Drug Development and Delivery for the Next 30 Years: Affordability in an Era of Precision Medicine

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Chairman Alexander, Ranking Member Murray, members of the Committee, I'd like to thank you for the opportunity to testify today about "The Cost of Prescription Drugs: How the Delivery System Affects What Patients Pay." I'm truly honored to be speaking to you today.

Bipartisan support for medical innovation, including strong support for FDA user fee agreements, an encouraging environment for translating basic medical research into promising new treatments, and an effective balance between strong upfront patent rights and rapid generic competition once those patents expire has made the U.S. the unquestioned global leader in medical innovation for the last several decades.[1]

Broadly speaking, robust generic competition, along with the advent of large and sophisticated payers, has kept the relative share of health care costs attributable to medicines broadly stable, even as new medicines have become a cornerstone of treatment for acute and chronic illness.[2]

However, there are real challenges facing the health care system today, specifically for patients with serious chronic illnesses who are facing high coinsurance or deductibles largely for what are called "specialty" medicines, and that challenge needs to be addressed.

Ironically, part of that challenge is due to the advent of highly effective new treatments for hepatitis C, cystic fibrosis, some cancers, and rheumatoid arthritis.

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[1] For a fuller discussion, see Biopharmaceutical Policy for American Leadership 52 in the 21st Century, Peter Huber & Paul Howard.
https://nationalaffairs.com/storage/app/uploads/public/doclib/20161209_UnleashingOpportunityInnovationPolicyBooklet.pdf

[2] Total U.S. health care spending in 2015 was $3.2 trillion dollars. Approximately two-thirds of those costs are attributable to hospital care (roughly 30%) and physician services (around 25%). Outpatient prescription drug spending has held steady at around 10% of total expenditures for decades. Adding in hospital administered drugs raises that share to 14-15%. Fein, Adam J., *The 2017 Economic Report on U.S. Pharmacies and Pharmacy Benefit Managers*, Drug Channels Institute, 2017.

A wave of even more powerful treatments, including gene therapies, new immune-oncology therapies, and regenerative medicine approaches are already on the horizon and likely to be approved by the FDA over the next 5 to 10 years. The outlook for innovation has never been brighter, even as the industry has become a lightning rod for product pricing controversies.[3]

But I would remind critics that having too many effective therapies is an enviable problem to have, and can largely be addressed by enhancing market competition and creating new financing and reimbursement tools that allow payments for treatments to be pegged to their real-world outcomes—like lowering costs elsewhere in the health care system, improving patient survival or quality of life, or simply delivering a comparable outcome to existing technologies less expensively.[4]

The U.S. health care system is in dire need of competition to reduce wasteful and ineffective care, and new technological platforms can allow the rapid analysis of large volumes of patient data – enabling competition not only between medicines, but among providers and different payment platforms. In short, Congress should create incentives that reward providers who use medicines (both generic and branded) and technology to deliver care as efficiently as possible, while also empowering patients with the information they need to identify high quality providers.[5]

Fixing drug prices in a silo is inadvisable because we want technology to substitute for labor, including unnecessary hospitalizations, doctor's visits, or

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[3] Many critics point to European drug price controls as the solution – ignoring the outsized role that the U.S. market plays in global innovation generally. See To Lower Drug Prices, Innovate, Don't Regulate. https://www.nytimes.com/roomfordebate/2015/09/23/should-the-government-impose-drug-price-controls/to-lower-drug-prices-innovate-dont-regulate

[4] For a fuller discussion of the role of analytics, diagnostics, and outcomes based payments see Precision Medicine in the Era of Health Care Reform. https://www.manhattan-institute.org/sites/default/files/R-PH-0416.pdf

[5] As researchers in a Health Affairs blog wrote in 2015:

Adherence to treatment guidelines and quality remain highly variable across providers in a wide variety of oncology domains, including end-of-life care, prostate cancer, ovarian cancer, and colorectal cancer screening.

