April 6, 2020

BY ELECTRONIC DELIVERY

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Ave., SW
Washington, D.C. 20201

Re: Medicare and Medicaid Programs; Contract Year 2021 and 2022 Policy and Technical. Changes to the Medicare Advantage Program, Medicare Prescription Drug Benefit Program, Medicaid Program, Medicare Cost Plan Program, and Programs of All-Inclusive Care for the Elderly
CMS-4190-P

Dear Administrator Verma:

Haystack Project is a 501(c)(3) 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 toward effective, accessible treatment options for all Americans. 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.

The Rare Cancer Policy Coalition (RCPC) is a Haystack Project initiative that brings together rare cancer patient organizations. RCPC gives participants 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 rare cancer coalition developed just to focus attention on reimbursement, access and value issues across the rare cancer community. Working within the 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 treatment inadequacies can be catastrophic.

Haystack Project and RCPC (collectively, Haystack) appreciate the opportunity to comment on the above-referenced Proposed Rule. We hope that a greater understanding of the unique issues affecting our lives and families will inform the Centers for Medicare & Medicaid Services' (CMS') final rule as well as its future policies and initiatives.

We believe the primary obstacle to CMS' effective implementation of policies that reduce costs while enhancing, or at least not compromising, care for individuals with rare diseases is the risk of unintended consequences to these populations. We have, therefore, outlined some of the challenges patients with rare disorders face. Our comments focus on two key points of critical importance to patients and caregivers impacted by rare and ultra-rare disorders and rare cancers:

- Haystack appreciates that CMS proposes a maximum cost sharing of 25-33% on specialty tier drugs, but remains concerned that exempting specialty tier products from tier exceptions to preferred, non-specialty cost-sharing levels will disproportionately disfavor complex patients with rare diseases and rare cancers; and
- Expanding the set of costs that Part D and Medicare Advantage plans (PDPs and MA plans, respectively) include within medical loss ratio (MLR) calculations could have unintended consequences that disproportionately burden access for patients with rare diseases and rare cancers.

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. Countless lives have been improved or saved by new therapies stimulated by the set of statutory incentives for orphan drugs. Although, millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure, there are many diseases for which treatments that are already here or in the pipeline cannot reach patients due to the realities of today's reimbursement structures.

- 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;
- Diagnosing a patient with a rare disorder is usually a multi-year process involving a series of primary care clinicians, specialists, and diagnostic testing regimens – extreme rarity of a disorder compounds the resources required for diagnosis;
- If a diagnosed condition has no FDA-approved option, treatment often involves off-label use of existing products, including therapies categorized as "specialty" products; 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 and whether patients can afford to absorb their cost sharing burden.

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preferred, non-specialty cost-sharing levels will disproportionately disfavor complex patients with rare diseases and rare cancers.

As CMS noted in its Proposed Rule, PDP specialty tier cost-sharing currently reaches levels as high as 50%, and can greatly impact a patient's ability to purchase treatments needed to manage serious medical conditions. Haystack applauds CMS' proposal to set a maximum cost-sharing for all drugs, including those on the specialty tier, at between 25% and 33%. CMS further proposes to:

- Allow PDPs to establish up to two specialty tiers;
- Permit PDPs to design and implement an exceptions process that exempts specialty tier drugs from tiering exceptions to non-specialty tiers;
- Require PDPs implementing two specialty tiers to designate one tier as "preferred" with lower cost-sharing; and
- Ensure that PDP tiering exception processes permit exception requests from the higher-cost specialty tier to the lower-cost specialty tier.

Haystack understands that CMS anticipates that an additional specialty tier could improve the ability for PDPs to negotiate more favorable pricing in exchange for "preferred" specialty tier placement. We are concerned that unless CMS provides guidance to ensure that the cost-sharing advantage for any preferred specialty tier is sufficiently robust, it will be of minimal benefit in spurring negotiations toward lower prices.

