



January 27, 2022

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Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 212441

RE: CMS-9911-P: Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2023

Haystack Project appreciates the opportunity to comment on the above-referenced Proposed Rule (the NBPP). The health insurance marketplaces created under the Affordable Care Act are critical to achieving the goal of equitable, affordable access to quality health care for all Americans.

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate efforts that address systemic value and access barriers. Our core mission is to evolve health care payment and delivery systems to make innovation and quality treatments accessible to all Americans living with or caring for someone with a rare or ultra-rare condition. We strive to amplify the patient and caregiver voice in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic.

Haystack Project appreciates the Administration's thoughtful approach to balancing the goal of facilitating and maintaining a robust, competitive insurance market with the over-arching objective of ensuring that all patients have access to the care they need at a cost they can afford. We similarly applaud CMS for its proactive and intentional focus on protecting health care access for individuals with high-cost conditions. Our comments reflect our general support for the policy refinements outlined in the NBPP and offer our insights and recommendations on ensuring that the benefits to patients within the ACA marketplace confer equally to individuals with rare and ultra-rare diseases and rare cancers.

Haystack supports CMS' increased clarity and focus on identifying and addressing discriminatory plan design

The nondiscrimination provisions applicable to marketplace plans offer critical protections to our patient communities relying on ACA plans for access to care. Unfortunately, the lack of clarity on the types of mechanisms constituting a discriminatory benefit design and

uncertainties on CMS enforcement have limited the impact of the provision on the real world experience of rare and ultra-rare disease patients seeking access to the targeted therapies or off-label treatment regimens and specialist care required to treat their condition. We support CMS' efforts to increase clarity for issuers and other stakeholders and believe that the steps proposed in the NBPP can have a meaningful impact on affordable access to treatment.

In particular, Haystack appreciates that CMS recognizes the interplay between clinical evidence and health care costs and clearly articulates the general requirement that plan benefit limitations and coverage requirements be grounded in clinical evidence rather than driven by economic considerations. The additional layer of clarity on the types of evidence plans and issuers must consider is of particular importance to **individuals** with rare and ultra-rare conditions and rare cancers. We strongly agree that a non-discriminatory plan design – **and its implementation mechanisms** - must be “clinically based, that incorporates evidence-based guidelines into coverage and programmatic decisions and relies on current and relevant peer-reviewed medical journal article(s), practice guidelines, recommendations from reputable governing bodies, or similar sources.”¹

We strongly support the proposed evidence sources outlined in the NBPP and urge CMS to (1) include opinion of recognized, disease-specific experts as an evidence source for therapies used in treating or managing a rare condition, including rare cancers; and (2) develop a mechanism through which patients and clinicians can report on and resolve real world experiences that demonstrate a **discriminatory impact** of plan design that may not be apparent within the resources available for CMS review. Haystack and its members recognize that the impacts of general coverage inclusions and exclusions on rare disease patients are most often related to implementation rather than design and not readily ascertainable in plan documentation. Examples of a discriminatory impact include:

- **Step therapy protocols.** Step therapy is a well-accepted, frequently encountered utilization management strategy. Payers require patients to “step” through older, less costly treatments before allowing access to newer, often more innovative or targeted, and inevitably more expensive options. This may not be a problem in disease states for which several treatments are available, including generic options. However, individuals with extremely low prevalence conditions rarely have an FDA-approved treatment available, and any off-label uses of existing drugs are seldom found in the sources listed in the various compendia and other sources plans commonly rely on to determine coverage. This means that individuals with very rare conditions do not have the same protection from inappropriate step protocols that individuals with common conditions have, and the steps designed for more common diseases are frequently inappropriate within the context of off-label use in rare conditions. This is particularly true when step therapy protocols require failure on a treatment that is not useful in that disease and/or that may be harmful to the patient. Haystack does not expect that plans would maintain

¹ [Federal Register :: Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2023](#)

up-to-date clinical information on every treatment for every rare disease. We do, however, urge CMS to consider whether plans maintain an expedited review process and permit emergency doses for rare disease patients in determining whether plan designs are nondiscriminatory.