Problems range from underuse of highly effective therapies and procedures to overuse of ineffective ones. Thus, while today's typical cancer patient is likely better off than her counterpart from earlier years, not all patients are receiving the most effective care.… Rewarding physicians for patient health improvement moves physician incentives closer to the values and needs of patients.

debilitating stays in a nursing home.[6] Bending the curve of health care cost growth and delivering state-of-the-art care can, and must, go hand in hand if we are to meet America's most pressing health care challenges.

There is no accounting or discount scheme that will enable us to grapple with the scourge of Alzheimer's short of medicines that delay, or perhaps even prevent it entirely. Innovation is our best hope for lowering costs *and* improving outcomes.

With that in mind, I'd like to frame my remarks with some observations that I hope will guide our discussion today.

We stand at the precipice of a Golden Age of medicine, with new treatments that allow us to treat diseases at their molecular and genetic roots, where we can begin to speak of lasting remissions, sharply reduced disability, and even true cures—as from gene therapy.

Nonetheless, broadly speaking, the vast majority of prescriptions in the U.S. today are highly affordable, with roughly 30% having a zero-dollar copay. Most Americans who take prescription drugs regularly say they are affordable. In fact, close to 90% of all retail prescriptions in the U.S. today are for generics[7], which have saved payers hundreds of billions of dollars over the last decade.

Apart from a sharp surge in drug spending in 2014, when a new class of highly effective medicines for hepatitis C were introduced, drug price growth has been moderate, especially when we disaggregate price increases from increased utilization. A growing number of Americans are taking medicines, which is unsurprising given that age is one of the leading risk factors for developing a chronic illness. Payers, however, have been able to leverage large purchasing networks to increase manufacturer rebates as a share of gross revenues.

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[6] See Michael Mandel, Rising Labor Costs Accounted for 47 percent of Increased Personal Health Care Spending in 2015. **http://www.progressivepolicy.org/blog/rising-labor-costs-accounted-47-percent-increased-personal-health-care-spending-2015/**

[7] Fein, Exhibit 2: Unbranded and Branded Generics, Share of U.S. Prescriptions, 2005-2021.

As a result, the Centers for Medicare and Medicaid Services (CMS) expects that medicine's cost growth will closely track overall health care spending growth over the next decade.[8]

When we drill down into the market, however, costs attributable to so-called specialty medicines are rising significantly faster than for traditional drugs, and today constitute close to 30% of all drug revenues. Prices for these medicines are rising significantly faster than other costs, although they also treat especially serious chronic diseases. They also face less generic competition, including, at least for now, from biosimilars.

That overall drug spending has not risen faster is a testament to the success of insurers and pharmaceutical benefit managers (PBMs) cost containment strategies. They have employed utilization management tools like prior authorization, drug tiering and coinsurance, and larger deductibles for non-preferred medicines manage the uptake of specialty drugs.

To retain formulary access for specialty medicines, companies often offer quite substantial rebates. One PBM, Express Scripts, noted in the last year that it held contain price increases for its members to under 3%.

How is it possible, then, that payers can complain about a drug pricing crisis, while pharmaceutical firms note that drug spending, and especially net pricing after accounting for rebates and utilization increases, are fairly stable?

The he-said, she-said debate can be resolved by simply noting that there are an increasing number of patients with high deductible plans, where medicines are part of a single combined medical and pharmacy deductible, and of patients with traditional insurance who are prescribed medicines where they pay coinsurance based on the list prices of these medicines, and thus do not benefit from PBM-negotiated discounts.

For patients who may need a medicine that is excluded from the PBMs formulary entirely, short of manufacturers' patient assistance programs, they may have to bear the full costs of these medicines themselves. (PBMs respond that they pass along these rebates to employers and other payers, helping to keep overall health insurance increases lower than they would otherwise be.)

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[8] Howard and Feyman, Drug Price Controls Hurt Patients Most. https://www.manhattan-institute.org/html/issues-2016-drug-price-controls-hurt-patients-most-7949.html

To summarize: Patients with serious chronic illnesses may find themselves caught between the hammer of rising cost control efforts at a time of rapid therapeutic innovation.

There are some promising signs that payers and manufacturers recognize that the status quo is unsustainable, and are edging toward agreement that patients with serious diseases should have access, at the point of purchase, to PBM-negotiated discounts.

