CHAPTER 1

Addressing high prices of drugs covered under Medicare Part B

RECOMMENDATIONS

1-1 The Congress should require the Secretary to cap the Medicare payment rate for Part B drugs and biologics that are approved under the accelerated approval program (with limited circumstances for the Secretary to waive the payment cap) if:

- postmarketing confirmatory trials for the product are not completed within the deadline established by the manufacturer and the Food and Drug Administration,
- the product's clinical benefit is not confirmed in postmarketing confirmatory trials, or
- the product is covered under a "coverage with evidence development" policy.

In addition, the Congress should give the Secretary the authority to cap the Medicare payment rate of Part B drugs and biologics that are approved under the accelerated approval program if their price is excessive relative to the upper-bound estimates of value.

COMMISSIONER VOTES: YES 17 • NO 0 • NOT VOTING 0 • ABSENT 0

1-2 The Congress should give the Secretary the authority to establish a single average sales price–based payment rate for drugs and biologics with similar health effects.

COMMISSIONER VOTES: YES 17 • NO 0 • NOT VOTING 0 • ABSENT 0

1-3 The Congress should require the Secretary to:

- reduce add-on payments for costly Part B drugs and biologics paid based on average sales price in order to minimize the relationship between average sales price and add-on payments, and
- eliminate add-on payments for Part B drugs and biologics paid based on wholesale acquisition cost.

COMMISSIONER VOTES: YES 17 • NO 0 • NOT VOTING 0 • ABSENT 0

CHAPTER 1

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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. It also covers certain drugs and biologics furnished by suppliers. In 2021, fee-for-service Medicare and its beneficiaries paid about $43 billion for Part B–covered drugs and biologics. From 2009 to 2021, Medicare Part B spending on drugs and biologics grew at an average rate of about 9 percent per year. (Hereafter, we use the term *drugs* to refer to drugs and biologics unless otherwise noted.)

The largest factor contributing to growth in Part B drug spending has been the rise in the average price paid by Medicare, driven by the introduction of new, higher-priced drugs; increased prices for existing products; and shifts in the mix of drugs furnished to beneficiaries. Manufacturers set prices based on what they believe the U.S. health care market will bear, and they have established increasingly high launch prices for many new treatments, whether or not evidence exists that the product is comparatively more effective than existing standards of care. Likewise, prices have grown rapidly for some older drugs, even those with therapeutic alternatives, despite a lack of evidence of increased effectiveness.

In this chapter

While the recently enacted Inflation Reduction Act gave Medicare certain tools to influence the price the program and beneficiaries (through cost sharing) pay for certain Part B–covered drugs, the program continues to lack the authority to pay for Part B drugs in a way that promotes price competition among Part B drugs with therapeutic alternatives or that balances a drug's net clinical benefit with an appropriate reward for innovation and affordability for beneficiaries and taxpayers. In addition, concern remains that Medicare's payment formula for Part B drugs (specified in Section 1847A of the Social Security Act)—a 6 percent add-on to the drug's average sales price (ASP)—can create financial incentives that favor prescribing higher-priced drugs in some circumstances.

In this chapter, the Commission makes recommendations to address high launch prices for certain accelerated approval drugs that have limited clinical evidence, little or no price competition among products with therapeutic alternatives, and misaligned financial incentives associated with the percentage add-on to Medicare Part B's payment rate.

Addressing high launch prices for drugs with limited clinical evidence by capping the payment of select Part B "accelerated approval" drugs and biologics

Drugs come to the market faster under the accelerated approval pathway than under traditional approval because the Food and Drug Administration (FDA) approves them based on intermediate clinical or surrogate endpoints that are *reasonably likely to predict* a clinical benefit, but before the clinical benefit has been verified. Consequently, some accelerated approval drugs are approved before evidence exists of their effect on the Medicare population, and some manufacturers establish high pricing relative to their accelerated approval drug's expected clinical benefit. Thus, Medicare's spending for these drugs is relatively high, affecting beneficiaries and taxpayers. In addition, some manufacturers' postmarketing studies that are conducted to confirm an accelerated approval drug's clinical benefit are delayed.

The accelerated approval pathway is intended to expedite the approval of potentially promising products for cancer and other complex or rare conditions; incentives for drug development in these areas are important. At the same time, tools are needed to ensure that the Medicare program is not overpaying for products approved on an accelerated basis if a product's clinical benefit is not confirmed. Such tools are particularly relevant for products approved on this pathway since some may not have any competitors. Also, manufacturers need an incentive to complete postmarketing confirmatory trials on a timely basis so that information about a product's effects on health

outcomes is available as soon as possible to providers who may prescribe it and beneficiaries who may receive it. Section 1847A of the Social Security Act, which specifies the payment methodology for Part B–covered drugs, does not differentiate Medicare payment for a drug approved under the FDA's traditional process versus one on an accelerated approval pathway. A targeted approach to capping Medicare's payment of select accelerated approval drugs would balance these trade-offs. Setting the payment cap based on net clinical benefit would reward companies with very promising drug products, acknowledging the advances they provide over the status quo.

To maintain financial rewards for innovation while improving access and affordability of care for beneficiaries and taxpayers and spurring manufacturers to complete their required confirmatory trials on time, the Commission recommends that the Congress require the Secretary of Health and Human Services to cap the Medicare payment rate of Part B drugs and biologics (with limited circumstances for the Secretary to waive the payment cap) that are approved under the accelerated approval program if:

- postmarketing confirmatory trials for the product are not completed within the deadline established by the manufacturer and the FDA,
- the product's clinical benefit is not confirmed in postmarketing confirmatory trials, or
- the product is covered under a "coverage with evidence development" policy.

In addition, the Congress should give the Secretary the authority to cap the Medicare payment rate of Part B drugs and biologics that are approved under the accelerated approval program if their price is excessive relative to the upper-bound estimates of value.

There are two key implementation issues for Medicare to consider in setting a cap on a new drug's Part B payment rate: how to set the cap on a drug's payment rate and how to operationalize the cap.

- The cap could be set based on a drug's net clinical benefit and cost compared with the standard of care. Such an approach would take into account a new drug's potential effect on beneficiaries' outcomes and costs.
- The payment cap could be put into effect using a rebate under which manufacturers would reimburse Medicare for the difference between the Medicare payment amount and the cap based on claims utilization for the accelerated approval diagnosis. The rebate could also be structured

to permit the beneficiary to share in the rebate through a reduced cost-sharing percentage. As of 2023, CMS is using a similar rebate approach for Part B drugs to implement the ASP inflation rebate established by the Inflation Reduction Act of 2022.

Spurring price competition by establishing a single ASP-based payment for Part B drugs and biologics with similar health effects

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. By contrast, products that are assigned to their own billing code and paid according to their ASP—single-source drugs, 505(b)(2) drugs, originator biologics, and biosimilars—do not face the same incentives for price competition.

To promote price competition among drugs with similar health effects, the Commission recommends that the Congress give the Secretary the authority to establish a single ASP-based payment rate for groups of drugs and biologics with similar health effects. Such a policy is consistent with the Commission's long-held position that Medicare should pay similar rates for similar care.

To implement this policy, the Secretary could develop reference groups of products that:

- have similar FDA-approved indications or off-label use according to Medicare claims data or have medically accepted (compendia-listed) off-label use;
- work in a similar way (e.g., same drug classification, mechanism of action); and
- are listed similarly by clinical guidelines (e.g., classification of products, recommended vs. not recommended).

The Secretary also could first focus on applying reference pricing to those groups for which all of a given product's indications could be included in the group. The Secretary could begin with those reference groups for which implementation would be the most straightforward: (1) biosimilars and originator biologics; (2) 505(b)(2) drugs and related brand-name and generic drugs; and (3) drugs for which reference pricing has been implemented or considered previously (including erythropoietin-stimulating agents and viscosupplements for the treatment of osteoarthritis). In most instances, the Secretary could set the reference price based on the volume-weighted ASP of drugs assigned to the reference group.

Improving financial incentives by modifying add-on payments for Part B drugs and biologics

Under Section 1847A of the Social Security Act, Medicare pays providers for most Part B drugs at a rate of the ASP plus 6 percent (ASP + 6 percent). In addition, Medicare makes a separate payment for drug administration services under the physician fee schedule or outpatient prospective payment system. Like all Medicare services, the Medicare program's payment for Part B drugs (but not beneficiary cost sharing) is subject to the 2 percent sequester through March 2032. When the sequester is in effect, the statutory payment rate of ASP + 6 percent translates into a net payment from the perspective of the provider of ASP + 4.3 percent (with the beneficiary paying 20 percent of ASP + 6 percent and the Medicare program paying 80 percent of ASP + 3.9 percent (i.e., ASP + 6 percent reduced by the 2 percent sequester)).

While clinical factors play a central role in prescribing decisions, at the margins, financial considerations can also play a role in providers' choice of drugs. Medicare's percentage add-on to ASP may create incentives for use of higher-priced drugs when less-expensive therapeutic alternatives are available. Since a percentage add-on generates more revenue for the provider when applied to a higher-priced product than a lower-priced product, selection of the higher-priced product could generate more profit for the provider, depending on their acquisition costs for the two products. The percentage add-on may also affect a provider's decision to initiate or continue drug treatment in some circumstances.

To improve financial incentives under the ASP payment system, the Commission recommends an approach that would minimize the relationship between price (ASP) and add-on payments by reducing add-on payments for costly drugs. The Commission developed a general framework to illustrate how such an approach could be operationalized. In developing this approach, we sought to:

- reduce or eliminate the percentage add-on for moderate- and high-priced drugs to minimize the relationship between price (ASP) and add-on payments,
- retain a portion of the percentage add-on for all but the most expensive drugs to accommodate price variation or other factors that might lead to some purchasers acquiring drugs at a price greater than ASP, and
- avoid applying a flat fee for low-cost drugs, which would constitute a substantial increase in payment rates relative to the price of the drug and potentially create incentives for overuse.

Our illustrative approach would maintain the current ASP add-on for lower-priced drugs, reduce the percentage add-on and add a fixed fee for mid-priced drugs, and place a fixed-dollar cap on the add-on for the highest-priced drugs. Overall, this approach would improve financial incentives by reducing the difference in add-on payments between differently priced drugs, with the largest reduction occurring among the highest-priced products.

In addition, the Commission recommends eliminating add-on payments for drugs lacking ASP data that are paid based on wholesale acquisition cost (WAC). Because WAC is generally a higher price than ASP and does not reflect discounts, eliminating the WAC add-on would reduce excess payments and improve financial incentives. ■

Background

Medicare Part B covers drugs and biologics that are administered by infusion or injection in physician offices and hospital outpatient departments. It also covers certain drugs and biologics furnished by suppliers. In 2021, traditional fee-for-service (FFS) Medicare and its beneficiaries paid about $43 billion for Part B–covered drugs and biologics. From 2009 to 2019, Medicare Part B spending on drugs and biologics grew at an average rate of about 9 percent per year. Between 2019 and 2021, spending growth slowed to 5 percent per year on average, but this slower growth reflected the decline in FFS enrollment over the period. Controlling for the number of FFS beneficiaries, Part B spending on drugs and biologics between 2019 and 2021 grew nearly 9 percent per year on average. (Hereafter, we use the term *drugs* to refer to drugs and biologics unless otherwise noted.)

Prescription medicines that Part B covers play a crucial role in managing or treating many conditions, including cancer, rheumatoid arthritis, macular degeneration, and others. Important pharmacologic breakthroughs—such as immunotherapy for melanoma, second-generation androgen receptor antagonists for prostate cancer, and new drugs for myeloma—have contributed to patients' increased life expectancy (Schnog et al. 2021). Some products—such as vaccines for COVID-19 and treatments for age-related macular degeneration—are transformative and represent large advancements in the standard of care and health outcomes (Finger et al. 2020). At the same time, many new drugs represent only modest improvements over existing treatments or have efficacy similar to products already on the market. For example, six studies that reviewed newly approved cancer drugs over various time periods found that, among the group of new products included in each study, the median or mean gain in overall survival was roughly two to four months (Schnog et al. 2021). In addition, manufacturers sometimes develop new products that are modifications of existing products (e.g., different formulations or routes of administration, modifications of delivery devices like inhalers or injector pens) as ways to potentially improve products' utility, extend patents or marketing exclusivity, or increase product revenues (Berger et al. 2016, Feldman 2018, Sumarsono et al. 2020).

An important driver of Part B drug spending is the price Medicare pays for drugs. The largest factor contributing to growth in Part B drug spending has been the rise in the average price paid by Medicare, driven by the introduction of new, higher-priced drugs; increased prices for existing products; and shifts in the mix of drugs furnished to beneficiaries. Manufacturers set prices based on what they believe the U.S. health care market will bear, and they have established increasingly high launch prices for many new treatments, whether or not evidence exists that the product is comparatively more effective than existing standards of care. Likewise, prices have grown rapidly for some older drugs, even those with therapeutic alternatives, despite a lack of evidence of increased effectiveness. Cost sharing for high-priced products can deter appropriate uptake, and Medicare program spending on high-priced products can crowd out valuable alternative uses of taxpayer resources.

Research suggests that drug launch prices have been increasing without commensurate gains in efficacy. For example, Howard and colleagues analyzed the prices of new anticancer drugs that were launched from 1995 to 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). However, the authors did not find a statistically significant relationship between launch prices and survival benefits. Similarly, a study by Vokinger and colleagues of 65 cancer drugs found no significant relationship between a drug's price and the product's level of clinical benefits (as measured by the American Society for Clinical Oncology's value framework scores) in the U.S. and in several European countries (England, Switzerland, and Germany) (Vokinger et al. 2020).

Prices also have grown rapidly for some older products, despite a lack of evidence of increased efficacy. In a report from the Institute for Clinical and Economic Review (ICER), researchers determined that, among the top drugs with price increases in 2020 that contributed to the largest increase in U.S. spending (including all prescription drugs, not just Part B drugs), 9 of 12 drugs lacked adequate new evidence to demonstrate a substantial clinical benefit that was not yet previously known.[1] The 2020 price increases of these products, even after rebates and other price concessions, resulted in an additional $1.7 billion in spending (Rind et al. 2022).

Drug prices in the U.S. are substantially higher than in other countries. The Assistant Secretary for Planning and Evaluation found that Medicare Part B's payment rates (106 percent of average sales price (ASP), or ASP + 6 percent) in 2018 were, on average, about double the average prices in 19 high-income countries included in the Organisation of Economic Co-operation and Development (Department of Health and Human Services 2020). Similarly, a study by Hwang and colleagues compared the ASP for 67 Part B drugs with prices from 4 other high-income countries (Japan, Germany, Switzerland, and the U.K.). Median prices in the comparator countries were roughly 45 percent to 60 percent lower than ASP (Hwang et al. 2019).

Higher prices in the U.S. are the result of both higher launch prices and higher price inflation once products are on the market. According to research by Vokinger and colleagues on 65 new drugs approved between 2009 and 2019 to treat solid-state tumors and hematologic cancers, launch prices were substantially higher in the U.S. than in England, Germany, and Switzerland (Vokinger et al. 2021). Among the group of cancer drugs included in the study, the U.S. median monthly treatment costs at launch, adjusting for currency and inflation, were 45 percent higher than in Germany, 57 percent higher than in Switzerland, and 63 percent higher than in England. After launch of these products, prices tended to increase faster than inflation for most products (74 percent) in the U.S., but not in England, Germany, or Switzerland (Vokinger et al. 2021).

Historically, Medicare has had only an indirect influence on how Part B–covered drugs are priced. Under the Part B payment system based on ASP, the program is a price taker. The recently enacted Inflation Reduction Act of 2022 gives Medicare certain tools to influence the price that the program and beneficiaries pay for certain Part B–covered drugs; however, some challenges remain. Additional policies that would set caps on payment for new drugs with uncertain clinical evidence and promote price competition among products with similar health effects would provide Medicare with additional pricing tools that would help the program strike a balance between maintaining incentives for innovation and ensuring affordability for beneficiaries and taxpayers. However, because Medicare operates within a context involving other payers as well as federal and state laws, agencies, and policies, many influences over drug prices are outside Medicare's purview, including funding for biomedical research and development (R&D), patent policy, tax policy, and the Food and Drug Administration's (FDA's) drug approval process.

Medicare coverage of Part A and Part B drugs

Section 1862(a)(1)(A) of the Social Security Act requires that the Medicare program cover Part A and Part B items and services that are included in a Medicare benefit category, are not statutorily excluded, and are "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member." (CMS considers a service reasonable and necessary if the service is safe and effective, not experimental or investigational, and appropriate for beneficiaries.) Based on statutory and regulatory text, FFS Medicare covers on-label use of a drug that the FDA has approved that is reasonable and necessary for the beneficiary. According to the *Medicare Benefit Policy Manual:*

> *Use of the drug or biological must be safe and effective and otherwise reasonable and necessary. . . . Drugs or biologicals approved for marketing by the Food and Drug Administration are considered safe and effective for purposes of this requirement when used for indications specified on the labeling. Therefore, the program may pay for the use of an FDA approved drug or biological, if:*
>
> - *It was injected [furnished] on or after the date of the FDA's approval;*
> - *It is reasonable and necessary for the individual patient; and*
> - *All other applicable coverage requirements are met* (Centers for Medicare & Medicaid Services 2021).

In addition, beginning in 1994, the Omnibus Budget Reconciliation Act of 1993 expanded Medicare coverage of Part B cancer drugs for indications not approved by the FDA if the drug's off-label use is supported by selected third-party drug compendia. Medicare may cover off-label use of noncancer drugs if the use is recognized, following Medicare's review of the peer-reviewed literature, as an appropriate treatment. Part B drug coverage is limited to products that are furnished

"incident to" a physician's service, provided that the drugs are not usually self-administered by the patients who take them.

Some Part B drugs are covered without the need for an explicit coverage policy. If the product is used for indications that the FDA approved and can be reimbursed on the basis of an existing billing code or a bundled payment system (e.g., the inpatient prospective payment systems (IPPS)), Medicare may cover it without an explicit coverage policy.

For other products, either CMS or Medicare's administrative contractors (MACs) make explicit coverage determinations under which a formal review of the medical, technical, and scientific evidence is conducted to evaluate the relevance, usefulness, and medical benefits of an item or service to Medicare beneficiaries, with opportunities for public participation. MACs develop the majority of explicit coverage policies through the local coverage determination (LCD) process that determines coverage of items and services that apply only in the contractor's regional jurisdiction. CMS develops coverage determinations for items and services that apply nationwide through the national coverage determination (NCD) process. Outcomes of the coverage process include (1) Medicare coverage of an item or service with no restrictions, (2) coverage for beneficiaries with certain clinical conditions or when furnished by certain providers or facilities, (3) leaving the coverage determination to the discretion of the MACs, or (4) Medicare not covering the service. CMS can initiate an NCD internally or can initiate one at a stakeholder's request under certain circumstances[2] or when a service's rapid diffusion is anticipated and the evidence may not adequately address questions regarding its impact on Medicare beneficiaries. The Commission's previous review of NCDs and LCDs for drugs found (1) the coverage policies appear to be aligned with the FDA's label indications, and (2) some policies delineate off-label conditions (for noncancer drugs) and the types of facilities or providers that Medicare will cover (Medicare Payment Advisory Commission 2022b).

A small subset of NCDs links a service's national coverage to participation in an approved clinical study or to the collection of additional clinical data. This policy is referred to as *coverage with evidence development* (CED), and its goal is to expedite early beneficiary access to innovative technology while ensuring that patient safeguards are in place. CED allows coverage of certain items or services when additional data gathered in the context of clinical care would further clarify the impact of these items and services on the health of Medicare beneficiaries. Because CED provides Medicare the opportunity to generate clinical evidence that otherwise might not have been collected, it enables the program to ultimately develop better, more evidence-based policies. CED also provides an opportunity to collect clinical evidence for groups that are often underrepresented in clinical trials, including older beneficiaries and minorities. As of May 2022, CMS applied CED to 21 items and services, and since the program's inception in 2005, 3 CED policies have been applied to drugs.[3]

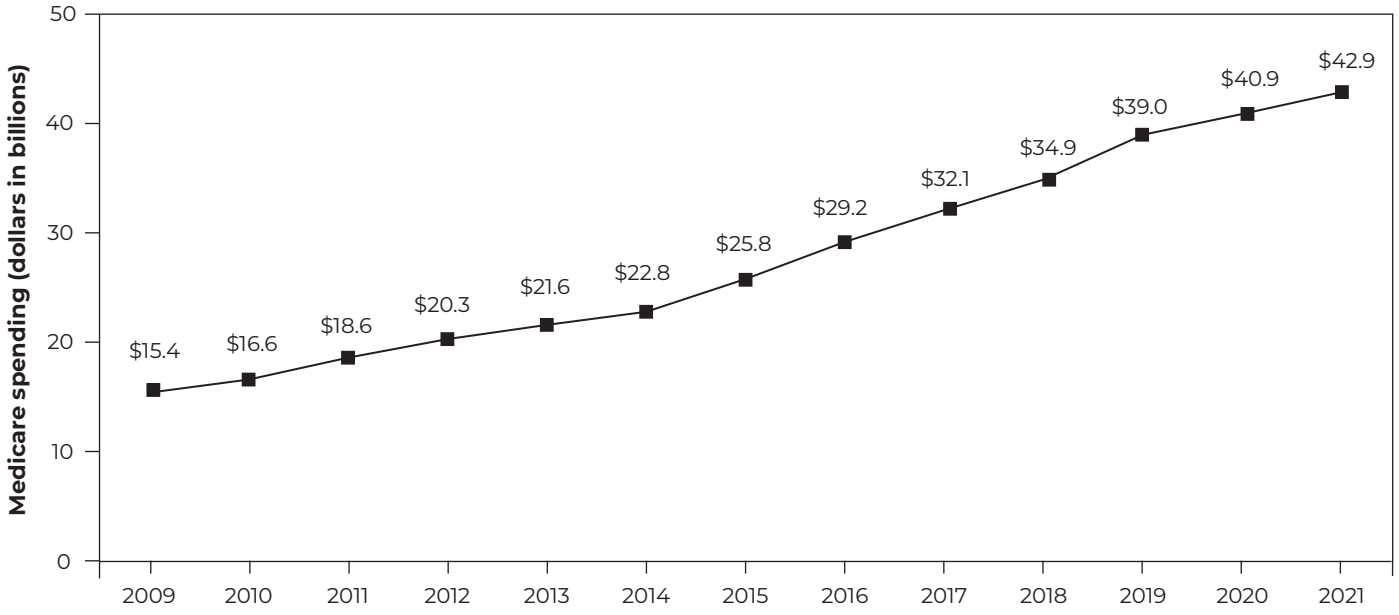
For Part B drugs, FFS Medicare generally bases payments on manufacturer-reported or provider-reported data

FFS Medicare largely acts as a price taker for Part B–covered drugs and historically has been able to do little to affect the amount the program pays for these products. Part B covers drugs that are administered by infusion or injection in physician offices and hospital outpatient departments, as well as certain drugs furnished by suppliers. Under FFS Medicare, these providers purchase drugs in the marketplace for whatever price the provider is able to negotiate. FFS Medicare pays these providers a prospectively determined rate for a Part B–covered drug, regardless of what the provider paid for the product. In many cases, FFS Medicare makes a separate payment for each drug administered, although in some cases Medicare bundles payment for drugs with payment for other services.

