



COMMENTS OF THE PHARMACEUTICAL RESEARCH AND
MANUFACTURERS OF AMERICA

SUBMITTED TO THE DEPARTMENT OF
HEALTH AND HUMAN SERVICES

CONCERNING HHS BLUEPRINT TO LOWER DRUG
PRICES AND REDUCE OUT-OF-POCKET COSTS

July 16, 2018

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Attention: HHS-OS-2018-0010
July 16, 2018

VIA ELECTRONIC FILING – via regulations.gov

The Honorable Alex M. Azar II
Secretary of Health and Human Services
200 Independence Avenue, S.W.
Washington, D.C. 20201

Re: HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs

Dear Secretary Azar:

The Pharmaceutical Research and Manufacturers of America (PhRMA) appreciates the opportunity to comment on the Department of Health & Human Services (HHS) request for information (RFI), *HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs*. PhRMA represents the country's leading innovative biopharmaceutical research companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. Since 2000, PhRMA member companies have invested more than \$600 billion in the search for new treatments and cures, including an estimated \$65.5 billion in 2016 alone.

Antitrust Statement

At the outset of our comments, it is important to note that numerous questions in the RFI raise competitively sensitive topics for members and that PhRMA's advocacy activities on behalf of its members in responding to the RFI are limited by the antitrust laws and PhRMA's antitrust compliance policy. In particular, PhRMA as a trade association does not permit any discussion about members' current and future drug pricing strategies, relationships with customers or anticipated responses in the marketplace to any proposed changes to law or regulation. PhRMA's comments have been prepared with these guidelines in mind and in compliance with the antitrust laws and thus set forth PhRMA's advocacy views regarding potential government reforms identified in the RFI that HHS could initiate.

The RFI comes at a time when we are in a new era of medicine in which breakthrough science is transforming patient care and enabling us to more effectively treat chronic disease, the biggest cost driver in our health care system. In this new era of medicine, many diseases previously

regarded as deadly are now manageable and even curable. Today, more than 7,000 medicines are in development worldwide, of which 80 percent have the potential to be first in class and 42 percent are personalized medicines.¹ At the same time we are experiencing these scientific breakthroughs, changes in the supply chain and in health insurance benefits have left some patients facing increased out-of-pocket costs due to rising list prices, and high deductibles and coinsurance.

The RFI creates a unique opportunity for policymakers to take a wide view and address all the factors that are influencing the cost of medicines. It recognizes that the powerful entities making up the biopharmaceutical supply chain, such as pharmacy benefit managers (PBMs) and insurers, play a large role in influencing the cost of medicines because they design prescription drug formularies and cost-sharing structures and retain a sizable share of spending on medicines.² This broad perspective expands the opportunity for HHS to solve the problems patients face. PhRMA is committed to helping solve these problems and supports efforts to make the fundamental policy changes needed to achieve solutions.

The RFI also recognizes the importance of lowering the amount that patients are charged for medicines at the pharmacy counter. This would reduce financial burdens on patients and help achieve the health benefits and cost savings available through improved adherence to needed treatments and reduced abandonment of prescriptions at the pharmacy counter.³ Patients' out-of-pocket cost for medicines is determined by payers' choices, including how they decide to allocate the large, rapidly growing discounts⁴ they obtain from manufacturers. At present, payers' choices have meant that patients rarely benefit from these discounts at the pharmacy counter.⁵ Here, too, PhRMA supports fundamental policy changes to achieve solutions that will help patients and produce better, more efficient health care.

¹ Long G. The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development. Analysis Group. 2017; Tufts Center for the Study of Drug Development (CSDD). Personalized Medicine Gains Traction But Still Faces Multiple Challenges. *Tufts CSDD Impact Report*. 2015;17(3).

² Berkley Research Group, The Pharmaceutical Supply Chain: Gross Drug Expenditures Realized by Stakeholders. January 2017. Available at: <http://www.thinkbrg.com/newsroom-publications-vandervelde-blalock-phrma.html>

³ Goldman DP, Joyce GF, Escarce JJ, et al. Pharmacy benefits and the use of drugs by the chronically ill. *JAMA*. 2004;291(19):2344-2350.; Doshi JA, Li P, Ladage VP, Pettit AR, Taylor EA. Impact of cost sharing on specialty drug utilization and outcomes: a review of the evidence and future directions. *Am J Managed Care*. 2016;22(3):188-197.

⁴ Fein AJ. The gross-to-net bubble topped \$150 billion in 2017. Drug Channels Institute. April 24, 2018. Available at: <https://www.drugchannels.net/2018/04/the-gross-to-net-rebate-bubble-topped.html>

⁵ PhRMA. Commercially-insured patients pay undiscounted list prices for one in five brand prescriptions, accounting for half of out-of-pocket spending on brand medicines. May 2017. Available at: <http://www.phrma.org/report/commercially-insured-patients-pay-undiscounted-list-prices-for-one-in-five-brand-prescriptions-accounting-for-half-of-out-of-pocket-spending-brand-medicines>. Notably, less is spent on medicines than other categories of care but payers' choices mean insurance often covers a smaller share of medicines' cost, leaving patients with a higher share.; Avalere Health analysis of the U.S. HHS, Agency for Healthcare Research and Quality, Medical Expenditure Panel Survey. 2014. Analysis includes individuals with any source of health care coverage, public or private; this includes individuals who had health coverage without coverage for prescription drugs, which can be expected to account for less than 2 percent of those with health coverage.

The RFI identifies potential policy changes that would remake key aspects of the market for prescription medicines. The reforms would have far-reaching impact on the cost of and access to medicines in the United States (U.S.), significantly affecting manufacturers, the supply chain and patients. They also would make large-scale changes to Medicare Parts B and D and address price differences for medicines between the U.S. and other countries caused by foreign governments' free riding on American biopharmaceutical innovation.

In some cases, the ideas raised in the RFI identify ways to remove obstacles to better functioning of private markets. This market orientation, which preserves the real successes of today's system while addressing its problems, is vital to achieving cost savings, continued medical advances and good patient access to needed treatment. Market-oriented policies identified in the RFI would build on important steps the Trump Administration and Congress have already taken to increase competition, including policies to accelerate U.S. Food and Drug Administration (FDA) review of generics; adoption and implementation of the prescription drug, generic drug, and biosimilar user fee legislation; FDA's recent finalization of two manufacturer communications guidance documents intended to facilitate broader opportunities for value-based contracting; Centers for Medicare & Medicaid Services' (CMS) policy providing appropriate reimbursement of biosimilars in Medicare Part B; and changes to address Medicare overpayments to 340B entities.

PhRMA and its member companies support improving the status quo for Americans who rely on medicines. Addressing market distortions created by current law and regulation and enacting reforms to change the supply chain incentives that favor high list prices and high out-of-pocket costs, even as overall spending on medicines is held down,⁶ would have positive consequences for patients and payers. Change also can create broader opportunities for value-based agreements between private payers and manufacturers. Antiquated public policies have constrained these agreements, preventing the biopharma sector from fully participating in the broader movement to promote value-based payment in health care. Addressing foreign governments' systematic free riding on American-supported biopharmaceutical innovation would be another ground-breaking change that would benefit patients and payers.

While some of the policies suggested in the RFI would improve the current system, other policies would restrict patient care and impede innovation. PhRMA opposes changes that would harm access or increase out-of-pocket costs for beneficiaries. We urge caution particularly when making changes that would impact the vulnerable patients who depend on Medicare and Medicaid. The wrong changes to these programs could hurt seniors, children and people with disabilities.

PhRMA is committed to working with the Administration, Congress, patients and payers to advance solutions that will improve affordability of medicines and health care, improve patients' access to needed treatments, and sustain the medical advances Americans expect and need.

⁶ IQVIA. 2017 Medicine Use and Spending. April 2018.

An overview of our comments is set out below, followed by detailed comments in each section.

Innovation and Spending on Medicines: Continued advances in medicines have revolutionized the treatment of numerous serious health conditions, saving lives, improving quality of life, and reducing the need for hospitalization.⁷ While medicines' role in effective health care has grown sharply and hundreds of new medicines have been brought to patients over the last decade, spending on medicines has grown more slowly than spending for other types of care, and medicines' share of national health spending has remained stable.⁸ However, during recent years, publicly reported list prices for medicines have increased more rapidly than the actual prices paid, resulting in a growing gap between list and net prices.⁹ This gap has had important consequences for federal programs and has adversely impacted patients who often pay cost sharing based on list price. Policy changes discussed below could help address these trends and improve the current system for both patients and payers.

Rebates: While the current drug distribution and payment system has successfully constrained overall spending on medicines, it could work better for patients, payers, and manufacturers. Today's system is characterized by a complex web of financial transactions and proprietary contracts and has evolved over time with changes in drug benefits as well as changes in the size, role, and structure of PBMs. As the RFI correctly observes, many entities in the system earn revenue based on a percent of the list price. This hurts patients and increases costs and we believe it must change. We also recognize that government reforms to this system will require careful consideration and input from all stakeholders to ensure an orderly transition to a system that focuses on net prices of medicines and their value to patients. As a first step, we support reforms to ensure that patients benefit from rebates at the point of sale and to discourage supply chain entities from being paid based on list price.

Drug Pricing Demonstrations: As HHS considers potential tests of innovative ways to encourage value-based care and lower medicines prices, it will be important to establish in rulemaking the appropriate role for the CMS Innovation Center (CMMI) and to prioritize holistic approaches that recognize the role that appropriate use of medicines plays in improving patient outcomes and reducing spending in other parts of the health care system. We encourage HHS to establish regulations that define small scale, voluntary, and limited duration testing; clearly

⁷ PhRMA. A decade of innovation in rare diseases: 2005-2015. 2015. Available at: <http://phrma-docs.phrma.org/sites/default/files/pdf/PhRMA-Decade-of-Innovation-Rare-Diseases.pdf>; Lacey MJ, Hanna GJ, Miller JD, Foster TS, Russell MW; Truven Health Analytics. Impact of pharmaceutical innovation in HIV/AIDS treatment during the highly active antiretroviral therapy (HAART) era in the US, 1987-2010: an epidemiologic and cost-impact modeling case study. December 2014. Available at: <http://truvenhealth.com/Portals/0/Assets/Life-Sciences/White-Papers/pharma-innovation-hiv-aids-treatment.pdf>; Roebuck MC. Medical cost offsets from prescription drug utilization among Medicare beneficiaries [commentary]. *J Managed Care Pharm.* 2014;20(10):994-995; Afendulis CC, Chernew ME. State-level impacts of Medicare Part D. *Am J Managed Care.* 2011;17 Suppl 12:S.

⁸ PhRMA analysis of CMS, NHE 2016. December 2017.; Altarum Institute. Projections of the prescription drug share of national health expenditures including non-retail. May 2018. Available at: https://altarum.org/sites/all/libraries/documents/Projections_of_the_Prescription_Drug_Share_of_National_Health_Expenditures_June_2018.pdf

⁹ IQVIA. 2017 Medicine Use and Spending. April 2018.

articulate that CMMI may not unilaterally make permanent, structural changes to Medicare and Medicaid; and lay out a transparent model design and evaluation process.

Medicare Part D: PhRMA shares the Administration’s goals of strengthening the Part D benefit and lowering out-of-pocket costs for patients. Preserving the success of the program will require targeted and measured reforms that uphold Part D’s competitive, market-based structure and improve affordability without compromising beneficiaries’ access to medicines. Some reform proposals advanced by the Administration—including passing through to beneficiaries a share of negotiated rebates at the point of sale and establishing an annual maximum out-of-pocket (MOOP) spending limit—would provide immediate and visible financial relief to patients facing high pharmacy costs. Other proposals—specifically, changes to the protected classes, eliminating the two drugs per class requirement, and removing coverage gap discounts from the calculation of true out-of-pocket (TrOOP) spending—would harm access, increase costs for beneficiaries, and jeopardize the health of seniors and persons with disabilities.

Medicare Part B: The Medicare Part B benefit provides access to medicines for vulnerable patients who suffer from a range of serious illnesses and who often have few available treatment options through a structure that provides much needed flexibility for physicians to tailor treatment plans to optimize care for these patients. As HHS considers changes to this program, it will be critical to preserve beneficiary access to a range of treatment options and timely delivery of complex care at the site of service that is best for the patient. Increasing hospital consolidation is driving up the cost of care, for both Medicare and commercial patients, and we encourage CMS to consider approaches that would address this dynamic. At the same time, HHS should avoid increasing patient costs and reducing access by moving Part B covered drugs into the Part D benefit, or by relaunching the competitive acquisition program (CAP) in ways that impose formulary or utilization management tools that would block patients from getting the care they need and place administrative burden on physicians.

Medicaid and Affordable Care Act Taxes: Prescription medicines represent a small share of Medicaid spending and provide substantial value to the program.¹⁰ However, manufacturers’ Medicaid rebate liability and tax obligations have increased dramatically with implementation of the Affordable Care Act (ACA). Numerous government analysts and economists have documented the negative consequences of the Medicaid drug rebate program in shifting costs and increasing prices for other customers.¹¹ The Administration’s proposal to repeal the cap on Medicaid rebates at 100 percent of Average Manufacturer Price (AMP) is essentially a new tax on the industry and would not achieve the Administration’s goal to lower list prices; instead it would deepen the price distortions caused by the rebate program.

340B Drug Discount Program: PhRMA and our member companies strongly support the 340B program and the important role it plays in our health care safety net. The 340B program is particularly crucial to supporting the care provided by qualifying federally-funded clinics (known

¹⁰ Menges Group analysis of 2016 CMS 64 and state drug utilization files.

¹¹ The Council of Economic Advisers. Reforming Biopharmaceutical Pricing at Home and Abroad. February 2018.

as “grantees”), who are typically required to reinvest revenue derived from the 340B program into helping the communities they serve. In contrast, hospitals, which now account for the clear majority of 340B sales, have no such requirements. Hospitals’ use of 340B has led to growth in the program that economists have found is increasing costs for patients and the overall health care system. The program now needs to be updated to keep it on a sustainable footing. The Administration has authority to make reforms and should update its guidance on important aspects of the program: a clearer patient definition in line with the statute, meaningful limits on hospital child sites, reforms to the contract pharmacy policy, and eligibility standards for private hospitals. Additionally, we urge Health Resources and Services Administration (HRSA) and CMS to develop more comprehensive and effective duplicate discount prevention guidance.

Cost-Sharing Assistance Cards: Cost-sharing assistance cards have become a crucial lifeline for patients with commercial insurance who are increasingly facing high cost sharing for their medicines due to high deductibles or coinsurance. Manufacturers provide these cards as a response to an insurance benefit design system that would otherwise leave many patients abandoning their medicines at the pharmacy counter. Maintaining availability of cost-sharing assistance cards for patients should be a key part of the administration’s efforts to promote access to affordable medicines for patients. Thus, the Administration should not seek to change the current exclusion of cost-sharing assistance cards from the determination of the AMP and Best Price, as is contemplated in the RFI.

Value-Based Arrangements: PhRMA appreciates HHS’s recognition of the regulatory barriers that can inhibit value-based arrangements, and the recent action by FDA which made a significant advance towards removing one of these obstacles for manufacturer. We encourage HHS to address the remaining barriers by issuing an Anti-Kickback Statute safe harbor for value-based arrangements and clarifying the rules for reporting of Medicaid Best Price. We urge continued reliance on the market as the best mechanism for determining a medicine’s value, as many payers assess their own needs in light of available evidence, and avoidance of centralized government approaches that would harm patient access and lead to suboptimal outcomes. We also urge caution as HHS considers long-term financing approaches and indication-based coverage and pricing to ensure any of these approaches support continued innovation and patient access, as well as market-based competition.

National Spending Estimates: Estimates of national health care spending should accurately reflect spending on medicines net of aggregate discounts and rebates to inform policymakers as they make decisions regarding health care spending controls and other payment and reimbursement issues. Although projections of prescription medicine spending included in the National Health Expenditure (NHE) data attempt to capture spending on medicines net of discounts and rebates, they systematically overestimate prescription medicine spending.¹² The actuaries at CMS should reassess their methodology for projecting drug spending, consider

¹² CMS. Accuracy Analysis of The Short-Term (10-Year) National Health Expenditure Projections. February 2018.; PhRMA analysis of CMS. NHE 2016. December 2017.

reporting total drug spending instead of retail drug spending, and break out spending by ingredient costs versus distribution and supply chain costs.

Direct-to-Consumer Advertising: FDA should not pursue any required disclosure of list prices in direct-to-consumer (DTC) pharmaceutical advertising. Such a requirement could confuse patients since the list price often does not represent what they would actually be required to pay, and the requirement could also have the unintended and harmful consequence of deterring patients from seeking care. Moreover, any such requirement would raise significant legal issues including serious First Amendment concerns.

Biosimilar Development, Approval, Education, and Access: PhRMA members support the development and delivery of safe and effective biologics, including biosimilars. The approval pathway outlined in the Biologics Price Competition and Innovation Act of 2009 (BPCIA) and the implementation of the Biosimilar User Fee Act (BsUFA II) goals are helping to provide more predictable and timely access to biosimilar products that will result in increased biopharmaceutical competition in the marketplace. PhRMA urges targeted revisions to the Purple Book to provide more certainty and transparency for stakeholders, supports FDA's continued efforts to increase the public's understanding of both biologics and biosimilars, and encourages FDA to address PhRMA's comments on the draft guidance on interchangeability as it finalizes that guidance.

Availability of Reference Product Samples: Reference product sponsors should not deny access to product samples to delay generic or biosimilar entry. FDA could exercise its existing statutory authority to evaluate whether Risk Evaluation and Mitigation Strategies (REMS) have impacted the availability of generics or biosimilars and whether there are steps the agency might take to address any such issues without undermining the safety issues that resulted in the REMS. Although FDA should take appropriate measures within its current statutory authority, legislation may be useful to fully address product sample access issues.

Fixing Global Freeloading: Foreign governments mandate price controls and other harmful trade practices to artificially depress the market value of U.S. innovative medicines, resulting in U.S. patients bearing a disproportionate share of the cost to develop medical advances. Recognizing the global benefits of addressing free riding by other wealthy countries, PhRMA proposes four actions that this Administration could take to end the most unfair and discriminatory trade practices faced by the U.S. innovative biopharmaceutical industry: (1) Securing strong commitments in global, regional and bilateral negotiations (including the ongoing North American Free Trade Agreement (NAFTA) renegotiations) to drive and sustain 21st century biopharmaceutical innovation; (2) Enforcing and defending existing trade commitments (such as those negotiated with South Korea and Australia); (3) Ensuring that foreign government pricing and reimbursement policies are transparent, provide due process and appropriately value U.S. innovation; and (4) Leveraging all available trade tools to combat abuse of compulsory licensing.

* * * *

SECTION I: INNOVATION AND SPENDING ON MEDICINES

Medicines have revolutionized the treatment of numerous serious health conditions, saving lives, improving quality of life, and reducing the need for hospitalization.¹³ The U. S. is by far the global leader in the development of new medicines.¹⁴ American patients benefit from earlier and wider access to new medicines compared to patients in other countries, where governments restrict access.¹⁵ For example, nearly 90 percent of newly launched medicines from 2011 to 2017 were available in the U.S., compared to just two-thirds in the United Kingdom (U.K.), half in Canada and France, and one third in Australia.¹⁶

Continued advances in medicines are indispensable to addressing some of our society's biggest health and economic challenges.¹⁷ Likewise, better use of medicines, such as improved adherence to needed treatments, offers the opportunity for better results for patients and an estimated \$213 billion per year in health savings.¹⁸ Several policies identified in the RFI could help achieve these important results.

As medicines' role in effective health care has grown sharply and many new medicines have been brought to patients, retail and physician-administered medicines combined have remained 14 percent of total U.S. health spending.¹⁹ Biopharmaceutical innovator companies, which develop the safe and effective new medicines that improve patients' lives, accounted for less than half of all spending on prescription medicines—or about 7 percent of total health care spending in

¹³ PhRMA. A decade of innovation in rare diseases: 2005-2015. 2015. Available at: <http://phrma-docs.phrma.org/sites/default/files/pdf/PhRMA-Decade-of-Innovation-Rare-Diseases.pdf>; Lacey MJ, Hanna GJ, Miller JD, Foster TS, Russell MW; Truven Health Analytics. Impact of pharmaceutical innovation in HIV/AIDS treatment during the highly active antiretroviral therapy (HAART) era in the US, 1987-2010: an epidemiologic and cost-impact modeling case study. December 2014.; Roebuck MC. Medical cost offsets from prescription drug utilization among Medicare beneficiaries [commentary]. *J Managed Care Pharm.* 2014;20(10):994-995; Afendulis CC, Chernew ME. State-level impacts of Medicare Part D. *Am J Managed Care.* 2011;17 Suppl 12:S.

¹⁴ National Science Foundation, National Science Board, 2018. Available at: <https://www.nsf.gov/statistics/2018/nsb20181/data/appendix>; TEconomy for PhRMA, analysis of Pitchbook data. April 2018. Companies and Deals. PitchBook Data Inc.; European Commission. The 2016 EU industrial R&D Investment Scoreboard, 2016. Available at: <http://iri.jrc.ec.europa.eu/scoreboard16.html>

¹⁵ See, e.g., Zhang Y, Hana CH, Hernandez I. Comparing the Approval and Coverage Decisions of New Oncology Drugs in the United States and Other Selected Countries. *J Manag Care Spec Pharm.* 2017;23(2):247-254.

¹⁶ PhRMA analysis of IQVIA data.

¹⁷ Alzheimer's Association. Changing the trajectory of Alzheimer's disease: how a treatment by 2025 saves lives and dollars. 2015. Available at: https://www.alz.org/help-support/resources/publications/trajectory_report

¹⁸ IMS Institute for Healthcare Informatics. Avoidable costs in U.S. healthcare: the \$200 billion opportunity from using medicines more responsibly. June 2013.

¹⁹ Altarum Institute. Projections of the prescription drug share of national health expenditures including non-retail. May 2018.

2015.²⁰ Generic manufacturers and intermediaries in the pharmaceutical supply chain retain the other half of spending on medicines.²¹

The ability to bring important medical advances to patients while holding medicines' share of health spending nearly constant is made possible by the highly competitive structure of the U.S. market. Fierce market competition among medicines achieves sizable discounts from brand manufacturers and shifts utilization from brand drugs to generics and biosimilars.²² As a result of these forces:

- In 2017, total net drug spending grew just 0.6 percent, and prices for brand-name medicines increased 1.9 percent after discounts and rebates, even as many new treatments reached patients.²³
- In 7 of the last 10 years, net retail prescription drug costs grew more slowly than total health care costs—and, on average, spending for retail prescription drugs has grown more slowly than growth for other major types of care, and more slowly than total health expenditures.²⁴
- In 2017, 90 percent of all prescriptions filled were generics, up from 80 percent in 2011.²⁵ IQVIA projects U.S. brand sales will be reduced by \$105 billion due to competition from generics and biosimilars between 2018 and 2022.²⁶ There is no similar type of cost containment for other health care services.

While growth of net spending on acquiring medicines from manufacturers has been lower than other health care costs, and was lower than inflation in 2017,²⁷ multiple data sources show that (1) growth in manufacturer rebates and discounts that lower payers' cost of acquiring medicines has

²⁰ Berkley Research Group, *The Pharmaceutical Supply Chain: Gross Drug Expenditures Realized by Stakeholders*. January 2017. Available at: <http://www.thinkbrg.com/newsroom-publications-vandervelde-blalock-phrma.html>

²¹ In some instances, middlemen who played no role in a medicine's development and took no risk in purchasing it are paid more than the company that developed a medicine through years of research and clinical trials. A recent study reports that for 20 medicines administered in hospital outpatient departments commercial insurers pay hospitals up to three and a half times the medicines' acquisition cost. The Moran Company. *Hospital Charges and Reimbursement for Drugs: Analysis of Markups Relative to Acquisition Cost*. October 2017. While these markups are recorded as spending on drugs that typically is attributed to manufacturers in policy debates, in fact this is spending that is determined by and goes to middlemen, not spending that either goes to or is determined by biopharmaceutical companies.

²² Generics and biosimilars are a form of cost containment that applies only to the biopharma sector. For instance, the price of one widely used statin dropped by about 92 percent from 2005 to 2013 when generic versions came to market. Over the same period, the average charge for percutaneous transluminal coronary angioplasty, a surgical procedure to treat cardiovascular disease, increased by almost 66 percent.

²³ IQVIA. *2017 Medicine Use and Spending*. April 2018.

²⁴ PhRMA analysis of CMS. *NHE 2016*. December 2017.

²⁵ IQVIA. *2017 Medicine Use and Spending*. April 2018.

²⁶ *Id.*

²⁷ PhRMA analysis of CMS. *NHE 2016*. December 2017.; IQVIA. *2017 Medicine Use and Spending*. April 2018.

been substantial and (2) an increasing share of these discounts and rebates are retained by intermediaries involved in distributing and paying for prescription medicines:

- Compared to list price growth, rebates and other discounts reduced average net price growth for brand medicines by nearly three-quarters in 2017.²⁸
- The distribution chain accounts for a significant share of prescription drug spending, retaining more than one third of spending on brand medicines in 2015.²⁹
- Additionally, manufacturers' gross-to-net reductions³⁰ have more than doubled since 2012, totaling more than \$150 billion in 2017.³¹

This ongoing growth in the difference between the list and the actual net prices paid, combined with a shift of funds to the supply chain, can adversely affect patients using medicines. Health plans typically base patients cost sharing at the pharmacy counter on a medicine's list price rather than the lower discounted price paid by the plan when patients face deductibles or coinsurance. This contrasts with out-of-pocket spending for doctors and hospitals, which is based on negotiated rates. Notably, more than half of commercially insured patients' out-of-pocket spending for brand medicines is based on list price.³² We are encouraged that some payers recognize that sharing savings with patients at the pharmacy counter is a "best practice"³³ and have undertaken initiatives to do so, although to date they affect only a small share of patients. These changes should not, however, be paired with changes that increase the reliance on coinsurance, thereby reducing the potential for patient savings.

²⁸ IQVIA. 2017 Medicine Use and Spending. April 2018.

²⁹ Vandervelde A, Blalock E; Berkeley Research Group. The pharmaceutical supply chain: gross drug expenditures realized by stakeholders. 2017. Available at:

http://www.thinkbrg.com/media/publication/863_Vandervelde_PhRMA-January-2017_WEB-FINAL.pdf

³⁰ Defined as "rebates, off-invoice discounts, copay assistance, price concessions, and other reductions like distribution fees, product returns, the 340B Drug Pricing Program, and more." (Drug Channels Institute)

³¹ Fein AJ; Drug Channels Institute. The gross-to-net bubble topped \$150 billion in 2017. April 2018.

³² PhRMA. Commercially-insured patients pay undiscounted list prices for one in five brand prescriptions, accounting for half of out-of-pocket spending on brand medicines. Available at:

<http://www.phrma.org/report/commercially-insured-patients-pay-undiscounted-list-prices-for-one-in-five-brand-prescriptions-accounting-for-half-of-out-of-pocket-spending-brand-medicines>

³³ Seeking Alpha. Express Scripts Holding (ESRX) Q4 2016 Results – Earnings Call Transcript. February 15, 2017. Available at: <http://seekingalpha.com/article/4046365-express-scripts-holding-esrx-q4-2016-results-earnings-call-transcript>; Seeking Alpha. CVS Health (CVS) Q4 2016 Results – Earnings Call Transcript. February 9, 2017. Available at: <http://seekingalpha.com/article/4044425-cvs-health-cvs-q4-2016-results-earnings-call-transcript?part=single>

SECTION II: REBATES (RFI p. 22698)

The RFI correctly identifies a clear problem: while the current system of rebates, list prices, and net prices has constrained overall drug spending, it could work better for patients, payers, and manufacturers. Reforming this system will not be easy and we commend the Trump Administration for taking on this challenge. The drug channel, which is characterized by a complex system of money flows and proprietary contracts, has evolved over time with changes in drug benefits as well as changes in the role and structure of PBMs. Government reforms to this system should be made only after careful consideration of incentives the current system has created, which now appear to favor brand medicines with high list prices and large rebates over lower cost brand medicines. We recommend developing government policies that move to a system that either prohibits or discourages entities in the supply chain from retaining compensation based on a percentage of the list price of the drug. Given the complexity of the current system, transformational change is unlikely to occur immediately and major reforms will need to be phased in over time. A transition period will be necessary given the current complex set of contractual relationships between private entities in the supply chain.³⁴ Even so, moving to a system where the supply chain does not retain compensation based on a percentage of the list price may be simpler to operationalize than government policies aimed at a wholesale move away from rebates.

REBATES: Role of Rebates in the Current System (RFI p. 22698)

Rebates are the primary lever currently used to enable differential and competitive pricing for pharmaceuticals in the commercial market and in government programs.³⁵ Market observers note that with differential pricing, manufacturers may (in accordance with applicable laws) adjust the cost of a medicine to a payer based on a wide range of factors, such as formulary access, number of covered lives, patient adherence, and the value delivered by the medicine to patients and payers alike.³⁶ According to economic theory, a firm's ability to offer different prices to different purchasers typically enhances consumer wellbeing, particularly when it facilitates the expansion of service or increases the output of a good or service.³⁷ As an example, passengers sitting near each other on an airplane typically pay different prices for their flight, depending on the conditions under which they made the purchase, and on the value they derive from the flight. A business traveler that needs to be in a given location at a specific time will typically be charged more than a leisure traveler with more flexibility in his or her schedule. The airline's ability to

³⁴ For an overview of these complex relationships see Fein A. Follow the Dollar: The U.S. Pharmacy Distribution and Reimbursement System. *Drug Channels*. Feb. 3, 2016. Available at: <https://www.drugchannels.net/2016/02/follow-dollar-us-pharmacy-distribution.html>

³⁵ Vandervelde A, Blalock E; Berkeley Research Group. The pharmaceutical supply chain: gross drug expenditures realized by stakeholders. 2017.

³⁶ Greenwalt L. Declining Value of Rebate Efficiency. Amundsen Consulting. Available at: <https://www.marketingweb.iqvia.com/rebate-efficiency-payer-access/>

³⁷ Varian HR. (2007). Handbook of Industrial Organization, Chapter 8: Price Discrimination. Armstrong M, Porter R (Ed.). North Holland: Elsevier B.V.; Carlton D, Perloff J. (2000). Modern Industrial Organizations (3rd ed.). Reading, MA: Addison-Wesley.

charge different fares depending on the conditions of purchase facilitates the expansion of travel opportunities.

Differential pricing for medicines facilitates the expansion of sales to customers beyond those that might be willing (or able) to purchase them if payers were prevented from negotiating discounts based on the conditions of consumer demand for access to a wide range of medicines. In many cases, differential pricing is the result of robust negotiation between PBMs and manufacturers, who may negotiate favorable formulary placement and other coverage terms in exchange for steeper discounts.³⁸ Robust negotiation can thus expand access for patients, and as discussed earlier in this letter, has also helped constrain overall spending on medicines in the U.S. In fact, total drug spending grew just 0.6 percent in 2017 and prices for brand-name medicines increased 1.9 percent after discounts and rebates, even as many new treatments reached patients.³⁹ In contrast, in 2017 the consumer price index for medical care overall increased by 2.5 percent.⁴⁰

Current structure allows PBMs to retain significant share of rebates and other price concessions

While the current system has helped to control overall spending and allows for differential pricing, the growth in rebates may have created incentives for payers to favor medicines that carry higher rebates,⁴¹ thus leading to an environment in which list prices are rising rapidly even as net prices have held steady.⁴² This may be the result of the types of arrangements PBMs commonly negotiate with their health plan and employer clients, which allow PBMs to retain a portion of negotiated rebates and other price concessions as compensation for their services.⁴³ Because the portion of the rebate retained by the PBM, as well as the administrative fees they charge their clients, may be based on a percentage of a medicine's list price, PBMs may have incentives to establish formularies that favor medicines with high list prices and large rebates over lower cost medicines.⁴⁴ Under the current system, if a manufacturer were to independently lower the list price of a medicine and abandon the trend towards higher and higher rebates, the revenues PBMs earn on that medicine would likely decline.⁴⁵ Since PBMs can influence medicine affordability and availability through their decisions about formulary coverage,

³⁸ Roehrig, C. The Impact of Prescription Drug Rebates on Health Plans and Consumers. April 2018. Available at: <https://altarum.org/publications/the-impact-of-prescription-drug-rebates-on-health-plans-and-consumers>

³⁹ IQVIA. 2017 Medicine Use and Spending. April 2018.

⁴⁰ Bureau of Labor Statistics. Consumer price index—all urban consumers, history table. Available at: <https://www.bls.gov/cpi/tables/supplemental-files/historical-cpi-u-201801.pdf>

⁴¹ Hoey DB. Rebates to pharmacy benefit managers are a hidden contributor to high drug prices. November 2016. Available at: <https://www.statnews.com/2016/11/28/rebates-pharmacy-benefit-managers-contribute-high-drug-prices/>

⁴² IQVIA. Understanding the Drivers of Drug Expenditure in the U.S. September 2017.

⁴³ Roehrig C. The Impact of Prescription Drug Rebates on Health Plans and Consumers. April 2018.

⁴⁴ Hoey DB. Rebates to pharmacy benefit managers are a hidden contributor to high drug prices. November 2016.

⁴⁵ Johnson CY. In May, Trump predicted the pharmaceutical industry would cut prices in two weeks. It hasn't happened yet. *The Washington Post*. June 26, 2018. Available at: https://www.washingtonpost.com/news/wonk/wp/2018/06/25/in-may-trump-predicted-the-pharmaceutical-industry-would-start-cutting-prices-in-two-weeks-its-been-three/?noredirect=on&utm_term=.82a4ec23bbb9

utilization management, and formulary tier placement (which establishes cost sharing), a hypothetical manufacturer's unilateral decision to lower list price could result in a significant reduction in formulary access for that manufacturer and significantly impact affordability and access for patients.⁴⁶ This threat has been identified by Secretary Azar as an impediment to the Administration's goal of bringing list prices down.⁴⁷

The complex set of rebates and fees can make it difficult for payers to assess whether they are fully benefiting from all price concessions that PBMs negotiate. While a share of rebates is generally passed on to plan sponsors, smaller employers and health plans may not benefit from the price concessions negotiated by the PBM, particularly if the PBM decides not to classify certain fees or other concessions as 'rebates.' For example, one benefits consultant has observed that PBMs are increasingly changing the contractual definition of rebates to exclude certain administrative fees, allowing the PBM to retain these payments rather than passing them back to the plan sponsor. These administrative fees can be as high as 25 to 30 percent of the total amount paid in rebates and fees by the manufacturer to the PBM and in some cases may not be reported to the plan sponsor by the PBM.⁴⁸ Lack of transparency over PBM-retained fees in contracts between employers and PBMs has led many plan sponsors to question the share of rebate savings being passed through, how much the PBM is retaining for administrative fees, and whether the PBM is disclosing and passing on other price concessions, such as savings from price protection rebates.⁴⁹

Many patients do not directly benefit from significant price negotiations in the market today

Currently, savings generated from confidential price negotiations between manufacturers and payers do not always make their way directly to patients facing high cost sharing for their medicines. Unlike care received at an in-network hospital or physician's office, health plans typically base cost sharing for prescriptions filled in the deductible or with coinsurance on undiscounted list prices, rather than on prices that reflect negotiated rebates and discounts. Enrollment in high-deductible health plans and use of coinsurance for prescription medicines has grown sharply in recent years, increasingly exposing patients to high out-of-pocket costs based on undiscounted prices, creating scenarios in which medicines appear to be more costly than other

⁴⁶ Nisen M. Pharma's Quieter Price War Continues. *Bloomberg Businessweek*. August 3, 2017. Available at: <https://www.bloomberg.com/news/articles/2017-08-03/pbm-formularies-quieter-drug-price-war-continues>

⁴⁷ Alex Azar, HHS, Testimony Before the United States Senate Committee on Finance: Prescription Drug Affordability and Innovation: Addressing Challenges in Today's Market, June 26, 2018.; Alex Azar, HHS, Testimony Before the United States Senate Committee on Health, Education, Labor and Pensions: Full Committee Hearing: The Cost of Prescription Drugs: Examining the President's Blueprint 'American Patients First' to Lower Drug Prices, June 12, 2018.

⁴⁸ Dross D. Will Point-of-Sale Rebates Disrupt the PBM Business? *Mercer*. July 31, 2017. Available at: <https://www.mercer.us/our-thinking/healthcare/will-point-of-sale-rebates-disrupt-the-pbm-business.html>

⁴⁹ Midwestern Business Group on Health. Drawing a Line in the Sand: Employers Must Rethink Pharmacy Benefit Strategies. September 2017. Available at: https://higherlogicdownload.s3.amazonaws.com/MBGH/4f7f512a-e946-4060-9575-b27c65545cb8/UploadedImages/Specialty%20Pharmacy/DMJ_MBGH_Line_in_the_Sand_RV12_9617.pdf

health care services. High cost sharing is a cause for concern, as a substantial body of research clearly demonstrates that increases in out-of-pocket costs are associated with both lower medication adherence and increased abandonment rates, putting patients' ability to stay on needed therapies at risk.⁵⁰

Over the past 10 years, patient cost sharing has risen substantially faster than health plan costs. For workers with employer-sponsored health insurance, out-of-pocket spending for deductible and coinsurance payments increased by 230 percent and 89 percent, respectively, compared to a 56 percent increase in payments by health plans.⁵¹ Whereas cost sharing for prescription medicines once consisted almost entirely of copays, use of deductibles and coinsurance has increased rapidly, particularly for new medicines that represent the most innovative therapies and treat the sickest patients. The share of patient out-of-pocket drug spending represented by coinsurance more than doubled over the past ten years in the commercial market, while the share accounted for by deductibles tripled.⁵²

The increased share of total medication costs that patients are paying through deductibles and coinsurance exposes patients to undiscounted list prices and creates affordability challenges for many patients.⁵³ Patients enrolled in high-deductible health plans may be asked to pay thousands of dollars out of pocket before any of their prescriptions are covered, while patients with coinsurance are responsible for as much as 30 to 40 percent of the total cost of their medicines, reducing adherence to needed therapy.⁵⁴ Again, in sharp contrast, patients typically get access to payer-negotiated discounts on in-network hospital and physician office visits when they are in the deductible or required to pay coinsurance.

⁵⁰ IMS Institute for Healthcare Informatics. Emergency and Impact of Pharmacy Deductibles: Implications for Patients in Commercial Health Plans. September 2015; Doshi JA, Li P, Huo H, et al. High Cost Sharing and Specialty Drug Initiation Under Medicare Part D: A Case Study in Patients with Newly Diagnosed Chronic Myeloid Leukemia. *American Journal of Managed Care*. 2016;22(4 Suppl):S78-S86; Brot-Goldberg ZC, Chandra A, Handel BR, et al. What Does A Deductible Do? The Impact of Cost-Sharing on Health Care Prices, Quantities, and Spending Dynamics. NBER Working Paper 21632, October 2015; Eaddy MT, Cook CL, O'Day K, et al. How Patient Cost-Sharing Trends Affect Adherence and Outcomes. *Pharmacy & Therapeutics*. 2012;37(1):45-55.

⁵¹ Claxton G, Levitt L, Long M, et al. Increases in Cost-Sharing Payments Have Far Outpaced Wage Growth. Peterson-Kaiser Health System Tracker. October 4, 2017. Available at: <https://www.healthsystemtracker.org/brief/increases-in-cost-sharing-payments-have-far-outpaced-wage-growth/#item-start>

⁵² Claxton G, Levitt L, Long M; Kaiser Family Foundation. Payments for Cost Sharing Increasing Rapidly Over Time. Peterson-Kaiser Health System Tracker. April 2016. Available at: <http://www.healthsystemtracker.org/insight/examining-high-prescription-drug-spending-for-people-with-employer-sponsored-health-insurance/>

⁵³ Claxton G, Levitt L, Long M; Kaiser Family Foundation. Increases in cost-sharing payments continue to outpace wage growth. Peterson-Kaiser Health System Tracker. July 2018. Available at: <https://www.healthsystemtracker.org/brief/increases-in-cost-sharing-payments-have-far-outpaced-wage-growth/>

⁵⁴ Sky-High Deductibles Broke the U.S. Health Insurance System. *Bloomberg*. June 2018. Available at: <https://www.bloomberg.com/news/features/2018-06-26/sky-high-deductibles-broke-the-u-s-health-insurance-system>; 2017 Employer Health Benefits Survey. Kaiser Family Foundation. September 2017. Available at: <https://www.kff.org/report-section/ehbs-2017-section-9-prescription-drug-benefits/>

Payers themselves have begun to recognize that using the undiscounted price of a medicine to set cost sharing is problematic for patients: recent statements from the two largest PBMs note that high deductibles for medicines put patients in a “very difficult position” and indicate that sharing rebate savings directly with patients should be considered as a “best practice.”⁵⁵ In addition, several private health plans and PBMs have already announced that they plan to offer point-of-sale rebate sharing to their commercial clients, indicating that the technical capacity exists to share these savings and the operational challenges are not insurmountable.⁵⁶ These changes should not, however, be paired with changes that increase the reliance on coinsurance, thereby reducing the potential for patient savings.

Current structure results in patients subsidizing plan costs

Due to the growing gap between list and net prices, patients’ cost sharing for medicines is increasingly based on prices that do not reflect plan sponsors’ actual costs. For example, market analysts report that negotiated discounts and rebates can lower the net price of insulin by up to 50 to 70 percent,⁵⁷ yet health plans require patients with deductibles to pay the full undiscounted price. As a result, a patient in a high-deductible health plan who pays the list price each month for insulin may be paying hundreds—or even thousands—more annually than their insurer.

As a hypothetical example, imagine a patient enrolled in a high-deductible health plan who takes an insulin with a list price of \$400. The patient’s insurer has negotiated a 65 percent rebate, which substantially reduces the cost to the plan. However, because the patient has not yet met his deductible, his insurer does not provide any coverage for his prescription, and the patient’s bill reflects the insulin’s full cost of \$400. Despite paying nothing for this patient’s insulin, the insurer still collects the rebate, earning over \$200.⁵⁸

Unfortunately, as the number of patients with deductibles and coinsurance rises, this situation is becoming more common. Analysis by Amundsen Consulting shows that more than 55 percent of patients’ out-of-pocket spending for brand medicines is based on the list price of the medicine, even though their health insurer may be receiving a steep discount.⁵⁹

⁵⁵ Seeking Alpha. Express Scripts Holding (ESRX) Q4 2016 Results – Earnings Call Transcript. February 15, 2017.; Seeking Alpha. CVS Health (CVS) Q4 2016 Results – Earnings Call Transcript. February 9, 2017.

⁵⁶ Aetna to Offer Point-of-Sale Pharmacy Rebates to Three Million Customers. *Managed Care*, March 27, 2018; Johnson CY. UnitedHealthcare Will Provide Drug Rebates Directly to Members in Some Plans. *The Washington Post*, March 6, 2018.

⁵⁷ Barrett P, Langreth R. The Crazy Math Behind Drug Prices: Intermediaries that Negotiate to Lower Prices May Cause Them To Increase Too. *Bloomberg Businessweek*. June 29, 2017. Available at: <https://www.bloomberg.com/news/articles/2017-06-29/the-crazy-math-behind-drug-prices>; Langreth R, Keller M, Cannon C. Decoding Big Pharma’s Secret Drug Pricing Practices. *Bloomberg*. June 29, 2016. Available at: <https://www.bloomberg.com/graphics/2016-drug-prices>.; SSR Health. US Brand Net Pricing Growth 0.2% in 3Q17. December 18, 2017.

⁵⁸ PhRMA. Follow the Dollar. November 2017. Available at: <http://phrma-docs.phrma.org/files/dmfile/Follow-the-Dollar-Report.pdf>

⁵⁹ IQVIA. Patient Affordability Part One: The Implications of Changing Benefit Designs and High Cost-Sharing. May 2018. Available at: <https://www.iqvia.com/locations/united-states/patient-affordability-part-one>

Health plans typically use some portion of negotiated rebates to reduce premiums for all enrollees, rather than to directly lower costs for patients facing high cost sharing due to deductibles and coinsurance. According to one actuarial firm, this results in a system of “reverse insurance,” whereby payers require patients with high drug expenditures to pay more out of pocket, while rebate savings are spread out among all health plan enrollees in the form of lower premiums.⁶⁰ Asking sicker patients with high drug costs to subsidize premiums for healthier enrollees is the exact opposite of how health insurance is supposed to work.

