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Submitted via email to: IRARebateandNegotiation@cms.hhs.gov

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RE: Medicare Drug Price Negotiation Program: Draft Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price (MFP) in 2026 and 2027

Dear. Dr. Seshamani:

The Massachusetts Biotechnology Council ("MassBio") appreciates this opportunity to submit comments on the Medicare Drug Price Negotiation Program: Draft Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price (MFP) in 2026 and 2027 (the "IPAY 2027 Draft Guidance").

MassBio represents the premier global life sciences and healthcare hub of Massachusetts, which has a vibrant biomedical research and development community that is a global leader for medical discovery and innovation. MassBio's 1,700+ member organizations are dedicated to preventing, treating, and curing diseases through transformative science and technology that brings value and hope to patients. MassBio's mission is to advance Massachusetts' leadership in the life sciences to grow the industry, add value to the healthcare system, and improve patient lives.

MassBio remains concerned about the impact the Medicare Drug Price Negotiation Program (the "Negotiation Program") will have on the future development of innovative and life-saving therapies, as well as on the world-leading small and emerging biotech companies based in Massachusetts. Given the potential impact on innovation and thus on vulnerable patient access to life-saving therapies, we continue to urge CMS to adopt a "do no harm" approach in implementing this program that errs on the side of mitigating against the potential disincentives created by the program's framework, and that allows the agency to make corrections as needed to preserve innovation. In particular, with respect to the IPAY 2027 Draft Guidance, we urge CMS to:

- Continue to explore opportunities to preserve incentives to develop innovative new therapies for rare disease;

- Provide additional predictability and transparency with respect to the process for establishing an MFP for selected drugs;
- Establish processes to effectuate the MFP in a manner that facilitates manufacturer compliance with statutory requirements without resulting in duplicate discounts; and
- Evaluate the impact of the IRA on the innovation ecosystem, particularly in Massachusetts.

I. CMS Should Explore Opportunities to Preserve Incentives to Develop Innovative New Therapies for Rare Disease.

MassBio remains concerned that the narrow scope of the orphan drug exclusion creates a strong disincentive for developers to continue to develop new indications and formulations for existing orphan therapies.

Rare diseases, as defined by the FDA are conditions that impact fewer than 200,000 patients nationwide, and are inherently under-researched, under-diagnosed, and under-treated. Although much progress has been made since the enactment of the Orphan Drug Act (ODA) 40 years ago, over 90 percent of known rare diseases still do not have therapies or treatments. While there has fortunately been a recent surge in the development of drugs for rare disease populations,[1] with much of this development occurring in Massachusetts,[2] these new indications still require costly clinical trials, regulatory approvals, and adherence to regulatory requirements. The narrow scope of the orphan drug exclusion creates a strong disincentive to undertake these investments, even though the science is there and could benefit vulnerable populations. Furthermore, because of the limited scope of the exclusion, companies may be disincentivized from developing therapies for rare diseases to begin with, and to instead prioritize indications with larger patient populations from the outset.

For these reasons, we are concerned that CMS appears to have walked back its commitment to "consider[] whether there are additional actions the agency can take in its implementation of the Negotiation Program to best support orphan drug development."

MassBio continues to urge CMS to implement the orphan drug exclusion in a way that promotes and is consistent with the underlying purposes and goals of the ODA: to create the necessary financial incentives to accelerate the development of rare disease drug development. Specifically, CMS should exercise its regulatory discretion to start the pre-negotiation period for orphan drugs upon loss of the orphan drug exclusion (i.e., when the product obtains approval for a new indication for a different disease or condition), rather than when the product was initially approved. In addition, once an orphan drug is selected for negotiation, CMS should ensure that

[1] The annual number of orphan drug designation requests has steadily increased from 2012 through 2016 and has remained greater than 500 annually since 2016. In 2020, the Office of Orphan Products Development received 753 new requests for designation, a 41% increase from 2019. *See* https://www.fda.gov/news-events/fda-voices/rare-disease-day-2021-fda-shows- sustained-support-rare-disease-product-development-during-public#:~:text=The%20annual%20number%20of%20orphan,a%2041%25%20increase%20from%202019.

[2] D, Seiffert, *Massachusetts owns the orphan drug market. Here's the proof,* Boston Business Journal (Nov. 9, 2015), https://www.bizjournals.com/boston/blog/bioflash/2015/11/massachusetts-owns-the-orphan-drug-market-here-s.html.

its consideration of the statutory factors adequately values the benefit the therapy brings to patients with rare disease.

