March 10, 2023

The Honorable Xavier Becerra
Secretary of Health and Human Services
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

The Honorable Chiquita Brooks-LaSure
Administrator Centers for Medicare and Medicaid Services
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

Re: Advancing Interoperability and Improving Prior Authorization Processes Proposed Rule (CMS-0057-P)

Dear Secretary Becerra and Administrator Brooks-LaSure:

On behalf of the more than 25 million Americans living with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Centers for Medicare & Medicaid Services (CMS) for the opportunity to provide comments on CMS's proposed rule captioned above (Proposed Rule).[1] NORD is the leading independent patient advocacy organization representing all individuals and families affected by rare diseases in the United States. Alongside our network of over 330 disease-specific member organizations, more than 18,000 Rare Action Network advocates across all 50 states, and our national and global partners, NORD improves lives, empowers patients and caregivers, and provides the right information and resources at the right time to our community.

A rare disease is defined as a disease or condition that affects less than 200,000 people in the United States.[2] Rare diseases are often serious, chronic, and complex. People with rare disorders typically see many providers and specialists, often across multiple health systems and state lines, over the course of their lives. However, due to our community's small and fragmented populations, patients often find that their disease and treatment needs are not well understood by providers or payers. As a result, cumbersome prior authorization protocols can be especially challenging and even dangerous to our community. With this perspective in mind, NORD applauds CMS for taking steps to improve the electronic exchange of health care data and reduce the patient and provider burdens related to prior authorization requirements.

Our comments on specific provisions of the proposed rule are included below.

[1] https://www.federalregister.gov/documents/2022/12/27/2022-26956/medicare-program-contract-year-2024-policy-and-technical-changes-to-the-medicare-advantage-program
[2] Section 526, Federal Food, Drug and Cosmetic Act [21 USC 360bb]

I. Improving Prior Authorization Processes

Utilization management protocols, such as prior authorization, are tools that can help control costs and prevent the overuse of health care services. However, when used improperly or without consideration of a patient's unique medical situation or history, utilization management can delay necessary treatment by weeks or even months and aggravate health inequities. According to a survey of rare disease patients and caregivers conducted by NORD in 2019, 61% of people with rare disorders have been denied, or faced delays accessing treatments that required pre-approval from an insurance company (including prescription drugs, medical devices or other treatments). In addition, 18% of respondents reported they were denied a referral to a specialist. Respondents earning less than $20,000 per year were twice as likely to be denied referral to a specialist compared to those earning $100,000 or more. [3]

Delayed access due to cumbersome prior authorization processes can harm people with rare disorders in several ways. For example, rare diseases often are particularly difficult to diagnose and treat. From the onset of symptoms, it takes on average six years for a patient with a rare disorder to receive an accurate diagnosis, with many patients experiencing countless tests, misdiagnoses, some incorrect treatments, and potentially loosing trust in the medical system.[4] An accurate diagnosis is the first step in managing a rare disease appropriately and identifying specific resources and interventions for the best possible clinical outcomes. As time goes on without answers, illnesses may progress, leaving patients to manage worsening, more severe or additional symptoms. This, in addition to the toll to mental and emotional health that patients and caregivers experience while devoting resources, time, and energy to finding an accurate diagnosis, contributes to what is known in the rare disease community as the "diagnostic odyssey."

Unfortunately, NORD has heard time and time again from our community that prior authorization processes play a significant part in extending the diagnostic odyssey. For example, exome sequencing (ES) is a type of genetic testing that can be used when other testing does not result in a diagnosis. ES has been found to yield a 25-68% success rate in diagnosing inherited rare diseases. [5, 6] However, multiple studies have found that prior authorization protocols delay access to ES.[7, 8] Indeed, a retrospective review of 115 cases of pediatric ES tests that were ultimately authorized by insurance found that, on average, prior authorization requests resulted in a delay of 104.4 days – with income again being negatively

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[3] https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf
[4] Blöß S, Klemann C, Rother AK, Mehmecke S, Schumacher U, Mücke U, et al.Diagnostic needs for rare diseases and shared prediagnostic phenomena: Results of a German-wide expert Delphi survey. PLoS ONE. 2017; 12(2):e0172532. Available from: http://dx.plos.org/10.1371/journal.pone.0172532.
[5] Malinowski J, Miller D, Demmer L et al. Systematic evidence-based review: outcomes from exome and genome sequencing for pediatric patients with congenital anomalies or intellectual disability. Genetics in Medicine. 2020;22(6):986-1004. doi:10.1038/s41436-020-0771-z
[6] National Organization for Rare Disorders (NORD). Genetic Testing 101 For Rare Diseases [Webinar Slides]; 2018. https://www.youtube.com/watch?v= eEdV2vwz160. Accessed October 26, 2020.
[7] Smith HS, Franciskovich R, Lewis AM, et al. Outcomes of prior authorization requests for genetic testing in outpatient pediatric genetics clinics. Genet Med. 2021;23(5):950–955. http://doi.org/10.1038/s41436-020-01081-x.
[8] Lennerz JK, McLaughlin HM, Baron JM, et al. Health care infrastructure for financially sustainable clinical genomics. J Mol Diagn. 2016;18(5):697–706. http://doi.org/10.1016/j.jmoldx.2016.04.003.

correlated with the length of time for payors to reach a decision.[9] With this context in mind, NORD is pleased that CMS is committed to reducing the burden that unnecessary prior authorization protocols place on patients.

