



May 9, 2016

Andrew M. Slavitt
Acting Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building, Room 445-G
200 Independence Avenue, SW
Washington, DC 20201

BY ELECTRONIC DELIVERY

Re: Medicare Program; Part B Drug Payment Model [CMS-1670-P]

Dear Acting Administrator Slavitt:

The Biotechnology Innovation Organization (BIO) appreciates this opportunity to submit comments on the Centers for Medicare and Medicaid Services' (CMS's) proposed rule entitled *Medicare Program; Part B Drug Payment Model* (the "Proposed Rule").¹

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or to prevent them in the first place. In that way, our members' novel therapeutics, vaccines, and diagnostics not only have improved health outcomes, but also have reduced healthcare expenditures due to fewer physician office visits, hospitalizations, and surgical interventions.

BIO represents an industry that is devoted to discovering new treatments and ensuring patient access to them. Accordingly, we closely monitor changes to Medicare's reimbursement rates and payment policies for their potential impact on innovation and patient access to drugs and biologicals. To these ends, we are gravely concerned that CMS has issued a Proposed Rule that will diminish Medicare providers' ability to obtain Part B therapies, and in turn, threaten patient access to needed medicines. Given these concerns, and the significant deviation of CMS's proposed approach from the statutory requirements and congressional intent with respect to Center for Medicare & Medicaid Innovation (CMMI) demonstrations, **BIO strongly urges the Agency to withdraw the Proposed Rule in its entirety.** In its place, CMS should establish an inclusive dialogue with stakeholders to identify discrete opportunities for Part B changes in an evidence-based manner and work collaboratively to develop any future demonstration programs with a scope and approach that align with Congress's intent in authorizing CMMI.

¹ 81 Fed. Reg. 13,230 (Mar. 11, 2016).

I. Overview of BIO's Comments Regarding the Proposed Rule.

Throughout these comments, BIO expresses grave concerns that the Proposed Rule will threaten patient access to needed therapies and result in increased overall Medicare expenditures. In this introductory section of the letter, we identify and summarize our four primary concerns with the Proposed Rule, which will be discussed in detail throughout the remainder of this letter in an order that parallels that of the Proposed Rule itself. Namely, BIO is very concerned that the Proposed Rule:

- (1) Will threaten patient access to the most appropriate medicine for them, especially for those patients who seek treatment in the community setting and/or who live in rural areas ([see section VI.B](#));
- (2) Does not prioritize patients' quality of care, nor provide for an accurate evaluation of how the Proposed Rule would impact patients ([see section XI](#));
- (3) Does not align with the statutory provisions that govern CMMI demonstrations ([see section III](#)) and relies on waiver proposals that either exceed CMMI's statutory authority or pose serious constitutional questions regarding the underlying statute ([see section X](#)); and
- (4) Would establish novel pricing mechanisms, not all of which are "value-based," and none of which are ready for widespread implementation and use ([see section VII](#)).

Each of these concerns are summarized here, in turn.

First, CMS's Phase I proposals will diminish providers' ability to obtain Part B therapies at or below the Medicare reimbursement rate, and in turn, threaten patient access to needed medicines. This is of particular concern for patients who are treated by providers practicing in a community setting and those who live in rural areas. The Average Sales Price (ASP) calculation methodology—and the derivative Part B payment methodology—inherently recognizes that not all providers are able to acquire Part B therapies at the same price.² This is particularly true for smaller practices and/or providers who practice in rural settings, who generally lack the economies of scale and purchasing power necessary to obtain medicines at prices closer to ASP. The blunt and arbitrary cut in provider reimbursement proposed under Phase I of the Proposed Rule is likely to hurt these community providers the most, an impact that will be exacerbated by the mandatory application of sequestration to the proposed reimbursement rate. If providers are unable to obtain therapies at or below the Medicare reimbursement rate, the Proposed Rule will incentivize patients to: compromise on therapies based on whether their provider is able to obtain the most appropriate therapy; seek care in a higher-cost setting (i.e., hospital outpatient departments) that may be farther away and require them to switch providers; or forgo care entirely. Thus, the Proposed Rule will undermine, not achieve, CMS's stated goals of improving patient care and decreasing overall Medicare expenditures.

² The Medicare Payment Advisory Commission (MedPAC)—an independent advisory to Congress on issues of Medicare payment policy—has noted that, even at ASP+6%, certain providers still are not able to obtain all Part B therapies at or below the Medicare reimbursement rate. [See](#) MedPAC, Report to Congress: Impact of Changes in Medicare Payments for Part B Drugs (2007), available at http://www.medpac.gov/documents/jan07_partb_mandated_report.pdf.

Second, the Proposed Rule's minute focus on the cost of Part B therapies obscures the broader value these therapies have with regard to improving patients' health outcomes and reducing overall healthcare expenditures. This singular, imbalanced focus on costs is contrary to CMMI's historical approach to demonstration projects and to the type of demonstrations enumerated in the CMMI statute, which holistically target the full spectrum of patient care, focusing primarily on improving quality of care and care continuity. BIO also raises concerns with the absence of robust, reliable quality measures to track patient access and quality of care in the Proposed Rule. The inclusion of such measures and a robust framework in which to assess them is not only a requirement of all CMMI demonstrations, but is a critical component of responsible, thoughtful policymaking.³ Moreover, BIO believes ethical issues also must be considered, especially because the research design may not produce scientifically sound results. In such circumstances, the research can subject participants to risk without advancing knowledge. We question whether CMS has considered the implications the Proposed Rule may have with regard to the Federal Policy for the Protection of Human Subjects ("Common Rule"),⁴ a concern also voiced by Senator Grassley.⁵

Third, the Proposed Rule is inconsistent with the statutory requirements for a CMMI demonstration. Specifically, contrary to statute, the Proposed Rule is not limited to a "defined population," and CMS has not produced evidence that the diverse patient population treated by Part B therapies suffers from "deficits in care."⁶ In fact, there is evidence to suggest just the opposite: the existing Part B reimbursement methodology is functioning as Congress intended, both in terms of aligning provider payment for Part B therapies with the actual costs to obtain these therapies, and in terms of facilitating patient access to these therapies.⁷ There also is evidence to demonstrate that the existing ASP methodology does not drive provider decision-making, contrary to CMS's conjecture.⁸ Additionally, the Proposed Rule's size and scope do not align with the statutory requirements for a CMMI "Phase I" test, as the proposed provisions would immediately apply to virtually all Medicare providers before CMMI had met any of the criteria that would allow for such a dramatic model expansion. Moreover, BIO has serious legal questions regarding CMS's proposed waivers, including that, as a threshold matter, CMS is not authorized to use any waivers in the context of the Proposed Rule since statute limits waivers to "Phase I" testing. BIO also expresses concern that the Proposed Rule was developed in the absence of the robust stakeholder engagement envisioned by CMMI's authorizing statute.⁹

Fourth, despite their description as "value-based," many of the models described in Phase II of the Proposed Rule are actually blunt cost-containment tools that run counter to

³ See Social Security Act (SSA) § 1115A(b)(4).

⁴ See 42 C.F.R. 46.101(b)(5); 80 Fed. Reg. 53,933, 53,957 (Sept. 18, 2015).

⁵ Letter to the Hon. Sylvia Burwell, Secretary, Dep't of Health & Human Servs. from the Hon. Charles E. Grassley, U.S. Sen. (Apr. 29, 2016).

⁶ SSA § 1115A(b).

⁷ The Moran Company, Trends in Weighted Average Sales Prices for Prescription Drugs in Medicare Part B, 2006-2014, Figure 4, p. 5 (2015), available at http://phrma.org/sites/default/files/pdf/asp_report_2015.pdf.

⁸ S. Pershing et al, *Treating Age-Related Macular Degeneration: Comparing The Use Of Two Drugs Among Medicare And Veterans Affairs Populations* 34 HEALTH AFFAIRS 229, 234 (2015).

⁹ SSA § 1115A(a)(3).

patient-centric care. For example, CMS’s proposals around reference pricing and indications-based pricing do not capture the complexities—including the individualized nature—of treating patients with Part B therapies, and will result in diminished patient access to the most appropriate treatment regimen for them. Moreover, CMS’s proposals around outcomes-based risk sharing, while conceptually closer to true “value-based” arrangements, are **not** ready for widespread implementation at this time. This is because there are fundamental and unresolved legal, operational, and other issues associated with their use. Especially since there is no “one-size-fits-all” value-based model, any experimentation with such models must involve only voluntary arrangements of limited scope, beginning in the private sector. In the meantime, CMS—and its sister agencies within the Department of Health and Human Services—should prioritize creating a safe and predictable regulatory environment to enable these models to succeed in the private sector.

These four primary concerns, as well as BIO’s numerous additional methodological concerns with the Proposed Rule, are described in greater detail throughout the balance of this letter. Each of these four concerns, on its own, would be grounds to recommend that CMS withdraw the Proposed Rule in its entirety. Thus, considering them together, **BIO urges CMS, in the most strenuous terms, to withdraw the Proposed Rule.** We recommend that the Agency, instead, initiate an inclusive dialogue with stakeholders to identify discrete opportunities to improve the quality of patient-centered care delivered under Part B while reducing or maintaining overall Medicare expenditures. BIO looks forward to participating in such a dialogue with CMS in the near future.

II. Table of Contents

III. The Proposed Rule is inconsistent with the statutory requirements for a CMMI “Phase I” test, including the requirement to consult with stakeholders.

7

A. The Proposed Rule is not limited to a “defined population,” and CMS has not produced evidence that the diverse patient population treated by Part B therapies suffers from “deficits in care leading to poor clinical outcomes or potentially avoidable expenditures.” 7

B. The Proposed Rule outlines a large-scale program change, as opposed to a true “test.” 9

C. The Proposed Rule was developed and released without the stakeholder engagement necessary for thoughtful model development, in stark contrast to CMMI’s other demonstrations and the statutory requirement for stakeholder consultation. 11

IV. Participation: Proposed Drugs Paid under Part B to be included in the Demonstration. CMS’s proposal to include all Part B drugs, biologicals, and biosimilars will threaten patient access to these vital therapies. 13

A. BIO recommends that CMS consider the significance of how biologicals are used to treat patients with some of the most complex, chronic conditions, including rheumatoid arthritis, Crohn’s disease, cancer, and rare diseases (e.g., lysosomal storage disorders, hemophilia, and multiple sclerosis). 13

B. CMS proposes to include almost all Part B therapies in the Proposed Rule—subjecting them to significantly lower reimbursement rates—despite the Agency’s own recognition that inadequate reimbursement can directly impact a therapy’s availability in the marketplace.	15
V. Participation: Proposed Participants, Selected Geographic Areas, and Sampling. CMS’s approach to provider participation in the Proposed Rule will limit patient access to needed medicines on the basis of where they live, ignoring what is most appropriate given each patient’s individual clinical circumstances.	15
A. PCSAs are not an accurate reflection of the distribution of specialists in a given region.....	16
B. PCSAs disadvantage patients who reside in rural settings.	17
C. The use of PCSAs as the unit of measure for provider randomization may incentivize shifts in care settings that are based solely on the arm into which a specific practice location is placed.....	18
D. The utilization of PCSAs introduces biases that may not be able to be resolved by CMS’s proposed stratified randomization.....	19
E. CMS has not established any opportunities to beta test the complicated data system that the use of PCSAs will require.....	20
VI. Payment Methodology: Phase I. Phase I of the Proposed Rule threatens providers’ ability to obtain Part B therapies, and therefore threatens patient access to these needed therapies, negatively impacting patient health outcomes and potentially increasing overall Medicare program expenditures.	20
A. Phase I of the Proposed Rule inappropriately focuses on a single aspect of patient care, rather than promoting a holistic approach to efficient, effective care, as envisioned by CMMI’s authorizing statute.	21
B. Phase I of the Proposed Rule will undermine, not achieve, CMS’s stated goals as well as certain aims of the Administration.	22
1. CMS’s Phase I proposal threatens providers’ ability to obtain Part B therapies and may drive the existing trend of shifting the site of care away from the community setting, increasing overall expenditures for patients and the Medicare program.	23
2. By threatening providers’ ability to obtain Part B therapies, CMS’s Phase I proposal endangers patient access to needed therapies.	26
3. CMS’s Phase I proposal undermines the Administration’s longer-term goals to speed innovative therapies to market.	27
C. CMS’s Phase I proposals will create arbitrary “winners” and “losers.”	28
D. CMS’s Phase I proposal lacks important procedural protections to ensure patients are able to gain access to the most appropriate therapies for them.	29
VII. Payment Methodology: Phase II.....	30
A. Introduction.	30
1. Any experimentation with value-based arrangements must involve only voluntary arrangements of limited scope beginning in the private sector.	31

2. CMS—and its sister agencies within HHS—must work with stakeholders to establish a safe and predictable regulatory environment to enable these models to succeed in the private market.	32
3. Any experimentation with value-based arrangements should occur via a thoughtful, transparent, and collaborative process, and should start by defining what constitutes a “value-based arrangement” in the first instance.	33
B. “Value-Based” Strategies. Not all of the strategies described in the proposed rule are “value-based” and none are ready for widespread implementation.	36
1. Reference pricing is not a value-based purchasing strategy.	36
2. BIO members are concerned with aspects of CMS’s “indications-based pricing” proposal, which appears more like LCA than a true indications-based arrangement.	39
3. . Numerous considerations remain with respect to outcomes-based risk-sharing arrangements.	43
4. Any Patient Cost Sharing Models Must Ensure that Patients Have Equal Access to Medicines that Truly Deliver the Greatest Value to Them.	45
VIII. Payment Methodology: Development of a Clinical Decision Support Tool. BIO Has Serious Concerns Regarding CMS’s Proposal to Develop a Clinical Decision Support Tool.	46
IX. Provider, Supplier, and Beneficiary Protections: The proposed Payment Exceptions Review process is not sufficient to ensure that patients are able to access the medicines that are most appropriate for their individual clinical circumstances.	47
X. Proposed Waivers of Medicare Program Rules. BIO has serious legal questions regarding CMS’s proposed “waivers.”	48
A. CMS’s proposed “Waiver” of SSA § 1847A(b)(1) cannot stand as it either exceeds CMMI’s statutory authority or the statute risks vulnerability on constitutional grounds should it be viewed as unlawfully permitting a unilateral abrogation of duly enacted statutory text by the executive branch contrary to Art. I, §7.	49
B. Given the many open questions regarding the structure and operation of models under Phase II of the Proposed Rule, CMS’s remaining waiver proposals are overbroad and premature and thus provide insufficient notice to enable public comment.	53
XI. Evaluation. CMS does not establish a sufficiently detailed evaluation framework under which the Agency can identify the impact of the Proposed Rule on providers and patients, and fails to address certain requirements for patient protection.	54
XII. Regulatory Impact Analysis. The Regulatory Impact Analysis for the Proposed Rule is not sufficiently detailed to reflect its potential effects on providers and patients.	56
XIII. Conclusion.	57

III. The Proposed Rule is inconsistent with the statutory requirements for a CMMI “Phase I” test, including the requirement to consult with stakeholders.

While the following sections of this letter describe BIO’s response to each of the provisions of the Proposed Rule, as a threshold matter, we would like to express our serious concern that the Proposed Rule is inconsistent with a number of requirements set forth in Section 1115A of the Social Security Act (SSA)—CMMI’s authorizing statute. Specifically, we raise serious concerns with the fact that the Proposed Rule: (1) does not target “a defined population” for which evidence demonstrates there are existing “deficits in care;” (2) is not a true “test” as described by statute; and (3) was developed in the absence of the robust stakeholder consultation that the statute requires. Each of these issues is described in greater detail in the subsections below. We describe the legal issues with CMS’s proposed exercise of CMMI’s waiver authority in [section X](#), below.

At the outset, we would also like to clarify that while CMS uses the terms “Phase I” and “Phase II” to refer to the two parts of the Proposed Rule—proposed modifications to the ASP add-on percentage for Part B drugs, and applying value-based purchasing tools, respectively—section 1115A of the SSA uses the term “Phase I” to refer to the model testing phase and the term “Phase II” to refer to model expansion. Throughout the letter, we have referenced the statutory “Phases” in quotation marks, and the current proposal’s phases without quotations, throughout this letter in an effort to help avoid further confusion.

A. The Proposed Rule is not limited to a “defined population,” and CMS has not produced evidence that the diverse patient population treated by Part B therapies suffers from “deficits in care leading to poor clinical outcomes or potentially avoidable expenditures.”

Section 1115A explicitly requires that a model selected for testing by CMMI: (1) address a “defined population”; (2) that this population suffer from “deficits in care”; and (3) that these care deficits “lead[] to poor clinical outcomes or potentially avoidable expenditures.”¹⁰ However, the Proposed Rule presented no evidence to this effect, and thus, does not satisfy these baseline requirements. In fact, this statutory limitation was not even referenced. We address each of these three statutory requirements, in turn.

First, rather than applying to a “defined population,” as the statute requires, the Proposed Rule would apply broadly to beneficiaries administered virtually any Part B therapy—which, collectively treat a broad range of diseases and conditions—by providers located across a staggering 75 percent of the country.

Second, the Proposed Rule fails to identify a unifying “deficit in care” across this extraordinarily broad patient population. While CMS notes that “[t]he ASP methodology may encourage the use of more expensive drugs because the 6 percent add-on generates more revenue for more expensive drugs,”¹¹ the Agency has not identified robust evidence

¹⁰ SSA § 1115A(b).

¹¹ 81 Fed. Reg. at 13,231.

that supports a link between the current reimbursement methodology and inappropriate prescriber behavior with regard to prescription drugs, let alone that the current reimbursement methodology is associated with any “deficits in care.”

In fact, there is evidence to suggest that the ASP methodology does not drive provider decision-making. For example, in a 2015 study published in *Health Affairs*, researchers found that, when Medicare providers were confronted with two therapies of similar efficacy, providers prescribed the **less** expensive therapy more frequently, “despite financial incentives for physicians to prescribe the more expensive agent.”¹² The study observed prescribing patterns from 2005 to 2011 and found this to be a relatively consistent trend. Additionally, the study compared Medicare providers’ prescribing patterns for these two therapies to prescribing patterns over the same time period by providers for the Department of Veterans Affairs (VA): the latter prescribed both therapies with similar frequencies, while the more expensive therapy enjoyed greater relative use among VA patients. As the researchers noted, this finding was significant since “[i]n the VA, neither patients nor physicians face financial incentives to choose one drug over the other.”¹³ BIO urges CMS to consider this evidence, which is a rebuke of CMS’s belief that the current Part B reimbursement methodology provides perverse incentives.

Third, the Proposed Rule fails to articulate how the current ASP-based reimbursement methodology in any way “lead[s] to poor clinical outcomes or potentially avoidable expenditures.” Moreover, BIO is deeply concerned that CMS does not address the existence of evidence to the contrary, which demonstrates that the ASP system is meeting its original goal of cost-containment, while also facilitating patient access to the most appropriate therapies. After the implementation of the ASP methodology, the Medicare Payment Advisory Commission (MedPAC) noted that “[a]s intended by the policy, payment rates for drugs were reduced to levels closer to provider purchase prices....”¹⁴ In fact, as discussed in more detail later in this letter ([see section VI.B.1](#)), MedPAC has since noted that, even at ASP+6%, certain providers are not able to obtain all Part B therapies at or below the Medicare reimbursement rate. The establishment of the ASP methodology also has helped to temper growth in Part B spending compared to the overall growth in healthcare spending: A 2015 Moran analysis that compared volume-weighted ASP to the Consumer Price Index for medical care (CPI-M) by quarter, from 2006 through the third quarter of 2014, found that while CPI-M has gradually been increasing from 2006 to the present, the volume-weighted ASP has maintained a flatter line.¹⁵

As a threshold matter, we note that CMS cannot proceed without meeting these clear statutory requirements. BIO also raises serious concerns with the prudence of proposing changes to policy in the absence of robust, substantiating evidence to support the premise on which such policy proposals are made. We instead strongly recommend that CMS consider the advantages of waiting for the evidence generated by existing and pending Medicare reform efforts that could provide valuable insights into how best to ensure the

¹² S. Pershing et al. (2015). [Supra](#) note 8 at 234.

¹³ [Id.](#) at 235.

¹⁴ MedPAC, Report to Congress: Impact of Changes in Medicare Payments for Part B Drugs (2007), *available at* http://www.medpac.gov/documents/jan07_partb_mandated_report.pdf.