Medicare pays physicians and hospital outpatient departments for Part B drugs based on the manufacturer's ASP, which reflects the average price realized by the manufacturer for sales to most U.S. purchasers, net of rebates, discounts, and price concessions, with certain exceptions. ASP is determined by the manufacturer's pricing decisions and is generally unrelated to a product's clinical value. Medicare pays physicians and outpatient hospitals for most separately payable Part B drugs

FIGURE 1–1

Medicare Part B drug spending has grown rapidly since 2009



Note: Data include Part B–covered drugs furnished by several provider types, including physicians, suppliers, and hospital outpatient departments, and exclude those furnished by critical access hospitals, Maryland hospitals, and dialysis facilities. "Medicare spending" includes program payments and beneficiary cost sharing. Data reflect all Part B drugs whether they were paid based on the average sales price or another payment formula. Data exclude blood and blood products (other than clotting factor).

Source: MedPAC and Acumen LLC analysis of Medicare claims data.

based on 106 percent of the ASP, or ASP + 6 percent.[4] For 340B hospitals, Medicare paid a lower rate (ASP – 22.5 percent) for Part B drugs (except for those with pass-through status) between 2018 and 2022; however, the Supreme Court recently ruled that CMS's approach to establishing this lower payment amount was not consistent with its statutory authority.[5] In calendar year 2023, payment for Part B drugs furnished in 340B hospitals has reverted to ASP + 6 percent.

Medicare FFS pays some providers for Part B drugs as part of a broader payment bundle. For example, under the hospital outpatient PPS (OPPS), hospitals are paid for a subset of Part B–covered drugs—those that are low cost or that function as supplies to a service—as part of the ambulatory payment classification (APC) payment for other services. The APC payment rates are determined based on a relative weight-setting process, in which CMS estimates the average cost of services associated with each APC, including bundled drugs.[6] Similarly, under the end-stage renal disease (ESRD) PPS, Medicare makes a single patient-level adjusted payment to ESRD facilities that bundles composite rate services and other ESRD-related services, including drugs, that were separately billable under the prior payment method. Including drugs in the ESRD payment bundle has spurred price competition and use of less costly products among some ESRD drug groups.

Medicare's payment systems, including for Part B drugs, are determined by statutory provisions that generally do not consider a service's comparative clinical effectiveness. Medicare's payment for Part B drugs is determined without any requirement for evidence demonstrating that the product in question is equally or more effective than other available covered treatment options. Likewise, Medicare lacks authority to adjust the payment rate for a Part B drug when new evidence does not confirm its clinical benefit or significant safety concerns are discovered. Some researchers have called on Medicare to adopt "dynamic pricing" policies that would adjust a drug's payment

rate over time as clinical evidence about the drug evolves (Pearson and Bach 2010, Robinson 2022).

Medicare Part B currently has limited tools to manage drug prices

Historically, Medicare Part B has lacked tools to influence launch prices for new products or spur price competition among competing brand alternative products. For these products, Medicare Part B pays each product an ASP-based rate under the product's own billing code. For sole-source drugs, this policy means that Medicare will pay whatever launch price the manufacturer establishes for a product without generic competitors. Even for therapeutic classes in which there are multiple brand products, Medicare pays each product under its own billing code based on its own ASP, which permits manufacturers to establish high launch prices for "me-too" products (i.e., a brand product launched in the same therapeutic class as an already existing product that is generally used for the same therapeutic purpose and is structurally related) and does little to spur price competition.

In contrast, for brand drugs with generic competitors, Medicare Part B pays for the brand product and its generic equivalents in the same billing code based on 106 percent of a volume-weighted ASP. This policy creates incentives for providers to select the lower-cost product within a billing code, which in turn lowers the volume-weighted ASP in future calendar quarters, leading to substantial price reductions in payment rates for brand products after generic entry.

Medicare pays for biosimilars differently than it does for generic drugs. Each biosimilar receives its own billing code and is paid 100 percent of its own ASP, plus 6 percent or 8 percent of the originator's ASP. Medicare payment rates for originator biologics and their biosimilars have declined to some degree, but not to the extent observed with generic drugs. In 2017, the Commission recommended that biosimilars and originator biologics be paid in a consolidated billing code at the same rate to spur price competition among these products (Medicare Payment Advisory Commission 2017).

The recently enacted Inflation Reduction Act of 2022 makes changes to how Medicare and beneficiaries pay for some Part B drugs. Beginning in 2028, the Secretary will have the authority to negotiate the price of certain high-expenditure single-source products that lack generic or biosimilar competitors and that have been on the market for at least 9 years for drugs or at least 13 years for biologics. In addition, beginning in January 2023, Part B drug manufacturers are required to pay Medicare a rebate if the price of their product increases faster than inflation. The legislation includes additional provisions that affect Part B drugs, such as limiting beneficiary cost sharing for Part B–covered insulin and certain changes to payment for biosimilars. (See text box, pp. 14–15, for a summary of the Part B drug provisions in the Inflation Reduction Act.) However, some challenges remain. The program continues to lack tools to influence launch prices of new drugs, including those with limited clinical evidence. In addition, because Medicare pays for single-source drugs and biologics in separate billing codes based on their own ASP, concerns remain about a lack of price competition among products with similar health effects during the period before they are eligible for negotiation. With respect to Medicare's payment to providers, concerns remain that the percentage add-on can create financial incentives that favor prescribing higher-priced drugs in some circumstances.

Part B drug spending has been growing rapidly

Medicare Part B spending on prescription drugs is substantial and has been growing rapidly. Between 2009 and 2021, FFS Medicare Part B drug spending grew about 9 percent per year, from $15.4 billion to $42.9 billion (Figure 1-1). Although Part B drug price growth slowed between 2019 and 2021, rising about 5 percent per year on average, this slower growth reflected the decline in FFS enrollment over the period. Controlling for the number of FFS beneficiaries, Part B drug spending grew nearly 9 percent per year on average between 2019 and 2021.

Price has been the largest driver of Part B drug spending growth

Overall, the largest factor contributing to spending growth has been the change in the average price Medicare pays for Part B drugs, which reflects increased prices for existing products; the introduction of new, higher-priced drugs; and shifts in the mix of drugs.[7] Between 2009 and 2021, spending on separately

The Inflation Reduction Act of 2022 will make several changes related to Medicare payment and cost sharing for Part B drugs

The Inflation Reduction Act makes several changes to payment and cost sharing for Part B–covered drugs that have gone into effect or will go into effect in mid-2023 or 2024, including changes to payment for biosimilars, a manufacturer inflation rebate, and changes to cost sharing for Part B–covered insulin. In addition, beginning in 2028, Medicare will have authority to negotiate prices for certain Part B drugs.

Temporary increase to biosimilar payment rates

In general, biosimilars are paid 100 percent of a biosimilar's average sales price (ASP) plus 6 percent of the originator biologic's ASP. The Inflation Reduction Act increases the biosimilar add-on percentage from 6 percent to 8 percent for five years. New biosimilars launched before 2028 (for the first five years on the market) and existing biosimilars (for five years beginning October 1, 2022) will receive the 8 percent add-on, as long as the biosimilar's ASP does not exceed the originator's ASP.

Limit on payment rate for new biosimilars when ASP data are not yet available

Effective July 1, 2024, in the initial quarters when ASP data are not yet available, a new biosimilar's payment rate of 103 percent of its wholesale acquisition cost will be capped by the payment rate for the originator biologic.

Limit on coinsurance and deductible for Part B–covered insulin

Starting July 1, 2023, there will be a $35 limit on monthly cost sharing for Part B–covered insulin and the Part B deductible will not apply.

Manufacturer Part B inflation rebate

Beginning January 1, 2023, manufacturers of Part B single-source drugs, biologics, and biosimilars are required to pay Medicare a quarterly rebate if their product's ASP grows faster than inflation. Beginning April 1, 2023, for products that incur a rebate, beneficiary cost sharing will be based on the lower, inflation-adjusted ASP. Certain types of products are excluded from the policy (e.g., low-cost drugs, preventive vaccines, drugs experiencing a shortage or supply chain disruption, and biosimilars meeting certain criteria). Certain Part B utilization is also exempt from a rebate (including utilization subject to a 340B discount or Medicaid rebate and utilization for which payment is packaged). The per unit rebate amount will equal the difference between the actual ASP + 6 percent payment amount and the inflation-adjusted payment amount (that is, what ASP + 6 percent for a product would have been if ASP had grown at the same rate as inflation between the benchmark period in 2021 and the current period).

Negotiation of prices for certain Part B drugs

Beginning in 2028, the Secretary will have authority to negotiate certain Part B drug prices. (This authority also applies to Part D drugs beginning in 2026.)

Negotiation applies to high-expenditure drugs that have been on the market for many years. Eligible drugs are single-source drugs that have no direct generic or biosimilar competitors and that are at least 9 years postapproval for drugs and at least 13 years postapproval for biologics. Eligible drugs are defined as being in the top 50 of Part B or Part D expenditures. Each year, the Secretary will select a specified number of eligible drugs for negotiation. The specified numbers of drugs subject to negotiation are 10 Part D drugs for 2026, 15 Part D drugs for 2027, 15 Part B or Part D drugs for 2028, and 20 Part B or Part D drugs for each subsequent year. To select among drugs for negotiation, the Secretary is required to rank eligible drugs by total expenditures under Part B and Part D and select the most highly ranked drugs. Some products are excluded, such as vaccines, certain orphan drugs, low-expenditure drugs (less than $200 million annually, indexed to inflation), plasma-

(continued next page)

The Inflation Reduction Act of 2022 will make several changes related to Medicare payment and cost sharing for Part B drugs (cont.)

derived products, and, through 2028, certain small biotechnology products. In addition, the Secretary can delay application of negotiation for a biologic for up to two years at the request of its manufacturer if the Secretary determines there is a high likelihood of imminent biosimilar competition (with the manufacturer liable for certain rebates if biosimilar entry does not occur).

When the Secretary negotiates a drug's price, referred to as the maximum fair price, with the manufacturer, the statute directs the Secretary to consider two types of information:

- ***Manufacturer-provided information***—including research and development costs, market data, unit costs of production and distribution, prior federal financial support for discovery and development of the drug, data on patents and existing or spending exclusivity, national sales data, and information on clinical trials.

- ***Evidence about alternative treatment***—including the drug's comparative effectiveness and alternative treatments, including with respect to subpopulations, and the extent to which the drug addresses unmet need.

The Inflation Reduction Act also places limits on the maximum fair price. It cannot exceed a specified percentage of the nonfederal average manufacturer price. In general, the specified percentage is set at one of three levels depending on the length of time since the drug received Food and Drug Administration (FDA) approval: 75 percent for "short-monopoly drugs" (less than 12 years since FDA approval); 65 percent for "extended-monopoly drugs" (at least 12 years but less than 16 years since FDA approval); and 40 percent for "long-monopoly drugs" (at least 16 years since FDA approval). For Part B drugs, the maximum fair price also cannot exceed the ASP from a specified reference year.

The Act provides a process for updating a product's maximum fair price after the initial year that the price is effective. The maximum fair price for subsequent years is equal to the initial maximum fair price indexed to inflation. In addition, the statute permits the Secretary to renegotiate a product's maximum fair price under certain circumstances, including when a new indication is added; a drug product switches categories (e.g., to "extended-monopoly" or to "long-monopoly" categories); the Secretary determines that "material changes" have occurred in the factors considered; or renegotiation is likely to result in a significant change in the negotiated price.

Manufacturers of Part B drugs are required to participate in negotiation when their drug is selected by the Secretary. Manufacturers that do not comply face tax penalties based on a percentage of their sales up to 95 percent, depending on the number of days of noncompliance.

For Part B drugs, manufacturers are required to make the maximum fair price available to Medicare providers such as hospitals, physicians, and other providers furnishing these drugs to Medicare beneficiaries. For drugs subject to negotiation, Medicare will pay providers 106 percent of the maximum fair price. ■

payable Part B drugs (excluding vaccines, certain Part B drugs that were separately payable for only part of the period, and certain drugs that were billed in not-otherwise-classified codes) climbed, on average, by about 10.8 percent per year (Table 1-1, p. 16).[8]

Medicare's average annual payment per drug increased at an average rate of 7.7 percent per year. The number of beneficiaries using Part B drugs also increased between 2009 and 2021, by an average of 3.2 percent per year, while the number of Part B drugs received per

TABLE 1–1

Growth in the average payment per Part B drug was the largest factor contributing to spending growth for separately payable Part B drugs, 2009–2021

	2009	2021	Average annual growth, 2009–2021
Total payments: Separately payable* Part B drugs, excluding vaccines (in billions)	$11.4	$39.1	10.8%
Number of beneficiaries using a Part B drug (in millions)	2.5	3.6	3.2
Average total payments per beneficiary who used a Part B drug	$4,585	$10,790	7.4
Average number of Part B drugs per user	1.35	1.31	–0.3
Average annual payment per Part B drug per user	$3,396	$8,241	7.7

Note: This analysis includes Part B drugs paid based on the average sales price as well as the small group of Part B drugs that are paid based on other methods. "Vaccines" refers to 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 and exclude data for critical access hospitals, Maryland hospitals, and dialysis facilities. Yearly figures presented in the table are rounded; the average annual growth rate was calculated using unrounded data.
*For purposes of this analysis, spending on separately payable Part B drugs excludes any drug that was bundled in 2009 or 2021 (i.e., drugs that were packaged under the outpatient prospective payment system in 2009 or 2021 were excluded from both years of the analysis, regardless of the setting in which the drug was administered), vaccines, drugs billed under not-otherwise-classified billing codes, and blood and blood products (other than clotting factor). Because of these exclusions, total spending reflected in this table is lower than spending in Figure 1-1 (p. 12).

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

user declined slightly during this period (by about 0.3 percent per year).

Medicare spending on Part B drugs

In 2021, Medicare and its beneficiaries paid about $42.9 billion for Part B–covered drugs.[9] Although there are roughly 900 billing codes for Part B drugs, spending is concentrated. In 2021, Part B drug spending for the top 10 products that accounted for the most aggregate spending, which were all biologics (including some with biosimilars), accounted for $17.4 billion, or 41 percent of total Part B drug spending. Spending on the top 20 products accounted for $22.9 billion, or about 53 percent of total Part B drug spending.

The top 20 Part B drugs tend to be concentrated in certain therapeutic areas, though some are used to treat multiple conditions (Table 1-2). Eleven of the top 20 Part B drugs are for cancer patients: 8 drugs that treat cancer and 3 supportive drugs that treat cancer side effects. The top 20 also include 5 products to treat rheumatoid arthritis or other inflammatory disorders and 3 products used to treat macular degeneration and other eye conditions. Also among the top 20 are one product for multiple sclerosis, one extremely high-cost product (spending of over $380,000 per patient year) for rare autoimmune conditions, one product for immunodeficiency or neuropathy, and one influenza vaccine product.

The patterns of spending among the top 20 products illustrate the effect of high launch prices on Medicare spending. For example, two products—Keytruda and Opdivo—were approved in late 2014 and were the first products belonging to a newer class of immune-oncology biologics. Spending on these products in 2021 was $4.0 billion for Keytruda and $1.6 billion for Opdivo, reflecting the products' substantial launch prices followed by additional price inflation after launch. In 2021, average annual Medicare spending per user for these products exceeded $60,000. Other recently launched cancer products in the top 20, such as Darzalex and Tecentriq, also had substantial average annual spending per patient, exceeding $80,000 and $50,000 per patient year, respectively.

TABLE 1–2

The top 20 highest-expenditure Part B drugs accounted for over half of total Part B drug spending in 2021

Part B drugs	Indication	Number of beneficiaries who used product, 2021	Total spending (in billions), 2021	Average spending per user, 2021	Average annual ASP growth 2005–2023[c]
Keytruda	CA	63,200	$4.0	$62,900	2.4%
Eylea	MD	312,200	3.4	11,000	–0.9
Prolia/Xgeva	CA SE, OS	627,600	1.8	2,800	4.4
Opdivo	CA	25,600	1.6	61,500	2.4
Darzalex	CA	18,800	1.5	81,400	3.9
Rituxan[a]	AR, CA, ID	64,900	1.3	20,100	3.5
Lucentis[a]	MD	115,200	1.0	9,100	–3.5
Orencia	AR, CA SE	31,700	1.0	31,200	5.4
Avastin[a]	CA, MD	191,200	0.9	4,600	1.2
Neulasta[a]	CA SE	85,700	0.9	10,100	–1.9
Tecentriq	CA	12,700	0.7	51,700	1.3
Remicade[a]	AR, ID	53,900	0.6	12,000	–2.3
Soliris	AI	1,700	0.6	382,700	1.7
Ocrevus	MS	12,800	0.6	47,600	0.9
Entyvio	ID	16,000	0.5	32,900	3.5
Herceptin[a]	CA	18,500	0.5	27,600	2.5
Gammagard	IMD, NE	18,800	0.5	27,000	2.5
Cimzia	AR, ID	21,500	0.5	23,300	2.4
Alimta	CA	17,500	0.5	27,300	–2.1
Fluzone High-Dose[b]	VA	7,596,800	0.5	62	7.5
Top 20 drugs			22.9		
All Part B drugs			42.9		

Note: ASP (average sales price), CA (cancer), MD (macular degeneration and other eye disorders), SE (side effects), OS (osteoporosis), AR (arthritis), ID (inflammatory disorders), AI (autoimmune), MS (multiple sclerosis), IMD (immune deficiency), NE (neuropathy), VA (vaccine). "Total spending" includes Medicare program payments and beneficiary cost sharing. Number of beneficiaries, total spending, and average spending per user displayed in the table are rounded; average spending per user was calculated using unrounded numbers.
[a]Spending and utilization data for 2021 reflect the originator biologic and its biosimilars (except for Lucentis, which experienced biosimilar entry after 2021). The average annual growth rate of ASP is based on the ASP-based payment rate for the originator biologic.
[b]Fluzone High-Dose is a preventive vaccine paid based on 95 percent of the average wholesale price (AWP). Percent change in the AWP-based payment rate rather than the ASP-based payment rate is displayed in the table.
[c]Average annual ASP growth between 2005 and 2023 is calculated using payment rates from the first quarter of each year. For products not on the market for the full period from 2005 to 2023, the average annual growth rate was calculated using the following alternate base years: 2018 (Ocrevus, Tecentriq), 2017 (Darzalex), 2016 (Keytruda, Opdivo, Entyvio), 2013 (Eylea), 2012 (Prolia/Xgeva), 2011 (Fluzone High-Dose), 2010 (Cimzia), 2008 (Lucentis, Soliris, Gammagard), and 2007 (Orencia).

Source: MedPAC analysis based on claims data, publicly available ASP payment rate files, and outpatient prospective payment system Addendum B from CMS.

Price inflation among products that have been on the market for a longer period also contributes to spending growth. For example, between 2005 and 2023 (or since launch if after 2005), Darzalex, Entyvio, Orencia, and Prolia/Xgeva have all experienced ASP growth of between 3.3 percent and 5.4 percent per year on average (Table 1-2). Fluzone High-Dose, which is paid 95 percent of the average wholesale price, also experienced substantial price growth (7.5 percent per year on average over the analysis period). While some

TABLE 1–3

Prices for certain biologics have declined due to biosimilar entry, after substantial price growth for these products during the preceding 10-year period

	First biosimilar entry	Percent change in originator biologic's ASP		Biosimilars' payment rate as a percentage of originator biologic's payment rate (2023 Q1)	Biosimilar market share (2022 Q3)
		In 10 years before biosimilar entry	Since biosimilar entry (through 2023 Q1)		
Neupogen and biosimilars	2015 Q3	71%	–2%	24–41%	83%
Remicade and biosimilars	2016 Q4	54	–58	71–130*	26
Neulasta and biosimilars	2018 Q3	117	–66	67–108*	43
Procrit/Epogen and biosimilars	2018 Q4	35	–33	98	52
Avastin and biosimilars	2019 Q3	42	–13	45–48	77
Herceptin and biosimilars	2019 Q3	69	–23	40–71	74
Rituxan and biosimilars	2019 Q4	68	–14	40–61	59
Lucentis and biosimilars	2022 Q3	–31	–14	99	N/A

Note: ASP (average sales price), Q (quarter). N/A (not available). An originator biologic is a drug product derived from a living organism. A biosimilar product is a follow-on product that is approved by the Food and Drug Administration (FDA) based on the product being highly similar to the originator biologic. The biosimilars included in the analysis are Zarxio, Nivestym, and Granix for originator Neupogen; Inflectra, Renflexis, and Avsola for originator Remicade; Fulphila, Udenyca, Ziextenzo, and Nyvepria for originator Neulasta; Retacrit for originator Procrit//Epogen; Mvasi and Zirabev for originator Avastin; Ontruzant, Herzuma, Ogivri, Trazimera, and Kanjinti for originator Herceptin; Truxima, Ruxience, and Riabni for originator Rituxan; and Byooviz for originator Lucentis. 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 in the U.S. market. "First biosimilar entry" date reflects the earliest market date for a product approved by the FDA as a biosimilar to the originator biologic.
*As of the first quarter of 2023, there was one biosimilar for Remicade and three biosimilars for Neulasta with Medicare payment rates that exceeded the originator's payment rate, while the other biosimilars had payment rates below the originator's payment rate (data not shown).

