



Submitted electronically

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Chiquita Brooks-LaSure, Administrator
Centers for Medicare & Medicaid Services Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 21244

CMS-1770-P -- Medicare and Medicaid Programs; CY 2023 Payment Policies Under the Physician Fee Schedule and Other Changes to Part B Payment Policies; Medicare Shared Savings Program Requirements; Medicare and Medicaid Provider Enrollment Policies, Including for Skilled Nursing Facilities; Conditions of Payment for Suppliers of Durable Medicaid Equipment, Prosthetics, Orthotics, and Supplies (DMEPOS); and Implementing Requirements for Manufacturers of Certain Single-Dose Container or Single-Use Package Drugs To Provide Refunds With Respect to Discarded Amounts

Dear Administrator Brooks-LaSure:

Haystack Project appreciates the opportunity to submit comments to the Centers for Medicare & Medicaid Services' (CMS') above-referenced proposed rule updating and refining the physician fee schedule and other Part B payment policies (the Proposed Rule).

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease advocacy organizations to highlight and address systemic access barriers to the therapies they desperately need. 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. Our core mission is to evolve health care payment and delivery systems, spurring innovation and quality in care toward effective, accessible treatment options for Americans living with rare or ultra-rare conditions. 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.

We appreciate CMS' outreach efforts and commitment to identifying and resolving inequities in access to quality care, and moving the Medicare program toward high-value, patient-centered care. Our comments provide a brief summary of the challenges patients with rare and ultra-rare diseases face, and focus on ensuring that efforts to refine Medicare policies and payment mechanisms result in improved quality, efficiency, and access for all beneficiaries regardless of the rarity of their medical condition(s).

Background

Approximately 7,000 rare diseases have been identified to date, 90-95% of which have no FDA-approved treatment.

- 80% of rare diseases are genetic in origin, and present throughout a person’s life, even if symptoms are not immediately apparent
- Approximately 50% of the people affected by rare diseases are children
- 30% of children with a rare disease will not live to see their 5th birthday
- Approximately half of identified rare diseases do not have a disease-specific advocacy network or organization supporting research and development.

The preference for focusing on “common” conditions permeates our health system from provider education underscoring the “common things are common” approach to diagnoses through the population-level priorities that drive health policy, including Medicare’s Quality Payment Program and Shared Savings Program. While this approach may appear pragmatic from a utilitarian perspective, it drives unduly-long diagnostic journeys for rare disease patients. In addition, systemic reimbursement carrots and sticks frequently exact unintended burdens on the health and lives of our patient communities from symptom onset through diagnosis and treatment access.

While each rare disease, by definition, impacts a patient population of under 200,000 (the primarily ultra-rare conditions within the Haystack community impact 20,000 or fewer, and often even 2,000 or fewer) rare diseases cumulatively affect approximately 30,000,000 or 1 in 10 individuals in the U.S. A 2021 Report to Congress from the Government Accountability Office (GAO) entitled “RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial” was compiled in collaboration with EveryLife Foundation for Rare Diseases and the National Organization for Rare Disorders to assess the challenges rare disease patients face accessing diagnostic and treatment services as well as the personal and economic costs associated with treatment delays. The GAO Report identified multiple factors tending to delay rare disease diagnoses, including:

Diagnostic delays and failures place patients at high risk for compromised health outcomes, including disease progression, exposure to inappropriate interventions, emergence of comorbid conditions, and even death. They are also costly. Although approximately 70% of rare diseases are genetic, and the costs for genomic sequencing has dropped from \$95,263,072 in 2001 to under \$1,000, the diagnostic journey has remained long and complex. According to an economic study which included a survey of 1360 patients with 379 rare diseases^{1 2} cited to in the GAO Report and administered by EveryLife Foundation and Lewin Group, patients:

- saw an average of 4.2 primary care physicians and 4.8 specialists for their rare disease diagnosis
- made an average of 2.4 out-of-state trips related to their diagnosis
- visited an emergency room an average of 3.7 times and
- were hospitalized an average of 1.7 times for reasons related to their rare disease, and prior to diagnosis.³