¹⁵ The Moran Company Analysis for BIO (2015). [Supra](#) note 7.

appropriate use of needed medicines. For example, results from the Oncology Care Model (OCM)—which maintains the existing reimbursement rate for Part B therapies but offers providers shared-savings if quality- and cost-based benchmarks are met or exceeded—will provide evidence that is directly relevant to the issue of whether, and how, providers' reimbursement can be reformed to more closely align with the value of the care they deliver. Results from the OCM also may offer important insights into the role that the appropriate utilization of therapies can have in the efficient, effective care that a patient with a complex disease receives. After all, these medicines, while critical to patient care, are often only part of a broader treatment regimen that can span months or years in the case of patients with rheumatoid arthritis, Crohn's disease, cancer, or rare diseases (e.g., lysosomal storage disorders, hemophilia, and multiple sclerosis). CMS, by rushing to propose and implement the Proposed Rule, in particular Phase I, ignores the real benefits of learning from existing and soon-to-begin demonstration programs in the Medicare population.

In sum, CMS has not articulated the evidence required for the development of a CMMI demonstration (i.e., "that the model addresses a defined population for which there are deficits in care leading to poor clinical outcomes or potentially avoidable expenditures"), nor has the Agency articulated evidence to support its theories that ASP+6% drives inappropriate prescribing behavior or to counter the contrary evidence. As a result, the model described in the Proposed Rule is not a proper "Phase I" test. If, on the other hand, the Agency did consider evidence to this effect and did not, for some reason, articulate it in the Proposed Rule, the rule itself would be improper for failing to disclose this information to the public.¹⁶

B. The Proposed Rule outlines a large-scale program change, as opposed to a true "test."

As the Agency is no doubt aware, section 1115A establishes a two-phase process for the development of new care-delivery models. Specifically, the statute requires that the Agency begin its consideration of models through "Phase I" testing. Only those models that are tested, evaluated, and found to meet certain statutory criteria can then be considered for "Phase II" expansion. We are extremely concerned that the purported demonstration outlined in the Proposed Rule bypasses the carefully crafted pathway for testing models established by section 1115A, and instead rushes to implement a new, wholly untested model on a nationwide basis that has the very real risk of restricting patient access to care.

Our concern is based largely on the fact that the Proposed Rule would immediately apply to virtually all providers across the entire country— a scope that seems more in line with the model expansion phase (i.e., "Phase II") of a CMMI demonstration. Yet, CMS has not met any of the criteria for model expansion, first and foremost because a pilot with a limited scope and duration has not been conducted, and thus, necessarily, the Secretary cannot make the statutorily required determination that the pilot: (1) reduces spending without reducing quality of care or improves quality of care without increasing spending; (2)

¹⁶ Meaningful comment on proposed rules may be precluded by an agency's failure to disclose especially relevant information. It is a longstanding principle of administrative law that "[i]t is not consonant with the purpose of a rule-making proceeding to promulgate rules on the basis of inadequate data, or on data that, critical degree [sic], is known only to the agency." Portland Cement Ass'n v. Ruckelshaus, 486 F.2d 375 (D.C. Cir. 1973).

the CMS Actuary certifies that expansion would reduce or not result in an increase in net program spending; and (3) if expanded, would not deny or limit the coverage or provision of benefits.¹⁷ Absent meeting these criteria, an initial demonstration that includes almost all Part B providers is inconsistent with CMMI's authority to conduct such demonstrations in the first place.

In sum, "Phase I" testing requires an actual test—and an evaluation thereof—before the model can be expanded under "Phase II." Yet, while CMS identifies improvements in the quality of patient care as a primary goal of the Proposed Rule, the Agency has yet to test the impact of the model on patient care, and—as discussed in greater detail in [section XI](#), below—the Proposed Rule also lacks a well-defined structure to accurately and reliably evaluate quality of care going forward.

Any such efforts to **launch** models on a nationwide, or near-nationwide, basis are inconsistent with section 1115A's two-phase process, and render it impossible for the Agency to comply with the statutory obligation to ensure that a proposed model does not reduce the quality of beneficiaries' care, restrict their access to benefits, or increase Medicare spending **before** it is expanded. Indeed, in its evaluation of CMMI's Medicare-Medicaid dual eligible models, MedPAC noted that "the large scope . . . makes the demonstrations appear to be large-scale program changes rather than true demonstrations."¹⁸ This is particularly notable given that the dual-eligible models are state-specific and could have covered a maximum of only one-third of all dual eligibles (who were then given the opportunity to opt out). Applying this same logic here, there is no way that a model that covers nearly all Part B drugs and mandates participation by virtually all Part B providers across three-quarters of the country could be considered a "test."

Courts examining demonstration proposals under other Medicare or Medicaid demonstration authorities have similarly emphasized the importance of testing new care models with a limited scope. For example, a court reviewing the Medicare Cataract Demonstration project highlighted the project's limited scope in determining that it was a true experiment permitted by the applicable demonstration authority.¹⁹ Among other things, the court highlighted that the program was limited in location to only three geographic areas and that "patient as well as health care provider participation is strictly voluntary."²⁰ According to the court, "Congress has provided the Secretary with the authority to develop experiments in an effort to increase Medicare's efficiency, and the

¹⁷ SSA § 1115A(c)(1)-(3).

¹⁸ MedPAC, Report to the Congress: Medicare and the Health Care Delivery System at 64 (June 2012), *available at* http://www.medpac.gov/documents/reports/jun12_entirereport.pdf. *See also, e.g.*, MedPAC Staff Report, CMS Financial Alignment Demonstration for Dual Eligible Beneficiaries at 6 (Sept. 13, 2013), *available at* <http://www.medpac.gov/documents/april-2016-meeting-presentation-status-report-on-cms-s-financial-alignment-demonstration-for-dual-eligible-beneficiaries.pdf?sfvrsn=0> ("We'll start with the scope of the demonstration. Most of the 26 state proposals included enrollment of the majority, or entire subgroups of dual eligibles in the state into the demonstration. The Commission commented that the scope of the demonstration was too broad and represented a program change because approximately 3 million dual eligible would be enrolled in the demonstration if CMS approved every state's proposal.").

¹⁹ *Am. Acad. Of Ophthalmology, Inc. v. Sullivan*, 998 F.2d 377, 384 (6th Cir. 1993) (emphasis added).

²⁰ *Id.*

Cataract Demonstration is just such an experiment, **limited in duration and location, as well as being vastly restricted in scope and application.**²¹

That CMMI intends to apply the proposed Part B Drug Payment Model on a broad, nationwide scale necessitates additional scrutiny into whether the above considerations are met by the Proposed Rule. We do not believe that, in granting CMMI with the demonstration and waiver authorities under section 1115A, Congress intended to create, in essence, a legislative body within CMS. Nor did Congress intend for CMMI to disregard the clear statutory requirements for the selection, testing, and expansion of demonstration programs. To the contrary, Congress provided specific criteria, and required each of these elements to be met—without exception—before CMMI undertook a new demonstration or considered expanding an existing one.

CMMI's authority to launch or expand a demonstration is conditioned on meeting these criteria, and the Proposed Rule's effort to impose large-scale program change, as opposed to a true "test," is therefore inconsistent with this authority. We find this deficiency to be particularly troubling given that the statute prevents model expansion unless CMS determines that the model will **not** result in an access challenge,²² yet evidence articulated throughout this letter shows that the Proposed Rule inherently presents risks to quality of care by threatening patient access to critical Part B therapies, which are indicated to treat a range of serious conditions, including rheumatoid arthritis, Crohn's disease, cancer, and rare diseases (e.g., lysosomal storage disorders, hemophilia, and multiple sclerosis). Moreover, as described in further detail in [section X](#), below, CMS's proposed waivers present serious legal issues.

C. The Proposed Rule was developed and released without the stakeholder engagement necessary for thoughtful model development, in stark contrast to CMMI's other demonstrations and the statutory requirement for stakeholder consultation.

The Agency's process also was out of alignment with the statutory requirements that CMMI "consult . . . clinical and analytical experts with expertise in medicine and health care management" and "use open door forums or other mechanisms to seek input from interested parties."²³ The statutory requirements underscore the need for broad engagement as the foundation for responsible public policy development.

BIO has expressed concerns in the past with CMMI's process for identifying the topics and structure of potential demonstrations, and the majority of these comments have urged the Agency to provide a more predictable, formal process for obtaining stakeholder input. However, in this instance, CMS forewent even the basic format for stakeholder engagement used to develop previous models, like the OCM, the Medicare Value-based Insurance Design, and Medication Therapy Management demonstrations. Specifically, in contrast to these previous models, in which CMS engaged stakeholders months and even years leading

²¹ *Id.* (emphasis added).

²² SSA § 1115A(c)(3).

²³ SSA § 1115A(a)(3).

up to their release, the Agency appears to have developed the Proposed Rule largely in the absence of broad stakeholder input. For example, the only advanced notice that BIO had of the Proposed Rule before it was released was a contractor transmittal that was inadvertently posted, and then removed, from the contractor's website on February 5. This transmittal gave the impression that the model was fully developed and steps to implement it as proposed were underway—all before it was released for public comment. Such action is inconsistent with general principles of notice-and-comment rulemaking.²⁴

In a program as complex as Medicare Part B, where the ramifications of unintended consequences can have immediate impacts on patients' lives, it is critical that CMS establishes a process that is inclusive of, and responsive to, diverse stakeholder input. No individual organization, CMS included, has all of the information that is needed to assess the implications of policy changes, and a holistic account of multiple perspectives is more likely to result in policy development that accomplishes goals as broad as improved quality of care and reduced overall Medicare expenditures. The Proposed Rule was shortchanged of this perspective, to its detriment and that of the stakeholders who would be most affected by it.

In fact, a diverse group of stakeholders—primarily representing patient and provider advocacy organizations—expressed immediate and significant concern with the Proposed Rule based, in part, on the lack of stakeholder engagement to develop it in the first place.²⁵ Moreover, in considering both the lack of process and the extremely short timeframe proposed for implementation, questions have emerged with regard to the motivations for the Proposed Rule. This concern, together with the Agency's noncompliance with the statutory consultation requirements, further underscores the need to withdraw the Proposed Rule, and to develop any future opportunities for Part B changes, including any changes applicable to prescription drugs, through an inclusive, evidence-based process.

²⁴ The Administrative Procedure Act (APA) requires that an agency genuinely consider the public feedback solicited through notice-and-comment rulemaking. "Consideration of comments as a matter of grace is not enough. It must be made with a mind that is open to persuasion." See *Advocates for Hwy. & Auto Safety v. Fed. Hwy. Admin.*, 849 F.2d 1288, 1292 (D.C. Cir. 1994) (internal citations omitted). This requirement helps ensure that agency decision-making is better informed and that the public perceives the participatory process as credible. See JAMES L. CREIGHTON, *THE PUBLIC PARTICIPATION HANDBOOK: MAKING BETTER DECISIONS THROUGH CITIZEN INVOLVEMENT* at 11, 41 (2005) ("A bogus participatory process destroys the credibility of all future attempts to provide genuine participation on other issues If the agency has already made a decision, public participation is a sham.") See also *ADMINISTRATIVE LAW AND PRACTICE* (3D ED.), § 4:41 (2010) (describing the general prohibition on "sham" rulemaking).

²⁵ Letter from diverse stakeholder groups to the Hon. Mitch McConnell, Majority Leader, U.S. Senate, the Hon. Larry Reid, Minority Leader, U.S. Senate, the Hon. Paul Ryan, Speaker of the U.S. House of Representatives, and the Hon. Nancy Pelosi, Minority Leader, U.S. House of Representatives (Mar. 17, 2016), *available at* <http://blog2.communityoncology.org/userfiles/76/file/0-67463100-1458227275.pdf>.

IV. Participation: Proposed Drugs Paid under Part B to be included in the Demonstration. CMS's proposal to include all Part B drugs, innovator biologicals, and biosimilars will threaten patient access to these vital therapies.

A. BIO recommends that CMS consider the significance of how biologicals are used to treat patients with some of the most complex, chronic conditions, including rheumatoid arthritis, Crohn's disease, cancer, and rare diseases (e.g., lysosomal storage disorders, hemophilia, and multiple sclerosis).

In the Proposed Rule, CMS proposes to include almost all Part B products, including yet-to-be-launched biosimilars, with the intention of "achiev[ing] savings through behavioral responses to the revised pricing, as we hope that the revised pricing will remove any excess financial incentive to prescribe high cost drugs over lower cost ones when comparable low cost drugs are available."²⁶ First and foremost, CMS does not support this statement with evidence. Rather, such statements are founded on the false premise that providers' prescribing decisions are primarily influenced by the ASP methodology, when in reality, these decisions are guided by the individual clinical circumstances of their patients, the providers' expertise and experience, and their medical training, as we describe in [section III.A](#), above.

Second, in a press release accompanying the release of the Proposed Rule, the Agency noted that a primary purpose of the Proposed Rule is to "drive the prescribing of the most effective drugs."²⁷ Yet the incentives the Proposed Rule will establish will undermine this goal, not achieve it: by limiting providers' ability to acquire Part B therapies at or below the Medicare reimbursement rate, the Proposed Rule will limit patient access to these therapies, regardless of clinical appropriateness. Moreover, the statements that the Agency has made about the Proposed Rule and in the preamble, considered together, seem to imply that CMS believes there is a significant degree of equivalence between Part B therapies, such that patients can easily be switched between such therapies. This implication is very concerning as it illustrates a fundamental misunderstanding of the role of Part B-covered therapies—many of which are biologicals—in the treatment of patients with complex, chronic conditions, such as rheumatoid arthritis, Crohn's disease, cancer, and rare diseases (e.g., lysosomal storage disorders, hemophilia, multiple sclerosis), as well as other serious conditions (e.g., age-related macular degeneration).

Patients have highly individualized responses to biologicals because they are complex molecules, or mixtures of molecules, manufactured in living systems. The complexity of biologicals is precisely what can allow them to be so effective against diseases that evolve as they interact with the body's own immune system. Congress recognized the differences between even reference biologics and their biosimilars in enacting the biosimilars pathway under the ACA, as evidenced by their creation of a reimbursement methodology under

²⁶ 81 Fed. Reg. at 13,233.

²⁷ Press Release, CMS, CMS proposes to test new Medicare Part B prescription drug models to improve quality of care and deliver better value for Medicare beneficiaries (Mar. 8, 2016), <https://www.cms.gov/Newsroom/MediaReleaseDatabase/Press-releases/2016-Press-releases-items/2016-03-08.html>.

which each biological product (whether biosimilar or reference biological) has the same dollar markup over that product's ASP. We also note that six Part B therapies have FDA-approved companion diagnostics, underscoring the personalized medicine approach to developing medicines to treat these serious conditions—an approach Congress and the Administration, including CMS, readily supports.

Even in instances in which two biologicals may have the same clinical indication(s), switching a patient from one to the other can negatively impact health outcomes by, for example, increasing negative side-effects and the number of episodes/flare-ups a patient experiences after such a switch occurs.²⁸ In fact, a recently published literature review on the issue of non-medical switching found that “[n]on-medical switching was more often associated with negative or neutral effects than positive effects on an array of important outcomes. Among patients with stable/well-controlled disease, non-medical switching was associated with mostly negative effects.”²⁹ Thus, non-medical switching can lead to increased consumption of healthcare resources, such as increased physician office visits and hospitalizations, which, in turn, can increase overall Medicare costs, undermining CMS's rationale for proposing this strategy.

Additionally, by making it more difficult for providers to obtain Part B therapies at or below Medicare reimbursement, the Proposed Rule could incentivize the use of compounded/repackaged off-label therapies over FDA-approved therapies, to the detriment of patient safety. This issue is discussed in more detail later in this letter ([see section VII.B.1](#)). Even more importantly, patients do not always have an alternative therapy to which they can be switched. In particular, in the case of therapies that treat rare diseases (e.g., lysosomal storage disorders), there is frequently only one therapy indicated to treat the disease, and the loss of access to that therapy (or a delay in access) leaves these vulnerable patients without access to any approved treatment at all.

By ignoring the fundamental realities of the flexibility that patients and their providers need to choose the most appropriate Part B therapy for them, the Proposed Rule threatens patient access to these therapies and does so on the basis of geographical factors, putting certain patients' health at risk based on where their provider practices (discussed in greater detail in [section V](#), below).

²⁸ See D.T. Rubin, M. Skup, S.J. Johnson, J. Chao, and A. Gibofsky, Abstract Presentation at the Digestive Disease Week® Gastroenterology Conference, Washington, D.C.: Tu1305 Analysis of Outcomes After Non-Medical Switching of Anti-Tumor Necrosis Factor Agents (2015). See also Global Health Living Foundation, Patient Perspectives on Medication Switching for Non-Medical Reasons (2015), available at http://www.50statenetwork.org/wp-content/uploads/2015/04/GHLF-Switching-Stable-Patients-Survey_Summary.pdf.

²⁹ E. Nguyen et al, *Impact of non-medical switching on clinical and economic outcomes, resource utilization and medication-taking behavior: a systematic literature review*, 1 CURRENT MED. RES. & OPINIONS 1-10 (2016) (publication ahead of print).

B. CMS proposes to include almost all Part B therapies in the Proposed Rule—subjecting them to significantly lower reimbursement rates—despite the Agency’s own recognition that inadequate reimbursement can directly impact a therapy’s availability in the marketplace.

In the Proposed Rule, CMS’s proposal to continue to pay for drugs in shortage at the statutory default reimbursement rate is a clear recognition that adequate reimbursement plays an important role in ensuring patient access to needed medicines.³⁰ Drug shortages can have a serious and significantly negative impact on patients, threatening their health if they are unable to obtain access to the therapies they need, or requiring them to switch to therapies that are not in shortage but may be less effective—and sometimes more expensive—or have more negative side effects.³¹ Though the causes of drug shortages are often multidimensional, inadequate reimbursement can contribute, by incentivizing: (1) increasingly fewer manufacturers to produce a specific therapy (in the case of multi-source therapies), which can lead to shortage if one of the few (or the only) manufacturers in the market has an unexpected manufacturing issue; and/or (2) very tight tailoring of supply to match current demand, which can lead to shortages if there is a sudden increase in demand. Recent history has demonstrated that regulators’ reaction to drug shortages, if not carefully calculated, can actually worsen their impact and lengthen their duration.³² In a 2013 article in the *Journal of Clinical Pharmacy and Therapeutics*, FDA’s Janet Woodcock and Marta Wosinska identified perverse market dynamics as largely responsible for shortages in generic sterile injectable drugs. Yet, notwithstanding these realities and the Agency’s own recognition of the link between inadequate reimbursement and drug shortage, CMS chose to press ahead with its Proposed Rule, seeming to look past the potential negative implications for patients (i.e., delaying or effectively denying patient access to medicines in shortage).

V. Participation: Proposed Participants, Selected Geographic Areas, and Sampling. CMS’s approach to provider participation in the Proposed Rule will limit patient access to needed medicines on the basis of where they live, ignoring what is most appropriate given each patient’s individual clinical circumstances.

As described above, given the lack of evidence supporting the need for this demonstration, BIO is concerned that CMS did not consider piloting a study to determine what, if any, impact the statutory Part B reimbursement rate has on provider behavior and what solutions might be most appropriate to address any such issues that were identified. This general approach is one that the Agency has employed in the past: for example, for the Comprehensive Primary Care-Plus model, CMMI initially studied 500 practices for four years

³⁰ See 81 Fed Reg. at 13,236.

³¹ See, e.g., M.L. Metzger, A. Billett & M. P. Link, *Perspective: The Impact of Drug Shortages on Children with Cancer—The Example of Mechlorethamine*, 367 NEW ENG. J. OF MED. 2461-2463 (2012). See also M. Lenders et al, *Patients with Fabry Disease after Enzyme Replacement Therapy Dose Reduction and Switch-2-Year Follow-Up*, 27 J. OF THE AM. SOC. OF NEPHROLOGY 952-962 (2016).

³² See, e.g., P. Loftus, *U.S. Drug Shortages Frustrate Doctors, Patients*, WALL ST. J., May 31, 2015, available at <http://www.wsj.com/articles/u-s-drug-shortages-frustrate-doctors-patients-1433125793>. See also M. Koba, *The U.S. has a drug shortage -- and people are dying*, FORBES, Jan. 5, 2016, available at <http://fortune.com/2015/01/06/the-u-s-has-a-drug-shortage-and-people-are-dying/>.

before proposing to scale-up the demonstration to 5,000 practices.³³ Instead, for the Proposed Rule, the Agency proposes to mandate participation by the vast majority of Part B providers from the start. CMS has a trove of data available to analyze the impact of various policies on provider and patient access, and should have engaged stakeholders where existing Agency data were insufficient to do so (for example, discussions directly with patients/collected from patient surveys would have provided valuable information on patient experiences with access to care).

In addition, given the potential for significant disruptions in patient care, CMS should have proposed to pilot any changes to Part B payment for drugs in an environment that could be studied carefully, and in the absence of confounding variables. This would have allowed the Agency to understand the impact of such changes on prescribing behavior, and on patient access to needed therapies. For this reason, BIO has concerns regarding CMS's selection of PCSAs as the primary unit of measure for provider randomization, which we believe will disproportionately threaten access to needed medicines for those patients who live in rural areas and/or receive care in community settings. Similarly the use of PCSAs will not yield clear data on any changes in practice patterns that occur in response to the Proposed Rule.