Source: MedPAC analysis of Medicare ASP payment rate files publicly available on CMS website and Medicare claims data for physicians and outpatient hospitals.

products have experienced substantial price increases over many years, price growth varies across products. As shown in the Commission's 2022 data book, a price index for Part B drugs, which measures postlaunch price growth at the individual product level, finds that, on average, Part B drug prices increased 16 percent cumulatively over the 10-year period from 2010 to 2020, with a 37 percent increase for biologics and an 18 percent decrease for drugs (Medicare Payment Advisory Commission 2022a).[10]

Biosimilar entry has led to some price competition. Recently, some biologics, including several in the top 20 (Rituxan, Lucentis, Avastin, Neulasta, Remicade, and Herceptin), have faced biosimilar entry. Biosimilars have resulted in savings because originators have generally lowered their prices in response to biosimilar competition and because biosimilar prices are in some cases substantially below innovators' prices (Table 1-3). However, the extent to which originator biologics have reduced their prices has varied substantially. Some originator biologics have reduced prices only modestly since biosimilar entry despite the availability of biosimilar competitors at substantially lower prices. For example, since the introduction of biosimilars for Neupogen, Avastin, and Rituxan that are less than half the price of the originator biologics, the prices of the originators have fallen just 2 percent, 13 percent, and 14 percent, respectively, and the ASP-based payment rate for the originator Avastin actually increased 4 percent between January 2022 and 2023 (data not shown). Note that for all but one originator biologic now facing

biosimilar competition (Lucentis), recent price declines have come after many years of price increases prior to the entry of biosimilars.

In 2021, spending for drugs that had yet to be assigned to their own billing code totaled nearly $142 million.[11] Medicare claims do not provide a way to identify these products because claims data do not provide the drug's national drug code (which is a universal product identifier). To better track spending trends and conduct pharmacoepidemiology research, including examining the adoption of newly launched accelerated drugs, the Secretary could consider requiring providers to report the drug's national drug code on claims for drugs that lack their own billing code. Moreover, coding these claims would further the ability of researchers and Medicare to use claims data to conduct pharmacoepidemiologic analyses if providers reported national drug codes for all drugs—including those assigned their own billing code.

The relationship between Medicare payment and drug research and development

As we discussed in our June 2022 report to the Congress, the price that Medicare and other entities pay for drugs is one of many factors that influence manufacturer research and development (R&D) investment (Medicare Payment Advisory Commission 2022b). According to the Congressional Budget Office (CBO), manufacturer R&D investment is influenced by the lifetime global revenues a new drug is expected to generate, the expected cost of developing the new drug, and any policies that affect supply or demand for the drug (Congressional Budget Office 2021b). Expected global revenues from new drug development depend on the prices and volume of sales that companies expect in different markets and the likelihood that drug development efforts will succeed (Congressional Budget Office 2021b). Several studies have found a positive relationship between market size, as measured by expected revenue or other related proxies, and R&D investment, such as the number of products undergoing clinical trials or the number of new products launched (Blume-Kohout and Sood 2013, Cerda 2007, Dubois et al. 2015).

Not only is the amount of R&D investment of interest, so too are the types of products R&D is focused on. In response to the establishment of Medicare Part D, several studies found increased clinical trial activity among drugs intended to treat clinical conditions prevalent among Medicare beneficiaries (Blume-Kohout and Sood 2013, Dravone et al. 2020). However, Dravone and colleagues found that the increase in clinical trial activity following the introduction of Part D was most pronounced among "less scientifically novel" products, while clinical trials for products that were in the most scientifically novel category (meaning the first use of a targeted base action) increased modestly (Dravone et al. 2020).

R&D is influenced by many factors beyond Medicare policy, including federal regulatory policies related to drug approval and patents and intellectual property; federal tax policy; payment policies of other payers in the U.S. and internationally; the cost of drug development, including capital availability and costs; and collaboration between pharmaceutical manufacturers and academic institutions (Congressional Budget Office 2021b). In addition, the federal government contributes to innovation both indirectly (through its substantial funding of basic science research) and directly (through its funding of drug development research for some products) (Galkina Cleary et al. 2018, Sampat and Lichtenberg 2011).[12]

Some stakeholders have raised concerns that reducing Medicare spending for drugs would lead to lower expected manufacturer profitability and reduce incentives for product improvement or innovation (Frank and Ginsburg 2017). CBO addressed this issue in a working paper discussing how the agency analyzes legislation that may affect drug development (Congressional Budget Office 2021a). CBO assumes that policies that reduce earnings for drug manufacturers would lead to some reduction in the number of new drugs developed (however, CBO explicitly makes no assumptions about the types of new drugs affected or the effect on health outcomes).[13]

However, under current Medicare policy, drug manufacturers are largely able to set their own prices even when incremental benefits to Medicare beneficiaries are low or are not well established. Implementing payment policies that focus on a drug's net clinical benefit could drive R&D investment toward products that have potential for larger effects on patient health and expected profitability. For example,

Sachs and Frakt suggest that some drug payment policies, including reference pricing, have the potential to shift R&D toward drugs that provide more value (Sachs and Frakt 2016). It is important, therefore, for Medicare to design payment policies that strike an appropriate balance between creating incentives for innovation and ensuring that the program is getting good value for beneficiaries and taxpayers.

Addressing high launch prices for drugs with limited clinical evidence by capping the payment of select Part B "accelerated approval" drugs and biologics

The FDA's accelerated approval program allows drugs to come to market faster than under the traditional approval process. Although this pathway was originally used for HIV drugs, approximately 85 percent of accelerated approvals in the last decade have been granted in oncology (Beaver and Pazdur 2021). This pathway is intended to expedite the approval of potentially promising products for cancer and other complex or rare conditions by reducing the development or review time needed to bring a potentially innovative drug to market; incentives are important for drug development in these areas. Under this pathway, the FDA approves drugs based on intermediate clinical or surrogate endpoints that are *reasonably likely to predict* a clinical benefit, but before the clinical benefit has been demonstrated. The FDA requires manufacturers to conduct postmarketing studies to verify and describe the clinical benefit and risk profile. After the completion of a drug's confirmatory trial(s), an accelerated approval drug generally fits into one of three categories: (1) converts to traditional approval based on confirmatory studies that document a drug's clinical benefit; (2) continued marketing authorization under accelerated approval even though the required confirmatory trials do not end up finding a clinical benefit, a so-called "dangling" approval; or (3) voluntary withdrawal by the manufacturer or involuntary withdrawal by the FDA.[14]

Concerns with how Medicare pays for accelerated approval drugs

Whether and how accelerated approval drugs impact clinical outcomes is uncertain at the time of approval. Concerns about how Medicare pays for accelerated approval drugs include the following:

- Accelerated approval is not based on measures of clinical benefit related to how a patient feels, functions, or survives. Thus, products approved under the accelerated approval pathway have more uncertainty about their clinical benefit than products approved under the traditional pathway.
- Completion of postmarket confirmatory clinical trials is often delayed.
- Over time, an increasing number of drugs have been approved under the accelerated approval pathway, and Medicare spending for such drugs is significant. Medicare's Part B payment rate for a drug may exceed the payment justified by its net clinical effectiveness (Medicare Payment Advisory Commission 2022b).

It is important that Medicare's payments for accelerated approval drugs strike an appropriate balance between creating incentives for innovation and ensuring good value and affordability for beneficiaries and taxpayers. This need is particularly relevant for products with accelerated approval since some may not have any competitors. At the same time, Medicare's payment policies could help create incentives for companies to complete postmarketing confirmatory trials on a timely basis. That way, information about a product's effects on health outcomes is available as soon as possible to providers who may prescribe the product and beneficiaries who may receive it.

FDA accelerated approval is not based on measures of clinical benefit related to how a patient feels, functions, or survives

The FDA instituted its accelerated approval program to allow for earlier approval of drugs that treat serious conditions and fill an unmet medical need. Under the program, the FDA approves drugs based on a surrogate or intermediate clinical endpoint that is *reasonably likely* to predict a clinical benefit.[15] The use of such endpoints can considerably shorten the time to receiving FDA approval. However, products approved under the accelerated approval pathway have more uncertainty about their clinical benefit than products approved under the traditional pathway. According to researchers, roughly 40 percent of drugs granted accelerated approval in the U.S. between 2007 and

2021 were rated (according to international health technology assessments) as providing moderate or greater therapeutic value compared with existing therapies. The share of cancer and noncancer drug indications rated as having high added therapeutic value were 36.0 percent (27 of 75) versus 53 percent (8 of 15), respectively (Vokinger et al. 2022).[16] One aspect of this uncertainty pertains to whether there is, in fact, any relationship between the selected surrogate endpoint and the intended clinical outcome. For example, researchers concluded that most trial-level validation studies of surrogate endpoints in oncology find low correlations with patients' overall survival (Prasad et al. 2015).[17] According to the FDA, using surrogate endpoints creates a risk that patients could be exposed to a drug that later is shown not to provide an actual clinical benefit.[18] Further, because accelerated approval may rely on smaller or shorter clinical trials than used under traditional approval, this pathway may result in less information about the likelihood of rare or delayed adverse events (Food and Drug Administration 2014). Because of the use of surrogate outcomes and other design features (e.g., use of single-arm trials and trials with relatively small sample sizes), clinicians and patients generally have less data with which to judge the benefits and risks of products approved under the accelerated approval pathway compared with drugs approved under traditional pathways.

Completion of postmarket confirmatory clinical trials is often delayed

Sponsors conduct postmarket confirmatory trials while these drugs are available to the public on a timeline agreed to by the FDA and the sponsor. Some drug manufacturers never complete required postmarket confirmatory clinical trials or do so only after long delays.[19] Table 1-4 (p. 22) gives examples of Part B products and indications with late confirmatory trials. For example, the FDA approved Opdivo for a specific type of colorectal cancer under the accelerated approval pathway in July 2017 with a final report due in September 2021 (as of April 2023, the product remains marketed under its accelerated approval). According to the Commission's analysis, about 30 percent of accelerated approval drug indications with incomplete confirmatory trials are past their original planned completion dates, including two that are more than five years past those dates.[20] Our analysis of FDA data found that among the 290 unique accelerated approval drug indications approved under the accelerated approval pathway between 1992 and 2022, the agency has converted 56 percent to traditional approval and has withdrawn approval from 13 percent; the remainder have not yet converted (e.g., confirmatory trials are still under way).[21] Because current law does not differentiate Medicare payment between a drug approved under traditional versus accelerated approval, some sponsors may have little incentive to complete postapproval confirmatory trials promptly. According to OIG, two common challenges that affect sponsors' abilities to complete confirmatory trials are advances in the standard of care, which can make it difficult for a drug's confirmatory trial to detect clinical benefit attributable to the drug, and changes in the ownership of a drug application (Office of Inspector General 2022).

In addition to drugs with late confirmatory trials, some accelerated approval drugs remain on the market despite their postapproval confirmatory trials not confirming a clinical benefit—"dangling" accelerated approvals. For example, according to researchers, the use of Keytruda for hepatocellular carcinoma, which was approved in November 2018 with a postmarket confirmatory study due in October 2019, is an example (as of April 2023) of such a product (Beaver and Pazdur 2021). In 2021, the FDA's advisory committee voted in favor of maintaining accelerated approval of Keytruda for hepatocellular carcinoma (based on results of the ongoing confirmatory trial); in 2022, the manufacturer announced additional results from the confirmatory trial; and, as of April 2023, this indication remains under accelerated approval (Cohen et al. 2022).

Some manufacturers have ultimately withdrawn their product many years after their accelerated approval. For example, Romidepsin for peripheral T-cell lymphoma was approved in 2011, and its confirmatory trial's final report was scheduled to be completed by April 2019; its manufacturer withdrew the indication in 2021 because the trial did not meet its primary efficacy endpoint (progression-free survival). Part B spending for Romidepsin's withdrawn indication in 2020 and 2021 was nearly $10 million. Sulfamylon, an antimicrobial agent (covered under Part D) that controls bacterial infection in the treatment of burns, was approved in 1998; in December 2021, the manufacturer (Viatris) sent the FDA a letter asking to withdraw the drug because

TABLE 1–4

Examples of Part B accelerated approval drugs that missed the deadline for completion of their confirmatory trials

Drug name	Drug's clinical indication	Date of accelerated approval	Due date of final report	Average spending per user, 2021
Beleodaq	Treatment of peripheral T-cell lymphoma	7/3/2014	1/31/2021	$124,100
Folotyn	Treatment of relapsed or refractory peripheral T-cell lymphoma	9/24/2009	6/30/2017	140,300
Jemperli	Treatment of mismatch repair deficient recurrent or advanced solid tumors	8/17/2021	10/31/2022	27,000
Keytruda	Treatment of hepatocellular carcinoma for patients who have been previously treated with sorafenib*	11/9/2018	10/31/2019	62,900
Libtayo	Treatment of basal cell carcinoma	2/9/21	2/28/2022	66,200
Opdivo	Treatment of microsatellite instability-high or mismatch repair deficient metastatic colorectal cancer	7/31/2017	9/30/2021	61,500
Pepaxto	Treatment of relapsed or refractory multiple myeloma	2/26/2021	2/28/2022	21,000
Rybrevant	Treatment of locally advanced or metastatic non–small cell lung cancer	5/21/2021	2/28/2023	43,700
Zepzelca	Treatment of metastatic small cell lung cancer**	6/15/2020	2/28/2021	46,600

Note: Deadline date based on date of final report submission for accelerated approval. If more than one trial was required, the final report date is based on the latest date. Average spending per user is determined across all indications (i.e., accelerated and traditional approval indications) of the drug.
*The product's confirmatory trial is complete but the Food and Drug Administration has continued its marketing authorization.
**In 2020, the manufacturer announced that the confirmatory trial did not meet its primary endpoint. In 2021, the manufacturer announced the initiation of new confirmatory trials with estimated study completion dates of 2025 and 2026 (Food and Drug Administration 2022b).

Source: MedPAC analysis of data from the Food and Drug Administration on accelerated approvals as of December, 31, 2022 (Food and Drug Administration 2022a), with the status of an indication updated as of April 15, 2023, using data from the Food and Drug Administration found at https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approval-program.

a confirmatory study was not feasible (Food and Drug Administration 2022c). As of December 31, 2022, the FDA has withdrawn both products.

Over time, an increasing number of drugs have been approved under the accelerated approval pathway, and Medicare spending for such drugs is significant

Since 2010, the number of unique drug indications approved through the FDA's accelerated approval pathway has grown dramatically. Between 2006 and 2010, just 25 drugs were given accelerated approval; that number climbed to 40 approvals between 2011 and 2015, 108 between 2016 and 2020, and 37 in 2021 and 2022. Nearly 30 percent of accelerated approvals (82 of 290) occurred between 2020 and 2022. Some of this increase is linked to multiple accelerated approvals of a given drug for the same condition but with different dosing schedules (Keytruda with 17 approvals).

As of December 31, 2022, there were 73 Part B drugs with 134 clinical indications that were approved

through the accelerated approval pathway, including 8 of the top 20 highest-expenditure Part B drugs (Table 1-2, p. 17): Alimta (with 3 indications), Avastin (with 2 indications), Darzalex (with 1 indication). Darzalex Faspro (with 1 indication), Keytruda (with 34 indications), Opdivo (with 11 indications), Remicade (with 1 indication), and Tecentriq (with 3 indications). Of these 134 clinical indications approved through the accelerated approval pathway, 44 indications have not converted. Two immune-oncology drugs, Keytruda and Opdivo, account for about one-quarter of all Part B indications that have not converted (six indications and five indications, respectively).

Medicare spending for accelerated approval drugs that have not converted to traditional approval is substantial. In 2021 alone, according to the Commission's analysis, Medicare spending totaled $363 million for 7 accelerated approval indications for 4 of the top 20 drugs (Table 1-2, p. 17):

- Darzalex Faspro spending for light chain amyloidosis was $67 million, representing 6 percent of the drug's total spending in that year;
- Keytruda spending for gastric, hepatocellular, and Merkel carcinoma indications was $123 million, accounting for 3 percent of the drug's $4.0 billion in total spending in that year;
- Opdivo spending for hepatocellular carcinoma was $48 million, representing 3 percent of the drug's $1.6 billion in total spending in that year; and
- Tecentriq spending for breast and urothelial carcinoma indications was $125 million, accounting for 19 percent of the drug's $660 million in total spending in that year.

These findings—the share of total spending associated with accelerated approval indications among the top 20 drugs—are conservative. This analysis does not include the spending for accelerated approval indications that overlap with traditional approval indications, particularly for Keytruda and Opdivo. In addition, these findings do not account for Medicare spending among non–top 20 drugs. For example, in 2021, spending totaled nearly $245 million for the accelerated approval indications of select non–top 20 drugs (Aliqopa, Amondys 45, Beleodaq, Blenrep, Folotyn, Pepaxto, Monjuvi, Polivy, Romidepsin, Rybrevant, Yervoy, Zepzelca, and Zynlonta). Furthermore, these numbers do not account for utilization of these drugs by Medicare Advantage enrollees.

Since Medicare lacks tools to influence the prices of new drugs, manufacturers have significant market power to set a new drug's price because the statute requires that Medicare assign the drug to its own billing code and set a payment rate based on its individual ASP. Manufacturers typically set a high launch price for drugs approved under the accelerated approval program, yet these prices may not reflect the expected clinical benefit of the product. According to CBO, drug manufacturers set launch prices for new drugs to maximize future net revenues, taking into account manufacturing and distribution costs. The amount spent on R&D for a particular product does not influence the price a drug company establishes for that product because R&D costs associated with a new product have already been incurred (commonly referred to as "sunk costs") (Congressional Budget Office 2021b).[22] Other factors that manufacturers may consider when establishing a drug's price include the competitiveness of the market, the drug's uniqueness (e.g., first-in-class products), its net clinical benefit compared with existing therapies, the pricing of existing therapies, how the price established may affect physicians' willingness to prescribe the product, and payers' reimbursement policies (e.g., how payers set payment rates or use tools such as formularies or prior authorization) (Robinson 2022). A congressional report on how the manufacturer of Aduhelm established its initial launch price provides an example of how manufacturers consider drug pricing (House Committee on Oversight and Reform and House Committee on Energy and Commerce 2022). That report indicated that the manufacturer of Aduhelm (a first-in-class product approved under the accelerated approval pathway for Alzheimer's disease) considered several competing factors before setting the product's initial launch price at $56,000 (per patient year) in June 2021. The manufacturer considered comparative clinical and cost-effectiveness, maximizing patient volume, pushback from payers and other stakeholders, and revenue maximation. The company's initial price of $56,000 was consistent with price levels suggested by revenue maximation, whereas the other considerations suggested lower pricing, according to the report.

Some high-priced products that are given accelerated approval are later withdrawn from the market after failing confirmatory trials. As of April 2023, 19 Part B drugs with 24 clinical indications have been withdrawn. The length of time that these indications were marketed (i.e., years between their dates of accelerated approval and withdrawal) averaged 6.8 years. Examples of relatively costly Part B accelerated approval products with Medicare spending in the year prior to their withdrawal include the following:

- Blenrep (average spending $52,800 per user in 2021) for multiple myeloma was approved in August 2020 and withdrawn in November 2022. In 2021, total spending for this accelerated approval indication was $36 million.
- Imfinzi (average spending $55,000 per user in 2020) for urothelial carcinoma was approved in May 2017 and withdrawn in February 2021. In 2020, total spending for this accelerated approval indication was $4 million.
- Opdivo (average spending $62,200 per user in 2020) for hepatocellular carcinoma was approved in September 2017 and withdrawn in July 2021. In 2020, total spending for hepatocellular carcinoma was $68 million.
- Tecentriq (average spending $51,700 per user in 2021) was approved for two indications of urothelial carcinoma; the first indication was approved in May 2016 and withdrawn in April 2022, and the second indication was approved in April 2017 and withdrawn in December 2022. The drug was also approved for one indication for breast cancer in March 2019 and withdrawn in October 2021. In 2021, total spending for the urothelial carcinoma and breast cancer accelerated approvals was $65 million and $60 million, respectively.[23]

Setting a cap on Medicare's payment of select accelerated approval Part B drugs

To maintain financial rewards for innovation while improving access and affordability of care for beneficiaries and taxpayers and spurring manufacturers to complete their required confirmatory trials on time, Medicare should cap the payment rate of certain Part B drugs and biologics that are approved under the accelerated approval program. To implement this policy, the Congress would need to provide the Secretary with statutory authority to apply a payment method to Part B drugs other than the ASP-based method in Section 1847A of the Social Security Act.

Setting a cap on payment of accelerated approval Part B drugs would help to make Medicare a more prudent purchaser of health care services while ensuring access to high-quality care for Medicare beneficiaries. In most instances, the Secretary could set a cap on payment based on the clinical benefit and cost of the accelerated approval drug relative to the standard of care. Capping a drug's payment based on its net clinical benefit would limit beneficiaries' and taxpayers' financial risk of using products with uncertain benefit (Lederer and Dusetzina 2021). This targeted application of a payment cap for accelerated approval drugs balances the tradeoffs between incentives for manufacturers' innovation and affordability and access for beneficiaries and taxpayers. On the one hand, Part B accelerated approval drugs offer beneficiaries earlier access to drugs that may improve clinical outcomes. On the other hand, the prices for those products are not necessarily commensurate with their benefits, even if surrogate outcomes were assumed to perfectly translate to clinical outcomes. In addition, capping payments would also provide strong incentives for the completion of postapproval trials; under current Medicare payment policies, there is no incentive to do so.

RECOMMENDATION 1-1

The Congress should require the Secretary to cap the Medicare payment rate for Part B drugs and biologics that are approved under the accelerated approval program (with limited circumstances for the Secretary to waive the payment cap) if:

- **postmarketing confirmatory trials for the product are not completed within the deadline established by the manufacturer and the Food and Drug Administration,**
- **the product's clinical benefit is not confirmed in postmarketing confirmatory trials, or**
- **the product is covered under a "coverage with evidence development" policy.**

In addition, the Congress should give the Secretary the authority to cap the Medicare payment rate of Part B drugs and biologics that are approved under the accelerated approval program if their price is excessive relative to the upper-bound estimates of value.

RATIONALE 1-1

The accelerated approval pathway is intended to expedite the approval of potentially promising products for cancer and other complex or rare conditions; incentives for drug development in these areas are important. At the same time, tools are needed to ensure that the Medicare program is not overpaying for products approved on an accelerated basis if a product's clinical benefit is not confirmed. Such tools are particularly relevant for products approved on this pathway, as some may not have any competitors. Manufacturers also need an incentive to complete postmarketing confirmatory trials on a timely basis so that information about a product's effects on health outcomes is available as soon as possible to providers who may prescribe it and beneficiaries who may receive it. The accelerated approval cap policy seeks to balance these trade-offs through the targeted application of the payment cap. Furthermore, by designing the payment cap based on net clinical benefit, the approach would reward companies with very promising drug products, acknowledging the advances they provide over the status quo.

