July 31, 2023

MassBio
MASSACHUSETTS BIOTECHNOLOGY COUNCIL

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William N. Parham, III, Director
Paperwork Reduction Staff
Office of Strategic Operations and Regulatory Affairs
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, Maryland 21244

Richard L. Revesz, Administrator
Office of Information and Regulatory Affairs
Office of Management and Budget
262 Old Executive Office Building
Washington, D.C. 20503

RE: Information Collection Request (ICR) Form for Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act (IRA) (CMS-10847, OMB 0938-NEW)

Dear Mr. Parham and Mr. Revesz:

The Massachusetts Biotechnology Council (MassBio) appreciates the opportunity to submit comments in response to the Centers for Medicare & Medicaid Services (CMS) proposed information collection request (ICR) for the Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act (IRA).[1]

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 1,600+ 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 is deeply concerned about the impact 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. MassBio therefore appreciates this opportunity to submit the following recommendations designed to ensure that the data collected by CMS through the ICR Form are clear, useful, and of high quality.

As a threshold matter, MassBio supports the revisions CMS made in response to our comments submitted in response to the 60-day notice. For instance, we appreciate that all respondents who are not Primary Manufacturers will now be able to use a separate user-friendly web application that is accessible from an entry point on CMS.gov, rather than being required to register via the HPMS system. This will reduce the burden on the public of submitting information and promote robust participation in this process. This will be particularly important for patients and caregivers to be able to submit information in a manner that has a minimal burden.

We also strongly support CMS's clarification that outcomes such as cure, survival, progression-free survival, or improved morbidity may be considered when comparing the selected drug to a therapeutic alternative. We also support the express recognition of health equity considerations for purposes of assessing comparative effectiveness for specific populations. By looking at patient-centered outcomes, particularly through the lens of health equity related to specific populations, CMS will be able to consider populations that generally lack access to adequate therapeutic options.

[1] 88 Fed. Reg. 42,722 (July 3, 2023). *See also* https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10847.

While we appreciate these changes, MassBio remains concerned that CMS has not addressed many of our other comments. Accordingly, some of the proposed reporting requirements will impose unnecessary burden on respondents and/or result in the collection of data that are unnecessary or unclear without further revision. We therefore continue to urge CMS to make additional changes to further improve the quality and utility of the information collected, while minimizing the associated reporting burden.

I. CMS Should Revise Questions Regarding Research and Development (R&D) Costs to Align with Industry Standards.

MassBio remains concerned that CMS's proposed reporting methodology is not consistent with how manufacturers track R&D costs and will thus result in the collection of information that is unclear and not useful. In particular, by focusing solely on R&D expenditures made by the Primary Manufacturer, CMS's proposed definition overlooks contributions made by the Secondary Manufacturer and others. Drug development is often a collaborative process, involving investments by both small biotech companies and larger pharmaceutical companies. By looking only at the expenditures made by the manufacturer that holds the NDA/BLA, CMS is ignoring a large portion of R&D costs. This approach is also not supported by the statute, which looks at research costs of the "manufacturer," a term that's defined quite broadly.[2] CMS should therefore collect information regarding R&D spend from across the innovation ecosystem, including directly from Secondary Manufacturers.

In addition, CMS's proposed definition for abandoned or failed drug costs suggests the agency will consider failed or abandoned product costs only for products with some relation to the selected drug at issue (i.e., same active moiety, active ingredient, mechanism of action and therapeutic class). Although these categories capture many of the costs incurred in the development of a given drug, to improve the clarity and utility of the information collected, MassBio continues to urge CMS to clarify that it will also solicit information from manufacturers regarding R&D costs for abandoned and/or failed research *that is not attributable to any particular product* across a manufacturer's selected drugs.

II. In Soliciting Information Regarding Therapeutic Alternatives, CMS Should Provide Greater Clarity Regarding the Information Solicited and Consider Only On-Label Indications.

We continue to urge CMS to provide additional transparency on the information it is soliciting regarding therapeutic alternatives to improve the quality and utility of the information collected. For instance, CMS should solicit information regarding the extent to which a given therapy is uniquely suited to treat specific conditions. For example, small-molecule drugs, in particular, are able to effectively accomplish delivery across the "blood-brain barrier," which is essential for the treatment of many mental health conditions. To aid its determination of therapeutic advance and comparative effectiveness, CMS should also specifically solicit information on the ability of a selected drug to assist patients with respect to other important patient-centered measures, including the ability of patients to function and be independent. This is particularly important for therapies that treat debilitating disorders, such as rare disease, for which improvements in function translate into enormous improvements to quality of life.

To improve the clarity and utility of the data collection, we also continue to urge CMS to consider as potential therapeutic alternatives only those products with the same *on-label* indications that are actually used in clinical practice as alternatives for that indication. MassBio is concerned that CMS's proposal to consider off-label therapeutic alternatives will result in the collection of data that are incomplete and not useful.[3] To obtain approval

[2] See Social Security Act (SSA) § 1191(c)(1) (referencing SSA § 1847A(c)(6)(A), which in turn references SSA § 1927(k)(5)) (defining a manufacturer to mean "any entity which is engaged in—(A) the production, preparation, propagation, compounding, conversion, or processing of prescription drug products, either directly or indirectly by extraction from substances of natural origin, or independently by means of chemical synthesis, or by a combination of extraction and chemical synthesis, or (B) in the packaging, repackaging, labeling, relabeling, or distribution of prescription drug products.").

[3] In the revised ICR Form, CMS proposes the following definition: "A therapeutic alternative must be a pharmaceutical product that is clinically comparable to the selected drug. CMS will consider different therapeutic alternatives for each

for an on-label indication, a manufacturer must complete clinical trials that result in extensive data regarding the safety and efficacy of the drug for that indication. The same is not true for off-label indications. CMS's proposed approach would thus compromise CMS's ability to make a direct comparison across products by considering indications for which there may be insufficient data.

III. CMS Should Provide More Clarity Regarding the Health Equity Data Solicited.

As noted above, MassBio appreciates CMS's express recognition in the revised ICR Form of the importance of health equity to assessing the comparative effectiveness of selected drugs on specific populations. We are concerned, however, that CMS has not provided sufficient clarity to facilitate the collection of information that is clear and useful to CMS. Specifically, in collecting information on the impact of therapies on specific populations, CMS should specifically note that it is collecting comparative effectiveness information regarding issues specific to rare disease populations, individuals with mental illness, and individuals with late stages of cancer. These are populations that generally lack access to adequate therapeutic options, and for whom the value of a new therapy is particularly critical. For instance, for rare disease, health utility and services research and data are limited given small populations and specialized knowledge base, and where there is an on-label therapy for a rare disease, it is often either the first on-label therapeutic option, or the first such option to be approved in decades. By highlighting and considering such factors, CMS can better align its efforts with advancing health equity and ensuring more equitable healthcare outcomes for all.

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MassBio thanks CMS and OMB for your consideration of our comments. We would be more than happy to answer any questions you may have regarding these comments or to provide additional information.

Best regards,

Kendalle Burlin O'Connell, Esq.
CEO & President
MassBio

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indication, as applicable. Therapeutic alternatives may be a brand name drug or biological product, generic drug, or biosimilar and may be on-label or off-label to treat a given indication. CMS will begin by identifying therapeutic alternatives within the same drug class as the selected drug based on properties such as chemical class, therapeutic class, or mechanism of action before considering therapeutic alternatives in other drug classes. In cases where there are many potential therapeutic alternatives for a given indication of the selected drug, CMS may focus on a subset of therapeutic alternatives that are most clinically comparable to the selected drug."