May 24, 2023

VIA ELECTRONIC SUBMISSION

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244-1850

Re: Small Biotech Exception Information Collection Request (CMS-10844, OMB 0938-NEW)

Dear Administrator Brooks-LaSure:

BeiGene, Inc. (BeiGene) appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services (CMS) Medicare Drug Price Negotiation Program: Small Biotech Exception Information Collection Request (ICR) (CMS-10844, OMB 0938-NEW).[1]

BeiGene is a global, science-driven biotechnology company focused on developing innovative and affordable cancer medicines to improve treatment outcomes and patient access. We are driven by a commitment to expand the highest quality therapies to individuals around the world through courage, persistent innovation, and challenging the status quo.

BeiGene is an active member of the Biotechnology Innovation Organization (BIO), and submitted comments on CMS's March 15, 2023 initial guidance[2] on the Inflation Reduction Act (IRA) Medicare drug price negotiation program through BIO as our representative organization.[3] In the following comments, we reaffirm BIO's feedback on CMS's initial guidance and offer additional comments to inform CMS's implementation of the small biotech portions of the IRA in 2026 and the program's successive years.[4]

BeiGene offers the following comments herein:

- BeiGene notes Congress's intent, in enacting the IRA, to safeguard innovation in small biotech companies, and we encourage CMS to implement the IRA in manners that protect small biotechs, in furtherance of Congressional intent.

- BeiGene urges CMS to set forth and solicit input on its full methodology for implementing the small biotech exception in 2026 and later years; additionally, we

[1] 88 Fed. Reg. 24,805 (Apr. 24, 2023).
[2] CMS, Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026, and Solicitation of Comments. Department of Health & Human Services (Mar. 15, 2023) (hereinafter referred to as Initial Guidance).
[3] Biotechnology Innovation Organization (BIO), Comment Letter on Medicare Drug Price Negotiation Program: Initial Memorandum, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2026 (Apr. 14, 2023), *available a*t https://www.bio.org/letters-testimony-comments/bio-comments-centers-medicare-medicaid-services-cms-medicare-drug-price.
[4] We are submitting these comments through the Small Biotech Information Collection Request (ICR) process, as many of the comments specifically pertain to the small biotech exception. As of the date of this comment letter, we are not aware of anticipated rulemakings or other administrative mechanisms with which to engage and share our thoughts on these topics; however, we remain eager and willing to engage with the agency on the feedback we share below.

encourage CMS to ensure adequate processes to validate agency data/methods and appeal agency small biotech determinations, as necessary.

- BeiGene encourages CMS to ensure strong stakeholder engagement, including robust comment opportunities, for all aspects of the Medicare drug price negotiation program.

I. BeiGene supports Congress's intent to protect innovation in small biotech companies under the IRA, and we encourage CMS to implement the relevant IRA provisions in furtherance of Congress's intent.

Congress, in enacting the IRA, included several provisions protecting small biotech companies and their drugs over a five-year window, including (i) an exception for small biotech drugs from the definition of negotiation-eligible drugs for price applicability years 2026, 2027, and 2028,[5] and (ii) a price floor for small biotech drugs that have initial price applicability years in 2029 or 2030.[6] In the months leading up to enactment of the IRA, throughout Congressional conversations around drug pricing reform in 2022, Congress made clear that it intended to ensure that any drug pricing legislation would include meaningful protections for drugs from small biotech companies.

Congressional testimony around this time characterizes small biotech companies and the drugs developed by such companies as reflecting valuable, "real innovation" in the scientific ecosystem. For example, an expert witness testifying at the Senate Finance Committee hearing on prescription drug pricing noted that the proposed Build Back Better Act – a precursor to the IRA with a similar small biotech exception[7] – contained a small biotech exclusion in order to be "thoughtful" and "protect[] innovation that is truly innovative […] made by very small biotech companies [….] [T]he legislation is […] making a distinction [around] things that [represent] real innovation …." [8] Additionally, Rep. Schrader, a proponent of the drug pricing reform framework included in the IRA, stated during a House of Representatives debate that the IRA's drug pricing framework will "preserve scientific creativity and the innovation ecosystem."[9]

In this manner, Congress affirmed its desire to ensure meaningful protections for smaller innovative firms that fuel "scientific creativity" and the "innovation ecosystem." Indeed, small innovators, or small biotechs, serve as pioneers in medical research and therapeutic development for the benefit of patients. Unlike certain larger players in the pharmaceutical market, these entities often operate on limited resources, taking substantial financial and other risks to bring novel therapies to market. Congress memorialized its intent to protect such innovative companies, so they can recoup their investments and continue groundbreaking work, when it deliberately afforded small biotech companies several categories of negotiation and pricing protections under the IRA – spanning a five-year window. For instance, small biotech drugs are exempted from negotiations and price applicability for the years 2026 through 2028. Additionally, for the years 2029 and 2030, small biotech drugs newly subject to price

[5] Social Security Act (SSA) § 1192(d)(2).
[6] *Id.* § 1194(d).
[7] Kaiser Family Foundation (KFF), *Explaining the Prescription Drug Provision in the Build Back Better Act* (Nov. 23, 2021), *available at* https://www.kff.org/medicare/issue-brief/explaining-the-prescription-drug-provisions-in-the-build-back-better-act/.
[8] *Hearings to Examine Prescription Drug Price Inflation, Focusing on the Urgent Need to Lower Drug Prices in Medicare; Before the S. Committee on Finance*, 117th Cong. (Mar. 16, 2022) (statement of R. Conti).
[9] 168 Cong. Rec. 7,686 (daily ed. Aug 12, 2022) (statement of Rep. Schrader).

applicability receive the benefit of a price floor, while they prepare to adjust to the full impact of the drug price negotiation program. These IRA provisions offer important protections to small biotechs and, consistent with Congressional intent, should be broadly construed in order to protect and reward small biotech innovation. Indeed, given the relative paucity of companies that serve as small biotechs in the current pharmaceutical industry, CMS should interpret the statute liberally in order to afford protections to companies during this five-year window, as they prepare for potential consequences of the IRA negotiation program.