IMPLICATIONS 1-1

Spending

- This recommendation would decrease federal program spending relative to current law.

Beneficiary and provider

- This recommendation would be expected to generate savings for beneficiaries through lower cost sharing but should not adversely affect beneficiaries' appropriate access to needed Part B drugs. The policy would be expected to result in more timely development of evidence of the clinical outcomes of accelerated approval drugs for beneficiaries and providers. This recommendation would not be expected to affect providers' willingness and ability to serve beneficiaries.

Applying a payment cap according to the status of a drug's confirmatory trial and Medicare's coverage requirements

Under the recommendation, the Secretary would be required, with rare exceptions, to apply a payment cap to accelerated approval drugs under three circumstances. First, the Secretary would cap Part B accelerated approval drugs that miss the deadline that the manufacturer set in collaboration with the FDA to complete their accelerated approval postmarket confirmatory trial. Table 1-4 (p. 22) provides examples of drugs with particular accelerated approval indications that have missed their deadline for completion (of their final report).

The Secretary could base a confirmatory study's completion date on either the "trial completion" date or the "final report submission" date specified in the FDA's approval letter to the drug sponsor. In cases in which an accelerated approval drug has multiple confirmatory trials for a given clinical indication, the Secretary could base the "deadline" on the trial with the latest date or the date of the trial whose population is most relevant to Medicare (e.g., adults vs. pediatric patients).

Second, the Secretary would cap the payment of accelerated approval drugs whose clinical benefit was not verified in postapproval confirmatory trials. Per statutory and regulatory provisions (noted on p. 10), the Secretary currently can cover and pay for off-label use of cancer and noncancer drugs in certain circumstances:

- The statute requires that Medicare cover Part B cancer drugs for indications not approved by the FDA if the drug's off-label use is supported by selected third-party drug compendia.[24] Use of Avastin for breast cancer is an example of Medicare off-label coverage of a cancer drug. When the FDA withdrew Avastin's breast cancer accelerated approval indication, CMS announced that it would still cover and pay for the product (Yukhananov and Selyukh 2011). In 2021, Medicare's Part B spending for Avastin for breast cancer totaled about $4 million, which represents less than 1 percent of the biologic's FFS Medicare spending for cancer-related conditions.
- FDA-approved drugs used for noncancer indications other than what is indicated on the official label can be covered under Medicare if the MAC determines the use to be medically accepted, taking into consideration the major drug compendia, authoritative medical literature, or accepted standards of medical practice. These decisions are generally made by the MAC on a case-by-case basis (Centers for Medicare & Medicaid Services 2021).

Thus, the Secretary would cap the payment rate of accelerated approval drugs whose clinical benefit was not verified in postapproval confirmatory trials, including the off-label use of such drugs that remain supported by compendia (as is the case for Avastin for breast cancer) as well as the on-label use of drugs whose marketing authorization under the FDA continues (i.e., "dangling" drugs).[25]

Third, the Secretary would cap accelerated approval drugs that the Secretary covers under a "coverage in evidence development" (CED) policy. As previously noted, Medicare applies CED when there is insufficient clinical evidence that an item or service is reasonable and necessary for the treatment of an illness or a disease. In 2022, CMS established CED for anti-amyloid monoclonal antibody drugs for the treatment of Alzheimer's disease.[26] Under this policy, the Secretary would apply a payment cap on such drugs approved under accelerated approval and subject to CED until they convert to traditional approval. Medicare's Part B payment rate would revert to current law once the manufacturer's postmarketing confirmatory trials confirm the drug's clinical benefit or once the Secretary withdraws the CED policy.

As part of this policy, the Congress would prescribe very limited circumstances in which the Secretary could waive the payment cap—for example, circumstances outside a manufacturer's control, such as a public health emergency that significantly affects patient recruitment. For all three circumstances in which the Secretary is required to apply a payment cap (based on the status of a drug's confirmatory trial and Medicare's CED requirements), the Secretary could consider a drug's financial impact on beneficiaries and taxpayers. To reduce the administrative burden on CMS, the Secretary could waive the cap for drugs with a very small financial impact on beneficiaries and taxpayers (e.g., $100,000 in a given year). When spending for such a drug exceeds the dollar threshold, the Secretary would establish a cap.

However, policymakers should develop clear and consistent criteria for any waivers of the policy cap. Unless carefully designed, such waivers could undo the policy's intent to ensure that the Medicare program is not overpaying for products approved on an accelerated basis if a product's clinical benefit is not confirmed and that manufacturers have an incentive to complete postmarketing confirmatory trials on a timely basis. In addition, clear and consistent criteria would help support innovation by reducing uncertainty for manufacturers.

Applying a cap on accelerated approval drugs with a price that is excessive relative to upper-bound estimates of value

Under the recommendation, the Secretary would have the authority to cap the payment at launch for selected accelerated approval drugs with an excessive price relative to the upper-bound estimates of value for Medicare beneficiaries. Such a policy would balance the goal of providing access to beneficiaries for needed medicines while protecting beneficiaries and taxpayers from the manufacturer setting an excessive price relative to the drug's upper bound of estimated value.

Some have raised concerns that giving the Secretary such flexibility might have an adverse impact on research and innovation and might lead to manufacturers being uncertain about whether to use the FDA accelerated approval pathway, which together might outweigh the benefits of Medicare acting as a prudent purchaser. In recognition of that concern, the Commission envisions that the Secretary would apply this policy sparingly, so that it serves as a safeguard available to the Medicare program in rare circumstances to manage products with an excessive price and small net clinical benefit (as assessed in health technology assessments outlined in the text box on pp. 29–31) compared with the standard of care that would result in a substantial budget impact on beneficiaries and taxpayers.[27] Examples of accelerated approval drugs whose pricing has been judged to be high relative to their net clinical benefit include:

- Aduhelm, for the treatment of Alzheimer's disease. The manufacturer originally priced the product at $56,000. Based on an assessment of this product (using an optimistic treatment benefit scenario) compared with the standard of care (supportive care), researchers concluded that this price was substantially above the estimate of a value-based price for the product; to achieve a cost-effectiveness threshold of $100,000 and $150,000 per equal value of life years gained, the

TABLE 1–5 How should the cap on payment be determined?

Approach	Ease of implementation	Advantages/disadvantages
A drug's net clinical benefit and cost compared with the standard of care	Requires identifying the standard of care and evidence on outcomes and costs for the new drug and the standard of care	Would best capture the new drug's potential effect on beneficiaries' outcomes
Some increment (e.g., 100 percent or less) of the payment rate under current law for the standard of care	Requires identifying the standard of care	Does not account for the new drug's potential to improve outcomes compared with the standard of care
A fixed percentage discount off the drug's payment rate under current law	Easiest to implement because the cap is based on a percentage of the manufacturer's launch price for the new drug	Might incentivize manufacturers to launch at higher prices Does not account for the new drug's potential effect on beneficiary outcomes

Source: MedPAC.

manufacturer's price of $56,000 per year would have to be discounted by between 60 percent and 74 percent (Institute for Clinical and Economic Review 2021).

- Folotyn, for treatment of peripheral T-cell lymphoma. At the time of its approval, many observers raised concerns about Folotyn, particularly about its high price relative to other therapies (roughly three times the monthly cost of other available chemotherapy products) and the lack of evidence about its clinical benefit (Pollack 2009).

Implementation issues for Medicare regarding a payment cap

There are two key implementation issues for Medicare to consider in setting a cap on a new drug's Part B payment rate: how to set the cap on a drug's payment rate and how to operationalize the cap. Medicare would need to develop a clear, transparent, timely, and predictable decision-making framework that ensures transparency and opportunities for public input. For example, Medicare should obtain input from a wide range of stakeholders in listening sessions and a formal public comment period when first developing its general methods for setting the cap on a drug's payment rate and operationalizing the cap.

Determining the payment cap A key design issue is how Medicare would determine the payment cap for a particular drug (Table 1-5). One approach would be for the Secretary to set a cap based on the accelerated drug's net clinical benefit and cost compared with the standard of care.

Such an approach would enable Medicare to set a higher cap for drugs that have a greater expected benefit, unlike a cap based on a percentage (100 percent or less) of the payment rate under current law for the standard of care or applying a fixed percentage discount off the drug's payment rate under current law. A cap based on a drug's net clinical benefit recognizes important and transformative therapies (which, in turn, incentivizes the development of better drugs) while ensuring that beneficiaries and taxpayers do not overpay; that is, Medicare's payment is at a rate that is not deemed excessive relative to the drug's expected benefit.

A clear, public, predictable, transparent, and timely process will need to be established for Medicare to

assess a drug's net clinical benefit and cost compared with the standard of care. For Medicare's payment and coverage determinations, CMS has developed methods to assess a new technology's clinical benefit (see text box on setting payment caps relative to a drug's comparative clinical effectiveness and cost). Specifically, the Secretary would need to develop a standard set of methods, informed by public input, that could be used across products to assess their clinical and economic outcomes, including approaches to (1) determine the standard of care and (2) assess the costs and health outcomes of the accelerated approval drug and the standard of care. In the Commission's June 2005 report to the Congress, we concluded that Medicare could play an important role in advancing the field of cost-effectiveness—an approach that could be used to compare the relative costs and benefits of alternative interventions—by helping to standardize the methods in these analyses in an open process (Medicare Payment Advisory Commission 2005). The Secretary could look for opportunities to harmonize the methods that Medicare uses across different policies that assess a product's or service's clinical evidence. In addition, as part of the process to set the payment cap, we envision that the drug's manufacturer, along with other stakeholders, would have the opportunity to provide clinical and cost (i.e., pricing) information about the new drug.

Another approach would base the cap for a new drug on a percentage of the price for the standard of care. Such an approach would be somewhat easier to implement because it would not require evidence about a new drug's outcomes and costs compared with one or more identified standards of care. While this feature would make the policy more straightforward to implement, basing the cap on the price of the standard of care would not account for a new product's potential for a greater clinical benefit than the standard of care.

A third approach would base the cap on a fixed percentage of the manufacturer's ASP for the new product. This approach would be the easiest to implement, but manufacturers may respond to such a policy by increasing their launch price (ASP) to partially or fully offset the effect of the cap. Under this approach, manufacturers could continue to price the product as high as the market will bear, despite the limited evidence underlying the product's clinical benefits. Thus, this method could result in a higher payment rate than under the other two alternatives, and that payment rate could have no relationship to the drug's expected clinical benefit.

In most instances, sources of evidence will be available for the Secretary to establish a cap based on the accelerated approval drug's net clinical value compared with the standard of care. As discussed in the text box, sources of evidence to conduct health technology assessments include data from clinical trials submitted by manufacturers for FDA approval and meta-analyses of the new drug and the standard of care. In those few instances in which sufficient data are not available to conduct such assessments, the Secretary could have the flexibility to cap the new drug's payment based on a percentage of the price for the standard of care (e.g., 100 percent or less), applying a fixed discount to the drug's payment rate under current law, or some combination of both approaches.

In situations where an accelerated approval drug fails to demonstrate clinical benefit in a postapproval clinical trial, the Secretary should use a method to set the cap that best aligns the drug's payment to its clinical benefit (as assessed by the results of the failed confirmatory study and other relevant peer-reviewed clinical studies). With the failure to find a clinical benefit over the standard of care, setting the cap based on a percentage of the price for the standard of care could be a reasonable, practical approach in these circumstances.

How to operationalize the cap The payment cap could be operationalized using a rebate under which manufacturers would reimburse Medicare for the difference between the Medicare payment amount and the cap based on claims utilization for the accelerated approval diagnosis. This rebate approach is used for Part B drugs beginning in 2023 to implement the manufacturer discarded drug refund and inflation rebate policies.[28] Providers would enter the diagnosis code for the product's clinical indication (as specified by CMS), which is consistent with information they already report on drug claims. Thus, under this approach, the total payment the provider receives for the drug (i.e., the combined Medicare

Set a payment cap based on a new drug's comparative clinical effectiveness and cost

One approach to setting a cap on a drug's payment rate could be based on the new drug's net clinical benefit and available cost information compared with the standard of care. Cost-effectiveness analysis (CEA) is one approach that considers evidence on a product's net clinical effectiveness and cost compared with the standard of care. CEAs assess trade-offs involving benefits, side effects, and costs inherent in alternative options by measuring the effect (outcome) of a medical intervention in terms of the quantity of health gained. The results of CEAs are typically summarized in a series of incremental cost-effectiveness ratios that show, for one intervention compared with another, the cost of achieving an additional unit of health (outcome). To estimate expected health effects and costs, CEAs require data on each treatment's clinical effectiveness, health outcomes, and health care resource use and costs. The results of such an analysis of comparative clinical effectiveness and cost-effectiveness could inform the payment cap for the accelerated approval drug (Sachs et al. 2022).

CMS has current experience in conducting analyses that assess a new technology's net clinical benefit compared with the standard of care. For example, on an annual basis for the inpatient and end-stage renal disease prospective payment systems, the agency assesses whether new technologies meet certain criteria, including substantial clinical improvement compared with the standard of care, to qualify for new technology add-on payments. Under the national coverage determination process, CMS reviews the clinical evidence for the technology in question and has the option to sponsor a technology assessment—a systematic analysis of the performance characteristics, safety, effectiveness, outcomes, and appropriateness of a service—from an external entity such as the Agency for Healthcare Research and Quality. In several instances, when determining coverage for certain preventive services (fecal occult blood tests, computed tomography colonography, and DNA stool testing for colorectal cancer screening), CMS sponsored external groups, including universities, other government agencies, and health care providers (e.g., cancer centers) to conduct technology assessments that assessed the cost-effectiveness of these screening technologies.

For drugs that are first in class, including some accelerated approval drugs, a key design element would be identifying the standard of care—that is, the treatment that is accepted by medical experts as a proper treatment for a certain type of disease and is widely used by health care professionals. For example, it may be feasible to obtain clinical evidence for the drug in question and its standard of care from separate clinical trials. These clinical studies, particularly for accelerated approval drugs, could be small, single-arm designs with limited follow-up. However, health technology assessments of clinical benefits and cost assessments authored by researchers that conduct health technology assessments demonstrate that it is feasible to assess the cost-effectiveness of first-in-class drugs. Examples of researchers identifying the standard of care for first-in-class products include:

- In clinical and cost assessments of Yescarta, a first-in-class CAR–T (chimeric antigen receptor T-cell) agent approved for adults with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy, the standard of care included so-called salvage therapies, such as rituximab, dexamethasone, cytarabine, and cisplatin, and stem-cell transplantation (Choe et al. 2022, Institute for Clinical and Economic Review 2018). In clinical and cost assessments of Aduhelm, a first-in-class disease-modifying anti-amyloid drug for Alzheimer's disease, the standard of care included nonpharmacologic interventions and pharmacologic interventions (Institute for Clinical and Economic Review 2021). Other researchers have also defined the standard of care in a similar fashion when determining the clinical and cost-effectiveness of anti-amyloid agents (Boustani et al. 2022, Ross et al. 2022).

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Set a payment cap based on a new drug's comparative clinical effectiveness and cost (cont.)

Potential sources of clinical evidence for a given treatment include randomized and nonrandomized clinical trials that manufacturers conduct and submit to the Food and Drug Administration for new drug approvals, cross-sectional studies, and meta-analyses (the statistical analysis of the results from more than one trial for the purpose of integrating the findings). CMS uses these sources when making national coverage determinations and in its assessments of an item's or service's clinical benefit under the inpatient, outpatient, and end-stage renal disease prospective payment systems. Organizations that conduct health technology assessments also use these sources when conducting cost-effectiveness assessments.

Other design issues must also be considered, such as:

- The process for identifying the standard of care, or a treatment that is accepted by medical experts as proper for a certain disease and is widely used by health care professionals. Omission of relevant comparators can produce misleading results. For example, researchers may overestimate the cost-effectiveness of an intervention (and underestimate its incremental cost-effectiveness ratio) because the intervention has not been compared with other available cost-effective alternatives (Drummond et al. 2015).

- The method of defining costs. Costs would include direct medical (e.g., cost of medical services to payers and patients) but also could include direct nonmedical (e.g., transportation costs) and non–health care costs (also referred to as indirect costs, such as productivity losses and caregiver burden). The assignment of prices to pharmaceuticals (as well as other medical items and services) to which the new product being evaluated is compared will affect the results and conclusions from CEAs. We envision that the price of the existing drugs under consideration would be based on each product's average sales price or other measures that are net of discounts, rebates, and other price concessions; for newly launched drugs, wholesale acquisition cost could initially be used. As we discussed in our June 2022 report to the Congress, if comparator products are priced high relative to their net clinical benefit, those high prices will carry through into the price determination of the new product (Medicare Payment Advisory Commission 2022b).

- The time horizon. Researchers must choose the period of time to measure a service's costs and outcomes. The time horizon of the analysis should extend far enough into the future to capture important health effects, and the choice of a time horizon should not bias the analysis in favor of one intervention over another (Drummond et al. 2015).

- The uncertainty of clinical events, costs, and outcomes. Sensitivity analyses vary the assumptions of the clinical, cost, and outcome data

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program payment and beneficiary cost sharing) would be unaffected by the cap; the provider would continue to receive the same total payment regardless of indication.[29] Once a manufacturer verified the drug's clinical benefit, the cap on the payment would cease and the payment rate would revert to current law.

The rebate could also be structured to permit the beneficiary to share in the rebate. Similar to how the Medicare Part B drug ASP inflation rebate is structured (under the Inflation Reduction Act of 2022), the beneficiary's cost-sharing percentage could be reduced upfront to a percentage of the drug's ASP that is equivalent to 20 percent of the capped price.[30] Medicare's share of the payment could then be increased to compensate for the lower cost sharing, so that the provider payment is unaffected and Medicare obtains the rebate payment from the manufacturer on the back end.

Set a payment cap based on a new drug's comparative clinical effectiveness and cost (cont.)

to test the robustness of the results, to identify the data elements to which the results are particularly sensitive, and to test the point at which one intervention becomes more costly or more effective than another.

Other design issues specific to CEA are discussed in the Commission's June 2022 report to the Congress, including the perspective of the analysis and the discounting of costs and outcomes (https://www.medpac.gov/document/june-2022-report-to-the-congress-medicare-and-the-health-care-delivery-system/).

There is no exhaustive research on the use of CEAs by commercial payers, pharmacy benefit managers (PBMs), or other purchasers. Nonetheless, reports in peer-reviewed journals and lay press suggest an increasing interest in determining the net clinical benefit and cost-effectiveness of medical interventions. Medical professional societies and other organizations have developed practice guidelines incorporating findings from CEAs (Neumann and Cohen 2015). For example, the American College of Cardiology and the American Heart Association described how both organizations can address the cost and value of care when making guideline recommendations and developing performance measures (Anderson et al. 2014). The sponsorship of nonprofit and for-profit organizations that conduct cost-effectiveness analyses by federal government agencies, commercial payers, purchasers, and PBMs suggests that these organizations are seeking information on the cost-effectiveness of health care services (Glassman et al. 2020, Neumann and Cohen 2015). Medicare organizations that take on financial risk, including Medicare Advantage plans and accountable care organizations, have flexibility in using cost-effectiveness in the design of their medical and pharmacy management programs. Stakeholders have raised concerns surrounding the use of CEA by payers and purchasers. For example, some contend that it could affect beneficiary access. A more detailed discussion of these concerns can be found in our June 2018 report at https://www.medpac.gov/wp-content/uploads/import_data/scrape_files/docs/default-source/reports/jun18_ch10_medpacreport_sec.pdf.

Some manufacturers use cost-effectiveness analysis to predict the price that purchasers will be willing to pay for a new drug (Neumann et al. 2015, Neumann et al. 2005). For example, in setting the launch price of $2.8 million for a new gene cell therapy, the manufacturer said: "When pricing Zynteglo, we took into consideration the therapy's benefit to patients and society, including measures of positive clinical outcomes as well as expected quality of life improvements, health systems' cost savings, and societal impact of patients and families living lives more fully" (Casey 2022).[31] In addition, as one component of their pricing strategy, some manufacturers show the value of a new drug to formulary committees and other purchasers. ■

Spurring price competition by establishing a single ASP-based payment for Part B drugs and biologics with similar health effects

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. For example, after the launch of generic zoledronic acid (a drug used to treat high blood calcium levels, bone metastases, and osteoporosis), the ASP for the branded product and generics assigned to the same billing code declined by roughly 55 percent in four quarters. By contrast, products that are assigned to their own billing code and paid according to their ASP—single-source drugs, 505(b)(2) drugs, originator biologics, and

biosimilars—do not face the same incentives for price competition. In addition, the 6 percent add-on to ASP can create incentives for some providers to choose higher-priced products over lower-priced products (Dusetzina and Mello 2021).

Thus, the current system does not spur competition among therapeutically similar single-source drugs and biologics. Despite the availability of products with similar health effects, several of the top 20 Part B products ranked by expenditure have ASPs that have either remained the same or increased over more than a decade. For example, Orencia and Cimzia, biologics indicated for the treatment of rheumatoid arthritis, have experienced significant ASP growth (5.4 percent per year since 2007 for Orencia and 2.4 percent per year since 2010 for Cimzia) despite the availability of several other biologics for the treatment of rheumatoid arthritis.

In addition, the current system does not always spur competition among originator biologics and their biosimilars. Since the availability of biosimilars, the ASP for some originator biologics has declined. Others, however, do not face much price competition. For example, the originator biologic Rituxan, used to treat cancer and rheumatoid arthritis, has faced biosimilar competition since the fourth quarter of 2019 but has reduced its price, as measured by ASP, by only 14 percent. As of the first quarter of 2023, the payment rates for Rituxan's biosimilars ranged from 39 percent to 60 percent lower than the originator's payment rate. Biosimilars accounted for 59 percent of the market share as of the third quarter of 2023. Addressing the issue of price competition, in 2017 the Commission recommended that the Congress establish consolidated billing codes to pay for a reference biologic and its biosimilars (Medicare Payment Advisory Commission 2017).

