

CHAPTER

3

**Medicare payment strategies
to improve price competition
and value for Part B drugs**

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Chapter summary

Medicare Part B covers drugs and biologics that are administered by infusion or injection in physician offices and hospital outpatient departments (HOPDs). Medicare Part B also covers certain other drugs provided by pharmacies and suppliers (e.g., inhalation drugs; certain oral anticancer, oral antiemetic, and immunosuppressive drugs; and certain home infusion drugs). Medicare pays for most Part B drugs and biologics at a rate of 106 percent of the average sales price (ASP + 6 percent). In 2017, the Medicare program and beneficiaries together paid about \$32 billion dollars for Part B–covered drugs and biologics. (Hereafter, we use the term *drugs* to refer to drugs and biologics unless otherwise noted.)

Medicare Part B drug spending has grown rapidly, increasing at an average rate of 9.6 percent per year between 2009 and 2017. Nearly two-thirds of the growth in Part B drug spending between 2009 and 2016 was accounted for by price growth, which reflects increased prices for existing products and shifts in the mix of drugs, including the launch of new high-cost drugs. In 2017, the Commission recommended several improvements to payment for Part B drugs, including an ASP inflation rebate that would address price growth in the years after products launch, consolidated billing codes for biosimilars and originator biologics that would spur price competition among these products, and a voluntary alternative to the ASP payment system that would use vendors to negotiate lower prices and share savings with providers and beneficiaries.

In this chapter

- Background on Medicare Part B coverage of drugs
- Spurring price competition with reference pricing
- Addressing high launch prices with binding arbitration
- Conclusion

The policies in the June 2017 recommendation—which aim to spur competition, address price growth, and lower prices—would be important steps forward; nonetheless, several additional issues remain that increase spending for both the Medicare program and beneficiaries. Under the ASP + 6 percent payment system, a new drug receives its own payment rate based on its own ASP. The payment system is not designed to spur price competition among single-source drugs with similar health effects. Also, a drug’s payment rate may not have any relationship to its clinical effectiveness. Fee-for-service (FFS) Medicare acts as a price taker and lacks tools to arrive at payment rates for new drugs that balance an appropriate reward for innovation with value and affordability for beneficiaries and taxpayers. In addition, concern exists about provider incentives under the ASP payment system. The 6 percent add-on to ASP may create incentives for some providers to select higher priced products, although studies examining this issue are limited.

Building on our June 2017 recommendation, this chapter examines two strategies that were elements of that recommendation—reference pricing and binding arbitration. We explore the potential to apply these two approaches more broadly in an effort to improve price competition and value for Part B drugs. Both of these strategies would require that the Congress change the statute to give CMS the authority to implement them.

Reference pricing

In 2017, the Commission concluded that the structure of the ASP payment system, with an originator biologic assigned to one billing code and its biosimilars assigned to different codes, does not spur price competition among these products. Consequently, the Commission recommended that the Congress give the Secretary the authority to use consolidated billing codes under which an originator biologic and its biosimilars would be assigned the same billing code and paid the same rate.

We have also found that the structure of the ASP payment system does not promote price competition among some groups of drugs with similar health effects, such as leukocyte growth factors and erythropoiesis-stimulating agents. Building on the Commission’s 2017 consolidated billing code recommendation, we discuss Medicare’s use of internal reference pricing, a policy that aims to spur price competition among single-source products with similar health effects and reduce drug prices. Applying this policy to Part B drugs, Medicare would establish a reference payment amount for groups of drugs with similar health effects currently assigned to separate billing codes. The reference payment amount could be set at the median, average, minimum, or other point along the range of prices within the drug group. Because there is typically a limit on what physicians or outpatient departments would receive in payment and because there can be large differences

in cost sharing, internal reference pricing gives the provider and patient strong incentives to consider lower cost therapeutic substitutes within each group. Between 1995 and 2010, Medicare implemented internal reference pricing strategies that set payment rates for groups of drugs with similar health effects based on the least costly product in each group. Since 2010, due to judiciary rulings and statutory changes, Medicare Part B no longer uses such policies for Part B drugs and pays for each single-source drug according to its own ASP payment rate.

Binding arbitration

For costly new drugs that face limited competition, such as the first drug in a class or a product that offers added clinical benefit over existing treatments, manufacturers have significant market power to set prices and payers currently have very limited ability to influence those prices. The Commission's June 2017 recommendation called for the development of a voluntary alternative to the ASP payment system (referred to as the Drug Value Program (DVP)), in which private vendors would obtain lower prices for Part B drugs through the use of tools, including binding arbitration for high-cost products with limited competition. Arbitration is a process by which two parties agree to accept the decision of a neutral third party in a dispute, such as a dispute over the price of a drug.

Although the Commission has recommended the inclusion of binding arbitration within the DVP, there may be benefits to expanding binding arbitration beyond the DVP. Since the DVP would be voluntary for providers, some Part B drug spending would remain under the traditional ASP system unaffected by the DVP. Thus, expanding binding arbitration beyond the DVP would increase its potential impact on Part B drug spending. Because Part A providers such as inpatient hospitals also face challenges with negotiating prices for drugs with few alternatives, there also could be benefits to extending prices achieved through binding arbitration to Part A providers.

In this chapter, we explore a potential policy that would permit the Secretary to enter into binding, baseball-style (i.e., final-offer) arbitration with drug manufacturers for Part B drugs with limited competition under certain circumstances. We describe how such an approach could work and discuss some of the key design elements and policy choices that would be involved. Under the potential policy, the Congress could specify criteria for when a Part B drug is eligible for arbitration based on its cost (e.g., exceeding specified thresholds) and whether it faces limited competition. If a product met the criteria, the Secretary could request that the manufacturer enter into binding arbitration. A system could be in place to select a neutral arbitrator or arbitration panel. The Secretary and

manufacturer could each submit an offer price to the arbitrator and the arbitrator could choose one of those two prices after considering supporting information submitted by the two parties and criteria specified by the Congress. The new arbitration price could become the basis of Medicare payment for the Part B drug, which could be operationalized by adjusting the Medicare payment rate and requiring that the manufacturer honor that price for Medicare patients or by instituting a manufacturer rebate paid to Medicare.

Binding arbitration is one of the few potential tools available to affect the price of drugs with limited competition. Binding arbitration has the potential to incorporate value, affordability, and an appropriate reward for innovation into the determination of Medicare's payment for Part B drugs. Whether arbitration is an effective process for arriving at a value-based payment would depend on how the arbitration process is designed. The Congress would need to specify a number of design elements for the binding arbitration process. The success of a binding arbitration process would also hinge on the ability to involve neutral arbitrators.

Both strategies—reference pricing and binding arbitration—would be somewhat complex to implement, but have the potential to yield substantial savings. Each strategy is a distinct policy and could be adopted on its own. However, packaging both strategies together, along with the Commission's June 2017 recommendation policies, could provide added benefits since the various policies would complement each other by addressing different factors driving Medicare Part B drug spending growth. Some stakeholders raise concerns that policies aimed at reducing Medicare spending for Part B drugs would reduce incentives for innovation. However, others argue that the current prices for some products adversely affect affordability and access and exceed what is necessary to provide appropriate incentives for innovation. Each strategy would be expected to lower beneficiary cost sharing and could be structured to promote beneficiary access. Finally, both reference pricing and binding arbitration could also be applied to pay for Part D drugs, although how each could be applied would differ from their use in Part B. ■

**TABLE
3-1**

Price growth accounted for nearly two-thirds of spending growth for separately payable Part B drugs between 2009 and 2016

	2009	2016	Average annual change 2009-2016
Total payments for separately payable Part B drugs excluding vaccines (in billions)	\$12.8	\$26.1	10.7%
Number of beneficiaries receiving Part B drug	2,840,166	3,750,634	4.1
Average payment per user	\$4,524	\$6,962	6.4
Average number of drugs per user	1.41	1.36	-0.5
Average annual payment per drug per user	\$3,206	\$5,119	6.9

Note: This analysis includes all Part B drugs paid the average sales price plus 6 percent as well as the small group of Part B drugs that are paid based on the wholesale acquisition cost, average wholesale price, or reasonable cost or those that are contractor priced. "Vaccines" refers to the three Part B-covered preventive vaccines: influenza, pneumococcal, and hepatitis B. Data include Part B drugs furnished by physicians, hospitals paid under the outpatient prospective payment system, and suppliers. Excluded from the analysis were any Part B drugs that were bundled or packaged in 2009 and/or 2016 (i.e., drugs that were packaged under the outpatient prospective payment system, regardless of the setting where they were furnished, and drugs furnished by dialysis facilities), drugs billed under not-otherwise-classified billing codes, blood and blood products (other than clotting factor), and data for critical access hospitals and Maryland hospitals. The average annual growth rates displayed in the table may differ slightly from the average annual growth rates calculated using the 2009 and 2016 values displayed in the table due to rounding. Total payments reflect Medicare program payments and beneficiary cost sharing.

Source: MedPAC analysis of Medicare claims data for physicians, hospital outpatient departments, and suppliers.

Background on Medicare Part B coverage of drugs

Medicare Part B covers drugs and biologics that are administered by infusion or injection in physician offices and hospital outpatient departments (HOPDs). Medicare Part B also covers certain other drugs provided by pharmacies and suppliers (e.g., inhalation drugs; certain oral anticancer, oral antiemetic, and immunosuppressive drugs; and certain home infusion drugs). Medicare pays for most separately payable Part B drugs and biologics at a rate of 106 percent of the average sales price (ASP + 6 percent). In 2017, fee-for-service (FFS) Medicare and beneficiaries together paid about \$32 billion dollars for Part B-covered drugs and biologics. (Hereafter, we use the term *drugs* to refer to drugs and biologics unless otherwise noted.)

Medicare program and beneficiary spending on Part B drugs has grown rapidly

Medicare Part B drug spending has grown rapidly, increasing by an average annual rate of 9.6 percent between 2009 and 2017. Drugs furnished in physician

offices account for the majority of Part B drug spending, but spending on drugs furnished in HOPDs has grown rapidly in recent years. Of total Part B spending in 2017 (including beneficiary coinsurance), about \$18.0 billion was for drugs administered in physician offices, about \$12.3 billion for drugs administered in HOPDs, and \$1.8 billion for drugs furnished by suppliers.¹ Between 2009 and 2017, Part B drug spending increased at an average annual rate of 17 percent in HOPDs and 7 percent in physician offices (data not shown). The faster spending growth in HOPDs partly reflects a shift in site of service from physician offices to HOPDs, particularly for oncology drugs.

Price growth is the largest driver of Medicare Part B spending growth. Nearly two-thirds of the growth in Part B drug spending between 2009 and 2016 was accounted for by price growth, which reflects increased prices for existing products and shifts in the mix of drugs, including the launch of new high-cost drugs. As shown in Table 3-1, focusing on drugs that were separately payable and excluding vaccines, Medicare Part B drug spending grew at an average annual rate of 10.7 percent between 2009 and 2016, with 6.9 percentage points of the growth due to

**TABLE
3-2**

Medicare expenditures and ASP growth for the top 10 Part B drugs as of 2017

	2017		Part B spending (billions)		Change in spending 2009-2017		Cumulative change in ASP	
	Number of users	Average spending per user	2009	2017	Dollars (billions)	Percent	2009-2017	2017-2019
Eylea	229,600	\$10,700	—*	\$2.5	\$2.5	N/A*	0%*	-2%
Rituxan	70,800	24,900	\$1.2	1.8	0.6	50%	53	17
Opdivo	29,000	51,000	—*	1.5	1.5	N/A*	3*	6
Neulasta	91,800	15,300	0.8	1.4	0.6	85	89	14
Remicade	56,800	23,700	0.8	1.3	0.5	61	44	-7
Prolia/Xgeva	467,700	2,700	—*	1.2	1.2	N/A*	14*	13
Avastin	218,300	4,900	1.1	1.1	0.0	0	28	10
Lucentis	105,300	9,900	0.9	1.0	0.1	16	-6	-4
Keytruda	21,600	48,100	—*	1.0	1.0	N/A*	2*	5
Herceptin	20,800	37,800	0.3	0.8	0.5	138	53	13

Note: ASP (average sales price), N/A (not applicable). Change in ASP was calculated based on ASP in effect for payment purposes as of the first quarter of each year. Data include Part B-covered drugs furnished by physicians, suppliers, and hospital outpatient departments, but exclude those furnished by critical access hospitals, Maryland hospitals, and dialysis facilities. "Part B spending" includes Medicare program payments and beneficiary cost sharing. *Product was not on the market in 2009. For these products, we calculate the percent change in ASP from 2009 to 2017 from the earliest January for which an ASP payment rate was available to January 2017 (2013 for Eylea, 2016 for Opdivo, 2012 for Prolia/Xgeva, and 2016 for Keytruda).

Source: MedPAC and Acumen LLC analysis of Medicare claims data and MedPAC analysis of average sales price files from CMS.

an increase in price (as measured by the average annual payment per drug per user).² This 6.9 percent increase reflects a combination of factors—increased prices for existing products, the launch of new higher priced products, and other changes in the mix of drugs used.

