***VIA ELECTRONIC SUBMISSION***

July 31, 2023

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

RE: **RE: Information Collection Request (ICR) for Negotiation Data Elements (CMS-10847)**

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to provide comments on the Centers for Medicare & Medicaid Services' (CMS') revised Information Collection Request for Negotiation Data Elements (the ICR).

Haystack Project is a 501(c)(3) non-profit organization enabling our membership of 140+ rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. 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. We strive to amplify the patient and caregiver voice in these disease states where unmet need is high and treatment delays and inadequacies can be catastrophic.

As we have previously stated, patients with rare and ultra-rare conditions rely on health system and public policy priorities that give investors a level of comfort 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. Without this, there is little reason for Haystack Project's patient and caregiver communities to hope that resources will be invested in advancing the treatments we need.

We appreciate that CMS considered our previous comments and revised the ICR to:

- Enable broader input on unmet medical need to include unmet needs associated with patient subpopulations as well as a general need within a condition that is not adequately addressed by available therapeutic options.

- Include more specific questions focused on soliciting input on unmet needs, off-label uses, and health equity considerations.

- Include consideration of side effects and adverse outcomes associated with alternative treatments.

- Remove references to fraud and abuse penalties within the certification statement that may have discouraged patients, caregivers, and researchers from providing input on selected drugs.

Haystack Project remains concerned that the overall framework for negotiation as outlined in the Initial Guidance largely drives the data elements and will inevitably compromise efforts to achieve a fair price for treatments used in rare and ultra-rare conditions that is aligned with value. We are similarly – if not more - concerned that the policies and statutory interpretations CMS has adopted will go further in detering innovation in ultra-rare conditions than contemplated within the IRA.

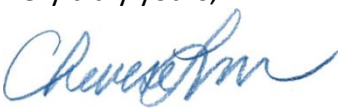
Our concerns are more fully outlined in Haystack Project's May 22, 2023, which we have attached to and incorporated in this submission.

**Conclusion**

Haystack Project appreciates the opportunity to submit feedback on the ICR and views this process as critical to ensuring that CMS implementation of the drug price negotiation program is consistent with the language and intent of the IRA. Our member organizations have significant concerns that the decisions CMS makes now will impact the set of new treatment options in rare and ultra-rare conditions into the foreseeable future.

Once again, we thank you for your consideration of our comments. If you have any questions, please contact our policy consultant M Kay Scanlan, JD at 410.504.2324.

Very truly yours,



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***VIA ELECTRONIC SUBMISSION***

May 22, 2023

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

RE: **RE: Information Collection Request (ICR) for Negotiation Data Elements (CMS-10847)**

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to provide comments on the Centers for Medicare & Medicaid Services' (CMS') Information Collection Request for Negotiation Data Elements (the ICR).

Haystack Project is a 501(c)(3) non-profit organization enabling our membership of 140+ rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. 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. We strive to amplify the patient and caregiver voice in these disease states where unmet need is high and treatment delays and inadequacies can be catastrophic.

Patients with rare and ultra-rare conditions rely on health system and public policy priorities that give investors a level of comfort 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. Without this, there is little reason for Haystack Project's patient and caregiver communities to hope that resources will be invested in advancing the treatments we need. Our comments (attached) to CMS' Initial Guidance implementing the Drug Price Negotiation Program under the Inflation Reduction Act (IRA) articulate our concern that the negotiation processes will fail to consider treatment value for rare patients and ultimately negate the incentives that have enabled development of new treatments and maintained commercial viability of existing therapies.

Haystack Project is concerned that the sufficiency of the data elements within the ICR and the burden associated with providing that information are inextricably linked to and vastly impacted by the Initial Guidance. Haystack Project remains concerned that the most impactful policies and interpretations within the Initial Guidance were finalized without opportunity for public notice and comment and create a drug price negotiation program that has a greater potential to disrupt access to current and future treatments than the plain language of the IRA

likely contemplates much less requires. Moreover, the Initial Guidance policies increase the ICR's burden on manufacturers and decrease the extent to which the information collected aligns with the IRA's apparent goal of ensuring that Medicare drug prices reflect treatment value without disrupting incentives toward innovation, including longstanding statutory incentive frameworks that have driven innovation in rare disease therapies.

The ICR represents a procedural and substantive guardrail to ensure that public comment is fully considered. This guardrail is important when the information collection is based on underlying policy determinations and interpretative rules rather than the plain meaning of the statute; it is crucial when those policies and rules were not subjected to public notice and comment.

Our comments emphasize the need for CMS to:

- Reconsider use of moiety or active ingredient rather than NDA/BLA to identify negotiation-eligible drugs because is particularly harmful for securing approvals for small population conditions, further building on IRA provisions harmful to rare and especially ultra rare diseases

- Revise its Primary/Secondary Manufacturer framework so as not to inappropriately over burden rare disease manufacturers and other entities that either license their developed products or acquire and commercialize new treatments

- Re-work the process for non-manufacturer submissions on alternative therapeutic options which, as proposed, is so onerous and limited that it appears designed to discourage patient, advocacy organization and clinician input.

