HAYSTACK PROJECT
The Voices of Rare & Ultra Rare

April 8, 2019

VIA ELECTRONIC SUBMISSION THROUGH www.regulations.gov

The Honorable Alex Azar
Secretary
Department of Health and Human Services 200 Independence Ave. SW
Room 600E
Washington, DC 20201

RE: Removal of Safe Harbor Protection for Rebates Involving Prescription Pharmaceuticals and Creation of New Safe Harbor Protection for Certain Point-of-Sale Reductions in Price on Prescription Pharmaceuticals and Certain Pharmacy Benefit Manager Service Fees

Dear Secretary Azar:

The Haystack Project is a patient and caregiver driven non-profit organization committed to educate and advocate for innovation in, and access to, treatments for extremely rare conditions.
Our policies and priorities are driven by our core membership of leading rare disease patient advocacy organizations, and focused on healthcare delivery innovation with an eye toward spurring innovation, quality in care, and access to treatment option.

We appreciate the opportunity to comment on the Department of Health and Human Services (HHS') Office of Inspector General (OIG) Proposed Rule modifying the scope of the discount safe harbor under the federal Anti-kickback Statute (AKS), and hope that a greater understanding of the unique issues affecting our lives and families will inform the Administration's policies and initiatives.

BACKGROUND

Over 35 years ago, Congress recognized that commercial realities associated with research and development discouraged innovation in treating serious medical conditions affecting small populations. Although, millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure, there are many for whom treatments that are already here or in the pipeline cannot reach patients due to the realities of today's reimbursement structures, including the AKS.

- Of the approximately 7,000 rare diseases identified to date, 95% have no FDA-approved treatment option;
- 80% of rare diseases are genetic in origin, and present throughout a person's life, even if symptoms are not immediately apparent;
- Approximately 50% of the people affected by rare diseases are children;

- 30% of children affected by a rare disease will not live to see their 5th birthday; and
- Approximately half of identified rare diseases do not have a disease-specific advocacy network or organization supporting research and development.

Advances in research and development such as regenerative medicine, gene therapy, and other targeted therapy innovations offer a renewed hope that a treatment could be on the horizon for any disease, no matter how rare. This sense of optimism is, however, tempered by increasing discussions about whether payers, public and private, will be willing and able to pay the high cost of these very specialized treatments.

The Haystack Project applauds the policy priorities driving the Proposed Rule and view its adoption as an important step toward reducing systemic inefficiencies that tend to drive patient out-of-pocket costs higher. We urge OIG to recognize the unique challenges and realities of individuals with very rare diseases as it devises new policies and re-examines old ones.:

- The Haystack Project supports HHS' proposal to eliminate the safe harbor protecting rebates between manufacturers, and pharmacy benefit managers and/or Part D plans.

- The Haystack Project supports a safe harbor for manufacturer discounts and rebates that are passed to patients. We urge the Administration to extend this safe harbor protection to enable financial assistance, both from charitable entities and directly from a manufacturer, when there is only one treatment for a rare cancer or extremely rare disease.

- OIG should facilitate stakeholder exploration of creative financing arrangements, such as outcomes-based pricing strategies, so long as the patient is aware of the arrangement and shares in any cost savings; and

- OIG should ensure that stakeholders have adequate time to efficiently adapt their business arrangements to ensure that patients actually receive the benefits intended within the Proposed Rule.

The Haystack Project supports HHS' proposal to eliminate the safe harbor protecting rebates between manufacturers and pharmacy benefit managers and/or Part D plans.

The Haystack Project agrees with the Proposed Rule's underlying premise – that the current system of discounts and rebates in Part D is inefficient and has led to practices that run at odds with the policies underlying the Anti-Kickback Statute (AKS) and the discount safe harbor. Rebates play an integral role in formulary decisions involving multiple products, including determining tier placement and even formulary inclusion. Although the limited treatment options addressing specific rare diseases reduces the impact the rebate incentive framework has on covered patient treatment options, we agree that these rebates increase costs to manufacturers, plans, and patients without offsetting value. In fact, in many respects the role of manufacturer rebates to PBMs has evolved to more closely resemble the incentives the AKS was designed to eliminate than the discounts the safe harbor sought to protect.