Looking at all Part B-covered drugs, a price index constructed by our contractor Acumen LLC isolates price growth that occurs at the individual product level. This measure reflects only a product's own price growth over time, not changes in price due to the introduction of new products or the changes in the mix of products used. Our price index finds that across Part B drugs, the price of individual products (as measured by the average sales price) grew an average of 1.9 percent per year between 2009 and 2016. Underlying this overall trend in the price index are different patterns by type of product. On average, the price index for Part B-covered biologics increased by 3.8 percent per year while the price index for nonbiologics declined by 1.4 percent per year over this period. The nonbiologic group includes single-source drugs and drugs with generic competition. The downward

price trend for nonbiologics in part reflects patent expiration and generic entry for some of these products.

Part B drug spending is concentrated in a small number of expensive products. In 2017, Medicare spending (including beneficiary cost sharing) for the top 10 drugs paid under the ASP system totaled about \$13.6 billion, about 43 percent of all Part B drug spending that year (Table 3-2). Notably, all 10 of these products are biologics. Many of these products are used to treat cancer or its side effects, while some treat macular degeneration, rheumatoid arthritis, and other inflammatory conditions.

The patterns of spending growth within the top 10 products illustrate two factors driving spending growth: new products with high launch prices and existing products with price inflation. For example, two products—Opdivo and Keytruda—are recent market entrants (approved in late 2014) and belong to a new class of immuno-oncology biologics. Spending on these products in 2017 reached \$1.5 billion and \$1 billion, respectively, reflecting the products' substantial launch prices. Average 2017 Medicare annual spending per user for these products was about \$51,000 and \$48,000, respectively.³

Price growth among biologics that have been on the market longer has also driven spending growth. For example, between 2009 and 2017, ASPs increased 44 percent for Remicade, 53 percent for Rituxan and Herceptin, and 89 percent for Neulasta. Although we lack data on Medicare expenditures beyond 2017, we do have ASP + 6 percent payment rates through the first quarter of 2019. Between January 2017 and January 2019, the ASPs for 5 of the top 10 products increased by 10 percent or more.

Price declines have occurred among a few of the top 10 products; however, these declines have been modest given the existence of competing products and the magnitude of spending on these products. For example, Eylea and Lucentis are competing products used to treat macular degeneration and related eye conditions that accounted for \$3.5 billion in 2017 Part B drug spending. Eylea's ASP declined 2 percent since its launch, and Lucentis's ASP declined 11 percent between 2009 and 2019 (the difference in this number and the numbers in Table 3-2 reflects rounding). Remicade is an originator biologic for rheumatoid arthritis and certain other inflammatory conditions. It faced entry by two biosimilars in late 2016 and mid-2017. Remicade's ASP declined 7 percent between 2017 and 2019; however, that decrease followed a 55 percent increase in Remicade's ASP between 2005 and 2017 (data not shown). Remicade's ASP + 6 percent payment rate in the first quarter of 2019 remains 24 percent to 34 percent higher than the biosimilars' payment rates.

How Medicare pays for Part B drugs

Medicare pays physicians and outpatient hospitals for the Part B-covered drugs they furnish to beneficiaries. By statute, Medicare pays physicians for most Part B drugs at a rate of ASP + 6 percent.⁴ By regulation, Medicare also pays ASP + 6 percent for separately payable Part B drugs furnished in hospital outpatient departments. ASP reflects the average price realized by the manufacturer for sales to all purchasers net of rebates, discounts, and price concessions, with certain exceptions. Thus, Medicare acts as a price taker, with payment based on a market-based price. Medicare pays providers 106 percent of the ASP for the drug regardless of the actual price a given provider pays for it. In addition to paying ASP + 6 percent for the drug, Medicare makes a separate payment to providers for the act of administering the drug to the patient (e.g., for infusing or injecting the product) at a rate determined under the physician fee schedule or hospital outpatient prospective payment system (OPPS).

Using the data submitted by manufacturers to CMS, the agency updates the Medicare Part B drug payment rates for each product with available ASP data on a quarterly basis; these payment rates are publicly available on CMS's website. There is a two-quarter lag in the data used to set ASP + 6 percent payment rates. This lag is necessary to permit time for manufacturers to submit ASP data and for CMS to calculate and implement the new payment rates.⁵

If a drug lacks ASP data, Medicare has alternative methods for paying for the product. For new single-source drugs that initially lack ASP data, Medicare pays a rate of wholesale acquisition cost (WAC) plus 3 percent for the first two to three quarters the product is on the market, consistent with a recent Commission recommendation that the payment rate for drugs paid based on WAC be lowered from WAC plus 6 percent to WAC plus 3 percent. For drugs that lack ASP data for reasons other than being new, such as the manufacturer not reporting ASP data or the manufacturer has no sales in a particular reporting quarter, the payment method varies and may be 106 percent of WAC, 95 percent of average wholesale price, or invoice priced.

Payments for single-source drugs and originator biologics, multiple-source drugs, and biosimilars are set differently. Each single-source drug and originator biologic is paid under its own billing code at 106 percent of its own ASP; brand and generic versions of a multiple-source drug are assigned to the same billing code and paid the same rate equal to 106 percent of the volume-weighted average ASP; and each biosimilar is paid under its own billing code at a rate of 100 percent of its own ASP plus 6 percent of the originator biologic's ASP.⁶

There is no consensus on the original intent of the 6 percent add-on to ASP. One hypothesis is that the 6 percent was intended to address price variation across purchasers and maintain access for purchasers who may pay above-average prices. Another thought is that the percentage add-on was intended to provide protection for providers when price increases occur and the payment rate has not yet caught up. Some stakeholders have also offered a variety of other rationales, suggesting that the 6 percent add-on was intended to help pay for drug storage and handling costs, the financing costs associated with maintaining drug inventory, or financial counseling services that some providers offer patients.

The Secretary does not routinely collect providers' acquisition costs for Part B drugs. However, on a few occasions, the Office of Inspector General (OIG)

2017 Commission recommendation on Part B drugs

In 2017, the Commission recommended a set of policies that seeks to improve the current average sales price (ASP) payment system in the short term while developing, for the longer term, a voluntary, market-based alternative to the ASP payment system. Specifically, the recommended short-term actions would:

- **Improve ASP data reporting.** Currently most, but not all, Part B drug manufacturers are required to report ASP data to CMS. The Commission recommended requiring all manufacturers to report ASP data, with civil monetary penalties for failure to report.
- **Reduce payment rates for drugs that lack ASP data.** The Commission recommended reducing the payment rate from 106 percent to 103 percent of wholesale acquisition cost for new single-source Part B drugs that initially lack ASP data and for existing drugs that lack ASP data. (CMS has adopted this policy for new drugs effective January 2019, but has not adopted it for other drugs that lack ASP data and may need additional statutory authority to do so).
- **Establish an ASP inflation rebate.** This policy would require a manufacturer to pay a rebate if the ASP for its drug grew at a rate in excess of an inflation benchmark.

- **Establish consolidated billing codes.** This policy would group an originator biologic and its biosimilars into the same billing code to maximize price competition.

Over the longer term, the Commission recommended that Medicare develop the Drug Value Program (DVP) as a voluntary, market-based alternative to the ASP payment system for physicians and outpatient hospitals. The DVP would seek to lower prices for Part B drugs by permitting private vendors to use tools (such as a formulary and, in certain circumstances, binding arbitration) to negotiate prices with manufacturers and by improving incentives for provider efficiency through shared savings opportunities. Under the program, a small number of DVP vendors would negotiate prices for Part B drugs, but vendors would not ship products to providers. Providers that chose to enroll in the DVP would continue to buy drugs in the marketplace but at the DVP-negotiated price, and Medicare would reimburse those providers at the same negotiated price. To encourage enrollment in the DVP, providers would have shared savings opportunities through the DVP, while the ASP add-on would be reduced gradually in the ASP system. Savings achieved through the DVP would also be shared with beneficiaries (through lower cost sharing) and with DVP vendors and Medicare. ■

compared the acquisition costs of selected drugs among a sample of providers with Medicare's payment rates under ASP. In the first quarter of 2010, OIG estimated that physician acquisition costs for Lucentis were on average 5 percent below the Medicare payment rate (Office of Inspector General 2012b). In the first quarter of 2009, acquisition costs for end-stage renal disease drugs among independent dialysis facilities averaged 10 percent below Medicare payment rates (Office of Inspector General 2010).

To get a sense of how physicians' drug acquisition costs compared with Medicare's payment rate, in our June

2016 report to the Congress, we analyzed proprietary invoice price data for 34 high-expenditure Part B drugs from IMS Health Incorporated for the clinic channel of purchasers (e.g., physicians and HOPDs). That analysis found that for two-thirds of the 34 drugs, at least 75 percent of the volume was sold to clinics at an invoice price below 102 percent of ASP.⁷ In addition, the analysis found evidence suggesting that some manufacturers responded to the sequester by changing their pricing patterns in a way that mitigated the effect of the sequester for some providers.⁸ Beginning April 2013, the sequester effectively reduced Medicare's payment rate for Part

B drugs from 106 percent of ASP to 104.3 percent of ASP. Analysis of the IMS data found that across the 34 drugs, the median of the 75th percentile invoice price as a percent of ASP fell when the sequester was implemented (from around 103 percent of ASP before the sequester to 101.5 percent of ASP in the second quarter 2013). This decrease suggests that providers' ability to purchase Part B drugs was generally maintained after the implementation of the sequester because manufacturers appear to have adjusted their prices to take into account the lower Medicare payment amount.

The Commission's June 2017 recommendation and next steps

In 2017, the Commission recommended several improvements to payment for Part B drugs. The recommendation included an ASP inflation rebate that would address price growth in the years after products launch, consolidated billing codes for biosimilars and originator biologics that would spur price competition among these products, and a voluntary alternative to the ASP payment system that would use vendors to negotiate lower prices and share savings with providers and beneficiaries (see text box for a summary of the recommendation). In addition, the recommendation included policies to require all manufacturers to report ASP data and to reduce payment for drugs that lack ASP data from WAC plus 6 percent to WAC plus 3 percent (see text box, pp. 64–65, for a discussion of overpayments for drugs lacking an ASP reporting requirement).

The policies in the June 2017 recommendation that aim to spur competition, address price growth, and lower prices would be important steps forward; nonetheless, several additional issues remain that increase spending for both the Medicare program and beneficiaries. Under the ASP + 6 percent payment system, a new drug receives its own payment rate based on its own ASP. The payment system is not designed to spur price competition among single-source drugs that have similar health effects. A drug's payment rate may not have any relationship to its clinical effectiveness. FFS Medicare currently lacks tools to arrive at payment rates for new drugs that balance an appropriate reward for innovation with value and affordability for beneficiaries and taxpayers. In addition, concern exists about provider incentives under the ASP payment system. The 6 percent add-on to ASP may create incentives for some providers to select higher priced products, although studies examining this issue are limited.

Building on our June 2017 recommendation, this chapter examines the potential of more broadly applying two strategies that were elements of that recommendation—reference pricing and binding arbitration—in an effort to improve price competition and value for Part B drugs. Both of these strategies would require that the Congress change the statute to give CMS the authority to implement them.

- **Reference pricing.** This policy would apply reference pricing to Part B single-source drugs with similar health effects in order to spur price competition among products and reduce prices.
- **Binding arbitration.** This policy would permit the Secretary to enter into binding, baseball-style (i.e., final-offer) arbitration with a drug manufacturer for a high-cost Part B drug with limited competition under certain circumstances. This policy would provide a way to incorporate value, affordability, and an appropriate reward for innovation in Medicare payment rates.

The Commission's June 2017 recommendation as well as the strategies discussed in this chapter would be expected to reduce Medicare payment rates for some Part B drugs and yield savings for beneficiaries and taxpayers.

Some stakeholders raise concerns that policies aimed at reducing Medicare spending for Part B drugs would reduce incentives for innovation. While arguments can be made that any effort to reduce drug prices lessens incentives for innovation, there is an inherent need to strike a balance between incentives for innovation and affordability and access. A presumption of arguments against reducing drug prices is that current prices strike the appropriate balance. But others argue that the current level of prices for some products adversely affects affordability and access and exceeds what is necessary to provide appropriate incentives for innovation. Kapczynski and Kesselheim contend that policies that lower drug prices for some products would improve patient access to care and that the net gains to population health would dwarf possible risks to pharmaceutical innovation (Kapczynski and Kesselheim 2016). Frank and Ginsburg point to the economic principle of diminishing returns and note that “at some point, perhaps already reached, the yield from additional resources going into R&D [research and development] no longer justifies what society is paying in the form of higher prices to support this” (Frank and Ginsburg 2017). In addition, Nichols acknowledges

Overpayments for drugs that lack an ASP reporting requirement

Manufacturers of Part B drugs that do not have a Medicaid rebate agreement are not required to report average sales price (ASP) data. In June 2017, the Commission recommended that all manufacturers of Part B drugs be required to report ASP data. In the physician office, products that lack ASP data and that are not new are paid according to the statute using generally higher pricing metrics such as wholesale acquisition cost plus 6 percent (WAC + 6 percent) or other methods that were in effect on November 1, 2003 (i.e., 95 percent of the average wholesale price (AWP) or invoice pricing). Under the outpatient prospective payment system, products that lack ASP data are also paid based on WAC or AWP.

Sodium hyaluronate products (which are injected into the knee to treat pain resulting from osteoarthritis) are regulated as devices and may not be subject to Medicaid Rebate Agreements. Over time, we have

observed fewer of these products being listed in Medicare's ASP payment rate files posted on the CMS website. In the second quarter of 2018, there were 10 products with billing codes, and 7 of those products had payment rates listed in CMS's ASP payment rate files on its website. By the second quarter of 2019, there were 11 products with billing codes but only 3 products had payment rates listed in CMS's ASP payment rate files on its website.

