Biotechnology Innovation Organization
1201 New York Ave., NW
Suite 1300
Washington, DC, 20005
202-962-9200

March 27, 2023

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Baltimore, MD 21244–1850

RE: Small Biotech Exception (CMS-10844)

Dear Administrator Brooks-LaSure:

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to comment on the proposed Information Collection Request (ICR) on the Small Biotech Exception (CMS-10844) issued by the Centers for Medicare & Medicaid Services (CMS) on January 18, 2023. We note that many of our comments, which are outlined in more detail below, will also apply to the ICR on the Medicare Part D Manufacturer Discount Program Agreement (CMS-10846) and the information that manufacturers must submit to qualify as a specified manufacturer or specified small manufacturer for phased-in discounts for Part D drugs under the new Manufacturer Discount Program.

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 prevent them in the first place. As a result, 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 membership includes biologics and vaccine manufacturers and developers that have worked closely with stakeholders across the spectrum, including the public health and patient advocacy communities, to support policies that help ensure access to innovative and life-saving medicines and vaccines for all individuals.

Small biotech manufacturers are at the forefront of America's innovation ecosystem. At BIO, we represent hundreds of biopharmaceutical manufacturers, with the vast majority of our members being small manufacturers who are working on some of the most important and cutting-edge scientific and medical research. Emerging biotech manufacturers are exceptionally productive, taking into account

their lower market capitalization, and are responsible for nearly 80 percent of clinical drugs in the pipeline. Many of these companies have yet to achieve a revenue stream and rely on private capital and outside investment to fund their clinical trials and research. Ensuring that these manufacturers benefit from the small biotech provisions in the Inflation Reduction Act (IRA) is critical as America seeks to maintain its global competitiveness in this strategically important growth sector. Even more important is ensuring that these small manufacturers have the policy and regulatory environment they need to bring the next generation of medicines to the patients whose lives can be transformed by breakthroughs in new treatments and therapies. We look forward to working with you to ensure small biotech manufacturers benefit from these provisions in a manner that is consistent with Congressional intent.

Under the IRA, a drug is exempt from negotiation for initial price applicability years 2026, 2027, and 2028 if spending on the medicine comprises: (1) a small percentage of Medicare program spending, and (2) a significant proportional share of a company's Medicare business. This is referred to as the Small Biotech Exception. This critical protection recognizes that small biotech manufacturers with a single product that represents the vast majority of their Medicare revenue will be disproportionately impacted by negotiation, which could have an immediate and tangible impact on the ability of such manufacturers to invest in future R&D – and in particular, in areas that predominantly affect the Medicare population.

In the proposed Small Biotech Exception ICR's Supporting Statement, CMS notes that Small Biotech Exception information must be submitted to CMS before the selected drug list for initial price applicability year 2026 is published by September 1, 2023, and that the timeline for submission of this information will be provided in program instruction. In separate initial guidance on the Drug Price Negotiation Program released on March 15, 2023, CMS states that it "anticipates that this deadline will be in June 2023 but will publish a specific deadline on the CMS IRA website in the future."

CMS does not address how or when it will notify manufacturers regarding its determination of whether a drug, based on the information the manufacturer submits through the proposed ICR, qualifies for the Small Biotech Exception. To that end, our comments that follow focus on ensuring predictability and transparency for small biotech manufacturers that apply for the exception. This includes the following process attributes:

- Clear process (i.e., who submits, what to submit, when to submit) for applying for or recertifying a drug qualifies for the Small Biotech Exception.
- Appropriate and fair timelines to submit information to qualify.
- Consistency and clear criteria in evaluating submissions to qualify for the Small Biotech Exception.
- Proper and timely notification regarding qualification if the drug meets the Small Biotech Exception requirements.
- Proper and timely notification if the drug does NOT meet the Small Biotech Exception requirements, as well as a clear dispute process for appeal of such decision.

- Clarity regarding the form and manner that CMS will use to notify manufacturers if they meet – or do not meet – the criteria for the Small Biotech Exception.