A. PCSAs are not an accurate reflection of the distribution of specialists in a given region.

BIO agrees with the criticisms that CMS makes with regard to calculating participation at the level of the state, Core Based Statistical Area (CBSA), Dartmouth Atlas of Health Care's Hospital Referral Regions (HRR), or zip code. However, we believe there are equally significant concerns with utilizing PCSAs as the unit of participation. PCSAs are defined "based upon patterns of Medicare Part B primary care services (specifically, patterns linking the residence of Medicare Part B beneficiaries with the practice locations for evaluation and management visits to Medicare participating physicians in primary care specialties)."³⁴ Yet, CMS does not address the likelihood that there are meaningful differences in the patterns linking the residence of patients with primary care offices and those linking the residence of patients with specialists offices. This is concerning given that Part B drugs are more often prescribed by specialists, and thus, the Proposed Rule will significantly impact these provider types, especially those who treat patients with complex, chronic conditions.

We ask the Agency to consider the following example: we compared the availability of specialists in Kentucky, a state that is 41% rural, and Massachusetts, a state that is 8% rural.³⁵ These states are divided into a similar number of PCSAs (approximately 163 and

³³ See CMS, *Comprehensive Primary Care Initiative* (2016), available at: <https://innovation.cms.gov/initiatives/comprehensive-primary-care-initiative/> (providing additional information about this model).

³⁴ 81 Fed. Reg. at 13,238.

³⁵ We conducted this analysis using the "Lists of Population, Land Area, and Percent Urban and Rural in 2010 and Changes from 2000 to 2010" and "Percent urban and rural in 2010 by state," both published by the U.S. Census Bureau. See *2010 Census Urban and Rural Classification and Urban Area Criteria*, U.S. CENSUS BUREAU, <https://www.census.gov/geo/reference/ua/urban-rural-2010.html> (last visited Mar. 22, 2016) (analysis on file at BIO).

158, respectively), yet the number of practicing specialists varies dramatically.³⁶ This example underscores that, while PCSAs are designed to consider adequate access to primary care providers, they are not a classification that takes into account access to specialists, nor do they account for the geographical differences in access to specialists. In fact, in Kentucky, over 44% of the PCSAs have three or fewer practicing specialists, while in Massachusetts, only 18% of PCSAs have three or fewer specialists.³⁷ This is a stark contrast that is not captured by the PCSA metric, yet could significantly affect CMS's evaluation of the impact of the Proposed Rule. As another example, PCSAs do not capture patients' access to hemophilia treatment centers (HTC), which provide highly specialized and effective care for patients with this condition. According to the Centers for Disease Control and Prevention (CDC), there are 23 states with only one HTC and five others with only two such centers in the entire state. It follows that the great majority of PCSAs in these 28 states do not include an HTC.³⁸ The concerns engendered by these two examples are compounded by the fact that the term "specialist" is used to describe any medical specialty, from oncologist to gastroenterologist, and thus the presence of one or more specialists may not be an adequate proxy for patient access to appropriate care.³⁹

B. PCSAs disadvantage patients who reside in rural settings.

BIO also has significant concerns that the use of PCSAs as the unit of measure will disadvantage providers and patients in rural areas of the country, simply based on where they live. For example, generally speaking, the more rural a state, the fewer the PCSAs it will be allotted, and the fewer specialists in each PCSA.

We ask the Agency to consider the example of a patient in a rural area whose provider is unable to obtain a needed Part B therapy at cost under the study arm into which the provider has been randomized, or chooses to forego doing so based on a determination that the potential financial loss is too high of a business risk to the practice. In this circumstance, the patient will be required to travel **much** farther to obtain care—potentially delaying or effectively denying that patient care in the end—than a similar patient who lives in an urban area, who may only need to travel a few miles to find another provider. For example, a study by the American College of Rheumatology found that, in smaller "micropolitan" areas (defined as populations of at least 40,000), the closest practicing rheumatologist was more than 200 miles away.⁴⁰ We ask the Agency to consider also the

³⁶ This is an **estimate** of the number of PCSAs in each state, respectively, because BIO realizes that the dataset we used may not correspond exactly to the data used by CMS (e.g., the Agency may be using a slightly updated list of PCSAs by state, and thus, our report of the number of PCSAs by state may deviate from a CMS estimate by a small number).

³⁷ This conclusion is based on a BIO analysis of The Dartmouth Atlas of Health Care. *See PCSA Data Download – 2010 (Census Tract Basis)*, DARTMOUTH ATLAS OF HEALTHCARE, <http://www.dartmouthatlas.org/Tools/Downloads.aspx?tab=42> (last visited Mar. 22, 2016) (Dataset: PCSA v3.1 layer attributes (File 4), Variables PG_DOC to PH65_UP) (analysis on file at BIO).

³⁸ We conducted this analysis using the "Hemophilia Treatment Center (HTC) Directory," published by the U.S. Centers for Disease Control and Prevention. *See Hemophilia Treatment Center (HTC) Directory*, U.S. CENTERS FOR DISEASE CONTROL AND PREVENTION, https://www2a.cdc.gov/ncbddd/htcweb/Dir_Report/Dir_Search.asp (last visited May 6, 2016) (analysis on file at BIO).

³⁹ Additionally, we note that these analyses did not account for whether a specific physician type accepted Medicare patients (and new Medicare patients), a factor that could have a significant impact on patient access.

⁴⁰ Am. Col. of Rheumatology, *Regional Distribution of Adult Rheumatologists*, 65 ARTHRITIS & RHEUMATISM 3017-3025 (2013).

example of a patient with a mental health condition who has spent time cultivating a relationship with a specific provider: disruptions in access to that provider, especially for a patient residing in a rural setting, can have meaningful and serious repercussions for the patients' short- and long-term health outcomes.

Beyond simply creating access disruptions for beneficiaries in these geographies, the proposal also does not address how CMS intends to capture access data and correct for geographic variances that might explain differences in utilization patterns and overall costs between, and within, the test and control arms.

C. The use of PCSAs as the unit of measure for provider randomization may incentivize shifts in care settings that are based solely on the arm into which a specific practice location is placed.

In the Proposed Rule, CMS notes that the Agency "analyzed CY 2014 claims data . . . and observed that almost all claims for an individual provider or supplier were billed within a single PCSA."⁴¹ However, it is unclear if this is true across provider practices. An analysis of existing PCSAs demonstrates that there are several PCSAs within individual cities, making it more likely that a large provider practice, with several different locations, may be subject to more than one arm of the demonstration (e.g., there are approximately 9 PCSAs in Boston, 34 that cover Philadelphia, and 8 in Washington, D.C., alone).⁴² Not only could this result in an undue administrative burden on a given practice, but it can drive patients to care settings based on the study arm in which the practice participates, despite the potential for longer travel times/distances for patients, potentially contributing to diminished adherence to treatment, in turn, compromising patient health outcomes. Further, this can create challenges to managing leakage between the study and control arms.

To illustrate, we ask the Agency to consider the example of a large Accountable Care Organization, such as a Medicare Shared Savings Program (MSSP) participant. CMS proposes to include all participants in ongoing demonstrations in the Proposed Rule. Thus, MSSP participants—which likely include multiple sites of care to achieve the goal of integrated, coordinated patient care—can be required to participate in several different study arms and/or the control arm, introducing a perverse incentive with regard to the sites of care at which providers treat patients (e.g., patients may be encouraged to seek care at the locations that participate in the control arm of the demonstration, potentially creating delays in care because of the increased patient volume in only certain parts of the Accountable Care Organization).

As a second example, we ask CMS to consider an independent analysis that shows that, of those PCSAs with hematology/oncology providers, 81% also have hospital outpatient departments.⁴³ Shifts in care—which have repercussions for Medicare and patient

⁴¹ 81 Fed. Reg. at 13,238.

⁴² This conclusion is based on a BIO analysis of PCSA data. *See supra* note 37.

⁴³ This conclusion is based on BIO analysis of HRSA's Primary Care Service Area Data Download—2010 (Census Tract Basis), CMS's Hospital Outpatient Prospective Payment System data, and CMS's Provider Utilization and Payment data. *See Primary Care Service Area Data Download—2010 (Census Tract Basis)*, HEALTH RESOURCES AND SERVICES ADMINISTRATION, <http://datawarehouse.hrsa.gov/data/datadownload/pcsa2010download.aspx> (last visited Apr 13, 2016) (Dataset: 2013 Zip5 to PCSA v3.1 crosswalk, Columns E-H) (analysis on file at BIO). *See also*

spending as well as patient outcomes (described in detail in [section VI.B.1](#))—that occur within a PCSA can be extremely difficult to detect under the proposed methodology. Similarly, we also ask the Agency to consider off-campus provider-based departments that bill as part of a hospital for purposes of Medicare. Independent analysis demonstrates that 10% of PCSAs have at least one off-campus department of a disproportionate share (DSH) hospital that is located in a different PCSA.⁴⁴ Thus, in these cases, the PCSA-based model may not provide accurate information about where patients are receiving care, and how the setting of care has shifted as a result of the Proposed Rule. Moreover, off-campus sites that are located in PCSAs may share certain characteristics that would further confound CMS analyses, including that such sites could be concentrated in PCSAs with higher Part B therapy utilization. In fact, research has shown a correlation between certain DSH hospitals' acquisitions of these locations and increased overall utilization after the acquisition, attributed entirely to the hospital but resulting from utilization at the off-campus site.⁴⁵

D. The utilization of PCSAs introduces biases that may not be able to be resolved by CMS's proposed stratified randomization.

In the Proposed Rule, CMS does not address how the Agency will overcome hurdles to accounting for several sources of bias that will result from the proposed PCSA methodology. For example, CMS proposes to utilize a stratified randomization based on two criteria: number of beneficiaries furnished Part B products, and mean drug expenditures per beneficiary. However, the cut points proposed for each strata are fairly broad: CMS proposes a single cut point of 1,500 beneficiaries per PCSA; and two cut points for mean dollars expended on drugs per-beneficiary, per-PCSA, \$500 and \$3,000. Additionally, analysis demonstrates that spending varies dramatically between PCSAs: the top 1% of PCSAs by spending account for 25% of physician spending in Part B.⁴⁶ Given the breadth of these criteria, it is unclear that this approach will guarantee an equal distribution of spending on drugs in each study arm.

Moreover, the stratified randomization will not ensure an equal distribution of provider types in each study arm, nor will it guarantee an equal distribution of providers participating in various other Medicare programs (e.g., OCM, MSSP), since it is not constructed to adjust for either of these factors. This is particularly concerning given a

Hospital Outpatient PPS: Details for title: CMS-1633-FC; CMS-1607-F2, CENTERS FOR MEDICARE AND MEDICAID SERVICES, <https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/HospitalOutpatientPPS/Hospital-Outpatient-Regulations-and-Notices-Items/CMS-1633-FC.html> (last visited Apr 13, 2016) (Dataset: 2016 OPPTS Final Rule Cancer Adjustment Analysis File, Columns I, AA, Q-Y) (analysis on file at BIO). *Medicare Provider Utilization and Payment Data: Physician and Other Supplier Data CY 2013*, CENTERS FOR MEDICARE AND MEDICAID SERVICES, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Medicare-Provider-Charge-Data/Physician-and-Other-Supplier2013.html> (last visited Apr 13, 2016) (Dataset: Medicare Physician and Out Supplier PUF, CY2013, Tab Delimited format, Columns AB-AL) (analysis on file at BIO)

⁴⁴ This conclusion is based on a BIO analysis of PCSA and other relevant data. See *supra* note 43.

⁴⁵ Berkeley Research Group, Trends in 340B Covered Entity Acquisitions of Physician-based Oncology Practices (2014), available at http://www.communityoncology.org/UserFiles/BRG_BIO_340B_Study%204-14.pdf.

⁴⁶ This finding is based on an analysis conducted by BIO using publicly available data. See *Physician and Other Supplier Data CY 2013*, CMS, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Medicare-Provider-Charge-Data/Physician-and-Other-Supplier2013.html> (last updated Oct. 15, 2015) (analysis on file at BIO). See also HRSA, *Primary Care Service Area Data Download – 2010 (Census Tract Basis)* (2013), available at <https://datawarehouse.hrsa.gov/data/datadownload/pcsas2010download.aspx> (Dataset: 2013 Zip5 to PCSA v3.1 crosswalk) (analysis on file at BIO).

recent *New England Journal of Medicine* analysis that noted that the multitude of existing Medicare demonstrations “are reaching a scale at which distortions generated by overlapping models could create real problems” with regard to evaluation efforts.⁴⁷ For purposes of the Proposed Rule, these significant confounding variables will make it very challenging to accurately interpret any analyses that evaluate changes in expenditures, quality of care, and changes in care setting that result from the Proposed Rule—assessments that CMMI is required to undertake to continue any of its demonstrations.⁴⁸ CMS’s inability to conduct such assessments given the proposed structure of Phase I further supports BIO’s strong recommendation that the Agency withdraw the Proposed Rule.

E. CMS has not established any opportunities to beta test the complicated data system that the use of PCSAs will require.

The process of associating a PCSA with the applicable payment amount based on the study (or control) arm in which it is participating is likely to require changes to current billing and claims-processing systems. In fact, the February 5 transmittal, which was posted and immediately withdrawn from CMS’s website, acknowledges this need. Despite the predictable complexity of implementing such a policy, CMS does not propose to provide a reasonable amount of time for providers to make the logistical and operational changes needed to submit claims under the Proposed Rule. Moreover, proposing to implement the provisions of the Proposed Rule in such a rushed manner has the potential to undermine CMS’s ongoing efforts to reduce waste, fraud, and abuse in the Medicare program, and could actually, and inadvertently, place many providers into a state of noncompliance without the ability to rectify this in a timely manner. Given that CMS is silent on these critical issues in the Proposed Rule, we reiterate our ardent recommendation that the Agency withdraw the Proposed Rule, and instead work with stakeholders to develop and implement demonstration programs that improve the quality of patient care in Part B in a responsible, measured, and practical manner.

VI. Payment Methodology: Phase I. Phase I of the Proposed Rule threatens providers’ ability to obtain Part B therapies, and therefore threatens patient access to these needed therapies, negatively impacting patient health outcomes and potentially increasing overall Medicare program expenditures.

BIO’s primary concern with the proposed Phase I payment methodology described in the Proposed Rule is that it will diminish Medicare providers’ ability to obtain Part B therapies—and establish perverse incentives to shift the site of patient care—and, in turn, threaten patient access to needed medicines through their preferred provider/setting. This concern alone is sufficient grounds for BIO to urge CMS, in the strongest terms, to withdraw the Proposed Rule. Yet, in addition to these concerns, BIO questions whether the Proposed Rule can meet CMS’s stated goals in putting it forward.

⁴⁷ Robert E. Mechanic, *When New Medicare Payment Systems Collide*, 374 *New Eng. J. Med.* 1706, 1709 (2016).

⁴⁸ SSA § 1115A(b)(3)(B).

Additionally, we question the Agency's focus, approach, and process for developing the Proposed Rule. In particular, BIO is very concerned that Phase I of the Proposed Rule: (1) does not focus holistically on the full spectrum of patient care and thus runs contrary to the spirit of the demonstration authority that Congress granted CMMI; (2) will undermine CMS's goals to improve the quality of patient care and decrease overall expenditures; (3) will create divergent winners and losers; and (4) lacks certain key procedural protections to ensure patients are able to obtain the therapies most appropriate for them. We address each of these concerns, in turn. As noted in [section III.C](#), above, we also are very concerned that the model was developed without input from the very stakeholders on whom it will have the greatest impact.

A. Phase I of the Proposed Rule inappropriately focuses on a single aspect of patient care, rather than promoting a holistic approach to efficient, effective care, as envisioned by CMMI's authorizing statute.

Phase I of the Proposed Rule takes a piecemeal approach to Medicare payment reform, focusing on a single aspect of a single type of treatment intervention. Moreover, considered within the context of the current structure of the Medicare program, the scope of ASP is small: payment for Part B drugs and biologicals is less than 3% of total Medicare spending.⁴⁹ In fact, for therapies with total payments per day of more than \$10,000, spending on the +6% of the payment rate is only 1.4% of total Medicare Part B spending on drugs.⁵⁰ BIO is concerned with this singular focus on cutting short-term costs since it obscures the longer-term implications on patient health outcomes and overall expenditures. Additionally, the focus of the Proposed Rule does not align with the types of models that Congress described in great detail in CMMI's authorizing statute, which target care coordination, delivery-of-care reforms, and improvements to patients' quality of care.⁵¹ The legislative history behind this provision similarly speaks broadly of "foster[ing] patient-centered care, improve[ing] quality, and slow[ing] the rates of Medicare cost growth."⁵²

Indeed, the overly narrow focus of Phase I of the Proposed Rule is particularly perplexing given that such a focus contradicts the Agency's approach to Medicare demonstrations thus far. For example, we note that the Oncology Care Model (OCM) focuses on practice transformation and the Bundled Payments for Care Improvement (BPCI) Initiative focuses on the full range of inpatient/acute care services. Given CMS's previous preference for developing demonstrations with a more holistic focus on patient care, we believe that beneficiaries will be best served by the Agency withdrawing the Proposed Rule in its entirety, and instead, engaging in an inclusive dialogue with stakeholders to identify opportunities for thoughtful, evidence-based demonstration programs in the future.

Moreover, in proposing this piecemeal approach to Part B reform, CMS appears to ignore the major payment and delivery-of-care reforms that are being developed now to

⁴⁹ MedPAC, Presentation at Sept. 10-11, 2015 Public Meeting: Medicare Drug Spending, *available at* <http://medpac.gov/documents/september-2015-meeting-presentation-medicare-drug-spending.pdf?sfvrsn=0>.

⁵⁰ The Moran Company analysis for BIO (2015). *Supra* note 7.

⁵¹ SSA § 1115A(b)(2)(B).

⁵² SEN. COMM. ON FINANCE, CHAIRMAN'S MARK, AMERICA'S HEALTHY FUTURE ACT OF 2009 (Sept. 2009), *available at* <http://www.mwe.com/info/news/ahfa0909.pdf>.

implement the Medicare Access and CHIP Authorization Act of 2015 (MACRA) by 2019, which are meant to broadly achieve the goals of increasing quality and decreasing overall expenditures. CMS does not address how these ongoing initiatives—in which many stakeholders have been participating avidly—will be impacted by, or interact with, the Proposed Rule. CMS also fails to address the concurrent timing of the proposed implementation of the Proposed Rule and the planned implementation of MACRA (given the proposed five-year timeline of the former, there will be overlap with the initial implementation efforts around the Merit-based Incentive Payment System (MIPS)).

B. Phase I of the Proposed Rule will undermine, not achieve, CMS’s stated goals, as well as certain aims of the Administration.

In the preamble to the Proposed Rule, CMS states that the goal of the Proposed Rule is to “spend[] our dollars more wisely for drugs paid under Part B, that is, a reduction in Medicare expenditures, while preserving or enhancing the quality of care provided to Medicare beneficiaries.”⁵³ While BIO generally supports this goal, we fundamentally disagree with the Agency that the Proposed Rule, if implemented, will achieve it. In fact, we strongly urge CMS to withdraw the Proposed Rule in its entirety precisely because it will undermine this goal: by limiting or effectively denying patient access to needed therapies covered under Part B, the Proposed Rule may actually increase overall Medicare expenditures and diminish patient access to high-quality care.⁵⁴

There is historical evidence to suggest that changes in Medicare reimbursement can negatively impact patient access to needed therapies. Specifically, Medicare beneficiaries’ access to intravenous immunoglobulin (IVIG) infusions was negatively impacted, in part, as a result of the implementation of the ASP methodology codified in the Medicare Modernization Act of 2003 (MMA).⁵⁵ While a confluence of market dynamics contributed to the observed reduction in access among Medicare beneficiaries, the initial inability of providers to obtain and administer this therapy at or below the Medicare reimbursement was a noted contributing factor.

⁵³ 81 Fed. Reg. at 13,231.