- **NDC “blocks” and “lock-outs”** – It is relatively common for plans to systematically block coverage of newly approved drugs for 6-12 months or longer under the rationale that formulary inclusion requires review of the plan’s pharmacy and therapeutics committee. These blocks apply to patients newly-seeking treatment as well as to those who have benefited from the treatment through clinical trial participation, open label extensions, and expanded access programs. Haystack recognizes that the mechanism has utility and may be a reasonable approach in more common conditions. Access delays for new drugs offering incremental benefits in efficacy, safety, or convenience over existing treatments may be frustrating, but they are generally not harmful to the patient. In rare conditions and rare cancers, however, declining access to what may be their only on-label treatment should be viewed as a failure to deliver essential health benefits. It is an example of the types of unintended consequences rare disease patients face throughout their health care journey and illustrates how applying policies with seeming equality drives real world inequities that can harm patients. An expedited formulary review process applicable to newly-approved treatments for rare diseases without on-label treatment options would mitigate the disparate impact that blocks and lock-outs exact on patients.

Haystack recommends that CMS identify a mechanism through which patients can report real-world experiences of discriminatory plan design and/or discriminatory impact associated with coverage and benefit implementation. This would be helpful to patients, potentially reduce burden to clinicians, and enable CMS to identify additional examples of presumptively discriminatory plan design and implementation mechanisms.

Haystack commends CMS for addressing adverse tier structures for prescription drugs in a manner that protects patients with high-cost conditions.

The patient communities represented by Haystack member organizations are extremely diverse with respect to disease onset, progression, long-term prognosis, and impact on caregivers. High out-of-pocket costs for prescription drugs are, however, a near-universal experience for rare and ultra-rare disease treatments. Haystack and its member organizations appreciate that CMS recognizes the inherent unfairness of what feels to families like an insurer-imposed financial penalty on families struggling with a serious rare or ultra-rare disease or a rare cancer. We agree that formulary tier structures should be grounded in clinical evidence rather than driven by cost considerations, and that issuers should expect to cover and enable access to treatments that are prescribed by the patient’s provider and recommended by disease-specific experts or, if available, consistent with clinical practice guidelines.

Haystack fully supports CMS' proposed presumption of discriminatory plan design when formulary tiering structures place most or all treatments for specific, high-cost conditions on tiers with high out-of-pocket costs. In fact, consistent enforcement of this policy in connection with rare disease treatments would represent a substantial step toward reducing the significant financial burden on rare disease patients and their families.

Haystack supports CMS' proposal to require issuers to provide potential enrollees with information on availability of telehealth services and to monitor plan use of telehealth with benefit design.

Haystack agrees that individuals shopping for a marketplace plan should have all information that may be relevant to their decision, including information related to availability and use of telehealth services. We also fully support CMS' proposal to view telemedicine visits as an adjunct to, rather than a replacement for face-to-face provider access in determining network adequacy.

We strongly support telemedicine as an option that patients can choose in consultation with their clinicians. For rare and ultra-rare disease patients, telehealth services have proven to be a valuable adjunct to in-person visits throughout the COVID-19 pandemic. It has enabled broader access to a continuity of coordinated care that includes disease-specific expertise from local specialists as well as those outside the patient's geographic area without the burden of travel. For many patients and their families, telemedicine has offered increased convenience and incrementally decreased the burden families face in caring for an individual with a serious rare condition.

In the early months of the pandemic, several Haystack member organizations reached out to patients with a survey assessing the patient experience with telehealth services during the Public Health Emergency (PHE). The majority of patients responding to the survey were able to access telehealth with relative ease and felt that the telehealth service flexibilities helped protect them from COVID-19 exposure. For individuals with rare conditions, the increased ease in accessing specialist care underscores the need to continue many of these flexibilities permanently. For example, one patient noted the care they have received through telehealth during the PHE:

Medication changes, local tests were ordered, met with neurosurgeon to determine surgery is needed. We live in Alaska and frequently have to fly to Seattle for care. We have been able to visit with specialists via telehealth and it's saved us considerable money and provided us with additional opportunities to see experts regarding care.

