

Getting to the Root of High Prescription Drug Prices

Drivers and potential solutions



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ABSTRACT

ISSUE: Historic increases in prescription drug prices and spending are contributing to unsustainable health care costs in the United States. There is widespread public support for elected officials to address the problem.

GOAL: To document the drivers of high U.S. prescription drug prices and offer a broad range of feasible policy actions.

METHODS: Interviews with experts and organizations engaged with prescription drug development and utilization, pricing, regulation, and clinical practice. Review of policy documents, proposals, and position statements from a variety of stakeholders.

FINDINGS AND CONCLUSIONS: Congress and regulators can undertake a wide range of policy actions to begin to rebalance incentives for innovation and price competition, prioritize patient access and affordability, and maximize the availability of information to patients, providers, and payers.



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EXECUTIVE SUMMARY

Historic increases in prescription drug spending and prices are contributing to unsustainable health care costs in the United States. Caught in the middle are patients. Faced with rising out-of-pocket drug costs, some must choose between taking life-saving drugs or paying the rent. Because the pharmaceutical market is complex and diverse, it will be challenging to rein in costs while still encouraging drug innovation. But it is certainly possible. There are many practical policies that could significantly curtail prices while incentivizing robust research and development.

This report documents 10 major problems that play a role in high U.S. prescription drug prices. These problems, along with their specific drivers, are creating barriers to health care access that affect patients, providers, and payers. We also identify a broad range of feasible policy actions that would curb high drug prices. The report is intended not as an exhaustive list of recommendations but as a means of fostering discussion and consensus among policymakers and stakeholders.

Our findings and conclusions are based on interviews with subject matter experts and organizations that are engaged with prescription drug development and utilization, pricing, regulation, and clinical practice (see [appendix](#)). We also reviewed policy documents, proposals, and position statements from a variety of stakeholders and performed an extensive literature review.

The 10 major problems are:

- High launch prices and high annual increases for patented brand-name drugs.
- Brand-name drugs, with Orphan Drug Act market exclusivities, are introduced with high launch prices and experience high annual price increases.
- Some manufacturers create, or take advantage of, natural monopolies for drugs that enable them to significantly increase prices.
- The lack of robust competition among manufacturers of generic drugs results in less price competition and higher prices.
- The lack of price competition among biologics and biosimilars results in higher prices.
- Anticompetitive behavior by some manufacturers undermines competition, resulting in higher prices.
- Some manufacturers use current patent-protection policies for brand-name drugs to extend monopoly pricing.
- Patients, providers, and payers lack information about the comparative effectiveness of drugs at the point in time when critical health care decisions are made.
- The pharmaceutical distribution system does not make essential pricing information available to patients, providers, and payers at the point of care—information that patients and their providers need when deciding on the best course of treatment.
- Federal law imposes limitations on state authority to negotiate prices for Medicaid and implement other price-related measures to reduce high drug prices.

This report also discusses a broad range of feasible policy actions that have been proposed by various stakeholders, experts, and researchers and could be further developed by policymakers to address high drug prices. Some of the actions identified will have a direct impact on pricing, while others may have an indirect impact but could lead to other favorable outcomes.

Our goal is for policymakers and stakeholders to use this resource to help identify the range of factors driving high prescription drug prices and reach consensus on the most significant problems affecting patients' access to affordable drugs. With a greater understanding of the issue, policymakers and stakeholders will be better positioned find a path to bipartisan solutions.

BACKGROUND

When looking to solve pricing problems in a market as complex as pharmaceuticals, policymakers and stakeholders require an understanding of the various forces and trends driving those prices. Following is a summary of some of the key developments and legislation that relate to drug prices. It is not intended to be exhaustive, and stakeholders are encouraged to dig deeper into these issues as they work to address problems in the pharmaceutical market.

Major Legislation That Shapes Today's Pharmaceutical Markets

Legislators must navigate a delicate balance when developing laws that affect the pharmaceutical market. As they look to bolster commercial drug research and development, they need to also ensure enough competition to keep prices in check. At the same time, lawmakers must consider how changes in prescription drug coverage could potentially affect the market and the health of their constituents.

Laws Affecting Prescription Drug Market and Competition

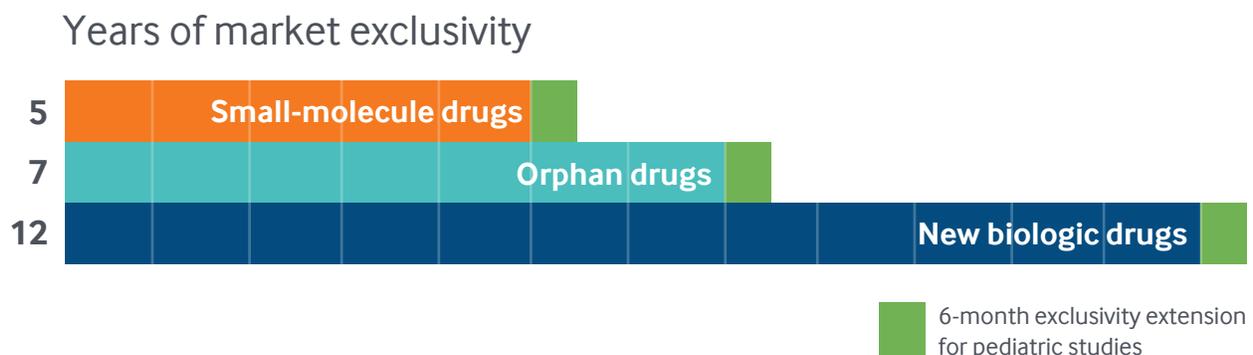
In 1983 and 1984, Congress enacted two laws designed to promote the development of new, innovative drugs and to create a competitive market through a generic drug approval process. The goals were to balance incentives that encourage research and development of innovative products, through patents and exclusive market rights, with new regulatory processes that establish and maintain price competition once market protections expire. Both laws have had minor amendments over the intervening years.

The first law, the Drug Price Competition and Patent Term Restoration Act—commonly referred to as the Hatch-Waxman Act—extended patent terms and introduced market-exclusivity protections for certain types of drugs. These policies were intended to ensure that drug manufacturers are given a period to sell patented, innovative products without direct competition so they can recoup their development costs and gain a return on investment.

The law also provides for a generic drug approval system that ensures safe, therapeutically equivalent generic drugs are available at lower prices when patents and other market exclusivities expire. In response to Hatch-Waxman and the expansion of prescription drug coverage, as explained below, all states enacted laws that played a major role in ensuring pharmaceutical access and competition. These laws generally required, with limited exceptions, mandatory substitution of generics, when available, to ensure access and price competition.

The second law, the Orphan Drug Act, provides several incentives for the development of drugs for rare diseases and conditions. Congress found that incentives were necessary because drugs for rare diseases were thought to be of limited commercial value to pharmaceutical companies due to their small patient population. The act provides a research and development tax credit and a seven-year market-exclusivity period for developing a drug for a rare disease or condition, which is defined as affecting less than 200,000 people.

Market Exclusivity Periods, by Drug Type



Additional legislation addresses a new class of drugs called biologics. These drugs, which are typically created from human or animal proteins, are more complex than small-molecule drugs synthesized from chemical compounds. Because of this complexity, biologic drugs do not have strictly generic alternatives, although equivalents, known as biosimilars, exist. In 2010, Congress passed the Biologics Price Competition and Innovation Act as part of the Affordable Care Act (ACA) to establish a 12-year market-exclusivity period for biologics and create a pathway for biosimilar approvals.

Currently, market incentives for new pharmaceuticals development vary by drug types and include:

- the underlying 20-year patent on the drug product, its use, or the process for manufacturing the product
- market-exclusivity periods of seven years for orphan drugs, five years for all new small-molecule drugs, three years for new clinical uses of small-molecule drugs, and 12 years for new biologic drugs
- extensions of patents on approved small-molecule drugs for up to five years, with a maximum of 14 years
- extensions of six months to existing patents and market-exclusivity periods for conducting pediatric studies
- the right to delay U.S. Food and Drug Administration (FDA) approval of competing generic drugs by claiming patent infringement
- the right to acquire the exclusive rights to drugs discovered through government-funded biomedical research and to set prices without restriction.

Laws Expanding Prescription Drug Coverage

To ensure patients can get needed prescription drugs, Congress over the past 30 years also significantly expanded pharmaceutical coverage through Medicaid, Medicare, and nongrandfathered individual and small-group health insurance. Starting with Medicaid in the early 1990s and then Medicare in 2006, and continuing with amendments to the ACA, Congress enacted policies to expand drug coverage and access.

Outpatient prescription drug coverage is an optional benefit in traditional Medicaid, although all 56 Medicaid programs have elected to offer prescription drug coverage. To encourage states to adopt prescription drug coverage, Congress enacted the Medicaid Drug Rebate Program in 1990 to make drugs more affordable to the states through the Medicaid program. Then, in 2006, Congress amended Medicare to significantly expand coverage for prescription drugs through the Medicare Part D program. Most recently, as part of the ACA, Congress included prescription coverage as an essential health benefit that is required to be covered under most private health insurance plans.

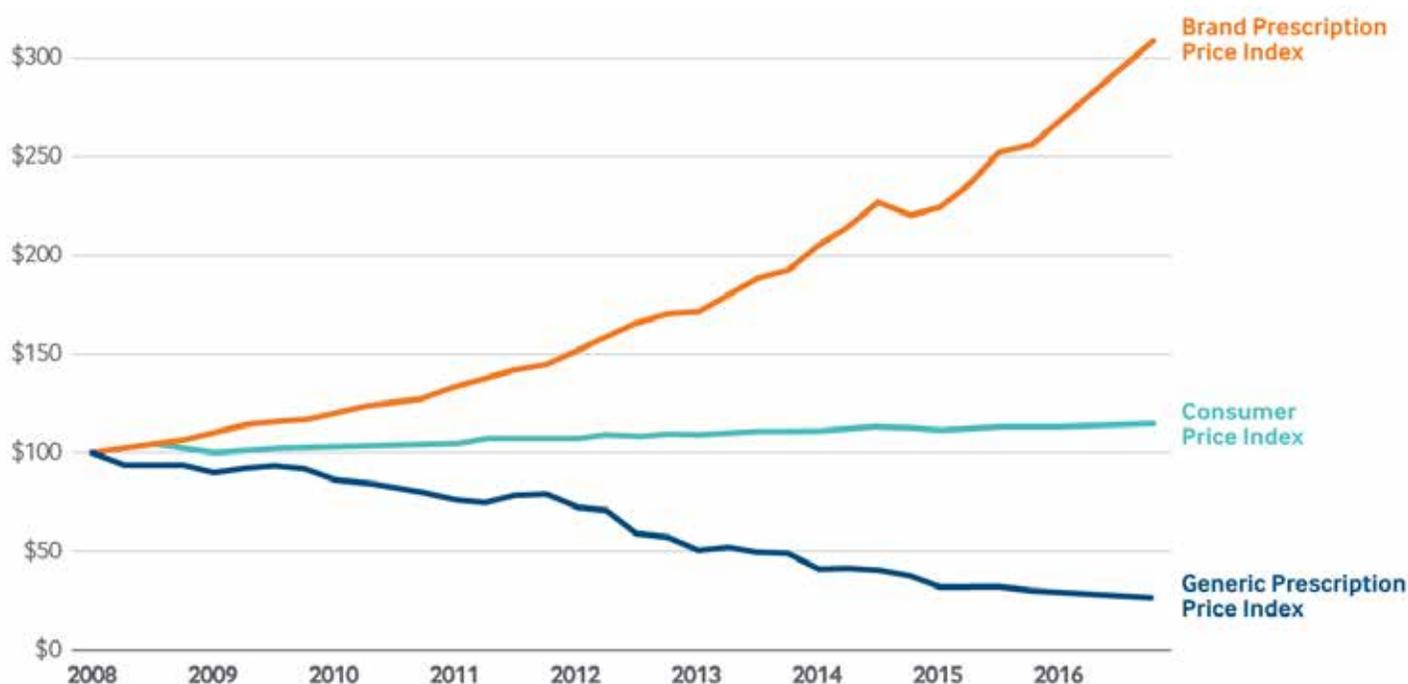
Trends in Drug Spending and Pricing

Since prescription drug coverage expanded, the U.S. has seen historic increases in drug spending as well as significantly higher drug prices. Drug spending is the amount of money paid for prescription drugs, whereas drug prices are what manufacturers charge for the drugs. Overall prescription drug spending increased 9.0 percent in 2015 following record growth in 2014 of 12.4 percent, the highest levels since 2001.¹ Spending increased by 5.8 percent in 2016, about half the rate of growth of the previous two years; this slower growth can be attributed to lower price increases for brand-name drugs and fewer new drugs entering the market.

