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Ensuring Affordable and Valuable Pharmaceutical Innovation for Americans

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Key Points:

- The challenge for public policy is to sustain the pace of medical innovation while ensuring that valuable new technologies remain affordable and accessible.
- The U.S. is by far the largest market for pharmaceuticals in the world and the engine of global pharmaceutical innovation. Other countries, in effect, free ride off the innovation stimulated by the American market.
- Despite stable or falling net prices paid to prescription drug manufacturers over the past decade, novel medicines lie increasingly beyond the financial reach of American patients.
- Blunt price controls are not the solution to the worsening affordability of prescription drugs or to global free-riding: Schaeffer Center research suggests that introducing European-style pricing policies would reduce Americans' life expectancy.
- Instead, aligning drug prices with the actual value provided to patients stimulates innovation that benefits patients and discourages innovation that does not.
- Legislation to increase drug price transparency, coupled with better information about value, can help payers and consumers spend their money wisely.
- Affordable and generous insurance for prescription drugs ensures that drugs remain within the financial reach of American families.

Chairman Sanders, Ranking Member Cassidy, and Honorable Members of the Committee, thank you for the opportunity to testify today about drug prices and the assessment of medical technologies.

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, I co-wrote the chapter in the *Handbook of Health Economics* on intellectual property and biomedical research, and I co-authored the book *Valuing Health* on modern methods for valuing medical technology. 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 in Global Context

In December of 1984, a young boy from Indiana named Ryan White was diagnosed with AIDS, as a result of a transfusion with infected blood. While his doctors gave him just six months to live, Ryan outlasted those predictions and lived six more years. In the immediate wake of his untimely passing in 1990, Congress passed the Ryan White Care Act, which has since ensured affordable care for generations of HIV/AIDS patients. While the Act played a critical role in the fight against HIV almost immediately, its full value would not be realized until five years after its passage, when highly active antiretroviral therapy (HAART) emerged as a life-saving treatment for patients with HIV. The Ryan White Care Act put effective medical care within reach for many HIV+ patients that would otherwise have gone without it, while medical innovation brought new forms of treatment that changed the lives of patients and their families. Today, 9 out of 10 patients receiving care through the Ryan White program enjoy viral loads so low that they are no longer infectious. Thanks to breakthrough medical innovation, and to forward-thinking public policy that made it affordable to many, HIV+ patients treated with HAART in a timely fashion can now expect to live well into their 70s and beyond.

The case of HIV illustrates a pair of health policy truisms. Increasing patient access through bold expansion of affordable care means little when there are no valuable cures or treatments to access. At the same time, breakthrough medical therapies provide little value if high cost-sharing pushes them out of patients' reach. The challenge for public policy is to sustain the pace of medical innovation while ensuring that valuable new technologies remain affordable and accessible to the patients who need them.

At first blush, it may seem impossible to navigate the narrow straits between affordability and innovation. Medical innovation investment carries high risk that drives up the cost of discovery. Among investigational medicines that undergo human trials, 90% will fail to launch. Pharmaceutical and medical device firms will undertake these costs only if they expect to recoup the cumulative costs of their investments and receive a reasonable rate of return. However, these returns on innovation must ultimately be paid by all Americans, through out-of-pocket payments, health insurance premiums, and taxes. In this respect,

therefore, greater rewards for innovators lead to more innovation but less affordability. The converse is also true: bluntly lowering prices makes new medicines more affordable for today's patients, but limits innovation for future generations of patients.

This trade-off between innovation and affordability has played out in the different approaches taken across the globe. There is little doubt that U.S. consumers access newer drugs sooner and more often than their overseas counterparts. Academic research shows how this tendency results in more and earlier new drug launches in the U.S., and correspondingly fewer and later launches in other countries. Schaeffer Center research suggests that introducing European-style pricing policies would ultimately lower innovation and cost American consumers just over half a year of life expectancy, about what would be lost if American surgeons suddenly forgot how to perform heart bypass surgery.[1]

Meanwhile, academic research finds that the American healthcare system performs better than its European counterparts in treating disease. For example, American mortality rates from breast, colorectal, and prostate cancer have fallen faster than European rates. Indeed, an analysis of cancer care across 16 countries found countries where cancer spending has grown more rapidly have also experienced faster declines in cancer mortality rates. According to our research, where the U.S. lags is in the prevention of chronic diseases like heart disease, hypertension, and diabetes. Faster growth in American obesity appears to have played an outsized role in driving these differences. In short, America's relatively low life expectancy appears to be in spite of, not because of, its healthcare system.

