February 13, 2023

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

RE: CMS-4201-P
Medicare Program; Contract Year 2024 Policy and Technical Changes to the Medicare Advantage Program, Medicare Prescription Drug Benefit Program, Medicare Cost Plan Program, Medicare Parts A, B, C, and D Overpayment Provisions of the Affordable Care Act and Programs of All-Inclusive Care for the Elderly; Health Information Technology Standards and Implementation Specifications

Dear Administrator Brooks-LaSure:

Haystack Project is pleased to offer its comments on the Centers for Medicare & Medicaid Services' (CMS') proposed rule revising and refining policies in Medicare Advantage (MA) and the Medicare Prescription Drug Benefit (Part D). We appreciate that CMS' policy refinements were informed by responses to the Agency's July 2022 Request for Information and crafted to improve beneficiary protections, increase access to care, and facilitate health equity.

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 associated with 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.

Haystack Project's 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. 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. It is, therefore, imperative that these Medicare beneficiaries have all available tools and information when choosing between FFS and MA, comparing specific MA and/or Part D plans, and navigating access to the specialists and treatment options they need to address their specific rare condition.

Our comments offer insights and recommendations from the rare disease community so that CMS can continue to build upon its efforts to ensure that the Medicare program's benefits confer equally to individuals regardless of their race, financial resources, health care needs, or the rarity of their health condition(s).

Utilization Management Requirements

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. Rare disease patients face substantial challenges from symptom emergence through treatment and/or management of their condition.

Haystack Project supports CMS' efforts to increase the transparency of MA plans' utilization management and prior authorization policies and ensure that MA enrollees have full access to coverage available for Medicare FFS beneficiaries. Rare and ultra-rare disease patients within the Medicare population are particularly vulnerable when utilization management strategies cause substantial delays in treatment access. Our ultra-rare disease communities have expressed ongoing concern with inappropriate step therapy protocols and NDC "blocks" or "lock-outs" to constrict and delay access to rare disease treatments.

- **Step therapy protocols.** Step therapy is a frequently encountered utilization management strategy within commercial and Medicaid plans that was recently adopted by MA plans. Patients must "step" through older, less costly treatments before allowing access to newer, often more innovative or targeted, and inevitably more expensive options. Haystack Project continues to believe that this utilization management tool is inappropriate for the highly complex Medicare population. This is particularly true for rare disease patients for whom step therapy protocols may require failure on a treatment that is not useful (or even harmful) for their specific condition.

- **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 for commercial insurers seeking to manage multiple treatment options for more common conditions. In this context, there is little chance of harm to patients. In rare conditions, however, there is no useful purpose for delaying access to what may be the only on-label treatment under the guise of needing to "review" whether it should be covered.

In addition to recommending that CMS address and resolve the access hurdles identified above, Haystack appreciates and fully supports CMS proposals, including:

- Requiring that when no applicable Medicare statute, regulation, National Coverage Determinations (NCD), or Local Coverage Determinations (LCD) establishes when an item or service must be covered, MA organizations must include current evidence in widely used treatment guidelines or clinical literature made publicly available to CMS, enrollees, and providers when creating internal clinical coverage criteria.

    - We ask that CMS implement this requirement in a treatment- and condition-specific manner so that, for example, LCD articulating coverage for a drug used to treat one condition would not be interpreted as denying coverage for all other uses.

- Prohibiting MA plans from denying coverage for an item or service unless the denial is reviewed by a clinician with expertise appropriate for the particular item/service.
    - We applaud CMS for proposing this policy refinement; MA plan consultation with disease-specific experts **before** denying a claim could relieve a significant burden for both rare disease patients and their providers.
- Streamlining prior authorization requirements to ensure continuity of care for beneficiaries.
- Requiring that MA plans implement a Utilization Management review committee to ensure that its prior authorization and other utilization management tools are grounded in science.
    - Haystack project urges CMS to require that UM policies and procedures are developed in consultation with contracted providers.