For the four products that appeared in CMS's ASP payment files in the past but no longer do so, we can compare the product's last payment rate listed in the CMS ASP payment rate files with the current WAC + 6 for the product. This comparison indicates that WAC + 6 percent is substantially higher than the last ASP + 6 payment for these four products: 15 percent, 91 percent, 97 percent, and 245 percent higher. Since these four products are not currently

(continued next page)

the importance of striking the right balance between encouraging innovation—by granting temporary monopoly pricing power—and ensuring affordability by encouraging postmonopoly competition (Nichols 2015). This researcher goes on to contend that “the [drug] cost problem is sufficiently serious and escalating that it is impossible to believe that we are being well served by the current configuration of innovation encouraging policies and actual pricing choices that specialty drug manufacturers are making” (Nichols 2015). Sachs and Frakt also suggest that some drug payment policy changes including internal reference pricing could have the potential to shift the mix of innovation toward drugs that provide more value (Sachs and Frakt 2016). It is also notable that the government already contributes to innovation indirectly through its substantial funding of basic science research and directly for some products through its funding of specific drug development research (Galkina Cleary et al. 2018, Sampat and Lichtenberg 2011).

Spurring price competition with reference pricing

The current ASP payment system maximizes price competition among generic drugs and their associated brand products by assigning these products to a single billing code, which we call a consolidated billing code.⁹ By contrast, products that are assigned to their own billing code and paid according to their ASP do not all face the same incentive to compete based on price and quality and generate the best price for beneficiaries (who are liable for 20 percent cost sharing) and taxpayers. In addition, the 6 percent add-on to ASP creates incentives for some providers to choose higher priced products over lower priced products. Thus, the current system does not spur price competition among:

- Therapeutically similar single-source drugs and biologics. There are examples of therapeutically similar products that are among the Part B 20 highest

Overpayments for drugs that lack an ASP reporting requirement (cont.)

listed in CMS's ASP payment files, we are not able to observe the current rates being paid for these products in the physician office setting. However, the payment rates for these products in the outpatient hospital setting continue to be published. These outpatient prospective payment system (OPPS) rates for the four products increased by the large percentages mentioned previously and appear to be set at WAC + 6 percent in the period when they are not listed in CMS's ASP payment files posted on its website. OPPS payments for two additional products that have never been listed in CMS's ASP payment files also appear to be based on WAC + 6 percent. (The OPPS payment rate for a new product that was first marketed in 2019 appears to be WAC + 3 percent, as would be expected in the first two to three quarters a new product is on the market).

WAC-based payment for sodium hyaluronate products has the potential to lead to substantial overpayments for this class of drugs. In total, the class of sodium

hyaluronate products accounted for over \$460 million of Part B drug spending in 2017. About \$170 million of that spending in 2017 was on the four products that have experienced substantial increases in the OPPS payment rates in 2018 or 2019, coinciding with the products no longer being listed in CMS's ASP payment rate files. An additional \$20 million of that spending in 2017 was on products that have never been listed in the ASP payment rate files. Although we cannot be certain why these products are not being listed in CMS's files, a possible explanation may be that manufacturers are choosing not to report because they are not required to do so, and by not reporting, providers could receive higher WAC-based payments for these products. The Commission's recommendation to require all manufacturers to report ASP data would be an important step to ensure against overpayments as a result of manufacturers choosing not to report ASP data. ■

expenditure products, whose ASPs have either remained the same or increased since 2010.¹⁰

- An originator biologic and its biosimilars. We have observed little decline in the ASPs of the originator biologics, but lower and declining ASPs for the biosimilars. As described in the text box (pp. 66–67): (1) the ASP for the originator product Neupogen has remained roughly the same between the first quarter of 2016 and the first quarter of 2019, while the ASP for its biosimilar Zarxio has declined by 34 percent, and (2) the ASP for the originator product Remicade has declined by 7 percent between the first quarter of 2017 and the first quarter of 2019, while the ASP for its biosimilar Inflectra has declined by 43 percent. Use of the more costly originator products Remicade and Neupogen accounted for 91 percent and 32 percent, respectively, of the market in the third quarter of 2018 (the most recent calendar quarter for which utilization data are available). To spur competition between the

originator biologic and its biosimilars, in 2017, the Commission recommended that the Congress require the Secretary to use a common billing code policy to pay for an originator biologic and its biosimilars. Such a policy would also address the incentive that the 6 percent add-on creates for some clinicians to select the more costly product.

Background on reference pricing

Research suggests that in many therapeutic classes, the approval of a new brand-name drug or biologic leads to higher list prices not just for the new product but also for the existing products. For example, according to researchers, competition between two or more brand-name products in the same class does not usually result in substantial price reductions (Kesselheim et al. 2016). Other researchers reported that the prices of first-generation disease-modifying therapies for the treatment of multiple sclerosis increased many times higher than

Under separate payment codes, price competition between an originator biologic and its biosimilar is not maximized

There has been little decline in the average sales prices (ASPs) of the originator biologics (Neupogen and Remicade), but lower and declining ASPs for their biosimilars. Applying either consolidated billing code or reference pricing policies would generate more price competition than under the current policy of assigning each product to its own billing code.

As of February 2019, two Part B originator biologics—Neupogen and Remicade—face biosimilar competition.

Neupogen was the first Part B product to experience biosimilar entry, with the biosimilar Zarxio entering in late 2015 and another product, Granix, that is similar to Neupogen, entering earlier.¹¹ Medicare payment rates for Zarxio and Granix are roughly 40 percent lower than the payment rate for the originator, Neupogen (Table 3-3). Utilization has shifted away from Neupogen, with Zarxio and Granix accounting for 67 percent of utilization as of third quarter 2018 (this number differs from figures in Table 3-3 due to rounding).

(continued next page)

**TABLE
3-3**

Medicare payment rates and utilization for originator Neupogen and biosimilars

	Medicare payment rate			Payment rate per unit for product as share of originator Neupogen's rate		Share of total units billed accounted for by:	
	Neupogen (originator)	Zarxio (biosimilar)	Granix*	Zarxio (biosimilar)	Granix*	Zarxio (biosimilar)	Granix*
2016 Q1	\$1.01	\$0.97	\$0.77	96%	76%	9%	16%
2016 Q3	1.00	0.87	0.77	87	76	28	18
2017 Q1	1.00	0.78	0.71	78	71	34	17
2017 Q3	1.01	0.73	0.64	72	64	38	19
2018 Q1	1.00	0.69	0.61	69	61	44	19
2018 Q3	1.02	0.65	0.59	64	58	50	18
2019 Q1	1.00	0.64	0.58	63	58	N/A	N/A

Note: Q (quarter), N/A (not available).

*Although Granix is not a biosimilar in the U.S. (because it was approved under the standard Food and Drug Administration approval process for new biologics), we include it here because it was approved as a biosimilar to Neupogen in Europe and it functions as a competitor to Neupogen and Zarxio in the U.S. market.

Source: MedPAC and Acumen LLC analysis of Medicare claims data and MedPAC analysis of average sales price files from CMS.

prescription drug inflation between 1993 and 2013, and they concluded that the price increases may have been a response to the introduction of competing treatments with higher prices (Hartung et al. 2015).

Reference pricing is a tool that some payers use to spur price competition among therapeutically similar drugs and other medical services and to lower the average price

paid. Under reference pricing, a payer establishes the price (reimbursement rate) that it is willing to pay for a given drug or procedure—the reference price. Payers use two approaches to reference pricing—internal reference pricing and international reference pricing.

Under internal reference pricing, a payer establishes the reference price for groups of drugs with similar health

Under separate payment codes, price competition between an originator biologic and its biosimilar is not maximized (cont.)

Remicade’s experience with biosimilar entry has been different. The payment rates for Remicade’s two biosimilars (Inflectra and Renflexis) are lower than Remicade’s (roughly 20 percent to 25 percent lower as of the first quarter of 2019), but the biosimilars account for only a small share of the market (9 percent of utilization as of the third quarter of 2018) (Table

3-4). The originator Remicade’s ASP declined 7 percent between 2017 and 2019 (Table 3-2, p. 60). However, Remicade’s ASP remains high from a historical perspective since its ASP grew substantially from 2009 to 2017 (at a cumulative growth rate of 44 percent). ■

**TABLE
3-4**

Medicare payment rates for originator Remicade and biosimilars

	Medicare payment rate			Payment rate per unit for biosimilars as share of originator Remicade’s rate	Share of total units billed accounted for by biosimilars
	Remicade (originator)	Inflectra (biosimilar)	Renflexis (biosimilar)		
2017 Q1	\$82	\$100	N/A	122%	0%
2017 Q3	86	80	N/A	94	4
2018 Q1	86	76	\$76	88	6
2018 Q3	84	65	69	77–83	9
2019 Q1	77	57	62	75–81	N/A

Note: Q (quarter), N/A (not available).

Source: MedPAC and Acumen LLC analysis of Medicare claims data and MedPAC analysis of average sales price files from CMS.

effects, a price that is typically based on the payer’s own prices. It is a concept that could be used for both medical benefits under Part B and outpatient drugs under Part D, but the recipients of the reference price would differ. In Part B, Medicare would pay medical providers the reference price, while under Part D, plans would pay the reference price to pharmacies. In either situation, if the provider and patient select a therapy priced higher than the reference price, the patient typically pays any difference as additional cost sharing. Compared with other drug management strategies (e.g., formularies), internal reference pricing does not restrict the selection of drugs within a given therapeutic class.

Under international reference pricing, a payer uses the prices that other countries pay for a drug in order to derive

a reference price or to negotiate with the manufacturer the price of that product. An example of international reference pricing is the potential model that CMS is considering testing through the Center for Medicare & Medicaid Innovation—the international pricing index (IPI) model—that would determine a payment rate for Part B drugs based on a target price that is linked to international prices from 14 countries. (See text box (p. 69) for a description of the IPI and text box (p. 70) for a summary of the study by the Assistant Secretary for Planning and Evaluation (ASPE) that informed the IPI on differences between Medicare and international prices for Part B drugs.)

As shown in Table 3-5 (p. 68), some of the design elements that payers consider when establishing both pricing strategies are similar, such as the frequency of

**TABLE
3-5**

Design elements of internal and international reference pricing

Design element	Internal reference pricing	International reference pricing
Scope of policy	Can include broad groups of products with similar health effects (e.g., single-source products) or narrower groups of products with the same active ingredients.	Applied on a drug-by-drug basis, not necessary to define groups of clinically similar products.
Source of reference price	Typically payer's own pricing data are used, but can use pricing data of other domestic purchasers.	Uses other countries' pricing data that can be obtained from secondary data sources, manufacturers, or websites of the reference countries.
Countries included in the reference basket	Does not use other countries' pricing data.	Reference countries are typically selected based on economic characteristics and geographic proximity. Countries included in the basket may vary depending on availability of new drugs.
Setting the reference price	Reference price for a group of clinically similar products typically based on the distribution of a payer's prices for the products in the group (e.g., reference price set at the median, weighted average, or least costly product).	Reference price for drugs under question is based on distribution of other countries' prices (e.g., reference price cannot be lower than the lowest price observed in countries included in reference basket).
Frequency of updating the reference price	Both internal and international reference pricing consider frequency of updating the reference price, which can include quarterly and annually.	Both internal and international reference pricing consider frequency of updating the reference price, which can include quarterly and annually.

Source: MedPAC analysis of published literature on internal and external reference pricing.

updating the reference price and the calculation of the reference price. The two approaches differ with respect to their source of pricing data and application. Internal reference pricing, which uses a payer's own pricing data, aims to spur price competition among products with similar health effects by incentivizing the selection of lower priced products, which, in turn, spurs price competition and lowers drug prices. By contrast, under international reference pricing, (ostensibly lower) drug prices from other countries are used to set the price of drugs under question.

Currently, Medicare does not use either type of reference pricing policy to pay for Part B drugs, though it has in the past:

- Between 1995 and 2010, Medicare Part B implemented two internal reference pricing policies—referred to as the least costly alternative (LCA) and functional equivalence policies—to pay for groups of drugs (prostate cancer drugs and anti-anemia biologics)

with similar health effects. Since 2010, because of court rulings and statutory changes, Medicare Part B no longer uses either reference pricing policy and pays for each drug according to its own ASP. Because the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 requires that biologics and single-source drugs (without generic competition) be paid based on their ASP and not averaged with other products' ASP, a change in the statute would be necessary for the Secretary to use internal reference pricing to pay for Part B drugs.

- In 2008, at least three national Part D sponsors (Health Net, Silver Script, and Sterling) used internal reference pricing for certain drugs—generally brand-name drugs with a generic equivalent. However, CMS prohibited the use of reference pricing in 2009 after beneficiary advocates argued that plan enrollees could not accurately calculate their out-of-pocket costs because Medicare's Plan Finder tool did not provide the incremental cost-sharing amounts. The Secretary

The international pricing index model for Part B drugs

In an advance notice published in 2018, CMS described a potential model the agency is considering testing through the Center for Medicare & Medicaid Innovation. CMS indicates the model, referred to as the international pricing index (IPI) model, would shift from paying physician and outpatient hospitals for Part B drugs to paying private vendors for these products. The prices Medicare pays these vendors for Part B drugs would be reduced over a five-year period to levels closer to international prices.