The diagnostic and treatment access challenges common to rare disease patients generally can be an overwhelming burden for people of color and other underserved populations, including rural communities. Communities of color face significant disparities in symptom severity, disease progression

¹ <https://everylifefoundation.org/burden-study/>

² <https://ojrd.biomedcentral.com/articles/10.1186/s13023-022-02299-5>

³ EveryLife Foundation and the Lewin Group, “National Economic Burden of Rare Disease.” p 19.

and mortality for rare diseases such as systemic lupus erythematosus and myasthenia gravis even though these conditions tend to occur across populations. Other rare diseases such as sickle cell anemia, thalassemia, and sarcoidosis disproportionately impact people of color. The growing number of beneficiaries with sickle cell disease (SCD) are primarily young, medically complex, and likely impacted by social determinants of health (SDOH):

- 75.5% utilized the emergency department, and 59.3% had an inpatient stay
- Hospital utilization was higher for individuals aged 18-45
- Common comorbidities in Medicare FFS patients with SCD include:
 - o hypertension (65.8%)
 - o fibromyalgia (64.9%)
 - o depression (51.3%)
 - o chronic kidney disease (47.0%).
- over 70% of Medicare FFS beneficiaries with SCD were dual-eligible
- over 80% are under 65 years of age.⁴

Medicare’s SCD patients’ experience within the opioid use epidemic illustrates the inequities confronting rare disease patients within communities of color as well as the high potential for unintended harms for rare disease patients when new population-level policies and/or health system refinements are implemented. CMS’ policy to curb the opioid abuse crisis was firmly grounded in public policy imperatives and proactively excluded cancer patients. SCD patients experienced access hurdles and denials of adequate pain management treatment until CMS recognized that “[t]he complex nature of SCD pain management may be exacerbated by ongoing efforts to address the opioid epidemic” and determined to exclude SCD patients from efforts to restrict opioid access.⁵ Proactive consideration of rare disease patients generally, with an eye toward efficient diagnosis and access to treatment, is a necessary step toward ensuring equity for Medicare’s rare disease patients.

This example illustrates the fact that even the most well-meaning policies can have unintended consequences for rare disease patients. Patients with other rare diseases, especially impacting very small numbers of patients like porphyria also manifest with acute episodes of pain requiring opioids and yet remain without reliable access to the pain management care they need. The rarer the condition, the more likely policymakers will not see the unintended consequences of their otherwise well-reasoned policies.

Haystack applauds CMS’ proposal to retain beneficiary coverage for each COVID-19 monoclonal antibody until termination of the relevant Emergency Use Authorization (EUA).

Many rare diseases and rare cancers are associated with severe immune compromise. For these patients, the monoclonal antibodies for which FDA granted EUAs to treat or prevent COVID-19 have been crucial in reducing the incidence of severe illness and even death. CMS’ decision to establish coverage under the Medicare Part B COVID-19 vaccine benefit, as opposed to standard Part benefits, has ensured equitable access to these life-saving treatments.

The December 2021 EUA granted to a monoclonal antibody product for pre-exposure use has conferred longer-term immunity for immune-compromised patients, just as vaccines have for the general population. We were concerned that the policy finalized in the 2022 PFS, i.e., to continue paying for these monoclonal antibodies under the Part B vaccine benefit until the end of the calendar year in which the PHE ends, would leave beneficiaries unable to afford the Part B copayment without the protection they need.

We appreciate CMS' reconsideration of the 2022 policy and strongly support its proposal to continue paying for monoclonal antibodies for pre-exposure prophylaxis prevention of COVID-19 and their administration under the vaccine benefit even after the products are FDA approved and the relevant EUA declarations are terminated. This proposal facilitates health equity for individuals unable to achieve COVID-19 immunity with traditional vaccines alone.

Haystack Project urges CMS to ensure that Medicare's rare disease patients retain access to telemedicine options for care/consultation from distant specialists.

