# Getting to Lower Prescription Drug Prices

## The Key Drivers of Costs and What Policymakers Can Do to Address Them

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The Commonwealth Fund

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## ABSTRACT

**ISSUE:** Unsustainably high prescription drug prices are a concern for patients, employers, states, and the federal government. There is widespread public support for addressing the problem, and enacting policies to lower drug prices has been a top concern for Congress and the administration over the past three years. Despite this attention, structural changes have not been enacted to rein in drug prices.

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

**METHODS:** Interviews with experts and organizations engaged on policies related to prescription drug pricing. Review of policy documents, white papers, journal articles, proposals, and position statements.

**KEY FINDINGS:** Action in five areas is key to increasing access to and affordability of medications for Americans: 1) allow the federal government to become a more responsible purchaser; 2) stop patent abuses and anticompetitive practices that block price competition; 3) build a sustainable biosimilar market to create price competition; 4) fix incentives in the drug supply chain and make the supply chain more transparent; and 5) ensure public accountability in the government-funded drug development process. Congress and regulators have a wide range of tools at their disposal to address high drug prices and spending.


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# CONTENTS

**ABSTRACT** . . . . . 2

**INTRODUCTION** . . . . . 5

**THE STATE OF U.S. PRESCRIPTION DRUG PRICING** . . . . . 5

Annual Prescription Drug Price Increases Remain High as Launch Prices Continue to Rise . . . . . 5

Patients, Employers, Taxpayers, and States All View Drug Costs as Unsustainably High . . . . . 6

**FEDERAL ACTION ON PRESCRIPTION DRUG PRICING SINCE 2017** . . . . . 7

**ADDRESSING THE DRIVERS OF HIGH DRUG PRICES** . . . . . 8

**1. Allow Government to Become a More Responsible Purchaser** . . . . . 8

**2. Stop Patent Abuses and Anticompetitive Practices That Block Price Competition** . . . . . 11

**3. Build a Sustainable Biosimilar Market to Create Price Competition** . . . . . 14

**4. Fix Incentives in the Drug Supply Chain and Make the Drug Supply Chain More Transparent** . . . . . 16

**5. Ensure Public Accountability in the Government-Funded Drug Development Process** . . . . . 20

**CONCLUSION** . . . . . 23

**NOTES** . . . . . 24

**APPENDIX A. TRUMP ADMINISTRATION INITIATIVES ON DRUG PRICING** . . . . . 34

The Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs and the President's Budget Requests . . . . . 34

FDA Focused on Competition . . . . . 34

Prescription Drug Importation Proposals . . . . . 35

Medicare and Medicaid Drug Spending Dashboard . . . . . 36

Advanced Notice of Proposed Rulemaking for an International Pricing Index . . . . . 36

Direct-to-Consumer Advertising Price Transparency Final Rule . . . . . 36

Proposed Rule to Eliminate Safe Harbor Protection for Rebates . . . . . 36

Voluntary Medicare Part D Senior Savings Model to Lower Insulin Out-of-Pocket Costs . . . . . 36

**APPENDIX B. CONGRESSIONAL ACTION ON DRUG PRICING** . . . . . 38

**Enacted Legislation** . . . . . 38

Bipartisan Budget Act (H.R. 1892) – February 2018 . . . . . 38

Correcting Inflation Rebates for Medicaid Line Extensions . . . . . 38

Closing the Medicare Part D Coverage Gap Early and Increasing Manufacturer Drug Cost Liability . . . . . 38

Applying the Brand/Biologic Coverage Gap Discount to Biosimilars . . . . . 38

Pharmacy Gag Clauses and Increased Biologic Patent Transparency (H.R. 2553 and H.R. 2554) – October 2018 . . . . . 39

Medicaid Services Investment and Accountability Act (H.R. 1839) – April 2019 . . . . . 39

Fair and Accurate Medicaid Pricing Act (H.R. 3276) – September 2019 . . . . . 39

The CREATES Act (H.R. 965) – December 2019 . . . . . 39

**Legislation That Passed the U.S. House of Representatives** . . . . . **40**

The Elijah E. Cummings Lower Drug Costs Now Act (H.R. 3) – December 2019 . . . . . 40

Other Legislation That Passed the House . . . . . 40

**Legislation That Passed the U.S. Senate** . . . . . **40**

Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act of 2017 (S. 1052) – August 2017 . . . . . 40

**Legislation Reported Out of the Relevant Committees** . . . . . **40**

Senate HELP Committee – Lower Health Care Costs Act (S. 1895) – July 2019 . . . . . 40

Senate Finance Committee – Prescription Drug Pricing Reduction Act (S. 2543) – July 2019 . . . . . 41

**Other Legislation Reported Out of a Committee** . . . . . **41**

**Legislation Enacted by States on Drug Pricing** . . . . . **41**

**APPENDIX C. EXCLUSIVITIES AND PATENT-GAMING STRATEGIES** . . . . . **42**

**ABOUT THE AUTHORS** . . . . . **44**

**ACKNOWLEDGMENTS** . . . . . **45**

## INTRODUCTION

**Our 2017 report, *Getting to the Root of High Prescription Drug Prices: Drivers and Potential Solutions,***[1] described the activities and behaviors of pharmaceutical manufacturers and other parts of the prescription drug supply chain that led to unaffordable prescription drug prices. Three years later, we find that pharmaceutical manufacturers' behavior is unchanged and that drug prices remain unsustainable.

During the past four years, the Trump administration has announced a number of proposals to address high drug prices but has implemented only small measures within its authority. Congress has held many hearings and enacted some modest pieces of legislation. To date, each chamber has also advanced separate major drug pricing bills: the House passed H.R. 3, the Lower Drug Costs Now Act, and, although it was never brought to the floor for a vote by the full Senate, the Senate Finance Committee approved S. 2543, the Prescription Drug Pricing Reduction Act (see the Appendices) — but Congress has not enacted the structural changes necessary to rein in drug prices.[2] The result has been persistent public pressure on elected leaders to lower prescription drug prices, as those prices have continued to rise annually and new drugs have been introduced at unprecedented high prices. In an April 2020 poll, 65 percent of Americans agreed that the Trump administration had made "not very much" progress or "none" on controlling rising drug costs.[3]

As we detail in this paper, pharmaceutical manufacturers continue to extend monopoly protection of brand and biologic prices, delay or prevent competition, and continue unsustainable price growth. This is caused by patent gaming and manipulation of exclusivity periods and enabled by outdated drug coverage design that manufacturers use to their advantage to incentivize use of more expensive products and crowd out generic or biosimilar products. Overall, the pharmaceutical market no longer maintains the right balance of incentives to drive the invention of new products while maintaining effective generic and biosimilar price competition.

While further action is needed to substantially reduce high introductory prices and limit annual increases of prescription drugs, this will not address the drivers of high prices. We recommend a policy focus on the following five areas:

1. Helping government to become a more responsible purchaser.
2. Stopping patent abuses and anticompetitive practices that block price competition.
3. Building a sustainable biosimilar market to create price competition.
4. Fixing incentives in the drug supply chain and making the supply chain more transparent.
5. Ensuring public accountability in the government-funded drug development process.

Below we discuss, in no particular order, some potential actions in each of these areas that Congress and the administration could consider taking.

## THE STATE OF U.S. PRESCRIPTION DRUG PRICING

### Annual Prescription Drug Price Increases Remain High as Launch Prices Continue to Rise

**Less than 10 percent of all prescriptions written in the United States are for a brand drug or biologic.**[4] **Brand products, however, make up 80 percent of all U.S. drug spending.**[5] Between 2013 and 2015, drug prices increased annually by nearly 10 percent, which is over six times the inflation rate.[6] In an effort to mollify lawmakers and the public, several large brand pharmaceutical manufacturers began to publicly report their list price increases between fall 2016 and early 2017.[7] Since 2018, larger drugmakers have honored their voluntary pledges to keep future price increases below 10 percent, with increases across the market and in manufacturers' portfolios averaging 5.2 percent in early 2020 — although some drugs are rising by 9.9 percent annually while others increase by 5 percent twice a year.[8] Still, this price growth remains well above annual inflation as measured by the Consumer Price Index for Urban Consumers (CPI-U).[9]

Although the average annual increase rate for list prices has remained below 10 percent, there is evidence that drug manufacturers have been launching new drugs at higher introductory prices.[10] Median launch prices tend to vary from year to year but the upper quartile of launch prices can be expected to grow reliably. For example, while the median wholesale acquisition cost (or the estimated list price) for a newly launched branded pharmaceutical product decreased from $1,133 to $722 from 2017 to 2019, the upper quartile in launch prices leapt from $8,095 to $15,310.[11] In 2017, Sprinraza, a drug for spinal muscular atrophy, launched at $125,000 per injection, adding up to $750,000 in the first year and $375,000 in the following years.[12] After more frequently occurring doses in the first year of treatment, patients must receive a maintenance injection every four months for the rest of their lives.[13] The most expensive drug ever approved by the U.S. Food and Drug Administration (FDA) was launched in 2019: a gene therapy called Zolgensma came to market at a per-patient price of $2.125 million for a onetime infusion therapy that cures spinal muscular atrophy.[14]

Drug manufacturers sometimes argue that net prices — which include all rebates and discounts a manufacturer offers — are more representative of the prices paid by health plans. However, in Medicare Part B, patient cost-sharing is based on list price. Additionally, there is evidence that net prices have also increased, especially for high-cost specialty drugs.[15] For example, the median net cost of the 49 highest-volume brand drugs increased 76 percent between January 2012 and December 2017, which is a nearly 10 percent compound annual growth rate.[16] In another example, the average net price per prescription of brand-name specialty drugs grew 12 percent in Medicaid and 22 percent in Medicare Part D between 2010 and 2015.[17] In recent years, net price growth for all branded products has been well above inflation.[18]

## Patients, Employers, Taxpayers, and States All View Drug Costs as Unsustainably High

***Patients.*** Nearly 80 percent of Americans said prescription drug prices were unreasonable in 2019.[19] New polling shows that over one-third of U.S. adults perceive that prescription drug prices have "increased a lot" since 2017, with only 7 percent saying prices have dropped at all.[20]

### HOW MANY WAYS CAN A PHARMACEUTICAL PRICE BE MEASURED?

**List Price:** The price of a drug initially set by the manufacturer. It does not include any discounts or rebates paid to pharmacy wholesalers, pharmacy benefit managers (PBMs), providers, or patients. When manufacturers disclose their price increases, they are disclosing their list price increases.

**Wholesale Acquisition Cost (WAC):** Also known as the estimated list price, it is defined in federal law as an estimate of the price a wholesaler pays a manufacturer for drugs purchased from the wholesaler's supplier, which is typically the manufacturer of the medicine. Publicly disclosed wholesale acquisition costs may not reflect all available discounts.

**Net Price:** The price of a drug that includes all rebates and discounts a manufacturer offers to pharmacy wholesalers, PBMs, providers, or patients. The net price may vary widely depending on whom the manufacturer is selling to and at what volume.

**Average Sales Price (ASP):** As defined in federal law, the price measure used for Medicare Part B reimbursement. It is a calculation of the weighted average of all of a manufacturer's nonfederal sales, net of any price adjustments. In other words, the average sales price is the weighted average net price of a drug after factoring in all rebates, chargebacks, or discounts a manufacturer supplies to entities along the supply chain. Rebates and discounts lower the Medicare Part B drug reimbursement to providers. However, Medicare Part B patients still have to pay coinsurance for the drug based on the drug's or biologic's list price

Beyond the financial impact, almost one-third (29%) of Americans have neglected to take their medication as prescribed due to its cost.[21] When patients discover that an expensive prescription is not covered on their plan's formulary, about half of low- and middle-income patients opt against filling the prescription.[22] This noncompliance may contribute to increased rates of hospitalization, sickness, and death.[23] Other polls show that nearly one-quarter (23%) of adults have not had enough money to pay for a prescription in the past year,[24] while 13 percent know someone who has died in the past five years after not being able to pay for needed treatment, including prescription drugs.[25]

***Businesses.*** Employers, which provide coverage for nearly half (49%) of the U.S. population, are also confronting the increasing cost of prescription drugs.[26] The National Alliance of Healthcare Purchaser Coalitions and EmployersRx[27] recently published a report based on a series of employer surveys and roundtables. The report found that 60 percent of respondents think the price they pay for prescription drugs is "costly and unsustainable" and that lack of affordability of prescription drugs has impacts on employee stress, financial instability, performance, and absenteeism, in addition to other indirect health care costs.[28] National polling of 500 small- and medium-sized businesses by Public Private Strategies and the Commonwealth Fund found that 40 percent of small-business owners rank prescription drug prices among the biggest challenges when it comes to providing health care to employees.[29]

***Federal and state government.*** The government, vis-à-vis the American taxpayer, is the last and largest group among those paying more every year for high-cost drugs. State and federal governments accounted for 45 percent of all health care spending in 2018 (compared to 20% for private businesses).[30] Much of federal spending for prescription drugs is through the Medicare Part D prescription drug benefit. Since at least 2014, the growth rate of spending in this program has outstripped that of national health expenditures as a whole.[31]

Drug spending in Medicare Part B, comprising mostly high-priced, hospital-administered medications such as biologics and infusion therapies, has also risen steeply over the past several years.[32] Increases in Medicare Part B average annual drug spending were above CPI-U inflation between 2006 and 2017.[33]

Medicaid has also increasingly had to contend with high-priced specialty pharmaceutical products as an increasingly large portion of the program's overall prescription drug spend.[34] In the absence of major federal action to bring down high drug prices, most states have sought to address the issue in their respective statehouses and courts and through governors' executive orders, as well as Medicaid waivers, state plan amendments, or Medicaid provider bulletin announcements.[35]

## FEDERAL ACTION ON PRESCRIPTION DRUG PRICING SINCE 2017

The burden of prescription drug prices on patients, employers, taxpayers, and states has grown. The increase in prescription drug spending observed between 2010 and 2014 was only partly attributable to higher pharmaceutical prices.[36] However, in recent years, the increase in prescription drug spending has been mostly attributable to higher drug prices.[37] While the U.S. House of Representatives, several Senate committees, and the Trump administration have identified drug pricing reform as a priority over the past two years, very little action has been finalized that will have a significant impact on lowering pharmaceutical prices.