⁵⁴ There is historical evidence to suggest that changes in Medicare reimbursement can impact patient access to needed therapies. Specifically, Medicare beneficiaries’ access to intravenous immunoglobulin (IVIG) infusions was impacted, in part, as a result of the implementation of the ASP methodology codified in the Medicare Modernization Act of 2003 (MMA). While a confluence of market dynamics contributed to the observed reduction in access among Medicare beneficiaries, the initial inability of providers to obtain and administer this therapy at or below the Medicare reimbursement was a noted contributing factor. For example, University of Chicago researchers studying the impact of the MMA found that “following the change in Medicare reimbursement at the start of 2005, the average number of IVIG claims among Medicare eligible individuals grew more slowly than in the non-Medicare eligible population, despite growing at the same rate in the three years prior.” See Tomas Philipson & Anupam B. Jena, *The impact of Medicare Modernization Act Reimbursement Changes on the Utilization of Intravenous Immune Globulin* (2007) (unpublished manuscript) (on file at BIO). A 2007 analysis by the U.S. Department of Health and Human Services (HHS) Assistant Secretary for Planning and Evaluation (ASPE) found that “IGIV reimbursement levels appear inadequate in some circumstances to cover costs. Physician’s offices and hospital outpatient infusion clinics receive their normal reimbursement for infusion services, but, in some cases, do not recover the full IGIV purchase costs. The shortfall in IGIV purchase costs discourages these providers from offering infusions.” See HHS ASPE, *Analysis of supply, distribution, demand, and access issues associated with Immune Globulin Intravenous (IGIV)*, at p. 4-31 (2007), available at <https://aspe.hhs.gov/pdf-report/analysis-supply-distribution-demand-and-access-issues-associated-immune-globulin-intravenous-igiv>.

⁵⁵ Pub. L. No. 108-173 (Dec. 8, 2003).

For example, University of Chicago researchers studying the impact of the MMA found that “following the change in Medicare reimbursement at the start of 2005, the average number of IVIG claims among Medicare eligible individuals grew more slowly than in the non-Medicare eligible population, despite growing at the same rate in the three years prior.”⁵⁶ A 2007 analysis by the U.S. Department of Health and Human Services (HHS) Assistant Secretary for Planning and Evaluation (ASPE) also found that “IGIV [Immune Globulin Intravenous] reimbursement levels appear inadequate in some circumstances to cover costs. Physician’s offices and hospital outpatient infusion clinics receive their normal reimbursement for infusion services, but, in some cases, do not recover the full IGIV purchase costs. The shortfall in IGIV purchase costs discourages these providers from offering infusions.”⁵⁷

With this historical precedent in mind, BIO is very concerned that Phase I will make it more difficult for some providers to obtain Part B therapies at or below Medicare reimbursement rates. This can result in delaying or effectively denying patient access to certain therapies, and/or forcing patients to obtain therapies in higher-cost care settings that may be less convenient (e.g., require more travel time). In the longer-term, Phase I may contribute to the increasing trend in hospital/provider consolidation, as providers who continually operate negative margins with respect to Part B therapies may struggle to keep their practices financially viable, moving care permanently out of the community setting. These issues, collectively, will negatively impact patient access and may undermine the Administration’s aims related to cancer and personalized medicine. We address each of these concerns, in turn.

1. CMS’s Phase I proposal threatens providers’ ability to obtain Part B therapies and may drive the existing trend of shifting the site of care away from the community setting, increasing overall expenditures for patients and the Medicare program.

Phase I of the Proposed Rule threatens providers’ ability to obtain Part B therapies, and therefore threatens patient access to these needed therapies. Indeed, analyzing the proposal in the context of mandatory sequestration, the preamble to the Proposed Rule itself recognizes that Phase I will threaten providers’ ability to acquire Part B drugs. Although BIO acknowledges that CMS does not have authority over the sequestration policy enacted by Congress in 2011, we find the lack of a discussion of the impact of this existing policy on the Agency’s Phase I proposals troubling. Specifically, taking into account the impact of sequestration on Medicare payment for Part B drugs, under the Proposed Model, providers would be reimbursed $ASP + 0.86\% + \$16.53$. This blunt reduction in the effective Medicare reimbursement rate will differentially impact providers who practice in the community setting and/or in rural areas. These providers may already have challenges obtaining therapies at or below the statutory reimbursement rate of $ASP+6\%$, challenges that are compounded by the impact of sequestration and the fact that prompt-pay discounts

⁵⁶ T. Philipson & A.B. Jena. *The impact of Medicare Modernization Act reimbursement changes on the utilization of intravenous immune globulin* (University of Chicago).

⁵⁷ HHS ASPE (2007). *Supra* note 54.

offered to wholesalers are often are not passed on to providers (which further diminishes their effective reimbursement rate by one to two percent of ASP).

Given this reality, CMS's own logic in the preamble of the Proposed Rule counsels that the Agency not move forward with this demonstration. Specifically, CMS recognizes that "[a] flat add-on fee alone . . . that does not vary with the cost of the drug **may potentially increase the risk of having payments fall below acquisition costs**, particularly for providers and suppliers whose acquisition costs are near or above a drug's ASP."⁵⁸ Yet, a "flat add-on fee alone" is **exactly** what the Proposed Rule puts forward in practice.

Part B drug reimbursement must be benchmarked to providers' costs for obtaining these therapies in order to ensure that they are able to plan for, and balance, the financial realities of running a practice and providing their patients with timely access to needed therapies. The +6% in the current ASP-based reimbursement methodology is needed to cover the costs of drug procurement, handling, storage, inventory, preparation, bad debt, and waste disposal.⁵⁹ In 2007, MedPAC reported that "there are some drugs [physicians] cannot purchase at the payment rate," meaning that the Medicare reimbursement rate, even with the additional 6%, is less than their acquisition cost.⁶⁰ MedPAC also noted that, for most physicians, the difference between ASP+6% and their costs for drugs is "slim." However, under sequestration, providers are already operating at less than ASP+6%: the effective Medicare reimbursement rate is ASP+4.3% for Part B medicines. Paradoxically, the impact of sequestration on the effective reimbursement rate will make it impossible for CMS to conduct the "test" the Agency describes as the goal of Phase I (i.e., comparing expenditures under ASP+6% versus ASP+2.5% plus \$16.80).

Phase I of the Proposed Rule will further diminish providers' ability to provide Part B therapies as a sustainable business practice. For those provider practices that have a large percentage of Medicare Part B beneficiaries, the dramatic decrease in Medicare reimbursement may be unsustainable. In the short-term, this will mean that practices increasingly refer patients to hospital outpatient departments to receive their Part B medicines. In the medium- and longer-term, practices may not be able to keep their doors open to Medicare patients or may need to merge with hospitals to continue treating Medicare patients. By increasing the hurdles for community-based provider practices to remain financially viable, Phase I is likely to exacerbate the trend in the site-of-care shift from community to hospital outpatient department settings, through both increased referrals and increased provider/hospital consolidation. A 2016 published by Milliman found that the portion of chemotherapy infusions delivered in hospital outpatient departments

⁵⁸ 81 Fed. Reg. at 13,240 (emphasis added).

⁵⁹ For example, see Ted Okon et al., Problems Facing Cancer Care with Medicare's Definition of Average Selling Price, 1 Community Oncology 59, 59-60, 63 (2004). See also Medicare Reimbursement of Physician-Administered Drugs: Hearing Before the H. Comm. On Ways and Means 109th Cong. 53-63 (2006) (statement of Frederick M. Schnell, M.D., President, Community Oncology Alliance).

⁶⁰ MedPAC (2007). Supra note 14.

increased from 15.8% to 45.9% in the Medicare population from 2004 to 2014.⁶¹ This 45.9% rate in 2014 is up from 33% in 2011.⁶²

Phase I of the Proposed Rule can therefore result in increased overall Medicare expenditures by exacerbating this trend toward shifting care into hospital outpatient departments. The same 2016 Milliman study found that chemotherapy infusions are more expensive when administered in a hospital outpatient department setting as compared to a physician's office: specifically, the study found that, compared to patients receiving all chemotherapy infusions in a physician's office, those receiving all chemotherapy infusions in a hospital outpatient facility had a per-patient, per-year cost that was 34% higher.⁶³ These findings support and expand upon an earlier Milliman study, which found that both Medicare and its beneficiaries pay more for chemotherapy infusions when administered in hospital outpatient departments than in community oncology practices (\$6,500 versus \$650, respectively).⁶⁴ The results of this study were recently corroborated by a 2016 study conducted by researchers at the University of Chicago, which found that "increased vertical provider consolidation results in statistically significant increased inflation adjusted spending and prices on outpatient prescription drug-based cancer treatment."⁶⁵

In fact, Congress recently passed legislation—the Bipartisan Budget Act of 2015 (BiBA)—which included a provision (section 603) aimed at curtailing the existing financial incentives to treat patients in a more expensive setting of care. In a letter to stakeholders released after the law was passed, Chairman Fred Upton, of the House Committee on Energy and Commerce, explained that these provisions were included in BiBA to address "years of non-partisan economists, health policy experts, and providers expressing concern over the Medicare program's HOPPS [Hospital Outpatient Prospective Payment System] paying more for the same services provided at HOPDs [hospital outpatient departments] than in other settings."⁶⁶ The Chairman went on to elaborate that concerns were further raised that "this payment inequity drove the acquisition of standalone or independent practices and facilities by hospitals, result[ing] in higher costs for the Medicare system and taxpayers, and also result[ing] in beneficiaries needlessly facing higher cost-sharing in some settings than in others." While the legislation aims to mitigate the financial incentives to shift care through consolidation, it only applies to hospitals that newly acquire provider practices. This leaves largely intact the financial incentives for providers to refer patients to existing hospital outpatient department settings. Thus, BIO is very concerned that Phase I

⁶¹ Milliman, Cost Drivers of Cancer Care: A Retrospective Analysis of Medicare and Commercially Insured Population Claim Data 2004-2014, Figure 7: Volume of chemotherapy infusion claims by site of service, p. 19 (Apr. 2016), available at <http://www.communityoncology.org/pdfs/Trends-in-Cancer-Costs-White-Paper-FINAL-20160403.pdf>.

⁶² The Moran Company, Results of Analyses for Chemotherapy Administration Utilization and Chemotherapy Drug Utilization, 2005-2011 for Medicare Fee-for-Service Beneficiaries (May 2013).

⁶³ Milliman (2016). See *supra* note 61 at Figure 8: PPPY costs for chemotherapy patients based on site of their infused chemotherapy service, 2004 to 2014, p. 21.

⁶⁴ Milliman, Site of Service Cost Differences for Medicare Patients Receiving Chemotherapy (Oct. 2011).

⁶⁵ R.M. Conti, M.B. Landrum, and M. Jacobson The impact of provider consolidation on outpatient prescription drug-based cancer care spending, HEALTH CARE COST INSTITUTE (2016), available at <http://www.healthcostinstitute.org/files/HCCI-Issue-Brief-Impact-of-Provider-Consolidation.pdf>.

⁶⁶ Letter to Members of the Health Care Community on behalf of the Hon. Fred Upton, Chairman, House of Representatives Comm. on Energy & Commerce & the Hon. Joseph Pitts, Chairman, Subcommittee on Health, House Comm. on Energy & Commerce. 2016 (February 5), available at: <http://energycommerce.house.gov/sites/republicans.energycommerce.house.gov/files/114/Letters/20160205SiteNeutralLetter%5B1%5D.pdf>.

of the Proposed Rule will undermine the clear Congressional intent expressed in BiBA section 603 to restrain financial incentives to provide care in one setting over another.

2. By threatening providers' ability to obtain Part B therapies, CMS's Phase I proposal endangers patient access to needed therapies.

By increasing providers' challenges to obtain Part B therapies and by exacerbating the shift in the site of care from the community to hospital outpatient departments, Phase I of the Proposed Rule may require patients to travel longer distances to receive care, pay higher cost-sharing amounts, be forced to work with a new provider to continue their care, and otherwise face barriers in accessing needed therapies. Studies have consistently shown that distance and access to transportation can be significant barriers to accessing health care, especially for those with limited financial means.⁶⁷ This can result in a significant and measurable detrimental impact on the quality of life for patients with complex, chronic conditions, such as rheumatoid arthritis, Crohn's disease, cancer, and rare diseases (e.g., lysosomal storage disorders, hemophilia, and multiple sclerosis).

Moreover, the potential negative impact of the Proposed Rule, in particular Phase I, on patient access to needed therapies undermines the requirements established in ACA section 3601. Specifically, statute dictates that none of the ACA's provisions—of which CMMI's demonstration authority is one—"result in a reduction of the guaranteed benefits under [Medicare]."⁶⁸ However, the Proposed Rule, in particular Phase I, can practically result in reduced access to Part B benefits for patients—a guaranteed benefit—depending on where their providers practice.⁶⁹ Notably, this section of the ACA also requires that any savings generated should "extend the solvency of the Medicare trust funds, reduce Medicare premiums and other cost-sharing for beneficiaries, and improve or expand guaranteed Medicare benefits and protect access to Medicare providers."⁷⁰ While Phase I may meet this standard—though that is unclear, as CMS does not address these statutory requirements in the Proposed Rule—Phase II does not: CMS recognizes in the Proposed Rule that it is unclear "whether rebate distributions could be returned to the Medicare Part B Trust Fund, the beneficiary, the provider or supplier, or a combination of the three."⁷¹ The Proposed Rule's noncompliance with these provisions further supports BIO's strong recommendation that CMS withdraw the Proposed Rule in its entirety.

Additionally, BIO is very concerned that CMS does not propose robust, reliable metrics to assess the impact of the Proposed Rule on patient access to needed medicines or the quality of patient care. Thus, the negative consequences of the Proposed Rule likely will go unnoticed by CMS. In [section XI](#), BIO identifies the lack of existing quality measures to serve as a bulwark against decreased quality of care under Phase I, and notes that, despite

⁶⁷ See, e.g., S.T. Syed, B.S. Gerber & L.K. Sharp, *Traveling towards disease: transportation barriers to health care access*, 38 J. OF COMMUNITY HEALTH 976-93 (2013).

⁶⁸ ACA § 3601(a).

⁶⁹ Under the Medicare statute, items and services in Part B benefit categories, including Part B drugs, are "guaranteed" to beneficiaries. See SSA § 1832(a)(1) (providing that the benefits provided under Part B "shall" consist of medical and other health services, defined, in turn, to include Part B drugs and biologicals). See also SSA § 1861(s) (defining "medical and other health services").

⁷⁰ ACA § 3601(b).

⁷¹ 81 Fed. Reg. at 13,246.

CMS's goal of improving the quality of care, the Agency does not establish a robust framework to measure changes in quality of care that result from each phase of the Proposed Rule, nor does the Agency establish a mechanism to refine the Proposed Rule based on such observed changes in care quality. The absence of such a framework, paired with the accelerated timeframe on which CMS proposes to implement a Final Rule, creates significant concerns with regard to a patient's ability to obtain needed medicines under CMS's proposed provisions.

3. CMS's Phase I proposal undermines the Administration's longer-term goals to speed innovative therapies to market.

Innovative biopharmaceutical treatments and cures are among the healthcare interventions most likely to improve patient health outcomes and offset costs across the healthcare system. For example, innovative therapies have: contributed to substantial gains in life expectancy for patients (e.g., cancer patients lived a combined 23 million years longer between 1988 and 2000, thanks to investments in cancer research);⁷² resulted in an improvement in the 10-year survival rate for people with chronic myeloid leukemia (from 20% a decade ago to 80% today);⁷³ and improved patients' quality of life. Limiting patient access to these critical therapies reverses the gains that medical science has made possible over the last several decades.

Over the longer-term, inadequate reimbursement for innovative therapies will negatively impact the ecosystem that sustains biopharmaceutical innovation. This can lead to diminished investment in research and development for therapies that may be covered by Part B. Considering that treatments and cures are the most likely healthcare interventions to improve the health outcomes of patients suffering from the diseases and conditions identified above—and are the most likely to offset costs across the healthcare system—creating a disincentive for innovation in this space would undermine CMS's efforts to achieve improved quality of care and decrease overall expenditures over time.

In fact, the Administration has recognized the potential impact of innovative therapies in the fight against serious diseases, and has prioritized the development of treatments for cancer. In the press release describing the "Cancer Moonshot," shortly after the President's 2016 State of the Union Address, the White House noted that "the science is ready for the concerted new effort this initiative will deliver."⁷⁴ However, the incentives that the Proposed Rule will establish will undermine the translation of the most advanced, groundbreaking science into innovative treatments for cancer patients, many of whom are covered by Medicare.

Indeed, BIO is perplexed that CMS is proposing to cut reimbursement to providers for furnishing innovative therapies that treat some of the most complex, chronic conditions **at the same time** that the Administration is devoting resources to finding new, innovative treatments and cures for these same conditions (e.g., through the Cancer Moonshot and the

⁷² D.N. Lakdawalla et al., *An economic evaluation of the war on cancer*, 29 J. OF HEALTH ECON. 333-346 (2010).

⁷³ W. Yin et al, *Value of Survival Gains in Chronic Myeloid Leukemia*, 518 AM. J. OF MG'D CARE S257-264 (2012).

⁷⁴ The White House, FACT SHEET: Investing in the National Cancer Moonshot (Feb. 1, 2016), *available at* <https://www.whitehouse.gov/the-press-office/2016/02/01/fact-sheet-investing-national-cancer-moonshot>.

Precision Medicine Initiative). BIO strongly cautions CMS that the Proposed Rule will undermine these ongoing initiatives: literature demonstrates that adequate reimbursement associated with specific therapeutic areas is tied to increased research and development investment in such areas.⁷⁵

C. CMS's Phase I proposals will create arbitrary "winners" and "losers."

BIO is very concerned that Phase I of the Proposed Rule will create divergent "winners and losers" among provider groups based on certain characteristics of the individual products they prescribe and the relative utilization of these products across a given patient population, **not** based on providers' delivery of efficient, effective high-quality care. For example, the Proposed Model's methodology will benefit therapies with more frequent dosing schedules—a feature of a product that is not necessarily unrelated to the value the therapy offers to a patient.⁷⁶ Specifically, the per-day flat add-on that CMS is proposing would reward products that required a greater number of administrations over a period of time (e.g., per week or per month) than those less frequently administered. Such a system could potentially reward inefficiencies in patient care, as a greater number of administrations would necessitate more patient visits—potentially increasing the frequency and/or magnitude of negative side effects, disrupting patients' daily routine, and resulting in higher overall costs to both the patient and the program. Thus, paradoxically, though CMS intends to utilize the Proposed Rule to remove so-called "perverse incentives" from the existing Part B reimbursement methodology, the Agency is actually introducing such incentives through its Phase I proposal.

Additionally, how the flat fee is calculated and updated could create arbitrary "winners" and "losers." For example, it is unclear, from the text of the Proposed Rule, how the flat fee will be calculated and applied in instances in which a Part B-covered therapy is provided in an amount that is meant to cover multiple administrations (e.g., in the case of certain clotting factors, patients can be provided with a month's supply of Part B-covered medicine at a time). If CMS calculates a single flat add-on payment for the entire supply, it would create a disincentive for providers and patients to obtain the larger supply, despite the efficiencies a larger supply may present for patients. However, the opposite could occur if CMS calculates the flat add-on for every single day between refills of the medicine, whether or not the therapy needs to be administered on each of those intervening days. Additionally, CMS's proposal to update the flat-fee amount each year by the percentage increase in the CPI-M for the most recent 12-month period is problematic since this index does not specifically reflect market pressures on prescription medicines.⁷⁷ Thus, it may over- or under-estimate an appropriate update to the flat add-on, to the detriment of the goal of appropriate utilization of needed medicines.

⁷⁵ M.E. Blume-Kohout & N. Sood, *Market size and innovation: Effects of Medicare Part D on pharmaceutical research and development*, 97 J. OF PUB. ECON. 327-336 (2013).

⁷⁶ The Moran Company, *Distribution of ASP+6% Values (2011)* (analysis on file at BIO).

⁷⁷ 81 Fed. Reg. at 13,241.

D. CMS's Phase I proposal lacks important procedural protections to ensure patients are able to gain access to the most appropriate therapies for them.

First and foremost, BIO is very concerned that CMS does not propose any evaluation metrics specific to monitoring patient access to needed medicines or quality of care, in complete contrast to the Agency's assertions that improving or maintaining quality of care is of paramount interest. BIO discusses these concerns—as well as concerns with regard to the lack of protections to minimize patients' exposure to risk—in detail in [section XI](#) as they relate to both Phases I and II.