**CMS' decision to identify negotiation-eligible drugs based on moiety or active ingredient rather than NDA/BLA overburdens manufacturers and dilutes the nexus between a "monopolist" drug and its value to patients.**

Haystack Project had anticipated that CMS would identify negotiation-eligible drugs on the basis of NDA/BLA approvals given the statutory reference to NDA/BLA approval date in identifying negotiation-eligible drugs. CMS' decision to broadly define qualifying single source drug' for negotiation eligibility purposes was unexpected and will likely negate existing incentives for securing approvals in small population conditions and place burdens on industry stakeholders that were not likely contemplated when the statute was enacted.

- Under CMS' definition, a drug with an NDA/BLA approval could be negotiation-eligible earlier than the 9 or 13 years outlined in the IRA if a reference drug is negotiation-eligible. In fact, drugs, including orphan drugs with statutory exclusivity, approved after selection and negotiation would be subject to the maximum fair price.

- This is not a simple implementation of a statutory requirement; it appears to be an Agency policy determination driving a statutory interpretation beyond and in likely conflict with the plain language of the IRA.

- Haystack Project members have brought us anecdotal reports of manufacturers shutting down research and development efforts toward new indications for existing drugs and re-focusing efforts away from ultra-rare to more robust orphan indications due to perceived inability to recoup research costs on a drug subject to an MFP at or shortly after approval.

- Unless CMS retracts its determination to include all NDAs/BLAs for a product as a singular qualifying single source drug for negotiation purposes, our patients have little hope that manufacturers will be able to justify to their shareholders that investing in NDA/BLA approvals for ultra-rare uses of existing treatments is a sound business decision.

- CMS' definition of qualifying single source drug will place information collection burdens on manufacturers that Congress did not consider in drafting the IRA.

  - The scenario examples set forth in the Initial Guidance contemplate requiring the primary manufacturer (NDA/BLA holder) to assume full responsibility and liability for participation in the negotiation process, submission of complete, accurate information and access to the MFP regardless of their role in commercialization activities.

  - Manufacturers often develop drug candidates and then license one or more current or future indications to a commercialization partner. In these instances, research and development costs are split across multiple entities and a manufacturer with data on those costs may not have access to data on sales volume, revenue, and other data elements required within the ICR.

- The MFP is a single price for a drug under the Medicare program. The IRA negotiation process outlines considerations such as alternative therapies, unmet need, and the extent to which a treatment represents an advance in therapeutic options.

  - Had CMS adhered to the NDA/BLA driven approach to drug selection outlined in the IRA, data collected on a drug's value to patients would be clearly related to the NDA/BLA and the patients and conditions to which it applies.

  - Aggregating NDAs/BLAs into a single negotiation-eligible drug reduces the nexus between data collected and the true value of the treatment to patients.

        - The value determination will place unwarranted emphasis on large patient populations in disease states with multiple treatment options.

        - Any value in treating rare and ultra-rare patients will be diluted and ultimately rendered irrelevant. This would be the case even if the drug was the only approved option in treating a life-threatening disease.

    - Information on alternative therapies is indication-specific. CMS' decision to utilize costs of alternative therapies in calculating an initial offer does not appear reasonable unless the selected drug is defined by an NDA/BLA rather than moiety or active ingredient.

        - Aggregating NDAs/BLAs with multiple, potential diverse, indications and patient populations would lead to a MFP that aligns with the NDA/BLA with the largest patient population.

        - Applying an aggregated alternative-therapies-based initial offer to an NDA/BLA in a small disease population for which alternative treatments are either more costly or nonexistent would, for practical purposes, ignore the considerations the IRA outlines as part of the negotiation process. The negotiated price, as applied to that NDA/BLA would be driven by value, time on the market, research costs, and other factors applicable to a different drug treating a different condition.

            - Haystack Project believes that this result is bad for rare and ultra-rare patients waiting for a treatment to come to market and that the MFP, as applied to that NDA/BLA, would be arbitrary rather than negotiated.

- CMS' definition of unmet medical need is narrow and fails to consider unmet needs associated with patient subpopulations, or a general need within a condition that is not adequately addressed by available therapeutic options.

    - Failure to determine unmet need based on NDA/BLA will make it impossible for CMS to incorporate actual, real-world unmet needs across divergent patient populations and disease states. Once again, aggregating unmet need will yield a result that provides an inaccurate, arbitrary result for indications with multiple, effective therapies as well as those indications for which few options exist.

**Haystack Project remains concerned that CMS' Primary/Secondary Manufacturer structures will overburden manufacturers, particularly the small biotech and pharmaceutical manufacturers that have historically developed rare disease treatments.**

Arrangements between an early-stage innovator and a larger manufacturer with commercialization expertise are common in the rare and ultra-rare disease space. Agreements between manufacturers are generally based on contracts negotiated and executed well before the parties perform any manufacturing, distribution, and/or marketing activities, and are based on the laws and regulations in place at the time. Neither the IRA, the ICR, nor CMS' Initial Guidance provide for any mechanism through which a primary manufacturer can secure information required within the ICR from a secondary manufacturer.