Since 2017, the president and other administration officials have frequently spoken publicly about the need to address the issue of drug pricing, announcing new initiatives and executive orders, proposing various policies, and calling for congressional action.[38] The administration laid out its *Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs*[39] in May 2018, in addition to issuing annual budget requests that included several proposals to reduce federal and beneficiary out-of-pocket spending on prescription drugs.[40] Ultimately, the FDA and CMS addressed some peripheral barriers to competition in the generic and biosimilar drug markets and began a modest pilot where insulin out-of-pocket costs are artificially lowered for seniors in participating Medicare Part D plans. However, few of the initiatives proposed by the executive branch — including those that do not require congressional action — have been implemented, while the few that were carried out have not produced a measurable impact on drug prices. This includes the administration's September 24, 2020,

announcement that it would create a demonstration that tests sending Medicare beneficiaries a one-time-use prescription drug copay card worth $200 to help offset out-of-pocket costs.[41]

Over the same period, and particularly in 2019, Congress was active in considering legislation to address drug pricing, holding numerous hearings and debating several comprehensive pieces of legislation. The House of Representatives passed legislation to require the Secretary of the Department of Health and Human Services (HHS) to negotiate certain prescription drug prices, among other policies, and key Senate committees advanced legislation meant to address drug pricing. Despite this activity, Congress managed to pass only a few changes into law, with three major bills currently waiting in the wings for potential future action. The successes included some relief for Medicare Part D beneficiaries and other patients at the pharmacy counter. In addition, bills that passed address pharmaceutical company behavior that took advantage of Medicaid rebate policies and revise FDA safety requirements and regulations to lessen barriers to generic drug development. More comprehensive legislation to address high drug prices is either stalled in committee or waiting to be scheduled for debate by the full Senate.

See Appendix A for detailed coverage of Trump administration and congressional action on prescription drug prices from 2017 to 2020.

## ADDRESSING THE DRIVERS OF HIGH DRUG PRICES

As this report lays out, much remains to be done to address the root causes of high and increasing drug prices. Both the administration and Congress have announced various proposals and priorities, but mostly the few changes achieved so far only marginally affect drug pricing. To see real improvement, actions need to be taken to address the following drivers of high prescription drug pricing. There are several policy options within each area that could make an impact. By seeking consensus among stakeholders, policymakers can determine which options are most achievable.

### 1. Allow Government to Become a More Responsible Purchaser

**The U.S. is the only country in the 34-member Organisation for Economic Co-operation and Development that lacks government regulation of prescription drug prices.[42] HHS, unlike the Veterans Health Administration and TRICARE, has few administrative tools to influence utilization management or product selection once Medicare (or a Medicare Administrative Contractor) approves coverage of a treatment. Additionally, Medicare reimbursement policies and benefit design do not incentivize industry self-regulation of brand drug or biologic prices, and they can even thwart the ability of competitors to trigger price reductions.**

***The federal government's restriction on negotiating prescription drug prices.*** Medicare is the largest payer for pharmaceuticals in the U.S. as measured by total spending.[43] The prices that Medicare plan sponsors pay for a medicine may vary greatly and can fluctuate from year to year. In part, this is because Medicare does not centralize the purchase or reimbursement of medicines, even for the most expensive drugs.

Each Medicare plan issuer must negotiate the treatment price with drug manufacturers, wholesale distributors, and pharmacy benefit managers (PBMs). The negotiated price is often influenced by the market share that the plan can provide for the manufacturer and the rebate the PBM or plan can negotiate off the manufacturer's list price. In contrast, the Veterans Health Administration and the Department of Defense's TRICARE health coverage programs negotiate directly with drug manufacturers using inclusion on each program's centralized formulary as leverage.[44] HHS, however, is prohibited by law from negotiating directly with drug companies.

This limitation sets the U.S. apart from other high-income countries, which employ use of centralized drug price negotiations, coverage determination, and drug value to control drug prices and spending.[45] Blocking Medicare from directly negotiating drug prices results in much higher costs for Medicare, patients, and taxpayers

relative to what is paid in other developed countries. The Congressional Budget Office (CBO) has estimated that the federal government could save $456 billion over 10 years by establishing direct drug price negotiation on a selection of the most costly drugs.[46] While most of that savings ($448 billion) would be in the Medicare program, $12 billion would also be saved through lower spending in Affordable Care Act (ACA) individual marketplace plans and federal employee health plans.

***Unreasonable price increases.*** In its June 2019 report, the Medicare Payment Advisory Committee found that Medicare Part B drug spending increases between 2009 and 2016 were partially attributable to "increased prices for existing products."[47] In addition, the Kaiser Family Foundation found that the prices of 60 percent of drugs covered by Medicare Part D increased more than the inflation rate in 2017. The federal government could do more to discourage these drug price increases, such as imposing a penalty on drug companies that increase list prices faster than the inflation rate. In 2019, the House passed and the Senate Finance Committee approved an inflation penalty structured as a rebate from drug companies to Medicare equal to the price differential that exceeded the inflation rate.[48] This approach would deter drug manufacturers from pushing large price increases.[49]

***Misguided Medicare Part B incentives.*** Under Medicare Part B, providers are reimbursed for drugs administered in physicians' offices or other outpatient facilities based on the drug's average sales price plus a percentage add-on. This reimbursement structure incentivizes providers to choose higher-priced drugs in two ways.[50,51] One, providers typically make more revenue on higher-priced drugs because they are often able to negotiate rebates or discounts from manufacturers on these drugs, meaning they pay less for the drug than the average sales price they are reimbursed.[52] Manufacturers are better able to offer large rebates or discounts on higher-priced drugs; generics don't usually pay rebates. And two, the percentage add-on, which is currently 6 percent, ensures that choosing a higher-priced drug results in a larger add-on payment for the physician.[53]

Specialists whose revenue is highly connected to administering drugs as opposed to procedures (for example, oncologists, infectious disease specialists, urologists) have the largest ability to drive drug spending in Part B.[54]

The Medicare Part B reimbursement system also has significant implications for biosimilars since the price of biologics has been driving spending in Part B (see "Build a Sustainable Biosimilar Market to Create Price Competition," below).[55]

***Drug wastage in Medicare Part B.*** When physicians administer treatments, the amount may be less than the total supply contained within a single-use vial. Manufacturing larger dose single-use vials can command higher payment, even if physicians more commonly use a smaller dose than is manufactured. The excess product is discarded, but the government and patient still pay for the entire vial. In 2017, the Centers for Medicare and Medicaid Services (CMS) began to require physicians, hospitals, and other providers to report the exact amount discarded from any single-use vial or package on Medicare Part B claim submissions.[56] CMS has used this data to publicly display which single-use vials are most commonly used, but Medicare does not require drugmakers to provide refunds for the unused amounts. Thus, Medicare currently pays a higher price for drug vials that routinely contain more medicine than is needed or required. In 2016, this amounted to nearly $3 billion spent on drug wastage.[57]

***Part D formulary and benefit issues.*** Part D plans often favor high-cost brand drugs in formulary coverage and placement decisions, as well as utilization management strategies. One study found that among 222 multisource (generic) drugs covered by all formularies in Medicare Part D, 70 percent had more utilization restrictions than reference brand drugs.[58] For instance, CMS could have saved $2.9 billion between 2012 and 2015 if two PBMs, CVS Caremark and Express Scripts, had not excluded generic substitutes from their Part D formularies, only making the reference brand available.[59]

## POTENTIAL CONGRESSIONAL POLICY ACTIONS THAT COULD HELP GOVERNMENT BECOME A MORE RESPONSIBLE PURCHASER

### Leverage Government Purchasing Power

- Direct the HHS secretary to negotiate directly with pharmaceutical manufacturers.
- Benchmark the price of high-cost drugs in Medicare to an external reference price.
- Create a federal/state purchasing pool to negotiate prices for Medicaid or Medicare Part B drugs. The pool could be designated as a PBM.
- Mandate that PBMs update their maximum allowable cost schedule to reflect generic drug price changes.[60]
- Consider limiting Medicare participation for those manufacturers that increase prices above the CPI-U inflation rate.

### Require Drug Manufacturers to Moderate Drug Prices by Passing Through Rebate Savings

- Enact rebates or penalties that manufacturers must pay to the government if they raise the price of a product faster than annual CPI-U inflation rates over the course of a calendar year.
- Change the Medicare Part D standard benefit so that out-of-pocket costs are based on net rather than list prices. Then require that Part D basic plans be actuarially equivalent to the defined standard benefit so that out-of-pocket costs are calculated as a share of net drug prices.
- Tie Medicare Part D rebates to health plan beneficiary cost-sharing amounts.
- Apply a "waste rebate" requiring drugmakers to reimburse Medicare for unused portions of vials.

### Incentivize Higher-Value, Lower-Cost Drugs

- Change Medicare Part B reimbursement to incentivize provider uptake of lower-cost biosimilar products where available.
- Consider making the interchangeable biosimilar the default product (i.e., chosen first over the reference biologic) for Medicare Part B patients starting a biologic regimen.
- Direct CMS to implement the "least costly alternative" policy in Medicare Part B, which bases the payment rate on the lowest cost drug of those drugs that are clinically comparable.
- Ensure Medicare Part B does not pay higher prices for drugs than commercial payers by requiring all calculations used to set Part B reimbursement to include all discounts available to commercial payers.
- Combine multiple brand products for the same indication that have been demonstrated to be of relatively equivalent efficacy in clinical trials into a single reimbursement code.
- Increase health plans' share of Medicare Part D costs or liabilities throughout the Part D benefit.
- Require automatic generic substitution in Medicare Part D.
- Exclude manufacturers' discounts in the Medicare Part D coverage gap from beneficiaries' true out-of-pocket spending.
- Allow CMS to establish an evidence-based national formulary for preferred drugs similar to the Veterans Health Administration, based on safety, efficacy, and cost, in that order.
- Require increased, timely, public-facing, post-market efficacy data submissions, which can be used to assess value and give the FDA more ability to impose penalties on manufacturers that fail to report.

## POTENTIAL ADMINISTRATIVE POLICY ACTIONS THAT COULD HELP GOVERNMENT BECOME A MORE RESPONSIBLE PURCHASER

### Promote the Uptake of Biosimilars and Generic Complex Products in Medicare

- Require Medicare Part D plans to add a formulary tier for biosimilars and allow reference products to make up no more than 10 percent of the products on that tier or require Medicare Part D plans to make biosimilar copays lower than reference biologic copays.
- Require Medicare Part D plans to add FDA-approved generic and biosimilar drugs to their formularies as soon as the generic or biosimilar comes on the U.S. market.
- Clarify FDA guidance on the more complicated approval process for "complex generic" products — generics that have a complex active ingredient, formulation, or delivery mechanism or products that are a drug–device combination.
- Remove step therapy requirements for biosimilars or generic complex products.