Providing Specific Denial Reasons

NORD is supportive of CMS's proposal that payers provide a "specific reason" for denying a prior authorization request. However, we believe that CMS should be clearer with respect to what constitutes a "specific reason." We are concerned that, without robust guardrails, payers may provide vague or non-informative answers that are not helpful to patients and providers in subsequent appeals processes. For example, if the reason for a prior authorization denial is "missing documentation," then the payer should specify *which documents* are missing to help expeditiously address the issue. We request that CMS clarify in the final rule that payers must provide detailed, actionable information when providing reasons for prior authorization denials.

Prior Authorization Timeframes

NORD is strongly supportive of efforts to improve the speed of prior authorization decisions. However, we recommend that CMS revise the timeline from what is outlined within the Proposed Rule. CMS has proposed that impacted payers "must provide notice of prior authorization decisions as expeditiously as a patient's health condition requires, but no later than 7 calendar days for standard requests. We also propose that Medicaid fee-for-service and CHIP fee-for-service programs must provide notice of prior authorization decisions as expeditiously as a patient's health condition requires, but no later than 72 hours for expedited requests unless a shorter minimum time frame is established under state law."[10] People with rare disorders often depend on regular, and *timely,* treatment in order to maintain their health. A delay of seven days is still too long for many in our community. Therefore, we recommend that CMS require payers to return decisions within 24 hours for expedited requests, and within 72 hours for standard requests. We also urge CMS to develop rulemaking that sets limits on the frequency of prior authorization requirements when a patient is on a stable treatment.

Exclusion of Prescription Drugs

While NORD supports the provisions within this proposed rule regarding prior authorization, we are disappointed that CMS has not included outpatient prescription drugs within the scope of this proposed rule. Many patients living with rare diseases depend on access to outpatient prescription drugs and/or biologics for the treatment and management of their condition. If this rule is finalized as written, these patients will continue to experience unnecessary delays in accessing their therapies.

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[9] Lee G, Yu L, Suarez CJ, Stevenson DA, Ling A, Killer L. Factors associated with the time to complete clinical exome sequencing in a pediatric patient population. Genet Med. 2022 Oct;24(10):2028-2033. doi: 10.1016/j.gim.2022.06.006. Epub 2022 Aug 11. PMID: 35951015.

[10] 87 Fed. Reg. at 76296

II. Application Programming Interfaces (APIs)

NORD is broadly supportive of CMS's proposal to advance the usage of Fast Healthcare Interoperability Resources (FHIR) based application program interfaces (APIs). As stated above, the care needs of many people with rare disorders can be incredibly complex and involve multiple specialists at any given time, and sequentially, over the course of a lifetime. Eighty percent of respondents to NORD's 2019 survey, for example, reported being treated by at least one specialist in addition to their primary care provider.[11] We believe that well implemented APIs are a valuable tool that will enable members of our community to receive better coordinated treatment and more readily access information about their medical care. We are also pleased that CMS has expanded upon the requirements proposed in the December 2020 Proposed Rule and is proposing to include Medicare Advantage within the scope of this proposal, in addition to Medicaid, CHIP, and qualified health plans on federally facilitated exchanges. [12]

In the creation, release, and upkeep of these new APIs, it is important that CMS and application developers consider challenges related to the use of electronic health records for people with rare disorders, including interoperability challenges and the lack of ICD-10 codes for many rare diseases. Of the more than 7,000 known rare diseases, only approximately 500 have an ICD-10 code.[13] As a result, patients' symptoms and their diagnoses may be coded as the most similar condition that *does* have an ICD-10 code, vital diagnostic and treatment information may be disproportionately contained in the notes fields as unstructured text, or codes may be used to describe underlying symptoms, rather than the true diagnosis. The lack of ICD-10 codes has implications for reimbursement for care, and for research into the prevalence and cost implications of rare diseases.