Second, and specific to Phase I of the Proposed Rule, BIO is very concerned that CMS did not propose an exceptions or appeals process that applies to Phase I of the Proposed Rule ([see section XI](#) for a more detailed discussion with regard to the proposed Phase II exceptions process). These processes are a critical patient protection as they help to ensure that a patient's individual clinical circumstances are taken into account in instances in which the application of a broader initiative may unduly restrict access to the most appropriate therapy for that patient. The absence of exceptions or appeals processes in Phase I is especially concerning given the potential for Phase I to threaten patient access to needed therapies (discussed above). Thus, BIO remains concerned that maintaining or improving the quality of patient care is not the main priority of the Proposed Rule, despite the fact that statute directs CMS to focus on demonstrations that prioritize this goal.⁷⁸

Third, CMS's implementation timeline and lack of a phase-in process does not demonstrate a responsible approach to policymaking. In contrast to the other demonstrations that CMMI has proposed, and other proposed rules CMS has released, the Agency does not establish a transition process for providers. For example, in the recently-released *Merit-Based Incentive Payment System and Alternative Payment Model Proposed Rule*, CMS specifically recognizes the need for "a full year of measurement and sufficient time to base adjustments on complete and accurate information."⁷⁹ However, CMS has expressed an interest in implementing the Proposed Rule as soon as possible, despite the complexity and broad scope proposed. The Agency also fails to propose a process for interim evaluation of the Proposed Rule, especially in the first several quarters after implementation. CMS also ignores the question of how patients will be informed about the impact of the Proposed Rule on their care. These oversights further support BIO's strong recommendation that CMS withdraw the Proposed Rule, and instead engage stakeholders in a dialogue to identify evidence-based, thoughtful mechanisms to improve the quality of patient care in Part B and decrease overall Medicare expenditures.

⁷⁸ Specifically, statute states that "[t]he Secretary shall focus on models expected to reduce program costs under the applicable title **while preserving or enhancing the quality of care received by individuals** receiving benefits under such title" (emphasis added). [See SSA § 1115A\(b\)\(2\)\(A\)](#).

⁷⁹ CMS, Medicare Program: Merit-Based Incentive Payment System and Alternative Payment Model Incentive under the Physician Fee Schedule, and Criteria for Physician-Focused Payment Models [prepublication version] at 77 (Apr. 27, 2016).

VII. Payment Methodology: Phase II

- A. Introduction. Many of the “value-based” models described in Phase II are actually blunt cost-containment tools that run counter to patient-centric care; True value-based arrangements are not ready for widespread implementation at this time given the many unresolved legal, operational, and other issues associated with their use, further underscoring the need to withdraw the Proposed Rule at this time; CMS should instead prioritize creating a safe and predictable regulatory environment to enable these arrangements to succeed in the private sector; Especially since there is no “one-size-fits-all” value-based arrangement, any experimentation with such models must involve only voluntary arrangements of limited scope beginning in the private sector.**

In the second phase of the model, CMS “propose[s] to implement VBP tools for Part B drugs using value-based pricing and clinical decision support tools.”⁸⁰ As a threshold matter, we take issue with CMS’s broad characterization of the models described in Phase II as “value-based.” Many of these tools—including reference pricing and CMS’s version of indications-based pricing—are actually blunt, one-size-fits-all cost-containment strategies that run counter to patient-centric care and other HHS initiatives, such as the Precision Medicine Initiative. Moreover, these models, as described, would involve CMS making clinical and cost-effectiveness determinations that well exceed the Agency’s expertise, as illustrated by many of the problematic aspects of the proposal.

BIO members are genuinely interested in pursuing true value-based arrangements that achieve better outcomes for patients at the same (or lower) cost. Indeed, BIO and its members have been working with CMS over the past several months to identify and work towards solutions for overcoming potential regulatory considerations related to the use of value-based arrangements in the private sector, and we look forward to continuing this dialogue with the Agency. However, these models are not ready for widespread implementation at this time, particularly given the many unresolved legal, operational, and other issues associated with their use.

Thus, while we agree with CMS that there is at least the potential for some of these models to be pursued successfully in the private sector, these models are still nascent and remain relatively small-scale in order to allow the participants to work through the sizable operational, fiscal, legal, and other challenges with their implementation before taking on greater degrees of risk. For example, even for the most sophisticated payors, there is a significant challenge associated with implementing the systems that are essential to track and assess the volume of data necessary to operationalize these arrangements. Moreover, even the studies cited in the preamble to the Proposed Rule note that there are currently only a limited number of true value-based arrangements in place in this country “and these have been modest efforts that would be difficult to bring to greater scale.”⁸¹ Moreover,

⁸⁰ 81 Fed. Reg. 13,242.

⁸¹ See, e.g., 81 Fed. Reg. at 13,244 (citing P.J. Neumann et al, *Risk-Sharing Arrangements That Link Payment For Medications To Health Outcomes Are Proving Hard To Implement*, 30 HEALTH AFFAIRS 2329–2337 (2011)).

"[g]aining a clear understanding of the status and performance of the models is challenging . . . because little formal evaluation has occurred."⁸²

Until there is empirical evidence that these arrangements work and establish value—particularly for patients—they should not be expanded beyond the private sector. In the meantime, it is critical that CMS—and its sister agencies within the Department of Health and Human Services—work with stakeholders to establish a safe and predictable regulatory environment, including by establishing safe harbors and issuing guidance to address the government price reporting, anti-kickback statute, product communications, and other regulatory considerations. Only then will these models flourish in the private market.

That said, even if value-based arrangements prove to be successful in the private sector, we note that it will be exceedingly difficult to establish true value-based arrangements in the context of Medicare Part B. Throughout the discussion of Phase II of the Proposed Rule, as well as in proposed 42 C.F.R. § 511.305, CMS does not always clearly state whether a given arrangement would be established between CMS and providers (i.e., alternative payment models), between CMS and manufacturers (i.e., value-based arrangements), or between CMS and beneficiaries (i.e., value-based insurance design). While this could potentially vary based on the type of arrangement pursued, we note that the pursuit of arrangements directly between CMS and manufacturers would be unprecedented, exceedingly difficult to adopt, and likely extremely problematic from a patient access standpoint.

Not only would the direct involvement of manufacturers implicate some of the regulatory barriers described above (e.g., Best Price), but currently there is no direct contractual relationship between CMS and the manufacturers of Part B drugs. Rather, these products are purchased by providers/suppliers, who are, in turn, reimbursed by Medicare for the administration/dispensing of these products to beneficiaries. Any efforts to upend this system could result in a reduction or delay in access to critical therapies for Medicare beneficiaries, particularly given the failures of the competitive acquisition program (CAP),⁸³ and the absence of any distribution option (e.g., specialty pharmacy) under Part B.

These concerns, collectively, further underscore the need for CMS to withdraw the Proposed Rule. We describe each of these concerns in greater detail, in turn.

1. Any experimentation with value-based arrangements must involve only voluntary arrangements of limited scope beginning in the private sector.

We cannot emphasize enough the need for any experimentation with value-based arrangements to begin with small-scale, voluntary pilot programs—as opposed to the mandatory, nationwide approach described in the Proposed Rule. The reason for this is two-fold. First, there is no “one-size-fits-all” value-based arrangement. Rather, value-based models are extremely complex and, to be both effective and worthwhile, their

⁸² Id.

⁸³ See, e.g., American Medical News, Lack of Interest Derails Medicare Part B drug program (Nov. 10, 2008), <http://www.amednews.com/article/20081110/government/311109988/1/>.

structure necessarily must vary based on the characteristics of the product and/or therapeutic area in question. For example, because the outcomes of interest necessarily vary by product, the metrics, data collection methodologies, disease states, patient populations, and care settings necessarily also vary from arrangement to arrangement. Consequently, these models are very challenging, if not impossible, to scale up, particularly in the short term.

Second, establishing these arrangements is labor-intensive for all parties—including payors, manufacturers, and providers—given the need to develop data-collection protocols, negotiate arrangements, assess product performance, and design procedures to adjudicate disputes. Moreover, extensive systems are needed to track and match outcomes and/or other data points. Particularly given CMS's limited resources and staffing, this effort will only be worthwhile where the benefits of the value-based arrangement—in particular for patients—are large enough to justify the implementation efforts and costs.

We therefore agree with CMS's recognition of the need for these models to be "properly structured and operated" to mitigate the "risk of abuse" as well as the fact that not all drugs may be "suitable candidates" for the application of the "specific tools" identified by the Agency.⁸⁴ To these ends, the selection of both the arrangements and the drugs to which such arrangements would apply must be made on a **voluntary** basis to enable the private market to continue to determine which drugs are suitable for these arrangements. In the meantime, CMS should turn its attention towards actions the Agency can take to enable these arrangements to succeed in the private sector.

2. CMS—and its sister agencies within HHS—must work with stakeholders to establish a safe and predictable regulatory environment to enable these models to succeed in the private market.

We are very concerned there is no thoughtful discussion in the Proposed Rule of how CMS proposes to address the barriers to value-based arrangements outside of those posed by the ASP statute itself (SSA § 1847A). CMS's failure to clearly address the regulatory requirements that currently constrain the adoption of value-based arrangements—in the Proposed Rule and otherwise—creates significant uncertainty. It also represents a missed opportunity, as CMS—together with its sister agencies within HHS—could significantly contribute to the successful development of value-based arrangements in the private sector by waiving, or otherwise addressing, some of the regulatory considerations that can impede the development and uptake of value-based arrangements today. It is in this way that CMS can play a foundational role in the more widespread development and use of these arrangements.

For example, provisions of the Medicaid Drug Rebate Statute (SSA § 1927), including the provisions regarding Medicaid Best Price, present critical considerations in any value-based arrangement design. Given the absence of waiver authority or safe harbors in this area, CMS will need to work with manufacturers and other stakeholders to ensure that the pursuit of value-based arrangements does not result in a Medicaid Best Price (or other

⁸⁴ 81 Fed. Reg. at 13,242.

Medicaid pricing metrics) that are inaccurate (e.g., artificially high or low). We also urge CMS to establish a formal process for manufacturers to obtain clear government price reporting guidance, including for the treatment of value-based arrangements, as we have discussed with the Agency previously.

In addition, while CMMI does have the authority to waive title XI of the SSA, there is absolutely no discussion as to whether CMMI intends to waive any such provisions, even though this title includes both the anti-kickback statute⁸⁵ and beneficiary inducement provisions of the civil monetary penalties statute,⁸⁶ either of which could be implicated by value-based arrangements between non-governmental entities. For example, to maximize their utility, certain value-based arrangements may be combined with implementation of adherence/persistency programs, which, depending on how they are structured, could be viewed as remuneration in exchange for a referral for, or purchase of, an item or service that may be paid for under a Federal health care program (here, Medicare).⁸⁷ Moreover, there remains uncertainty as to whether the potentially applicable safe harbors (e.g., for discounts and other reductions in price) would apply to such arrangements.⁸⁸ BIO has asked the HHS Office of Inspector General (OIG) to consider updating its regulatory safe harbors to reflect the emergence of value-based arrangements, and we now encourage CMS to work with the OIG on this endeavor.⁸⁹

Another critical area that CMS does not discuss in the Proposed Rule relates to FDA's limitations on promotion of off-label indications by manufacturers—which is another key regulatory consideration, and one that CMS should work with FDA to address.⁹⁰

Each of these areas represents an opportunity for CMS, and the Department as a whole, to contribute to the success of value-based arrangements going forward.

3. Any experimentation with value-based arrangements should occur via a thoughtful, transparent, and collaborative process, and should start by defining what constitutes a "value-based arrangement" in the first instance.

It also is absolutely imperative that any experimentation with value-based arrangements occur through a thoughtful, transparent, and collaborative process that

⁸⁵ SSA § 1128B(b).

⁸⁶ SSA § 1128A(a)(5).

⁸⁷ See, e.g., OIG, Advisory Opinion No. 14-06 (Aug. 7, 2014) (concluding that an arrangement involving per-fill fees for the dispensing of specialty drugs—including care coordination services—would "represent compensation for the Local Pharmacies generating business, including Federal health care program business, rather than solely compensation for bona fide, commercially reasonable services," and thus the OIG "cannot conclude that the Proposed Arrangement would pose no more than a minimal risk of fraud and abuse under the anti-kickback statute.").

⁸⁸ See 42 C.F.R. § 1001.952; SSA § 1128B(b)(3)(A). For example, if CMS were to disagree with the government price reporting treatment of a value-based arrangement, it is unclear whether the arrangement would meet the requirement under the statutory discount safe harbor that the reduction in price is properly disclosed.

⁸⁹ BIO. 2016. *Comments Submitted in Response to the Solicitation of New Safe Harbors and Special Fraud Alerts Proposed Rule* (February 22, 2016), available at: <https://www.regulations.gov/#!docketDetail;D=HHSIG-2016-0001>.

⁹⁰ Questions also remain regarding the ability of manufacturers to relate certain pharmacoeconomic data to payors, particularly given that the FDA has not issued guidance on FDAMA 114.

involves a diverse group of stakeholders, including patients, providers, and manufacturers. Indeed, in contrast to the closed-door process used by CMS to craft the Proposed Rule, one of the studies referenced in support of Phase II expressly notes that the development of value-based models should involve a “transparent process.”⁹¹

It is not enough to solicit informal public input on each of the proposals before they are finalized, or to provide a 45-day period of public notice before any finalized proposals are actually implemented.⁹² As we describe throughout our comments, implementing these models would be an enormous undertaking that necessarily involves resolving an array of complex operational, legal, and other questions—many of which are detailed in these comments. Furthermore, depending on how they were structured, such models could represent sweeping changes to current payment policies. Reserving the right to implement such policies through a brief, informal comment period would introduce yet further uncertainty for innovators considering whether to invest in high-risk research projects. Thus, in accordance with the public-consultation requirements articulated in section 1115A, CMS must begin any consideration of these models with open door forums, requests for information, and other open-ended solicitation of stakeholder feedback. Moreover, any models ultimately developed for Medicare Part B absolutely must be subject to notice-and-comment rulemaking in order to provide stakeholders with ample notice and opportunity to comment, as well as the opportunity to see the Agency’s response to any comments provided.

Perhaps most critically, broad stakeholder engagement is necessary to define what constitutes a “value-based” arrangement in the first instance. The significant uncertainty surrounding this issue is illustrated by the fact that CMS has identified “reference pricing” as a “value-based purchasing” model, which it is not, as we describe below. From BIO’s perspective, at a minimum, any “value-based” arrangement must: (1) be patient-centered and clearly produce additional value to the patient; (2) encourage both the continued development of innovative new medicines, as well as advancements in “beyond the pill” innovations that further improve patient care (e.g., medication adherence programs, predictive modeling, personalized medicine, innovative financing arrangements with payors and others); and (3) measure value across the healthcare system, including across both the pharmacy and medical benefits, using validated, meaningful metrics and robust, program-wide data. Designing arrangements based on these criteria will help ensure that value-based arrangements benefit all stakeholders, including providers, manufacturers, and patients. We discuss these three criteria, in turn.

This first criterion—that a value-based arrangement remain patient-centric—is the most critical and fundamental. To these ends, value-based arrangements must endeavor to maximize the value that biopharmaceutical products have to offer patients. Specifically, any value-based arrangement must include—as its central premise—that the arrangement both: (1) produces tangible benefits to patients, including by delivering the right therapy to the right patient at the right time, and by supporting patient adherence to that therapy; and

⁹¹ See 81 Fed. Reg. at 13,244 (citing P.J. Neumann et al., *Risk-Sharing Arrangements That Link Payment For Medications To Health Outcomes Are Proving Hard To Implement*, 30 HEALTH AFFAIRS 2329–2337 (2011)).

⁹² 81 Fed. Reg. at 13,243.

(2) does not harm patients (e.g., as the result of diminished access to care or increased cost-sharing). The development of these models also should take into consideration recommendations for incorporating the patient voice into payment reform developed by patient advocacy organizations, such as the National Health Council's Patient-Centered Value Model Rubric.⁹³ Relatedly, any arrangements adopted must also be economically and operationally viable for providers—and not merely viewed as another administrative burden—to mitigate the risks that the arrangement could result in patient access and provider consolidation issues akin to those associated with the proposed design of Phase I, described in [section VI](#) of this letter.

This second criterion—that the arrangement encourages the development of new therapies and “beyond the pill” innovations—also is critical to the success of these arrangements. To these ends, we are concerned that the Proposed Rule discusses the need to remove risk and obtain savings for the Medicare program, while providing absolutely no discussion of the upside risk for manufacturers, or any limitation on manufacturers' downside risk. As interested partners in the development of value-based arrangements, BIO and its member companies are very concerned about the development of models that impose solely downside risk on manufacturers and providers. Not only does this stand in stark contrast to the approach taken to develop alternative payment models (APMs)—which include only upside risk for participating entities, at least in the initial phases—but models that impose only downside risk on manufacturers entirely overlook the value that biopharmaceutical products have to offer, and have the very real potential to stifle innovation in this sector going forward. Value-based arrangements must therefore identify both an upside risk for manufacturers and providers/prescribers (e.g., increased upfront payment or opportunity for shared savings), as well as a limitation on their downside risk (e.g., cap on risk, risk-adjustment methodologies). At a minimum, it is essential that manufacturers' risk be mitigated for outcomes beyond their control, such as how a particular drug is prescribed or used.

The third criterion—that the arrangements measure value across the healthcare system, including across both the pharmacy and medical benefits, using validated, meaningful metrics assessed using robust, program-wide data—is a fundamental component of value-based arrangements generally, and is essential to the ability of these arrangements to maximize the potential of biopharmaceutical products for the Medicare program, in particular. Notably, in the Proposed Rule, CMS does not provide any details regarding: the types of metrics that would be used for purposes of value-based arrangements; how such metrics would be identified or validated; how data used to track performance on the metrics will be collected, shared, or utilized; who will pay for or conduct the data collection; or whether performance would be assessed across care settings. Yet, given the data-driven nature of value-based arrangements, resolving these questions is critical to the success of these arrangements, and must be addressed, taking into account the potential operational and systems barriers, as well as what steps will be taken to protect patient privacy. Relatedly, CMS does not explain if or how the Agency will assist stakeholders (e.g., manufacturers, providers) to set up the systems, or obtain the data, necessary to support the value-based arrangements described in the Proposed Rule. In addition to any

⁹³ This rubric is available here: <http://www.nationalhealthcouncil.org/sites/default/files/Value-Rubric.pdf>.

“educational activities to support implementation and testing of value-based purchasing strategies,”⁹⁴ technical assistance and potentially even reimbursement for the cost of establishing these systems would be necessary to successfully implement these models.

B. “Value-Based” Strategies. Not all of the strategies described in the Proposed Rule are “value-based” and none are ready for widespread implementation.

CMS proposes four principal types of alternative purchasing strategies, including: (1) reference pricing; (2) indications-based pricing; (3) outcomes-based risk-sharing; and (4) discounting or eliminating patient coinsurance amounts. We respond to each of these proposals, in turn.

1. Reference pricing is not a value-based purchasing strategy.

In the Proposed Rule, CMS states that “providing equal payment for therapeutically similar drug products is one form of value-based pricing that we propose to implement as part of phase II of the model. The private market capitalizes on this concept through reference pricing, which refers to a standard payment rate—a benchmark—set for a group of drugs.”⁹⁵ BIO strongly disagrees with this characterization of the reference pricing methodology. Reference pricing is not a value-based arrangement, or even a market-based arrangement; rather it is a reprise of least-costly alternative (LCA), a policy about which BIO has expressed, and maintains, very serious concern.⁹⁶

In sum, LCA policies limit beneficiaries’ access to drugs or biologicals based on cost, not whether they are “reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member.”⁹⁷ BIO is not alone: CMS’s last attempt to utilize an LCA policy was stricken down on the grounds that it circumvented the minutely detailed reimbursement rates that Congress had established for prescription drugs under Part B.⁹⁸ Although CMMI technically has the authority to waive the underlying statutory provision at issue in that litigation, we do not believe that Congress intended CMMI to be a vehicle for the Executive Branch to unilaterally rewrite the Medicare statute, as described in greater detail in [section X](#), below, or for it to circumvent a decision of the judiciary.⁹⁹

⁹⁴ 81 Fed. Reg. at 13,244.

⁹⁵ 81 Fed. Reg. at 13,245 (internal citations omitted).

⁹⁶ See, e.g., Biotechnology Industry Organization Comments in Response to *Medicare Program; Revisions to Payment Policies Under the Physician Fee Schedule and Other Revisions to Part B for CY 2009; Proposed Rule [CMS-1403-P]* (Aug. 29, 2008), available at <http://www3.bio.org/healthcare/medicare/20080829.pdf>; Biotechnology Industry Organization Letter to Bruce Quinn, MD, Contractor Medical Director, National Heritage Insurance Company Re: Draft Local Coverage Determination for Gonadotropin-Releasing Hormone Analogs (June 2, 2006), available at <http://www3.bio.org/healthcare/medicare/20060602.pdf>.

⁹⁷ SSA § 1862(a)(1)(A).

⁹⁸ See *Hayes v. Sebelius*, 589 3d 1279 (D.D.C. 2009) (holding that CMS could not use a least costly alternative policy in an LCD to by-pass the statutory reimbursement policy for prescribed drugs Congress enacted under the Medicare Modernization Act of 2003).

