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February 13, 2023
Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 21244

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:

On behalf of the EveryLife Foundation for Rare Diseases, we are pleased to provide comments relevant to the health equity and utilization management components of the proposed rule on Medicare Advantage and the Medicare Prescription Drug Benefit.

EveryLife's policy priorities are informed by the needs of our community and our shared mission of advancing the equitable development of, and access to, lifesaving diagnoses, treatments, and cures. To inform our policy work, we convene the Community Congress, a forum for collaboration across stakeholders, representing over 200 individual rare disease patient advocacy organizations in addition to over 90 other healthcare and biotechnology organizations.

Over 30 million Americans live with one or more rare diseases, 95% of which have no approved treatment. Rare disease patients and families navigate how to manage expenses from multiple inpatient and outpatient encounters, costs for prescription therapies and medical devices, and the support services that are critical for their health and wellbeing. The National Economic Burden of Rare Disease Study in the United States estimated in 2019 the overall annual economic burden of rare disease exceeded $966 billion[1]. Among the Medicare enrollee data studied, 11.5 percent of enrollees had one of the 379 rare diseases included in the study. The 6.8 million Medicare enrollees with a rare disease made up 43 percent of the total direct costs identified in the study.[2]. Medicare enrollees accounted for $192.7 billion in excess direct medical costs at a price of $28,185 per person[3].

[1] https://ojrd.biomedcentral.com/counter/pdf/10.1186/s13023-022-02299-5.pdf

Medicare will continue to play a large role within the rare disease community. The proposed rule makes much needed improvements to address healthy equity and utilization management challenges experienced by the rare disease community. We offer the following comments on the progress the rule makes in addressing the issues and further action CMS can take to support the rare disease community.

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

We applaud CMS for working towards achieving policy goals that promote health equity for all enrolled in its programs. By clarifying and expanding population definitions, refining best practices for provider directories, improving digital health literacy procedures, and enhancing Quality Improvement (QI) programs to promote equity in care, we can positively address the needs of the rare disease community. Stigmatization may be increased and complex for marginalized communities with rare diseases or those without a diagnosis because of their intersectionality of identities. The following comments propose opportunities to further achieve greater health equity for the rare disease community.

We propose expanding the definition of *"otherwise adversely affected by persistent poverty and inequity"* to include employment status due to the physical, social, and economic impacts it has on the rare disease community. The National Economic Burden of Rare Disease Study (2019) found that absenteeism and forced retirement totaled $149 billion and $136 billion, respectively. Patients with rare diseases may have an increased likelihood of developing a severe functional impairment or disability that restricts their ability to work, limits their employment opportunities, and lowers their earnings. In 2019, the estimated indirect costs of rare diseases due to productivity loss was $437 billion.

Including employment status in the list of population definitions would recognize the disparities faced by individuals unable to earn a living wage due to physical, social, or economic restraints. It will provide opportunities to assess and understand the barriers faced by this patient population and strategies that can be taken to ensure they can still receive a timely diagnosis, treatment, and cure. We commend CMS for continuing to recognize and address the needs of all individuals.

We applaud CMS for taking steps to advance health equity through expanded QI program requirements. We recommend including activities that provide opportunities for increasing knowledge about the diagnostic odyssey, rare diseases and health-related quality of life in the list of example activities to incorporate into existing QI programs. On average, rare disease patients wait approximately 6.3 years before receiving a confirmed diagnosis. It is estimated that patients with rare diseases see 16.9 specialists since their first rare disease symptom appeared. According to a Definitive Healthcare 2021 survey, the healthcare commercial intelligence company found that a lack of rare disease education and lack of awareness of rare disease symptoms were the most common challenges faced by healthcare providers when addressing rare disease patients.

The education and training of healthcare providers in the recognition of rare disease symptoms and understanding of treatment options is essential for ensuring equity for patients with rare diseases. By establishing opportunities for healthcare providers to recognize symptoms and culturally and linguistically communicate next steps, reducing the average wait time for receiving a confirmed diagnosis and the estimated number of specialists being involved since the onset of the first symptom can be accomplished. We recognize and appreciate the approaches CMS is taking to promote health equity across its policies and programs.

Utilization Management Requirements: Clarifications of Coverage Criteria for Basic Benefits and Use of Prior Authorization, Additional Continuity of Care Requirements, and Annual Review of Utilization Management Tools (§§ 422.101, 422.112, 422.137, 422.138, and 422.202)

Utilization management techniques create hurdles that the rare disease population must overcome to receive access to a treatment that many have fought for decades to exist. A 2020 study of Medicare Part D formularies found that 76 percent of orphan drugs were subject to prior authorization, highlighting the unique challenges the rare disease community faces when trying to access their life-saving treatments.[4] Prior authorization can lead to care delays, somewhat or significant negative impacts on clinical outcomes and can lead to increased hospitalizations.[5] We appreciate the proposals within the rule to address utilization management requirements, to ensure that the polices are putting the science and patient first. We offer the following comments and recommendations on how the proposals would impact the rare disease community.