Prescription drug spending in the U.S. was \$457 billion in 2015, or 16.7 percent of overall personal health care services, and included \$328 billion (72%) for retail drugs and \$128 billion (28%) for nonretail drugs. It is important to acknowledge that, as coverage expands, spending will increase.² Growth in health care expenditures between 2013 and 2018 is projected to be 5.2 percent, whereas prescription drug expenditure growth is projected to be 7.2 percent.

New trends in drug pricing have also emerged. While prescription drug use has clearly gone up in recent years because of a growing and older population and greater use of drugs in health care for all age groups, about a third of the rise in drug spending from 2010 to 2014 was due to either price increases or a shift toward higher-price drugs.

Prices for Brand-Name Prescription Drugs Doubled Between 2008 and 2016



Source: Kaiser Family Foundation analysis of data from Express Scripts 2015 Prescription Price Index.

Together, these trends drove average prices to increase at a higher rate than general inflation.⁵

Price increases for currently marketed drugs continue to outpace inflation, with an average annual price increase of nearly 10 percent over the past three years, compared to 2.3 percent inflation rate.⁴ Among widely used brand-name drugs, approximately 97 percent had price increases in excess of general inflation.⁵ These trends are expected to continue if no action is taken.

Impact of High Prices of Drugs on Patient Affordability and Access

As drug spending and prices have risen, the burden on patients has grown as well. Prescription drugs are the single largest health care expense for consumers with commercial insurance.⁶ Rising prescription drug costs account for approximately 22 percent of every commercial (nongovernment) premium dollar, outpacing physician, inpatient, and outpatient hospital services.⁷

When drug prices rise, patients feel the negative consequences in terms of financial burdens and loss of access. In 2016, one-third (33%) of Americans went

without recommended care, did not see a doctor when sick, or failed to fill a prescription because of costs.⁸ One in four (26%) of Americans who take prescription drugs report difficulty affording their medications.⁹ More than half of older Americans report not filling a prescription in the past two years due to cost.¹⁰

Americans want action. In late 2016, a large majority (77%) said prescription drug costs are unreasonable, with widespread support for a variety of actions to keep costs down, including:

- requiring drug companies to release information to the public on how they set their drug prices;
- allowing the federal government to negotiate with drug companies to get a lower price on medications for people on Medicare;
- limiting the amount drug companies can charge for high-cost drugs;
- allowing Americans to buy prescription drugs imported from Canada; and
- creating an independent group that oversees the pricing of prescription drugs.¹¹

Another survey found that eight in 10 (81%) older Americans think drug prices are too high, with nearly nine in 10 wanting their elected officials to do something about it.¹²

Distribution System of Prescription Drugs in the U.S.

The complexity and lack of transparency in the U.S. distribution system for prescription drugs also contribute to high prices. The current system involves many entities and a complicated flow of payments and rebates. The main entities are:¹³

- The companies that manufacture or own the rights to manufacture prescription drugs.
- Wholesalers, to whom manufacturers typically sell their products after production. Wholesalers will distribute the product to providers, including retail pharmacies, hospitals, and clinics.
- Pharmacy benefit managers (PBMs), the intermediary between the third-party payer and the manufacturer.

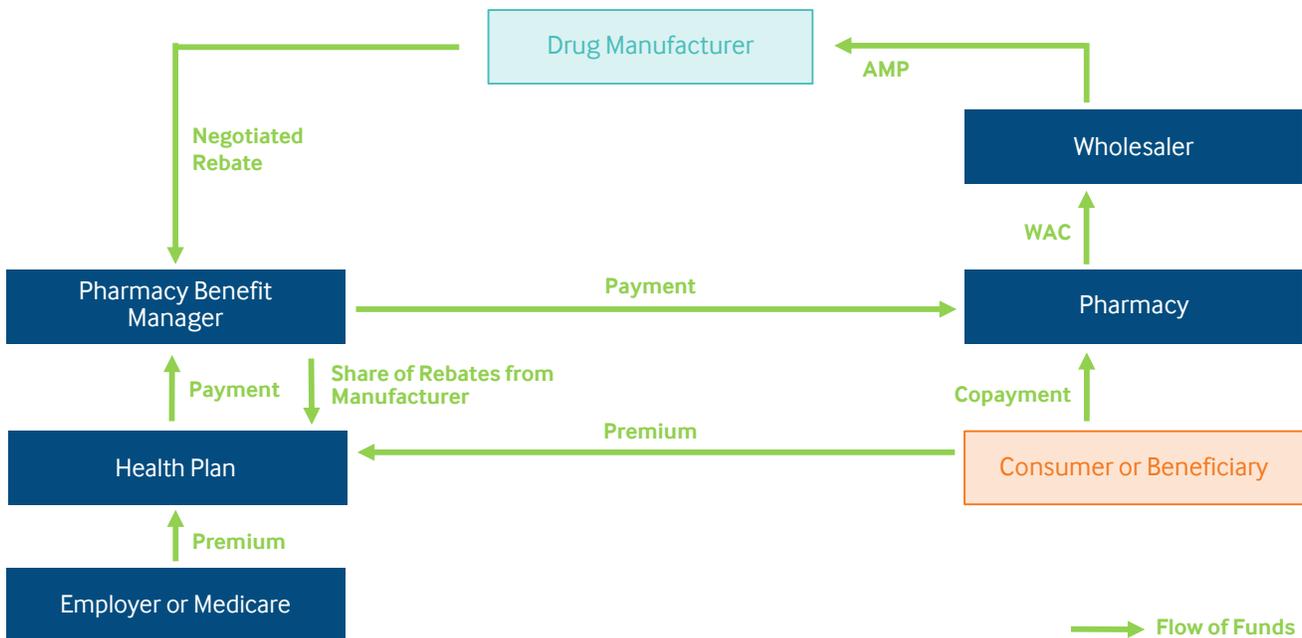
PBMs generally handle the billing, negotiation of drug prices, and creation of pharmacy networks. PBMs also have the authority to design their own formularies.

- Third-party payers, including health plans, employers, Medicare, and Medicaid. These payers provide health care coverage to people and reimburse providers and distributors for health care products and services.
- Retail or mail pharmacies, where most consumers access their prescription drugs. Specialty pharmacies generally distribute specialty drugs along with services to administer those drugs.

Generally, the manufacturer establishes a list price for a drug, and then wholesalers purchase and distribute the drug to retail pharmacies, mail-order pharmacies, and providers. PBMs and payers (or group-purchasing organizations acting on behalf of hospitals and health systems) can negotiate with manufacturers for rebates or discounts on the list price. Manufacturers generally

The Flow of Payment for Brand-Name Drugs Is Complex

Excludes federally mandated rebate programs



Note: AMP = Average Manufacturer Price, WAC = Wholesale Acquisition Cost.
Source: Congressional Budget Office.

tie their price concessions to the number or volume of products sold, with higher sales volumes yielding higher discounts from the list price. The price concessions are called rebates—general discounts from the wholesale acquisition cost (the list price from a manufacturer to a wholesaler or a direct purchaser without discounts).

PBMs retain a portion of the rebate in exchange for developing the formulary and negotiating with manufacturers. It is not known how much of the manufacturer rebates or discounts are passed on by PBMs and payers to consumers, as these concessions are considered proprietary information.

Aside from this complicated distribution system, a lack of price transparency and availability of information about the comparative value of similar therapeutic drugs makes the drug marketplace less efficient. It also undermines the goal of robust price competition to ensure patient access to the most important drugs. Some of the lack of transparency may lie in the practice of providing rebates after drugs are purchased. These rebates vary from payer to payer and from PBM to PBM.

GUIDING POLICY GOALS

U.S. prescription drug pricing over the past decade reflects a distortion of the policies enacted by Congress to balance innovation and price competition and to enable access to affordable medicine. Addressing the high prices of drugs will support efforts to reduce or manage health care costs. Guided by the principles outlined below, policymakers can establish a more rational drug pricing system that meets the needs of all stakeholders: patients and consumers, public and private health care purchasers, health care providers, and the pharmaceutical industry.

Rebalance Incentives for Innovation and Price Competition

Pharmaceutical markets are most efficient when appropriate incentives for innovation are balanced with vigorous price competition. Pharmaceutical markets also

work best when information is available to all parties involved: manufacturers, patients, providers, and payers. While discovery and development of innovative therapies is a cornerstone of the U.S. health care system, it should not be at a price that leaves patients without access. Furthermore, incentives should be focused on driving innovation in developing new treatments and cures.

Prioritize Patient Access and Affordability

Drugs should be accessible and affordable to patients when they need them. Given the increasing role of pharmaceutical products in our health care system, pharmaceutical prices must be reasonable and sustainable for patients, government programs, and taxpayers.

Maximize Availability of Information to Improve Patient Care

Greater availability of information on the clinical value, comparative effectiveness, and pricing of prescription drugs would allow patients to become more active participants in decisions about their health care. Shared decision-making involves determining which drug will be the most clinically effective as well as the best ways to ensure adherence to a drug regimen given each patient's unique characteristics and socioeconomic circumstances. Drug manufacturers should be able to clearly articulate and justify their drug pricing decisions in a clear, straightforward manner to the public.

PROBLEMS, DRIVERS, AND ACTION STEPS TO ADDRESS HIGH DRUG PRICING

In addition to providing an overview of the problems and drivers contributing to high prescription drug pricing and related access problems, this report offers a broad range of possible actions that have been proposed by various stakeholders, experts, and researchers (see appendix). Some of these actions would have a direct impact on pricing, while others would have an indirect impact by leading to other favorable outcomes, such as increased patient engagement in their health care. Because some of these problems overlap, the actions do as well.

Problem 1: High Launch Prices and High Annual Increases for Patented Brand-Name Drugs

Drivers: Patents, Market Exclusivity, and Federal Support for Drug Development

New brand-name drugs are granted patent and market-exclusivity protections to give drug manufacturers time to recoup the costs of developing new treatments and cures and to receive a return on investment. During this period of market protection, brand-name drug manufacturers have total discretion in setting their introductory prices and annual increases. The only price competition that can occur is limited and comes from clinically comparable brand-name drugs, also known as comparator drugs. In 2014, 33 new brand-name drugs were launched in the U.S., and only eight had a direct price competitor at launch.¹⁴

Introductory prices for new brand-name drugs have reached unprecedented levels over the past decade.

One study found that oral anticancer drugs introduced in 2014 were six times more expensive at launch, when adjusted for inflation, than drugs introduced in 2010 (\$11,325 and \$1,869 per month, respectively).¹⁵ However, it is not only anticancer drugs that are being launched with high price tags. Recently, several drugs have been introduced to treat psoriasis. Stelara was the first to enter the psoriasis market in 2009, at a cost of nearly \$46,000 per year. Then a comparator drug, Cosentyx, was launched in 2015 at the same price.¹⁶ In 2016, a direct competitor to Cosentyx was approved, called Taltz. The launch price for Taltz was set at more than \$50,000 per year.¹⁷

These skyrocketing prices have real-life implications. In a survey of insured cancer patients, 42 percent reported that their anticancer drugs presented a significant financial burden. To reduce their out-of-pocket costs, at least one in five used less medication than their clinicians prescribed.¹⁸

Many manufacturers are using patent protection and market-exclusivity protections to significantly increase some brand-name drug prices annually, even when there have been no significant improvements to the drug. Between 2014 and 2015, retail prices for 268 brand-name prescription drugs widely used by older Americans rose by an average of 15.5 percent, 130 times the rate of

general inflation.¹⁹ Upward price swings on specialty pharmaceuticals used to treat complex conditions are often even higher. For example, first-generation disease-modifying treatments for multiple sclerosis, originally costing \$8,000 to \$11,000, now cost about \$60,000 per year.²⁰ Prices of top-selling drugs for multiple sclerosis, Copaxone and Avonex, have seen significant increases. Copaxone's price doubled, or nearly doubled, between 2010 and 2014.²¹

These frequent price increases leave few options for payers, providers, and patients. In some cases, no clinically comparable drugs may be available. Even if a choice does exist, patients or their providers may not be willing to switch drugs, which could disrupt treatment. To maintain access for patients, payers must continue covering the drug while increasing premiums or cost-sharing, or both.

Some manufacturers develop new drugs by leveraging federally funded research and discoveries.

Through the National Institutes of Health (NIH) and other agencies, the federal government funds a large portion of pharmaceutical research and development, with substantial investments in the basic science and translational research that leads to new drug discoveries, as well as late-stage development. The government typically awards grants to researchers at academic medical centers and other institutions, and some projects are conducted by federally employed scientists. New discoveries are commonly licensed to drug manufacturers for additional development and testing, as well as marketing and commercialization. Drug manufacturers still benefit from market-exclusivity protections in these situations: they can introduce these new drugs, developed primarily with federal funds, at high launch prices even when spared considerable research and development costs.