II. BeiGene urges CMS to solicit input on, and make available to stakeholders, its full intended small biotech methodology for all program years; to make applicable data available for validation; and to afford a clear appeal process for contesting small biotech determinations.

To date, the guidance CMS has provided on the small biotech exception has been limited in scope and nature – with an emphasis on the first year of the negotiation program (i.e., 2026). Through the Small Biotech ICR, CMS has solicited feedback on the burdens associated with providing specified information to help determine small biotech eligibility. While the ICR and CMS's initial guidance represent a first step in illuminating the agency's plans with regard to small biotech provisions under the IRA, CMS has not yet expounded on its overall intended methodology to assess small biotechs and provide them with the enumerated statutory relief in 2026 *and* later years of the program. Small biotech companies need to understand this methodology as soon as possible in order to make important clinical and business decisions. Indeed, small biotech companies, perhaps more so than other industry stakeholders, confront potentially enormous consequences of agency implementation decisions. This is the case because, oftentimes, a small biotech company has a single product as its driving force and focus. Agency decisions that would adversely impact that product's price and/or accessibility could dramatically, and adversely, impact the business and its future.

Relatedly, any data the agency intends to use to determine eligibility for the small biotech exception should be made available to manufacturers for validation. We note that the IRA designates that only total drug expenditures in 2021 will be considered in small biotech exception determinations,[10] and the initial guidance further specifies that CMS plans to use the 2021 prescription drug event (PDE) data for such purposes.[11] It is not clear whether CMS intends to rely on other, non-publicly available data to further inform its small biotech determinations, and CMS has said that small biotechs must reapply annually for qualification/eligibility.[12] Any other data CMS intends to use should be made available to manufacturers for review and validation. More broadly, CMS's methodology for all years should be made clear as soon as possible, to allow potential small biotech companies to assess their eligibility and make appropriate plans.

Finally, CMS should establish a robust, fair, and transparent dispute resolution process. This mechanism should allow manufacturers to appeal and contest determinations made by CMS regarding their eligibility for small biotech relief in 2026 and later years. This process should be timely, efficient, and during the pendency of any dispute, manufacturers should receive the

[10] SSA § 1192(d)(2)(A)(i), (ii).
[11] Initial Guidance § 30.2.1.
[12] *Id*.

benefits of the small biotech exception if they believe in good faith their drug qualifies under the statutory criteria. Affording manufacturers an adequate appeals and dispute resolution process is both a matter of fairness and is a critical element of a drug price negotiation program run with integrity and credibility.

We seek clarity as soon as possible on CMS's complete methodology for determining whether a drug satisfies the small biotech exception – across all years of the IRA negotiation program – for transparency, predictability, and planning purposes. We continue to seek and look forward to robust opportunities to engage with the agency around these important determinations.

III. BeiGene supports strong stakeholder engagement and encourages CMS to consider comments on all aspects of the Medicare drug price negotiation program.

BeiGene appreciates CMS's stated commitment to transparency and robust stakeholder engagement[13] in the implementation of the IRA negotiation program. However, we are concerned that critical portions of the initial guidance – namely those that relate to the identification and selection of drugs for the negotiation program and the small biotech exception – were issued as final without public comment.

In order for CMS to successfully implement a program with such complexity, breadth and changes to our current healthcare system, the agency should solicit robust input from diverse stakeholders – including patients, caregivers, health care professionals, and others – on legal and policy considerations related to implementation. Indeed, the agency's implementation decisions, including those related to which drugs qualify, will undoubtedly impact patient access to medicines and healthcare equity.

Key stakeholders that participate in the Medicare program – including drug manufacturers, health care professionals, and patients – should be afforded meaningful opportunities to engage in CMS's plans for implementation of the negotiation program. We consider such active participation to be not merely a courtesy and helpful practice, but rather an essential and foundational procedural right. While we can appreciate the agency's desire for expediency in implementing the first year of the program and the 2026 price applicability year, it is nonetheless critical that the agency promptly begin engaging the public in planning for later years of the program as well.

Stakeholders must have clarity as soon as possible on the manner in which CMS intends to implement later years of the program so they may plan accordingly. Given the unprecedented nature of this program, which stands to fundamentally reshape Medicare, clinical and research priorities, and the entire biopharmaceutical marketplace, it is of utmost importance that CMS provide clarity on its implementation intentions as soon as possible. We urge the agency to solicit and consider comments on all aspects of the program during all implementation years, and to do so expeditiously.

***

[13] CMS, Medicare Drug Price Negotiation Program: Next Steps in Implementation for Initial Price Applicability Year 2026 at 1 (Jan. 11, 2023), *available at* https://www.cms.gov/files/document/medicare-drug-price-negotiation-program-next-steps-implementation-2026.pdf.

BeiGene appreciates the opportunity to comment on the Small Biotech Exception ICR (CMS-10844 / OMB 0938-0982). We thank you in advance for your consideration of these comments and are available to discuss them in further detail. Should you have any questions or concerns, please contact Rebecca Davison, at rebecca.davison@beigene.com or (862) 335-1854.

Sincerely,

John P. Halliwell
Executive Director, U.S. Government Affairs & Public Policy