Finally, NORD urges CMS to include outpatient prescription drugs within the items and services subject to the proposed Patient Access, Provider Access, and Payer-to-Payer APIs, or to swiftly promulgate further rulemaking to that effect. While we recognize that there are technical challenges associated with including prescription drug prior authorization data within the APIs, we believe that the impact of this rule will be severely limited without that information. Many patients living with rare diseases depends on access to outpatient prescription drugs and/or biologics for the treatment and management of their condition. We believe that leaving prior authorizations for prescription drugs out of the rule will significantly limit its effectiveness; it will do nothing to alleviate ongoing access barriers to vital therapies, and will also, quite simply, be confusing to patients.

Patient Access Application Programming Interface (API)

NORD is supportive of CMS's proposal to require impacted payers to include information about patients' prior authorization decisions within the Patient Access API. As CMS notes, all communications to patients, as well as the API's user interface itself, should be accessible to all patients. This includes people with disabilities, older adults, individuals with limited English proficiency, and those with limited health literacy or who are impacted by social risk factors that may create barriers to the full benefit of these applications. We encourage CMS to partner closely with advocates in the patient community to create a broad range of beta-testers and stakeholders. This bench of advocates should be consulted continuously

[11] https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf
[12] CMS-9123-P
[13] https://pink.pharmaintelligence.informa.com/PS145630/ICD-Code-Breaking-Rare-Disease-Advocates-Want-Simpler-Shorter-Nomination-Process?vid=Pharma&processId=fb194882-71c8-49b0-95ae-f318e0c65bbb

throughout the APIs development and rollout. It is also important for CMS to ensure that people who do not have access to their data through the API (for example, patients living in areas without consistent internet access) are still able to access timely prior authorization-related information.

The benefits of this technology will not be fully realized if patients are unaware of how to decipher information regarding their prior authorization requests, or how to take appropriate action in response. Therefore, we urge CMS to provide ongoing patient education to support users as they navigate the data through the API. Privacy and consent information should be clear and easily understandable within the user interface. In addition, information regarding prior authorization decisions should be presented in such a way that it is actionable to patients as they navigate their treatment options.

Provider Access API

We support steps to facilitate the accurate and seamless exchange of data among providers on the same care team across institutions and geographies. Complete and timely records provide healthcare providers invaluable insights into the care of their patients, including breakdowns in referrals and challenges accessing necessary diagnostics and therapies. We believe the provider access API will be an important step in this direction. However, given the resource constraints that can limit IT implementation and adoption projects in particular in rural and safety-net hospitals, we urge CMS to track and counteract any equity issues that may manifest from operationalizing this proposed rule. Specifically, we encourage CMS to provide enhanced technical and financial support to resource-strained health care settings, such as those providing significant care to underserved populations, to ensure these providers can fully benefit and to limit any negative impacts from the implementation of the Provider Access API.

Payer-to-Payer Data Exchange

NORD agrees with CMS that "data exchange among payers is a powerful way to help patients accumulate their data over time… which can help to promote better patient care."[14] We are supportive of CMS's proposals to require impacted payers to implement and maintain a payer-to-payer FHIR API and make information on prior authorization available on that system. However, we are disappointed that CMS is not at this time proposing that payers "review, consider, or honor the active prior authorization decisions of a beneficiary's former payer."[15] All efforts should be made to limit the number of times that patients and their providers need to resubmit information to payers.

CMS has also requested comment on whether "prior authorizations from a previous payer should be honored by the new payer."[16] We strongly encourage CMS to move forward with rulemaking on this topic. Continuity of care is critically important to patients with chronic, serious, and rare disorders, and lapses in treatment due to coverage transitions can be devastating. With appropriate patient consent, we urge CMS to require payers to review the records of a prior payer before requesting a new prior authorization. We would also encourage CMS to require payers to review information that was previously submitted to the current payer in the prior authorization process. If a patient's condition and care needs have not materially changed, then they should not be required to go through repeated authorizations in order to continue on their treatment. Through this process, patients may be able to avoid unnecessary,

[14] 87 Fed. Reg. at 76269
[15] 87 Fed. Reg. at 76270
[16] 87 Fed. Reg. at 76271

duplicative utilization management while both payers and providers save significant duplicative administrative burdens. We suspect that this will dramatically reduce the paperwork burden on impacted payers. For example, a report examining prior authorization requests in Medicare Advantage in 2021 found that only 6% of the 35 million requests submitted by enrollees were denied. Of those denied that were subsequently appealed, 82% were fully or partially approved.[17] CMS should consider the extent to which the burden and delays of these authorizations could have been avoided through more rigorous data review.

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NORD appreciates the opportunity to comment on the Proposed Rule. Should you have any questions or wish to discuss anything in these comments, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs at hross@rarediseases.org.

Sincerely,

Heidi Ross
Vice President, Policy & Regulatory Affairs

[17] https://www.kff.org/medicare/press-release/medicare-advantage-plans-denied-2-million-prior-authorization-requests-in-2021-about-6-of-such-requests/