorpe and Thomas L. Johnson, "Accelerated Approval Drugs Are Not Driving Medicaid Spending" Health Affairs, June 3, 2022.

[8] FDA, "Accelerated Approval Program," Last Updated January 30, 2023. Accessed May 5, 2023. https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approval-program#:~:text=The%20FDA%20instituted%20its%20Accelerated,based%20on%20a%20surrogate%20endpoint.

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To further illustrate the gravity of the issue, CMS recently announced the decision that Medicare would cover monoclonal antibodies targeting amyloid plaque for the treatment of Alzheimer's only if they have received traditional (i.e., not accelerated) approval from the U.S. Food and Drug Administration (FDA); drugs receiving Accelerated Approval would only be covered for patients in clinical trials. This decision, as we described at the time, was an "enormous setback for Alzheimer's patients and an unprecedented and dangerous infringement on the FDA's scientific autonomy and decision making." This dangerous precedent of CMS substituting its own judgement for FDA's could lead to a dangerous spiral of lack of confidence in the U'S''s gold standard drug approval process, access restrictions or continued unmet need for patients suffering from all manner of diseases and ailments, and a disinvestment of an important industry where our country is far and away the global leader.

Still more troubling for patients suffering with unmet medical need is that CMS's Alzheimer's decision is neither isolated nor unique among policymakers. A recent proposal was floated by the Center for Medicare and Medicaid Innovation (CMMI), to reduce Medicare spending on drugs entering the market via accelerated approval, if confirmatory phase 4 trials are not complete. This troubling approach has the potential to decrease patient access to drugs for serious conditions with unmet needs. Specifically, for investigational products, the proposal would disincentivize future product development and investment. Additionally, for products that are currently approved under the accelerated approval pathway, the program may discourage sponsors from pursuing the required post-approval studies and maintaining the product approval in the United States. The healthcare consultancy Vital Transformation has found that threats such as these at both the federal and state level could result in as many as two-thirds of accelerated approval therapies failing to reach patients – affecting as many as 3.6 million patients.[9]

BIO strongly opposes efforts to restrict access to innovative therapies approved under the accelerated approval pathway. This pathway is often the only mechanism for approving effective therapies to address critical unmet patient need in challenging and serious disease states. Any efforts to undermine this pathway would have serious, detrimental effects on vulnerable patient populations and hinder innovation.

Critics miss that using the same well-established evidentiary standard as for traditional approvals, the pathway has facilitated approval of treatments for many severe diseases, such as a variety of cancers (including rare cancers), Human Immunodeficiency Virus (HIV), various bacterial infections, Multiple Sclerosis, Sickle Cell Disease, and others. Moreover, drugs earning accelerated approval must meet the same statutory standards of evidence for safety and effectiveness as those granted traditional approval. In using the accelerated

[9] https://vitaltransformation.com/2022/06/calculating-the-value-and-impact-of-accelerated-approvals/

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approval pathway, a sponsor must show that the drug demonstrates substantial evidence of an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on an intermediate clinical endpoint that can be measured earlier than irreversible morbidity or mortality (IMM) that is reasonably likely to predict a clinical benefit.

Further, drug manufacturers are required to conduct with due diligence phase 4 post-marketing trials to verify the clinical benefit of the drug. FDA may withdraw the accelerated approval if evidence demonstrates that the product is not shown to be safe or effective. This happens if the post-marketing trials do not verify clinical benefit or are not conducted with due diligence.

For all these reasons, we urge the Committee to oppose efforts to restrict access to innovative therapies approved under the accelerated approval pathway, which is often the only way forward for approving effective therapies to address critical unmet patient need.

Government Action that Harms Access: The Need to Limit CMMI Authority

BIO believes that innovation is key to bringing cures and treatments to patients suffering from unmet medical need. To that end, we believe innovation in existing payment systems may be just as critical as innovation in the laboratory to deliver tomorrow's cures. Today's 20th Century payment systems often have difficulty delivering 21st Century treatments that do not fit neatly into decades-old legacy payment systems. Concomitantly, we support CMMI's goal to "foster healthcare transformation."

At the same time, BIO believes that great amount of authority invested in CMMI must be wielded to truly innovate the American health care system rather than to facilitate an end run around the Congress' authority to oversee the Medicare program. As illustrated above in its recent approach to cutting spending on drugs approved through the accelerated pathway, CMMI's broad testing authority, and CMS's increasingly aggressive approach to using that authority, results in unchecked ability of the Agency to make rapid, broad, and unpredictable changes to payment policy. Several recent CMMI announcements (Radiation Oncology Model, International Pricing Index Model, and past mandatory demonstrations such as the Part B Drug Payment Model and Comprehensive Care for Joint Replacement Model), illustrate the negative consequences such action can have for patients, providers, and other stakeholders. Reforms are urgently needed to establish CMMI safeguards so the agency can still propose and test new payment models, but protect against sweeping, unilateral policy changes that undermine care quality and patient access to needed care.

BIO has long supported bipartisan Congressional efforts to establish transparency and important guardrails around CMMI demonstration initiatives. These necessary CMMI reforms fall into five broad categories:

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(1) **Limiting mandatory Phase I tests:** Mandatory models pose heightened risks for negative, unintended consequences for patient care, care quality, and care continuity. Phase I models should be tested on a voluntary basis to minimize and assess the potential risks to beneficiaries.

(2) **Placing reasonable limits on the scope and duration of CMMI models:** Set appropriate limits on the number of beneficiaries that can be included in any early-stage test (the lesser of either 10% of the defined population or 500,000 beneficiaries) and limit the length of time the demonstration can run to no more than 5 years.

(3) **Reaffirming the need for Congressional approval of any legislative changes required to expand a model**: Under the statute, CMMI may waive certain provisions of law in order to test models but may not make permanent changes to the law. To reaffirm Congress' role in making changes to Medicare law, legislation should clarify that Congress must approve any changes to existing statute (if needed) when CMMI expands a model (Phase II).

(4) **Providing for judicial review of key CMMI decisions:** Current CMMI statute precludes key mechanisms for accountability at CMMI by limiting judicial review of CMMI decisions. Reforms are needed to allow for judicial review and promote CMMI accountability for important decisions (e.g., regarding model expansions).

(5) **Improving accountability and stakeholder engagement and establishing stronger safeguards for beneficiaries:** CMMI models should be developed with input from impacted stakeholders prior to their announcement through a request for applications or proposed rule. Stronger safeguards are also needed at model launch to protect beneficiaries, including a monitoring and evaluation strategy appropriate to the risks associated with the model and providing for notification to impacted beneficiaries.

Conclusion

We thank the Subcommittee for the opportunity to provide this statement for the record for the hearing, "*Examining Policies that Inhibit Innovation and Patient Access.*" We look forward to working with the Committee to address these important issues and stand ready to help the Subcommittee in any way we can to assure access to new cures and treatments for Americans suffering from diseases of all kinds.

Testimony Submitted for the Record
U.S. House Committee on Ways & Means Committee
Subcommittee on Health
Hearing: "Examining Policies that Inhibit Innovation and Patient Access"
May 10, 2023

Lauren Aronson
Executive Director, The Campaign for Sustainable Rx Pricing (CSRxP)

Chairman Buchanan, Ranking Member Doggett, and members of the House Ways & Means Committee Subcommittee on Health, the Campaign for Sustainable Rx Pricing (CSRxP) thanks you for the opportunity to submit testimony for the record on fostering innovation and patient access to care. We strongly support bipartisan efforts to lower the unsustainable growth in prescription drug prices because, without more affordable therapies, patients simply cannot access the treatments they need and benefit from the innovations occurring today.

The Campaign for Sustainable Rx Pricing (CSRxP) is a nonpartisan coalition of organizations committed to fostering an informed discussion on sustainable drug pricing. Our members represent organizations including consumers, hospitals, physicians, nurses, pharmacists, employers, pharmacy benefit companies and insurance providers. We are committed to developing bipartisan, market-based solutions that promote competition, transparency, and value to improve affordability while maintaining patient access to innovative prescription drugs that can improve health outcomes and save lives. We believe innovation and affordability must go hand in hand.

Prescription drug prices are out of control and continue to grow at unsustainable rates. Twenty-two cents of every health care dollar goes toward prescription drugs – with drugs contributing more to health care costs than any other type of health care service.[1] Drug companies increased prices on nearly 1,000 drugs through the first three weeks of January 2023 even though far too many Americans still cannot afford their medications.[2] These price increases to start the new year follow years of unsustainable price increases imposed by Big Pharma on consumers and taxpayers. During the period of July 2021 to July 2022, for example, drug makers raised prices in excess of inflation for 1,216 drugs, with an average price increase of 31.6 percent.[3] The average price increase was nearly $150 per drug (10.0 percent) in January 2022 and was $250 (7.8 percent) in July 2022.[4]

Unsustainably high price increases are not the only significant drug pricing problem that U.S. patients and their families face today. Pharmaceutical companies are setting records for skyrocketing prices of new drugs at launch. The median annual price among new drugs approved by the Food and Drug Administration (FDA) in 2022 was more than $220,000 – a significant jump even from 2021 when the median launch price was $180,000.[5]

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[1] AHIP. Where Does Your Health Care Dollar Go? September 6, 2022.
[2] Alltucker, Ken. "Why drugmakers have raised prices on nearly 1,000 drugs so far this year." *USA Today*. January 30, 2023.
[3] U.S. Department of Health and Human Services Assistant Secretary for Planning and Evaluation Office of Health Policy. "Price Increases for Prescription Drugs, 2016 – 2022." September 30, 2022.
[4] *Ibid*.
[5] Beasley, D. "U.S. new drug price exceeds $200,000 median in 2022." *Reuters*. January 5, 2023.

Despite efforts from the pharmaceutical industry to suggest otherwise, drug manufacturers – and drug manufacturers alone – are the drivers of the unsustainable growth in prescription drug prices and the needlessly high spending on drugs that consumers, taxpayers, and businesses face today. Drug makers set high list prices at launch and increase those list prices at rates far above inflation. Spending on high-priced drugs places significant strain on patients, federal health programs, and taxpayers. High-priced drugs also substantially burden the many small businesses and large employers who seek to offer affordable health insurance to their employees because, as prescription drug expenditures increase, cost-sharing and premium costs also rise.[6] Far too often patients experience the unfortunate and unfair choice of purchasing the medications they need to get well and stay healthy and paying their bills. Patients simply should never be presented with such a choice.

Indeed, patients cannot benefit from innovations in healthcare if the treatments they need are unaffordable and inaccessible due to cost. Simply put, needlessly high-priced prescription drugs create barriers to care and inhibit patient access to treatment. Thus, to foster healthcare innovation and patient access to medically necessary treatment, CSRxP ardently believes that significant actions must be undertaken to address today's prescription drug pricing crisis. The Inflation Reduction Act (IRA) of 2022 took major steps toward holding Big Pharma accountable for the escalating costs of Medicare Part D and lowering prescription drug prices for the millions of Americans who face financial uncertainty affording their medications, in part through adopting policies long advocated by CSRxP including keeping drug companies' price hikes for Medicare-covered drugs at rates below inflation and capping Part D out-of-pocket costs for beneficiaries. Still, more must be done to improve prescription drug affordability for American consumers and taxpayers.

CSRxP thus welcomes bipartisan legislation from this Subcommittee and the Congress that lowers costs and promotes patient access to affordable prescription drugs while at the same time fostering healthcare innovation. In this light, we offer the following policies for consideration to help address the critical prescription drug pricing problem that American consumers and taxpayers face today.

1. **Address out-of-control prescription drug launch prices.** As underscored above, the median annual price among new drugs approved by FDA in 2022 was more than $220,000 – a large increase from a year earlier when the median new drug launch price was extremely high at $180,000.[7] To help thwart the relentless growth in launch prices, Congress should direct the U.S. Department of Health and Human Services (HHS) to issue an annual report on launch prices of new drugs and launch pricing trends in order to systematically monitor escalating launch prices and their impacts on consumers and taxpayers. With this information, patients, their providers, and taxpayers will have improved information on the affordability of the treatment options available to them.

2. **Increase manufacturer transparency in prescription drug pricing.** Today little to no transparency exists in how pharmaceutical companies price their therapies. Indeed, manufacturers regularly justify their pricing decisions by citing industry-funded research claiming that it costs $2.6 billion to bring a new drug to market, even though the industry offers the public no way to independently verify this estimate and gives the government, employers,

[6] American Academy of Actuaries. "Prescription Drug Spending in the U.S. Health Care System." March 2018.
[7] Beasley, D. "U.S. new drug price exceeds $200,000 median in 2022." *Reuters*. January 5, 2023.

and insurers no tools to determine whether a drug is affordably and reasonably priced.[8] Therefore, Congress should enact legislation increasing transparency in manufacturer prescription drug pricing. To that end, CSRxP supports the bipartisan Fair Accountability and Innovative Research (FAIR) Drug Pricing Act – this legislation will shine a light on how manufacturers price their products. CSRxP urges Congress to enact both pieces of legislation, which importantly will require manufacturers to publicly disclose pricing information and justify price increases for their high-priced drugs.

3. **Foster the availability of lower cost biosimilars to compete with expensive reference brand biologics.** Robust competition from interchangeable biologics and biosimilars can place pressure on brand manufacturers to lower list prices and reduce overall costs on high-priced biologics. The HHS Assistant Secretary for Planning and Evaluation (ASPE) determined, for example, that Medicare Part B expenditures on prescription drugs increased at a rapid average annual rate of 7.7 percent from 2005 to 2014.[9] During that period, specialty biologic medicines that in most cases faced little to no competition from interchangeable biosimilar and biosimilar products grew at a particularly fast rate, climbing from 39 percent to 62 percent of total spending, with a substantial share of the growth due to price increases rather than number of patients using the medications.[10] Indeed, the Congressional Budget Office (CBO) has recognized the value that competition from biosimilars and interchangeable biosimilars can have in prescription drug costs for patients and payers:

> *"The availability of therapeutic substitutes provides insurance plans and PBMs with leverage to negotiate lower prices. When alternatives are limited, such as when a new drug is the first to treat a particular condition, then insurance plans and PBMs have limited leverage to negotiate lower prices. As competing products enter the market, payers gain the flexibility to exclude a given drug or to limit patients' use of that drug through higher cost sharing or other utilization management tools (emphasis added)."*[11]

Given the significant potential for lower patient out-of-pocket expenditures and overall healthcare spending, Congress should enact legislation that fosters a more robust marketplace for biosimilars, for example, by reducing the market exclusivity for brand reference biologics from 12 year down to 7 and implementing policies that encourage prescribing and coverage of these lower cost therapies.

4. **Thwart anti-competitive intellectual property abuse by brand drug makers.** Published research clearly suggests that Big Pharma's abuse of the U.S patent system is particularly contributing to high drug costs and spending. One analysis found, for example, that despite representing less than one percent of U.S. prescriptions, biologics account for nearly half of all drug spending largely due to lower overall biosimilar competition resulting from current market regulation and

[8] DiMasi JA, Grabowski, HG, Hansen RA. Innovation in the pharmaceutical industry: new estimates of R&D costs. *Journal of Health Economics* 2016; 47:20-33.
[9] HHS Assistant Secretary for Planning and Evaluation. "Medicare Part B Drugs: Pricing and Incentives." March 8, 2016.
[10] *Ibid*.
[11] CBO. "Prescription Drugs: Spending, Use, and Prices." January 2022, pages 19 – 20.

efforts by the brand industry to undermine the patent system.[12] Absent significant actions undertaken to stop this abuse, the analysis concluded that consumers will pay an extra $25 billion on drugs over the next decade.[13] Similarly, an investigation by the House Committee on Oversight and Reform determined that brand drug companies raised prices more than 250 times on 12 of the best-selling drugs in Medicare, leading to median prices almost 500 percent higher than when they were brought to market.[14] Brand drug makers obtained more than 600 patents on these 12 drugs to effectively block competition from more affordable alternative generic and biosimilar therapies for decades, imposing substantial and unnecessary costs on Medicare beneficiaries and U.S. taxpayers for years.[15]

Given the significant role that intellectual property abuse plays in the prescription drug pricing crisis, CSRxP urges enactment of legislation to thwart the anti-competitive IP abuses of Big Pharma. In particular, we support the Interagency Patent Coordination and Improvement Act of 2023 (S. 79), the Affordable Prescriptions for Patients Act of 2023, the Preserve Access to Affordable Generics and Biosimilars Act, and the Stop STALLING Act. Each of these important pieces of legislation will take significant actions to combat the patent and other intellectual property abuses of Big Pharma, thereby facilitating greater access to more affordable prescription drugs for patients.

Conclusion

In conclusion, CSRxP again wishes to thank you for the opportunity to provide testimony on this vital issue. CSRxP firmly believes that without major actions by this Subcommittee and others, the brand pharmaceutical industry will continue to excessively profit from their unfair and unsustainable pricing practices that increase drugs costs and risk access for the patients who need them. CSRxP looks forward to our continued work with the Subcommittee and Congress to develop bipartisan, market-based policies that promote transparency, foster competition, and incentivize value to improve affordability for consumers while at the same time maintaining access to the treatments that can improve health outcomes and save lives. Please find further information on the drug pricing problem and ways to rein in high drug prices at our website (www.csrxp.org).

[12] Roy, Avik. "The Growing Power of Biotech Monopolies Threatens Affordable Care." Foundation for Research on Equal Opportunity. September 15, 2020
[13] *Ibid*.
[14] House Committee on Oversight and Reform Majority Staff Report. Drug Pricing Investigation. December 2021.
[15] *Ibid*.

May 24, 2023

Ways and Means Committee Chairman Jason Smith
Ways and Means Committee Ranking Member Richard Neal
Health Subcommittee Chairman Vern Buchanan
Health Subcommittee Ranking Member Lloyd Doggett
1139 Longworth HOB
Washington D.C. 20515

RE: Hearing on Examining Policies that Inhibit Innovation and Patient Access

We at Caregiver Action Network (CAN) are encouraged to see the committee focusing on ways to protect innovation and patient access - such policies significantly impact not only patients, but also their families, caregivers, and communities.

The caregiver burden is often overlooked in policy discussions, but caregiving is a critical part of our healthcare system. When new treatments, diagnostics, and other tools are approved by the FDA, this burden is lessened. However, when it comes to caring for Alzheimer's patients, innovative new tools have been made inaccessible for a majority of patients. And the ripple effects of limited access are and will be devastating.

Despite recent news of the "strongest Alzheimer's Phase 3 data release to date," the biggest hurdle patients face in accessing innovative treatments is one put in place by Medicare. CMS' decision to limit coverage for FDA approved Alzheimer's treatments unless patients meet strict eligibility criteria will add to the caregiver burden significantly.

Approximately 11 million Americans are providing unpaid care for their loved ones with Alzheimer's disease. Caregiving presents daily physical and emotional challenges; and it takes a huge toll on careers, relationships, and family life. These family caregivers are juggling work with their caregiving responsibilities and logging more than 15 billion hours of caregiving annually.

As people living with Alzheimer's cognitive abilities erode over time, the task of caring for them becomes far more difficult – and takes a significant toll on their caregiver. Caregivers for Alzheimer's patients report disproportionately higher rates of stress and depression. They have increased risk for heart disease and stroke; they suffer higher mortality rates than the general population; and they must spend about $5,000 more than the average person on their own annual health care costs.

A study from the University of Michigan found that more than half of Americans 50 years and older are caregivers to one or more people, 65 years or older. Younger families feel the brunt too. Twenty-five percent of Gen Z and Millennial caregivers were in caregiving roles for the first time during the pandemic. Overall, 20 percent of caregivers surveyed were new to caregiving, and 60 percent of them were Gen Z or Millennials.

The worst part is that CMS is setting a disturbing precedent for millions of patients and their caregivers by strictly limiting coverage to only patients who can participate in clinical trials, or whose physicians are

possibly able to enter them into patient registries – a time consuming, burdensome undertaking. This will undoubtedly lead to most Alzheimer's patients and future caregivers being left behind.

Further, the consequences of this decision will reverberate throughout the healthcare system and put in jeopardy the development of future treatments for rare and chronic diseases that impact tens of millions of patients and families.

Medical science has progressed, but by limiting access to an entire class of Alzheimer's therapies today, CMS is shutting the door on the development of new and better treatments in the future. Congress must take notice – and act.

As you discuss access to innovative treatments, it is critical that Congress recognizes how CMS decision to limit coverage for Alzheimer's treatments will cause more long-term harm than good. Patients living with the disease today will continue their decline, family and caregivers will see their quality of life continue to erode, and we will have arrested future progress towards the development of new treatments that can bring new hope.

Copied below you'll find our infographic on the many ways the CMS NCD/CED requirements hinder patient access and increase the caregiver burden. We hope to serve as a resource as you work on this important issue.

Sincerely,

Lisa Winstel
Interim CEO
Caregiver Action Network

MEDICARE COVERAGE IS CRITICAL TO CAREGIVERS

Why "Coverage with Evidence Development (CED)" Hinders Patient Access & Increases the Caregiver Burden

CAREGIVER ACTION NETWORK

The experts at the Food and Drug Administration (FDA) determine which medicines, therapies, and medical devices are safe and effective. The Centers for Medicare and Medicaid Services (CMS) covers the vast majority of FDA-approved medical services, therapies, and equipment.

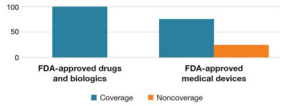
When CMS denies Medicare coverage of FDA-approved drugs, patients and their family caregivers lose.

What is an NCD?

A national coverage determination (NCD) is a Medicare coverage policy that explains the conditions for CMS coverage for specific "items and services that are reasonable and necessary for the diagnosis or treatment of an illness or injury." NCDs limit access by specifying eligible patients, healthcare providers, and other conditions that must be met. NCDs are especially rare for FDA-approved drugs, which have only been subjected to the process a handful of times. Even when CMS has issued NCDs for therapeutics, the agency has almost always provided coverage according to a drug's label.

NCD outcomes for FDA-approved drugs and devices, by type of approval

Percent with CMS coverage. 1999-2011

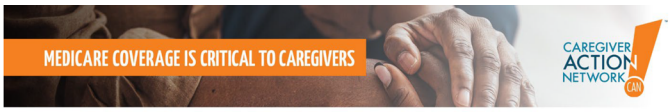


What is a CED?

Coverage with Evidence Development (CED) is a coverage policy CMS can impose within the context of an NCD that further restricts coverage (i.e., requiring beneficiaries be enrolled in a CMS-approved clinical study or a patient registry to receive coverage.) **Of the hundreds of NCDs from 2005-2021, only 26 require CED.** CMS has utilized CEDs almost exclusively for medical devices because clinical evidence is often limited at the time of launch and long-term effectiveness depends on how they are adopted.

According to CMS, CED is intended to expedite access to innovative new therapies while additional evidence is collected. As CMS explains, this process is not intended to "duplicate or replace the FDA's authority in assuring safety [and] efficacy," as the FDA approval process is extremely rigorous.

For patients and their family caregivers, CED for an FDA-approved drug often seems duplicative, leads to confusion, and undermines confidence in the FDA approval process.



CED can have several unintended consequences, including:

LIMITING PATIENT ACCESS:

Limiting the type of patients who may benefit from a certain type of treatment, diagnostic tool, or therapy, means that many others will not have access. Family caregivers' top priority is to ensure that their loved ones have access to all tools and therapies they need.

THREATENING R&D:

The time and money required to put a drug through the FDA-approval process is significant. If CMS requires additional data—beyond ongoing reporting commitments to FDA—as a condition for coverage, research and investment incentives will be reduced.

INCREASING COSTS:

A review of CEDs found that CMS does not directly fund the clinical research detailed in NCDs, therefore public and private funding streams are required to develop and conduct the research in question.

EXTENDING TREATMENT DELAYS FOR PATIENTS:

One expert explained that, for CED, "generally, a maximum of four years until study completion and final decision seems to distinguish successful from unsuccessful cases." For most disease states, timely and accurate diagnosis is critical to help ensure timely, effective treatment.

UNCLEAR OR CONFUSING DATA REQUIREMENTS:

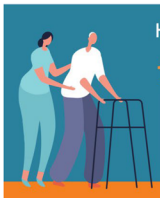
Currently, data collection mechanisms are designed and implemented without a specific timeline for coverage reconsideration. This is exemplified by the lengthy and widely varying program duration for the three out of 26 programs with retired data collection requirements (4 to 12 years) and the long duration of the two CED programs resulting in NCD revocation (10 and 13 years) since 2005.

INCREASING HEALTH INEQUITIES:

CEDs could add to the significant health equity gaps for rural and underserved communities, and people of color. Participants in approved studies may receive better treatment than those not participating, and treatments likely will not be available in all geographical areas. A recent Avalere analysis provides a detailed look at the health equity issue.

ETHICAL ISSUES:

Various stakeholders have questioned whether it is ethical to restrict access to FDA-approved therapies to patients participating in registries and clinical trials while withholding coverage for others. Patients and caregivers must weigh a lot of factors in order to determine whether a clinical study is right for them. Further, when CMS requires randomized control trials, there is a risk that patients will receive placebo.



How Can CMS Better Help Patients and Caregivers?

1. Clearly articulating a pathway to coverage.
2. Utilizing a fit-for-purpose data collection process that is designed to answer specific, necessary questions.
3. Reducing health equity gaps by allowing flexibility for all appropriate providers to participate, even if entities have minimal data infrastructure capabilities.
4. Clearly defining the time limits or stopping rules to ensure that patients aren't waiting for decades for treatments.

Learn more at caregiveraction.org

Click Therapeutics

May 15, 2023

House Committee on Ways and Means Subcommittee on Health
Chairman Vern Buchanan
Ranking Member Lloyd Doggett
1100 Longworth House Office Building
Washington, DC 20510

Dear Chairman Buchanan and Ranking Member Doggett:

Click Therapeutics commends the work of the House Committee on Ways and Means Subcommittee on Health for examining our nation's crisis with barriers to innovation.

To leverage the important advantages of digital therapeutics, the federal government must establish a structure that enables patients and clinicians to identify genuine DTx products, ensures reliable access to these products, and provides actuarially sound reimbursements for DTx products and the clinicians responsible for authorizing and/or utilizing digital therapeutics.

Prescription digital therapeutics (PDTs) are a new class of medical treatments that utilize software to improve patient outcomes. These innovative therapies can help patients manage chronic conditions, such as diabetes and depression, by delivering personalized interventions through mobile applications or other digital devices. However, policies that inhibit innovation and patient access to PDTs are a major barrier to the adoption and growth of this emerging field.

One policy that inhibits innovation in the PDT industry is the lack of clarity around regulatory approval. The FDA has not yet established a clear framework for the approval and regulation of PDTs, which creates uncertainty and slows down the development process. In addition, the reimbursement landscape through the Centers for Medicare and Medicaid Services (CMS) for PDTs is unclear, which further disincentivizes companies from investing in research and development.

Another policy that inhibits patient access to PDTs is the lack of insurance coverage for these treatments. Many insurance providers do not cover the cost of PDTs, even though they may be more cost-effective than traditional treatments in the long run. This creates a barrier to access for patients who may benefit from these treatments but cannot afford to pay out of pocket.

Additionally, state regulations may vary, which further complicates the development, approval, and distribution of PDTs. Some states may require additional licensing or regulatory approvals

for these therapies, while others may not. This creates an inconsistent regulatory environment that can be challenging for companies to navigate.

The lack of clarity around reimbursement for PDTs can create a significant barrier to patient access, as many patients may not be able to afford the cost of these treatments out of pocket. This can limit the ability of PDTs to reach their full potential in improving patient outcomes and reducing healthcare costs.

To address these policy barriers, stakeholders including regulators, insurance providers, and policymakers must work together to create a regulatory framework that incentivizes innovation and ensures patient access to PDTs. This can include developing clear guidelines for regulatory approval, establishing reimbursement policies that incentivize the use of PDTs, and promoting consistent state regulations to support the growth of this emerging field.

We look forward to further engaging with your committee on these critical issues. Please contact Rich DeNunzio at RdeNunzio@ClickTherapeutics.com for any further information or insights.