Certain innovative contracting arrangements tied to clinical outcomes may require rebates

We support HHS’ efforts to encourage more innovative contracting arrangements, such as voluntary value-based arrangements between payers and manufacturers.⁶¹ It is important that efforts to reform government rules to address misaligned incentives be pursued in tandem with efforts to promote new approaches to value-based arrangements. In particular, arrangements in which price negotiations are tied to clinical outcomes would require the ability to provide a price concession after a drug is purchased. For example, a hypothetical manufacturer may independently agree to vary the final price of a medicine, so that a payer pays less if patients taking the medicine do not achieve certain health outcomes. In such a case, the manufacturer would adjust the final price paid by the payer using a rebate. As another example, a manufacturer might independently agree to provide an unlimited amount of a medicine to a payer for no more than a certain annual payment limit. This might also be most easily implemented through a rebate. In this case, the payer would continue to pay the pharmacy the usual price for the medicine, but once the agreed upon maximum payment amount is reached, the manufacturer would rebate back the full price of the medicine. Given the potential of such arrangements to drive improved efficiency for the health care system, reforms should allow for continued use of rebates or similar mechanisms in these circumstances.

REBATES: Principles for Government Reform of the Drug Distribution and Payment System (RFI p. 22694)

For the reasons described above, we share the concerns raised in the RFI that the current system’s incentives appear to favor high list prices with rebates instead of focusing on the net price.⁶² Changes are needed to ensure that the system works better for patients and does not leave them with artificially high out-of-pocket costs.

⁶⁰ Girod CS, Hart SK, Wertz S. 2017 Milliman Medical Index. May 2017. Available at: <http://www.milliman.com/uploadedFiles/insight/Periodicals/mmi/2017-milliman-medical-index.pdf>

⁶¹ FDA. Statement from FDA Commissioner Scott Gottlieb, M.D., on new efforts to advance medical product communications to support drug competition and value-based health care. June 12, 2018.

⁶² RFI p. 22694.

Below we suggest several goals that should guide any future government reform.

1. **Patients should benefit directly at the point of sale from negotiated rebates and other price concessions.** Patients in the deductible or facing coinsurance should pay cost sharing that reflects the steep discounts that many manufacturers provide to PBMs and payers. Their cost sharing should not be calculated based off the list price of the drug. Policy changes made to move towards providing this benefit to patients should be executed in a way that is cognizant of the benefits of keeping proprietary pricing information confidential, which the Federal Trade Commission has identified as important to the effective functioning of competitive markets.⁶³ The confidentiality of those agreements allows for vigorous negotiations that has helped hold net prices steady.⁶⁴
2. **Rebates should not be allocated solely to premiums.** In both Medicare Part D and most commercial coverage, rebate dollars are typically directed to lowering premiums instead of reducing cost sharing for patients who use prescription medicines. This means that patients taking medicines with large rebates are subsidizing coverage for other beneficiaries—which is effectively a tax on the sick.⁶⁵ Government policies should encourage rebate dollars to flow back to patients taking prescription drugs, either directly through rebate pass through (as discussed directly above) or through other means of enhancing the level of coverage provided by the prescription drug benefit.
3. **Payers should have sufficient tools and information to ensure PBM incentives are well aligned with plan interests.** Some PBM contracts with employers and group health plans offer little opportunity for assessing whether the PBMs incentives are well aligned with payer priorities and responsibilities to plan enrollees. For example, despite regulatory requirements, employers may not know the share of savings being retained by PBMs as administrative fees, or whether the PBM is sharing the benefit of other types of price concessions with employers, such as savings from price protection rebates.⁶⁶ Some contracts provide limited audit rights for payers. In other cases, payers simply may not know the specific questions to ask. Additional education for employers, such as sharing of best practices for engaging with PBMs, could help put payers on stronger footing when negotiating with PBMs. This could promote greater supply chain efficiency and help reduce overall spending on prescription drugs.
4. **Underlying incentives for compensation arrangements should discourage payment tied to list price.** Currently, PBMs, wholesalers, and pharmacies are often compensated as a percentage of list price, or in the case of PBMs, as a percent of rebates that are

⁶³ Koslov T, Jex E. Price Transparency or TMI? Federal Trade Commission, July 2, 2015.

⁶⁴ IQVIA. 2017 Medicine Use and Spending. April 2018.

⁶⁵ Girod CS, Hart SK, Weltz S. 2017 Milliman Medical Index. May 2017. Available at: <http://www.milliman.com/uploadedFiles/insight/Periodicals/mmi/2017-milliman-medical-index.pdf>

⁶⁶ Midwestern Business Group on Health. Drawing a Line in the Sand: Employers Must Rethink Pharmacy Benefit Strategies. September 2017.

themselves a percentage of the list price. Reforms to the current system should be made with an aim to move toward a compensation structure that is not linked to list price. As policymakers attempt to inject payment for value into all parts of our health care system, all participants in the drug supply chain can and should be paid based on the value they provide. As each of these participants—wholesalers, pharmacies, and providers of PBM services—deliver substantial value, they should be entitled to compensation based on that value. However, it does not make sense that their compensation is always, or even in most cases, proportional to the list price of a drug.

5. Medicare Part D reforms must be consistent with the noninterference clause.

Changes to the Part D program must not violate the noninterference clause in the Part D statute (Section 1860D-11 (i)(1) of the Social Security Act), which states that the Secretary may not “interfere with the negotiations between drug manufacturers and pharmacies and [stand-alone prescription drug plans (PDP)] sponsors.”

REBATES: Public Policy Changes to Improve the Current System (RFI p. 22698)

PhRMA recognizes that the current system needs to evolve and has advocated for several policy changes that would put the current system on a more sustainable path that would be better aligned with the needs of patients and payers.

We note that the current system is complex and care must be taken to avoid unintended consequences from government reform. It is important to recognize that transformational change of the type the Administration is proposing will take time and that reforms need to occur in a step-wise manner to avoid system disruption that would jeopardize patient care. Given the significance of the impact of these transformational changes to the system and to patients, it is critical that specific policy proposals be developed with engagement and feedback from all stakeholders. We also recognize that there may be other approaches that would be consistent with the principles outlined above, and we welcome other ideas and look forward to working with the Administration to improve the drug payment and distribution system.

Passing through rebates at the point of sale

The Administration could immediately lower out-of-pocket costs for millions of beneficiaries by requiring Part D plans to apply a substantial portion of negotiated rebates to reduce cost sharing at the point of sale. This government policy change is discussed in more detail in the Medicare Part D section of our comments. Several private insurers and PBMs have announced plans to offer point-of-sale rebate sharing to their commercial clients, signifying that the infrastructure and the capacity to implement this policy already exist.⁶⁷ Analysis that accounts for the potential anticipated behavioral changes from adoption of this policy shows that passing through of rebates

⁶⁷ Aetna to Offer Point-of-Sale Pharmacy Rebates to Three Million Customers. *Managed Care*. March 27, 2018; Johnson CY. UnitedHealthcare Will Provide Drug Rebates Directly to Members in Some Plans. *The Washington Post*, March 6, 2018.

could save the federal government money.⁶⁸ It could also improve adherence for conditions like diabetes, thereby generating savings in other parts of Medicare.⁶⁹ Separately, we also urge the Trump Administration to consider government reforms to encourage further take-up of rebate pass through in the commercial market. Actuarial research of the impact of rebate pass through in the commercial market has found that sharing negotiated savings could save certain commercially insured patients enrolled in plans with high deductibles and coinsurance between \$145 and \$800 annually, while increasing premiums by 1 percent or less.⁷⁰

Increasing PBM transparency and accountability

The RFI notes the Administration’s focus on incentives for intermediaries in the drug payment channel, such as PBMs. Specifically, the RFI asks whether PBMs should be obligated to act solely in the interest of the entity for which they manage pharmaceutical benefits, and what effect this “fiduciary duty” would have on PBM’s ability to negotiate drug prices. Fiduciary duty is a concept under Employee Retirement Income Security Act of 1974 (ERISA)—as regulated by the Department of Labor (DOL)—that is linked to the functions an entity performs with respect to a group health plan. For example, an employer plan sponsor is often a fiduciary of its group health plan because it exercises discretion or control over administering the plan.⁷¹ Fiduciary duty may be one potential option to address PBM incentives, depending on implementation. However, the Administration should consider a range of federal policy options that could help drive market-based approaches to greater efficiency and better alignment of PBM incentives with payer interests. We recommend that HHS work with DOL to explore opportunities to increase PBM accountability to their plan sponsors in the commercial market, and that HHS consider additional opportunities to increase accountability in Medicare Part D. For instance, in the employer market, the Administration could consider increasing PBM reporting requirements to include certain information about their compensation structure to group health plan sponsors.⁷² Similarly, in Medicare Part D, CMS can use its authority to expound upon what types of arrangements between Part D plan sponsors and first-tier entities (such as PBMs) are “acceptable to CMS.”⁷³ We support the Administration’s efforts to ensure that the drug payment system is efficient and effective for plan sponsors and patients across markets. We recommend that the agency engage

⁶⁸ Milliman. Reducing Part D Beneficiary Costs Through Point-of-Sale Rebates. January 2018. Available at: <http://www.phrma.org/report/reducing-part-d-beneficiary-costs-through-point-of-sale-rebates>

⁶⁹ IHS Markit. Passing a Portion of Negotiated Rebates Through to Seniors with Diabetes Can Improve Adherence and Generate Savings in Medicare. May 14, 2018.

⁷⁰ Bunge A, Gomberg J, Petroske J. Sharing Rebates May Lower Patient Costs and Likely Has Minimal Impact on Premiums. October 12, 2017. Available at: <http://www.phrma.org/report/point-of-sale-rebate-analysis-in-the-commercial-market>

⁷¹ Service providers, such as PBMs, have typically not been viewed as fiduciaries, but instead as parties in interest subject to certain arrangements with plans.

⁷² The 2014 ERISA Advisory Council recommended that DOL consider extending regulations to health and welfare plan arrangements with PBMs, and thereby deem such arrangements “reasonable” only where PBMs disclose direct and indirect compensation. PBM Compensation and Fee Disclosure. *Advisory Council on Employee Welfare and Pension Benefit Plans Report*. November 2014.

⁷³ Part D regulations define “first-tier entity” as “any party that enters into a written arrangement, acceptable to CMS, with a Part D plan sponsor or applicant to provide administrative services or health care services for a Medicare eligible individual under Part D.” 42 CFR § 423.501 (emphasis added).

with all relevant stakeholders as specific proposals are developed to more fully assess the full implications of any particular approach.

Delinking supply chain payment from list price

The questions in the RFI suggest that HHS is considering how rebates may be contributing to the rise in list prices. As discussed above, currently all intermediaries in the pharmaceutical supply chain profit from higher list prices, while patients are often left paying cost sharing based on the higher list price.⁷⁴ HHS has asserted that these incentives have contributed to the rise in list prices even as net prices have remained stable.⁷⁵ PhRMA shares HHS' concerns about incentives created by the current system and believes reforms focused on delinking payment for intermediaries from the list price may be simpler to operationalize than government policies aimed at a wholesale move away from rebates.

Instead of enacting policies that would eliminate rebates altogether, HHS could focus on reforms to either prohibit or discourage entities in the supply chain from receiving fees for services based on a percentage of the list price of a drug. For example, regulatory reforms could require PBMs, wholesalers and pharmacies be paid a flat fee. This shift could have several advantages. It could help make supply chain intermediaries less sensitive to changes in list prices and thus could help realize HHS' goal of lowering list prices.⁷⁶ PhRMA, as a trade association, is not involved in and cannot comment on the individual pricing decision of our members. HHS has, however, noted its concerns that the current system—in which robustly negotiated rebates are tied to a percentage of list price—deters decreases in list price.⁷⁷ In a recent article, Adam Fein, an expert on the pharmaceutical supply chain, stated that it would be “difficult, perhaps impossible,” to lower list prices because “cutting the list price means wholesalers make less money, pharmacies make less money, PBMs make less money and payers get fewer rebate dollars.”⁷⁸ Government reforms that support a move away from supply chain payment based on a percentage of list price could also push more of the rebate through to the plan sponsors. Plan sponsors should then use that savings to pass through a share of the rebate and devote some of the remaining money towards lower prescription drug cost sharing more generally.

⁷⁴ PhRMA. Commercially-insured patients pay undiscounted list prices for one in five brand prescriptions, accounting for half of out-of-pocket spending on brand medicines. 2017. Available at: <https://www.phrma.org/report/commercially-insured-patients-pay-undiscounted-list-prices-for-one-in-five-brand-prescriptions-accounting-for-half-of-out-of-pocket-spending-brand-medicines>

⁷⁵ RFI p. 22698.; IQVIA Institute for Human Data Science. Understanding the Drivers of Drug Expenditure in the U.S. September 2017.

⁷⁶ RFI p. 22698.

⁷⁷ Alex Azar, HHS, Testimony Before the United States Senate Committee on Finance: Prescription Drug Affordability and Innovation: Addressing Challenges in Today's Market, June 26, 2018.; Alex Azar, HHS, Testimony Before the United States Senate Committee on Health, Education, Labor and Pensions: Full Committee Hearing: The Cost of Prescription Drugs: Examining the President's Blueprint 'American Patients First' to Lower Drug Prices, June 12, 2018.

⁷⁸ Johnson C. In May, Trump predicted the pharmaceutical industry would cut prices in two weeks. It hasn't happened yet. *Washington Post*. June 25, 2018.

REBATES: The Role of the Anti-Kickback Statute Safe Harbors (RFI p. 22698)

Any changes to the Anti-Kickback Statute safe harbors would have to be approached with caution

The RFI raises the possibility of revising the discount safe harbor to “restrict the use of rebates and reduce the effect of rebates on list prices.”⁷⁹ However, the discount safe harbor is limited as a tool to address the misaligned incentives in the drug channel.

First, and most importantly, the Anti-Kickback Statute applies only to Federal health care programs.⁸⁰ Changes to the discount safe harbor would not directly impact the commercial market. As noted above, the need for government action to reform the privately-negotiated rebate system spans both the commercial market and Medicare Part D.

Second, the discount safe harbor is just one part of a complex statutory and regulatory framework. The Federal Anti-Kickback Statute is a criminal law that broadly prohibits the knowing and willful offer, solicitation, payment, or receipt of anything of value to induce the purchase of an item or service paid for by a federal health care program.⁸¹ Recognizing that, by its terms, the statute was overly broad, Congress enacted ten statutory exceptions (including a broad exception for discounts) and authorized the promulgation of additional regulatory safe harbors. Currently, there are 28 such safe harbors, including separate safe harbors for discounts and rebates, administrative fees, service fees, and discounts to certain managed care organizations (MCOs), among others. Thus, the discount safe harbor is just one of many available safe harbors that may apply to the complex set of arrangements within the drug channel.⁸²

As noted above, we encourage the Administration to think holistically about the complicated system of money flows and contracts within the system, not simply about rebates. Moreover, the administration must recognize that changes to that complicated system cannot be accomplished effectively by immediately disrupting that system. To that end, any changes to the Anti-Kickback Statute safe harbors should be undertaken with caution, and only after careful consideration of the following principles. Consider whether such changes:

1. **Provide clarity on all drug channel payments.** Because PBMs negotiate rebates, administrative fees, and other service fee arrangements, they may rely on a variety of safe harbors or statutory exceptions for protection. If safe harbor protection in one category were removed, they could conceivably shift arrangements from one category to another in order to continue to extract payment and attempt to maintain safe harbor protection.⁸³

⁷⁹ RFI p. 22698.

⁸⁰ 42 U.S.C. § 1320a-7b(b).

⁸¹ 42 U.S.C. § 1320a-7b(b).

⁸² Moreover, failure to satisfy the requirements of a safe harbor does not render an arrangement illegal; rather, these arrangements are subject to a case-by-case evaluation. 64 FR 63518, 63546 (Nov. 19, 1999).

⁸³ In a parallel example of the shifting between rebates and administrative fees, one benefits consultant has observed that PBMs are increasingly changing the contractual definition of rebates to exclude certain administrative fees, allowing the PBM to retain these payments rather than passing them back to the plan sponsor. Dross D. Will Point-of-Sale Rebates Disrupt the PBM Business? *Mercer*. July 31, 2017.

Stakeholders therefore need clear guidance regarding all channel arrangements including administrative fees and service fees, not rebates alone. Moreover, as described above, we suggest that any reforms of the safe harbors be undertaken with the goal of delinking compensation based on list price (including both fees and rebates) throughout the supply chain, and not focus solely on rebates.

2. **Sufficiently encourage PBM compliance.** Because lack of safe harbor protection does not necessarily render an arrangement illegal, and large PBMs both have incredible market power and may not have been subject to the same level of enforcement scrutiny historically as other stakeholders, it is critical that the Administration take steps to ensure PBM compliance, such as enhanced oversight of PBMs, penalties for PBM non-compliance, and a clear articulation of the Administration’s expectation that PBMs comply with the terms of any new guidance.
3. **Provide ample time for implementation.** Finally, meaningful change will require renegotiation of contracts throughout the drug channel. Because the Anti-Kickback Statute is a criminal law, with criminal penalties, any policy attempting to use the safe harbors as a lever should recognize the magnitude of this change and provide ample time for contract renegotiation and implementation—for example, two plan years or such longer period as may be required for expiration of existing contracts.

SECTION III: DRUG PRICING DEMONSTRATIONS (RFI p. 22694)

PhRMA recognizes HHS’s interest in developing “demonstration projects to test innovative ways to encourage value-based care and lower drug prices.” As HHS works to develop directions for CMS, it will be important to consider that drugs are a small, stable share of overall health care spending, which lead to savings in other parts of the health care system, and to focus on holistic approaches to improving cost and value of care. Total retail and non-retail drug spending is expected to remain constant at about 14 percent of total health care expenditures from 2015 through 2025, even as many new treatments reach patients.⁸⁴

It will also be important to establish in rulemaking the appropriate role for CMMI, and to recognize which demonstrations can be successfully and appropriately implemented by CMMI, given its authority. CMMI should not be used to undermine key patient protections and important structural elements of public programs. In addition, CMMI is not the ideal place for tests of value-based arrangements, as CMMI does not have the necessary authority to address key barriers that can impede these arrangements. Instead, as discussed below, CMS should provide regulatory relief from the barriers to value-based arrangements—including lack of clarity in Federal price

Available at: <https://www.mercer.us/our-thinking/healthcare/will-point-of-sale-rebates-disrupt-the-pbm-business.html>

⁸⁴ CMS. NHE Data.; Altarum Institute, A Ten Year Projection of the Prescription Drug Share of National Health Expenditures Including Non-Retail. October 2014, addendum update May 2017.

reporting metrics, and lack of clear protection for value-based arrangements under the Anti-Kickback Statute.⁸⁵

Prioritize holistic models that address broader cost drivers and quality of care deficits

The goal of payment and delivery reforms should be to improve care for patients, first and foremost. This includes ensuring that patients are well-informed about their health care options, have access to the full range of treatment options, and are engaged in their treatment choices. It is also important to recognize that medicines are a small share of overall health care spending. Total retail and non-retail drug spending grew just 0.6 percent in 2017⁸⁶ and is expected to remain constant at about 14 percent of total health care expenditures from 2015 through 2025,⁸⁷ even as many new treatments reach patients. Because holistic models can focus on overall health care spending, including administrative costs, they offer a greater opportunity for meaningful savings.

Holistic models allow medicines to demonstrate their value through offsets in other parts of the health care system, which are generally a result of better patient outcomes. For example, patients who were adherent to prescribed medicines for four chronic conditions (heart failure, hypertension, diabetes and dyslipidemia) exhibited savings of \$3 to \$10 in non-drug spending for each additional dollar spent on medicines, due to fewer emergency department visits and inpatient hospital days.⁸⁸ Similarly, patients with rheumatoid arthritis (RA) who responded to tumor necrosis factor inhibitors had lower all-cause medical, pharmacy, and total costs (excluding biologics) up to 3 years from initiation of therapy.⁸⁹ As CMS considers new models, it is important to recognize and include the role that prescription drugs can play in improving quality of life for beneficiaries and reducing system-wide costs.

It is also important to ensure that reforms do not inadvertently drive provider consolidation. As the CMS Administrator herself and other leaders within the Department have noted, "The complexity of many of the current [CMMI] models might have encouraged consolidation within the health-care system, leading to fewer choices for patients."⁹⁰ The impact assessment from the Merit-Based Incentive Payment System (MIPS) proposed rule also shows how value-based payment, if not properly constructed, can have a disproportionately negatively impact on smaller practices. CMS estimated that 87.0 percent of solo practitioners and 69.9 percent of 2-9 clinician groups would have a negative MIPS adjustment, compared to 18.3 percent of groups

⁸⁵ Detail about how to address the barriers to value-based arrangements is available in the following section: VALUE-BASED ARRANGEMENTS: Value-Based Arrangements and Price Reporting.

⁸⁶ IQVIA. 2017 Medicine Use and Spending. April 2018.

⁸⁷ CMS. NHE Data.; Altarum Institute. Projections of the prescription drug share of national health expenditures including non-retail. May 2018.

⁸⁸ Roebuck MC, et al. Medication adherence leads to lower health care use and costs despite increased drug spending. *Health Affairs*. 2011;30(1):91-9

⁸⁹ Grabner M, Boytsov NN, et al. Costs associated with failure to respond to treatment among patients with rheumatoid arthritis initiating TNFi therapy: a retrospective claims analysis. *Arthritis Research & Therapy*. 2017;19(1): 92.

⁹⁰ Verma S. Medicare and Medicaid Need Innovation. *W.S.J.* September 19, 2017. Available at: <https://www.wsj.com/articles/medicare-and-medicare-need-innovation-1505862017>

with 100 or more eligible clinicians.⁹¹ Concerns over this expected impact ultimately led to a less stringent MIPS final rule.

Provider consolidation increases costs for the health care system at large. Generally, a one percentage point increase in the proportion of medical providers affiliated with hospitals and/or health systems was associated with a 34 percent increase in average annual costs per person and a 23 percent increase in average per person price of treatment.⁹² Physician-administered chemotherapy medicines are an example of how the shift from the community to hospitals contributes to higher spending. From 2004 to 2014, chemotherapy infusions in hospital outpatient departments increased dramatically—from 6 to 46 percent for commercial patients and from 16 to 46 percent for Medicare patients. Drug spending was more than twice as high in the hospital setting. Had this consolidation not occurred, spending would have been 5.8 and 7.5 percent lower for commercial and Medicare infused chemotherapy patients, respectively.⁹³ Market-driven reforms that improve coordination at the provider level can help to address these challenges.

We offer several recommendations for holistic models that CMS might pursue in our comments on the CMMI New Direction RFI (New Direction RFI).⁹⁴

Codify CMMI “Guiding Principles” in Rulemaking

PhRMA supports the establishment of Guiding Principles for CMMI in the Innovation Center New Direction RFI,⁹⁵ and encourages CMMI to codify its guiding principles through formal notice and comment rulemaking prior to issuing any new demonstrations to improve the predictability and transparency of the model testing process. CMMI model tests must maintain protections for beneficiaries and achieve scientific rigor. To achieve this, CMMI should strengthen the processes and standards it uses to test new payment and delivery models. Establishing principles in regulation will facilitate more effective collaboration with stakeholders across the health care industry by clearly communicating requirements for CMMI model tests. It would also reduce regulatory burdens by providing greater predictability in future payment policy. Most importantly, these guidelines will help to minimize potential unintended consequences for beneficiaries.

⁹¹ CMS. Medicare Program; MIPS and Alternative Payment Model (APM) Incentive Under the Physician Fee Schedule, and Criteria for Physician Focused Payment Models. Proposed Rule. May 9, 2016. 81 FR 28372. Table 64.

⁹² Health Care Cost Institute & National Academy for State Health Policy. The Impact of Provider Consolidation on Outpatient Prescription Drug-Based Cancer Care Spending. April 2016.

⁹³ Kathryn F, et al. Cost Drivers of Cancer Care: A Retrospective Analysis of Medicare and Commercially Insured Population Claim Data 2004-2014. Milliman. 2016.

⁹⁴ PhRMA. Comment Letter responding to CMS, Innovation Center New Direction. November 20, 2017. Available at: <https://www.phrma.org/policy-paper/request-for-information-center-for-medicare-and-medicare-services-innovation-center-new-direction>.

⁹⁵ CMS. Innovation Center New Direction. September 2017.

Regulation should be used appropriately to implement the statute and to provide clarity on how agencies will apply the law. Many aspects of CMMI's authorizing statute have yet to be clearly defined, such as the parameters of a Phase I test. To effectively implement the CMMI guiding principles outlined in the New Direction RFI, like small scale, voluntary testing, CMMI could issue regulations that explain how it will define model populations and provide much-needed clarity and predictability. Regulations should also clearly articulate how CMMI will work with Congress to establish proof of concept for models and make recommendations for changes to the Medicare and Medicaid programs, and to clarify that CMMI may not unilaterally make permanent, structural changes to Medicare and Medicaid.

CMMI is also required by law to collect input from interested parties through open door forums or other mechanisms, and has engaged stakeholders at various points through meetings, RFI's, and technical expert panels. However, CMMI has never publicly described a process that it will consistently follow for engaging stakeholders in model development, implementation and evaluation. As a result, the level of interaction between CMMI and stakeholders has been inconsistent across models. Some models, like the Oncology Care Model (OCM), were developed over a period of years with multiple opportunities for public comment. Others, like the Comprehensive Care for Joint Replacement (CJR) model, were rapidly deployed with limited opportunities for input. CMMI should publish regulations outlining the process for model development and stakeholder engagement to help to address this concern.

PhRMA encourages CMMI to establish regulations that define small scale, voluntary, and limited duration testing; clearly articulate that CMMI may not unilaterally make permanent, structural changes to Medicare and Medicaid; and lay out a transparent model design and evaluation process. We also encourage CMMI to consider principles for use of waivers, facilitating access to medical innovation, and protecting commercially sensitive information. Our specific recommendations for the guiding principles are outlined in our comments on the CMMI New Direction RFI.⁹⁶

Address barriers to innovative value-based arrangements through regulatory changes

We note that HHS may be considering a test of value-based purchasing arrangements among “demonstration projects to test innovative ways to encourage value-based care and lower drug prices.”⁹⁷ As the health care market shifts to demand that providers and other stakeholders share greater risk for the cost of care, insurers are increasingly pursuing value-based arrangements with biopharmaceutical manufacturers. Value-based arrangements have potential to benefit patients and the health care system by improving health outcomes and other endpoints that matter to patients, reducing medical costs, and reducing the cost of medicines.

⁹⁶ PhRMA. Comment Letter responding to CMS, Innovation Center New Direction. November 20, 2017. Available at: <https://www.phrma.org/policy-paper/request-for-information-center-for-medicare-and-medicaid-services-innovation-center-new-direction>.

⁹⁷ RFI p. 22694.

As we describe below, permanent changes are needed to enable an expansion of value-based arrangements both inside and outside of federal health care programs, and CMMI is limited in its ability to address the barriers to value based arrangements. Medicaid Best Price is outside of CMMI's authority. While CMMI can waive the Anti-Kickback Statute, such a waiver could discourage beneficial VBCs outside of CMMI, which would not have the benefit of an Anti-Kickback Statute waiver. We recommend creation of a new safe harbor to the federal Anti-Kickback Statute. If the Administration believes that these regulatory changes must be evaluated, the U.S. Government Accountability Office (GAO) could conduct a study after such regulatory changes are in place, to evaluate the impact, including the impact on manufacturer outcomes.

We also discuss below other key considerations in Medicare Parts B and D that may be relevant to demonstrations that HHS is considering.

SECTION IV: MEDICARE PART D (RFI p. 22694)

For more than a decade, Medicare Part D has successfully provided seniors comprehensive prescription drug coverage with low and stable premiums, and its unique market-based structure has kept overall program costs far below initial projections. With the multitude of changes that have taken place in the insurance and pharmaceutical markets over the past 10 years, it makes sense to now consider whether Part D is due for a “tune up.” PhRMA is pleased that the Administration has expressed interest in modernizing Part D and we share the Administration's goal of updating and improving the Part D benefit. We believe that any reforms of Part D should be developed with a focus on ensuring that Medicare beneficiaries have access to and can afford the medicines they need, no matter what health conditions they are facing. Today, exposure to high cost sharing—often tied to an undiscounted “list price” for the medicine—presents affordability challenges that jeopardize patient adherence to needed medicines, which in turn increases costs in other parts of the Medicare program. PhRMA believes that reforms are needed both to improve affordability for beneficiaries facing high out-of-pocket costs at the pharmacy counter, and to realign and strengthen incentives to ensure long-term program sustainability.

It is critical that improvements to Medicare Part D be undertaken in the right way, with targeted and measured reforms that protect beneficiaries' access to medicines they need. Some reform proposals advanced by the Administration—including passing through to beneficiaries a share of negotiated rebates at the point of sale and establishing an annual MOOP spending limit—would provide immediate financial relief to patients facing high pharmacy costs. Other proposals—specifically changes to the protected classes, eliminating the two drugs per class requirement, and removing coverage gap discounts from the calculation of TrOOP spending—would harm access, increase costs for beneficiaries, and jeopardize the health of seniors and persons with disabilities.

MEDICARE PART D: Growing Affordability Challenges Threaten Patients' Access to Medicines (RFI p. 22695)

Despite consistently low and stable premiums in Medicare Part D, many beneficiaries experience high out-of-pocket expenses for prescription medicines. Increasingly, Part D beneficiaries are exposed to high and unpredictable cost sharing, with no limit on the amount they are required to pay out of pocket each year. For beneficiaries with a serious illness or multiple chronic conditions, out-of-pocket expenses for prescription medicines can easily add up to many thousands of dollars annually, resulting in seniors with chronic or life-threatening illnesses such as diabetes, schizophrenia, multiple sclerosis, and cancer *walking away from the pharmacy counter without filling vital prescriptions*.⁹⁸ High cost sharing for medicines puts patients at risk of delayed treatment initiation, gaps in therapy, and premature discontinuation, which research has consistently shown leads to poor health outcomes, increased use of hospital services and other costly medical care, and higher overall Medicare spending.

1. High Cost Sharing, Not Premiums, Drives Affordability Challenges in Part D

The average Part D premium has been growing at a low rate since the program's inception and is substantially lower than initial projections. Part D premiums average just \$33.50 a month in 2018, slightly less than the average premium in 2017. Low and stable Part D premiums are one key reason why the program has been so popular, with several surveys showing that about 90 percent or more of Part D beneficiaries are satisfied with their coverage.⁹⁹ However, if current cost-sharing trends continue, and the affordability challenges many patients are facing are not addressed, the popularity of the program could soon begin to erode.

Given Part D plan sponsors' strong incentive to keep premiums low, they use the rebates they negotiate with pharmaceutical manufacturers to reduce overall plan costs, rather than to directly reduce beneficiary cost sharing at the pharmacy counter. At the time Part D was implemented, CMS believed plan sponsors would apply a portion of the rebate savings negotiated for a medicine directly at the point of sale, thereby lowering the cost sharing for beneficiaries taking that medicine. However, the agency has observed that plans seldom share rebate savings directly with patients, choosing instead to apply aggregate rebate savings as direct and indirect remuneration (DIR) at the end of the year to reduce overall benefit costs and lower premiums for all enrollees.¹⁰⁰ As noted earlier in our letter, according to one actuarial firm, this practice of using savings from negotiated rebates to keep premiums low has led to a system of "reverse insurance," whereby plans require patients with high drug expenditures to pay more out of pocket, while rebate savings are spread out among all beneficiaries in the form of lower

⁹⁸ Amundsen Consulting. Medicare Part D Abandonment: Deep Dive into Branded Product Abandonment. November 2017.

⁹⁹ Morning Consult Survey for Medicare Today. Nearly Nine in 10 Seniors Satisfied with Medicare Part D. July 2017; MedPAC. Report to the Congress: Medicare Payment Policy. Chapter 15: Status Report on Part D. March 2013.

¹⁰⁰ 82 Fed. Reg. at 56419.

premiums.¹⁰¹ *In effect, not sharing a portion of the rebate savings with beneficiaries at the point of sale has resulted in chronically ill Medicare patients with high drug costs subsidizing premiums for healthier enrollees, which is the inverse of how health insurance is intended to work.*

2. For Many Part D Beneficiaries, Cost Sharing for Medicines is Unpredictable and Unaffordable

Beneficiaries have higher and more unpredictable out-of-pocket costs for their medicines in Medicare Part D than for the hospital and physician services they receive in Parts A and B. Those enrolled in fee-for-service Medicare have the option of purchasing supplemental Medigap coverage to limit their Parts A and B out-of-pocket costs. In addition, Medicare Advantage (MA) enrollees have the added benefit of an annual out-of-pocket spending limit for A and B services. These options are not available in the Part D program and beneficiaries have no such safeguards against high out-of-pocket costs. Instead, they face multiple affordability challenges including high cost sharing for brand prescriptions, high annual out-of-pocket costs, and the uneven distribution of out-of-pocket costs throughout the year.

High Cost Sharing for Brand Prescriptions

Financial barriers to treatment are particularly acute for Part D beneficiaries whose medicines are subject to coinsurance (cost sharing set as a percentage of the medicine's cost), particularly when those drugs are covered on a plan's non-preferred or specialty drug tiers. Most Part D plan sponsors impose up to 33 percent coinsurance for medicines on the specialty tier and coinsurance for non-preferred tier medicines can be as high as 40 to 50 percent.¹⁰² What's more, the coinsurance percentage is typically applied to a medicine's undiscounted "list price," even if the Part D sponsor or their PBM has negotiated a substantial rebate for the product.

High coinsurance rates impose a substantial financial burden for beneficiaries, who are typically living on modest or fixed incomes. It is not uncommon for beneficiaries who do not receive the low-income subsidy (LIS) to find out that the required cost sharing for their brand medicines is \$250 or more. For example, more than half of all new brand osteoporosis prescriptions, more than 40 percent of all new brand autoimmune and oral antidiabetic prescriptions, and more than 30 percent of all new brand antipsychotic prescriptions brought to a pharmacy in 2016 had cost sharing greater than \$250.¹⁰³ Not surprisingly, many of these prescriptions went unfilled. Requiring plan sponsors to pass through a share of the negotiated rebates at the point of sale would immediately lower out-of-pocket costs for millions of beneficiaries currently paying coinsurance for their brand medicines.

¹⁰¹ Girod CS, Hart SK, Weltz S. 2017 Milliman Medical Index. May 2017. Available at: <http://www.milliman.com/uploadedFiles/insight/Periodicals/mmi/2017-milliman-medical-index.pdf>

¹⁰² Cubanski J, Damico A, Neuman T. Medicare Part D in 2018: The Latest on Enrollment, Premiums and Cost-Sharing. Kaiser Family Foundation. May 2018.

¹⁰³ Amundsen Consulting. Medicare Part D Abandonment: Deep Dive into Branded Product Abandonment. November 2017.

High Annual Out-of-Pocket Costs

Analysis by the Kaiser Family Foundation shows that more than one million non-LIS beneficiaries incurred out-of-pocket spending high enough to reach catastrophic coverage in 2015, more than twice the number in 2007.¹⁰⁴ Annual out-of-pocket expenses for these patients were significant—more than \$3,000, on average—and exceeded \$5,200 for one in 10 beneficiaries. Such high out-of-pocket expenses are persistent from year to year for patients with complex health care needs: Medicare Payment Advisory Commission (MedPAC) analysis indicates that 70 percent of beneficiaries who reached catastrophic coverage in one year reached it in the following year as well.¹⁰⁵

Many Medicare beneficiaries, including seniors and individuals with disabilities, live on modest fixed incomes. In 2016, the median per capita income for the Medicare population was \$26,200 and more than a quarter of beneficiaries had incomes below \$15,250.¹⁰⁶ With no limit on annual out-of-pocket spending in Part D, even patients who reach catastrophic coverage continue to face high out-of-pocket costs. For the more than one million non-LIS beneficiaries who reached catastrophic coverage in 2015, 40 percent of their total out-of-pocket spending occurred in the catastrophic portion of the benefit.¹⁰⁷ Establishing an annual out-of-pocket spending limit in Part D would provide true catastrophic coverage for beneficiaries with multiple chronic conditions and significant, life-threatening illnesses.

Uneven Distribution of Out-of-Pocket Costs

Expenses for beneficiaries with high annual out-of-pocket costs are heavily concentrated at the beginning of each calendar year. Patients with a serious illness or multiple chronic conditions can rapidly progress through the deductible, initial coverage phase, and the coverage gap within a month or two, resulting in a large upfront cost burden. For example, one study found that Part D beneficiaries with RA, multiple sclerosis, or chronic myeloid leukemia (CML)—whose average annual out-of-pocket spending ranged from \$3,900 to \$6,300—incurred 25 to 40 percent of these costs in January alone and between 54 and 66 percent of these costs in the first three months of the year.¹⁰⁸ According to the authors, the average out-of-pocket cost for the first prescription filled during the calendar year “nearly equaled or exceeded the average monthly social security benefit” for two of these three conditions. A policy that allowed beneficiaries to more evenly

¹⁰⁴ Cubanski J, Neuman T, Orgera K, et al. No Limit: Medicare Part D Enrollees Exposed to High Out-of-Pocket Drug Costs Without a Hard Cap on Spending. Kaiser Family Foundation. November 2017.

¹⁰⁵ MedPAC. Report to the Congress: Medicare and the Health Care Delivery System. Chapter 6: Improving Medicare Part D. June 2016.

¹⁰⁶ Jacobson G, Griffin S, Newman T, et al. Income and Assets of Medicare Beneficiaries, 2016-2035. Kaiser Family Foundation. April 21, 2017.

¹⁰⁷ Cubanski J, Neuman T, Orgera K, et al. No Limit: Medicare Part D Enrollees Exposed to High Out-of-Pocket Drug Costs Without a Hard Cap on Spending. Kaiser Family Foundation. November 2017.

¹⁰⁸ Doshi JA, Pengxiang L, Pettit AR, et al. Reducing Out-of-Pocket Cost Barriers to Specialty Drug Use Under Medicare Part D: Addressing the Problem of “Too Much Too Soon.” *American Journal of Managed Care*. 2017;23(3 Suppl):S39-S45.

spread their annual out-of-pocket spending over the course of the year would help alleviate this substantial upfront financial burden.

3. Changes in Plan Benefit Design Have Shifted More Costs to Patients

The implementation of Part D brought about significant improvements in medication affordability, particularly for seniors with multiple chronic conditions. Early evaluations of the Part D program found significant reductions in rates of cost-related medication nonadherence, including behaviors such as delaying or not filling prescriptions due to cost or skipping doses to make a prescription last longer. However, more recent analysis shows that the prevalence of such behaviors is once again on the rise. According to one study, seniors with four or more chronic conditions reported higher rates of cost-related nonadherence in 2011 than they did in 2007, suggesting an erosion of gains in medication affordability among the sickest Medicare beneficiaries.¹⁰⁹ In part, the authors attribute this deterioration to changes in Part D benefit design, such as increased use of deductibles and higher cost sharing, which have shifted cost burdens onto patients with chronic conditions.

The increased use of complex, multi-tiered formularies and growing prevalence of coinsurance expose patients to a disproportionately high share of the cost of their medicines. Today, the vast majority (95 percent) of PDPs use formularies with five coverage tiers, and 5 percent are now using a sixth tier.¹¹⁰ While most Part D plans have historically applied coinsurance to specialty tier drugs, in recent years plans have increasingly extended coinsurance to drugs on lower tiers. As a result, the percentage of Part D drugs subject to coinsurance jumped by nearly 20 percentage points between 2015 and 2018. ***Today, 62 percent of all drugs covered by PDPs are covered on a coinsurance tier.***¹¹¹

Meanwhile, the share of brand medicines covered on a plan's preferred drug tier continues to decrease. In 2018, less than one-quarter (23 percent) of brand medicines covered by PDPs were placed on the preferred brand tier, while 32 percent and 44 percent were placed on the non-preferred and specialty tiers, respectively. Relative to the fixed-dollar copays commonly applied to medicines on the preferred drug tier, the increased use of coinsurance-based non-preferred and specialty tiers results in higher and less predictable cost sharing for beneficiaries who rely on brand medicines.

In 2017, CMS also began allowing plan sponsors to offer a "blended" non-preferred drug tier, which consists of both brand and generic drugs. Typical coinsurance on the non-preferred drug tier is 40 percent, but can be as high as 50 percent. ***Allowing plans to include a large number of lower-cost generic drugs on the blended tier results in significantly lower average cost sharing across the tier, with the lower cost sharing for generics masking the disproportionate cost sharing that beneficiaries face for brand medicines.*** For example, despite CMS guidance that

¹⁰⁹ Naci H, Soumerai SB, Ross-Degnan R, et al. Medication Affordability Gains Following Medicare Part D Are Eroding Among Elderly with Multiple Chronic Conditions. *Health Affairs*. 2014;33(8):1435-1443.

¹¹⁰ Avalere Health. 2018 Medicare Part D Formularies: An Initial Analysis. November 2017.

¹¹¹ Id.

the average maximum allowable cost sharing for non-preferred medicines cannot exceed a non-discriminatory threshold of \$100,¹¹² across all PDPs with coinsurance on the blended non-preferred tier (representing 98 percent of all enrollment in PDPs), an average of 72 percent of brand medicines placed on this blended tier had cost sharing that resulted in at least \$100 in out-of-pocket costs for beneficiaries in 2018. Similarly, 15 percent of brand medicines placed on these tiers by these same plans resulted in cost sharing of at least \$500 and more than 5 percent resulted in cost sharing of more than \$1,000.¹¹³

4. High Cost Sharing Adversely Impacts Beneficiary Access and Adherence to Medicines

As the Congressional Budget Office (CBO) has affirmed, medication adherence plays an important role in reducing the use of other health care services in Medicare.¹¹⁴ On the other hand, medication nonadherence is associated with poor clinical outcomes and higher overall health care costs.¹¹⁵ Research consistently shows that patients facing high cost sharing are less likely to initiate or adhere to their prescribed medication regimens:

- Analysis by Amundsen Consulting shows that 38 percent of all new specialty prescriptions filled by Part D beneficiaries beginning therapy for the first time were abandoned at the pharmacy in 2016, and that the likelihood of abandonment was strongly associated with out-of-pocket cost.¹¹⁶ When beneficiary cost sharing exceeded \$250, 71 percent of new specialty prescriptions were abandoned. This level of cost sharing was not uncommon, as nearly 40 percent of all new Part D prescriptions for specialty medicines had cost sharing of more than \$250. Even among patients with debilitating or life-threatening illnesses, abandonment rates were alarmingly high. For example, more than 6 out of 10 new oncology prescriptions and more than 7 out of 10 new antipsychotic and multiple sclerosis prescriptions were abandoned at the pharmacy counter when their cost sharing exceeded \$250.
- Researchers at the University of Pennsylvania examined the impact of high cost sharing on initiation of tyrosine kinase inhibitors (TKIs), which have revolutionized the treatment of CML. The analysis found that Part D enrollees who did not receive the LIS and were diagnosed with CML were less likely than enrollees who did receive subsidies (and paid only nominal out-of-pocket costs) to fill a prescription for a TKI within six months of

¹¹² CMS. Announcement of CY 2018 MA Capitation Rates and MA and Part D Payment Policies and Final Call Letter and RFI. April 3, 2017.

¹¹³ Analysis by Avalere Health for PhRMA. June 2018.

¹¹⁴ CBO. Offsetting Effects of Prescription Drug Use on Medicare's Spending for Medical Services. November 2012.

¹¹⁵ Boswell KA, Cook CL, Burch SP, et al. Associating Medication Adherence with Improved Outcomes: A Systematic Literature Review. *American Journal of Managed Care*. 2012;4(4):e97-e108.

¹¹⁶ Amundsen Consulting. Medicare Part D Abandonment: Deep Dive into Branded Product Abandonment. November 2017.

diagnosis (45.3 percent versus 66.9 percent). Additionally, non-LIS beneficiaries took twice as long to fill a prescription for a TKI (an average of 50.9 days versus 23.7).¹¹⁷

- Another academic study found that for Part D enrollees with RA, high cost sharing was associated with treatment interruptions. Among enrollees who used a Part D biologic in the prior year, those facing high cost sharing were less likely to continue using a Part D biologic relative to those beneficiaries receiving cost sharing subsidies. When Part D enrollees with RA did fill a Part D biologic, those facing high cost sharing were twice as likely to experience an interruption in treatment (defined as a gap of more than 30 days) compared to beneficiaries receiving subsidies.¹¹⁸

5. Competitive Incentives Are Key to the Long-Term Sustainability and Affordability of Part D

Medicare Part D, which relies on private market competition to hold down costs for beneficiaries and taxpayers, has been a tremendously successful program. Part D was designed to encourage broad participation of beneficiaries and plan sponsors, and provides beneficiaries with the freedom to choose among dozens of competing private plans, who take on the financial risk of managing Part D costs and compete for enrollment based on premiums, coverage, quality, and service. Having private Part D plan sponsors assume financial risk has been an important part of the program's success. As MedPAC has noted, "When competing plans bear risk, they have an incentive to strike a balance between offering benefits that are attractive to beneficiaries and managing their enrollees' drug spending so that plans' premiums will be affordable."¹¹⁹ To avoid upsetting this balance, potential changes to the benefit should be examined carefully, with an eye towards fully understanding how such changes could impact the competitive incentives built into the Part D program.