Submissions for Initial Price Applicability Year 2026. CMS has indicated that the current Small Biotech Exception ICR is focused only on initial price applicability year 2026. However, as discussed further below, given that the agency has not made publicly available the data on which it will rely with regard to total expenditures for the purpose of determining eligibility for the Small Biotech Exception under Sec. 1192 (d)(2,) or whether a drug meets the test of a high spend drug under Sec. 1192 (d)(1), it is impossible for small biotech manufacturers to make a reasonable inference regarding whether a submission is warranted in the current or future years on the basis of the requisite statutory thresholds. Therefore, for clarity, we recommend that any company that believes it qualifies for the Small Biotech Exception under Sec. 1192 (d)(2) should be able to apply and be approved for this exception this year regardless of whether the drug meets the test of a high spend drug under Sec. 1192 (d)(1). This will provide important certainty and predictability for small biotech manufacturers. Such certainty is critical as most small biotech manufacturers have only one or a limited number of products on the market. We also believe the statute contemplates such an approach, as the exception in Sec. 1192 (d)(2)(A) refers to a "qualifying single source drug" that meets either the test in Sec. 1192 (d)(2)(A)(i) or Sec. 1192 (d)(2)(A)(ii), and these tests refer to Medicare expenditures in 2021, and the data for any small biotech company is readily available to CMS. To provide additional predictability and mitigate uncertainty for small biotech manufacturers, CMS should also clearly articulate the specific criteria manufacturers should consider in determining whether to apply for the Small Biotech Exception for initial price applicability year 2026.

Clarity on Process and CMS Response to Small Biotech Manufacturer. CMS should specify not only the timeline for when the submission of information by the small biotech manufacturer is due, but also the timeline for CMS review and response to the manufacturer, in situations where CMS grants the exception as well as situations where CMS does not. To promote certainty for small biotech manufacturers, CMS should commit to responding to each manufacturer as far in advance of September 1, 2023, as possible.

- *Clear Timelines.* We suggest the following as a timeline that would allow for appropriate transparency, clarity, and completion of the process in advance of the September 1, 2023, publication of drugs selected for negotiation:
    - Submission by small biotech manufacturers due June 10, 2023;
    - CMS response to small biotech company (affirmative or negative) due by June 30, 2023;
    - Small biotech company response to negative determination by July 20, 2023;
    - Final CMS response to small biotech company by August 10, 2023.

- *Clarity on Data Source for 2021 Drug Spending and Availability of Data for Manufacturers.* CMS should clarify what data source it will use for identifying 2021 total expenditures for the qualifying single source drug, as the agency has stated that the drug dashboard data published at *cms.gov* is not being used for the IRA negotiation provisions; we also understand CMS is

considering use of Prescription Drug Event (PDE) data. We recommend that CMS provide the data it will be using for 2021 to manufacturers so that this data can be validated by manufacturers that apply for (or will apply for) the Small Biotech Exception.

- *CMS Response and Justification for Decision.* CMS should provide clarity on the form and content of its expected response and notification to small biotech manufacturers applying for the exception, specifically whether the response will be by letter, email, or other form of official communication. Further, if CMS determines that it does not agree that a small biotech drug qualifies for the exception, CMS's response should outline in sufficient detail how such a determination was made, including on which expenditure data the agency relied and other information, as relevant, that led to a negative determination. Further, CMS should indicate if the rationale for the denial is restricted to initial price applicability year 2026 or all years for which the Small Biotech Exception applies.

- *Dispute Resolution.* CMS should provide a dispute resolution process where the manufacturer can respond to and appeal a negative determination by CMS. Specifically, the small biotech manufacturer should have the opportunity to provide additional data or information to the agency to support its application for the Small Biotech Exception.

- *Flexibility.* Given that this is a new program and process, and that only a limited number of small biotech manufacturers will be providing submissions to CMS, we recommend that the agency allow for a flexible approach. For example, if CMS determines that information submitted by the small biotech manufacturer is incomplete or unclear, we urge CMS to engage in a dialogue with the manufacturer to resolve any outstanding issues to complete their submission. Further, for the first year of the program, we encourage CMS to allow a small biotech manufacturer to submit information after the information submission deadline, such as in good faith circumstances where a small biotech manufacturer may later realize that it should qualify for the exception.

- *One-time Qualification.* We request that manufacturers should not need to reapply in subsequent years if a drug has previously received the Small Biotech Exception and there is no material change in the manufacturer's circumstances. As an alternative, manufacturers could submit an attestation that nothing in their application has materially changed from the prior year and if there has been a material change the manufacturer could submit an updated form.

- *Clear Definition of Acquired.* We recommend CMS include a definition for what it means to be "acquired" pursuant to Sec. 1192(d)(2)(B)(ii). CMS should consider defining an acquisition as the transfer of substantially all assets of the manufacturer. Further, CMS should specify whether the acquiring manufacturer meeting the definition of a specified manufacturer will be determined at the time of acquisition. If the acquisition results in a change in eligibility for the small biotech exemption, an updated form should be submitted.