For example, the federal government spent \$484 million developing the cancer drug Taxol, which was then marketed under an agreement with Bristol-Myers Squibb starting in 1993. In 10 years, the manufacturer earned \$9 billion in revenue and paid the federal government \$35 million in royalties.²²

Nearly half of basic research is funded through federal government investments, with roughly 75 percent of new innovative drugs supported by federal funding.^{23,24} This system of public investment has raised concerns that the economies of developing drugs with government grants are not being passed on to consumers and payers—and that needed medications are not being made accessible to all patients for a reasonable price.

Xtandi, a prostate cancer drug, is an example of how U.S. patients are not benefiting from federal drug development funding. The University of California, Los Angeles, discovered Xtandi and was granted three patents. These patents would not have been possible without funding provided by the NIH and the Department of Defense. Eventually, the three patents were licensed to a private manufacturer, Medivation, which pursued and was granted FDA approval. Today, the average U.S. wholesale price of Xtandi is more than \$129,000 a year. Other developed countries pay no more than half this price.²⁵

Actions That Would Have Direct Impact on Launch Prices and Annual Increases

1. *Alter patent protections and market exclusivities to introduce price competition, with the goal of reducing patented brand-name drug prices.*
 - a. Shorten the market-exclusivity period for brand-name biologics from 12 to seven years (or something more on par with the small-molecule drug exclusivity periods). Some experts believe that a lengthy market-exclusivity period discourages manufacturers from developing biosimilars. The U.S. is the only country that allows a 12-year exclusivity period. Biologics cannot be patented at this time, but their manufacturing processes can be. Most biologics sold in the U.S. do not have a generic alternative, and that is attributed to the exclusivity period.
 - b. Reduce or eliminate patent extensions that were created under the Hatch-Waxman Act to rebalance innovation incentives with competition. A more focused strategy would be to eliminate patent extensions only for drugs that are clinically comparable to a drug on the market but that have no added comparative value.
 - c. Terminate market-exclusivity protections before they would naturally expire when manufacturers recoup a multiple of their research and development investment. Instead of basing market exclusivity solely on a length of time, this proposal would tie market exclusivity to a proxy to ensure that manufacturers adequately recoup their investment and earn compensation.
 - d. Eliminate or reform provisions of the Hatch-Waxman Act that delay the introduction of generic products. These provisions include the 30-month delay in the first generic approval, in the case of patent infringement challenges by the brand-name company, and the 180-day exclusivity period for the first generic approved before a second generic can be approved.
 - e. Eliminate the six-month market exclusivity for conducting pediatric trials or substitute alternative incentives. The current exclusivity protects all approved indications of the drug and not just the pediatric indication. While this incentive has encouraged more pediatric trials and useful changes to labels, the compensation to manufacturers is unrelated to the cost of the trials. One alternative to the market exclusivity could be tax credits to offset clinical trial costs. Another alternative is to require pediatric trials without an incentive.
 - f. Expand FDA authority, notwithstanding patent or market-exclusivity protection, to permit importation or reimportation when a drug becomes inaccessible due to its high price or short supply and is available at a lower cost in another country. Under certain conditions, the FDA would authorize importation or reimportation only from countries recognized for having comparable drug-approval systems and adequate safety controls in place in their distribution systems.
2. *Require reasonable pricing of patented brand-name drugs when there are federally funded investments in the development of these drugs, so that the public benefits from any tax dollars devoted to drug research.*
 - a. Use the current authority in the Bayh-Dole Act, or amend the act's provisions, to establish allowable

constraints on the prices that manufacturers can charge or require manufacturers to enter into a contract for government manufacturing. The 1980 Bayh-Dole Act gives the government certain patent rights for inventions arising from federally funded research and development.²⁶ Under the law, certain rights are reserved for the government to protect the public's interest. These rights include a license for the government to make and use a drug or to have another entity do so on the government's behalf. When prices are unreasonable, the federal government could use Bayh-Dole provisions to manufacture the drug or have it manufactured on the government's behalf.

3. Alter how federal and state government programs purchase patented brand-name drugs for the purpose of lowering prices and increasing access for patients.

- a. Authorize Medicare to negotiate Part D drug prices with manufacturers directly, potentially using established prices. Negotiations could follow various models, including:
 - Establish a drug's ceiling price in a manner similar to that used by the Federal Ceiling Price program to purchase drugs for the Department of Veterans Affairs (VA), the Department of Defense, the Public Health Service, and the Coast Guard.
 - Identify default "fall back" prices if the manufacturer and the Department of Health and Human Services (HHS) are unable to come to agreement on price. Fall-back prices could potentially be based on VA-negotiated prices in the federal supply schedule or average prices paid by Organisation of Economic Co-operation and Development (OECD) member countries.
 - Settle negotiation disputes via binding arbitration if the manufacturer and HHS are unable to come to agreement on price during an allotted period.
 - Limit negotiations to specialty-tier drugs or other drugs that meet certain criteria, such as drugs that exceed a certain threshold for patient cost-sharing or that have excessive price increases.

- Create a Medicare-sponsored Part D plan that would serve as a public option to compete directly with private Part D plans.
- Use reference pricing, which would permit HHS to set a benchmark price for clinically comparable drugs that are interchangeable.

Several factors need to be considered when determining how to implement these models. First, any of these models could be scalable, meaning that they could be tested through demonstration projects for a subset of drugs and then expanded more broadly. Second, the scope of the models could vary, from including all drugs under the Part D benefit to focusing only on a small category of drugs based on certain criteria, such as price, spending thresholds, utilization, or potential for improved outcomes. Third, model implementation could be either voluntary or mandatory. Under the former approach, HHS could solicit proposals from manufacturers, negotiate agreements, and offer those to Part D plan sponsors on a voluntary basis.

Another factor is the role that HHS would play in negotiations. HHS could directly negotiate, or responsibility could be delegated to an independent entity.

A final factor is whether Medicare would exclude any drugs from the formulary if a satisfactory price is not agreed on. Currently, Medicare Part D plans may impose restrictions, such as utilization management or prior authorization, on formulary drugs but must cover the six protected classes of drugs required by law.

- b. Align Medicare and Medicaid drug prices for dual-eligibles, or low-income Medicare beneficiaries who are also eligible for Medicaid. Prior to Part D Medicare drug coverage, dual-eligible beneficiaries received prescription coverage through the Medicaid Drug Rebate Program. Now these beneficiaries access drug benefits through commercial Medicare Part D plans. There is a substantial difference in the prices paid for brand-

- name drugs under the two programs, with Medicare paying higher prices than Medicaid. Under this model, Medicare would pay prices at least as low as those Medicaid pays.
- c. Require inflation-based limits on price increases for drugs purchased through Medicare Part B and/or Part D. Currently, Medicaid receives lower prices for drugs than Medicare. This is partly attributed to Medicaid's limits on price increases that are tied to inflation. Instituting a similar inflation cap in Medicare could lead to moderate price increases and potentially generate savings. Under this proposal, manufacturers would be allowed to increase annual prices only at or below inflation; if prices increased above inflation, then Medicare would receive additional rebates.
 - d. Apply best-price provisions, currently used by Medicaid, as a model for all other federal health programs, potentially using prices in other countries as a reference. This would allow drug prices to be based on the best price in any country with a comparable standard of living.
 - e. Establish purchasing pools among some or all public payers. This could include pooling drug purchasing for federal health programs or creating a federal-state Medicaid purchasing pool. Under both models, the purchasing pool could be designated as a PBM. Several proposals describe approaches to federal-state collaboration that would pool purchasing power among public health programs. One model expands the Department of Defense Pharmacy Benefit Program to create a governmentwide PBM for all federal health programs.
 - f. Establish alternative government purchasing programs for drugs that protect public health, such as vaccines and drugs that prevent and limit the spread of serious infections.
 - The federal government could contract for the bulk purchase of certain drugs directly from manufacturers to expand access and limit annual price increases. One successful model to replicate is the Vaccines for Children (VFC) program.
 - The federal government could expand or authorize programs similar to the Ryan White AIDS Drug Assistance Program (ADAP) to expand access and ensure affordability. ADAP is a state- and territory-administered program that provides FDA-approved medications to low-income people living with HIV who have limited or no health coverage. A similar program could be authorized for treatment of other conditions, such as hepatitis C.
 - The federal government could negotiate a bulk purchase price and make the drug available to public payers, similar to how it purchases naloxone, which counteracts opioid overdoses. Manufacturers benefit by gaining access to a large patient population. The government could exercise its authority under 28 U.S.C. 1498, which grants federal rights for use. Or it could exercise its authority under 28 U.S.C. 1498, which permits the government to infringe patents without being subject to an injunction, as long as it pays reasonable compensation to the patent owner.
4. *Alter how government programs purchase patented brand-name drugs with the purpose of tying purchases to improved clinical value or health outcomes.*
- a. Authorize Medicare Part B and Part D to negotiate drug prices using alternative purchasing models. Current federal law prohibits Medicare Part D plan sponsors from excluding drugs from coverage and restricts the ability to test value-based purchasing strategies to address the high cost of drugs. Negotiation strategies that consider clinical value, known as value frameworks or value-based purchasing, take many models and structures, including:
 - Use of a payment bundle, in which HHS would combine payment for health services and drugs into a single per treatment rate.
 - Adoption of outcomes-based pricing, which would permit HHS and a manufacturer to enter into risk-sharing agreements that link patient outcomes to payments.

- Adoption of indications-based pricing, which would allow HHS to set a price based on the comparative effectiveness of the drug for different diseases.
- Use of reference pricing, which would enable HHS to set a benchmark price for clinically comparable drugs and determine which drugs are interchangeable.
- Adoption of drug coverage tied to evidence development, which would permit HHS to make coverage conditioned on the collection of additional population-level evidence, possibly from a pre-specified study to support continued, expanded, or withdrawal of coverage.

HHS could develop these frameworks in partnership with a contractor or, an independently established panel of experts could develop them. The agency could use the frameworks in isolation or in combination, and it could vary which frameworks to use based on the drug or condition addressed. In addition, the considerations related to Medicare negotiating Part D drug prices (highlighted above), are also applicable in designing these alternative purchasing models.

- Authorize, by legislation, a least-cost alternative model for drugs covered under Medicare Part B. Under a least-cost alternative policy, Medicare would not pay the additional cost of a more expensive drug when a clinically comparable, lower-cost drug is available. However, a beneficiary could continue treatment with a higher-priced drug by choosing to pay the additional cost.
- Apply the national coverage determination (NCD) process to Medicare Part B, which reimburses for all FDA-approved physician-administered drugs. Through the NCD process, Medicare can choose to evaluate whether an item or service is “reasonable and necessary” and can choose not to cover it if the benefits do not outweigh potential harms. If the NCD process were applied to Part B prescription drugs, Medicare might make step therapy a coverage requirement. When clinically comparable drugs are

available, physicians and patients would be required to try the lesser-priced options before moving to more-expensive ones.

Actions That Would Have Indirect Impact on Launch Prices and Annual Increases

5. Institute changes to protect patient access and affordability, in the absence of action to directly moderate prices.

While these actions insulate patients from high prices, some might also insulate manufacturers from criticism for their high prices. This would enable drug companies to set prices even higher in the absence of direct competitors for their products.

- Accelerate closing of the Medicare Part D coverage gap or “doughnut hole.” Prior to the ACA, Medicare beneficiaries were responsible for the full cost of their medications while in the Medicare Part D coverage gap. Currently, beneficiaries in the doughnut hole receive a 50 percent discount from manufacturers on brand-name drugs. One proposal to accelerate the closing of the coverage gap is to increase manufacturer discounts to 75 percent, effectively closing the gap for brand-name drugs earlier than under current law.
- Limit out-of-pocket cost-sharing for prescription drugs, so that patients are less likely to fall into medical debt paying for drugs. Many states have acted to limit out-of-pocket maximums for prescription benefits in the individual market, ranging from \$1,250 to \$3,500 per year. These limits apply after a patient meets a prescription drug deductible and are usually implemented through monthly caps on spending.
- Require insurance coverage for the first dollar of certain prescription drug costs—for example, those for preventive medicines or drugs for managing chronic diseases.
- For public payers, reduce or waive cost-sharing for certain drugs based on comparative-effectiveness research. Cost-sharing based on therapeutic value may help to shift utilization from less-effective

drugs to those of higher value. This proposal may indirectly affect the prices of drugs considered to be less effective but with arbitrarily high prices.