While CMS might assume that manufacturers can contract with each other to accommodate the IRA requirements, the substantial liability and potential monetary penalties placed on primary manufacturers creates an extremely unlevel playing field. This construct also increases the level of risk associated with investment and partnering opportunities in rare disease treatments initially developed by a pre-clinical manufacturer.

Haystack Project urges CMS to refine its approach given that the burden associated with providing information a manufacturer has no legal recourse to access, much less disclose, is both enormous and avoidable.

**The ICR appears to purposefully discourage the public input on alternative therapies and unmet need that it purports to indicate is crucial to the negotiation process.**

The ICR provides for public input into the consideration of alternative therapeutic options. Unfortunately, the process for submission, limitation of information content and quantity, and certification requirement will substantially deter patient advocacy organization input.

- The 30-day comment period is far too short for organizations like Haystack Project to collect specific, meaningful input from our member organizations and incorporate the feedback into a comprehensive comment.

- Most patients with rare and ultra-rare conditions have no FDA-approved treatment options and rely on off-label uses of existing treatments. These uses are rarely included within the compendia CMS lists as acceptable sources of information on off-label indications.

    - CMS should ensure that rare and ultra-rare disease patients can provide information on their off-label treatments, potential alternatives, unmet need, and the extent to which their prescribed therapy has improved their quality of life, slowed disease progression, or otherwise improved outcomes.

- CMS has not articulated how the information and scientific evidence it collects will be used to inform decisions on therapeutic alternatives or what evidence is particularly important in the negotiation process.

- Rare and ultra-rare disease patients will find it difficult to challenge CMS identification of an alternative treatment option unless CMS provides information on the treatments it is considering. For example, CMS may focus on a high-volume indication and identify multiple treatment options that could be substituted for the selected drug.

    - Our patient communities cannot provide information on whether or not those therapies are, in fact, actual options in treating their condition or contraindicated/ineffective unless we know what those alternatives are.

    - Without that information, patient advocacy organizations may be able to identify condition-specific options or state that there are no alternative therapies. The ICR and CMS' Initial Guidance do not provide information on how this relatively nonspecific information would be weighed against data on alternative therapies for more common conditions.

- Haystack Project expects that CMS' decision to use the HPMS system for ALL information collection activities associated with the drug price negotiation program will make it difficult for patient advocacy organizations to weigh-in throughout the process.

    - How will CMS notify the public that a comment period is available? Patients and patient advocacy organizations do not have current access to HPMS. We urge CMS to use the notice and comment processes established within regulations.gov to provide notice and receive comments from the public.

- Limitations on the number of words or citations that can be submitted to CMS are unlikely to improve the quality or relevance of information received. We urge CMS to remove those limitations and to encourage stakeholder input relevant to the drug price negotiation.

- The ICR's Section J, *Certification of Submission for Respondents Who Are Not Primary Manufacturers Required for All Respondents Who Are Not Primary Manufacturers***,** is an onerous requirement that implies potential civil or criminal liability. It is inappropriate and unnecessary when applied to patients, patient advocacy organizations and clinicians.

    - Patients and their advocacy organizations must certify that the information is complete and accurate, yet CMS does not provide any guidance on what would constitute a complete submission from patient stakeholders.

    - Non-manufacturer stakeholders must also certify that they will "timely notify CMS if I become aware that any of the information submitted in this form has changed." A potential commenter may infer an obligation to inform CMS about changes in medication or symptoms, appearance or reduction of side effects,

changes in out-of-pocket costs, emergency room visits, and other health care encounters.

- There does not appear to be a simple, identified process through which non-manufacturer stakeholders would submit updated information.

- Finally, patients and patient advocacy organizations must acknowledge that they "also understand that any misrepresentations may also give rise to liability, including under the False Claims Act."

    - Haystack Project expects that this provision will significantly deter stakeholders from providing CMS with information that would improve the negotiation process and the data upon which the Agency will rely.

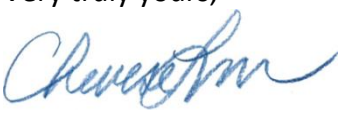
    - If CMS expects that this certification requirement is a necessary part of its information collection, it should narrow the set of stakeholders to which it would apply.

**Conclusion**

Haystack Project appreciates the opportunity to submit feedback on the ICR, and view this process as critical to ensuring that CMS implementation of the drug price negotiation program is consistent with the language and intent of the IRA. Our member organizations have significant concerns that the decisions CMS makes within the next several months will determine the set of new treatment options in rare and ultra-rare conditions and rare cancers for the foreseeable future. More importantly, the decisions likely to have the greatest impact have been made without public notice and comment or a meaningful dialogue between CMS and the rare and ultra-rare disease community.

Once again, we thank you for your consideration of our comments. If you have any questions, please contact our policy consultant M Kay Scanlan, JD at 410.504.2324.

Very truly yours,



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