



March 27, 2023

Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 212441

**RE: Small Biotech Exception
CMS 10844**

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to comment on the proposed Information Collection Request (ICR) on the small biotech exception to the drug negotiation provisions of the Inflation Reduction Act.

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access unique to rare diseases or particularly pronounced in extremely rare diseases. Haystack Project is committed to educating policymakers and other stakeholders about the unique circumstances of extremely rare conditions with respect to product development, commercialization, and fair access to care. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for all Americans living with or caring for someone with a rare or ultra-rare condition.

Our rare disease communities struggle to navigate health system challenges in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic. Our comments offer our insights and recommendations to enable CMS to build upon its efforts to ensure that the benefits of reduced drug prices for the general Medicare population confer equally to all beneficiaries regardless of the rarity of their health condition(s).

Background

While countless lives have been improved or saved by new therapies enabled by Congress' set of incentives for orphan drugs, 95% of the 7,000 rare diseases identified to date have no FDA-approved treatment option. Patients suffering from rare diseases that are currently untreatable have maintained hope that the incentives toward innovation, coupled with increased scientific understanding of disease mechanisms, would stimulate progress toward treatment and, eventually, a cure.

As you know, the economic calculation of research and development costs, projected risk, and population-based revenue estimates must be accompanied by an analysis of whether it is possible to successfully clear reimbursement mechanisms and hurdles that may tip the scales for or against pursuing a specific drug candidate for an orphan indication. For patient populations approaching the 200,000 orphan disease limit, current incentives may be sufficiently robust to mitigate clinical trial and reimbursement risks. However, as affected populations dwindle below 20,000 or even into and below the hundreds, the balance can be far more fragile.

Patients rely on payers, and society in general, to lay a strong foundation that gives investors a measure of certainty that research efforts will result in patient access to treatment innovations. To date, most treatments developed to address extremely rare conditions have been discovered by small biotechnology entities focused primarily on research. The significant costs of going from bench to market cannot be raised without strong investor interest and efforts usually require partnering with an established biotechnology or pharmaceuticals manufacturer. Unless investors (including industry partners) have reason to believe that the costs of research and development can be recouped, either through the price of the new drug, its use in other patient populations, or both, there is little reason for us to hope they will invest their limited resources in advancing the treatments we need.

Haystack Project fully recognizes that CMS has been charged with implementing the Inflation Reduction Act provisions related to price negotiation, including the small biotech exception, as Congress directed. We urge CMS to exercise its implementation discretion in a manner that, to the extent possible, avoids disrupting the fragile balance between risk and reward that has fueled hope in our patient communities.

Increased clarity on CMS' process

In addition to a clear timeline, our patient communities and the small biotech companies manufacturing the treatments patients rely upon need greater clarity on CMS' process for making this important determination. We urge CMS to:

- *Ensure that manufacturers know how and when they will be informed of CMS' receipt of a submission and determinations on completeness and eligibility for the exception.* CMS' communication could be by email, letter, or other mechanism, but it is essential that manufacturers know what they are looking for and when to look for it.
- *Provide a substantive response to submissions when it determines that a small biotech manufacturer's drug is ineligible for the exception.* The response should be sufficiently detailed to enable manufacturers to provide any data or other information that may refute a negative CMS determination.
- *Implement a dispute resolution process that manufacturers can understand and utilize in the event of a negative determination.*

- *Accept manufacturer submissions through a dedicated email “inbox.”* Haystack understands that CMS intends to develop an HPMS tool that manufacturers would use to submit information on the Small Biotech Exception ICR form. Unfortunately, creating new processes within short implementation timeframes increases the likelihood for delays, errors, and inadvertent inclusion or exclusion of information. Emailed submissions with automated receipt response can ensure that manufacturers know that the information they intended to send was received.
- *Maintain open lines of communication between specific CMS personnel making determinations on small biotech exception eligibility and manufacturers submitting information to qualify their drugs.* Our patient and caregiver communities know all too well that the decisions on our access to treatments are often made within closed processes that do not include our participation. The IRA implementation processes are new to industry, patients, and CMS, and are therefore vulnerable to miscommunications, inadvertent submission errors, and other missteps that could prove dispositive. A clear and open line of communication between CMS staff and manufacturers can avoid unintended delays and erroneous determinations.
- *Streamline continuing eligibility for the small biotech exception.* Under the IRA, a drug determined to be eligible for the exception would lose its eligibility only if the manufacturer is acquired by a manufacturer that does not qualify for the exception. We urge CMS to apply the exception to drugs for each year upon receipt of a simple statement certifying that the manufacturer has not been acquired by another entity. A new eligibility submission should only be required when an acquisition has occurred, and the new manufacturer seeks to qualify for the exception.