We also urge CMS to develop a mechanism through which patients and clinicians can report on and resolve real world experiences that place significant burdens to access to care.

Health Equity in Medicare Advantage (MA) (§§ 422.111 and 422.112)

Health inequities within the rare disease community

The diagnostic and treatment access challenges common to rare disease patients generally can be an overwhelming burden for people of color, individuals with limited financial resources, and other underserved populations, including rural communities.

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 cannot fulfill their mission to advocate on behalf of ***all*** patients impacted by a rare condition. Haystack urges CMS to incorporate the experience of rare disease patients as it addresses health disparities and inequities.

Health Equity: Part D coverage prohibitions deny access to the standard of care for rare disease patients unable to afford out-of-pocket costs of necessary, off-label treatments.

Ninety-five percent of rare diseases have no available FDA-approved treatment. This means that patients must rely on off-label use of therapies indicated for more common conditions to reduce disease burden and/or slow disease progression. Patients and their providers are left fighting for off-label access from their insurers. This can be a tremendous burden for those insured through commercial plans or Medicaid, and frequently entails use of reconsideration, appeals, and formulary exception processes before access to treatment is granted. Medicare beneficiaries, however, may have no "light at the end of the tunnel" if the treatment(s) they need is generally covered through Part D. Unlike Medicaid, ACA plans, and employer-sponsored plans, Part D plans are ***prohibited*** from covering off-label uses not listed in compendia - there is no appeal or reconsideration mechanism available to overcome the "fact" that a treatment is a covered Part D drug for most patients needing it, but not for patients with rare conditions that fail to gain recognition in compendia.

This Part D coverage inequity is an inconvenience for individuals with sufficient resources to pay for the treatments they need, a hardship for those relying on retirement savings and acquired assets, and an absolute bar to medical care for individuals with limited means. Haystack Project continues to advocate for parity in coverage for Medicare beneficiaries with low-prevalence conditions. We urge CMS to work with Congress to align the statutory definition of "medically accepted use" for low-prevalence conditions with sources likely to include the standard of care, i.e., FDA label, compendia, peerreviewed literature, and opinion of disease experts identified by relevant specialty societies. Over a decade ago, oncologists and cancer patients faced a similar situation and Congress amended the Act to deem certain uses of anti-cancer treatments as "medically accepted" if those uses are listed in compendia (NCCN is an oncology-specific compendium) or there were two or more peer-reviewed articles supporting an off-label use. The circumstances that drove that legislation for oncology are analogous to the situation that rare disease patients find themselves in today.

Haystack Project similarly urges CMS to consider implementing a "rare disease" category of special needs plans that would include the care coordination and collaboration available in SNPs generally, as well as enhanced Part D benefits that provide coverage for off-label treatments that are consistent with the standard of care but have not achieved compendia inclusion. Although this approach would not fully resolve the Part D care gap, we believe that it would be a step in the right direction. In addition, access to SNPs focused on the needs of rare disease patients could alleviate some of the burdens our patients face in navigating the health care system.

In 2021, 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 and examined 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, many are treated for a condition they do 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]

The collaborative and coordinated care available within MA SNPs could improve efficiencies in treatment access and reduce costs associated with treatment delays that are all too common in extremely low prevalence conditions. Rare disease patients generally:

- 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.
- 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. As noted above, ***individuals relying on Part D coverage for***

[1] GAO Report.

off-label treatments supported by clinical guidelines and/or disease-specific expert consensus often find that the treatment they need to manage the rare disease they have is simply not a covered benefit.

Health Equity: Addressing digital health literacy

CMS proposes to require that MA organizations identify and offer digital health education to enrollees with low digital health literacy to assist with accessing telehealth benefits. Haystack Project recognizes that benefits of telehealth and the increased access it can provide, particularly to individuals with rare conditions. We strongly support CMS initiatives to ensure that Medicare beneficiaries have the tools they need to get the maximum value from remote care opportunities.

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). Most 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.