Sincerely,

Rich DeNunzio

COUNCIL FOR AFFORDABLE
HEALTH COVERAGE

April 14, 2023

Meena Seshamani, M.D., Ph.D.
Deputy Administrator and Director of the Center for Medicare
Center for Medicare & Medicaid Services
7500 Security Blvd.
Baltimore, MD 21244

RE: Medicare Drug Price Negotiation Program Guidance

Submitted electronically to IRARebateandNegotiation@cms.hhs.gov

Dear Dr. Seshamani:

Thank you for the opportunity to provide comments on the March 15,2023 memorandum entitled "Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments."

The Council for Affordable Health Coverage (CAHC) has long supported reduced drug costs, greater access to drug therapies and fostering innovation to help treat and cure disease. CAHC (www.cahc.net) is a broad-based alliance with a primary focus: bringing down the cost of health care for all Americans. Our members include employers, medical providers, patient groups, insurers, agents and brokers, technology companies, pharmaceutical manufacturers, and pharmacy benefit managers who collectively represent tens of millions of lives in the private market.

We are submitting comments on two aspects of the Initial Memorandum:

1. The process CMS is using to implement the program is opaque and lacks critical stakeholder input to ensure the principles of good government are followed. This must be improved.

2. The provisions related to Orphan products must be modified to ensure implementation of the Medicare negotiation program does not harm our most vulnerable patients.

Process

While most businesses are seeking to provide more transparency and accountability for their constituents, the Administration's implementation of the Inflation Reduction Act (IRA) moves in the opposite direction. Under Section 1198 of the Inflation Reduction Act, Congress instructed HHS to implement the negotiation law by "program instruction or other forms of program guidance" for 2026, 2027 and 2028. Congress also limited administrative and judicial review.

This is not the regular process established by Congress to encourage citizen participation in a transparent government for the people.

Enacted in 1946, the Administrative Procedures Act ensures citizens have a right to be heard by government, that government responds to their concerns, and that parties harmed by government have access to recourse. The law includes requirements for informing the public of rules and providing for public participation in the rule making process by publishing notices of proposed and final rulemaking in the Federal Register and the opportunity for the public to comment on notices of proposed rulemaking.

While CMS must produce the program guidance as instructed, CMS was not instructed by Congress on the process for seeking out and responding to stakeholder input. We are disappointed that CMS has solicited feedback in limited venues, on limited sections of the law, from a discrete panel of stakeholders who already support the program. A law of this magnitude and complexity should have robust stakeholder feedback, including diverse views from every party impacted. We are concerned to learn that some stakeholders have been told their comments may not even be read, much less responded to.

Additionally, the inclusion of language that bars manufacturers from being transparent about government activities during the negotiation process is an egregious overreach of government censorship. CMS proposes a sweeping policy that would restrain manufacturer speech by placing limits on what a manufacturer can use or disclose from CMS offers, requires a "certificate of data destruction" of any and all material related to the negotiation process- including the manufacturer's own written notes or emails, and prevents manufacturers from audio or video recording any oral conversations between CMS and the manufacturer. This proposal seriously undermines transparency and the ability to validate information if a conflict arises. The need to shield CMS decision-making process from scrutiny will erode public confidence in the price-setting process and should be removed.

Recommendation: We encourage CMS to open the process up to sunshine by:

- **Making your meetings transparent (with recorded minutes and records of attendees) with any stakeholder willing to participate.**
- **Provide responses to stakeholder questions through the program guidance process. Congress provided $3 billion in funding to implement the program. We suggest spending some of this money on outreach and response to legitimate concerns and questions. CMS is undermining its credibility by failing to respond to stakeholders.**
- **Work with Congress to change the law to require the program be implemented through the regular rulemaking process. While the guidance route may be expeditious for CMS employees, it undermines citizen trust in the program and the Agency itself. There is little reason to continue using program guidance rather than the normal rulemaking process in the program's second or third year.**
- **Remove the gag clause on manufacturers to destroy all information related to the negotiation process.**

There is nothing in law that precludes CMS from having a more transparent, accountable process. We encourage you to open up the process and commit to the responsible implementation of the law.

Orphan Products

More than 10,000 rare diseases impact more than 30 million people (about the population of Texas) in the United States. Fewer than 5% of these diseases have any FDA-approved therapies. Half of those afflicted with a rare disease are children and thirty percent of them will die before their fifth birthday due to the lack of available treatments. For most of the 1 in 10 Americans with a rare disease, surgeries or other medical procedures will not help – they need prescription drugs to either keep their disease from progressing, to get better, or hopefully with new advancements in drug development, be cured. The rare disease ecosystem is extremely sensitive to changes in policy and incentives that drive investment in these patient populations which are high risk and have unmet needs.

Under the IRA, if a chemical drug has been FDA-approved for at least seven years (at least 11 years for a biological product) it may be eligible for price "negotiation." There are exceptions. For example, drugs with a generic or a biosimilar substitute are exempt from price limits.

The law states that CMS must exclude from price controls a drug "...for only one rare disease or condition and for which the only approved indication (or indications) is for such disease or condition" (Section 1191(e)(3)(A)).

The intent behind this language was to continue to incentivize drug development for rare diseases, carving out products that treat rare diseases from the damage that price controls will cause to access and innovation where it is needed most. However, drug developers often continue research on approved products for other indications and conditions. Particularly in the rare disease community, where studying potential new uses of repurposed products can lead to faster access and less expensive therapies for diseases in need of treatments, this is especially important. More than 60 percent of oncology medications approved more than a decade ago, for example, received additional approvals to treat new indications in later years. Yet under the IRA, if a product indicated to treat one rare disease was studied and approved for the treatment of another rare disease, the product could be subject to price limits, thereby eliminating the incentive to study approved products on additional patient populations and disease groups.

The IRA makes clear that companies developing orphan drugs are now at increased risk of market failure – the opposite of what the Orphan Drug Act sought to address through tax, market exclusivity, and other incentives. Companies are unlikely to invest in products that could be subject to price limits because they are unlikely to see a return on their investment. Stack that negative incentive on top of others that already make this a difficult landscape for drug development – fewer patients (many of them pediatric), high cost of clinical trials, difficulty designing trials that meet the FDA's demands and regulatory uncertainty – and it makes the market for orphan products very unfavorable. Faced with this uncertainty, investors and manufacturers are unlikely to develop follow-on treatments for the more than 30 million Americans (and their caregivers) who all share the same desire: to have treatments developed for their diseases.

We are concerned that the March 15th guidance language on the single rare disease or condition indication is even more restrictive than what was intended by Congress and will undoubtedly have a damaging impact on development of rare disease drugs. Resolving the problems created by the orphan drug language in the IRA must be done quickly and transparently.

Recommendation: In the program guidance, CMS states the Agency is considering whether there are additional actions it can take to best support orphan drug development. CMS has sufficient flexibility through program guidance in implementing the law to immediately help. Given the limited nature of the exclusion for orphan drugs in the MDPNP, CMS should delay the start of the price negotiation clock for when an excluded orphan product loses its exemption from price controls due to FDA approval of an additional indication until it is proven that the changes do not threaten patient access or innovation. This will help incentivize new research and the discovery of therapies to treat rare diseases. It is pro science and does not conflict with the goal of the law – namely, to reduce prices for products without generic or biosimilar competition.

Conclusion

CAHC encourages CMS to rework the rule to ensure that scientific discovery, product development and patient access are unharmed, and to work with Congress to promote transparency and accountability in rulemaking that will ensure people trust a program of this size and scope is implemented appropriately. While we share your goal of lowering the cost of healthcare, achieving this goal must be approached systemically and not in a way that creates a slew of unintended consequences, namely harming our most vulnerable patients.

If you have questions about these comments, please do not hesitate to contact me.

Sincerely,

Joel C. White
President
Council for Affordable Health Coverage

May 10, 2023

The Honorable Vern Buchanan
Chairman
Subcommittee on Health
House Ways and Means Committee
1139 Longworth House Office Building
Washington, D.C. 20515

The Honorable Lloyd Doggett
Ranking Member
Subcommittee on Health
House Ways and Means Committee
1139 Longworth House Office Building
Washington, D.C. 20515

Dear Chairman Buchanan and Ranking Member Doggett:

I write on behalf of the Council for Innovation Promotion (C4IP) regarding the Health Subcommittee's upcoming hearing on "policies that will have negative effects on medical innovation and reduce patient access to therapies."

C4IP is a bipartisan coalition chaired by two former directors of the U.S. Patent and Trademark Office (USPTO) appointed by presidents of different parties. C4IP believes a strong and effective patent system is the single most important driver of U.S. innovation. Patents bolster U.S. economic competitiveness while incentivizing the creation of products and technologies, including medical innovations, that benefit the entire world.

We hope that the Subcommittee on Health's hearing will not be an opportunity for harmful attacks on the U.S. patent system. Misinformed activists increasingly argue that drug companies are "gaming" the patent system by filing duplicative patents meant to exclude generic competitors from entering the market with affordable treatment options.

This is just false. The USPTO only issues patents for new, non-obvious, and useful innovations. And patents within the same "patent family" expire at the same time. So, obtaining multiple patents on the same drugs wouldn't extend the length of time that the original drug formula is protected from copycats.

More importantly, this misleading narrative relies on the mistaken assumption that filing multiple patents for a single medicine is a sign of malfeasance. On the contrary, additional patents granted after a drug's initial FDA approval reflect critical, life-saving "follow-on" research.

In the years after a medicine is first approved, developers often continue working to improve its formula, dosage, and delivery mechanism to reduce side effects and boost patient adherence. Sometimes, developers even discover that a medicine initially approved to treat one condition can treat one or more other illnesses. This is particularly common in certain fields, like oncology. Lifesaving post-approval development should be celebrated and encouraged, not bemoaned.

Crucially, if a post-approval discovery yields a new patent, the original formulation of a medicine is not impacted. Patenting an extended-release version of a particular drug, for instance, would not alter the exclusivity period of the original version.

Stripping inventors of the ability to patent follow-on discoveries, as some activists advocate, would hamper IP-driven innovation in all sectors of the U.S. economy. Virtually every step taken to improve medical science, manufacturing, or technology is follow-on. Inventors build on their own progress and the progress of others.

Just like inventors in any other sector, drug developers would have little reason to invest in the R&D and clinical trials necessary to make incremental improvements to existing medications if follow-on discoveries couldn't be patented. Lawmakers must resist turning follow-on research into a financial liability. We should all want to increase -- rather than shrink -- the number of medical advances generated by post-approval research.

Lastly, attacks on follow-on patents reflect a broader belief that the patent system somehow stands between patients and lifesaving, innovative therapies. Nothing could be further from the truth. Rather than reducing patient access to therapies, robust patent rights underlie the R&D pipeline that continues to deliver breakthrough treatments and cures. Last year, the FDA approved 37 novel drugs. In 2021, it approved 50.

These new medicines would not have been possible if inventors and investors lacked confidence in the reliability of the patent system. Without a predictable period of market exclusivity, the enormous investment required to develop just one new drug could not be justified.

And without this initial innovation, patients will never benefit from cheaper generics down the road. The United States has one of the highest generic use rates in the world -- nine in 10 U.S. prescriptions are filled with generics. Far from being broken, the intellectual property system is working as lawmakers intended, by giving innovators enough protection to invest in new products, while also ensuring those products ultimately become available to consumers as cheap generics once patent protections expire.

C4IP appreciates the Subcommittee's focus on promoting greater innovation and competition while ensuring patient access to breakthrough therapies. But efforts to weaken our nation's world-leading patent system -- and IP protections more broadly -- work directly against these goals.

We hope the upcoming Health Subcommittee hearing will be a forum for an evidence-based debate about healthcare policy, not baseless attacks on patent protections.

Sincerely,

Frank Cullen
Executive Director
Council for Innovation Promotion

DIGITAL THERAPEUTICS ALLIANCE

May 22, 2023

House Committee on Ways and Means
Subcommittee on Health
Chairman Vern Buchanan
Ranking Member Lloyd Doggett
1100 Longworth House Office Building
Washington, DC 20510

Dear Chairman Buchanan and Ranking Member Doggett:

The Digital Therapeutics Alliance (DTA) commends the work of the House Committee on Ways and Means Subcommittee on Health for examining our nation's crisis caused by barriers to innovation and patient access.

To leverage the important advantages of digital therapeutics (DTx), the federal government must establish a structure that enables patients and clinicians to identify genuine DTx products, ensures reliable access to these products, and provides actuarially sound reimbursements for DTx products and the clinicians responsible for authorizing and/or utilizing digital therapeutics.

Prescription digital therapeutics (PDTs) are a new class of medical treatments that utilize software to improve patient outcomes. These innovative therapies can help patients manage chronic conditions, such as diabetes and depression, by delivering personalized interventions through mobile applications or other digital devices. However, policies that inhibit innovation and patient access to PDTs are a major barrier to the adoption and growth of this emerging field - hence depriving patients of critically important clinical impacts that DTx products can deliver at scale.

One policy that inhibits innovation in the PDT industry is the lack of clarity around regulatory approvals. The FDA has not yet established a clear mechanism to track the approvals of PDTs, which creates uncertainty and a lack of clarity for healthcare decision makers and ecosystem players. In addition, the reimbursement landscape through the Centers for Medicare and Medicaid Services (CMS) for PDTs is unclear, which further disincentivizes companies from investing in research and development.

Another policy that inhibits patient access to PDTs is the lack of insurance coverage for these treatments. Many insurance providers do not cover the cost of PDTs, even though they may be more cost-effective or clinically impactful than traditional treatments over time. This creates a barrier to access for patients who may benefit from these treatments but cannot afford to pay

out of pocket, thus limiting the ability of PDTs to reach their full potential in improving patient outcomes and reducing healthcare costs.

Additionally, state regulations may vary, which further complicates the development, approval, and distribution of PDTs. Some states may require additional licensing or regulatory approvals for these therapies, while others may not. This creates an inconsistent regulatory environment that can be challenging for companies to navigate.

To address these policy barriers, this committee needs to proactively work with stakeholders including regulators, insurance providers, and policymakers to create a regulatory and reimbursement framework that incentivizes innovation and ensures patient access to clinically-validated PDTs. This can include developing clear guidelines for regulatory approvals, establishing reimbursement policies that incentivize the use of PDTs, and promoting consistent state regulations to support the growth of this emerging field.

We look forward to further engaging with your committee on these critical issues. Please contact Sara Elalamy at sara@dtxalliance.org for any further information or insights.

Sincerely,

Sara Elalamy
Director of U.S Government Affairs
Digital Therapeutics Alliance

GAITBETTER
Reduce Fall Risk • Improve Mobility

May 24, 2023

The Honorable Jason Smith
Chairman
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Richard Neal
Ranking Member
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Vern Buchanan
Chairman
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Lloyd Doggett
Ranking Member
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

Dear Chairmen Smith and Buchanan and Ranking Members Neal and Doggett,

On behalf of GaitBetter, thank you for the opportunity to submit a statement for the record for the May 10th hearing on "*Examining Policies that Inhibit Innovation and Patient Access*." As GaitBetter's head of U.S. sales, I have first-hand experience regarding the barriers that are thwarting patient access to innovative interventions. As such, I very much appreciate that you are seeking public input regarding federal government policies, practices, and programs that have "negative effects on medical innovation and reduce patient access to therapies." Thank you in advance for your attention to the issues, challenges, and recommendations I outline below.

About GaitBetter

GaitBetter, a medical technology company with locations in Maryland and Israel, has developed a patented and clinically-proven virtual reality-based motor-cognitive training solution that has helped reduce falls in older adults by 70%[1]. The technology, created by world-leading neuroscientists, physical therapists, and experts in older adults in the Laboratory for Gait Analysis and Neurodynamics at Tel Aviv Sourasky Medical Center, currently is being used by the U.S. Department of Veterans Affairs as well as by several leading hospital systems across the country. However, due to myriad federal government policies, practices, and programs we have been unable to scale the deployment of the technology, leaving seniors without access to a clinically-proven intervention that can prevent falls and reduce the serious concomitant injuries and death that can accompany them.

[1] GaitBetter. "Case Study: Maccabi Health Services Reducing Falls in Older Adults By 70%." 2022, https://www.gaitbetter.com/case-study-maccabi/

Falls are Common and Costly in the U.S.

Falls are a common problem among older Americans and a leading cause of morbidity, mortality, and use of health care services in the U.S. Falls conservatively account for more than $50 billion in annual health care expenditures, with Medicare and Medicaid paying for an estimated 75% of the costs.[2,3] According to the Centers for Disease Control and Prevention (CDC):[4]

- More than 25% of older adults fall at least once per year and 20% of these falls result in a serious injury, such as a fracture or a traumatic brain injury. [5]
- Each year, three million older adults are treated by the nation's emergency departments for fall injuries.
- Falls among adults 65 and older caused almost 37,000 deaths in 2020[6] – the leading cause of injury death for that population.

Existing interventions to prevent and reduce falls have been insufficient as fall rates, fall injuries per capita, and fall death rates per capita continue to rise.[7] Current falls prevention approaches result in low adherence rates and either focus on short-term individualized therapy or longer-term wellness activities in a group setting, with neither providing optimum results in preventing falls over a large population.

The GaitBetter Falls-Prevention Technology

GaitBetter provides motor-cognitive therapy by adding semi-immersive virtual reality (VR) to existing treadmills, which are used for gait training for falls prevention. The GaitBetter system has a small footprint and easily transforms any existing treadmill into a powerful motor-cognitive training device. In less than two minutes, patients are set up to use the system, ensuring that maximum therapy is received during a visit. Additionally, since patients are secured in a safety harness, therapists can simultaneously attend to multiple patients, increasing efficiency and maximizing the clinic workforce.

The trainee walks on the treadmill and sees her two feet in a simulation projected on a TV screen in front of her. As she walks, she encounters virtual challenges to practice both cognitive and

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[2] Department of Housing and Urban Development (HUD). *Overcoming Obstacles to Policies for Preventing Falls by the Elderly Final Report.* 2017, https://www.hud.gov/sites/dfiles/HH/documents/OvercomingObstaclesFalls.pdf
[3] Centers for Disease Control and Prevention. *Facts About Falls*, https://www.cdc.gov/falls/facts.html
[4] Centers for Disease Control and Prevention. *Older Adult Falls Prevention,* https://www.cdc.gov/falls/index.html
[5] Centers for Disease Control and Prevention. *Facts About Falls,* https://www.cdc.gov/falls/facts.html
[6] Santos-Lozada, Alexis R. "Trends in deaths from falls among adults aged 65 years or older in the US, 1999-2020." JAMA 329.18 (2023): 1605-1607.
[7] HUD. *Overcoming Obstacles to Policies for Preventing Falls by the Elderly Final Report.*

motor skills at the same time. She works on obstacle negotiation and decision making in an environment that is very similar to the real world. Patients' feet movements are analyzed in real-time to drive a VR simulation displayed on a screen (there is no VR headset). A proprietary computer vision algorithm uses a single camera to capture accurate feet tracking, which boosts motor-learning. The patented artificial intelligence algorithm personalizes the intervention for each patient. The individual receives strong/measurable feedback and can track progress over time. This gamification is so motivating that more than 85% of older adults complete the training. Patients easily transfer acquired skills to daily living.

GaitBetter Training Components		
Motor	**Motor-Cognitive**	**Cognitive**
• Gait speed • Step length/Clearance • Endurance • Dynamic balance • Symmetry • Variability	• Obstacle negotiation • Motor planning • Problem solving • Balance strategies • Coordination	• Multitasking • Memory • Response time • Attention • Environment sensory input processing

To see a demonstration of the technology, please visit: https://www.gaitbetter.com/

The Science Behind GaitBetter

The basis of the GaitBetter technology started more than 15 years ago with a longitudinal study of more than 250 dementia-free older adults who had never had a fall.[8] The mental and physical characteristics of this group were closely measured and then time to first fall and number of falls were tracked for five years. ***This study found that the risk of falls only correlated to low levels of cognition performance, specifically executive function***.

Based on this ground-breaking research, a team from the Neurology and Physical Therapy Departments at Tel Aviv University developed the predecessor of GaitBetter. Clinical effectiveness of the intervention was evaluated in a randomized-controlled trial of more than 300 older adults and the results were published in the Lancet.[9] ***The results showed that individuals who used the intervention experienced a 50% reduction in falls after six months, which is two times more effective than existing interventions (exercise classes, multifactorial interventions, physical therapy).***

[8] *Mirelman, Anat, et al. "Executive function and falls in older adults: new findings from a five-year prospective study link fall risk to cognition." PloS one 7.6 (2012).*
[9] *Mirelman, Anat, et al. "Addition of a non-immersive virtual reality component to treadmill training to reduce fall risk in older adults (V-TIME): a randomised controlled trial." The Lancet 388.10050 (2016): 1170-1182.*

GaitBetter's Effectiveness

By leveraging the latest research on the neuroscience of aging, GaitBetter has demonstrated a fall reduction rate that is up to three times more effective than existing interventions.[10] The reason for this efficacy is brain plasticity. The research team behind GaitBetter performed functional MRI scans and found unique changes in brain activation in the frontal regions, suggesting that GaitBetter training makes our brains more efficient and better able to handle the multitasking required for successful walking.

GaitBetter Utilization and Demand

As soon as the 2016 Lancet paper was published, requests came from clinics around the world asking where and how they could access such a successful technology. Based on this demand, GaitBetter was founded in 2018 and licensed the technology from Tel Aviv University. Our first installation was in May 2019 in Israel. Once we reached a certain level of growth in the company, we expanded into the U.S. with our first installation at Spaulding Rehabilitation Hospital in Boston, Massachusetts in March 2021. As of May 2023, there are 100 systems installed across the U.S. and Israel and we have treated more than 6,000 patients.

Our success in Israel has been explosive. Due to a value-based health care system that prioritizes population health and an environment that incentivizes and rewards innovation that improves outcomes, GaitBetter is now accessible to more than 90% of the Israeli population, available throughout the country. GaitBetter has been implemented in hospitals, outpatient clinics, adult day care centers, and senior living communities. Israeli citizens and the government are benefitting from this proven-effective way to prevent falls among older individuals.

GaitBetter Faces Significant Challenges in the U.S. Market Due to U.S. Federal Policy

Unfortunately, due in large part to outdated Medicare payment policy and bureaucracy at the Administration for Community Living (ACL) and the Veterans Administration (VA), our growth in the U.S. has been limited. While we have received significant interest in the technology, when potential customers learn that Medicare does not provide payment for its use, they often indicate they are unable to proceed with a purchase. For those customers that have purchased the technology, they struggle to make the purchase revenue neutral due to the lack of Medicare payment. Moreover, when we have sought to sell the technology to senior centers and other locations funded through the ACL, we have been told they cannot purchase anything that has not

[10] Sherrington, C, et al. *Evidence on physical activity and falls prevention for people aged 65+ years: systemic review to inform the WHO guidelines on physical activity and sedentary behaviour.* Int J Behav Nutr Phys Act. 2020.

received an "evidence-based" designation by the agency, which has proven impossible because ACL has shut down its review of new falls prevention interventions. We have interested clinical leaders in several VA locations, including Baltimore, the James A. Lovell Federal Health Center in Chicago, and San Francisco, but they have been hindered by an opaque and bureaucratic evaluation and procurement process. We are stuck in the valley of death while tens of thousands of Medicare beneficiaries and dual eligible and U.S. veterans have preventable falls every day, costing them, their families, and the government millions of dollars, in addition to pain and suffering.

Medicare Challenges

The Centers for Medicare and Medicaid Services (CMS) has recognized the problem of falls among the Medicare population and seeks to reduce the occurrence of falls among both patients within the health care system and individuals in the community. However, the current Medicare payment system fails to provide coverage and reimbursement in a manner that proactively supports beneficiary access to clinically-proven technology to reduce falls.

While our technology is proven-effective in published randomized clinical trials, under current Medicare reimbursement policy there is no additional payment available for clinics that wish to use it. There are several existing CPT codes that cover gait training; however, they are reimbursed at a standard rate that does not factor in the purchase or utilization of a technology like GaitBetter. As such, therapists and therapy clinics receive the same level of reimbursement whether or not they use an innovation like the GaitBetter semi-immersive VR technology. Specifically:

- Medicare has no higher paying CPT codes to address combined motor-cognitive therapy; existing codes address either physical therapy **or** cognitive therapy but not both.
- Medicare has no CPT or HCPCS codes for physical therapy that include or reflect the cost of equipment such as GaitBetter, which provides additional clinical value to the patient.
- While Medicare does have a code that allows physical therapists to bill for treating more than one patient at a time it is not adequately reimbursed for the services provided, which limits its use.
- GaitBetter is not considered Durable Medical Equipment (DME): Because GaitBetter is installed with a standard treadmill in an outpatient therapy clinic, it is not considered DME, so reimbursement for the provider or patient is not available through that pathway.

Due to these limitations with existing CPT codes, the installation and monthly subscription fee currently are considered unreimbursed cost-centers for outpatient therapy clinics, which have limited capital equipment budgets and low margins. In an effort to try to solve this problem ourselves, in June 2022 we applied to the American Medical Association (AMA) for a new Category III CPT Code, a temporary code for utilization of "emerging technologies, services, and procedures." This would serve as an add-on code to cover the cost of adding semi-immersive VR

to Gait Training (CPT 97116). The AMA CPT Editorial Panel approved our application in September 2022 and released the new add-on code (CPT 0791T) in December 2022. We have since met or emailed all 12 Medicare Administrative Contractors (MACs) to explain the GaitBetter technology, educate them about the new CPT 0791T add-on code, and explain the potential number of beneficiaries that could benefit from this intervention within their region.

All the MACs indicated that they will not provide any guidance on coverage and reimbursement until CPT 0791T goes live on July 1st, 2023. We are hopeful that the MACs will assign a payment to the code so therapists and therapy clinics then will have an incentive to purchase and utilize the technology since it would be covered and reimbursement. Unfortunately, in our experience, payment drives practice and without additional payment for the technology, it will have a very low adoption rate, despite its clear clinical benefit.

In addition to seeking payment for the technology through the MACs, which handle payments for physician office and standalone outpatient clinic settings, we are hopeful that Medicare will provide payment through the Hospital Outpatient Prospective Payment System, which reimburses for gait training therapy provided in hospital outpatient settings. Again, however, there is no existing code that naturally fits a technology like GaitBetter and we have to appeal to CMS staff to create a new technology Ambulatory Payment Classification (APC) to cover the costs of the GaitBetter software and equipment. We plan to meet with CMS staff this summer and apply for a New Technology APC by the September 1st deadline with the hope CMS will approve the application for payment to become available in January 2024. This would have a significant positive impact on the resources available to hospital outpatient clinics to purchase and deploy GaitBetter to the benefit of the Medicare beneficiaries they serve.

Finally, we are working with the Actuarial Research Corporation (ARC), a veteran-owned small business that is led by former senior professionals from the Medicare Office of the Actuary, to accurately capture the value of utilizing GaitBetter to improve existing gait training practices and reduce the number of falls among Medicare beneficiaries and dual eligibles. Based on ARC's initial analysis, we conservatively estimate that GaitBetter would reduce Medicare falls-related costs by $1.1 billion on an annual basis.

ACL Catch-22

We have also found ourselves almost completely shut out of state and local federally-funded senior centers because those organizations are only allowed to use federal funds to purchase fall prevention interventions that have been identified as "evidence-based" by the ACL. However, the ACL has not been open to reviewing any new interventions since June 2022, effectively preventing any federally-funded entities from purchasing GaitBetter. We have done everything required by ACL to seek an agency review but because the agency has halted such reviews we remain in limbo. The following provides a timeline of our efforts to secure ACL evidence-based review:

- **September 2021**: We submitted a Letter of Intent (LOI) to the National Council on Aging (NCOA) (the organization manages the "evidence-based' program).

- **November 2021**: Based on that LOI, we were invited to submit a Stage 1.

- **January 2022**: Based on our Stage 1 application, we were recommended for a Stage 2 application.

- **March 2022**: We submitted a Stage 2 application and received constructive feedback in May 2022.

- **June 2022**: We met with the technical assistance team to prepare for resubmission based on feedback.