Confidentiality of Proprietary Information, Publication of Drugs Qualifying for Small Biotech Exception. CMS should fully protect the confidentiality of all proprietary information submitted in relation to this ICR. At the same time, CMS should outline its approach for sharing with the public information regarding the small biotech drugs the agency determines qualify for the exception. Further, BIO recommends that CMS publish a summary list of the small biotech drugs and manufacturers that qualified. Such information will be important for understanding the impact of this IRA provision and provide further certainty to small biotech manufacturers. We believe that more detail on how or why a specific manufacturer's drug qualified as a small biotech drug should only be released if that manufacturer chooses to do so.

Health Plan Management System (HPMS) Submission: In the supporting statement, CMS states that it plans to develop an automated tool within HPMS for manufacturers to submit the Small Biotech Exception ICR Form. CMS also notes that if completion of this tool is delayed, the agency will accept responses to this ICR by mail. We recommend that CMS consider submission by email to a dedicated inbox rather than using regular mail as a backup approach. Further, given this is a new process for manufacturers, we recommend that CMS allow for submissions via email as an alternative to using HPMS even if the tool is functional.

ICR Form – Who Submits/Control Group Information. The language in question 4/4a of the ICR form raises confusion, as it appears to request information about members of the control group even if no coverage gap discount program (CGDP) is in place for these members. We recommend that the language for the ICR small biotech exception form align with that for the Part D Medicare Discount Program ICR – see 2nd column below:

Small Biotech Exception	Medicare Discount Program
Question 4: Did the entity that had a Coverage Gap Discount Program agreement effective on December 31, 2021, for the covered Part D drug for which the Submitting Manufacturer seeks the Small Biotech Exception (i.e., either the Submitting Manufacturer or the entity identified in Question 3b, as applicable) have other members in its controlled group as of December 31, 2021? For the purpose of this information collection request, "controlled group" means all corporations or partnerships, proprietorships and other entities treated as a single employer under 26 U.S. Code section 52(a) or (b). Yes [ ] No [ ]	
Question 4a: If yes, provide the following information as of December 31, 2021, for **each such** member of the controlled group <u>of the entity</u> that had the Coverage Gap Discount Program agreement effective	*Did the submitting Manufacturer have a Coverage Gap Discount Program Agreement under 42 USC § 1395w-114a effective on December 31, 2021 AND have any other corporation or business (whether or

on December 31, 2021, for the covered Part D drug which the Submitting Manufacturer seeks the Small Biotech Exception:	not incorporated) in its controlled group, as of December 31, 2021, that had a Coverage Gap Discount Program Agreement under 42 USC § 1395w-114a effective on December 31, 2021? For the purpose of this information collection request, "controlled group" means all corporations or partnerships, proprietorships and other entities treated as a single employer under 26 U.S. Code section 52(a) or (b). Y/N If yes, complete the subsequent five (5) fields in this section for **each** such additional Manufacturer belonging to the same controlled group as of December 31, 2021.

It is critical that manufacturers know the appropriate entity that should submit the form for the small biotech exemption. This is particularly important (1) in circumstances where multiple companies are part of a "control group" and are considered a single manufacturer for purposes of qualifying for the Small Biotech Exception; and (2) in instances where a manufacturer seeking to qualify for the Small Biotech Exception that may have been acquired by another manufacturer.

CMS indicated during its March 22, 2023 meeting with BIO that the small biotech exception form should be submitted by the New Drug Application (NDA) or Biologics License Application (BLA) holder of the covered Part D drug for which the small biotech exemption is being sought. We recommend that CMS make this clear in its guidance and on the accompanying form.

ICR Form – Certification. The language in the certification section appears to be overly broad – we recommend amending as follows: "I reviewed the submission and made a reasonable inquiry regarding its content. I understand the information contained in this submission is being provided to ~~and will be relied upon by CMS for Medicare reimbursement purposes, including to~~ determine whether the covered Part D drug of the Submitting Manufacturer qualifies for the Small Biotech Exception, as described in section 1192(d)(2) of the Social Security Act."

Thank you for your consideration of these comments. We look forward to ongoing dialogue with you on these and other issues of importance to small biotech drug manufacturers.

Sincerely,

/s/

Crystal Kuntz
Vice President, Healthcare Policy & Research