In addition, 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, 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 by individuals with very rare conditions 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 likely drive access to technologies that might improve patient outcomes.

We have 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 appreciate that MA provider directories will include information on availability of ASL interpretation and urge CMS to ensure that providers are sufficiently reimbursed for telemedicine services that are accessible for hearing and

vision impaired patients.

We recognize that educational outreach to ensure that patients have sufficient knowledge to utilize telemedicine and other remote care modalities will help reduce inequities. Unfortunately, lack of reliable broadband wireless technologies and/or devices with data and video capabilities may be a greater impediment to access for low-income families and those in rural areas. We urge CMS to ensure that technology-focused beneficiary education is fit to purpose with respect to the patient's real-world access to broadband wireless services.

Medicare Advantage (MA) Provider Directories

CMS proposes to require that MA organizations include provider linguistic and cultural capabilities (including American Sign Language) in the set of information on provider networks that beneficiaries receive at enrollment and annually thereafter. Oftentimes, the greatest challenge patients with rare and ultra-rare diseases face is simply finding a provider with the expertise to diagnose and treat their condition. There is a shared understanding within the stakeholder community that patients, including those with rare and ultra-rare conditions, would benefit from a reliable, one-stop source of information to identify in-network specialists with disease-specific expertise. The American Medical Association (AMA}, for example, recently voted to adopt a policy urging Medicare Advantage plans to maintain accurate provider directories. AMA board member Scott Ferguson, MD noted that "[p]atients face a false appearance of choice when Medicare Advantage plans create networks that are too thin and directories that are too flawed. A comprehensive and authoritative source of accurate information is needed from federal authorities to support patients in Medicare Advantage."

Haystack Project supports CMS' proposed changes and further urges the Agency to require plans to include granularity on provider specialties, whether the provider is accepting new patients, average wait time to secure a new patient appointment, and hospital affiliation.

In addition, we strongly support CMS' proposal to codify its interpretation of Section 1852(d)(1)(A) of the Act to mean that in the event an in-network provider or service is unavailable or inadequate to meet an enrollee's medical needs, the MA organization must cover care outside the provider network and apply cost-sharing applicable to in-network providers. We urge CMS to apply a definition of "unavailable" that takes into account the specific patient, their medical condition, and the urgency of their medical need.

Clinical Trial-Related Provisions

Haystack Project appreciates that CMS is seeking to clarify MA coverage for costs associated with clinical trial participation. Under the proposed rule:

- MA plans would cover the routine care costs associated with participation in clinical trials falling within the Clinical Trials National Coverage Determination 310.1 (NCD) (NCD manual, Pub. 100–03, Part 4, section 310).
    - The costs associated with this coverage and all reasonable and necessary items and services used to diagnose and treat complications from participating in clinical trials will be shifted to Medicare FFS
    - MA enrollees participating in clinical trials are not subject to Part A and B deductibles.

- MA plans may not require prior authorization for participation in a Medicare-qualified clinical trial not sponsored by the plan or impede an enrollee's participation in a non-plan-sponsored clinical trial under NCD 310.1.

Haystack Project and its member organizations recognize the importance of clinical trial participation. Individuals with extremely rare conditions are particularly aware that clinical trial participation can enable early access to promising therapies. We therefore support proposed clarification as it applies to coverage for routine care costs associated with clinical trial participation. We strongly oppose the proposal to shift costs of routine care and the "investigation" item or service from MA plans to FFS within the context of clinical trials initiated to satisfy an NCD with CED requirement because:

- The rationale for shifting costs from MA plans to Medicare FFS is within the Medicare statute's provision requiring FFS to pay for newly-covered items and services (due to legislative change or National Coverage Determination) that reach a "significant cost" threshold until the costs for the coverage change are incorporated into MA capitation rates for the contract year.
- NCD 310.1 ***expanded*** Medicare coverage to include routine care costs associated with participation in certain clinical trials. Although it is unclear whether or not the costs associated with this coverage are significant, the NCD did, in fact, result in expanded coverage.
- National Coverage Determinations do not necessarily result in expanded coverage. In fact, any NCD that includes a CED requirement for indications that are within the FDA-approved label for a drug or biological, or that were initiated to curb off-label uses of that treatment, is a coverage restriction. There is no coverage expansion upon which a determination of significant cost can be made, and any determination would appear to be outside of CMS' authority under the statute.
    - Routine care costs associated with a clinical trial initiated to resolve a CED requirement would, however, potentially fit within NCD 310.1.