- **July 2022: We were notified that the review program had been paused in June**.

- **October 2022**: We reached out to ACL via email to request a meeting and were told that the review process remains indefinitely on hold. Alarmingly, the response from ACL said "if" the review process is resumed then they would consider meeting – not "when."

- **April 2023:** We reached out to NCOA to inquire as to whether reviews would be restarting and were referred to ACL. The ACL representative indicated the agency was still undergoing an internal evaluation process and that they would reach out to us once the process wraps up and could provide guidance to us on next steps.

Twenty months ago we began the process to secure evidence-based review by ACL so state and local senior centers interested in purchasing GaitBetter could do so with federal funding and make the intervention available to the seniors they serve. Community-based deployment of GaitBetter would help advance falls prevention among seniors who have not yet fallen and, in turn, decrease adverse medical events and the associated costs. ***We understand and appreciate the need for agencies to periodically conduct reviews of their operations; however, ACL has not provided an alternative pathway for innovative technology to get to seniors in federally-funded settings while the agency conducts its internal evaluation. A year has passed since the review process was halted and in that year millions of Medicare beneficiaries experienced a fall, with an estimated three million of them requiring hospital care.***

VA Bureaucracy

We were introduced to the VA when a delegation visited to our Israeli headquarters in late 2019. Representatives from VA were excited by the potential to improve the health of older veterans since, in the words of one VA therapist, "*I'm tired of treating them for a fall, sending them home,*

and then having them return because of a fall six months later." Unfortunately, the process of working with the VA has mostly been challenging. It took approximately three years (from late 2019 to launch in September 2022) to initiate a Quality Improvement Study with the VA Baltimore[11]. As part of a 30-day trial, we installed a GaitBetter system in the James A. Lovell Federal Health Center in Chicago in June 2022. Based on very positive results from Veterans and staff, the purchase was approved in July 2022. However, initial payment was not received until nine months later, in April 2023, which makes doing business with the VA for a small start-up like GaitBetter very financially challenging. Since September 2022, the San Francisco VA has been attempting to purchase three GaitBetter systems but has been stymied by an opaque procurement process.

Attempts to engage with higher levels of the VA have been equally difficult. The Gerofit program, which helps aging Veterans maintain physical and mental health, would seem to be a good fit for GaitBetter; however, VA rules require equipment purchases under this program to be only for research purposes – meaning veterans at risk for a fall but who are not enrolled in a research study would not be able to utilize it. We submitted a request to the VA Innovation Center in February 2021 and followed up but never received a response. In November 2022, we made a submission to the VA Pathfinder Innovation program and again, despite follow-up, have not received feedback or actions, including very limited information about the review process or timeframe. Again, while the VA bureaucracy is blocking access to GaitBetter, veterans coast-to-coast continue to fall and have expensive and preventable injuries. We must do better for those who have served our country.

How Congress Can Help

We appreciate your attention to – and interest in – addressing the barriers to innovation and patient access. We are concerned and frustrated that within the U.S. system we have been unable to bring to bear the benefit that the GaitBetter system has for individuals at-risk for falling. To that end, we respectfully urge you and your colleagues to:

- Communicate with MACs and urge them to provide timely payment for new Category III codes that support the utilization of new technology, such as GaitBetter.
- Encourage CMS to ensure that New Technology APCs are approved for software and equipment that are proven effective interventions, like GaitBetter.
- Direct the ACL to immediate resume consideration of all pending "evidence based" review applications.
- Contact the VA and request that VA Pathfinder Innovation complete its review of pending submissions, like GaitBetter's, in 30 days.

[11] "Veterans getting help to improve balance in VA 'Gait Better' study", Crisfield-Somerset County Times, March 30, 2023 (https://baytobaynews.com/somerset/stories/veterans-getting-help-to-improve-balance-in-va-gait-better-study,104591)

Summary

Again, on behalf of GaitBetter and the millions of Medicare beneficiaries and Veterans at risk for a fall, thank you for this opportunity to provide input regarding the barriers to innovation and patient access. GaitBetter stands ready to be a resource to you and your colleagues and we welcome an opportunity to discuss with you further the policy changes we believe are necessary to improve our nation's incentives for innovation. In particular, there are many lessons learned from our experience in Israel that we would be happy to share. Please feel free to contact me at any time.

Geneoscopy Written Testimony: House Ways and Means Committee Subcommittee on Health Regarding Policies that Inhibit Innovation and Patient Access

Geneoscopy Written Testimony Regarding Policies that Inhibit Innovation and Patient Access
House Ways and Means Committee
Subcommittee on Health
Submitted by
Geneoscopy, Inc.
St. Louis, MO

Thank you for the opportunity to provide written comments regarding policies that inhibit innovation and patient access to medical technology that improves health and saves lives. Geneoscopy is a start-up biotech company based in St. Louis, MO, and our first product is a stool-based colorectal cancer (CRC) screening technology that is currently under review by the FDA. Like many small biotech companies, we worry about the time we will have to wait for revenue flow between approval by the FDA and coverage for our test by the Centers for Medicare and Medicaid Services (CMS) and private insurance. We believe there are unnecessary bureaucratic hurdles that companies like ours encounter in our efforts to bring life-saving technology to patients.

About Geneoscopy

Geneoscopy was founded in 2015 with a vision to improve how gastrointestinal diseases are prevented, detected, and treated. Geneoscopy was started by an MD/PhD candidate at the Washington University School of Medicine in St. Louis, MO who developed a groundbreaking technology to isolate and interrogate RNA. As mentioned, Geneoscopy's initial product is a non-invasive CRC screening test that detects CRC and high risk pre-cancerous polyps – advanced adenomas (AA).[1]

[1] https://pubmed.ncbi.nlm.nih.gov/11916153/

The Promise of New Technology

As technological innovations in the field of preventive screening and diagnostics advance for the country's deadliest diseases, more effective screening modalities become available. For example, Geneoscopy's non-invasive, at-home CRC screening test using mRNA technology has demonstrated the potential to improve the detection of CRC and AA above and beyond existing tests on the market. Geneoscopy's CRC-PREVENT pivotal clinical study demonstrated 94% sensitivity for CRC and 45% sensitivity for AA, representing the highest sensitivity profile reported for any non-invasive CRC screening test in a prospective clinical study.[2] When it comes to screening, more choice is better as it leads to greater compliance. Geneoscopy's clinical trial showed that the new technology worked successfully for people across demographic groups all over the country and has the real potential to advance the vital goal of increasing access to critically needed screening for historically underserved populations. In Geneoscopy's trial, 30% of participants had annual household income below $50,000 and 9% were on Medicaid.[3]

Colorectal Cancer is the Problem: Screening and Early Detection are the Solution

CRC is the third most diagnosed cancer and the second leading cause of cancer death in our country.[4] This year alone, the American Cancer Society estimates there will be 153,020 new cases and about 52,550 deaths nationwide.[5] Everyone is at some risk for developing CRC, however, some groups are at an elevated risk. Of particular concern, African Americans have the highest CRC incidence and mortality rates of all racial groups in the U.S. African Americans are approximately 20% more likely to develop CRC and an estimated 40% more

[2] https://www.prnewswire.com/news-releases/geneoscopys-non-invasive-colorectal-cancer- screening-test-demonstrates-high-sensitivity-and-specificity-in-large-pivotal-clinical-trial- 301717145.html
[3] https://doi.org/10.1158/1940-6207.CAPR-20-0294
[4] https://www.cdc.gov/cancer/colorectal/statistics/
[5] https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21772

likely to die from it than most other populations.[6]

CRC is also the most preventable cancer if people get screened for it regularly. CRC almost always develops from precancerous polyps (abnormal growths, also called adenomas) in the colon or rectum. If these pre-cancerous polyps can be detected and removed through CRC screening, CRC can be prevented before it develops. Moreover, every 1% increase in adenoma detection leads to a 3% decrease in CRC incidence and a 5% decrease in CRC mortality risk.[7] Screening can also identify early-stage cancer. When found at an early stage before it has spread, CRC is more treatable, and the five-year relative survival rate is about 90%. The percentage of individuals diagnosed with advanced-stage CRC has increased from 52% in the mid-2000s to 60% in 2019.[8] Survival rates are lower when cancer has spread outside the colon or rectum.[9]

Unfortunately, many patients avoid screening, and their cancer is diagnosed at later stages. Approximately 40% of patients fail to get screened in part because they do not want to have a colonoscopy, which is the gold standard for CRC screening in the U.S. A colonoscopy is frequently met with patient aversion due to its required bowel preparation, sedation, and potential time away from work.[10] Non-invasive screening tests that can be used at home, such as Geneoscopy's test, serve as important alternatives to colonoscopy for average-risk patients.

Access to Screening for Patients

A key hurdle to bringing life-saving screening tests to patients is CMS coverage and appropriate reimbursement. Additionally, many commercial insurance providers refuse to cover a

[6] https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21772
[7] https://jamanetwork.com/journals/jama/fullarticle/2792977
[8] https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21772
[9] https://www.cancer.org/cancer/colon-rectal-cancer/detection-diagnosis-staging/detection.html
[10] https://www.sciencedirect.com/science/article/pii/S2211335519300750

test until after CMS has done so. Start-up companies like Geneoscopy take risks when developing new technologies and face the "valley of death" when coverage fails to come quickly after FDA approval. Unfortunately, many innovative companies such as Geneoscopy fail to survive the valley of death because of undue delays in coverage. To keep pace with biotech innovation, CMS should follow through on its promise to offer a new predictable pathway for coverage after it repealed the Medicare Coverage of Innovative Technology (MCIT) rule. We strongly believe CMS should issue the Transitional Coverage for Emerging Technologies (TCET) proposed rule to provide a coverage pathway for FDA-approved breakthrough designated products and we are grateful that this committee is helping to highlight this important issue.

Conclusion

New technology and screening tools like Geneoscopy's CRC screening test hold the exciting promise of improving CRC screening rates, enabling early-stage detection of CRC and AA, and, in turn, reducing morbidity and mortality associated with CRC. Once the FDA approves a breakthrough designated test, we believe CMS should take steps to ensure it is covered immediately upon FDA approval. Patients cannot wait to get access to the latest advances in cancer screening; delays by the agency can make the difference between life and death.

We appreciate your consideration of our testimony as you explore ways to support access to innovative technologies for patients. We stand ready to be a resource to you and the committee. Thank you.

HALEON | healthpartner

May 24, 2023

The Honorable Jason Smith
Chairman
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Richard Neal
Ranking Member
Ways & Means Committee
1139 Longworth House Office Building
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The Honorable Vern Buchanan
Chairman
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Lloyd Doggett
Ranking Member
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

Dear Chairmen Smith and Buchanan and Ranking Members Neal and Doggett,

On behalf of Haleon, we appreciate this opportunity to submit a written statement for the record of the May 10th hearing on "*Examining Policies that Inhibit Innovation and Patient Access*." We thank you for undertaking an examination to identify the federal government policies that "have negative effects on medical innovation and reduce patient access to therapies." As a global leader in consumer health with over-the-counter (OTC) brands trusted by millions of consumers globally, we have a long-standing commitment to promoting innovation and expanding patient access to self-care products. To that end, we are appreciative of your interest in uncovering and addressing the barriers to innovation and patient access and stand ready to work with you and your colleagues to help ensure that all Americans have access to the OTC products they need to advance their health and well-being.

About Haleon

Haleon is united by its purpose to **deliver better everyday health with humanity**. **Haleon's products** are built on trusted science, innovation, and deep human understanding and span five major categories: Oral Health, Pain Relief, Respiratory Health, Digestive Health, and Vitamins, Minerals and Supplements. Haleon's world-class portfolio of long-standing brands include **Advil, Sensodyne, Voltaren, Theraflu, Flonase, Polident, Parodontax, and Centrum.**

Haleon supports federal policies, programs, and investments that help consumers have affordable access to OTC products. Haleon stands ready to work with federal policymakers in Congress and the Biden-Harris Administration to ensure that OTC products are accessible to all consumers and that federal policies and programs facilitate access to OTC products, particularly those that help consumers manage pain, maintain oral health and hygiene, get the dietary vitamins and supplements they need, improve digestive health, and treat symptoms associated with cold, flu, and allergies.

FDA Additional Condition for Nonprescription Use (ACNU)

We understand that the hearing generally focused on reimbursement and related policies that are thwarting access to innovations in medicine, devices, and other aspects of health care and that it is your colleagues on the Energy and Commerce Committee who have jurisdiction over Food and Drug Administration (FDA) policy; however, because FDA policy is inextricably linked to access to innovations and is part of the broader health care ecosystem, we respectfully seek to elevate to your attention an issue currently pending before the FDA.

Ensuring Innovations in Over-the-Counter Medications Reach Consumers

Over-the-counter (OTC) or nonprescription medications provide consumers with safe, effective, and reliable options for self-care, which can be more convenient to access and more affordable than prescription products. Pain management, allergy relief, weight loss, and smoking cessation are all health concerns for which consumers have benefitted from access to products that have transitioned or "switched" from prescription to OTC (also known as Rx-to-OTC switch).

Background on Regulation of OTC Products

In the United States, there are only two legal classes of drugs: prescription and nonprescription; both are regulated by the FDA. For a drug to be sold OTC, the drug label must be sufficient for a consumer to properly select, (i.e., decide to use or not use a product) and use safely without the supervision of a healthcare provider. In June 2022, the Food and Drug Administration (FDA) issued a proposed rule, Nonprescription Drug with an Additional Condition for Nonprescription Use (ACNU),[1] seeking to expand the types of products available as nonprescription medicines by allowing product developers to propose applying certain additional conditions of use to support appropriate consumer self-selection and use of these when labeling alone provides insufficient assurance.

An example of an ACNU is a mandatory online questionnaire that consumers would need to complete to determine eligibility for purchase of the OTC product. Questions could include medical and medication history. Upon successful completion of the questionnaire, a code would

[1] https://www.fda.gov/drugs/over-counter-otc-nonprescription-drugs/fda-announces-proposed-rule-nonprescription-drug-product-additional-condition-nonprescription-use

be generated to enable the purchase of the OTC product. If unable to complete the questionnaire, the consumer would not be able to purchase the product.

As a world-leading consumer healthcare company, Haleon supports the FDA's intent behind its proposed rule. We support policies and programs to improve public health, promote greater access to new nonprescription medicines, and increase the options available for consumers to manage their own health and wellness. Further, Haleon supports changes to our regulatory system that promote innovative approaches to self-care and those that leverage advances in technology to support consumer decision-making. **However, we have concerns that the proposed rule, as currently drafted, will thwart innovation rather than facilitate access. The United States is already behind many other countries in enhancing access to OTC products. As such, we believe the ACNU proposed rule requires three changes as it is finalized to achieve the desired and intended public health outcomes**.

Proposed ACNU Rule Challenges and Proposed Solutions

Haleon has identified three issues in the proposed ACNU regulation that must be addressed in order to achieve the FDA's public health goals, support innovation, expand access, and align with ensuring a least- burdensome approach for sponsors.

(1) Simultaneous Marketing Status

As proposed in the rule, an ACNU constitutes a 'meaningful difference' such that simultaneous prescription and OTC marketing is permitted for the same drug product. Meaning, the same drug – identical dose, indication, and modality – can be sold both as prescription and nonprescription. **This is clearly contrary to the statutory language that defines prescription and nonprescription drugs and the FDA's historical application of this legal definition.**

It has been over a decade since FDA last solicited feedback from key stakeholders on the ACNU concept; perhaps reflecting that gap in dialogue, this simultaneous marketing provision is based on an outdated model of the OTC market. While in 2012 when FDA commenced the development of this regulation most OTC companies were integrated with prescription pharmaceutical companies, that is no longer the case. As such, the proposed rule is built on an outdated drug sponsor model and undermines the public health benefits by failing to incentivize innovation and reflect the significant additional investment required to design, develop, and effectively implement a technology-enabled Rx-to-OTC switch for the retail setting. **To that end, the final rule should not include the provision permitting simultaneous marketing and should limit availability only to the nonprescription option.**

(2) Data Requirements to Justify Use of ACNU

The proposed rule requires the drug sponsor to illustrate the reasons why ACNU is necessary – placing the burden on the company. However, there are situations where the

requirement for an ACNU will likely be obvious from the outset and that, in such instances, the FDA should not require the sponsor to undertake duplicative or unnecessary research or data collection. **The timing and degree of information required to support such decision should be discussed and agreed to as early as possible during the development program; this will enable the design and execution of efficient development programs that support a pragmatic, least-burdensome approach to the generation of supporting data. There should be no requirement to generate new data for the purposes of demonstrating that the product requires an ACNU.**

(3) **Post-marketing Reporting**

The proposed rule would establish post-marketing reporting requirements for an OTC drug with an ACNU that are in addition to the requirements that currently apply to an OTC product. Well-established processes for the post-marketing surveillance of OTC products should be applied to ACNU drugs. **Additional new reporting requirements are burdensome and costly and are not necessary to ensure safe and effective use of OTC drugs approved with an ACNU. As such, we urge that the final rule eliminate these additional post-marketing requirements and that the existing post-marketing requirements for OTC products be applicable.**

Expanding Access Requires Action

For every $1 spent on an OTC product, $7 of healthcare expenditure is saved. Expanding access to a wider array of OTC medicines, including those with an ACNU, helps to advance self-care and promote preventive health efforts, which together will improve public health and support more efficient expenditure of limited public and private healthcare resources. The United States currently faces a significant shortage of physicians, nurses, and other healthcare providers, especially in rural areas where access to a physician could be hours away. Furthermore, many drugs remain prescription-only products in the United States, while in other high-income countries they are approved for OTC; Americans deserve easier access to these therapies. As such, there is a significant public health imperative to ensure broader access to proven safe and effective OTC options.

Unfortunately, as written, the proposed ACNU rule significantly increases the costs of technology-enabled Rx-to-OTC switches, disincentivizing innovation, prolongs the approval process, and places unnecessary burdens on product developers. Haleon is committed to working collaboratively with policymakers to ensure new OTC medicines are safe, effective, and accessible to consumers, particularly individuals and families in rural and other underserved communities. **We stand ready to work with members of Congress and the Biden Administration to secure the three aforementioned changes to ensure that the final regulation helps FDA and the nation fully realize the intended benefits and achieve the desired public health outcomes.**

May 24, 2023

Representative Jason Smith
Chairman, House Ways & Means Committee
1011 Longworth House Office Building
Washington, DC 20515

Representative Richard Neal
Ranking Member, House Ways & Means Committee
1011 Longworth House Office Building
Washington, DC 20515

Representative Vern Buchanan
Chairman, House Ways & Means Health Subcommittee
2307 Rayburn House Office Building
Washington, DC 20515

Representative Lloyd Doggett
Ranking Member, House Ways & Means Health Subcommittee
1011 Longworth House Office Building
Washington, DC 20515

RE: Written Statement from Members of the Transplant Community Regarding the House Ways & Means Health Subcommittee May 10, 2023, on "Examining Policies that Inhibit Innovation and Patient Access."

Dear Representatives Smith, Buchanan, Neal, and Doggett:

As members of the transplant community, we appreciate the opportunity to submit a written statement in response to the House Ways & Means Health Subcommittee held on May 10, 2023, on the topic of "Examining Policies that Inhibit Innovation and Patient Access." We are writing to bring your attention to recent administrative policy actions by MolDX, a Medicare contractor, that has limited access to non-invasive post-transplant testing for transplant patients. These decisions may significantly impact the long-term outcomes of transplant patients and may have the long-term effect of cooling innovation in transplant care.

Transplant patients represent some of the most vulnerable individuals within our healthcare system, facing critical health challenges. With over 100,000 people languishing on waiting lists and 17 lives lost daily due to the scarcity of available organs. Access to transplantation is undeniably a matter of life and death. Keeping a close eye on transplanted organs through post-transplant surveillance is crucial. It helps to detect organ rejection at an early stage, which can extend the lifespan of the transplanted organ and ultimately save numerous lives.

The field of transplant medicine has experienced significant advancements through the introduction of innovative non-invasive diagnostic tests, such as donor-derived cell-free DNA (dd-cfDNA) and gene expression profiling (GEP). These tests have been extensively validated and provide evidence-based methods to detect potential subclinical rejection in transplanted organs. Regrettably, the recent decisions by MolDX to issue a new billing article in March 2023 that restricts access to these necessary tests jeopardize the health and well-being of transplant patients. Timely identification of organ rejection or injury is paramount in maintaining

transplanted organs' functionality and ensuring transplant recipients' long-term success. By limiting the availability of molecular diagnostic testing for post-transplant patients, MolDX's actions may inadvertently delay diagnoses and impede the delivery of timely, appropriate care, thereby compromising patient outcomes.

Regular biopsies and follow-up care present significant transportation and logistical challenges for transplant patients who live hours away from their transplant center, hindering timely access to critical diagnostic procedures and monitoring for detecting organ rejection or injury. Additionally, these barriers impose financial burdens and require taking time off work, resulting in lost wages and increased stress for patients and their families. Molecular diagnostic testing offers a transformative solution by providing non-invasive monitoring that can be conducted remotely, minimizing the need for multiple hospital visits, and reducing the impact on patients' work schedules and financial stability. This enables patients to receive crucial post-transplant care without facing additional hardships.

We are gravely concerned by the decisions made by MolDX, as they not only contradict the applicable local coverage determinations (LCDs) but were also implemented without allowing for public comment. This lack of transparency and exclusion of input from the transplant community, including patients, healthcare providers, and experts in the field, is deeply troubling. By silencing these vital voices, the resulting policy fails to fully consider the diverse and complex needs of the transplant community.

As leaders of the House Committee on Ways and Means members, we request your support and collaboration to rectify this situation. We urge the committee to work with our organizations to rescind the MolDX billing article, reinstate coverage based on the initial coverage determination for dd-cfDNA and GEP, and provide additional oversight to ensure that Medicare and its contractors' use of billing articles does not fundamentally change coverage without public input.

Patients who have undergone transplants rely on a supportive ecosystem that encourages innovation and guarantees access to cutting-edge innovations, including molecular diagnostic tests. Thanks to the leadership demonstrated by the Ways & Means Committee, we can collaborate to safeguard patients' access, protect the vulnerable, and foster an environment that promotes innovation in post-transplant care.

We appreciate your commitment to enhancing healthcare for Americans and are available to offer any additional information or help you may need.

Sincerely,

Alport Syndrome Foundation
Ava's Heart
Heartfelt Help Foundation
Lung Transplant Foundation

National Kidney Donation Organization
Texas Kidney Foundation
The HeartBrothers
The Mended Hearts, Inc.
Transplant Families
Transplant Life Foundation
Transplant Recipients International Organization (TRIO)

incubate

The Honorable Jason Smith
Chairman
U.S. House Committee on Ways & Means
Washington, D.C. 20515

The Honorable Richard Neal
Ranking Member
U.S. House Committee on Ways & Means
Washington, D.C. 20515

Re: Health Subcommittee on Examining Policies that Inhibit Innovation and Patient Access

Dear Chair Smith and Ranking Member Neal:

As the U.S. House Committee on Ways and Means Health Subcommittee examines policies that inhibit innovation and patient access, Incubate is sharing the perspective of the early-stage life sciences ecosystem and private capital. Incubate is a coalition of early-stage life sciences venture capital firms representing the patient, corporate, and investment communities. Our primary aim is to educate policymakers on the role of venture capital in bringing promising treatments to patients in need.

While we are sharing research and views on several topics discussed at the hearing, we urge the Committee and the Congress more broadly to immediately end the Small Molecule Penalty included in the *Inflation Reduction Act of* 2022. Adding disincentives for any type of research and development skews investment, altering the medicines available to patients. Incubate's resources on the "small molecule penalty" are available on our website, https://incubatecoalition.org/the-small-molecule-penalty/. This single change will help allow science – not Washington – to pursue the treatments and cures patients are hoping for.

This hearing comes at a critical time. Broader economic pressures are already limiting capital for aspiring bio-entrepreneurs. We need Washington to take immediate and bipartisan action to assure investors, scientists and patients that government will aid, not inhibit the next generation of medicine.

CMS Draft Guidance on Drug Price Negotiation:
Before the Committee, Incubate is reiterating its position that the IRA lacks transparency in the price setting process and disincentivizes small molecule drug development and post-approval research, ultimately sending a perverse signal to the market. The CMS' initial implementation establishes a distorted calculation of the maximum fair price (MFP) and does not mitigate any concerns or address fundamental issues created by the legislation.

For more information, please consider our previous submission to the Centers for Medicare & Medicaid Services' (CMS) on "Drug Price Negotiation Program Guidance: Initial Memorandum,

Implementation of Section 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments".[1]

CMMI's Release of New Drug Payment Models:
Incubate is also concerned that the CMMI's proposed demonstration model to change the way drugs approved via the Food and Drug Administration's (FDA) accelerated approval pathway are reimbursed would place a lower value on certain drugs approved through the pathway despite FDA's determination that they are safe and effective. This change would also discourage investment in some of the hardest-to-treat diseases and, as a result, undermine the fundamental purpose of the pathway, which is to allow for earlier approval of drugs that treat serious conditions with unmet needs.

CMS NCD:
We believe that the CMS' Final National Coverage Decision (NCD) on Certain Treatments for Alzheimer's Disease decision severely restricts patient access to FDA-approved medicines for Alzheimer's disease and sends a signal to the market that novel therapies in these disease areas are not valued. As a result, we expect companies will alter their research and development of new treatments.

Despite consistent warnings that the coverage decision could undercut the investment needed to bring innovative therapies to patients, CMS doubled down on its efforts to second-guess FDA decision-making - making it even harder for patients to access the medicines their physicians deem appropriate. For more information on our position, please consider our previous submission.[2]

WTO TRIPS Waiver:
The importance of intellectual property (IP) protections in early-stage life sciences development remains a core belief of Incubate. We strongly agree there will be significant industry repercussions if IP is weakened, which could ultimately lead to decreased investment and fewer new, life-changing or life-saving drugs.

To view additional resources on our perspective, please find Incubate's testimony and blog in response to the U.S. International Trade Commission's Investigation No. 332-596, COVID-19 Diagnostics and Therapeutics, Supply, Demand, and TRIPS Agreement Flexibilities. [3] [4]

Small Molecule Penalty:
In its current form, the IRA alters the existing system of patents and exclusivities that ensures successful medicines have, on average, 14 years of profitability for future R&D after the medicines are approved. The law implements price controls after nine years for small molecule drugs while biologics are given 13 years.

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[1] https://incubatecoalition.org/incubate-submits-comment-to-cms-regarding-its-draft-guidance-for-the-medicare-drug-price-negotiation-program/
[2] https://incubatecoalition.org/incubate-comment-on-the-proposed-decision-memo-for-monoclonal-antibodies-directed-against-amyloid-for-the-treatment-of-alzheimers-disease/
[3] https://incubatecoalition.org/incubate-testifies-at-usitc-hearing/
[4] https://incubatecoalition.org/incubate-takes-the-stand-testifying-on-global-importance-of-ip/

Resources to help educate policymakers about this "small molecule penalty," are available on our website, including a detailed explainer on the downstream impacts of the penalty on Incubate's podcast, Making Medicine, *The Inflation Reduction Act: A Big Issue for Small-Molecule Medicine.*[5] [6] We call on Congress to fix the IRA's small molecule penalty, as outlined in Incubate Executive Director John Stanford's recent STAT article.[7][8]

Hearing from the investment community on the impact of the IRA in their quarterly earnings calls, we capture shifts and changes in industry investment in our Life Sciences Investment Tracker to measure the immediate and longer-term impacts of the IRA on the ecosystem.[9] The tracker follows two key indicators: public shifts in activity specifically due to new price controls and public announcements of decreased R&D activity in the broader investment environment.

Furthermore, BioCentury conducted an industry survey, comprised of 69 biopharma companies and 10 venture capital or other investment organizations, which found that one third (34%) of biotechs and investors expect the IRA to create major changes or existential crisis for their businesses.[10] Similar concerns are reflected in our investment tracker and across our membership.

We would welcome the opportunity to discuss our feedback on any of these topics in greater detail with the Health Subcommittee; please reach out to John@incubatecoalition.org with any questions you may have.