Haystack strongly supports continued access to 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. Audio-only and audiovisual visits initiated from the patient's home have 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 were useful in avoiding 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 Haystack's survey appeared to view telehealth as vital throughout the PHE, but also recognized its value as an adjunct to in-person care thereafter. The PHE has given patients increased flexibility to see their healthcare providers from home or other convenient location, reducing the costs of transportation, missed work or school, child-care, and other expenses associated with in-person appointments. Increased access to telemedicine has also given patients access to disease specialists they may have previously been unable to see due to distance. It is not uncommon for rare disease patients to find that there are just a handful of disease-specific specialists in the entire country. Individuals in rural, low-income, and other underserved areas will lose meaningful access to these

experts unless the telehealth flexibilities remain in place.

Last year, the Government Accountability Office (GAO) compiled 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 identified 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.⁶ Making access to appropriate experts through telemedicine could make a meaningful difference in addressing some of these findings.

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 ensure that providers are sufficiently reimbursed for telemedicine services that are accessible for hearing and vision impaired patients.

Lack of reliable broadband wireless technologies and/or devices with data and video capabilities have also made it difficult for low-income families and those in rural areas to take advantage of the expanded availability of telemedicine other than through flexibilities permitting telephone-only visits. Returning to the pre-pandemic requirements on initiation site and audiovisual capabilities will disproportionately constrict care that the most vulnerable patients have relied upon.

CMS should ensure that its refined policies on coding/payment for remote therapeutic monitoring services does not deter use of technology to diagnose and manage rare disease patients

Haystack recommends that CMS not implement its proposal to require clinician reporting of one of the device/supply codes prior to including remote therapeutic monitoring services on claims for reimbursement. Rare disease patients face significant challenges in obtaining a diagnosis and accessing care. They are, therefore, highly motivated to engage with technology for condition management and determined to utilize new approaches to improve health outcomes.

As the pandemic emerged, rare disease patients and their families were, of necessity, taking on more demanding and active roles as the hands and eyes of clinicians. The pandemic has demonstrated that,

⁶ GAO Report. [GAO-22-104235, RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial](#)

with guidance, tools and support, families can take on proactive and impactful roles and responsibilities that optimize patient care. Haystack Project 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. Use of wearables, monitors, and layperson friendly medical equipment can provide key patient information that may not be ascertained from periodic in-person visits. Without reimbursement to level the playing field on access to emerging technologies, a family's financial resources will enable or limit the impact that technology can have on improving patient outcomes.

The proposed approach would limit remote monitoring to specific body systems and have a chilling effect on use of these services to diagnose and manage rare disease patients. It also overlooks the potential that the device/supply enabling clinician monitoring could be integrated into a device dispensed through the durable medical equipment benefit and not be an expense that clinicians can or should include on a Medicare claim.

CMS should ensure that clinicians participating in the Shared Savings Program are not penalized for initiating the diagnostic journey or providing evidence-based care for rare disease patients.

Haystack generally supports initiatives that incentivize clinicians to provide efficient, cost-effective, high-quality care. Individuals with rare and ultra-rare diseases are particularly vulnerable to changes in how care is received as well as provider reimbursement; We are concerned that reimbursement mechanisms that, like the Shared Savings Program, shift risk to clinicians could have unintended consequences to individuals with rare conditions for which benchmark costs are unavailable. Although we recognize that Medicare program savings can result from aligning incentives toward reducing costs associated with common health conditions, we have learned that these frameworks tend to discourage use of the resources required to diagnose and treat individuals with rare and extremely rare diseases.

Haystack Project continues to advocate for specific carve-outs applicable to rare disease patients as well as incentives to reward timely diagnosis, treatment planning, and care coordination. We believe that a carve-out is a pragmatic mechanism given that it is virtually impossible to reliably benchmark costs associated with treating Medicare beneficiaries with rare disorders, and even more so if the patient suffers from an additional chronic condition. In addition:

- 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.
- The relatively small population size for many rare disorders precludes availability of clearly articulated, scientifically-validated treatment standards that would form the basis of a reliable benchmark.
- Patients with rare disorders may not have access to a specialist with experience in treating their condition, leaving their care to a set of providers in various specialties that address specific disease symptoms. It is, therefore, difficult to assess which costs to assign to a specific clinician.