The Haystack Project appreciates that HHS is exploring and addressing the role of rebates in incentivizing higher list prices and patient out-of-pocket costs. We support a system that is aligned with

facilitating affordable access for patients with rare diseases, and believe that encouraging manufacturers to lower their list prices and/or provide rebates at the point of sale could provide meaningful savings for beneficiaries, and improve access, affordability, and medication adherence. For patients with rare diseases requiring high-cost treatments, manufacturer funds now allocated to rebates would be better spent easing the cost burden for patients.

While we commend the Administration's efforts to lower list prices and improve transparency, we are concerned that the breadth of changes to distribution channel arrangements contemplated under the Proposed Rule, together with its short implementation timeline, could trigger PBM and plan behavior changes with unintended consequences for patients with higher prescription drug costs, including those with extremely rare diseases. We urge HHS to consider implementing appropriate guardrails to ensure that our patients retain affordable access to Part D coverage and the medications they need, and that they are well-informed (and not misled or confused) on the pharmacy benefits provided in each plan, as well as out-of-pocket costs, when determining which plan best meets their needs.

The Haystack Project supports a safe harbor for manufacturer discounts and rebates that are passed to patients, and urges the Administration to extend this safe harbor protection to enable financial assistance, both from charitable entities and directly from a manufacturer, when there is only one treatment for a rare cancer or extremely rare disease.

We believe that treatments for extremely rare diseases, particularly when there are limited options, do not pose the same set of fraud and abuse considerations that are present when stakeholders may have a commercial rationale for "incentivizing" treatment decisions. As patients, families and caregivers, we face a reality with few, if any, options. The high cost of prescription drugs and high out-of-pocket costs for medicines create significant challenges for our patient community. We have consistently supported HHS and CMS efforts to increase prescription drug price transparency and devise more robust mechanisms to ensure that patients receive the benefits of manufacturer price concessions.

The increased availability of health insurance has relieved some of the financial burden of disease for individuals living with rare cancers, extremely rare diseases, and other chronic conditions and that can represent catastrophic economic hardships for families. Unfortunately, insurers have increasingly imposed financial hurdles that disproportionately impact patients requiring costly medications.

The public policy considerations at the core of the anti-kickback statute are designed to protect patients from medical decisions influenced by incentives in a financial arrangement. We ask that OIG consider our reality – when we have *one* treatment available to reduce the disease burden of a very rare disease, our decision is clear. Without financial assistance, we may not be able to receive this medically necessary care. Put simply, no incentive is required, and none can influence the actual decision on what treatment is needed; financial assistance helps families make the necessary possible. Without a safe harbor that recognizes their unique situation, many patients with rare diseases can only receive treatment if they can afford to absorb their share of its cost.

We recognize that charitable entities represent a potential source of assistance for patients in need. Unfortunately, very rare diseases seldom have a charitable organization dedicated to ensuring treatment access. The rarer the disease, the less likely it is that a manufacturer's donations to a charitable entity

will ever reach any patient their therapy is indicated to treat. Distributing the funds "fairly" across patients with similar conditions results in a numbers game that we cannot win.

We urge OIG to help level the playing field for patients needing treatment for a very rare disease through a safe harbor permitting a manufacturer, either directly or through a third party to offer assistance to patients if (a) the patient requires the medication for a labeled indication associated with a very rare disease; (b) the product is an orphan drug; (c) there are limited treatment options available so that the financial assistance enables access but does not incentivize the treatment decision; and (d) the manufacturer implements uniform means testing to assess the need for assistance. Similarly, we request that manufacturers be permitted to contribute toward the costs patients incur accessing treatment such as genetic testing to ensure appropriate fit with a targeted therapy and/or travel to a center of excellence with expertise in the specific disease or treatment.

OIG should facilitate stakeholder exploration of creative treatment financing arrangements, such as outcomes-based pricing strategies, so long as the patient is aware of the arrangement and shares in any cost savings.

We are concerned that without multi-stakeholder creativity toward ensuring that new, high-cost treatments deliver on value, high costs may reduce coverage and, consequently, discourage innovators from developing new targeted treatments. Over the last year, innovators introduced two cellular therapies (CAR-T), and the first US gene therapy product for an inherited condition. For individuals with very rare conditions, these advances signal a new era in drug discovery; for payers, they usher in a new potential that health care innovation could outpace our ability to pay its associated costs. In fact, the Centers for Medicare & Medicaid Services (CMS) has recently proposed coverage limits and restrictions to Medicare patient access to these treatments, even when used for on-label indications or medically-accepted off-label indications. We believe that all payers, including Medicare and Medicaid should have the ability to devise win-win arrangements with industry that ensure patient access to treatment innovations. Value- or outcome-based treatment pricing strategies may present an attractive option for emerging treatments in rare diseases, and we urge OIG to maintain flexibility while also offering clear parameters to guide stakeholders considering these arrangements.