Thank you for the opportunity to comment on these important issues.

Sincerely,

John Stanford
Executive Director
Incubate Coalition

[5] https://incubatecoalition.org/the-small-molecule-penalty/
[6] https://incubatecoalition.org/episode-19-the-inflation-reduction-act-a-big-issue-for-small-molecule-medicine/
[7] https://www.statnews.com/2023/03/06/congress-must-fix-ira-small-molecule-penalty/
[8] https://incubatecoalition.org/congress-must-fix-the-iras-small-molecule-penalty/
[9] https://incubatecoalition.org/life-science-investment-tracker/
[10] https://www.biocentury.com/article/647205?editionId=clfbtldic6cy60ao7evcqyibt&editionType=daily&utm_source=bctoday&utm_campaign=product&utm_medium=email

Written statement for the Record
of
The National Association of ACOs
for the
House Committee on Ways and Means Subcommittee on Health
hearing on
"Examining Policies that Inhibit Innovation and Patient Access"

May 10, 2023

The National Association of ACOs (NAACOS) appreciates the opportunity to submit comments in response to the health subcommittee's hearing on "Examining Policies that Inhibit Innovation and Patient Access." NAACOS represents more than 400 accountable care organizations (ACOs) in Medicare, Medicaid, and commercial insurance working on behalf of health systems and physician provider organizations across the nation to improve quality of care for patients and reduce health care cost. NAACOS members serve over 8 million beneficiaries in Medicare value-based payment models, including the Medicare Shared Savings Program (MSSP) and the ACO Realizing Equity, Access, and Community Health (REACH) Model, among other alternative payment models (APMs).

NAACOS appreciates the committee's leadership and commitment to driving innovation in the health care system. Value-based payment reforms have a long history of bipartisan support which has generated over $17 billion in gross savings for Medicare over the last decade and improved the quality of care for millions of patients. As the committee continues to discuss long-term approaches for advancing innovation and value in health care, we urge the subcommittee to consider the following recommendations:

Support Legislation to Continue Driving Innovation in Medicare. The Value in Health Care Act is a bipartisan bill that Reps. Darrin LaHood (R-IL) and Suzan DelBene (D-WA) will be re-introducing in 2023 to help grow participation and drive innovation in Medicare. A key aim of the bipartisan Medicare Access and CHIP Reauthorization Act (MACRA) was to speed the transition to patient-centered, value-based care by encouraging physicians and other clinicians to transition into APMs. While MACRA was a step in the right direction, more needs to be done

to drive long-term system transformations. The Value in Health Care Act is the next step that helps drive innovation by:[1]

- Extending value-based care incentives and ensuring that qualifying thresholds remain attainable for clinicians.
- Removing barriers to participation in value-based care models, such as eliminating regulatory burdens for clinicians and improving financial methodologies.
- Evaluating parity between APMs and Medicare Advantage requirements and program flexibility.
- Supporting continued innovation in the MSSP by encouraging CMS to establish a voluntary full risk track that includes options for providers to seek capitated payments for primary care services.

Provide a Broader, More Predictable Pathway for More Types of Clinicians to Engage in APMs. NAACOS appreciates the subcommittee's interest in finding bipartisan solutions to improve the Center for Medicare and Medicaid Innovation (CMMI). Congress established CMMI in 2010 to develop and test innovative payment and service delivery models. While CMS' population health models have seen encouraging growth over the last 10 years, there has been insufficient model development for all types of physicians and other clinicians.

CMMI has tested over 50 models, expanding our understanding of how to shift payment and care processes to improve patient outcomes. However, few models have met the criteria for expansion and lessons learned are not always translated into new models. Unfortunately, little is known about the parameters that must be met for expansion and the model evaluations fail to consider key aspects of innovating care.

Congress should work with CMMI to ensure that promising models have a more predictable pathway for being implemented and becoming permanent and are not cut short due to overly stringent criteria. In February, NAACOS and other stakeholders sent a letter to committee leaders outlining the following recommendations for improving CMMI, including:[2]

- Directing CMS to redesign its evaluation strategies to better isolate specific innovations while controlling for other variables.
- Broadening the criteria by which CMMI models qualify for Phase 2 expansion (e.g., does the model positively address health equity or effectively expand participation to more provider types).
- Directing CMMI to engage stakeholder perspectives during APM development, such as leveraging the Physician-Focused Payment Model Technical Advisory Committee (PTAC).

Encourage Specialist Integration within Total Cost of Care Models. NAACOS supports the Administration's goal of having all Medicare and most Medicaid patients in accountable care relationships by 2030. To successfully achieve this goal, policymakers must allow providers to

[1] https://www.naacos.com/assets/docs/pdf/2023/NAACOSValueHealthCareAct_OnePager.pdf
[2] https://www.naacos.com/assets/docs/pdf/2023/118thCongressValue-BasedCareRecsCoalitionLetter.pdf

coordinate care across the continuum of care. Over the last decade, we have learned that concurrent episode models and total cost of care models result in a complex set of overlapping rules. This leads to provider and patient confusion and increased burden. Designing specialty payment approaches within a total cost of care arrangement can create the proper incentives to encourage coordinated care across the care continuum. In April, NAACOS responded to a request for information from the PTAC encouraging CMS to work with ACOs on the following priorities, including:[3]

- Sharing data on cost and quality performance for specialists with ACOs.
- Supporting total cost of care ACOs with shadow or nested bundled payments for those who elect these arrangements.
- Addressing policy and program design elements that currently are prohibitive to this work.

We appreciate the opportunity to express our views and look forward to working with the subcommittee to ensure that high-quality, coordinated, and person-centered care is accessible to all Medicare beneficiaries.

[3] https://www.naacos.com/assets/docs/pdf/2023/FinalPTACSpecialtyEngagementRFIComments040623v2.pdf

NATIONAL CONSUMERS LEAGUE
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May 23, 2022

Ways and Means Committee Chairman Jason Smith
Ways and Means Committee Ranking Member Richard Neal
Health Subcommittee Chairman Vern Buchanan
Health Subcommittee Ranking Member Lloyd Doggett
1139 Longworth HOB
Washington D.C. 20515

RE: Hearing on Examining Policies that Inhibit Innovation and Patient Access

The National Consumers League (NCL), America's pioneering consumer advocacy organization, applauds the committee's efforts to address patient access issues. This topic is critical for millions of patients and families battling any and all diseases and conditions – particularly those with little to no treatment options.

This issue has been top of mind for the Alzheimer's community as it faces significant and unique access issues to new FDA approved therapies that have been demonstrated in clinical trials to be both safe and effective.

Very little progress has been made in the development of treatments for Alzheimer's. Alzheimer's patients, families, and caregivers have battled this condition with no treatment options. The new innovative medications represent real progress. We are concerned, however, that this progress is being stifled by the Centers for Medicare and Medicaid (CMS)'s decision to severely limit coverage for these treatments.

And as I discuss in a blog post published late last year, CMS has placed itself in direct conflict with the FDA, whose medical experts approved the treatment as safe and effective. In fact, we are concerned that this puts the FDA's entire accelerated approval pathway in the crossfire, sounding an alarm to millions of patients and their families hoping for medical breakthroughs.

The science and medical ecosystem will continue to progress, but we are concerned that by strictly limiting access to an entire class of Alzheimer's treatments, CMS is putting future scientific breakthroughs at risk and may even create a ripple effect throughout the entire healthcare system.

Any drug that emerges from the rigorous development pipeline could essentially be blocked from patients with this precedent in place. As you continue your work on this issue, we ask that you urge CMS to keep pace with the science and give hope to Alzheimer's patients and families. Thank you for your attention to this critical issue.

Sincerely,

Sally Greenberg

Sally Greenberg
Chief Executive Officer
National Consumers League

Ochsner Health

May 24, 2023

The Honorable Jason Smith
Chairman
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Richard Neal
Ranking Member
Ways & Means Committee
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The Honorable Vern Buchanan
Chairman
Health Subcommittee, Ways & Means Committee
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1139 Longworth House Office Building
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The Honorable Lloyd Doggett
Ranking Member
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

Dear Chairmen Smith and Buchanan and Ranking Members Neal and Doggett,

On behalf of Ochsner Health (Ochsner), thank you for this opportunity to submit a written statement for the record of the May 10th hearing on "*Examining Policies that Inhibit Innovation and Patient Access*." We appreciate that you have prioritized identifying the policies and practices of the federal government that "have negative effects on medical innovation and reduce patient access to therapies." Unfortunately, due to existing Medicare and Medicaid payment structures we have been thwarted in our efforts to scale our proven effective digital health offerings. We thank you in advance for your consideration of our views and recommendations and stand ready to serve as a resource to you and your colleagues and you continue your examination of this important issue.

About Ochsner

As one of the nation's leading non-profit, academic health systems, Ochsner Health is dedicated to delivering accessible, affordable, convenient, and effective person-centered care to the more than one million patients we serve who come from every state in the nation and 62 countries across the world. Headquartered in New Orleans, Ochsner consistently is named both the top hospital and top children's hospital in Louisiana by U.S. News & World Report. Ochsner is one of the leading health systems in the country for cancer care, cardiology, neurosciences, liver and heart transplants, and pediatrics. An independent academic medical center, Ochsner performs groundbreaking clinical research on new treatments and emerging medical technologies in nearly 700 active clinical trials.

Ochsner delivers a comprehensive range of services throughout Louisiana, Mississippi, and the

Gulf South region with a clinically integrated network of 48 owned, managed, and affiliated hospitals, and more than 370 health centers and urgent care clinics. Ochsner offers a wide variety of specialized and nationally ranked services with its more than 4,700 affiliated physicians, including 1,800 employed physicians practicing in more than 90 specialties and subspecialties and more than 38,000 employees. In 2022, Ochsner had 22,000 patients enrolled in digital medicine.

Ochsner's Digital Health Innovations

As you may know, Louisiana regularly ranks near the bottom of the U.S. in nearly all health indicators, with a population that has a high prevalence of several risk factors for poor health outcomes, such as obesity, tobacco use, diabetes, and hypertension. In response to the challenge of improving care and outcomes while reducing costs, in 2015 Ochsner created an innovation lab, innovationOchsner (iO) to improve health through innovation. iO has developed numerous digital medicine programs that are transforming the patient experience, enhancing health, and improving well-being, while decreasing costs and reducing the stress on providers of care.

Particularly for individuals who are managing multiple complex diagnoses and chronic disease, these programs are facilitating access to care and improving the patient experience by allowing them to receive the care they need, when and where they need it – principally through wearable technologies, remote patient monitoring (RPM), and virtual provider visits. And, critically, our pioneering telehealth program is meaningfully increasing patient access to care for people in rural and other underserved areas of Louisiana and Mississippi where, in certain cases, no such access existed before. For many patients, telehealth and digital medicine are the standard of care and a preferred way in which they interface with the health care system.

Ochsner's Hypertension Digital Medicine (HTNDM) program uses a digitally connected blood pressure (BP) cuff to automatically transmit BP readings from the patient's home to a dedicated Ochsner care team, which includes a pharmacist and health coach. ***This program has been shown to be at minimum three times more effective than traditional care at achieving BP control at 180 days, while also increasing patients' medication adherence and patient activation, and reducing the total cost of care***.[1] ***Among Medicare and commercial HTNDM participants, an actuarial analysis conducted by a third party, Santa Barbara Actuarial Associates, found a decrease in health care utilization, specifically emergency room visits and hospital admissions, resulting in medical claims savings of $204 per patient per month in Year 1***.

[1] Richard Milani *et al.*, *New aspects in the management of hypertension in the digital era,* CURRENT OPINION IN CARDIOLOGY VOL. 36, NO. 4, JULY 2021, *https://journals.lww.com/co-cardiology/Abstract/2021/07000/New_aspects_in_the_management_of_hypertension_in.6.aspx*.

Similarly, our Digital Diabetes Medicine (DDM) program uses a Bluetooth-enabled digital glucometer to monitor a diabetic patient's A1C and other health indicators. This program also has achieved results that are better than traditional care methods, including reductions in A1C, decreases in hypoglycemic events and diabetes distress, and increases in adherence to recommended health maintenance activities.[2] ***Among DDM Medicare and commercial participants, an actuarial analysis also conducted by Santa Barbara Actuarial Associates, found a similar decrease in health care utilization, specifically emergency room visits and hospital admissions, resulting in medical claims savings of $163 per patient, per month in Year 1***.

The Connected Maternity Online Monitoring (MOM) program provides pregnant patients with a Bluetooth-enabled BP cuff and scale that interfaces with their electronic health record. This allows patients to perform remote monitoring during pregnancy, and as appropriate, decrease the number of in person prenatal visits, while increasing the frequency of monitoring for potential pregnancy complications. ***Analysis of data from early implementation of the program demonstrates that not only does it allow for earlier detection of hypertension in pregnancy, but also increases compliance with post-partum BP monitoring in the initial days and weeks following delivery.***

Fully deploying telehealth and digital medicine to our patients during the public health emergency (PHE) helped us to maintain continuity and coordination of care, as well as allowed for supplemental access to primary care. In many cases, we have been able to reach patients who previously have had limited or no access to such services***. The ability to scale our proven effective digital health programs was possible principally because of the federal telehealth and copayment waivers permitted during the PHE and an FCC PHE telehealth grant program, created by Congress, which covered the costs of the digital devices. However, outside of the PHE, many barriers thwart access to and utilization of these proven effective programs for people living with one or more chronic conditions***.

Barriers and Recommended Solutions

As noted earlier, we are proud that our digital medicine offerings support and empower patients to manage their own health. A key factor stymying the scaling of our digital medicine programs are both the manner and amount that the Centers for Medicare and Medicaid Services (CMS) pays for RPM. Current Medicare payment policy is incredibly restrictive and confusing with respect to when providers can use the RPM codes. For example, under current payment policy, it is unclear if a beneficiary can participate in more than one RPM program at a time; as such,

[2] Richard Milani *et al.*, *Improving Management of Type 2 Diabetes Using Home-Based Telemonitoring: Cohort Study*, NATIONAL LIBRARY OF MEDICINE (June 10, 2021), https://pubmed.ncbi.nlm.nih.gov/34110298/.

patients with multiple chronic conditions and their providers have to choose between their diseases when the patient likely would benefit from participating in two or more digital medicine programs contemporaneously. Further, Medicare beneficiaries need access to both the device(s) and the monitoring service from their providers. Yet, CMS payment policy fails to cover 85% of the costs associated with the device and care – paying only 15% of what it costs Ochsner to provide these programs, which is not financially sustainable.

Another related payment challenge is the imposition of a 20% coinsurance for Medicare beneficiaries, which means whenever the care team reviews the patient's data to inform disease management and the provider bills for the service, the beneficiary may be subject to an out-of-pocket fee. Similarly, for any beneficiaries who do not have supplemental coverage, they are charged out-of-pocket costs (coinsurance or full price) for any digital devices they may need. Further, for a patient with multiple chronic conditions, if they were able to participate in more than one program, they would incur even greater cost-sharing expenses – likely resulting in patients declining participation.

Unfortunately, given the demographics of the Ochsner patient population, affordability of care is a serious impediment to our ability to manage chronic disease for too many of our patients. ***According to Kaiser Family Foundation, approximately 17% of Medicare beneficiaries in fee-for-service have no type of supplemental coverage, which makes paying out-of-pocket costs more challenging and contributes to health disparities.***[3] Coinsurance often stands in the way of patients seeking and receiving the care they need, particularly for Medicare patients with limited resources.

- For Ochsner's Medicare beneficiaries who do not have supplemental coverage to cover out-of-pocket cost-sharing, we have found many decline the opportunity to participate in our digital medicine programs when they learn they would have to pay coinsurance.

- Over the course of the PHE, with the copayments waived due to flexibilities provided by CMS, we experienced a significant increase in enrollment and participation among patients who need these programs, which in turn will help improve their health and reduce costs over time.

- ***As noted above, recently when we issued our annual reconsent to treat, which includes a cost notification of potential out-of-pocket expenses, 43% of beneficiaries who are currently participating, decided to opt-out of the program(s) when they learned that following the end of the PHE they could have 20% cost-sharing requirements.***

[3] https://khn.org/news/article/medicare-enrollment-blitz-doesnt-include-options-to-move-into-medigap/

Due to restrictions on what Medicare providers can offer for free to beneficiaries, providers generally cannot provide digital devices to patients at no cost and providers cannot – outside of the PHE – waive the coinsurance for the RPM benefit/service or the out-of-pocket cost for digital devices. ***This means beneficiaries in digital medicine and RPM programs can accrue a significant amount of cost-sharing for and as such, many of them choose not to enroll. This financial barrier hinders access to care, exacerbates existing health disparities, stands in the way of achieving equity, and otherwise limits the benefits of these programs to beneficiaries who can afford them***. Digital medicine programs save money and improve outcomes – these are the types of services we want Medicare beneficiaries to utilize. Cost-sharing requirements exist to guard against over-consumption of care and services, yet in this case, we want to facilitate the utilization of these services and cost-sharing stands in the way of patient uptake.

Recommendations

We have first-hand experience that demonstrates that the current payment system for digital medicine does not meet the needs of beneficiaries, providers, or the Medicare program. These challenges stand in the way of our implementing person-centered chronic disease management care plans. However, with a few modest changes that we believe CMS has the authority to make, the program would be more accessible for beneficiaries and scalable – allowing more providers to offer it to beneficiaries and, in turn, generating greater savings for the Medicare program.

We believe there are several ways in which CMS could support the further deployment of digital medicine programs such as ours.

One option would be for CMS under the Center for Medicare to allow for the following changes to be permitted for participants in the Medicare Shared Savings Program (MSSP) as there are built in checks and balances related to quality, cost, and outcomes.

- **Create a Per Beneficiary Per Month Payment for RPM and Waive Cost-Sharing:** The current system of individual CPT codes is not efficient for providers or the Medicare program. A single, fixed amount paid per month (e.g., $55 for single disease program), like a subscription model, allows Medicare to pay for a service and permits providers to engage with beneficiaries and their data as frequently as necessary. Given the significant barrier that out-of-pocket costs pose to beneficiary participation and to ensure equitable access, cost-sharing for this secondary prevention service should be waived.

- **Permit and Reimburse Multiple RPM Services Contemporaneously:** At a minimum, Medicare should reimburse providers for multiple RPM services performed contemporaneously for a single patient if a physician, or clinician under general supervision of the physician, recommends multiple remote monitoring services for the patient based on the patient's

specific diagnoses. Recognizing there likely are economies of scale, the per beneficiary per month amount provided for each subsequent program would be prorated; a provider would not be paid three times the standard amount but rather a reduced proportion thereof for each subsequent program (e.g., $55 for single disease program, $75 for two disease programs).

Another path forward would be for CMS to convene a group of RPM stakeholders, including providers and beneficiaries, to discuss the benefits of RPM and further uncover the challenges to scaling RPM and discuss alternative payment models that would reduce burdens on providers and decrease costs for beneficiaries.

A third option would be for CMS, through its Center for Medicare and Medicaid Innovation, to work with providers, such as Ochsner, to design a pilot program or model that would utilize an alternative benefit design to facilitate access to RPM programs for a range of providers, not just MSSP participants.

We respectfully request that you urge CMS to take action to improve its payment policy so it supports – rather than impedes – the deployment of RPM technology for the management of multiple chronic conditions contemporaneously; doing so would advance the bipartisan goal of leveraging technology and innovation to improve chronic disease management, decrease costs, and increase access to care.

Conclusion

On behalf of our physicians, nurses, and other health professionals and the patients and the communities we serve, thank you again for your consideration of our recommendations. We commend you for your interest in identifying policies that are adversely impacting medical innovation and reducing patient access to therapies. We believe digital medicine holds great promise to improve management and treatment of chronic conditions and maintain that with our recommended changes Medicare beneficiaries with multiple chronic conditions will have improved access to care and experience better outcomes; in turn, the total cost of care will be reduced.

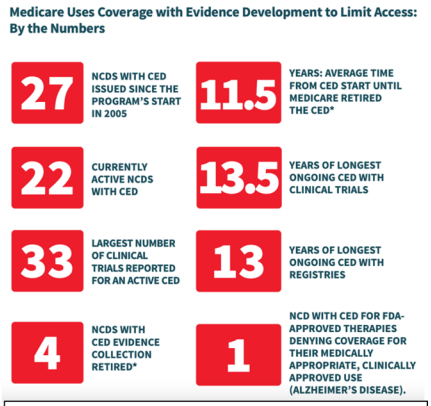
Partnership to Fight Chronic Disease
Written Testimony
United States House Committee on Ways and Means, Health Subcommittee
May 10, 2023 Hearing on "Examining Policies that Inhibit Innovation and Patient Access"

Submitted for the Record May 24, 2023 by Candace DeMatteis, JD MPH,
Policy Director, Partnership to Fight Chronic Disease

Chairman Buchanan, Ranking Member Doggett and members of the Subcommittee, thank you for the opportunity to provide written testimony for the record. The Partnership to Fight Chronic Disease is an internationally recognized organization of patients, providers, community organizations, business and labor groups, and health policy experts committed to raising awareness of the number one cause of death, disability, and rising health care costs: chronic disease. We share the concerns expressed by many members of the subcommittee and witnesses during the hearing over the Centers for Medicare and Medicaid Services (CMS) unprecedented decision to limit access to an entire class of innovative, disease-course-modifying treatments for Alzheimer's disease.

Recently, PFCD and 57 other organizations sent a letter to Members of this Subcommittee and your colleagues in Congress raising serious concerns over CMS' actions to limit patient access to these innovative therapies. As the letter states, "Allowing this unprecedented action to stand will set a new standard that, according to Secretary Becerra, CMS must then consistently apply to new FDA-approved drugs." A copy of this letter is accompanies this written testimony and is submitted for inclusion in the record.

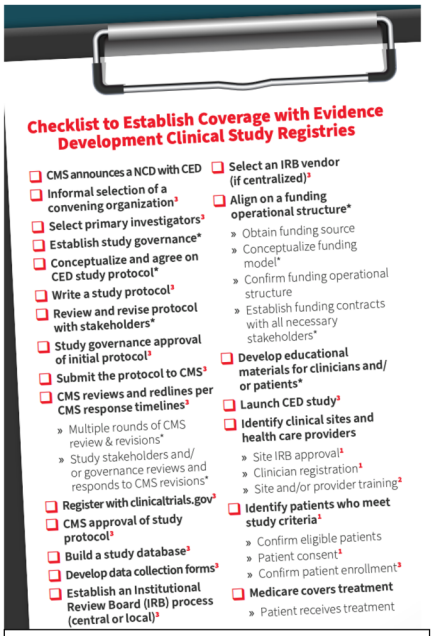
CMS' record with Coverage with Evidence Development (CED) is troubling. Though CED's stated purpose is to collect additional data that Medicare will use to reassess coverage in the future. But the CED process has taken 11.5 years on average before retiring evidence collection and during that time Medicare beneficiaries have limited access and no clear time frame for when the CMS will revisit coverage limitations. The CED clinical study requirements favor large, urban medical centers and often leave rural populations without access. Moreover, CED clinical trials and clinical study registries have historically had a poor record of enrolling diverse participants. With an estimated 2,000 people a day progressing from mild to moderate Alzheimer's disease and beyond the reach of these therapies, delays in access cause irreversible harm.



Full infographic available at:
https://www.fightchronicdisease.org/alzheimers

CMS also has downplayed the significance of requiring beneficiaries to participate in a clinical registry as not limiting access to these innovative Alzheimer's therapies for patients. The reality is that registries take a significant amount of time to establish and receive CMS approval, require providers to pay fees to participate, and require patients to identify providers participating in approved registries who are accessible and accepting new patients. For example, the National Oncologic PET Registry took 18 months to establish. The IDEAS PET Registry and the NEW IDEAS PET registry took 23 and 24 months, respectively.

Notably, although the FDA is set to review one of the new therapies for traditional approval in early July 2023, no registries even currently exist to enroll patients and they typically take more than a year after FDA approval to establish. As the visual here describes, the process to establish a registry is arduous and time-consuming. In contrast, veterans eligible for this therapy and prescribed it by their physicians are already receiving treatment.



Full infographic available at:
https://www.fightchronicdisease.org/alzheimers

Medicare beneficiaries, particularly those living with early-stage Alzheimer's disease, deserve better. We appreciate the work the subcommittee and individual Members are doing to urge CMS to reconsider its harmful decision and correct course. We urge you to continue to use your authority to push for this change, not only will it benefit your constituents living with Alzheimer's, but it could also protect other patients from the harmful precedent this decision sets.

Beneficiaries living with Alzheimer's disease deserve to have the same access to treatment as beneficiaries living with any other illness. Accordingly, CMS' unprecedented decision to restrict access by so limiting coverage for an entire class of Alzheimer's disease therapies must not be allowed to stand.

PARTNERSHIP TO FIGHT CHRONIC DISEASE

A VISION FOR A HEALTHIER FUTURE

May 18, 2023

Dear Members of Congress,

The Partnership to Fight Chronic Disease and 57 undersigned organizations appreciate your attention and ongoing efforts to urge the Centers for Medicare and Medicaid Services ("CMS") to reconsider its ill-advised National Coverage Decision requiring Coverage with Evidence Development for an entire class of Alzheimer's disease therapies. During recent hearings with Secretary Becerra on the Biden Administration's FY2024 budget, Members of Congress, on a bipartisan basis, asked Secretary Becerra important questions that highlighted CMS's intransigence and downplayed the unprecedented nature of CMS's actions.

Every day in America, 2,000 people progress from mild to moderate Alzheimer's disease and any who could have benefitted become ineligible for these treatments. Time is of the essence, and we urge you to continue to press CMS to reconsider its insistence on coverage with evidence development for current and future therapies in this class.

Unfortunately, the testimony of Secretary Becerra in response to the many questions posed to him in recent hearings give even more reason for alarm over CMS's decision, both in terms of the devastating impact on the Alzheimer's disease community and in the precedent-setting move that threatens Medicare coverage for other diseases as well. Soon after CMS announced its NCD on an entire class of potential therapies for Alzheimer's disease, CMS staff rushed to reassure concerned patients and advocates that this was a unique case and did not signal a change of policy.[1]

More recently, however, when asked by members of Congress about this action, Secretary Becerra defended CMS's action, stating that CMS was "simply following the law" that Congress enacted and added that "CMS has to remain consistent in the way it treats any drugs."

CMS's action on an entire class of Alzheimer's therapies is not "consistent" with past policy. In fact, CMS has never before required Medicare coverage be dependent on evidence development for any FDA-approved therapeutic for medically appropriate use according to its

[1] Alzheimer's Forum. Drilling Down into the CMS Aduhelm Decision—A Primer. Apr 22, 2022. Available at https://www.alzforum.org/news/community-news/drilling-down-cms-aduhelm-decision-primer

label.[2][3] Allowing this unprecedented action to stand will set a new standard that, according to Secretary Becerra, CMS must then consistently apply to new FDA-approved drugs. Some of the "most innovative drugs coming to market," including the new Alzheimer's therapies, gene and stem cell therapies, and immunotherapy aimed at infectious diseases and cancers are among therapeutic areas noted by experts as potential ones to target for strict Medicare coverage limitations.[3]

Further, the reach of this new precedent can be seen in the Center for Medicare and Medicaid Innovation's (CMMI's) recently announced mandatory "demo" to pay less for drugs receiving FDA approval through the accelerated approval pathway. CMMI cited CMS's decision on Alzheimer's therapies and differential approach to coverage for those approved using accelerated approval as supporting CMMI's proposed model to discount payments for drugs approved under the FDA's accelerated approval pathway. Specifically, the CMMI report notes, "CMS has also narrowed Medicare coverage of AAP drugs through its Coverage with Evidence Development (CED) process… providing coverage only to beneficiaries enrolled in qualifying clinical trials."[4]

Requiring Medicare beneficiaries already coping with early-stage Alzheimer's disease to enroll and participate in a clinical study to qualify for Medicare coverage will disproportionately affect already underserved beneficiaries and exacerbate well-documented health disparities. People living in rural areas, African Americans and Hispanics who already have higher disease prevalence, and people with disabilities all stand to lose under this proposal. Requiring enrollment in a clinical study for coverage is not coverage. If allowed to stand, CMS's actions will limit the opportunity to delay Alzheimer's disease to people who can either afford to pay out of pocket or have the means and supports to identify clinical study locations and manage the logistics needed to qualify, enroll, gain access to coverage, and maintain coverage through continued study participation. Even those who could access a study have to wait for these studies to commence – which typically take more than a year to establish before enrollment even begins.