Recent changes made to the standard Part D benefit under the Bipartisan Budget Act of 2018 (BBA) weaken the competitive incentives that have made the program successful. Beginning in 2019, the BBA will reduce the amount a plan sponsor pays towards a beneficiary's costs in the coverage gap from 25 percent to 5 percent for brand medicines—an 80 percent reduction. This reduction in liability, combined with plan sponsors' zero liability in the coverage gap for LIS,¹²⁰ sharply reduces the degree to which Part D's private sector plans are at risk for the cost of delivering the benefit, weakening incentives for plans to manage drug spending beyond the initial coverage limit, and threatening to undermine Part D's market-based structure. CMS recently reflected on the BBA's impact in the final Calendar Year (CY) 2019 Call Letter, noting that "we

¹¹⁷ Doshi, JA., Li, P, Huo, H, et al. Medicare Part D Cost-sharing And Specialty Drug Initiation In Newly Diagnosed Chronic Myeloid Leukemia Patients. *Value in Health*. 2016;19(3):78-86.

¹¹⁸ Doshi, JA, Hu, T, Li, P, et al. Specialty Tier-Level Cost-sharing and Biologic Use in the Medicare Part D Initial Coverage Period among Beneficiaries with Rheumatoid Arthritis. *Arthritis Care & Research*. 2016.

¹¹⁹ MedPAC. Report to the Congress: Medicare and the Health Care Delivery System. Chapter 6: Sharing Risk in Medicare Part D. June 2015.

¹²⁰ CMS. Instructions for Completing the Prescription Drug Plan Bid Pricing Tool for Contract Year 2019. April 6, 2018.

have significant concerns about the impact these changes will have on drug costs under Part D in 2019 and future years, particularly as plan liability in the gap significantly decreases for brand name drugs beginning in 2019.”¹²¹

Actuaries and economists have also questioned whether plan sponsors have begun to overemphasize offering the lowest possible premium at the expense of benefit designs that are affordable for high-cost beneficiaries with significant chronic or life-threatening conditions. First, plan sponsors’ practice of applying all rebates as DIR rather than using them to reduce cost sharing at the point of sale suppresses premiums, but as noted above, actuaries have observed that this also creates a system of “reverse insurance,” where chronically ill beneficiaries with high spending subsidize costs for healthier enrollees.¹²² Notwithstanding the savings from lower premiums, this practice results in higher cost sharing that drives up out-of-pocket spending for millions of beneficiaries with chronic and other serious illnesses. In the absence of change, the value of the benefit will erode over time for the sickest beneficiaries, as these patients bear an ever-larger share of the cost of their medicines.

Second, systematic trends in plan sponsors’ bidding practices suggest that plans keep premiums low by shifting risk to the government. Economists have found that relative to actual spending, plan sponsors systematically bid too low on the amount of spending expected in catastrophic coverage, while bidding too high for expected spending in the other phases of the benefit.¹²³ Underbidding on catastrophic spending allows plan sponsors to suppress growth in premiums, while still receiving reimbursement for a large share of their actual incurred catastrophic coverage costs through additional reinsurance payments made during reconciliation. Since retrospective reconciliation payments are not reflected in plan sponsors’ bids, this allows plans with high reinsurance costs to continue offering low premiums. A higher share of Part D payments in 2016 were made through retrospective reconciliation, rather than the prospective risk-based capitation system, suggesting that plan sponsors’ liability for managing the benefit may be shrinking.¹²⁴

MEDICARE PART D: Targeted and Measured Reforms Will Improve Affordability, While Preserving the Success of Part D (RFI p. 22694)

Preserving the success of the Medicare Part D program requires targeted and measured reforms that uphold Part D’s competitive market-based structure and improve affordability without compromising beneficiaries’ access to medicines. Two Part D reforms included in the President’s Fiscal Year (FY) 2019 budget proposal would provide much needed financial relief for beneficiaries facing high cost sharing and high annual out-of-pocket costs:

¹²¹ Announcement of CY 2019 MA Capitation Rates and MA and Part D Payment Policies and Final Call Letter. April 2, 2018.

¹²² Girod CS, Hart SK, Weltz S. 2017 Milliman Medical Index. May 2017.

¹²³ Jung J, Feldman R. Growing Reinsurance Payments Weaken Competitive Bidding in Medicare Part D. *Health Services Research*. Epub ahead of print, May 7, 2018; MedPAC. Report to the Congress: Medicare and the Health Care Delivery System. Chapter 6: Sharing Risk in Medicare Part D. June 2015.

¹²⁴ MedPAC. Report to the Congress: Medicare Payment Policy. Chapter 14: The Medicare Prescription Drug Program (Part D): Status Report. March 2018.

1. Requiring plan sponsors to pass through a substantial share of negotiated rebates at the point of sale would immediately lower out-of-pocket costs for millions of beneficiaries.
2. Establishing a maximum annual limit on beneficiary out-of-pocket spending would provide a true catastrophic benefit to protect the sickest patients.

In addition, there are several program improvements not specifically contemplated in the RFI that the Administration and Congress could pursue to improve patient affordability and support the long-term stability of the program:

1. Reverse changes made under the BBA that threaten to undermine Part D's successful market-based structure by substantially scaling back plan liability and potentially crowding out privately-negotiated rebates with statutorily-mandated price controls.
2. Fix the looming out-of-pocket "cliff," which will cause a sharp spike in the catastrophic threshold in 2020.
3. Allow beneficiaries to more evenly spread their out-of-pocket payments over the course of the year.

These critical benefit design reforms are the optimal long-term approach for improving affordability in Part D, but allowing manufacturers the option of providing cost-sharing assistance also could help reduce some patients' out-of-pocket costs in the near term.

Applying a Share of Negotiated Rebates at the Point of Sale

The Administration could immediately lower out-of-pocket costs for millions of beneficiaries by requiring Part D plans to apply a substantial portion of negotiated rebates to reduce cost sharing at the point of sale. At the time Part D was implemented, CMS expected that plan sponsors would share a large portion of rebate savings directly with beneficiaries in this manner.¹²⁵ Instead, CMS has observed that plan sponsors prefer to report rebates as end-of-year DIR in order to lower plan liability, push down premiums, and increase profits.¹²⁶ Both CMS and MedPAC have raised questions about this practice and CMS has expressed concern that reporting all rebates as DIR provides incentives for plan sponsors to steer utilization towards medicines with high rebates, even when lower cost alternatives are available.¹²⁷

Passing through a substantial portion of rebates at the point of sale is the most important step the Administration can take to ensure that beneficiaries directly benefit from the significant price negotiations taking place in the Part D market. This policy change would immediately and visibly lower out-of-pocket costs for millions of seniors, lower government cost-sharing subsidies and reinsurance payments, and realign stakeholder incentives by reducing plans'

¹²⁵ 70 Fed. Reg. 4194, 4244.

¹²⁶ 82 Fed. Reg. 56419-56420.

¹²⁷ 82 Fed. Reg. 56420.

preference for medicines with high rebates.

Plan sponsors and their PBMs have claimed that sharing a portion of rebates at the point of sale would be too administratively complex and would significantly increase costs to the federal government.¹²⁸ These claims are inaccurate. First, several private insurers and PBMs have announced plans to offer point-of-sale rebate sharing to their commercial clients, signifying that the infrastructure and the capacity to implement this policy already exist.¹²⁹ Second, the cost estimates cited by opponents of this policy change have not accounted for how anticipated behavioral impacts among stakeholders could reshape the market.

Only one study, conducted by actuaries at Milliman, has taken these behavioral changes into account and it concluded that passing through 50 percent of rebates at the point of sale could save the government between \$8B (assuming a modest market response) and \$73B (assuming a strong market response) over the next 10 years.¹³⁰ According to Milliman, savings would be driven by expected changes in formulary strategies that would shift coverage towards medicines with the lowest net costs, as opposed to the highest negotiated rebate. With rebates less “treasured” by plan sponsors and their PBMs, over time their importance to the prescription drug supply chain could change. Importantly, Milliman’s estimates do not account for additional savings likely to accrue to Medicare Parts A and B due to improved medication adherence. A recent study by IHS Markit found that passing through a share of rebates just for diabetes medicines alone could reduce overall health care spending (including Parts A and B) for Medicare beneficiaries with diabetes by \$20B over the next 10 years.¹³¹

PhRMA looks forward to continuing to engage with the Administration on an approach to rebate pass through that preserves the incentives for market-based competition in Part D and protects the confidentiality of commercially-sensitive data.

Establishing a Maximum Annual Out-of-Pocket Spending Limit

Current law requires MA plans to apply a MOOP limit on annual patient cost sharing for services covered under Parts A and B. Extending the MOOP to Part D, and establishing a similar out-of-pocket limit for PDP enrollees, would provide parity with coverage for Medicare Parts A and B services and offer catastrophic protection for patients whose conditions require treatment with medicines, rather than surgical or other medical interventions. It would also harmonize coverage

¹²⁸ See PCMA and AHIP comment letters submitted in response to CMS-4182-P: Medicare Program; Contract Year 2019 Policy and Technical Changes to the Medicare Advantage, Medicare Cost Plan, Medicare Fee-for-Service, the Medicare Prescription Drug Benefit Programs, and the PACE Program (“Proposed Rule”). January 16, 2018.

¹²⁹ Aetna to Offer Point-of-Sale Pharmacy Rebates to Three Million Customers. *Managed Care*. March 27, 2018; Johnson CY. UnitedHealthcare Will Provide Drug Rebates Directly to Members in Some Plans. *Washington Post*. March 6, 2018.

¹³⁰ Milliman. Reducing Part D Beneficiary Costs Through Point-of-Sale Rebates. January 2018. Available at: <http://www.phrma.org/report/reducing-part-d-beneficiary-costs-through-point-of-sale-rebates>

¹³¹ IHS Markit. Passing a Portion of Negotiated Rebates Through to Seniors with Diabetes Can Improve Adherence and Generate Savings in Medicare. May 14, 2018.

standards between Part D and other insurance markets. Today, when beneficiaries age into Medicare from commercial coverage, they often lose the financial protection of an annual cap on out-of-pocket spending. There is no clinical justification or policy rationale for penalizing Part D enrollees who incur high annual drug expenditures with unlimited cost sharing once they turn 65.

CMS already has the legal authority to create a Part D MOOP for beneficiaries enrolled in Medicare Advantage prescription drug plans (MA-PDs). In establishing the Part A/B MOOP for local MA plans, CMS relied on two MA provisions, both of which have Part D counterparts: (1) the prohibition on discriminatory MA benefit designs in Social Security Act (SSA) § 1852(b)(1)(A), which closely resembles the Part D non-discrimination provision in SSA § 1860D-11(d)(2)(D); and (2) the SSA § 1857(e)(1) authority to add “necessary and appropriate” terms to contracts with MA plans, which is incorporated into Part D via § 1860D-12(b)(3)(D).¹³² Therefore, CMS’ legal authority for establishing the MA MOOP is fully applicable to a Part D MOOP.

Additionally, the Part D statute states that CMS may waive Part D provisions to the extent they duplicate or conflict with MA provisions.¹³³ This waiver authority applies here for two reasons:

1. The Part A/B MOOP was established to avoid discouraging individuals with higher than average health care costs from enrolling in MA, so that the plan does not violate the non-discrimination requirement.¹³⁴ Similarly, unlimited Part D cost sharing can also discourage individuals who use above-average levels of services from enrolling in an MA-PD, and thus conflicts with the cap on Part A/B cost sharing.
2. The absence of a Part D MOOP undercuts MA plans’ ability to coordinate Part C and D benefits. Sicker enrollees may cut back on Part D medicines—skipping doses or not filling prescriptions—as their out-of-pocket costs increase without limit on the Part D side, which in turn may cause avoidable complications and increase their use of Part C services such as hospitalizations.¹³⁵ Further, the lack of a Part D counterpart to the A/B MOOP may lead beneficiaries with high health care costs to use Part B drugs even if there are Part D drugs that would be more clinically appropriate. These scenarios illustrate the problems this perverse incentive system can create for coordinating MA plans’ Part C and D benefits.

Accordingly, CMS has ample authority to waive Part D requirements to the extent they would otherwise impede its ability to create a Part D MOOP. CMS should use that authority; from both

¹³² 74 Fed. Reg. 54634, 54657 (Oct. 22, 2009) (proposed MA and Part D rule for 2011, proposing mandatory MOOPs for local MA plans); 75 Fed. Reg. 19678, 19710-11 (April 15, 2010) (final rule for 2011, finalizing MOOP proposal).

¹³³ SSA § 1860D-21(c)(2).

¹³⁴ 74 Fed. Reg. at 54657. Under SSA § 1852(b)(1)(A), CMS may not approve an MA plan if “the design of the plan and its benefits are likely to substantially discourage enrollment by certain MA eligible individuals”).

¹³⁵ CBO. Offsetting Effects of Prescription Drugs Use on Medicare’s Spending for Medical Services. November 2012.

a legal and health care policy perspective, a Part D MOOP would be a sound strategy offering substantial benefits to the MA program and its current and future enrollees.

Distributing Beneficiary Cost Sharing More Evenly Throughout the Year

For beneficiaries with high annual out-of-pocket costs, the structure of the Part D benefit results in an uneven cost distribution, with the highest costs heavily concentrated at the beginning of each calendar year. Therefore, even with a Part D MOOP, beneficiaries will still face significantly high costs in the early months of the year that threaten initiation of treatment and continued adherence to therapy before reaching catastrophic coverage. To make spending more manageable, the Administration could pursue mechanisms that would allow beneficiaries to more evenly spread their annual out-of-pocket payments over the course of the year. In conjunction with a maximum annual out-of-pocket spending limit, this policy change would allow beneficiaries to more accurately predict and budget for their monthly out-of-pocket expenses.

Restoring Competitive Incentives Undone by the Bipartisan Budget Act

The BBA passed by Congress in February 2018 made a significant change to the Part D benefit, reducing plan liability in the coverage gap from 25 percent to just 5 percent for brand medicines. Plan sponsors now have less of a stake in managing Part D expenses above the initial coverage limit, which reduces their incentives to provide benefit designs that are affordable for high-cost beneficiaries with significant chronic or life-threatening illnesses. By limiting the role of competing private plan sponsors and privately-negotiated rebates, BBA threatens a successful market-based program and puts Part D's future in jeopardy. ***The Administration should immediately work with Congress on legislation to mitigate these harmful changes and restore the competitive incentives that have been vital to Part D's success.***

Fixing the Out-of-Pocket Cliff

Another important step that the Administration could take to improve Part D affordability and predictability would be to work with Congress to address the looming out-of-pocket cliff. Changes made under the ACA temporarily slowed the annual rate of increase in the catastrophic threshold, which is the level of TrOOP spending beneficiaries must reach to exit the coverage gap and enter catastrophic coverage. At the end of 2019, the temporary suppression of the growth rate is set to expire, which will cause the catastrophic threshold to jump up suddenly in 2020, as if the growth rate had never been slowed in the first place. This steep increase (roughly \$1,250, based on current projections)¹³⁶ in the catastrophic threshold is known as the out-of-pocket cliff.

With the help of Congress, the Administration should act this year to phase in the impact of the out-of-pocket cliff and prevent the significant increase in out-of-pocket costs beneficiaries will otherwise face in 2020. Given the annual contracting cycle for Part D, as a practical matter, this

¹³⁶ 2018 Annual Report of the Board of Trustees of the Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Fund. June 5, 2018.

benefit cut to seniors needs to be addressed before the end of the first quarter of 2019 so plan sponsors can reflect the change to current law in their 2020 plan-year bids.

Allowing Manufacturers to Provide Cost-Sharing Assistance in Part D

The RFI solicits feedback on whether there are circumstances under which allowing beneficiaries of Federal health care programs to utilize copay discount cards would advance public health benefits.¹³⁷ Currently, government guidance limits the use of manufacturer cost-sharing assistance cards—referred to in the RFI as copay discount cards and also known as copay discount cards or copay coupons—in Federal health care programs.¹³⁸ This guidance—issued by HHS Office of Inspector General (OIG)—suggests that manufacturers can be held liable under the Federal Anti-Kickback Statute if they offer such programs to Part D beneficiaries.¹³⁹ While PhRMA believes that the reforms described above are the optimal long-term approach for addressing affordability challenges in Part D, allowing manufacturers to voluntarily offer cost-sharing assistance cards could provide another alternative for reducing some seniors’ out-of-pocket costs in the near term.

The immediate financial relief provided by cost-sharing assistance programs could advance public health goals by improving appropriate medication use among Part D enrollees. A substantial body of research demonstrates that lowering out-of-pocket costs for medications plays an important role in improving adherence, promoting better health outcomes, and reducing spending on non-prescription drug services, especially for patients with chronic conditions.¹⁴⁰ As the CBO has affirmed, better use of medicines plays an important role in reducing the use of other health care services in Medicare. CBO credits every 1 percent increase in the utilization of prescription medicines with a 0.20 percent decrease in Medicare Parts A and B spending.¹⁴¹ Subsequent research suggests that for Medicare beneficiaries with chronic conditions such as diabetes, hypertension, high cholesterol, and congestive heart failure, this offsetting effect may be three to six times as large.¹⁴²

In the commercial market, cost-sharing assistance programs already provide an important source of financial support for patients and have been shown to improve medication use. Multiple studies report that use of cost-sharing assistance is associated with higher adherence and lower

¹³⁷ RFI p. 22698.

¹³⁸ 70 Fed. Reg. 70623, 70625 (Nov. 22, 2005).

¹³⁹ 70 Fed. Reg. 70623, 70625 (Nov. 22, 2005) (“...the core question is whether the anti-kickback statute would be implicated if a manufacturer of a drug covered under Part D were to subsidize cost-sharing amounts (directly or indirectly through a PAP) incurred by Part D beneficiaries for the manufacturer’s product. . . . Simply put, these subsidies would be squarely prohibited by the statute...”).

¹⁴⁰ Eaddy MT, Cook CL, O’Day K, et al. How Patient Cost-Sharing Trends Affect Adherence and Outcomes: A Literature Review. *Pharmacy & Therapeutics*. 2012;37(1):45-55.

¹⁴¹ CBO. Offsetting Effects of Prescription Drug Use on Medicare’s Spending for Medical Services. November 2012.

¹⁴² Roebuck MC. Medical Cost Offsets from Prescription Drug Utilization Among Medicare Beneficiaries. *Journal of Managed Care & Specialty Pharmacy*. 2014;20(10):994-995.

rates of therapy discontinuation.¹⁴³ For patients facing high risk of prescription abandonment due to high cost sharing, another study found that cost-sharing assistance programs typically reduced patients' monthly out-of-pocket costs to a level where they were much less likely to abandon therapy.¹⁴⁴

MEDICARE PART D: Part D Reforms Must Not Compromise Patients' Access to Medicines (RFI p. 22694)

PhRMA shares the Administration's goal of modernizing the Part D program to provide beneficiaries with more affordable and predictable out-of-pocket costs. However, we strongly dispute the Administration's assertion that the 5-part plan outlined in the President's FY2019 budget proposal must be implemented as a whole. *Certain elements of the 5-part plan—as well as other ideas raised for consideration in the RFI—directly contradict the Administration's stated goals of lowering out-of-pocket costs and putting patients first and would have the perverse consequence of harming the sickest and most vulnerable beneficiaries who rely on their Part D coverage.* Rather than improving the affordability and accessibility of prescription medicines, certain Administration policy proposals would increase costs for patients already facing high out-of-pocket burdens and create new access barriers for vulnerable beneficiaries. These include:

1. Excluding coverage gap discounts from the calculation of TrOOP spending.
2. Reducing the minimum number of required drugs per class from two to one.
3. Increasing coverage restrictions in the protected classes

1. Exempting Coverage Gap Discounts from TrOOP Spending Would Make Medicines Less Affordable for Chronically Ill Beneficiaries

Excluding manufacturer coverage gap discounts from the calculation of TrOOP spending would exacerbate, rather than address beneficiary affordability challenges, and undermines the Administration's goal of reducing out-of-pocket costs for Medicare beneficiaries. By prolonging the amount of time spent in the coverage gap, this change would directly harm millions of chronically ill patients, increasing out-of-pocket spending by hundreds of dollars for those who most rely on medicines to manage their health. Higher out-of-pocket costs for this population would have the unintended consequence of increasing prescription abandonment, medication nonadherence, and premature discontinuation of therapy, leading to poor health outcomes and higher costs elsewhere in the Medicare program.

¹⁴³ Daugherty J, Maciejewski KL, Farley JF. The Impact of Manufacturer Coupon Use in the Statin Market. *Journal of Managed Care Pharmacy*. 2013;19(9):765-772; Daubresse M, Andersen M, Riggs KR, et al. Effect of Prescription Drug Coupons on Statin Utilization and Expenditures: A Retrospective Cohort Study. *Pharmacotherapy*. 2017;37(1):12-24.

¹⁴⁴ Stamer CI, Alexander GC, Bowen K, et al. Specialty Drug Coupons Lower OOP Costs and May Improve Adherence at the Risk of Increasing Premiums. *Health Affairs*. 2014;33(10):1761-1769.

Under current law, 2.3 million non-LIS beneficiaries are estimated to reach catastrophic coverage in 2019.¹⁴⁵ *If the calculation of TrOOP were changed to exclude manufacturer coverage gap discounts, 69 percent (1.6 million) of these beneficiaries would remain in the coverage gap longer and their average annual out-of-pocket costs would increase by 27 percent (from \$2,635 to \$3,364).*¹⁴⁶ Patients with chronic illnesses—particularly those with congestive heart failure, diabetes, hypertension, high cholesterol, and kidney and liver failure—would be the most affected by the TrOOP change, while the relatively healthy would be unaffected. This proposed change to TrOOP would exacerbate the trend towards less meaningful coverage for sicker beneficiaries, which may threaten the future of Medicare Part D as a successful, market-based coverage model. And for many patients, this policy change would result in annual out-of-pocket costs that exceed 10 percent of the median per capita income of Medicare beneficiaries, which was \$26,200 in 2016.¹⁴⁷

Similarly, researchers at the University of Pennsylvania simulated the impact of the TrOOP change for chronically ill beneficiaries with RA, multiple sclerosis, and CML and concluded that this policy change would subject Part D enrollees to higher, more concentrated out-of-pocket costs during the early months of the year. They also observed that chronically ill beneficiaries who remained in the coverage gap for an extended period because of the TrOOP change would be substantially worse off as a result. For example, annual out-of-pocket spending for beneficiaries with RA would increase by 15 percent, from \$3,949 to \$4,540.¹⁴⁸

As the Commonwealth Fund recently noted, “any proposals to change Medicare must proceed with caution. Already-high financial burdens mean any changes to the program must be assessed to safeguard beneficiaries’ access and affordability.”¹⁴⁹ The Administration’s proposal to eliminate coverage gap discounts from TrOOP clearly fails to meet this standard.

2. Eliminating the Two Drug Per Class Requirement Limits Access to Medicines and Could Interrupt or Delay Treatment

PhRMA is greatly concerned about eliminating the Medicare Part D coverage requirement that formularies include at least two drugs per therapeutic class or category. The dilution of this core beneficiary protection in Part D is inappropriate for a host of reasons. It could greatly limit and impede access for patients with complex medical conditions, leading to further health complications when their treatment regimens are compromised. Further, it could result in

¹⁴⁵ Xcenda. Analysis for PhRMA of the 2015 Medicare Part D Event Research Identifiable Files, 10% Sample. Modeling of patient completed by Xcenda based on standard benefit parameters for 2019. Part D and Medicare Advantage Part D Non-LIS enrollment estimates from the Congressional Budget Office April 2018 Medicare baseline.

¹⁴⁶ Id.

¹⁴⁷ Jacobson G, Griffin S, Newman T, et al. Income and Assets of Medicare Beneficiaries, 2016-2035. Kaiser Family Foundation. April 21, 2017.

¹⁴⁸ Doshi JA, Pengxiang L, Pettit AR, et al. Reducing Out-of-Pocket Cost Barriers to Specialty Drug Use Under Medicare Part D: Addressing the Problem of “Too Much Too Soon.” *American Journal of Managed Care*. 2017;23(3 Suppl):S39-S45.

¹⁴⁹ Schoen C, Davis K, Willink A. Medicare Beneficiaries’ High Out-of-Pocket Costs: Cost Burdens by Income and Health Status. The Commonwealth Fund. May 2017.

additional paperwork and red tape for physicians who treat Medicare patients due to an increase in appeals requests to access appropriate off-formulary medicines.

Having a broad range of treatment options is fundamental to providing good care to all patients, but particularly so for the Medicare population, who are more likely to be affected by multiple chronic conditions. It is critical that patients who are stabilized and well-managed on a therapy—or a combination of therapies—maintain access to the appropriate medicines to prevent further complications, poorer disease outcomes, and greater utilization of other health care services such as emergency department visits and hospitalizations. Commercial insurers clearly recognize the importance of ensuring patients have access to a range of therapies: for a wide variety of medicines commonly used by both commercial and Part D enrollees—including those to treat diabetes, asthma, mental illness, HIV, autoimmune disorders, and multiple sclerosis—100 percent of commercial plans provide coverage for two or more medicines per class.¹⁵⁰

Each person is unique with genetic and molecular variations that may affect how they respond to or tolerate any given medication. Even within the same class, patients often respond to drugs differently or certain drugs may not be compatible with other prescribed therapies, necessitating a broader range of treatment options than just one per class. A review of 29 studies evaluating the impact of non-medical switching found that among patients with stable, well-controlled disease, the practice of switching to a different, chemically distinct medicine for reasons other than lack of clinical efficacy/response led to poor side effects or nonadherence and was associated with mostly negative outcomes.¹⁵¹ Of course, these negative outcomes can translate into higher medical costs immediately or in the future, making the practice of non-medical switching seem penny wise and pound foolish.

For autoimmune conditions, such as RA, multiple sclerosis, or inflammatory bowel disease, there is no one-size-fits all approach to treatment. Recognizing that treating physicians and their patients are in the best position to determine appropriate therapies, the physician must always have the authority to decide which product is dispensed to the patient. In addition to patient benefits, there are greater economic savings that result when patients find the right therapy and remain adherent. As an example, patients with RA who responded to tumor necrosis factor inhibitors had lower all-cause medical, pharmacy, and total costs up to three years from initiation of therapy.¹⁵²

¹⁵⁰ Avalere Health. Analysis for PhRMA of the MMIT database of 2018 commercial formularies. June 2018.

¹⁵¹ Nguyen E, Weeda E, Sobieraj D, et al. Impact of Non-Medical Switching on Clinical and Economic Outcomes, Resource Utilization and Medication-Taking Behavior: A Systematic Literature Review. *Current Medical Research and Opinion*. 2016;32(7):1281-1290.

¹⁵² Grabner, M, Boytsov NN, Huang Q, et al. Costs Associated with Failure to Respond to Treatment among Patients with Rheumatoid Arthritis Initiating TNFi Therapy: A Retrospective Claims Analysis. *Arthritis Research & Therapy*. 2017;19(1):92.

Similarly, there is limited clinical evidence to indicate which antipsychotic will be most efficacious for an individual patient.¹⁵³ Instead, treatment response is heterogeneous and patients may experience clinically meaningful differences when exposed to different therapies. Because of varied pharmacokinetics and differences in treatment response, clinical guidelines suggest that medication regimens should be determined on an individual basis and that a trial-and-error process involving multiple antipsychotics will often be needed to find the optimal regimen.¹⁵⁴ For patients diagnosed with schizophrenia, formulary restrictions and non-medical switching may lead to treatment interruptions and nonadherence, which contribute to worsened prognosis, increased hospitalization, increased rates of relapse, attempted suicide, and impaired occupational and social functioning.¹⁵⁵

In the era of curative direct-acting-antivirals (DAAs), researchers are constantly learning more about optimal strategies for treating the various forms of the hepatitis C virus (HCV)—particularly in difficult-to-treat subpopulations. No one treatment regimen is appropriate to treat the broad spectrum of patients living with HCV. There are seven known genotypes of the virus, as well as various subtypes, each associated with different treatment guidelines and recommended DAAs.¹⁵⁶ While some DAAs are pan-genotypic, others are specifically indicated for just one or two genotypes, for certain subtypes of HCV, or for drug resistant forms of HCV. For patients who have failed a previous HCV treatment and those living with comorbid conditions such as HIV; chronic liver disease; liver cancer; or renal impairment, limiting access to one DAA in the class is contrary to clinical guidelines, which recommend the use of different DAAs for different subpopulations. Taken together, the diversity of HCV highlights the importance of patient access to multiple treatment options.

Although there may be many medicines within an individual therapeutic class, the particular therapy that is best suited for a patient is often determined by a specific biological marker or genetic mutation. With the advent of personalized medicines and targeted therapies—where the

¹⁵³ Chien WT, Yip ALK. Current Approaches to Treatments for Schizophrenia Spectrum Disorders, Part I: An Overview and Medical Treatments. *Neuropsychiatric Disease and Treatment*. 2013;9:1311-1332.

¹⁵⁴ Lehman AF, Lieberman JA, Dixon LB, et al. Practice Guideline for the Treatment of Patients with Schizophrenia, Second Edition. *American Journal of Psychiatry*. 2004;161(2 Suppl):1-56.

¹⁵⁵ Keith SJ, Kane JM. Partial Compliance and Patient Consequences in Schizophrenia: Our Patients Can Do Better. *Journal of Clinical Psychiatry*. 2003;64:1308-1315; Velligan DI, Weiden PJ, Sajatovic M, et al. The Expert Consensus Guideline Series: Adherence Problems in Patients with Serious and Persistent Mental Illness. *Journal of Clinical Psychiatry*. 2009;70(suppl 4):1-46; Svarstad BL, Shireman TI, Sweeney JK. Using Drug Claims Data to Assess the Relationship of Medication Adherence with Hospitalization and Costs. *Psychiatric Services*. 2001;52(6):805; Morken G, Widen JH, Grawe RW, Nonn JH, Grawe JK. Using Drug Claims Data to Assess Rehospitalisation in Recent-Onset Schizophrenia. *BMC Psychiatry*. 2008;8:32; Novick D, Haro JM, Suarez D, et al. Predictors and Clinical Consequences of Non-Adherence with Antipsychotic Medication in the Outpatient Treatment of Schizophrenia. *Psychiatry Research*. 2010;176(2-3):109-113; Haynes VS, Zhu B, Stauffer VL, et al. Long-Term Healthcare Costs and Functional Outcomes Associated with Lack of Remission in Schizophrenia: A PostHoc Analysis of a Prospective Observational Study. *BMC Psychiatry*. 2012;12:222.

¹⁵⁶ CDC. Hepatitis C Questions and Answers for Health Professionals: Management and Treatment. Available at: <https://www.cdc.gov/hepatitis/hcv/hcvfaq.htm>; American Association for the Study of Liver Diseases and the Infectious Disease Society of America. HCV Guidance: Recommendations for Testing, Managing, and Treating Hepatitis C. May 24, 2018. Available at: <https://www.hcvguidelines.org/>

underlying molecular drivers of disease help identify and direct precise, targeted treatment choices—limiting the number of covered medicines in a therapeutic class reduces the vast potential of breakthrough science to revolutionize care.¹⁵⁷ This is particularly true for many forms of cancer, where the underlying genetic mutations driving cancer cell growth can be targeted by specific personalized medicines. In the treatment of chronic myelogenous leukemia, for example, identification of a specific mutation led to the development of a class of medicines called TKIs that has nearly tripled the 5-year survival rate from 31 to 89 percent.¹⁵⁸ However, cancer cells often develop resistance to specific medicines over time, making it very important for patients to have additional targeted options available as cancer cells mutate and stop responding to treatment.¹⁵⁹

Furthermore, limiting the scope of coverage within formularies could disproportionately affect and acutely impact patients with rare diseases. Using the U.S. Pharmacopeia Medicare Model Guidelines (UPS MMG) as an example, many agents for rare diseases are not classified at a class level that is granular enough that would be sufficient to ensure patients have access to the most appropriate therapies for their particular condition. Many of these are specific therapies that do not have FDA-approved therapeutic alternatives and are not interchangeable. Most are placed in catch-all “other” classes, and if only one product is required to be covered across a broad, heterogenous class, that could jeopardize the health of a rare disease patient who cannot access treatment in a timely manner if subject to additional utilization management because the product they need is not covered.

Some medicines are approved by FDA specifically for treatment of a condition after another medicine in the class has been tried and failed. If only one medicine per class were required to be covered, patients who are not responsive to the sole medicine covered could experience further treatment delays when subject to utilization management requirements or lengthy appeals processes. Additionally, there are agents that have a narrow therapeutic index, and patients who are stabilized on a medication should not be abruptly switched. What may appear to be minor changes in dose or formulation for medicines within the same class can have a sizable impact on clinical response and may lead to serious therapeutic failures or adverse drug events that could be life-threatening.

Epilepsy is one such condition where not only do patients cycle through several different medicines to find the one that effectively manages their condition, but the anti-epileptic medications to control seizures must also be carefully dosed and monitored. Patients with

¹⁵⁷ Personalized Medicine Coalition. The Personalized Medicine Report: 2017 Opportunities, Challenges, and the Future. November 2017.

¹⁵⁸ American Cancer Society. Thirty-One Percent 5-Year Survival Rate Reported for Cases Diagnosed During 1991-1992. Cancer Facts & Figures. Available at: <http://www.cancer.org/research/cancerfactsfigures/cancerfactsfigures/cancer-facts-figures-2012>; Druker BJ, Guilhot F, O'Brien SG, et al. Five-Year Follow-Up of Patients Receiving Imatinib for Chronic Myeloid Leukemia. *New England Journal of Medicine*. 2006;355(23):2408-2417.

¹⁵⁹ American Cancer Society. Treating Chronic Myeloid Leukemia by Phase. December 2017. Available at: <http://www.cancer.org/cancer/leukemia-chronicmyeloidcml/detailedguide/leukemia-chronic-myeloid-myelogenous-treating-treating-by-phase>

epilepsy often must cycle through four or more antiepileptic drugs (AEDs) both as monotherapy and in combination, and even still, more than half of all patients with newly-diagnosed partial onset seizures fail to achieve seizure control with current first-line monotherapy AEDs.¹⁶⁰ Although there are many therapeutic options for seizures available today, the National Institutes of Health notes that the choice of medication will vary considerably based on several factors including the type of seizures experienced, the lifestyle and age of the patient, frequency of the seizures, interactions with other medicines taken for comorbid conditions, and drug side effects.¹⁶¹

Any efforts to lower the minimum number of medicines that need to be covered in each class would be inconsistent with the medical needs of patients and current clinical recommendations. Although medicines may have the same basic mechanism of action, small differences at the molecular level and the site of action mean that medicines within the same class can have variances that may impact how a medicine works and the patient responds. The need to retain coverage flexibility is particularly pronounced for biologic products, which are large protein molecules that differ in functional and structural binding locations, and therefore biologic response. It is imperative to maintain the coverage requirements to ensure beneficiaries have access to a broader range of medicines within a class to best meet their health needs.

3. Part D Plan Sponsors Use the Same Utilization Management Tools as Commercial Insurers

The RFI raises the question of whether Part D plans have a sufficient level of flexibility to manage high-cost medications, including those in the protected classes, and suggests that private payers in the commercial market have more robust utilization management tools.¹⁶² The fact is that commercial and Part D plans use the same utilization management tools to manage access to high-cost medications to ensure appropriate utilization, medical necessity, and potential adverse reactions. In comparison to private payers, Part D plans require utilization management as often or more frequently for many classes of medications.

In creating the Part D program, the Medicare Modernization Act authorized the use of utilization management by Part D plans, saying that PDP sponsors may have “a cost-effective drug utilization management program, including incentives to reduce costs when medically appropriate.”¹⁶³ This authority is further communicated in guidance to Part D plans in chapter six of the Medicare Prescription Drug Benefit Manual, which details the use of utilization management tools for which Part D plans must receive CMS approval, the use of these tools when CMS approval is not required, and guidelines for the application of prior authorization

¹⁶⁰ Brodie MJ, Barry SJ, Bamagous GA, et al. Patterns of Treatment Response in Newly Diagnosed Epilepsy. *Neurology*. 2012;78(2):1548-1554.

¹⁶¹ NIH National Institute of Neurological Disorders and Stroke. The Epilepsies and Seizures: Hope Through Research. Available at: https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Hope-Through-Research/Epilepsies-and-Seizures-Hope-Through#3109_30.

¹⁶² RFI p. 22695.

¹⁶³ SSA § 1860D-4 (c)(1)(A).

specifically.¹⁶⁴ The Part D manual also specifies that use of utilization management should be consistent with best practices, appropriate guidelines, and current industry standards—presumably in relation to the broader commercial market.

In fact, the data show that Part D plan sponsors do consistently manage utilization, both in terms of tier placement and use of utilization management tools. Over the past four years, plan sponsors have placed an increasing proportion of brand medicines on higher tiers. In 2018, PDPs placed an average of 44 percent of brand medicines on the specialty tier compared to 35 percent in 2015, while the proportion of brand medicines placed on the preferred tier dropped from 27 percent to 23 percent over that same time horizon.¹⁶⁵ Part D plan sponsors also apply utilization management tools at a consistent rate as well. For example, across three classes of medicines identified as “high cost” by payers—immune suppressants, immunomodulators, and multiple sclerosis treatments—all PDPs use step therapy or prior authorization for at least one drug in 2018.¹⁶⁶

When compared to employer plans, Part D plans commonly employ utilization management at consistent or higher rates than the commercial market, including for “high cost” and protected class medicines. For example, PDPs use prior authorization or step therapy on 44 percent of oncology medications on average, while employer plans include these requirements on an average of 32 percent of these medications.¹⁶⁷ Across all oncology subclasses—including alkylating agents, antiandrogens, antiangiogenic agents, antimetabolites, enzyme inhibitors, and molecular target inhibitors—PDPs are more likely to apply utilization management to oncology medicines than employer-sponsored plans. Despite protected class status, use of prior authorization and step therapy for atypical antipsychotics is consistent for both markets—on average, employer plans apply these tools to 14 percent of medications in this class, compared to 13 percent for PDPs.¹⁶⁸

MEDICARE PART D: Changes to the Six Protected Classes Would Harm Vulnerable Beneficiaries and Are Not Warranted on Clinical, Fiscal, or Legal Grounds (RFI p. 22695)

PhRMA opposes any changes to the six protected classes policy that would reduce beneficiary access to critical medications. We dispute the claim that Part D plan sponsors do not have sufficient tools to manage utilization in the protected classes and question the legality and appropriateness of policy changes that would allow cost considerations to outweigh clinical need—particularly in the case of vulnerable beneficiaries. Increased restrictions on use of medicines in the protected classes are unlikely to produce substantial financial savings in Part D. Instead, such restrictions may disrupt therapy and hinder beneficiary access to medicines, leading to worse clinical outcomes, increased need for costly emergency and hospital care, and higher

¹⁶⁴ Medicare Prescription Drug Benefit Manual, Chap. 6, § 30.2.2.

¹⁶⁵ Avalere Health. 2018 Medicare Part D Formularies: An Initial Analysis. November 2017.

¹⁶⁶ Avalere Health. Analysis for PhRMA of PlanScape database of 2018 Part D formularies. June 2018.

¹⁶⁷ Avalere Health. Analysis for PhRMA of MMIT and PlanScape databases of 2018 commercial and Part D formularies. June 2018.

¹⁶⁸ Id.

overall Medicare costs. In short, restricting access to protected class medicines is flawed from a clinical, fiscal, and legal standpoint and would harm vulnerable beneficiaries.

1. Robust Coverage Protections for the Protected Classes Are Needed to Ensure Vulnerable Beneficiaries Have Access to a Full Range of Necessary Medicines

Access to clinically critical medicines for vulnerable patients has been a cornerstone of the Part D program. When Congress established Medicare Part D, it recognized that robust access to certain medicines is central to the wellbeing of beneficiaries who need those therapies, and that their prescribers need access to the full range of treatment options. For example, a Senate exchange that took place just before enactment of the legislation that created Part D emphasized the many layers of patient protections Congress had purposely built into the program to ensure broad coverage of medications for patients—such as those facing HIV/AIDS, epilepsy, or mental illness—“who need exactly the right medicine for them.”¹⁶⁹

One of the key safeguards referenced in this exchange and others—the Part D non-discrimination provision—remains the law today and prohibits CMS from approving a plan if “the design of the plan and its benefits (including any formulary and tiered formulary structure) are likely to substantially discourage enrollment by certain Part D eligible individuals.”¹⁷⁰ As CMS has explained, it “instituted this policy because it was necessary to ensure that Medicare beneficiaries reliant upon these drugs would not be substantially discouraged from enrolling in Part D plans and to mitigate the risks and complications associated with an interruption of therapy for these vulnerable populations.”¹⁷¹

For beneficiaries relying on medicines in the protected classes, many treatments are not interchangeable and there is often little clinical evidence to indicate which medication will be most efficacious for any particular patient. Instead, treatment response is heterogeneous and seemingly “similar” patients may experience clinically meaningful differences when exposed to different therapies. Medicines in the same class often have different side effects as well, or may be counter-indicated when combined with a patient’s other therapies.

The clinical considerations that led to the original six protected classes policy are as relevant and as pressing today as when the protected classes were established. Maintaining the existing protected classes policy remains clinically necessary for minimizing adverse outcomes that may otherwise result from therapy interruptions or delays. When patients are unable to receive the medication best suited to their individual needs, worsening of symptoms, avoidable hospitalization, poor prognosis or impaired quality of life all are likely. Delaying optimal treatment for even a short time while trying ineffective treatments may cause irreversible damage. There is no clinical basis for preventing a patient from accessing the particular treatment option that would be most effective—or least harmful—on a timely basis.

¹⁶⁹ 149 Cong. Rec. S5882-03 (Nov. 25, 2003).

¹⁷⁰ SSA § 1860D-11(e)(2)(D).

¹⁷¹ Prescription Drug Benefit Manual, Ch. 6 § 30.2.5.

2. Part D Plan Sponsors Already Have Effective Tools to Manage Utilization in the Protected Classes

Part D plan sponsors have always been able to manage access to most drugs in the six protected classes. Plan sponsors routinely apply restrictions like prior authorization and step therapy to promote selection of certain products over others and are permitted to exclude multi-source brands, extended-release products, and certain medication forms and dosages from their formularies. Plan sponsors can also structure their formularies and beneficiary cost-sharing requirements to influence product selection and negotiate rebates with manufacturers. In classes where generic drugs are available, plan sponsors have been highly effective in driving high rates of generic utilization.

In some instances, coverage of medicines in the protected classes is already *more* restrictive in Part D than in the commercial market. For example, an analysis by Avalere Health compared access to anticonvulsants between commercial health plans and Part D PDPs and found that—despite anticonvulsant’s status as a protected class—PDPs had less generous formularies and lower levels of access.¹⁷² Specifically, PDP formularies covered fewer anticonvulsants on average than commercial plans (62 percent versus 80 percent) and a substantially smaller share of all brand anticonvulsants (42 percent versus 76 percent). Relative to commercial plans, PDPs also subjected a larger share of anticonvulsants to either prior authorization or step therapy (13 percent versus 11 percent).

Other analysis similarly demonstrates that utilization management for protected class medicines is no less prevalent in Part D than in the commercial market. As mentioned earlier, relative to employer-sponsored plans, Part D PDPs apply prior authorization or step therapy to a similar share of atypical antipsychotics (14 percent and 13 percent, respectively) and a larger share of oncology medicines (44 percent versus 32 percent).¹⁷³ Furthermore, wide variation in the use of utilization management across Part D plan sponsors shows that clinical discretion, not regulation, determines how often these tools are applied in the protected classes. For example, among the top 10 largest PDPs, the share of covered atypical antipsychotics subject to prior authorization or step therapy ranges from 5 percent to 65 percent.¹⁷⁴

A solid body of evidence shows that Part D plans already negotiate successfully to impact therapeutic choices and secure competitive pricing for medicines in the protected classes. MedPAC reports that over the 2006 to 2014 period (the latest data available), prices for protected class drugs grew more slowly than for Part D prices overall.¹⁷⁵ Part D plans have also been highly successful in driving generic utilization within the protected classes. According to an

¹⁷² Avalere Health. An Analysis of Access to Anticonvulsants in Medicare Part D and Commercial Health Insurance Plans. June 2013.