⁹⁹ See id. Given the timing of the LCA litigation, which occurred prior to the negotiation and passage of the ACA, had Congress wished to grant CMS the authority to pursue LCA policies—through CMMI or otherwise—it could have made that known in the statute. However, in the debate leading up to the passage of the ACA, the severe shortcomings of cost effectiveness decisions were exposed and ultimately explicitly excluded as a basis of analysis

BIO also is extremely concerned regarding CMS's proposals to make determinations of therapeutic similarity as part of the Agency's reference-pricing proposal, which has the potential to usurp healthcare providers' clinical judgment, expertise, and ability to take into account an individual patient's circumstances and health history. Moreover, in order for reference pricing to work, there would have to be a determination that exceeds mere similarity, to assess true clinical equivalence. Yet, CMS does not have the clinical expertise nor resources to make these complex determinations.¹⁰⁰ We therefore do not support the proposition of CMS substituting its potentially reimbursement-driven decision-making on therapeutic similarity for those made based on substantial clinical evidence and expertise.

This concern is heightened by CMS's proposed process for identifying "therapeutically similar drug products" for purposes of its proposed reference pricing strategy. Specifically, treating therapies as "therapeutically similar" merely because they are in the same drug category and/or class (which CMS appears to suggest as a potential mechanism to implement this strategy) is inappropriate both because : (1) there is no universally recognized system for classifying drugs by categories and class, and the most commonly utilized systems, such as the U.S. Pharmacopeia categories and classes, have distinct disadvantages, particularly as applied to Part B drugs; and (2) no matter which classification system is used, it is not always the case that all therapies in a category treat the same conditions or share all of the same indications, or have similar side-effect profiles, making any assessment of the comparative efficacy or effectiveness of such therapies an apples-to-oranges comparison.

This proposal is particularly unworkable for biologicals, and thus will have an especially negative effect on patients who rely on these therapies. For example, in the Proposed Rule, CMS defines therapeutically similar drugs as those that "are generally members of the same drug class that work on the same biochemical processes but have different **chemical structures**."¹⁰¹ This definition **cannot** be appropriately applied to biological therapies because, relative to chemically-synthesized drugs, these products are very large, complex molecules (or mixtures of molecules), manufactured in living systems, and thus are not categorized based on "chemical structure."

Furthermore, as noted previously, patients have highly individualized responses to biologicals, meaning that even two biologics in the same class can elicit very different patient responses. Indeed, even in instances in which two biologicals may have the same clinical indication(s), switching a patient from one to the other can negatively impact health outcomes by, for example, increasing negative side-effects and the number of

in evaluating therapies, and Congress made the decision that innovative payment methodologies should focus on quality, rather than purely cost. Thus, while the Department of Health and Human Services' Office of Inspector General has recommended that CMS consider seeking legislative authority to implement LCA policies for Part B drugs, Congress, to date, has not expressly granted CMS this authority. See OIG, Least Costly Alternative Policies: Impact on Prostate Cancer Drugs Covered Under Medicare Part B, OEI-12-12-00210 (Nov. 2012), available at <http://oig.hhs.gov/oei/reports/oei-12-12-00210.pdf>.

¹⁰⁰ Even FDA, which has the capability to make determinations of therapeutic equivalence, only does so in limited circumstances (i.e., in the case of a generic and the brand product).

¹⁰¹ 81 Fed. Reg. at 13,243, n. 20 (emphasis added).

episodes/flare-ups a patient experiences after such a switch occurs.¹⁰² In addition to having very serious consequences for a patient's quality of life, this can lead to increased consumption of healthcare resources, such as increased physician office visits and hospitalizations, which, in turn, can increase overall Medicare costs, undermining CMS's stated rationale for proposing this strategy. The challenges of establishing something akin to therapeutic equivalence for biologics is demonstrated by the approval pathway for biosimilars, which recognizes that, even products approved as "biosimilars" differ from their reference products.¹⁰³

Moreover, reference pricing is not a relevant conceptual framework when considering the realities of clinical care, in particular in the context of the increasing reliance on combination therapy regimens to treat a number of conditions. For instance, in the oncology space, combination regimens have become the standard of care for major cancers. Particularly given that each drug in these regimens may have subtle differences that affect patients differently, we have serious concerns regarding a reference pricing model that could encourage providers to swap out a specific drug in the regimen, or to pursue a different regimen entirely, as either approach could have an extremely deleterious patient impact.

While CMS claims that the proposed reference pricing scheme "give[s] prescribers incentives to use the drug product that provides the most value for the patient";¹⁰⁴ this proposal would actually result in **diminishing** patient access to appropriate therapies, not enhancing it, for the following two reasons.

First, the reference pricing strategy does not assess value in a way that is relevant to an individual patient. Instead, CMS proposes to base the determination of "value" on an average across all patients by assessing, for example, "effectiveness," in the broadest possible terms. Thus, this strategy encourages providers to apply that broad "value" determination across their entire patient population, seemingly regardless of how the specific clinical circumstances of an individual patient might impact clinical decision-making.

Second, CMS provides no details regarding how the benchmark reimbursement rate would be established under such a system, which will impact the degree to which this proposal exacerbates our patient access and quality-of-care concerns. That said, no matter how the benchmark is established, providers whose patients require a therapy that is more expensive than whatever benchmark price is set by CMS will be forced to obtain that therapy below cost, or to encourage the patient to seek care in a different—and potentially higher-cost and less convenient—setting. Because CMS would have sole discretion to establish the reimbursement benchmark, the Agency could effectively deny patient access to certain therapies by setting a payment rate that financially penalizes providers who select

¹⁰² See D.T. Rubin et al (2015). *Supra* note 28.

¹⁰³ FDA may affirmatively designate a biosimilar as interchangeable with a reference product *only* after an additional determination that: (1) it can be expected to produce the *same* clinical results as the reference product *in any given patient*; and (2) for a biological product that is administered more than once to an individual, the risk in terms of safety or diminished efficacy of alternating or switching between use of the biological product and the reference product is not greater than the risk of using the reference product without alternating or switching. Public Health Service Act § 351(k)(4).

¹⁰⁴ 81 Fed. Reg. at 13,243.

certain therapies, no matter whether it was the most appropriate for an individual patient. In such a situation, CMS would be usurping the decision-making role of patients and providers, effectively denying patients access to a safe and effective therapy in the care setting of their choosing, solely on the basis of the therapy's cost. This countermands all of CMS's efforts—in accordance with CMMI's authorizing statute—to improve **value** for Medicare beneficiaries.

Along these lines, BIO is particularly troubled that the Agency's reference pricing proposal appears to favor the use of compounded/repackaged off-label therapies over FDA-approved therapies by generally reducing payment rates for FDA-approved therapies, while increasing the payment rate for the off-label repackaged alternative. The FDA, Congress, and other governmental bodies have recognized the importance of ensuring patient access to safe medicines that are produced under conditions designed to ensure their safety, efficacy, and quality. CMS's proposed changes should not undermine this critical principle but, instead, reflect and reinforce it.

Finally, the proposed reference pricing strategy will have a negative impact on future innovation. For example, if finalized, this policy would create a disincentive for the dedication of R&D resources to drug categories and classes where a therapy already exists, even if new therapies could decrease side-effects or improve efficacy for a subpopulation of patients with that condition. In the past, this type of cumulative innovation has led to major improvements in the standard of care and even to cures, including in the areas of HIV, hepatitis C, pediatric cancer, and chronic myeloid leukemia.¹⁰⁵ Stifling this innovation ecosystem, as the reference pricing proposal would do, can impede progress toward better treatments and cures. Meanwhile, a 2012 review article studying the impact of reference pricing on European markets concluded that there is limited, if any, impact on long-term expenditures. The researchers attributed this finding to the reality that reference pricing "does not affect other drivers of increasing pharmaceutical expenditure," including, for example, the impact of increased utilization by an aging population, and improved treatments that allow patients to live for decades with previously fatal conditions.¹⁰⁶

In sum, the reference pricing proposal has the very real potential to do harm without doing any good.

2. BIO members are concerned with aspects of CMS's "indications-based pricing" proposal, which appears more like LCA than a true indications-based arrangement.

CMS proposes "using value-based pricing to vary prices for a given drug based on its varying clinical effectiveness for different indications that are covered under existing

¹⁰⁵ See, e.g., D.N. Lakdawala et al, *An economic evaluation of the war on cancer*, 29 J. of Health Econ. 333-346 (2010); FDA, Consumer Update: Faster, Easier Cures for Hepatitis C, available at <http://www.fda.gov/ForConsumers/ConsumerUpdates/ucm405642.htm> (last updated July 28, 2014); H. Samji et al., *Closing the Gap: Increases in Life Expectancy among Treated HIV-Positive Individuals in the United States and Canada*, 8 PLoS ONE e81355 (2013).

¹⁰⁶ P. Dylst, A.G. Vulto & S. Simoens, *Reference pricing systems in Europe: characteristics and consequences*, Figure 1, p. 130. 2 GENERICS AND BIOSIMILARS INITIATIVE J. 127-31 (2012).

Medicare authority.”¹⁰⁷ We have serious concerns with respect to this “indications-based pricing” proposal, which appears more like LCA than a true indications-based arrangement. This provides yet another reason for CMS to withdraw the proposed rule and instead prioritize working with its sister agencies and other stakeholders to create a safe and predictable regulatory environment that enables experimentation with these models in the private sector. As noted previously, BIO looks forward to continuing our dialogue with the Agency to identify workable solutions in this area.

Specifically, BIO has concerns regarding CMS’s proposal “to use indications-based pricing where appropriately supported by studies and reviews of evidenced-based clinical practice guidelines.”¹⁰⁸ While we generally agree with many of the Agency’s broader statements about the type of evidence that qualifies as “high quality evidence” for this purpose (e.g., comprehensive; ideally based on randomized trial designs; research findings that are valid, competent, reliable, and generalizable to the Medicare population),¹⁰⁹ BIO is very concerned about the suggestion that the comparative-effectiveness and cost-effectiveness reports developed by the Institute for Clinical and Economic Review (ICER) would be used to inform the reimbursement for each indication of a given product.¹¹⁰

Not only does BIO have serious concerns about the methodology and non-transparent process employed by ICER to establish these drug reviews, we are concerned that CMS has provided no rationale as to why reports by ICER, in particular, were selected for this purpose. We also note that the use of any cost-effectiveness reviews by CMS to inform the development of indications-based pricing models suggests that CMS’s version of indication-based pricing conflates two models and is actually just another version of LCA in disguise. This impression is bolstered by the example provided in the preamble text, which suggests that CMS’s “indications-based pricing” will depend on the **relative** performance of each indication versus therapies on the market for that indication.¹¹¹

We cannot emphasize enough BIO’s very serious concerns with the use of LCA of any flavor, which has the very real potential to impede patient access and stifle innovation for all of the reasons described in [section VI](#), above. It is therefore essential that any indications-based arrangements focus solely on how a therapy performs for one indication relative to another indication, and not relative to another therapy.

Moreover, while BIO members are interested in exploring true indications-based arrangements—at least on a voluntary basis in the private sector—we are deeply concerned that CMS does not address any of the many complex and difficult challenges with, or

¹⁰⁷ 81 Fed. Reg. at 13,242.

¹⁰⁸ 81 Fed. Reg. at 13,243.

¹⁰⁹ 81 Fed. Reg. at 13,244.

¹¹⁰ 81 Fed. Reg. at 13,245 (“The Institute for Clinical and Economic Review (ICER) is currently producing reports on high-impact drugs that analyze comparative effectiveness and cost-effectiveness before calculating a benchmark price for each drug. ICER’s reports reflect the dependence of the value of medications on evidence available for certain target populations.”) (internal citations omitted).

¹¹¹ 81 Fed. Reg. at 13,243 (“For example, if a drug is introduced with indications for treating two types of cancer and this drug did no better in clinical trials *than existing treatments* for the first type of cancer and significantly better *than existing treatments* for the second, our use of indications-based pricing might result in lower payments when the drug is used to treat the first type of cancer and higher payments when the drug is used to treat the second type.”) (emphasis added).

limitations to, the use of such arrangements in the Proposed Rule. Perhaps most notably, as with all value-based arrangements, indications-based arrangements are not appropriate for all products or indications. This is the case for a number of reasons, including:

- As the Agency expressly recognizes, “the quality of available evidence can vary for any given drug or indication.”¹¹² Value-based arrangements of any type should not be pursued for those drugs or indications for which high-quality evidence does not exist.
- Some disease states, such as cancer, are exceedingly complex, making the adoption of indications-based pricing arrangements particularly difficult to pursue.¹¹³ We were therefore concerned that CMS, in the Proposed Rule, illustrates the concept of indications-based pricing using an oncology-specific example (i.e., a product approved to treat two different types of cancer with differing degrees of comparative effectiveness).¹¹⁴
- There are a number of significant regulatory and other barriers to pursuing indications-based arrangements for products with only one FDA-approved indication (or, in this context, only one FDA-approved indication relevant to the Medicare Part B program).
- Data limitations counsel against the use of these arrangements where the product’s indications are all for a single disease state. Indeed, as ICER notes in their report, “[v]ery distinct indications make it easier to use existing data systems to identify the different indications for which a single drug is used.”¹¹⁵

There also are significant legal and operational concerns that must be addressed before these models could succeed. These include, for example:

1. Government Price Reporting: As CMS is no doubt aware, government price types—including not only ASP, but also Medicaid Best Price, Average Manufacturer Price (AMP), and the 340B ceiling price—are calculated and reported based on the National Drug Code (NDC). The current NDC regime assigns a single NDC to each drug, regardless of indication. This has implications for indications-based pricing arrangements because, as ICER noted in their report, “if sufficiently low enough, the price assigned to a drug within an indication-specific framework could interfere with existing reimbursement mechanisms used by Medicaid and Medicare as well as impact the mandated price to 340B eligible entities and lead to unintended market disruption.”¹¹⁶ Specific to physician-administered drugs, as ICER notes in its report, if indication-specific pricing were applied to physician-administered drugs under the current buy-and-bill model, the lower price for the less-effective indication could

¹¹² 81 Fed. Reg. at 13,244.

¹¹³ Indications for therapies that treat cancer currently rely on a reference to the site (e.g., organ) in which the initial lesion presented. However, emerging scientific evidence suggests that cancers may be more appropriately categorized based on their genetic profile, and how the human immune system interacts with them, rather than by where a tumor may have first appeared. Given the disconnect between how uses of a product are approved by the FDA and the realities of the emerging science, there is much work that HHS can do to better understand clinical practice before considering the adoption of value-based arrangements pertaining to these products.

¹¹⁴ 81 Fed. Reg. at 13,243.

¹¹⁵ Institute for Clinical and Economic Review (ICER), *Indication-Specific Pricing of Pharmaceuticals in the United States Health Care System* (March 2016).

¹¹⁶ Id.

lower the ASP-based reimbursement for the drug as a whole, so that reimbursement amounts for the more expensive indication may be insufficient to cover the cost of the drug.

2. Patient Cost-Sharing: Related to the potential for indications-based pricing to have a negative impact on provider reimbursement is the very real potential that patients could face differential cost-sharing obligations based on their specific condition or indication. However, as ICER highlights, “patients with the same purchaser [having] different co-pays for the same drug depending on their condition, or their sub-population with the same condition” would be perceived as inequitable.¹¹⁷
3. Coding: As ICER noted in its report, establishing indications-based pricing may not be possible “absent changes to the way in which drugs are identified during billing.”¹¹⁸ For example, indications-based pricing absolutely cannot work with the blended Healthcare Common Procedure Coding System (HCPCS) codes that CMS has proposed to use for biosimilar biologics that share a reference product. However, changes in coding, or the creation of additional codes, has the very real potential to further increase the administrative burden of value-based arrangements on healthcare providers and others.
4. Off-Label Promotion: It also is not clear if/how an indications-based model would work for unapproved indications, which is a concern in light of the FDA’s restrictions on manufacturers’ promotion of unapproved indications. Reimbursement and contracting system infrastructure is not in place to track off-label indications and FDA restrictions prohibit much of this activity. For this reason, ICER has suggested that “[d]rugs that have significant off-label uses, including ones that may be supported by research, guidelines, and compendia, are unlikely to be suitable candidates for indication-specific pricing.” Nonetheless, there remains the potential that a provider may, in his or her clinical judgment, prescribe **any** drug—even drugs without significant off-label uses—for an off-label indication. It thus remains an open question what price (if any) would apply to those indications and/or whether there would be any flexibility for manufacturers to enter into contract negotiations for indications that represent off-label use.
5. Data Systems: As noted in the ICER report, “data systems necessary to administer some models of indication-specific pricing may be difficult to develop and use, raising uncertainty about resources that must be devoted to the successful implementation of indication-specific pricing strategies.”¹¹⁹ For instance, ICER further notes that “linking drug prices with indications is extremely difficult,” given that “the point at which payment is made is far removed from the point at which the drug is delivered to an individual patient for a specific indication.” This is true for both physician-administered and pharmacy-dispensed medications. Specifically, according to ICER, “standard pharmacy claims data for indication-specific pricing are not useful” and

¹¹⁷ Id.

¹¹⁸ Id.

¹¹⁹ Id.

"[m]edical benefit claims for drugs are generally associated with a 3-month time lag," while "[e]ven electronic medical records (EMRs) rarely contain indication information in a format that links through to the pharmacy benefit."

6. Potential for Abuse: As ICER noted in its report, "[i]f two prices are available in the marketplace, purchasers may be incentivized to buy the drug at the lowest price with the intent of using it for the indication that should merit a premium price."¹²⁰ CMS notably did not address how the Agency would prevent/penalize diversion in the Proposed Rule (i.e., providers/suppliers purchasing the product for the lower priced indication and then billing for the higher priced indication). CMS also did not address how the Agency would communicate to providers, and the public, the rationale for having two prices for the same therapy. Indications-based pricing/reimbursement would then put CMS in the crosshairs of public scrutiny over its inability to enforce, track use, and rationalize variable pricing for the same medicine.

3. Numerous considerations remain with respect to outcomes-based risk-sharing arrangements.

CMS also proposes that the Agency will enter into voluntary agreements with manufacturers to link healthcare outcomes with payment. Specifically, the Agency is interested in building off of private-sector "outcomes-based risk-sharing agreements" that "link payment for drugs to patient health outcomes."¹²¹ BIO is generally supportive of this type of arrangement, at least in concept, as we believe it is the most likely of the proposed models to meet our criteria for value-based arrangements—in particular that the arrangement remain centered on maximizing the value of biopharmaceuticals for **the patient**. That said, while BIO members are interested in exploring these arrangements in the private sector, we note that there remain considerable challenges to doing so.

Indeed, the article CMS cites in describing these arrangements is titled "Risk-Sharing Arrangements That Link Payment For Medications to Health Outcomes Are Proving Hard to Implement." As one might expect from the title, this article found that there are actually very few true outcomes-based risk-sharing arrangements in place in this country, and that these models are generally very small in scale. The principal lesson the authors draw from their research is that "risk sharing for pharmaceuticals is appealing in theory but hard in practice."¹²² This challenge stems from an array of operational, legal, and other considerations that must be addressed before these arrangements can get off the ground. This provides yet another reason for CMS to withdraw the Proposed Rule and instead prioritize establishing a safe and predictable regulatory environment to facilitate the successful adoption of these models in the private sector. Challenges to the adoption of outcomes-based risk-sharing models include:

1. Government Price Reporting: As is true with indications-based arrangements, there are a number of ways in which manufacturers could potentially account for

¹²⁰ Id.

¹²¹ 81 Fed. Reg. at 13,244.

¹²² P.J. Neumann et al, *Risk Sharing Arrangements that Link Payment for Drugs to Health Outcomes are Proving Hard to Implement*, 30 HEALTH AFFAIRS 2329-37 (2011).

outcomes-based risk-sharing arrangements in their government price reporting calculations. Because CMS has not issued specific guidance with respect to such treatment, manufacturers are permitted to employ reasonable assumptions in their calculations.¹²³ However, significant risk remains given the novelty and variability of these arrangements and the unknown perspective of enforcement authorities. The government price reporting treatment of these arrangements is an issue that CMS, in consultation with OIG and other stakeholders, should directly address in order to facilitate the adoption and use of value-based arrangements going forward.

2. Patient Cost-Sharing: Consistent with BIO's criteria for value-based arrangements, to the extent that an outcomes-based risk-sharing arrangement resulted in varying tier structure and/or reimbursement rates, beneficiaries should not face any increases in out-of-pocket costs that would otherwise occur as a result.
3. Methodology: While CMS suggests that outcomes-based risk-sharing arrangements "tie the final price of a drug to results achieved by specific patients **rather than using a predetermined price based on historical population data**,"¹²⁴ we note that the "value-based" arrangements present in the marketplace today often rely on historical data to set pre-determined prices. This reflects the challenges of obtaining real-time quality/outcomes data on a patient-by-patient basis, as well as the government price reporting considerations associated with moving away from per-unit pricing.
4. Metrics: The specification and determination of treatment effects in non-randomized settings is extremely challenging, given that these effects often are heavily influenced by disease and other factors outside the control of a given manufacturer that can compromise outcomes (e.g., local practice styles, poor patient treatment adherence). BIO therefore agrees, in concept, with the idea of relying on data provided by manufacturers to "create an accurate picture regarding clinical value for a specific drug," as well as manufacturer-provided "outcomes measures for any outcomes-based risk-sharing pricing agreement."¹²⁵ However, we note that manufacturers are somewhat limited in their ability to provide both types of information to CMS.