Historically, Medicare has covered drugs and biologicals for their medically accepted uses. The NCD process has rarely been initiated to evaluate coverage of these products, yet they are covered by Medicare FFS in the absence of any NCD or LCD. CMS has not stated or implied any change to this longstanding policy. Patients and their providers have, from Medicare's inception, relied on the implied policy that Medicare covers medically accepted uses of drugs unless CMS or its contractors propose and finalize coverage restrictions.

Haystack Project has significant concerns that any expansion in use of CED to manage utilization of emerging treatments will disproportionately burden patients with very rare diseases. Small population studies are inherently viewed as inferior to the clinical trials that can be performed to develop treatments for common conditions, sponsors rely on accelerated approval mechanisms (without accelerated approval, the approval process would be prohibitively long and costly), and treatments are likely to be launched with a relatively high price tag. All of these factors increase the chance that a specific rare disease treatment will, like CAR-T, be the target of an NCD initiated by CMS or at the request of an MA organization. Unfortunately, the stakes are even higher for rare disease patients without an FDA-approved treatment since any potential off-label uses would be subject to national noncoverage during and beyond resolution of the CED study requirements.

We are similarly concerned that the language of CMS' proposed policy may incentivize MA plans to initiate the NCD process for each and every new treatment that might increase their costs. Managed care organizations within the Medicaid program are accustomed to seeking a carve-out for costs associated with newly-approved treatments, and this practice is consistent with the statutory framework in Medicaid. The Medicare statute, however, is clear in enabling NCD-based cost shifting to

FFS only when new coverage is created through legislation or an NCD, and the new coverage is associated with a significant cost.

We urge CMS to implement its clarification on MA coverage for routine care costs in clinical trials and without deeming each additional NCD with CED requirements as potentially imposing a "significant cost" to MA organizations.

Medicare Advantage (MA) and Part D Marketing (Subpart V of Parts 422, 423)

Haystack Project appreciates that CMS' proposed rule responds to recent reports of deceptive marketing practices among MA plans seeking to enroll Medicare beneficiaries by enhancing the the information that patients are provided when selecting or switching plans. We generally support the proposed changes directed at increasing patients' health literacy, enabling informed decisions, and regulating communications from third-party marketing organizations (TPMOs) to Medicare beneficiaries.

We urge CMS to also ensure that newly-eligible Medicare beneficiaries and those currently enrolled in Medicare FFS receive information on the impact that MA enrollment may have on their ability to secure supplemental coverage if they determine to switch back to FFS.

Gross Covered Prescription Drug Costs

CMS stated in its proposed rule that it intends to remove the two references to "actually paid" in the definition of ''gross covered prescription drug costs'' at § 423.308. Haystack Project appreciates that CMS has moved toward transparency in its approach to implementing the negotiation process under the Inflation Reduction Act (IRA). The patient community has expressed an interest in learning more about the IRA and its potential impact on their out-of-pocket costs.

We urge CMS to release guidance on IRA implementation, in draft form, as soon as possible, and to ensure that the comment period is sufficient to ensure that patient advocacy organizations can provide meaningful feedback.

Conclusion

Once again, Haystack and its member organizations appreciate the opportunity to submit comments as CMS finalizes its refinements to the MA and Part D program requirements. 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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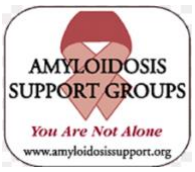
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