6. Align incentives and payment policies that could increase the ability of public payers to improve medical utilization of patented brand-name drugs.

- a. Increase Medicare Part D plan sponsors' responsibility for covering catastrophic drug expenses to encourage prudent purchasing and management of high-cost drugs. The federal government currently covers 80 percent of the costs of catastrophic drug spending, with the Part D plan sponsor assuming 15 percent of the expenses and the beneficiary responsible for 5 percent. The federal government coverage shields Part D plan sponsors from high costs and serves as a disincentive for sponsors to negotiate aggressively with manufacturers. With the goal of creating an incentive to lower drug prices, this action increases the financial risk for Part D Plan sponsors by decreasing the federal government's reinsurance of catastrophic costs from 80 percent to a lower percentage.
- b. Align payment with the most commonly used dosage. Many drugs are packaged in sizes that are greater than the most commonly used dosage, resulting in waste. This proposal requires manufacturers to package drugs that are reimbursed under Medicaid and Medicare in the most common dosage or face a reduced reimbursement.

7. Ensure the availability of comparative-effectiveness information on patented brand-name drugs for patients, providers, and payers to empower shared decision-making on treatment options.

- a. Build prescriber education and clinical decision-support tools that make comparative-effectiveness information available to patients and providers at the point of care.
- b. Authorize the Patient-Centered Outcomes Research Institute (PCORI) or a federal agency to conduct comparative-effectiveness research that incorporates price information.

- c. Authorize and appropriate federal funding for comparative-effectiveness research.
- d. Incentivize or require manufacturers to submit comparative-effectiveness research to FDA as part of their drug-approval applications or post-approval.
- e. Require a manufacturer awarded the three-year New Clinical Investigation Exclusivity incentive to demonstrate significant clinical benefit over existing therapies manufactured by the applicant in the five-year period preceding submission of the application.

8. Ensure availability of price information on patented brand-name drugs that enables informed and prudent purchasing for patients, providers, and payers.

- a. Require transparency in drug pricing and in price increases for patients, providers, and payers.
- b. Eliminate practices that obscure pricing to encourage the industry to engage in more straightforward, open-pricing practices.
- c. Build prescriber education and clinical decision-support tools that promote the availability of price information for patients and providers at the point of care.

9. Ensure availability of other information that enables informed and prudent use of patented brand-name drugs by patients, providers, and payers.

- a. Publish information on the federal government's investments into the research and development for all drugs.
- b. Proactively share information on the drug development pipeline that would be beneficial to payers, including potential pricing of the drug product.
- c. Restrict or eliminate direct-to-consumer advertising. This can be accomplished by changing how the FDA regulates advertising to prohibit the use of misleading information. In addition, changes to the tax code could reduce or eliminate the tax deduction for a manufacturer's advertising expenses.

Problem 2: Brand-Name Orphan Drugs Have High Launch Prices and High Annual Price Increases

Drivers: Multiple Orphan Drug Exclusivities and Repurposing of Off-Patent Drugs

During the past decade, manufacturers have significantly increased their use of the Orphan Drug Act's incentives.

The Orphan Drug Act was enacted to provide incentives for the development of drugs for rare diseases or conditions—those affecting fewer than 200,000 people. For each rare disease or condition for which a drug is approved, the manufacturer receives a research and development tax credit and, most importantly, a seven-year period of market exclusivity. In some circumstances, manufacturers of orphan drugs also receive a priority review voucher, which allows the manufacturer to have another of its new drugs reviewed under the FDA's priority review system. During this period, the manufacturer has total discretion in setting prices. The only price competition allowed is from clinically comparable orphan drugs, which occurs infrequently.

The number of orphan drugs on the market has grown steadily in the past 10 years. Of the nearly 600 drugs approved with orphan indications since 1983, nearly half (289) were approved between 2007 and 2016.²⁷ Even though the focus of the Orphan Drug Act is rare diseases and conditions, seven of the top 10 best-selling drugs in the U.S. are approved for at least one orphan disease.²⁸

Prices of orphan drugs have also dramatically increased. A recent study found that the median launch price of orphan drugs has doubled every five years since 1983. The inflation-adjusted median cost per patient per year at market entry for orphan drugs increased from \$1,573 between 1983 and 1984 to \$100,555 between 2010 and 2014, representing a 64-fold increase when adjusted for inflation.^{29,30} Between 2012 and 2014, the prices of 45 orphan drugs increased 30 percent on average.³¹

Manufacturers are obtaining multiple approvals for orphan-drug indications for existing products and increasing prices for the new indication as well as for all previous indications. Under the statute, manufacturers can split a disease into several sub-diseases, which each qualify as a rare disease. Approximately one in four

orphan drugs have multiple orphan-drug approvals, resulting in exclusivity periods that, in many cases, overlap.³² By obtaining as many market-exclusivity periods as possible for a product, the manufacturers monopolize the market, severely hindering the ability of manufacturers to introduce brand-name and generic competitors.³³

For example, the FDA initially approved Humira in 2002 for rheumatoid arthritis. Since 2008, the product has received five orphan-drug approvals. The first, in 2008, was for treatment of juvenile rheumatoid arthritis in patients age 4 and older. In 2014, Humira was approved for treatment of juvenile rheumatoid arthritis in patients ages 2 to 4. The latest orphan-drug approval for Humira was granted in 2016. There are currently no generics available.

Manufacturers repurpose older drugs originally developed for non-orphan diseases as treatments for orphan diseases and increase the price of the drug for all indications. More than 70 orphan drugs were initially approved for non-orphan diseases.³⁴ The older drug's approval for the orphan drug seven-year market-exclusivity period permits the manufacturer to increase the price of the drug regardless of whether it is for the older indications or the new orphan indication.³⁵

In one example, H.P. ActharGel (corticotropin) received orphan drug approval for treatment of infantile spasms, with a price increase of more than \$20,000 per vial.³⁶ Since the 1950s, corticotropin has been used for treatment of several other diseases, including multiple sclerosis, arthritis, and inflammatory conditions of the eye, and it was used off-label as a treatment for infantile spasms. Typically, one vial cost \$1,650 before it received orphan-drug approval. Now the higher price is applied for all uses, including off-label and prior approvals.

Another example is hydroxyprogesterone caproate, which was originally approved by the FDA in 1956 and widely used for decades to prevent miscarriages and gynecological disorders. The manufacturers withdrew the drug from the market for commercial purposes in 1999, and manufacturing then shifted to compounding pharmacies. In 2003, a study by NIH showed that hydroxyprogesterone caproate was effective

in preventing preterm birth in women with at-risk pregnancies. KV Pharmaceutical conducted a follow-up clinical trial and received FDA approval in 2011 for hydroxyprogesterone caproate, branded as Makena, as an orphan drug to reduce the risk of premature birth prior to 37-week gestation for women with a single fetus who had at least one previous premature birth. Makena was introduced to the market with a significant increase over what hydroxyprogesterone caproate was previously priced at, from \$15 to \$1,500 per dose, with a typical treatment regimen priced at \$25,000.³⁷

Actions That Would Have Direct Impact on Orphan-Drug Pricing

1. *Alter patent protections and market exclusivities to introduce price competition, with the goal of reducing orphan drug prices.*
 - a. Require multiple orphan designations on the same drug product to run simultaneously or permit a manufacturer to only receive a single market-exclusivity period for a product's first orphan drug indication.
 - b. Terminate market exclusivity once a manufacturer recoups a multiple of its research and development investment. Instead of basing the market-exclusivity period solely on duration, this proposal would tie market exclusivity to a proxy to ensure manufacturers adequately recoup their investment and earn a return.
 - c. Prohibit off-patent drugs from gaining market exclusivity under the Orphan Drug Act, while still allowing manufacturers to access other incentives, such as the research-and-development tax credit.
2. *Alter how federal and state government programs purchase orphan drugs, to lower prices and increase access for patients.*
 - a. Authorize Medicare to negotiate Part D drug prices with manufacturers directly. See problem 1, action 3a, on page 13 for details.
 - b. Align Medicare and Medicaid drug prices for dual-eligibles so that Medicare pays at least as low a price as Medicaid does. See problem 1, action 3b, on page 13 for further explanation.
 - c. Require inflation-based pricing limits for drugs purchased through Medicare Part B and/or Part D. Currently, the Medicaid program receives lower prices for drugs than Medicare. See problem 1, action 3(c), on page 14 for further explanation.
 - d. Apply best-price provisions to all other federal health programs, potentially using prices in other countries as a reference. See problem 1, action 3(d), on page 14, for further explanation.
 - e. Establish purchasing pools among some or all public payers. See problem 1, action 3(e), on page 14 for further explanation.
 - f. Establish alternative government purchasing programs for drugs that protect public health. See problem 1, action 3(f), on page 14 for further explanation.
3. *Alter how government programs purchase orphan drugs, for the purpose of tying purchases to improved clinical value or health outcomes.*
 - a. Authorize Medicare Part B and Part D to negotiate drug prices using alternative purchasing models that take clinical value into consideration. See problem 1, action 4(a), on page 14 for further explanation.
 - b. Authorize, by legislation, a least-cost alternative model for drugs covered under Medicare Part B. Under such a policy, Medicare would not pay the additional cost of a more expensive drug when a clinically comparable, lower-cost drug is available. However, a beneficiary could continue treatment with a higher-priced drug by choosing to pay the additional cost.
 - c. Apply the national coverage determination (NCD) process in Medicare Part B to lower spending on FDA-approved physician-administered drugs. See problem 1, action 4(c), on page 15 for further explanation.

4. Require reasonable pricing when there are federal investments in the development of orphan drugs so that the public benefits from tax dollars devoted to drug research.

- a. Use the current authority granted in the Bayh-Dole Act, or amend the Bayh-Dole provisions, to establish allowable constraints on the prices that manufacturers can charge or require manufacturers to enter into a contract for government manufacturing. See problem 1, action 2(a), on page 13 for further explanation.

Actions That Would Have Indirect Impact on Orphan-Drug Pricing

5. Require additional information when companies seek orphan drug status.

- a. Require manufacturers to provide information about any additional orphan drug indications the company intends to seek approval for, as well as drug utilization data on both orphan and non-orphan indications. The purpose of collecting this information is to ensure that a manufacturer is not stratifying diseases and misusing the incentives provided under the Orphan Drug Act.

6. Restrict use of the orphan drug tax credit or replace with a grant-and-access pathway.

- a. Instead of guaranteeing manufacturers a tax credit for the clinical trial costs of orphan drugs, a grant-and-access pathway would award manufacturers grants to cover clinical trial expenses, assuming the manufacturers agree to price caps and a target rate of return on orphan drugs. This proposal is a variation on a rate-on-return model, which dictates a specified gain on an investment over a specified period. Receiving grants to cover clinical trial costs may be more financially attractive to manufacturers than a tax credit.

7. Institute changes to protect patient access and affordability, in the absence of action to directly moderate prices.

See problem 1, action 5 (a-d), on pages 15–16, for details on the following proposals:

- a. Accelerate closing of the Medicare Part D coverage gap.
- b. Limit out-of-pocket cost sharing for prescription drugs so that patients are less likely to fall into medical debt paying for prescription drugs.
- c. Require first-dollar coverage for certain prescription drug coverage in private insurance, such as that for preventive medicines or drugs to manage certain chronic diseases.
- d. For public payers, reduce or waive cost sharing for certain drugs based on comparative-effectiveness research.

8. Align incentives and payment policies that could increase the ability of public payers to improve medical utilization with orphan drugs.

- a. Increase Medicare Part D plan sponsors' responsibility for covering catastrophic drug expenses to encourage prudent purchasing and management of high-cost drugs. See problem 1, action item 6(a), page 16 for further explanation.
- b. Align payment with the most commonly used dosage. Many drugs are packaged in sizes that are greater than the most commonly used dosage, resulting in waste. This proposal would require manufacturers to package their drugs that are reimbursed under Medicaid and Medicare in the most common dosage or face a reduced reimbursement.

9. Ensure the availability of comparative-effectiveness information for patients, providers, and payers to empower shared decision-making on treatment options with orphan drugs.

See problem 1, action item 7(a-e), on page 16 for specific actions steps and details.

10. Ensure availability of price information on orphan drugs that enables informed and prudent purchasing for patients, providers, and payers.

See problem 1, action item 8(a-c), on page 16 for specific actions steps and details.

Problem 3: Some Manufacturers Create, or Take Advantage of, Natural Monopolies for Drugs That Enable Them to Significantly Increase Prices

Drivers: Lack of Competition and Monopolies

Because of manufacturers' actions, as well as market forces, there are currently 182 drugs that no longer have patent protection or any associated generics.

