



January 6, 2021

The Honorable Ami Bera
The Honorable Mike Kelly
The Honorable Roger Marshall
The Honorable Brad Wenstrup

The Honorable Tony Cardenas
The Honorable Ron Kind
The Honorable Markwayne Mullin

Health Care Innovation Caucus
United States House of Representatives
Washington, DC 20515

Dear Health Care Innovation Caucus Members,

Haystack Project appreciates the opportunity to provide input on the Health Care Innovation Caucus' efforts to modernize Stark and Anti-kickback statutes and Medicaid Best Price for those participating in Value Based partnerships. We agree that this exercise is critical for building on the late 2020 final rules to simplify and modernize these laws.

Haystack Project is a 501(c)(3) non-profit organization enabling some 70 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 rare and ultra-rare patients. 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.

We have discussed and commented previously on the issues raised in the CMS and OIG rulemaking on Stark and Anti-kick statutes and Medicaid Best Price. We provide the following comments to open the dialogue between Haystack Project and your offices as you build on last year's work. We provide comments on the three topics requested.

(1) Feedback on the finalized CMS and OIG rulemaking on Stark and Antikickback Laws

The OIG rulemaking primarily addressed value-based arrangements. These arrangements can present significant risk to care for patients with rare and ultra-rare conditions, including (and, possibly, particularly) those still within a potentially lengthy patient journey to diagnosis. When value-based enterprise (VBE) participants assume downside risk, there is a heightened risk for cherry-picking patients, discharging highly complex, rare, and/or costly patients, and stinting on the care patients with high medical needs receive. Therefore, it is critical that ALL VBE should:

- prioritize informed consent for patients with transparency on the VBE arrangements
- examine how provider risk assumption might impact care
- provide patients with detailed information on how to opt out of inclusion in VBE by withholding or withdrawing consent.

Furthermore, all such arrangements must protect the patient/provider decision making process by requiring that all VBEs operate in a manner that ensures non-interference with health care decisions. There is a clear gap in federal oversight on VBEs, particularly those associated with downside risk. OIG and CMS appear to believe that downside risk for providers is a safeguard in and of itself. It, however, only mitigates risk that providers would increase utilization. For rare and ultra-rare patients, the greater concern is that providers would stint on care.

Many rare and ultra-rare conditions are chronic and progressive. Treatment deficiencies may not impact outcomes or increase overall costs within the short timeframes ordinarily in place for VBEs. Downside risk could have a serious impact on provider willingness to recommend the necessary diagnostic tests and referrals to diagnose a rare or ultra-rare condition. Providers would be incentivized to address patient symptoms with lowest-cost interventions and disease progression may not be captured within the short-term VBE evaluation/remuneration period.

It is also critical that any efforts ensure patients receiving care within a VBE are not disadvantaged by capitated rates or other risk arrangements when a new treatment option becomes available. The safe harbors should require that VBEs implement “carve out” mechanisms to ensure that patients have access to new treatments.

(2) Suggested Policy and Legislative Text to Modernize Stark and Anti-Kickback Laws

OIG safe harbors must address the realities patients with extremely rare disorders face so that the assistance patients need to (i) access treatment or (ii) undergo precision diagnostics to determine that a treatment path is appropriate are not mischaracterized as a prohibited inducement.

The anti-kickback statute and/or its safe harbors should recognize that rare and ultra-rare diseases may have few, or even a single, treatment option. The significant disease burden and potentially poor prognosis these patients live with day-to-day is more than enough incentive to seek treatment and choose an FDA-approved therapy. Manufacturer assistance simply enables access to the treatment patients need; it does not “incentivize” choice of treatment.

Part D excludes treatments for ultra-rare and rare diseases, even if they are the standard of care, if the uses are off-label (as most existing treatments for very rare conditions are). This ends up foreclosing access for any patients unable to afford the total cost of these medications. Manufacturers cannot contribute for fear that they would be promoting off-label use. This requires a statutory fix to ensure that the definition of “covered Part D drug” reflects uses that are standard of care in rare and ultra-rare disorders.

(3) Range of policy and legislative options to improve Medicaid Best Price to facilitate value-based arrangements for treatments.

Value-based arrangements should not be used to deter access to treatments for rare and ultra-rare diseases by, for example, limiting access to those patients fitting within the contours of both the labeled indication(s) and outcomes-based pricing eligibility. These arrangements could, however, be very

helpful in expanding access to treatments in rare disorders for which manufacturers may not otherwise pursue labeled indications.

There should be transparency for patients obtaining access through value-based arrangements, and patient cost-sharing and/or obligations should not be increased due to value-based payment mechanisms.

Haystack also believes outcomes-based arrangements would have greater utility if there were mechanisms through which payers could distribute treatment costs of successful therapies across private/public payers. For example, a curative or disease modifying treatment for a progressive disease leading to disability would save funds for Medicare and/or Medicaid; should public payers bear some of cost if they primarily benefit from savings?

We strongly urge these changes be made as these challenges will persist for our community. They are a critical lifeline between our patients and the extremely rare disease experts that are few and far between for each condition.

Once again, we thank you for the opportunity to think critically about what is needed for our rare and ultra-rare communities. We look forward to discussing these issues in greater detail with your staff.

Sincerely,

A handwritten signature in black ink that reads "Jim Caro". The signature is fluid and cursive, with the first letters of the first and last names being capitalized and prominent.

Jim Caro
Chief Executive Officer
Haystack Project
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