Patients responding to the survey appeared to view telehealth as vital through the PHE, and useful as an adjunct to in-person care thereafter, and many expressed concerns that the ability to receive remote care from out-of-state providers could be restricted once again after the PHE resolves. Individuals relying on marketplace plans frequently cite concerns and frustrations

related to limited networks and inability to seek consultations from disease-specific experts. This is particularly true in ultra-rare diseases with experts primarily located in a limited set of “Centers of Excellence.” Use of telemedicine to enable patient access to consultation services and treatment plan reviews from disease-specific experts should be recognized as a plan enhancement for network adequacy purposes.

Haystack has also heard from patients and caregivers regarding the barriers individuals with hearing and/or visual impairments face in seeking care. Usher Syndrome, for example, is a very rare (approximately 25,000 US patients) inherited disease causing combined hearing loss and vision loss from retinitis pigmentosa. For these patients, it is essential that remote care includes access to an ASL interpreter if they have sufficient remaining vision, or a tactile sign interpreter if they do not. We urge CMS to clarify that plan information on telemedicine availability should include details on accessibility for hearing and vision impaired patients.

We fully agree with CMS that telemedicine should not be implemented in a manner that reduces access to face-to-face visits or imposes differential cost-sharing burdens for patients choosing in-person care over telemedicine. Although most patients enjoy the convenience of telemedicine, it does not work for everyone and, depending on how it is implemented, telemedicine has potential to either reduce *or* perpetuate health disparities.

Haystack supports CMS’ proposed elimination of the option for states to permit issuer substitution of benefits between EHB categories

Haystack strongly supports CMS’ decision to eliminate the ability for states to permit plan designs that substitute benefits between EHB categories. We commend CMS for its careful analysis and its decision to prioritize the coverage needs of patients with high-cost conditions over any future interest states may have in exercising flexibilities that alter the set of benefits conveyed by marketplace coverage.

Network Adequacy Standards

Haystack strongly supports CMS’ NBPP proposals to strengthen and clarify network adequacy standards. Network adequacy has presented a critical equity issue for patients with rare and ultra-rare diseases and rare cancers that are magnified in communities of color and other underserved populations. Specifically, we appreciate that CMS intends to:

- Include appointment wait time standards in evaluating network adequacy;
- Expand the list of provider specialties subject to time and distance standards; and
- Require that providers included in network adequacy assessments are contracted within the network tier with the lowest cost-sharing.

The PHE underscored the need for, and temporarily provided, Haystack’s patient community with increased access to specialists across state lines and out of network. It eased the burden on physicians having to justify to payers the need to see patients outside their local area, as

well as the significant burden patients face when it is necessary to travel for in-person specialist care. The rare and ultra-rare community served by ACA plans needed these flexibilities long before Covid and will need them long after the PHE is over. Haystack encourages CMS to continue its efforts to strengthen and clarify network adequacy standards. We would specifically call out the need to consider including standards that ensure that a network is adequate to serve the needs of all of its enrollees, including those with very rare conditions who have a limited set of clinicians with sufficient expertise to treat or manage their condition. One pragmatic approach would be to encourage plans to adopt a mechanism similar to what is applied to Medicaid patients seeking out-of-state coverage. Upon a showing that an individual needs care that cannot be sufficiently delivered within the network, the plan would authorize out-of-network care with in-network cost-sharing.

We urge CMS to reconsider its policy permitting issuer implementation of copayment accumulator programs and other mechanisms that impact patient out-of-pocket burdens.

Most of the identified rare diseases do not have any FDA-approved treatments available to treat, manage, or cure the condition. The ACA marketplace has substantially reduced the number of uninsured rare disease patients who, while fortunate to have an available FDA-approved treatment, were unable to access treatment due to the high cost of developing and commercializing therapies for low-prevalence conditions. Although individuals with relatively common diseases can generally count on the emergence of generic substitutes for higher-cost branded drugs to reduce their out-of-pocket costs, orphan drugs addressing very rare conditions may remain without generic competition and replaced only if a new branded therapy is advanced to improve outcomes. For these patients, copayment accumulator programs function solely as a revenue stream for issuers with patients remaining burdened with disproportionately high out-of-pocket costs. We urge CMS to re-examine this policy so that funds intended to reduce financial impediments to needed medical care have an actual impact on the patient's out-of-pocket costs over the plan year.