II. CMS Should Provide Additional Transparency and Predictability Regarding the Process for Establishing the MFP for Selected Drugs.

As CMS proceeds with implementation of the Negotiation Program, MassBio urges the agency to pursue an approach that creates the greatest degree of certainty for developers by adopting a predictable, transparent methodology for applying the statutory factors, which should then be updated over time to recognize the value of continued innovation. As we have explained in prior comment letters, investment in the drug development process and the innovation ecosystem is significantly impacted by the long-term market dynamics. Thus, to enable developers and their investors to make informed investments today, CMS's methodology should reflect the value that a product provides over its lifecycle and create incentives to invest in new therapeutic areas with unmet need.

MassBio is concerned that the IPAY 2027 Draft Guidance remains vague regarding certain key elements of the process for establishing an initial offer. For one, we are concerned about the lack of predictability and transparency surrounding the selection of therapeutic alternatives. Relative to the IPAY 2026 guidance, CMS is proposing to reserve even greater discretion for the agency to select a broad range of therapeutic alternatives. Given that CMS's identification of therapeutic alternatives plays an outsized role in determining the initial offer, it is critical that this selection process not only be predictable, but consistent with the statutory directive to compare selected drugs to true therapeutic alternatives. We therefore urge the agency to limit the selection of therapeutic alternatives to those products that are in the same category and class and share common clinical use. We also urge the agency to clarify how the agency will weight net prices across multiple indications and therapeutic alternatives, as applicable, in determining the starting point for the initial offer.

We are similarly concerned that CMS has provided very little information about the adjustments CMS makes to the starting point based on the various statutory factors. Manufacturers are required, as part of the Negotiation Program, to submit large volumes of information to CMS for use in establishing the MFP. However, as with the IPAY 2026 revised guidance, it remains unclear in the IPAY 2027 Draft Guidance how this information is applied. To help manufacturers of selected drugs prepare for the data submission for IPAY 2027 and beyond, and to ensure that CMS is receiving the most pertinent information, we urge CMS to provide greater detail in this regard.

III. CMS Should Establish Processes for MFP Effectuation that Facilitate Manufacturer Compliance Without Resulting in Duplicate Discounts.

MassBio appreciates CMS's efforts to support the effectuation of the MFP, including through the creation of the Medicare Transaction Facilitator (MTF). We are concerned, however, that elements of CMS's proposal may impose undue burden on manufacturers or complicate manufacturers' ability to effectuate the MFP without resulting in duplicate discounts. We therefore urge CMS to make the following revisions to the IPAY 2027 Draft Guidance:

- Make the payment facilitation function of the MTF mandatory for all parties; and
- Take proactive steps to prevent duplicate discounts between the Negotiation Program and the 340B drug discount program.

We also urge CMS to provide more information about the MTF and its functionality in a timely manner to enable manufacturers to develop informed MFP Effectuation Plans by the June 2025 deadline.

A. CMS Should Mandate Use of the MTF for Payment Exchange To Facilitate MFP Effectuation.

In the IPAY 2027 Draft Guidance, CMS reiterates that while the primary manufacturer must participate in the data exchange functionality of the MTF, "any potential payment facilitation functionality of the MTF would be voluntary for dispensing entities and Primary Manufacturers…." MassBio disagrees with this approach. Making the MTF's payment functionality optional would increase the burden on manufacturers and may compromise CMS's ability to successfully effectuate the MFP.

Notably, manufacturers have generally not had direct contractual relationships with retail pharmacies. Consolidating payment functionality in the MTF would thus avoid the need for each primary manufacturer to enter into a contractual relationship with each retail pharmacy that opts out of the MTF payment functions. However, the failure to *require* pharmacy participation undermines this benefit by nonetheless requiring manufacturers to develop a mechanism to effectuate the MFP with non-participating pharmacies.

We note that retail pharmacies would also benefit from this consolidated approach as it would streamline the payment reconciliation process across manufacturers and selected drugs—products that are, generally speaking, high-volume products. Such a requirement would also promote enrollee access to selected drugs by removing operational barriers that could affect enrollees' experience with the MNP. We note that CMS could achieve this aim by imposing contractual requirements on Part D plans that require their network pharmacies to participate in the payment functionality of the MTF.

B. CMS Should Take an Active Role in Preventing Duplicate Discounts Across the Negotiation Program and 340B Drug Discount Program.

