NORD®
National Organization for Rare Disorders

Alone we are rare. Together we are strong.®

July 29, 2026

The Honorable Mehmet Oz, M.D.
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

Re: CMS-2454-IFC Medicaid Program; Community Engagement Requirement for Certain Individuals

Dear Administrator Oz,

On behalf of Americans living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Centers for Medicare and Medicaid Services (CMS) for the opportunity to provide comments on the Interim Final Rule (IFR) for Medicaid Community Engagement Requirements. NORD welcomes the opportunity to engage with CMS and others on the rule so that we can collectively protect rare disease patients from the possibility of losing Medicaid coverage.

NORD is a federation of non-profit rare disease patient organizations and healthcare institutions, including Centers of Excellence (COEs), dedicated to improving the health and well-being of people living with rare diseases. NORD was founded more than 40 years ago, after the passage of the Orphan Drug Act (ODA), to formalize the coalition of patient advocacy groups that were instrumental in passing that landmark law. Our mission has always been, and continues to be, to improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.

NORD has specific comments regarding the scope and implementation of the Medicaid Community Engagement Requirement that we gathered from our community. We are concerned that under the current program design, eligible individuals are at risk of losing critical access to care due to administrative barriers that fail to reflect the realities of complex medical conditions. Our comments are organized around the following areas: (I) Medical Frailty – ensuring rare disease patients and the rare disease experience are fully captured, including through the NORD Rare Disease Database, multiple identification pathways beyond ICD-10 codes, clinical trial participation, and permanent and stable exemptions; and, (II) State Implementation and Operability – including operationalizing the two-part medically frail test, self-attestation limitations, and the good faith effort exemption.

I. Medical Frailty – Ensuring Rare Disease Patients are Captured

NORD believes that the proposed two-part test for exemption under medical frailty does not comport with congressional language and intent. H.R.1 does not state that an individual's ability to work or

complete other community engagement activities must be impaired for the individual to be considered "medically frail or otherwise have special needs" or to have a "serious or complex medical condition." In 2013, CMS purposefully expanded the term "medically frail," "to ensure that all people with disabilities are included in the medically frail definition."[1] The addition of a community engagement impairment standard narrows the scope of medical frailty in opposition of demonstrated congressional intent.

CMS defines medical frailty to include serious or complex medical conditions, disabilities, and functional impairments, and instructs states to use diagnosis codes, pharmacy data, and clinician attestations to identify qualifying individuals.[2] Many rare diseases fall squarely within this framework. A rare disease is one that affects fewer than 200,000 people per condition. Many rare conditions are chronic, progressive, and life-threatening. Approximately 95% of rare diseases have no FDA-approved treatment.[3] Conditions with identified treatments, whether on or off label, may still require lifelong care and management. For individuals with rare diseases regardless of treatment availability, disruption of healthcare coverage poses a risk of clinical destabilization, organ damage, hospitalization, or death.

Some rare disease patients, however, may face structural barriers to medical frailty recognition. While more than 10,000 rare diseases are known, only approximately 500 have specific ICD-10 diagnostic codes. Most are categorized under broad or nonspecific codes, making it difficult for individuals to demonstrate medical frailty through diagnosis-based verification alone. Rare disease patients often endure diagnostic journeys averaging 5–7 years before receiving a definitive diagnosis, which further lessens their ability to be identified by ICD-based criteria.[4] Without better and explicit CMS guidance on medical frailty, which would include those conditions which don't have an associated IDC-10 code, many of these rare disease patients are at risk of losing coverage under this new requirement.

A. The NORD Rare Disease Database as a Clinical Resource for State Qualifying Condition Lists

The rule requires each state to maintain a written list of qualifying conditions for medical frailty determinations along with a process for individuals whose condition is not on that list to request consideration. As stated, CMS already defines medical frailty. To ensure rare disease patients eligible for Medicaid are not denied support based on different medical frailty qualifications, CMS should also support the states with a de minimis list of serious or complex medical conditions, disabilities, and functional impairments. The accuracy and comprehensiveness of state lists and processes that will determine whether rare disease patients who qualify as medically frail will be critical. **NORD respectfully requests that CMS encourage state Medicaid programs to include the NORD Rare Disease Database[5] in their state verification plans required under §435.557 to help reviewers identify whether a given rare condition meets criteria for medical frailty and ensure no qualifying individual is overlooked due to**

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[1] Medicaid, Children's Health Insurance Programs, and Exchanges: Essential Health Benefits in Alternative Benefit Plans, Eligibility Notices, Fair Hearing and Appeal Processes for Medicaid and Exchange Eligibility Appeals and Other Provisions Related to Eligibility and Enrollment for Exchanges, Medicaid and CHIP, and Medicaid Premiums and Cost Sharing, 78 Fed. Reg. 4594 (proposed Jan. 22, 2013) (to be codified at 42 C.F.R. §440.315); 42 CFR §440.315(f).