Haystack response to CMS' solicitation of comments on health equity

The COVID-19 pandemic cast a spotlight on the profound impact that race and racial inequities have on health outcomes and the additive detriments associated with social determinants of health. Our member organizations represent a diverse set of rare and ultra-rare disorders, some of which have known disparate impacts on communities of color. Unfortunately, Haystack and its member organizations face the same hurdles in identifying and quantifying these impacts as CMS has in addressing them. We do know that unless registry participation, outreach, and engagement is sufficiently representative of the total patient population, advocacy organizations remain uninformed of disparate disease burdens, treatment response, and access to care, and cannot advocate on behalf of *all* patients impacted by a rare condition. Haystack believes that CMS should take a partnership approach to addressing health disparities and inequities and that patient advocacy organizations can play a strong role in narrowing care gaps due to social determinants of health and systemic perpetuation of racial inequities.

Our member advocacy organizations have asked for support in illuminating and addressing the needs of non-white patients in their communities, and Haystack is responding with its Health

Equity in Access to Treatments initiative. The goal of this program is to develop a “best practices” guide to empower our patient advocacy organizations to (i) evaluate their organization’s inclusiveness and representativeness, (ii) address care gaps, and (iii) incorporate the lived experience of all patients into their advocacy. Ultimately, we hope that each of our 70+ patient organizations will leverage their learnings to proactively drive initiatives toward reducing inequities related to systemic racism and social determinants of health that drive disparate access to treatment and health outcomes.

We appreciate CMS’ proposals to improve collection and extraction of data relevant to social determinants of health and underserved populations, including the proposal to collect and extract new data elements including zip code, race, ethnicity, individual coverage health reimbursement arrangement (ICHRA) indicator, and a subsidy indicator in states where HHS is operating the risk adjustment program. The proposed collection of “z codes” could enable increased granularity and improve CMS’ ability to direct equity initiatives to areas with highest need for the interventions. In implementing this proposal, CMS should focus first on educating providers on the z codes and how to properly report them.

Haystack’s outreach efforts have revealed several areas of concern to patients that, if adequately addressed, could close care gaps and reduce health inequities.

- Patients face uncertainties in accessing off-label treatments used within the standard-of-care due to limited inclusion of rare disease considerations in the compendia that payers generally rely on. The rarer the disease, the less likely it is that medically accepted treatments will be published in compendia. Patient access programs are not generally available since a manufacturer offering free or discounted drug in this patient population would face off-label promotion scrutiny and potential liability. This leaves patients with few options unless they receive care from a provider willing to navigate the reconsiderations and appeals processes.
- Receiving care in the home through telemedicine is often the best option for low-income and rural patients and their families. SDOH can, however impede availability of this option due to lack of broadband internet capabilities and financial impediments to maintaining reliable housing and utilities.
- We suspect that the unduly lengthy journey from emergence of symptoms to diagnosis is even longer for patients in communities of color and other underserved populations. Unfortunately, our member organizations do not have the data to quantify those inequities or identify clear causative factors.
- Patients and caregivers have faced significant challenges in accessing care throughout the pandemic and have often taken on more demanding and active roles as the hands and eyes of clinicians. With guidance, tools and support, families can take on proactive and impactful roles and responsibilities that optimize patient care.

- Haystack believes that technology can be leveraged to reduce the diagnostic journey for rare and ultra-rare disease patients as well as to ensure that all patients have access to the expertise needed to effectively treat or manage their condition.
- In rare disease patients, subtle changes in disease symptoms and/or progression could have profound impacts on longer-term outcomes. Encouraging plans to deploy wearables, monitors, and layperson friendly medical equipment would enhance remote monitoring capabilities and provide key patient information that may not be ascertained from periodic in-person visits,

Conclusion

Once again, Haystack and its member organizations appreciate the opportunity to submit comments as CMS finalizes the NBPP for calendar year 2023. We look forward to working with the Agency as it continues to refine ACA marketplace policies.

If you have any questions or would like to discuss the issues raised in our comments, please contact our policy consultant, Kay Scanlan, at (410) 504-2324.

Very truly yours,



Deanna Darlington
CEO and Ex Officio Board Member