First, as noted previously, manufacturers' ability to discuss the clinical value of their drugs can, in some instances, be limited by the FDA's limitations on off-label promotion, as often the metrics of interest to a payor (e.g., Medicare) may not be in the product's label. Second, appropriate, validated measures do not currently exist for every therapy, condition, or indication, and—even where there are appropriate measures—evaluating a drug's performance using these metrics in the context of an outcomes-based risk-sharing arrangement has proven difficult (e.g., because of the

¹²³ See National Drug Rebate Agreement § II(i) (permitting manufacturers to make reasonable assumptions in their calculation of AMP [Average Manufacturer Price] and Best Price, in the absence of specific statutory guidance, federal regulations, and the terms of this agreement).

¹²⁴ 81 Fed. Reg. at 13,244 (emphasis added).

¹²⁵ 81 Fed. Reg. at 13,244.

varying course of the disease, or because the measurement is subject to interrater variability).¹²⁶

Moreover, metrics with longer time horizons—which may be the most appropriate, clinically, for the drug in question—are extremely difficult to execute and enforce in the context of an outcomes-based risk-sharing arrangement, and can create potential government price-reporting implications if the outcome of interest occurs outside of the three-year restatement period.

5. Data Systems: As with indications-based pricing, risk-sharing arrangements require high-quality information systems, databases, and operational and analytic expertise, as these arrangements often involve matching data from across the healthcare system (e.g., for pharmacy and medical services), which often is housed in separate systems in payor databases. Linking payment to specific metrics also generally necessitates an upgrade to existing data infrastructure.

4. Any patient cost-sharing models must ensure that patients have equal access to medicines that truly deliver the greatest value to them.

CMS also is proposing a “value-based pricing strategy that involves discounting or eliminating patient coinsurance amounts for services that are determined to be high in value in an attempt to tailor incentives.”¹²⁷ BIO supports the idea, at least in concept, that beneficiaries should see a financial benefit for selecting the therapy that is the most “valuable” among truly comparable choices, as well as CMS’s express proposal that cost-sharing would not exceed 20%. However, our support of this concept—commonly referred to as value-based insurance design (VBID)—extends only so far as the models are designed correctly. Specifically, patients must have equal access to the medicines that truly deliver the greatest value to them. We therefore have two important concerns regarding CMS’s proposal.

First, we are concerned that this proposal will not actually benefit Medicare beneficiaries. To help cover Medicare’s cost-sharing requirements, most Medicare beneficiaries have a supplemental form of coverage, including Medigap policies and employer- or union-sponsored retiree health plans.¹²⁸ Accordingly, a reduction in cost-sharing, for many beneficiaries, would not translate to a direct reduction in patient out-of-

¹²⁶ PJ Neumann et al., *Risk Sharing Arrangements that Link Payment for Drugs to Health Outcomes are Proving Hard to Implement*, 30 HEALTH AFFAIRS 2329-2337 (2011) (“Only certain types of outcomes may prove suitable. Ideally, they should be objective, clearly defined, reproducible, and difficult to manipulate.”).

¹²⁷ 81 Fed. Reg. at 13,244.

¹²⁸ See Kaiser Family Foundation, *Medigap Enrollment Among New Medicare Beneficiaries: How Many 65-year olds enroll in plans with first-dollar coverage* (Apr. 2015), available at <http://files.kff.org/attachment/issue-brief-medigap-enrollment-among-new-medicare-beneficiaries>. We note that, while many low-income beneficiaries receive supplemental coverage through the Medicaid program, this assistance does not always fully cover the cost of Medicare cost-sharing. For instance, a Medicaid and CHIP Payment and Access Commission (MACPAC) report found that, in 2012, 39 states had policies that allowed them to pay less than the Medicare cost-sharing for at least one service type if the Medicaid payment rate for that service was lower than the Medicare rate. MACPAC, *2015 Report to Congress on Medicaid and CHIP, “Chapter 6: Effects of Medicaid Coverage of Medicare Cost Sharing on Access to Care”* (2015), available at <https://www.macpac.gov/wp-content/uploads/2015/03/Effects-of-Medicaid-Coverage-of-Medicare-Cost-Sharing-on-Access-to-Care.pdf>.

pocket costs. In addition, we note that patient out-of-pocket costs often do not influence which Part B therapies are prescribed; rather prescribing decisions are made based on the best therapy for the patients' condition, as we describe above. In sum, we wonder if this proposal would actually be meaningful from a patient perspective.

Second, we are concerned that the proposal would not benefit, and might actually harm, those beneficiaries who lack supplemental coverage and thus could be affected by it. In particular, if this policy were to be combined with any of CMS's other Phase II proposals—such as reference pricing or CMS's "indications-based pricing"—beneficiaries could actually see cost-sharing rates that exceed 20% of the statutory reimbursement amount.

We also are concerned about the methodology that will be used to assess which therapy is the most "valuable." It is particularly critical, where the beneficiary's cost-sharing is involved, to ensure that the metrics employed for this purpose are not only patient-centric, but also specific to the beneficiary's condition. Metrics that over-generalize (e.g., that determine the most "valuable" product across an entire therapeutic area), are likely to underappreciate the value of a given therapy for a particular indication/condition in an individual clinical circumstance. Along these lines, BIO has concerns that CMS is "not proposing manufacturer-specific or NDC-specific cost sharing amounts."¹²⁹ We believe that this approach has the potential to ignore the unique value different products may have for specific patients by collectively evaluating the "value" of all products that share a HCPCS code. In sum, we are concerned that CMS's proposal would prove to be inequitable, in that some patients would benefit while others would not. Also, while not addressed in the Proposed Rule, under no circumstances should any VBID policy interfere with beneficiaries' existing access to patient assistance programs.

VIII. Payment Methodology: Development of a Clinical Decision Support Tool. BIO Has Serious Concerns Regarding CMS's Proposal to Develop a Clinical Decision Support Tool.

BIO supports initiatives that aim to improve the availability of relevant information to providers and patients at the point of clinical decision making. However, given the limitations of similar initiatives pursued by CMS—most notably the Quality and Resource Use Reports (QRURs) distributed for purposes of the physician Value-Based Modifier Program—we have serious concerns about CMS's ability to develop useful, meaningful, and evidence-based tools in this space.

As an initial matter, we question whether there is a need for CMS to develop its own Clinical Decision Support (CDS) tool. While we agree that such resources can have significant clinical utility, the details of how evidence is judged, by whom, and how often it is updated will determine whether these tools facilitate, or hinder, patient access to high-quality care. Currently, medical specialty societies have developed and maintain such tools, and are doing so in an increasingly sophisticated manner. These organizations, as opposed to CMS, have the ability to develop and update CDS tools to keep pace with the evolution of

¹²⁹ 81 Fed. Reg. at 13,244.

medicine and to incorporate emerging real-world data on the utilization of medicines. They can closely monitor changes in the standard of care, and can be sufficiently nimble to adapt the format and content of CDS tools to be as responsive to provider needs as possible. Thus, CMS's efforts in this space may be redundant, and even detrimental, to the extent that they detract from the uptake of these specialty-specific resources.

In considering the Agency's proposals, we also note our strenuous objection to any mandatory use of CDS tools, either now or in the future, as providers should always retain the ability to make treatment decisions using their clinical judgment, based on the best treatment option for the individual patient.

Moreover, while BIO generally supports CMS's proposal to communicate data back to each healthcare provider regarding his/her performance on specific measures relative to some type of average, in developing this component of the CDS tool, CMS must first address existing concerns with the QRURs currently provided to Medicare providers (e.g., providers have described the information included in QRURs as confusing and not immediately relevant to their clinical practice). In establishing this second CDS component, we also urge CMS to ensure that providers are only compared to other, similar providers, defined as those: (1) of the same specialty, and where applicable, subspecialty; (2) participating in a similar sized practice (and potentially in a similar geographical area (e.g., rural vs. urban)); and (3) treating similar patient populations, including with regard to the population's underlying health and prognoses (i.e., that provider comparisons include a risk-adjustment component). The information on provider performance that the CDS tool relays to individual providers will not be meaningful or useful unless providers can be certain that the comparisons being made are appropriate. Also, under no circumstances should this information be used to mandate the use of CDS tools—either directly or indirectly.

IX. Provider, Supplier, and Beneficiary Protections. The proposed Payment Exceptions Review process is not sufficient to ensure that patients are able to access the medicines that are most appropriate for their individual clinical circumstances.

In the Proposed Rule, CMS proposes to establish a "Pre-Appeals Payment Exceptions Review" process that will allow providers and patients an opportunity to dispute payments made under Phase II before proceeding through the existing Medicare appeals process. BIO is concerned that this proposal is inadequate to protect patient access to needed medicines, as the ability to engage in a pre-appeal exceptions process is hardly a guarantee of such access.

In particular, we are concerned with the scope of the process: the Exceptions Review process only applies to Phase II of the Proposed Rule, neglecting to protect patients in Phase I (discussed in more detail in [section VI.D](#)). The Exceptions Review process also only allows patients and providers to request an exception to payment made under Phase II. However, patients may require the opportunity to request exceptions based on any aspect of a demonstration program's design that impedes access to needed care. For example, CMS's determinations with regard to therapeutic class under the proposed reference pricing scheme could dramatically impact patient access to appropriate medicines. Yet, under the

proposed Exceptions Review process, patients and providers would only be allowed to submit exceptions based on the payment for an individual medicine, but not challenge the broader determination that resulted in the negative impact to patient access in the first place.

In addition to the scope of the Exceptions Review process, BIO is concerned with the lack of detail included in the Proposed Rule with regard to operationalizing such a process. For example, it is unclear what metrics the proposed contractor will use to evaluate exceptions requests, and there are no details as to how burdensome the process may be for patients and providers to navigate.

Similarly, it is unclear whether patients will have access to the medicine in question during the duration of the exceptions process, a critical provision that minimizes disruptions in care or delays in the initiation of care. This protection is particularly important given that CMS proposes to allow the contractor up to 5 business days to respond to an exceptions request. BIO finds this timeframe to be exceedingly lengthy, and reflective of a lack of understanding of the needs of patients who utilize Part B therapies. These patients are some of the sickest and most vulnerable, who often suffer from complex, chronic conditions such as rheumatoid arthritis, Crohn's disease, cancer, and rare diseases (e.g., lysosomal storage disorders, hemophilia, and multiple sclerosis). Delays, even relatively short delays, in access to needed medicines can seriously impact these patients' short- and longer-term health outcomes.

Given the proposed delay in access associated with the Exceptions Review process, BIO is equally concerned that utilizing this process will ultimately delay, rather than facilitate, patient access to needed therapies, especially in instances in which patients must subsequently navigate the existing appeals process.

X. Proposed Waivers of Medicare Program Rules. BIO has serious legal questions regarding CMS's proposed "waivers."

BIO has serious legal questions regarding CMS's proposed waivers. We address our concerns with each of the Agency's specific waiver proposals, in turn. However, as a threshold matter, we note that CMS is not authorized to use any waivers in the context of the Proposed Rule, as the CMMI statute limits waivers to "Phase I" testing.

The Proposed Rule would represent a large-scale program change, rather than a true "Phase I" test. As noted in [section III.B](#), above, section 1115A does not allow CMMI to skip ahead to a "Phase II" expansion without engaging in the "Phase I" testing. But, even when a model is properly expanded in accordance with the statutory provisions, CMMI's waiver authority ends with Phase I, because it applies "solely for purposes of carrying out [Phase I testing]."¹³⁰

¹³⁰ Section 1115A's waiver authority is available only "as may be necessary solely for purposes of carrying out this section **with respect to testing models described in subsection (b)**." SSA § 1115A(d)(1) (emphasis added). Notably, subsection (b) describes the testing of models under "Phase I." The expansion phase of CMMI's demonstrations, on the other hand, is described in subsection (c) of the statute.

A. CMS’s proposed “Waiver” of SSA § 1847A(b)(1) cannot stand as it either exceeds CMMI’s statutory authority, or the statute risks vulnerability on constitutional grounds should it be viewed as unlawfully permitting a unilateral abrogation of duly enacted statutory text by the executive branch contrary to Art. I, §7.

In the Proposed Rule, CMS proposes to “waive portions of section 1847A(b)(1) of the Act which specify the six percent add-on percentage for payments determined under section 1847A of the Act.”¹³¹ Moreover, putting together the proposed waiver provisions,¹³² and the provision describing the proposed model structure,¹³³ CMS has actually proposed to **rewrite** section 1847A(b)(1) to replace “6” with “2.5.” Indeed, according to CMS itself “[w]aiving the fixed add-on percentage will allow the agency to **modify** the add-on percentage for payment determinations made under section 1847A of the Act” across virtually all Medicare Part B-covered products.¹³⁴ This proposal is deeply concerning and cannot stand for one of the following two reasons: it either exceeds CMMI’s statutory authority, or, if the Agency’s statutory authority is so broad to allow a unilateral abrogation of duly enacted statutory text by the executive branch, the statute itself risks vulnerability on constitutional grounds. We discuss these two alternatives, in turn.

First, the proposed waiver would be found unlawful if CMS were found to be acting beyond their statutory authority. We believe this is, indeed, the case here because the Agency has proposed to “modify” (rather than merely waive) statutory text. By contrast, Section 1115A(d)(1) allows CMMI only to “**waive** such requirements of title[] . . . XVIII . . . as may be necessary solely for purposes of carrying out this section with respect to testing the models described” in CMMI’s authorizing statute.¹³⁵ Thus, while the list of statutory provisions that may be waived using this authority is rather lengthy—to include all of title XVIII—the Agency’s only permissible regulatory action with regard to these provisions is strictly limited to waiver.¹³⁶ Indeed, had Congress intended for CMS to take actions other than waiver (e.g., modification), they would have specified as much in the statute.¹³⁷

¹³¹ 81 Fed. Reg. at 13,251.

¹³² 42 C.F.R. § 511.400, et seq. (proposed).

¹³³ 42 C.F.R. § 511.205(c) (proposed).

¹³⁴ 81 Fed. Reg. at 13,251 (emphasis added) (“The waiver for the add-on encompasses single source drugs, biologicals, multiple source drugs and biosimilars” as well as drugs paid based on Wholesale Acquisition Cost. CMS also proposes to waive the detailed statutory reimbursement methodologies in section 1842(o) of the SSA).

¹³⁵ SSA § 1115A(d)(1) (emphasis added).

¹³⁶ BLACK’S LAW DICTIONARY (10TH ED. 2014) (defining “waive” to mean “[t]o refrain from insisting on (a strict rule, formality, etc.); to forego”). See also *FDIC v. Meyer*, 510 U.S. 471, 476 (1994) (“In the absence of a statutory definition, we construe a statutory term in accordance with its ordinary or natural meaning.”).

¹³⁷ Elsewhere in the ACA, where Congress intended to authorize an agency to waive *or modify* a statute, it said so explicitly. See, e.g., ACA § 10408 (enacting a provision that would allow the Director of the National Institutes of Health to “waive **or modify** a matching fund requirement under certain circumstances) (emphasis added). Similar examples exist in the Social Security Act that predate the ACA. See SSA § 1135(b) (authorizing the Secretary of HHS to “temporarily waive **or modify**” the application of certain statutory provisions under certain circumstances.) (emphasis added). The case law supporting the axiom that “Congress knows how to say” is extensive. See, e.g., *Dole Food Co. v. Patrickson*, 538 U.S. 468, 476 (2003) (observing that Congress knows how to refer to an ‘owner’ “in other than the formal sense,” and did not do so in the applicable Act); *Whitfield v. United States*, 543 U.S. 209, 216 (2005) (“Congress has included an express overt-act requirement in at least 22 other current conspiracy statutes, clearly demonstrating that it knows how to impose such a requirement when it wishes to do so.”).

We think it unlikely, however, that Congress did, in fact, intend for CMMI to do anything other than waive broad statutory requirements with its 1115A authority. Indeed, the legislative history behind the enactment of section 1115A describes waivers historically operated in the Medicare context. Notably, these waiver authorities had been used solely to *waive* general Medicare requirements (e.g., the physician self-referral law), not to *modify* or redraft them.¹³⁸ Similarly, CMS's longstanding section 1115 waiver authority—also referenced in the ACA's legislative history—had historically been used to allow State Medicaid programs to forgo broad federal requirements that would otherwise limit states' ability to operate and test new models of care (e.g., comparability, freedom of choice, and statewideness), or to **add** populations and services not eligible for Federal matching payments under the Medicaid State plan.¹³⁹ By contrast, as far as we are aware, there is no precedent for CMS developing or approving a waiver that seeks not only to cancel statutory text, but also to overwrite or modify it—as CMS itself has described its proposed actions here.

That said, while there is a general principle that statutes must be interpreted to avoid raising constitutional questions,¹⁴⁰ if section 1115A could be read to authorize CMS's proposal to overwrite duly enacted statutory text, it risks vulnerability on constitutional grounds should it be viewed as unlawfully permitting a unilateral abrogation of duly enacted statutory text by the executive branch contrary to Art. I, § 7 of the United States Constitution.

Article I, §7, often referred to as the "Presentment Clause," sets out a clear, elaborate process for the enactment, amendment, and repeal of federal legislation, namely bicameralism and presentment. This provision "provide[s] for an exhaustive legislative procedure by dividing lawmaking authority amongst three institutions: the House, the Senate, and the President."¹⁴¹ The Supreme Court has described this process as "a single, finely wrought and exhaustively considered procedure,"¹⁴² holding that "the power to enact statutes may only 'be exercised'" according to that procedure.¹⁴³ Importantly, this procedure provides the veto as the President's only official tool to cancel legislative text.

¹³⁸ See, e.g., CMS, Physician-Hospital Collaboration Demonstration, https://www.cms.gov/Medicare/Demonstration-Projects/DemoProjectsEvalRpts/downloads/phcd_646_solicitation.pdf; CMS, Medicare Modernization Act, Section 646, Medicare Health Care Quality Demonstration Programs, https://www.cms.gov/Medicare/Demonstration-Projects/DemoProjectsEvalRpts/downloads/MMA646_FactSheet.pdf. See also CMS, Presentation: Medicare Shared Savings Program Accountable Care Organization: Preparing to Apply for 2017 (Apr. 5, 2016), available at <https://www.cms.gov/Outreach-and-Education/Outreach/NPC/Downloads/2016-04-05-MSSP-Presentation.pdf> (describing waivers to the Skilled Nursing Facility three-day rule).

¹³⁹ See MACPAC, Waivers, <https://www.macpac.gov/subtopic/waivers/> (last visited Apr. 12, 2016).

¹⁴⁰ See *NFIB v. Sebelius*, 567 U.S. ____ (2012) ("And it is well established that if a statute has two possible meanings, one of which violates the Constitution, courts should adopt the meaning that does not do so. Justice Story said that 180 years ago: "No court ought, unless the terms of an act rendered it unavoidable, to give a construction to it which should involve a violation, however unintentional, of the constitution." *Parsons v. Bedford*, 3 Pet. 433, 448-449 (1830). Justice Holmes made the same point a century later: "[T]he rule is settled that as between two possible interpretations of a statute, by one of which it would be unconstitutional and by the other valid, our plain duty is to adopt that which will save the Act." *Blodgett v. Holden*, 275 U. S. 142, 148 (1927) (concurring opinion).").

¹⁴¹ Mitchell J. Widener, *The Presentment Clause Meets the Suspension Power: The Affordable Care Act's Long and Winding Road to Implementation*, 109 B.U. PUB. INT. L.J. 109, 114-115 (2015).

¹⁴² *INS v. Chada*, 462 U.S. 919, 951 (1983).

¹⁴³ *Clinton v. New York*, 462 U.S. 417, 439 (1998) (quoting *Chada*, 462 U.S. at 951).

While courts have long upheld congressional delegation of legislative authority to Executive Branch agencies in order to implement and build upon duly enacted statutory text, provided certain criteria are met,¹⁴⁴ this functionalist view of separation of powers is less appropriate when Congress delegates the power to **negate** legislative bargains struck under the strict constitutional structure.¹⁴⁵

Any efforts by the executive branch to not only waive, but also overwrite or modify statutory text raise even greater constitutional concerns. Under its express terms, Art. I, § 7 prohibits abrogation or amendment of statutory text without bicameralism and presentment. Thus, any statute that would permit the Secretary to overwrite express statutory requirements via administrative rulemaking stands in stark contrast to the Constitution's clear directive that "[a]mendment and repeal of statutes, no less than enactment, must conform with Art. I."¹⁴⁶ This is particularly true with respect to a statute that confers the authority for an agency to overwrite statutory text that is especially precise in its terms.

