



## **Please Support Creation of Safe Harbors that Recognize the Unique Challenges in the Rare Disease Community**

Drug and biological manufacturing stakeholders have growing concerns about whether industry funds donated to enable patient cost-sharing assistance will trigger anti-kickback investigations. The Office of the Inspector General (OIG) for the Department of Health and Human Services (HHS) has asked for input on the need for new safe harbors to separate inappropriate incentives from acceptable financial arrangements.

**Issue:** We ask that safe harbors and OIG enforcement activities consider our reality – when we have one treatment available to address our disease, our decision is clear. While we may not be able to afford treatment without financial assistance, there is no “but-for relationship” threatening our treatment decisions. Put simply, financial assistance when there is only one treatment choice cannot influence the actual decision about appropriate therapy.

While charitable entities represent a potential source of assistance for patients in need, many rare, and most very rare diseases, are without a dedicated charitable organization capable of administering a cost-sharing assistance program. The rarer the disease, the less likely it is that a manufacturer’s donations to a broader charitable entity will ever reach any patient their therapy is indicated to treat.

Without a safe harbor, patients with rare diseases can only receive treatment if they can afford to absorb their share of its cost.

**Solution:** 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.

**Background:** 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. OIG should develop a safe harbor, or redesign its existing ones to enable manufacturers to assist with cost-sharing burdens that would otherwise prevent us from getting the only treatment available to address our rare disease.

A properly-crafted safe harbor would help level the playing field for patients needing treatment for a very rare disease by permitting a manufacturer to directly offer assistance to patients if (a) the patient requires the medication for a labeled indication associated with a 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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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. Our core mission is to evolve healthcare delivery innovation with an eye toward spurring innovation, quality in care, and treatment options for all Americans.

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