The Negotiation Program statute exempts manufacturers from providing access to the MFP to covered entities when the 340B ceiling price is lower than the MFP for a given selected drug.[3] Although the statute requires manufacturers to provide access to the MFP if it is lower than the 340B ceiling price, this provision further requires that the MFP be offered in a "nonduplicated amount."[4] The proper implementation of this provision is necessary to ensure the proper functioning of the MNP as contemplated by Congress. However, CMS has not proposed policies that would enable MFP effectuation in accordance with these deduplication provisions.

[3] SSA § 1193(d)(1).
[4] SSA § 1193(d)(2)

In the IPAY 2027 Draft Guidance, CMS proposes that manufacturers may avoid duplication of the MFP and 340B discounts by identifying claims from the data elements transmitted by the MTF. However, CMS is not proposing to require dispensing entities to identify claims as 340B-eligible at the point-of-sale (POS) using available 340B claims modifiers. CMS instead expects manufacturers to work this out directly with covered entities. This is not a realistic proposal.

MassBio members have long been frustrated by the Health Resources and Services Administration's (HRSA's) lack of oversight the 340B program's duplicate discount prohibition. Without such oversight, covered entities have little motivation to avert the occurrence of double discounts, which can result in their loss of 340B discounts. Meanwhile, rapid growth of the 340B program and, in particular, the explosion in 340B contract pharmacy utilization complicates manufacturers' ability to prevent and identify duplicate discounts. And a growing number of states—including Massachusetts—have proposed legislation that would prohibit the use of modifiers for the identification of 340B claims in the absence of a federal mandate, undercutting the imperative for transparency.[5]

For these reasons, CMS must actively implement the 340B nonduplication provision by requiring POS identification of 340B claims. CMS can achieve this by imposing requirements on Part D plans regarding the types of claims that they may adjudicate. Specifically, CMS could designate the lack of a 340B claims modifier as a "defect" that prevents the claim from being a "clean claim" subject to the prompt payment standard.

IV. CMS Should Evaluate the IRA's Impact on the Innovation Ecosystem in Massachusetts.

We continue to urge CMS to carefully examine the impact of the law in Massachusetts. In light of Massachusetts' unique role as the hub of companies directly engaged in research, development, and manufacturing of innovative products, Massachusetts will be a "canary in the coal mine" in terms of changes to the system, and will thus be a good test case to see how IRA implementation affects the biotech industry.

As noted in prior comments above, in early 2023, MassBio surveyed its membership regarding the IRA's immediate impacts, and member perspectives regarding certain regulatory and legislative policies that could mitigate those impacts. MassBio plans to continue to survey our membership and perform other data-driven approaches to monitor the impact of the law, and we hope to have the opportunity be a resource for CMS as it begins to track the impact of the IRA. Likewise, as CMS proceeds with implementation of the law, MassBio urges the agency to similarly prioritize building the necessary infrastructure to track the impact of the IRA on the innovation ecosystem. This will be vital given the long-standing relationship between innovation and increased access to life-saving therapies, and the need for the agency to "do no harm" in the implementation of this new program. For instance, CMS could track the following metrics, using CMS's own data and certain data available from the FDA, to assess the IRA's impact over time:

- Number of new technology add-on payment applications for drugs and biologicals;

[5] States May Consider 340B Legislative Proposals in 2024, Avalere (December 1, 2023), https://avalere.com/insights/states-may-consider-340b-legislative-proposals-in-2024.

- Requests for pass-through status under the Hospital Outpatient Prospective Payment System;
- Number of new NDCs in average sales price (ASP) reporting data;
- Number of NDA/BLA submissions (tracking proportion of small-molecule vs. large-molecule over time);
- Number of supplemental NDA/BLA submissions;
- Number of applications for orphan drug designation (ODD);
- Percent of products with an ODD that are approved by FDA;
- Number of applications for breakthrough therapy designation;
- Number of applications for fast-track designation; and
- Number of applications for regenerative medicine advanced therapy designation.

We further urge CMS to publicly report these data to inform both the public and policymakers in Congress, and to establish a dynamic framework pursuant to which significant decreases in the metrics captured above trigger reconsideration of the negotiation process implemented by the agency.

V. Conclusion

MassBio thanks CMS for your consideration of our comments. Please don't hesitate to contact me at (617)-674-5148 or kendalle.oconnell@massbio.org if you have any questions or would like any additional information to consider our comments.

Best regards,

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