Under the IPI model, the government would determine a payment rate for a Medicare fee-for-service (FFS) Part B drug based on a target price linked to international prices. According to estimates by the Assistant Secretary for Planning and Evaluation (ASPE), in the first quarter of 2018, acquisition costs for certain Part B drugs in the U.S. were, on average, about 1.8 times higher than in other countries (Office of the Assistant Secretary for Planning and Evaluation 2018). Over a five-year period, the IPI model would phase in a target price for Part B drugs, which the agency states would result in about a 30 percent reduction in spending. The target price would be calculated by multiplying the IPI—the ratio of Medicare spending under average sales price (ASP) to international prices (holding volume and the mix of drugs constant)—and a factor that would phase in a spending reduction of about 30 percent over time.¹² The percentage reduction between the target price and ASP would vary for each drug. If a product's ASP

was lower than the target price, CMS would set the payment amount to the ASP for that drug.

The IPI target prices would apply to certain Part B drugs furnished in selected geographic areas. CMS indicates that it intends to select geographic areas that account for about 50 percent of Part B drug spending. In those areas, the model would be mandatory for physicians and outpatient hospitals, which would be required to acquire Part B drugs that they furnish to Medicare FFS beneficiaries through IPI vendors. CMS indicates it would phase in the group of products included in the model over time, focusing first on single-source drugs and biologics. The agency states that it could begin by including most of the products that appeared in the ASPE report, which accounted for over 50 percent of Part B drug charges in 2017 (Office of the Assistant Secretary for Planning and Evaluation 2018).

Under the IPI model, Medicare would pay the vendor for Part B drugs at the payment rate established based on the international target price. Vendors would negotiate with manufacturers over their own acquisition costs for drugs, but those negotiations would not affect Medicare payment rates. The vendor's negotiated price would determine whether the vendor made a profit or loss given the Medicare payment rate established by CMS. The advance notice mentions the potential for IPI model vendors to pursue indication-specific pricing or outcome-based arrangements, but does not mention pharmacy management tools such as a formulary, step therapy, or prior authorization.¹³ ■

has the authority to let Part D plans use internal reference pricing.

- Medicare has never used international reference pricing to pay for covered drugs, and a change in the statute would be necessary for the Secretary to use this approach.

Building on the Commission's 2017 recommendation that would group an originator biologic and its biosimilars

in the same billing code to maximize price competition, the remainder of this section focuses on Medicare's use of internal reference pricing for single-source drugs with similar health effects.

Applying internal reference pricing to spur price competition in Medicare

Internal reference pricing is a tool that some payers and purchasers use to spur price competition among therapeutically similar drugs and other medical services.

Assistant Secretary for Planning and Evaluation study found the average sales prices for certain Part B drugs were, on average, 1.8 times higher than in other countries

In 2018, the Assistant Secretary for Planning and Evaluation (ASPE) published a study that compared the prices that Medicare paid for selected Part B drugs with the prices paid in other countries. Key design elements of this study include the following:

- The analysis used international pricing data from IQVIA that provided ex-manufacturer prices (i.e., the price a manufacturer is paid for its product).
- The drugs included in the analysis were compiled from the top 20 drugs in terms of 2016 Medicare spending to physician offices, hospital outpatient departments, or overall. The final list of 27 drugs included only sole-source drugs (excluded products include vaccines, blood products, and contrast agents; products not physician administered; and products that lacked IQVIA data).
- The analysis compared Medicare average sales prices (ASPs) in the third quarter of 2018 with prices paid internationally in the first quarter of 2018.
- The analysis included 16 countries in the reference basket: Austria, Belgium, Canada, Czech Republic, Finland, France, Germany, Greece, Ireland, Italy, Japan, Portugal, Slovakia, Spain, Sweden, and the U.K. ASPE defined the reference basket based on all countries (except the U.S.) included in the so-called Group of

Seven (Canada, France, Germany, Italy, Japan, and the U.K.) and all countries in Germany's basket (15 countries), but excluded 2 of the countries (Denmark and the Netherlands) because IQVIA data were not available.¹⁴

Key findings of this analysis include the following:

- Only 11 of the 27 drugs in the analysis were sold in all 16 comparator countries.
- Medicare Part B ASPs were 1.8 times that of the average international ex-manufacturer price. Medicare and its beneficiaries spent an additional \$8.1 billion (47 percent more) for the studied products than they would have if payments based on ASP were scaled by the international price ratios that ASPE calculated.
- U.S. prices are lower for Gammagard, and prices are similar for six products. For the remaining 20 products, U.S. prices exceed the average international price by more than 20 percent. Moreover, for 14 of the 20 products, U.S. prices are at least double (i.e., more than 100 percent above) the average international price.
- Germany and Canada had the highest prices for six drugs and Japan for five drugs. No other country had the highest price for more than three drugs. However, France and the U.K. had the lowest price for four products, and Japan, Sweden, and Slovakia had the lowest prices for three drugs. ■

For drugs covered under medical benefits, payers establish a reference price for a group of drugs with similar health effects assigned to separate payment codes. For example, the reference price can be based on the average, median, or volume-weighted average of the payments of all the products in the reference group. When the reference price is based on the least costly product of all the products in the group, the reference pricing policy is referred to as the LCA policy.

If a therapy is prescribed that is priced higher than the reference price, the patient typically pays any difference as cost sharing. Because there is a reference price on what clinicians or outpatient departments would receive in payment and there are potentially large differences in cost sharing, reference pricing gives all parties strong incentives to consider lower cost therapeutic alternatives. If beneficiaries are aware of their potential cost-sharing obligations, reference pricing in this context also provides strong incentives for beneficiaries to ask their prescriber about lower cost therapies.

Under Part B, reference pricing policies could also take the form of assigning products that result in similar health effects to the same billing code—a consolidated billing code—or paying a single reference price for products with similar health effects that are assigned to their own billing codes.¹⁵ The reference pricing and consolidated billing policies are strategies in which a payer sets a single payment rate for therapeutic groups of products that result in similar health effects.

Internal reference pricing is a concept that can also be used to pay for Part D drugs. When applying internal reference pricing to Part D drugs, a plan and pharmacy benefit manager (PBM) design their formulary to include a maximum amount they will pay to pharmacies for a therapeutic category. Rather than exclude certain drugs, the formulary may allow an enrollee access to a broader range of therapies, but the enrollee must pay more in cost sharing for higher priced drugs. The plan's pharmacy and therapeutics committee would provide input on which therapies could substitute for one another, on which agent is preferred for the class (the basis for the maximum payment amount), and on preferred cost-sharing amounts.

The Commission has held that Medicare should pay similar rates for similar care. With respect to groups of products with similar health effects, this principle might warrant that Medicare use a reference pricing or consolidated billing code policy when paying for these products under Part B. Table 3-6 (p. 72) presents examples of groups of competing products, with each product paid under a separate billing code based on its separate ASP. We derived these groups from reference pricing policies implemented by Medicare and commercial payer policies or policies suggested by the Congressional Budget Office (CBO) and OIG. The pricing behavior exhibited by some manufacturers—the ASPs for all of the products have not substantially declined between 2009 and 2019—suggests that applying a reference policy could spur price competition among these products. In 2017, Medicare spending for all the products in the eight therapeutic groups included in Table 3-6 totaled nearly \$12 billion.¹⁶ In addition to these products, there are other examples of groups to consider under a broader consolidated billing code policy.

Applying reference pricing policies to Part B drugs would be expected to generate more price competition among products than paying for each product based on its own ASP. Drug manufacturers would have an incentive to

lower their price relative to their competitors' to make their product more attractive to providers and garner market share. Both CBO and the Department of Health and Human Services OIG have said that use of LCA policies would result in savings for beneficiaries and taxpayers. OIG estimated savings of \$275 million for beneficiaries and \$1.1 billion for the program by using an LCA policy (in 2008 and 2009) to pay drugs that treat wet age-related macular degeneration (Office of Inspector General 2011). CBO estimated savings of almost \$500 million between 2010 and 2019 if an LCA policy had been used for drugs that treat osteoarthritis of the knee (Congressional Budget Office 2008).

Researchers have also found savings from applying reference pricing policies to drugs (Robinson et al. 2017). For example, a 2014 literature review (published by the Cochrane Library) of 17 studies of internal reference pricing policies used in 7 countries (including the U.S.) concluded that the policy generally reduced payers' total spending in the short term (through 2 years) by shifting use from more costly drugs that required higher cost sharing to drugs paid at the reference price (Acosta et al. 2014). In a 2012 literature review, Lee and colleagues reviewed 16 studies of internal reference pricing policies used in 6 countries and concluded that the policies reduced the average price of drugs included in the reference groups by 7 percent to 24 percent (Lee et al. 2012).

Between 1995 and 2010, Medicare used LCA policies to pay for selected Part B drugs

The medical directors associated with the Medicare administrative contractors (MACs) established LCA policies between 1995 and 2010 to set the payment rate for certain Part B drug classes, including luteinizing hormone-releasing hormone agonists for prostate cancer. Under the LCA policy, the MACs used the prevailing Medicare payment policy to determine Medicare's payment rate (i.e., ASP-based payment) for each product and then set the payment rate for all the products with similar health effects based on the least costly product.¹⁷

The contractors' medical directors generally based LCA determinations on the premise that "if two services are clinically comparable, then Medicare does not cover the additional expense of the more costly service, when this additional expense is not attributable to that part of an item or service that is medically reasonable and necessary" (National Government Services 2009). LCA policies were implemented in local coverage decisions in which the

**TABLE
3-6**

Between 2009 and 2019, ASPs of single-source products with similar health effects have not substantially declined

	Average annual ASP growth from January to January of each year (2009-2019)	First year of pricing data (if not 2009)
Erythropoiesis-stimulating agents: <i>Biologics that stimulate production of red blood cells</i>		
Aranesp (darbepoetin alfa)	2.3%	
Epogen (epoetin alfa)	2.6	
Antivascular endothelial growth factors: <i>Biologics that treat wet age-related macular degeneration and other conditions</i>		
Eylea (aflibercept)	-0.3	2013
Lucentis (ranibizumab)	-1.1	
Targeted immune modulators: <i>Biologics that treat selected immunologic diseases</i>		
Remicade (infliximab originator biologic)	3.0	
Orencia (abatacept)	10.4	
Rituxan (rituximab)	5.9	
Leukocyte growth factors: <i>Biologics that stimulate proliferation and differentiation of normal white blood cells</i>		
Neupogen (filgrastim originator biologic)	4.1	
Neulasta (pegfilgrastim)	8.0	
Granix (tbo-filgrastim)	-7.2	2015
Immune globulins: <i>Products that treat primary humoral immunodeficiency and other selected conditions</i>		
Gamunex-C/gammaked	1.2	
Gammagard liquid injection	1.8	
Privigen	1.7	
Luteinizing hormone-releasing hormone agonists for prostate cancer: <i>Products that treat prostate cancer</i>		
Trelstar (triptorelin pamoate)	3.8	
Zoladex (goserelin acetate implant)	10.6	
Lupron (leuprolide acetate suspension)	1.2	
Botulinum toxins: <i>Products that treat various focal muscle spastic disorders and excessive muscle contractions</i>		
Botox (onabotulinumtoxinA)	1.2	
Myobloc (rimabotulinumtoxinB)	2.8	
Xeomin (incobotulinumtoxinA)	-1.2	2012
Viscosupplements using hyaluronate for osteoarthritis of the knee		
Orthovisc	-1.9	
Hyaluronan, Hyalgan, or Supartz	-2.0	
Synvisc or Synvisc-One	0.4	

Note: ASP (average sales price). For each group, table includes only up to the three leading products as measured by 2017 Part B spending. We include Granix in this table because, in the U.S., it was approved under the standard Food and Drug Administration approval process for new biologics. However, the product was approved as a biosimilar to Neupogen in Europe, and it functions as a competitor to Neupogen and Zarxio (Neupogen's biosimilar) in the U.S. market.

Source: MedPAC analysis of data from CMS's average sales price quarterly pricing files, 2009-2019.

medical director decided to cover a particular product in its geographic jurisdiction. LCA policies were established based on the Secretary's authority from Section 1862(a) (1)(A) of the statute that states that "no payment may be made under Part A or Part B for any expenses incurred

for items or services which . . . are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member." Simply put, LCA policies were applied under the premise that Medicare should not pay for the

additional cost of a more expensive product if a clinically comparable product costs less. Although the statutory platform for making LCA determinations was based on Medicare's reasonable and necessary (coverage) authority, the policy affected the payment rate of a product. The MACs' medical directors established LCA policies in the local coverage determination process within their geographic jurisdiction.

In applying LCA policies to Part B drugs, the MACs' medical directors generally followed these steps: (1) determined that the product was a Medicare-covered benefit; (2) determined that the product was "reasonable and necessary" for the treatment of an illness or injury; (3) reviewed clinical evidence (from the Food and Drug Administration (FDA) and other sources) to determine whether the product is clinically similar to other Medicare-covered products; and (4) established the payment rate for each drug covered under the LCA policy under the prevailing Medicare payment policy and set the payment rate for all the products based on the product with the lowest ASP.