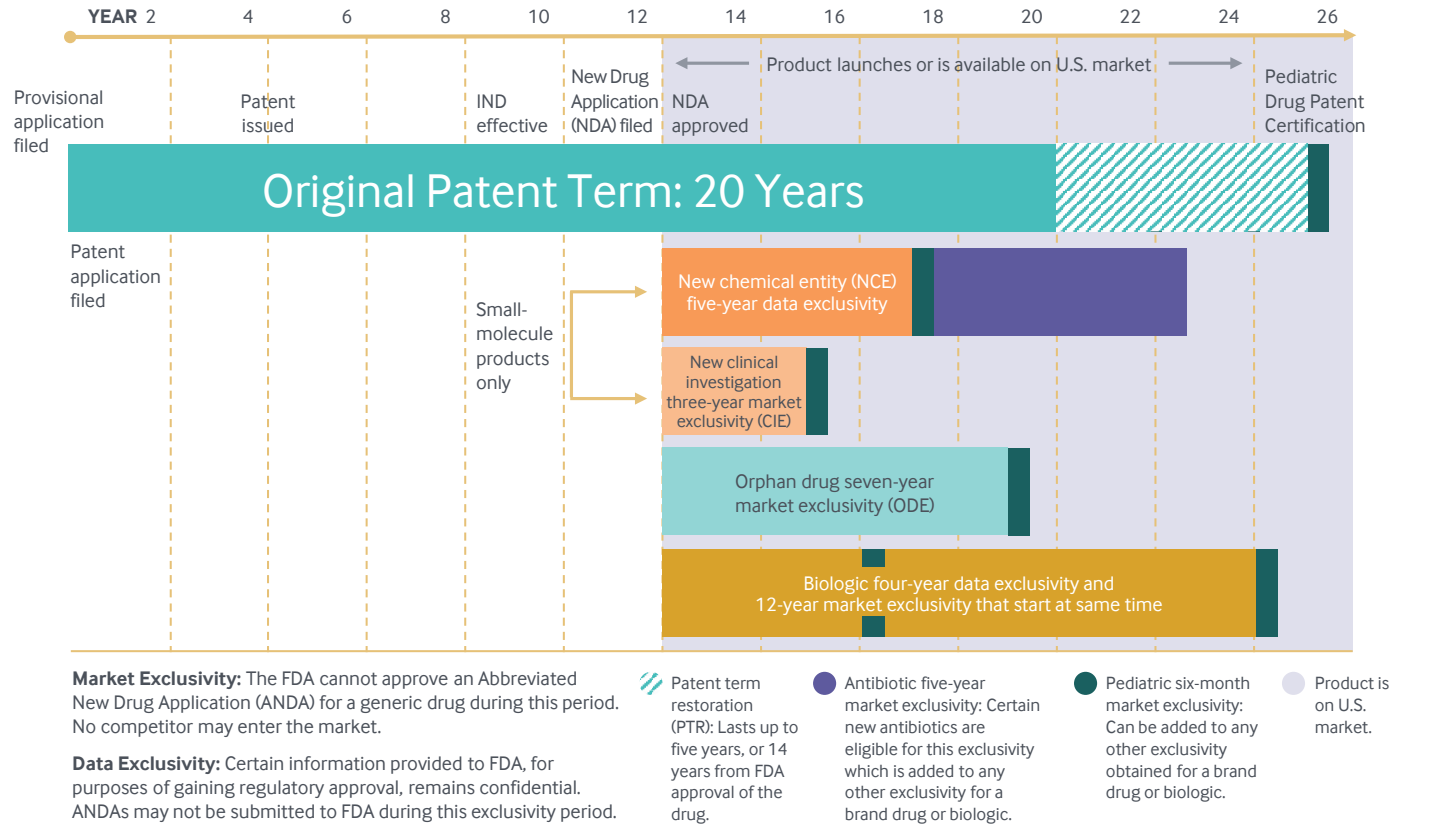
In addition, the design of the Medicare Part D standard pharmacy benefit may be encouraging plans and PBMs to favor high-cost drug products. The more costs a Part D beneficiary incurs in a year, the more quickly she will move through the phases of the Part D benefit: deductible, initial coverage, coverage gap, and catastrophic coverage. Once the beneficiary reaches the catastrophic phase, the federal government is liable for 80 percent of patient drug costs and plans are responsible for 15 percent. Thus, insurers with drug formularies that favor patients choosing high-priced drugs are more successful at shifting their liability more quickly to the federal government, creating a perverse incentive.[61] This practice not only crowds out generic and biosimilar manufacturers from the market, it raises costs for the Medicare program and enables and even rewards drugmakers that keep drug prices higher than competitor products, costing Americans in the long run. The role of rising drug prices is illustrated by the Part D drug spending rate consistently increasing at a much higher annual rate than the number of Part D prescriptions filled.[62]

## 2. Stop Patent Abuses and Anticompetitive Practices That Block Price Competition

**Pharmaceutical companies use patent and market-exclusivity laws to block product competition, sometimes for decades, enabling high annual price increases even when there have been no significant improvements to the drug or biologic.**

***Patent gaming and market exclusivity challenges.*** The Drug Price Competition and Patent Term Restoration Act of 1984 (commonly referred to as the Hatch-Waxman Act) sought to encourage drug development and medical innovation, while also promoting price competition that would make prescription drugs more affordable. Its passage quickly led to a substantial U.S. generic drug market that made medicine much more affordable for patients. In 1984, only 35 percent of the top-selling drugs in the U.S. had generics after patent expiration, and generic prescriptions made up only 19 percent of total prescriptions.[63] By 2016, generics constituted 89 percent of all prescriptions written and almost all top-selling drugs

Exhibit 1. Original Patent Term Protection and FDA Exclusivities for Brand Drugs and Biologic Products



had generics after patent expiration.[64] Despite generics accounting for roughly nine in 10 of prescriptions, they make up only 27 percent of U.S. drug spending, indicating how affordable generic prices are relative to brand and biologic products.[65]

The FDA, however, can only approve generic drugs when there are no patents blocking an approval, and when all market exclusivities for the reference brand drug have expired.[66] When Congress passed the Hatch-Waxman Act, policymakers figured that 14 years of market monopoly for a drug is the maximum number of years a brand would need to generate a motivating return on investment. However, now it is not uncommon for blockbuster and other popular drugs to have a market monopoly for two decades, if not more.[67]

This results from various gaming strategies, including excessive patenting of the same general product and, to a lesser extent, stacking of market exclusivities provided by the FDA through the Federal Food, Drug, and Cosmetic Act.[68] Brand and biologic pharmaceutical manufacturers use patent protections and exclusivities as a first-line defense to block or delay generic and biosimilar price competition.[69]

In 2018, the top 12 best-selling drugs in the U.S. had already been on the market for 20 years and continued to have patent applications filed to extend protection for several more years.[70] Herceptin, one of the top-selling drugs for many years, had patents first filed in 1985 and its market monopoly extended until 2019, equating to 34 years without competition[71] (Exhibit 2).

## Exhibit 2. Patent and Pricing History for Selected Top-Selling Prescription Drugs

| Product | Year First Patent Filed for Product | U.S. Patents Granted / U.S. Patent Applications | Years Patents Could Block Competition | Year Product Gained U.S. Generic Competitor | On the U.S. Market Since | Wholesale Acquisition Cost (WAC) Compound Annual Growth Rate (CAGR) Since Launch | WAC Price Increase 2012–2018* | WAC Price Increase From Launch | Product Type |
|---|---|---|---|---|---|---|---|---|---|
| **Herceptin** | 1985 | 108 / 186 | 48 | — | 1998 | 4.5% | 46% | 112% | biologic |
| **Enbrel** | 1990 | 39 / 68 | 48 | — | 1998 | 8.8% | 169% | 531% | biologic |
| **Humira** | 1994 | 132 / 247 | 39 | — | 2002 | 9.9% | 172% | 432% | biologic |
| **Lantus** | 1994 | 49 / 74 | 37 | — | 2000 | 11.4% | 136% | 715% | biologic (insulin glargine) |
| **Lyrica** | 1995 | 69 / 118 | 42 | — | 2004 | 11.2% | 165% | 396% | small molecule |
| **Copaxone** | 1995 | 68 / 143 | 44 | 2015 | 1996 | 9.9% | 105% | 825% | complex small molecule |
| **Imbruvica** | 2006 | 88 / 165 | 29 | — | 2013 | 8.0% | 49% 2013 launch–2018 | 69% | small molecule |

Sources: I-MAK, *Overpatented, Overpriced: How Excessive Pharmaceutical Patenting Is Extending Monopolies and Driving Up Drug Prices* (Aug. 2018); I-MAK, *Overpatented, Overpriced: Special Humira Edition* (Aug. 2018); I-MAK, *Overpatented, Overpriced: Special Edition: Lantus* (Oct. 2018); I-MAK, *Overpatented, Overpriced: Special Edition: Enbrel* (November 2018; I-MAK, *Overpatented, Overpriced: Data on America's Top 12 Drugs* (March 2020); I-MAK, *Overpatented, Overpriced: Imbruvica's Patent Wall* (July 2020).

Sources for Price Increases: Price increases and CAGR were calculated using wholesale acquisition cost (WAC) prices from Medi-Span Price Rx database. In the case of Copaxone, WAC pricing data was compiled using Medi-Span Price Rx, Medi-Span Price Rx Inactive, and Gold Standard Elsevier Drug Database.

## POTENTIAL CONGRESSIONAL POLICY ACTIONS THAT COULD STOP PATENT ABUSE AND ANTICOMPETITIVE PRACTICES

### Require More Responsible Patent Licensing/ Discourage Patent Abuse

- Require automatic review of secondary patents by the U.S. Patent and Trademark Office's (USPTO's) Patent Trial and Appeal Board when they are provided to the FDA.
- Require demonstration of greater clinical benefit for new formulation patents or other stricter patenting standards.
- Reduce Medicare Part B reimbursement for a brand drug if the manufacturer enters into a pay-for-delay settlement. Alternatively, create stiffer penalties.
- Reduce the USPTO's reliance on post-grant user fees, or patent issuance and renewal fees, which account for nearly all of the USPTO's revenue.[72]
- Prevent patents from being approved for changes based on common pharmacological experimentation and knowledge.
- Similar to what other countries have done, raise the patent standards for additional uses of existing compounds contained within a product.[73]
- Strengthen the USPTO *inter partes* review as well as the process for evaluating patent validity.[74]
- Codify the definitions of "product hopping," "patent thicketing," "evergreening," and "secondary patent" to empower the Federal Trade Commission (FTC) to challenge these actions as anticompetitive. (See Appendix C for more on these terms.)
- Mitigate product hopping by requiring manufacturers to keep the original formulation of the branded product on the market past the date of generic entry to ensure sufficient market share can move to the generic product.
- Allow and provide additional funding for the FTC to use its equitable remedy authority to keep companies from engaging in patent abuse.
- Limit the number of continuing patent applications that may be filed for the same invention.
- Increase the time patent examiners spend reviewing patent applications.
- Publicly identify all patents relating to a compound in one centralized, publicly accessible, online database.
- Prohibit overuse of the citizen petition process by limiting the number of petitions that may be filed per year for a drug.
- Prohibit citizen petitions that are usually found to be frivolous or fine manufacturers when their citizen petition is found to be frivolous.
- Fine any manufacturers that file a citizen petition found to be frivolous or for the primary purpose of delaying generic or biosimilar approval.
- Codify the requirement that a citizen petition is to be submitted within 60 days after the petitioner knew or reasonably should have known the information forming the premise of the petition.

### Build More Competitive Markets

- Reduce biologic exclusivity from its current 12 years.
- Grant a five-year New Chemical Entity market exclusivity only if a product's molecular structure contains a significant change from the existing drug.
- Revisit which types of products (like off-patent drugs) need additional exclusivity periods.
- Require demonstration of greater clinical benefit for granting the New Clinical Investigation exclusivity.
- Examine whether FDA exclusivities should be reformed.
- Review the FDA's market and data exclusivities and how they are applied to ensure sufficient balance between rewarding scientific innovation and promoting competition.

## POTENTIAL ADMINISTRATIVE POLICY ACTIONS THAT COULD STOP PATENT ABUSE AND ANTICOMPETITIVE PRACTICES

### Allow Smarter Oversight of Patent Approvals

- Require manufacturers to share more data in patent applications.
- Increase scientific expertise of USPTO examiners to allow them to review patents with greater efficiency.
- Establish a Patent Quality Taskforce to provide recommendations to Congress.
- Request to adequately fund the USPTO in the president's budget.
- Reform the citizen petition process to prevent abuse.

### Encourage a More Competitive Market

- Maximize the use of the USPTO Patent Trial and Appeal Board.
- Have the FTC, Department of Justice (DOJ), and FDA evaluate the sustainability of the generic drug market and provide recommendations to Congress.
- Prioritize funding for the FTC to challenge patent settlement rules and to review settlements.
- Publish guidance on FDA-designated "complex generic" products, which have a more complicated approval process than traditional generic drugs.

The length of the monopoly period that originator products enjoy has grown substantially in the last 35 years as drugmakers invest foremost in finding new ways to extend a product's life cycle. Contraction of company product pipelines, coupled with brand products experiencing rapid price deflation when generic competitors enter the market, has led brand manufacturers to focus on product life-cycle management strategies, such as finding new ways to patent the same product.

While rewarding invention is important, decades of market protection allow brand and originator biologic product prices to increase uninhibited, even when there have been no new significant innovations or discoveries associated with the product to justify the long monopoly period. To bring down drug prices and encourage greater scientific innovation, market monopolies must not be allowed to continue for decades. (See Appendix C for details on patent-gaming strategies and types of exclusivities granted by the FDA.)