Indeed, research suggests that in many therapeutic classes, 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:

- Between 2005 and 2017, the mean cumulative price increase of 24 Part B anticancer drugs was 36.5 percent. Using multivariate regression, researchers reported that new supplemental FDA approvals, new off-label indications, and new competitors did not influence rates of changes in each drug's ASP (Gordon et al. 2018).[32]
- A systematic review of 10 original studies on competition among branded drugs found no evidence of a price-lowering effect of new drug entry on intraclass brand-name products (Sarpatwari et al. 2019).
- The mean annual increase in the net prices (measured using data from SSR Health) of drugs (available in January 2007) in six therapeutic classes was 4.5 percent between 2007 and 2018.[33] When the authors included drugs that entered the market after 2007, the estimates for net price increases rose (Hernandez et al. 2020).

One reason some new drugs that are not first in class have not experienced price competition could be that lowering prices has not historically resulted in selling more units of a drug. Instead, some manufacturers with lower market share in a given therapeutic class have raised their drug's price to make up for lost market share. Drugs in the class with larger market shares can, in turn, follow with price hikes (Herper 2020). According to San-Juan-Rodriguez and colleagues, the rising prices for existing products could reflect manufacturers' opportunism in response to new, higher-priced agents (San-Juan-Rodriguez et al. 2019).

Internal reference pricing is a tool that some payers use to spur price competition among therapeutically similar drugs (and other medical services) to lower the average price paid. Under such a policy, a payer establishes the price (reimbursement rate) that it is willing to pay for a group of drugs with similar health effects—the reference price, which is typically based on the payer's own prices.

There is substantial precedent for the use of policies to spur price competition among drugs with similar health effects. Health plans and pharmacy benefit managers (PBMs) commonly use policies such as formularies and step therapy to spur price competition among products with similar health effects. Medicare's use of internal reference pricing would pursue the same objective—more price competition among drugs with similar health effects—and would not be expected to deter innovation. Furthermore, as others have noted, spurring price competition among products with similar health effects could increase the relative profitability of a new drug versus me-too products, potentially increasing incentives for the development of new innovative drugs (Sachs and Frakt 2016). Some observers have raised

concerns that reference pricing could have an adverse impact on access if a clinician were unwilling to supply expensive drugs that were coded with and paid the same rate as other, less expensive products. However, though reference pricing would create incentives for clinicians to use the lower-priced products within a code, the clinician would continue to have the choice to select the product most appropriate for the patient. Further, if the reference price were established based on the weighted average price for drugs in the reference group, providers would earn a profit when choosing the lower-priced product, which could help to offset the additional cost of using the higher-priced product if needed for a particular patient. In addition, a payment exception process can be employed in limited circumstances to reimburse a provider based on the ASP of the higher-priced product if the clinician provides justification that the product is medically necessary, such as in instances in which there has been documented clinical failure of a lower-priced alternative.

Internal reference pricing approaches are frequently used by other countries (Australia, Canada, Japan, and many European countries).[34] As of 2017, 22 of 28 European Union member states used internal reference pricing (Vogler et al. 2017). An earlier study reviewing drug pricing policies used in 20 European countries reported that in 2011, 16 European countries used internal reference pricing. Of these 16 countries, 8 defined reference groups based on a product's active substance while another 8 had a broader classification system that defined groups of drugs based on therapeutic classes (Dylst et al. 2012).

In the past, Medicare used internal reference pricing policies to pay for Part B drugs, but it no longer does so. Between 1995 and 2010, Medicare implemented two reference pricing policies—referred to as the least costly alternative (LCA) and functional equivalence policies—to pay for groups of drugs with similar health effects (prostate cancer drugs and anti-anemia biologics). Since 2010, because of judicial 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 (MMA) requires that biologics and single-source drugs (without generic competition) be paid based on their ASP and not averaged with other products' ASPs, a change in the statute would be necessary. Consequently, the Secretary would require statutory authority to apply either reference pricing (or consolidated billing policies) to groups of drugs with similar health effects. (A detailed description of Medicare's prior application of reference pricing approaches can be found in our June 2019 report at https://www.medpac.gov/wp-content/uploads/import_data/scrape_files/docs/default-source/reports/jun19_ch3_medpac_reporttocongress_sec.pdf.)

Using reference pricing to establish a single ASP-based payment rate for groups of drugs with similar health effects would likely reduce spending for Part B drugs

The Commission has long held that Medicare should pay similar rates for similar care. As such, this principle might warrant that Medicare Part B use reference pricing, an approach that sets a single reference price for products with similar health effects that are currently assigned to their own billing codes. Such an approach would spur price competition among products with similar health effects. Compared with other drug management strategies (e.g., formularies), reference pricing does not restrict the selection of drugs within a given therapeutic class. By contrast, Medicare Advantage (MA) plans have several mechanisms to promote more efficient prescribing of Part B provider-administered drugs—through use of prior authorization and contracting arrangements that direct enrollees to more efficient sites of care. Anderson and colleagues noted that in four clinical scenarios where similarly or equally effective Part B drugs exist and are substantially different in terms of cost, older adults with MA coverage who receive treatment for the given condition more often receive the low-cost drug alternative compared with older adults with FFS coverage (Anderson et al. 2021).

Under reference pricing policies for Part B drugs, manufacturers would have incentive to lower their prices relative to competitors to make their products more attractive to providers and garner market share. Federal government agencies have estimated that applying reference pricing policies to Part B drugs would result in savings for beneficiaries and taxpayers.

- OIG estimated that using an LCA policy in 2008 and 2009 to pay for drugs that treat wet age-related macular degeneration (Avastin and Lucentis) would have saved beneficiaries $275 million and Medicare $1.1 billion (Office of Inspector General 2011).

- CBO projected that if Medicare had used an LCA policy between 2010 and 2019 for drugs that treat osteoarthritis of the knee, the program would have saved almost $500 million (Congressional Budget Office 2008).
- OIG has twice recommended that the Secretary apply LCA policies to prostate cancer drugs paid under Part B.[35] In 2004, OIG estimated that if Medicare's contractors had applied LCA policies to all applicable prostate cancer drugs, beneficiaries and taxpayers 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 Part B drugs that treat prostate cancer because of judicial rulings and statutory changes in 2010, utilization shifted in favor of costlier products; if an LCA policy had been in place, OIG estimated that Medicare would have achieved one-year savings of nearly $27 million and nearly $7 million for beneficiaries (Office of Inspector General 2012).

Researchers have also estimated significant savings from reference pricing:

- Dickson and colleagues estimated Medicare savings of $7 billion for setting a "domestic reference price" for new drugs based on the payment rates of three existing drugs that are clinically comparable (i.e., of similar therapeutic class, mechanism of action, and indication) (Dickson et al. 2021). The domestic reference price would be calculated as the inflation-adjusted launch price of its comparators, weighted by the relative utilization of each comparator, and adjusted by an innovation premium based on the average time since approval for comparators. Under their approach, the domestic reference price of the 66 drugs analyzed was not always lower than the launch price of the new drug. However, across all Part B and Part D drugs, the researchers estimated that this approach would have yielded Medicare savings of $7 billion between 2015 and 2019.
- After adjusting for sociodemographic and clinical characteristics and regional effects, Anderson and colleagues found that, compared with FFS beneficiaries, MA enrollees were more likely to receive the lower-cost drug in four therapeutic drug classes (Anderson et al. 2021). The authors estimated that if FFS use aligned with MA prescribing patterns, FFS spending (in 2016 dollars) would be reduced by (1) $204 million for anti-vascular endothelial growth factor used to treat macular degeneration (representing 8 percent of FFS spending for this drug group), (2) $28 million for bone resorption inhibitor treatment of osteoporosis (representing 6 percent of FFS spending for this drug group), (3) $101 million for bone resorption inhibitor treatment of malignant neoplasms (representing 20 percent of FFS spending for this drug group), and (4) $6 million for intravenous iron treatment of anemia (representing 7 percent of FFS spending for this drug group).
- The Committee for a Responsible Federal Budget proposed "clinically comparable drug pricing," under which Part B drug payment would be set at a single price for groups of drugs within the same therapeutic class (Committee for a Responsible Federal Budget 2021). For any such group, Medicare would set the payment for all drugs at a volume-weighted average price, which would be calculated quarterly using each product's quarterly ASP, weighted by the average annual usage of each product, and amortized based on each drug's standard dosing. The researchers estimated that for drugs that treat macular degeneration, rheumatoid arthritis, and prostate cancer, their policy would reduce Medicare FFS spending between 2021 and 2030 by $81 billion and result in $29 billion in savings for the MA program. Most of these estimated savings come from the macular degeneration and rheumatoid arthritis groups, due to the high price differential for the drugs in these groups and their significant use among FFS beneficiaries.

Establishing a single ASP-based payment rate for groups of drugs and biologics with similar health effects

To promote price competition, Medicare should establish a single ASP-based payment rate for groups of drugs and biologics with similar health effects. The Congress would need to give the Secretary the authority to apply internal reference pricing approaches to Part B drugs. Reference pricing would not be applied to all Part B drugs; rather, the Secretary would consider the ease of implementing reference

pricing and apply the policy to groups of products that may have similar indications.

RECOMMENDATION 1-2

The Congress should give the Secretary the authority to establish a single average sales price–based payment rate for drugs and biologics with similar health effects.

RATIONALE 1-2

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. By contrast, products that are assigned to their own billing code and paid according to their ASP—single-source drugs, 505(b)(2) drugs, originator biologics, and biosimilars—do not face the same incentives for price competition. Thus, the current system does not spur competition among therapeutically similar single-source drugs and biologics or among originator biologics and their biosimilars. This recommendation builds on the Commission's June 2017 recommendation to apply a reference pricing policy to pay for biosimilars and originator biologics. Establishing a single ASP-based payment to drugs and biologics with similar health effects is consistent with the Commission's long-held position that Medicare should pay similar rates for similar care.

IMPLICATIONS 1-2

Spending

- This recommendation would decrease federal program spending relative to current law. We envision that the Secretary would first focus on applying reference pricing to those groups for which all of a given product's indications could be included in the group. The Secretary could begin with those groups for which implementation would be the most straightforward: (1) biosimilars and originator biologics, (2) 505(b)(2) drugs and related brand-name and generic drugs, and (3) drugs for which reference pricing has been implemented or considered previously (including erythropoietin-stimulating agents and viscosupplements for the treatment of osteoarthritis of the knee). In most instances, the Secretary could set the reference price based on the volume-weighted ASP of drugs assigned to the reference group.

Beneficiary and provider

- The recommendation is expected to generate savings for beneficiaries through lower cost sharing. The policy would not be expected to adversely affect beneficiaries' appropriate access to needed Part B drugs. Payments to providers are expected to decrease through increased price competition of drugs and biologics with similar health effects, but profitability might increase (due to the two-quarter lag in ASP payment rates and declining prices and providers choosing the lower-priced product). This recommendation is not expected to affect providers' willingness and ability to serve beneficiaries.

Implementation issues for Medicare regarding reference pricing

A reference pricing policy would require establishing a transparent and predictable process that permits opportunities for public comment. Key issues that Medicare would need to consider in applying reference pricing to Part B drugs include setting the payment rate, defining reference groups, and establishing a process for medical exceptions.

Setting the payment rate There are several ways for Medicare to determine the reference price.

- Under method 1, the reference price would be calculated using the same volume-weighted approach that CMS currently uses when determining the payment rate for generic drugs and their associated brand drug assigned to a single billing code. In 2016 and 2017, CMS used a similar volume-weighted approach to pay for all biosimilar products associated, but not grouped, with a given reference biologic.

- Under method 2, also an approach that CMS currently uses, the reference price would be based on the lower of (1) the volume-weighted ASP of all drugs within the reference group or (2) the ASP for the individual drug.[36]

- Under method 3, the reference price could be based on the payment rate of the least costly product within the reference group. CMS used such an approach to pay for prostate cancer drugs and anti-anemia drugs between 1995 and 2010.

An advantage of basing payment on the volume-weighted ASP (as done in method 1) compared with the

TABLE 1–6

Illustrative example of reference pricing using a weighted average approach for a hypothetical group of three drugs

		(1)	(2)	(3)	(4)	(5)	(6)	(7)	(8)
		Number of 10 mg billing units		ASP + 6%		Number of:		Reference price (ASP + 6% per billing unit that equalizes cost of year of therapy)	Cost of year of therapy at new payment rate (2) × (7)
	Dosage	Per admin	Per year	Per billing unit	Per year of therapy (2) × (3)	Billing units from Medicare claims	Year-equivalent billing units (5) / (2)		
Drug A	20 mg every 2 weeks	2	52	$15	$780	300,000	5,769	$12.65	$658
Drug B	10 mg every 1 week	1	52	$10	$520	500,000	9,615	$12.65	$658
Drug C	10 mg every 1 week	1	52	$25	$1,300	50,000	962	$12.65	$658

Note: ASP (average sales price), admin (administration). Illustrative example of reference pricing for three hypothetical drugs with similar health effects using a weighted average approach to setting the reference price. All products, prices, and utilization are hypothetical.

Source: MedPAC-constructed illustrative example.

other options is that it would more likely give providers time to adjust to the new payment rates without creating financial disruption, especially for practices that already purchased the higher-priced drug before the policy went into effect. Furthermore, the volume-weighted approach is similar to how Medicare handles payment for brand drugs with generic equivalents, whose prices have significantly declined over time due in part to this approach.

While a weighted average approach has a number of advantages overall, there may be circumstances where it would be beneficial for the Secretary to also have discretion to set the reference price using method 2—that is, the lesser of the volume-weighted ASP for the group of products or the individual product's ASP. If extremely large price differences existed between therapeutic alternatives (e.g., a low-priced drug that has experienced generic entry and an expensive single-source brand drug), using a weighted average reference price could lead to a very large increase in the low-priced drug's payment rate. If the Secretary had the option to apply method 2 in this circumstance, more efficient payment rates could result. An example of this approach occurred with albuterol and levalbuterol in 2007, when the Congress legislated use of the lesser-of approach to set Medicare's payment rate.[37]

To determine the reference price using the volume-weighted average, Medicare could weight each drug's ASP-based payment according to its utilization under Part B—that is, by the number of units of each drug obtained from Part B Medicare claims data. Thus, the reference price would be influenced over time by Medicare beneficiaries' use of each drug.

Some reference groups may be composed of products that vary in dosage size and frequency of administration. In calculating the reference price across products, Medicare would need to establish

a method to determine an equivalent dose across products. Potential sources of dosing information include the drug's FDA label dosing, actual use by Medicare beneficiaries derived from Part B drug claims data, or a combination of data from both sources. To implement reference pricing, we envision that the Secretary would be given the authority to determine the equivalent units when the dosing units among drugs in a given reference group are different.

To illustrate how reference pricing could work, Table 1-6 presents an example of three hypothetical drugs with similar health effects. This example assumes that the reference price is established based on the volume-weighted ASP across the products (method 1). In this hypothetical example, the three products with similar health effects have different dosing schedules. Drug A is administered every other week at a dose of 20 mg while drugs B and C are administered weekly at a dose of 10 mg. To account for the products' different dosing schedule, we calculate their price (ASP + 6 percent) for a year of therapy (column 4). Across the three products, the price per year of therapy ranges from a low of $520 (drug B) to a high of $1,300 (drug C). To create a weighted average payment rate across the products, we use Medicare claims data to determine the number of billing units of each drug furnished and convert these billing units into years of therapy equivalents. Next, using data for all the products, we calculate the weighted average price (ASP + 6 percent) per treatment year ($658).[38] Next, for each product, we calculate the billing code–level payment rate (column 7) that would result in a payment amount of $658 for each product per year of treatment (column 8). Comparing the original payment rates (column 3) and the reference-priced payment rates (column 7), the table shows that, under reference pricing, the payment rate per billing unit would decline for drug A (from $15 to $12.65) and drug C (from $25 to $12.65) and increase for drug B ($10 to $12.65).

The example in Table 1-6 is static, reflecting the first quarter when reference pricing is undertaken. Over time, we would expect the reference price to decline, as volume shifts to the lower-priced product and manufacturers of higher-priced products have the incentive to lower prices.

Defining reference groups Medicare would need to develop a process for defining groups of drugs with similar health effects. For example, such a process could organize reference groups by clinical indications and drug classifications and could include Medicare Part B–covered drugs and biologics that:

- have similar FDA-approved indications or off-label use according to Medicare claims data or medically accepted (compendia-listed) off-label use;
- work in a similar way (e.g., same drug classification, mechanism of action); and
- are listed similarly by clinical guidelines (e.g., classification of products, recommended vs. not recommended).

Medicare's efforts to define reference groups would be similar to what health plans and PBMs commonly do when they identify a group of therapeutic alternatives for the purpose of developing a formulary or fail-first/step therapy policies. For example, for certain drugs (including viscosupplements and targeted immune modulators), Aetna covers and pays for the more costly product only for patients who have a contraindication, intolerance, or ineffective response to the less costly product. While Medicare Part B would be identifying drugs with similar health effects for a different purpose (to determine which products should be paid a similar rate), the processes utilized by health plans and PBMs to identify similar drugs could have applicability for Medicare. For example, like health plans and PBMs, Medicare Part B could develop its own pharmacy and therapeutics committee to advise it on the definition of particular reference groups. CMS could also consider seeking a technology assessment from groups with clinical expertise. Such processes would need to be clear and transparent and provide opportunities for public comment from beneficiaries, clinical experts, and others.

In defining reference groups, Medicare could consider the ease of implementing reference pricing. It would be relatively straightforward to define groups of products that have similar indications. Each drug in the reference group would be paid based on its reference price across all uses. However, in some cases, drugs that are therapeutic alternatives and candidates for inclusion in a reference group may not have the same universe of indications. If substantial differences existed in indications across products that are therapeutic alternatives, Medicare could consider establishing reference groups for specific indications

TABLE 1–7

Medicare's payment system for originator biologics and their biosimilars results in wide price variation across similar products

Originator biologic and biosimilars	Number of products (originator biologic and biosimilars)	Estimated range of payment rates across an originator biologic and its biosimilars: Average annual payment per beneficiary per year based on first-quarter 2023 ASP payment rates and same annual average dose		
		Lowest-priced product	Highest-priced product	Yearly difference between lowest- and highest-priced product
Neupogen	4	$729	$3,001	$2,272
Remicade	4	7,697	14,111	6,414
Neulasta	5	4,212	6,867	2,656
Procrit/Epogen	2	2,369	2,424	54
Avastin*	3	15,489	34,576	19,086
Herceptin	6	13,022	32,456	19,434
Rituxan	4	9,138	22,856	13,718
Lucentis	2	6,857	6,893	36

Note: ASP (average sales price). Yearly difference between lowest- and highest-priced product is calculated using unrounded figures.
*The estimated annual price for the originator biologic Avastin and its biosimilars is based on the average dose for non-ophthalmological indications (e.g., cancer diagnoses). The dosing for non-ophthalmological indications is much larger than for ophthalmological indications, and the vast majority of Avastin biosimilar administrations in 2021 were for non-ophthalmological indications.

Source: MedPAC analysis of Medicare ASP payment rate files for first quarter 2023 publicly available on CMS website and Medicare claims data for physicians and outpatient hospitals.

or groups of indications. However, such an approach would result in indication-specific pricing, an approach under which payers establish a price for each drug's clinical indication. Indication-specific pricing is not used under the ASP-based payment rate for Part B drugs. Rather, Medicare pays one ASP for all of a drug's clinical indications. Adopting an indication-specific policy would likely be complex to administer. For example, some drugs are distributed and purchased without knowledge of their ultimate use, which makes it difficult to link prices with indications (Pearson et al. 2017).

Consequently, we envision that the Secretary could first focus on applying reference pricing to those groups for which all of a given product's indications could be included in the group. The Secretary could begin with those groups for which implementation would be the most straightforward: (1) biosimilars and originator biologics, (2) 505(b)(2) drugs and related brand-name and generic drugs, and (3) drugs for which reference pricing has been implemented or considered previously (including erythropoietin-stimulating agents and viscosupplements for the treatment of osteoarthritis).

Applying reference pricing to pay for an originator biologic and its biosimilars The use of reference pricing for originator biologics and biosimilars would promote price competition and eliminate the wide variation in the price that Medicare and beneficiaries currently pay for similar products. As shown in Table 1-7, prices vary widely among products with biosimilar competitors. For example, the amount that Medicare and beneficiaries would pay for the originator biologic Avastin and its three biosimilar competitors (with the

Drugs approved under the 505(b)(2) pathway

A 505(b)(2) application is a type of new drug application (NDA) that contains full reports of investigations of safety and effectiveness, at least some of which come from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. In some cases, drugs approved under Section 505(b)(2) share significant portions of labeling with generic drugs that are paid as multiple-source drugs under Section 1847A of the Social Security Act. The 505(b)(2) pathway is a hybrid between the generic approval process (under 505(b)(j)) and a full NDA under 505(b)(1). CMS proposed but did not finalize a proposal to revise the definition of a multiple-source drug in regulation text by amending the applicable regulatory text to state that multiple-source drugs may include drugs described under Section 505(b)(2) (Centers for Medicare & Medicaid Services 2020). CMS indicated that some stakeholders expressed concern about potential payment reductions and about reduced incentives for innovation of and access to 505(b)(2) drugs, while others expressed support for the proposal.

As of January 2023, CMS assigns many products approved under the 505(b)(2) pathway to their own billing code, rather than grouping them in a multiple-source billing code with other similar brand and generic versions of the drug. The phenomenon of separate billing codes and payment rates for 505(b)(2) products stems from CMS's effort to identify 505(b)(2) products that should receive a separate billing code based on the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (Centers for Medicare & Medicaid Services 2023). Beginning in January 2023, CMS established new separate billing codes for many 505(b)(2) products that were paid under a shared billing code in 2022, including bortezomib, calcium gluconate, cefazolin sodium, cefepime hydrochloride, daptomycin, decitabine, fulvestrant, glucagon hydrochloride, linezolid, meropenem, micafungin, midazolam, morphine, moxifloxacin, triamcinolone acetonide, and vancomycin (Table 1-8, pp. 40–41).