¹⁷³ Avalere Health. Analysis for PhRMA of MMIT and PlanScape databases of 2018 commercial and Part D formularies. June 2018.

¹⁷⁴ Avalere Health. Analysis for PhRMA of PlanScape database of 2018 Part D formularies. June 2018.

¹⁷⁵ MedPAC. Report to the Congress: Medicare Payment Policy. March 2017.

analysis of CMS data by the Pew Charitable Trusts, *the generic utilization rate in the protected classes is higher than for non-protected classes* (92 percent versus 84 percent).¹⁷⁶ MedPAC agrees that protected class status does not affect plan sponsors' ability to drive utilization of generics and reports that accounting for generic substitution, cumulative prices for protected class medicines *decreased* by 13 percent between 2006 and 2014, compared to an 8 percent increase for all Part D drugs.¹⁷⁷

Similarly, a 2016 report by the QuintilesIMS Institute, now IQVIA, also found that plan sponsors successfully negotiate significant cost reductions for medicines most commonly used by Part D beneficiaries, including medicines in two of the protected classes.¹⁷⁸ Across the 12 most commonly used therapeutic classes of medicines—including antidepressants and anticonvulsants—Part D plan sponsors negotiated an average rebate of 35.3 percent. Accounting for negotiated rebates, the analysis found that the final net costs to the plan sponsor for antidepressants and anticonvulsants were roughly half of the list price. Given the conclusive evidence that Part D sponsors have been able to, and do, negotiate rebates and drive appropriate generic utilization within the protected classes, it is wholly inappropriate as a matter of public policy to allow a vague but vocal interest in “flexibility” on the part of health plans and PBMs to outweigh the Federal government’s legitimate (and statutory) interest in preventing discrimination against the sickest patients.

3. Restricting Access to Protected Class Medicines is Unlikely to Produce Substantial Savings in Part D and Could Increase Medicare Costs Overall

Cost containment is clearly one of the Administration’s primary motivations in pursuing changes to the protected classes; however, allowing plan sponsors to place additional restrictions on access to medicines in the protected classes is unlikely to produce substantial Part D savings, and could have the unintended consequence of increasing Medicare spending overall.

Two factors limit the potential for the proposed revision of the protected classes policy to yield Part D savings. First, the current high rate of generic utilization sharply limits the ability of plan sponsors to further drive utilization to lower cost therapies. Second, the remaining share of brand utilization in the protected classes is primarily comprised of medications without generic alternatives. In these instances, plan sponsors who seek to use more restrictive utilization management to force non-medical (*e.g.*, cost-based) switching run the risk of disrupting established treatment regimens and worsening clinical outcomes for their most vulnerable beneficiaries. As an example, the Pew Charitable Trust reports that 90 percent of antiretroviral and 22 percent of antineoplastic prescriptions are for brand medicines without generic alternatives, “indicating widespread clinical use that may inhibit PDPs’ ability to exclude these drugs from formularies.” Furthermore, even in cases where generic drugs are available in a class,

¹⁷⁶ The PEW Charitable Trusts. Policy Proposal: Revising Medicare’s Protected Classes Policy. March 2018.

¹⁷⁷ MedPAC. Report to the Congress: Medicare Payment Policy. March 2017.

¹⁷⁸ QuintilesIMS Institute. Estimate of Medicare Part D Costs After Accounting for Manufacturer Rebates: A Study of Original Branded Products in the U.S. October 2016.

generic therapy may be clinically inappropriate due to safety and efficacy concerns. For example, federal clinical guidelines for the use of antiretrovirals recommend newer combination brand therapies over older generic monotherapies, due to the increased risk of virologic failure and drug resistance associated with monotherapy.¹⁷⁹

Restricting access to protected class medications may also have the unintended consequence of increasing overall Medicare costs. In particular, stand-alone PDPs—which are not responsible for their enrollees’ medical care—may lack the financial incentives to consider the downstream consequences of formulary exclusions and utilization management in the six protected classes, including discontinuation of therapy, poor medication adherence, and increased consumption of inpatient and outpatient services.¹⁸⁰ Higher costs and poor clinical outcomes resulting from access restrictions or suboptimal medication use in the six protected classes are supported by a wide body of evidence. For example:

- Multiple studies demonstrate that Medicare beneficiaries with schizophrenia and low adherence to antipsychotics require significantly more inpatient care and incur significantly higher psychiatric hospital expenditures.¹⁸¹
- Compared to adherent patients, individuals 65 and older with epilepsy who are nonadherent to their anticonvulsant medications experience more seizures and more than \$2,600 in higher health care costs from increased inpatient and emergency department use.¹⁸²
- Among individuals with a transplanted organ, nonadherence to immunosuppressants increases the odds of transplantation organ failure seven-fold, leading to increased health care utilization or premature death.¹⁸³
- Prior authorization requirements for antiretrovirals increase administrative costs for providers and can lead to patients experiencing delays in receiving their medications.¹⁸⁴

¹⁷⁹ National Institutes of Health. Guidelines for the Use of Antiretroviral Agents in Adults and Adolescents Living with HIV. March 27, 2018. Available at: <https://aidsinfo.nih.gov/guidelines/html/1/adult-and-adolescent-arv/0>

¹⁸⁰ Lavetti K and Simon K. Strategic Formulary Design in Medicare Part D Plans. Working Paper. June 24, 2016.

¹⁸¹ Offord S, Lin J, Wong B, et al. Impact of Oral Antipsychotic Medication Adherence on Healthcare Resource Utilization Among Schizophrenia Patients with Medicare Coverage. *Community Mental Health Journal*. 2013;49(6):625-629; Roberto P, Brandt N, Onukwugha E, et al. Adherence to Antipsychotic Therapy: Association with Hospitalization and Medicare Spending Among Part D Enrollees with Schizophrenia. *Psychiatric Services*. 2017;68(11):1185-1188.

¹⁸² Ettinger AB, Manjunath R, Candrilli SD, et al. Prevalence and Cost of Nonadherence to Antiepileptic Drugs in Elderly Patients with Epilepsy. *Epilepsy & Behavior*. 2009;14(2):324-329.

¹⁸³ Butler JA, Roderick P, Mullee M, et al. Frequency and Impact of Nonadherence to Immunosuppressants After Renal Transplantation: A Systematic Review. *Transplantation*. 2004;77(5):769-776.

¹⁸⁴ Casalino LP, Nicholson S, Gans DN, et al. What Does it Cost Physician Practices to Interact with Health Insurance plans? *Health Affairs*. 2009;28(4):w533-w543.; Raper JL, Willig JH, Lin H, et al. Uncompensated Medical Provider Costs Associated with Prior Authorization for Prescription Medications in an HIV Clinic. *Oxford Journal of Clinical Infectious Diseases*. 2010;51(6):718-724.

HIV patients who face drug benefit design changes are also nearly six times more likely to face treatment interruptions than those with more stable coverage, increasing their risk of virologic rebound, drug resistance, and increased morbidity and mortality.¹⁸⁵

In sum, allowing plan sponsors to place additional restrictions on access to medicines in the protected classes is penny wise and pound foolish. Changes to the existing six protected classes policy are unlikely to generate substantial savings for Part D and could have the unintended consequence of increasing spending in other parts of Medicare.

4. Conditioning Protected Class Status on Changes in List Price Violates Part D's Non-Discrimination Clause

In the discussion of incentives to lower or not increase list prices, the RFI asks broadly what CMS “[should] consider doing, under current authorities, to create incentives for Part D drug manufacturers committing to a price over a particular lookback period.”¹⁸⁶ Among other things, the RFI asks whether drugs that have been subject to a price increase over a specified lookback period should be allowed to be included in the protected classes, and whether drugs that have not had a price increase over a lookback period should be treated differently for purposes of protected class exceptions criteria.¹⁸⁷

We understand the importance HHS attaches to reforms that could encourage manufacturers not to increase list prices. PhRMA supports several policies, which we discuss in detail in Section II of our comments that could help to promote competition and improve patient affordability. At the same time, it is critically important that CMS not undercut foundations of the Part D program that have protected its most vulnerable beneficiaries and made the program successful. We discuss these foundations below.

First, excluding a drug from the protected classes that otherwise belongs there (or otherwise tying protected class status to whether a drug's list price increases over a specified period) is not an appropriate or legally sound way to advance the administration's goal of lowering prices, as two statutory provisions may prevent this: the Part D law's non-discrimination clause (Social Security Act (SSA) § 1860D-11(e)(2)(D)) and its protected classes clause (SSA § 1860D-4(b)(3)(G)). CMS developed the protected classes doctrine at the beginning of the Part D benefit to carry out the Part D law's non-discrimination clause, which prohibits CMS from approving any Part D plan with a design (including a formulary or formulary structure) that is “likely to substantially discourage enrollment by certain [Medicare beneficiaries].”¹⁸⁸ CMS instituted the protected classes policy “because it was necessary to ensure that Medicare beneficiaries reliant upon these drugs [in the six protected classes] would not be substantially discouraged from enrolling in certain Part D plans, as well as to mitigate the risks and complications associated with an

¹⁸⁵ Das-Douglas M, Riley ED, Ragland K, et al. Implementation of the Medicare Part D Prescription Drug Benefit is Associated with Antiretroviral Therapy Interruptions. *AIDS and Behavior*. 2009;13(1): 1-9.

¹⁸⁶ RFI p. 22698.

¹⁸⁷ 83 Fed. Reg. at 22698.

¹⁸⁸ SSA § 1860D-11(e)(2)(D).

interruption of therapy for these vulnerable populations.”¹⁸⁹ This statement remains true. Excluding a drug otherwise within the six protected classes from protected class status due to a list price increase would therefore violate the non-discrimination clause (even if CMS issued regulations to create an exception to SSA § 1860D-4(b)(3)(G)), by permitting Part D plans with benefit designs that discouraged enrollment by some of Medicare’s most vulnerable beneficiaries. Accordingly, the law does not permit compromising its non-discrimination principles in order to promote lower drug prices, and we believe CMS has other tools that will advance the goal of patient affordability and market competition more effectively.

Moreover, tying a drug’s protected class status to whether its list price has increased finds no support in the text of the relevant statutory provisions, which instruct CMS to develop criteria to identify classes “of clinical concern”¹⁹⁰ and ban discriminatory benefit designs that could discourage enrollment by certain beneficiaries.¹⁹¹ In interpreting and applying these provisions, CMS may not consider factors Congress did not authorize it to consider¹⁹²—such as list price movements—and must respect Congress’ determination that criteria based on “clinical concern[s]” are in the best interest of the Part D program.

Second, any approach to discouraging list price increases that CMS adopts must not violate the noninterference clause of the Part D statute.¹⁹³ As we state elsewhere in this letter, the noninterference clause is a cornerstone of the Part D program and a key reason for the program’s success. Any policies pursued by CMS must not (1) interfere in the private negotiations between manufacturers, plan sponsors, or pharmacies; or (2) create a formulary or price structure. For this and other policy reasons, we urge CMS to instead consider the policies discussed in the section of this comment letter on rebates, which we believe provide effective tools for discouraging list price increases.

SECTION V: MEDICARE PART B (RFI p. 22697)

The Medicare Part B benefit provides crucial access to medicines for vulnerable patients who suffer from a range of serious illnesses. It covers a subset of outpatient prescription medicines that are usually administered by a physician to treat patients with complex, serious, often rare conditions who currently have few or no alternative treatment options. The structure of the Part B benefit provides much needed flexibility for physicians to tailor treatment plans to optimize care for these patients. As HHS considers changes to this program, it will be very important to

¹⁸⁹ Medicare Prescription Drug Benefit Manual, Chap. 6, § 30.2.5.

¹⁹⁰ SSA § 1860D-4(b)(3)(G).

¹⁹¹ SSA § 1860D-11(e)(2)(D).

¹⁹² See, e.g., *Nalco Co. v. EPA*, 786 F. Supp. 2d 177, 187 (D.D.C. 2011) (rejecting EPA’s enforcement action as arbitrary and capricious where it acted based on its stated desire “to level the marketplace for competitors,” but the authorizing statute “does not give EPA jurisdiction to control or modify the marketplace”).

¹⁹³ SSA § 1860D-11(i) (“In order to promote competition under [Part D] and in carrying out this part, the Secretary—(1) may not interfere with the negotiations between drug manufacturers and pharmacies and PDP sponsors; and (2) may not require a particular formulary or institute a price structure for the reimbursement of covered part D drugs”).

preserve its strengths in supporting beneficiary access to a range of treatment options and timely delivery of complex care at the site of service that is best for the patient. Preserving drug coverage under Part B is crucial for beneficiaries with serious illnesses.

As HHS considers changes to Medicare Part B, it should pursue approaches that improve value holistically, across the treatment continuum the patient experiences, and empower patients and consumers to make informed choices rather than restricting their choices and treatment options. The Department also should avoid introducing misaligned incentives that would undermine the existing market-based and transparent Average Sales Price (ASP) system. The President’s Blueprint states “Millions of Americans face soaring drug prices and higher out-of-pocket costs, while manufacturers and middlemen such as PBMs and distributors benefit from rising list prices...”¹⁹⁴ and calls for bold action to bring down prices for patients and taxpayers, such as increasing transparency and fixing incentives that may be increasing prices for patients.¹⁹⁵ In light of HHS’ goals, it is noteworthy that several unique features of Medicare Part B contribute to transparency, stable prices in the program, negotiation, access to care, and predictable cost sharing for beneficiaries:

- **ASP reflects robust negotiation in the commercial market, resulting in savings for beneficiaries and the Medicare program.** Medicare Part B drug reimbursement generally is not based on manufacturer list price or Wholesale Acquisition Cost (WAC). Rather, for most drugs, reimbursement is based on ASP, which reflects the weighted average of all manufacturer sales prices,¹⁹⁶ and includes rebates and discounts that are privately negotiated by health care providers and payers. As a result, it serves as a mechanism for passing discounts negotiated in the commercial market on to Medicare beneficiaries and the Medicare program. Due to this market-based competition, ASP prices are often substantially lower than list prices. Looking at discounts for the 25 medicines with the highest spending under Part B, the ASP represents a weighted average discount of 21.2 percent off the list price.¹⁹⁷
- **ASP moderates price growth.** CMS’ own analysis of the market-based ASP pricing mechanism found that in the third quarter of 2018, the ASP-based Part B payment amount for 11 of the top 50 drugs decreased; and, for most of the higher volume drugs, ASP changed 2 percent or less. CMS notes, “In general, among the top drugs with a decrease, there are a number of competitive market factors at work—multiple manufacturers, alternative therapies, new products, recent generic entrants, or market shifts to lower priced products.”¹⁹⁸ A long range analysis of the ASP system supports this finding. The volume weighted ASP for Part B medicines has remained steady year

¹⁹⁴ RFI p. 22692.

¹⁹⁵ RFI p. 22695.

¹⁹⁶ Medicaid and certain other federal discounts and rebates are excluded from ASP. There are special rules for certain classes of drugs (e.g., DME infusion drugs, vaccines, and biosimilars).

¹⁹⁷ Estimates based on analysis by PhRMA using the July 2018 ASP Pricing File, 2018 Medispan files, and 2018 Medicare Part B Drug Spending Dashboard.

¹⁹⁸ CMS, 2018. Available at: <https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Part-B-Drugs/McrPartBDrugAvgSalesPrice/2018ASPFiles.html>

over year, and price growth for Medicare Part B drugs is below overall medical inflation.¹⁹⁹

- **ASP is transparent.** Manufacturers report sales to CMS on a quarterly basis, and CMS then calculates and posts the ASP for all Part B medicines in a public data file on the CMS website.
- **Part B offers a predictable cost-sharing structure and supplemental coverage offsets out-of-pocket costs for many beneficiaries.** Cost sharing for Part B medicines is set at 20 percent of the Medicare reimbursement rate. A majority of beneficiaries (over 80 percent) carry supplemental coverage that helps to defray their out-of-pocket costs for Part B medicines, an option that is not available for Part D plans.²⁰⁰ Recent analysis from Avalere found that, as a result of supplemental coverage, beneficiaries typically have lower out-of-pocket costs for oncology medicines covered in Part B than in Part D.²⁰¹
- **The Part B reimbursement rate covers costs associated with storing and handling the medicine.** Changes to the Part B reimbursement rate would affect providers' ability to stock and handle Part B drugs. As a result, patients would be forced to receive care in more costly settings.²⁰² Under the current statutory model, the 6 percent add-on rate also accounts for variability in provider practice size, patient population, and location.²⁰³ Critics of the system argue that the add-on creates perverse prescribing incentives; however, there is no compelling evidence to show that doctors make inappropriate prescribing decisions based on reimbursement rates.
- **Part B facilitates access to care for beneficiaries with serious illnesses.** Due to the nature of many medicines in Part B and the diseases that they treat, patients often need to try multiple therapies before finding the appropriate treatment, and physicians and patients need maximum flexibility to tailor treatments to meet patients' needs, consistent with clinical evidence.
- **New and innovative payment models are already being explored in Part B.** CMMI has implemented a number of programs that address Medicare expenditures in Part B more broadly. For example, the Oncology Care Model (OCM) aims to lower Medicare costs by coordinating care more closely for oncology patients and testing a performance-based payment system. OCM is just one of many CMMI models that affect prescribing

¹⁹⁹ The Moran Company. Trends in Weighted Average Sales Prices for Prescription Drugs in Medicare Part B, 2006-2015. December 2016.

²⁰⁰ Analysis of the 2013 Medicare Current Beneficiary Survey conducted by The Moran Company for PhRMA. June 2017.

²⁰¹ Avalere Health. Moving Certain Part B Drugs to Part D, A Proposal Being Evaluated by The Trump Administration, Would Have Disparate Financial Impacts on Patients. May 2018.

²⁰² Letter to HHS Secretary Alex Azar. March 14, 2018. Available at: <https://www.rheumatology.org/Portals/0/Files/Physician-Group-Letter-to-HHS-Drug-Pricing-Proposals.pdf>

²⁰³ American Action Forum. Primer: Medicare Part B Drug Payment System. June 19, 2018. Available at: <https://www.americanactionforum.org/research/primer-medicare-part-b-drug-payment-system/>.

of Part B medicines. In a recent report, Avalere notes that “[a]lthough CMS did not design the programs covered in this brief specifically to address Part B drugs, providers participating in these programs may modify their Part B prescribing, utilization, and treatment patterns in an effort to ensure that expenditures for all included Medicare services fall under the applicable spending benchmark.”²⁰⁴

These dynamics successfully balance patient access with controlling costs as evidenced by the fact that Part B medicines remain a small and stable share of Medicare spending. Spending on Part B medicines was just 3 percent of total Medicare spending in 2015 (11 percent of all Part B spending),²⁰⁵ even as patients gained access to important new treatment advances. HHS should not pursue policy changes to Part B that would reduce access to care or undermine the aspects of the program that have worked well to promote transparent, market-based reimbursement for physician-administered medicines. As discussed below, we are concerned that several of the specific proposals in the RFI could harm patient access to care and undermine delivery of high-quality care in clinically appropriate settings.

MEDICARE PART B: Part B to Part D (RFI p. 22694)

Whether and when a drug is covered under the Part B benefit or the Part D benefit is a distinction that is clearly defined in Medicare law. Generally, Medicare Part B covers medications that require administration by a physician or in a hospital outpatient setting, such as chemotherapy. Many patients who use Part B medicines have serious conditions that require intensive management such as cancer, RA and other autoimmune conditions, severe infections, multiple sclerosis, macular degeneration, genetic disorders, and other rare diseases. Often, these patients are reliant on physician-administered medicines (e.g., intravenous infusions, interocular injections) because they have few or no other treatment options. By contrast, Medicare Part D covers nearly all other types of drugs not otherwise covered by Part B, and most Part D drugs are self-administered products (e.g., oral pills or liquids, simple subcutaneous injections) that are obtained by the patient through a pharmacy.

Moving Part B drugs exclusively to the Part D benefit could increase out-of-pocket costs for many patients and reduce access to care. It also poses operational and administrative challenges for providers, as well as safety issues for patients, because most Part B medicines have complex storage, handling, and preparation requirements that require specific clinical expertise. For the reasons described below, PhRMA does not support moving medicines from Part B to Part D.

²⁰⁴ Avalere Health. Medicare Part B Drug Payments Implicated in CMMI Models. August 2016. Available at: <http://avalere.com/expertise/life-sciences/insights/medicare-part-b-drug-payments-implicated-in-cmmi-models>.

²⁰⁵ Analysis of 2017 Medicare Trustees Report and June 2017 MedPAC Databook conducted by Price Waterhouse Cooper for PhRMA.

Increased Costs for Patients

All else equal, moving Part B drugs to the Part D benefit could increase costs for most patients. If products are shifted from Part B to Part D, beneficiaries would experience an increase in their monthly Part D premiums as Part D coverage broadened to cover more medicines. Beneficiaries would experience these increases whether or not they are prescribed a Part B medicine as the cost of additional benefits would be spread across all policies. We estimate that if all Part B medicines were moved to Part D, Part D premiums could increase by nearly 40 percent.²⁰⁶

Shifting coverage of Part B medicines to Part D could also introduce short-term volatility into the Part D market. The overall cost of the Part D benefit would likely increase as plans begin to cover a broader range of medications, and premium changes would likely be less stable year over year as plans adjust to incorporating additional medicines into their bids. In addition, more patients could reach the catastrophic phase of the Part D benefit and this will increase federal spending on reinsurance in the Part D program.²⁰⁷

For some beneficiaries, out-of-pocket costs at the point-of-sale would also increase. A government-commissioned study previously examined moving a subset of Part B drugs to Part D and concluded that “as drugs move from Part B to Part D...costs for beneficiaries rise. The increase in beneficiary out-of-pocket costs is an important concern in examining the effects of the proposed consolidation, as it could impede beneficiary access to needed medication.”²⁰⁸ On net, previous analyses suggest that Medicare would likely see only a small decrease in total spending as a result of this policy, and that this decrease comes at the expense of beneficiaries, shifting significant costs to them via excessive cost sharing.²⁰⁹ More recently, an analysis from Avalere Health found that average out-of-pocket costs were about 33 percent higher for Part D-covered new cancer therapies than for those covered in Part B in 2016.²¹⁰ Beneficiaries who carry supplemental coverage (a majority of Part B patients) are particularly likely to see higher out-of-pocket costs if their medicines are shifted into Part D.

Reduced Access to Care

Moving Part B medicines to Part D could also reduce patient access to treatment for many life-threatening and debilitating conditions. A subset of Medicare beneficiaries are not currently enrolled in Part D prescription drug coverage. Analysis of similar proposals in the past found that thousands of patients would be without coverage of physician-administered medicines as a result of the shift.²¹¹ Approximately 12 percent of beneficiaries either do not have drug coverage or have coverage that is less generous than Part D, and could be at risk for losing coverage for their

²⁰⁶ Holcomb, Katie et al. Impact of Moving Medications from Medicare Part B to Part D. June 2018

²⁰⁷ Id.

²⁰⁸ Acumen, LLC. Estimating the Effects of Consolidating Drugs under Part D or Part B. August 2011.

²⁰⁹ Id.

²¹⁰ Avalere Health. Avalere Analysis Highlights Complexities of Transitioning Medicare Part B Drugs into Part D. May 21, 2018. Available at: <http://avalere.com/expertise/life-sciences/insights/avalere-analysis-highlights-complexities-of-transitioning-medicare-part-b-d>

²¹¹ Acumen, LLC. Estimating the Effects of Consolidating Drugs under Part D or Part B. August 2011.

Part B medicines if they were shifted to the Part D benefit.²¹² If these patients decided to enroll in Part D as a result of the shift, they may be subject to late enrollment penalties that further increase their premium costs.

If Part B medicines shift to Part D, patients who rely on Part B medicines may experience new barriers to accessing the medication that they and their doctor have identified as the best treatment for their disease or condition. Unlike Part B, which covers all medically necessary services and treatments, Part D plans are generally not required to cover all the medicines within a therapeutic class. Imposing coverage restrictions on Part B medicines would have a significant negative impact on patients. For example, one study examining a proposed model, found that using a cost-effectiveness-based standard to restrict access could result in 62 to 93 percent of patients with RA, multiple sclerosis, non-small cell lung cancer and/or multiple myeloma losing access to the treatments their physicians determined were best for them.²¹³ Although Part D plans typically do not apply this type of rigid cost-effectiveness standard, they do impose cost sharing and utilization management policies (like prior authorization requirements) based on presumptions of treatment equivalence that may not always be clinically appropriate and can have a similar effect on patient access. For medications where time is a critical factor in treatment, any delays due to benefit verification, prior authorization, or lack of coverage will have negative effects on patient outcomes.

Applying the Part D coverage floor to Part B drugs would also be of concern. The Part D statute requires plans to offer a minimum of two drugs in each USP MMG category or class.²¹⁴ Due to issues with the classification of rare genetic disorders, there is potential for plans to exclude coverage for certain diseases altogether.

In addition, restrictions in Part D plans like prior authorization or step therapy can increase administrative burden for providers and delay access to treatment. For example, an analysis of Part D formulary coverage for biologic Disease-Modifying Antirheumatic Drugs (DMARD) used to treat RA found that coverage for individual products ranged from 30 to 100 percent of plans, meaning some products were covered by a minority of Part D plans. While all plans covered at least one product, nearly all plans (97 percent) required prior authorization to access DMARD products.²¹⁵ Many of the treatments covered in Part B are complex biologics with few or no other treatment options and the utilization management techniques employed by many Part D plans for these medicines have the potential to delay or prevent patient access,²¹⁶ undermining adherence,

²¹² MedPAC. Report to Congress: Medicare Payment Policy. March 2017.

²¹³ Xcenda. Applying Cost-Effectiveness Thresholds to the Real World: Implications on Access for Medicare Beneficiaries. May 30, 2018. Available at: <https://www.xcenda.com/insights/phrma-issue-brief-applying-cost-effectiveness-on-access-for-medicare-beneficiaries>

²¹⁴ See 42 U.S.C.S. § 1395w-104(b)(3)(C) (LexisNexis 2018) (codified at 42 C.F.R. § 423.120(b)(2) (LexisNexis 2018))

²¹⁵ Yazdany, Jinoos, et al. Coverage for High-Cost Specialty Drugs for Rheumatoid Arthritis in Medicare Part D. *Arthritis & Rheumatology* 2015;67(6):1474-1480.

²¹⁶ American Society of Clinical Oncology. American Society of Clinical Oncology Statement on the Impact of Utilization Management Policies for Cancer Drug Therapies. *Journal of Oncology Practice*. 2017;13(11):758-762.

which will lead to poorer outcomes and increasing long-term medical costs for the health care system.²¹⁷

Furthermore, Part D plans have cost sharing as high as 33 percent for the specialty tier, and there is no mechanism for addressing access when there are no reasonable alternative medications with lower cost sharing. Under current CMS regulation and guidance, when a product is placed on a specialty tier in Part D, patients cannot seek formulary exceptions (a process available for medicines in other tiers) by demonstrating medical need for the specialty tier drug. Such an exception might be appropriate when a patient has failed on medicines available on lower-cost tiers, for example. Many of the products in Part B have the potential to fall into the specialty drug category,²¹⁸ and would then be exempt from the exceptions process, creating another barrier to access.

Operational Challenges

If coverage for physician-administered medicines shifts to Part D, it could change the providers' process for acquiring these medicines in a way that undermines their ability to customize dosing in response to changing individual patient needs, e.g., using lab values. Experience with Part D covered vaccinations suggests that physicians may not have an administratively simple way to bill Medicare Part D plans. Instead of purchasing medicines directly, providers may need to work with a specialty pharmacy to order medicines for their patients. Currently, more than half of providers prescribe Part D covered vaccines to seniors, but refer beneficiaries to pharmacies to purchase them.²¹⁹ In some cases (e.g., certain cancer treatments), physicians may need to make adjustments to the dosing and administration frequency of Part B products at the point of care that aren't easily accommodated in a specialty pharmacy or retail model. Inability of physicians to modify and customize dosing in response to individual clinical outcomes during administration was one of the major complaints that physicians had with CMS' CAP, as discussed in greater detail below. The potential exists to increasingly complicate the entire patient experience from lab work, to medication experience, to the interaction between a patient and their provider and pharmacy.

These operational issues can also compound barriers to patient access. Here another lesson can be drawn from Part D covered vaccines. Physicians who prescribe and administer these vaccinations are often unable to verify beneficiary coverage and cost-sharing liability when they are not included in the Part D plan's network. Also, when physicians cannot file Part D claims,

²¹⁷ Iuga, A. O., & McGuire, M. J. Adherence and health care costs. *Risk Management and Healthcare Policy*. 2014;7:35–44.

²¹⁸ CMS determines a threshold for a specialty drug in their annual call letter and only allows Part D drugs to be placed on specialty tiers if the majority of prescription drug events exceeds the dollar threshold. CMS, Available at: <https://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovGenIn/Downloads/CY-2016-Specialty-Tier-Methodology.pdf>. In 2018 the threshold was \$670 per month. CMS, Available at: <https://www.cms.gov/Medicare/Health-Plans/MedicareAdvtgSpecRateStats/Downloads/Announcement2018.pdf>

²¹⁹ Avalere Health. Shifting Drugs from Medicare Part B to Part D: Learnings from Medicare Coverage of Vaccines. June 2018.

the patient must sometimes pay the provider up front and then submit for reimbursement from their Part D plan, creating a potential financial burden. Another option is for patients to purchase the vaccine directly from the pharmacy and then transport the vaccine to their treating physician's office for administration.²²⁰ In a survey conducted by GAO, 8 in 10 physicians cited the amount of time used to identify beneficiaries' coverage and submit claims as a barrier to administering Part D vaccines. This ambiguity about cost may be discouraging patients from getting vaccinated, despite recommendations from their provider. GAO found that more than 60 percent of physicians report that beneficiaries decline shingles vaccinations about half the time or more. By comparison, only 1 in 10 physicians report that beneficiaries decline pneumococcal vaccinations, which are covered under Part B, half the time or more.²²¹ Physicians and patients would likely face similar billing and reimbursement barriers if Part B drugs were covered under Part D. While access barriers have been shown to interfere with the administration of a one-time vaccine, switching complicated physician-administered medicines could cause even more disruption for medicines that need to be administered more often—monthly or even weekly.

Of particular concern are the safety issues surrounding patient transportation and handling of complex medications. Under a Part B to Part D scenario, in some cases patients might be encouraged to pick up their medicine at a pharmacy and bring it to an infusion center for administration. This would not only be unnecessarily complicated and time consuming, but poses a significant potential public health hazard in the form of unintended exposures. Many of these medicines have intricate storage, handling, and administration requirements that are best met by a medical professional in a clinical setting for quality, safety, and liability reasons. Improper handling has the potential to be extremely wasteful and put patients at risk. By contrast, the current structure of the Part B benefit facilitates safe and effective use of Part B therapies. This is one of many reasons why Medicare and most commercial plans cover physician administered drugs in the medical benefit and reimburse through a buy-and-bill system.

Challenges Associated with Reducing or Eliminating the Drug Add-on Payment

Proposals to reduce or eliminate the percentage add on to ASP-based payment could make it financially untenable for physicians to provide certain medicines to Medicare beneficiaries. When CMS proposed a Part B drug payment demonstration that would have reduced the ASP add on in 2016, a survey of oncologists, hematologists, and rheumatologists found that physicians expected to realize a financial loss on approximately 40 percent of the products they administer if the Part B Drug Payment Model went into effect.²²² Such losses could lead community practices to close, consolidate, or refer patients to the hospital setting. For patients, this would mean traveling longer distances to obtain care and accelerating the shift to the hospital setting where treatment is more expensive for both beneficiaries and the Medicare program. A study from 2012 found that the average cost sharing for Medicare beneficiaries receiving chemotherapy was 24 percent

²²⁰ Id.

²²¹ Id.

²²² Xcenda. Provider Survey: Potential Impact of CMS Part B Drug Payment Model Proposed Rule. Conducted for PhRMA. April 2016.

higher in the hospital outpatient setting versus a physician's office.²²³ Other data showing cost increases associated with increased provider consolidation are included in our comments below on Site Neutrality for Physician-Administered Drugs. Past proposals to implement these types of changes in Part B coverage and reimbursement have been rejected over concerns that patients may experience treatment delays and higher costs, and that care could shift to more expensive settings.

Concerns with Shifting Part B Medicines to Part D Based on OECD Country Prices

The RFI asks whether Part B medicines should be shifted to Part D when prices in Organization for Economic Co-operation and Development (OECD) countries are lower than prices paid by Part B providers. PhRMA is deeply concerned with this concept, which links Medicare policy decisions to policies in other countries that artificially suppress prices through government-dictated access restrictions and arbitrary cost-effectiveness thresholds.²²⁴ At the same time, because many OECD countries have regulations that effectively prohibit the sale of medicines at U.S. prices, this would move many Part B medicines to Part D with the harms described above.

It is important to recognize that foreign price controls often lead to significant access barriers. Experience in several OECD countries have shown the dangers of the government attempting to make centralized, one-size-fits-all judgments of value. Restrictions imposed by the U.K.'s National Institute for Health and Care Excellence (NICE) have created substantial barriers between patients and life-saving treatments—recent analysis shows that from 2013 to 2017, nearly 92 percent of oncology treatments were given some kind of access restriction.²²⁵ Patients who live in countries that impose centralized value judgements also have access to fewer treatment options—recent data shows that nearly 90 percent of newly launched medicines were available in the U.S., compared to just two-thirds in the U.K., half in Canada and France, and one-third in Australia.²²⁶

MEDICARE PART B: Part B Competitive Acquisition Program (RFI p. 22697)

Relaunching a CAP in Part B could also reduce patient access to needed therapy and inhibit physicians' ability to provide Part B medicines in their offices. Below, we outline concerns with CMS' 2006-2008 CAP as well as a recent MedPAC proposal to relaunch CAP called the Drug Value Program (DVP). As CMS considers potential approaches it will be important to ensure that any proposal does not undermine the core strengths of the current, market-based ASP system, including: supporting and empowering patients and their physicians in making informed

²²³ Avalere Health. Total Costs of Cancer Care by Site of Service: Physician Office vs. Hospital Outpatient. March 2012.

²²⁴ See the additional discussion of foreign price controls in the International section of our comments.

²²⁵ Hughes K and N Jeswani. HTAs Recommendations for Oncology Have Grown More Restrictive Over Time. Avalere Health. June 2018. Available at: <http://avalere.com/expertise/life-sciences/insights/htas-recommendations-for-oncology-have-grown-more-restrictive-over-time>.

²²⁶ Haninger K. New analysis shows that more medicines worldwide are available to U.S. patients. PhRMA. The Catalyst blog. June 2018. Available at: <https://catalyst.phrma.org/new-analysis-shows-that-more-medicines-worldwide-are-available-to-u.s.-patients>

decisions from the range of available treatment options, avoiding disruptions or delays in delivery of medically beneficial care in the optimal treatment setting, and avoiding increases in patient costs that can lead to treatment abandonment.

To achieve this goal, it will be important for any competitive bidding proposals to be voluntary for physicians, and workable from the perspective of physicians and patients by reducing administrative burden and supporting quality patient care. It should not use formulary and utilization management tools that prevent patients from accessing care and place unnecessary administrative burden on physicians.

CMS' 2006-2008 CAP

CMS' original Part B CAP faced several challenges, including:

- **Initial payment rates exceeded reimbursements under the ASP system:** Based on Medicare claims processed through the National Claims History File as of June 2008, the cost of drugs administered through CAP exceeded 106 percent of ASP by approximately 3.2 percent in the aggregate for 2006 and 2007. This occurred in part because product utilization under CAP differed from that under buy and bill, which CMS had not accounted for in its payment methodology. CMS also adjusted CAP payments using the producer price index for prescription drugs, which resulted in further overpayments to vendors.
- **Vendor interest:** CMS received bids from vendors under the original CAP solicitation. However, only one vendor (Bioscrip) signed a contract with the agency to participate in CAP.²²⁷
- **Provider attrition:** CAP suffered from a very high provider attrition rate. At its peak enrollment, the program served just one thousand physician practices. 45 percent of practices participating in the CAP in 2006 opted not to participate in 2007, and 53 percent of practices participating in 2007 opted not to participate in 2008.²²⁸

In 2008, CMS postponed implementation, citing contractual issues with bidders.²²⁹

A criticism of the original Part B CAP was that it could have the effect of limiting physicians' ability to tailor treatment to meet the needs of their patients. Concerns with this aspect of the program contributed to low overall enrollment and higher dissatisfaction in targeted specialties like oncology. Oncologists in particular may need to alter the dose, formulation, or drug regimen at the point of care depending on the status of the patient on the day they present for treatment.

²²⁷ RTI International. Evaluation of the Competitive Acquisition Program for Part B Drugs: Final Report. December 2009. Available at: https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Reports/downloads/CAPPartB_Final_2010.pdf.

²²⁸ Id.

²²⁹ CMS. Competitive Acquisition for Part B Drugs & Biologicals.

CAP required physicians to place an order with the CAP vendor in advance of the patient visit, leaving open the possibility that physicians may not have the appropriate product(s) available if a change was needed the day of treatment.

CAP did include two provisions that were essential to preserving flexibility and access to treatment. The “Furnish as Written” provision allowed physicians to write for a specific National Drug Code (NDC) (e.g., to obtain a specific formulation of a drug that may not be supplied by the CAP vendor). Under this provision, the physician would obtain the drug privately and bill Medicare using the ASP methodology. Similarly, the “Emergency Restocking” provision allowed physicians to administer a CAP drug to a Medicare beneficiary from the physician’s own inventory and replace the drug by ordering from the vendor.

Physicians may have lacked the flexibility necessary to administer clinically appropriate treatment absent these two provisions. Use of these two provisions was unexpectedly high under CAP, particularly for patients who had multiple Part B drug claims and those with cancer and chronic conditions. Patients with seven or more CAP drug claims in 2006 received 40 percent of their CAP medicines under emergency restocking.²³⁰ For treatment of some cancers, 30 percent of claims were billed outside of the normal CAP billing procedure. Similarly, for patients with chronic conditions such as RA and asthma, one-third of their claims billed outside of the normal CAP billing process.²³¹

Experience with the 2006-2008 Part B CAP underscores the importance of preserving clinical flexibility and patient access under such a program. Even with these provisions, oncologists and other targeted specialists (e.g., rheumatologists, ophthalmologists, and other non-primary care specialties) had lower enrollment and higher dissatisfaction. Nearly one-third of oncology specialists and one-quarter of other targeted specialists said that they were dissatisfied with CAP, compared with just 17 percent of non-targeted specialists.²³² Further, 30 percent of oncology specialists believed their patients were inconvenienced by CAP.²³³

MedPAC’s DVP

The MedPAC has proposed an alternative to CAP that seeks to resolve some of the challenges with the 2006-2008 program. However, MedPAC’s proposal would severely limit access to treatment for Medicare beneficiaries via restrictive formularies, prior authorization, and step therapy. It would also undermine Part B’s market-based reimbursement system by imposing a binding arbitration process to set prices for innovative new medicines. Finally, it could threaten the viability of community practices and encourage costly consolidation by using changes to the ASP reimbursement system to drive physicians into the program. We strongly urge HHS to avoid policies that would have similar consequences for patients and the Medicare program.

²³⁰ RTI International. Evaluation of the Competitive Acquisition Program for Part B Drugs: Final Report. December 2009.

²³¹ Id.

²³² Id.

²³³ Id.

Like CAP, MedPAC’s proposed DVP would establish third-party vendors to negotiate prices for Part B drugs. However, these vendors would be permitted to establish formularies and utilization management requirements such as prior authorization and step therapy that could make it more challenging for Part B patients, many of whom have serious and complex conditions, to access the medications they need.

Part B’s current structure ensures the availability of a range of treatment options. Due to the personalized nature of many medicines in Part B and the diseases that they treat, patients often need to try multiple therapies before finding the appropriate treatment, and physicians and patients need maximum flexibility to tailor treatments to meet patients’ needs, consistent with clinical evidence. For this reason, imposing formularies or other utilization management tools would put patient access to treatment at risk. As discussed above, prescribing flexibility is essential to the management of complex conditions like cancer, RA, rare diseases and other conditions treated with Part B medicines. For example:

- Comorbid conditions can impact a patient’s tolerance for the toxicity of certain cancer medications. Patients with heart disease and congestive heart failure may require different medications than patients without these comorbid conditions to avoid serious and life-threatening complications.
- Patients with RA respond differently to biologic DMARD products, making choice of treatments critically important. Physicians frequently try a series of treatments until one is found that the patient responds to. In addition, RA patients often stop responding to one treatment over time, requiring them to shift to a different option.²³⁴
- Many patients with multiple sclerosis who are receiving an infused Part B medication may be on their second or third line of treatment. Step therapy requirements could force these patients to revisit therapies their physician has already determined are ineffective in managing their disease.

A recent survey of physicians underscores these concerns. 88 percent of oncologists and rheumatologists believe a CAP or DVP program would take care decisions away from the person in the best position to make that decision; more than 87 percent believe it would limit their ability to provide the best care to patients; and 75 percent of providers believe it would increase the administrative burden for their practices.²³⁵

²³⁴ American College of Rheumatology. 2012 Update of the 2008 American College of Rheumatology Recommendations for the Use of Disease-Modifying Antirheumatic Drugs and Biologic Agents in the Treatment of Rheumatoid Arthritis. *Arthritis Care & Research*. 2012;64(5):632.

²³⁵ Community Oncology Alliance. COA Physician Survey: Medicare Part B Proposals Will Harm Patients, Increase Costs and Bureaucracy. May 2018. Available at: <https://www.communityoncology.org/portfolio-items/coa-physician-survey-medicare-part-b-proposals-will-harm-patients-increase-costs-and-bureaucracy/?portfolioCats=64%2C60%2C67%2C70%2C59%2C69%2C66%2C65%2C63%2C61%2C68%2C51%2C58>

Policies like CAP are intended to make Part B more competitive. However, MedPAC’s DVP proposal would do the opposite by imposing a binding arbitration process to regulate prices for innovative new medicines. As one commissioner noted, “I am absolutely opposed to arbitration because the message that the Commission is sending is that we believe in free market, but then we don’t.”²³⁶ Another commissioner also noted that having a regulated price can interfere with market forces that would help to keep costs down.²³⁷

Finally, MedPAC’s DVP would encourage physician participation by reducing the add-on payment to ASP for those physicians who seek to remain in the buy and bill system. The add-on fee accounts for the variability in provider negotiating leverage and therefore the price at which products are purchased; it also helps cover complex storage and handling, other overhead costs, and ongoing patient monitoring. If the add-on payment were reduced further, some providers (particularly those in small practices or rural communities) would likely lose money on many products they administer. As described above, past proposals to implement these types of changes in Part B coverage and reimbursement have been rejected over concerns that patients may experience treatment delays and higher costs, and that care could shift to more expensive settings. These payment policies have the potential to lead to further physician-hospital consolidation, which MedPAC has previously noted, increases Medicare prices paid for physician services.²³⁸ For example, there has been substantial consolidation among outpatient oncology providers and hospitals or health systems. The shift in cancer care to hospital-based settings has led to higher costs to Medicare and its beneficiaries.²³⁹ For further discussion of hospital consolidation see our comments on site-neutral payments below.

MEDICARE PART B: HCPCS Codes as an Incentive to Commit to a Particular Pricing Scheme (RFI p. 22698)

Part B reimbursements are based on a proven, market-based metric that should be preserved. ASP is transparent and dynamic—it reflects commercially negotiated discounts and, as a result, changes over time in response to fluctuations in the market. As CMS notes in its quarterly analysis of the ASP pricing file, “there are a number of competitive market factors at work—multiple manufacturers, alternative therapies, new products, recent generic entrants, or market shifts to lower priced products”²⁴⁰ that contribute to price stability and even decrease ASP for several products quarter over quarter. As a result, ASP has built-in protections against price

²³⁶ Transcript: MedPAC Public Meeting. Comments by Amy Bricker, April 6, 2017. pp. 65-66. Available at: <http://www.medpac.gov/docs/default-source/default-document-library/transcript-04-17-ealizecombinedd14010adfa9c665e80adff00009edf9c.pdf?sfvrsn=0>

²³⁷ Transcript: MedPAC Public Meeting. Comments by Kathy Buto, April 6, 2017. p. 71. Available at: <http://www.medpac.gov/docs/default-source/default-document-library/transcript-04-17-combinedd14010adfa9c665e80adff00009edf9c.pdf?sfvrsn=0>

²³⁸ MedPAC. Report to Congress: Medicare and the Health Care Delivery System. Chapter 10 – Provider Consolidation: the role of Medicare policy. June 2017.