***Manufacturers' use of citizen petitions.*** One way drug manufacturers extend a product's life cycle is through the FDA's citizen petition process, which allows interested parties (including competitors) to bring concerns to the FDA's attention or request that the agency take certain actions on generic or biosimilar approval applications, such as rejecting an application or delaying a generic approval. Other actions could include requests to apply over-the-counter status to a prescription drug or require a warning label on a drug product's packaging. Manufacturers filed over 90 percent of citizen petitions between 2011 and 2015,[75] and the FDA has expressed frustration that the process can take up valuable human resource hours and slows down the agency's work.[76] Deterring or allowing the FDA to deny citizen petitions more quickly would speed competitor products to market and lower overall pharmaceutical costs in public programs.[77]

## 3. Build a Sustainable Biosimilar Market to Create Price Competition

**Barriers to biosimilar commercialization and adoption in the U.S. have led to a biologic market with inadequate competition, a shrinking biosimilar pipeline in the U.S., and increasingly high drug spending in Medicare Part B, Medicaid, and commercial insurance markets.**

Some of the highest-priced drugs on the market today are biologics, and most of them have no competition to date. Biologics are more expensive to develop, produce, and use compared to small-molecule drugs, in part because the active ingredients in biologics are large molecules, or proteins modeled after those made within living systems, such as microorganisms or plant cells. Biologics also tend to be physician-administered drugs that are injected or infused, which makes their manufacture, distribution, and storage different from small-molecule drugs. While small-molecule drugs can be simpler to produce, the complexity of biological manufacturing necessitates several process patents, in addition to patents on agents, which offer additional market protection for these products. A biologic manufacturing facility takes $200 to $400 million to build, and the costs of the materials to manufacture biologics may be 20 to 100 times more than those for chemical drugs.[78]

The ACA created the first abbreviated pathway in the U.S. for biosimilars, or highly similar biological products, to be FDA-approved. The ACA also created a 12-year market exclusivity period for reference biologics, during which a biosimilar may become FDA-approved, even if it cannot enter the market.[79] Unlike generic small-molecule drugs, pharmacy-level biosimilar substitution is only permitted with the approval of a physician, or if the biosimilar has received approval from the FDA as interchangeable with the biologic. To date, there are no approved interchangeable biologic products, creating a barrier to more widespread use of biosimilars.

## POTENTIAL CONGRESSIONAL POLICY ACTIONS THAT COULD CREATE A MORE COMPETITIVE BIOSIMILAR MARKET

### Change Medicare Reimbursement and Cost-Sharing

- Adjust Medicare Part B reimbursement for biosimilars and originator biologic products to incentivize the use of the biosimilar over the originator biologic.
- Modify copayments for Medicare beneficiaries with incomes at or below 135 percent of the federal poverty level to encourage the use of biosimilars when available.
- Direct HHS to reduce or eliminate cost-sharing for biosimilars.

### Shorten Patent and Exclusivity Periods That Block Biosimilar Market Entry

- Shorten the biologic FDA market exclusivity period from its current 12 years.
- Cap the number of years a biologic may enjoy cumulative patent protection or shorten patent extension periods.

### Lower the Cost of Biosimilar Development

- Provide tax incentives or subsidies to develop biosimilars but only to manufacturers unaffiliated with the originator biologic.

## POTENTIAL ADMINISTRATIVE POLICY ACTIONS THAT COULD CREATE A MORE COMPETITIVE BIOSIMILAR MARKET

### Change Medicare Reimbursement

- Create a shared Medicare Part B reimbursement billing code for both a reference biologic and all corresponding biosimilars.

### Lower the Cost of Biosimilar Development

- Consider allowing biosimilar manufacturers the option of using studies comparing the biosimilar to a non-U.S.-licensed reference product during the FDA approval process.
- Review the current development paradigm for biosimilars, which is based on the totality of evidence, with a more efficient paradigm, such as a confirmation of sufficient likeness. The goal would be to emphasize analytical likeness rather than specific studies between a biosimilar and an originator.[80]

### Encourage Growth of Biosimilar Market Share via Physician Education

- Scale up the FDA's physician education campaign on the safety and efficacy of biosimilar products and interchangeability.
- Revisit the FDA's nonproprietary label name guidance for interchangeable biosimilars to encourage switching or substitution.[81]
- Incentivize clinical trials or require and enforce post-market surveillance and outcome reporting of patients who switch from a biologic to a biosimilar product.

### Remove Barriers to Biosimilar Uptake by Medicare Beneficiaries

- Encourage health plans to lift prior authorization requirements for biosimilars.
- Prevent Medicare plans from requiring patients to fail first on the (rebated) originator biologic before the plan will cover the biosimilar.
- Require Medicare plans to add FDA-approved biosimilar drugs to their formularies as soon as the biosimilar comes on the U.S. market.
- Require Medicare Part D plans to favor a biosimilar over a reference biologic via formulary management or copay rules.
- Require automatic interchangeable substitution in newly diagnosed Medicare beneficiaries.

Currently, the U.S. biosimilar market has three main obstacles to becoming sustainable[82]:

***Cost of FDA approval.*** The biosimilar (and interchangeable) pathway requires less data from manufacturers about their products than is required for a new biologic. But the pathway still requires far more data and costly clinical research than a generic drug; a biosimilar is about eight to 100 times more expensive to develop and approve than a generic drug, on average.[83] Thus, strong market incentives are important for biosimilar manufacturers in the same way they are for biologic and brand manufacturers.[84]

***Blocked market access.*** Biosimilars must compete against reference biologics that have patents and market exclusivities. High-priced biologics that have little if any market competition make up the majority of Medicare Part B drug spending.[85] Price inflation for biologics in Part B was 54 percent between 2006 and 2018, outstripping the pace of price growth for other drugs in Part B.[86] The top 10 most expensive drugs in Part B are reference biologics.

***Commercialization challenges.*** The FDA has made progress in recent years, tripling the number of biosimilar approvals to 26 total with 16 biosimilars reaching the U.S. market.[87] For some biosimilars, commercialization has been delayed or manufacturers sued for patent infringement.[88] This is far from the situation in Europe, where regulatory agencies have approved more biosimilars, and biosimilars have been able to achieve a healthy market share.[89] Many of the patent abuse activities discussed in this report are also used by biologic manufacturers to block biosimilar market entry.

Additionally, current reimbursement policy may not sufficiently encourage uptake of biosimilar products over biologics. When a generic enters the market, uptake of the generic over the brand happens relatively quickly, usually within three years.[90] However, current Medicare reimbursement policies still reward physicians and hospitals with higher reimbursement for brand reference biologics than for the available biosimilar competitors, creating an incentive for physicians to continue to prescribe the higher-price product. Additionally, physician discomfort with using biosimilar products is another obstacle to uptake.[91] In recent years, the FDA started an education campaign to try to change physician perceptions.[92]

Finally, FDA requirements for naming and labeling biosimilars and interchangeables highlight the differences between those products and their reference products. These policies may make providers uncertain about whether they should prescribe the biosimilar and, therefore, hinder or deter substitution. This may also make it more difficult for pharmacies to substitute interchangeables without consulting the physician.[93]

## 4. Fix Incentives in the Drug Supply Chain and Make the Drug Supply Chain More Transparent

**Contractual arrangements among wholesale distributors, PBMs, and insurers favor high-priced drugs that allow for kickbacks to be paid, create a market barrier for competitors, and erode the negotiating power of smaller insurers and independent pharmacies.**

The pharmaceutical supply chain is what moves a pharmaceutical product from the manufacturer to the patient. It is made up of multiple arrangements that differ based on whether a product is:

- Branded, generic, or biologic
- Complex,[94] compounded, or small-molecule
- Administered or dispensed.

***Contractual pressures and restrictions.*** Contracts between manufacturers and PBMs or large health plans are proprietary. They can be designed to stifle price competition not only by deterring and excluding generic and biosimilar products, but also by favoring the highest-priced drug in a group of therapeutically equivalent or interchangeable products.[95] Closed-door negotiations at multiple levels along the supply chain help to obfuscate the true cost of prescription drugs, effectively allowing drug coverage decisions to be heavily weighted toward higher-priced drugs that afford higher rebates. These contracts effectively block lower-priced therapies from gaining market share.

> ***Manufacturers are using several schemes to hamstring biosimilar competition. . . . [R]estrictive contracting, rebating, and distribution agreements deter coverage and reimbursement. . . . [T]he net result is a lopsided playing field that disincentivizes biosimilar developers from making the sizable investment in bringing such products to market. I am concerned this will lead to reduced competition in the long run and unsustainable costs.***[96]
>
> **Scott Gottlieb, M.D.**
> ***Former FDA Commissioner***

Some of the tools used by manufacturers to influence the supply chain include but are not limited to:

- Rebates to sway formulary and coverage decisions by PBMs and insurers
- Discounts for high-volume orders with PBMs or prompt payment by wholesalers
- Dispensing fees to PBMs and pharmacies.

Some contracts even impose restrictive arrangements that threaten to withdraw existing rebates to payers unless the complex generic or biosimilar is effectively excluded from formulary tiers that offer lower cost-sharing or from formulary coverage altogether.[97]

Over time, this structure drives up the total cost of pharmaceutical spending for patients and taxpayers over the long run.[98] Moreover, in creating an anticompetitive environment, prices stay high and continue increasing, as entities along the supply chain pass the balance of the higher cost on to patients and taxpayers.[99]

***Prices not necessarily tied to value.*** Contracts along the supply chain that offer manufacturer rebates and discounts to payers, including PBMs and health insurers, have become a line of defense that brand and biologic manufacturers use to protect their market share after patent protections and market exclusivities have been exhausted and a competitor finally reaches the U.S. market. These various agreements create wide price variability for the same product, so that product price is often more dependent on coverage or geographical markets than on efficacy or value.

Rebates paid by manufacturers can make up 40 percent or more of a drug's list price.[100] Between 2010 and 2019, rebates paid by drug manufacturers to Medicare Part D plan sponsors grew by over 18 percent (Exhibit 3).[101] Rebates made up 25 percent of Part D drug spending in 2018, up from 11 percent in 2008.[102] However, these price concessions corresponded with an 18 percent average annual increase in cost liabilities to taxpayers over the same period.[103]

***No fiduciary duty on part of PBMs.*** Importantly, rebate savings that PBMs and plans receive from manufacturers generally do not get passed on to patients, public program beneficiaries, or taxpayers.[104] These secret PBM rebate agreements solely help payers pay lower net drug prices and manufacturers secure favorable formulary placements.[105] Thus, there is no requirement that PBMs have a legal obligation, or fiduciary duty, toward the entity for which they are managing pharmaceutical benefits, nor do they have an obligation to act in the best interest of the consumer.[106] Policymakers have debated whether imposing fiduciary duty on PBMs would prevent them from capitalizing on incentives that may ultimately drive up costs for plans or patients.[107] Any action around this topic should consider the effect of vertical integration between PBMs and insurers and PBM consolidation in general.[108]

### THE PHARMACEUTICAL DRUG SUPPLY CHAIN: KEY TERMS

**Drug Rebate:** A negotiated payment from a pharmaceutical manufacturer to a PBM, which then shares a portion (usually at least 90%)[109] with the health plan sponsor. Rebates are given in exchange for the manufacturer's product being favored by the PBM or plan over a competitor product. For example, rebates can incentivize a payer to cover a drug in a formulary, place the drug on a formulary tier with a low copay, or waive utilization management or step therapy protocols. The rebate amount is the difference between a drug's list price and the drug's net price.

**Dispensing Fee:** A negotiated fee paid by pharmaceutical manufacturers to PBMs and retail pharmacies for inclusion in pharmacy networks or inventory. The dispensing fee may be a flat fee, or it may be a percentage of the medicine's list price. The dispensing fee accounts for the majority of PBM revenue in Medicare Part D.[110]

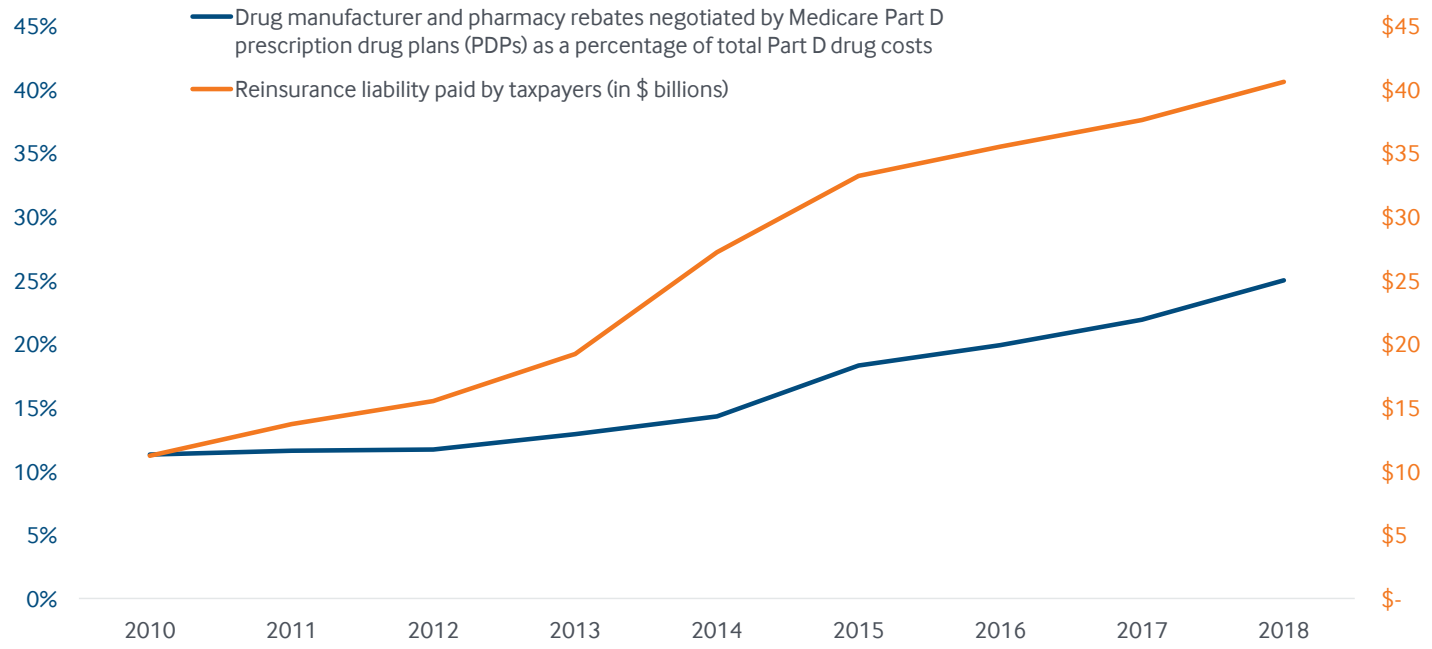
**Chargeback:** A discount that drug manufacturers agree to give a pharmacy or customer on a drug. The wholesale distributor then charges the manufacturer the price difference between the discount given to the customer and the price the wholesaler negotiated with the manufacturer, known as the wholesale acquisition cost. This arrangement prevents the wholesaler from losing money when pharmacies pay a lower price than the WAC.