The patient and provider community continue to reach out to CMS, to provide data showing evidence of benefits, to highlight the unprecedented nature of their actions, the disparate

[2] Ibid.

[3] CERSI Summit - Panel 2: Cross-Agency Synergy to Accelerate Access to Medical Products, https://www.youtube.com/watch?v=2acW0KMYCII&t=1398s (see, e.g., minutes 20:00-22:32)

[4] Secretary Xavier Becerra. US Department of Health and Human Services. A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans. Available at https://innovation.cms.gov/dataandreports/2023/eo-rx-drug-cost-response-report.

impact on vulnerable populations, and the tremendous unmet need that CMS is making worse. We need your help to convince CMS to reconsider by continuing to ask Administration officials tough questions, to urge the Biden White House to push for action, and, if necessary, to pass bipartisan legislation that ensures CMS reconsiders this decision and avoids such actions in the future.

The Partnership to Fight Chronic Disease and the 57 signed organizations stand ready to provide additional information and support needed to continue raising awareness and motivating action around CMS reconsideration of this NCD with CED decision.

Signed,

Alliance for Aging Research
Alliance for Patient Access
Alzheimers Orange County
American Senior Alliance
American Society of Consultant Pharmacists
Autoimmune Association
Caregiver Action Network
Caring Ambassadors Program
Center for Global Health Innovation
Center for Patient Advocacy Leaders (CPALs)
Chronic Care Policy Alliance
Chronic Disease Coalition
Dementia Alliance of North Carolina
EveryLife Foundation for Rare Diseases
Firefly Fund
Friedreich's Ataxia Research Alliance (FARA)
Genetic Alliance
Genome Creative, LLC
Georgia Bio
Global Alzheimer's Platform Foundation. Inc.
Global Coalition on Aging Alliance for Health Innovation
Global Healthy LIving Foundation
Great Lakes Hemophilia Foundation
Haystack Project
HealthyWomen
Kaleidoscope Fighting Lupus

Latino Alzheimer's and Memory Disorders Alliance
Dementia Alliance International
LEAD Coalition (Leaders Engaged on Alzheimer's Disease)
Lewy Body Dementia Association
Lupus and Allied Diseases Association, Inc.
Lupus Foundation of America
Michigan State University Alzheimer's Alliance
Minnesota Society of Clinical Oncology
Myositis Support and Understanding
National Association of State Long Term Care Ombudsman Programs (NASOP)DC Ombudsman
National Consumers League
National Grange
National Kidney Foundation of Wisconsin
Nevada Oncology Society
No Patient Left Behind (NPLB)
Noah Homes Inc
NTM INFO & RESEARCH
NutriStyle
Ohio Council For Cognitive Health
Oregon Health and Science University
Partnership to Fight Chronic Disease
Patients Rising Now
Prevent Blindness Wisconsin
PSC Partners Seeking a Cure
RetireSafe
Rio Grande Valley Diabetes Association
Second Wind Dreams
Texas Healthcare and Bioscience Institute
The Balm In Gilead, Inc
UsAgainstAlzheimer's
Voices of Alzheimers
Wisconsin State Grange

PATIENTS FOR AFFORDABLE DRUGS NOW™

Statement of David E. Mitchell
Founder and President, Patients For Affordable Drugs Now

Submitted To The

Committee on Ways and Means Subcommittee on Health
U.S. House of Representatives

for a hearing on

Medical Innovation and Patient Access to Therapies

May 10, 2023

Section I. Background and Introduction

My name is David Mitchell. I am the founder of Patients For Affordable Drugs Now. We are a bipartisan organization focused on policies to lower prescription drug prices. We don't accept funding from any organizations that profit from the development or distribution of prescription drugs.

Our job is to collect and amplify the stories of patients struggling to pay high drug prices, and to help them share their experiences with policymakers and elected officials. You can read more than 33,000 stories on our website today. And we have built a community of more than 400,000 patients and allies who support policies to lower drug prices.

More importantly for the Committee, I have an incurable blood cancer, and prescription drugs are keeping me alive — literally.

My doctors currently have me on a four-drug combination of infused and oral cancer drugs which carry a combined list price of more than $900,000 per year. Just one of my oral drugs, called Pomalyst, is priced at more than $23,000 for 21 capsules, which I must buy every 28 days. And because people on Medicare like me pay our costs in Part D based on list price, I will spend

more than $17,000 out of pocket this year — just for Pomalyst. For people with my cancer — multiple myeloma — drugs account for 60 percent of the cost of treatment.[1] Sixty percent.

I am a very lucky man — these drugs are currently keeping my cancer at bay, and I tolerate them well. But eventually I will fail on this combination, too. When that happens, I will need a new treatment. Fortunately, there are options out there and more in development.

The point is: I need these innovative drugs. I care deeply about innovation and new drug development. My life depends on it. Without innovation, I will die sooner than I hope to. That is just an unfortunate fact.

But my more than 12-year journey as a cancer patient has taught me one irrefutable fact: Drugs don't work if people can't afford them.

Section II. The Price of Drugs and Need for Change

Despite historic reforms enacted last year, too many drugs are still too expensive in the United States, and there is no justification for the high prices. When drug makers hike prices, they don't do so because the drug suddenly becomes more innovative or clinically effective. Drug companies raise prices because they can. We let them. Starting this year we will curb these price increases in Medicare through the Inflation Reduction Act, but there is currently no direct mechanism to rein in these increases in the commercial market.

The result is that Americans pay nearly four times what people in other wealthy nations pay for the exact same brand-name drugs.[2]

Consequently, nearly 40 percent of people in this country report having difficulty affording their medications.[3] When their prescription drug prices are too high, patients don't adhere to their drugs and this harms their health[4].

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[1] Tran, D., Kamalakar, R., Manthena, S., & Karve, S. (2019, November 13). Economic Burden of Multiple Myeloma: Results from a Large Employer-Sponsored Real-World Administrative Claims Database, 2012 to 2018. *Blood*, 134, 3414. https://doi.org/10.1182/blood-2019-131264

[2] Schondelmeyer, S., Purvis, L. (2021). Trends in Retail Prices of Brand Name Prescription Drugs Widely Used by Older Americans, 2006 to 2020. *AARP Public Policy* Institute https://www.aarp.org/content/dam/aarp/ppi/2021/06/trends-in-retail-prices-of-brand-name-prescription-drugs-widely-used-by-older-americans.10.26419-2Fppi.00143.001.pdf

[3] Nguyen, A. (2021, March 22). Survey: Americans Struggle to Afford Medications as COVID-19 Hits Savings and Insurance Coverage. *GoodRx*. https://www.goodrx.com/blog/survey-covid-19-effects-on-medication-affordability/

[4] Congressional Budget Office. (2023). How CBO Estimated the Budgetary Impact of Key Prescription Drug Provisions in the 2022 Reconciliation Act. https://www.cbo.gov/system/files/2023-02/58850-IRA-Drug-Provs.pdf

Due to high drug prices, Americans face challenges affording other expenses, such as food and housing. One survey found that over 20 percent of people took on debt or declared bankruptcy because of their medications.[5]

High drug prices disproportionately harm communities of color. One in two Latinos in the United States takes a prescription medication, and 20 percent are uninsured.[6] Black Americans are more likely to live with chronic pain, diabetes, and high blood pressure than white Americans and are nearly two times more likely to be uninsured.[7]

Too many Americans with and without insurance are struggling everyday to pay high prices.

But instead of lowering prices, the drug industry maintains that this unacceptable status quo in which they can charge whatever prices they please is necessary in order to guarantee they can invest in future research and development (R&D). In other words, drug companies take the position that patients must suffer poor health and financial harm in the short term in order for drug companies to preserve the possibility of potential innovation in the future.

This is a false choice, aiming to prey upon the hopes and fears of patients and their families. We can achieve balance to have innovative, safe, and effective drugs at prices all people can afford. The Inflation Reduction Act is an example of policy that takes us in the right direction.

Section III: The Inflation Reduction Act Protects and Incentivizes Innovation

Implementation of the Inflation Reduction Act (IRA) is a critical step towards achieving a future where the need for innovation is balanced with affordability. For the first time, people on Medicare will be protected from catastrophic financial hardship due to drug costs with a $2,000 cap on out-of-pocket costs. For the first time, drug companies will not be able to raise prices at will in Medicare year after year. And finally, after years of effort to achieve reform, the government will be able to negotiate lower prices for a subset of the most costly drugs on behalf of beneficiaries.

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[5] Nguyen, A. (2021, March 22). Survey: Americans Struggle to Afford Medications as COVID-19 Hits Savings and Insurance Coverage. *GoodRx*. https://www.goodrx.com/blog/survey-covid-19-effects-on-medication-affordability/

[6] UnidosUS Action Fund. (2021, January). *A Vicious Cycle of Health Inequity: How High Prescription Prices Hurt Latino Health and Prosperity*. https://www.lowerdrugpricesnow.org/wp-content/uploads/UNIDOS-RX-REPORT-Vicious-Cycle.pdf

[7] Patients For Affordable Drugs Now. (2020, December 14). *High Prescription Drug Prices Perpetuate Systemic Racism. We Can Change It*. https://patientsforaffordabledrugsnow.org/2020/12/14/drug-pricing-systemic-racism/

Big drug corporations and their allies tell us that the IRA will devastate the industry and obliterate innovation — but this is another false narrative repeated by opponents of reform who are actually *proponents* of higher prices and the harmful status quo for patients.

In fact, the IRA improves and builds upon existing incentives for innovation in two key ways:

- Currently, drug companies can pursue profits by recycling — and jacking up the prices on — old blockbuster products that have no competitors despite enjoying monopoly periods far beyond what is intended under law. The Medicare price hike rebates and negotiation provisions in the IRA address this directly by penalizing price increases higher than inflation and by targeting negotiation to old drugs that should have competition, but don't. This much-needed shift in incentives will force drug companies to invest instead in new and truly innovative products that can command high prices.

- In contrast to the industry's current process of setting arbitrary prices, the Centers for Medicare & Medicaid Services (CMS) will begin to negotiate prices on a subset of very old and costly drugs based on the *clinical benefit* of a drug and other key factors to be sure we reward important and high quality new drugs with prices befitting their contribution to health and an appropriate profit. It is axiomatic that to stimulate and reward innovative new drug development, we should pay more for high value drugs and less for low value drugs. The IRA negotiating process is structured to encourage truly innovative, high value drugs.

The IRA also preserves existing incentives in law for innovation:

- Drug companies can continue to set prices at launch and enjoy years of market exclusivity to ensure they are well-compensated for investment and risk before possibly being eligible for Medicare negotiation.
- Medicare must cover all drugs in six protected classes, which even the Pharmaceutical Research and Manufacturers of America (PhRMA) acknowledges ensures access to these drugs.[8-9]
- Medicare must cover at least two drugs in each class of drugs.[10]

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[8] Centers for Medicare & Medicaid Services. (2019, May 16). *Medicare Advantage and Part D Drug Pricing Final Rule (CMS-4180-F)*. https://www.cms.gov/newsroom/fact-sheets/medicare-advantage-and-part-d-drug-pricing-final-rule-cms-4180-f

[9] Powaleny, A. (2015, December 10). Medicare Part D's six protected classes. *PhRMA*. https://catalyst.phrma.org/medicare-part-d-six-protected-classes

[10] *What Medicare Part D drug plans cover*. (n.d.). CMS.gov. Retrieved May 3, 2021 from https://www.medicare.gov/drug-coverage-part-d/what-medicare-part-d-drug-plans-cover

- Medicaid must cover every drug offered by a manufacturer in the United States if the manufacturer agrees to give Medicaid a best-price guarantee.[11]

Section IV. The Truth About Innovation and Drug Prices

Of course, the reason the biopharmaceutical industry seeks to undermine the IRA is because *it opposes any reforms that curb its unilateral power to dictate prices for brand drugs.* It insists it must be able to set prices as high as it wants for virtually as long as it wants or it won't be able to attract investment. So it rolls out its well-worn claim that any limits on its ability to set high prices will destroy innovation and access to new drugs.

No one cares more about innovation than patients. But if you pull back the curtain on this fear-mongering, the industry's arguments don't hold up.

Experts from both sides of the aisle agree it's possible to rein in the pharmaceutical industry's abusive pricing power without threatening valuable innovation.[12-13] Here are six reasons why:

1) Biopharma corporations enjoy profit margins that are almost three times the average of the S&P 500.[14] Brand-name pharmaceutical companies could lose $1 trillion in sales over 10 years and remain the most profitable industry in the United States.[15] There is more than enough headroom to lower drug prices and leave drug companies with plenty of profit to attract investment and fund research and development. And if drug pricing legislation curbs profits, the industry can maintain or even increase R&D investment by shifting the billions spent on stock buybacks, marketing, advertising, and lobbying.[16]

2) It doesn't cost nearly as much as the industry says it does to develop a new drug. Pharma claims it costs $2.87 billion to bring a new drug to market. But that's based on industry-

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[11] Kaiser Family Foundation. (2019, May 1). *Medicaid's Prescription Drug Benefit: Key Facts.* https://www.kff.org/medicaid/fact-sheet/medicaids-prescription-drug-benefit-key-facts/

[12] Frank, R. G. (2019, November 13). Drug companies exaggerate — controlling drug prices won't threaten innovation. *The Hill*. https://thehill.com/opinion/healthcare/470266-drug-companies-exaggerate-controlling-drug-prices-wont-threaten-innovation

[13] Waikar, S. (2020, September 2). Pharma Companies Argue That Lower Drug Prices Would Mean Fewer Breakthrough Drugs. Is That True?. *Kellogg School of Management at Northwestern University*. https://insight.kellogg.northwestern.edu/article/pharma-companies-argue-lower-drug-prices-fewer-breakthrough-drugs

[14] Yardeni Research. (2021, January 19). *S&P 500 Sectors & Industries Profit Margins (quarterly)*. https://www.yardeni.com/pub/sp500margin.pdf

[15] West Health. (2019, November 14). *New Analysis Finds Large Drugmakers Could Lose $1 Trillion in Sales and Still Be the Most Profitable Industry*. https://www.westhealth.org/press-release/new-analysis-finds-large-drug-makers-could-lose-1-trillion-in-sales-and-still-be-the-most-profitable-industry/

[16] Angelis, A. et al. (2023). High drug prices are not justified by industry's spending on research and development. *BMJ*. http://press.psprings.co.uk/bmj/february/drugprice.pdf

funded research and undisclosed source data.[17-18] Independent studies have found the cost to develop a drug is likely less than $1 billion.[19-20]

3) A tremendous amount of research and development is coming from taxpayers. The National Institutes of Health (NIH) is the single largest biomedical research agency in the world. NIH-funded research is associated with all 356 new drugs that were approved by the FDA from 2010 to 2019.[21] Former NIH Director Francis Collins has said: "Finding new treatments thus requires NIH to play a lead role — by investing in the early stage of therapeutic development to 'de-risk' such projects."[22] Drug companies argue high drug prices are required to reimburse the industry for the financial and scientific risk it takes on during research and development. In reality, the U.S. government takes on most of those early risks, further undermining the industry's argument for high prices.

   Our experience with COVID-19 vaccines illustrates this point with crystal clarity. Drug companies were not investing in vaccines in the 1990s and early 2000s because vaccines were seen as too risky and offering low profits[23]. Drug makers only mobilized in response to Operation Warp Speed when the government offered billions of dollars in contracts to develop COVID-19 vaccines and therapeutics. Our government de-risked the investment and privatized the profit so now COVID vaccines are among the highest grossing drug products of all time.

   One noted industry expert, Jack Scannell, summed it up this way: "Before we pat the drug industry on the back too much, one has to recognize it got involved in this partly because the whole thing has been de-risked by government."[24]

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4) Pharma's claims that patients will suffer an alarming loss of new drugs as a result of the IRA simply isn't supported by the facts. According to the CBO, the Inflation Reduction Act will decrease the number of new drugs over the next 30 years by only about 15 out of 1,300 expected – that's less than 2 percent. Since only 10 to 15 percent of "new" drugs represent true therapeutic advancements, of the 15 new drugs foregone, only one or two might actually be true innovations[25-26]. The loss of a few drugs each year will have minimal to no impact on the health of Americans.

5) The industry has plenty of money for innovation. In the wake of the Inflation Reduction Act passage, investors are upbeat. Drug company stocks are doing fine.[27] The industry is flush with cash and has great access to capital.[28]

   - Pfizer is buying biotech company Seagen for $43 billion. [29]
   - Sanofi is buying a diabetes product company for $2.9 billion. [30]
   - Novartis is spending $15 billion in a stock buyback. [31]

   Even in the face of the Inflation Reduction Act, drug companies are reporting *increased* investment in R&D. For example, in 2022 10-K filings[32], J&J reported an 11.8% increase in R&D spending in 2022, Merck reported an 11% increase in R&D spending, and Moderna reported a 65% increase in R&D spending and projected further increases in 2023.

6) Big Pharma threatens that patients in the U.S. will lose access to newly developed drugs. It points out that more drugs are available — and are available faster — in the United

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States than in other wealthy countries. It frequently references a white paper from the White House Council of Economic Advisers (CEA) to explain why: "Drug manufacturers usually pursue market access in the United States before other markets due to the higher prices in the United States."[33] The CEA could also have mentioned the other big reason drug companies file for approval first in the United States: It is the *largest* market in the world.[34-35]

Given that U.S. prices for brand-name drugs are almost four times what many other wealthy nations pay, we can lower prices by a meaningful amount and still offer the highest prices by far in the largest market in the world, preserving the incentive to file first for approval in the United States. [36]

Section V: Recent Innovative Payment Strategies

Let me be clear. P4ADNow supports investment into medicines for rare and severely under-funded diseases such as Alzheimer's. We applaud drug companies that are researching and developing truly innovative therapies. And those companies will continue to be paid the highest prices in the world for their successes—even with IRA provisions that improve affordability for patients.

As a country, we should reward drug companies that invent truly innovative products—but our health system cannot afford to pay drug companies arbitrarily high prices for ineffective or even unsafe drugs. Patients and families grappling with Alzheimer's disease, for example, must have safe and effective treatments. But the way to increase innovation is not to provide incentives for non-innovative or unsafe therapies.

As a cancer patient relying on three drugs that came to market through the accelerated approval pathway, I urge lawmakers to preserve options and incentives for drug companies to bring therapies to market as quickly as possible. But we must also be sure to confirm that accelerated approval drugs are, in fact, safe and effective through timely confirmatory trials. And as a patient on Medicare, I am also glad CMS has tools to protect beneficiaries from paying a portion of an often limited or fixed budget towards a medication that has not been shown to be safe and

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effective–or worse, a drug that might cause harm. Like many other facets of the drug pricing system—utilization of these tools requires balance and discretion. And I am troubled by characterizations of any effort by CMS to create thoughtful guardrails on potentially unsafe drugs as an assault on innovation and patient access.

Section VI: Conclusion

Pharma's threats to innovation and access don't hold up. It is clear that we can balance our system to have fair prices and profits and still get the innovation we need.

Equally important, we must remember that people can't afford existing drugs they need right now. High prices represent a huge barrier to access and better health. More than 1.1 million people on Medicare could die over the next decade because they cannot afford to pay for their prescriptions.[37] America needs policies that maximize public health while ensuring fair private profit.

It is truly ironic that this committee is holding this hearing after the House of Representatives voted last week to cut next year's NIH budget by $10 billion dollars—20 percent! Given the key role of the NIH in developing basic science and the pipeline for new drugs, the House bill will likely do far more harm to new drug development than any of the provisions in the IRA. According to *Medpage Today*, "Lawrence Tabak, DDS, PhD, acting director for the NIH, acknowledged that cuts would have a 'chilling effect on the entire biomedical research enterprise' and deter young people from pursuing careers as scientists.'" The hypocrisy of this hearing in light of last week's House vote is breathtaking.

To be clear, those who try to undermine the Inflation Reduction Act and other thoughtful efforts to ensure patients can afford the drugs they need are–practically speaking–advocates for higher drug prices. The key drug price provisions of the IRA are overwhelmingly supported by more than 80 percent of Americans–Democrat and Republicans alike. [38] Elected officials tamper with these reforms at their own political risk.

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House Committee on Ways and Means Subcommittee on Health
Hearing on Examining Policies that Inhibit Innovation and Patient Access
Written Statement for the Hearing Record

Personalized Medicine Coalition

May 23, 2023

Chairman Buchanan, Ranking Member Doggett, and distinguished members of the Health Subcommittee of the House Committee on Ways and Means, the Personalized Medicine Coalition (PMC) appreciates the opportunity to submit comments on the potential impacts of government policies on medical innovation. PMC is a nonprofit education and advocacy organization comprised of more than 220 institutions from across the health care spectrum who support this growing field. In our following statement, we encourage Congress to thoughtfully consider how policies around drug price negotiations, breakthrough devices, and drugs that receive accelerated approval can incentivize continued innovation in personalized medicine and facilitate patients' timely access to the pharmaceutical, diagnostic, and other technologies underpinning this approach to care.

Personalized medicine, also called precision or individualized medicine, is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients. Personalized medicine is playing an important role in transforming care and patient outcomes for a range of serious and life-threatening diseases and conditions, helping to shift patient and provider experiences away from trial-and-error toward a more streamlined process for making clinical decisions. By ensuring that only patients who will benefit from a particular intervention receive it, personalized medicine can also make the health care system more efficient.

As personalized approaches to treatment and prevention have emerged, new types of drugs, tools, and technologies using a patient's genetic and other personal health information have challenged existing regulatory frameworks and processes. Chimeric antigen receptor (CAR) T-cell therapies in oncology, gene therapies for pediatric rare diseases, next-generation sequencing technologies, and biomarker imaging with molecular diagnostics are just a few of the innovations that are unlocking a new era of personalized care. To facilitate patients' timely access to personalized medicine, PMC advocates for flexible coverage policies and adequate payment rates for personalized medicine treatments, diagnostic tools, and technologies that recognize the value these technologies provide to patients, the health care system, and society.

Mitigating Potential Impacts of Medicare's Drug Price Negotiation Program

With the passage of the *Inflation Reduction Act (IRA)* in 2022, Congress gave the Centers for Medicare & Medicaid Services (CMS) the authority to negotiate the prices for certain drugs as soon as 2026. CMS' implementation of the drug price negotiation program represents an

unprecedented new federal authority that will significantly alter how personalized medicine will be evaluated and incentivized under Medicare. Multiple analyses, including those from the Congressional Budget Office, have called attention to the potential consequences of the Medicare drug price negotiation program, such as canceled research and development and disincentives to invest in small molecule medicines and therapeutic areas that require incremental innovation.[i,ii,iii,iv]

Due to smaller patient subpopulations, personalized medicines that address the root causes of disease can sometimes be expensive and risky to develop. Now an important part of health care, personalized medicines have accounted for at least a quarter of new drug approvals for each of the past eight years.[v] In 2022, over half of U.S. Food and Drug Administration (FDA)-approved personalized medicines were indicated for certain cancers, and over one-third were indicated for rare diseases.[vi] There are more than 10,000 rare diseases, including rare cancers, and more than 90 percent of them do not have an FDA-approved treatment.[vii] With companies expected to focus on treatments for larger patient populations where return on investment can be easier, treatment pipelines for cancers and rare diseases are expected to be impacted by Medicare's drug price negotiation program.[viii,ix]

Research conducted after approval of a new drug is important to advancing personalized medicine. After initial approval of a targeted therapy by FDA, further research provides greater understanding of patients' responses to treatment based on results from molecular diagnostics and other biomarkers. This research leads to new or improved treatment indications that contribute to progress in personalized medicine, but smaller patient subpopulations can make it difficult to recoup investment in this research. The potential downstream impacts of the negotiation program are expected to curtail post-approval research.

Over the past eight years, PMC has identified more than 120 expanded indications significant to advancing personalized medicine. Notably, these expanded indications have had an upward trend in the average time since a drug's initial approval. Given this trend, PMC is concerned that implementation of the negotiation program, which by statute makes drug products eligible for negotiation after nine years (or 13 years for biological products), may further stifle post-approval research for expanded indications that provide patients with personalized medicine treatment options. By limiting the exemption of orphan drugs from negotiation to those with only one approved orphan indication for a single disease or condition, the negotiation program could also stifle post-approval research into additional orphan indications for rare disease patients who lack treatments due the risk of losing this exemption.

In March, CMS released its *Medicare Drug Price Negotiation Program Initial Guidance*[x] outlining how the agency will select drugs for negotiation, gather and use relevant data, and carry out the negotiation process to establish a maximum fair price (MFP) for selected drugs for the initial price applicability year (IPAY) of 2026. PMC believes the initial guidance lacks clear descriptions for CMS procedures and methodology. Based on this lack of detail, we are concerned that CMS could implement this new program in ways that unintentionally undermine the incentives for developing innovative medicines, including drugs with personalized medicine treatment strategies that direct them toward patients who are most likely to benefit and away from those who are not. PMC submitted comments to CMS urging the agency to establish a

consistent and transparent methodology for determining a drug's MFP that considers the value of personalized medicine to patients and society and allows a more robust exchange of information with stakeholders that meaningfully considers patients' perspectives on value. While CMS has indicated it plans to release updated guidance as soon as early July, we are concerned that the tight statutory deadlines under the *IRA* for the agency to implement this new program may limit CMS' ability to respond to these and other comments raised by the public.

Medicare's drug price negotiation program could also have an outsized effect on patients' access to new and existing treatments that extends beyond the Medicare program and possibly narrows patients' treatment options. Although Medicare plan sponsors will be required to include selected drugs on their formularies, plans could use restrictive utilization management or other cost-control practices to manage their increased liability and deny coverage for negotiated products vital to a patient's personalized health care. To ensure patients are protected from plan attempts to offset costs, our Coalition has encouraged CMS to establish guardrails and conduct oversight to ensure the clinical appropriateness of any utilization management or formulary changes and to mitigate unintended consequences on beneficiaries' access to both negotiated and non-negotiated drugs.

Congress and the administration should take every step possible to prevent, monitor, and correct for potential impacts of the negotiation program on patients and the health care system. We believe information should be collected on potential unintended impacts to ensure the program does not disincentivize the development of new treatments for unmet medical needs, research on expanded indications that provide additional benefits to patients, or patient access to personalized medicine through cost-control practices.

Facilitating Timelier Medicare Coverage of Breakthrough Devices

A study by the Stanford Byers Center for Biodesign found it takes an average of five years for medical devices to achieve nationwide coding, coverage, and payment.[xi] For devices addressing areas of unmet medical need, the newness of the device and, in some cases, small patient population sizes, can create challenges to gathering the clinical evidence needed for coverage and reimbursement determinations, subsequently increasing the time between introduction to the market and patient access. For personalized medicine to realize its full potential at a moment of rapid progress in science and medicine, patients need timelier access to innovative medical technologies they stand to benefit from.

Devices that receive breakthrough designation and marketing authorization from the FDA provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions. A new Medicare coverage pathway expediting patient access to medical products designated as breakthrough devices and authorized by the FDA could mitigate the upfront evidence burden required to meet the current coverage standard while prioritizing patients' unmet medical needs. We believe such a pathway could benefit patients by helping to provide timelier coverage for, and thus access to, certain diagnostic and screening tests as well as other enabling technologies underpinning personalized medicine, such as digital health technologies that leverage artificial intelligence.

Over the past several years, PMC has been engaged on efforts at CMS to develop such a pathway for breakthrough devices. Since the repeal of the Medicare Coverage of Innovative Technology (MCIT) pathway, we have eagerly awaited the Biden Administration's proposed rule on Transitional Coverage for Emerging Technologies (TCET), which was introduced as a replacement for MCIT and was slated to be released in April of 2024. TCET has the potential to provide Medicare beneficiaries access to a broader range of treatment options and to enable patients, in consultation with their doctor, to make informed, personalized decisions about their care. We look forward to reviewing CMS' expected proposal and evaluating its ability to advance personalized medicine.