Our primary concern, however, is that patients with rare diseases and rare cancers disproportionately rely on higher-cost products relegated to PDP specialty tier cost-sharing. We agree with CMS' longstanding concern, reiterated in the Proposed Rule, that permitting PDPs to exempt specialty tiers from the exceptions process

> could potentially be discriminatory for Part D enrollees with certain diseases only treated by specialty tier-eligible drugs, and thus in conflict with the statutory directive under section 1860D-11(e)(2)(D) of the Act that CMS disapprove any "design of the plan and its benefits (including any formulary and tiered formulary structure) that are likely to substantially discourage enrollment by certain part D eligible individuals under the plan.

CMS' discussion of the impact on premiums and overall PDP cost-sharing related to exceptions from specialty to non-specialty tier cost-sharing highlights the fact that exempting these products from tier exceptions is discriminatory. CMS noted that if patients requiring specialty drugs are treated as all other PDP enrollees and granted preferred cost-sharing upon a showing that drugs for treatment of their condition that are on lower tiers are ineffective or dangerous, premiums and cost-sharing would increase for all enrollees. The path CMS proposes is to allow plan design to ensure that the burden of higher-cost treatments falls on the patients needing those products by foreclosing their ability to access treatments at non-specialty cost-sharing regardless of their ability to achieve a medical benefit from non-specialty products. Haystack is concerned that this structure is inherently discriminatory.

We urge CMS to finalize a policy that levels the playing field for patients that have no therapeutic options outside specialty tier products. Formulary structures with differential cost-sharing should encourage appropriate patient use of products that provide value with respect to outcomes and costs, rather than to discourage enrollment among patients without low-cost treatment options.

Expanding the set of costs that Part D and Medicare Advantage plans (PDPs and MA plans, respectively) include within medical loss ratio (MLR) calculations could have unintended consequences that disproportionately burden access for patients with rare diseases and rare cancers.

Haystack is concerned that the proposed expansion of cost categories that can be included within a PDP or MA plan's medical loss ratio (MLR) will adversely impact the value of these plans to all but the healthiest patients. CMS' longstanding policy of requiring that all costs identified within the MLR be direct claims paid to providers for covered services furnished to enrollees was implemented to ensure that issuers and plans were incentivized to minimize administrative costs and provide a robust benefit to Medicare beneficiaries.

We are particularly concerned that CMS' proposal could be interpreted as sufficiently broad to permit MA plans and PDPs to report costs associated with pharmacy benefit manager (PBM) services due to the nexus between those services and patient access to covered drugs. The use of PBM services to implement a plan's pharmacy benefit arose based on perceived "value-add" with these entities based on expertise and negotiation power. Plans rely on PBMs to reduce costs associated with providing covered drugs. PBM prior authorization and step therapy requirements, as well as formulary structures, are, and should be, designed to focus utilization management tools on therapies and disease states where avoidable costs exist and are sufficient to exceed the administrative costs implementing those tools. Utilization management tools are associated with "cost" -- PBMs charge plans for implementing them, providers encounter costs associated with the inherent paperwork burden, and patients can face delayed access and higher out-of-pocket costs.

Haystack strongly urges CMS to clarify that all utilization management strategies and. tools implemented by plans, PBMs, or other entities remain outside the scope of MLR cost calculations. If utilization management tool costs are untethered from administrative costs that a plan must absorb, it will disrupt the fragile balance between cost-effective checks and balances and patient/provider burden. Haystack has previously noted that increased use of step therapy and other utilization management tools, both within the "protected classes" under Part D and in the Medicare Advantage program creates a substantial access burden for individuals with extremely rare conditions and their treating clinicians. Absent the requested CMS clarification, we expect that these access burdens and their disproportionate impact on the patients and caregivers within Haystack's member organizations will accelerate. Patients with extremely rare disorders may not have access to a specialist with experience in treating their specific condition, leaving their care to a set of providers in various specialties that address specific disease symptoms. It is, therefore, relatively easy for providers to avoid the burden of battling through formulary exceptions and appeals by simply referring the patient to a different specialty.

Conclusion

We look forward to working with you to ensure that innovations toward treating and curing ultra-rare disorders reach the patients who need them. We urge the Administration to devise any necessary protections to ensure that efforts toward reducing the costs patients with commonly-encountered conditions pay for MA plans and PDPs does not impede or complicate treatment access for individuals with rare conditions.

If you have any questions or need additional information, please contact Saira Sultan at 202-360-9985.

Respectfully submitted,