February 26, 2018

Ms. Patrice Drew  
Office of Inspector General, Regulatory Affairs,  
Department of Health and Human Services,  
Attention: OIG-127-N,  
Room 5541C, Cohen Building  
330 Independence Avenue SW  
Washington, DC 20201

RE: Solicitation of New Safe Harbors and Special Fraud Alerts  
OIG-127-N

The Haystack Project is an unincorporated association of patient and caregiver advocates that is committed to educate and advocate for reimbursement policies that recognize the unique circumstances of extremely rare conditions and their treatments.

The core mission of the Haystack Project is to evolve healthcare delivery innovation with an eye toward spurring innovation, quality in care, and treatment options for all Americans. Like other public policies intended to minimize the ability of “bad actors” to game the healthcare system, implementation of the anti-kickback statute can have an unintended and disproportionate impact on rare and ultra-rare diseases.

This week is Rare Disease Week, with February 29th (or the 28th in non-leap years) designated as Rare Disease Day. Hundreds of advocates are in DC and at their state capitols this week addressing issues important to the rare disease community.

While countless lives have been improved, or saved by new therapies enabled by Congress’ set of incentives for orphan drugs, millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure:

- 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, development, and patient access.

Similarly, despite dramatically increased availability of novel treatment options, many patients with rare diseases still face hurdles accessing lifesaving and life-improving FDA-approved therapies. These hurdles are often related to reimbursement structures such as high cost-sharing and/or payer coverage delays and restrictions on what may be the only treatment available to
reduce a patient’s disease burden. The potential that a treatment’s cost will be priced beyond a patient’s financial ability to pay a front-loaded copayment increases as the number of individuals impacted by the disease decreases -- a recent study examining the relationship between disease rarity and treatment cost found, not surprisingly, that the cost of orphan drugs in European markets is inversely proportional to disease prevalence.¹

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.

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. While we generally 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, our comments center on two areas of concern.

- OIG should develop a safe harbor, or redesign its existing ones, to enable financial assistance, both from charitable entities and directly from a manufacturer, so that patients can access the rare disease treatment they need; and

- 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 to payers.

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The increased availability of health insurance has relieved some of the financial burden of disease for individuals living with rare diseases and other chronic conditions that can represent catastrophic economic hardships for families. Unfortunately, insurers have increasingly imposed financial hurdles that disproportionately impact patients requiring costly medications. Out-of-pocket limits are an essential mechanism to ensure that families are not overwhelmed by high medical costs, but annual limits are invariably front-loaded. For the vast majority of Americans, a medical expense of $7,500 or more in a single month to access a covered treatment is an impenetrable barrier to access.

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. This but-for relationship, however, is not associated with the treatment decision in any way. Put simply, no incentive is needed, and none can influence the actual treatment decision. 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. Without a safe harbor, however, 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. It is 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 to directly 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.

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Patients with extremely rare conditions, and the providers treating them, face systemic challenges that too often creates extreme disadvantages for therapies administered during inpatient stays. Increased focus on the high costs associated with treating very rare diseases has led us to reconsider our belief that insufficient reimbursement concerns were limited to inpatient stays paid under a DRG system. 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. Private payers and the Centers for Medicare & Medicaid Services are considering mechanisms to account for the high-cost of these products while ensuring value for the healthcare system. We believe that all payers 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, such as those contemplated for CAR-T 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.

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.²

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 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 require that any cost savings to payers are also reflected in the patient’s cost-sharing responsibility.

Finally, while we believe that creative financial arrangements may be essential for continuing payer coverage of high-cost treatments, and see clear OIG guidance as a necessary component to enabling these arrangements, we remain concerned with the disproportionate impact any unintended consequences may have on individuals with extremely rare diseases. For example, broad-brush application of “value” as a general strategy to control or set prices for treatments, particularly for rare diseases, can inject concerns with a very real potential to chill innovation. This concern is grounded in evidence -- researchers have observed that price thresholds would

slow drug innovation by 23-32 percent with as much as a 60 percent reduction in Research and Development (R&D) early stage projects.\textsuperscript{3} \textsuperscript{4}

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.

\textit{Conclusion}

We appreciate the opportunity to respond to OIG’s request for input on safe harbors. 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.