²³⁹ Milliman. Comparing Episode of Cancer Care Costs in Different Settings: An Actuarial Analysis of Patient Receiving Chemotherapy. August 2013.; Milliman. Cost Drivers of Cancer Care: A Retrospective Analysis of Medicare and Commercially Insured Population Claim Data 2004-2014. April 2016.

²⁴⁰ CMS, 2018. Available at: <https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Part-B-Drugs/McrPartBDrugAvgSalesPrice/2018ASPFiles.html>

inflation. Demanding that manufacturers “commit to a price over a particular lookback period” would not only undermine the market-based nature of the ASP reimbursement system, but is also unnecessary. Accordingly, PhRMA opposes tying eligibility for Healthcare Common Procedure Coding System (HCPCS) codes to price commitments.

PhRMA does support issuance of HCPCS codes for Part B drugs on a quarterly basis overall, to reduce administrative burdens and improve patient access to new medicines. Under the current process, CMS assigns HCPCS codes to new drugs on an annual basis. Manufacturers must apply for a HCPCS code for a new drug (either a drug already approved by FDA or shortly expected to be approved) by the first business day of January of year 1 to have the drug considered for a HCPCS code that would take effect on January 1 of year 2. If the drug was not yet approved by FDA when the application was submitted, it must then be approved by March 31 of year 1 or it will not be considered for a HCPCS code that (if granted) would take effect January 1 of year 2. Therefore, a drug approved by FDA on April 1 of year 1 can only be considered for a HCPCS code in year 2 (and would require the full application to be resubmitted in year 2) and then (assuming a code is granted) it would not take effect until January 1 of year 3—21 months after its approval.

The delay in the current HCPCS process creates unnecessary administrative burden for both payers and providers, and results in uncertainty in reimbursement that could be detrimental to patient access to medical advances. Until a HCPCS is assigned, providers must bill for newly approved products using an unlisted or miscellaneous HCPCS code. Because these codes are not specific to a single drug or technology, claims which include unlisted or miscellaneous codes require manual review by payers. This manual claim review process often requires the provider to include additional information on the claim form, such as the drug name, strength, route of administration, and the NDC.

CMS should assign new HCPCS codes for Part B drugs on a quarterly, rather than annual basis. This is simple and doable. In fact, based on an application process separate from the application process for ordinary HCPCS codes, CMS already assigns new drugs a special type of HCPCS code called a “C code” on a quarterly basis (but currently these codes only apply in hospital outpatient departments and therefore do not substitute for an ordinary HCPCS code). The C code process illustrates how simple it would be for CMS to reform the ordinary HCPCS application process for new drugs. Doing so would cut needless complexity and red tape and facilitate patients’ access to important new drug therapies that often treat life-threatening or otherwise very serious diseases.

MEDICARE PART B: Site Neutrality for Physician-Administered Drugs (RFI p. 22697)

Payment differentials, and differences in the cost of goods (e.g. the 340B program), incentivize hospital systems to acquire physician practices.²⁴¹ Consolidation leads to increased market

²⁴¹ Baker LC, Bundorf MK, Kessler DP. Vertical integration: hospital ownership of physician practices is associated with higher prices and spending. *Health Affairs*. 2014;33(5):756-63.

power, which allows hospitals to charge more for the same care, which drives up costs of care for patients with both public and private insurance.²⁴²

Spending on hospitals is increasing rapidly, driving up overall health care costs and premiums. Hospitals accounted for \$1.1 trillion in U.S. health spending in 2016, representing 32 percent of NHE, far more than any other category.²⁴³ When hospitals purchase physician practices, prices and spending increase, often without any corresponding increase in quality of care.²⁴⁴ A recent analysis of Medicare Fee-for-Service claims data between 2008 and 2015 showed a significant shift in site of care for outpatient drug therapies from the physician office to the 340B hospital outpatient setting.²⁴⁵ Physician-administered chemotherapy medicines are an example of how the shift from the community to hospitals contributes to higher spending. From 2004 to 2014, chemotherapy infusions in hospital outpatient departments increased dramatically, from 16 percent to 46 percent for Medicare patients. Drug spending was more than twice as high in the hospital setting. Had this consolidation not occurred, spending would have been 7.5 percent lower for Medicare infused chemotherapy patients.²⁴⁶

To address some of these concerns, in 2016 CMS finalized sections of the Bipartisan Budget Act of 2015 requiring that payments to certain entities for covered services, including physician administered medicines, be site-neutral. Recognizing that a system where Medicare pays for the same service at a higher rate if it is provided in a hospital outpatient department versus a physician's office creates perverse incentives for hospitals to acquire physician offices, CMS issued a regulation stating that certain services provided by certain off-campus hospital outpatient departments would no longer be paid under the Hospital Outpatient Prospective Payment System (HOPPS).²⁴⁷ The policy became effective in January 2017 but included some exceptions, most notably grandfathering in off-campus sites billing under HOPPS prior to November 2015, and some facilities with new or developing off-campus departments.²⁴⁸ While CMS has taken some steps to correct policies that incentivize shifts in site of service, additional consideration should be given to potential policies that would help address and prevent anticompetitive behavior that drives increased drug and overall health care spending.

²⁴² Bai G, Anderson GF. A more detailed understanding of factors associated with hospital profitability. *Health Affairs*. 2016;35(5):889-97.

²⁴³ CMS. NHE Data. 2009-2025 Expenditures and Projections.

²⁴⁴ Post B, Buchmueller T, Ryan AM. Vertical Integration of Hospitals and Physicians: Economic Theory and Empirical Evidence on Spending and Quality. *Medical Care Research and Review*. 2017: 1-35.

²⁴⁵ Berkeley Research Group. Site of Care Shift for Physician-Administered Drug Therapies. June 2017. Available at: https://www.thinkbrg.com/media/publication/943_943_Vandervelde_Site-of-Care-Oct-16-2017_WEB_FINAL-2.pdf

²⁴⁶ Fitch, Kathryn, et al. Cost Drivers of Cancer Care: A Retrospective Analysis of Medicare and Commercially Insured Population Claim Data 2004-2014. Milliman. 2016.

²⁴⁷ CMS. CMS Finalizes Hospital Outpatient Prospective Payment System Changes to Better Support Hospitals and Physicians and Improve Patient Care. 2016.

²⁴⁸ CMS. CMS Clarifies Site-Neutral Medicare Reimbursement Exceptions. 2017.

MEDICARE PART B: Site Neutrality Between Inpatient and Outpatient Setting (RFI p. 22697)

PhRMA appreciates the administration's interest in understanding payment policies that may drive patients or physicians to prefer treatment in the outpatient or inpatient setting. We urge HHS to consider how prospective payment systems like the Inpatient Prospective Payment Systems (IPPS) can affect site of care because reimbursement rates are set using historic cost information and may not accurately reflect the resources associated with the current standard of care. IPPS creates three challenges for reimbursement of new medicines, further documented in our comments on the 2019 IPPS Proposed Rule.²⁴⁹ First, existing Medicare Severity Diagnosis Related Groups (MS-DRGs) may not capture the additional resource utilization associated with a new therapy. In addition, CMS' standards for new technology add-on payments (NTAP), can exclude important new therapies because of small sample sizes or clinical evidence requirements that are unrealistic for therapies that are new to market, particularly if they were approved under an expedited FDA pathway. Finally, even if a manufacturer can clear the bar for approval of a NTAP, the combination of NTAP and outlier adjustments may still be insufficient to facilitate patient access to a beneficial new test or treatment. We encourage HHS to adopt a more holistic approach to accommodating new technologies in future years that considers the multiple levers at the administration's disposal to accurately calibrate reimbursement under the IPPS.

CMS might also consider whether payment distortions are inconveniencing patient and harming clinical outcomes, increasing costs for the health care system. HHS should consider whether improvements in care could be made through payment changes that encourage moving appropriate patients to a different care setting (outpatient infusion or home health), discharging patients earlier when clinically appropriate, or avoiding unnecessary hospitalizations.

SECTION VI: MEDICAID AND AFFORDABLE CARE ACT TAXES (RFI p. 22695)

Medicaid is a joint state and federally funded program that provides comprehensive health coverage to more than 70 million low-income Americans, including children and their parents, pregnant women, the elderly and people living with disabilities.²⁵⁰ When Congress created the Medicaid Drug Rebate Program in 1990, authorized by Section 1927 of the Social Security Act, it had two main goals—to lower state and federal expenditures for outpatient prescription drugs and to increase Medicaid beneficiary access to prescription drugs.²⁵¹ The Medicaid rebate statute requires biopharmaceutical manufacturers to provide substantial mandatory rebates in exchange for guaranteed state coverage of all covered outpatient drugs with limited exceptions. Under the

²⁴⁹ PhRMA comments on Medicare Program; Hospital IPPS for Acute Care Hospitals and the Long-Term Care Hospital Prospective Payment System and Proposed Policy Changes and FY 2019 Rates; Proposed Quality Reporting Requirements for Specific Providers; Proposed Medicare and Medicaid Electronic Health Record (EHR) Incentive Programs (Promoting Interoperability Programs) Requirements for Eligible Hospitals, Critical Access Hospitals, and Eligible Professionals; Medicare Cost Reporting Requirements; and Physician Certification and Recertification of Claims; CMS-1694-P.

²⁵⁰ CMS. March 2018 Medicaid & CHIP Enrollment Data Highlights.

²⁵¹ H. Rpt. 101-8 8 1, 101st Congress, 2d Session (Oct. 16, 1990).; As of 2018, about 600 biopharmaceutical manufacturers participate in the program along with all fifty states.

statute, participating manufacturers must enter into a national rebate agreement with CMS, and must also enter into agreements to provide discounts to 340B covered entities and to cap prices on sales of their drugs to four federal agencies (the Department of the Veterans Affairs (VA), the Defense Department (DoD), the Public Health Service, and the Coast Guard).²⁵²

Medicaid rebates for brand medicines have two components: a basic rebate and an additional inflation rebate if the price of a drug rises faster than inflation (based on changes in the Consumer Price Index-Urban). For brand drugs, the basic rebate is the greater of (a) 23.1 percent of the AMP or (b) the difference between AMP and the Best Price (the manufacturer's lowest net price for the drug to any customer with limited exceptions). For example, if a manufacturer's lowest net price to a customer included in the Best Price determinations in a quarter is 70 percent of AMP, then 70 percent of AMP would be the Best Price for every state Medicaid program; the additional rebate is capped by statute at 100 percent of AMP. The additional rebate is added to the basic rebate to get the total unit rebate amount (URA) on each unit dispensed to a Medicaid patient. Manufacturers may also negotiate voluntary supplemental rebates with states in addition to these mandatory rebates.

Prescription medicines represent a small share of Medicaid spending and provide substantial value to the program. In 2016, Medicaid programs spent on average just 5 percent of their budgets on retail prescription medicines, due in large part to the significant rebates received from manufacturers.²⁵³ In contrast, Medicaid programs spent about 9 percent on administrative costs and 31 percent on long term care services.²⁵⁴ Manufacturers provided \$42 billion in rebates in 2017, representing a more than 50 percent discount to states and the federal government.²⁵⁵ Many states put manufacturer rebates back into their general fund and do not earmark the money for Medicaid or prescription drug purposes, shielding them from the true net cost of medicines. The Medicaid population is particularly vulnerable, with significant health care needs compared to those with private insurance.²⁵⁶

Research has shown that better use of prescription medicines can create savings to Medicaid. For example, researchers have found that a 1 percent increase in prescription drug utilization decreases inpatient Medicaid costs by as much as 0.31 percent.²⁵⁷ Another analysis found that treating HIV/AIDS to viral load suppression saves state Medicaid programs an estimated \$1 million per treated patient by preventing future transmissions.²⁵⁸ It is also estimated that a new

²⁵² SSA 1927(a), 1927(k)(2).

²⁵³ MACPAC. MACSTATS. 2016.

²⁵⁴ The Menges Group analysis of FY2016 CMS 64 reports and State Drug Utilization data files. Brand and generic expenditure totals are net of rebates. Data used were predominantly derived from CMS 64 reports.

²⁵⁵ John Coster Presentation at Alliance for Health Policy, May 11, 2018.

²⁵⁶ MACPAC. MACStats: Medicaid and CHIP Data Book. December 2016.

²⁵⁷ Roebuck, C, Dougherty, S. Impact of Medication Adherence on Hospitalization in Medicaid. Poster Presentation at ISPOR 21st Annual International Meeting, Washington, DC. May 23, 2016.

²⁵⁸ Truven Health Analytics. Impact of pharmaceutical innovation in HIV/AIDS treatment during the highly active antiretroviral therapy (HAART) era in the U.S., 1987-2010: an epidemiologic and cost-impact modeling case study. December 2014.

medicine that delays the onset of Alzheimer’s disease by five years could reduce Medicaid spending by \$77 billion by 2050.²⁵⁹

Since the enactment of the ACA, the Medicaid program has grown and changed significantly, and manufacturer obligations have also increased because of new branded prescription drug taxes, increases in rebate amounts, and the expansion of the 340B program, which has increased 340B discounts. While manufacturer rebates have held down net prescription drug expenditures in Medicaid, the growth in manufacturer rebates and tax obligations is significant. Consequently, for reasons we discuss below, any measures to increase Medicaid rebates or to tax the industry further may not serve the intended purpose of reducing list prices. As CBO and many economists have suggested, imposing mandatory rebates and taxes on drug manufacturers can lead to higher prices for other customers.²⁶⁰

The President’s drug pricing initiative should further the goals of the Medicaid program and therefore avoid any changes that could ration care or otherwise limit vulnerable Medicaid patients’ access to prescription drugs. Accordingly, while we recognize that states have a desire to experiment with Medicaid coverage requirements, we do not support any new approaches that risk compromising Medicaid patients’ access to medicines by creating a closed formulary.

MEDICAID AND AFFORDABLE CARE ACT TAXES: ACA Taxes and Rebates (RFI p. 22695)

Since the passage of the ACA, Medicaid has undergone a period of significant growth with an additional 16 million enrollees. Thirty-three states, and the District of Columbia, have adopted the ACA Medicaid expansion.²⁶¹ Independent analysts estimate that the ACA will increase prescription drug rebates and industry taxes that brand manufacturers pay by almost \$70 billion through 2021.²⁶² Some of the more significant changes to Medicaid under the ACA include:

- The Medicaid minimum basic rebate increased from 15.1 percent of AMP to 23.1 percent of AMP

²⁵⁹ Alzheimer’s Association. Changing the Trajectory of Alzheimer’s Disease: How a Treatment by 2025 Saves Lives and Dollars. Available at: https://www.alz.org/help-support/resources/publications/trajectory_report

²⁶⁰ CBO. How the Medicaid Rebate on Prescription Drugs Affects Pricing in the Pharmaceutical Industry. January 1996.; RxEconomics for Pharmaceutical Industry Labor-Management Association. Medicaid Drug Rebates in Medicare Part D Low-income Subsidy: An Economic Analysis of the Proposed Policy and Its Implications for Multi-Employer Plans. June 18, 2013.

²⁶¹ Kaiser Family Foundation. Status of State Action on the Medicaid Expansion Decision. Available at: <https://www.kff.org/health-reform/state-indicator/state-activity-around-expanding-medicaid-under-the-affordable-care-act>

²⁶² PwC. Implications of the U.S. Supreme Court Ruling on Healthcare. Available at: <https://www.pwc.com/us/en/health-industries/health-research-institute/publications/pdf/implications-of-the-US-Supreme-Court-ruling-on-healthcare.pdf>

- Medicaid rebates were extended to Medicaid MCOs²⁶³
- The definition of AMP was altered to increase rebate amounts
- Medicaid rebates cover an even larger population due to the expansion of Medicaid and the extension of rebates to Medicaid MCO enrollees
- A new annual fee on sales by brand drug manufacturers that are reimbursed or purchased by certain federal programs (Medicaid, Medicare Part B, Medicaid Part D, and VA and DoD drug programs)²⁶⁴
- Expansion of 340B hospital eligibility which has driven program growth in subsequent years

While the Blueprint recognizes that, “drug spending has been held down in the Medicaid program,” the adverse consequences of rebate expansion, coupled with the creation of the branded prescription drug industry tax cannot be ignored.²⁶⁵ Government actuaries and economists have documented the unintended consequences of the Medicaid Drug Rebate Program in shifting costs to other parts of the pharmaceutical market and increasing prices for other customers. Secretary Azar himself has stated that, “both industry practices and government rules—encourages higher and higher list prices.”²⁶⁶ It is also likely that the same problems created by the Medicaid Drug Rebate Program also apply to the ACA industry tax. In fact, the Blueprint recognizes this, stating that “this expansion of [branded prescription drug fees plus Medicaid rebates] may have placed pressure on list prices by forcing drug manufacturers to raise prices overall.” Similarly, the Blueprint notes that “the additional billions of dollars in [340B] discounted sales and the cross-subsidization necessary may have created additional pressure on manufacturers to increase list price.” Similar concerns have also been raised by:

- CBO, which analyzed the Medicaid Drug Rebate Program’s impact on state and federal drug spending and on the broader pharmaceutical marketplace, found that “spending on prescription drugs by non-Medicaid patients may have increased as a result.”²⁶⁷
- GAO noted that “following enactment of the rebate program, discounts for outpatient drugs decreased significantly because manufacturers raised the prices they charged large private purchasers.”²⁶⁸ GAO also predicted that the larger the group entitled to a rebate, the “greater the incentive” is for manufacturers to increase prices.

²⁶³ In FFY 2016, almost half (49 percent) of all Medicaid expenditures were in Medicaid managed care organizations, up from 24 percent in 2010. Available at:

<https://www.healthmanagement.com/blog/medicaid-managed-care-spending-2016/>

²⁶⁴ Internal Revenue Service. Annual Fee on Branded Prescription Drug Manufacturers and Importers.

²⁶⁵ HHS, American Patients First.

²⁶⁶ Sec. Azar Blueprint remarks, May 14, 2018.

²⁶⁷ CBO. How the Medicaid Rebate on Prescription Drugs Affects Pricing in the Pharmaceutical Industry. January 1996.

²⁶⁸ GAO. Prescription Drugs: Expanding Access to Federal Prices Could Cause Other Price Changes. August 2000.

- The RxEconomics Literature Review found “compelling evidence that the Medicaid Drug Rebate Program has prompted reductions in the rebates extended to private payers, resulting in higher drug prices in [other markets].”²⁶⁹
- The Heritage Foundation, which in reviewing the impact of new ACA taxes, including fees on pharmaceutical companies, medical device manufacturers, and health insurance companies, found that these new taxes “will ultimately be passed on to [middle-income families] through higher prices.”²⁷⁰

The Best Price provision of the Medicaid Drug Rebate Program, designed to give the lowest net unit price given to any other customer (with limited exceptions) to every Medicaid program, has been shown to limit the discounts given to other customers. The Council of Economic Advisors recently highlighted issues related to the Medicaid Drug Rebate Program noting that the “Medicaid Best Price program can create artificially high prices in the private sector under certain conditions.”²⁷¹ Further, Best Price has posed a challenge to innovation in the pharmaceutical marketplace: numerous sources have found that Medicaid’s Best Price rebate provision makes it “unfavorable for drug manufacturers to enter into value-based contracts for their drugs,”²⁷² and is “in effect setting a floor under prices.”²⁷³

According to third party analysts, the additional rebate (which penalizes AMP increases exceeding the inflation rate) creates perverse incentives for high launch prices. CBO has opined that, “new drugs may be launched at a slightly higher price because of the Medicaid rebate.”²⁷⁴ They also indicate that, “the larger Medicaid’s anticipated share in total sales of a drug, the more important that effect is.”²⁷⁵

Since the enactment of the Medicaid Drug Rebate Program, economists and federal analysts have documented that while the program has reduced prescription drug expenditures in Medicaid, there are negative effects such as increased costs to private payers. Additionally, government experts found that proposals to extend Medicaid rebates to other government programs will likely increase Medicaid spending and negatively affect other drug payers, such as employers in the

²⁶⁹ RxEconomics for Pharmaceutical Industry Labor-Management Association. Medicaid Drug Rebates in Medicare Part D Low-income Subsidy: An Economic Analysis of the Proposed Policy and Its Implications for Multi-Employer Plans. June 18, 2013.

²⁷⁰ The Heritage Foundation. Obamacare: Impact on Taxpayers. April 2010. Available at: <https://www.heritage.org/health-care-reform/report/obamacare-impact-taxpayers>

²⁷¹ The Council of Economic Advisors. Reforming Biopharmaceutical Pricing at Home and Abroad. February 2018.

²⁷² American Action Forum, Current Impediments to Value-Based Pricing for Prescription Drugs. June 2017.

²⁷³ Medicaid Best Price: Health Policy Brief. *Health Affairs*. August 10, 2017. Available at: [https://www.healthaffairs.org/pb-](https://www.healthaffairs.org/pb-assets/documents/Collections/Collection_CMWF_Prescription_Drug_Pricing_May_2018.pdf)

[assets/documents/Collections/Collection_CMWF_Prescription_Drug_Pricing_May_2018.pdf](https://www.healthaffairs.org/pb-assets/documents/Collections/Collection_CMWF_Prescription_Drug_Pricing_May_2018.pdf)

²⁷⁴ CBO. How the Medicaid Rebate on Prescription Drugs Affects Pricing in the Pharmaceutical Industry. January 1, 1996.

²⁷⁵ Id.

commercial market. Specifically, CBO writes that “drug manufacturers would be expected to set higher ‘launch’ prices for new drugs as a way to limit the effect of the new rebate.”²⁷⁶

MEDICAID AND AFFORDABLE CARE ACT TAXES: Proposals Related to Maximum Rebate Amount (RFI p. 22695)

The Administration notes that imposing additional liabilities on the biopharmaceutical industry can lead to unintended consequences and cost shifting. However, the RFI also discusses developing proposals to repeal “the Affordable Care Act’s Maximum Rebate Amount provision, which limits manufacturer rebates on brand and generic drugs in the Medicaid program to 100% of the Average Manufacturer Price.”²⁷⁷ This cap is a modest safeguard that simply keeps Medicaid rebates from exceeding the payment a manufacturer receives for a drug and from making drugs a profit center for Medicaid. The proposed repeal of the Medicaid rebate cap could lead to further cost shifting for other customers, deepening the price distortions caused by the Medicaid Drug Rebate Program.

Medicaid rebates represent a discount of over 50 percent for all medicines and CBO estimates brand rebates average a discount of 63 percent of AMP.²⁷⁸ The current cap limits Medicaid rebates to 100 percent of AMP.²⁷⁹ For some medicines, the Medicaid rebate is already so large that the net cost of the drug (prior to any dispensing fees) is zero. Simply, manufacturers already are providing free drugs to the Medicaid program after rebates; therefore, repealing the cap would provide Medicaid with rebates that exceed the state’s payment to the dispensing pharmacy.

Since rebate liability is imposed on individual manufacturers—and over 600 pharmaceutical companies participate in the Medicaid rebate program—removing the cap on rebates is essentially creating a new industry tax that will force manufacturers to pay the government a fee for participation in Medicaid, a program that serves over 70 million people. As the previous reports cited above found, increasing Medicaid rebates and industry taxes will further distort prices in the commercial market and create perverse incentives and pressure to increase launch prices. Finally, manufacturers already hold up their end of the statutory coverage-rebate bargain by paying significant rebates to states for drugs utilized by the Medicaid population. The federal

²⁷⁶ CBO. *Require Manufacturers to Pay a Minimum Rebate on Drugs Covered Under Part D of Medicare for Low-Income Beneficiaries*. December 8, 2016.

²⁷⁷ RFI at 22695.

²⁷⁸ CBO. *Options for Reducing the Deficit: 2017-2026*. December 2016. This 63 percent of AMP figure includes the two components of the Medicaid rebate on a brand name drug: (1) the “basic rebate” (23.1 percent of AMP or [AMP minus Best Price], whichever is higher); and (2) the “additional rebate” (the current-quarter AMP minus the inflation-adjusted AMP from the drug’s baseline period, which usually is the first full quarter after the drug’s launch). This does not take into account supplemental rebates that States may negotiate from manufacturers on top of the federal rebate required under the rebate statute. See also HHS Office of the Assistant Secretary for Planning and Evaluation, *Report to Congress, Prescription Drugs: Innovation, Spending, and Patient Access*, 10 (Dec. 7, 2016) (“About half of Medicaid gross spending on prescription drugs is returned to the federal government and the states in the form of manufacturer rebates”).

²⁷⁹ § 2501(e) of the ACA.

government and states should not ‘profit’ off of this new tax by repealing this cap—a policy which will not achieve the goal of lowering list prices and could potentially backfire.

MEDICAID AND AFFORDABLE CARE ACT TAXES: New Approaches (RFI p. 22693)

The Blueprint recognizes that drug spending has “been held down in the Medicaid program by other tools,” and the “program’s rules prohibit the use of closed formularies, but states [may] use preferred drug lists.”²⁸⁰ Prescription drugs have consistently been a low share of Medicaid spending over the last decade due in large part to the significant rebates states receive from manufacturers, even as the program has undergone extensive expansions.²⁸¹ The Blueprint notes that states have multiple cost containment strategies to manage prescription drug spending, but states have expressed a need for more flexibility to limit drug coverage than they currently have under the rebate statute. While we support states’ engaging in testing new approaches to providing the best care to their population, we strongly oppose any proposals that ration access to prescription drugs in Medicaid through a closed formulary. The rebate statute reflects a carefully-crafted bargain that guarantees large rebates in exchange for coverage of all covered outpatient drugs.

Today, almost all states have created preferred drug lists and utilize prior authorization to negotiate extra voluntary supplemental rebates from manufacturers.²⁸² Despite the Medicaid rebate statute’s coverage requirements, some states place significant restrictions on medicines in the form of prior authorization or delays in coverage. Additionally, now that Medicaid MCOs serve most Medicaid patients, CMS should consider additional transparency requirements with regards to coverage and access. We strongly encourage CMS to preserve and work to improve access to medicines for vulnerable Medicaid patients. Medicaid patients, compared to those with other types of insurance, have higher rates of complex and chronic health conditions that often require access, without delay, to a broad range of medicines as prescribed by their physicians in order to achieve optimal therapeutic results. In addition to poorer health status, Medicaid patients tend to be more financially vulnerable, with few to no alternative options to obtain the medicines they need. Patients who access health insurance through employers or the individual market often have more options to select different coverage or pay out of pocket when needed.

As the Administration is considering new approaches to Medicaid financing and coverage of new medicines, PhRMA urges the Administration to consider the following principles:

- **Access:** Access for vulnerable Medicaid beneficiaries must be preserved.
 - Medicaid patients must have a streamlined and timely appeals or exceptions process to access any available therapy that recognizes the enormous pressures facing many physicians or prescribers who treat Medicaid patients.

²⁸⁰ RFI p. 22693.

²⁸¹ CMS. NHE Accounts. 2017.

²⁸² CMS, HHS. Medicaid Pharmacy Supplemental Rebate Agreements (as of March 2017). 2017.

- Medical providers, in consultation with patients, should be able to determine the medicines that best meet patients’ needs. Cost sharing for prescription drugs should not be an access barrier in Medicaid.
- Any transition to a new approach should ensure patients do not experience a disruption in coverage.
- **Statutory Bargain:** Under the law, manufacturers pay rebates on covered outpatient drugs in Medicaid in exchange for guaranteed coverage that cannot be broken.
 - CMS should not negotiate directly with companies or interfere in private negotiations between manufacturers and states or MCOs.

Any new approach that the Administration is considering must have a rigorous and independent evaluation that looks at the broad impacts of the new approach. A February 2018 GAO report found that the “federal government did not require complete and timely evaluations from the states,” so results on the new approaches were not complete and often not made available to the public.²⁸³ New approaches to prescription drug financing must include an analysis of beneficiary access and satisfaction as well as changes in adherence and health outcomes. Only looking at prescription drug spending changes is insufficient to fully evaluate how any new approach will impact the Medicaid population and its patients at large.

SECTION VII: 340B DRUG DISCOUNT PROGRAM (RFI p. 22698)

The 340B Drug Discount Program was created by Congress in 1992 to restore the voluntary drug discounts for uninsured or vulnerable patients that manufacturers provided before the passage of the Medicaid drug rebate statute. As part of the 340B program, manufacturers provide steep discounts averaging about 50 percent²⁸⁴ on most outpatient medicines to certain types of clinics (known as “grantees”) and to qualifying hospitals as a condition of their medicines being covered by Medicaid. PhRMA and our member companies strongly support the 340B program, which when used to benefit patients, plays a significant role in our health care safety net. The 340B program is particularly crucial to supporting the care provided by grantees, which serve our nation’s most vulnerable patients. These grantees are on the front lines of public health threats and represent a lifeline for many vulnerable patients without another source of care.

Safety-net clinics must generally meet federal requirements to reinvest any profit derived from reselling 340B medicines into care for uninsured or vulnerable patients as part of their grant requirements. In contrast, current 340B program rules lack any standards for how 340B discounts should be used by 340B hospitals. Hospital use of 340B is concentrated in the disproportionate share (DSH) hospitals that comprise 80 percent of all 340B sales.²⁸⁵ The lack of program standards for how DSH hospitals use 340B discounts, combined with the significant growth of the program driven by these hospitals, has greatly transformed the 340B program. It is no longer

²⁸³ GAO. Medicaid Demonstrations: Evaluations Yielded Limited Results, Underscoring Need for Changes to Federal Policies and Procedures. February 20, 2018.

²⁸⁴ CBO. Prices for Brand-Name Drugs Under Selected Federal Programs. June 2005.

²⁸⁵ Hatwig C. Apexus Update, 340B Health Summer Conference, 2016.

accurate to characterize the program as primarily focused on care for vulnerable patients by safety-net providers. Instead, more than two thirds of DSH hospitals that participate in 340B provide below national average levels of free and reduced cost treatments to uninsured or vulnerable patients, when compared to all hospitals.²⁸⁶ As a 2014 *Health Affairs* study on 340B put it, the program has evolved “from [a program] that serves vulnerable communities to one that enriches hospitals.”²⁸⁷

340B DRUG DISCOUNT PROGRAM: The Growth of 340B (RFI p. 22698)

Today’s 340B program is unrecognizable in size and character as compared to the program that was created in 1992. It took 15 years after 340B’s enactment (2007) for annual 340B sales to reach \$3.9 billion. Yet in the last 10 years, between 2007 and 2017, 340B sales at the 340B price grew by nearly 400 percent to \$19.3 billion.²⁸⁸ The MedPAC May 2015 Report to Congress provides data showing that between 2005 and 2013, 340B sales grew seven times faster than total U.S. medicine spending.²⁸⁹ Between 2002 and 2017, the number of 340B designated contract pharmacy arrangements increased from 279 to 51,963.²⁹⁰ Nearly 90 percent of that growth came after HRSA’s 2010 sub-regulatory guidance authorizing unlimited contract pharmacy networks. From 2013 to 2017, the number of hospital entities participating in the program tripled.²⁹¹ Yet over that same period, 340B purchases as a share of hospitals’ total drug purchases consistently and steadily increased,²⁹² while hospitals’ uncompensated care dropped.²⁹³

This growth has not been accompanied by evidence that patients are more likely to benefit from 340B discounts. In fact, a 2018 study in the *New England Journal of Medicine* found the opposite: 340B-eligible hospital status was associated with serving lower proportions of low-income patients in hematology-oncology and ophthalmology and did not show clear evidence of increased care for, or lower mortality among, low-income patients.²⁹⁴

²⁸⁶ Alliance for Integrity and Reform of 340B. *Benefiting Hospitals, Not Patients: An Analysis of Charity Care Provided by Hospitals Enrolled in the 340B Discount Program*. Spring 2016.

²⁸⁷ Conti R, Bach P. The 340B Drug Discount Program: Hospitals Generate Profits by Expanding to Reach More Affluent Communities. *Health Affairs*. 2014;33(10):1786-1792.

²⁸⁸ Fein A. EXCLUSIVE: The 340B Program Reached \$19.3 Billion in 2017 – As Hospitals’ Charity Care Has Dropped., *Drug Channels Blog*. May 7, 2018.

²⁸⁹ Analysis of data from MedPAC. Report to the Congress: Overview of the 340B Drug Pricing Program. May 2015, pp. 11-12.

²⁹⁰ HRSA OPA Database, January 2017.

²⁹¹ GAO. Drug Discount Program: Status of Agency Efforts to Improve 340B Program Oversight. May 15, 2018.

²⁹² Fein A. 340B Purchases Were More than Half of the Hospital Market in 2016. *Drug Channels Blog*. May 19, 2017.

²⁹³ Fein A. EXCLUSIVE: The 340B Program Reached \$19.3 Billion in 2017 – As Hospitals’ Charity Care Has Dropped. *Drug Channels Blog*. May 7, 2018.

²⁹⁴ Desai S, McWilliams JM. Consequences of the 340B Drug Pricing Program. *N Engl J Med*. 2018.

340B DRUG DISCOUNT PROGRAM: HRSA Rulemaking Authority (RFI p. 22699)

As the RFI and Blueprint recognize, the 340B program has grown substantially since its inception, and its current size may have created additional pressures on manufacturers to increase list prices.²⁹⁵ The RFI asks whether providing HHS with general 340B rulemaking authority could materially affect the elements of the program affecting drug pricing. To be clear, HRSA already has authority to make reforms and it should exercise its authority to update its guidance on four key aspects of the program as described below—a clearer patient definition in line with the statute, meaningful limits on hospital child sites, a reassessment of the contract pharmacy policy, and a more comprehensive and effective duplicate discount prevention guidance. Based on existing guidances, HHS believes it already has authority to provide interpretive guidance in these areas, and it should take action in these key areas promptly. Moreover, as discussed below, currently Apexus—a contractor to HHS—is issuing guidance on 340B issues instead of the government itself issuing guidance. This causes confusion about the status of the guidance and accordingly we would recommend that interpretive guidance on key 340B program issues come solely from HHS.

However, in response to the RFI’s questions, we support providing HHS with appropriate 340B rulemaking authority in those areas where such authority would be useful in aligning the program with the text and purpose of the 340B law. It would be important for Congress to provide legislative guidance on the use of such authority and to monitor its use carefully to ensure that it is not used in a way that further promotes unwarranted growth or otherwise adds to the program’s unintended consequences; that departs from the program’s mission to serve low income and vulnerable patients; that imposes needless burdens on any stakeholders; or harms grantees.

340B DRUG DISCOUNT PROGRAM: The Unintended Consequences of the 340B Program (RFI p. 22699)

The size of the 340B program creates market-distorting incentives that affect consumer prices for medicines, shift care to more expensive hospital settings, and accelerates provider market consolidation. A growing body of evidence from nonpartisan, independent sources, including *The New England Journal of Medicine*, *Journal of the American Medical Association (JAMA)*, the GAO, and others, points to data showing that the 340B program is driving up costs for everyone. Costs are being driven up in at least three ways:

1. **Cost shifting that distorts market prices:** Economists who study the 340B program suggest the current size of the program is leading to cost shifting and higher prices for consumers.²⁹⁶ A study in *JAMA* noted that list prices for medicines are likely higher than they otherwise would be “to offset revenue losses incurred as a larger number of drug sales become eligible

²⁹⁵ Conti R, Bach P. Cost Consequences of the 340B Drug Discount Program. *JAMA*. 2013;309(19):1995-1996.

²⁹⁶ Conti R, Rosenthal M. Pharmaceutical Policy Reform — Balancing Affordability with Incentives for Innovation. *N Engl J Med*. 2016;374:703-706.

for 340B discounts (and thus, fewer drugs are sold at full price).”²⁹⁷ Some therapeutic areas are particularly impacted by 340B. For example, for certain cancer drugs, sales to 340B hospitals account for 33 percent of *all* Medicare Part B reimbursement.²⁹⁸

2. **Perverse incentives to prescribe more medicines or more expensive medicines:** Because the discount on a 340B drug is typically a significant discount on the drug’s list price, 340B hospitals make more money when patients take more medicines or when more expensive medicines are prescribed. A 2015 GAO study found that this incentive was driving up costs in Medicare Part B.²⁹⁹ While the Administration took an important first step towards addressing these incentives in Part B in the 2018 HOPPS rule,³⁰⁰ hospitals continue to be able to profit from the 340B discounts for other payers.³⁰¹ This potential profit seems to be creating the same incentives in the commercial market. A recent study from the actuarial firm Milliman found higher spending on outpatient medicines for commercially insured patients at 340B hospitals compared to non 340B hospitals.³⁰² These perverse incentives extend to contract pharmacies. A recent GAO study found that some contract pharmacies receive higher reimbursement for brand 340B prescriptions.³⁰³ Rena Conti, an expert on drug pricing, raised concerns about this GAO finding in a recent interview with *Politico*, noting, “here’s a policy that is maximizing revenue for hospitals and contract pharmacies and perversely going against the intent of the program, which is to provide accessible and affordable health care for vulnerable people.”³⁰⁴
3. **Shifting care from community-based physicians to higher-cost settings:** Many hospitals have leveraged their ability to generate revenue from 340B by buying community-based physician practices and then obtaining 340B discounts for prescriptions written by those physicians.³⁰⁵ These off-site hospital clinics (known as “child sites”) are often located in wealthier areas than the 340B hospitals themselves³⁰⁶ and have no requirement to treat uninsured or vulnerable patients. These shifts in ownership and site of treatment not only undermine community-based practices but also drive concentration in provider markets,

²⁹⁷ Conti R, Bach P. Cost Consequences of the 340B Drug Discount Program. *JAMA*. 2013;309(19):1995-1996.

²⁹⁸ Drugs sold to 340B hospitals account for 33 percent of *all* Part B reimbursement for breast cancer and multiple myeloma drugs. Vandervelde A, Blalock E. Measuring the Relative Size of the 340B Program: 2012-2017. Berkeley Research Group. July 2017.

²⁹⁹ GAO. Medicare Part B Drugs: Action Needed to Reduce Financial Incentives to Prescribe 340B Drugs at Participating Hospitals. GAO-15-442. June 2015.

³⁰⁰ 80 Fed Reg 59216, (Dec. 14, 2017).

³⁰¹ A Vandervelde and E Blalock. Site of Care Shift for Physician-Administered Drug Therapies. Berkeley Research Group. October 2017.

³⁰² Milliman. Commercial Payers Spend More on Hospital Outpatient Drugs at 340B Hospitals. March 2018.

³⁰³ GAO. Drug Discount Program: Federal Oversight of Compliance at 340B Contract Pharmacies Needs Improvement. June 2018.

³⁰⁴ Karlin-Smith S. Perverse incentives? Why some 340B pharmacies are opting for branded drugs. *Politico*. July 2, 2018.

³⁰⁵ Desai S, McWilliams JM. Consequences of the 340B Drug Pricing Program. *N Engl J Med*. 2018.

³⁰⁶ Conti R, Bach R. The 340B Drug Discount Program: Hospitals Generate Profits By Expanding To Reach More Affluent Communities. *Health Affairs*. 33(10).

leading to higher prices for payers, the government, and patients. For cancer care, an analysis by IMS Health found that average commercial costs for administering cancer medicines are typically twice as high at hospital outpatient departments compared to treatment by community-based oncologists.³⁰⁷

340B DRUG DISCOUNT PROGRAM: Hospital Outpatient Prospective Payment System (RFI p. 22684)

Last year the Administration took an important first step to try to address some of these market distortions. Citing analysis from the GAO³⁰⁸ and MedPAC³⁰⁹ regarding the discrepancy between hospitals' discounted acquisition costs and their full reimbursements for 340B medicines, CMS' 2018 HOPPS final rule took steps to address these incentives by reducing the reimbursement for Medicare Part B drugs for a subset of 340B hospitals. While more still needs to be done to address the program's perverse incentives to prescribe more medicines and more expensive medicines, it is critically important that the reimbursement change remain in place and HRSA follow CMS' lead and begin reforms to address other areas of the program that lead to growth and distort the market, like the overly broad patient definition, and flawed contract pharmacy policy and child site guidances discussed below.

340B DRUG DISCOUNT PROGRAM: Improvements to the 340B Program are Urgently Needed in Key Areas to Refocus the Program to its Intended Purpose (RFI p. 22684)

Guidance released by HRSA has led to legally questionable policies in fundamental parts of the program. Based on evidence from GAO, OIG, analysis in the *New England Journal of Medicine*, *JAMA*, and others,³¹⁰ immediate changes are needed in each of the following areas to help refocus the program to its intended purpose:

Patient Definition: The 1996 patient definition should be clarified and updated to more clearly define who is entitled to manufacturer discounts on 340B medicines

The 340B program was originally created to provide manufacturer discounts on covered outpatient drugs to safety-net facilities that serve low-income, uninsured, and other vulnerable patients. Unlike hospitals, grantees are good stewards of that mission and have strict requirements

³⁰⁷ IMS Institute for Healthcare Informatics. Global Oncology Trend Report: A Review of 2015 and Outlook to 2020. June 2016.

³⁰⁸ GAO. Medicare Part B Drugs: Action Needed to Reduce Financial Incentives to Prescribe 340B Drugs at Participating Hospitals. GAO-15-442. June 2015.

³⁰⁹ MedPAC. Report to the Congress: Overview of the 340B Drug Pricing Program. May 2015.

³¹⁰ HHS OIG. Contract Pharmacy Arrangements in the 340B Program. February 2014; GAO, Medicare Part B Drugs: Action Needed to Reduce Financial Incentives to Prescribe 340B Drugs at Participating Hospitals. GAO-15-442. June 2015; Desai S, McWilliams JM. Consequences of the 340B Drug Pricing Program. *N Engl J Med*. 2018; Conti R, Bach P. Cost Consequences of the 340B Drug Discount Program. *JAMA*. 2013;309(19):1995-1996; Hirsch BR, Balu S, Schulman KA. The Impact Of Specialty Pharmaceuticals As Drivers Of Health Care Costs. *Health Affairs*. 2014;33(10):1714-1720.

on how they use the revenue generated through the 340B program to help those vulnerable or uninsured populations.

Under the 340B law, a covered entity has access to a 340B discount under the program if the medicine is used for the covered entity's own "patient."³¹¹ The 340B law further prohibits covered entities from reselling or otherwise transferring medicines purchased under the 340B program to anyone but a "patient" of the covered entity (a practice specified in 340B law as "diversion").³¹²

Despite this centrality of "patient" to defining the program's scope and assuring that statutory program integrity requirements are met, it has been a quarter of a century since the 340B program was created, and the patient definition still needs correction³¹³—despite a clear consensus that the lack of specificity in the current (1996) patient definition invites abuse. For example:

- "[S]ome 340B covered entities may have interpreted the [patient] definition too broadly, resulting in the potential for diversion of medications purchased under the 340B Program.... This [never finalized] clarification provides covered entities with more explicit guidance regarding the relationship between a covered entity and an individual that makes that individual a 'patient' of the covered entity." (HRSA, 2007.³¹⁴)
- "HRSA officials told us that the [patient] definition currently includes individuals receiving health care services from providers affiliated with covered entities through 'other arrangements' as long as the responsibility for care provided remains with the entity. However, HRSA does not define 'other arrangements,' and officials told us what is meant by responsibility for care also needs to be clarified. As a result of the lack of specificity in the guidance, HRSA has become concerned that some covered entities may be broadly interpreting the definition to include individuals such as those seen by providers who are only loosely affiliated with a covered entity and thus ... for whom the entity does not actually have the responsibility for care." (GAO, 2011.³¹⁵)
- "[C]overed entities ... use different methods to identify 340B-eligible [patients and] prescriptions to prevent diversion in their contract pharmacy arrangements. In some cases, these different methods lead to differing determinations of 340B eligibility.... [T]wo covered entities may categorize similar types of prescriptions differently (*i.e.*, 340B-eligible versus not 340B-eligible) [T]here is inconsistency within the 340B

³¹¹ 42 U.S.C. § 256b(a)(5)(b).

³¹² 42 U.S.C. § 256b(a)(5)(B).

³¹³ We support the general approach to defining a 340B "patient" reflected in HRSA's proposed (now withdrawn) omnibus guidance, taking into account considerations for HRSA grantees in the 340B program. 80 Fed. Reg. 52300 (Aug. 28, 2015).

³¹⁴ 72 Fed. Reg. 1543, 1544 (Jan. 12, 2007).