We believe a successful TCET pathway would be voluntary for breakthrough device manufacturers and would provide predictable coverage at the time of marketing authorization. Timely coverage will improve patients' access to these technologies and facilitate the post-market collection of any additional evidence needed to inform future coverage and reimbursement decisions. To help minimize the burden of post-market evidence collection on device manufacturers, clinicians, and patients, we believe it will be important for this pathway to foster alignment between CMS and the FDA on evidence needs and facilitate early alignment between CMS and device manufacturers on how to resolve any evidence gaps. Providing adequate resources for FDA's and CMS' respective workforces will also be important to help foster this alignment.

CMS has indicated that its forthcoming TCET proposal will build on prior CMS initiatives, such as coverage with evidence development (CED). CMS covers promising therapeutics and services for Medicare beneficiaries under CED on the condition that they are furnished in a setting of ongoing data collection. Although this mechanism has been around for nearly two decades, it lacks transparency in how it is applied, and CMS can require ongoing evidence collection without any specified time frame for retiring these requirements. It is extraordinarily difficult to retire CED requirements.[xii] As a result, CED has created barriers that prevent wider access to medical breakthroughs, limiting data for research and ultimately hampering innovation.[xiii] Thus, PMC is concerned that if CMS requires CED for breakthrough devices because limited initial evidence is available, CED could diminish the potential for the TCET pathway to improve patients' access to personalized medicine. To resolve some of the challenges and uncertainties with CED, CMS must include a time limit for any evidence collection requirements.

PMC believes breakthrough devices should also be eligible for coverage under the TCET pathway regardless of whether they fall within a defined benefit category. Some diagnostic and screening tests, like those used for preventive screening, may not fall within a defined Medicare benefit category. In developing a new coverage pathway within its existing authorities, we recognize that CMS faces statutory constraints in providing coverage for breakthrough devices that do not fall within a defined Medicare benefit category. For this reason, we have previously supported the inclusion of a provision in legislation introduced in the previous Congress that would codify a transitional coverage and payment pathway for breakthrough devices under the Medicare program, including for "specified" breakthrough devices that do not fall into a defined Medicare benefit category.

Ensuring the Accelerated Approval Pathway Continues to Benefit Patients

To realize the benefits of rapid advances in science and facilitate patients' access to new treatments in a timely manner, the regulatory approval processes in the United States have evolved. As one of four expedited pathways available at FDA, the Accelerated Approval Program allows FDA to approve certain drugs that treat serious or life-threatening diseases and offer meaningful therapeutic benefit to patients over existing treatments before confirmatory trials are completed. Between 2011 and 2017, the majority of newly approved drugs were associated with at least one expedited FDA review pathway.[xiv] With the increasing identification of new molecular drug targets, the use of these pathways in personalized medicine is expected to grow. As of March 2023, FDA had approved 295 products under accelerated approval, including numerous drugs for patients with cancers and rare conditions like sickle cell disease or Duchenne muscular dystrophy.[xv] Dozens of these treatments have been personalized medicines.

Accelerated approval is critical for providing patients with access to new safe and effective drugs that fill unmet medical needs. Since its inception in 1992, millions of patients with serious or life-threatening illnesses have received faster access to new drugs and better outcomes under the program.[xvi] For patients with progressive diseases and unmet medical needs, time is of the essence. In cancer, accelerated approval can give patients access to new treatments around four years faster on average and as much as 12 years faster for certain drugs.[xvii] With more than 90 percent of rare diseases lacking an FDA-approved treatment, timelier access to novel treatments can offer meaningful advantages in treating or managing a patient's disease.

To gain approval in this pathway, a drug must still meet FDA's standards for safety and efficacy. Accelerated approval simply permits FDA to accept a different type of data when deciding that a drug's benefits outweighs its risks. While traditional approval relies on a direct demonstration of clinical benefit, accelerated approval relies on surrogate or intermediate clinical endpoints that are expected to predict clinical benefit and can be measured earlier in shorter, smaller clinical trials. Under accelerated approval, FDA accepts a level of uncertainty that the surrogate endpoint will not be confirmed to be predictive of clinical benefit. In order to resolve those uncertainties, FDA requires drug companies to conduct confirmatory studies after approval. Validated surrogate endpoints and post-marketing requirements can also be associated with drugs that received traditional approval, so these features are not unique to the accelerated approval pathway.

Designing, enrolling, and completing post-marketing studies can be very complex. Limited understandings of a disease, small numbers of patients, and a lack of regulatory precedent can also create challenges in evaluating the long-term clinical benefit of new drugs. Although the accelerated approval pathway has been criticized due to delays in the completion of confirmatory studies and a failure to complete some studies by sponsors, the pathway has been largely successful in bringing to market treatments where the expected clinical benefit of the drug was confirmed. One analysis has estimated that 33 percent to 66 percent of products approved under the Accelerated Approval Program may not have come to market or been developed at all without the flexibilities available through this pathway.[xviii]

Earlier this year, in response to President Biden's executive order on lowering prescription drug costs, the CMS Innovation Center (CMMI) announced it would consider for testing a new Accelerating Clinical Evidence Model. This mandatory model would aim to incentivize manufacturers to complete confirmatory trials for their drugs approved under the Accelerated Approval Program by adjusting payments to providers. In 2022, Congress also granted new authorities to FDA to ensure that sponsors of accelerated approval drugs are complying with post-approval requirements, and the effect of these authorities in resolving concerns with the completion of confirmatory studies has yet to be realized. Coupled with the new drug price negotiation program, the Accelerating Clinical Evidence Model could further force manufacturers to limit research into expanding drugs' indications for unmet medical needs and shift investment away from disease areas where treatments are more difficult to develop under traditional approval.

Accelerated approval offers a vital approach for advancing treatments for patients with diseases that stand to benefit from advances in personalized medicine. We encourage Congress to protect this pathway to ensure that personalized medicines can be reviewed in a flexible and timely manner by FDA and provide greater opportunities for novel drug development to benefit patients.

Conclusion

PMC appreciates the opportunity to discuss the potential impacts of government policies on innovation and patient access to personalized medicine. We look forward to working with Congress on policies that improve the health care system and bring us closer to a future in which every patient has access to and benefits from an individualized approach to health care.

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[xviii] Vital Transformation. *Preliminary Findings: Calculating the Value and Impact of Accelerated Approvals.* June 15, 2022. https://vitaltransformation.com/wp-content/uploads/2022/06/AA-Project_BIO_Deck_v9.pdf. (Accessed May 22, 2023.)

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Statement Submitted for the Record
U.S. House Committee on Ways & Means Committee
Subcommittee on Health
Hearing: "Examining Policies that Inhibit Innovation and Patient Access"
May 10, 2023

Jon Bloom, M.D.
Chief Executive Officer, Podimetrics

Chairman Smith, Ranking Member Neal, and members of the House Ways & Means Committee Subcommittee on Health, Podimetrics thanks you for the opportunity to submit a statement for the record on fostering innovation and patient access to care. We commend your leadership and strongly support bipartisan efforts to encourage innovation in healthcare and facilitate patient access to novel treatments and medical technologies so that all Americans can live longer and healthier lives.

Based in Somerville, Massachusetts, Podimetrics is a tech-enabled services company dedicated to ending the diabetes-related amputation pandemic. Through early detection of diabetic foot ulcers (DFUs), Podimetrics' technology has enabled healthcare providers to demonstrate a reduction of unnecessary diabetes-related amputations. Increasing data from partnerships Podimetrics has with the Veterans Health Administration (VHA) and some commercial health plans suggest that high percentages of at-risk patients have avoided diabetes-related amputation and averted significant health care spending with use of Podimetrics' SmartMat technology.[1] [2] [3]

Diabetes-related amputation is one of the most costly and debilitating complications of the disease – and one that disproportionately impacts lower income individuals from racial and ethnic minorities often residing in rural communities across the country. Direct medical costs for diabetes care in the U.S. totaled approximately $237 billion in 2017 and about one-third of those costs were attributed to diabetic foot disease and associated lower extremity amputation.[4] [5] Patients undergoing diabetes-related amputation have higher five-year mortality rates than patients with coronary artery disease, breast cancer, and colorectal cancer.[6] **According to the American Diabetes Association (ADA), more than 154,000 diabetes-related amputations occur annually – a 75 percent increase over the past decade – and up to 85 percent of those amputations are preventable but happen due to patient challenges accessing high-quality care.**[7] The significant increase in the number of Americans suffering from diabetes-related amputation has led the ADA to identify a "diabetes-related amputation pandemic" across the U.S.

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[1] VA News. How Innovation and Partnership are Ending Diabetic Limb Loss at VA. Accessed October 11, 2022.

[2] Isaac et al. Lower resource utilization for patients with healed diabetic foot ulcers during participation in a prevention program with foot temperature monitoring. *BMJ Open Diab Research & Care*. 2020.

[3] Frykberg et al. Feasibility and Efficacy of Smart Mat Technology to Predict Development of Diabetic Plantar Ulcers. *Diabetes Care.* 2017 Jul;40(7):973-980. DOI: 10.2337/dc16-2294

[4] CDC. By the Numbers: Diabetes in America. Accessed January 8, 2023.

[5] Hicks et al. "Burden of Infected Diabetic Foot Ulcers on Hospital Admissions and Costs." *Ann Vasc Surg*. 2016 May; 33: 149 – 158. 2016 Feb. 22. doi: 10.1016/j.avsg.2015.11.025

[6] American Diabetes Association. Amputation Prevention Alliance. Accessed January 8, 2023.

[7] *Ibid*.

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Innovative medical technologies and treatments can prevent many of the diabetes-related amputations from occurring today. Consequently, Podimetrics strongly supports and encourages bipartisan efforts from this Subcommittee and the Congress to facilitate coverage and improved patient access for novel technologies and therapies that can detect and prevent diabetic-related foot ulcers and associated amputations in federal health programs. As part of this work, we particularly urge the establishment of a clear pathway for Medicare coverage and reimbursement of innovative medical technologies and technology-enabled services given that one does not exist today so that patients can benefit from access to novel platforms that can prevent diabetes-related foot ulcers and associated amputations.

I. Diabetic Foot Ulcers and Associated Amputations Are Highly Debilitating for Patients and Extremely Expensive to Treat

Approximately 37.3 million U.S. adults currently have diabetes.[8] Diabetic foot ulcers (DFUs) and associated amputations are among the most debilitating and expensive complications of the disease. Published research has found, for example:

- **Patients with diabetes-related amputation have a significantly higher risk of mortality – experiencing higher five-year mortality rates than patients with coronary artery disease, breast cancer, and colorectal cancer.** [9] One study found that the five-year mortality rate was very high among patients with any amputation (major and minor combined), ranging from 53 percent to 100 percent, and in patients with major amputations, ranging from 52 percent to 80 percent. [10] A separate analysis determined that patients undergoing major and minor diabetes-related amputations suffer mortality rates of 13.62 percent, 30.25 percent, and 50.55 percent at one-, three-, and five-year intervals, respectively. [11]

- **Diabetes-related amputation severely limits a patient's ability to meaningfully engage in routine daily acts of living.** Patients with diabetes-related amputation are much more likely to experience moderate to severe dysfunction in activities of daily living, such as walking capability.[12] Patients with diabetes-related amputation also are at increased risk for undergoing re-ulceration and re-amputation, typically resulting in further functional limitations and challenges in activities of daily living. [13] [14]

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[8] Centers for Disease Control and Prevention. By the Numbers: Diabetes in America. Accessed January 8, 2023.

[9] American Diabetes Association. Amputation Prevention Alliance. Accessed January 8, 2023.

[10] Thorud et al. "Mortality After Nontraumatic Major Amputation Among Patients with Diabetes and Peripheral Vascular Disease: A Systematic Review." *J Foot Ankle Surgery* 2016 May-Jun; 55(3):591-9. Doi: 10.1053/j.jfas.2016.01.012

[11] Rathnayake A, Saboo A, Malabu U, and Falhammar H. Lower extremity amputations and long-term outcomes in diabetic foot ulcers: A systematic review. *World J Diabetes.* 2020 Sep 15; 11(9): 391-399. Doi: 10.4239/wjd.v11.i9,391

[12] Rathnayake A, Saboo A, Malabu U, and Falhammar H. Lower extremity amputations and long-term outcomes in diabetic foot ulcers: A systematic review. *World J Diabetes.* 2020 Sep 15; 11(9): 391-399. Doi: 10.4239/wjd.v11.i9,391

[13] *Ibid*.

[14] Frykberg et al. "Feasibility and Efficacy of the Smart Mat Technology to Predict Development of Diabetes Plantar Ulcers." *Diabetes Care.* 2017;40(7):973-980. https://doi.org/10.2337/dc16-2294

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- **The cost of a single amputation resulting from, or related to, a diabetic foot ulcer can be more than $100,000 and the mean cost from a healthcare public payer perspective is $44,200.**[15] [16] [17] The extremely high spending on treating diabetic foot ulcers and associated amputations drives a significant amount of the total U.S. healthcare spending on diabetes. Direct medical costs for diabetes care in the U.S. totaled approximately $237 billion in 2017 and about one-third of those costs were attributed to diabetic foot disease and associated lower extremity amputation.[18] [19]

II. Low-Income Individuals from Racial and Ethnic Minorities, More Often in Rural Areas, Are the Most Likely to Suffer a Diabetes-Related Amputation

Published research demonstrates that individuals from historically underserved communities are the most likely to suffer from diabetic foot ulcers and undergo diabetes-related amputation. Specifically:

- **Low-income individuals:** Across the U.S., adults with a family income below the federal poverty level have the highest prevalence rate of diabetes according to the CDC, with prevalence rates of 14.1 percent (less than 100% FPL), 10.8 percent (100% - 299% FPL), 7.8 percent (300% - 499% FPL), and 5.6% (500% FPL or more).[20] Moreover, prevalence rates between low-income and non-low-income populations in the U.S. generally have widened further over the past decade.[21] Specifically with respect to diabetes-related amputations, data show that patients in the lowest income quartile regions of the U.S. have a 38.5 percent higher odds of undergoing a major diabetic amputation compared to the highest income regions of the country.[22] Reflecting this trend across the U.S., a separate study published in *Health Affairs* found that residents in poorer neighborhoods of Los Angeles County, California were twice as likely to have a major amputation as those in wealthier neighborhoods of the County.[23]

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[15] Gorman, Anna. Diabetic Amputations A 'Shameful Metric' Of Inadequate Care. California Healthline. May 1, 2019.

[16] Hicks et al. "Burden of Infected Diabetic Foot Ulcers on Hospital Admissions and Costs." *Ann Vasc Surg*. 2016 May; 33: 149 – 158. 2016 Feb. 22. doi: 10.1016/j.avsg.2015.11.025

[17] Frykberg et al. Feasibility and Efficacy of Smart Mat Technology to Predict Development of Diabetic Plantar Ulcers. *Diabetes Care.* 2017 Jul;40(7):973-980. DOI: 10.2337/dc16-2294

[18] CDC. By the Numbers: Diabetes in America. Accessed January 8, 2023.

[19] Hicks et al. "Burden of Infected Diabetic Foot Ulcers on Hospital Admissions and Costs." *Ann Vasc Surg*. 2016 May; 33: 149 – 158. 2016 Feb. 22. doi: 10.1016/j.avsg.2015.11.025

[20] CDC. By the Numbers: Diabetes in America. Accessed January 8, 2023.

[21] American Diabetes Association. "Income-Related Inequalities in Diabetes Have Widened Over the Past Decade, CDC Study Finds." June 28, 2021.

[22] Skrepnek G, Mills J, Armstrong D. A Diabetic Emergency One Million Fee Long: Disparities and Burdens of Illness among Diabetic Foot Ulcer Cases within Emergency Departments in the United States, 2006-2010. *PLOS ONE.* August 6, 2015. Doi.org/10.1371/journal.pone.0134914

[23] Stevens C, Schriger D, Raffetto B, Davis A, Zingmond D, and Roby D. Geographic Clustering Of Diabetic Lower-Extremeity Amputations In Low-Income Regions of California. *Health Affairs*. August 2014. https://doi.org/10.1377/hlthaff.2014.0148

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- **Rural residents:** Diabetes prevalence is 17 percent higher in rural areas of the U.S. compared to urban areas.[24] Particularly in the "diabetes belt" that covers much of the southeastern U.S., the CDC estimates that 11.7 percent of the population has diabetes compared to 8.5 percent of people living in other parts of the country.[25] [26] Reflecting the broader trend of disproportionate diabetes disease burden in rural communities, one study concluded people living in rural areas in the U.S. have approximately 35 percent higher odds of undergoing diabetes-related major amputations compared to those residing in urban areas.[27] A separate study found that individuals with diabetic foot ulcers residing in rural areas across the U.S. were associated with a 51.3 percent greater odds of suffering major amputation and 41.4 percent higher odds of inpatient death.[28]

- **Blacks across the U.S.:** Blacks have a significantly higher overall diabetes prevalence rate than Whites in the U.S.: 12.1 percent versus 7.4 percent.[29] Critically, Blacks are up to four times more likely to experience a diabetes-related amputation than Whites.[30] In the Medicare population specifically, the amputation rate among Black Medicare beneficiaries —5.6 per 1,000—was nearly three times higher than the rate among other beneficiaries (2.0) during the period of 2007 to 2011, according to Dartmouth Atlas research.[31]

- **Individuals who identify as Black and rural:** Individuals who identify as both Black and rural are at especially high risk for diabetic foot ulcers and undergoing diabetes-related amputations. One study determined that Black Medicare beneficiaries living in rural areas have a 28 percent risk of undergoing a major leg amputation or death after hospital admission for a diabetic foot ulcer compared to 17.6 percent of the overall national cohort – a relative difference of more than 50 percent.[32] Similarly, Dartmouth Atlas data show that the diabetes-related amputation rate varied by a factor of more than seven among black Medicare beneficiaries in different geographic regions of the country, ranging from about 2 per 1,000 in San Diego (2.1) and Las

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[24] Bolin J and Ferdinand A. "The Burden of Diabetes in Rural America." Rural Health Research Gateway. March 2018.

[25] CDC. Appalachian Diabetes Control and Translation Project. Accessed January 9, 2023.

[26] CDC. CDC identifies diabetes belt. March 7, 2015.

[27] Brennan M, Powell W, Kaiksow F. Association of Race, Ethnicity, and Rurality With Major Leg Amputation or Death Among Medicare Beneficiaries Hospitalized With Diabetic Food Ulcers. *JAMA Netw Open.* 2022;5(4):e228399. doi:10.1001/jamanetworkopen.2022.8399

[28] Skrepnek G, Mills J, Armstrong D. A Diabetic Emergency One Million Fee Long: Disparities and Burdens of Illness among Diabetic Foot Ulcer Cases within Emergency Departments in the United States, 2006-2010. *PLOS ONE.* August 6, 2015. Doi.org/10.1371/journal.pone.0134914

[29] CDC. By the Numbers: Diabetes in America. Accessed January 8, 2023.

[30] American Diabetes Association. "Amputation Prevention Alliance." Accessed October 10, 2022.

[31] Goodney et al. "Variation in the Care of Surgical Conditions: Diabetes and Peripheral Artery Disease." A Dartmouth Atlas of Healthcare Series.

[32] Brennan M, Powell W, Kaiksow F. Association of Race, Ethnicity, and Rurality With Major Leg Amputation or Death Among Medicare Beneficiaries Hospitalized With Diabetic Foot Ulcers. *JAMA Netw Open.* 2022;5(4):e228399. doi:10.1001/jamanetworkopen.2022.8399

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Vegas (2.2) to 14 or more per 1,000 in Lynchburg, Virginia (14.0), Meridian, Mississippi (14.2), and Tupelo, Mississippi (16.1).[33]

- **Hispanic Individuals:** Hispanic individuals are more than twice as likely as non-Hispanic Whites to develop Type 2 diabetes: 17 percent versus 8 percent, respectively.[34] Reflecting this disparity, Hispanic individuals are 1.5 times more likely to experience a diabetes-related amputation than Whites.[35] In one of the largest states in the nation, California, Kaiser data shows Hispanic and Blacks are more than twice as likely to undergo diabetes-related amputations compared to Whites in the State.[36]

- **American Indians and Native Alaskans:** Native Americans have a greater chance of developing diabetes than any other racial group and are at higher risk for undergoing a diabetes-related amputation.[37] Specifically, data show that American Indian / Native Alaskan Medicare beneficiaries have a 1.8 to 1.9 times higher risk of major diabetes-related amputation than Whites.[38]

In sum, widely published research plainly demonstrates that individuals from underserved communities are most likely to contract diabetes and undergo diabetes-related amputations. Those who are low-income individuals from racial and ethnic minorities, often living in rural areas across the U.S., are at disproportionately higher risk of DFU and therefore are at disproportionately higher risk for suffering functional limitations and mortality due to their increased likelihood of undergoing a diabetes-related amputation.

III. Patient Access to Innovative Technology Can Meaningfully Help Prevent Diabetic Foot Ulcers and Diabetes-Related Amputations

Data from the Veterans Health Administration and commercial health plans suggests that patient access to and use of Podimetrics' innovative temperature-sensing SmartMat technology may detect early signs of and reduce the risk of diabetic foot ulcers and diabetes-related amputations, including the following:[39] [40] [41]

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[33] Goodney et al. "Variation in the Care of Surgical Conditions: Diabetes and Peripheral Artery Disease." A Dartmouth Atlas of Healthcare Series.

[34] CDC. Hispanic or Latino People and Type 2 Diabetes. Accessed January 9, 2023.

[35] American Diabetes Association. Amputation Prevention Alliance. Accessed January 8, 2023.

[36] Stevens C, Schriger D, Raffetto B, Davis A, Zingmond D, and Roby D. Geographic Clustering Of Diabetic Lower-Extremeity Amputations In Low-Income Regions of California. *Health Affairs*. August 2014. https://doi.org/10.1377/hlthaff.2014.0148

[37] CDC. Native Americans with Diabetes. Accessed January 9, 2023.

[38] American Diabetes Association. Amputation Prevention Alliance. Accessed January 8, 2023.

[39] VA News. How Innovation and Partnership are Ending Diabetic Limb Loss at VA. Accessed October 11, 2022.

[40] Frykberg et al. "Feasibility and Efficacy of the Smart Mat Technology to Predict Development of Diabetes Plantar Ulcers." *Diabetes Care*. 2017;40(7):973-980. https://doi.org/10.2337/dc16-2294

[41] Isaac et al. Lower resource utilization for patients with healed diabetic foot ulcers during participation in a prevention program with foot temperature monitoring. *BMJ Open Diab Research & Care*. 2020.

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- **Early diabetic foot ulcer detection:** 97 percent of non-acute plantar diabetic foot ulcers not caused by acute injury (such as burn or laceration) were detected on average over 5 weeks (37 days) before wound clinical presentation.[42]

- **Marked lower extremity amputation reduction**: 71 percent of all amputations were eliminated.[43]

- **Moderate and severe ulcer reduction:** Patients experienced a 91 percent relative risk reduction in moderate and severe ulcers.[44]

- **Substantial cost savings:** A health plan achieved an estimated $12,000 in savings using the SmartMat relative to the same cohort one year prior. Savings were estimated from reduced rates of hospitalization, ED visits, and outpatient visits, as detailed below.[45]

- **All-cause hospitalization reduction:** There was a 52 percent relative risk reduction in all-cause hospitalizations.[46]

- **ED visit reduction:** There was a 40 percent relative risk reduction in ED visits.[47]

- **Outpatient visit reduction:** There was a 26 percent relative risk reduction in outpatient visits.[48]

These data are consistent with three prior randomized controlled trials funded by the National Institutes of Health (NIH) demonstrating the use of temperature monitoring to reduce the incidence of diabetic foot ulceration – the leading cause of diabetic amputation – by approximately 70 percent in high risk populations.[49] [50] [51] As a result, the use of temperature to predict and prevent diabetic foot

[42] Frykberg et al. "Feasibility and Efficacy of the Smart Mat Technology to Predict Development of Diabetes Plantar Ulcers." *Diabetes Care.* 2017;40(7):973-980. https://doi.org/10.2337/dc16-2294
[43] Isaac et al. Lower resource utilization for patients with healed diabetic foot ulcers during participation in a prevention program with foot temperature monitoring. *BMJ Open Diab Research & Care*. 2020.
[44] *Ibid*.
[45] *Ibid*.
[46] *Ibid*.
[47] *Ibid*.
[48] *Ibid*.
[49] Lavery LA, Higgins KR, Lanctot DR, et al. Home monitoring of foot skin temperatures to prevent ulceration. Diabetes Care 2004;27:2642–7.
[50] Frykberg et al. "Feasibility and Efficacy of the Smart Mat Technology to Predict Development of Diabetes Plantar Ulcers." *Diabetes Care.* 2017;40(7):973-980. https://doi.org/10.2337/dc16-2294
[51] Lavery LA, Higgins KR, Lanctot DR, et al. Preventing diabetic foot ulcer recurrence in high-risk patients: use of temperature monitoring as a self-assessment tool. Diabetes Care 2007;30:14–20.

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complications is supported by three clinical practice guidelines[52] [53] [54] as well as a comparative effectiveness report by the Agency for Healthcare Research and Quality.[55]

In sum, the partnerships between Podimetrics and the VA and commercial health plans as well as peer-reviewed, NIH-funded clinical trials suggest that patient access to innovative new medical technology may lead to earlier detection of diabetic foot ulcers, and as a result, may prevent occurrence of diabetes-related amputation. Thus, by facilitating access to innovative prevention and treatment technologies services like the Smart Mat, diabetes patients at risk for developing diabetic foot ulcers and undergoing associated amputation may have meaningful improvements in quality of life and lower healthcare costs.

IV. Policy Recommendations

The American Diabetes Association maintains that up to 85 percent of the more than 154,000 diabetes-related amputations that occur annually – an increase of 75 percent over the past decade – are preventable but result from patient challenges accessing high-quality care.[56] Therefore, enrollees in federal health programs should have access to high-quality prevention and treatment for diabetic foot ulcers and associated amputations, which will lead to improved health outcomes, lower spending, and significant advancements in health equity. Specifically:

1. **Medicare Pathway for Coverage and Reimbursement of Innovative Medical Technology and Technology-Enabled Services:** No separate pathway exists today for Medicare coverage of innovative technologies and devices. The lack of a clearly established pathway has led to delays and barriers in patient access to novel medical treatments and technologies, which can prolong their lives and improve their overall health outcomes. The Centers for Medicare and Medicaid Services (CMS) intends to publish a notice soon that would establish expedited Medicare coverage for innovative technologies and devices called Transitional Coverage for Emerging Technologies (TCET). The Subcommittee and Congress should work with CMS to ensure that TCET and other potential clearly defined policies provide expedited Medicare coverage and reimbursement of innovative medical technologies and technology-enabled services with sound evidence of clinical benefit so that patients can access these treatments without barriers and delays.

2. **CMS Diabetes Prevention and Treatment Services Strategy:** The National Strategy on Hunger, Nutrition, and Health states that the CMS intends to develop a strategy to expand access to

[52] Frykberg RG, Zgonis T, Armstrong DG, et al. Diabetic foot disorders. A clinical practice guideline (2006 revision). J Foot Ankle Surg 2006;45:S1–66.
[53] Lavery LA, Davis KE, Berriman SJ, et al. WHS guidelines update: diabetic foot ulcer treatment guidelines. Wound Repair Regen 2016;24:112–26.
[54] Bus SA, Lavery LA, Monteiro-Soares M, et al. Guidelines on the prevention of foot ulcers in persons with diabetes (IWGDF 2019 update). Diabetes Metab Res Rev 2020;36 Suppl 1:e3269.
[55] Sydney M Dy et al., "Preventing Complications and Treating Symptoms of Diabetic Peripheral Neuropathy," *Comparative Effectiveness Review,* No. 187, (2017), Agency for Healthcare Research and Quality. https://www.ncbi.nlm.nih.gov/books/NBK442335/, accessed November 2021
[56] American Diabetes Association. Amputation Prevention Alliance. Accessed January 8, 2023.

diabetes prevention and treatment services for individuals enrolled in Medicare, Medicaid, Children's Health Insurance Program (CHIP), and Marketplace plans.[57] As part of this comprehensive strategy, the Subcommittee and Congress should encourage CMS to include innovative prevention and treatment services and technologies specifically targeting individuals at risk for diabetic foot ulcers and associated amputations in each of these federal health programs.

