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March 11, 2023  
The Honorable Chiquita Brooks-LaSure  
Administrator  
Centers for Medicare & Medicaid Services  
Department of Health and Human Services  
P.O. Box 8013  
Baltimore, MD 21244-1850

VIA ELECTRONIC DELIVERY to: [IRAREbateandNegotiation@cms.hhs.gov](mailto:IRAREbateandNegotiation@cms.hhs.gov)

**Re: Medicare Part D Inflation Rebate Comments**

Haystack Project appreciates the opportunity to submit its comments in response to the Centers for Medicare & Medicaid Services' (CMS') Medicare Part D Inflation Rebate Guidance.

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease advocacy organizations to highlight and address systemic access barriers to the therapies they desperately need. Our core mission is to evolve health care payment and delivery systems, spurring innovation and quality in care toward effective, accessible treatment options for Americans living with rare or ultra-rare conditions. 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.

Haystack Project supports health policy refinements that make it possible for all patients to receive the medications they need without compromising the financial sustainability of our payer systems or chilling innovation in disease states with high unmet needs. Our comments offer insights and recommendations from Haystack Project's over-130 ultra-rare disease patient advocacy organization members so that CMS can continue to build upon its efforts to ensure that Medicare coverage and benefits confer equally to individuals regardless of the rarity of their health condition(s).

**Background**

Of the approximately 7,000 rare diseases identified to date, 95% have no FDA-approved treatment option. Advances in research and development such as regenerative medicine, gene

therapy, and other targeted therapy innovations offer a renewed hope for Haystack Project's patient and caregiver communities that a treatment might be on the horizon for any disease, no matter how rare. Unfortunately, treatments targeted to extremely rare conditions are, by necessity, associated with high costs when compared to drugs developed for more common, well-understood disease states. We have significant concerns that unless CMS fully considers the unique challenges associated with developing and manufacturing rare disease treatments as it implements provisions of the Inflation Reduction Act (IRA), our patients will suffer disproportionately from its unintended consequences.

As you know, Congress tackled the incentive framework for orphan drugs to counter the commercial realities associated with research and development toward treatments for serious medical conditions affecting small populations. Countless lives have been improved or saved by new therapies since then. The economic calculation of research and development costs, projected risk, and population-based revenue estimates must include a realistic assessment of reimbursement mechanisms and payment structures that can tip the scales for or against pursuing a specific drug candidate for an orphan indication.

While the Orphan Drug Act (ODA) clearly boosted interest in pursuing rare disease treatments, its incentives are a fixed set of counterbalances to the inherent risk associated with rare disease research and development. When patient populations approach the 200,000 orphan disease limit, the ODA incentives may be sufficiently robust to mitigate clinical trial and reimbursement risks. As affected populations dwindle below 20,000 or even into and below the hundreds, however, the balance is far more fragile. Innovators newly considering a pipeline candidate in an ultra-rare disease state face substantial uncertainties on whether Medicare and other payers will maintain sufficient payment to ensure commercial viability. The inflation rebates will add an additional layer of uncertainty and risk.

Haystack Project and its member organizations appreciate that CMS must implement the inflation rebate provisions of the IRA within an extremely limited timeframe. We generally support many of the policies outlined in CMS' guidance as applied to most treatments covered under Medicare Part D. We are, however, concerned that the unique circumstances associated with treatments for extremely rare diseases will drive risks and uncertainties that will not only discourage new product development but threaten financial viability of existing treatments. This would be catastrophic for our patient communities.

### **Reducing or Waiving the Rebate Amount in the Case of a Part D Rebatable Drug on the Shortage List**

Section 1847A(i)(3)(G) provides that CMS reduce or waive the rebate amount with respect to a Part D rebatable drug for an applicable calendar quarter in two cases: (1) when a Part D rebatable drug is described as currently in shortage on the shortage lists authorized under section 506E of the Federal Food, Drug, and Cosmetic Act (FD&C Act) at any point during the calendar quarter; or (2) for a biosimilar biological product when the Secretary determines there

is a severe supply chain disruption during the calendar quarter, such as that caused by a natural disaster or other unique or unexpected event.

CMS states that it intends to structure this policy to provide a period of financial relief for manufacturers in certain circumstances without creating incentives for manufacturers to intentionally maintain their drug or biological in shortage for the purpose of avoiding an obligation to pay a rebate.