In some instances, the MACs' medical directors would pay the higher rate for the more costly product when the physician could document that the more costly product was medically necessary. In addition, there was an opportunity for the beneficiary to choose the more costly product. Specifically, if the physician informed the beneficiary in advance and in writing that Medicare was likely to deny payment for the more costly product and if the beneficiary signed an advance beneficiary notice for the product, then the beneficiary could pay an additional sum if he or she and the physician chose a more costly service or product. Under these circumstances, the beneficiary's liability would include the 20 percent coinsurance and the difference in the Medicare payment between the more costly and least costly product.

In 2008, a beneficiary challenged the proposed application of an LCA policy for an inhalation drug, arguing that the statute requires that if the drug is reasonable and necessary, Medicare must pay the statutorily defined payment rate for the drug—ASP + 6 percent. The government argued that the reasonable and necessary statutory provision confers great discretion on the Secretary and that the LCA policy is permissible because the provision explicitly addresses payment and expenses.

Two federal courts agreed with the beneficiary and ruled that Medicare cannot use LCA policies to pay for Part B

inhalation drugs, asserting that the statute's provision that sets the payment rate for Part B drugs based on its ASP precludes Medicare from applying LCA policies. These rulings apply to instances in which CMS has set a drug's payment based on the ASP of the least costly alternative. Effective April 2010, the MAC's medical directors rescinded the LCA policies applied to Part B drugs, and since then, Medicare's payment rate for products previously paid for under an LCA policy (e.g., prostate cancer drugs) is 106 percent of the product's ASP.

According to federal agencies, applying reference pricing policies to Part B drug payment could reduce Medicare spending for beneficiaries and taxpayers. OIG has twice recommended that the Secretary apply LCA policies to prostate cancer drugs. In 2004, OIG reported that not all carriers included one of the prostate cancer drugs (leuprolide acetate) in their LCA policy and recommended that CMS encourage all Medicare contractors to include this product when applying LCA policies to this drug group. OIG estimated that if such a policy had been implemented, Medicare and beneficiaries would have saved \$40 million per year (Office of Inspector General 2004). In 2012, OIG reported that after LCA policies were removed for a group of drugs that treat prostate cancer, utilization patterns shifted dramatically in favor of costlier products, and the agency concluded that spending for these products was higher in the absence of LCA policies.¹⁸ OIG estimated one-year savings of nearly \$7 million for beneficiaries and nearly \$27 million for Medicare if an LCA policy was used to pay for these prostate cancer drugs (Office of Inspector General 2012a). Neither study addressed the effect of the LCA policies on beneficiaries' use of other medical services.

Between 2003 and 2005, Medicare used the functional equivalence standard in the hospital OPPS

The "functional equivalence standard" is another name for a reference pricing policy under which payment for products with similar health effects assigned to separate payment codes is based on the least costly item. In 2003, in the rule-making process for the hospital OPPS, CMS set the payment rate nationally for a new biologic (darbepoetin alfa) at the rate of an existing, less costly product (epoetin alfa) after concluding that both anti-anemia products were clinically comparable because they used the same biological mechanism to produce the same clinical result—stimulation of the bone marrow to produce red blood cells. CMS did not initially set the payment rate of the new product by using the functional equivalence

standard. Rather, in the 2003 proposed hospital OPPS rule, CMS said that it would continue the new biologic’s transitional (higher) pass-through payments. In response, a product developer argued that because both the old and the new biologics are substitutes, they should be paid at the same rate. In the final rule, CMS reviewed the clinical evidence, concluded that the biologics were functionally equivalent, and set the payment rate of the new biologic at the same rate as the older one (Centers for Medicare & Medicaid Services 2002). The agency implemented this payment policy on its authority to make adjustments necessary to ensure equitable payments to the transitional pass-through payments of the hospital OPPS.¹⁹

This policy withstood a lawsuit from the product developer of the new biologic. An appeals court dismissed the case, concluding that CMS’s statutory rationale for the decision was not subject to judicial review (U.S. Court of Appeals 2004). Subsequently, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) limited use of the functional equivalence standard. The Congress prohibited use of this standard for drugs and biologics in the hospital outpatient setting unless the standard was in place before the law’s enactment.

Medicare continued to use the functional equivalence standard in 2004 and 2005. In response to passage of the MMA, the payment rate for each biologic was set based on its ASP beginning in 2006.

Examples of reference pricing implemented by employers and other payers

Reference pricing for drugs is an emerging structure of benefit design for commercial payers and employers (Robinson 2018). For example, a self-insured employer-based purchaser (the Reta Trust, a national association of 55 Catholic organizations that purchase insurance for their employees) implemented reference pricing for about 1,300 pharmacy benefit drugs in 2013 in part to address the diminishing effectiveness of the formulary to account for price variation and price increases within its formulary’s drug tiers.²⁰ The program included the following key elements:

- The reference price was based on the least costly drug in each therapeutic category.
- The reference pricing program focused on drug classes with extensive price variation among therapeutically equivalent products.
- Therapeutic classes were defined according to the criteria of the American Hospital Formulary Service

Pharmacologic–Therapeutic Classification, which is used to classify drugs for Medicaid and Medicare Part D formularies.

- A payment exception process (reviewed by clinical staff at the PBM) paid for a higher priced product if a clinician provided medical justification.
- Absent a clinical exception, patients who used a higher priced drug paid the price difference themselves.

To assess the effect of this reference pricing strategy, researchers compared the drug use and spending of Reta Trust members with a control group using multivariable difference-in-difference regressions and found that this policy:

- increased the probability by 7 percentage points that Reta Trust members selected the lowest priced product compared with the control group;
- decreased the average purchase price paid by nearly 14 percent (equivalent to a decrease of \$9.24 per monthly prescription); and
- increased Reta Trust members’ out-of-pocket spending by about 5 percent compared with the control group (equivalent to an \$0.84 increase in copayments per prescription) (Robinson et al. 2017).²¹

The authors did not assess the effects of reference drug pricing on the use of medical services because they lacked data on patients’ use of these services.

A state employee health plan (for Arkansas state and public school employees and retirees) also uses a reference pricing policy “when evidence shows one product in a class of drugs is not any more effective than the other drugs within the same therapeutic class” (ARBenefits 2019). This state employee health plan uses a design similar to the design of the Reta Trust policy, including basing the reference price on the lower cost product and requiring that the patient pay the difference between the higher and lower cost product (in the form of a higher copayment) if a higher priced product is preferred. Researchers compared costs before and after implementation of this reference pricing policy for one therapeutic group (proton pump inhibitors) and found reductions in members’ copayments (by 6.7 percent) and in the net cost per member per month (49.5 percent) (Johnson et al. 2011).

There is no exhaustive research on the use of reference pricing policies by commercial payers. We did not find any publicly available information that major commercial payers were using internal reference pricing for single-source products with similar health effects. However, for certain drug groups, a major commercial payer applies a strategy that is similar to an LCA policy. For example, the payer concluded that there is a lack of reliable evidence that any one brand of targeted immune modulators is better than other brands for medically necessary indications and that the least costly brands are as likely to produce equivalent therapeutic results as the more costly brands. Consequently, the payer considers a higher cost product to be necessary only if the member has a contraindication, intolerance, or ineffective response to one of the least costly brand products (Aetna 2019). Several payers and purchasers have applied internal reference pricing for surgical and diagnostic procedures, which has resulted in spending reductions of 20 percent for joint replacement, 18 percent for cataract removal, 21 percent for colonoscopy, 17 percent for arthroscopy, 12 percent for computed tomography, and 32 percent for laboratory assays (Robinson et al. 2017).

In recent years, commercial payers have relied on tiered formularies with differing levels of patient coinsurance and copayments as a tool to moderate drug spending. Formularies and reference pricing are similar in that both strategies identify drugs with similar health effects. With a tiered formulary, not all drugs may be included on the formulary, whereas with reference pricing, all drugs in the therapeutic group are available.

Examples of reference pricing implemented by other countries

Both internal and international reference pricing approaches are more frequently used by other countries (in Australia, Canada, Japan, and many European countries) than in the U.S. For example, researchers conducted a review of the drug pricing policies used in 20 countries and reported that 16 European countries used internal reference pricing in 2011.²² Of these 16 countries, 8 defined reference groups based on the active substance while another 8 had a broader classification system that defined groups of drugs based on therapeutic classes (Pew Charitable Trusts 2017).

International reference pricing is commonly applied in Europe. For example, a review of 31 countries (as of 2013) found that international reference pricing was used by Iceland, Norway, Switzerland, and all 28 European Union members, with the exception of Sweden and the

U.K. (Rémuzat et al. 2015).²³ According to Rémuzat and colleagues, there is some variation in the application of international reference pricing among these countries:

- Most (23) countries used international reference pricing as the main criterion for price setting or negotiations with manufacturers, while 6 countries (Belgium, Finland, Germany, Italy, Poland, and Spain) reported that international prices were one factor among many in the decision-making/negotiation process.
- The drugs that the policy may affect varies across countries. In some countries, the policy is used for specific categories of drugs, such as new, innovative products (e.g., France, Germany, and Spain), while in other countries the policy is used more broadly, applying to all outpatient drugs (brand and generics) and high-cost and orphan drugs used in the inpatient setting (e.g., the Netherlands).
- The number of reference countries included in a country's basket varied from 1 (in Luxembourg) to 31 (in Hungary and Poland). The most referenced countries were France, the U.K., and Germany.
- The reference price calculation methods differed across countries. The three main calculation methods were average price, lowest price, and average of the three or four lowest prices of all countries in the basket.
- Most countries used ex-manufacturer (i.e., the price a manufacturer is paid for its product) prices to calculate the reference price, followed by the pharmacy purchasing price.
- When different dosages and package sizes were approved at different prices in the reference countries, the same or closest package size or dosage was generally used as a reference.
- The time frame that prices were reevaluated varied from every three months to every five years (Rémuzat et al. 2015).
- Some countries use both internal and international reference pricing.

Case studies of two countries' application of reference pricing: Australia and Germany

Australia and Germany are similar in their drug pricing: Both countries apply internal reference pricing to therapeutic groups of drugs with similar health effects,

and both countries engage in price negotiation with manufacturers for new innovative products (e.g., first drug in a class). For a new, innovative drug, Australia considers information about its comparative clinical effectiveness and cost-effectiveness while Germany considers information about its comparative clinical effectiveness.

Australia—For a product to be paid for by the Australian Government Department of Health and Ageing (DHA), manufacturers submit an application to the Pharmaceutical Benefits Advisory Committee (PBAC), an independent statutory committee. The PBAC assesses whether the product is both clinically effective and (for products that are not yet covered) cost-effective compared with other treatments.²⁴ The Australian Minister for Health decides whether the drug will be included in the Pharmaceutical Benefits Scheme (PBS) based on the recommendation of the PBAC.

Reference pricing is applied to drugs considered to be of similar safety and efficacy for pricing purposes. The lowest priced product sets a benchmark price for either the other brands of that drug or the other drugs within the same subgroup of therapeutically related drugs. Patients pay any difference between the price of the drug purchased and the reference price. If a patient cannot take a product in the therapeutic group due to clinical reasons certified by the clinician, the government pays the contribution on the patient's behalf.

For innovative products that have been approved by the PBAC, the government enters into a negotiation with the manufacturer to set the price at which the product will be paid for on the PBS. The pricing of innovative products is informed by the cost-plus method, which grants a gross margin based on the costs of manufacturing (see <http://www.pbs.gov.au/industry/useful-resources/pbs-forms/pb11b.pdf> for cost information reported by the manufacturer). A margin on costs of around 30 percent is usually considered reasonable for new drug listings, but higher margins may be recommended for low-volume products, and lower ones may be recommended for high-volume products. If a product has more than one indication and a cost-effectiveness that varies across indications, a weighted average price is set according to expected volumes of use across the indications. The price of each covered drug is reviewed annually. A manufacturer is required to submit cost and other data if it wants the price of a given product to change.

Germany—Before 2011, Germany was one of the few European Union countries where pharmaceutical

manufacturers were largely free to set the prices for their new drugs. To address increasing drug spending and rising drug prices, in November 2010, the German parliament passed the Act to Reorganize the Pharmaceuticals' Market in the Statutory Health Insurance System (AMNOG). Consequently, since 2011, products with new active ingredients (or a new combination of active ingredients) are subject to a comparative clinical benefit assessment under the AMNOG:²⁵

- At the time of a drug's market launch, manufacturers are required to submit a dossier to the Federal Joint Committee (a group consisting of clinicians, providers, and health insurance funds that is responsible for coverage decisions) that demonstrates a new drug's added clinical benefit relative to a comparator therapy. (The Federal Joint Committee can also assess the benefit of products that were on the market before January 1, 2011, but remain under patent.)
- For most new drugs, the Federal Joint Committee commissions the Institute for Quality and Efficiency in Health Care (IQWiG) (an independent scientific body that conducts evidence-based assessments of health services and products) to evaluate the new product's added clinical benefit. Specifically, the assessment compares the clinical benefit (as measured by patients' improvement in health status, reductions in the duration of the disease, survival gains, reduction of side effects, and improvements in quality of life) of the new product relative to a comparative therapy.²⁶ Within three months after the product's market launch, this evaluation is completed and published on the internet.
- Within six months after the product's launch, the Federal Joint Committee, after considering IQWiG's assessment and comments from the manufacturer and other stakeholders, publishes a detailed decision document concerning the added value of the new drug. There are six classifications concerning the extent of the additional benefit: (1) major additional benefit, (2) considerable additional benefit, (3) minor additional benefit, (4) nonquantifiable additional benefit, (5) no additional benefit, and (6) less benefit. Based on this classification, one of two courses of action concerning the price setting of a pharmaceutical will follow:
 - If the Federal Joint Committee decides the product has no added clinical benefit, then the product is paid using internal reference pricing. The

Federal Joint Committee establishes the reference price, which is set near the 30th percentile in the distribution of prices within each therapeutic class, high enough to ensure that patients have more than one choice but low enough to ensure that the payer does not have to pay the highest prices within the class. There must be at least three products in a reference pricing group. If there is not a reference price group, the National Association of Statutory Health Insurance Funds negotiates with the pharmaceutical company a rebate to the ex-manufacturer price such that the payment does not lead to higher annual therapy costs than a comparator product (Spitzenverband 2019).²⁷ If negotiations fail to arrive at a price within six months, an arbitration committee sets the reimbursement amount within three months.