A recent white paper published by the Duke/Margolis Center for Health Policy discussing both the potential benefits of, and challenges with, value- or outcome-based treatment pricing noted the importance of adequate data:

> Data collection, accessibility, and interoperability are related challenges for the execution of VBP arrangements. The difficulty of monitoring and analyzing the type of patient data needed to execute VBP arrangements can be considerable. Many payers do not have access to the EHR data or lab results that would be needed to track longitudinal outcomes, and those that do often still face data that is incomplete or does not reliably capture information on outcomes of concern for the agreement, such as patient adherence, toxicity, desired endpoint, etc.
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> With increasing pressure to demonstrate value for high-cost therapies, interest in "Real-World Evidence" including patient-reported outcomes are increasingly seen as crucial to

determining effectiveness and patient satisfaction in chronic conditions such as cancer, multiple sclerosis, chronic obstructive pulmonary disease, and rheumatoid arthritis.[1]

Clearly, value-based arrangements should be developed with consideration of the availability of sufficient data to identify a price-point, and a clear understanding of patient-centered outcomes and the ability to incorporate those outcomes in determining a treatment's success. We believe that innovative pricing strategies can increase access to treatment options while reducing costs for patients and payers, so long as the arrangements are the result of voluntary participation and innovator-payer negotiation with a focus on outcomes that are important to patients.

We further urge OIG to ensure transparency for patients so that there is clear awareness of any value-based arrangements between the manufacturer, provider, and payer, and to carry over the policies within the Proposed Rule by requiring that any cost savings to payers are also reflected in the patient's cost-sharing responsibility.

We urge OIG to offer stakeholders the safety net they require in exploring value-based purchasing arrangements, so long as patient protections are implemented. We believe that this is new ground for public and private insurers, requiring a cautious look at the impact on patients, their access to treatment, and the financial impact on patients and families.

HHS should ensure that stakeholders have adequate time to efficiently adapt their business arrangements to ensure that patients actually receive the benefits intended within the Proposed Rule.

Currently, rebates play a major role in arrangements between manufacturers, plans, and PBMs that shape plan premiums, formulary structure, and service agreements. The Haystack Project is concerned that the Proposed Rule contemplates large-scale business model refinements yet affords little time to permit plans, manufacturers, and PBMs to make adjustments to their business model, much less incorporate the charge-back mechanisms needed to apply rebates to the point of sale. Without a reliable, efficient mechanism for applying chargebacks, patients will not benefit from the discounts and rebates contemplated in the proposal.

We are similarly concerned that the impending changes could trigger over-reactions that constrict access or significantly increase premiums. For example, many high-cost prescription drugs previously had a lower net cost to Part D plans due to high rebates. If these products are removed from formulary, placed on a higher tier, or subject to prior authorization and/or step therapy protocols, the patients who could benefit most from the overriding goals of this proposal – those with rare conditions -- may actually face higher out-of-pocket costs, fewer plan choices, and higher premiums.

We believe that the proposed timeframe for implementing this proposal will make it difficult for manufacturers, PBMs, pharmacies, and plans to devise the system-wide efficiencies and contractual arrangements necessary to ensure that patients benefit and drug costs are reduced.

[1] https://healthpolicy.duke.edu/sites/default/files/atoms/files/value_based_payment_background_paper_-_october_2017_final.pdf

Conclusion

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. Without this, many investors will simply choose to take the easy road by funding things that are not rare. As HHS continues to refine its implementation of the American Patients First blueprint, we urge it to include us in its decisions. We urge the Administration to not only devise any necessary protections to ensure that efforts toward reducing the costs of commonly-encountered conditions does not impede or complicate treatment access for individuals with rare conditions, but to proactively address the unique characteristics of our patients and the treatments they need.

We appreciate the opportunity to respond to the Proposed Rule. As the voice of rare and ultra-rare disease stakeholders, we look forward to working with you in the future to facilitate patient access to important treatment advances, and to further inform your policies and guidance to stakeholders with respect to the impact on individuals with extremely rare diseases. If you have any questions or would like to discuss our comments and recommendations, please contact Saira Sultan at 202-360-9985.

Respectfully submitted,

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