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Examining Policies that Inhibit Innovation and Patient Access

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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.