VIA ELECTRONIC SUBMISSION

July 20, 2020

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS-2482-P
P.O. Box 8016
Baltimore, MD 21244-8016

Re: Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and Supporting Value-Based Purchasing (VBP) for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third-Party Liability (TPL) Requirements

Dear Administrator Verma,

Haystack Project and the Rare Cancer Policy Coalition (RCPC) (collectively, Haystack Project) appreciate the opportunity to submit comments to the Centers for Medicare & Medicaid Services’ (CMS’) proposed rule referenced above (the Proposed Rule).

Haystack Project is a non-profit organization enabling 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, and effective, accessible treatment options for all Americans.

The Rare Cancer Policy Coalition (RCPC) is a Haystack Project initiative that brings together rare cancer patient organizations to provide a platform for focusing specifically on systemic reimbursement barriers and emerging landscape changes that impact new product development and treatment access for rare cancer patients. It is the only coalition developed specifically to focus attention on reimbursement, access and value issues across the rare cancer community. Working within Haystack Project enables RCPC participants...
and rare and ultra-rare patient advocates to leverage synergies and common goals to optimize advocacy in disease states where unmet need is high and inadequacies in treatment and/or access can be catastrophic.

For our patient communities, the emergence of new, innovative therapies targeting specific disease mechanisms offer renewed hope for treatment options, and even a cure to the life-limiting and life-threatening conditions they face. This hope, however, is tempered by our concern that the rising cost of prescription drugs generally will impede patient access to novel, high-cost treatments addressing very rare disorders and rare cancers. We recognize that alternative health care financing structures could offer payers an important tool to improve access to high-cost, targeted treatments without burdening patients with unaffordable up-front out-of-pocket costs or compromising health care system sustainability.

Our comments reflect our commitment to individuals with rare and ultra-rare diseases, including rare cancers, and their families. We are, however, concerned that the inherent complexities within the proposed rule limit the ability of patients and patient organizations to offer meaningful input within the 30-day comment period. Haystack Project, therefore, urges CMS to extend the comment period to ensure that stakeholders are able to consider the Agency’s proposals, their impact on patients, and the safeguards required to ensure that incentives are aligned toward patient access and improved outcomes.

**Haystack Project supports CMS’ proposal to permit manufacturers to report separate best prices under value-based payment (VBP) arrangements and urges the Agency to consider additional mechanisms and flexibilities to support VBP adoption for treatments addressing extremely rare conditions.**

The potential that a VBP agreement could skew reporting on best price from quarter to quarter has presented a significant barrier to payer and manufacturer adoption of VBP agreements. CMS’ pragmatic approach of proposing to allow manufacturers to report separate best prices under VBP agreements without impacting best price calculations for sales outside of VBPs should help facilitate adoption of these arrangements.

Haystack Project expects that VBP arrangements would tend to proliferate, and even predominate, in the subset of novel new treatments targeted to extremely rare conditions and rare cancers. This would include the gene therapies that tend to be one-time treatments for rare diseases that offer improved long-term outcomes for patients and downstream savings for payers. VBP arrangements offer payers and patients an opportunity to mitigate the financial risks for payers while encouraging broader coverage and patient access to these high-value/high-cost treatments. These arrangements can also facilitate broader collection of real-world data on patient outcomes that can be particularly helpful when novel therapies are introduced with limited long-term data on clinical effectiveness and durability of response.
Haystack Project understands that CMS offered both the bundled sale concept and the multiple best price reporting option to accommodate broader adoption of VBPs. When a particular treatment addresses a very rare condition, however, it may be prescribed and administered to just a handful of patients each year. One or two failures to meet outcome-based measures within a quarter could, therefore, negate the benefits of separate reporting for sales related to a VBP arrangement. On a quarter-to-quarter basis, the VBP-related best price could vary from $0 to the full cost of the treatment. We strongly urge CMS to ensure that other methods are available to accommodate the unique factors associated with extremely rare disorders.