**Point-of-Sale Discount:** When a portion of the negotiated rebate paid to PBMs by pharmaceutical manufacturers is passed through to patients making a purchase during the deductible phase of coverage or when paying a coinsurance amount for a prescription.

**Prompt Pay Discount:** A payment from a pharmaceutical manufacturer to a wholesale distributor that provides a discount off the purchase price if a manufacturer receives payment from the wholesaler within a specified time frame.

**Volume or Market Share Discounts:** Payments by a pharmaceutical manufacturer to a wholesale distributor that provide a discount off the PBM purchase price based on the PBM or pharmacy selling a specific volume of the manufacturer's drug or drugs, or achieving a certain share of a specified market (for example, the Medicare Part D market).

## Exhibit 3. Growth of Medicare Part D Prescription Drug Plan Rebates and Taxpayer Liability




Data: Boards of Trustees, Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds, *2020 Annual Report of the Board of Trustees of the Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds*, April 22, 2020, Table IV.B8 and Table IV.B10.

## POTENTIAL CONGRESSIONAL POLICY ACTIONS THAT WOULD FIX INCENTIVES IN THE DRUG SUPPLY CHAIN

### Prohibit Anticompetitive Practices

- Prevent PBMs from receiving remuneration from manufacturers tied to a drug's list price.
- Ban anticompetitive contracting clauses between manufacturers, PBMs, and plan sponsors.
- Prohibit kickbacks or manufacturer rebates to PBMs and payers that are tied to preferential formulary placement or utilization management of competitor products and are not completely passed through to patients.
- Require PBMs and plans to pass through manufacturer rebates at the point of sale or time of purchase to reduce out-of-pocket costs for patients.

### Prevent Perverse Incentives in the Drug Supply Chain

- Remove the tax deduction that drug manufacturers use for spending on direct-to-consumer advertising.
- Levy taxes on drug company spending on direct-to-consumer broadcast advertising.
- Require PBMs to act in the interest of and have fiduciary responsibility for those for whom they are managing pharmaceutical benefits (that is, patients).

### Increase Transparency in the Drug Supply Chain

- Require retail pharmacies to regularly report their average acquisition costs of drugs to CMS to ensure a more robust National Average Drug Acquisition Cost dataset.
- In the commercial market, ban patient assistance programs, manufacturer copay coupons, and loyalty cards used at the point of sale. (Copay coupons are already banned in Medicare and Medicaid.) Alternatively, require pharmaceutical companies to report information on these programs to regulators.
- Require and fund the FTC and DOJ to publish a joint report on the effect of mergers and acquisitions of PBMs, retail pharmacy chains, and insurers on drug purchasing, distribution, and pricing.
- Require PBMs to disclose all direct and indirect compensation to the government and plan sponsors.

## POTENTIAL ADMINISTRATIVE POLICY ACTIONS THAT WOULD FIX INCENTIVES IN THE DRUG SUPPLY CHAIN

### Prohibit Anticompetitive Practices and Promote Competition

- Prohibit manufacturers from setting or paying fees, rebates, or discounts in contracts with plans and PBMs that are calculated based on or relative to a drug's price.
- Prohibit manufacturers from paying rebates or giving discounts in exchange for contracting that is exclusionary or anticompetitive.
- Prohibit PBM clawbacks, or collecting payments extracted from drug manufacturers tied to preferential formulary placement and not passed through to the payer or the patient.
- Promote use of evidence-driven formularies in Medicare and Medicaid to promote brand–brand competition.
- Encourage plans in the federal health insurance marketplace to maximize generic and interchangeable substitution.

### Promote Oversight and Transparency in the Drug Supply Chain

- Expand oversight by HHS, the Office of Inspector General, and the Government Accountability Office of anticompetitive supply chain discounting arrangements.
- Ban brand and biologic drug price copay coupons or accumulators in the federal individual marketplace.
- Require wholesalers to report exclusive purchasing agreements or contracts to the FTC for monitoring.
- Establish supply chain transparency and reporting requirements for PBMs, wholesalers, and pharmaceutical manufacturers.

## 5. Ensure Public Accountability in the Government-Funded Drug Development Process

**The pharmaceutical research and development process is heavily subsidized by the federal government, but private companies that bring pharmaceuticals to market do not account for this support in pricing their products.**

***Federal funding of early-stage research.*** Federal agencies spent $43 billion in 2018 on medical and health research and development, much of it on competitive grants given for early-stage research.[111] Findings from federally funded research are the basis for the product development work done by private pharmaceutical companies.[112] U.S. tax dollars, allocated through grants from the National Institutes of Health (NIH), were used in the scientific discovery of every pharmaceutical product approved by the FDA between 2010 and 2016.[113]

Promising drug-related discoveries are bought or licensed by pharmaceutical companies, which then develop formulations to test via time-consuming clinical trials with the hope of successfully meeting research goals or demonstrating safety and efficacy. Companies then bring successful formulations to market — increasingly with assistance from federal applied-research scientists — often launching new medicines at high prices.[114] Drugmakers generally do not invest in the riskiest, early-stage phase of research (known as basic research), which is most often funded with NIH grants.[115]

***Tax incentives for drug development.*** In addition to funding scientific findings via grants, the federal government encourages drug development by providing tax incentives. Drugmakers may write off some of the amount they spend each year on research and development using one or a combination of three different mechanisms (see the box on page 21 for more information):

- The Orphan Drug tax credit
- The research and development tax credit; and
- The deduction of research or experimental expenditures from gross income.

### EXISTING FEDERAL AUTHORITY TO LOWER DRUG PRICES OR INCREASE ACCESS

***March-in rights:*** The Bayh-Dole Act of 1980 includes a provision that allows federal agencies march-in rights, or the ability to cancel a company's exclusive license if a product is not reasonably priced. If a license is canceled, the government must reasonably compensate the patent holder (for example, by paying royalties). The NIH has never invoked march-in rights.[116] Debate continues about potentially amending the provision to make it more accessible to policymakers as a tool to reduce drug prices.

***Reasonable pricing requirement for NIH exclusive license agreement:*** Similarly, an NIH policy to add a "reasonable pricing" clause to Public Health Service exclusive license agreements with drug manufacturers, which created an upfront price accountability mechanism, was abandoned altogether by the agency in 1995 after six years, following an industry lobbying effort.[117] The NIH director at the time, Harold Varmus, stated the clause deterred industry collaboration with federal researchers, distracted from the NIH's research mission, and conflicted with its statutory mission to transfer technologies to the private sector for commercialization.[118]

***Section 1498 compulsory licensing:*** Under federal patent law, the government can manufacture a product owned by a company for the purpose of serving a public good. Section 1498 of 28 U.S. Code allows the government sovereign immunity, or access to the intellectual property of any product under patent without the consent of the patent or intellectual property owner. This grant of a compulsory license may happen as long as the government provides reasonable compensation to the patent owner.. The authority was last used in the 1970s but has been nearly invoked a few times in recent years. For instance, in 2001, HHS discussed using Section 1498 to obtain the generic antibiotic ciprofloxacin, or Cipro, as a potential treatment for anthrax exposure. The government has successfully leveraged the possibility of invoking Section 1498 to negotiate with manufacturers a reduced price for specific drugs.

***Consumer payments to the industry.*** Because of these different federally funded and tax expenditure programs, the American people pay four times for drugs:

- Through taxpayer-funded research
- Through the corporate tax code
- As a consumer purchaser of the drug
- Through taxpayer-funded drug coverage under Medicare, Medicaid, TRICARE, and other federal and state health programs.

***Manufacturers' research and development priorities.*** Despite federal subsidies, some drug manufacturers claim high research and development costs as justification for high launch prices and price increases. However, on aggregate, marketing expenditures by private companies are greater than their spending on research and development.[119] Even though 90 percent of all drugs that enter human testing fail, the majority of these failures occur early and at relatively low cost.[120] Importantly, pharmaceutical company revenue after product approval far exceeds the money manufacturers spend on research and development.[121]

Due to the uncertainty inherent in the drug discovery process, manufacturers find it less risky to use the research and development tax credits to prolong or extend an existing product's market monopoly. Novel drugs approved by the FDA only accounted for between 8 and 18 percent of total drug approvals between 2005 and 2015.[122] During the same period, over 75 percent of drugs associated with new patents were for products already on the market.[123]

Increasingly, manufacturers are focusing their drug development efforts on products, such as biologic and rare-disease drugs, that are more specialized and treat smaller groups of patients.[124] In 2018, 58 percent of new pharmaceuticals approved by the FDA were for a rare disease indication, and 29 percent were for a biologic.[125] Both orphan and biologic products are able to retain monopoly periods longer and more easily dissuade generic competition because the markets for these products are small, and generic drug margins thrive on volume. Not coincidentally, these product categories are where much of the high drug spending is concentrated.

### THREE WAYS THE TAX SYSTEM INCENTIVIZES DRUG DEVELOPMENT

***Research and development credit:*** Companies may claim a nonrefundable tax credit of 14 percent to 20 percent for qualified research expenditures spent over a year that exceed a base amount. Disqualifying a base amount from inclusion in the calculation of the tax credit amount is meant to incentivize companies to conduct research beyond what they otherwise would. Qualified research is generally anything related to the process of experimentation or development of new or improved functions, performance, reliability, or quality.[126]

***Deductions of qualified research expenses:*** The tax code allows companies to deduct "research or experimental expenditures" from gross income in the tax year the research and development is conducted. This deduction is inclusive of any research dollars spent outside the U.S. The deduction is given in place of the company having to depreciate assets created by research and development over time. However, any deductions must be reduced by the amount of tax credits claimed to avoid double-dipping on the same expenses.

***Orphan drug credit:*** For drugs indicated for rare diseases, companies may claim a nonrefundable orphan drug credit equal to 25 percent of the amount spent for qualified clinical testing expenses paid or incurred during the tax year. This includes clinical testing that happens outside the U.S. if there is an insufficient population in the U.S. on which to test a drug. A manufacturer can claim either the orphan drug credit or the research and development credit for the same clinical testing expenses, but not both.

## POTENTIAL CONGRESSIONAL POLICY ACTIONS THAT COULD ENSURE ACCOUNTABILITY IN DRUG DEVELOPMENT

### Increase Transparency of Drug Development Costs

- Require manufacturers to publicly disclose their drug development costs.
- Require manufacturers to repay the tax credits and research grants they receive for developing a drug if revenue from the drug exceeds a particular threshold; this may require manufacturers to report annual revenues for drugs to the federal government.
- Amend the march-in construct in the Bayh-Dole Act of 1980 to require additional reporting of federal funds throughout the research and development process.

### Tie Public Funding Incentives to the Value of the Drug

- Condition incentives on manufacturers agreeing to set a government-validated value-based price.

### Prevent Overpricing on Products Developed with Public Funds

- Create fairer royalty agreements with taxpayer-funded institutions that lead to successful product development.[127]
- Direct HHS to negotiate prices with drug manufacturers for products that benefit from public-funded research.
- Amend the Bayh-Dole Act and other relevant statutes to clarify how to determine a reasonable price for products developed with government funding.
- Amend the Bayh-Dole Act and other relevant statutes to clarify the process for and conditions necessary for the government to use march-in rights.

