January 30, 2023

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

RE: CMS–9899–P
Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2024

Dear Administrator Brooks-LaSure:

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

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 unique to rare diseases or particularly pronounced in extremely rare diseases. Haystack Project is committed to educating policymakers and other stakeholders about the unique circumstances of extremely rare conditions with respect to product development, commercialization, and fair access to care. 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 living with or caring for someone with a rare or ultra-rare condition.

Our rare disease communities struggle to navigate health system challenges in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic. Our comments offer our insights and recommendations to enable CMS to build upon its efforts to ensure that the benefits to patients within the ACA marketplace confer equally to individuals

regardless of their race, financial resources, health care needs, or the rarity of their health condition(s).

Background

Health care for individuals with rare and ultra-rare conditions can be relatively high-cost and often requires highly specialized clinicians to deliver quality care. Approximately 7,000 rare diseases have been identified to date, 90-95% of which have no FDA approved treatment. Cumulatively, rare diseases affect approximately 30,000,000 or 1 in 10 individuals in the U. Health care for individuals with rare and ultra-rare conditions can be relatively high-cost and often requires highly specialized clinicians to deliver quality care. Rare disease patients face substantial challenges from symptom emergence through treatment or management of their condition. These patients:

- See an average of 4.2 primary care physicians and 4.8 specialists before receiving an accurate diagnosis.
- Make an average of 2.4 out-of-state trips related to their diagnosis.
- Visit an emergency room an average of 3.7 times and - are hospitalized an average of 1.7 times for reasons related to their rare disease prior to diagnosis.
- Face a heightened risk of misdiagnosis.
- Have a very limited set of clinicians with disease-specific expertise, making it difficult for many patients to identify an experienced provider within their network, or even their state.
- Disproportionately rely on off-label use of treatments indicated for more common conditions to address disease symptoms and/or progression. Due to disease rarity, these off-label uses are seldom included within compendia.

In 2021, the Government Accountability Office (GAO) complied a report to Congress entitled "RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial" in collaboration with EveryLife Foundation for Rare Diseases and the National Organization for Rare Disorders. The report assessed the challenges rare disease patients face accessing diagnostic and treatment services as well as the personal and economic costs associated with treatment delays. Among its many findings, the GAO found that rare disease patients are unable to access specialists due to geography or failure to receive a referral for follow-up care at initial symptoms, and often progress to more severe disease states by the time they receive an accurate diagnosis. Forty-one percent of rare disease patients also receive at least one misdiagnosis, and many are treated for a condition they did not have, and approximately 7 percent of rare disease patients reported that they were given a false psychological/psychiatric diagnosis that further impeded and delayed their treatment.[1]

[1] GAO Report. GAO-22-104235, RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial

Individually, these access challenges can present inconveniences, frustration, and delays in receiving care. Cumulatively, they can present an overwhelming burden for patients and their families.

Haystack Project generally supports CMS' proposals to expand access to coverage and streamline enrollment/re-enrollment processes.

Haystack Project appreciates that the Administration's ACA-related efforts have emphasized refinements that reduce health inequities and protect health care access for individuals with chronic conditions and other high-cost health care needs. We support CMS' policy refinements to ensure that individuals have the information they need to enroll in a plan that meets their health care needs, including:

- Repealing the regulatory provisions prohibiting assisters (Navigator, Non-Navigator Assistance Personnel, and Certified Application Counselors) from going door-to-door or using other unsolicited means of direct contact to provide enrollment assistance to consumers Haystack Project agrees that repealing the prohibition would enable assisters to reach a broader consumer base for enrollment assistance.
- Requiring agents to create and provide CMS with documentation that a consumer/representative has reviewed and confirmed the accuracy of eligibility application information before submission.
- Requiring that agents document and maintain (for at least 10 years) a record of consumer consent that includes name (consumer and agent), date of consent, and a description of the scope, purpose, and duration of consent.
- Permitting exchanges to find an applicant ineligible for advance premium tax credit (APTC) ***only if*** the applicant has a "failure to file and reconcile" (FTR) delinquent status for the two consecutive years for which tax data would be used to verify household income and family size.
- Modifying the Verification Process Related to Eligibility for Insurance Affordability Programs
    - Require Exchanges to accept enrollee's attestation of projected household income where IRS tax return data is unavailable.
    - Require that income inconsistencies receive an additional, automatic 60-day extension to the deadline for providing documentation to verify household income.
- Permitting Exchanges to implement a "new special rule" that consumers eligible for a special enrollment period due to loss of Medicaid or CHIP coverage will have up to 90 days after coverage loss to enroll in an Exchange plan.

Haystack Project continues to support CMS' elimination of the option for states to permit issuer substitution of benefits between EHB categories.

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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.

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Haystack Project is disappointed that CMS has not addressed areas of particular concern to individuals with very rare diseases.

Self-insured and large employer flexibilities to deem specific prescription drugs as "nonessential health benefits."

In addition to the difficulties rare disease patients face in receiving a diagnosis, identifying an in-network specialist with disease-specific expertise, and identifying a treatment plan to manage disease symptoms and progression, individuals insured through employer-sponsored coverage are increasingly confronted with noncoverage when an FDA-approved therapy becomes available. Self-insured and large employer plans have leveraged benefit flexibilities (e.g., determining that specific prescription drugs are "nonessential" health benefits) to deny patient access to what may be the only therapeutic option to reduce disease burden and/or slow disease progression. The unfairness of declaring that what may be the only treatment available to address a patient's life-threatening or life-limiting condition is "nonessential" is compounded by the fact that the significant costs incurred to pay out-of-pocket do not count toward the plan's deductible or out-of-pocket maximum. This can be catastrophic for families impacted by a rare condition with a treatment that is deemed to be a non-essential health benefit. The policy likely widens health disparities between patients with sufficient financial resources and families with limited means. We urge CMS to revisit this policy and either revoke it or otherwise ensure that impacted patients have access to the treatment they need at a cost they can afford.

Patients with rare diseases and chronic conditions remain vulnerable to "discriminatory" plan designs implemented in the form of utilization management strategies and formulary design.

Haystack Project supported CMS' efforts to improve enforcement of the ACA prohibitions on discriminatory plan benefit design. In particular, Haystack appreciated that CMS articulated the general requirement that plan benefit limitations and coverage requirements be grounded in clinical evidence rather than driven by economic considerations. We strongly agree that a non-discriminatory plan design – **and its implementation mechanisms** - must be grounded in scientific evidence, including specialty society and disease-specific expert recommendations.

As we noted in our comments to the 2023 proposed rule, extremely rare diseases and their treatment regimens are not included in the compendia that many plans rely upon exclusively to determine coverage. Similarly, 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. We again 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*** or plan design that may not be apparent within the resources available for CMS review. Examples of a discriminatory impact that disproportionately impedes access to care for individuals with rare diseases 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 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.

Once again, Haystack Project urges CMS to 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 Project urges CMS to implement additional policy refinements to reduce health inequities.

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.

Haystack Project 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.

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. Social determinants of health 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 2024. 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, M Kay Scanlan, JD at (410) 504-2324.

Very truly yours,

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