The establishment of manufacturer-specific codes for 505(b)(2) products has led to wide variation in Medicare's payment rates for some 505(b)(2) and related brand-name and generic products. Pemfexy, a 505(b)(2) version of the brand drug Alimta (chemical name pemetrexed), is an example of this variation. In the Food and Drug Administration's (FDA's) review of Pemfexy's application for 505(b)(2), the FDA states, "Pemfexy is a ready-to-dilute liquid intravenous formulation which was designed to eliminate the reconstitution step of the Reference Listed Drug (RLD) Alimta. Pemfexy is expected to have the same efficacy and safety profile" (Food and Drug Administration 2019a, Food and Drug Administration 2019b). In the first half of 2022, Alimta's marketing exclusivity expired and generic forms of pemetrexed and the 505(b)(2) Pemfexy entered the market. As of January 2023, Alimta and its generic equivalents are paid under the same billing code at a rate of about $28 per 10 mg (based on the volume-weighted average sales price for the brand and generic) (Table 1-8, pp. 40–41). In contrast, Pemfexy is paid three times more, $82 per 10 mg, because it is paid under its own billing code. In another example, as of January 2023, Medicare pays different rates for bortezomib (brand and generics J9041) and its three 505(b)(2) drugs (J9046, J9048, and J9049).

(continued next page)

same average annual dose for non-ophthalmological indications) would vary by $19,000 per beneficiary depending on whether the lowest- or highest-priced version of the product were administered. Across the eight Part B products with biosimilars, the highest-priced product for four of the products was at least double the price of the lowest-priced product; for two products, the highest-priced product was at least 50 percent more expensive than the lowest priced. In June 2017, the Commission recommended that Medicare pay originator biologics and their biosimilars the same average rate using a combined billing code, a

Drugs approved under the 505(b)(2) pathway (cont.)

TABLE 1–8

Wide price variation among drug groups with branded, generic, and 505(b)(2) products

Billing code	Drug type	Billing unit	ASP for Q1 2023
Injection, acetaminophen			
J0131	Generics only	10 mg	$0.101
J0134	505(b)(2)	10 mg	0.139
J0136	505(b)(2)	10 mg	0.049
Bortezomib			
J9041	Brand and/or generics	0.1 mg	9.011
J9046*	505(b)(2)	0.1 mg	10.959
J9048*	505(b)(2)	0.1 mg	2.677
J9049	505(b)(2)	0.1 mg	7.130
Calcium gluconate			
J0610	505(b)(2)	10 ml	5.168
J0611*	505(b)(2)	10 ml	1.689
Cefazolin sodium			
J0689*	505(b)(2)	500 mg	1.162
J0690	Generics only	500 mg	0.730
Cefepime hydrochloride			
J0692	Generics only	500 mg	1.287
J0701*	505(b)(2)	500 mg	5.431
J0703*	505(b)(2)	500 mg	5.062
Daptomycin			
J0877*	505(b)(2)	1 mg	0.066
J0878	Brand and/or generics	1 mg	0.048
Decitabine			
J0893*	505(b)(2)	1 mg	1.576
J0894	Brand and/or generics	1 mg	1.276
Fulvestrant			
J9393*	505(b)(2)	50 mg	15.443
J9394*	505(b)(2)	50 mg	7.845
J9395	Brand and/or generics	50 mg	12.661
Glucagon hydrochloride			
J1610	Brand and/or generics	1 mg	173.775
J1611*	505(b)(2)	1 mg	162.012
Linezolid			
J2020	Brand and/or generics	200 mg	3.233
J2021*	505(b)(2)	200 mg	16.433
Melphalan			
J9245	Brand and/or generics	50 mg	220.661
J9246	505(b)(2)	1 mg	16.044
Meropenem			
J2184*	505(b)(2)	100 mg	2.126
J2185	Brand and/or generics	100 mg	0.621

Drugs approved under the 505(b)(2) pathway (cont.)

TABLE 1-8

Wide price variation among drug groups with branded, generic, and 505(b)(2) products (cont.)

Billing code	Drug type	Billing unit	ASP for Q1 2023
Micafungin sodium			
J2247*	505(b)(2)	1 mg	0.240
J2248	Brand and/or generics	1 mg	0.920
Midazolam hydrochloride			
J2250	Brand and/or generics	1 mg	0.157
J2251*	505(b)(2)	1 mg	0.308
Morphine sulfate			
J2270	Brand and/or generics	10 mg	2.461
J2272*	505(b)(2)	10 mg	7.451
J2274	Brand and/or generics	10 mg	9.101
Moxifloxacin			
J2280	Brand and/or generics	100 mg	10.264
J2281*	505(b)(2)	100 mg	9.728
Pemetrexed			
J9304	505(b)(2)	10 mg	81.563
J9305	Brand and/or generics	10 mg	27.681
Triamcinolone acetonide			
J3299	505(b)(2)	0.1 mg	47.612
J3300	505(b)(2)	1 mg	4.185
J3301	505(b)(2) or generics	10 mg	1.060
J3304	505(b)(2)	1 mg	16.954
Vancomycin hydrochloride			
J3370	Brand and/or generics	500 mg	2.856
J3371*	505(b)(2)	500 mg	6.399
J3372*	505(b)(2)	500 mg	6.491

Note: ASP (average sales price), Q (quarter).
*In 2022, CMS paid for the 505(b)(2) product under a shared billing code (i.e., with other brand, 505(b)(2), and generic products).

Source: Medicare ASP payment rate file for first quarter 2023 publicly available on CMS website.

These clinically comparable products are likely candidates for reference pricing. In the clinical review of each product, the FDA concluded that:

- Dr. Reddy's bortezomib (J9046) is qualitatively and quantitatively similar to brand-name Velcade and that "the benefit and risk of Bortezomib for Injection is expected to be the same as that of the listed drug Velcade" (Food and Drug Administration 2019b).
- Fresenius Kabi's bortezomib (J9048) "has the same indication, dosage form, strength, and route of administration (IV) as the innovator drug" (Food and Drug Administration 2013).
- "The Hospira [bortezomib] product is nearly identical to the listed product, as the Hospira product has the same active ingredient and inactive ingredient, is the same dosage form and has the same routes of administration and concentration of bortezomib following reconstitution as the Listed Drug, Velcade" (Food and Drug Administration 2017). ■

form of reference pricing. The creation of a reference group containing only an originator biologic and its biosimilars is straightforward, consistent with the Commission's 2017 recommendation that reference pricing for those products be mandatory.

Applying reference pricing to pay for 505(b)(2) products and related brand-name and generic products As discussed in the text box on 505(b)(2) drugs (pp. 39–41), another potential use for reference pricing is for drugs approved under 505(b)(2), a pathway that is a hybrid between the generic approval process (under 505(b)(j)) and a full new drug application (NDA) (under 505(b)(1)). A 505(b)(2) application is a type of NDA that contains full reports of investigations of safety and effectiveness, but at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. According to researchers, most 505(b)(2) applications consist of changes (e.g., a new dosage form, route of administration, or inactive ingredient) to a previously approved reference drug (Freije et al. 2020). Manufacturers of some 505(b)(2) products may adopt these changes (e.g., different inactive ingredients) to evade patents of the reference drug (Wosinska and Frank 2022). The provisions of 505(b)(2) were created, in part, to help avoid unnecessary duplication of studies already performed on a previously approved ("reference" or "listed") drug. Some stakeholders refer to products approved under the 505(b)(2) pathway as "line extensions" or "505(b)(2) generics" (Centers for Medicare & Medicaid Services 2019, Wosinska and Frank 2022).

Because of statutory provisions in the MMA, Medicare currently assigns a unique billing code and pays for many 505(b)(2) products based on their own ASP rather than placing them in an existing billing code and paying them the same average rate as brand and generic forms of the drug. CMS recently announced that it has identified 505(b)(2) products that should receive a separate billing code based on the MMA, which resulted in a substantial increase in the number of 505(b)(2) products receiving their own manufacturer-specific payment rate in January 2023 (Centers for Medicare & Medicaid Services 2023). As of January 2023, CMS created a manufacturer-specific (unique) billing code for 19 505(b)(2) drugs that the agency, in 2022, assigned to a shared billing code (with related brand and generic versions of the drug) (Table 1-8, pp. 40–41). The establishment of manufacturer-specific codes for 505(b)(2) products has led to wide variation in Medicare's payment rates for some 505(b)(2) products and related brand-name and generic products (see text box on 505(b)(2) approval process, pp. 39–41). Examples of likely candidates for reference pricing include (1) Velcade, its generics, and its three 505(b)(2) products, and (2) Alimta, its generics, and its four 505(b)(2) products, which include Pemfexy and Actavis.

Applying reference pricing to pay for groups of products for which the Secretary already has some current or prior experience Another category of products on which the Secretary could initially focus are those for which the Secretary already has some current or prior experience with reference pricing. One example is viscosupplements that treat osteoarthritis of the knee (e.g., GenVisc 850 and Gel-One), which CBO included as a policy option in its budget options publication (Congressional Budget Office 2008). While most viscosupplement products have their own billing code, a few viscosupplement products (Hyalgan, Supartz, and Visco-3) are currently grouped together in a combined billing code and paid at an average rate, based on a statutory grandfathering provision that required products that were grouped together as of October 1, 2003, to remain grouped together. Under new authority, CMS could apply reference pricing more broadly to products in this therapeutic class rather than to just a small subset of the products. Other groups of drugs with which the Secretary has reference-pricing experience include erythropoietin-stimulating agents (e.g., Epogen and Aranesp) and prostate cancer drugs (e.g., Lupron, Trelstar, Zoladex, Firmagon, and 505(b)(2) products including Camcevi).

Other groups of drugs to apply reference pricing The Secretary could consider reference pricing for other groups of clinically similar products. Examples include anti-vascular endothelial growth factors that treat wet age-related macular degeneration and other conditions (e.g., Eylea, Lucentis); targeted immune modulators that treat rheumatoid arthritis (e.g., Orencia and Rituxan); leukocyte growth factors that stimulate white blood cells (e.g., Neupogen, Granix, Neulasta); iron products (e.g., Injectafer and Feraheme) and products that treat osteoarthritis and bone cancer (e.g., Evenity, Prolia, Xgeva, Zometa). (See Table 4-6 of the Commission's

June 2022 report (at https://www.medpac.gov/wp-content/uploads/2022/06/Jun22_Ch4_MedPAC_Report_to_Congress_v2_SEC.pdf) for additional potential groups and spending implications.)

How Medicare would define groups of products that are clinically similar—narrowly or broadly—is a key design issue. For example, a group could be defined that would broadly apply to both short-acting erythropoiesis-stimulating agents (ESAs) (Epogen and its biosimilar Retacrit) and long-acting ESAs (Aranesp and Mircera). Alternatively, two groups could be defined—one for short-acting agents and another for long-acting agents. More products in a given group will likely lead to greater price competition. Designing groups more broadly would have a greater effect on Medicare spending than groups defined narrowly.

Another issue relates to whether a repackaged drug used for an off-label indication should be included in a given reference group. One example is the off-label use of Avastin, a cancer treatment that is repackaged by compounding pharmacies into smaller doses for treatment of eye disorders, including wet age-related macular degeneration. Medicare may cover off-label use of FDA-approved drugs and biologics if it determines the use to be medically accepted, which the program has done for off-label Avastin use for ophthalmological indications.[39]

Establishing a process for medical exceptions CMS could establish a process for determining exceptions to reference pricing policies when a beneficiary's clinical circumstances support the medical necessity of a more costly product. Our recommendation for establishing consolidated billing codes for the original biologic and its biosimilars discussed the potential for a medical exception process (Medicare Payment Advisory Commission 2017). We said that under such a policy, the clinician would continue to have the choice to prescribe the product most appropriate for the patient, with Medicare's payment based on the reference price. The Congress could consider allowing the Secretary to provide a very limited payment exception process under which Medicare would reimburse the provider based on the ASP of the higher-priced product if the clinician provided justification that the product was medically necessary, such as instances for which clinical failure of a particular product has been documented (Medicare Payment Advisory Commission 2017). A payment exceptions process addresses the concern that beneficiary access under a reference pricing policy could be harmed if some providers were unwilling to supply the higher-cost product to a beneficiary for whom the product was a medical necessity. Providers could submit medical justification to the regional MACs, and the exception process could be coupled with Medicare's existing appeals process that gives beneficiaries, providers, or their representatives the right to appeal the MACs' coverage and payment decisions.

The degree to which a payment exceptions process would be needed may depend on the method chosen to establish the reference price. If a weighted average payment rate (method 1) were chosen, there may be less need for an exceptions process than under the other two methods. With the weighted average approach, clinicians who chose the lower-cost product would be paid at the higher weighted average payment rate and earn additional revenues, which the clinician could use to offset the additional cost of a higher-priced product if needed by a particular patient. In contrast, with the least costly alternative (method 3) or the lesser of a product's own ASP or the weighted average price (method 2), the lowest-cost product would continue to be paid based on its own ASP. Thus, with these two approaches, there are fewer opportunities for the profits associated with the use of less costly drugs to offset any additional costs of more costly drugs. Consequently, an exceptions process might be more important if these two methods were chosen.

Unless carefully designed, a payment exceptions process could create incentives for the use of higher-priced products when the beneficiary's clinical circumstance does not support an exception. Since the add-on of a higher-priced product generates more revenue for the provider than the add-on of a lower-priced product, selection of the higher-priced product could generate more profit, depending on the provider's acquisition costs for the two products. In 2017, the Commission said that to minimize such unintended effects:

- the clinician's payment from Medicare when an exception is granted could be set at the higher-cost product's ASP without an add-on payment (i.e., 100 percent of ASP); and

- the Medicare program would pay the provider 80 percent of the ASP of the exception (higher-cost) product that was furnished, and the beneficiary would pay the provider 20 percent of the exception (higher-cost) product's ASP + 0 percent (Medicare Payment Advisory Commission 2017).[40]

Other issues For a drug newly approved by the FDA, the Secretary would need a clear, transparent, and timely process for evaluating the drug's comparative clinical effectiveness against existing drugs that are the standard of care and for determining whether the drug should be included in an existing reference product group.[41] The Secretary already has experience under the prospective payment systems for inpatient, outpatient, and ESRD services to assess 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.

Additional design elements would be involved in establishing reference pricing policies:

- how frequently the reference price would be updated (e.g., quarterly, annually);
- providing pricing information to beneficiaries and clinicians (to make them sensitive to the difference in out-of-pocket spending); and
- whether Medigap policies could cover beneficiary cost sharing that is greater than the reference price.

Improving financial incentives by modifying add-on payments for Part B drugs and biologics

The percentage add-on payment to Medicare Part B's ASP payment rates has garnered attention because of concern that it may create incentives for use of higher-priced drugs when lower-priced alternatives exist. While clinical factors play a central role in prescribing decisions, at the margins, financial considerations can also play a role in providers' choice of drugs. Evidence from several studies examining utilization patterns for certain products suggests that the percentage add-on to ASP likely affects prescribing patterns in some circumstances. A policy to reduce and restructure add-on payments would improve financial incentives.

Medicare's percentage add-on payment to ASP

Under Section 1847A of the Social Security Act, Medicare pays providers for most Part B drugs at a rate of ASP + 6 percent. In addition to the payment for the drug, Medicare also makes a separate payment for drug administration services under the physician fee schedule or hospital outpatient prospective payment system (OPPS).

The 6 percent add-on is often thought of as the profit margin that providers make on Part B drugs, but the actual profit margin may be greater or less than 6 percent (including possibly negative margins in some circumstances), depending on a variety of factors. If a provider purchases a drug at a price equal to ASP, the profit margin on the drug is 6 percent. A provider may purchase a drug at a price other than ASP for several reasons. Since ASP is an average, some providers will pay more and some will pay less than the average if there is price variation across purchasers (e.g., due to volume discounts). Because of two-quarter lags in the ASP payment rates, the provider's margin is reduced when a drug's price increases (and the margin increases when the drug's price declines) until the ASP payment rates catch up two quarters later. In addition, prompt-pay discounts paid by manufacturers to wholesalers (which are anecdotally reported in the range of 1 percent to 2 percent) can create a gap between ASP and the provider's acquisition costs, as these discounts are subtracted from ASP but are reportedly not fully passed on to purchasers.

As with other Medicare services, the current 2 percent sequester reduces Medicare program payments for Part B drugs. From the perspective of the provider, the statutory payment rate of ASP + 6 percent becomes a net payment of ASP + 4.3 percent after application of the sequester. Because the sequester applies to the Medicare program's payment and not beneficiary cost sharing, the Medicare beneficiary continues to pay 20 percent of ASP + 6 percent and the Medicare program pays 80 percent of ASP + 3.9 percent when

the 2 percent sequester is in effect.[42] The sequester was first implemented in April 2013 but was suspended from May 2020 to March 2022 and reduced to 1 percent from April to June 2022 by the Congress in response to the COVID-19 public health emergency, and then was reinstated at 2 percent beginning July 2022, effective through March 2032.

Information on providers' acquisition costs for Part B drugs is very limited, but a few older studies of certain drugs found that pharmaceutical manufacturers' pricing patterns responded to policy changes. For example, when the ASP payment system was adopted in January 2005, the Commission found evidence suggesting that pharmaceutical manufacturers responded to the new payment system by narrowing the variation in invoice prices across purchasers (Medicare Payment Advisory Commission 2006). In addition, a Commission analysis of IMS Health invoice price data from 2012 to 2015 found evidence suggesting that manufacturers responded to implementation of the sequester in 2013 by changing their pricing to mitigate the effect of the sequester on providers' margins (Medicare Payment Advisory Commission 2016). (See text box in the Commission's June 2022 report to the Congress for a more detailed discussion, https://www.medpac.gov/wp-content/uploads/2022/06/Jun22_Ch4_MedPAC_Report_to_Congress_v2_SEC.pdf.)

There is no consensus on the original intent of the percentage add-on to ASP. Some analysts have suggested that the add-on was intended to cover price variation across purchasers or other factors that can result in a provider's purchase prices exceeding ASP. Another perspective is that the add-on was in part intended to cover costs associated with drug wastage or spillage. However, high-expenditure drugs tend to be packaged in single-use containers, and Medicare pays providers for the wasted amount of drug dispensed from single-use containers.[43] Another view is that the add-on was intended to cover drug storage and handling costs, although it seems unlikely that these costs would vary across products based on a percentage of each product's price.[44] Still others have suggested that the add-on was intended to cover the financing costs associated with maintaining a drug inventory.

Because Medicare Part B covers a diverse set of products ranging in price from very inexpensive to extremely expensive, the size of ASP add-on payments varies widely across Part B drugs. In 2021, about 35 million Part B drug administrations received a 6 percent add-on, and those add-on payments accounted for about $1.6 billion of the total $29 billion in payments for those drugs (excluding drugs acquired by outpatient hospitals under the 340B program).[45] Most Part B drug administrations involve low-cost products with small add-ons. In 2021, nearly half of Part B drug administrations involved an add-on of less than $1; 63 percent of Part B drug administrations involved an add-on of less than $10 (Figure 1-2, p. 46). Examples of products with small add-on payments include corticosteroids injections, vitamin B-12, and contrast agents. However, the bulk of add-on payment spending is concentrated among lower-frequency, high-priced drugs. For example, about 15 percent of drug administrations had an add-on payment exceeding $100, and those administrations accounted for more than 80 percent of add-on spending (Figure 1-2). Furthermore, less than 2 percent of drug administrations had an add-on payment exceeding $500, and those administrations accounted for 25 percent of add-on spending. Examples of products with some of the highest add-ons include CAR–T (chimeric antigen receptor T-cell) products, certain clotting factors, and certain products for rare conditions.

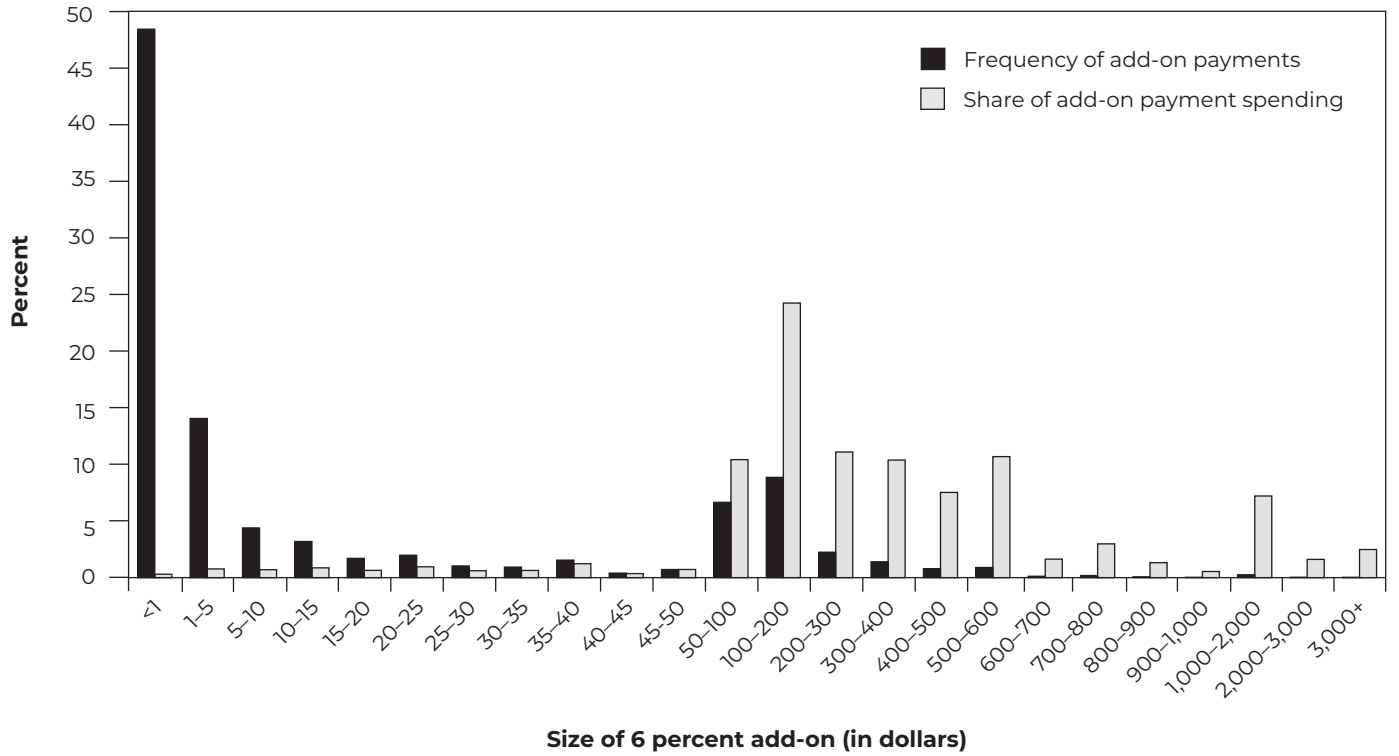
When a provider furnishes a Part B drug, in addition to being paid ASP plus a percentage add-on for the drug, the provider also receives a separate payment for drug administration services. Medicare Part B pays providers for drug administration services under the physician fee schedule and OPPS. For example, under the physician fee schedule in 2023, payment for an injection is about $74 for a chemotherapy product and $14 for a nonchemotherapy product, while payment for the first hour of infusion is $132 for a chemotherapy product and $65 for a nonchemotherapy product. Additional payments are made if more than one drug is furnished or if an infusion lasts longer than the initial hour. Hospital outpatient departments generally receive higher drug administration payment rates than physician offices. In addition, drug administration payment rates may vary based on the location of the injection (e.g., injections in the eye and in the knee).