### Explore Alternate Drug Development Pathways and Improve Existing Drug Development Incentive Tools

- Remove barriers that encumber nonprofit pharmaceutical organizations from participating in U.S. drug development and commercialization.
- Explore policies that incentivize data-sharing or open science models among drug developers.[128]
- Use the tax code to incentivize high-risk drug development, such as for neurological conditions.
- Reward manufacturers of wholly new products with direct tax incentives in exchange for marginal cost pricing.[129]

### Review Tax Incentives for Drug Development

- For the research and development tax credit, clarify how companies calculate the base amount of qualifying research to exclude activities companies would have done anyway and are unnecessary for taxpayers to fund.
- Allow smaller biotech firms with net operating losses tax incentives for drug development in lieu of tax credits, which they cannot take advantage of.[130]
- Comprehensively review all the tax incentives given to drug manufacturers and biotech firms to ensure they are equitable and advance competition and needed drug development.
- Modify research and development tax incentives so that they provide a greater incentive for novel drug development with higher investment risk or products for neglected diseases.
- Raise the threshold for qualifying research activities and raise the level of documentation needed to support research and development tax credits.

## POTENTIAL ADMINISTRATIVE POLICY ACTIONS THAT COULD ENSURE ACCOUNTABILITY IN DRUG DEVELOPMENT

### Reexamine the Processes for Licensing Results of Government-Funded Research

- Impose conditions on government technology transfer rules under the Bayh-Dole Act.
- Issue guidance to clarify how to determine what is a reasonable price for products developed with government funding.
- Issue guidance and regulations to clarify the process for and conditions necessary for the government to use march-in rights.

For example, orphan drugs are estimated to have a higher success rate than nonorphan drugs. They also cost less to develop. After smaller trial sizes and tax advantages are factored in, orphan drugs cost less than half ($0.8 and $1.0 billion) of estimated median drug development costs ($1.9 billion and $2.6 billion).[131] This is a significant change from 1983, when the Orphan Drug Act was enacted and pharmaceutical manufacturers rarely invested in rare-disease drugs because these products were believed to be unprofitable.

GAO experts surveyed in 2017 reported that manufacturers' focus on niche markets came at the expense of less lucrative disease areas.[132] The current incentive structure for drug development enables a focus on those drugs with naturally less competition, which can lead to a focus on products for narrower populations or personalized medicine rather than products important for public health.[133]

***Merger and acquisition growth model.*** Finally, despite the government contribution to pharmaceutical research and development, tax incentives can be dwarfed by the ability of large drugmakers to acquire smaller biotech firms or the intellectual property of promising products that small firms have developed.[134] This is one factor contributing to stagnant research and development spending by the largest drug companies for more than a decade.[135] Pharmaceutical manufacturer spending on research in the U.S. peaked at $25.5 billion in 2007, subsequently declining through 2014, according to the latest data available.[136] Members of the Pharmaceutical Research and Manufacturers of America (PhRMA), a pharmaceutical trade group, reported worldwide spending on both research and development grew from $53 billion to $54 billion between 2008 and 2014.[137]

Lowering the cost of drug development could spur greater investment in novel therapies because the investment risk in such therapies would be lower. As the industry is already generously subsidized, cutting down on the time it takes to develop a drug may be most efficacious for incentivizing novel drug development to more effectively compete with the increasingly expensive merger and acquisition model of growth that currently brings a higher return with less risk.

To help speed the development process and spur more invention, policymakers have options for helping manufacturers and federal grantees share, or more effectively share, scientific findings from failed efforts. Additionally, incentivizing companies to use or lose, or share any finding they do not want to develop, may lead to more novel research by manufacturers. While there is no evidence to suggest that lowering the cost of drug development would bring down medicine prices, it may make more drugs available with naturally lower price points, some of which may help prevent conditions that require costly treatment.

## CONCLUSION

While Congress and the Trump administration have focused on high drug prices since 2017, no action taken has significantly affected the factors driving high prescription drug costs. Over the past decade, prescription drug spending has been driven by higher-cost specialty products, despite the fact that the vast majority of prescription drugs dispensed were generic products.

Over time, pharmaceutical manufacturers have increasingly invested in extending monopoly protection of brand and biologic prices and delaying or crowding out competition, enabling originator prices to grow higher. This insulation of brand and biologic drugs from price competition is not just caused by patent gaming and exclusivity stacking but is enabled by outdated drug coverage design and reimbursement, which incentivizes plan sponsors, PBMs, and physicians to use the more expensive products and even crowd out generic or biosimilar products.

Overall, the pharmaceutical market no longer maintains a balance of incentives that encourage generic and biosimilar price competition while driving invention of new scientific innovation. Instead, the system incentivizes manufacturers to invest in innovative ways to protect, increase, and extend existing brand and originator biologic product margins.

The actions that Congress and the administration have taken over the past three years largely have been necessary. But none has succeeded in fixing the dynamics in today's pharmaceutical market that allow extended brand and biologic monopoly-like pricing.

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## APPENDIX A. TRUMP ADMINISTRATION INITIATIVES ON DRUG PRICING

### The Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs and the President's Budget Requests

Through the annual budget proposals to Congress and the release of the *American Patients First: The Trump Administration Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs* (the Blueprint) in May 2018, the administration has identified its priorities for addressing challenges that it sees as central to the problems in the U.S. pharmaceutical market.[138] The Blueprint, which included several previously announced Trump administration proposals along with some new policy initiatives, was described as a framework for further action by HHS, CMS, and the FDA. The framework included ideas such as value-based purchasing, shifting Medicare Part B drugs to Part D, designating PBMs as fiduciary agents, various reimbursement changes in Medicare Part B, and transparency efforts in the drug supply chain. Several of the initiatives included in the Blueprint and the budget are described in more detail below.

Alongside the Blueprint, the president's budget requests included several provisions to reduce federal and beneficiary out-of-pocket spending on prescription drugs.[139] In Medicaid, the administration proposed a pilot allowing up to five states to determine their own drug formularies and negotiate directly with drug manufacturers, and to implement drug price negotiations through their formularies. For the Medicare Part D drug benefit, the budgets proposed providing drug rebates to patients at the point of sale, decreasing out-of-pocket costs in the catastrophic phase, increasing flexibility in plan formularies, excluding rebates when calculating coverage gap spending, and consolidating coverage for certain self-administered drugs in Medicare Part B, where patient protections are less robust. The administration also proposed inflation-based limits on price increases in Medicare Part B and significantly reduced reimbursement for drugs implicated by pay-for-delay contracts. Lastly, the administration sought to address product hopping and end 180-day exclusivity for generics when a manufacturer enters a pay-for-delay or another anticompetitive contract. Other administration proposals and activities are described below.

### FDA Focused on Competition

The FDA under the leadership of former Commissioner Scott Gottlieb, M.D., was the most active federal agency within the Trump administration in terms of addressing the drivers of high drug prices and issuing procompetitive regulatory changes in the prescription drug marketplace. Commissioner Gottlieb announced a Drug Competition Action Plan in December 2017,[140] prior to the Blueprint's release and followed with a Biosimilars Action Plan in May 2018.[141] Under these initiatives, the FDA accomplished the following:

- Took steps to publicize drugs that had no competition.
- Clarified the processes for submission and review of generic drugs and biosimilars, which included:
    - rolling out a new policy to prioritize generics for drugs with inadequate competition
    - finalizing biosimilar naming and labeling guidance
    - issuing a final guidance on establishing interchangeability with biologics.
- Laid out how the FDA uses its limited authorities to deter companies from using Risk Evaluation and Management Strategies (REMS) to prevent competition. REMS are FDA safety requirements imposed on drugs with greater risk profiles and can include restrictions on distribution ("limited distribution network").
- Increased its efforts to educate providers about the safety and efficacy of biosimilars.
- Prioritized implementation of the Generic Drug User Fee program negotiated by the previous administration to eliminate the backlog of generic drug applications.

The FDA approved record numbers of generic drug applications over the past four years in addition to having approved 22 new biosimilars, building on the progress made in the previous administration to increase generic drug and biosimilar approvals.[142] As of September 2020, 17 of the 28 FDA-approved biosimilars

are on the U.S. market.[143] Ultimately, however, these substantial efforts and accomplishments must be paired with other systemic and statutory changes to directly address high and growing drug prices.

## Prescription Drug Importation Proposals

In July 2019, HHS announced that the FDA would establish two pathways for the importation of certain prescription drugs from overseas. Then, in December 2019, the FDA took the first steps to implement these pathways by publishing a draft rule and draft guidance laying out the details of how these processes would work.[144] Following the President releasing an executive order on July 24, 2020, FDA issued a final rule on September 24, 2020, without major changes from the December 2019 proposed rule.[145] The rule reiterates existing HHS authority, but reverses the FDA's position on its willingness to use the authority to allow jurisdictions, and in some cases wholesalers, to import certain drugs from Canada. The rule is unlikely to create significant savings in part because of the cost and difficulty for jurisdictions to implement it, and in part because of the small size of the Canadian pharmaceutical market.

**FDA Action on Prescription Drug Pricing**

| Action | Description | Status | Date |
|---|---|---|---|
| Publish directory of drugs without competition. | Identify uncompetitive drug markets with public-facing website and information. | **Completed** | June 2017[146] |
| Expand biosimilar education to providers. | Encourage greater prescribing of biosimilars through provider-targeted educational material. | **Ongoing** | Oct. 2017[147] |
| Update guidance on REMS.* | Address REMS loopholes, which allow branded manufacturers to withhold samples from makers of generic drugs. | **Completed** | Oct. 2017[148]<br>June 2018[149]<br>Feb. 2019[150]<br>April 2019[151] |
| Clarify guidance on manufacturer communication of health care economic information (HCEI) to payers.** | Ensure information on which payers base "coverage and reimbursement decisions is truthful and nonmisleading and that appropriate background and contextual information is provided to enable payers to make informed decisions."[152] This guidance does not address communications to physicians and patients. | **Completed** | June 2018[153] |
| Clarify biosimilar review processes. | Publish final guidance on naming and labeling of biosimilars and establishing interchangeability. | **Completed** | July 2018[154]<br>May 2019[155]<br>Feb. 2020[156]<br>Feb. 2020[157] |
| Streamline generic drug review and approval and prioritize review of Abbreviated New Drug Applications for reference drugs with no market competition. | Publish guidance to assist industry, establish Competitive Generic Therapy prioritization, cleare the Abbreviated New Drug Application backlog for generics drugs. | **Completed** | Oct. 2018[158]<br>June 2019[159] |
| Update FDA guidance on citizen petition criteria. | Revise 2014 guidance to align with FDA's current thinking on what constitutes a citizen petition and the factors the agency will consider in determining whether the primary purpose of a petition is to delay a drug application approval. | **Completed** | Sept. 2019[160] |
| Enable prescription drug importation. | Allow states to import prescription drugs from Canada. | **Rule finalized** | Sept. 2020[161] |
| Expand products and information detailed in and funtionality of the Purple Book: Database of FDA-Licensed Biological Products | Add exclusivity information and additional FDA-licensed allergenic, cellular and gene therapy, hematologic, and vaccine products to the Purple Book. | **Completed** | Aug. 2020[162] |

* REMS (Risk Evaluation and Mitigation Strategies) are additional FDA safety requirements for drugs with greater risk profiles and can include restrictions on access to the drug, sometimes referred to as "limited distribution networks."

** HCEI is a measure of the economic consequences of the outcomes achieved from use of the product.

### Medicare and Medicaid Drug Spending Dashboard

First launched in December 2015, the Medicare Drug Spending Dashboard interactive web tool included 40 drugs each from Medicare Parts B and D.[163] A Medicaid State Drug Spending Dashboard was launched in 2016.[164] Following the publication of the administration's Blueprint, CMS published a modified version of the dashboards, which were last updated in December 2019.[165] While the original dashboards showcased data for the highest-spending pharmaceutical products based on three-part selection criteria, the newer version includes all drugs covered by the programs.

### Advanced Notice of Proposed Rulemaking for an International Pricing Index

In October 2018, CMS published an Advanced Notice of Proposed Rule Making, previewing an international pricing index model for Medicare Part B drugs.[166] While many details of how this would work were not specified within the notice, it is generally understood that it would have phased in a new cap on reimbursement for Part B drugs at 126 percent of an average of certain international prices. The proposal was accompanied by a report asserting that the U.S. pays 1.8 times more than other countries do for the drugs Medicare Part B spends the most on.[167] The administration has yet to publish a draft or final rule to implement an international pricing index as of this report's publication. However, on July 24, 2020, the White House announced it would be releasing an executive order directing that prices paid for Medicare Part B drugs be tied to the lowest prices of select products. On September 13, 2020, the administration released executive orders directing HHS to test a payment model where certain high-cost prescription drugs and biologics would be reimbursed by Medicare Part B and D at the lowest price paid by a member country of the Organisation for Economic Co-operation and Development (OECD).[168] As of the publication date of this report, HHS has yet to issue any related guidance.