Despite the good news, however, there is no denying the sentiment that U.S. consumers unfairly pay higher drug prices than their peers overseas. On the one hand, we cannot readily observe the actual extent of the difference between U.S. and overseas prices. Too often, price comparisons in the public discussion rely on U.S. list prices, which are easily accessible, but almost never reflect what is truly paid for a drug. While researchers have a rough idea of the average discount paid in aggregate, this provides little insight into the actual prices of specific drugs. Economic principles predict that volume will be higher on drugs offering higher discounts. Therefore, applying the average discount to the list price of every individual drug will overstate U.S. prices.

Nonetheless, economic principles also predict that U.S. prices probably are higher than prices overseas, even if we do not know by exactly how much. The culprit is the problem of "free-riding." The U.S. is by far the largest market for pharmaceuticals in the world. Smaller market countries have rational, self-interested incentives to pay lower prices, knowing that their small size allows them to save money without meaningfully reducing global pharmaceutical innovation. In effect, their lower reimbursements "free-ride" off the

[1] Bypass surgery adds about 1.1 years of life to patients treated with it. The lifetime risk of cardiovascular disease is around 60%. Thus, even if every heart disease patient received bypass surgery, it would add just over half a year of life.

American market, which remains the engine of pharmaceutical innovation that benefits patients throughout the world.

Americans have understandably become frustrated by footing so much of the world's bill for innovation. Unfortunately, we have no reliable ways to coerce other countries to act against their own self-interest. And, while it may seem tempting to stop paying higher prices and to join with the free riders, the resulting slowdown in innovation would harm American patients and their families most of all. Fortunately, there are actions we can take to ensure that patients benefit from medical advances, today and in the future.

Ensuring Patient Access to Treatments: Net Prices Are Not the Problem

The deteriorating accessibility of prescription drugs in recent years threatens to derail the access advantages and health gains American consumers have so far enjoyed. Even patients with "good" insurance are struggling to access the therapies their doctors prescribe. Plans frequently impose co-insurance requirements, where patients pay a share of their drug's list price, exposing them to artificially inflated list prices even when drugs' true costs are much lower. Plans are also restricting access or denying it altogether for an increasing share of drug compounds. Since 2012, the three largest pharmacy benefit managers have excluded a sharply increasing number of drugs from their formularies – last year, each of them excluded from coverage more than 600 products. At the same time, the average manufacturer net prices of brand drugs—the amount manufacturers receive after rebates and discounts—have declined in each of the last five years.

If it is getting cheaper to buy these drugs from manufacturers, why are they growing harder for patients to access? Part of the answer can be seen in a 2021 analysis of the flow of money spent on insulin. Between 2014 and 2018, net manufacturer prices for insulin fell by 31%, but the total expenditure per unit of insulin remained nearly constant. Growing discounts and concessions offered by manufacturers were not being passed on to patients or taxpayers in the form of lower insulin expenditures. Instead, those savings were being pocketed by intermediaries in the pharmacy distribution system, including pharmacy benefit managers, pharmacies and wholesalers. Pending legislation aimed at increasing transparency in the distribution system will shed more light on the commercial practices that enable PBMs to divert savings like this and provide more insight into where our drug spending is going. Neither third-party payers nor consumers observe the net prices they themselves are paying for individual drugs. Even large self-insured employers may be unable to get simple answers about how much they are paying for a given drug, no matter how widely used. Transparency in pricing would be a major step towards ensuring that drug prices reflect the actual value provided to patients, and don't simply enrich intermediaries.

Some academics and federal agencies have asserted[2] that price transparency harms consumers, purportedly by providing a means for pharmaceutical firms to cooperate with

[2] See page 362.

each other in raising prices. This argument is specious. In the first place, there are no academic studies showing that pharmaceutical price transparency limits competition; the argument against transparency proceeds primarily by means of a flawed analogy to a 25 year-old study of the Danish ready-mix concrete industry. Moreover, the critique of price transparency rests on the quaint notion that confidential rebates yield vigorous price-competition that benefits consumers. On the contrary, our research illustrates how confidential rebates explain why competition among branded drug companies is currently associated with higher—not lower—list prices for drugs, and correspondingly higher costs for patients paying co-insurance for their medicines.

In addition to hitting American families in the pocketbook, higher out-of-pocket costs for drugs also harm health. 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.

Sustaining Innovation for American Patients and their Families

Fortunately, reforms that promote patient access do not have to lower medical innovation. Indeed, our research shows that generous prescription drug insurance unlocks affordability and access for patients while still enabling sufficient rewards for innovation. This is not to say, however, that all innovation should be unquestioningly rewarded. The goal is to encourage innovations that benefit patients and their families, and to discourage those that do not. These goals can best be achieved when prices reflect value to patients.