- For products with added therapeutic benefit: The National Association of Statutory Health Insurance Funds and the manufacturer negotiate the ex-manufacturer price. The negotiation process considers the evaluation of the IQWiG (including the proven additional benefit of the product relative to its comparator) as well as pricing from 15 European Union countries; the final price can reflect discounts and rebates to the ex-manufacturer price as well as price-volume agreements. If negotiations fail to arrive at a price within six months, an arbitration committee sets the reimbursement within three months.

Until this evaluation process is completed—the first 12 months after a drug’s launch—the price set by the manufacturer applies to the product. The payment rates derived from this process apply to persons with both statutory and private insurance and to self-paying patients.

Issues in implementing internal reference pricing in Medicare

For Medicare to apply reference pricing strategies, the program would need a clear legal foundation to apply them. Specifically, the Congress would need to restore the Secretary’s authority to apply reference pricing approaches. At present, the Secretary’s lack of flexibility to apply this approach stems from the MMA, which requires that biologics and single-source drugs (without generic competition) be paid based on their own ASP and not averaged with other products. Consequently, these products receive their own payment code.

In addition, the statute constrains Medicare’s use of comparative clinical effectiveness evidence (the foundation of reference pricing strategies) to pay for drugs. Medicare cannot use comparative clinical effectiveness evidence that the Agency for Healthcare Research and Quality produces to withhold coverage of prescription drugs. Since 2010, the Patient Protection and Affordable Care Act of 2010 constrains Medicare’s use of comparative clinical effectiveness research conducted by the Patient-Centered Outcomes Research Institute when making coverage decisions and setting payment rates.

Developing a clear and predictable decision-making framework; ensuring transparency and opportunities for public input

Reference pricing could be applied to existing groups of clinically similar products shown in Table 3-6 (p. 72). The Congress, when clarifying Medicare’s authority to apply reference pricing policies under Part B, could require that the Secretary establish a clear, public, predictable, transparent, and timely process and obtain public comment from a wide range of stakeholders, including beneficiaries, providers, and product developers. Some of the design elements that would be involved in establishing reference pricing policies include:

- how Medicare would define groups of products that are clinically similar;
- how Medicare would set a single payment (i.e., the reference price) for the products in a given group;
- how frequently the reference price would be updated;
- ensuring exceptions to reference pricing policies when a beneficiary’s clinical circumstances support the medical necessity for the more expensive service or product;²⁸
- providing pricing information to beneficiaries and clinicians (to make them sensitive to the difference in out-of-pocket spending);
- permitting a beneficiary to gain access to a more costly product by paying the difference (in the cost between the more costly product and the reference price) if that is his/her preference; and
- whether Medigap policies could cover beneficiary cost sharing that is greater than the reference price.²⁹

For a drug newly approved by the FDA, the Secretary would need a clear, transparent, and timely process for

evaluating its comparative clinical effectiveness compared with existing drugs that are the standard of care and for determining whether the drug should be included in an existing reference product group. The Secretary already has experience under the inpatient and outpatient hospital payment systems in developing the process and assessing whether new services represent clinical improvements compared with existing treatments. While a new drug's comparative clinical effectiveness is being considered, its payment rate could be based on prevailing Medicare payment policies (i.e., ASP + 6 percent), which would obviate delays in beneficiaries' access. Determining the overall length of time for the Secretary to implement this process would also need to be addressed.

To establish the payment rate for a reference group, CMS could determine the payment rate for each drug based on the prevailing payment policy and then set the payment rate for all the clinically similar products in the drug group based on, for example, the weighted average of all products within the group, at the 50th percentile of all ASPs of all the products within the group, or based on the ASP of the least costly product.

Regarding how Medicare would define groups of products, the program could seek advice and possibly contract with pharmaceutical and therapeutics committees to help develop and update groups of Part B products with similar health effects.

To motivate choice, providers and beneficiaries should receive up-to-date information on the payment rates for drugs that are paid for under reference pricing (Robinson 2018). As we noted earlier, reference pricing gives providers and beneficiaries strong incentives to consider lower cost therapeutic alternatives. There is evidence to suggest that physician practices of certain specialties, including oncologists, rheumatologists, and ophthalmologists, already consider the cost of alternative therapies in selecting Part B drugs and provide their beneficiaries financial counseling services, such as advising beneficiaries about their cost sharing based on their treatment choices (Office of Inspector General 2012a, Office of Inspector General 2012b, UVA Cancer Center 2018).

Addressing key concerns about reference pricing strategies

Two key concerns that stakeholders have raised about the application of reference pricing strategies for drugs are (1) the effect of the policies on manufacturers' incentives to

innovate and (2) the effect of the policies on beneficiaries' access to care.

Some stakeholders raise concerns that policies aimed at reducing Medicare spending for Part B drugs would reduce incentives for innovation. For example, Danzon and Ketcham argue that reference pricing policies applied to on-patent innovator drugs decrease the manufacturer's ability to recoup the costs of research and development, which in turn negates the intent of patents and undermines the incentives for product improvement or innovation (Danzon and Ketcham 2004). While arguments can be made that any effort to reduce drug prices lessens incentives for innovation, there is an inherent need to strike a balance between those incentives with affordability and access. Arguments against reducing drug prices presume that current prices strike the appropriate balance. However, others argue that the current level of prices for some products adversely affects affordability and access and exceeds what is necessary to finance innovation (Nichols 2015).

Proponents of reference pricing policies argue that such policies might actually increase manufacturers' incentive to develop more innovative products. Under the current process, development focuses on a stand-alone assessment of the safety and efficacy of a product. In a reference pricing environment, manufacturers would have to compare their product with other products in the clinical trials they sponsor. Some analysts have argued that determining the impact of any health care policy on the pace of innovation is difficult to ascertain because the socially optimal level of research and development is unknown.

A second key concern is that reference pricing strategies could have an adverse impact on beneficiary access. However, that concern would be addressed with a clinical exceptions policy. If a patient needed a particular drug, the patient could obtain an exception (certified by a clinician) and continue to have access to that drug with no increase in cost sharing. Some observers have argued that use of information about a service's comparative clinical effectiveness in the payment processes ignores the variability among individual patients in treatment efficacy, safety, and tolerability of treatment interventions and could result in "one-size-fits-all" policies. Acosta and colleagues found that the effects of reference pricing on health are uncertain due to a lack of rigorous evidence, while Lee and colleagues concluded that reference pricing did not increase use of medical services such as physician visits and hospitalizations (Acosta et al. 2014, Lee et al. 2012). Robinson and colleagues lacked the necessary data

to examine the impact of reference pricing on patients' health outcomes (Robinson et al. 2017). Some observers have also suggested that the cost sharing that patients may incur in order to access the product of their choice (absent a clinical exception certified by a clinician) will lead to nonadherence. To address the concern that reference pricing might lead to patients becoming noncompliant, seeing their physician more frequently, or being hospitalized more frequently, the Secretary could monitor and publicly report on the outcomes of affected patients.

Stakeholders have raised concerns specific to international reference pricing that include:

- The transparency of a drug's transaction price across countries. Accurate measurement of transaction (net) prices is increasingly problematic due to the growing use of confidential rebates and other risk- and cost-sharing measures between manufacturers and payers/countries. Indeed, such confidential (off-invoice/postsale discounts) rebates may be preferred by manufacturers to reductions in list prices, which would spill over to countries through international reference pricing. Manufacturers may design and implement pricing and marketing strategies to counteract the effects of international reference pricing. For example, manufacturers can list high prices in reference countries while providing those countries with confidential rebates or discounts. Because off-invoice rebates and other confidential agreements are not reflected in publicly available drug prices, payers may ultimately reference inaccurate higher prices. Docteur argues that international reference pricing may inflate manufacturers' list prices (Docteur 2008). ASPE notes that using list prices in its analysis may not accurately reflect the actual amount paid in the U.S. and other countries and that its results may be biased due to differences across countries in the use of postsale discounts (and other policies) that are not reflected in the manufacturers' list price (Office of the Assistant Secretary for Planning and Evaluation 2018).
- Prices from existing data sources are not measured consistently. Toumi and colleagues state that comparing prices across countries is difficult because available pricing data are varied (Toumi et al. 2014). For example, pricing data could vary depending on whether they reflect the pharmacy's purchasing price, pharmacy's retail price, or the manufacturer's list price. Adjusting heterogeneous prices can be problematic. In its report, ASPE states that some

countries' data are collected at the hospital level, while others' are collected only at a higher level such as the wholesale level (Office of the Assistant Secretary for Planning and Evaluation 2018).

- Difficulty in identifying the same product across countries. Manufacturers sometimes launch the same products in different countries using different commercial names, pharmaceutical formulations, dosages, and vial and package sizes (Young et al. 2017). Indeed, marketing nonidentical products may be a technique used by manufacturers to counteract the use of international reference pricing. Thus, international reference pricing may promote minor product differentiation (with no therapeutic advances) across markets. ASPE acknowledges that products available in the U.S. do not always align with products available in other countries.

Addressing high launch prices with binding arbitration

Launch prices for some drugs and biologics have increased rapidly in recent years, even after taking into account differences in the clinical effectiveness of the products. Howard and colleagues analyzed the launch prices of anticancer drugs from 1995 and 2013 and found that after controlling for inflation and differences in survival benefits, launch prices increased about 10 percent per year (about \$8,500 per year) (Howard et al. 2015). The authors did not find a statistically significant relationship between launch prices and survival benefits.

For costly new drugs that face limited competition, such as the first drug in a class or a product that offers added clinical benefit over existing treatments, manufacturers have significant market power to set prices and payers currently have very limited ability to influence those prices. Under Section 1847A of the Social Security Act, FFS Medicare lacks the authority to implement tools to arrive at drug payment rates that balance an appropriate reward for innovation with value and affordability for beneficiaries and taxpayers. Medicare's payment rate for a drug may have little relationship to a drug's clinical effectiveness compared with other available treatments. Under the Medicare Part B ASP + 6 percent payment system, FFS Medicare acts as a price taker, and a drug manufacturer with a new product with limited competition effectively sets its own Medicare payment rate.

Binding arbitration is an approach that could be considered to address high launch prices for products with limited competition. Arbitration is a process by which two parties agree to accept the decision of a neutral third party in a dispute, such as a dispute over the price of a drug. Arbitration was an element of the Commission's June 2017 recommendation to improve Medicare payment methods for Part B drugs. That recommendation called for the development of a voluntary alternative to the ASP payment system in which physicians and HOPDs could choose to enroll. Under that alternative program, which we refer to as the Drug Value Program (DVP), Medicare would contract with private vendors to negotiate prices for Part B drugs and would permit vendors to use tools such as a formulary to create negotiating leverage. Because leverage is particularly challenging for drugs with limited alternatives—such as the first product in a class or a product that provides a significant clinical improvement over existing treatments—the Commission recommended that the DVP include binding arbitration as a tool to help vendors and manufacturers arrive at an agreed-on payment rate for high-priced Part B drugs with little or no competition.

Background on arbitration

Arbitration is used to settle disputes in a wide range of areas including labor, communications, international taxes, and health care in certain circumstances. Its most familiar use is in Major League Baseball where binding arbitration serves as a vehicle to settle salary disputes between players and teams. Baseball arbitration uses an approach called “final-offer” arbitration, in which the arbitrator must pick one of the offers made by the disputants. This approach provides an incentive for parties to make reasonable offers since an unreasonable offer may increase the odds that the arbitrator will choose the other party's offer. Final-offer arbitration is credited with encouraging negotiated settlements between players and owners because only a small share of players eligible for arbitration have their salaries decided through an arbitration hearing while the vast majority reach a settlement outside of arbitration.³⁰

States are using a number of different approaches to address out-of-network surprise bills, including in some cases independent dispute resolution processes or arbitration.³¹ A recent analysis indicates that about 10 states include independent dispute resolution or arbitration systems as a part of their approach to settling disputes about payment rates and/or cost sharing when a patient receives a surprise out-of-network bill (Hoadley et al.

2019). The structure of these systems vary by state. New York and recently New Jersey use baseball arbitration. According to one study looking at the early experience with New York's program, the initial effect appears to be in the intended direction, with the study finding a lower frequency of out-of-network billing and lower payment rates for emergency department physicians providing services in network after implementation of the program (Cooper et al. 2018). Another way that state dispute resolution or arbitration programs vary is in whether participation in the dispute resolution system by insurers and providers is voluntary or mandatory. A study of some early state experiences with out-of-network dispute resolution systems found that voluntary systems (such as those in California and Texas) have not been as effective as mandatory systems because voluntary systems have received little use (Hall et al. 2016).