3. **Medicare and Medicaid Quality Measurement:** Currently, two Medicare Merit-based Incentive Payment System (MIPS) quality measures address diabetes-related foot care, but do *not* assess prevention of diabetic foot ulcers and associated amputation. In Medicare Advantage, the Health Effectiveness Data and Information Set (HEDIS) Comprehensive Diabetes Care measure does not evaluate foot care. Similarly, the 2023 and 2024 Core Set of Adult Health Care Quality Measures for Medicaid includes four diabetes-related measures, but none that address quality of care related to diabetic foot ulcers and amputations. Therefore, the Subcommittee and the Congress should encourage CMS to work with stakeholders to develop new quality measures that specifically assess effective preventive care and treatment for diabetic foot ulcers and amputations.

4. **Essential Health Benefits (EHBs) in Qualified Health Plans in the Marketplaces:** In a recent Request for Information (RFI), CMS indicated it is considering updating the EHB to account for scientific advancements and innovations in medicine that have occurred since the agency initially established the EHB standards. The Subcommittee Congress should encourage CMS to update the EHB to cover diabetes prevention and treatment services, including innovative technologies targeting individuals at risk for diabetic foot ulcers and associated amputations.

Conclusion

In conclusion, Podimetrics again wishes to commend you for your leadership in aiming to foster innovation in healthcare and facilitate patient access to innovative treatments and technologies that save lives and improve health outcomes. The strong bipartisan efforts of this Subcommittee and Congress will ensure that more Americans can live longer and healthier lives with improved access to novel medical therapies and technologies.

[57] Biden-Harris Administration National Strategy on Hunger, Nutrition, and Health, page 18. September 2022.

May 17, 2023

House Committee on Ways and Means Subcommittee on Health
Chairman Vern Buchanan
Ranking Member Lloyd Doggett
1100 Longworth House Office Building
Washington, DC 20510
WMSubmission@mail.house.gov

Dear Chairman Buchanan and Ranking Member Doggett:

Renalis commends the work of the House Committee on Ways and Means Subcommittee on Health for examining our nation's crisis with barriers to innovation, and appreciates the consideration of our input.

Introduction to Renalis

Renalis is a Cleveland-based company committed to developing pelvic health platforms to improve effectiveness and efficiency of patient and provider interactions, optimize patient outcomes, and decrease healthcare costs.

Renalis' first commercial platform will be an FDA-approved prescription digital therapeutic for Overactive Bladder (OAB) in women. Of the 33 million adult Americans suffering from some form of urinary incontinence, 75% to 80% of those are women. And about 23% of these women are over 60. In the future, the company plans to launch therapeutics for stress incontinence, bowel dysfunction, and chronic pelvic pain as well as, when applicable, will target solutions for all persons with a pelvis.

Digital Therapeutics that are Prescription Digital Therapeutics (PDTs)

To leverage the important advantages of digital therapeutics (DTx), the federal government must establish a structure that enables patients and clinicians to identify genuine DTx products, ensures reliable access to these products, and provides actuarially sound reimbursements for DTx prescription products and the clinicians responsible for authorizing and/or utilizing these beyond the pill therapeutics.

Prescription digital therapeutics (PDTs) are a new class of evidence-based medical treatments that utilize software to improve patient outcomes. These innovative therapies can help patients manage chronic conditions by delivering personalized interventions through mobile applications or other digital devices wherever that patient is located. PDTs provide beneficiaries access to high-quality care for a diverse set of conditions, including PTSD, ADHD, substance use and opioid use disorder, chronic back pain, diabetes, pediatric amblyopia, women's pelvic health, and more.

Two Policy Examples

1. **Lack of clarity around regulatory approval disincentives innovation**.
    - One policy that inhibits innovation in the PDT industry is the lack of clarity around regulatory approval. The FDA has not yet established a clear framework for the approval and regulation of PDTs, which creates uncertainty and slows down the development process. the reimbursement landscape through the Centers for Medicare and Medicaid Services (CMS) for PDTs is unclear, which further disincentivizes companies from investing in research and development.
    - Additionally state regulations may vary, which further complicates the development, approval, and distribution of PDTs. Some states may require additional licensing or regulatory approvals for these therapies, while others may not. This creates an inconsistent regulatory environment that can be challenging for companies to navigate.
2. **Lack of insurance coverage for PDT treatments**
    - Another policy that inhibits patient access to PDTs is the lack of insurance coverage for these treatments. Medicare and Medicaid do not currently cover reimbursement of PDTs, even though they may be more cost-effective than traditional treatments to the healthcare system. This creates a barrier to access for patients who may benefit from these treatments but cannot afford cash pay out of pocket.
    - The lack of clarity around reimbursement for PDTs can create a significant barrier to patient access, as many patients may not be able to afford the cost of these treatments out of pocket. This can limit the ability of PDTs to reach their full potential in improving patient outcomes and reducing healthcare costs.

Renalis' Recommendations to the Committee

To address these policy barriers, stakeholders including regulators, insurance providers, and policymakers must work together to create a regulatory framework that incentivizes innovation and ensures patient access to PDTs. This can include developing clear guidelines for regulatory approval, establishing reimbursement policies that incentivize the use of PDTs, and promoting consistent state regulations to support the growth of this emerging field.

Renalis welcomes the opportunity to discuss these recommendations in further detail. If you have any questions regarding these comments, please do not hesitate to contact me at: (312) 287-1951 or at: missy@renalis.health.

Respectfully submitted,

Renalis

By: /s/ Missy Lavender

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Statement of SPR Therapeutics
Before the Committee on Ways and Means
Subcommittee on Health
U.S. House of Representatives

Submitted for the record at a hearing on: "Medical Innovation and Access to Care"

May 22, 2023

Chairman Buchanan, Ranking Member Doggett, and distinguished members of the Health Subcommittee of the House Committee on Ways & Means, SPR Therapeutics appreciates the Subcommittee's interest in addressing challenges associated with patient access to innovative medical technologies.

SPR's mission is to improve the quality of patients' lives by providing them with a minimally invasive, non-opioid solution to manage acute and chronic pain. Our SPRINT® peripheral nerve stimulator (PNS) system has been FDA-cleared since 2016 and provides a 60-day implant designed to deliver substantial and sustained non-opioid pain relief without the need for nerve destruction, or a permanently implanted neurostimulator. Specifically, the SPRINT PNS system is indicated for implant periods of up to 60 days for symptomatic relief of chronic, intractable pain, post-surgical and post-traumatic acute pain; symptomatic relief of post-traumatic pain; and symptomatic relief of post-operative pain.[1]

SPR commends your efforts to confront obstacles that impede medical innovation and limit patient access to medically necessary innovative medical technologies.

Medicare Coverage Policies Restrict Utilization of Innovative Treatments for Pain Management

SPR is committed to offering providers and patients an innovative, safe and effective opioid alternative for pain management. However, there are several barriers that impede patient access to minimally invasive innovative pain relief treatment. Medicare Administrative Contractor (MAC) Local Coverage Determinations (LCDs) that are more restrictive than Medicare National Coverage Determinations (NCDs) impose significant barriers to patient access to innovative medical treatments, such as the SPRINT PNS system. For example, Noridian LCDs for Peripheral Nerve Stimulation (LCDs JE-L34328 and JF-L37360) incorporate coverage requirements well in excess of NCD 160.7 – Electrical Nerve Stimulators, such as requiring post-payment reviews for clinicians with low trial to permanent implant ratios. Such a policy assumes that temporary implantation is solely for evaluating permanent implantation efficacy, disregarding FDA-cleared PNS treatment technologies, such as the SPRINT PNS System, that offer sustained pain relief without the need for permanent implantation in many cases.

This post-payment review policy causes confusion and deters physicians from using PNS treatments like SPRINT due to concerns about denials and claw-backs. In other words, restrictive policies like these limit

[1] SPRINT is not indicated for the treatment of pain in the region innervated by the cranial and facial nerves.

access to Medicare beneficiaries and overlook studies showing prolonged pain relief from temporary PNS treatments. Accordingly, we urge Congress to take a broader look at MAC LCDs that further limit coverage beyond existing associated Medicare NCDs.

We also note that Medicare coverage policies regarding peripheral nerve stimulation only contemplate treatment for chronic, intractable pain. We believe there is now ample evidence to conclude that coverage under these policies should be extended to other FDA-cleared indications, such as acute post-operative pain. This narrow approach hinders progress in pain management and discourages providers from utilizing innovative non-opioid methods to treat post-acute pain.

Accordingly, we urge Congress to expand Medicare coverage for peripheral nerve stimulation technologies to include treatment for acute post-operative pain. By doing so, we can prioritize patient-centered care, reduce reliance on opioids, and promote effective and innovative pain management treatments for our patient communities.

Payer Prior Authorization Practices and Vague Coverage Criteria Inhibit Patient Access to Innovative Technologies

Undefined "experimental and investigational" criteria, along with “no prior authorization required” policies pose significant barriers to medically appropriate, minimally invasive, and non-opioid pain management services. It is our understanding that services are commonly denied because the service is deemed "experimental or investigational," without clear evidence-based criteria for how such determinations are made. This failure to cover has been especially egregious and increasingly observed among those insured by Anthem Medicare Advantage programs where more than 43% of the beneficiaries we have been able to track have been denied access to this treatment. Use of the "experimental or investigational" rationale is not rational. Recent data published in a highly regarded peer-reviewed journal demonstrate outcomes in pain management across myriad peripheral nerve targets and in more than 6,000 patients using this technology.[2] The impact is that patients are often deprived of innovative and promising medical technologies, while clinicians are left chained with fewer options to effectively manage their patients’ pain, often resorting to more costly and invasive treatments with suboptimal care outcomes.

Furthermore, commercial payers have commonly been influenced by Medicare coverage, and the current boldness demonstrated by Anthem and others potentially delays commercial coverage for multitudes of Americans. In other words, insufficient access to innovative medical technologies can lead to higher costs in our health care system, worse outcomes for patients, and burn-out among frustrated clinicians. Understandably, many clinicians, fatigued by the significant administrative burden of assuring coverage, eventually “give-up,” and resort to less effective treatments to avoid these constraining practices. Another concerning practice is the usage of the phrase “no prior authorization required.” This practice is commonplace among commercial payers, and has begun creeping into Medicare Advantage programs. This phrase is particularly dangerous because it misleadingly implies freedom from restrictive prior authorization practices, while actually placing the patient in a state of uncertainty and medical jeopardy. Indeed, in these situations, patients are left without any indication of the likelihood that their

[2] Huntoon, Marc A., Konstantin, Slavin V., et. Al. A Retrospective Review of Real-World Outcomes Following 60-Day Peripheral Nerve Stimulation for the Treatment of Chronic Pain. Pain Physician (2023) 26: 273-281. Available at: https://painphysicianjournal.com/current/pdf?article=NzY1OQ%3D%3D

care will be covered by their plan administrator, leaving the patient, their health care provider, and the health care facility to bear significant financial risk, should they advance to treatment and discover that there is no coverage. The majority of these patients continue to suffer, which unfortunately increases the continuance of, or advancement to, the use of opioids to manage pain. The ability of payers to circumvent the risk of non-coverage in this manner must stop.

Accordingly, we urge Congress to consider the impacts of vague payer coverage policies in patient care access. Greater transparency would enable patients to better understand the extent of their coverage, help providers make informed treatment decisions and improve access to innovative treatments for their patient communities, and facilitate improved guidance for manufacturers and product developers necessary to create strategies with clearer coverage pathways.

Further, when requests for prior authorization are denied for these treatments, patients are critically deprived of access to innovative treatments and similar technologies, with limited remedies. Consequently, the prior authorization process can become a mechanism that circumvents patient access to medically reasonable and necessary care. We strongly believe that payers should be required to provide explicit coverage requirements in the specific reason for denial in prior authorization requests, and eliminate the "no prior authorization required" option.

Accordingly, we urge Congress to address payer prior authorization practices to ensure fair and consistent application for patients with diverse care needs and in consideration of the benefits that innovative medical technologies can provide. Addressing these concerns would represent a significant stride towards improving patient access to innovative technologies while promoting fairness, consistency, and transparency in the prior authorization process.

"Independent" Review Organizations

According to the National Association of Independent Review Organizations (NAIRO), an independent review organization (IRO) "acts as a third-party medical review resource which provides objective, unbiased medical determinations that support effective decision making, based only on medical evidence." Unfortunately, our experience with IROs speaks to the contrary, as decisions about provision of care are clearly made without regard for the patient's condition. Rather, IRO decisions are more commonly associated with the payer's policy, which is often cloaked within the auspices and vagaries of "experimental and investigational" language. In an alarming example, 100% of Anthem patients who have appealed their denial for coverage and advanced to review by an independent third party, a "so-called" IRO, Maximus, denied care to 17 of the 17 patients who have taken this approach. We assert that it is extremely unlikely that this entire patient sample, identified as candidates for treatment by a physician specialized in non-opioid pain management, should be denied coverage. Another IRO, MCMC, has also denied 100% of patients seeking an independent opinion. We respectfully appeal to the Ways and Means Subcommittee on Health to conduct a thorough assessment of this seemingly unregulated space.

Accurate Classification of New Technologies is Essential for Ensuring Patient Access to Innovative Pain Management

Patient access to these technologies continues to be impaired by the incorrect classification by insurance payers of medical technologies, thereby leading to service denials and inhibited patient

access. For example, the confusion between Peripheral Nerve Stimulation (PNS), Percutaneous Electrical Nerve Stimulators (PENS), and Peripheral Subcutaneous Field Stimulation (PSFS), creates barriers to patient access due to significant differences in coverage policies for these technologies.

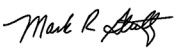
Payer coverage policies often mischaracterize PNS as PENS or PSFS, despite the considerable difference in their applications and supporting literature. PENS involves temporary needle insertion for minutes over multiple in-office treatments, while PSFS utilizes lead implantation to target subcutaneous afferent fibers of unnamed nerves. By contrast, PNS involves the temporary or permanent implantation of leads to target named peripheral nerves affecting the patient's pain, and the robust literature supporting the use of PNS is markedly stronger compared to PENS or PSFS. Critically, PENS and PSFS treatments are generally non-covered by payers, which means that when PNS is misclassified as these treatments, it results in service denials. Some of this confusion has occurred because overly simplistic FDA product classifications may not keep pace with innovation, as in the case above.

For these reasons, we urge Congress to take a closer look at how payers define and classify medically necessary and innovative technologies, to facilitate consistency and enhance patient access to medically appropriate pain management technologies.

Conclusion

On behalf of SPR Therapeutics, we thank you for your dedication to increasing medical innovation and patient access to medical technologies. If you have any questions, please contact me at mstultz@sprintpns.com or via phone at 612.770.0390.

Sincerely,



Mark Stultz, Senior Vice President, Market Development
SPR Therapeutics, Inc.

Comments for the Record
U.S. House of Representatives
Committee on Ways and Means
Subcommittee on Health
Hearing on Examining Policies that Inhibit Innovation and Patient Access
Wednesday, May 10, 2023 at 2:00 PM

Michael Bindner
The Center for Fiscal Equity

Chairman Buchanan and Ranking Member Doggett, thank you for the opportunity to address this issue.

Drug pricing puts too much of the research and innovation processes into the hands of companies whose main goal is profit. On top of this, the new Majority seeks to cut spending below the level of inflation, jeopardizing new research. This must be rethought.

Regardless of where drugs are developed or manufactured, their costs do not vary by where they are sold. Indeed, if a drug is manufactured in the United States, it may have a lower price in other markets - although usually manufacture has shifted to Asia. Prices are another matter. They are dictated by what the market will bear, given the regulatory environment of each market. As long as price is less than the cost, the drug will be sold. Sadly, this puts prices out of reach in the developing world.

PhARMA relies, in part, on claims that negotiation will lead to cost shifting. The dirty little secret in this debate is that single-payer solutions in the rest of the OECD have already resulted in price (not cost) shifting, where the rest of the world shifts its cost to the United States to the greatest extent possible (although they might anyway)

Most people with insurance don't notice this. Single payer healthcare, either through a public option or Medicare for All, will further bury this. For now, allowing drug price negotiation will give drug companies leverage to renegotiate their deals with the rest of the world. As a side note, how Medicare for All or a Public Option might work is explained in an attachment.

PhARMA also relies on the claims that new cures for pandemics and subsidizing the development of orphan drugs and new therapies requires the right to charge the most the market can bear. This ignores the fact that most basic research comes through government grants and contracts, not drug company profits. The latter fund commercial, not scientific, development.

An important part of decreasing cost to consumers is to expand funding, such as the President's ARPA-H proposal. Part of ARPA-H is the funding for research on orphan drugs and the lingering problem of their cost once research leads to product development. In comments to Senate Finance on March 16th of this year, we repeated our proposal in this area for NIH to retain ownership in any such drug and contract out its further development and manufacture. Keeping ownership in public hands ends the need for drug companies to charge extreme prices or increase prices for its existing formulary to fund development.

PhARMA would still make reasonable profit, but the government would eat the risk and sometimes reap the rewards. NIH/FDA might even break even in the long term, especially if large volume drugs which were developed with government grants must pay back a share of basic research costs and the attached profits, as well as regulatory cost.

Hospital consolidation and privatization (i.e., the closing of public hospitals - often at the behest of industry) limits access and drives poor patients to emergency rooms because they are uninsured. It is good of the Republican Majority to get on board in objecting to such bad behavior. It is even worse in some regions, where the only hospital system remaining is run by Catholic religious orders, which limits family planning and abortion access (with the blessing of public law).

When only Catholic hospitals are left in some states, due to consolidation, it makes this policy that more acute. In order for such hospitals to fully serve women, the drama of abortion politics must settle into compromise. There are proposals on both sides for a federal solution - either a federal law banning most abortions or permitting it in all cases. At some points, electoral stunts need to recede and real compromise must be sought.

In both scenarios, the need to take the issue away from the states is obvious. Justice Alito ignored the problems of both slavery and Jim Crow as reasons why there should not be abortion states and anti-abortion states. The respondents relied on the question of rights rather than on the question of powers. Had they examined the competencies of federal and state government on the question of who makes the rules on personhood, the answer is obviously that this responsibility must be federal.

A ruling along those lines would have ended the issue at the status quo - with no regulation of abortion unless Congress recognized the rights of the unborn as reservoirs of positive rights. They are already recognized as having the right to life against government action. It is the same as the right to life for adults - the right to not be executed without due process. It is why we do not execute pregnant women, as well as the right to seek redress for outside injury.

What they cannot claim is a right against the welfare of its mother - especially if the child is doomed due to a fatal defect. In such cases, termination is the only ethical solution - even in Catholic hospitals. Especially if the Catholic hospital is the only hospital for miles around.

For the larger issue, the right to an abortion in the very early stages should be federally guaranteed. After the embryo becomes a fetus - a little person in Latin - then pregnancies should be ended in a live birth, but with no medical intervention required to save the child (other than baptism or other religious blessing). This form of termination should have no upper limit. No one has a right to NOT be born.

Thank you again for the opportunity to add our comments to the debate. Please contact us if we can be of any assistance or contribute direct testimony.

Attachment - Hearing on Pathways to Universal Health Coverage, June 12, 2019

There are three methods to get to single-payer: a public option, Medicare for All and single-payer with an option for cooperative employers.

The first to set up a **public option** and end protections for pre-existing conditions and mandates. The public option would then cover all families who are rejected for either pre-existing conditions or the inability to pay. In essence, this is an expansion of Medicaid to everyone with a pre-existing condition. As such, it would be funded through increased taxation, which will be addressed below. A variation is the expansion of the Uniformed Public Health Service to treat such individuals and their families.

The public option is inherently unstable over the long term. The profit motive will ultimately make the exclusion pool grow until private insurance would no longer be justified, leading-again to Single Payer if the race to cut customers leads to no one left in private insurance who is actually sick. This eventually becomes Medicare for All, but with easier passage and sudden adoption as private health plans are either banned or become bankrupt. Single-payer would then be what occurs when

The second option is Medicare for All, which I described in an attachment to June 18th and 19th's comments and previously in hearings held May 8, 2019 (Finance) and May 8, 2018 (Ways and Means). Medicare for All is essentially Medicaid for All without the smell of welfare and with providers reimbursed at Medicare levels, with the difference funded by tax revenue.

Medicare for All is a really good slogan, at least to mobilize the base. One would think it would attract the support of even the Tea Partiers who held up signs saying, "don't let the government touch my Medicare!" Alas, it has not. This has been a conversation on the left and it has not gotten beyond shouting slogans either. We need to decide what we want and whether it really is Medicare for All. If we want to go to any doctor we wish, pay nothing and have no premiums, then that is not Medicare.

There are essentially two Medicares, a high option and a low one. One option has Part A at no cost (funded by the Hospital Insurance Payroll Tax and part of Obamacare's high unearned income tax as well as the general fund), Medicare Part B, with a 20% copay and a $135 per month premium and Medicare Part D, which has both premiums and copays and is run through private providers. Parts A and B also are contracted out to insurance companies for case management. Much of this is now managed care, as is Medicare Advantage (Part C).

Medicaid lingers in the background and the foreground. It covers the disabled in their first two years (and probably while they are seeking disability and unable to work). It covers non-workers and the working poor (who are too poor for Obamacare) and it covers seniors and the disabled who are confined to a long-term care facility and who have run out their assets. It also has the long-term portion which should be federalized, but for the poor, it takes the form of an HMO, but with no premiums and zero copays.

Obamacare has premiums with income-based supports (one of those facts the Republicans hate) and copays. It may have a high option, like the Federal Employee Health Benefits Program (which also covers Congress) on which it is modeled, a standard option that puts you into an HMO. The HMO drug copays for Obamacare are higher than for Medicare Part C, but the office visit prices are exactly the same.

What does it mean, then, to want Medicare for All? If it means we want everyone who can afford it to get Medicare Advantage Coverage, we already have that. It is Obamacare. The reality is that Senator Sanders wants to reduce Medicare copays and premiums to Medicaid levels and then slowly reduce eligibility levels until everyone is covered. Of course, this will still likely give us HMO coverage for everyone except the very rich, unless he adds a high-option PPO or reimbursable plan.

Either Medicare for All or a real single payer would require a very large payroll tax (and would eliminate the HI tax) or an employer paid subtraction value added tax (so it would not appear on receipts nor would it be zero rated at the border, since there would be no evading it), which we discuss below, because the Health Care Reform debate is ultimately a tax reform debate. Too much money is at stake for it to be otherwise, although we may do just as well to call Obamacare Medicare for All and leave it alone.

The third option is an **exclusion for employers**, especially employee-owned and cooperative firms, who provide medical care directly to their employees without third party insurance, with the employer making HMO-like arrangements with local hospitals and medical practices for inpatient and specialist care.

Employer-based taxes, such as a subtraction VAT or payroll tax, will provide an incentive to avoid these taxes by providing such care. Employers who fund catastrophic care or operate nursing care facilities would get an even higher benefit, with the proviso that any care so provided be superior to the care available through Medicaid or Medicare for All. Making employers responsible for most costs and for all cost savings allows them to use some market power to get lower rates.

This proposal is probably the most promising way to arrest health care costs from their current upward spiral – as employers who would be financially responsible for this care through taxes would have a real incentive to limit spending in a way that individual taxpayers simply do not have the means or incentive to exercise. The employee-ownership must ultimately expand to most of the economy as an alternative to capitalism, which is also unstable as income concentration becomes obvious to all.

Contact Sheet

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Committee on Ways and Means
Subcommittee on Health
Hearing on Examining Policies that Inhibit Innovation and Patient Access
Wednesday, May 10, 2023 at 2:00 PM

All submissions must include a list of all clients, persons and/or organizations on whose behalf the witness appears:

This testimony is not submitted on behalf of any client, person or organization other than the Center itself, which is so far unfunded by any donations.

U.S. Chamber of Commerce

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May 24, 2023

The Honorable Jason Smith
Chair
Committee on Ways and Means
U.S. House of Representatives
Washington, DC 20515

The Honorable Richard Neal
Ranking Member
Committee on Ways and Means
U.S. House of Representatives
Washington, DC 20515

The Honorable Vern Buchanan
Chair
Subcommittee on Health
U.S. House of Representatives
Washington, DC 20515

The Honorable Lloyd Doggett
Ranking Member
Subcommittee on Health
U.S. House of Representatives
Washington, DC 20515

Dear Chairmen Smith and Buchanan and Ranking Members Neal and Doggett:

The U.S. Chamber of Commerce ("Chamber") and its Global Innovation Policy Center ("GIPC") appreciate the opportunity to share this statement for the record regarding your committee's May 10 hearing on policies that would inhibit innovation and patient access.

The Chamber supports efforts to help ensure every American has equitable access to life-saving medicines, from vaccines for COVID-19 to new therapeutics to combat some of the world's most debilitating diseases. However, we are concerned that this Administration and some Members of Congress are pursuing policies that would lead to fewer life-saving drugs and less access to treatments for Americans. Further, we are concerned the Administration is pursuing an agenda that harms life-science innovation, misconstrues the respective roles of public and private funding of science, research, and development, and upends the successful legal frameworks that facilitate public-private partnerships and commercialization.

The Chamber's main concerns with the current anti-innovation policy landscape can be summarized in six main points:

1. Analysis and experience in other countries proves that market-restrictive policies like the Inflation Reduction Act's (IRA's) price controls deter future innovation, inhibit patient access, and limit patient choice;

2. In implementing the IRA, the Administration's interpretations and actions go beyond the statutory text and further exacerbate the law's negative effects;

3. The Administration seeks to further expand harmful, ill-conceived price control policies even before the IRA is fully implemented;

4. Taxpayer funding of R&D is dwarfed by private sector R&D investments.;

5. It is essential to understand how the legal frameworks supporting public-private partnerships, including the bipartisan Bayh-Dole Act of 1980, promote the development and commercialization of lifesaving, cost-effective innovations that benefit millions of Americans. As we cite with the Xtandi example, 99.977% of research costs were borne by the private sector; and

6. Indications are the Administration may advance so-called march-in rights or other forms of forced tech transfers to weaken the statutory intellectual property ("IP") rights of America's innovative companies.

The Chamber's concerns are outlined in more detail below.

I. Market-restrictive policies like the IRA's price controls have a negative impact on innovation that results in restricted access to new, innovative, and life-saving medications by American patients.

In March, the Chamber released its *2023 Patient Access Report (Phase One)* ("The Report"). As GIPC recently explained in a letter to HHS Secretary Xavier Becerra, the Report confirms what proponents of the free market system already know: marketplace competition and effective intellectual property protections give patients greater access to the latest life-saving medicines.[1] In contrast, the Chamber's research shows that market-restrictive policies like artificial price controls deter future innovation, inhibit patient access, and ultimately limit patient choice.

Reducing barriers to access has long been a health policy priority and focus for Congress and the business community. The Chamber supports appropriate, effective efforts to help mitigate and overcome obstacles to life-saving medicines. But government price setting will create additional access challenges for Americans.

The Chamber's Report cautions that the IRA's drug pricing penalties will harm patients by causing them to forfeit early and extensive access to the best life-saving medications. The Report's methodology shows that in other OECD countries which have implemented price controls, patients see fewer overall biopharmaceutical product launches, including biologics and oncology products, and have delayed access to medicines.[2] For example, prior to the enactment of the IRA's price controls, out of 104 new oncology products released globally, 80% were FDA-approved and made available in the U.S., while only 58% of those new medicines

[1] Ltr from David Hirschmann, President and CEO, Global Innovation Policy Center, to Secretary Xavier Becerra, March 22, 2023.
[2] The report found that fewer biopharmaceutical products overall launched in Canada, Japan, South Korea, Australia, and European Union member states than in the United States over the past 20 years.

were similarly available in Europe. Similarly, in several benchmark countries, patients can often wait up to several hundred days to receive access to life-saving treatments, waiting an average of 133 days in Germany and up to 500 days in Spain.