Haystack Project supports broad application of CMS' authority to adjust and/or waive imposition of rebates on drugs impacted by shortages. We also appreciate that CMS has asked whether there are "specific causes for or types of a shortage where CMS might reduce or waive the rebate amount differently, such as drugs that treat certain conditions or address critical need, and how CMS would identify such drugs."

We ask that CMS fully consider the impact of its guidance on rare disease treatments and urge the Agency to implement a set of safeguards and/or exceptions to address the realities associated with small population treatments, including, for example:

- New requirements for manufacturing and/or quality assurance can introduce significant costs that are allocated over a smaller volume of product. Manufacturers facing these challenges must increase prices to account for increased cost, attempt to "sell" the asset to a manufacturer able to accommodate the requirements, or stop manufacturing the treatment.
- Shortages and/or price increases in ingredients will present more of a challenge to manufacturers producing low-volume treatments as they do not have the purchasing power of their high-volume counterparts. This could result in a real-world ingredient shortage well in advance of official product shortage reporting.
- Introduction of new products to address an ultra-rare disease can have an enormous impact on the per-unit costs of continuing to manufacture an older treatment. For example:
  - o If rare disease X impacts 20,000 patients and is associated with 5 acute "attacks" per patient each year, a drug addressing the attacks could expect volume of 100,000 treatment episodes per year.
  - o A new treatment option that reduces the incidence of these attacks would be valuable to patients but would not eliminate the need for the older product.
  - o Unfortunately, many of the manufacturing costs for the older product are fixed regardless of volume. Without the ability to increase the product price, a manufacturer could not continue to offer the product.

Haystack Project urges CMS to implement a limited set of guardrails applicable to rare disease products that would protect manufacturers of products addressing small populations from

punitive rebates when (and to the extent that) increases in the costs of manufacturing a unit of product exceed the applicable CPI-U. Without this protection, Haystack Project fears that it will become increasingly difficult to protect or project the commercial viability of the treatments many within our patient communities rely on and most hope will be developed in the future.

### **Value-Based Arrangements Should Not Trigger or be Subject to Inflation Rebates.**

The Administration has prioritized a set of innovation models focused on further reducing the costs of drugs and biologics, including value-based arrangements for cell and gene therapies. These arrangements are likely to be increasingly adopted among commercial payers as a coverage and payment mechanism for high-cost treatments. Haystack Project expects that treatments for rare and ultra-rare conditions will be disproportionately impacted by value-based payments that rely on patient-specific outcomes for determining the actual price received for the treatment.

These arrangements are inherently associated with dips and peaks in drug “price” over time without any further manufacturer decision or action. In fact, it is likely that payers and manufacturers could improve their ability to identify likely responders over time. This could lead to imposition of a penalty in the form of inflation rebates based on improved patient selection, increased provider experience managing the patient, and other factors associated with real-world “value” to patients and payers.

We urge CMS to revise its guidance to accommodate and protect pricing arrangements aligned with value and improved patient outcomes.

### **CMS Should Enable Manufacturers to Avoid Inflation Rebates When AMP Fluctuations Are Outside their Control.**

Haystack Project expects that AMP fluctuations from quarter to quarter are particularly common for drugs treating rare and ultra-rare conditions. These fluctuations can occur for many reasons beyond the manufacturer’s control. For example, a greater number of patients being covered (or ceasing coverage) by a major payer, introduction or removal of mail-order pharmacy options, and other factors can have a significant impact on the AMP – the smaller the total patient population, the greater impact a single patient or payer will have.

We are concerned that patient access to necessary treatments will be impeded if CMS imposes inflation penalties on manufacturers when they have not increased their list price (or even changed contract terms). This was not the intent of the statute.

Haystack Project is similarly concerned with the interaction between inflation rebates and the increasing interest among Medicare, Medicaid, and other payers in reducing payment for accelerated approval treatments. Haystack Project has voiced its objection to this policy and

will continue to do so. If, however, payers subject accelerated approval treatments to a discount until confirmatory studies demonstrate clinical benefit, it would be unfair and counter-intuitive to impose an inflation penalty when the product receives traditional approval.

## **Conclusion**

Haystack Project appreciates the opportunity to provide feedback on this important guidance. We believe that our 130+ ultra-rare disease member community is uniquely positioned to offer CMS important insights it will need to implement the inflation rebates without compromising rare disease patient access to life-saving treatments. If you have any questions or need additional information, please contact me or our policy consultant, M Kay Scanlan, JD at 410-504-2324.

Very truly yours,



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