- Highly-specialized clinicians with expertise sufficient to manage the whole patient would appear to perform poorly when compared to clinicians managing more common conditions within the same specialty.

Last year’s GAO report cited a number of relevant findings that make a Shared Savings Program difficult if not impossible to implement without creating challenging incentives for providers:

- ***Overlap with other diseases.*** Rare disease symptoms are often non-specific and overlap with more common diseases. Patients not only face long diagnostic journeys, but often receive costly and potentially toxic treatments due to misdiagnoses.
- ***Lack of clinician knowledge.*** Because signs and symptoms of many rare diseases are not fully described or understood, patients and clinicians may fail to note significance of initial symptoms or discount patient/caregiver reports.
- ***Multiple disease presentations.*** Many rare diseases are without a single set of symptoms and are associated with symptom variability on an individual level as well as over time. Other rare conditions can impact multiple organ systems leading to care from multiple specialists before a correct diagnosis is made.
- ***Comorbid conditions.*** Comorbid conditions inject an additional layer of diagnostic complexity, particularly if the patient has two or more rare diseases. The GAO Report cited the example of acromegaly, a hormonal disease commonly accompanied by diabetes or cardiovascular disease. Acromegaly has a slow progression so that This rare disease follows a slow progressive course, so that individuals are generally not correctly diagnosed until they present with advanced disease and multiple comorbidities.

We urge CMS to devise a structural exception that would permit clinicians participating in two-sided risk arrangements, including the Shared Savings Program to treat patients with rare disorders, including administration of Part B drugs and prescribing of Part D drugs, without absorbing the incremental cost of this care that would otherwise apply under a shared-risk model. This carve-out/exception would be triggered when either:

- A new or existing patient presents with a diagnosis for a rare disease that is not associated with a disease-specific cost benchmark for shared-risk purposes that is based on an accepted standard of care for that disease; or
- The clinician identifies a patient with a set of symptoms requiring further follow-up through specialist referral and diagnostic testing and facilitates appointment(s) for those services.

The complex patient bonus should account for complexity associated with rare diseases and additional complexity associated with social determinants of health (SDOH).

Haystack appreciates that CMS recognizes the need to ensure clinicians treating Medicare’s sickest and most vulnerable patients are not penalized. We continue to support the complex patient bonus under the MIPS, and urge CMS to implement a presumption that patients with diagnosed rare disorders as well as those with significant symptoms requiring a definitive diagnosis are “complex.” CMS has utilized the

Hierarchical Condition Category (HCC) risk score to calculate complexity of a provider's patient population. The HCC score compares Medicare beneficiaries' FFS spending to the overall average for the entire Medicare population. The HCC score methodology has been used in other CMS programs to calculate risk adjustment.

We believe that the complex patient bonus remains important to:

- Protect access to care for complex patients
- Encourage high-quality care
- Mitigate any real or perceived disadvantage encountered by MIPS-eligible clinicians caring for complex patients.

The GAO report mentioned above also found implicit biases in rare disease patient care, where pre-existing judgments related to race, socioeconomic, or gender beliefs can interfere with a clinician's ability to accurately diagnose a disease or refer a patient for specialist follow-up.

We applaud CMS' attention to the needs of Medicare's most vulnerable patients, and believe that complexity is a function of both the patients' care needs and social determinants of health, i.e., these factors are additive. We urge CMS to broaden its sources for complexity beyond the set of diagnoses within the HCC to account for enhanced complexity in treating patients with rare conditions for which an HCC risk score is unavailable.

The Medicare Quality Payment Program (QPP) should more fully capture elements of high-quality care for individuals with rare diseases.

The QPP measures were intended to apply to *all* patients under a MIPS-eligible clinician's care. As Medicare's Quality Payment Program becomes an established factor in provider decisions on which patients to treat and how to treat them, we remain concerned that the focus on established indicia of "quality" for relatively common conditions represents a lost opportunity for meaningful improvement in rare disease diagnosis, treatment, and management. We believe that the QPP could increase attention on rare diseases and reduce the disparities in care quality and access experienced by individuals of color and other under-served patients.