Decades of economic research demonstrate that innovation follows pricing incentives. Where innovators expect higher returns, innovative effort and discovery follow. In contrast, innovators will avoid investing where they expect lower returns. As a result, aligning the price of every drug with the value it brings patients stimulates innovation that benefits patients and discourages innovation that does not. At a minimum, this requires a transparent and predictable approach to price-setting that rewards value. Predictability matters, because innovation investments follow what innovators expect prices will be, often many years in the future. Second, value must be measured in a way that holistically reflects what patients and their families care about. Doing otherwise stimulates the wrong kind of innovation.

Looking outside the U.S., many countries adopt pricing approaches that force a tradeoff between predictability and the holistic measurement of value. The United Kingdom,

Australia, and Canada employ relatively transparent systems that set prices based on three kinds of data: the clinical benefits of the new drug, the expected economic benefit of the new drug, and the likely cost impact of the new drug. Even though prices are not determined in a purely formulaic manner, drugs are more likely to be reimbursed when their prices result in sufficient economic benefit, and vice-versa. And, since economic benefit is computed using a known mathematical framework, this approach results in more predictable pricing outcomes.

However, while these countries employ a more predictable approach, they also rely on old-fashioned methods of economic analysis—for instance traditional cost-effectiveness calculations using quality-adjusted life-years (QALYs). While many have correctly observed the ethical challenges posed by the discriminatory nature of QALYs, our research demonstrates that traditional QALYs also get the mathematics and economics of value assessment wrong for patients.

On the other side of the coin are countries like France and Germany, which recognize the pitfalls of traditional economic evaluation of new medicines. For the most part, these countries focus on clinical benefits as the main criterion for reimbursement decisions, rarely if ever attempting to form specific economic estimates of value. While these countries avoid flawed estimates of value, their approach compromises predictability. In contrast to economic evaluation, which is focused on estimating a monetary benefit, clinical evaluation typically considers many dimensions of health improvement without a clear and quantitative method for weighing these different dimensions against each other. For example, one academic study found that only 2 out of the 5 official criteria specified for clinical benefit in France are statistically associated with the official rating of clinical benefit. Moreover, even if estimated clinical benefits are predictable, their effect on prices may not be. Under the German system, which uses a very specific, albeit complicated, process for measuring clinical benefit, there remains no clear quantitative relationship between measured clinical benefit and negotiated prices.

These tradeoffs also underscore the risks of so-called "reference pricing" approaches that tie American prices to those charged by other countries. In so doing, Americans would be forced to live with the vagaries of pricing systems designed and implemented elsewhere, around priorities that may differ from ours. Moreover, academic research finds that bringing reference pricing to the U.S. would likely inflate overseas prices but leave U.S. prices largely unchanged. The net result will be little if any benefit for American families in the short-term, and some degree of harm to long-term medical innovation in the bargain.

Instead, aligning prices with value encourages innovators to invest in areas that patients value. Achieving this outcome requires better information about value, which is ironic because we already have an overload of certain kinds of information about value. Prescription drugs nearly always arrive to market with studies estimating their value, often many of them, and they frequently reach divergent conclusions. Instead of even more studies, payers and consumers need an objective review and translation of the evidence on value. This might not result in a single, incontrovertible estimate of economic value, but even

a range of values, when objectively determined, would benefit the people and organizations ultimately footing the bill for prescription drugs. Better information about value, coupled with price transparency, helps ensure payers and consumers spend their money wisely.

While it is yet to be determined what the true impact of the Inflation Reduction Act (IRA) will be on biomedical innovation, there is strong evidence that cuts to Medicare's pharmaceutical spending will reduce discovery of new treatments as well as new uses for existing drugs. But there are ways to mitigate these adverse impacts. Most importantly, it is essential that Maximum Fair Price (MFP) determination hew to the principles of transparency and value to patients. Economic research provides transparent approaches that can be leveraged by CMS, and relying on economics no longer means relying on the old-fashioned QALY. For example, one new value assessment method based on research at the USC Schaeffer Center corrects the QALY's 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. This approach comports with federal law by avoiding value assessments that discriminate against vulnerable patients with disabilities or terminal illness.

Finally, 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) suggest the upward pressure on list prices will continue absent other market changes.

Sustaining Affordable and Valuable Innovation

Ultimately, the right policies need to focus on the affordability of good health, not simply of health care. This is especially true for diseases with few or no treatment options. The least affordable drugs are those that have not yet been discovered. For example, in the days before the discovery of effective vaccines, freedom from the most devastating consequences of COVID-19 could not be bought at any price. To be sure, affordable and generous insurance for prescription drugs remains part of any solution, because today's medicines already put good health within reach for millions of Americans suffering from chronic disease. Making prices transparent and generating actionable information on value will help wring out wasteful spending that fails to benefit patients and their families. Finally, rewarding drugs that do provide value helps sustain innovation and ensures good health will be increasingly within the reach of Americans for generations to come.