³¹⁵ GAO. Drug Pricing: Manufacturer Discounts in the 340B Program Offer Benefits, but Federal Oversight Needs Improvement. September 23, 2011.

program as to which prescriptions filled at contract pharmacies are treated as 340B-eligible.” (HHS OIG, 2014.³¹⁶)

- “HRSA has outlined three criteria for who is an eligible patient, but some of these criteria are not clearly defined.” (MedPAC, 2015.³¹⁷)
- “HRSA’s guidance addresses patient eligibility, but leaves room for interpretation as to which of the patient’s prescriptions might be eligible in a retail pharmacy setting. In these retail settings, we found that providers, in fact, are making different determinations of what prescriptions are eligible for the 340B discounts.” (Oral Testimony of Ann Maxwell, Assistant Inspector General, OIG, Senate Health, Education, Labor & Pensions (HELP) Committee, May 15, 2018.)
- “HRSA’s current patient definition guidance does not account for the complexity of contract pharmacy arrangements...In its 2014 report, OIG found wide variation in these [340B] eligibility determinations. Different determinations of 340B eligibility appear to stem from the application of the patient definition by 340B providers and their contract pharmacies to a wide variety of prescription-level scenarios. Depending on the interpretation of HRSA’s patient definition, some 340B provider eligibility determinations would be considered diversion and others would not.” (Testimony of Ann Maxwell, Assistant Inspector General, OIG, Senate HELP Committee, May 15, 2018.³¹⁸)

We urge HRSA to correct this problem and promptly eliminate the opportunities for abuse inherent in the current patient definition, which HRSA issued 22 years ago. Much has changed in the health care system since 1996, including a decrease in the number of uninsured Americans, much in part due to Medicaid expansion,³¹⁹ and the definition of this key term in the 340B program needs to be updated to reflect the current environment and to ensure a clear and reasonable interpretation of the statutory term “patient” is in place.

As highlighted by HRSA itself along with GAO and OIG, the 1996 patient definition is vague and lacks the specificity needed to provide clear direction to covered entities and manufacturers about who is a patient for 340B discount purposes.³²⁰ This has encouraged covered entities to take broad interpretations of the patient definition guidance and use 340B medicines for individuals who in many instances are those who Congress never intended to qualify for the program.

The 340B statute creates an absolute prohibition on covered entities transferring or selling 340B drugs to individuals who are not patients of the covered entity. Therefore, a clear definition of

³¹⁶ HHS OIG. Contract Pharmacy Arrangements in the 340B Program. February 5, 2014.

³¹⁷ MedPAC. Report to the Congress: Overview of the 340B Drug Pricing Program. May 2015.

³¹⁸ Ann Maxwell, HHS OIG, Testimony Before the United States Senate Committee on Health, Education, Labor and Pensions: Examining Oversight Reports on the 340B Drug Pricing Program, May 15, 2018.

³¹⁹ Medicaid and CHIP Payment and Access Commission. MACStats: Medicaid and CHIP Data Book, Exhibit 10. December 2017.

³²⁰ Debra Draper, GAO, Testimony Before the Committee on Health, Education, Labor & Pensions, U.S. Senate, May 15, 2018.; Testimony of Ann Maxwell, OIG, Testimony Before the Committee on Health, Education, Labor & Pensions, U.S. Senate, May 15, 2018.

“patient” is a key element of the program and critical to the integrity and long-term sustainability of the 340B program. HRSA has an obligation to update and clarify its 340B patient definition to address the current health care system and to incorporate clear and enforceable standards. HRSA should consider promptly finalizing a new patient definition that contains the core elements proposed by HRSA in its 2015 omnibus guidance (and any other elements necessary to comply with the statute). We believe finalizing such a definition through new guidance would make important strides in bringing the definition current and resolving many of the inconsistencies in the way stakeholders have interpreted this key term. As we indicated in our 2015 comments to HRSA,³²¹ the patient definition should also address the diverse arrangements and delivery modes of treatment provided by HRSA grantees in the 340B program.

Off-site Hospital Clinics (“Child Sites”): Current guidance on eligibility criteria for child sites is outdated, is driving up costs and consolidation, and should be updated

The 340B law defines the types of hospitals that can participate in the program with great specificity,³²² but never mentions participation of off-campus outpatient facilities associated with these hospitals. Although there is no basis in the statute for including these sites, in 1994, HRSA unilaterally issued guidance dramatically expanding the 340B program by permitting child sites to participate—even if as private DSH hospitals have interpreted, they are only loosely connected to the parent hospital and do not serve a needy population.³²³ Child sites have become a major source of the program’s growth and incentives. In 1994, there were a total of 34 child sites. By 2016 this had increased to over 15,000.³²⁴

In addition to accounting for much of the 340B program’s explosive growth, the hospital child site policy has shifted the program away from its original goal of helping get discounted medicines to uninsured and vulnerable patients.³²⁵ For example, a 2014 *Health Affairs* study found that child sites are converting 340B “from [a program] that serves vulnerable communities to one that enriches hospitals.”³²⁶ The authors of a recent *New England Journal of Medicine* Perspective on 340B state that “hospitals have purchased community practices in part ... to expand their footprint into wealthier neighborhoods to ‘profit’ from the 340B program.”³²⁷ Hospitals purchasing physician practices leads to higher costs for many payers and patients because commercial reimbursement for hospital-owned practices are typically higher due to their market power—thereby increasing costs on government payers, commercial insurers, and patients in the form of higher cost sharing and premiums.³²⁸

³²¹ 80 FR 52300.

³²² 42 U.S.C. § 256b(a)(4)(L)-(O).

³²³ 59 Fed. Reg. 47884, 47885 (September 19, 1994).

³²⁴ HRSA OPA Database, October 2016.

³²⁵ Vandervelde A, Blalock E. 340B Program Sales Forecast: 2016 – 2021. 2016. Available at: <http://340breform.org/userfiles/December%202016%20BRG%20Growth%20Study.pdf>.

³²⁶ Conti M, Bach P. The 340B Drug Discount Program: Hospitals Generate Profits by Expanding to Reach More Affluent Communities. *Health Affairs*. 2014;33(10): 1786-1792.

³²⁷ Gellad, WF, James AE. Discounted drugs for needy patients and hospitals—understanding the 340B Debate. *New England Journal of Medicine*. 2018;378(6):501-503.

³²⁸ As discussed earlier, while the administration recently made changes to address 340B hospitals’ incentives to increase spending in Medicare Part B, that change will likely have a minimal impact on

HRSA should revisit its 1994 guidance given the rampant growth in the number of child sites, the lack of any requirements that these clinics serve a safety-net role, and the evidence that they are leading to higher costs for many patients. Reforms are needed to align HRSA’s guidance with the 340B law’s text and its goal of improving eligible patients’ access to medications, including tightening the eligibility criteria to assess when these outpatient facilities are considered part of a covered entity for 340B program purposes.

Contract Pharmacy: Rampant growth of hospital use of contract pharmacy arrangements must be reined in through updated guidance

Contract pharmacies, which are not mentioned in the 340B statute—have expanded rapidly since HRSA’s 2010 expansion of its previous contract pharmacy guidance. The evidence to date shows significant problems with unlimited and unchecked expansion of 340B into the retail pharmacy setting, especially as driven by DSH hospital arrangements. First, covered entities and contract pharmacies generally are not abiding by the compliance safeguards suggested by HRSA in its 2010 guidance.³²⁹ A June 2018 study by the GAO found “weaknesses in HRSA’s oversight that impede its ability to ensure compliance with 340B Program requirements at contract pharmacies.”³³⁰ Second, many contract pharmacy arrangements have been cited for duplicate discount violations; moreover, HHS OIG has found that contract pharmacies make compliance with the duplicate discount ban more complicated and OIG and GAO have both found that contract pharmacies also increase the risk of diversion violations.³³¹ Third, and most concerning, OIG found that unlike grantees, 340B hospitals generally are not sharing discounts with uninsured patients through their contract pharmacies.³³² Without benefit to needy patients, as the 340B program was intended, the dramatic expansion of contract pharmacy arrangements into the for-profit, retail pharmacy sector represents an unreasonable and unnecessary risk to program compliance.

Contract pharmacies can generate higher returns by dispensing 340B prescriptions than non-340B prescriptions, however uninsured patients are not always offered the 340B discounted price at contract pharmacies contracting with DSH hospitals.³³³ Despite the fact that the 340B program was designed to ensure increased access to prescription medicines for vulnerable or uninsured patients, the 2014 OIG report found that the majority of hospitals in their study did not ensure that they passed 340B discounts back to uninsured patients who filled their prescriptions at a contract

incentives for future provider consolidation. The new Part B reimbursement changes are by definition limited to the less than one quarter of DSH hospitals’ 340B profits derived from Part B fee-for-service sales and the new policy will not impact newly acquired outpatient sites that are not paid under HOPPS.

³²⁹ HHS OIG. Contract Pharmacy Arrangements in the 340B Program, supra, at 1-2.

³³⁰ GAO. Drug Discount Program: Federal Oversight of Compliance at 340B Contract Pharmacies Needs Improvement. June 2018.

³³¹ HHS OIG. Contract Pharmacy Arrangements in the 340B Program, supra, at 1-2; GAO. Manufacturer Discounts in the 340B Program Offer Benefits but Federal Oversight Needs Improvement, supra, at 28-29.

³³² HHS OIG. Contract Pharmacy Arrangements in the 340B Program, supra, at 14 (only one-third of hospitals surveyed by OIG reported that they passed through 340B discounts to uninsured patients in at least one of their contract pharmacy arrangements, vs. 83 percent of surveyed grantees).

³³³ HHS OIG. Contract Pharmacy Arrangements in the 340B Program. February 2014.

pharmacy.³³⁴ In contrast, the grantee covered entities in the OIG study were more likely to have developed systems for their contract pharmacies to pass 340B discounts on to uninsured patients.³³⁵

Contract pharmacy expansion is also a troubling example of intermediaries diverting resources from 340B's intended purpose of assisting low-income or vulnerable patients. An industry of for-profit pharmacies and their third-party administrators and consultants has developed since 2010 with the goal of maximizing 340B dispensing.³³⁶ These entities financially benefit from taking a share of the markup between the legally mandated 340B price and the higher price paid by patients and insurers. Little to no oversight exists to monitor contract pharmacies and these third-party vendors.

The current unlimited use of contract pharmacies by hospitals is not sustainable and diverts savings from 340B to for-profit pharmacies and other intermediaries. HRSA should use its authority and revisit its current unlimited contract pharmacy policy, particularly as it applies to how contract pharmacies are used by some covered entities such as DSH hospitals. Any new policy must consider what role, if any, hospitals' contract pharmacies should play in a program that has grown significantly over the past eight years and has failed to benefit patients.

Hospital Eligibility: Hospital eligibility standards are outdated, and the requirements in statute are not well enforced

With 45 percent of all current acute care hospitals participating in a program that was first intended for true safety-net facilities,³³⁷ the eligibility criteria for DSH hospitals must be reexamined. DSH hospitals qualify for the 340B program based in part on their DSH percentage,³³⁸ an inpatient measure relating to the number of Medicaid and low-income Medicare patients treated in a hospital's inpatient unit. MedPAC reported that it had found little correlation between hospitals' DSH adjustment percentages and whether they had a high percentage of uninsured patients.³³⁹ While changes to the DSH metric must be made legislatively, it is an important issue that the Administration should consider given it has driven growth in the program and does not target the 340B program's intended patient population or even represent an outpatient care metric.

HRSA does have an important role to play in ensuring hospitals that are eligible for the 340B program meet the statutory criteria to be true safety-net facilities. To ensure discounts are for hospitals serving a truly indigent or vulnerable population, HRSA should issue meaningful

³³⁴ Id.

³³⁵ Id.

³³⁶ Senator Charles Grassley. Letter to Walgreens CEO Gregory Watson. July 21, 2013. Available at: <https://thebeatatcooleyhealth.files.wordpress.com/2013/08/walgreens-340b-letter-grassley.pdf>; Talyst. Benefits to Becoming a Contract Pharmacy. Available at: http://www.talyst.com/wp-content/uploads/Talyst_White_Paper_Benefit_Becoming_Contract_Pharmacy.pdf

³³⁷ MedPAC. Report to the Congress: Overview of the 340B Drug Pricing Program. May 2015.

³³⁸ See 42 U.S.C. § 256b(a)(4)(L)-(O).

³³⁹ MedPAC. Report to the Congress: Medicare Payment Policy. March 2007.

eligibility standards for hospitals not owned or operated by a state or local government. The statute requires that all 340B hospitals must be owned or operated by a unit of state or local government or be a private nonprofit hospital that (a) has been formally granted governmental powers by a state or local government; or (b) has a contract with a state or local government to provide health care services to low-income individuals who are not Medicare or Medicaid eligible. Unfortunately, there is little guidance, transparency, or oversight to enforce these requirements. In fact, HRSA does not even review or collect the contracts that make some hospitals eligible for 340B discounts. Instead, the responsibility falls on hospitals to self-report if they believe they no longer meet the requirements. GAO noted that “hospitals with contracts that provide a small amount of care to low-income individuals not eligible for Medicare or Medicaid could claim 340B discounts, which may not be what the agency intended.”³⁴⁰ This lack of oversight makes it difficult to ensure that contracts are meeting congressional intent. The legislative history states that a private nonprofit hospital that had “a minor contract to provide indigent care which represents an insignificant portion of its operating revenues” could not qualify for 340B under the state and local government contract test.³⁴¹ Yet HRSA is not enforcing this requirement, which could easily be done routinely when HRSA recertifies a hospital’s 340B eligibility.

340B DRUG DISCOUNT PROGRAM: Program Dynamics (RFI p. 22699)

Role of the Prime Vendor

The RFI asks specifically about the impact of the Prime Vendor Program. The 340B statute created the Prime Vendor Program “under which covered entities may enter into contracts with prime vendors for the distribution of covered outpatient drugs.”³⁴² Over the 15 years Apexus has been the recurring awarded Prime Vendor, the role of the 340B Prime Vendor Program has expanded to other areas including education and assistance for all program stakeholders. Importantly, none of these expanded activities are funded by fees paid by covered entities. Under the current model, Apexus is obliged to engage in additional revenue generating activities separate from its 340B communication and training programs. We have concerns that these conflicting obligations impact Apexus’ ability to share 340B program information with HRSA in an unbiased way.

We support the concept of Apexus providing basic facts about the program, such as answering the question “can a for-profit hospital participate in the 340B program?” However, we are concerned that due to HRSA’s failure to issue updated rules, Apexus’ current role has veered into setting policies through the posting of Frequently Asked Questions (FAQs).³⁴³ For example, on the Apexus website:

³⁴⁰ GAO. Manufacturer Discounts in the 340 Program Offer Benefits, but Federal Oversight Needs Improvement, GAO-11-836 (Sept.2011), p 23.

³⁴¹ U.S. House of Representatives Report accompanying H.R. Rep. 102-384 (II) (1992).

³⁴² Sec, 340B PHSA(a)(8).

³⁴³ Apexus. Frequently Asked Questions. May 23, 2018. Available at: <https://www.340bpvp.com/resource-center/faqs>

“HRSA relies on Apexus to communicate policy and provide award-winning education, training, and support to all 340B stakeholders.”

“Frequently Asked Questions: Apexus is communicating these HRSA FAQs with the intention of improving program compliance. Additional FAQs may be available to address specific circumstances by contacting Apexus Answers. The removal of an FAQ from the website does not imply that the FAQ is no longer supported by HRSA. Certain FAQs are best applied when details are presented in the appropriate context, according to a specific covered entity’s situation, and Apexus Answers can facilitate that level of communication and application.”

In some cases, it appears to be setting entirely new policies in key areas including patient definition.³⁴⁴ This raises concerns about why a third-party contractor—and not HHS—is issuing guidance. Both GAO and OIG have raised concerns that current program rules are overly broad and not well-enforced.³⁴⁵

In addition to ceding authority to Apexus for policy communication, HRSA has empowered Apexus with unique sales data and price negotiation access. Apexus has long advertised its unique status as the only group contracting option for those covered entities subject to the statutory group purchasing organization (GPO) prohibition.

“Q. Why is it permissible for Apexus to establish contracts for the non-GPO account for hospitals subject to the GPO prohibition?”

A: Certain hospitals must agree to not participate in a GPO for the purchase of outpatient covered drugs as a condition of eligibility for participation in the 340B program. Apexus, as HRSA’s contracted 340B Prime Vendor, is not considered a GPO and is permitted to perform such group purchasing functions on behalf of all entities who voluntarily participate in the prime vendor. The HRSA agreement enables Apexus to contract for outpatient covered drugs and other value-added products on behalf of participating covered entities.”

Under its distribution contracts, Apexus has price visibility and can enforce data reporting standards not available to other stakeholders. For example, Apexus states, “Pricing rules with the wholesalers are monitored by the Prime Vendor to support compliance of manufacturers and covered entities.” Solutions to improving the role of the prime vendor in the 340B program include HRSA clearly defining activities and providing adequate funding for any 340B Prime

³⁴⁴ See, for example, FAQ ID: 1442 “Q: May providers that have admitting privileges at our 340B participating hospital be considered eligible providers under the 'other arrangements' provision of patient definition?”.

³⁴⁵ GAO. Medicare Part B Drugs: Action Needed to Reduce Financial Incentives to Prescribe 340B Drugs at Participating Hospitals. GAO-15-442. June 2015.; HHS OIG. Contract Pharmacy Arrangements in the 340B Program. February 5, 2014.

Vendor Program (or supporting contractor) to eliminate conflicting areas of responsibility, as well as HRSA fulfilling its role as administrator of the program, by providing sufficient and updated program rules and official agency communications.

Inventory Control Models

The RFI specifically asks about 340B inventory control models. The most common 340B inventory model used today is a virtual inventory and replenishment model to track the dispensing and ordering of 340B medicines. Instead of using a physical inventory model where contract pharmacies fill 340B prescriptions from a designated inventory of 340B medicines that are separately stocked and apart from usual inventory, contract pharmacies utilize a “virtual replenishment model” to fill prescriptions from their existing stock, managing their inventory as usual. A 340B third-party administrator (TPA) then reconciles medicines dispensed to 340B patients, and replenishes the contract pharmacy’s stock using the covered entity’s 340B medicines.

As the program is currently structured, there are no requirements on the time frames or dates for when a claim must be identified and adjudicated as 340B. This allows, and results in, TPAs going back several years in the past, scrubbing adjudicated claims, and submitting them for 340B discounts. Due to HRSA’s outdated, vague patient definition and the insufficient methods to prevent duplicate discounts (see below), this type of activity leads to duplicate discounts and diversion. Providing contract pharmacies an easy way to reconcile their claims must be balanced in a way that maintains program integrity.

340B DRUG DISCOUNT PROGRAM: Duplicate Discounts Drive Program Integrity Issues (RFI p. 22699)

Current mechanisms to identify and prevent duplicate discounts are ineffective

The 340B program prohibits covered entities from purchasing a medicine at a 340B discount that also generates a Medicaid rebate claim.³⁴⁶ Consequently, the law creates an absolute prohibition on duplicate discounts.³⁴⁷ Despite this clear statutory imperative, current prevention methods do not stop or prevent 340B duplicate discounts. Two primary factors lead to duplicate discounts: 1) insufficient oversight of the 340B program, and 2) the creation and unfettered expansion of contract pharmacies.

The increasing use of contract pharmacies coupled with expansion of Medicaid rebates for medicines used by Medicaid MCO enrollees have exacerbated the problem of duplicate discounts—with HRSA and CMS thus far not taking effective steps to prevent this statutory violation. In 2014, HRSA released guidance that expressly excluded MCO drug utilization from the only mechanism HRSA has developed to prevent duplicate discounts (the Medicaid Exclusion

³⁴⁶ 42 U.S.C. § 256b(a)(5)(A).

³⁴⁷ Sec. 340B PHSA(a)(5)(i).

File (MEF)), stating that it needs to develop, in conjunction with CMS, a policy for MCOs.³⁴⁸ As of 2018, this policy has yet to be developed.

The FY 2017 HRSA covered entity audit data show that two-thirds of all DSH hospitals audited were noncompliant in at least one area, and many were noncompliant in multiple areas.³⁴⁹ It is not clear how HRSA addressed covered entity violations of program requirements, but at least one Congressional committee found little evidence for strong agency oversight citing that “HRSA rarely terminates covered entities from the 340B program through the audit process.”³⁵⁰

GAO released a report at the end of June on contract pharmacies which highlights these concerns in clarifying detail. The report found that because HRSA only assesses the potential for duplicate discounts in fee-for-service and not MCOs, “[u]ntil HRSA develops guidance and includes an assessment of the potential for duplicate discounts in Medicaid managed care as part of its audits, **the agency does not have assurance that covered entities’ efforts are effectively preventing noncompliance**”³⁵¹ (emphasis added).

Suggestions for Improving Prevention of Duplicate Discounts

HRSA has an explicit statutory mandate “to establish a mechanism to ensure that covered entities comply”³⁵² with the prohibition on duplicate discounting. We suggest the following for HRSA to comply with its statutory requirement and we are open to working with HRSA to develop other solutions:

1. **HRSA should work with CMS to address duplicate discounts, as HRSA stated it would do in 2014 guidance.** In 2014, HRSA stated it was “working with CMS to develop policy” to prevent duplicate discounts in MCOs.³⁵³ The notice encouraged covered entities and States to work together to develop alternative strategies for preventing duplicate discounts for MCO drugs.³⁵⁴ This policy or guidance has yet to be developed. In its June 2018 Report to Congress, GAO recommends that HRSA should issue guidance on the prevention of duplicate discounts in MCOs and that it should work together with CMS to achieve this. However, while HRSA concurs with the GAO’s

³⁴⁸ HRSA. 340B Drug Pricing Program Release No. 2014-1. December 12, 2014. The MEF mechanism requires that 340B covered entities either “carve in” (provide 340B drugs to Medicaid patients and report this practice to HRSA, so that these entities are listed on the Exclusion File and State Medicaid programs do not bill manufacturers for rebates on drugs furnished by these entities) or “carve out” (do not provide 340B drugs to Medicaid beneficiaries, so that drugs supplied by a 340B entity to a Medicaid patient triggers a Medicaid rebate, but not a 340B discount). Under the 2014 guidance, this mechanism no longer applies to prevent double discounts on 340B drugs provided to MCO beneficiaries.

³⁴⁹ HRSA OPA Database Program Integrity FY17 Audit Results. March 6, 2018.

³⁵⁰ Energy & Commerce Committee’s “Review of the 340B Drug Pricing Program.”

³⁵¹ GAO. Drug Discount Program: Federal Oversight of Compliance at 340B Contract Pharmacies Needs Improvement. June 2018.

³⁵² Sec. 340B PHSA(a)(5)(A)(ii).

³⁵³ HRSA. 340B Drug Pricing Program Notice: Clarification on use of the MEF. Release No. 2014-1. December 12, 2014.

³⁵⁴ HHS OIG. State Efforts to Exclude 340B Drugs from Medicaid Managed Care Rebates. June 2016.

findings, it provides insufficient excuses for why such guidance has not been issued and does not provide any concrete detail for when such guidance may be forthcoming.³⁵⁵ We agree with the GAO, that HRSA and CMS must work together now to enforce the law’s duplicate discount ban.

2. **Require a claim modifier.** According to OIG, there are two ways to identify 340B claims, a provider-level method or a claim-level method.³⁵⁶ The first option is to require covered entities to use the MEF not just for fee-for-service utilization but also for MCO utilization. The second option is to create a claims modifier for all public and private payers, including fee-for-service and MCOs in Medicaid.³⁵⁷ Last year, CMS began requiring hospitals subject to the new Medicare Part B 340B drug payment reduction, to identify 340B drugs, so many 340B hospitals are already using a claims modifier as part of Medicare reimbursement rules.³⁵⁸

HRSA created the contract pharmacy policy out of guidance and therefore, it should take action to implement GAO’s recommendations to improve duplicate discount prevention by issuing new or revised guidance. GAO highlighted HRSA’s authority to issue new guidance in its June 2018 report when it concluded that “Since the establishment of the 340B Program, HRSA has used interpretive guidance and statements of policy to provide guidance to covered entities regarding compliance....As such, **we continue to believe that further clarification, whether provided as interpretive guidance, audit procedures, or another format, is necessary to help ensure compliance with program requirements**”³⁵⁹ (emphasis added).

340B DRUG DISCOUNT PROGRAM: Impact of Commercial Rebates Paid on 340B Discounted Medicines (RFI p. 22699)

A prescription drug with a negotiated commercial rebate can also be subject to a 340B discount. While some manufacturers may include in their contracts with commercial plans that drugs purchased through the 340B program are not eligible for further rebates to the health plan, without a means to prospectively identify 340B-eligible claims at the point of sale (e.g., a claims identifier), these contract terms are difficult to operationalize and enforce. The 340B program is already growing; if manufacturers pay a rebate on a medicine that was already purchased at a large discount, it is likely that this compounds the distortive impact that economists say that 340B discounts already have on prescription medicine prices.³⁶⁰

³⁵⁵ GAO. Drug Discount Program: Federal Oversight of Compliance at 340B Contract Pharmacies Needs Improvement. June 2018.

³⁵⁶ HHS OIG. State Efforts to Exclude 340B Drugs from Medicaid Managed Care Rebates. June 2016.

³⁵⁷ CMS and HRSA could consider specific identifiers for Medicaid MCOs such as IDs on Medicaid patients with BIN/PCN number.

³⁵⁸ Fed. Reg Vol. 82, No. 217.

³⁵⁹ GAO. Drug Discount Program: Federal Oversight of Compliance at 340B Contract Pharmacies Needs Improvement. June 2018.

³⁶⁰ Conti R, Rosenthal M. Pharmaceutical Policy Reform — Balancing Affordability with Incentives for Innovation. *N Engl J Med.* 2016;374:703-706.; Conti R, Bach P. Cost Consequences of the 340B Drug Discount Program. *JAMA.* 2013;309(19):1995-1996.

SECTION VIII: COST-SHARING ASSISTANCE CARDS (RFI p. 22698)

Commercial health plans are increasingly using high deductibles, coinsurance, and multiple cost-sharing tiers that push more costs onto the sickest patients. High prescription medicine cost sharing may limit patients' access to needed treatments, reduce adherence, and lead to poor outcomes. Individual manufacturers provide cost-sharing assistance cards, which are referred to as "copay discount cards" in the RFI,³⁶¹ in response to a benefit design system that would otherwise leave many patients with unaffordable out-of-pocket costs for their medicines at the pharmacy counter. These cost-sharing assistance cards can improve patient access and adherence to prescription medicines by reducing patients' out-of-pocket burden. This assistance is essential to patient affordability for the sickest patients who need ongoing treatment for chronic conditions such as multiple sclerosis and RA, and rare diseases and conditions. Ensuring patients have affordable access to their medicines is a top priority for PhRMA. Maintaining availability of cost-sharing assistance cards for patients should be a key part of the Administration's efforts to promote access to affordable medicines for patients. Thus, the Administration should not seek to change the current exclusion of cost-sharing assistance cards from the determination of AMP and Best Price, as is contemplated in the RFI. Such a reform would be inconsistent with the statute, would likely raise Medicaid prices (through lower statutorily required rebates if cost-sharing assistance cards were included in the calculation of AMP) for some medicines, and could reduce the availability of this assistance.

The RFI asks about the potential role of cost-sharing assistance cards in government programs.³⁶² PhRMA's response to those questions are included above in our comments on Medicare Part D.

COST-SHARING ASSISTANCE CARDS: Need for Cost-Sharing Assistance Cards (RFI p. 22698)

In the last decade, commercial health plan designs have shifted more costs to patients through increased use of deductibles and coinsurance.

- When a patient is in the deductible, they typically must pay the list price of their medication up to the deductible amount. Since 2006, deductibles for patients in employer health plans have increased by 300 percent.³⁶³
- When patients pay coinsurance, they must pay a percentage of costs associated with their health care service or medicine. Patient out-of-pocket spending on coinsurance has increased 67 percent while spending on copays has decreased.³⁶⁴

³⁶¹ RFI p. 22698

³⁶² RFI p. 22698.

³⁶³ Claxton, G., Rae, M., Long, M., Damico, A. 2017 Employer Health Benefits Survey. Available at: <https://www.kff.org/report-section/ehbs-2017-section-7-employee-cost-sharing/#figure710>

³⁶⁴ Claxton, G., Levitt, L., Rae, M., Sawyer, B. Increases in cost-sharing payments continue to outpace wage growth. Peterson-Kaiser Health System Tracker. June 2018. Available at: <https://www.healthsystemtracker.org/brief/increases-in-cost-sharing-payments-have-far-outpaced-wage-growth>

- The share of employer health plans requiring a deductible for prescription medicines has more than doubled from 23 percent in 2012 to 52 percent in 2017.³⁶⁵

Deductibles and coinsurance leave patients with high and often unpredictable costs, particularly for their medicines. Average patient out-of-pocket costs for deductible and coinsurance claims for brand medicines are much higher than copay claims.³⁶⁶ In 2017, more than half of commercially insured patients' out-of-pocket spending for brand medicines was for medicines filled while a patient was in the deductible or with coinsurance, an increase of 20 percent from 2013.³⁶⁷ Patients with chronic conditions are disproportionately impacted by high out-of-pocket costs.³⁶⁸ Research has shown that just 7 percent of claims are responsible for over half of all patient out-of-pocket costs for brand medicines.³⁶⁹ Without cost-sharing assistance cards many of these patients would have trouble paying the out-of-pocket costs for their medicines.

In many cases, individual manufacturers provide cost-sharing assistance cards to lower patients' out-of-pocket burden at the pharmacy counter since patients are often not benefiting directly from rebates. When a patient pays cost sharing for prescription drugs in a deductible or with coinsurance, their cost sharing is typically based on the undiscounted list price. PBMs negotiate discounts on brand medicines on behalf of health plans and employers that substantially reduce the list price. For certain medicines used to treat chronic conditions like asthma, high cholesterol, HCV, and diabetes, these discounts and rebates can reduce list prices by as much as 30 to 70 percent.³⁷⁰ However, the discounts are given in the form of rebates paid directly to the PBM and are not commonly passed through to patients. This creates additional affordability challenges at the pharmacy counter. In contrast, when patients pay cost sharing for medical care from an in-network hospital or physician, deductible and coinsurance payments are based on discounted rates negotiated between the health plan and the provider. Research has shown that sharing

³⁶⁵ PwC. Health and Well-Being Touchstone Survey 2012-2017.

³⁶⁶ Devane, K., Harris, K., Kelly, K. Patient Affordability Part One: The Implications of Changing Benefit Designs and High Cost-Sharing. May 2018. Available at: <https://www.iqvia.com/locations/united-states/patient-affordability-part-one>

³⁶⁷ Id.

³⁶⁸ Cox C et al. Examining high prescription drug spending for people with employer sponsored health insurance. Kaiser Family Foundation. October 27, 2017. Available at: <https://www.healthsystemtracker.org/brief/examining-high-prescription-drug-spending-for-people-with-employer-sponsored-health-insurance/#item-start>

³⁶⁹ Devane, K., Harris, K., Kelly, K. Patient Affordability Part One: The Implications of Changing Benefit Designs and High Cost-Sharing. May 2018.

³⁷⁰ QuintilesIMS Institute. Estimate of Medicare Part D Costs After Accounting for Manufacturer Rebates. October 2016.; Gronholt-Pedersen J, Skydsgaard N, Neely J. Novo Nordisk Defends U.S. Diabetes Drug Pricing. *Reuters*. November 4, 2016. Available at: <http://www.reuters.com/article/us-novo-nordisk-prices-idUSKBN12Z184>; Silverman E. What the 'Shocking' Gilead Discounts on its Hepatitis C Drugs Will Mean. *W.S.J.* February 4, 2015. Available at: <https://blogs.wsj.com/pharmalot/2015/02/04/what-the-shocking-gilead-discounts-on-its-hepatitis-c-drugs-will-mean/>; Barrett P, Langreth R. The Crazy Math Behind Drug Prices: Intermediaries that Negotiate to Lower Prices May Cause Them To Increase Too. *Bloomberg Businessweek*. June 29, 2017. Available at: <https://www.bloomberg.com/news/articles/2017-06-29/the-crazy-math-behind-drug-prices>

manufacturer rebates with certain commercially insured patients who have deductibles and coinsurance can save patients up to \$800 annually.³⁷¹

Cost sharing for prescription drugs is also unique in that patients must pay the full cost sharing for their medicine to take their medicine home from the pharmacy. In the case of care provided at a hospital or physician's office, patients often pay their cost sharing after care is received and may be able to negotiate a discount with the provider or work out a payment plan to pay over time. Cost-sharing assistance cards are a private market solution to address the challenges patients faced at the pharmacy counter when asked to pay the full cost sharing required by their insurers up-front before getting their medicine.

Higher out-of-pocket costs for prescription medicines can have significant negative impacts on patient health.

- Patients with leukemia who faced high out-of-pocket cost for medicines on a specialty tier were less likely to initiate drug therapy than patients who received an LIS (53 percent versus 21 percent). Patients with high out-of-pocket costs also took twice as long to initiate treatment.³⁷²
- Research has shown that patients are more likely to abandon or delay starting their anticancer drugs as out-of-pocket costs increase.³⁷³ Only 10 percent of patients abandoned therapy when costs were less than \$10 but that rate tripled when costs were above \$100.
- New data shows that over half of patients did not start their new brand medicines when their out-of-pocket costs reach \$125.³⁷⁴ Most patients who abandoned their brand drugs do not fill another drug within 90 days, indicating they may not be receiving any treatment for their condition.³⁷⁵

³⁷¹ Bunger, A., Gomberg, J., Hunter, M., Petroske, J. Point of Sale Rebate Analysis in the Commercial Market: Sharing rebates may lower patient cost and likely has minimal impact on premiums. 2017. Available at: <http://phrma-docs.phrma.org/download.cfm?objectId=5F5FD190-AEDD-11E7-833F0050569A4B6C>

³⁷² Doshi JA, Li P, Huo H, et al. High cost sharing and specialty drug initiation under Medicare part D: A case study in patients with newly diagnosed chronic myeloid leukemia. *Am J Manag Care*. 2016;22(4 Suppl):s78-86.

³⁷³ Doshi J, et al. Association of Patient Out-of-Pocket Costs with Prescription Abandonment and Delay in Fills of Novel Oral Anticancer Agents. *ASCO*. Available at: <http://ascopubs.org/doi/abs/10.1200/JCO.2017.74.5091>

³⁷⁴ Devane K, et al. Patient Affordability Part Two: Implications for Patient Behavior and Therapy Consumption. May 2018. Available at: <https://www.iqvia.com/locations/united-states/patient-affordability-part-two>

³⁷⁵ IMS Institute for Healthcare Informatics. Emergence and impact of pharmacy deductibles: implications for patients in commercial health plans. September 2015. Available at: <https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/emergence-and-impact-of-pharmacy-deductibles.pdf>

- RAND researchers found that doubling copays reduced patients' adherence to mental health and asthma medicines by 25 to 32 percent. Their research also found that because of increased cost sharing, emergency room visits and hospitalizations also increased.³⁷⁶

Cost-sharing assistance cards help patients who face high out-of-pocket costs for their medicines and can mitigate patient abandonment rates while advancing public health benefits. Patients who utilize cost-sharing assistance cards for brand medicines, including specialty drugs, are asked to pay much higher cost sharing at the pharmacy counter compared to patients who do not use cost-sharing assistance.³⁷⁷ Coupons help to mitigate this higher cost sharing. Specialty drugs have the highest cost sharing and, in many cases, there are no lower cost alternatives available. Recent analysis by IQVIA found that cost-sharing assistance cards can mitigate patient abandonment rates by up to half.³⁷⁸ Research has also shown that cost-sharing assistance card use is very low among brand medicines with a generic available. In 2017, only 0.4 percent of commercial claims were filled with a cost-sharing assistance card for brand medicines with a generic alternative.³⁷⁹

Even when patients use cost-sharing assistance cards to lower their cost sharing, PBMs have ample tools to manage health insurers' spending on medicines. PBMs can, and often do, subject medicines to prior authorization and step therapy—only allowing medicines to be covered by insurance for patients who have successfully overcome those hurdles and for whom the PBM has determined need the medicine. Additionally, PBMs often use closed formularies that exclude certain medicines. Coverage for those medicines are only available to patients who have successfully gone through an exceptions process. PBM's ability to steer patients to the lowest cost medicine is a key reason why generics account for 90 percent of prescriptions that are dispensed.³⁸⁰ PBM's ability to use utilization management as part of efforts to drive high use of generics suggests that cost-sharing assistance does not subvert benefit design.

Up until recently, patients reached their deductible and out-of-pocket maximum at the same time, regardless of how they paid the cost sharing required for their medicines. But new programs from some PBMs and health insurers ignore cost-sharing assistance cards when calculating whether patients have reached their deductible or out-of-pocket maximum. In some cases, these programs lead to patients exhausting their cost-sharing assistance, potentially leaving them with unexpected out-of-pocket costs as high as several thousand dollars in order to continue taking their medicine. As discussed above, high out-of-pocket costs make patients more likely to abandon their medicines and become nonadherent, leading to increased health care costs for health plans and employers. These new programs threaten access for patients and could negatively impact patient health. These programs also single out cost-sharing assistance cards.

³⁷⁶ Goldman DP, Joyce GF, Escarce JJ, et al. Pharmacy benefits and the use of drugs by the chronically ill. *JAMA*. 2004;291(19):2344-2350.

³⁷⁷ K. Devane, et al., Patient Affordability Part Three: The Implications of Co-Pay Cards. May 2018. Available at: <https://www.iqvia.com/locations/united-states/patient-affordability-part-three>

³⁷⁸ Id.

³⁷⁹ IQVIA. An Evaluation of Copay Card Utilization in Brands after Generic Competitor Launch. February 2018. Available at: <https://www.iqvia.com/-/media/iqvia/pdfs/us-location-site/market-access/fact-sheet-evaluation-of-copay-card-utilization-post-loe.pdf>

³⁸⁰ IQVIA. 2017 Medicine Use and Spending. April 2018.

In contrast, patients with commercial insurance benefit from hospitals and doctors forgiving bad debt that patients owe towards the cost sharing in their medical benefit. Even when this cost sharing is not collected by the provider, it still counts towards patients' deductible and out-of-pocket maximum.

COST-SHARING ASSISTANCE CARDS: Price Reporting (RFI p. 22698)

The RFI asks about the impact of ending the current policy of excluding manufacturer-sponsored drug discount programs from the determination of AMP and Best Price.³⁸¹ PhRMA strongly recommends that HHS not change the current policy. As discussed below, eliminating the exclusion for manufacturer sponsored drug discount programs would not be consistent with the statute, would be operationally difficult, would likely raise prices in most cases for Medicaid, and could harm patients.

Best Price

Statutory change would be needed to include cost-sharing assistance cards in Best Price determinations. By law, the term “best price” means “the lowest [net-of-discount] price available from the manufacturer during the rebate period to any wholesaler, retailer, health maintenance organization, nonprofit entity, or governmental entity within the United States” subject to specified exclusions.³⁸² Accordingly, Best Price determinations do not take into account manufacturer discounts to patients as patients are not wholesalers, retailers, health maintenance organizations (HMOs), nonprofit entities, or government entities—as CMS recognized in developing the current regulations.³⁸³ Congress drafted the Best Price provision in the Medicaid rebate statute to give Medicaid “the benefit of the same discounts that other large public and private purchasers enjoy” and did not intend discounts to patients to trigger Best Price. We strongly recommend that Congress not change its current policy for the reasons detailed below.

- Requiring manufacturers to offer their best price to Medicaid is a policy grounded in the idea that Medicaid should benefit from negotiated discounts and should benefit from the best deal available to private insurers. Cost-sharing assistance cards are not negotiated with a payer and no payer pays a price net of cost-sharing assistance cards. Instead, insurers pay a price that is net of negotiated rebates and price concessions, which are already factored into Best Price. In contrast, cost-sharing assistance cards are offered to patients to fill in some of the plan-assigned cost sharing so that medicines are more affordable. Manufacturers typically do not directly control how much of a cost-sharing

³⁸¹ RFI p. 22696.

³⁸² 42 U.S.C. § 1396r-8(c)(1)(C)(i)(Social Security Act § 1927(c)(1)(C)(i))(emphasis added).

³⁸³ 77 Fed. Reg. 5318, 5336, 5362 (proposed 42 C.F.R. § 447.505(a)(explaining that CMS is “proposing to revise the term ‘best price’ at newly proposed § 447.505(a) so that it is consistent with the definition of best price found in section 1927(c)(1)(C) of the Act” and then proposing new language whereby best price is “the lowest [net] price available from the manufacturer during the rebate period to any wholesaler, retailer, provider, health maintenance organization, nonprofit entity or governmental entity in the United States” subject to certain exclusions.

assistance card is used for an individual fill of a prescription—that depends on a patients’ level of cost sharing.

- Factoring cost-sharing assistance cards into price reporting metrics would add more complexity to the current system. The amount an individual cost-sharing assistance card pays out is a function of several factors: whether a deductible applies to prescription drugs, the size of that deductible and whether the patient has other medical expenses that apply to that deductible, how many times the patient fills the prescription and whether other medical expenses cause the patient to reach their out-of-pocket maximum.

AMP

For most drugs, AMP is the average price for direct sales to retail community pharmacies and indirect sales to retail community pharmacies through wholesalers.³⁸⁴ Therefore AMP excludes manufacturer sales, discounts, rebates, and other price concessions to other parties—including patients—and a statutory change would be needed to include cost-sharing assistance cards in AMP calculations. It is not clear what HHS would hope to achieve by amending the statute to include patient discounts in AMP calculations. Such a change would lower AMP, which in turn would lower Medicaid rebates.³⁸⁵ This would *increase* net drug costs to State Medicaid programs, which we believe is counter to goals of the Administration’s reform efforts.

SECTION IX: VALUE-BASED ARRANGEMENTS (RFI p. 22696)

Our health care system is evolving to increasingly reward the value of services, rather than solely reimbursing based on the volume of services provided. As these medicines are becoming increasingly personalized, manufacturers and health plans are exploring innovative payment and coverage approaches in the competitive market that can help improve patient access and affordability. As HHS has repeatedly recognized, these approaches can advance its goal of moving from fee-for-service payments toward payment methods that reward quality and value. For example, CMS Administrator Seema Verma stated last year that innovative products “reinforce our belief that the current healthcare payment systems need to be modernized in order to ensure access to new high-cost therapies, including therapies that have the potential to cure the sickest patients.”³⁸⁶ CMS emphasized that “[a]s part of larger efforts to support the President’s priority [of lowering drug costs], CMS is working actively with all stakeholders . . . on innovative

³⁸⁴ 42 U.S.C. § 1396r-8(k)(1)(SSA § 1927(k)(1)). Drugs that are infused, injected, inhaled, instilled, or implanted and are not generally dispensed through a retail community pharmacy have a special AMP formula that includes many sales and price concessions that are not included in standard AMP (so that an AMP for these “5i” drugs can be calculated), but discounts to patients are not part of the 5i AMP. 42 U.S.C. § 1396r-8(k)(1)(A)(i)(IV)(SSA § 1927(k)(1)(A)(i)(IV)).

³⁸⁵ The rebate for a brand drug equals a basic rebate plus an additional rebate. The total rebate will decline if AMP declines, since this will cause the basic rebate to go down and the additional rebate to go down. The “basic rebate” is the greater of: (1) 23.1 percent of AMP or (2) AMP minus best price. Both of these amounts will decline with a decline in AMP. The “additional rebate” equals [the current-quarter AMP minus the inflation-adjusted AMP from the base period (usually the first full quarter after the drug’s launch)]; this will also decline with a decline in AMP, holding the base period AMP constant.

³⁸⁶ CMS. Innovative Treatments Call for Innovative Payment Models and Arrangements. (emphasis added).

payment arrangements” including “outcome-based pricing for medicines in relation to clinical outcomes.”³⁸⁷ In its 2016 final rule on Covered Outpatient Drugs, CMS stated that “[w]e recognize the value of such [value-based payment] arrangements, especially when they benefit patients,” and “since these arrangements are unique, we are considering how to provide more specific guidance.”³⁸⁸ Later that year, CMS announced that in subsequent guidance it would seek to generalize lessons learned from common questions and arrangements.³⁸⁹

PhRMA greatly appreciates that just last month, FDA issued final industry guidance on manufacturer communications with payers and communications consistent with the label. These guidances are a substantial and positive step forward for manufacturers’ ability to communicate about the value of their products.³⁹⁰ FDA’s final payer guidance includes recommendations designed to enable truthful, non-misleading, and appropriate manufacturer communications with payers across a product’s lifecycle, which will facilitate communications that can allow payers to provide coverage for new products and indications more quickly. In issuing this guidance, FDA recognized the important role that value-based arrangements can play in advancing patient care:

The goal is to advance public health benefits such as increased cost savings from informed and appropriate coverage and reimbursement decisions. In this way, we can help ensure patients have more timely access to cutting-edge medical technologies. We can facilitate access by helping to reduce the overall cost of providing these benefits to patients. And in promoting access, we will advance important public health goals.³⁹¹

FDA has made an important step towards addressing one key barrier to value-based arrangements. We urge HHS to continue this momentum, by modernizing the safe harbors to protect value based arrangements and addressing challenges to value-based arrangements associated with Medicaid rebate reporting. These agreements can offer important clinical gains and overall cost savings to payers, providers, and patients throughout the health care system—including Medicaid, Medicare, and their beneficiaries.