Furthermore, there are more than 500 patented drugs with only one marketed generic.³⁸ These natural monopolies create the opportunity for manufacturers to maintain patent-era pricing or engage in price gouging.

Monopolies may occur naturally or be forced. A manufacturer can obtain a natural monopoly with an older drug when other companies making the same drug withdraw from the market for commercial reasons, manufacturing difficulties, or safety reasons. Alternatively, a manufacturer may merge with or acquire other companies that made a drug, decreasing competition.

This problem has been explored in depth by the U.S. Senate Special Committee on Aging. The committee specifically found that drug manufacturers consider five strategies to ensure or create monopolies:³⁹

- Acquire a sole-source drug, meaning there is only one manufacturer for the drug.
- Ensure that the drug acquired is the gold standard, or the most effective or best-regarded treatment for a specific condition.
- Choose drugs for which there is a small market, or a relatively few patients and few competitors, if any at all. Because of their small numbers, consumers would have trouble organizing against high drug prices.
- Limit access to the drug by establishing a closed distribution system. This means that the drug is accessible only through certain channels, such as specialty pharmacies or certain distributors, thus limiting access not only for patients but for competitors as well.
- Increase prices astronomically to maximize profits.

Manufacturers such as Turing, Retrophin, Rodelis, and Valeant eliminated any opportunity for competition and established themselves as the one provider of life-saving medications. These manufacturers then increased prices, and the lack of competition left patients nowhere else to turn.

Actions That Would Have Direct Impact on Sole-Source Drug Pricing

1. *Provide targeted or narrow incentives that the FDA can implement to generate competition for sole-source drugs.*
 - a. Authorize the use of voucher programs, or FDA assistance with abbreviated new drug applications (ANDAs), or awards to encourage manufacturers to enter sole-source markets. These incentives could be limited to manufacturers that do not already have the 180 days of market exclusivity for being the first approved generic.
 - b. Increase competition by waiving fees and instituting a priority or expedited review for manufacturers that are the second and third entrants in generic markets. This provision aims to provide financial assistance to companies that take on the burden of entering a generic, sole-source market. The FDA could stop providing priority reviews or waiving fees once a drug has sufficient competitors.
2. *Provide authority for government intervention if a sole-source drug becomes inaccessible as a result of unaffordable prices.*
 - a. Permit drug importation or re-importation of a drug when there is a sole-source market in the U.S. Currently, importation is prohibited, except for personal use. Importation could create competition for generic sole-source drugs that are priced exorbitantly high.
 - b. Use current government contracting authority (28 U.S.C. 1498) to bulk purchase drugs when a sole-source drug has become unaffordable. The federal government also has authority (28 U.S.C. 1498) to infringe patents without being subject to an injunction, although the government would be

required to pay reasonable compensation to the patent owner for use by the federal government. Some states currently are using a similar strategy to address the opioid crisis. The state government leverages its authority to negotiate the bulk-purchase price of naloxone, the drug that reverses an overdose. The federal government could use this same strategy if a sole-source generic drug incurs an unaffordable price hike for a necessary medication. Alternatively, the Federal Supply System (a program that supports the health care acquisition requirements, including prescription drugs, of the Veterans Health Administration and other federal agencies) could be authorized to induce new manufacturers to enter the market by agreeing to long-term contracts for drugs once they have received FDA approval.

- c. Establish alternative government purchasing programs for drugs that protect public health. The federal government could: 1) centralize contracting and bulk-purchase certain drugs for public health, similar to how successful programs like Vaccines for Children operate; or 2) expand or authorize programs like the Ryan White AIDS Drug Assistance Program.

3. Alter how federal and state government programs purchase sole-source drugs for the purpose of lowering prices and increasing access for patients.

- a. Authorize Medicare to negotiate Part D drug prices with manufacturers directly. See problem 1, action 3a, on page 13 for details.
- b. Align Medicare and Medicaid drug prices for dual-eligibles so that Medicare pays at least as low a price as Medicaid. See problem 1, action 3b, on page 13 for further explanation.
- c. Require inflation-based pricing limits for drugs purchased through Medicare Part B and/or Part D. Currently, the Medicaid program receives lower prices for drugs than Medicare. See problem 1, action 3(c), on page 14, for further explanation.
- d. Apply best-price provisions to all other federal health programs, potentially using prices in other

countries as a reference. See problem 1, action 3(d), on page 14 for further explanation.

- e. Establish purchasing pools among some or all public payers. See problem 1, action 3(e), on page 14 for further explanation.

Actions That Would Have Indirect Impact on Sole-Source Drug Pricing

4. Provide authority for government monitoring and oversight of pharmaceutical markets that could become concentrated.

- a. Give the FDA responsibility to monitor markets to identify where a monopoly may develop. This might include maintaining a public list of generic drugs and their manufacturers (including distributors, labelers, and compounders), so the FDA can more quickly identify drugs at risk of shortage or drugs with a limited number of competitors. Generic drug manufacturers could also be required to report a discontinuance or interruption in the production of a drug at least 180 days prior to the event or as soon as practicable.
- b. Provide public notice when a market for a drug has only two or fewer manufacturers.
- c. Require additional FDA reporting about generic drug applications and the backlog. The FDA could provide more transparency to give manufacturers better insight into when their product might be approved and how many competitors they may face.
- d. Require the Federal Trade Commission (FTC) and the Department of Justice (DOJ) to regularly review markets and any potential anticompetitive behavior that affects drug prices.

5. Institute changes to protect patient access and affordability, in the absence of action to directly moderate prices

See problem 1, action 5 (a-c), on page 15 for details on the following proposals:

- a. Accelerate closing of the Medicare Part D coverage gap.

- b. Limit out-of-pocket cost sharing for prescription drugs so that patients are less likely to fall into medical debt paying for prescription drugs.
 - c. Require first-dollar coverage for certain prescription drug coverage in private insurance, such as for preventive medicines or drugs for managing certain chronic diseases.
6. *Align incentives and payment policies that could increase the ability of public payers to improve medical utilization of sole-source drugs.*
- a. Increase Medicare Part D plan sponsors' responsibility for covering catastrophic drug expenses to encourage prudent purchasing and management of high cost drugs. See problem 1, action item 6(a), page 16 for further explanation.

Problem 4: The Lack of Robust Competition Among Generic Drug Manufacturers Results in Less Price Competition and Higher Prices

Drivers: Blocking Access to Samples; Mergers and Acquisitions; Market Exits; and Delayed Market Entry

More than 500 drugs have only one marketed generic; this lack of competition keeps prices high. In 2016, the FDA approved 630 abbreviated new drug applications and tentatively approved 183—the highest number of generic drug approvals and tentative approvals in the history of the generic drug program.⁴⁰ Despite the existence of competition, approximately 22 percent of the top 200 generics drugs had price increases that exceeded inflation between 2005 and 2014.⁴¹

More than 180 off-patent drugs are currently without generic competition, which some experts attribute to a low return on investment in the generic market.⁴² In some cases, generic markets for drugs within a certain class may be small and unable to support multiple competitors. A recent analysis of Medicaid claims data showed that the generics with the largest price increases were those for which Medicaid spending was low.

Brand-name manufacturers misuse Risk Evaluation Mitigation Strategies (REMS) to block potential competitors from obtaining samples needed to conduct

bioequivalence studies, a key step in developing a generic drug. The FDA requires some manufacturers to adhere to a risk-mitigation strategy for safety reasons, but manufacturers may also voluntarily introduce one. Nearly half of drugs with REMS have limited distribution, which ultimately restricts access for generic manufacturers.⁴³ Without access to samples of brand-name products, generic manufacturers cannot conduct bioequivalence testing, which is required for FDA approval of a generic. Analysis suggests that curbing the abuses of REMS could result in savings of \$2.4 billion over 10 years through the introduction of more generics to the market.⁴⁴

Mergers and acquisitions among manufacturers have led to more-concentrated, less-competitive markets. For instance, Valeant Pharmaceuticals' acquisition strategy acquired 100 companies, and part of this strategy was to increase prices as the market was consolidated.⁴⁵ Valeant is not the only company with such a business strategy. It is expected that mergers will continue to increase.⁴⁶

Drug shortages can cause spikes in drug prices. For example, doxycycline, a commonly prescribed antibiotic, is available from multiple manufacturers. However, in 2013, a supply disruption occurred, leading to a shortage of the drug. The retail price for doxycycline increased more than 1,900 percent during the shortage.⁴⁷

Market exits reduce competition in the generic market. Market exits may be temporary, such as those required to fix drug-safety or manufacturing issues. Or they may be permanent, perhaps resulting from low profits. Either way, market exits limit competition and cause significant price increases. For instance, digoxin tablets are relatively easy to produce and have been used for decades to treat high blood pressure. But limited market competition is driving the cost of the drug up, and patients are facing higher cost-sharing.⁴⁸

Market exclusivities provided by the Federal Food, Drug and Cosmetic Act and current patent laws delay generic price competition, allowing brand-name drugs to maintain high prices. In recent years, asthma inhalers containing albuterol were able to get new patents because of a ban on chlorofluorocarbons (CFCs). Replacing CFC inhalers with newly patented

hydrofluoralkane (HFA) inhalers led to price increases by approximately 400 percent, from \$13 to \$50 per inhaler.⁴⁹

Actions That Would Have Direct Impact on Generic-Drug Pricing

1. Provide targeted or narrow incentives that the FDA can implement to generate competition among generic drugs.

- a. Employ the use of voucher programs or awards to encourage generic manufacturers to enter a sole-source market or offer products to relieve drug shortages. These incentives could be limited to manufacturers that do not already have 180 days of exclusivity for first generics.
- b. Increase competition by waiving fees and instituting priority or expedited review for generic manufacturers that are second and third entrants into generic markets. This provision aims to provide financial assistance to manufacturers that take on the burden of entering a generic sole-source market. The FDA could stop providing priority reviews or waiving fees once a drug has sufficient competitors.

2. Provide authority for government intervention if a generic drug becomes inaccessible because of unaffordable prices.

- a. Permit importation or reimportation of a drug when there is a sole-source market in the U.S. Currently, importation is prohibited, except for limited instances of personal use. However, importation could create competition for a sole-source drug that is priced exorbitantly high in the U.S.
- b. Provide the FDA with responsibility to monitor markets to identify where a monopoly may develop.
- c. Provide public notice when a market for a drug includes only two or fewer manufacturers.
- d. Use current government contracting authority (28 U.S.C. 1498) to bulk purchase drugs when a sole-source drug has become unaffordable. See problem 3, action 2(b), on page 21 for further explanation.
- e. Require additional FDA reporting about generic drug applications and the backlog. The FDA could provide more transparency to give manufacturers

better insight into when their products might be approved and how many competitors they may face.

- f. Require the FTC and the DOJ to regularly review markets and any potential anticompetitive behavior that affects drug prices.

3. Alter patent protections and market exclusivities to encourage the introduction of generics and price competition with the goal of reducing drug prices.

- a. Reduce or eliminate the patent extensions that were created under the Hatch-Waxman Act to rebalance innovation incentives with competition. A focused approach might only eliminate patent extensions for drugs that are clinically comparable to a drug on the market but have no added comparative value.
- b. Eliminate the provisions under the Hatch-Waxman Act that delay introduction of generic products, including the 30-month delay in generic approval in the case of patent infringement challenges by the brand-name company and the 180-day exclusivity period for the first generic approved.
- c. Eliminate the six-month market exclusivity for conducting pediatric trials or substitute alternative incentives. The current six-month market exclusivity protects all approved indications of the drug, not just the pediatric indication. While this incentive has encouraged more pediatric trials and useful changes to labels, the compensation to the manufacturers is unrelated to the cost of the trials. An alternative to the market exclusivity could be tax credits to offset clinical trial costs.
- d. Modify the five-year new chemical entity (NCE) patent extension period so that the FDA can accept generic applications for the brand-name product after three years. The brand-name manufacturer would still maintain market exclusivity for five years, but the FDA would complete its review of the generic before the end of the five-year period.

4. Alter how Medicare reimburses for generic drugs in Part B.

- a. Encourage the use of generic alternatives in Medicare, when available. Authorize, by legislation,

a least-cost-alternative model for drugs covered under Medicare Part B. Under such a policy, Medicare would not pay the additional cost of a more expensive drug when a clinically comparable, lower-cost drug is available. However, a beneficiary could continue treatment with a higher-priced drug by choosing to pay the additional cost.

- b. Alter the provider payment structure for drugs under Part B to provide less financial incentive for providers to prescribe high-cost drugs over lower-cost alternatives. Currently, Medicare pays providers the average sales price of the drug, plus 6 percent to cover administrative costs. Medicare might pay a flat fee for generics in lieu of the administrative load.