Unfortunately, some believe that government intervention and price setting is the most effective way to provide patients with access to life-saving innovations. This approach is embodied within the drug pricing provisions of the IRA. While the IRA claims to promote access by controlling prices through so-called "negotiation," the reality is that innovators are forced to comply with the government's arbitrary and coercive price control scheme or face crippling penalties. At the same time, incentives to develop generic and biosimilar medications, one of the key components in the innovative ecosystem in today's biopharmaceutical market, are altered in a way that changes market dynamics – embedding price controls in the U.S. market in a way that would affect future generations of medicines.

We are already seeing the IRA's anticipated harms. Several life-science innovators have ended product research and development programs, citing the new price controls. For example, Eli Lilly CEO Dave Ricks said the company had already dropped a blood cancer drug from its R&D pipeline because they "couldn't make the math work" in light of the Inflation Reduction Act.[3] Similarly, Novartis warned that the new law could discourage research in its most promising areas of research: RNA and radioligands.[4] Finally, Alnylam has stopped the development of a treatment for a rare eye disease due to the need "to evaluate impact of the Inflation Reduction Act." [5]

In addition, research by The Pharmaceutical Research and Manufacturers of America ("PhRMA") shows the IRA's pricing provisions may put the development of more than 400 new medicines at risk.[6] This research indicates these potential medicines under development target some of the most common, yet serious, chronic diseases affecting America's seniors, including Alzheimer's, diabetes, and congestive heart failure.[7] Unfortunately, this report, too, demonstrates the IRA's price controls are already having a "chilling effect" on research and development. According to the report, life science innovators believe the IRA's current framework will undermine advances critical to patient well-being.[8] In fact, when asked, some 82% "or more of companies with pipeline projects in cardiovascular, mental health, neurology and cancers expect substantial impacts on R&D decisions…."[9]

The impact of government price setting on product development also extends to disease areas beyond oncology such as cardiovascular disease. Despite advances in prevention and treatment, heart disease remains the leading cause of death in the United States,

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[3] Deena Beasley, *Drug companies favor biotech meds over pills, citing new U.S. law*, Reuters, January 13, 2023
[4] Ludwig Burger, *Novartis warns U.S. plan to curb drug prices could hit key research*, Reuters, January 20, 2023.
[5] Grogan, *supra* note 1.
[6] Medicines in Development, 2023 Report, Pharmaceutical Research and Manufacturers Association of America.
[7] *Id.*
[8] *Id.*
[9] *Id.*

representing a huge unmet need for safer and more effective treatment options. Price controls like those contained in the IRA will have a negative effect on the ability of America's innovative companies to continue developing new cardiovascular medicines especially at a time when the costs, complexities, and risks of running large-scale cardiovascular clinical trials are greater than ever.

It takes multiple years and tens of thousands of patients worldwide to conduct a pivotal Phase III cardiovascular clinical trial as well as additional years of post-approval real-world evidence studies for a new cardiovascular medicine to become established in clinical practice and treatment guidelines. It is for this reason that the number of cardiovascular medicines researched has declined across all phases of development in the last 20 years. From 2012-2017 alone, cardiovascular medicines comprised just 6% of all new drug launches. Since many of the cardiovascular medicines in development are small molecules drugs, the IRA's 9 years from initial approval for small molecules drugs to be subject to Medicare price setting, shortens the runway to recoup the large-scale investment necessary to run complex cardiovascular trials thus exacerbating the ongoing decline in cardiovascular research and development.

These are but a few of the most prominent examples of the innovative, life-saving products whose realization, availability and ultimately access are ironically threatened by the IRA's price controls that would purportedly improve access. Unfortunately, it may be the most vulnerable patients – including older Americans, those diagnosed with rare diseases, and underserved populations– who will pay the price for innovation lost to the IRA. Government intervention in the market establishment of prices undermines the innovation ecosystem that enabled the U.S. to become one of the most inventive countries in the world. Decisionmakers must consider the implications of price controls for patients before proceeding with the implementation of the IRA's framework, which would jeopardize U.S. leadership on biopharmaceutical innovation and access to treatments. The ability of American patients to access life-saving innovations in a timely manner depends on it. Surely this outcome—less innovative medicines and longer wait times—isn't what anyone wants.

II. Recent IRA implementation actions contemplated by the Administration are not supported by the statute and further undermine life-science innovation, devalue the living innovation ecosystem, and limits patient access to new, life-saving medications.

While we believe the IRA's statutory price controls are harmful to America's life-science ecosystem, we are concerned that interpretations and actions contemplated by the Administration in recent CMS guidance go beyond the statutory text and further exacerbate the law's negative effects. For example, CMS's proposed guidance anticipates establishing rules that would penalize life-science innovators for investing in extensive research and development to acquire patents for selected medications. Under the proposed guidance from CMS, the agency would "consider the length of the available patents…and may consider adjusting the preliminary price downward" if the patents last "for a number of years." Given the timelines set

forth in the IRA, this could include both patents on medications originally approved and patents secured for subsequent innovations.

This policy could penalize America's life-science innovators for engaging in both initial product innovation and in additional research and development into new treatments and new applications of existing treatments. These policy changes are inconsistent with the United States Government's deliberate, longstanding intellectual property policy decisions, on which companies and investors have relied for many years, to bolster innovation with patent protection in the U.S. Both theory and reality suggest that more patents in a therapeutic class expand innovation and economic growth, expand patient choice, and advance the public good with better health. Innovation is not a one-time, compartmentalized process. When a life sciences innovator files an initial patent application it often does so in the early stages of research and development, years before an intended product reaches the market and all aspects of its applications and treatments have been clinically tested. Extensive clinical trials and continuing investments in research and development are required to uncover subsequent health conditions that may be treated by the initial product. The result is living innovation, a tree that continues to bear fruit. From delivery efficacy and patient compliance to dosages, mitigation of side effects, extended-release formulations, and entirely new treatments, so-called "follow-on innovations" deliver invaluable benefits to patients and consumers. [10]

More than 60 percent of oncology medicines approved a decade ago went on to receive additional approvals, and more than 70 percent of these additional approvals occurred seven or more years after initial approval, and as such required significant investment in research and development on the part of the innovator. These new uses provide treatment options for different diseases, including rare diseases, or additional patient populations such as children. However, with the policies laid out in the IRA guidance, instead of these critical advances, companies will have to reconsider whether post-approval research is sustainable, given the commitment of time and resources.

One product that demonstrates the value of living innovation is Botox. When Botox was initially approved, it was to treat two rare eye muscle disorders. Today, there are more than 12 approved indications, including for overactive bladder.[11] Similarly, AZT was originally developed as a failed attempt at cancer treatment.[12] It was only years after its failed application as a cancer treatment—and untold investments in clinical trials and research—that its potential in

[10] Professor Kristen Osenga, *Are "patent thickets" to blame for high drug prices,* Richmond-Times Dispatch, Nov. 30, 2022 ("It's no secret that drug manufacturers regularly continue to innovate drugs long after they're originally proven safe and effective. There are countless legitimate reasons to do so. Sometimes, post-market research suggests that a particular dosage or delivery method could be superior to the original.").
[11] *Id.* AbbVie Inc., Press Release, AbbVie to Showcase Migraine Portfolio and Pipeline During the 16th European Headache Federation Congress (Dec. 6, 2022).
[12] Christopher M. Holman, Why Follow-On Pharmaceutical Innovations Should Be Eligible For Patent Protection, IP-Watch, Sep. 21, 2018, available at https://www.ip-watch.org/2018/09/21/follow-pharmaceutical-innovations-eligible-patent-protection/.

the fight against HIV/AIDS was discovered.[13] Without the ability to engage in continuous innovation and secure patent protections, it is questionable whether the new treatments for either of these life-science innovations would be available.

Each stage of innovation requires new investment and risk, which is made possible by incentives like the potential for patent protection. According to one study, the median cost of getting a new life science innovation to market was $985 million, with an average overall cost of $1.3 billion.[14] Other studies estimate the cost, based on the amount of research and clinical trials required, could be as high as $2.8 billion.[15] The reality is that cutting-edge medical treatment is costly, and the hope it gives to patients with previously uncurable diseases and illnesses is immense. To justify these substantial costs and investments in life-science innovations, many of which never materialize or become profitable, innovators must have access to potential patent protection, and the ability to recoup significant investments to enable future innovations and follow-on uses that arise later in the product's development lifecycle.

Simply put, given the significant costs associated with bringing any iteration of a product to market, without the ability to secure full scope of protection and additional protections for follow-on innovations, life science companies may be severely constrained in their ability to invest in new or improved versions of their medicines. Actions contemplated by the guidance, and especially its penalization of companies that secure additional legal rights, would undermine the living life-science innovation ecosystem and prevent new medicines and treatments for existing medicines from entering the market. This would ultimately harm the very people CMS wishes to protect: American patients suffering from debilitating diseases.

To be clear, the Chamber believes that the IRA's price-control provisions are unconstitutional. But the underlying defects in the statute are no justification for CMS to go even farther in guidance and undermine innovation and patient access even more than the IRA itself requires.

III. The Administration and some in Congress seek to rapidly expand the IRA's price control policies before the IRA itself is fully implemented and its impact on innovation can be fully understood.

Under the Administration's proposals, as reflected in his FY2024 budget request, the number of life-saving medications subject to price controls could be quadrupled to as many as 40 medicines. In addition, the Administration's s proposals would decrease the amount of time such products could sell at fair market prices before arbitrary price controls kick in. Finally, the proposal would extend price controls to the ***private sector*** market.

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[13] *Id.*

[14] *See generally* Wouters OJ, McKee M, Luyten J, *Estimated Research and Development Investment Needed to Bring a New Medicine to Market*, 2009-2018, JAMA, March 3, 2020

[15] Robert Zirkelbach, *The Cost of Innovation*, PHRMA, November 19, 2014.

These proposals send a signal to America's life-science companies that there is no support for the development of further innovations and cures. According to Nick Shipley, Chief Advocacy Officer for the Biotechnology Innovation Organization, the Administration's proposals would "further destabilize Medicare, slow critical investment in future research and development, stall drug innovation, and ultimately harm patients." This would, in sum, represent another blow to the millions of patients suffering from debilitating diseases who are depending on America's private sector to innovate new cures and treatments.

As the Chamber noted in the preceding section, anecdotal evidence already suggests that America's life-science innovators are abandoning research pipelines for new, life-saving medications. Common-sense suggests that trend will accelerate if Congress enacts additional price controls.

IV. Taxpayer Funding of R&D is Dwarfed by Private Sector R&D Investments.

The policies pushed by the Administration, some Members of Congress, and advocates for price controls reflect a failure to understand the true relationship between taxpayer funding and private sector expenditures on research, development, and commercialization. According to the Congressional Budget Office (CBO), the private sector invested $83 billion in pharmaceutical R&D expenditures in 2019.[16] Adjusting for inflation, that is 10 times the amount invested in the 1980s, illustrating the growing role the private sector plays in supporting the success of the America's innovation ecosystem.[17] The CBO report acknowledges that the federal government underpins biopharmaceutical R&D spending in three ways. First, the government can influence the demand for new drugs by subsidizing the purchase through federal programs, such as Medicare and Medicaid. Second, the government can help increase the supply of new drugs by funding "basic biomedical research that provides a scientific foundation for the development of new drugs by private industry." [18] Third, federal government policy can influence both the supply and demand for drugs by increasing the demand for a specific medicine while also creating incentives for the private sector to invest in the next generation of medicines.

The successful role of public-private partnerships isn't limited to the life-sciences innovation ecosystem. In fact, private sector funding underpins American innovation across all technology sectors. According to the National Science Foundation, the private sector is the main driver of research and development expenditure, accounting for almost 83% of investment from 2010-2019.[19] The National Science Foundation found that the majority of research and development involves experimental and applied research, both of which are

[16] *Research and Development in the Pharmaceutical Industry,* Congressional Budget Office.
[17] *Id.*
[18] *Id.*
[19] *The State of U.S. Science and Engineering 2022*, National Science Foundation.

dominated by the private sector.[20] And, while the federal government's investment in research and development increased in real dollars over the last decade, its total share dropped from 31% to 21%, meaning the private sector increasingly accounted for a larger and more impactful share of research.[21]

As industry experts and thought leaders have noted, these facts can only lead to one conclusion: our public-private partnerships are working, allowing innovative private sector actors and the federal government to contribute through their unique areas of specialization, which improves the efficiency of the innovation ecosystem overall. In other words, the data indicates that our system is working, and not because taxpayers are bearing a burden and companies are simply free-riders. On the contrary, the private sector pays market rates to license rights to intellectual property when useful discoveries emerge from government-funded research. The system works because the private sector assumes the risk of actual drug development and testing, a process of sunk investment that more often than not results in failure and significant financial loss.[22]

The role of the private sector in bringing publicly funded basic research to market is even more pronounced in the context of life-saving treatments. According to a recent paper published by several scholars, of the tens of thousands of National Institutes of Health ("NIH") funded grants from 2000, only 18 treatments were approved by the Food and Drug Administration ("FDA").[23] Of these 18 approved treatments, taxpayer funding totaled only $670 million. In contrast, private-sector funding totaled $44.3 billion. When applying these facts in a logistic regression analysis, they found a "positive and significant relationship between private sector funding and the likelihood of FDA approval…[while] [t]he relationship between public funding and the likelihood of FDA approval is….negative and not statistically significant."[24] In other words, compared to the significant resources invested by private enterprises, public funding had almost no impact on the product's ultimate approval and availability to the public.[25]

This evidence makes clear what proponents of strong public-private partnerships have known all along: that the private sector, subject to inherent market risks and potential economic failure, plays a significant and vital role in bringing new discoveries to patients, i.e.,

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[20] *Id.* ("The majority of R&D performance is in experimental development (65%) and applied research (19%), and the business sector dominates in both. With its focus on new and improved goods, services, and processes, the business sector performs 90% of experimental development, and 58% of applied research. Higher education institutions perform the largest proportion of basic research (46%). However, the share of basic research performed by the business sector increased from 18% in 2012 to an estimated 30% in 2019.").

[21] *Id.*

[22] *Id.*

[23] Schulthess, D., Bowen, H.P., Popovian, R. et al.,*The Relative Contributions of NIH and Private Sector Funding to the Approval of New Biopharmaceuticals*, Ther Innov Regul Sci 57, 160–169 (2023)

[24] *Id.*

[25] *The US Ecosystem for Medicines. How new drug innovations get to patients*, Vital Transformations (Showing that the private sector is responsible for inventing 90% of all medicines (45% pharma companies, 45% biotech), academia 8% and the government around 1%.).

the private sector and private resources play the indispensable role in turning a discovery into a medicine and making it widely available for public consumption and use. This Committee's leadership must recognize this basic fact, promote, and advocate for the continued growth of public-private partnerships, and resist efforts to undermine private sector research, development, and investment in life-saving medications.

V. Legal Frameworks that Promote Successful Public-Private Partnerships have Delivered Lifesaving, Cost-Effective Innovations to the Public and Must be Protected.

We are also concerned by attacks on the successful statutory framework which promotes public-private research and development partnerships. This framework is otherwise known as the Bayh-Dole Act, which, since its passage, has been a foundational element in America's success in research and development.[26] The Bayh-Dole Act enables public-private collaborations and allows expanded access to new, life-changing innovations that help make the U.S. the global innovation leader.[27]

By any measure, the Bayh-Dole Act has been highly successful. According to some estimates, since its passage the Bayh-Dole Act has contributed $1.9 trillion to the U.S. economy, supported 6.5 million jobs, and helped lead to more than 15,000 start-up companies.[28] In addition, the Bayh-Dole Act has allowed thousands of commercial products stemming from university research to be introduced to the public.[29] As The Economist put it, the Bayh-Dole Act "unlocked all the inventions and discoveries that had been made in laboratories throughout the United States…."[30]

The Bayh-Dole Act's success is even more pronounced in the case of life-science innovations, and its legal framework is considered foundational for biopharmaceuticals.[31] Prior to the enactment of Bayh-Dole, not a single pharmaceutical product had been created from federally funded inventions. In contrast, since Bayh-Dole's implementation, more than 200 new

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[26] *See Quaadman*, *supra* note 3 ("Bayh-Dole established a fair, appropriate, and pragmatic system for the federal government to transfer proprietary rights in research. It has been critical to the success of the United States in bridging the "valley of death" and ensuring that scientific knowledge translates into usable products, services, and technologies that both serve end-users and advance national strategic priorities.").

[27] Tom Wilbur, *IP Explained: Four things to know about the Bayh-Dole Act*, September 13, 2019 ("Adopted by Congress in 1980, the bipartisan Bayh-Dole Act allows institutions and grant recipients, such as universities, to hold the title to patents on inventions stemming from government-funded research and to license the rights to those inventions to private sector partners who further develop them for commercialization. These private sector partners, including biopharmaceutical companies, assume the full risk of developing and commercializing the technologies that may eventually prove to be viable products. This can generate royalties for the research institution, paid by the commercial developer, once a product is brought to market.").

[28] Home - The Bayh-Dole Coalition (bayhdolecoalition.org).

[29] *See* https://autm.net/surveys-and-tools/databases/statt.

[30] *Innovation's golden goose*, The Economist, December 14, 2002 (Describing how the Bayh-Dole Act was perhaps the most inspired piece of legislation enacted in the last half century.).

[31] Lou Berneman, *A plan to cut the price of some medicines could end up hurting more than it helps, The Morning Call*, October 19, 2022.

life-saving treatments and vaccines have been developed and brought to market.[32] This includes some of the technologies, therapeutics, and treatments which drove the development of COVID-19 vaccines, illustrating that both the public and private sectors play critical roles.[33]

One product that reflects the remarkable success of the Bayh-Dole Act in life-sciences innovation is Xtandi (enzalutamide), the only novel hormone therapy approved by the FDA to treat three types of advanced prostate cancer. UCLA, as the patentee, received less than $500,000 in taxpayer funding to support early-stage research that directly contributed to the initial discovery of Xtandi. In contrast, Astellas and its partners contributed almost $2.2 billion in pre-clinical studies and clinical trials to bring Xtandi to market. As a result of this collaborative public-private partnership, which proportionally cost taxpayers less than 0.023 percent of Xtandi's overall development cost, hundreds of thousands of patients have received a life-saving treatment that otherwise would not exist. Notwithstanding these freely available facts affirming the outstanding success of the Bayh-Dole mechanism, biopharmaceutical industry critics have targeted Xtandi in their attempts to support the false notion that the government pays twice. In truth, it would be fair to say the private sector paid four thousand four hundred times. Thankfully, the National Institutes of Health ("NIH") has realized the success of this public-private partnership which led to Xtandi's development and rejected calls to undermine it.[34]

The Bayh-Dole Act works well and provides countless benefits to the American public, including facilitating access to new, life-saving medications. At a time when America is engaged in a global competition for innovation leadership, we cannot risk upending highly successful legal frameworks based upon false narratives which misrepresent the role of taxpayer funding in the commercialization of products. The documented and growing effort of the Chinese government to outpace U.S. innovation, including in the biopharmaceutical sector, would be supported and enhanced by efforts to weaken our current, successful framework. The Chamber urges this Committee to resist any legislative actions which would weaken our tech transfer systems established under the Bayh-Dole Act and instead do anything and everything it can to support its continued success.

VI. The Federal Government Must Not Engage in Actions that Will Degrade and Undermine Successful Domestic and International Innovation Frameworks.

Separate and independent from preventing any legislative changes to the Bayh-Dole Act's successful framework, this Committee must resist any efforts by the Administration to impair tech transfer using "march-in" rights. As this Committee is well aware, during the Bayh-Dole Act's drafting process, lawmakers were concerned about private sector startups and market-dominant enterprises who failed to ***commercialize*** a partially taxpayer-funded

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[32] Wilbur, *supra* note 12.
[33] Joseph Allen, *Lawmakers Aim a Triple Whammy at American Innovation*, IP Watchdog, November 7, 2022.
[34] Jeannie Baumann, *Pfizer, Astellas' Xtandi Patents Avoid Seizure by NIH*, Bloomberg, March 21, 2023.

innovation.[35] Because of that, Congress included a ***very limited*** march-in provision which allows the government to force the patent owner to grant additional licenses if, for example, good faith efforts are not being made to bring the product to market.[36]

Unfortunately, in recent years, advocates for weakened intellectual property rights have advanced a false theory that march-in rights can be used as a form of price control. These advocates, including several Members of Congress, have asked the federal government to use march-in rights as a blunt tool to reduce the price of certain life science products.[37] The proponents of this theory claim that the government has the legal authority to "march-in" and revoke exclusive patent licenses at any time, for any reason, if it decides a product is too expensive. The government could then simply re-license the patent to companies that promise to sell the product at a reduced cost.

March-in rights, however, were never intended to be a mechanism whereby the government could dictate the price of a commercialized product. The late Senators Birch Bayh and Bob Dole—the lead sponsors and negotiators of the Act—both confirmed march-in rights were never intended to be a mechanism to control prices, noting that nothing in the text or legislative history supports such an assertion.[38] Senators Thom Tillis and Marsha Blackburn, two recognized experts on intellectual property law and tech transfer, have also recognized that using march-in rights in an attempt to lower product prices "contradicts the purpose and the function of the Bayh-Dole Act."[39]

If utilized, this false theory of march-in rights, would deter private sector partnerships thereby decimating America's life sciences innovation ecosystem and directly result in fewer life-saving products entering the market.[40] The Bayh-Dole Act's overwhelming success has allowed universities and research institutes to partner with the private sector, which has the expertise, capacity, and resources to commercialize technologies. Using march-in rights on available products would destroy the Bayh-Dole Act's delicate balance and harm future innovation.[41]

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[35] *See Issue Brief: March-In Rights Under the Bayh-Dole Act*, Bayh-Dole Coalition, February 2023.
[36] *Id.*
[37] Ltr. from Senator Warren et. al. to Secretary Xavier Becerra, February 18, 2022.
[38] Bayh-Dole Coalition *Issue Brief*, supra note 19.
[39] Ltr. from Senators Thom Tillis and Marsha Blackburn to Secretary Xavier Becerra, February 24, 2022 ("Stripping intellectual property rights for private actors simply because they are commercializing their applied research on terms opponents dislike contradicts the very purpose and function of the Bayh Dole Act. March-in rights were never intended to function as price controls nor does the statute allow it. The authors of the statute – Senators Bayh and Dole – have said as much. Every Republican and Democratic Administration dating back to President Clinton has agreed. The statute clearly doesn't sanction marching in to control prices of successfully commercialized products.").
[40] Stephen Ezell, *The Bayh-Dole Act's Vital Importance to the U.S. Life-Sciences Innovation System*, Information Technology and Innovation Foundation, March 14, 2019; *See also* Ltr. from Senators Tillis & Blackburn, *supra* note 23 ("March-in rights, exercised inappropriately, would destroy the development of new, innovative, and life-saving medications.").
[41] Bayh-Dole Coalition, *supra* note 19 ("If the government ever chose to misapply march-in rights for price control, confidence in universities or federal laboratories as reliable research partners would collapse. No company would

If anything, lawmakers should want private industry to save American taxpayers money by investing private funds in commercializing academic research and bringing new products to market. Bayh-Dole's successful commercialization framework ultimately benefits the public and delivers taxpayers the benefits of the basic research that their government marginally funded. This framework works because private sector actors believe it will operate as it has done the past 40 years: without the threat of forced tech transfer. If private companies were to become subjected to march-in rights, these innovators would lose faith in the system and would no longer make the risky investments needed to translate promising scientific discoveries into testable products, and ultimately deliver them to market. Again, as cited earlier, a change of policy could have taken 99.977% of research funding off the table in the development of Xtandi.

Thankfully, NIH has recently rejected calls to use march-in rights on Xtandi.[42] The NIH correctly found that the criteria under the law for using march-in is whether a product has been successfully commercialized.[43] [44] The decision by NIH is a critical victory for and affirmation of the success of Bayh-Dole's statutory framework.

Unfortunately, on the same day the NIH announced its Xtandi decision, Secretary Becerra announced the creation of a "whole of government" interagency task force on Bayh-Dole[45] whose purpose is to ""develop a framework for implementation of the march-in provision that clearly articulates guiding criteria and processes for making determinations where different factors, including price, may be a consideration in agencies' assessments."[46] As the NIH's decision on Xtandi demonstrates, the law and criteria for the use of march-in rights is already clearly articulated and settled. Given that, the Chamber believes this task force is unnecessary, duplicative, and serves no other legitimate purpose than to weaken the successful public-private partnerships under Bayh-Dole and therefore should be abandoned.

This Committee must reject calls for utilizing march-in rights as a mechanism to lower prices, should stand firm in defense of the Bayh-Dole Act's successful statutory frameworks, and should exercise appropriate oversight of the Administration's proposed task force on Bayh-Dole. Anything short of these actions would represent a failure to protect America's innovation and tech transfer ecosystem.

The Chamber also believes that given the vital role IP plays in supporting investment in innovation, U.S. leadership to advance strong, rules-based global IP standards is critical. The Chamber is grateful for the U.S. government's legacy efforts to promote and protect IP worldwide. However, the Chamber was alarmed by the U.S. government's unprecedented

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agree to license a university or federal laboratory invention under these circumstances. No venture capitalist would fund a startup company with that sword hanging over its head.").

[42] Baumann, *supra* note 24.

[43] Joe Allen, *Bayh-Dole Opponents Slam-Dunked Once Again*, IP Watchdog, March 23, 2023.

[44] *Id.*

[45] HHS and DOC Announce Plan to Review March-In Authority, March 21, 2023.

[46] *Id.*

support for the waiver of WTO TRIPS commitments related to COVID-19 vaccines, which will disrupt the IP ecosystem that enabled American industry's highly effective response to the pandemic and undermine future American innovation. This marks a radical departure from long-standing, bipartisan U.S. policy.

The decision by WTO members in June 2023 to waive IP commitments as applied to COVID-19 vaccines, and the ongoing negotiations over the extension of this "TRIPS Waiver" to COVID-19 therapeutics and diagnostics, have falsely branded IP rights as a barrier to access to innovation. While early U.S. support for these waiver measures may have been justified by some as a willingness to endorse "extraordinary measures" and a "no stone unturned" approach amid a global health crisis, the waiver's realization came long after its ostensible purpose was mooted by a large and growing surplus of COVID-19 vaccine supplies.

Proposals to expand the waiver to therapeutics and diagnostics will only compound threats to American competitiveness and sabotage investment in other IP-intensive sectors, including digital, green, and agricultural technologies that are central to the response to current and future crises. With renewed U.S. leadership at multilateral organizations in support of a strong, global framework of IP rules, it is not too late to stem the damage from the initial waiver and preserve American jobs, foster ingenuity, and protect U.S. national security.

VII. Conclusion.

The Chamber appreciates the opportunity to submit these comments on policies which are or will undermine the medical innovation ecosystem. We stand ready and willing to work with this Committee to find ways to ensure that life-saving medications are both available and accessible to all Americans. However, the Chamber cannot and will not support misguided, market-restrictive efforts that limit patient access and choice and fail to recognize and appropriately consider the private sector's chief role in bringing new, innovative, and life-changing products to market. The Chamber also remains concerned about proposals to expand international waivers for life saving therapeutics and diagnostics. The Chamber urges this Committee to continue its support for the proven and successful statutory frameworks which support public-private partnerships, to resist all efforts by the Administration and some Members of Congress to undermine support and confidence in their protections, to reject misguided expansions of the TRIPS waiver, and to reject any new policies which impose anti-innovation price controls on and harm the development of new, life-saving medications.

Sincerely,

Patrick Kilbride	Katie Mahoney
Senior Vice President	Vice President
Global Innovation Policy Center	Health Policy
U.S. Chamber of Commerce	U.S. Chamber of Commerce

cc: Members of the House Committee on Ways and Means

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