There is also growing agreement that reimbursement contracts for high cost, high value medicines should reflect evidence of their real-world performance, which may be very different than outcomes generated in clinical trials used for FDA approval—or for an entirely new indication, where evidence may be lacking at the time of approval.

Congress, HHS, and FDA have critical roles to play in encouraging the market to shift to new arrangements that lower barriers to patient access and encourage greater collaboration in getting the right medicine to the right patient at the right time – and at a price that is sustainable for patients, payers, and innovators.

I have three recommendations for Congress today.

First, fix the 340B drug discount program. 340B was originally designed to assist hospitals serving indigent patients, but has expanded to cover approximately 50% of the market for infused oncology medicines.

While hospitals acquire these drugs at large mandatory discounts, several studies suggest that they are billing commercial insurers a percentage of allowable charges, which is significantly higher than their acquisition price. As a result, such sales have become a major source of hospital revenues and an inducement for vertical hospital consolidation—i.e., for hospitals to acquire oncology practices and then charge far higher prices than standalone oncology practices (who charge Medicare ASP+6%).

Commercially insured patients and Medicare Part B patients thus may find themselves paying coinsurance on these highly inflated prices. Congress should reform 340B, returning it to its original intent to assist hospitals that largely serve indigent and uninsured populations, and ensure its rebates are extended to

vulnerable patients (like the uninsured), and commercially insured and Medicare Part B patients who may be treated at these hospitals and find themselves paying coinsurance. Reducing the financial arbitrage available to hospitals would also reduce the incentive for hospitals to acquire oncology practices, reduce pricing pressures on oncology payers and patients, and reduce pricing distortions in other parts of the market.

Second, stakeholders also seem to be in broad agreement that novel reimbursement contracts should reflect medicines' value, both through indication- or outcomes-based designs. Regulators should help accelerate the transition to these contracts by removing regulatory barriers that discourage companies from testing the waters.

Specifically, HHS and FDA should coordinate on creating safe harbors from federal regulations that would allow stakeholders to experiment with innovative new contractual arrangements. These might allow for reimbursement to track a medicine's real-world performance, or for pricing to evolve as the weight of evidence evolves.

For instance, recently Eli Lilly and Anthem petitioned HHS and the FDA to grant them safe harbor from regulations, like Medicaid Best Price and Stark anti-kickback rules, which prohibit them from experimenting with these types of contracts.

With the FDA at the table, regulators could also create standards for the collection of real-world evidence that would allow the agency to update a drug's label to reflect new information on safety and efficacy, expand to new label indications, and generally support the development of a "health care learning system." This system uses information on patient outcomes, medication regimens, and even delivery system reforms to create a rapid feedback loop that helps ensure that the right medicine reaches the right patient at the right time—and all in a framework pushing every dollar spent on patient care to be used as efficiently as possible.

Finally, I would encourage Congress to consider a broader menu of reforms that would allow payers to take a longer perspective on the value and costs of new medicines. Such reforms would include encouraging the uptake of value-based insurance designs; new financing tools for state Medicaid programs to purchase curative technologies rapidly, but spread the costs over longer periods of time; and multi-year private insurance contracts that may align payers' incentives with

patients' long-term health. Congress should also continue to empower patients with more information about both provider pricing and outcomes for specific indications – helping the market to reset on a competitive basis.

In conclusion, once we start asking questions about how to deliver better value to patients, to society, and to future generations, we are apt to look far past our current drug pricing debates – and towards the future of precision medicine.

For the last 30 years, the U.S. has benefitted from arrangements that have put us on the cusp of tremendous new medical achievements. The system is under strain because the pace of innovation is accelerating, while our health care system is still divided into payment silos, with a short-term framework that undervalues the long-term impact medicines can play in resolving our most pressing health care challenges – including cancer, major depression, diabetes, and Alzheimer's.

Rather than pointing fingers, I hope that Congress can construct arrangements that will serve patients better for the next 30 years, unleashing the full potential of precision medicine to improve and lengthen patients' lives, here and around the globe.

Thank you, and I'd be happy to answer any questions you may have.