Actions That Would Have Indirect Impact on Generic-Drug Pricing

5. Provide additional resources to fully fund FDA activities to review generic drug applications.

- a. The FDA's jurisdiction of products and activities is broad. The challenges to securing the safety of these products increases in complexity with a growing global market. To increase competition in the generic drug market, additional resources are necessary to continue the record number of reviews and approvals of generic drug applications. The FDA is making strides to improve the efficiency of the generic drug review process, and additional resources are needed to enhance access to highquality, lower-cost generics.

6. Require manufacturers to provide information necessary for the development of generic drugs.

- a. Require brand-name manufacturers to promptly disclose all patents on drug approval, including process and manufacturing patents, that a generic manufacturer may infringe or violate in developing a generic version.
- b. Require manufacturers to provide samples of drugs with REMS to generic manufacturers.

Problem 5: Lack of Price Competition Among Biologics and Biosimilars Results in Higher Prices

Drivers: Lack of Competition, Lack of Regulatory Guidance, and Discouraged Use

U.S. spending on biologic drugs has risen considerably over the past several years. In 2016, \$105.5 billion was spent on biologics, with some drugs having annual costs of \$250,000 per patient. However, because of the lack of a fully developed regulatory framework, very few biosimilars have been introduced.^{50,51,52,53} Without action, we expect this trend to continue. Studies have projected that the U.S. health care system would save as much as \$250 billion in revenue over a 10-year period (2014–2024) if 11 existing biosimilars were able to successfully enter the market.⁵⁴

The passage of the Biologics Price Competition and Innovation Act in 2010 set a landmark precedent by creating a pathway for biosimilar entry. However, the FDA is still in the developing stages of building and implementing this pathway for approval. In the mean time, the U.S. lags the rest of the world in bringing biosimilars to market. Biosimilars have been on the market in Europe since 2006, and they are common in China, India, and South Korea.

The FDA has approved four biosimilars, with the first approved in 2015. However, only two are currently on the market. These two are alternatives to Neupogen and have prices roughly 15 percent lower than Neupogen's.⁵⁵ In comparison, Europe has 23 biosimilars on the market for nine "originator reference" biologics and has experienced significant price decreases.^{56,57}

Biosimilars face similar barriers as small-molecule generic drug products, such as labeling issues, misuse of REMS, and patent challenges, which limit access to or discourage manufacturers from entering the market.

There are many outstanding issues that stand in the way of the FDA completing implementation of the biosimilar pathway. As a result, the biosimilar market is not as robust as it could be. In addition, critical issues have arisen, particularly in relation to biosimilars, including the following:

- patent challenges;
- labeling issues;
- concern over identical International Nonproprietary Names (INNs);
- misuse of REMS;
- transitional biological products;
- CMS classification and reimbursement of biologics and biosimilars; and
- interchangeability and substitution.

Biosimilar use is discouraged under certain state laws.

Some state laws require a pharmacist to communicate and, in certain cases, be granted consent by a patient or provider to substitute a biosimilar for a brand-name biologic that would be less expensive and clinically comparable under FDA standards of biosimilarity. However, without biologic products being approved as interchangeable by the FDA, states cannot require substitution the way it is done for small-molecule products. Once biosimilar approval rules and procedures are established, these state laws would become a barrier to patients' access to lower-priced biologics.

Actions That Would Have Direct Impact on Pricing of Biologics and Biosimilars

- 1. Eliminate barriers that restrict competition in the biologics market and deter introduction of biosimilars.*
 - Require the FDA to finalize guidance to spur competition in the biologics market, including guidance on labeling and interchangeability, identical international nonproprietary names (INNs), and transitional biological products.
 - Shorten the market-exclusivity period for brand-name biologics from 12 to seven years (or something more on par with the small-molecule brand-market exclusivity periods). The U.S. is the only country that allows a 12-year exclusivity period. Some experts believe that this lengthy period discourages manufacturers from developing biosimilars.
- 2. Alter how federal and state government programs purchase biologic and biosimilar drugs for the purpose of lowering prices and increasing access for patients.*
 - Create consolidated billing codes for biosimilar products. Most single-source drugs (including biologics but not biosimilars) have their own billing code under Medicare Part B. In 2015, HHS finalized a policy that all biosimilars associated with the same reference product will be grouped together in one billing code and paid the same rate. Under this policy, the reference biologic retains its own billing code. If HHS could issue policy that would apply a consolidated billing code for biosimilars and their reference biologic, it may drive price competition.
 - Authorize Medicare to negotiate Part D drug prices with manufacturers directly, potentially using established prices. See problem 1, action 3a, on page 13 for details.
 - Align Medicare and Medicaid drug prices for dual-eligibles so that Medicare pays at least as low a price as Medicaid does. See problem 1, action 3b, on page 13 for further explanation.
 - Require inflation-based pricing limits for drugs purchased through Medicare Part B or Part D. Currently, the Medicaid program receives lower prices for drugs than Medicare. See problem 1, action 3(c), on page 14 for further explanation.
 - Apply best-price provisions to all other federal health programs, potentially using prices in other countries as a reference. See problem 1, action 3(d), on page 14 for further explanation.
 - Establish purchasing pools among some or all public payers. See problem 1, action 3(e), on page 14 for further explanation.
 - Establish alternative government purchasing programs for drugs that protect public health. See problem 1, action 3(f), on page 14 for further explanation.

3. Alter how government programs purchase biologic and biosimilar drugs for the purpose of tying purchases to improved clinical value or health outcomes.

- a. Authorize Medicare Part B and Part D to negotiate drug prices using alternative purchasing models that take clinical value into consideration. See problem 1, action 4(a), on page 14 for further explanation.
- b. Authorize, by legislation, a least-cost-alternative model for drugs covered under Medicare Part B. Under such a policy, Medicare would not pay the additional cost of a more expensive drug when a clinically comparable, lower-cost drug is available. However, a beneficiary could continue treatment with a higher-priced drug by choosing to pay the additional cost.
- c. Apply the national coverage determination (NCD) process in Medicare Part B to lower spending on FDA-approved physician-administered drugs. See problem 1, action 4(c), on page 15 for further explanation.
- d. Establish an independent entity to set voluntary payment ranges based on research that assesses clinical value of the drug for the purpose of informing public and private payers in their negotiations with manufacturers. If the price of a drug under Medicare or another federal program is higher than the recommended payment range, then the manufacturer would be required to publicly justify the price. Similarly, states could require the same type of reporting for their Medicaid, Children's Health Insurance Program (CHIP), or other public programs.

Actions That Would Have Indirect Impact on Pricing of Biologics and Biosimilars

4. Eliminate barriers that restrict use of biosimilars.

- a. Encourage use of biosimilars in Medicare through guidance and rulemaking that achieve the following:

- instituting interchangeability and substitution standards;
- permitting appropriate medical utilization practices; and
- ensuring appropriate classification and reimbursement of biologics and biosimilars.

- b. Remove barriers at the state level that restrict use of biosimilars, including state standards for substitution and interchangeability, notice requirements, and pharmacy records retention.

5. Require manufacturers to provide information necessary to the development of biosimilar drugs.

- a. Require brand-name manufacturers to promptly disclose at the time of a drug's FDA approval all patents that a biosimilar manufacturer may infringe or violate in developing a biosimilar version.
- b. Require manufacturers to provide samples of drugs with REMS to biosimilar manufacturers.

6. Ensure the availability of comparative-effectiveness information for biosimilars for patients, providers, and payers to empower shared decision-making on treatment options involving biologics and biosimilars.

- a. Build prescriber education and clinical decision-support tools that make information available to patients and providers at the point of care.
- b. Authorize PCORI or a federal agency to conduct comparative-effectiveness research that incorporates price information.
- c. Authorize and appropriate federal funding for comparative-effectiveness research.
- d. Incentivize or require manufacturers to submit comparative-effectiveness research as part of their drug approval applications or post-approval.

7. Ensure availability of price information on biologics and biosimilars that enables informed and prudent purchasing for patients, providers, and payers.

- a. Require transparency in drug pricing and in price increases for patients, providers, and payers.
- b. Eliminate practices that obscure pricing to encourage the industry to engage in more straightforward, open pricing practices.
- c. Build prescriber education and clinical decision-support tools that promote the availability of price information for patients and providers at the point of care.

8. Institute changes to protect patient access and affordability, in the absence of action to directly moderate prices.

See problem 1, action 5 (a–c), on page 15 for details on some of these proposals.

- a. Accelerate closing of the Medicare Part D coverage gap.
- b. Limit out-of-pocket cost sharing for prescription drugs so that patients are less likely to fall into medical debt paying for prescription drugs.
- c. Require insurance coverage for the first dollar of certain prescription drug costs, such as for preventive medicines or those to manage certain chronic diseases.
- d. For public payers, reduce or waive cost-sharing for certain drugs based on comparative-effectiveness research.

Problem 6: Anticompetitive Behavior by Some Manufacturers Undermines Competition Resulting in Higher Prices

Drivers: Price Collusion and Shadow Pricing, Pay-for-Delay, and Product-Hopping

Some manufacturers are engaging in anticompetitive pricing behaviors that keep prices high, despite the competition existing among generic and brand-name manufacturers.

States and federal officials are investigating multiple generic drug companies for artificially inflating prices of diabetes and antibiotic drugs.⁵⁸ A 500-pill bottle of doxycycline, an antibiotic, increased from \$20 for to \$1,849 over six months.

Federal officials have filed charges against executives from a generic drug company for price fixing, rigging bids, and allocating customers for certain generic versions of doxycycline.⁵⁹ Similarly, three brand-name manufacturers—Sanofi, Novo Nordisk, and Eli Lilly—are facing accusations of price-fixing insulin products in response to apparent matching price increases. Over the past decade, the price of insulin has tripled.⁶⁰

Some manufacturers enter agreements with other manufacturers to suppress competition through pay-for-delay or reverse-payment patent settlements.

When a brand-name drug manufacturer pays a patent challenger to keep a generic competitor off the market until an agreed-on date, it is known as a pay-for-delay or reverse-payment settlement. The agreed-on date usually corresponds with the 180-day market-exclusivity period for first generics, or the agreement takes advantage of the 30-month approval delay under the Hatch-Waxman Act. The FTC has argued that pay-for-delay deals are anticompetitive and cost Americans about \$3.5 billion annually in higher health care costs.⁶¹ A 2012 Supreme Court opinion discouraged manufacturers from pursuing these arrangements, and the FTC reported that the number of such deals declined by half by 2014.⁶²

Some brand-name manufacturers engage in a practice referred to as “product hopping,” which involves creating a new product that is similar to the original product. With a goal of obstructing generic manufacturers, the brand-name manufacturer makes

modest reformulations that offer little or no therapeutic advantages and then withdraws the original product from the market, forcing consumers to switch to the reformulated drug. This practice enables the brand-name company to keep its market exclusivity and prevent consumers from obtaining the benefits of generic competition.

The Mylan-Warner Chilcott lawsuit presents an example of product-hopping. Mylan challenged Warner Chilcott in court, alleging that Warner Chilcott maintained a monopoly in the market for its antibacterial drug Doryx. Mylan argued that Warner Chilcott suppressed generic competition by making three successive, insignificant reformulations to the strength of the drug's tablets and then removing the older formulations from the market.⁶⁵

Actions That Would Have Direct Impact on Price Competition

1. Clarify federal law to prohibit anticompetitive behaviors that lead to higher prices.

- a. Clarify in federal law that shadow pricing and pay-for-delay arrangements are presumptively illegal, and increase FTC and DOJ resources to monitor, provide oversight, and investigate these and other settlements.
- b. Clarify in federal law that the FDA has authority to rescind the 180-day generic drug exclusivity period from any generic drug manufacturer that enters into anticompetitive, pay-for-delay settlements with a brand-name drug manufacturer.
- c. Establish a definition for product-hopping and require the FTC to monitor, provide oversight for, and investigate manufacturers engaging in these anticompetitive practices. In addition, require the FDA to study the effects of product-hopping on company profits, consumer access, physician prescribing behavior, and broader economic impacts.
- d. Terminate market exclusivity on any product found to be in violation of criminal or civil law through a federal or state fraud conviction or settlement in which the company admits fault.

Actions That Would Have Indirect Impact on Price Competition

2. Provide additional resources to federal agencies to monitor market dynamics, patient access, and manufacturer behavior.