Major League Baseball and out-of-network bills provide examples of how arbitration has been used to establish prices in situations where one party would otherwise have little negotiating leverage. Since Medicare and other payers also lack leverage to affect the price for drugs with limited competition, arbitration could have promise to address prices for such products. Clearly, there are differences between Major League Baseball, out-of-network claims, and drug pricing that would be expected to translate into differences in how an arbitration system is designed for these different purposes. For example, arbitration for out-of-network claims tends to occur at the level of an individual patient's claim and there is the potential for there to be a relatively large number of claims with relatively small dollar amounts per claim. In contrast, the use of arbitration for determining the price of a drug could occur at the level of the Secretary, with arbitration focusing on only a small number of products. The rules, criteria, and processes for arbitration for drug pricing could be designed to take into account the specific considerations and implications of drug-pricing decisions.

Although use of arbitration for drug pricing is not common, Germany offers an example of one such approach. In Germany, if a drug is found to have added clinical benefit over existing treatments, health insurers and manufacturers are given six months to negotiate the price, and if negotiations fail, they move to arbitration. In some circumstances, products found to be without added benefit over existing treatments go through negotiations and arbitration (e.g., if there are not enough products to form a comparator group for reference pricing). The arbitration process lasts up to three months and the

arbitration board consists of three neutral members, including the chair, plus one representative of the insurers and one of the manufacturer. The arbitration board's decision is based on a majority vote, with the chair's vote being decisive if a majority is not reached. The manufacturer and health insurers each offer a price, and the arbitration board chooses a price in the range between the two offers.³² The arbitration price goes into effect the 13th month the product is on the market. The parties can appeal to a court, but appeals do not have suspensive effect (Wenzel and Paris 2018). A process also exists for the parties to request that the price be revisited, generally after at least a year. In some cases, manufacturers have chosen to withdraw their product after an arbitration decision, with one motivating factor being concern about the effect that a lower German price could have on prices in other countries that use Germany as a reference price (Robinson et al. 2019).³³ These negotiation and arbitration processes apply to outpatient drugs; however, the prices arrived at through these processes also serve as a ceiling on prices manufacturers can charge to hospitals for inpatients.

Rationale for arbitration beyond the DVP

Although the Commission has recommended the inclusion of binding arbitration within the DVP, there may be a role for binding arbitration beyond the DVP. The Commission's recommended DVP design would be voluntary for providers. If the DVP were implemented, it is possible that a significant portion of Part B drug spending would remain under the traditional ASP system, unaffected by the DVP. Thus, if the DVP obtained a lower price through binding arbitration, it would not affect Medicare's ASP payment rates.

Some Medicare Part A providers (such as inpatient hospitals) are paid a bundled rate for all care provided, including drugs, based on a patient's case-mix group. Although bundles give providers an incentive to negotiate lower prices and use services efficiently, providers may have little leverage to negotiate favorable prices when a drug lacks competition. In addition, sometimes a drug can be covered under Part A or Part B depending on where it is administered. To the extent that a drug covered by Part B goes through arbitration, it would seem reasonable that Part A providers that also furnish that drug to Medicare beneficiaries should benefit from the lower price resulting from arbitration.

This chapter focuses on binding arbitration's potential use in FFS Medicare. However, we note that the concept of binding arbitration was first raised by researchers with

respect to Part D, and its use could also be explored for Part D (Frank and Newhouse 2008).

How binding arbitration could operate outside the DVP

In this chapter, we explore a potential policy that would permit the Secretary to enter into binding arbitration with drug manufacturers for Part B drugs with limited competition under certain circumstances. If this type of binding arbitration were available, there would be a number of important structural features for such a system. In the following sections, we discuss various design elements that would be involved in setting up such a system and some of the policy choices that would have to be contemplated.

- **Type of arbitration.** Two common forms of arbitration are conventional and final-offer arbitration, which is often referred to as "baseball arbitration." Under conventional arbitration, the arbitrator can select any award amount, whereas under baseball arbitration, the arbitrator picks the award amount from the offers made. The Commission has focused on baseball arbitration because it provides an incentive for parties to make reasonable offers since the arbitrator must pick one of the two offers. These incentives would make the process less risky for both the Secretary and manufacturers.
- **Selection of arbitrators.** Having neutral arbitrators with sufficient subject matter expertise would be essential to the success of an arbitration process. The arbitrator could be a single individual or panel of individuals. Some have suggested that a neutral third party propose a slate of arbitrators, with each party having the ability to veto certain arbitrators (Frank and Newhouse 2008). For example, a nonpartisan government entity (e.g., the Government Accountability Office) could propose a slate of five arbitrators with specialized expertise and without conflicts of interest and permit each side to strike one arbitrator, leaving a panel of three. Another component essential to this process would be the development of standards for what constitutes a conflict of interest and processes for how conflicts would be identified and handled.
- **Who would enter into binding arbitration and what would trigger it?** The Congress could establish the criteria for when the Secretary could seek arbitration for a product. For example, the Secretary could

be granted authority to seek arbitration if total Medicare Part B program expenditures for a product or the product's cost per patient (or per unit of health outcome) is estimated or projected to exceed specified dollar thresholds. These thresholds could be set at levels that would focus arbitration on those products for which it would have the most benefit by identifying products that have high total spending, a high cost per patient, or both. Because a small number of Part B drugs account for a large share of Part B drug spending, it would be possible to set criteria that could have a meaningful impact while involving a limited number of products. A second component of the criteria could be that the product faces limited competition (e.g., because few products with similar health effects exist). When these criteria are met, the Secretary could decide whether to request the manufacturer to enter arbitration.

- **Manufacturer obligation.** To give manufacturers a strong incentive to agree to participate in arbitration when requested by the Secretary, Medicare payment for a manufacturer's product could be conditioned on that manufacturer's participation in binding arbitration. Thus, if a manufacturer chose not to participate in arbitration for a particular product, that choice by the manufacturer would result in the Medicare program no longer paying for the product. While it is possible that a manufacturer could decline to participate in arbitration, the large size of the Medicare market and the high cost of the products that would meet the criteria for arbitration would be a strong disincentive for a manufacturer to decline Medicare payment for its product.
- **Timing of arbitration.** There may be benefits to granting the Secretary flexibility on the time period when the Secretary can first request arbitration for a product, either at a product's launch or later in a product's time on the market. For some products, it may be clear at launch that the product meets the criteria for arbitration, and, in that case, the arbitration process could begin quickly once the product has launched and the Secretary requests arbitration. If arbitration occurs at a product's launch, it would be important that access to the product not be delayed while the arbitration process is underway. The product could be paid its standard ASP-based payment amount while the process is underway. Once an arbitration price has been decided, several options exist for the effective date of that price. It could be effective on a

going forward basis immediately or (like Germany) after a specified time period, or it could be applied retroactively with the difference between the initial price and the arbitration price recouped. There could be situations in which a product at its launch does not appear to meet the cost criteria for arbitration, but later—after the product has been on the market—data indicate that it meets the criteria. Permitting the Secretary to request arbitration later in a product's market experience would ensure that arbitration is an available tool if a product's market size, usage, or pricing turns out to be different from initially expected.

- **Offer price.** If the Secretary and the manufacturer enter arbitration for a product, the Secretary and the manufacturer would submit offer prices to the arbitrator(s) who would choose one of those prices. How the Secretary would determine an appropriate offer price would be a key issue. This determination of an offer price could be left entirely to the Secretary or the Congress could specify factors the Secretary should consider or parameters the Secretary should use in developing an offer price. Another approach would be for the Congress to specify some bounds on the offer prices for both the Secretary and manufacturer—for example, by specifying a range in which an offer price should fall relative to various pricing benchmarks (e.g., ASP, prices in other countries, measures of price per unit of health outcome, and/or rate-of-return on investment).

The process by which the Secretary arrives at an offer price also could take several forms. The Secretary could seek input from neutral outside organizations with expertise in value-based pricing. Another approach would be for the Secretary to create the Department's own model of a value-based price. If the Department created its own model, it could use that same approach consistently across drugs for which it sought arbitration. The Secretary could also use a combination of approaches, seeking estimates from neutral outside experts as well as creating its own model. If manufacturers were required to submit a dossier on their products' comparative clinical effectiveness (as done in Germany) and cost (as done in some other countries like Australia), the Secretary could also consider such information in formulating an offer price. Since in the future high-priced breakthrough drugs may be developed for large populations, it would be important that

the Secretary be permitted to consider Medicare program affordability as one of many factors he or she considers in developing an offer price.

- **Pre-arbitration discussions.** The binding arbitration system described here does not necessitate direct negotiations between the Secretary and the manufacturer on price. The decision on price could be left entirely to the arbitrator. Without direct negotiations between parties, there could still be a role for informational meetings between the Secretary and a manufacturer before a product's launch. Such meetings could permit manufacturers to provide information on their new products and permit the manufacturer to ask questions about what the Secretary considers when deciding to pursue arbitration. The FDA–CMS parallel approval review program for devices is one example of a process for prelaunch consultations between CMS and manufacturers.³⁴

In other areas where binding arbitration is used, such as labor disputes, one benefit of binding arbitration is that it can encourage negotiated settlements and the avoidance of arbitration hearings. In applying binding arbitration to Part B drugs, there would be the question of whether (similar to Germany) the Secretary would be permitted to engage in pre-arbitration negotiations with the manufacturer to potentially reach agreement on a lower price for Medicare patients without entering arbitration. Because binding arbitration would be a fallback if negotiations fail, the Secretary would potentially have more leverage in negotiating with manufacturers under these circumstances than would otherwise be the case in the absence of arbitration. However, direct negotiation of prices between the Secretary and manufacturers is a controversial issue. An arbitration process could be feasible with or without permitting the Secretary to engage in pre-arbitration negotiations.
- **Length of arbitration process.** The length of time it takes to complete the arbitration process would depend on how it is structured. Certain design features—such as how the deadlines are spaced for parties to submit information and specific requirements about the content and amount of materials that parties can submit—affect the time involved. The arbitration system can be designed to be as expedient as judged appropriate. For example, in Germany, if price negotiations between insurers and a

drug manufacturer fail, an arbitration board makes its own determination on price within three months.

- **Criteria used by arbitrator.** An important feature of designing an arbitration system would be the criteria the arbitrator would use in making its decision between the parties' offers. Some potential criteria could include:
 - clinical benefit compared with existing treatments (which would provide an incentive for manufacturers to focus on the development of drugs that offer substantial clinical benefits over drugs with smaller added benefits)
 - prices of existing treatments
 - whether the drug addresses specific areas of need (e.g., new antibiotics)
 - whether the drug focuses on a rare condition and does not have other broader uses
 - cost of manufacturing the product
 - amount spent on the product's research and development by the manufacturer and other entities (e.g., government-sponsored research)
 - affordability for the Medicare program and beneficiaries
- **Operationalizing the award price.** Once the arbitrator decides on a price, the Medicare program would need to use that price as a basis for paying for Part B drugs. The arbitration price could be operationalized as an adjustment to the Medicare Part B drug payment rates or as a rebate paid by the manufacturer.
- **Approach 1: Part B payment rate based on the arbitration price and a manufacturer requirement.** The arbitration price could become the Medicare payment rate for a Part B drug. To ensure providers can acquire the product, manufacturers could be required as a condition of Medicare payment that they sell the product to providers for Medicare patients at a price no higher than the arbitration price. With this manufacturer pricing requirement, the 6 percent add-on to the Part B payment rate for the product could potentially be eliminated. To operationalize the manufacturer requirement, a back-end reconciliation process would be needed between providers and wholesalers, distributors,

or manufacturers to ensure that, for the volume of product furnished to Medicare patients, the price would be no higher than the arbitration price.

With this approach, the manufacturer requirement could also be extended to providers furnishing drugs under Part A. Although Part A providers are paid for drugs through larger payment bundles that create incentives for providers to be cost conscious and negotiate for lower prices, Part A providers may have little leverage with manufacturers when a product has limited alternatives. Making the arbitration price a ceiling on the price at which a manufacturer can sell drugs to these providers for their Medicare patients has the potential to assist Part A providers with their costs for expensive drugs with limited competition.

- **Approach 2: Manufacturer rebate.** Medicare could continue paying for Part B drugs under its standard approach of ASP + 6 percent, but manufacturers could be required to pay Medicare a rebate to achieve the price arrived at through arbitration. This approach would be relatively straightforward to implement and would accrue savings to Medicare Part B. However, this approach would not lower the drug acquisition prices paid by providers so it would not have the potential to assist Part A providers with drug costs.

Both approaches would have the potential to reduce beneficiary cost sharing. The first approach would automatically reduce cost sharing by lowering the Medicare payment amount on which the 20 percent cost sharing is calculated. Although not as automatic, the second approach—a manufacturer rebate—could be structured to lower beneficiary cost sharing. With the rebate approach, Medicare could reduce the cost sharing up front based on the arbitration price, with Medicare increasing its payment to the provider to make up the difference. The Medicare program would then receive rebates from the manufacturer afterward and keep the full amount of the rebate. The net result would be that the beneficiary would realize roughly 20 percent of the rebate through lower cost sharing and the program would realize 80 percent.