One important purpose of bicameralism and presentment is to promote the use of caution and deliberation and to decrease the "incidence of hasty and ill-considered legislation."¹⁴⁷ As the Supreme Court has explained:

The legislative steps outlined in Art. I are not empty formalities; they were designed to assure that both Houses of Congress and the President participate in the exercise of lawmaking authority. This does not mean that legislation must always be preceded by debate . . . But the steps required by Art. I, §1, 7 make certain that there is an opportunity for deliberation and debate. To allow Congress to evade the strictures of the Constitution and in effect enact Executive proposals into law by mere silence cannot be squared with Art. I.¹⁴⁸

In sum, lawmaking inevitably reflects compromises. As the specificity of the statutory text increases, so does the cost of reaching these compromises, thus "the less permissible it

¹⁴⁴ *Mistretta v. United States*, 488 U.S. 361, 371-723 (1989) (holding that, while "[t]he Constitution provides that '[a]ll legislative Powers herein granted shall be vested in a Congress of the United States,' and we long have insisted that 'the integrity and maintenance of the system of government ordained by the Constitution' mandate that Congress generally cannot delegate its legislative power to another Branch," "[o]ur jurisprudence has been driven by a practical understanding that in our increasingly complex society, replete with ever changing and more technical problems, Congress simply cannot do its job absent an ability to delegate power under broad general directives.") (citations omitted) (quoting U.S. CONST. ART. I, § 1 and *Field v. Clark*, 143 U.S. 649, 692 (1892)). As a general matter, to prevent the delegation of the broader "legislative power" that is constitutionally granted solely to Congress, the Court requires that any such delegation include an "intelligible principle" constraining the exercise of discretion by another branch. R. Craig Kitchen, *Negative Lawmaking Delegations: Constitutional Structure and Delegations to the Executive of Discretionary Authority to Amend, Waive, and Cancel Statutory Text*, 525 HASTINGS CONST. L.Q. 525, 535 (2013).

¹⁴⁵ Kitchen, *Negative Lawmaking Delegations*, *supra* note 144 at 543. ("The underlying premise is that, when the delegated power is one to *negate* law from having 'legal force or effect,' that delegation violates Article I, Section 7 and 'the Framers' decision that the legislative power of the Federal government be exercised in accord with a single, finely wrought and exhaustively considered procedure.") (citing *Chada*, 462 U.S. at 951 (1983)). *See also Clinton*, 524 U.S. at 439-40 (citing *Chada*, 462 at 951).

¹⁴⁶ *INS v. Chada*, 462 U.S. at 954.

¹⁴⁷ Kitchen, *Negative Lawmaking Delegations*, *supra* note 144 at 587.

¹⁴⁸ *INS v. Chada*, 462 U.S. 919, 958 n. 23 (1983).

should be to negate that text through a streamlined process such as unilateral Executive action."¹⁴⁹

Along these lines, few statutes are more specific and rule-like than section 1847A—which Congress enacted in order to “minutely detail[] the reimbursement rates for covered items and services.”¹⁵⁰ A unilateral executive action should not suffice to override this bargain painstakingly made by “constitutionally delineated actors,”¹⁵¹ even if an agency believes it has developed a better policy.¹⁵² Thus, were section 1115A to be read to authorize the re-writing of this precise language, including the carefully-crafted reimbursement methodology for biosimilars described in [section IV.A](#), above, it cannot stand on constitutional grounds—even in spite of the time-limited nature of the waiver authority in question¹⁵³—particularly given that it also limits administrative and judicial review,¹⁵⁴ thereby eliminating a procedural protection that would otherwise provide an “essential check on federal executive overreaching and interference.”¹⁵⁵

¹⁴⁹ Kitchen, *Negative Lawmaking Delegations*, *supra* note 144 at 594.

¹⁵⁰ See *Hayes v. Sebelius*, 589 F.3d 1279 (D.C. Cir. 2009) (finding, in striking down CMS’s Least Costly Alternative policy, that “we think it quite unlikely that Congress, having minutely detailed the reimbursement rates for covered items and services, intended that the Secretary could ignore these formulas whenever she determined that the expense of an item or service was not reasonable or necessary.”) (citing *Hays v. Leavitt*, 583 F. Supp. 2d 62, 71 (D.D.C. 2008)).

¹⁵¹ Kitchen, *Negative Lawmaking Delegations*, *supra* note 144 at 585.

¹⁵² See *MCI Telecommc’ns. Corp. v. Am. Tel. & Tel. Co.*, 512 U.S. 218, 220 (1994) (statutory delegation at issue, 47 U.S.C. § 203(b)(2)(1998 ed. & Supp. IV), allowed the FCC to “**modify** any requirement made by or under” that particular section of the Communications Act of 1934.) (emphasis added.) Notably, however, notwithstanding this this explicit statutory authority to “modify,” the Supreme Court held that the fundamental nature of change that FCC sought to make to the applicable statutory provision exceeded the bounds of permissible modification. *Id.* at 229 (“Since an agency’s interpretation of a statute is not entitled to deference when it goes beyond the meaning that the statute can bear. . . the Commission’s [proposed policy] can be justified only if it makes a less than radical or fundamental change” in the Act’s tariff-filing requirement, which the Court described as the “heart” of the relevant section of the Communications Act.).

¹⁵³ Unilateral executive efforts to alter legislative text have been found constitutionally impermissible, notwithstanding the fact that the cancelled provisions retained some continuing legal effect; it is enough that **any** legal force or effect of enacted statutory text is altered outside of Art. I, §7 that gives rise to the constitutional concern. See *Clinton v. New York*, 524 U.S. at 447 (finding that the partial retention of legal force or effect was insufficient to save the Line Item Veto Act because “[t]he cancellation of one section of a statute may be the functional equivalent of a partial repeal even if a portion of the section is not cancelled.”). See also *MCI Telecommc’ns. Corp.*, 512 U.S. at 231 (describing the FCC’s elimination of a “crucial provision of the statute for 40% of a major sector of the industry” as “much too extensive to be considered a ‘modification,’” and finding it instead to be a “fundamental revision of the statute.”).

¹⁵⁴ See SSA § 1115A(d)(2). While we recognize that the statute does provide for the waiver of judicial review regarding CMMI’s “selection of models for testing and expansion,” we do not believe that this language would preclude judicial evaluation of CMMI’s identification of the instant proposal in its entirety. Specifically, section 1115A(b)(2) envisions an initial step before a CMMI model may be “selected”—namely, the Secretary must first identify models that address a “defined population” for which there are “deficits in care leading to poor clinical outcomes or potentially avoidable expenditures.” It is only from the pool of models that the Secretary determines meet these statutory criteria that the Secretary may, in turn, “select” a model for “Phase I” testing. We therefore believe, particularly in light of the common law presumption of judicial reviewability, see *Abbott Laboratories v. Gardner*, 387 U.S. 136, 140–41 (1967), that judicial review should remain available to, among other things, the initial determination that a model “addresses a defined population for which there are deficits in care,” which the statute dictates must precede a model’s “selection.” See SSA § 1115A(b)(2).

¹⁵⁵ Kate R. Bowers, *Saying What the Law Isn’t: Legislative Delegations of Waiver Authority in Environmental Laws*, 257 HARVARD ENV’T L.R. 257, 294 (2010) (noting that “broad delegations of legislative authority must be limited by procedural protections, including but not limited to the opportunity for judicial review” and that “[t]he limitation on judicial review thus removes an essential check on federal executive overreaching and interference with the interests of state and local governments and individuals.”).

We note that we have similar concerns regarding CMS's proposed waivers of sections 1833(t) and 1842(o), which also reflect an intent to overwrite duly enacted statutory text.

B. Given the many open questions regarding the structure and operation of models under Phase II of the Proposed Rule, CMS's remaining waiver proposals are overbroad and premature and thus provide insufficient notice to enable public comment.

BIO finds CMS's two remaining waiver proposals problematic in that they are overbroad as well as premature, and thus provide insufficient notice to enable public comment. Specifically, CMS proposes to waive both:

1. The definitions of single source drug or biological, multiple source drug, and biosimilar biological product (SSA § 1847(c)(6)); and
2. The provisions that require assignment of NDCs to HCPCS codes based on whether a drug meets the definition of single source drug or biological, multiple source drug, or biosimilar (SSA § 1847(b)).

The supporting rationale that CMS provides for both proposals is that "the determination of the model's payment amounts may not be consistent with the statutory definitions of single source drug or biological, multiple source drug, or biosimilar biologicals."¹⁵⁶ The example that CMS provides to support this proposition is that, under Phase II of the Proposed Rule, "equal or benchmarked payment for therapeutically similar drug products that are used for a given indication . . . is unlikely to be consistent with the[se] statutory definitions."¹⁵⁷

As a threshold matter, we question the constitutionality of a waiver of this scope—which would, in effect, eviscerate the clear, detailed, and prescriptive process for establishing reimbursement rates and coding policies for Medicare Part B drugs, biologics, and biosimilars. Moreover, given the many unanswered questions regarding Phase II of the Proposed Rule, we find these waiver proposals to be premature. Specifically, without further details regarding the nature, scope, and operation of the Phase II models, the Proposed Rule provides insufficient notice regarding the necessity or intended use of the proposed waivers, and we are thus unable to respond with informed comments.¹⁵⁸ And, as for the limited details that do appear on the Proposed Rule, we have very serious concerns with the idea of establishing "equal or benchmarked payment for therapeutically similar drug products," for the reasons articulated in [section IV.A](#), and thus do not support the creation of waiver authority for this purpose.

¹⁵⁶ 81 Fed. Reg. at 13,251.

¹⁵⁷ *Id.*

¹⁵⁸ The notice-and-comment procedures outlined in the Administrative Procedure Act are intended to product more informed agency decision making by encouraging public participation in the administrative process. To obtain "meaningful" participation from the public, courts have consistently held that the notice of proposed rulemaking must "fairly apprise interested persons" of the issues in the rulemaking. *See, e.g., United Steelworkers v. Marshall*, 647 F.2d 1189, 1221 (D.C. Cir. 1980) (quoting *Am. Iron & Steel Inst. v. EPA*, 568 F.2d 284, 293 (3d Cir. 1977)). Notice may be found adequate for this purpose "if it appraises interested parties of the issues to be addressed in the rulemaking proceeding with sufficient clarity and specificity to allow them to participate in the rulemaking in a meaningful and informed manner." *Am. Med. Ass'n v. United States*, 887 F.2d 760, 767 (7th Cir. 1989).

Relatedly, we are unable to provide informed comments in response to CMS's proposal to waive section 1847B, which established the Part B drug CAP, as the Agency's "discuss[ion of] an alternative to the CAP" in the Proposed Rule consists only of a list of questions. We do, however, urge the Agency to very carefully consider whether it is wise to proceed with any alternatives explored in this area, given the clear failures of the statutory CAP program, which has been suspended since 2009.

These concerns underscore the need for CMS to withdraw the Proposed Rule and instead refocus on creating a safe and predictable regulatory environment supportive of the development of value-based arrangements in the private sector. Any waiver authority exercised for purposes of facilitating the adoption of these arrangements—including any waivers of title XI—should only be considered once these models are further developed in the private sector. In the meantime, CMS must withdraw its proposed rule because, among other things, its proposed waivers either exceed CMMI's statutory authority, or, if the Agency's statutory authority is so broad to allow a unilateral abrogation of statutory text by the executive branch, the statute itself risks vulnerability on constitutional grounds.

XI. Evaluation. CMS does not establish a sufficiently detailed evaluation framework under which the Agency can identify the impact of the Proposed Rule on providers and patients, and fails to address certain requirements for patient protection.

Under the CMMI statute, a "Phase I" testing model must analyze its effect on quality of care "including the measurement of patient-level outcomes" and on program spending.¹⁵⁹ To do that, CMMI would need to identify relevant outcomes metrics and set up a system for their collection **before the model's launch**—otherwise outcomes cannot be tracked throughout the testing. By contrast, the Proposed Rule does not establish an evaluation framework with any level of detail, yet is proposed to begin implementation in only a few short months.

In section VI of the preamble of the Proposed Rule, CMS identifies a list of broad questions that the Agency intends to evaluate to assess the impact of the Proposed Rule on providers and patients. CMS notes that the primary goals of evaluation will be "to test the hypothesis that [the Proposed Rule's] alternative payment designs would lead to both higher quality and more affordable care for Part B Medicare enrollees and reduced Medicare expenditures."¹⁶⁰ However, we do not agree that CMS will be able to evaluate changes in quality of care and patient access, as the Agency has not proposed any such metrics in conjunction with the Proposed Rule. Instead, the only two statements related to the evaluation of quality of care themselves raise questions about whether any outcomes measures would even be evaluated:

- "Our evaluation will focus on whether the intervention reduces costs while improving quality of care. It **could** include assessments of patient experience of care,

¹⁵⁹ SSA § 1115A(b)(4).

¹⁶⁰ 81 Fed. Reg. at 13,252.

prescribing and utilization patterns, health outcomes, Medicare expenditures, provider and supplier costs, and other potential impacts of interest to stakeholders.”¹⁶¹

- “Our key evaluation questions would include . . . Outcomes/Quality. What is the impact on quality of care, access to care, timeliness of care, and the patient experience of care?”¹⁶²

This is a significant deviation from the Agency’s work on other demonstration programs, in which quality measures were identified in advance of the demonstrations becoming operationalized, and stakeholders were provided an advanced opportunity to comment on these measures.

Since CMS has not proposed to utilize any new quality measures, one might assume that the Agency intends to utilize existing measures of quality to evaluate the impact of the demonstration on patients. BIO raises serious concerns with doing so, however, given that questions regarding the sufficiency of existing quality metrics to measure quality of care remain. To illustrate, the National Quality Forum’s (NQF’s) Measure Applications Partnership (MAP)—HHS’s own expert advisory group composed of diverse stakeholders who are tasked with “provid[ing] input to the [Department] on the selection of performance measures”—has identified critical gaps in the existence of quality measures that can meaningfully assess prevention and treatment of cardiovascular disease, diabetes, and cancer.¹⁶³

Additionally, robust measures of patient access to needed medicines are not currently employed in Part B. BIO also reiterates our concern, expressed earlier in this letter, that the reliance on PCSAs as the unit of measure for provider participation will confound analyses comparing Part B utilization before and under the Proposed Rule ([see section V](#)). Thus, given the absence of both robust, existing quality measures and proposed, new measures, together with the use of a flawed methodology for determining provider participation, BIO raises serious concerns that CMS can reasonably expect to measure the impact of the Proposed Rule on the quality of care patients receive. The inability to evaluate a demonstration program runs afoul of the statutory evaluation requirements with which CMMI must comply, and represents irresponsible policymaking and poor study design.¹⁶⁴

Given the lack of an adequate evaluation framework and the numerous methodological concerns raised by the Proposed Rule, it is possible that CMS will not be able to glean reliable data with regard to how the interventions proposed are affecting quality of care, patient access to needed medicines, and overall Part B spending. Yet that affect may be significant nonetheless, a serious concern that has been discussed throughout this letter.

¹⁶¹ 81 Fed. Reg. at 13,252 (emphasis added).

¹⁶² 81 Fed. Reg. at 13,252.

¹⁶³ National Quality Forum, Measure Applications Partnership: Cross-Cutting Challenges Facing Measurement: MAP 2015 Guidance, Figure 1, p. 6 (Mar. 2015), *available at* https://www.qualityforum.org/Setting_Priorities/Partnership/MAP_Final_Reports.aspx.

¹⁶⁴ Under statute, CMMI must evaluate each model, and such evaluate must “include an analysis of [] the quality of care furnished under the model, including the measurement of patient-level outcomes and patient-centeredness criteria.” See ACA § 3021(b)(4)(A)(i).

Moreover, because the Proposed Rule involves research that can create risks to patients (e.g., reduced access to a needed therapies to treat a serious disease), BIO believes ethical issues also must be considered, especially in a case where the research design may not produce scientifically sound results. In such circumstances, the research can subject participants to risk without advancing knowledge. Yet, it is unclear whether CMS has considered the implications the Proposed Rule may have with regard to the Federal Policy for the Protection of Human Subjects (“Common Rule”).¹⁶⁵ We understand that the Agency has received a letter from Senator Grassley on this topic; BIO urges CMS to address the issues raised in the Senator’s letter in a transparent and timely manner.¹⁶⁶

XII. Regulatory Impact Analysis. The Regulatory Impact Analysis for the Proposed Rule is not sufficiently detailed to reflect its potential effects on providers and patients.

BIO is concerned that the Regulatory Impact Analysis (RIA) included in the Proposed Rule is not sufficiently detailed to accurately capture the potential impact of Proposed Rule on providers and patients. Stakeholders must be able to refer to thorough RIAs when providing feedback to CMS to ensure that such feedback is meaningful in the context of the Agency’s proposal. Additional detail is particularly important here, given the Proposed Rule’s broad scope and the accelerated timeline on which the Agency has proposed to implement it. For example, CMS designed Phase I of the Proposed Rule to change prescriber behavior, yet does not model the very changes in behavior that the Agency is trying to affect. As another example, defining a provider practice as either “urban” or “rural”—the distinctions used in the RIA—dramatically oversimplifies the potential impact of geographical location on a provider practice’s ability to obtain Part B therapies at or below Medicare reimbursement.

Additionally, while CMS distinguishes between different specialty types and different types of hospitals in the RIAs, the Agency does not distinguish between specialty types that practice in the community versus the hospital setting, which can play a major role in determining a provider’s acquisition cost for Part B therapies. CMS has the data needed to run such analyses, thus BIO is perplexed as to why the Agency did not include these among the regulatory impact analyses conducted. We also question the accuracy of the analysis of the potential impact on Medicare Administrative Contractors (MACs), in particular, given the significant logistical and operational challenges of implementing Phase I, for which they will be responsible (discussed in greater detail in [section VI](#) above).

While CMS included a section in the RIA on the “effect of Part B Drug Payment Model Changes on Beneficiaries,” the Agency focused solely on the cost-related implications of the

¹⁶⁵ While research on public benefits is exempt from the common rule on protection of human research subjects, this exemption is premised on “alternative processes [being] in place in which ethical issues raised by research in public benefit or service programs are being addressed by the officials who are familiar with the programs.” 42 C.F.R. 46.101(b)(5); 80 Fed. Reg. 53,933, 53,957 (Sept. 8, 2015). The administrative record concerning HHS-funded demonstrations involving public benefit programs must therefore show an effort to “reduce risks to those necessary to achieve the research objective,” which includes considerations of whether the demonstration could be narrowed in order to reduce risks while still attaining the research objective. *Benio v. Shalala*, 30 F.3d 1057, 1071 (9th Cir. 1994) (internal citations omitted). This standard has not been met by the Proposed Rule.

¹⁶⁶ Letter to the Hon. Sylvia Burwell, Sec’y, Dep’t of Health & Human Servs. from the Hon. Charles E. Grassley, U.S. Sen. (Apr. 29, 2016).

Acting Administrator Slavitt

May 9, 2016

Page 57 of 57

Proposed Rule. BIO agrees that patients' out-of-pocket costs play a major role in patients' healthcare decision-making, but in order for patients to accrue such costs, they must have access to the medicine in the first place. Yet, CMS does not include a discussion of the potential impact on patients' access to appropriate therapies. Nor does CMS take into account other costs to patients who will be required to travel greater distances to access needed therapies or to receive care in costlier settings. Additionally, given the diversity of models CMS proposes between Phase I and Phase II, BIO is surprised that the Agency did not include distinct analyses of the potential impact of each phase, at a minimum, on patients' cost-sharing liabilities and access to needed medicines. Though it is BIO's **strong** recommendation that CMS **withdraw** the Proposed Rule in its entirety, if the Agency moves forward nonetheless, we urge that the RIA be expanded to include the issues discussed in this section, at a minimum, and made publicly available, to inform CMS's and stakeholders' perspectives moving forward.

XIII. Conclusion

BIO appreciates this opportunity to comment on the Proposed Rule and, for the reasons articulated throughout this letter, strongly urges the Agency to withdraw the Proposed Rule in its entirety. In its place, CMS should establish an inclusive dialogue with stakeholders to identify discrete opportunities for Part B reform in an evidence-based manner and work collaboratively to develop any future demonstration programs with a scope and approach that align with Congress's intent in authorizing CMMI.

Please contact us at (202) 962-9200 if you have any questions regarding our comments. Thank you for your attention to this very important matter.

Sincerely,

/s/

Laurel L. Todd
Vice President
Healthcare Policy & Research

Deborah M. Shelton
Deputy General Counsel for Healthcare