As the Administration considers value-based payment and coverage approaches, it is critical that the market determine value rather than the government or other centralized organizations. The competitive market is uniquely well-designed to make complex determinations about the value of medicines as the many heterogeneous payers assess their own needs in light of available evidence. In contrast, policies that would impose a centralized government determination of value would reduce and delay appropriate patient access and lead to suboptimal

³⁸⁷ CMS. Innovative Treatments Call for Innovative Payment Models and Arrangements. supra.

³⁸⁸ 81 Fed. Reg. 5170, 5253 (Feb. 1, 2016).

³⁸⁹ CMS Medicaid Rebate Release No. 99 to Manufacturers, July 14, 2016.

³⁹⁰ FDA. Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities. June 2018; FDA. Medical Product Communications That Are Consistent With the FDA-Required Labeling. June 2018.

³⁹¹ FDA. Drug and Device Manufacturer Communications with Payors, Formulary Committees, and Similar Entities—Questions and Answers. June 17, 2018.

outcomes. Experience in several European countries has shown the dangers of the government attempting to make centralized, one-size-fits-all judgments of value. Restrictions imposed by the U.K.'s NICE have created substantial barriers between patients and life-saving treatments—recent analysis shows that from 2013 to 2017, nearly 92 percent of oncology treatments were given some kind of access restriction.³⁹² Patients who live in countries that impose centralized value judgements also have access to fewer treatment options—recent data shows that nearly 90 percent of newly launched medicines were available in the U.S., compared to just two-thirds in the U.K., half in Canada and France, and one-third in Australia.³⁹³ Ensuring reforms are market based is essential to preserving patient access to a range of treatment options that they identify as high value.

Below we offer suggestions for HHS as it works to drive competition by addressing barriers to value-based arrangements (such as price reporting rules), and potential approaches to indication-based pricing and long-term financing arrangements to consider.

VALUE-BASED ARRANGEMENTS: Value-Based Arrangements and Price Reporting (RFI p. 22696)

PhRMA appreciates HHS' Interest in Value-Based Arrangements and related price reporting changes. As part of the broader shift to value in health care, private payers increasingly are pursuing results- or value-based contracts with biopharmaceutical companies. An expansion of these innovative arrangements would offer an effective, market-based approach to managing drug costs and spurring value-based care, while delivering savings for patients, private payers and the government. Currently, outdated regulations developed for a fee-for-service world are limiting the number, scale, and types of these arrangements.

PhRMA appreciates HHS' continued commitment to facilitating value-based arrangements. We believe that some additional HHS guidance, and a few regulatory changes, could help reduce challenges to value-based arrangements and permit broader adoption. Below we provide a brief overview of value-based arrangements—what they involve, why they matter, and the benefits they offer to federal health programs and their beneficiaries—and then discuss the specific topics on which the RFI seeks input.

Description and scale of value-based arrangements

PhRMA considers value-based arrangements for biopharmaceuticals to be voluntary arrangements between manufacturers and other private entities, such as health plans or risk-bearing providers, in which the price or price concession for a prescription medicine is linked to

³⁹² Hughes K and N Jeswani. HTAs Recommendations for Oncology Have Grown More Restrictive Over Time. Avalere Health. June 2018. Available at: <http://avalere.com/expertise/life-sciences/insights/htas-recommendations-for-oncology-have-grown-more-restrictive-over-time>

³⁹³ Haninger K. New analysis shows that more medicines worldwide are available to U.S. patients. PhRMA. The Catalyst blog. June 2018. Available at: <https://catalyst.phrma.org/new-analysis-shows-that-more-medicines-worldwide-are-available-to-u.s.-patients>

value as determined by the contracting entities. These arrangements can reduce insurers' cost exposure for treatment failures by allowing the manufacturer to share financial risk with the payer. By aligning payments for medicines more directly with their value in improving health outcomes and/or reducing the need for other health care services (such as hospitalizations), value-based arrangements make drug manufacturers accountable for the results their products achieve in a concrete way and can help improve patients' health and maximize the benefits of health care spending.

We recognize that our members can also enter into value-based arrangements with state Medicaid programs, thereby lowering budgetary costs for both the federal government and the state. While the majority of value-based arrangements are between private entities, the government can play an effective role in addressing barriers to innovative market-based arrangements.

Many types of value-based arrangements are occurring between manufacturers and health plans; outcomes-based contracts, which vary costs or discounts based on patient outcomes, are one example. Earlier this year, PhRMA released an issue brief which provides a taxonomy with some of the many possible types of value-based arrangements.³⁹⁴ The brief describes performance-based contracts such as outcomes-based contracts and conditional treatment continuation arrangements. It also describes indication-based pricing and regimen-based pricing (discussed in the next section) as well as expenditure caps, which are both types of variable pricing arrangements.

As evidence of the increasing proliferation of these contracts, a 2017 Avalere survey of 45 payers representing 183 million covered lives, found that more than half of payers surveyed either had an outcomes-based contract in place or were in negotiations.³⁹⁵ A survey by PwC found that one quarter of pharmaceutical company executives say their company has participated in a value-based arrangement. Of those who have participated, nearly one-third (32 percent) have engaged in more than 20 of these arrangements.³⁹⁶ The number of value-based contracts has been increasing. Only 7 private sector risk-sharing contracts were publicly announced from the late 1990s to 2013, but 16 were announced from 2015 through early 2017.³⁹⁷ PhRMA identified 39 publicly announced value-based contracts by 19 pharmaceutical companies for 25 medicines from 2009 through Q1 2018.³⁹⁸ Recent data from the Academy of Managed Care Pharmacy and PwC's survey confirm that only a portion of value-based arrangements are publicly announced.³⁹⁹

³⁹⁴ Id.

³⁹⁵ Avalere Health. Payer Perspectives on Outcomes-Based Contracting: Avalere 360. May 22, 2017.

³⁹⁶ PwC. Launching Into Value: Pharma's Quest to Align Drug Prices with Outcomes. September 2017. Available at: <https://www.pwc.com/us/en/health-industries/health-research-institute/publications/value-based-drug-pricing.html>.

³⁹⁷ PhRMA. Barriers to Value-Based Contracts for Innovative Medicines: PhRMA Member Survey Results. March 2017.

³⁹⁸ PhRMA. Value-Based Contracts 2009-2018 Q1. April 2018. Available at: <https://www.phrma.org/fact-sheet/value-based-contracts-2009-q1-2018>.

³⁹⁹ Duhig AM, S Saha, S Smith et al. The Current Status of Outcomes-Based Contracting for Manufacturers and Payers: An AMCP Membership Survey. *J Manag Care Spec Pharm*. 2018;24(5):410-415.

Looking forward, IQVIA estimates that there will be 65 outcomes-based contracts from 2018-2022.⁴⁰⁰

While the number of value-based arrangements in the competitive market continue to increase, manufacturers continue to face multiple obstacles to creation of these arrangements. Addressing these challenges would allow more, a greater variety of, and larger scale arrangements to occur.

Benefits of value-based arrangements

As recognized in a recent report by the Duke Margolis Center for Health Policy, “[m]any stakeholders view [value-based agreements] as potentially driving more efficient healthcare delivery, with reductions in overall costs while improving patient outcomes.”⁴⁰¹ Importantly, these arrangements also can increase patient access to new therapies, including breakthrough medications for rare and devastating diseases, which could ultimately improve patient outcomes. These products have the potential to transform patients’ lives by treating segments of the population in desperate need of medical advances—often people with progressively debilitating diseases who have lacked any effective treatment options. For instance, currently over 1,500 potential gene therapy treatments are in research and development by dozens of pharmaceutical companies, including nearly 600 targeting cancers and 500 for rare and debilitating or deadly conditions.⁴⁰² A payer that might otherwise decline to cover a new drug (or that would only cover the drug with significant utilization management restrictions or high cost sharing) due to uncertainties about the expected percentage of its patient population who would benefit from the drug might increase access to the drug if the manufacturer shared the risks of the drug’s performance. By reducing a payer’s risks (e.g., agreeing to pay a large rebate on units of the drug used by enrollees who do not respond to the drug or achieve a specified outcome, so that the payer cannot end up paying a high net price for low performing products), these agreements may make newer drugs more accessible to patients who will benefit from them and increase competition in relevant drug classes.⁴⁰³

There is evidence that payers and PBMs are experimenting with value-based arrangements to drive cost savings. CVS Health described several types of value-based arrangements as tools they used to keep specialty drug cost growth to 3.7 percent in 2017.⁴⁰⁴ Avalere found that one-third of payers engaged in these contracts experienced cost savings.⁴⁰⁵ Express Scripts also

⁴⁰⁰ IQVIA Institute. 2018 and Beyond: Outlook and Turning Points. March 2018.

⁴⁰¹ Duke Margolis Center for Health Policy. Overcoming the Legal and Regulatory Hurdles to Value-Based Payment Arrangements for Medical Products. December 2017. Available at: <https://healthpolicy.duke.edu/publications/overcoming-legal-and-regulatory-hurdles-value-based-payment-medical-products>.

⁴⁰² Steven Miller. Gene Therapy Holds Great Promise, But Big Price. September 21, 2017. Available at: <http://lab.express-scripts.com/lab/insights/drug-options/gene-therapy-holds-great-promise-but-big-price>.

⁴⁰³ See, e.g., Lee Staley. A Drug’s Worth: Why Federal Law Makes it Hard to Pay for Pharmaceutical Performance. 98 Boston Univ. Law Review 303, at 310. 2018 (“Tying reimbursement to health outcomes presents new opportunities for competition with rival manufacturers. . . .A manufacturer that can demonstrate sustained health benefits in post-market studies may distinguish itself from competitors).

⁴⁰⁴ CVSHealth. Drug Trend Report 2017.

⁴⁰⁵ Avalere Health. Payer Perspectives on Outcomes-Based Contracting: Avalere 360. May 22, 2017.

engages in several types of value-based arrangements and their Chief Medical Officer, Steve Miller, has recognized the benefit of risk-sharing through these arrangements stating, “[v]alue-based contracting can help to ensure that payors and patients are not on the hook when a treatment isn’t effective.”⁴⁰⁶

The short-term benefits of value-based arrangements fall into three categories:

- **Value-based arrangements can lower patients’ out-of-pocket costs.** From 2015 to 2017, patient copays were 28 percent lower than the market average for certain plans that announced a value-based arrangement. Although data was not detailed enough to directly link lower cost sharing to the value-based arrangement, the results provide a clear indication that such contracts may have led to lower patient cost sharing.⁴⁰⁷ Researchers have also found that value-based arrangements can improve patient access to medicines.⁴⁰⁸
- **Value-based arrangements can improve patient outcomes.** Because these arrangements allow manufacturers to reduce payers’ risk for suboptimal outcomes or offer new types of discounts that may not be available today, payers may be able to provide broader access to innovative medicines. These arrangements may also allow payers or manufacturers to provide more support for appropriate use of medicines by patients. All of these changes are improving patient outcomes—Avalere’s payer survey found that 38 percent of payers engaged in outcomes-based contracts experienced improvements in patient outcomes.⁴⁰⁹
- **Value-based arrangements can reduce costs for the health care system.** For example, if new value-based arrangements can improve the use of medicines for diabetes and help reduce the burden of this disease in the U.S. by only five percent, this could save \$9 billion annually in direct medical costs, and improve productivity by an additional \$3.4 billion. This would save the country more than \$12 billion annually.⁴¹⁰

In the longer term, if the number and scope of value-based agreements increase, they will likely generate more information on the effects of different products and treatment regimens on different patient populations and subpopulations.⁴¹¹ Real world evidence on how different

⁴⁰⁶ Steven Miller. Gene Therapy Holds Great Promise, But Big Price. September 21, 2017. Available at: <http://lab.express-scripts.com/lab/insights/drug-options/gene-therapy-holds-great-promise-but-big-price>.

⁴⁰⁷ PhRMA. Delivering Results for Patients: The Value of Value-Based Contracts. February 2018.

⁴⁰⁸ See, e.g., description of Entresto and Repatha contracts in: Seely E and Kesselheim A. Outcomes-Based Pharmaceutical Contracts: An Answer to High U.S. Drug Spending?” Commonwealth Fund. September 2017.

⁴⁰⁹ Avalere Health. Payer Perspectives on Outcomes-Based Contracting: Avalere 360. May 22, 2017.

⁴¹⁰ PhRMA. Delivering Results for Patients: The Value of Value-Based Contracts. February 2018.

⁴¹¹ For example, one study conducted in Sweden concluded that “stakeholders benefited from analysis of real-world (postmarket) data (in addition to pre-launch, trial-based data)” collected under a value-based pricing agreement. See Deloitte. Value-based Pricing for Pharmaceuticals: Implications of the Shift from Volume to Value. 2012. Available at: <http://deloitte.wsj.com/cfo/files/2012/09/ValueBasedPricingPharma.pdf>.

treatments affect patients with a certain disease (or subgroups of patients with a certain disease) will be available both to providers and patients making individualized, patient-centered treatment decisions, and to payers developing formularies and coverage policies. Over time, this should shift drug utilization toward drugs with greater clinical value and greater ability to reduce hospitalizations and other costly services, resulting in better health outcomes and lower overall health care spending.

Addressing barriers to value-based arrangements could lead to government savings

An expansion of value-based arrangements in MA or Medicare Part D could benefit the government through existing mechanisms. Removing barriers to these arrangements would facilitate broader participation in value-based arrangements by MA and Medicare Part D plans. In addition, under Part D's competitive, market-based structure, innovator companies contract directly with Part D plans, and MA (or MA-PD) plans. Some of these contracts may already reflect value-based arrangements and there continues to be growing interest in pursuing these types of arrangements.⁴¹² To the extent that value-based arrangements improve use of medicines, they could reduce MA plan spending, which could reduce MA plan bids.

Improved use of Part D medicines could reduce spending on medical services under Medicare Parts A and B. In addition, if value-based arrangements reduce plans' risk, they could permit lower plan bids.

Addressing barriers to value-based arrangements could also allow for an expansion of innovative arrangements in Medicaid, thereby reducing Medicaid costs. Manufacturers are negotiating value-based arrangements directly with at least one state through supplemental rebate agreements.⁴¹³ In addition, to the extent that manufacturers enter into value-based arrangements with Medicaid Managed Care plans, that could also reduce plan costs and the premiums that these plans charge to states.

Permanent regulatory reforms are needed to address barriers that inhibit value-based arrangements

PhRMA released a member survey last year which highlighted the regulations that our members believe need to be modernized to allow an expansion of value-based arrangements.⁴¹⁴ Price reporting metrics, the federal Anti-Kickback Statute, and FDA rules for manufacturer communications were all prioritized by our members. A 2016 survey of payers also identified the same barriers.⁴¹⁵ In addition, over the past year, the Academy of Managed Care Pharmacy, the

⁴¹² PhRMA discussions with Milliman.

⁴¹³ Gleason B. Value-Based Purchasing for Prescription Drugs Takes a Leap Forward in State Medicaid. Pharmaceutical Executive. June 29, 2018.

⁴¹⁴ PhRMA. Barriers to Value-Based Contracts for Innovative Medicines: PhRMA Member Survey Results. March 2017.

⁴¹⁵ Ward A, et al. Regulatory, Legal Uncertainties Are Barriers to Value-Based Agreements. *Health Affairs Blog*. November 4, 2016. Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20161104.057443/full/>.

Network in Excellence in Health Innovation, and the Duke-Margolis Center have all released papers recommending addressing these same barriers to value-based arrangements.⁴¹⁶ We greatly appreciate the recent action by FDA to issue final guidance on manufacturer communications with payers and communications consistent with the label. These guidances are a positive and substantial step forward for manufacturer communications for value-based arrangements.⁴¹⁷ Our suggested changes related to the other barriers are below.

- **Value-based arrangements should be clearly protected under the Anti-Kickback Statute.** Despite the potential benefits of these arrangements, the Federal Anti-Kickback Statute is chilling more widespread adoption. The Anti-Kickback Statute is a broadly worded statute that can inadvertently discourage beneficial low-risk health care arrangements through the threat of civil, criminal, and/or administrative sanctions.⁴¹⁸ To reduce the risk that the broadly worded Anti-Kickback Statute would deter beneficial arrangements, Congress authorized the development of regulatory safe harbors and requires annual solicitation of comments for updating such safe harbors.⁴¹⁹ It is important that the Anti-Kickback Statute safe harbors evolve to support new arrangements that, if properly structured, could help improve health outcomes, promote competition, and contain overall health care spending without raising risk of fraud and abuse. To date, OIG’s annual solicitations have elicited at least six proposals to develop a safe harbor for value-based arrangements. In addition, over the past year, the Healthcare Leadership Council released a paper recommending modernization of the Anti-Kickback Statute in the area of value-based care.⁴²⁰

⁴¹⁶ Academy of Managed Care Pharmacy. AMCP Partnership Forum: Advancing Value-Based Contracting. *Journal of Managed Care & Specialty Pharmacy*. November 17, 2017.; Network for Excellence in Health Innovation. Rewarding Results: Moving Forward on Value-Based Contracting for Biopharmaceuticals. March 2017. Available at: <https://www.nehi.net/publications/76-rewarding-results-moving-forward-on-value-based-contracting-for-biopharmaceuticals/view>; Duke Margolis Center for Health Policy. Overcoming the Legal and Regulatory Hurdles to Value-Based Payment Arrangements for Medical Products. Available at: <https://healthpolicy.duke.edu/publications/overcoming-legal-and-regulatory-hurdles-value-based-payment-medical-products>.

⁴¹⁷ FDA. Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities. June 2018; FDA. Medical Product Communications That Are Consistent With the FDA-Required Labeling. June 2018.

⁴¹⁸ Today, the risk of discouraging beneficial arrangements is even greater than in the past. As you know, the ACA added language to the Anti-Kickback Statute stating that “a claim that includes items or services resulting from a violation of this section constitutes a false or fraudulent claim for purposes of [the civil False Claims Act].” 42 U.S.C. § 1320a-7b(g).

⁴¹⁹ 42 U.S.C. § 1320a-7d (requiring an annual solicitation seeking proposals from the public for new or modified safe harbors and Special Fraud Alerts). Even before the 1996 law requiring the annual solicitation for safe harbor proposals, OIG acknowledged the Congressional expectation that it should “formally re-evaluate the anti-kickback regulations on a periodic basis, and . . . solicit public comment at the outset of the review process.” Medicare and State Healthcare Programs: Fraud and Abuse; OIG Anti-Kickback Provisions, 56 Fed. Reg. 35952 (July 29, 1991) (quoting H.R. Rep. No. 85, part 2, 100th Cong. 1st Sess. 27 (1987)).

⁴²⁰ Healthcare Leadership Council. Health System Transformation: Revisiting the Federal Anti-Kickback Statute and Physician Self-Referral (Stark) Law to Foster Integrated Care Delivery and Payment Models. February 2017. Available at: https://www.hlc.org/app/uploads/2017/02/HLC_StarkAntiKickback-White-Paper.pdf

The key safe harbors to the Anti-Kickback Statute that are applicable to manufacturers were developed over twenty years ago, and did not anticipate innovative, value-based approaches. We continue to seek creation of a new safe harbor to clearly protect value-based arrangements under the Anti-Kickback Statute and submitted this recommendation to HHS OIG in both 2017 and earlier this year.⁴²¹

- **Value-based contracts necessitate a more modern and flexible approach to price reporting.** Biopharmaceutical companies must adhere to a complex set of government price-reporting rules for calculating ASP in Medicare Part B and Best Price in Medicaid. These highly technical price-reporting rules were established prior to the introduction of innovative payment approaches. While the price-reporting rules do permit biopharmaceutical companies to make reasonable assumptions, to the extent there is ambiguity about how to capture innovative pricing methods in an ASP or Best Price framework this can create uncertainty for manufacturers and payers.

Recommendations for specific price reporting clarifications

Under the Medicaid rebate statute, a drug’s “Best Price” is generally the manufacturer’s single lowest net price during a quarter “to any wholesaler, retailer, provider, health maintenance organization, nonprofit entity, or governmental entity” (Best Price-eligible customers), subject to certain limited exemptions.⁴²² Just one sale during the quarter can therefore set the Best Price. The Medicaid rebates manufacturers must pay on brand drugs include a basic rebate (either 23.1 percent of AMP or AMP minus Best Price, whichever is higher) and an additional rebate (AMP minus the inflation-adjusted AMP from the drug’s base date, usually the first full quarter after launch). Given this rebate formula, a state Medicaid program’s net payment for a brand drug (the state’s payment to the pharmacy or other provider that dispenses or administers the drug, minus the Medicaid rebate it receives from the manufacturer) should be at least as low as—and usually much lower than—the manufacturer’s single lowest net price to any Best Price-eligible customer in any non-exempt sale.

In enacting the Medicaid rebate statute in 1990, Congress intended to put Medicaid in the same position as other large-volume payers:

[Under the Medicaid rebate bill] manufacturers would be limited to charging Medicaid the best price given any bulk purchaser . . . with savings returned to Medicaid through a quarterly rebate . . . [T]he Subcommittee on health and the environment heard testimony that Medicaid pays substantially more for many single-source drugs than do other large purchasers The Committee believes Medicaid, the means-tested entitlement

⁴²¹ PhRMA comments to OIG-125-N. Solicitation of New Safe Harbors and Special Fraud Alerts. February 2017, and to OIG-127-N. Solicitation of New Safe Harbors and Special Fraud Alerts. February 2018.

⁴²² Social Security Act (SSA) § 1927(c)(1)(C).

program that purchases basic health care for the poor, should have the benefit of the same discounts on single-source drugs that other large public and private purchasers enjoy.⁴²³

However, in 1990 Congress did not envision the type of value-based arrangements that are emerging today. Questions have come up about whether the type of pricing arrangements often associated with value-based contracts could sharply reduce Best Price and thus sharply increase the manufacturer's rebate liabilities, thereby serving as a disincentive to value-based contracts. For example, under a value-based agreement where the manufacturer pays a 90 percent rebate on a unit of drug used by a patient who does not respond to the drug, just one non-responding patient to the drug could set Best Price at 10 percent of the drug's usual price.

This unanticipated dynamic can limit the size of performance-based rebate that a manufacturer can offer to a PBM or health plan because of the risk that a poor outcome with a single patient will reset the Best Price, increasing the rebate owed for all Medicaid patients using the medicine. A very low Best Price can also lower a drug's 340B ceiling price (since the ceiling price formula is AMP minus the Medicaid rebate) further increasing the potential cost to companies. Because performance-based contracts can lead to a range of outcomes, and because the risk of a bad outcome is greater with a small population, the challenge associated with Medicaid Best Price is more of a barrier to arrangements with smaller payers or for low volume medicines, such as orphan medicines.

CMS clarification of certain Best Price issues is therefore important to reduce the risk that Best Price rules, which are intended to put Medicaid on an equal footing with other high-volume customers, could unintentionally discourage innovative value-based agreements. While manufacturers can already make reasonable assumptions when ambiguity exists about how to apply AMP or Best Price rules to particular arrangements, clearer guidance that reduces obstacles to value-based agreements could improve patient care and also curb spending without departing from the statute's Best Price provisions—presenting a rare opportunity that we hope HHS will seize.

While other challenges to value-based agreements—particularly lack of clear protection under the Anti-Kickback Statute—would still exist, steps such as issuing clearer guidance or making regulatory changes to price reporting terms to address Best Price uncertainties could help to expand the adoption of value-based agreements that offer the potential for significant health gains and overall health care cost savings.

⁴²³ H.R. Rep. 101-881, 1990 U.S.C.C.A.N. 2017, 2108 (Oct. 16, 1990).

As HHS considers how to address the barrier that Medicaid Best Price poses for value-based arrangements, we recommend it consider the following principles:

- To allow for more innovative approaches and risk sharing, a single poor outcome should not set a new price for Medicaid. This would allow manufacturers to share more risk with commercial health plans.
- Over time Medicaid should be able to derive benefits from value-based contracts.
- Approaches to reporting value-based arrangements should be as simple as possible. This would help avoid creating operational challenges for companies that may prevent development of innovative approaches.
- Manufacturers should continue to have flexibility to make reasonable assumptions in their price reporting, so reporting approaches can evolve to reflect changes in the dynamic market and contracting environment.

Turning to concrete approaches for reducing the risk that the Medicaid rebate statute's Best Price provisions would be construed in a way that needlessly deters value-based arrangements, below we describe three different ideas to reduce Best Price challenges to value-based contracting. These ideas could be implemented separately, or together, to provide manufacturers with greater clarity. The first approach relies on an "averaging" concept already reflected in CMS' regulations under the "bundled sales" definition at 42 CFR § 447.502; whereas the other two approaches (sections 2 and 3) provide two possible legal interpretations of the price reporting terms "unit" and "best price" that CMS could incorporate into its Medicaid rebate regulations, through notice and comment rulemaking, to help ensure that the Best Price regulations provide the same degree of flexibility as the statute itself, and thus do not discourage important value based arrangements unnecessarily.

Finally, in section 4, we respond to the RFI's question about the appropriate cutoff point for restating AMP and Best Price values from previous quarters.

1. Application of the "Bundled Sales" Definition to Value-Based Agreements

CMS could help facilitate value-based arrangements by issuing guidance and confirming the reasonableness of applying the "bundled sale" definition in 42 CFR § 447.502 to value-based agreements. Under this regulation:

Bundled sale means any arrangement... under which the rebate, discount, or other price concession is conditioned upon the purchase of the same drug [at the NDC-9 level], drugs of different types ... or another product or some other performance requirement (for example, the achievement of market share, inclusion or tier placement on a formulary), or where the resulting discounts or other price concessions are greater than those which

would have been available had the bundled drugs been purchased ... outside the bundled arrangement.

(1) The discounts in a bundled sale ... are allocated proportionally to the total dollar value of the units of all drugs or products sold under the bundled arrangement.

(2) For bundled sales where multiple drugs are discounted, the aggregate value of all the discounts in the bundled arrangement must be proportionally allocated across all the drugs or products in the bundle. (Emphasis added.)

The regulation thus defines “bundled sale” in a broad manner that includes agreements involving only one drug (NDC-9). The regulatory definition could thus encompass an agreement in which the manufacturer agrees to pay a high rebate on a drug if certain outcomes occur (e.g., the patient does not achieve the same improvement in a certain metric achieved in the drug’s clinical trials) conditioned upon the payer’s acceptance of a lower rebate on the drug if better outcomes occur. The regulation further requires that the price concessions in a bundled sale must be “unbundled” by allocating them proportionally across all of the units of product covered by the agreement—which in this example would result in the average rebate ultimately paid on the drug under the agreement being allocated to every unit covered under the agreement; the net price of each unit would thus reflect the outcomes patients achieved on average. This discount reallocation process required by the bundled sale definition would thus keep an isolated poor outcome under a value-based agreement from resulting in one unit having a very low unit price that could set the Best Price for the whole quarter.

CMS guidance should specifically recognize the reasonableness of the bundled sales approach by explicitly assuring manufacturers that higher and lower prices under such contracts must be averaged (via the proportional discount reallocation required by 42 CFR § 447.502) in calculating the net unit prices under the bundled sale that would go into AMP and Best Price determinations. This is a straightforward application of the existing regulation that may reduce the impact of an “outlier” result under certain value-based arrangements setting a new Best Price for the quarter, by explicitly assuring.

Specifically, CMS should issue clarifying sub-regulatory guidance describing a bundled sales example in which the manufacturer agrees to pay a higher rebate on a certain drug when patient outcomes fail to meet a specified benchmark, conditioned on the payer accepting lower rebates when patient outcomes do meet the benchmark. For example, the guidance could describe a value-based agreement between a manufacturer and a payer in which the manufacturer agrees to pay higher rebates (by way of example, 40 percent off list price) on a certain drug when patient outcomes do not meet a specified benchmark, conditioned on the payer accepting a lower rebate (10 percent off list price) when patient outcomes do meet the benchmark specified in the agreement. CMS could explain in such guidance that it would be reasonable to treat this agreement as a bundled sale under 42 CFR § 447.502, and thus to allocate the rebates proportionally to the total dollar value of all units of the drug covered by this agreement. If the rebates were allocated in accordance with 42 CFR § 447.502, and ultimately 50 percent of the

units covered by the agreement resulted in patient outcomes that meet the specified benchmark, then each unit would have a rebate of 25 percent off list price. Thus, if the product had a list price of \$100 and the manufacturer used list prices in estimating the net price of a payer, the net price to this payer of each unit covered by the bundled value-based agreement would be \$75 (which would be used in determining the manufacturer's Best Price for the relevant quarters).

By laying out this type of example in a manufacturer release, CMS could swiftly alert manufacturers that it was reasonable to categorize such an agreement as a bundled sale and thus to allocate rebates and discounts proportionally across all of the units covered by the agreement, thereby "smoothing out" the unit prices that are taken into account in determining AMP and Best Price and reducing the risk that a single poor outcome could set a new Best Price for the drug for the quarter. This smoothing procedure would not always reduce the risk of a poor outcome on one or a small number of units triggering a new and drastically low Best Price; in particular, if the agreement involved a low level of utilization (because, for example, the product treated a very rare disease, or the manufacturer was contracting with a health plan with low enrollment), then the risk of isolated poor outcomes driving Best Price could not be dismissed, as there would be few units to average. But in many or most cases, this approach could help to reduce Best Price risks and CMS guidance to this effect could therefore reduce manufacturer concerns and encourage broader adoption of value-based agreements. The CMS guidance could also advise manufacturers that this approach was not necessarily limited to outcomes-based agreements; another example of a value-based agreement to which the bundled sale definition in 42 CFR § 447.502 could reasonably be interpreted as applying would be an agreement where a manufacturer agreed to sell a product with multiple indications at a low price in circumstances where it was used for a lower-value indication, provided the customer agreed to a higher price when the product was used for a high-value indication.

2. Definition of "Unit"

CMS could also facilitate value-based agreements by amending the definition of "best price" at 42 CFR § 447.505, through notice and comment rule-making, to distinguish between drugs that the manufacturer prices on a per-unit basis (in particular, "traditional" types of arrangements such as fixed per-unit rebates or volume or market share-based per unit rebates) and those it does not price on a per-unit basis. Currently, a value-based agreement where the drug is not paid per unit is treated for Best Price purposes as if the drug is paid per unit, resulting in a distorted "Best Price" figure that experts have pointed out, does not accurately reflect the agreement's pricing arrangement.

For example, a report by the Duke Margolis Center for Health Policy provides a useful example of the problems with taking a value-based agreement in which the drug is not priced on a per-unit basis and forcing it into a per-unit model.⁴²⁴ A manufacturer may agree to an alternative payment

⁴²⁴ Duke Margolis Center for Health Policy. Overcoming the Legal and Regulatory Hurdles to Value-Based Payment Arrangements for Medical Products. December 2017. Available at: <https://healthpolicy.duke.edu/publications/overcoming-legal-and-regulatory-hurdles-value-based-payment-medical-products>

model where a drug is paid a fixed per-member per-month (PMPM) or per-patient per-month (PPPM) amount (also called capitated or subscription models), regardless of the number of units actually used. Under the current system, such an arrangement is discouraged because manufacturers are required to reduce a PMPM/PPPM arrangement to a “per-unit” basis for Best Price reporting purposes. In other words, a manufacturer could agree to supply however many doses were needed each month by the enrollees in a certain health plan at a fixed per-patient monthly rate of \$100; while it may turn out that the plan enrollees use 100-unit doses of the drug in month one and 200 in month two, that does not mean the manufacturer has agreed to supply the drug at a unit price of \$1.00, 50 cents, or any other figure. Yet the current regulations require that the manufacturer calculate a unit price after the fact and use that price in determining Best Price. This may produce a new Best Price—as the manufacturer cannot control the monthly utilization, which could go up or down each month for any number of reasons, thus generating volatile “unit prices”—and thereby discourage manufacturers from pursuing innovative arrangements that could provide customers with needed flexibility in managing drug costs.

Similarly, a manufacturer could enter into a “cost-to-cure” arrangement with a payer or health care provider, in which the manufacturer agreed to supply at a fixed price the doses of a certain drug needed to cure a patient of the disease the drug—however many doses were needed, over whatever period of time, to cure each patient covered by the agreement. Such an arrangement further highlights how innovative new therapies designed to cure disease and conditions, have “outgrown” dated pricing metrics such as a “per unit” basis, and regulatory and sub-regulatory price reporting rules that interpret the statute and can be changed by CMS without waiting for Congress—need to be reexamined and modernized. Here again, a manufacturer may now be required to calculate an after-the-fact “unit price” for the drug—even though the manufacturer was not selling units of the drug, but an outcome (a cure). Thus, to use the fictitious “unit price” in Best Price determinations, would turn a type of value-based agreement that could offer important benefits to payers, health care providers, and patients into a Best Price risk and deter adoption of these agreements.

This is not an unfortunate result dictated by the Best Price statute; it stems solely from regulatory language that does not appear in the statute, and could thus be amended through rulemaking.⁴²⁵ CMS could amend 42 CFR § 447.505 to fix this problem and clarify that Best Price is the lowest net price from the manufacturer to a wholesaler, retailer, provider, HMO, nonprofit or governmental entity during the rebate period in a non-exempt sale “for a unit of the drug,” and carve out value-based arrangements from the definition of “unit.” Notably, CMS set a precedent for excluding certain sales from the definition of a “unit” in the ASP context. Under Social Security Act 1847A(b)(2) and (c)(1), ASP is calculated “for a unit,” and CMS may establish units and methods for counting units. In the 2005 interim final rule regarding the CAP, CMS decided

⁴²⁵ Unlike the statute, the regulation states that Best Price is the lowest price available from the manufacturer during the rebate period to any wholesaler, retailer, provider, health maintenance organization, nonprofit entity or governmental entity in the U.S. “in any pricing structure (including capitated payments)” and that Best Price “must be determined on a unit basis” without regard to package size, special packaging, labeling, or identifiers on the dosage form or product or package. 42 CFR § 447.505(a), (d)(2).

to exclude drugs the manufacturer sells to a CAP vendor from the “unit” definition in 42 CFR § 414.802. CMS stated:

We were not convinced that we had the statutory authority to exclude sales of CAP drugs from the calculation of ASP . . . however, we recognized the commenters’ concerns about the effect of including CAP prices in the calculation of ASP and agree that the best outcomes for both [ASP] and [CAP] would be one in which prices under CAP did not affect payment amounts under [ASP]. We have decided to exclude, for the initial 3-year contract period under the CAP, units of CAP drugs [I]t is appropriate to implement the exclusion from the ASP calculation because the exclusion is necessary for implementing the CAP, a program that the Congress has expressly identified as an alternative to the ASP payment methodology.⁴²⁶

CMS has authority to interpret the “best price” definition by issuing a new regulatory definition (through notice and comment rulemaking) and could amend 42 CFR § 447.505 to specifically reference “unit” in the definition and separately define “unit” as follows:

(a) Definitions. For the purpose of this section, the following definitions apply:

Best price means, for a single source drug or innovator multiple source drug of a manufacturer (including the lowest price available to any entity for an authorized generic drug), the lowest price available from the manufacturer during the rebate period for a unit of the drug to any wholesaler, retailer, provider, health maintenance organization, nonprofit entity, or governmental entity in the United States in any unit pricing structure, in the same quarter for which the AMP is computed.

* * * *

Unit means a unit of the drug sold or discounted in a transaction in which the price or price concession is either a fixed per unit amount or percentage, or a per-unit amount that varies by volume, market share, or another factor other than health or quality outcomes associated with use of the drug or cost of caring for patients treated with the drug (such as a cap on cost of treatment or an agreement to share treatment costs).⁴²⁷

This regulatory change could help facilitate value-based agreements by ensuring that for reporting purposes, “Best price” would distinguish between drugs that the manufacturer prices on a per-unit basis in “traditional” types of arrangements from those it does not price on a per-unit basis. This is a straightforward

⁴²⁶ 70 Fed. Reg. 70478, 70479 (Nov. 21, 2005).

⁴²⁷ If these changes were made, it would be unnecessary to revise the language in § 447.505(d)(2) providing that Best Price “must be determined on a unit basis,” as Best Price determinations would only take into account products that were priced and sold per unit by the manufacturer.

interpretation of the statutory definition of “Best price” that the agency could do through notice and comment rulemaking.

3. Definition of “Best Price”

CMS could also amend the Best Price definition in 42 CFR § 447.505 to give effect to the statutory language limiting Best Price to a price available “during the rebate period.”⁴²⁸ To that end, CMS could amend § 447.505 to exclude price adjustments that are based on outcomes measured outside of the rebate period (defined as a calendar quarter). This would be an important and useful clarification because value-based arrangements often use metrics that are most appropriately measured over a period longer than a quarter. As a 2017 paper by the Network for Excellence in Health Innovation points out:

The full value of many pharmaceuticals . . . is often only realized over a longer period than . . . one-year For example, a drug may promise patients and payer the benefit of reduced hospitalizations, but these reductions may only occur in significant numbers as patients use the drug over a period of years. In such cases, a value-based contract may only make sense if it covers this longer time frame and the payer and manufacturer agree to adjust rebates periodically over a multi-year contract.⁴²⁹

CMS could revise 42 CFR § 447.505 to add language defining prices available “during the rebate period,” and exclude from that term price adjustments that are only available later because they are based on clinical or cost outcomes measured in a later period. For example, CMS could amend the regulation as follows:

Best price means, for a single source drug or innovator multiple source drug of a manufacturer (including the lowest price available to any entity for an authorized generic drug), the lowest price available from the manufacturer during the rebate period to any wholesaler, retailer, provider, health maintenance organization, nonprofit entity, or governmental entity in the United States in any pricing structure (including capitated payments), in the same quarter for which the AMP is computed. A price available “during the rebate period” does not include a price adjustment that is only available later based on clinical or cost outcomes measured in a later period.

⁴²⁸ Social Security Act § 1927(c)(1)(C)(i).

⁴²⁹ Network for Excellence in Health Innovation. Rewarding Results: Moving Forward on Value-Based Contracts for Biopharmaceuticals. March 2017. Available at: https://www.nehi.net/writable/publication_files/file/rewarding_results_moving_forward_on_value_based_contracting_for_biopharmaceuticals_copy1.pdf.

This new regulatory language defining “during the rebate period” would have a solid statutory basis, as it would interpret and implement statutory language in SSA §1927(c)(1) (C)(i) defining Best Price as the lowest price available from the manufacturer to Best Price-eligible customers “during the rebate period.”

4. Manufacturer Price Reporting Restatements

The RFI asks whether the period for manufacturers to restate AMP or Best Price values for a past quarter should be lengthened, to accommodate the possibility of extended evaluation timeframes for value-based agreements. Currently manufacturers generally may only restate the AMP and Best Price for a quarter in the 3-year period after the initial filing deadline (30 days after the end of the quarter).⁴³⁰ As noted above, value-based agreements may base price adjustments on outcomes over a period outside the calendar quarter. And if the outcome that determines price adjustments is for example, whether the patient’s disease is in remission one year after treatment, it may take considerably longer to determine whether, for each of the patients treated in a quarter, the disease was in remission one year after treatment. Therefore, it is logical to ask whether value-based agreements may need a longer restatement period than 3 years and we appreciate CMS raising this question. However, on reflection we suggest CMS keep the current three-year restatement window.

In establishing the three-year restatement window in 2003, CMS recognized “the potential burden for States and manufacturers to apply prior period adjustments during a 3-year retroactive timeframe,”⁴³¹ but still adopted the three-year timeframe to balance the need for accuracy of data against the need for finality:

a timeframe for manufacturers to submit revised pricing data to us ...streamlines the administration of the Medicaid drug rebate program. Due to recalculations involving hundreds of millions of State and Federal Medicaid dollars ... we believe it is essential that a standard timeframe be established within which manufacturers ... are permitted to submit revised drug prices. This timeframe will also assist States that would otherwise be required to retain their drug utilization data indefinitely to verify changes in rebate amounts resulting from retroactive manufacturer recalculations.⁴³²

The three-year restatement window still strikes a reasonable balance between the interest in finality and the interest in incremental improvements in data accuracy. With a longer period for restatements, the states would face a higher risk of reductions in their rebate revenue from past periods, which was a major concern to the states when this issue last arose; CMS stated that: “[t]his rule [establishing the three-year limit on restatements] will have a positive effect on the State

⁴³⁰ 42 CFR § 447.510.

⁴³¹ 68 Fed. Reg. 51912, 51914 (Aug. 29, 2003).

⁴³² 68 Fed. Reg. at 51912 (emphasis added).

Medicaid agencies. State Medicaid agencies are having difficulty fully funding their Medicaid programs. They will likely be relieved that we are setting forth a rule that will limit their fiscal vulnerability....⁴³³

CMS should therefore keep the three-year restatement window for value-based agreements and make clear that (notwithstanding any new data), restatements in AMP and Best Price are neither required nor permitted once the window closes. The interest in ensuring that rebates for a certain quarter are final after three years (thus reducing uncertainty for states and manufacturers and allowing them to close the books) outweighs any potential for improved accuracy that may come from extending the three-year deadline for value-based arrangements.

VALUE-BASED ARRANGEMENTS: Indication-Based Pricing and Coverage (RFI p. 22694, 22696)

As HHS recognizes, payers may cover or pay for a drug differently based on its indication. Variable coverage is generally considered to be a form of value-based insurance design (VBID), a concept in which payers provide better coverage for items and services that are higher value compared with those that are lower value.⁴³⁴ Indication-based pricing is an arrangement in which the net price of a medicine varies for different indications based on an agreement between the contracting entities.⁴³⁵ Indication-based pricing is occurring in the commercial market. Express Scripts and CVS Health have both announced that they are engaging in indication-based pricing.⁴³⁶ Regimen-based pricing, which is closely related to indication-based pricing, is an arrangement in which the net price of a medicine decreases when a patient must take a second medicine to make the treatment regimen more effective.⁴³⁷ Some pharmaceutical manufacturers have expressed an interest in regimen-based pricing, but we are not aware of any cases where such an arrangement is in place.⁴³⁸ Below we share principles that we suggest HHS consider as it further explores options related to indication-based coverage, indication-based pricing, and regimen-based pricing.

Indication-based coverage and VBID

PhRMA supports HHS providing health plans more flexibility to pursue VBID, provided that the flexibility brings with it certain requirements to help ensure that VBID can facilitate access to a full range of high-value care. Earlier this year, CMS finalized changes to the MA program,

⁴³³ 68 Fed. Reg. at 51916.

⁴³⁴ Chernew ME, Rosen AB, Fendrick AM. Value-Based Insurance Design. *Health Affairs*. 2007;26(2): w195-w203.

⁴³⁵ PhRMA. Delivering Results for Patients: The Value of Value-Based Contracts. February 2018.

⁴³⁶ Express Scripts. Right Drug, Right Price. March 17, 2016. Available at: <http://lab.express-scripts.com/lab/insights/drug-options/right-drug-right-price>; Brennan T. Aligning Drug Prices with Value: Value-Based Pharmacy Management. July 11, 2017. Available at: <https://payorsolutions.cvshealth.com/insights/aligning-drug-prices-with-value>.

⁴³⁷ PhRMA. Delivering Results for Patients: The Value of Value-Based Contracts. February 2018.

⁴³⁸ See, e.g. Hirschler B. New cocktails to test limits of cancer drug pricing. *Reuters*. August 3, 2015.

which included expanding flexibility under the uniformity requirements.⁴³⁹ We appreciated that these changes gave plans greater latitude for VBID in Medicare Advantage, offering plans the opportunity to better align incentives and help ensure health care financing and delivery are designed to improve access to high-value care. VBID also complements health plans' interest in exploring value-based arrangements, because both VBID and value-based arrangements encourage consideration of how the value of a medicine varies between different patients. We also appreciated that CMS also implemented certain patient protections, including requiring that similarly situated enrollees (e.g., all diabetics) are treated the same, requiring that plans ensure that cost-sharing reductions and targeted supplemental benefits are for health care services that are medically related to each disease condition, and ensuring that MA plans do not provide supplemental benefits for many disease conditions, while excluding other higher-cost conditions. These protections are critical to ensuring that VBID approaches in MA do not discriminate against or discourage enrollment of beneficiaries with certain conditions.