- **Process for revisiting arbitration price and addressing new products.** The arbitration process could include a process for the parties to request a

reconsideration at a later date. It may be in the interest of each party to have this option. For example, if new research comes out that suggests the clinical effectiveness of a drug is substantially more or less than initially thought, it could benefit one of the parties to request a new arbitration process. Another important issue would be what happens if a similar product to the one that underwent arbitration subsequently launches. Different approaches to that situation could be considered, such as applying the arbitration price to the new product or letting the products revert to the standard ASP payment system, with the potential to reenter arbitration if the pricing under the standard system rises.

- **Other design issues.** Other design features that would need to be considered include whether to allow the arbitrator to contract with a neutral third party to supplement or evaluate the information contained in each disputant's final offers (e.g., an independent fact finder) and what information from the arbitration process besides the arbitration price would be made public.

Implications and stakeholder concerns with binding arbitration

Binding arbitration is one of the few potential tools available to affect the price of drugs with limited competition. The binding arbitration process has the potential to incorporate value, affordability, and an appropriate reward for innovation into the determination of Medicare's payment for Part B drugs. Because the decision on Medicare's payment would ultimately be in the hands of a neutral arbitrator, it may help insulate the process from stakeholder pressure to some degree. Nonetheless, the Secretary would still likely face stakeholder pressure over when to invoke arbitration and at what level to set Medicare's offer price.

Whether arbitration is an effective process for arriving at a value-based payment would depend on how the arbitration process is designed. The Congress would need to specify a number of design elements for the binding arbitration process (as discussed above). Success of a binding arbitration process would also hinge on the ability to involve neutral arbitrators. Critics of binding arbitration argue that it would be challenging to find arbitrators with sufficient subject matter expertise who are without conflicts of interest. Putting the selection of arbitrators in the hands of a nonpartisan government agency could

help navigate that issue. With binding arbitration, there may also be concerns about whether a manufacturer might decline to participate in binding arbitration—and thereby decline to have its product covered by Medicare—and the implications of such a decision for beneficiary access. However, the large size of the Medicare market and the high cost of the products that would be eligible for arbitration would create a strong disincentive for a manufacturer to decline to have its product paid for by Medicare. As with other policies that would reduce drug prices, some stakeholders assert that arbitration would reduce the incentives for innovation. In contrast, if the arbitration process focuses on clinical effectiveness and the magnitude of clinical benefits over existing products, the process could improve the incentives for research and development aimed at products likely to have substantial added benefits over those with smaller added benefits. Furthermore, the establishment of criteria to help guide the arbitrator’s decision could include factors (such as market size, clinical benefit, unmet need, special populations, rate of return on investment) that are important for innovation.

Conclusion

Reference pricing and binding arbitration are two potential tools that could be considered to improve price competition and incorporate value into payment for Part B drugs. Reference pricing focuses on products with similar health effects, and binding arbitration focuses on expensive products with limited competition. Each approach is a distinct policy and could be adopted on its own. However, packaging both strategies together, along with the Commission’s June 2017 recommended policies, could provide added benefits because the various policies would complement each other by addressing different factors driving Medicare Part B drug spending growth. Medicare would need additional statutory authority to implement reference pricing and binding arbitration; the legislative provisions would influence each strategy’s effectiveness to improve price competition and value for Part B drugs. Finally, both reference pricing and binding arbitration could also be applied to pay for Part D drugs, although how each could be applied would differ from its use in Part B. ■

Endnotes

- 1 Spending on supplier-furnished drugs decreased by 11 percent in 2017 because of a statutory change in Medicare's payment formula for home infusion drugs and the entry of generics for a few high-expenditure products. Beginning January 2017, Medicare pays for Part B–covered home infusion drugs at a rate of ASP + 6 percent. Before that time, Medicare paid for these drugs based on 95 percent of the average wholesale price.
- 2 This analysis of the factors driving spending growth between 2009 and 2019 excludes any Part B drugs that were packaged into payment for other services, regardless of setting and year. This means that drugs that were packaged under the outpatient prospective payment system are excluded from the analysis, even if they were separately paid in the physician's office. We focused our analysis on this subset of drugs to ensure that shifts in a drug's status as separately paid or packaged or shifts in site of service did not skew our results. We also exclude vaccines to ensure that the analysis is not skewed by a substantial increase in the use and price of a new pneumococcal vaccine. For the period from 2009 to 2016, the average annual growth in spending for nonvaccine separately payable drugs was somewhat higher than for all Part B drugs (10.7 percent and 9.5 percent, respectively). Under the hospital outpatient prospective payment system, low-cost drugs (e.g., drugs with a cost per day of less than \$125 in 2019) and certain types of drugs regardless of cost (e.g., drugs that function as supplies for certain tests or procedures) are packaged into the payment for other services (unless they are new products and have received temporary pass-through status). Medicare Part B covers drugs that are administered in HOPDs when they are directly related and integral to a procedure or treatment and are required to be provided to a patient in order for a hospital to perform the procedure or treatment during a hospital outpatient encounter.
- 3 Because some beneficiaries begin treatment midyear and treatment carries into the following year, average spending per user in any given year understates the cost of a full year of treatment with the product.
- 4 Manufacturers calculate ASP based on sales to all purchasers, excluding nominal sales and prices that are exempt from the determination of the Medicaid best price (e.g., sales or discounts to other federal programs, 340B–covered entities, state pharmaceutical assistance programs, and Medicare Part D plans, as well as manufacturer coupons to consumers meeting certain criteria). Bona fide service fees are not considered price concessions for the purposes of ASP (for example, fees paid by the manufacturer to entities such as wholesalers or group purchasing organizations that are fair market value, not passed on in whole or in part to customers of the entity and are for services the manufacturer would otherwise perform in the absence of the service arrangement).
- 5 Manufacturers are required to report ASP data for a calendar quarter within 30 days after the close of that quarter. CMS then takes the data submitted by manufacturers and uses them to calculate the ASP + 6 percent payment rates for the next calendar quarter. For example, ASP data for the fourth quarter of 2018 were used to set the ASP + 6 percent payment rates for the second quarter of 2019. Manufacturers were required to report ASP data for the fourth quarter of 2018 by January 30, 2019. CMS then had two months to calculate, publish, and operationalize the new payment rates so they would go into effect at the start of the next calendar quarter, April 1, 2019.
- 6 Between 2016 and 2018, the Secretary assigned to a single billing code all biosimilar products that rely on a common originator product's biologics license under the Food and Drug Administration's approval process. Under this policy, all biosimilars associated with a particular originator product were paid under a single billing code and received a payment equal to 100 percent of the weighted average ASPs for the biosimilar products plus a constant add-on equal to 6 percent of the reference product's ASP. In 2018, the Secretary changed this policy and began assigning each biosimilar to its own billing code and paying each product based on its own ASP + 6 percent of the originator biologic's ASP.
- 7 The IMS Health Incorporated data were available by channel of purchaser. We examined the clinic channel, which included physician offices, hospital outpatient departments, dialysis clinics, nonhospital surgical centers, and public health service clinics. The IMS data for the clinic channel included discounted sales to 340B entities. To avoid reflecting 340B prices in our estimates, we did not use data on the average invoice price. Instead, we focused on invoice prices at the 75th percentile (i.e., the 75th percentile reflects the price at which 75 percent of the volume of a drug is sold at or below that price). The prices in the IMS data reflect all on-invoice discounts and rebates but not off-invoice rebates. As a result, in some cases the IMS data overstate the actual end price paid by the purchaser.
- 8 Like other Medicare services, Part B–covered drugs are subject to the budget sequester effective April 1, 2013, through 2027. The sequester reduces Medicare program payments by 2 percent but does not affect the beneficiary cost-sharing amount.
- 9 After a generic is launched (and assigned to the same billing code as its brand-name product), its lower price is averaged with the higher price of the brand product, which results in the ASP-based payment rate of the consolidated billing code falling over time as brand and generic products compete based on price.

- 10 For example: epoetin and darbepoetin (erythropoiesis-stimulating agents that treat anemia), aflibercept and ranibizumab (anti-vascular endothelial growth factors that treat eye conditions), and infliximab and rituximab (targeted immune modulators that treat immunologic conditions).
- 11 Although Granix is not a biosimilar in the U.S. (because it was approved under the standard FDA approval process for new biologics), we include it here because it was approved as a biosimilar to Neupogen in Europe and it functions as a competitor to Neupogen and Zarxio in the U.S. market.
- 12 Countries that CMS is considering including in the IPI are Austria, Belgium, Canada, Czech Republic, Denmark, Finland, France, Germany, Greece, Ireland, Italy, Japan, Netherlands, and the U.K.
- 13 In remarks at an October 26, 2018, event hosted by the University of Southern California–Brookings Schaeffer Initiative for Health Policy, the Secretary of the Department of Health and Human Services stated that the IPI model would not include formularies (https://www.brookings.edu/wp-content/uploads/2018/10/es_20181026_hhs_medicare_transcript.pdf).
- 14 The Group of Seven is an informal grouping of seven of the world’s advanced economies consisting of Canada, France, Germany, Japan, Italy, the U.K., and the U.S.
- 15 Alternatively, all drugs within a reference pricing group could have the same payment (e.g., median of prices across products), with beneficiaries’ cost sharing based on 20 percent of the reference price. In that case, the provider would get paid the same amount regardless of the product chosen and would have an incentive to choose the lower priced product.
- 16 In 2015, total Part B spending for these eight groups totaled \$9.5 billion.
- 17 In its interpretive manuals, CMS explained that Medicare’s authority to apply LCA policies was based on the general provision requiring the program to pay the expenses of items and services that are reasonable and necessary (Medicare Payment Advisory Commission 2010).
- 18 The prostate cancer drugs were triptorelin pamoate, goserelin acetate implant, and leuprolide acetate suspension.
- 19 See Social Security Act Section 1833(t)(2)(E).
- 20 Reference pricing was applied to 76 therapeutic classes composed of multiple generic and therapeutically similar brand-name drugs.
- 21 Before the implementation of reference pricing, Reta Trust members paid an average of 31 percent more in copayments per prescription compared with the control population (Robinson et al. 2017). After reference pricing was implemented, the use of the lowest priced reference drugs was 11.3 percent higher among Reta Trust members than among the control group (Robinson et al. 2017).
- 22 According to researchers, in 2011, the following 16 countries used reference pricing to pay for drugs: Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Finland, France, Germany, Hungary, Italy, Latvia, the Netherlands, Poland, Portugal, Spain, and Turkey. Austria, Norway, Sweden, and the U.K. did not use reference pricing (Dylst et al. 2012).
- 23 International reference pricing is considered in the following 29 countries: Austria, Belgium, Bulgaria, Croatia, Cyprus, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, the Netherlands, Norway, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, and Switzerland (Rémuzat et al. 2015).
- 24 For new products that DHA does not yet cover, the manufacturer is required to submit a clinical evaluation (that provides the best available evidence to support the comparative effectiveness and safety of the product) and an economic evaluation (cost-effectiveness analysis); for new forms of already covered products, an economic evaluation is usually not required.
- 25 All drugs approved by the European Medicines Agency are immediately available after launch for clinicians to prescribe (Robinson et al. 2019).
- 26 A new drug treating multiple indications may have multiple comparators.
- 27 There must be three therapeutically equivalent drugs to constitute a class for reference pricing (Robinson et al. 2019).
- 28 A mechanism for exceptions for patients who need higher priced products must be carefully designed. Exceptions that are too limited could lead to higher copayments for the most effective drug and to physicians prescribing less effective drugs. Too generous exceptions could reduce the savings by not shifting drug use toward less costly products (Acosta et al. 2014).
- 29 For example, Medigap policies F and G cover 100 percent of the costs known as Medicare Part B excess charges, the difference between what a doctor or provider charges and the amount Medicare will pay.

- 30 In Major League Baseball, out of 2,994 filings for arbitration between 1990 and 2016, only 246 (8 percent) were decided by an arbitration hearing (http://www.mlbplayers.com/ViewArticle.dbml?DB_OEM_ID=34000&ATCLID=211445796).
- 31 Some states have used dispute resolution or arbitration to address surprise billing situations. Other states have taken different approaches such as specifying the payment rate for out-of-network services based on a benchmark, prohibiting providers from balance billing, or requiring insurers to hold the patient harmless by paying a larger share of the payment to the provider (Hoadley et al. 2019). Research comparing the relative effects of the various approaches is limited.
- 32 According Ludwig and Dintsios, for the 16 products that completed arbitration through 2015, the arbitration price was closer to the insurers' offer price for 12 products and closer to the manufacturer's offer price for 4 products (Ludwig and Dintsios 2016). On average for the 16 products, the arbitration price was 20 percent below the midpoint between the insurers' and manufacturer's offer price (Wenzel and Paris 2018).
- 33 Manufacturers have the option to halt offering their product in the German market at any point, such as when the government has made a determination of the product's comparative effectiveness, during the negotiations process between insurers and the manufacturer, or in response to an arbitration decision. Between 2011 and 2017, of the 148 products that underwent a comparative effectiveness assessment, 29 products were withdrawn from the German market. Twelve products were withdrawn immediately without going through the negotiations and arbitration process and 16 were withdrawn after a pricing decision generally by the arbitration board. One product was withdrawn due to manufacturer bankruptcy (Robinson et al. 2019).
- 34 Although for a different purpose, the FDA–CMS parallel review program offers device manufacturers a voluntary opportunity to engage with FDA, CMS, and others about what type of evidence might be important to these agencies as they make decisions about product approval and coverage, which permits manufacturers to consider that feedback as they are designing their clinical trials (Food and Drug Administration and Centers for Medicare & Medicaid Services 2016).

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