Timelines for small biotech exception approval process

CMS' ICR appears to envision a single “due date” for companies to submit required information to CMS that would be applicable until the exception provision sunsets. The precise date for submitting information will be provided within program instructions developed by CMS, but it will be well in advance of the September 1, 2023, date for CMS publication of its selected drug list for initial price applicability year 2026. We ask that CMS:

- *Allow for small biotech exception submissions in each year for which the exception is applicable.* This will permit companies that failed to fully submit required information within the timeframe allowed to secure the exception for the drugs it was intended to benefit.
- *Set a date for small biotech companies to submit their information no earlier than mid-June 2023.* This will enable innovators and their industry partners (if any) to determine the entity responsible for submission and collect the required information. This is especially important for small biotech companies that are marketing one or more drug products as the data elements may require access to sources that are not familiar to smaller companies.

- *Inform small biotech companies on whether their submission is complete on a rolling basis throughout the submission period, ideally within 10 days of submission.*
- *Furnish a material response to submissions indicating whether the submission was successful.* The response should (as noted above) provide a clear and substantive rationale for CMS' decision if the Agency determines that the drug is ineligible for the small biotech exception.
- Permit a 3-week period for small biotech companies to respond to a negative CMS determination.

Increased clarity on information CMS will rely upon and confidentiality of information received by submitting manufacturers.

Haystack Project expects to provide additional input on the small biotech exception within its comments to CMS' recently-released initial guidance on the negotiation process. The ICR and accompanying explanations do not, however, fully implement the statutory language related to the IRA small biotech exception. In addition, it lacks sufficient clarity on the data sources CMS will rely upon and the confidentiality of manufacturer-submitted information. We urge CMS to:

- *Modify its "form" for small biotech exception qualification to fully comply with the statutory language.* The IRA provides for a two-pronged "test" conferring eligibility to the exception when drugs meet **either** prong. This means that a drug would be eligible for negotiation applicable to Part D drugs if it meets either the 1%/80% test on Part D expenditures or the 1%/80% test on Part B expenditures. The ICR, however, does not permit entities to qualify for the exception when a drug meets the "Part B Prong." The fact that CMS' selection for 2026 focuses on Part D drugs does make the Part B prong of the small biotech exception eligibility criteria irrelevant. In fact, the statute clearly envisions crossover between Part D and Part B - the Maximum Fair Price for selected and negotiated drugs will be applied to drugs acquired under the Part D benefit as well as to those administered by a clinician and covered under Part B.
- *Make the data CMS will use for 2021 expenditures publicly available.* CMS has noted that it will not use the drug dashboard data currently available on cms.gov to select or negotiate drug prices. Haystack Project strongly believes that transparency on the 2021 drug expenditure data will help CMS ensure that its determinations on eligibility for the small biotech exception are accurate and based on a shared understanding of the information CMS will rely upon.
- *Inform the public, including manufacturers, on CMS' approach for handling any proprietary information it might obtain from manufacturers seeking eligibility for the small biotech exception.* This is particularly critical for information submitted on drugs with more than one "manufacturer" given the potential for disclosure by one manufacturer of information that is "proprietary" to its partner manufacturer.

Conclusion

Haystack Project and its member organizations appreciate the opportunity to submit comments as CMS implements the small biotech exception to negotiation under the IRA. We look forward to a continuing dialogue with the Agency as it continues to implement the law so that all Medicare beneficiaries have access to the care they need.

If you have any questions or would like to discuss the issues raised in our comments, please contact me or our policy consultant, M Kay Scanlan, JD at (410) 504-2324.

Very truly yours,



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