### Direct-to-Consumer Advertising Price Transparency Final Rule

In October 2018, CMS proposed to require pharmaceutical manufacturers to include list prices of their drug products in their direct-to-consumer television advertisements.[169] The final rule was issued in May 2019[170] but was blocked in federal court following suit by Merck, Eli Lilly, and Amgen, when a judge found that HHS did not have sufficient authority to take this step.[171]

### Proposed Rule to Eliminate Safe Harbor Protection for Rebates

In January 2019, HHS proposed a rule to require that all rebates negotiated between pharmaceutical manufacturers and PBMs be passed through to patients.[172] These rebates have been criticized as incentivizing high list prices.[173] The CBO estimated that the rule would increase Medicare and Medicaid spending by nearly $200 billion as well as increase Medicare Part D premiums, which have remained stagnant for over a decade.[174] Under intense pressure from Congress over potential increases to Part D premiums, HHS withdrew the proposed rule in July 2019.[175]

### Voluntary Medicare Part D Senior Savings Model to Lower Insulin Out-of-Pocket Costs

For beneficiaries enrolled in Medicare Part D, out-of-pocket costs for insulin fluctuate from month to month, in part due to different cost-sharing levels for the different phases of the Part D benefit (that is, the initial coverage phase versus the coverage gap). This cost unpredictability can be challenging financially for beneficiaries, affecting medication adherence in a way that may lead to dangerous and costly side-effects or conditions.

In March 2020, CMS launched a model that enables beneficiaries enrolled in a participating Part D enhanced benefit plan to pay a maximum copay of $35 for a 30-day supply of formulary insulin throughout the benefit year or until the beneficiary hits the catastrophic coverage phase.[176] Manufacturers and plan sponsors must apply to participate in the program. Participating manufacturers will pay an additional discount above the 70 percent off the negotiated net price for the insulin when beneficiaries are in the coverage gap. The model is projected to increase medication adherence and save $250 million over five years, largely due to manufacturers paying the additional discount.

| HHS Action on Prescription Drug Pricing | | | |
|---|---|---|---|
| **Action** | **Description** | **Status** | **Date** |
| Medicare and Medicaid drug spending dashboards | Maintain an updated drug spending dashboard for Medicare Parts B and D. | **Update completed** | May 2018<br>Dec. 2019 |
| Clarifying guidance allowing Part D plans to use indication-based utilization management strategies[177] | Allow plans to employ utilization management, step therapy, or prior authorization requirements for a specific indication of a covered formulary drug, giving Medicare Part D plans additional negotiating leverage with manufacturers. | **Guidance issued** | July and Aug. 2018 |
| International pricing index model | Outline potential rule for international reference pricing. Propose a "Most Favored Nation" policy as outlined in an Executive Order released in Sept. 2020. | **No action** | Oct. 2018 (outline); Sept. 2020 (EO) |
| Direct-to-consumer drug price transparency final rule | Require drugmakers to publish list price in drug-to-consumer advertisements. | **Final rule struck down in court** | Oct. 2018 |
| Elimination of prohibition on Medicare Advantage plans using step therapy for Part B drugs[178] | Allow Medicare Advantage plans the option to use step therapy or a fail-first policy for new prescriptions or new administrations of Part B drugs.[179] Plans that choose this option must also offer beneficiaries the option to participate in drug management/care coordination activities.[180] | **Implemented** | Jan. 2019 |
| Proposed rule to eliminate safe-harbor protection for rebates | Limit PBMs to a flat processing fee rather than a fee that is a percentage of list price; also, require pass-through of rebates to patients. | **Proposed rule withdrawn** | Jan. 2019 |
| Voluntary senior insulin savings model | Lower insulin costs for seniors in certain plans by expanding manufacturer drug discounts in the Part D coverage gap. | **Requests for proposals for manufacturers and plans released** | March 2020 |
| Require certain community health centers to provide insulin and injectable epinephrine to low-income patients at section 340B Drug Pricing Program acquisition prices | Add a requirement to future community health center HHS grant recipients that they extend the prices they paid for insuline and injectable epinephrine, to certain low-income patients. | **Proposed rule issued** | Sept. 2020 |

# APPENDIX B. CONGRESSIONAL ACTION ON DRUG PRICING

## ENACTED LEGISLATION

### Bipartisan Budget Act (H.R. 1892) – February 2018

The Bipartisan Budget Act made a number of drug pricing–related changes as part of a larger legislative package.

### Correcting Inflation Rebates for Medicaid Line Extensions

Drug companies often make minor changes to their products (for example, a change in a drug's formulation) and then charge more for that product than the unchanged original product. These modified products are called "line extensions" or "new formulation drugs." Medicaid historically required manufacturers of such products to pay a rebate to offset increases above inflation. However, the ACA inadvertently allowed line extension drugs to be treated as new drugs, resetting the price baseline used to calculate the rebate to the line extension market entry price, rather than the product's original market launch price. The Bipartisan Budget Act included a technical correction to this error by adjusting the calculation of the inflation rebate to be the greater of the line-extension drug's additional rebate, or the highest additional rebate for any strength of the original branded drug.[181] The CBO estimated this correction would reduce Medicaid spending by $6.5 billion over 10 years.[182]

### Closing the Medicare Part D Coverage Gap Early and Increasing Manufacturer Drug Cost Liability

When originally enacted in 2003, the Medicare Part D drug benefit had a benefit design that exposed patients to 100 percent of drug spending after an initial coverage period until the beneficiary reached a catastrophic spending limit. In the catastrophic part of the Part D benefit, CMS and the Part D plan sponsor defrayed much of the cost of drugs for the beneficiary.[183] But for a period of time every year, Part D beneficiaries with moderate-to-high prescription drug costs were exposed to high out-of-pocket costs when they reached the coverage gap.

The ACA fixed the flaw in the original Part D design by phasing out the coverage gap, frequently called the donut hole, over 10 years. The ACA did this by increasingly transferring drug cost liability from the beneficiary to the sponsor over the 10-year time frame.[184] The Bipartisan Budget Act accelerated the ACA timeline and closed the coverage gap in 2019 one year ahead of schedule, completing the transition from patients paying for 100 percent of their drug costs while in the coverage gap, to paying 25 percent of their drug costs.[185]

However, the coverage gap closure will have little nominal effect on some Part D beneficiaries' out-of-pocket costs due to drug price inflation. One study estimates that for patients taking some drugs, such as those to treat rheumatoid arthritis, much of the cost savings from the coverage gap closure was lost to yearly price increases by drug manufacturers.[186]

Further, the Bipartisan Budget Act significantly reduced plan sponsor liability in the coverage gap from 25 percent to 5 percent, while correspondingly increasing the manufacturer discount from 50 percent to 70 percent. This benefit design change removed an incentive for Part D plans to offer beneficiaries high-cost drugs every year to facilitate patient movement through the coverage gap more quickly and offload the patient drug cost liability to the government, which covers 80 percent of the cost of catastrophic coverage. Additionally, shifting more liability to manufacturers for patient spend in the coverage gap may decrease manufacturer incentive to set high prices.[187] CBO estimated this reform would save Medicare Part D $11.8 billion over a decade.[188]

### Applying the Brand/Biologic Coverage Gap Discount to Biosimilars

The Bipartisan Budget Act made an additional change to the coverage gap that created parity between originator biologic and biosimilar products via the discount beneficiaries receive. Offering beneficiaries a 70 percent discount on biosimilar products in addition to biologic products removed the disincentive for beneficiaries or providers to choose the biosimilar over the reference biologic. Before the Bipartisan Budget Act, the 70 percent discount only applied to reference biologics, making

these higher priced originator products less expensive than biosimilar products. Biosimilar prices are, on average, 20 percent to 40 percent less expensive than reference biologics. The change made in the Bipartisan Budget Act aligns beneficiary lower out-of-pocket costs with the lower-list-price product, creating parallel incentives for the beneficiary and the taxpayer.

### Pharmacy Gag Clauses and Increased Biologic Patent Transparency (H.R. 2553 and H.R. 2554) – October 2018

Congress banned the inclusion of pharmacy gag clauses from contracts between PBMs and health insurers. These gag clauses prohibited pharmacists from informing patients if purchasing a medication without insurance would have a lower out-of-pocket cost.[189] Additionally, the law requires biologic and biosimilar manufacturers to report biosimilar patent litigation settlements to the FTC and DOJ, just as they must report drug patent settlements. The FTC continues to publish an annual report of all the patent settlement activity. The Know the Lowest Price Act of 2018 (S. 2553 – 115th Congress)[190] and the Patient Right to Know Drug Prices Act (S. 2554 – 115th Congress)[191] were enacted in October 2018.

### Medicaid Services Investment and Accountability Act (H.R. 1839) – April 2019

Previously, drug manufacturers would inaccurately classify a branded outpatient drug as a generic to reduce the rebate amount they are required to pay the government under the Federal Medicaid Drug Rebate Program. The minimum rebate for generic drugs is 13 percent of the average manufacturer price, and the minimum rebate for brand drugs is 23.1 percent of the average manufacturer price or the best price at which the brand manufacturer sells the drug. Congress passed H.R. 1839, allowing HHS to impose civil monetary penalties on manufacturers for the misclassification of a drug and to directly change the classification of a drug.[192] (HHS already has authority to end a manufacturer's ability to participate in the Medicaid program.) CBO estimated this increased enforcement ability will save the federal government $77 million over 10 years.[193]

### Fair and Accurate Medicaid Pricing Act (H.R. 3276) – September 2019

As part of the Continuing Appropriations Act of Fiscal Year 2020 (H.R. 4378), Congress made two changes to the way drugmakers calculate the average manufacturer price (AMP) for the purposes of the Medicaid Drug Rebate Program. The law prohibits a manufacturer from blending the sales of a brand drug with a generic that the brand manufacturer also makes (termed an authorized generic). Requiring that sales of the authorized generic be excluded from the calculation of the AMP for the branded drug raises the AMP of the branded drug and, consequently, increases the rebate amount the manufacturer must pay the government under the Medicaid Drug Rebate Program.

Additionally, the law closed the loophole that allowed the primary drug manufacturer that sold an authorized generic to a secondary manufacturer to include that selling price in the branded drug average manufacturer price for certain drugs. (Often in these situations, both manufacturers have a corporate relationship, such as the same parent company.) Specifically, the bill clarified that the secondary manufacturers do not count as wholesalers for the purposes of average manufacturer price calculation. The CBO estimated the federal government would save $3.15 billion over 10 years from this law.[194]

### The CREATES Act (H.R. 965) – December 2019

The most significant drug pricing legislation to be enacted in the last three years was the Creating and Restoring Equal Access to Equivalent Samples (CREATES) Act, which was included in a fiscal year 2020 consolidated appropriations bill (H.R. 1865) and signed into law on December 20, 2019.[195]

Different versions of the CREATES Act have been under consideration for several years, making slow but steady progress through the legislative process. The law was designed to prevent branded drug manufacturers from using FDA safety requirements, or REMS, to slow generic competition by refusing to share needed samples of their products with generic manufacturers.[196] Such refusals caused development bottlenecks for generics.[197] The CREATES Act established an exemption for drugs being

sold to generic drug developers and an avenue by which generic manufacturers can sue if samples are not provided in a timely, "commercially reasonable" manner.[198] The new law also codified the FDA's ability to waive requirements for generic and branded manufacturers to negotiate a single shared REMS if that requirement is preventing a generic from coming to market because one party is delaying the negotiation.

These changes address two specific ways generic competition has been stymied in the past but fall short of the systemic reform that is needed to address high drug prices.

## LEGISLATION THAT PASSED THE U.S. HOUSE OF REPRESENTATIVES

### The Elijah E. Cummings Lower Drug Costs Now Act (H.R. 3) – December 2019

The House passage of H.R. 3 marked the first time the House chamber passed legislation allowing the HHS secretary to directly negotiate drug prices with pharmaceutical manufacturers.[199] HHS would only negotiate the price of drugs when they have no competition on the U.S. market, and when the wholesale acquisition cost of the drug exceeds the median household income in the United States. In addition, the price would be capped using an international pricing index. If a drugmaker refused to negotiate or sold a drug above the negotiated price, an excise tax would be applied at a maximum of 95 percent of sales.[200] CBO estimated the negotiations provision at $456 billion in savings over 10 years.[201]

The legislation also included a benefit redesign, an out-of-pocket cap for beneficiaries in Medicare Part D, and an inflationary cap on price increases. CBO estimates the savings of these policy provisions at greater than $45 billion over a decade.[202]

H.R. 3 also included other provisions designed to increase transparency into PBM practices and manufacturer drug-pricing decisions, increase financial assistance to low-income beneficiaries facing high drug prices, and increase funding to federal research programs.[203]

### Other Legislation That Passed the House

The House also passed a few other stand-alone bills to address prescription drug pricing. H.R. 1503, the Orange Book Transparency Act, and H.R. 1520, the Purple Book Continuity Act, would increase transparency around the patents on prescription drugs and biologics to facilitate generic drugs and biosimilars coming to the market more easily. In addition, H.R. 2115, the Public Disclosure of Drug Discounts Act, would require more transparency of discounts and rebates being offered by PBMs.