- a. Require agencies, including the FDA, FTC, and DOJ, to proactively monitor, assess, and report on how the pharmaceutical markets are performing, including identifying anticompetitive behaviors, concentrated markets, and any potential sole source markets. The FDA would also be responsible for monitoring behaviors that produce market inefficiencies, which would help identify where competition may be needed. For example, maintaining a public list of generic drugs and their manufacturers (including distributors, labelers, and compounders) would allow the FDA to more quickly identify drugs at risk of shortage or drugs with a limited number of competitors. In addition, the GAO would be required to conduct a study of market competition in the brand, generic, and biosimilar markets.
- b. Require manufacturers to regularly report information on pricing, price increases, and patient access. This information would be used to issue publicly available annual reports that hold manufacturers accountable for their prices and for increases in those prices over time. This information would also be used by federal agencies to monitor for anticompetitive behaviors.

Problem 7: Some Manufacturers Use Current Patent-Protection Policies for Brand-Name Drugs to Extend Monopoly Pricing

Drivers: Extended Patent Protections, Patent Clustering, and Evergreening

Some manufacturers are using their long periods of patent protection and patent restoration to significantly increase brand-name drug prices, often on an annual basis, even when there have been no significant improvements in the drug. The Hatch-Waxman Act

extended patents on approved new drugs for five years, up to a maximum of 14 years. These patents were extended, or restored, to make up time lost from gaining FDA approval.

Some manufacturers develop a dense portfolio of patents to cover one drug, a practice often referred to as a patent cluster or patent thicket. Separate patents can be obtained for the drug molecule, for different use indications, or for the manufacturing process itself. By building a portfolio of patents for a single drug, manufacturers can protect their product and eliminate competition. A recent example is Mylan's EpiPen. While the epinephrine solution is not currently under patent, Mylan has secured four patents on EpiPen that do not expire until 2025.⁶⁴ The use of multiple patents makes it incredibly difficult for a competitor to develop an alternative.

Some manufacturers misuse patent law by evergreening, or suppress timely competition by adding patents that may not be novel. Evergreening occurs when manufacturers seek additional patents on variations of their original drugs. These patents may cover new forms of release, dosages, combinations, or formulations that are unrelated to the drug's effectiveness. This practice can extend a drug's price monopoly. For example, Suprenza is a weight-loss drug that was granted a patent extension in 2013, which extended protection into 2029. The new patent was for an orally dissolving tablet with a new speckled appearance, which was created by adding colored granules of water-soluble sugar. Critics argue that the colored speckles are not patentable.⁶⁵

Actions That Would Have Direct Impact on Drug Pricing Linked to Patent Issues

1. Alter patent protections to introduce price competition, with the goal of reducing drug prices.

- a. Eliminate or reform provisions under the Hatch-Waxman Act that delay the introduction of generic products. Changes to the law include addressing the 30-month delay in any additional generic approval in the case of patent infringement challenges by the brand-name company and the 180-day exclusivity period for the first generic approved.

- b. Reduce or eliminate patent extensions that were created under the Hatch-Waxman Act to rebalance innovation incentives with competition. A more focused strategy would be to eliminate patent extensions only for drugs that are clinically comparable to a drug on the market but have no added comparative value.

2. Reform U.S. Patent and Trademark Office (USPTO) policies on patent review and approval.

- a. Establish policies that would require patent applicants to demonstrate significant differences, originality, or additional benefit for secondary patents.
- b. Strengthen patent system review to ensure that competitors that oppose the grant of a patent can play a productive role in the administrative proceeding known as inter partes review (IPR). The IPR process for patent challenges at the USPTO is intended to be less costly and time-consuming than pursuing litigation. A review may not be instituted if the petition requesting the proceeding is filed more than one year after filing a complaint alleging patent infringement.
- c. Extend the window to file a post-patent grant opposition proceeding—known as post-grant review—in which third parties can challenge a patent's validity by submitting any additional information bearing on the patentability of the claimed invention. Once a patent is granted, there is a strong presumption of validity, and generic manufacturers are blocked from production until the patent is revoked. The post-grant review requires that the challenge be filed within nine months from when the patent was granted. However, it can be years after a patent is granted before that patent's relevance and importance to a generic manufacturer are known.
- d. Require brand-name manufacturers to promptly disclose at the time of a drug's FDA approval all patents that a generic manufacturer may infringe or violate in developing a generic version.

Problem 8: Patients, Providers and Payers Lack Information About Comparative Effectiveness of Drugs at the Point in Time When Critical Health Care Decisions Are Made

Driver: Lack of Information for Decision-Making

Information on how well a drug performs in comparison to clinically comparable drugs is limited.

Having comparative-effectiveness information would improve shared decision-making between patients and providers and help ensure that patients receive the most appropriate treatment for their situation.

Several value-based frameworks have emerged to tie the comparative benefit of a treatment to its costs. Many provider organizations and nonprofit organizations are promoting comparative-value assessments to provide the highest quality of care to patients; these include the American College of Cardiology in partnership with the American Heart Association, the American Society of Clinical Oncology, the Institute for Clinical and Economic Review, and Memorial-Sloan Kettering Cancer Centers.⁶⁶

Actions That Would Have Direct Impact by Increasing Information on Comparative Effectiveness

1. *Alter how government programs buy drugs for the purpose of tying purchases to improved clinical value or health outcomes.*

- a. Authorize Medicare Part D to tie drug coverage to comparative-effectiveness research. For instance, HHS might base whether to continue or expand coverage of a drug on the manufacturer's agreement to collect additional population-level evidence of the drug's comparative effectiveness.

Actions That Would Have Indirect Impact by Increasing Information on Comparative Effectiveness

2. *Ensure the availability of comparative-effectiveness information for patients, providers, and payers to empower shared decision-making on treatment options.*

- a. Build prescriber education and clinical decision-support tools that make information available to patients and providers at the point of care. Such tools could disseminate comparative-effectiveness research and price information on potential treatments, which would help patients and providers make informed choices.
- b. Authorize PCORI or a federal agency to conduct comparative-effectiveness research that incorporates price information.
- c. Authorize and appropriate federal funding for comparative-effectiveness research.
- d. Incentivize or require manufacturers to submit comparative-effectiveness research as part of their drug approval applications or post-approval. As part of this proposal, manufacturers would be required to compare costs and outcomes of a new drug versus existing therapies.
- e. Require a manufacturer awarded the three-year New Clinical Investigation Exclusivity incentive to demonstrate significant clinical benefit over existing therapies manufactured by the applicant in the five-year period preceding submission of the application.

Problem 9: The Pharmaceutical Distribution System Does Not Make Essential Pricing Information Available to Patients, Providers, and Payers at the Point of Care, Making It Difficult for Patients to Make the Best Decisions Related to Their Care

Drivers: System Complexity and Lack of Transparency

The current drug distribution system has many stakeholders, and each has a complex relationship with the others. Major players in the distribution system include manufacturers, wholesalers, pharmacy benefit managers (PBMs), retailers, and insurers.⁶⁷ The number of intermediaries in our drug distribution system creates a complex flow of payments and rebates.

There is a lack of transparency regarding the rebates and payments each entity charges or receives. This lack of transparency makes it difficult to determine if entities are inappropriately driving up drug prices. No regulations or requirements exist for these entities to disclose information on prices or rebates to each other or the public.

Actions That Would Have Indirect Impact by Increasing Availability of Pricing Information

1. Ensure availability of pricing information before or at the point of care to patients, providers, and payers to support and encourage prudent purchasing.

- a. Require transparency in drug pricing and in price increases for patients, providers, and payers. The federal government would require manufacturers and PBMs to report:
 - information on rebates to various payers;
 - prices paid by various payers; and
 - the level of patient assistance provided by manufacturers.

The federal government or an independent entity would monitor price increases and require manufacturers to justify price increases, with a goal of moderating annual increases. This information could be made publicly available, and the intent would be to promote open data and information-sharing.

- b. Eliminate practices that obscure pricing, to encourage the industry to engage in more straightforward, open-pricing practices. The use of consumer coupons provided by manufacturers in commercial settings could be eliminated, similar to the current prohibition on coupons in Medicare and Medicaid. The use of rebates between manufacturers, PBMs, and insurance companies might also be discontinued.
- c. Build prescriber education and clinical decision-support tools that promote the availability of price information for patients and providers at the point of care.

2. Ensure accurate and comprehensive information from manufacturers to patients, providers, and payers to support and encourage prudent purchasing.

- a. Regulate, restrict, or eliminate direct-to-consumer advertising, including disease-awareness activities, to eliminate misleading information.
- b. Remove tax incentives (like the deductibility of advertising expenses) for drug promotion activities.

Problem 10: Federal Law Imposes Limitations on State Authority to Negotiate Prices for Medicaid and Implement Other Price-Related Measures

Drivers: Lack of Flexibility for State Innovation

While there is some flexibility in Medicaid drug coverage, federal law limits states' authority to exclude a drug from coverage or use value-based purchasing strategies to address the high cost of drugs in the program. To have their products covered by Medicaid, manufacturers must agree to provide a discount of 23.1 percent on the average manufacturer price. When the manufacturer enters this rebate agreement with CMS, each state Medicaid program must cover all the manufacturer's drugs, providing manufacturers with a guaranteed market.

Some states have negotiated supplemental rebates with manufacturers to ensure placement of the manufacturer's product on the preferred drug list (PDL). The PDL is similar to how tiers are used in a drug formulary. States may also impose restrictions, such as utilization management or prior authorization, on drugs not on the PDL or drugs with no supplemental rebate.

In 2014, Medicaid net spending on prescription drugs totaled \$22 billion.⁶⁷

Actions That Would Have Direct Impact Through State Action

- 1. Provide states with flexibility to address high drug prices.*
 - a. Permit states to reimport high-priced drugs. Currently, reimportation of drugs from other countries is illegal, except for personal use.

- However, leaders from some border states, such as Vermont, have argued for the use of reimportation. While not all states may pursue this option, it could be beneficial to smaller border states that might not have as much purchasing power as a larger state in terms of implementing alternative purchasing strategies or negotiations.
- b. Allow states greater flexibility in purchasing drugs under the Medicaid Drug Rebate Program. Prescription drug coverage is optional in Medicaid, although every state has chosen to provide prescription drug coverage. In doing so, the states are required to cover every drug in the Medicaid Drug Rebate Program. If states were allowed flexibility to exclude drugs from their Medicaid formularies when a clinically comparable drug exists on the market, then states could engage with manufacturers in negotiations that would drive down prices. To ensure that patients were not disadvantaged or left without access, an exceptions process would be required when a patient has a medically necessary reason to take a drug that is excluded from the Medicaid formulary. In addition, many of the same models and tools described as purchasing strategies for Medicare could be applied in the Medicaid program at the state level. (See problem 1, actions 3 and 4, on pages 13–15).
 - c. Allow states greater flexibility in purchasing drugs under the Medicaid Drug Rebate Program to use alternative purchasing models. See problem 1, action 4(a), on page 14, for further explanation.
 - d. Permit Medicaid waivers and legislative changes to promote greater purchasing flexibility but still require a minimum level of coverage for enrollees. One approach could establish a minimum threshold of coverage to ensure beneficiary access while still allowing states to waive some requirements. Alternatively, waivers might allow states to opt out of the Medicaid drug rebate program, enabling them to increase their purchasing power.
 - e. Let states operate as PBMs to broaden their purchasing and negotiating powers. Currently, states' Medicaid programs may enter into purchasing pools to negotiate prices jointly, creating more purchasing power. There are also some efforts in states to involve other agencies or populations, along with Medicaid. This option could allow states more opportunity for different contracting approaches. The state could act as a negotiator and purchaser on behalf of all its coverage programs, thus increasing negotiation and purchasing power. If states were allowed flexibility to exclude drugs from the formulary when a clinically comparable drug existed on the market, then states could engage with manufacturers in negotiations that would drive down prices. To ensure patients were not disadvantaged or left without access, an exceptions process would be required when a patient has a medically necessary reason to take a drug that is excluded from the Medicaid formulary.
 - f. Pursue return-on-investment (ROI) pricing. ROI pricing allows states to estimate all costs that will be avoided across state spending programs because of coverage and use of a drug. Essentially, the state would expect to see some sort of return (or savings), by covering a certain product over a set amount of time. In this approach, states and manufacturers negotiate and agree on a set price, rather than the manufacturer setting a price. The price would be based on estimates of how much the state would spend over a given period for the drug, and the estimates of costs avoided because of coverage and use of the drug during the same period. In upcoming years, the ROI price would be adjusted to account for market changes. This approach would likely be better for drugs that have an established history of effectiveness. Overall, the goal of ROI pricing is to tie drug prices and payment to the value of the drug.

CONCLUSION

Unprecedented prescription drug prices are leaving many patients without affordable access to drugs when they need them. Because of high prices, patients are skipping doses and choosing to not fill their prescriptions. Many are also increasingly concerned about how they will afford their prescription drugs in the future.