Coverage Determinations
We applaud much of the proposed process for how best to create internal coverage policies in the absence of established criteria. The increased transparency into the decision-making process, the inclusion of clinical literature as current care standards, and the inclusion of the consideration of the physician's recommendations regarding medical necessity will all greatly improve a system that has long frustrated the rare disease community. The changes will help to streamline the decision-making process and provide much needed clarity on how coverage decisions are made. When determining internal coverage standards, we urge CMS to consider the unique challenges of the rare disease community.

Many rare diseases have limited published clinical literature and rely on a few specialists on how best to treat the condition***. A final rule on this issue should include language that acknowledges the unique challenges of identifying clinical literature for a rare disease and requires transparency on the efforts to identify the clinical literature for the internal coverage standard.***

We also urge CMS to require Medicare Advantage internal coverage plans not to exclude treatments from coverage or base medical necessity decisions based on the Food and Drug Administration's (FDA) approval pathway utilized for the treatment. Therapies for rare diseases are developed for areas of high unmet need and have unique complexities that decades of investments in strengthening the rare disease expertise at the FDA and multi-stakeholder partnerships have been focused on addressing.

Timelines and Continuity of Care
A common frustration when rare disease patients are faced with prior authorization requirements, denials and appeals is uncertain timelines for response. The EveryLife Foundation appreciates that the rule includes timeline requirements tied to assessing network adequacy. We urge CMS to further restrict and clarify expected timelines for response to prior authorization submissions and appeals to ensure all decisions are made in a timely and transparent manner. CMS should also explore methods to monitor compliance with expected timelines and provide a dedicated vehicle to collect instances of MA plan's violation of required timelines.

We applaud the addition of the continuity of care requirements for ongoing care to beneficiaries. Patients should not need to worry that switching insurances will result in losing care. We are thankful to

[4] https://www.ajmc.com/view/predictors-of-orphan-drug-coverage-restrictions-in-medicare-part-d
[5] https://www.ajmc.com/view/how-prior-authorization-can-impact-patients-with-rare-disease

see the guidelines outlined to help ensure that does not occur. ***We also urge CMS to require plans to cover newly approved treatments while going through the internal coverage determination process.*** Patients who have waited years for the hope of a new treatment, often report lengthy delays after approval before coverage policies are determined. Particularly in rare diseases, where there is likely no existing treatment, it is unethical to further delay treatment initiation.

Utilization Management Committee

The creation of the Utilization Management Committee can help improve consistency and transparency among Medicare Advantage plans. The impact of the proposed committees will be limited for the rare disease population unless the committees include appropriate expertise familiar with the unique nuances in rare disease research and therapy development. We ask the final version of the rule to require the new Committees to consult with experts in rare disease while conducting their reviews. We urge CMS to require accessible aggregate information on the plan's use of UM policies, information that could be produced annually in conjunction with the new Committee's review.

Looking Forward

In 2021, 94% of prior authorization requests were approved, but of the over 2 million requests that were denied, only 11% were appealed while appeals were met with an 80% success rate. The data aligns with the stories we consistently hear from rare disease patients[6]. There are several concerns highlighted by this data, including if 94% of requests are approved and this number is consistent year to year, are prior authorization policies needlessly disrupting care and causing clinical workforce burden. Additionally, CMS should investigate the reasons behind the low appeal number to determine if corrective actions are needed to improve transparency of the process and patient's understanding of their right to appeal. We urge CMS to partner with CMMI to explore innovative ideas to radically change the use of prior authorization policies, especially for vulnerable populations including those with rare diseases. Proposals such as a 'gold carding program' that rewards specialists with a history of high success rates by limiting the products that require prior authorization could lower the burden of UM policies on both the patient and provider. Patient advocacy and provider communities have long called attention to the growing problem with inappropriate use of UM policies and CMS and CMMI should incentivize the evaluation of new approaches.

Thank you for the opportunity to provide comments on health equity and the utilization management requirements within Medicare Advantage and Prescription Drug Plans. Please contact Jamie Sullivan, Senior Director of Policy at jsullivan@everylifefoundation.org if we can provide any additional information to inform your process.

Sincerely,

Dylan Simon

[6] https://www.kff.org/medicare/issue-brief/over-35-million-prior-authorization-requests-were-submitted-to-medicare-advantage-plans-in-2021/

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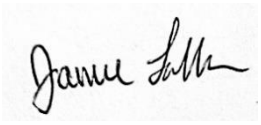

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