Medicare's add-on payment for drugs lacking ASP data

For some Part B drugs, CMS lacks ASP data on which to base the drug's payment. A lack of ASP data can occur

FIGURE 1–2

Most Part B drug add-on payments are small, but expensive drugs with large add-on payments account for most add-on spending



Note: Analysis includes all Part B–covered drugs paid under the ASP plus 6 percent system in 2021, excluding drugs billed through not-otherwise-classified Healthcare Common Procedure Coding System codes; drugs furnished by outpatient hospitals that were acquired through the 340B drug pricing program; drugs furnished by critical access hospitals or Maryland hospitals; and drugs furnished to beneficiaries with Medicare as a secondary payer.

Source: MedPAC analysis of Medicare claims data for physicians, hospitals, and suppliers.

in two scenarios. First, for new single-source drugs, in the first six to nine months on the market, ASP data are not yet available. Medicare may also lack ASP data for drugs that are not new for other reasons, such as a manufacturer not reporting ASP data. For example, a recent OIG report found that as of January 2023, many billing codes for skin-substitute products (30 out 68 billing codes) lacked an ASP-based payment rate because manufacturers were not reporting ASP data (Office of Inspector General 2023).[46]

For most drugs without ASP data, Medicare generally pays providers based on the manufacturer's list price—the wholesale acquisition cost (WAC)—plus an add-on percentage. When the ASP payment system was first established, Medicare paid all drugs lacking ASP data WAC + 6 percent. In June 2017, the Commission recommended that payment be changed from WAC + 6 percent to WAC + 3 percent (Medicare Payment Advisory Commission 2017). In 2019, CMS changed the payment amount for new drugs lacking ASP data to WAC + 3 percent, while drugs lacking ASP data for other reasons continue to be paid WAC + 6 percent.

The rationale for the Commission's 2017 recommendation to reduce the add-on percent payment to WAC + 3 percent stems from differences in the definition of WAC and ASP. WAC is the price at which the manufacturer sells to the wholesaler and, unlike ASP, does not reflect any discounts. As a result, WAC is generally higher than ASP. The Commission's 2017 analysis found that for a sample

of new drugs, payment rates generally fell when ASP data became available and payment rates moved from WAC + 6 percent to ASP + 6 percent. The Commission recommended a payment of WAC + 3 percent for drugs lacking ASP data because it would set payment at a level that was roughly in parity with ASP + 6 percent for the sample of new drugs we examined. In making the 2017 recommendation of WAC + 3 percent, the Commission indicated that WAC-based payment might be further reduced if the ASP add-on were reduced in the future.

Does the percentage add-on influence use of high-cost drugs?

The percentage add-on to Medicare Part B's ASP payment rates has garnered attention because of concern that it may create incentives for use of higher-priced drugs when lower-priced alternatives exist (Bach and Ohh 2018, Dusetzina and Mello 2021, Hutton et al. 2014, Sanghavi et al. 2014). Prescribing decisions depend on a variety of clinical factors; for example, drugs can vary in terms of their effectiveness in treating patients with certain conditions or comorbidities, and they can differ in terms of side effects. While clinical factors play a central role in prescribing decisions, at the margins, financial considerations can also play a role in providers' choice of drugs. Since a percentage add-on generates more revenue for the provider when applied to a higher-priced product than a lower-priced product, selection of the higher-priced product could generate more profit for the provider, depending on their acquisition costs for the two products. At the same time, other financial considerations might create an incentive to use lower-priced drugs in some situations. For example, when selecting a drug, a provider may take into account the cost sharing associated with each drug and the patient's ability to pay, which might lead to choosing a lower-priced drug for some patients. Also, the financial capital required to acquire and keep an inventory of a high-priced drug can be a disincentive for some providers to furnish expensive drugs.

Evidence from several studies examining utilization patterns for certain products with therapeutic alternatives suggests that the percentage add-on to ASP likely affects prescribing patterns in some circumstances. A study by Jacobson and colleagues examining oncologists' prescribing patterns for lung cancer found a modest increase in the use of the most expensive cancer drug after January 2005, when Medicare began paying for Part B drugs based on ASP + 6 percent (Jacobson et al. 2010). A study by Conti and colleagues of drugs used to treat colorectal cancer found that use of the chemotherapy drug irinotecan declined (by just under 20 percent) after it went generic in 2008 compared with use of an alternative higher-priced brand drug, oxaliplatin (Conti et al. 2012). The authors suggested that physician reimbursement incentives may have been a driver of those utilization changes, but they also stated that there were changes in recommended treatment regimens over this period, which could also have contributed to these trends. When the LCA policy for certain prostate cancer drugs was removed in 2010 and Medicare began paying for the drugs based on 106 percent of their own ASPs, the Department of Health and Human Services OIG found a shift from the lowest-priced prostate cancer drug toward higher-priced competitor products (Office of Inspector General 2012). Hambley and colleagues examined utilization of several iron products among Medicare beneficiaries between 2015 to 2017, a period that included a shortage of the low-priced product iron dextran during the early part of 2016 (Hambley et al. 2020). The study found increasing market share for a high-priced iron product, ferric carboxymaltose, even after the shortage of iron dextran subsided, which the authors suggest may have been related to its higher add-on payment. Gupta and colleagues found that after the FDA approved denosumab (a bone resorption inhibitor drug) in 2018 for skeletal-related events in patients with multiple myeloma, the product rapidly diffused among FFS beneficiaries with multiple myeloma, despite lack of evidence of superiority compared with its lower-cost alternatives, zoledronic acid and pamidronate (Gupta et al. 2020). The authors questioned the routine use of denosumab except in patients with renal dysfunction or in those unable to tolerate the lower-cost agents.

The percentage add-on may also affect a provider's decision to initiate or continue drug treatment rather than opt for nondrug treatment, watchful waiting, or palliative care. Although studies have not evaluated this question directly, some have looked at whether large reimbursement changes—specifically, the payment rate changes that occurred when the MMA changed the Part B drug payment rates from

95 percent of average wholesale price (AWP) to ASP + 6 percent—affect utilization of drugs. A study by Elliot and colleagues found that when reimbursement for androgen suppression therapy (AST) declined by 64 percent between 2003 and 2005, AST use declined among nonindicated, low-risk patients (from 10 percent to 6 percent receiving AST) but remained steady among higher-risk patients with metastatic disease (Elliott et al. 2010). A study by Colla and colleagues found some reduction in patients with a poor prognosis receiving chemotherapy in physician offices in the last 14 days and 3 months of life, but not in hospital outpatient departments, after the payment rate was reduced from 95 percent of AWP to ASP + 6 percent (Colla et al. 2012). The authors attributed the decrease in chemotherapy provision to physician offices' response to reduced drug profit margins, hypothesizing that physician offices were more responsive to the payment reduction than outpatient hospitals because physicians' income is more directly related to chemotherapy use in the physician office setting than in the hospital outpatient setting, where physicians may be salaried employees.

Experience with payment changes under the ESRD payment system also illustrates more broadly how financial incentives can affect utilization, product selection, and price competition. Medicare's implementation of the ESRD prospective payment system (PPS), which eliminated separate payment for ESAs and included them as part of the broader payment bundle, led to more judicious use of ESAs by nephrologists and dialysis facilities. ESA use declined by 23 percent between 2010 and 2012 (one year before and after, respectively, implementation of the PPS) without adverse effects on beneficiary outcomes (Medicare Payment Advisory Commission 2022c).[47] Inclusion of drugs in the ESRD payment bundle also led to price competition and incentivized providers to shift to lower-priced products.[48]

Reducing add-on payments for Part B drugs

To improve financial incentives, Medicare should minimize the relationship between price and add-on payments for drugs paid based on ASP and eliminate add-on payments for drugs paid based on WAC. As discussed later in this section, our approach for drugs paid based on ASP would maintain the current add-on for the lowest-cost products, reduce the percentage add-on and add a fixed fee for mid-priced drugs, and place a fixed-dollar cap on the add-on for the highest-priced drugs. Add-on payments for drugs that lack ASP data and are paid based on WAC should also be eliminated because WAC is an undiscounted list price that is generally higher than ASP.

RECOMMENDATION 1-3

The Congress should require the Secretary to:

- **reduce add-on payments for costly Part B drugs and biologics paid based on average sales price in order to minimize the relationship between average sales price and add-on payments, and**
- **eliminate add-on payments for Part B drugs and biologics paid based on wholesale acquisition cost.**

RATIONALE 1-3

Because a percentage add-on payment generates more revenue for the provider when applied to a higher-priced product, Medicare's current payment for Part B drugs may create incentives for use of higher-priced drugs when less expensive therapeutic alternatives are available. The percentage add-on payment may also affect a provider's decision to initiate or continue drug treatment in some circumstances. Reducing the add-on payment for costly Part B drugs would improve financial incentives under the ASP payment system by minimizing the relationships between price (ASP) and add-on payments. For drugs lacking ASP data and paid based on WAC, providers already receive a payment that is generally higher than ASP since WAC is a list price that does not reflect any discounts. Eliminating the add-on to WAC would reduce excessive payments for these drugs, thereby reducing the financial incentives to use such drugs when less expensive alternatives are available.

IMPLICATIONS 3

Spending

- This recommendation would decrease federal program spending relative to current law by at least $250 million over one year and at least $1 billion over five years.

Beneficiary and provider

- We do not expect this recommendation to have an adverse effect on beneficiaries' access to care. Providers that furnish Part B drugs would generally

experience reduced add-on payments, except for low-priced drugs. The reduction would result in increased financial pressure for some providers, depending on factors such as manufacturers' pricing response to the policy. Overall, the policy is not expected to affect providers' willingness and ability to serve beneficiaries.

Implementation issues for Medicare regarding reducing add-on payments

In implementing a policy to reduce the ASP add-on, the Secretary would have to consider how to structure the reduced add-on to improve financial incentives. Over the years, the Commission has explored a number of approaches to modify the percentage add-on to ASP. In 2017, the Commission recommended reducing the add-on as part of its recommendation to develop what we described as the Drug Value Program (DVP). As recommended, the DVP would be a voluntary, market-based alternative to the ASP payment system. It would rely on private vendors to negotiate drug prices using tools like a formulary, and it would share savings with providers that choose to enroll. The Commission recommended that the percentage add-on be reduced beginning no later than 2022, regardless of the status of the DVP, in order to create pressure for DVP development and implementation and to encourage provider enrollment in the DVP. Our report suggested that the ASP add-on could be reduced gradually, by 1 percentage point per year (i.e., ASP + 5 percent in 2022, ASP + 4 percent in 2023, and ASP + 3 percent in 2024 and onward).

In our June 2022 report, the Commission continued to explore approaches to modify the percentage add-on. We observed that policies to modify the ASP add-on involve trade-offs. Eliminating the percentage add-on would reduce any incentives for providers to use a higher-priced drug when a lower-priced drug with similar health effects is available to treat a particular patient. At the same time, however, eliminating a percentage add-on might result in Medicare's payment rate being lower than acquisition costs for some products or some providers. An alternative to fully eliminating the percentage add-on is a "hybrid approach," with a reduced percentage add-on and flat fee. Such an approach would improve incentives by reducing the difference in add-on payments between higher-cost and lower-cost drugs, while also reducing the potential for unintentionally harming providers' ability to acquire drugs for the Medicare payment amount.

However, as we discussed in our June 2022 report, under a hybrid approach (i.e., a reduced percentage add-on and fixed fee), concerns exist related to incentives for very high-priced and very low-priced drugs. The majority of Part B drug administrations are for very low-priced drugs. Under the hybrid approach, the flat add-on for very low-priced drugs could be large relative to the price of such drugs, potentially creating incentives for overuse of these products when treatment might not otherwise be initiated. On the other end of the spectrum, some Part B drugs are extremely high priced (e.g., a few are currently priced over $400,000 per patient year, and in the future launch prices may be even higher for certain types of products like gene therapies). A percentage add-on is particularly inefficient for high-priced drugs. If one rationale for an ASP add-on is price variation across purchasers, paying a percentage add-on for expensive drugs could result in a large dollar add-on payment that is not in line with actual price variation. Even if prices currently vary across purchasers for high-priced drugs, changes to Medicare add-on payments could spur manufacturers to reduce or eliminate the variation. As noted previously, the Commission's analysis of prior payment changes for Part B drugs found that manufacturers have changed pricing patterns in response to payment policy changes. In addition, the existence of a large add-on on top of an already expensive drug also raises concerns from a beneficiary cost-sharing perspective, particularly when the purpose of large add-on payments is unclear. A hybrid approach could be combined with caps on the add-on for high-priced and low-priced drugs as a way to address concerns about incentives for differently priced drugs.

An illustrative hybrid approach to reduce add-on payments for drugs paid based on ASP

We modeled an approach that would convert a portion of the percentage add-on to a fixed fee and place additional limits on the add-on amounts for high-priced and low-priced products. Under this illustrative policy, the ASP add-on payment per drug per administration day would be the lesser of 6 percent, 3 percent plus $24, or $220. This illustrative policy reflects the pre-sequester payment amount, just like the 6 percent add-on policy that it would

replace in Section 1847A of the Social Security Act. We note that policymakers would need to determine the appropriate level of the percentage add-on. The specific percentages and dollar amounts outlined here are illustrative; other percentages and amounts could achieve the Commission's objective of improving Medicare's payment for drugs paid based on ASP.

In developing this approach, we sought to:

- reduce or eliminate the percentage add-on for moderate- and high-priced drugs to minimize the relationship between price (ASP) and add-on payments;
- retain a portion of the percentage add-on for all but the most expensive drugs, to accommodate price variation or other factors that might lead to some purchasers acquiring drugs at a price greater than ASP; and
- avoid applying a flat fee for low-cost drugs, which would constitute a substantial increase in payment rates relative to the drug's price and potentially create incentives for overuse.

Our illustrative approach was developed by first reducing the percentage add-on from 6 percent to 3 percent. This choice is consistent with the level articulated in the Commission's June 2017 report. On the one hand, the more the add-on percentage is reduced, the more incentives are improved by reducing the difference in add-on payments between higher- and lower-cost drugs. On the other hand, a higher percentage add-on gives more cushion for providers if prices vary across purchasers or if manufacturers raise prices.

We arrived at the $24 flat fee by estimating the budget-neutral equivalent of a 3 percent add-on (i.e., the average of 3 percent of ASP across all drug administrations, using the aggregate residual to set the flat-fee amount). We then applied a hybrid approach, with two limits on add-on payments. First, add-on payments under our illustrative policy could be no greater than 6 percent (current policy). This limit was intended to address concerns that a $24 flat fee could lead to a very large add-on for very low-priced drugs. Second, add-on payments could be no greater than a fixed dollar amount. This fixed-dollar cap was intended to address concerns about excessive add-on payments for very expensive drugs. Under our illustrative policy, we set the fixed-dollar cap at the 75th percentile of add-on payments in 2021, or $220. About 25 percent of Part B drugs in 2021 had an average add-on payment greater than $220, accounting for less than 6 percent of all drug administrations but more than half of total add-on payments. Determining the appropriate level for an add-on cap is a policy judgment, and policymakers could consider other points in the distribution. We note that the dollar amounts we modeled were intended for the first year of our illustrative policy. Policymakers would need to determine how the flat-fee and the fixed-dollar cap amount would be updated each year. One option would be to update the amounts annually based on an inflation benchmark.[49]

This illustrative add-on policy was developed as a potential modification to Part B drug payment rates specified in Section 1847A of the Social Security Act, which specifies a payment rate of 106 percent of ASP for most Part B drugs. Pursuant to the Budget Control Act of 2011 and subsequent legislation, however, all Medicare program payments under traditional FFS Medicare, MA, and Part D—including Part B drug payments—are subject to a 2 percent sequester through March 2032. If policymakers were to adopt the illustrative policy in Section 1847A of the Social Security Act, the Part B drug payment amount (ASP plus the revised add-on amount) would be subject to the sequester through March 2032 (similar to other Medicare services) and Medicare's portion of the payment would be reduced by 2 percent. Policymakers could choose to offset the effect of the sequester on drugs receiving lower add-on payments under the illustrative policy.[50] For example, for the most costly products, policymakers could consider designing the fixed-dollar add-on cap such that Medicare's net payment rate would not fall below ASP while the 2 percent sequester was in effect.[51] Similarly, policymakers could consider the effects of the sequester while determining at what level to set the reduced percentage add-on and fixed fee (e.g., 3 percent + $24, or alternative amounts).

Recently, payment for Part B drugs furnished by 340B hospitals changed from ASP − 22.5 percent to ASP + 6 percent. Although 340B hospitals are now paid ASP + 6 percent, we assume the restructured add-on would not apply to 340B drugs because the Commission has a separate standing recommendation to modify payment

TABLE 1–9

ASP add-on payment amounts for differently priced drugs under current policy and under the Commission's illustrative policy

	Add-on payment amount in dollars		Add-on payment amount as percentage of ASP	
ASP per drug administered	Current policy: 6%	Policy option: Lesser of: (6%, 3% + $24, $220)	Current policy: 6%	Policy option: Lesser of: (6%, 3% + $24, $220)
10	0.60	0.60	6.0	6.0
100	6	6	6.0	6.0
800	48	48	6.0	6.0
1,000	60	54	6.0	5.4
3,000	180	114	6.0	3.8
5,000	300	174	6.0	3.5
6,500	390	219	6.0	3.4
10,000	600	220	6.0	2.2
13,500	810	220	6.0	1.6

Note: ASP (average sales price). "ASP per drug administered" is defined as the ASP unit price times the number of units of the drug administered to the patient on a particular day. For drugs furnished by suppliers (e.g., nebulizer drugs and certain oral drugs), the data reflect ASP per prescription rather than ASP per administration. Under current policy, the ASP add-on payment per drug per administration day is 6 percent; under the illustrative policy, it would be the lesser of 6 percent, 3 percent plus $24, or $220. Add-on payment amounts include Medicare program payments and beneficiary cost sharing and are calculated before application of the sequester, which would reduce the total payment by 1.6 percent.

Source: MedPAC.

for Part B drugs furnished by 340B hospitals. That recommendation would reduce Medicare payments for 340B drugs by 10 percent and direct program savings toward safety-net hospitals (see text box on the Commission's standing recommendation regarding 340B drugs, p. 53).[52]

The effect of the illustrative policy on add-on payments for differently priced Part B drugs is displayed in Table 1-9. For drugs with an ASP per administration under $800, add-on payments are unchanged from current policy. For drugs with an ASP per administration greater than $800, add-on payments are reduced to 3 percent + $24. Add-on payments are also capped at $220, which limits the add-on for drugs with an ASP per administration greater than $6,533. Thus, for products with an ASP greater $800, incentives to use a higher-priced product compared with a lower-priced product would be reduced under this illustrative policy. For example, comparing two drugs, one with an ASP per administration of $1,000 and the other of $3,000, the difference in add-on payments between the two products would be reduced from $120 ($180 – $60) under current policy to $60 ($114 – $54) under the illustrative approach. The largest reduction in the add-on differential occurs among higher-priced drugs. For example, comparing a drug with an ASP per administration of $6,500 and one with an ASP per administration of $13,500, the add-on differential is essentially eliminated, falling from $420 ($810 – $390) under current policy to $1 ($220 – $219) under the illustrative approach.

Table 1-10 (p. 52) shows the effect of the illustrative add-on policy on overall Part B drug spending. These estimates are based on 2021 utilization data without any assumptions about how the illustrative policy might affect prescribing behavior or manufacturer pricing decisions. Overall, we estimate that the illustrative policy would reduce aggregate Part B drug payments for non-340B drugs by 2.3 percent. The Medicare program and beneficiaries could realize

TABLE 1–10

Simulated impact of the Commission's illustrative policy reducing add-on payments for drugs paid based on ASP: Total Part B drug payments by type of provider

	2021 Total payments for Part B drugs paid ASP + 6% (in billions)	Percentage change under illustrative policy
All	$28.7	–2.3%
Physician	20.5	–2.3
Oncology	7.7	–2.7
Ophthalmology	4.2	–1.6
Other	2.8	–2.1
Primary care	2.5	–2.3
Rheumatology	2.5	–2.1
Neurology	0.5	–2.8
Urology	0.3	–1.8
Hospital outpatient departments	6.2	–2.7
Suppliers	2.0	–1.7

Note: ASP (average sales price). Under current policy, the ASP add-on payment per drug per administration day is 6 percent; under the illustrative policy, it would be the lesser of 6 percent, 3 percent plus $24, or $220. Total payments include Medicare program payments and beneficiary cost sharing. Analysis includes all Part B–covered drugs paid under the ASP + 6 percent system, excluding drugs billed through not-otherwise-classified Healthcare Common Procedure Coding System codes. Part B drugs acquired by hospitals under the 340B drug pricing program are excluded from the analysis. Data for critical access hospitals, Maryland hospitals, and beneficiaries with Medicare as a secondary payer are excluded from the analysis.

Source: MedPAC analysis of Medicare claims data for physicians, hospitals, and suppliers.

additional savings to the extent that the illustrative policy resulted in substitution of lower-cost drugs for higher-cost drugs. However, estimated savings would be lower if the $220 fixed-dollar cap on the add-on payment resulted in some drugs being furnished in smaller, more frequent doses. In implementing reduced add-on payments for drugs paid based on ASP, CMS could monitor drug administration patterns across providers and products to help ensure that the policy did not incentivize shifts toward more frequent administrations than would otherwise occur. As shown in Table 1-10, the effects of the illustrative policy would vary across clinical specialty. We estimate that all specialties and categories of providers that furnish some Part B drugs would experience a decline in Part B drug payments, ranging from 1.7 percent to 2.8 percent.