We also remain concerned that stakeholders generally, and the patient community in particular, have not been afforded an adequate opportunity to consider potential unintended consequences of more widespread adoption of VBPs. This makes it nearly impossible for our patient communities to offer meaningful insights on the safeguards and guardrails needed to ensure that the novel treatments emerging to address extremely rare conditions and rare cancers reach the patients who need them. Haystack strongly urges CMS to continue and expand its outreach and engagement efforts with the patient community to identify a set of VBP requirements that appropriately align incentives from a patient-centered perspective. Only those VBPs that are able to demonstrate that the arrangement would result in improved access and lower patient out-of-pocket costs should benefit from exclusion from best price calculations.

*Haystack Project urges CMS to require that evidence- or outcomes-based measures and the definition of “substantial” in a VBP arrangement be identified through patient engagement and meaningful input from patients potentially benefiting from the subject treatment.*

Haystack Project appreciates that CMS proposes to require that a VBP be an “agreement intended to align pricing and/or payments to an observed or expected therapeutic or clinical value in a population.” Within the context of a rare disorder, including a rare cancer, the concept of “value” is inherently disease-specific, and can even vary from patient to patient. We strongly urge CMS to incorporate a patient-centric perspective into the refinement of the Agency’s VBP definition in its Final Rule.

Use of evidence- or outcomes-based measures can only reflect “value” if there is a mandate for meaningful, substantive input from patients on factors and outcomes that are most important from the patient perspective. This means that VBP arrangements should be transparent to patients, and designed to reflect high-priority outcomes from the patient perspective.

*Haystack Project urges CMS to withdraw its proposal to require manufacturers to deduct the value of cost-sharing assistance from best price and AMP calculations.*

Patients with very rare disorders and rare cancers can be disproportionately impacted by high out-of-pocket costs, lengthy coverage denial appeals processes, and burdensome prior
authorization hurdles generally associated with treating complex and chronic conditions. Our patient communities, for the most part, do not have access to the generic substitutes that lower costs for patients with common conditions. Patients requiring branded medications to manage or treat their conditions often rely on copay coupons, discount cards, charitable assistance, and other assistance as their only means to afford their medications. More often than not, there is no opportunity to decide which, among many, treatment options to pursue, much less whether or not their treatment is cost-effective. The utilization management strategies payers employ to drive cost-effective treatment utilization, therefore, have no impact on deciding how to treat most rare disease patients. They function only to erect financial barriers to patient access to any treatment at all. Manufacturer assistance programs, including copayment assistance, for these patients is most appropriately viewed as enabling rare disease patients to realize the same value from their health insurance coverage as individuals with more common conditions achieve.

Haystack Project agrees with CMS’ stated intent of ensuring that cost-sharing assistance fully accrues to the patient’s benefit. We are, however, very concerned that CMS’ proposal would require manufacturers to guarantee that the full value of their assistance accrues to the patient’s benefit as it is something over which they have limited control. The proliferation of copay accumulators, for example, creates a situation where a manufacturer could fully intend that all value accrue to the patient and remain unaware that a portion of that value actually accrues to the payer. Given the potential for false reporting penalties and the lack of transparency between payers and manufacturers, we are concerned that manufacturers would simply stop offering this assistance to patients who truly need it.

Conclusion

Haystack Project appreciates CMS’ interest in devising and adopting policies to enable patient access to high-cost/high-value treatment options without compromising health system sustainability. Now more than ever, science and innovation are progressing at a rapid pace that offers individuals with serious, life-limiting conditions and their families real hope of seeing a treatment or cure in their lifetime. We are encouraged by CMS’ pragmatic approach to VBP arrangements, and urge the Agency to consider our request to extend the comment period on this important Proposed Rule so that the patient community and other stakeholders can more fully respond.

We look forward to a continuing dialogue to ensure that all patients are able to receive appropriate care, no matter how rare their disease or condition. If you have questions or need further information, please do not hesitate to contact Saira Sultan at 202-360-9985.