These bills have not been passed by the Senate, though some have been included in bills considered by committees.

## LEGISLATION THAT PASSED THE U.S. SENATE

### Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act of 2017 (S. 1052) – August 2017

The Senate passed a bill that would have required the FDA to consider patient-focused data, such as patient preferences, patient-reported outcomes, and patient experiences, as part of the risk-benefit assessment of new drugs. Following approval of a drug, the FDA would have had to include a description of how this patient data was considered. The House did not consider the bill.

## LEGISLATION REPORTED OUT OF THE RELEVANT COMMITTEES

### Senate HELP Committee – Lower Health Care Costs Act (S. 1895) – July 2019

Chairman Lamar Alexander (R-TN) and ranking member Patty Murray (D-WA) of the Senate Committee on Health, Education, Labor, and Pensions successfully passed the Lower Health Care Costs Act (S. 1895) out of committee by a bipartisan vote of 20 to three, although the bill has yet to be scheduled for a vote on the floor by the full Senate.[204] Among other proposals to reduce health care costs, the bill included provisions to stop anticompetitive behaviors; protect access to generic drugs and biosimilars; and establish transparency requirements for the orange and purple books, FDA compendiums of all the prescription

drugs and biologics approved by the agency, along with their known patent information. The CBO estimates that the prescription drug provisions would save the government $4.6 billion over a 10-year budget window.[205]

### Senate Finance Committee – Prescription Drug Pricing Reduction Act (S. 2543) – July 2019

Senate Finance Committee chairman Chuck Grassley (R-IA) and ranking member Ron Wyden (D-OR) reported the sweeping Prescription Drug Pricing Reduction Act out of committee with a bipartisan vote of 19 to nine, but the bill has yet to be scheduled for a Senate floor vote.[206] The most recent version of the legislation would establish an inflationary limit to list-price increases under Medicare Parts B and D, institute an out-of-pocket cap for Medicare Part D beneficiaries, redesign the Medicare Part D benefit structure to shift liability in the catastrophic phase toward plans and manufacturers, and increase transparency into PBM practices and manufacturer drug-pricing decisions.[207] The CBO estimated the inflationary cap in the bill would save Medicare nearly $70 billion over 10 years on its own,[208] and estimated $3.2 billion in savings on the entire bill.[209]

This bill was passed out of committee but has yet to be brought to the floor for a vote by the full Senate. The chairmen of both committees continue to press for enactment of their respective bills in this Congress.

## OTHER LEGISLATION REPORTED OUT OF A COMMITTEE

The House Energy and Commerce Committee, the House Ways and Means Committee, and the House and Senate Judiciary Committees have also reported out legislation that could help address high drug prices. Included were bills to prevent the abuse of the FDA citizen petition process to delay generic drugs coming to market; narrow the cases in which existing products could seek orphan drug designations; give the FDA authority to require that outdated drug labels be updated; increase the role of the FTC in pursuing pay-for-delay agreements, patent thickets, and product hopping; and create more transparency in the prescription drug supply chain. As of the publication of this report, these bills have not yet been considered by the full House of Representatives or Senate.

## LEGISLATION ENACTED BY STATES ON DRUG PRICING

As the drug-pricing debate developed in Congress, state legislatures, governors, and attorneys general addressed the issue as well.[210] Enacting at least 45 laws in 2019 alone, states — which bear significant liability for drug spending in Medicaid — are addressing high prices using some of the mechanisms discussed at the federal level, such as price transparency requirements, drug importation, reform of PBM drug-purchasing incentives, and volume-purchasing agreements.[211]

Other laws use mechanisms uniquely available to the state. These include the following:

- Putting a global reimbursement cap on the state's overall Medicaid drug spending by targeting the highest-cost drugs
- Implementing drug affordability review boards to monitor and review high drug costs
- Instituting commercial cost-sharing requirements for insulin patients
- Prohibiting brand drug coupons or copay accumulators that do not count toward a patient's deductible or copay requirements in commercial insurance regulated by the state.[212]

While states can implement measures that control state spending, there is little that most states can do, given their limited purchasing power and Medicaid drug coverage requirements, to target the root of high prescription drug–launch prices or stymie overall drug price increases. Additionally, for many drugs, Medicaid is a small revenue center relative to Medicare and the commercial market, making the Medicaid program a less powerful tool to influence U.S. drug-pricing trends.

## APPENDIX C. EXCLUSIVITIES AND PATENT-GAMING STRATEGIES

Broadly, there are four main patent-gaming strategies and six types of exclusivities granted by the FDA. These strategies can be used alone or in combination with one another.

Drug manufacturers have six market exclusivities, some of which can be combined, or stacked, if the right criteria are met. These include:

1. *New chemical entity data exclusivity* grants five years' exclusivity, starting from the time of the FDA's approval of the new drug application, for chemically synthesized (not biologic) drugs that contain a new active moiety, or ingredient.
2. *New clinical investigation market exclusivity* grants an additional three years' exclusivity to chemically synthesized (not biologic) drugs when a new application or supplement new drug application contains reports of new clinical studies conducted by the manufacturer to demonstrate a new use for the drug that's essential to the FDA approval of that application.
3. *Pediatric market exclusivity* grants six months of additional exclusivity (added to other FDA exclusivities the product may qualify for) if a brand or biologic manufacturer has conducted and submitted pediatric studies on a drug's active moiety.
4. *Orphan drug market exclusivity* grants seven years of additional exclusivity (added to other FDA exclusivities the product may qualify for) to brand and biologic uses designated and approved to treat diseases or conditions affecting fewer than 200,000 individuals in the U.S., or when the manufacturer is not reasonably expected to recover development costs.
5. *Antibiotic market exclusivity* grants five years of additional market exclusivity (added to other FDA exclusivities the product may qualify for) to brand and biologics designated and approved by the FDA as qualified infectious disease products.
6. *Biologic exclusivity* grants four years of data exclusivity and concurrently 12 years of market exclusivity for new biologic products. It begins when the FDA approves a biologic license application.

During a data exclusivity period, a generic drug application cannot be submitted to the FDA, and certain data submitted to the FDA for the purposes of gaining regulatory approval cannot be disclosed. A market exclusivity bars a generic or biosimilar drug application from being approved by the FDA and, thus, no competitor can enter the market.

Four basic strategies are employed by brand and originator biologic manufacturers, often in combination, to extend a product's patent protection in the U.S. market.

**Patent thicketing.** When brand manufacturers take out as many patents for the original brand compound or product as possible, it is known as patent thicketing. They will seek to patent manufacturing techniques, routes of administration (for example, oral, injection), and delivery devices (for example, pen, auto-injectors, wearable injectors), as well as the molecule itself. The dense portfolio of patents is referred to as a patent thicket; this practice has grown dramatically over the past 20 years.[213] For example, the number of granted patents for pharmaceutical products doubled between 2005 and 2015, despite a similar number of products produced.[214]

**Patent evergreening.** This occurs when brand manufacturers obtain a secondary, or later-issued, patent for peripheral features or incremental improvements of the main drug ingredient or primary patent, such as creating a new dosage, combination, or formulation unrelated to the effectiveness or science of the drug.[215] This effectively extends the market monopoly beyond the known patent life. As a generic drug may only apply for FDA approval if the reference brand product no longer has patent protection, a brand manufacturer can extend the period of monopoly by taking out as many patents as possible, even if the patents are not for innovative changes or product improvements.

**Product hopping.** Also known as product switching, this practice involves a brand manufacturer shifting prescribing patterns to a new, similar, or follow-on product with a later-expiring patent just before the patent of a product the market currently uses is set to expire. Under a hard product switch, the older product is removed from the market entirely. With a soft switch, the older product is kept on the market alongside the new product. In both cases, the brand manufacturer will target all marketing to the new product. Examples of this include moving the older drug to an over-the-counter drug designation, getting approved for a new indication, product bundling, or other business model innovations that do not substantially change the science of the drug itself but sufficiently change the product's treatment in the market. In some cases, the products are also eligible for new market exclusivities, further extending a manufacturer's monopoly of a market.

**Reverse payment patent settlements/pay-for-delay.** After a generic drug has been developed and approved, a brand manufacturer still has the ability to block or delay it from entering the U.S. market if the brand manufacturer comes to an agreement with the manufacturer of the first-to-file generic (that is, the first manufacturer to file a generic product application) to extend the brand monopoly period. These agreements suppress market competition through a reverse-payment patent settlement, sometimes known as pay-for-delay. The result is a generic manufacturer agreeing not to bring an FDA-approved product to the U.S. market for several years in exchange for a significant payout, royalty, or other arrangement with the manufacturer of the reference product. In some cases, the generic manufacturer has the same parent company as the brand drug.

These settlements between two drug manufacturers block all other generic manufacturers from bringing a product to market to compete. While both parties to the settlement reap larger profits than they would absent the settlement, patients and the U.S. health care system pay billions more for drugs due to this anticompetitive practice. In 2013, the FTC successfully challenged pay-for-delay settlements as a violation of anti-trust law before the Supreme Court. Since the court's ruling in the FTC's favor, the number of these settlements has dropped, but they still remain a potent way to delay competition. Due to resource constraints, the FTC only investigates the seemingly most egregious pay-for-delay settlements, while urging Congress to pass legislation banning the anticompetitive practice outright.

## ABOUT THE AUTHORS

**Rep. Henry A. 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."

**Bill Corr** has spent the bulk of his impressive career advocating for better health care access at almost every level of society. Most recently, he served as deputy secretary of the U.S. Department of Health and Human Services (HHS) from 2009 to 2015. Corr returned to the department after serving as executive director of the Campaign for Tobacco-Free Kids, a privately funded organization established to focus the nation's attention and action on reducing tobacco use among both kids and adults. From March 1998 until 2000, Corr served as chief counsel and policy director for Senate Minority Leader Tom Daschle. Before working in the Senate, he served as the chief of staff for HHS. In that capacity, he was principal adviser to Secretary Donna E. Shalala on all major policy and management issues and initiatives. He also was deputy assistant secretary for health for the Department and counselor to the secretary prior to becoming chief of staff. From 1989 until 1993, Corr served as chief counsel and staff director for the Subcommittee on Antitrust, Monopolies and Business Rights of the Senate Committee on the Judiciary under Chairman Howard M. Metzenbaum. Corr also served as counsel to the Subcommittee on Health and the Environment of the House of Representatives Committee on Energy and Commerce under Chairmen Paul Rogers and Henry A. Waxman.

**Jeremy Sharp** has extensive policy experience in both the executive and legislative branches of the federal government. Most recently he served as Deputy Commissioner for Policy Planning, Legislation, and Analysis at the U.S. Food and Drug Administration. Prior to that he served at the U.S. Department of Health and Human Services as Counselor to the Secretary for Science and Public Health, under both Secretary Kathleen Sebelius and Secretary Sylvia Burwell, and he also served as Deputy Assistant Secretary for Legislation. His work in the executive branch spanned issues including prescription drug pricing, tobacco control, substance abuse, mental health, food safety, drug and device regulation, pharmacy compounding, and biosimilar and interchangeable product policy. His legislative career included serving as Legislative Director for Senator Christopher J. Dodd and as a professional staff member on the Senate Health, Education, Labor and Pensions Subcommittee on Children and Families during the enactment of the Affordable Care Act and the Family Smoking Prevention and Tobacco Control Act, among other legislation. Prior to that, he staffed Congresswoman Lois Capps and Senator Evan Bayh, and in the interim worked for the public health nonprofit Trust for America's Health. Jeremy received his bachelor's degree from Georgetown University's School of Foreign Service.

**Ruth McDonald** brings both policy and communications expertise from her experience serving both the legislative and executive branches of government, as well as her work on corporate, nonprofit, and political campaigns. Most recently, Ruth worked at the health policy consulting firm Avalere Health, where she helped companies, advocacy organizations, and trade associations navigate or create changes to the legislative and regulatory landscape. Previously, she served in the Office of Health Policy at the U.S. Department of Health & Human Services for the Assistant Secretary for Planning and Evaluation (ASPE). She also served nearly seven years as a staff member in the U.S. House to Majority Leader Steny H. Hoyer (Md.-05) and Congressman Jim Cooper (Tenn.-05) during the enactment and early implementation of the Affordable Care Act. Ruth earned an MPH in health management and policy and an MPP degree from the University of Michigan. She received her BA in the history of science and medicine from the University of Illinois at Urbana-Champaign.

**Kahaari Kenyatta** uses data to dissect problems, uncover new questions, and identify strategies to address challenges across the health care and public health ecosystems. With Waxman Strategies, Kahaari has supported clients' research and advocacy objectives on issues including women's health, prescription drug pricing policy, tobacco control, FDA issues, and health care costs, access, and affordability. Before coming to Waxman Strategies, Kahaari worked as an analyst at Ipsos Healthcare in Washington, D.C., where he managed large market research projects for top biopharmaceutical firms. Previously, Kahaari engaged in several community health research projects for nonprofit and academic entities in New York City, Camden, N.J., and Philadelphia, Pa. Kahaari earned his BA from the University of Pennsylvania where he studied health and societies with a concentration in public health.

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