As HHS considers providing additional flexibility for health plans, we encourage the above principles to be retained. We suggest that HHS also adopt the following measures to help ensure that VBID can facilitate access to a full range of high-value care:

- VBID should not lead to cost sharing increases for other covered items or services or reductions in the number of medicines on a health plan's formulary;
- VBID cost sharing must be based on an appropriate assessment of value, not price;
- Determination of high-value care should be based on the full body of available evidence, based on a range of study designs; and
- Determination of high-value care must incorporate relevant clinical quality and patient-centered measures and account for changes in evidence, medical practice, and innovations.

Finally, we urge HHS to consider extending plan flexibility to Part D benefits in future rulemaking. We recognize the programmatic complexity of doing so, but also note the absurdity of plans offering enrollees with diabetes zero cost sharing for endocrinologist visits, but charging 33 percent coinsurance for a biopharmaceutical anti-diabetic agent that could avoid the need for some physician or hospital visits all together. Because VBID can complement plans' efforts to implement value-based arrangements—and plans may use the same infrastructure to support both efforts—allowing plans greater flexibility to pursue VBID designs may also encourage more value-based arrangements between plans and biopharmaceutical companies.

⁴³⁹ [CMS-4182-P] Contract Year 2019 Policy and Technical Changes to the Medicare Advantage, Medicare Cost Plan, Medicare Fee-for-Service, the Medicare Prescription Drug Benefit Programs, and the PACE Program.

Indication- and regimen-based pricing

As described above, we recognize that health plans and some manufacturers are exploring indication- and regimen-based pricing. As HHS continues to explore these concepts, it will be important to develop approaches that continue to support patient access, support continued innovation, and encourage market competition on value, rather than a myopic focus on lowering prices. To this end, we urge consideration of the following principles:

- Market negotiations should determine the price of each indication between each payer and manufacturer—not government price setting or centralized value assessment.
- Confidentiality of net prices should be maintained to avoid driving all prices in the market to price for a single indication and undermining the objective of variable pricing by indication.
- When negotiating indication-based prices, health plans should make rigorous evaluations that consider the full range of available evidence (including real-world evidence) for the medicine.
- HHS should carefully evaluate any potential impacts to ASP reporting that may result from indication based pricing approaches.

To the extent that HHS pursues indication-based pricing or coverage in Part D, it will be important to consider how this policy would interact with existing beneficiary protections and other structural aspects of the Part D program. For this reason, CMS should also consider the following principles for Part D:

- Beneficiaries should continue to have access to a broad range of pharmacies and should be able to fill prescriptions at the pharmacy of their choice.
- Cost-sharing information for medicines with indication-based prices should be incorporated into Plan Finder and should be easily accessible and understandable for beneficiaries.
- PDPs may require access to medical claims or other diagnostic data necessary to determine the indication a medicine is prescribed for.

VALUE-BASED ARRANGEMENTS: Long-Term Financing (RFI p. 22697)

HHS suggest that states and other payers' budgets may be challenged by new high-cost treatments, which provide benefits over an extended period of time. However, there are examples of other services which can be high cost and provide a benefit which extends over several years. Organ transplants often cost \$500,000 to \$1 million per patient and neonatal intensive care units

can cost \$500,000 in some cases, yet insurers have mechanisms such as reinsurance to manage these costs, rather than spreading the costs over time.⁴⁴⁰

HHS asks about how Medicaid or Medicare should account for the cost of disease averted by a curative therapy paid for by another payer. We oppose efforts that would spread payment for a medicine from public to private payers or vice versa. Such approaches would be extremely complex to implement, undermining any potential benefits. We also believe they are unnecessary. For example, medicines that cure Medicaid patients of disabling conditions can help these individuals develop a higher functioning level which may enable them to earn a higher income and purchase their own insurance. A potential new gene therapy for hemophilia, which would be administered one time, could also lead to substantial savings for payers. In a retrospective study of U.S. health insurance claims between January 2004 and December 2012, annual payers' costs peaked at just under \$400,000 for hemophilia A and roughly \$450,000 for hemophilia B patients.⁴⁴¹ Finally, we are concerned that spreading payment between public and private payers requires changes to federal health care programs that would essentially require creation of a new, single payer for these medicines. This could encourage commercial payers to deny coverage for these medicines, with the aim of pushing payment for these medicines off to the new payer.

While we have concerns about long-term financing arrangements across public and private coverage, we do recognize that long-term financing arrangements with an individual payer or across multiple insurers within a specific market, e.g., in the commercial market, could support greater patient access or allow patients to spread their costs over multiple years. This is a viable option that could be considered for the appropriate therapy and patient population.

- **Long-term financing in the commercial market:** Long-term payment approaches may be possible in the commercial market today. As an example, Express Scripts is reportedly exploring such an arrangement with a gene therapy company.⁴⁴² These types of arrangements are at a very early form of development, and a range of different groups could take the role of spreading the payment over time; this is essentially a financing function, and other entities may be in a better position to offer this service than a pharmaceutical manufacturer.
- **Long-term financing in Medicaid:** As HHS considers new types of arrangements and considers the current barriers to long-term financing in Medicaid, we recommend that these arrangements be voluntary for both states and manufacturers. Also, HHS guidance on these new arrangements should ensure proper coverage and reimbursement for

⁴⁴⁰ Bentley TS, Phillips SJ. 2017 U.S. organ and tissue transplant cost estimates and discussion. Milliman Research Report. August 2017.; M Glabman. Million-Dollar Claim Club. *Managed Care Magazine*. March 1, 2009.

⁴⁴¹ Eldar-Lissai A, Hou Q, Krishnan S. The Changing Costs of Caring for Hemophilia Patients in the U.S.: Insurers' and Patients' Perspectives. *Blood*. 2014;124(21):199.

⁴⁴² Weintraub A. Payers point to Spark's gene therapy as a model for innovative pricing plans. *FiercePharma*. January 12, 2018. Available at: <https://www.fiercepharma.com/financials/payers-point-to-spark-s-gene-therapy-as-a-model-for-innovative-pricing-plans>.

medicines in the Medicaid program. Additionally, all arrangements should operate within the current Medicaid drug rebate statute coverage requirements.

Manufacturers that are exploring long-term financing approaches often describe these approaches as being complemented by an outcomes-based contract or other performance-based arrangements.⁴⁴³ Some manufacturers have identified the same barriers for these arrangements as for value-based arrangements, including the Anti-Kickback Statute, and federal price reporting rules.⁴⁴⁴

SECTION X: NATIONAL SPENDING ESTIMATES (RFI p. 22697)

Reports asserting that drug costs are the primary driver of increases in national health care spending are often based on analyses of medicines' undiscounted list prices.⁴⁴⁵ These reports paint an inaccurate picture of the true drivers of national health care spending growth. Even as medicines have played an increasingly important role in health care, changing the course of disease and producing better results for patients, the share of total health care spending devoted to prescription drugs has remained constant at 14 percent.⁴⁴⁶ In addition, medicines play a crucial role in controlling future health care costs: researchers have found that every additional dollar spent on medicines for adherent patients with congestive heart failure, high blood pressure, diabetes and high cholesterol generated \$3 to \$10 in savings on emergency room visits and inpatient hospitalizations.⁴⁴⁷

In reality, growth in spending on prescription medicines in recent years has fallen to historic lows.⁴⁴⁸ Reports that capture the net price of medicines, which properly account for the discounts and rebates negotiated by PBMs and plan sponsors, have found that net price increases for brand medicines have remained in the low single digits for the past several years, increasing just 1.9 percent in 2017, lower than the rate of inflation.⁴⁴⁹ Estimates of national health care spending should accurately reflect spending on medicines net of aggregate discounts and rebates in order to appropriately inform policymakers as they make decisions regarding health care spending controls and other payment and reimbursement issues.

⁴⁴³ Daniel G, Leschly N, Marrazzo J, McClellan M. Advancing Gene Therapies And Curative Health Care Through Value-Based Payment Reform. *Health Affairs Blog*. October 30, 2017. Available at: <https://www.healthaffairs.org/doi/10.1377/hblog20171027.83602/full/>.

⁴⁴⁴ Id.

⁴⁴⁵ See, e.g., U.S. Senate Homeland Security & Governmental Affairs Committee, Minority Office. *Manufactured Crisis: How Devastating Drug Price Increases Are Harming America's Seniors*. March 2018.

⁴⁴⁶ Altarum Institute. *A Ten Year Projection of the Prescription Drug Share of National Health Expenditures Including Non-Retail*. October 2014, addendum update May 2017.

⁴⁴⁷ Roebuck MC, et al. Medical Adherence Leads to Lower Health Care Use and Costs Despite Increased Drug Spending. *Health Affairs*. January 2011.

⁴⁴⁸ Drug Channels. *Who Best Managed the Drug Spending Slowdown in 2017: CVS Health, Express Scripts, MedImpact, or Prime Therapeutics?* May 2018. Available at: <https://www.drugchannels.net/2018/05/who-best-managed-drug-spending-slowdown.html>

⁴⁴⁹ IQVIA. *2017 Medicine Use and Spending*. April 2018.

NATIONAL SPENDING ESTIMATES: Accuracy of National Spending Data (RFI p. 22697)

Although projections of prescription medicine spending included in the NHE data attempt to capture spending on medicines net of discounts and rebates, they systematically overestimate prescription medicine spending.

As part of their recent review of the accuracy of NHE projections made between 1997 and 2016, CMS actuaries found that the projections for prescription drug spending overestimated drug spending on average and were more inaccurate than the projections made for other types of health spending.⁴⁵⁰ In an analysis of NHE projections released since 2000, we found that estimates of prescription drug spending growth made just one-year prior to the publication of actual spending amounts overestimated retail drug spending two-thirds of the time.⁴⁵¹

Improving the Accuracy and Comprehensiveness of National Spending Data

The RFI asks whether the Medicare Trustees Report, annual NHE publications, Uniform Rate Review Template, and other publications could more accurately collect and report gross and net drug spending in medical and pharmacy benefits.⁴⁵² Given the trends detailed above, the actuaries should reassess their methodology for projecting drug spending, including assumptions about the growth of rebates and discounts. As the actuaries themselves have noted, “drug sector growth is historically much more volatile than that of any other sector.”⁴⁵³ CMS should seek the input of outside experts to improve the accuracy of their projections of prescription drug spending and ensure that their estimation methods reflect up-to-date information about the biopharmaceutical market. The Secretary should consider convening a technical panel on the Medicare Trustees Reports so experts in their field can review CMS’ assumptions about pharmaceutical spending growth and provide feedback in a public setting.

Currently, NHE data on prescription drug spending is of limited use because it only captures spending on retail medicines. In order to provide a more comprehensive view, the actuaries should consider reporting total drug spending, by including spending for provider-administered medicines in addition to spending for retail medicines. There are a number of sources that attempt to report total medicine spending, including estimates previously released by the Assistant Secretary for Planning and Evaluation.⁴⁵⁴ However, these estimates use different methodologies and provide conflicting conclusions about the amount of national spending

⁴⁵⁰ CMS. Accuracy Analysis of The Short-Term (10-Year) National Health Expenditure Projections. February 2018.

⁴⁵¹ PhRMA analysis of CMS. NHE 2016. December 2017.

⁴⁵² RFI p. 22697.

⁴⁵³ CMS. Accuracy Analysis of The Short-Term (10-Year) National Health Expenditure Projections. February 2018.

⁴⁵⁴ HHS Office of the Assistant Secretary for Planning and Evaluation. Observations on Trends in Prescription Drug Spending. 2016.

attributable to medicines.⁴⁵⁵ Including spending for medicines administered by hospitals and physicians as part of the NHE could help remedy this confusion.

Additionally, the actuaries at CMS should consider breaking out prescription drug spending in the NHE into ingredient costs versus distribution and supply chain costs. Over the last decade, with the growth in use of generic medicines, the relative costs of distribution have grown. In addition, recent evidence suggests a shift toward greater spending for services provided by intermediaries. In 2015, brand and generic manufacturers accounted for 70 percent of net drug expenditures, while participants in the pharmaceutical supply chain realized 27 percent.⁴⁵⁶ These distribution and management costs account for a growing share of prescription drug spending, and tracking this trend as part of the annual NHE data release would help policymakers better assess the drivers of pharmaceutical spending growth.

Reporting of Part D Net Price Data for Small Molecule, Biologics, and High-Cost Drugs

The RFI asks about how the Medicare Trustees Report and other publications could report drug spending more accurately and whether average Part D rebate amounts “should be reported separately for small molecule drugs, biologics, and high-cost drugs.”⁴⁵⁷ Importantly, Part D rebate data is subject to several confidentiality provisions: (1) 18 U.S.C. § 1905, the Trade Secrets Act, which generally prohibits federal agencies from disclosing proprietary and confidential data submitted to the government by private parties; (2) Social Security Act (SSA) § 1860D-15(d)(2) and (f)(2), which protects data submitted by Part D plan sponsors to CMS for Part D payment purposes; and (3) SSA § 1860D-2(d)(2), which protects against disclosure of certain aggregate price concession data in a form that could identify a manufacturer or drug pricing.

Any disclosures of average Part D rebate data must conform fully to all of these protections, and compliance with all these provisions would become increasingly difficult: (1) the more granular the categories at which “average” rebate data is disclosed; and (2) the more information HHS discloses, or that is already publicly available, that could be analyzed in conjunction with average Part D rebate data HHS discloses and potentially provide insight into Part D rebates or pricing information regarding a specific drug or manufacturer. Beyond these legal issues, HHS should also bear in mind that the smaller and more granular the categories at which average rebate data is disclosed, the larger the risk that these disclosures would undercut vigorous competition between manufacturers to offer discounts and the higher the resulting Part D drug costs.⁴⁵⁸

⁴⁵⁵ Pew Charitable Trusts. A Look at Drug Spending in the U.S. February 2018. April 2018. Available at: <http://www.pewtrusts.org/en/research-and-analysis/fact-sheets/2018/02/a-look-at-drug-spending-in-the-us>

⁴⁵⁶ Vandervelde A, Blalock E; Berkeley Research Group. The pharmaceutical supply chain: gross drug expenditures realized by stakeholders. May 2017. Available at: http://www.thinkbrg.com/media/publication/863_Vandervelde_PhRMA-January-2017_WEB-FINAL.pdf

⁴⁵⁷ 83 Fed. Reg. at 22697.

⁴⁵⁸ See, e.g., Federal Trade Commission letter to the Honorable mark Formby, Mississippi House of Representatives, re SB 2445 (March 22, 2011) (noting that government disclosures of negotiated pricing information can “undercut vigorous competition on drug pricing” and undermine competition between drug

SECTION XI: DIRECT-TO-CONSUMER ADVERTISING (RFI p. 22695)

The RFI notes that HHS may request FDA to consider compelling biopharmaceutical companies to include list prices in DTC advertisements. Such a requirement would not benefit patients, could have the unintended and harmful consequence of deterring patients from seeking care, and would raise legal concerns.

As an initial matter, including the list price of medicines in DTC ads would not meet the Administration's aim of better informing patients. Such information would be potentially confusing to patients because list price is often not the relevant measure for what they actually pay. Patients picking up a prescription medicine often pay a co-pay dictated by their insurance company. Patients without insurance often receive assistance. Insurance companies usually do not pay the full list price because they receive substantial rebates and discounts.

Including list prices in DTC ads could deter patients from seeking care. Research shows that a major benefit of DTC ads is that they promote conversations between patients and their providers.⁴⁵⁹ If patients hear or see a list price in a DTC ad, they may erroneously assume that is the price they will be required to pay and that their out-of-pocket costs will be higher than they actually are. Mandating inclusion of list price information could thus mislead patients and would not result in transparency about their out-of-pocket costs. Instead, it could result in the unintended consequence of patients choosing to avoid talking with providers about their health care needs.

Alternative policies could yield meaningful cost and access-related information for patients. Information from stakeholders across the pharmaceutical supply chain have a greater effect on patient costs than medicine list prices. For example:

- Contracts with PBMs may prohibit pharmacists from informing consumers when their medicine's cash price is lower than the price the patient would pay through their insurance plan, or when manufacturer copay assistance could help reduce patient costs. Prohibiting such 'gag clauses' would give patients meaningful cost information.
- Providing real-time benefit information at the point of prescribing can help ensure patients and their providers make informed decisions about choice of treatment based on the patient's actual expected cost information.
- Requiring insurers to provide patients with easy access to information about their prescription drug benefit would also aid decision making by shedding light on formulary

manufacturers to offer discounts); Shepherd J, Is More Information Always Better? Mandatory Disclosure Regulations in the Prescription Drug Market. *Cornell Law Review Online*. 2013;99:1-22.

⁴⁵⁹ FDA. Patient and Physician Attitudes and Behaviors Associated with DTC Promotion of Prescription Drug – Summary of FDA Survey Research Results. November 19, 2004.; Prevention Magazine. 2012 Direct to Consumer Advertising Survey.

design and changes, cost sharing, access restrictions such as prior authorization, and the exceptions process, including rates of denials and appeals.

In addition to the policy concerns, any consideration of requiring disclosure of list prices in DTC ads must be squared with FDA's statutory authority and First Amendment restrictions against compelled speech.⁴⁶⁰ We do not believe that FDA currently has the statutory authority to impose such a requirement or that such a requirement would be constitutional. Moreover, any such proposal would be a substantive change to FDA's existing regulations and would necessitate notice and comment rulemaking.

SECTION XII: BIOSIMILAR DEVELOPMENT, APPROVAL, EDUCATION, AND ACCESS (RFI p. 22696)

PhRMA members support the development, and delivery of safe and effective biologics, including biosimilars. PhRMA appreciates the balance between incentives for innovation and the need for biosimilar competition struck in the BPCIA.⁴⁶¹ Additionally, PhRMA acknowledges Congress and FDA's continued efforts to implement the BPCIA through the BsUFA II⁴⁶² and associated BsUFA II Commitment Letter.⁴⁶³ These efforts will help provide earlier and more predictable access to biosimilar products, increasing biopharmaceutical competition in the marketplace.

PhRMA acknowledges that FDA has "prioritize[d] ongoing efforts to improve the efficiency of the biosimilar and interchangeable product development and approval process."⁴⁶⁴ In light of this prioritization, PhRMA reminds HHS that FDA already has an obligation, under the BsUFA II Commitment Letter, to produce certain information resources and development tools. We encourage FDA to implement these commitments promptly to assure an effective and efficient biosimilar approval process.

We are encouraged by HHS's solicitation of recommendations to improve the Purple Book.⁴⁶⁵ In addition to the information that FDA has committed to publish in the BsUFA II Commitment letter, PhRMA urges FDA to revise the Purple Book to state FDA's commitment to making and publishing prompt exclusivity decisions at the time of biologic approval.⁴⁶⁶ Prompt publication of this information is essential to provide certainty and transparency to all stakeholders. Specifically, prompt exclusivity decisions allow reference product sponsors the ability to understand much earlier whether their products will be entitled to exclusivity, and prompt

⁴⁶⁰ See, e.g., *NIFLA v. Becerra*, 138 S.Ct. 2361 (2018).

⁴⁶¹ BPCIA, Pub. L. No. 111-148 §§ 7001-7002, 124 Stat. 119, 804.

⁴⁶² Biosimilar User Fee Act Amendments of 2017, Pub. L. No. 115-52 §§ 401-407, 131 Stat. 1005, 2028.

⁴⁶³ FDA, Biosimilar Biological Product Reauthorization Performance Goals and Procedures FY 2018 Through 2022.

⁴⁶⁴ RFI at 22696.

⁴⁶⁵ Id.

⁴⁶⁶ PhRMA. Comments to Docket No. FDA-2013-D-1165 at 16-17. October 6, 2014. These comments provide greater detail on recommendations to revise the Purple Book to include prompt information on exclusivity determinations.

publication of those decisions allows potential biosimilar developers to know whether exclusivity will affect the timing of biosimilar application submission and approval. Thus, all stakeholders would benefit from this information and would be able to make more informed investment decisions. PhRMA also encourages FDA to include the name of the Biologic License Application (BLA) holder in the Purple Book.

PhRMA agrees that “[p]hysician education and patient confidence in biosimilar and interchangeable products is critical.”⁴⁶⁷ To that end, PhRMA supports FDA’s continued efforts to raise awareness of the agency’s role in the biosimilar approval process, increasing the public’s understanding of both biologics and biosimilars, and helping stakeholders understand the data and information that goes into biosimilarity determinations.

PhRMA supports FDA’s effort to create a regulatory framework for interchangeability. PhRMA recommends FDA finalize its guidance on interchangeability,⁴⁶⁸ with revisions to the draft guidance consistent with PhRMA’s comments and guided by the BPCIA and the science.⁴⁶⁹

SECTION XIII: AVAILABILITY OF REFERENCE PRODUCT SAMPLES (RFI p. 22695)

PhRMA appreciates the balance struck by the Hatch-Waxman Amendments to the Federal Food, Drug, and Cosmetic Act (FDCA), which established a framework where after a period of intellectual property (IP) protection, generics would be approvable.⁴⁷⁰ Although it is a different framework, the BPCIA relies on a similar premise allowing for the approval of biosimilars once reference product exclusivity has lapsed.⁴⁷¹ Both of these regimes then operate from the starting proposition that IP rights are key to innovation and thus must be respected. Of course, the other side of the balance struck by both Hatch-Waxman and the BPCIA is that once applicable protections have expired, generics and biosimilars should be eligible for approval. To ensure this is possible, reference product samples should be reasonably available under terms consistent with patient safety for bioequivalence and biosimilar testing to allow for their approval and licensure when permitted under statute. Reference product sponsors should not withhold samples to delay generic or biosimilar entry.

AVAILABILITY OF REFERENCE PRODUCT SAMPLES: REMS (RFI p. 22696)

Risk management is an integral part of sound clinical care and an important responsibility of biopharmaceutical innovators. The Food and Drug Administration Amendments Act of 2007

⁴⁶⁷ RFI p. 22696.

⁴⁶⁸ FDA. Draft Guidance for Industry, Considerations in Demonstrating Interchangeability With a Reference Product. January 2017.

⁴⁶⁹ PhRMA. Comments to Docket No. FDA-2017-D-0154. May 19, 2017.

⁴⁷⁰ Drug Price Competition and Patent Term Restoration Act of 1984, Pub. L. No. 98-147, 98 Stat. 1585 (as amended); PhRMA. Comments to Docket No. FDA-2017-N-3615. November 17, 2017.

⁴⁷¹ BPCIA, Pub L. No. 111-148 §§ 7001-7002, 124 Stat. 119, 804.

gave FDA authority to require a REMS.⁴⁷² FDA's REMS authorities allow FDA to impose safeguards to help ensure that medicines that carry high risk are prescribed, distributed and taken appropriately, while at the same time enabling patients to have continued access to the medicine by implementing a safety strategy to manage any known or potential serious risk associated with a medicine.

To impose a REMS, FDA must determine that the REMS is necessary to ensure the product's benefits outweigh its risks.⁴⁷³ REMS including elements to assure safe use (ETASU) are limited to when FDA has determined that, because of the drug's inherent toxicity or potential harmfulness, the drug may be approved only if, or would be withdrawn unless, the ETASU are required. In addition, if a REMS exists for an already approved drug without ETASU, ETASU will be required if the existing elements of a REMS are not sufficient to mitigate the risks.⁴⁷⁴ Any ETASU imposed shall, considering the risks that prompted the REMS, not be unduly burdensome on patient access to the drug and, to the extent practical, minimize the burden on the health care delivery system.⁴⁷⁵

As part of its ongoing REMS authority, FDA can evaluate the impact of one (or more) REMS with ETASU on the health care delivery system and also structure or revise REMS to minimize the impact to the system.⁴⁷⁶ PhRMA supports FDA exercising that authority to evaluate whether one or more REMS has had an impact on the availability of generics or biosimilars. After completing such an assessment, FDA could then consider whether there are particular steps the agency might take to revise or modify REMS to allow for sample access while not undermining the patient safety protections the REMS was imposed to provide. For example, depending on the risks the REMS was imposed to mitigate, FDA might require the generic or biosimilar applicant to submit protocols, informed consent documents, and other relevant materials to ensure the safety protections of the REMS were not undermined.

FDA should revise REMS to confirm that provision of samples to generic or biosimilar applicants who have obtained a Safety Determination Letter would not violate the REMS.⁴⁷⁷ FDA could also evaluate whether REMS supporting documents might appropriately include information about how generic or biosimilar developers might obtain product samples, including the

⁴⁷² Food and Drug Administration Amendments Act of 2007 § 901(b), 121 Stat. 823, 922 (as codified at 21 U.S.C. § 355-1).

⁴⁷³ 21 U.S.C. § 355-1(a)(1), (2)(A).

⁴⁷⁴ Id. § 355-1(f)(1)(A)-(B).

⁴⁷⁵ Id. § 355-1(f)(2)(C)-(D).

⁴⁷⁶ Id. § 355-1(f)(5)(B), (g)(4).

⁴⁷⁷ FDA. Draft Guidance for Industry, How to Obtain a Letter from FDA Stating that Bioequivalence Studies Protocols Contain Safety Protections Comparable to Applicable REMS for RLD. December 2014. We note that FDA has taken the position that the contents of a REMS may include only "safety-related elements."; Janet Woodcock. Letter to Kumar Sekar. August 17, 2013, Docket No. FDA-2009-P-0266. However, this position does not preclude FDA from including, in REMS supporting documents, a statement that providing samples, in certain circumstances (e.g., upon receipt of a Safety Determination Letter) for purposes of bioequivalence or biosimilar testing, does not violate the REMS. The existing template language in REMS approval letters merely reminding sponsors of 21 U.S.C. § 355-1(f)(8) may not provide sufficient assurance that supplying samples is not a violation of REMS.

information that the generic or biosimilar developer might be required to provide FDA in order to obtain a Safety Determination Letter. Finally, FDA might consider whether it is fully exercising its authority under the current statute.

AVAILABILITY OF REFERENCE PRODUCT SAMPLES: Additional Measures (RFI p. 22696)

Although PhRMA supports FDA taking appropriate measures within its existing statutory authority to address product sample access issues, legislation may be useful to fully address the issue. We take seriously the concerns raised about REMS and other distribution systems being used to delay generic entry. We are actively engaged with policymakers to develop policy solutions that ensure the timely transfer of samples to generic manufacturers without risking patient safety or establishing a tool that creates an incentive for predatory litigation.

PhRMA would support an appropriate statutory solution, but has concerns with the proposals introduced to date. For example, we are concerned that the CREATES Act⁴⁷⁸ would undermine the role of FDA in access decisions for products with REMS with ETASU and would encourage frivolous litigation. We would support a proposal that instead codifies within the FDCA an authorization process for access to samples for products with REMS with ETASU. We also would encourage safeguards in any new cause of action including affirmative defenses for license holders that offer samples at commercially reasonable terms as well as statutory assurance that providing samples would not violate REMS.

SECTION XIV: FIXING GLOBAL FREELOADING (RFI p. 22697)

The RFI appropriately identifies the problem of global free riding, whereby advanced economies are relying on U.S. patients to bear a disproportionate share of the cost to develop innovative medicines.⁴⁷⁹ Furthermore, as highlighted in the U.S. Trade Representative's recent Fact Sheet on its Engagement on Pharmaceutical and Medical Device Issues, too many countries are undervaluing and/or undermining U.S. IP.⁴⁸⁰ Recognizing these problems, the RFI asks what can be done to reduce the pricing disparity and spread the burden for incentivizing new drug development more equally between the U.S. and other developed countries.⁴⁸¹ In addition, the RFI seeks input on what policies the U.S. government should pursue in order to protect IP rights and address concerns around compulsory licensing in this area.⁴⁸²

To research, develop and deliver new treatments and cures for patients who need them around the world, biopharmaceutical innovators must be able to secure and effectively enforce patents and protect regulatory test data. They must be able to obtain timely marketing approval for new medicines and make those therapies available to patients according to reimbursement rules and

⁴⁷⁸ S. 974, 115th Congress (2017); H.R. 2212, 115th Congress (2017)

⁴⁷⁹ RFI p. 22697.

⁴⁸⁰ USTR Fact Sheet. USTR Engagement on Pharmaceutical and Medical Device Issues. April 2018.

⁴⁸¹ RFI p. 22967.

⁴⁸² Id.

procedures that are fair, transparent, reasonable, and non-discriminatory, and that appropriately value and reward patented pharmaceuticals. With the right policies and incentives in place at home and abroad, they can continue to bring valuable new medicines to patients and contribute powerfully to the American economy and jobs.

In recent years, however, America's biopharmaceutical sector has witnessed a surge in the number of trading partners that impose arbitrary or unreasonable pricing and reimbursement policies and/or steal U.S. IP. In many countries, governments are the principal payer of medicines and effectively dictate prices. Too often, this dominant position is used to benefit domestic drug companies and wholesalers at the expense of innovators in the U.S.

Foreign governments employ multiple price control measures in tandem to artificially depress the market value of U.S. innovative medicines, including:

- **International Reference Pricing**, where developed markets reference prices in poorer countries or countries that undermine incentives for innovation.
- **Therapeutic Reference Pricing**, where trading partners require innovative medicines to have similar prices as older medicines.
- **Health Technology Assessment**, where governments arbitrarily apply low thresholds on the value of innovative medicines.
- **Mandatory Price Cuts and Clawbacks**, which act as perverse incentives against developing treatments for new indications and patient-centered formulations.
- **Compulsory Licensing**, where governments threaten to steal IP as a negotiating ploy.
- **Discriminatory Practices**, by which U.S. companies are denied due process and a level playing field, including through non-transparent decisions and localization measures.

The 2004 Department of Commerce Report on this issue demonstrates how, as more countries enact price controls and similar measures, the burden for financing medical advances will be increasingly borne by U.S. patients and biopharmaceutical innovators, while patients abroad will suffer decreased access to improved therapies over the long term.⁴⁸³ Such threats significantly undervalue U.S. innovation and threaten good-paying U.S. jobs and the development of pioneering therapies.

In the Report—which the President's 2019 budget indicates is being updated—Commerce found that tackling foreign price controls in just a few countries could “increase[] the flow of [new

⁴⁸³ Department of Commerce. Pharmaceutical Price Controls in OECD Countries Implications for U.S. Consumers, Pricing, Research and Development, and Innovation. Commerce Report. December 2004.

medicines] by three to four per year,”⁴⁸⁴ generating increased competition in the U.S. marketplace and savings for U.S. patients. Economists agree, and have concluded time and again, that when biopharmaceutical companies have more resources to invest in research and development (R&D), it leads to more innovation and competition and better health outcomes. For example, lifting government price controls in other wealthy countries would:

- Increase the number of new treatments available by 2030 by 9 percent—equivalent to 8-13 new drugs in that year.⁴⁸⁵
- Increase life expectancy for an American aged 15-years-old today by 1.1 years.⁴⁸⁶
- Increase welfare gains of \$10 trillion for Americans and \$7.5 trillion for Europeans over the next 50 years, reflecting improved length and quality of life.⁴⁸⁷

In turn, there is overwhelming evidence that where there are more competing medicines, the market forces costs down:

- Within a year of the introduction of a breakthrough HCV cure, there were multiple competitors in the market that enabled payers to negotiate deep discounts for these medicines in exchange for favorable formulary placement. Competition drove rebates from about 22 percent in 2014 to discounts ranging from about 40-65 percent today, as well as lower WAC prices.⁴⁸⁸
- In the case of new cholesterol-lowering medicines, called PCSK9 inhibitors, despite initially claiming that the medicines could “wreak financial havoc,” Express Scripts, the nation’s largest PBM, ended up including them on its national list of covered medicines, thanks in part to substantial negotiated discounts and aggressive utilization management policies. According to the company, “[w]e were able over the course of tough negotiations to get good economics on both products.”⁴⁸⁹

⁴⁸⁴ Id.

⁴⁸⁵ Schwartz TT, et al. The Impact of Lifting Government Price Controls on Global Pharmaceutical Innovation and Population Health, Precision Health Economics. May 2018. Available at: <https://www.ispor.org/ScientificPresentationsDatabase/Presentation/81984?pdfid=54396>

⁴⁸⁶ Id.

⁴⁸⁷ Goldman D, Lakdawalla D. The Global Burden of Medical Innovation. The Brookings Institution. January 30, 2018. Available at: <https://www.brookings.edu/research/the-global-burden-of-medical-innovation/>

⁴⁸⁸ Silverman E. The Hepatitis C Scorecard: Gilead is Trouncing AbbVie, but at a Price, *W.S.J. Pharmalot Blog*. February 12, 2015. Available at: <https://blogs.wsj.com/pharmalot/2015/02/12/the-hepatitis-c-scorecard-gilead-is-trouncing-abbvie-but-at-a-price/>

⁴⁸⁹ Pollack A. Express Scripts Says It Will Cover 2 New Cholesterol Drugs. *New York Times*. October 6, 2015. Available at: https://www.nytimes.com/2015/10/07/business/express-scripts-says-it-will-cover-2-new-cholesterol-drugs.html?_r=1

As the Report also notes, “the benefits for consumers in the United States from deregulation of foreign drug prices and increased R&D would be expected to rise as a result of savings from hospitalization, fewer missed work days, and other medical cost savings. Obviously, aggressive reforms among the OECD countries would accelerate this effect.”⁴⁹⁰ For example:

- The use of cholesterol-lowering statin drugs has cut hospitalizations and saved the U.S. health care system at least \$5 billion.⁴⁹¹
- Every \$24 spent on new medicines for cardiovascular diseases in OECD countries saves \$89 in hospitalization costs.⁴⁹²
- Treating high blood pressure according to clinical guidelines would result in annual health system savings of about \$15.6 billion.⁴⁹³
- New HCV cures have the potential to reduce future U.S. health care spending by \$115 billion.⁴⁹⁴
- In the fight against Alzheimer’s disease, a new medicine that delays the onset of Alzheimer’s disease by five years would avoid \$367 billion annually in long-term care and other health care costs by 2050.⁴⁹⁵

In addition to lowering overall health care costs, appropriate use of medicines can increase worker productivity by reducing rates of absenteeism and short-term disability.⁴⁹⁶

Recognizing the benefits of addressing free riding by other developed countries, here are four recommended actions that this Administration could take to end the most unfair and discriminatory trade practices faced by the U.S. innovative biopharmaceutical industry.

⁴⁹⁰ Department of Commerce. Pharmaceutical Price Controls in OECD Countries Implications for U.S. Consumers, Pricing, Research and Development, and Innovation. Commerce Report. December 2004.

⁴⁹¹ Grabowski D, Lakdawalla D, et al. The Large Social Value Resulting From Use Of Statins Warrants Steps To Improve Adherence And Broaden Treatment. *Health Affairs*. October 2012.

⁴⁹² Lichtenberg F. Have newer cardiovascular drugs reduced hospitalization? Evidence from longitudinal country-level data on 20 OECD countries, 1995-2003. National Bureau of Economic Research. May 2008.

⁴⁹³ Cutler, DM, Long G, et al. The Value of Antihypertensive Drugs: A Perspective on Medical Innovation. *Health Affairs*. January 2007.

⁴⁹⁴ Milliman. An Actuarial Approach to the Incremental Cost of Hepatitis C in the Absence of Curative Treatments. September 2015. Available at:

http://www.milliman.com/uploadedFiles/insight/2015/20150915_Incremental-Cost-of-HCV-White-Paper.pdf.

⁴⁹⁵ Mandel M. The Folly of Targeting Big Pharma: The biggest driver of rising health-care spending is the cost of labor, not drugs. *W.S.J. Op. Ed.* December 10, 2015. Available at:

<https://www.wsj.com/articles/the-folly-of-targeting-big-pharma-1449792625>.

⁴⁹⁶ Carls GS, Roebuck MC, et al. Impact of medication adherence on absenteeism and short-term disability for five chronic diseases. *Journal of Occupational and Environmental Medicine*. July 2012.

1. Secure Strong Commitments in Global, Regional and Bilateral Negotiations

Global, regional, and bilateral trade and investment negotiations provide critical opportunities to build on the existing foundation of international rules and to secure commitments necessary to drive and sustain 21st Century biopharmaceutical innovation. Recognizing this opportunity, Congress has identified unreasonable foreign pricing and reimbursement policies as major concerns to be addressed in trade negotiations. Specifically, the Trade Promotion Authority (TPA) legislation, pursuant to which the Administration is renegotiating NAFTA, identifies as a principal negotiating objective for free trade agreements “to ensure that government regulatory reimbursement regimes are transparent, provide procedural fairness, are non-discriminatory, and provide full market access for United States products.” As noted in the TPA’s legislative history, ensuring full market access “includes setting the reimbursement amount based on competitive, market-derived pricing or an equivalent process, such as one that appropriately recognizes the value” of innovative products.

The existing NAFTA does not contain pharmaceutical pricing and reimbursement obligations, and yet such obligations are critically needed to address market access barriers faced by the U.S. innovative biopharmaceutical industry in our closest trading partners. In particular, Canada’s Patented Medicine Prices Review Board (PMPRB) imposes price caps solely on patented medicines in both the public and private segments of the Canadian market. This unfairly undervalues innovative U.S. medicines. Conversely no price caps are imposed on generics, thereby bolstering domestic Canadian generic interests. Canada has recently proposed sweeping regulatory changes to the PMPRB to remove the U.S. from its reference pricing system in favor of South Korea and other countries that are poorer and/or have onerous price control policies. They have also proposed a value assessment system for medicines in Canada modeled on existing systems abroad that have delayed access and produced poor health outcomes, like in the U.K. In turn, the Canadian Government is proposing to use these mechanisms to further drive down prices in the private insurance market. Obligations should be secured through NAFTA renegotiation to appropriately value innovation and ensure a level playing field.

In addition, Mexico has failed to fulfill its obligations under NAFTA and the World Trade Organization (WTO) Agreement on Trade-Related Aspects of Intellectual Property Rights (TRIPS) to ensure that regulatory data submitted to obtain marketing approval of pharmaceutical products in Mexico are protected against unfair commercial use and unauthorized disclosure. Mexico fails to provide effective regulatory data protection for biologic medicines. Despite numerous judicial orders in Mexico compelling federal agencies to provide such protection for biologics, the Mexican government has yet to implement this NAFTA obligation. Mexico should pass regulations to provide greater certainty regarding the extent and durability of Mexico’s commitment to protecting and promoting innovation.

2. Enforce and Defend Global, Regional, and Bilateral Rules

The Administration should use all available tools and leverage to ensure America's trading partners live up to their obligations in global, regional, and bilateral trade and investment agreements. Modernizing existing trade agreements and stepping up enforcement activity in the months ahead will be critical to end discriminatory pricing policies and to address longstanding IP challenges around the world—particularly in countries that are U.S. trade and investment agreement partners, that have made important unfulfilled WTO accession commitments and that benefit from U.S. trade preference programs.

In this regard, the Administration has already taken a strong initial step to secure a commitment from Korea to amend its Premium Pricing Policy to ensure that consistent with its obligations in the Korea-United States free trade agreement (KORUS), it does not discriminate against U.S. biopharmaceutical manufacturers.⁴⁹⁷ And yet, as outlined in PhRMA's 2018 Special 301 submission, there are many elements of Korea's pricing and reimbursement system that are not consistent with its commitment to appropriately recognize the value of patented pharmaceuticals. PhRMA and its members stand ready to engage with both the Korean and U.S. governments on broader reforms to Korea's pricing and reimbursement system to ensure that Korea faithfully and comprehensively implements its KORUS commitments to the U.S.

Furthermore, contrary to Korea's commitment in KORUS, recent Court decisions in Korea inappropriately restrict the availability of patent term extensions, which enable U.S. innovators to seek restoration of a portion of the patent term lost due to lengthy regulatory approval processes. Left standing, these decisions will negate the value of patent term extensions in Korea.

Similarly, in recent years, Australia has made significant changes to its pricing and reimbursement policies, making it more difficult for Australian patients to access innovative medicines. Of particular concern is an arbitrary and broad-based retroactive price reduction which was applied to all medicines listed on Australia's Pharmaceutical Benefits Scheme (PBS) for five or more years, and which disproportionately impacts foreign companies. Such *ad hoc* price cuts, along with other onerous conditions placed on PBS-listed medicines and price-depressing measures such as health technology assessment, are creating significant uncertainty and lost revenues for U.S. innovators.

Moreover, Australia is unfairly tipping the scales in commercial patent disputes by encouraging competitors to launch at risk and discouraging innovators from enforcing their patents. Since 2012, the Australian government has sought "market-sized damages" from innovators that have unsuccessfully sought to enforce patent claims. Those damages are designed to compensate Australia's PBS for any higher price paid for a patented medicine during the period of a provisional enforcement measure. It exposes innovators to significant additional compensation claims that are difficult to quantify and were not agreed to at the time provisional enforcement

⁴⁹⁷ USTR Fact Sheet. New U.S. Trade Policy and National Security Outcomes with the Republic of Korea. March 28, 2018.

measures were granted. The size of these additional claims equates legitimate patent enforcement with patent abuse. Allowing governments or other non-parties to a patent dispute to collect market-size damages undermines legal certainty, predictability, and the incentives patents provide for investment in new treatments and cures. Contrary to its trade agreements with the U.S., Australia is failing to value innovation appropriately and is seriously hampering innovative companies' ability to protect their patents.

3. Ensure that Foreign Government Pricing and Reimbursement Policies are Transparent, Provide Due Process, are Non-Discriminatory, and Appropriately Value U.S. Innovation

PhRMA members are, and seek to be, partners in solutions to health care challenges facing patients and their communities around the world. However, some governments have proposed or implemented pricing and reimbursement policies that discriminate against medicines made in America, do not appropriately value innovation, and lack predictable, transparent, and consultative processes. For example, just last year, Japan approved sweeping changes to its pricing policies that significantly undermine Japan's pro-innovation environment and its efforts to carry its fair share of the costs of global R&D efforts. Like earlier price-cutting measures, the new framework was developed behind closed doors without meaningful opportunities for input from key stakeholders, including the innovative pharmaceutical industry. Despite strong engagement by the U.S. government throughout 2017, Japan reduced the scope of products covered by its Price Maintenance Premium (a program intended to ensure that innovative medicines are not hit by draconian price cuts), and imposed new company requirements that benefit Japanese manufacturers over U.S. innovators in pricing.

Particularly onerous pricing practices in several developed economies include international reference pricing, therapeutic reference pricing, and health technology assessment. These practices dictate the terms of market access for our industry and can result in significant negative impacts on patients and America's biopharmaceutical industry, including by eviscerating the expected benefit of IP protections. Moreover, such measures can undermine the ability of biopharmaceutical innovators to bring new medicines to patients who need them and to invest in future treatments and cures.

The U.S. government can play a critical role in ensuring transparency and due process of pricing and reimbursement policies, as well as in highlighting the global benefits to patients that result from a reduction in trade barriers. The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 called for the Administration to develop a strategy to address foreign price controls on pharmaceuticals and related practices through bilateral and multilateral trade negotiations. PhRMA believes that the cornerstone of any such strategy must be a proactive U.S. trade policy focused on: (i) addressing discriminatory government price controls and related practices; and (ii) highlighting the global benefits for patients from the potential groundbreaking research that could result from a reduction in key trade barriers. Completing the update of the 2004 Commerce Report on Pharmaceutical Price Controls in OECD Countries will be an

important first step in identifying the worst offenders and developing a comprehensive strategy using all available levers to address this important issue.

4. Leverage All Available Trade Tools to Combat Abuse of Compulsory Licensing

Too often, foreign governments threaten compulsory licensing to compel innovators to lower pharmaceutical prices—even where the medicine is being sold at the price originally dictated by the government. For example, Colombia recently threatened to issue a compulsory license (CL) for an innovative cancer medicine, even though the medicine was being sold in the country at the price mandated by the government. While Colombia did not issue the CL, it did force a drastic mandatory price cut to levels as if the patent on the medicine did not exist.

Often with the support of multilateral organizations, countries around the world are issuing or currently considering CLs on a wide range of innovative medicines. Last year, Malaysia issued a CL for one HCV treatment, and Saudi Arabia took action with equivalent effect for another. Colombia is now assessing whether to grant another petition that ultimately is seeking the imposition of a CL on the whole class of HCV medicines. American inventions are at risk in Chile, El Salvador, Peru and Russia. The fact that CLs have now been issued in countries across Asia, Africa, and Latin America has emboldened governments to follow through on threats and diminished what little leverage innovators have in price negotiations.

Where specific and credible threats of compulsory licensing arise, the U.S. government must defend American innovators and engage relevant authorities abroad. The U.S. government must make common cause with other like-minded governments, and push back in multiple multilateral organizations and other fora that are seeking to erode IP protections. Furthermore, the U.S. government should not provide unilateral trade benefits like Generalized System of Preferences (GSP) or allow countries to accede to organizations such as the OECD until those countries have demonstrated that they are prepared to offer a level playing field to U.S. innovators.

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On behalf of PhRMA and our member companies, thank you for consideration of these comments. We look forward to working with you to address the many important issues discussed in the RFI.

Sincerely,



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James C. Stansel
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