Stakeholders have raised several concerns specific to policy proposals that would reduce the add-on for Part B drugs to improve financial incentives. First, some contend that small purchasers and those practicing in medically underserved communities will be unable to acquire drugs for the Medicare payment amount if the ASP add-on is changed. We note that manufacturers set their own prices and have an incentive to price products at a level commensurate with Medicare payment. Prior Commission analyses suggest that manufacturers are responsive to Medicare payment rate changes (such as the shift to the ASP payment system in 2005 and the implementation of the sequester in 2013), narrowing price variation or modifying pricing patterns in ways that help mitigate the effect on providers (Medicare Payment Advisory Commission 2022b). Moreover, our illustrative approach would keep in place the 6 percent add-on for lower-priced drugs and, for more expensive drugs, would apply a larger reduction to the add-on payment as prices increased.

The Commission's standing recommendation on Medicare payment for 340B drugs

In March 2016, the Commission recommended reductions to payments for Part B drugs furnished by 340B hospitals, with the savings directed to the Medicare uncompensated care fund.

The 340B Drug Pricing Program ("340B program") allows certain hospitals and other health care providers that meet certain criteria to obtain substantially discounted prices on covered outpatient drugs. The discount for each drug obtained through the 340B program is based on a ceiling price. The ceiling price is the maximum allowed amount a manufacturer can charge 340B hospitals. The formula for the ceiling price is the average manufacturer price (AMP) for a drug, less a unit rebate amount (URA). For brand drugs, the URA is the greater of 23.1 percent of AMP or the difference between AMP and best price; plus, if the product's price rises faster than inflation, there is an additional inflation rebate.[53] A report from the Office of Inspector General estimated that the 340B ceiling prices were 34 percent below Medicare ASP plus 6 percent (ASP + 6 percent) payments for Part B drugs furnished by 340B hospitals and other 340B entities in 2013 (Office of Inspector General 2015).

Prior to 2018, under the outpatient prospective payment system, Medicare paid 340B hospitals and non-340B hospitals the same rates for Part B drugs, even though 340B hospitals are able to purchase these drugs at steep discounts. Similarly, beneficiaries had cost-sharing liability of up to 20 percent of Medicare's payment rate for outpatient drugs received at both types of hospitals.

In the March 2016 report, the Commission recommended changes to Medicare's payment for 340B drugs. The Commission recommended that the Congress direct the Secretary of Health and Human Services to reduce Medicare payment rates for 340B hospitals' separately payable 340B drugs by 10 percent of the average sales price (ASP) and direct the program savings from reducing Part B drug payment rates to the Medicare-funded uncompensated care pool. A rationale for the rate reduction was to allow beneficiaries to share in the discounts that 340B hospitals receive from drug companies. Because the Commission did not want to reduce program payments to hospitals providing the most care to the uninsured, it recommended that the program savings from the payment reduction be redirected to the uncompensated care pool.

Subsequent to the Commission's recommendation, CMS lowered payments to 340B hospitals for separately payable non-pass-through drugs to ASP – 22.5 percent. In June 2022, the Supreme Court ruled that CMS's approach to reducing payment for 340B drugs was not consistent with its statutory authority. CMS has established a payment rate of ASP + 6 percent in 2023 for Part B drugs furnished by 340B hospitals. With payments reverting to ASP + 6 percent for 340B drugs, the Commission's 2016 position on 340B drugs remains a recommendation. We continue to believe that this approach is appropriate, and the specific level of payment reduction could be considered further as newer data become available. In addition, the Commission has recently begun to discuss alternative ways of identifying and supporting Medicare safety-net hospitals, including redistributing disproportionate share and uncompensated care funds to support such hospitals. Under this construct, 340B savings from our 2016 recommendation would similarly be distributed to support these hospitals. ■

Second, some stakeholders have cautioned that reducing the ASP add-on could accelerate a trend toward hospitals buying community oncology practices. The forces driving hospitals' acquisition of these practices are multifactorial and include the availability of 340B discounts at some hospitals, general reimbursement pressures, a movement toward integrated care models, and interest among some physicians in employment rather than running a practice. If a change to the ASP add-on resulted in

some practices having difficulty purchasing drugs at the Medicare payment rate, this circumstance might contribute to the trend toward more hospital-based oncology care. We note, however, that it is in drug manufacturers' interest to support community oncology practices since acquisition of practices by hospitals potentially subjects more manufacturer sales to 340B discounts.

Last, some stakeholders assert that the percentage add-on to ASP should not be reduced because it is used to cover some of physicians' drug administration costs, which they claim are not adequately paid for under the physician fee schedule payment rates. Data that could be used to compare physicians' cost for administering drugs with physician fee schedule payment rates are not available. However, Medicare's ASP-based payment is intended to cover drug acquisition costs and is separate from its payment for drug administration services under the physician fee schedule and hospital OPPS. If there are concerns about Medicare's payment for drug administration, CMS should use existing processes—such as the American Medical Association's Specialty Society Relative Value Scale Update Committee—to evaluate the adequacy of those rates. Using a percentage add-on to a drug's ASP to compensate physicians for drug administration costs would be inefficient: There is no evidence that the costs of a drug's administration are proportionate to the price of the drug. ■

Endnotes

1 On the basis of SSR Health data, the authors identified a list of prescription drugs that met each of the following criteria: (1) were among the top 250 drugs by 2020 U.S. sales revenue; (2) had list price increases that were more than 2 percentage points higher than the rate of medical inflation between the end of 2019 and the end of 2020; (3) had net price increases after accounting for rebates and other concessions; and (4) after net price increases were vetted with manufacturers, were found to be the top 10 drugs whose price increases—as opposed to volume increases—contributed to the largest increase in U.S. spending. Based on public input, an additional two drugs were included in the analysis.

2 Such circumstances include when practitioners, patients, providers, or other members of the public have raised significant questions to the Secretary about the health outcomes attributable to the use of services by Medicare beneficiaries.

3 In 2005, CMS applied CED to cover off-label use of colorectal cancer drugs (oxaliplatin, irinotecan, cetuximab, or bevacizumab), linking coverage to participation in nine clinical trials sponsored by the National Cancer Institute. As of September 2021, this CED was ongoing. In 2009, Medicare applied CED for pharmacogenomic testing for warfarin response. In April 2022, CMS applied CED to the use of antiamyloid mAb products.

4 Like all Medicare services, the Medicare payments for Part B drugs are subject to the 2 percent sequester through 2032. A statutory payment rate of ASP + 6 percent after application of the sequester results in a net payment from the perspective of the provider of ASP + 4.3 percent (with the Medicare program paying 80 percent of ASP + 3.9 percent and the beneficiary paying 20 percent of ASP + 6 percent).

5 The 340B Pricing Program allowed certain hospitals to obtain discounted prices from drug manufacturers on drugs and biologics other than vaccines. Under the hospital outpatient prospective payment system, new drugs, biologics, and biosimilars typically receive pass-through status for the first two to three years on the market. Between 2018 and 2022, 340B hospitals were paid ASP + 6 percent for drugs with pass-through status while other Part B drugs were paid ASP − 22.5 percent. CMS has not yet determined how the agency will remedy payment rates for past years based on the Supreme Court ruling.

6 CMS takes the charges for items and services, including bundled drugs, and multiplies them by department-level cost-to-charge ratios to estimate the average cost associated with each APC. In this way, an estimate of hospitals' average drug costs flows into the bundled payment rates under the OPPS.

7 By price, we mean the amount Medicare paid per drug per beneficiary over a one-year period. Because Part B drugs vary in their frequency of administration, we measure price in terms of payment over a one-year period in order to help control for dosing differences across products.

8 This analysis of separately payable Part B drugs between 2009 and 2021 excludes any drug that was bundled in 2009 or 2021. That is, if payment for the drug was packaged into the payment for another service in 2009 or 2021, that drug was excluded from both years of the analysis, regardless of the setting in which the drug was administered.

9 In addition to payment for a drug, Medicare makes a separate payment for administration of the drug under the physician fee schedule or OPPS. Medicare pays a dispensing or supplying fee to pharmacies that dispense inhalation drugs and oral anticancer, oral antiemetic, and immunosuppressive drugs to beneficiaries; Medicare also pays a furnishing fee to providers of clotting factors. Beneficiaries generally are responsible for a 20 percent copayment.

10 The Part B price indexes reflect growth in the ASP at the individual product level, which is a measure of average postlaunch price growth for Part B drugs. Growth at the individual product level is different from the change in the aggregate average price Medicare Part B pays for drugs (Table 1-2, p. 17), which reflects a broader set of dynamics (including changes in the price of existing products, rising launch prices of new products compared with older products, and shifts in the mix of drugs).

11 The amount of Medicare spending in not-otherwise-classified billing codes varies on a yearly basis. Over the 10-year period from 2012 to 2021, spending in not-otherwise-classified codes ranged from $142 million to $438 million per year.

12 Researchers found that NIH-funded projects directly or indirectly contributed to all of the 210 new molecular entities approved by the FDA from 2010 to 2016 (Galkina Cleary et al. 2018). The study found that the NIH-funded projects primarily focused on "molecular targets for new drugs and likely represents basic research or use-inspired basic research, as opposed to applied research." Nayak and colleagues examined public sector contributions to late-stage research and development for drugs approved by the

FDA from 2007 to 2018 and found that "publicly supported research had a major role in the late stage development of at least one in four new drugs" (Nayak et al. 2019).

13 In describing the assumptions of its simulation mode, CBO stated that "a 15 percent to 25 percent reduction in expected returns for drugs in the top quintile of expected returns is associated with a 0.5 percent average annual reduction in the number of new drugs entering the market in the first decade under the policy, increasing to an 8 percent annual average reduction in the third decade" (Congressional Budget Office 2021a).

14 According to the Office of Inspector General, the process to withdraw an accelerated approval drug can be lengthy (Office of Inspector General 2022). For example, for voluntary withdrawals, the FDA must make a finding about the proposed withdrawal, publish a Federal Register notice of its determination, and address any relevant abbreviated new drug applications and requests for continued access (National Organization for Rare Disorders 2021). The Food and Drug Omnibus Reform Act of 2022 includes several reforms to the accelerated approval process, including enabling the FDA to require that a postapproval study be under way prior to granting accelerated approval and expanding expedited withdrawal procedures.

15 For purposes of accelerated approval: (1) A surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure, that is thought to predict clinical benefit, but is not itself a measure of clinical benefit; (2) an intermediate clinical endpoint, which is also thought to predict clinical benefit, is the measurement of a therapeutic effect that can be assessed earlier than an effect on irreversible morbidity or mortality (Food and Drug Administration 2014).

16 Researchers determined the added therapeutic value of 90 accelerated approval drugs compared with existing therapies using health technology assessments from German, French, and Canadian health technology assessment agencies.

17 A meta-analysis of randomized clinical trials quantifying the association between surrogate endpoints and overall survival in medical oncology found that more than half of reported correlations were of low strength, 25 percent were of medium strength, and 23 percent were highly correlated with survival (Prasad et al. 2015).

18 For example, in 2014, Zydelig, a first-in-class (Part D) drug, was approved for relapsed follicular B-cell non-Hodgkin lymphoma and relapsed small lymphocytic lymphoma through the accelerated approval pathway, with a postconfirmatory trial due December 2019. In 2016, evidence about additional safety risks (including an increased rate of adverse events, including deaths, in the drug's clinical trials) led the manufacturer to terminate six clinical trials (Banerjee et al. 2023, Food and Drug Administration 2022d). The manufacturer voluntarily withdrew the two accelerated approval indications in 2022.

19 According to the FDA: "For drugs granted accelerated approval, postmarketing confirmatory trials have been required to verify and describe the anticipated effect on IMM (irreversible morbidity or mortality) or other clinical benefit" (Food and Drug Administration 2014).

20 Folotyn and ProAmatine are the two drugs whose confirmatory trials are more than five years late. Folotyn's confirmatory trial results (for treatment of T-cell lymphoma) is 66 months past its original date. The delay is linked to changes in the standard of care, the design and implementation of new trials, and changes in the drug's ownership (Office of Inspector General 2022). According to CMS dashboard data, Part B spending for Folotyn was $177 million between 2012 and 2021. ProAmatine's confirmatory trial results (for treatment of postural hypotension) is 93 months past its original final report date. The FDA attempted to withdraw this drug in 2010, but stakeholders asked that the drug remain on the market (Office of Inspector General 2022). The delay is also linked to changing ownership of the drug application (Office of Inspector General 2022).

21 The Commission's analysis includes all drugs, not solely Part B products administered by infusion or injection in physician offices and hospital outpatient departments, and is based on FDA data on accelerated approvals as of December, 31, 2022 (Food and Drug Administration 2022a), with the status of an indication updated as of April 2023 using FDA data found at https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approval-program.

22 A recent study of 60 new drug products approved between 2008 and 2018 found no association between the products' launch price and R&D costs (Wouters et al. 2022).

23 Average spending per user in 2021 is determined across all indications (i.e., accelerated and traditional approval indications) of the drug.

24 Researchers have raised several concerns about how compendia are assembled, the conflicts of interest on the part of their contributors, and substantial inconsistences both among and within these resources (Green et al. 2016).

25 A policy design issue concerns the timing of a cap for a drug whose initial confirmatory trial failed to show a clinical benefit but whose manufacturer has reached an agreement with the FDA for initiating additional trials. For example, in 2020, the confirmatory trial of Zepzelca (used to treat

small cell lung cancer) did not meet its primary endpoint of overall survival. However, based on several factors (including the initial trial's design), the FDA concluded that the drug's withdrawal is not appropriate at this time and agreed with the manufacturer on the initiation of subsequent confirmatory randomized trials in 2021 (Food and Drug Administration 2022b).

26 In 2022, CMS concluded there was insufficient evidence that coverage of anti-amyloid monoclonal antibody drugs for the treatment of Alzheimer's disease was reasonable and necessary. However, because of the prevalence of this disease among Medicare beneficiaries, the agency concluded that the CED paradigm was the most appropriate pathway to provide Medicare coverage while additional evidence is developed.

27 Health technology assessments evaluate issues related to the safety, efficacy, cost, and cost-effectiveness of a medical service, item, or a group of services or items.

28 To operationalize such an approach, the Secretary would need to develop a process to calculate and collect the manufacturer rebate amount, as the Secretary has done for other policies (e.g., discarded drug refund policy). To determine the rebate amount and to facilitate reduced beneficiary cost sharing, CMS could add a modifier or specific billing code so the agency, providers, and researchers could identify claims that qualify for lower beneficiary cost sharing and a retroactive manufacturer rebate to Medicare.

29 Alternatively, Medicare could create temporary drug billing codes for the period that the payment of an accelerated approval drug is capped.

30 Medicare is currently using this type of approach with the ASP inflation rebate; as of April 1, 2023, for products that incur a rebate, beneficiary cost sharing is based on the lower, inflation-adjusted ASP.

31 Zynteglo is a one-time cell-based gene therapy for adult and pediatric patients with beta-thalassemia (a type of inherited blood disorder that causes a reduction of normal hemoglobin and red blood cells in the blood) who require regular red blood cell transfusions. Press reports suggest that the manufacturer is entering into outcomes-based agreements with some commercial and government payers (Harris 2022). ICER's cost-effectiveness modeling concluded that this new treatment achieved commonly accepted value thresholds at an anticipated price of $2.1 million with an 80 percent payback option for patients who do not achieve and maintain transfusion independence over a five-year period.

32 The study examined 24 Part B anticancer drugs that were approved by the FDA between 1996 and 2012 and did not go off patent during the follow-up period (between 2005 and 2017). Over that period, the mean overall cumulative price increase for the 24 Part B drugs was 36.5 percent. Adjusting for the general inflation rate or the health-related inflation rate, the mean cumulative drug price increases were 19.1 percent and 8.4 percent, respectively. Using multivariate regression techniques, the researchers reported that the number of years after a drug's launch may have influenced price change rates. For every additional year after a drug's launch, there was an additional increase of 0.3 percent in inflation-adjusted price change and a 0.2 percent increase in health-related inflation-adjusted price change rates.

33 The authors included the following six classes: antineoplastic agents, insulins, lipid-lowering agents, multiple sclerosis therapies, noninsulin antidiabetic agents, and TNF inhibitors.

34 For case studies on the use of reference pricing in Australia and Germany, see the Commission's June 2019 report to the Congress (https://www.medpac.gov/document/http-www-medpac-gov-docs-default-source-reports-jun19_ch3_medpac_reporttocongress_sec-pdf/).

35 The prostate cancer drugs were triptorelin pamoate, goserelin acetate implant, and leuprolide acetate suspension.

36 Under the Medicare, Medicaid, and SCHIP Extension Act of 2007, CMS calculates the payment rate for albuterol and levalbuterol based on the lower of (1) the volume-weighted average of 106 percent of the ASP for both drugs or (2) the payment rate based on 106 percent of ASP for the individual drug.

37 In 2007, the ASP for levalbuterol was nearly 20 times that of albuterol. When CMS placed these products in the same billing codes in the third quarter of 2007 and set the payment rate based on the volume-weighted ASP across these products, the payment rate for albuterol, the product that accounted for the vast majority of utilization, increased five-fold while the payment rate for levalbuterol declined substantially (Medicare Payment Advisory Commission 2017). The Congress responded to the large increase in payment for albuterol in the Medicare, Medicaid, and SCHIP Extension Act of 2007 by specifying that the payment rate be based on 106 percent of the lower of (1) the volume-weighted ASP for both drugs or (2) the ASP for the individual drug.

38 Because the three products in this example have the same billing unit (10 mg) and the same dose per year, we would have arrived at the same result if we had taken the unit price of each of the three products (column 3) weighted by the total number of billing units for the year (column 5). In this example, we take the extra step of performing the analysis at the price per year of therapy level because that approach can accommodate more varied situations such as when products have different billing units or different dosing amounts per year.

39 The National Eye Institute funded a study that found that off-label Avastin and on-label Lucentis had equivalent effects on visual acuity when administered according to the same schedule (Catt Research Group et al. 2011).

40 The cost-sharing amount the beneficiary would pay under the exceptions policy would be less than the amount the beneficiary would have paid under current law (i.e., in the absence of a reference pricing policy) because payment under the exceptions policy would be set at 100 percent of ASP.

41 The statute constrains Medicare's use of comparative clinical effectiveness evidence to pay for drugs. Medicare cannot withhold coverage of prescription drugs using comparative clinical effectiveness evidence that the Agency for Healthcare Research and Quality produces. The 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.

42 For example, for a drug with an ASP + 6 percent of $106, under the sequester, Medicare's payment to the provider equals $106 × 0.98 × 0.80 and the beneficiary's payment equals $106 × 0.20.

43 For example, the top 10 drugs that account for the most Medicare Part B spending are all packaged by manufacturers in single-use containers.

44 For drugs provided by outpatient hospitals, some portion of the drug payment amount is intended to cover pharmacy overhead. With respect to payment for separately paid drugs under the OPPS, CMS has stated that the drug payment rate (currently ASP + 6 percent; in prior years, as low as ASP + 4 percent) includes payment for drug acquisition costs and pharmacy overhead (Centers for Medicare & Medicaid Services 2012).

45 This analysis of add-on payments excludes drugs furnished by 340B hospitals in 2021. Specifically, we exclude those drugs billed by OPPS hospitals using the JG or TB modifier (i.e., the modifiers that hospitals are required to include on claims for drugs acquired via the 340B drug pricing program).

46 Before 2022, manufacturers of skin substitutes and other products paid by the Medicare program as Part B drugs but approved by the FDA as devices were not required to report ASP data (although some manufacturers voluntarily reported ASP data). The Consolidated Appropriations Act, 2021, changed this requirement, instead requiring manufacturers of these products to begin reporting ASP data for sales occurring on or after January 1, 2022. The OIG report found noncompliance with this new ASP reporting requirement for 30 of 68 skin-substitute billing codes in January 2023. According to the report, Medicare program spending on these 30 skin-substitute billing codes lacking ASP-based payment rates was $256 million in the third quarter of 2022, almost two-thirds of all spending on skin substitutes that quarter (Office of Inspector General 2023).

47 For ESAs, some of this decline could also have stemmed from clinical evidence showing that higher doses of these drugs led to increased risk of morbidity and mortality, which resulted in the FDA changing the ESA label in 2011.

48 In at least one situation, switching was an explicit goal: Fresenius Medical Care, a large dialysis organization, announced its intent to have more than 70 percent of the company's ESA patients (110,000 patients) switched to epoetin beta (from epoetin alfa) by the end of the first quarter of 2016 (Reuters 2016). Several sources suggest that this company reduced its total ESA costs due to the switch (Reuters 2016, Seeking Alpha 2016).

49 For example, the flat-dollar portions of the add-on formula could be updated using a benchmark of inflation such as the consumer price index, an estimate of average drug price inflation, or the lesser of those two measures.

50 Note that, under the Commission's illustrative model, the payment rates for low-cost drugs would be unchanged from current levels (106 percent pre-sequester and 104.3 percent post-sequester), while the payment rates for all other drugs (i.e., the mid-priced and highest-cost groups) would be reduced and the sequester applied to those reduced amounts.

51 Under the Commission's illustrative model, the 2 percent sequester for the costliest Part B products (with ASPs in excess of about $13,530 per administration) would reduce Medicare's net payment rate below 100 percent of ASP. However, policymakers could design the $220 add-on cap such that net payments did not fall below 100 percent of ASP. For example, policymakers could apply a formula under which the fixed-dollar cap equaled the greater of $220 or, if the 2 percent sequester is in effect, 101.626 percent of ASP. The 101.626 percent is an artifact of (1) the sequester applying to both ASP and the add-on and (2) the sequester applying to only the Medicare program's portion of payment, not the beneficiary's cost-sharing liability. Thus, a payment amount of 101.626 percent of ASP subject to a 2 percent sequester on the Medicare program's portion of the payment results in a net payment to the provider of 100 percent of ASP.

52 The Commission's March 2016 recommendation would reduce payments for 340B drugs by a larger amount than a policy to change the ASP add-on would. The 2016

recommendation concerning payment for drugs furnished by 340B hospitals remains the standing recommendation of the Commission. However, until such time as the Congress acts on that recommendation, from an equity perspective, it could be argued that any reductions to Part B drug add-on payments that are made for non-340B providers should also be made for 340B providers.

53 Best price is defined as the lowest price for a drug available to nongovernment purchasers and reflects discounts, rebates, and other pricing adjustments.

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