There is widespread public support for elected officials to address the problems of high prescription drug prices. This report aims to foster discussion and consensus among policymakers and stakeholders to take significant and meaningful actions on this issue. Any effort must start with identifying common ground on the problems of drug pricing. Taking on these problems today should be guided by policy goals that rebalance incentives for innovation and price competition, prioritize patient access and affordability, and maximize availability of information to improve patient care.

As the U.S. moves forward in finding effective solutions that address the problems identified in this report, it is important to recognize how Congress was able to tackle similar, complicated prescription drug issues in the 1980s, when lawmakers pursued and passed bipartisan legislation that balanced the right incentives for innovation with price competition. Congress should take a page out of this earlier playbook, which succeeded in creating a generic drug market and incentives for finding new treatments and breakthrough cures.

NOTES

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The following subject matter experts and organizations directly or indirectly contributed to the development of this report through their policy work (including policy proposals, position statements, or policy research) and through interviews with the authors.

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American Heart Association (AHA)	Oregon Health and Science University, Drug Effectiveness Review Project (DERP)
American Medical Association (AMA)	Pacific Business Group on Health (PBGH)
Blue Cross and Blue Shield Association (BCBSA)	Partnership for Safe Medicines
Campaign for Sustainable Rx Pricing	Patients for Affordable Drugs
Center for American Progress (CAP)	Pew Charitable Trusts
Coalition for Affordable Prescription Drugs	Pharmaceutical Care Management Association (PCMA)
Council for Affordable Health Coverage	PhRMA
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GLOSSARY

Abbreviated new drug applications (ANDAs): The Food and Drug Administration's (FDA) application for a generic drug approval. Generic drug applications are "abbreviated" because they are generally not required to include preclinical (animal) and clinical (human) data to establish safety and effectiveness. Generic applicants must scientifically demonstrate that their product is bioequivalent (i.e., performs in the same manner) to the innovator drug.

Bayh-Dole Act: This 1980 law reserves for the government certain patent rights for inventions arising from federally funded research and development.

Best-price provisions: The lowest price available from the manufacturer during the Medicaid rebate period to any wholesaler, retailer, provider, health maintenance organization, nonprofit entity, or governmental entity in the United States in any pricing structure (including capitated payments), in the same quarter for which the average manufacturer price (AMP) is computed.

Biologic: Biological products include a wide range of products such as vaccines, blood and blood components, allergenics, somatic cells, gene therapy, tissues, and recombinant therapeutic proteins. Biologics can be composed of sugars, proteins, or nucleic acids or complex combinations of these substances, or may be living entities such as cells and tissues.

Biologics Price Competition and Innovation Act (BPCIA): This law established a 12-year market-exclusivity period for biologics and created an abbreviated licensure pathway for biological products that are demonstrated to be "biosimilar" to or "interchangeable" with an FDA-licensed biological product. Under the BPCIA, a biological product may be demonstrated to be biosimilar if data show that, among other things, the product is "highly similar" to an already-approved biological product.

Biosimilar: A biosimilar product is a biological product that is approved by the FDA based on a showing that it is highly similar to an FDA-approved biological product, known as a reference product, and has no clinically meaningful differences in safety and effectiveness compared to the reference product. Only minor differences in clinically inactive components are allowable in biosimilar products.

Comparative effectiveness: Research on comparative effectiveness allows for comparison of multiple drugs based on the effectiveness, harms, and benefits of different treatment options.

Comparator drug: These are marketed products that are clinically comparable to another drug based on the same indication and used as a reference in clinical studies.

Compounding pharmacy: Pharmacies that compound, a practice in which a licensed pharmacist, a licensed physician, or, in the case of an outsourcing facility, a person under the supervision of a licensed pharmacist, combines, mixes, or alters ingredients of a drug to create a medication tailored to the needs of an individual patient.

Evergreening: Evergreening occurs when a manufacturer seeks additional patents on variations of their original drug. These patents may cover new forms of release, dosages, combinations, or formulations that are unrelated to the drug's effectiveness and forms.

Federal Ceiling Price: The maximum price that manufacturers can charge four federal purchasers of pharmaceuticals: the Department of Veterans Affairs, the Department of Defense, the Public Health Service, and the Coast Guard.

First generic: The first FDA approval of a generic drug, permitting a manufacturer to market a generic drug product in the United States.

Formulary: A list of drugs covered by a prescription drug plan or another insurance plan offering prescription benefits.

Grant-and-access pathway: A process where manufacturers compete for federal grants to subsidize the costs of clinical testing. In return for the grant funding, manufacturers would no longer claim tax credits and would agree to price caps for marketed products based on the duration and costs associated with drug development, expected market size, and target rate of return.

Group purchasing organization: An entity that leverages the purchasing power of a group to negotiate contracts for medical products and services.

GLOSSARY (cont'd)

Hatch-Waxman Act (also known as the Drug Price Competition and Patent Term Restoration Act): This federal law extended patent terms and introduced market-exclusivity protections for certain types of drugs. These policies were intended to ensure that drug manufacturers are given a period to sell patented, innovative products without direct competition so they can recoup their development costs and gain a return on investment. The law also provides for a generic drug approval system that ensures safe, therapeutically equivalent generic drugs are available at lower prices when patents and other market exclusivities expire.

Inter Partes Review: Inter partes review is a proceeding conducted at the Patent Trial and Appeal Board to review the patentability of one or more claims in a patent limited to certain grounds, and only based on prior art consisting of patents or printed publications. This review is the mechanism for challenging the validity after the nine-month window for post-grant review has closed.

International Nonproprietary Names (INNs): INNs are used to identify pharmaceutical substances or active pharmaceutical ingredients. Each INN is a unique name that is globally recognized and is public property. A nonproprietary name is also known as a generic name.

Launch price: The price set by a manufacturer upon FDA approval of a drug.

Least-cost alternative: The payor does not pay the additional cost of a more expensive drug when a clinically comparable, lower-cost drug is available. Beneficiaries may choose to pay the additional cost for the more expensive treatment.

Market-exclusivity protections: Exclusive marketing rights granted by the FDA upon approval of a drug and can run concurrently with a patent or not. These protections prevent the submission or effective approval of ANDAs or other drug applications and are designed to promote a balance between new drug innovation and generic drug competition.

Medicaid Drug Rebate Program: The program requires a drug manufacturer to enter, and have in effect, a national rebate agreement with HHS in exchange for state Medicaid coverage of most of the manufacturer's drugs. This agreement requires manufacturers to pay a statutory rebate on those drugs for which payment was made under Medicaid. It also requires manufacturers to enter agreements with two other federal programs so that their

drugs can be covered by Medicaid: a pricing agreement for the Section 340B Drug Pricing Program, administered by the Health Resources and Services Administration, and a master agreement with the Secretary of Veterans Affairs for the Federal Supply Schedule.

Medicare Part B drugs: Medicare Part B generally reimburses for drugs administered by a health care professional in a physician's office, as part of a sustained clinical treatment such as chemotherapy, or for other specified drugs that cannot be patient self-administered, such as vaccines.

Medicare Part D drugs: The Medicare Part D benefit was designed to provide coverage for outpatient prescription drugs.

National coverage determination (NCD) process: The NCD process determines which items and services are covered by Medicare. The determinations are made through an evidence-based process, with opportunities for public participation. In some cases, CMS's own research is supplemented by an outside technology assessment and/or consultation with the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC). In the absence of a national coverage policy, an item or service may be covered at the discretion of the Medicare contractors based on a local coverage determination.

New Chemical Exclusivity (NCE): A five-year period of market exclusivity granted to new drug applications for products containing chemical entities never previously approved by FDA either alone or in combination.

New Clinical Investigation Exclusivity incentive: This incentive applies to new clinical studies that lead to new approved indications. The applicant is entitled to this exclusivity if an application or supplement contains reports of new clinical investigations conducted or sponsored by the applicant that were essential for approval. This is a three-year market exclusivity.

Off-label use: When a prescription drug is prescribed for uses or treatment other than what the FDA has approved.

Orphan Drug Act: A federal law that encourages manufacturers to produce drugs for treatment or cure of rare diseases and conditions. The act provides a research-and-development tax credit and a seven-year period of market exclusivity for the development of a drug for a rare disease or condition, which is defined as affecting less than 200,000 people.

GLOSSARY (cont'd)

Patent clusters (also known as patent thickets): To create a patent cluster, manufacturers develop a dense portfolio of multiple patents to cover one drug or device.

Pay-for-delay settlements (also known as reverse-payment settlements): A brand-name manufacturer pays a potential generic competitor to delay the selling of the generic version of the drug until the six-month first generic exclusivity period has expired.

Pharmacy benefit manager (PBM): PBMs are intermediaries between the third-party payer and the manufacturer. PBMs will generally handle the billing, negotiation of drug prices, and creation of pharmacy networks.

Post-grant review: A proceeding conducted at the Patent Trial and Appeal Board to review the patentability of one or more claims in a patent on any relevant grounds. This review is the mechanism for challenging the validity during the nine-month window immediately following patent issuance.

Preferred drug list (PDL): In the Medicaid program, states may maintain a preferred drug list. States may negotiate supplemental rebates with manufacturers to ensure placement of the manufacturer's product on the PDL.

Priority review voucher: FDA's priority review vouchers are incentives intended to spur the development of new treatments that would otherwise not be developed because of the cost. Manufacturers are given a "voucher" that allows them to have any one of their drugs reviewed under FDA's priority review system. These vouchers are available to manufacturers of a newly approved drug or biologic that targets a neglected tropical disease, a rare pediatric disease, and/or a medical countermeasure.

Product-hopping: Product-hopping occurs when a manufacturer makes modest reformulations to an existing product that offer little or no therapeutic value and then withdraws the original product from the market to obstruct competition and preserve monopoly profits.

Protected classes: In Medicare Part D, six classes of drugs must be covered: anticonvulsants, antidepressants, antineoplastics, antipsychotics, antiretrovirals, and immunosuppressants.

Rate-of-return model: A model for profitability based on incurring a set profit on an investment over a specified period that is expressed as a proportion of the original investment.

Rebates: In many pharmaceutical contracts and agreements to purchase drugs, manufacturers will pay a rebate on their product. The rebate is a reduction in price based on product sales.

Reference pricing: The practice of setting a price for reimbursement of drugs within a disease group based on therapeutic effectiveness or the least-cost alternative of drugs within a disease group. The payer pays the set price, called the reference price, for any drug in the class.

Retail drugs: Any drug that does not need to be administered in a hospital or physician office.

Return on investment (ROI) pricing: A pricing method that ties drug prices and payments to the drug's value. Through an ROI agreement, the payer, most likely the state, will estimate a return or savings from other areas of state spending, attributed to coverage and use of a certain drug.

Risk Evaluation Mitigation Strategies (REMS): REMS programs are intended to improve drug safety for certain products by ensuring that the benefits for patients outweigh the risks. They commonly include limited distribution systems.

Shadow pricing: The practice whereby drug manufacturers raise the prices of their drugs to match the highest-cost drug in the market, without explicitly colluding.

Shared decision-making: A process in which clinicians and patients work together to make decisions and select tests, treatments, and care plans based on clinical evidence that balances risks and expected outcomes with patient preferences and values.

Small-molecule drug: A substance able to enter cells easily because of its low molecular weight.

Sole-source drugs: Drugs produced by a single manufacturer that has no competition in the market.

Step therapy: The practice of prescribing a patient the most cost-effective drug first and, if that drug fails, progressing to more costly and riskier drugs.

Transitional biological products: Protein products that are transitioning from the drug statute to the biologics system, as described under the Biologics Price Competition and Innovation Act of 2009.

Translational research: Applies findings from basic science to enhance human health and well-being.

ABOUT THE AUTHORS

Rep. Henry Waxman is one of the most effective legislators of the last 40 years, with health care among his central concerns. During his time in Congress, Waxman used legislative tools to unmask the tobacco industry after years of deception and authored the Affordable Care Act, which has helped 20 million more Americans get health insurance. The Hatch-Waxman Act helped create the generic drug industry, while the Orphan Drug Act incentivized the growth of an industry that has given hope to the millions of Americans afflicted with rare diseases. Evident in all of Waxman's work is his commitment to concrete solutions that transform people's lives for the better. His tenacity has earned him widespread recognition from journalists, fellow elected officials, and President Obama, who described him as "one of the most accomplished legislators of this or any era."

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About The Commonwealth Fund

The Commonwealth Fund, among the first private foundations started by a woman philanthropist—Anna M. Harkness—was established in 1918 with the broad charge to enhance the common good.

The mission of The Commonwealth Fund is to promote a high performance health care system. The Fund carries out this mandate by supporting independent research on health care issues and making grants to improve health care practice and policy. An international program in health policy is designed to stimulate innovative policies and practices in the United States and other industrialized countries.

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