We urge CMS to

- develop measures and improvement activities that reflect quality care for rare diseases in the Merit-Based Incentive Payment System (MIPS)
- enable clinicians to earn MIPS "bonus points" for diagnosing and/or appropriately managing and treating patients with ultra-rare disorders
- include measures applicable to rare diseases in its Chronic Care Management MVP
- increase clinician awareness of potential for a rare disease diagnosis in primary care by developing one or more measures incentivizing efficient rare disease diagnoses within the Promoting Wellness MVP and
- develop an outlier-styled mechanism to account for rare disorder diagnosis and treatment costs under Advanced Alternative Payment Models.

The GAO report found a lack of availability or accessibility to diagnostic tests. Even when confirmatory diagnostic testing is available, it is often not accessible due to reimbursement hurdles. Since most rare diseases are without an FDA-approved treatment, payers (including Medicare) decline coverage due to lack of medical necessity. Clinicians seeking a definitive diagnosis to enable a treatment plan (on- or off-label therapies) face significant paperwork burdens associated with prior authorization and appeals processes.

Haystack recognizes that the QPP was designed to maximize its impact on the value of care for Medicare beneficiaries and, therefore, was built around relatively common conditions. To date, it has not been well-suited to capture either quality or costs of care for patients with extremely rare disorders that may be difficult to diagnose and costly to treat. From an operational standpoint, the program's shortcomings in addressing ultra-rare disorders may not impact its overall functionality in addressing quality care for the most commonly-encountered conditions in the Medicare population. The structure and criteria for implementing quality measures make it difficult, if not infeasible, to create measures reflecting care for each rare disease, or even for related subsets of rare and extremely rare conditions as the measures would fail to meet both the benchmark and case requirement thresholds. Moreover, clinicians would likely not elect to report on measures that do not apply to the majority of their patients unless the potential "point" value is comparatively high.

We suggest that CMS develop alternative means to reward clinicians treating patients with rare disorders, including practice improvement and advancing care information measures specific to extremely rare disorders. This might include incentivizing use of disease-specific patient registries, inclusion of communications regarding clinical trial participation within care planning, and use of FDA-approved therapies or evidence-based off-label treatments. Haystack is eager to work with CMS on measures reflecting the main components of quality care for people with rare and extremely rare disorders, including clinician activities that promote:

- Recognition of patients at risk for the disease
- Starting the appropriate evaluation
- Making the appropriate diagnosis and/or referring the patient to a specialist making the appropriate diagnosis
- Starting the appropriate treatment
- Scheduling the appropriate follow-up to ascertain treatment adherence/compliance and response.

Similarly, as CMS seeks to move Medicare-participating providers to risk-sharing arrangements, it must proactively consider the potential for unintended consequences to rare disease patients and, particularly, to rare disease patients within underserved populations. It is, therefore, imperative (as detailed above) that CMS develop a set of mechanisms through which these clinicians and their practices can confidently utilize the resources necessary and appropriate to treat rare disease patients without concern that they will personally or collectively bear the cost burden. The QPP could play an important role in advancing the quality of care for rare disease patients for which FDA-approved treatments are currently available, as well as advance overall understanding of disease processes for those conditions with no available treatment options. We strongly urge CMS to engage the stakeholder community, including rare disease patients and providers, to develop guardrails, protections, and payment mechanisms that improve care quality for rare disease patients.

Until CMS identifies and implements those mechanisms, we recommend that CMS allow clinicians treating patients with elusive diagnoses or rare disorders to do so without accumulating “costs” applicable within their shared-risk arrangement.

Conclusion

Once again, Haystack and its member organizations appreciate CMS’ increased consideration of the needs of Medicare’s most complex and vulnerable patients and its continuing commitment to reducing health disparities and inequities. We look forward to working with the Agency as it continues to refine payment policies under the Physician Fee Schedule.

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

Very truly yours,



Deanna Darlington
CEO and Ex Officio Board Member