[2] *Medicaid Program; Community Engagement Requirement for Certain Individuals*, 91 FR 33348, June 3, 2026 (Interim Final Rule) (to be codified at 42 CFR Parts 431, 435, 438, 457, and 600).

[3] *Rare Disease Drugs: FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development*, GAO-25-106774 (Nov. 18, 2024).

[4] *Rare Disease Facts and Statistics: Rare Disease at a Glance*, NORD Rare Disease Facts and Statistics | NORD (last visited, June 2026).

[5] *NORD Rare Disease Database*, NORD List of Rare Diseases | A-Z Database | NORD (last visited June, 2026).

their diagnostic odyssey, diagnostic unfamiliarity, or the absence of a specific ICD-10 code.

NORD's Rare Disease Database is the largest continuously updated database[6] of rare diseases available, containing more than 10,200 conditions; the database was built with the Monarch Initiative (MONDO), Orphanet, Online Mendelian Inheritance in Man (OMIM), and the NIH Genetic and Rare Disease Information Center (GARD).[7] Importantly, the NORD database is more comprehensive than any single federal database currently available to states. GARD's public-facing website represents a subset of its larger MONDO-derived list, and many conditions, particularly those without ICD-10 codes, are absent from resources states would otherwise consult. NORD's database is a reference tool that states and reviewers can use to:

- Verify whether a condition meets the Orphan Drug Act definition of rare;[8]
- Understand its clinical severity and treatment requirements; and
- Support individualized medical frailty determinations.

Use of the NORD database ensures rare disease patients are not excluded due to diagnostic unfamiliarity. By using the NORD Rare Disease Database as a reference for identifying medically frail patients, CMS can ensure that these individuals receive fair and consistent consideration regardless of the rarity of their diagnosis and the state in which they live.

NORD also requests CMS set minimum standards for the off-list consideration process that each state must maintain. At minimum, this process should include plain-language notice to patients, caregivers and providers that the process exists and how to access it, reasonable and defined timeframes for determination, written notice of the outcome with its factual basis, the right to a fair hearing, and acceptance of self-attestation during the pendency of any request.

B. Multiple Identification Pathways to Protect Enrollment for the Undiagnosed

NORD respectfully requests that CMS encourage states to use multiple identification pathways to determine medically frail individuals and not rely solely or primarily on diagnostic codes to capture both diagnosed and undiagnosed rare disease patients, ensuring that those with serious or complex health conditions actively seeking care but without an official diagnosis are not unintentionally disenrolled. Identification pathways should include, but not be limited to diagnosis codes, orphan drug prescription data, specialty procedure codes, specialist claims data, evidence of enrollment in third-party copay assistance or patient assistance programs, utilization history in claims data, and clinician attestation.

A Minnesota state law (MN ST § 62Q.451) provides a model for how states can consider other clinical information to identify a serious or complex health condition (in this case, a rare disease), when there is not a definitive diagnosis. Reviewers consider evidence of two or more primary care or specialty clinical consultations specific to the presenting complaint, documented developmental delay or regression, failure to thrive, or progressive multisystem involvement, and testing that failed to provide a definitive

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[6] *NORD Rare Disease Database*, NORD List of Rare Diseases | A-Z Database | NORD (last visited June, 2026).
[7] *NORD Rare Disease Database*, NORD List of Rare Diseases | A-Z Database | NORD (last visited June, 2026).
[8] Orphan Drug Act 21 USC 360bb(a)(2).

diagnosis or resulted in conflicting diagnoses.[9]

C. Clinical Trial Participation - A Gap the Rule Does Not Address

The rule is silent on clinical trial participation and leaves states without guidance on how to treat clinical trial participation in the context of compliance or exception determinations. With fewer than 5% of rare diseases having FDA-approved treatments, clinical trials are often the only available therapeutic option. Trial participation is often physically and mentally demanding, negatively impacting a patient's ability to meet community engagement requirements. Patients whose only therapeutic option is a clinical trial are left without a clear pathway to maintain coverage. Work requirement frameworks that fail to account for clinical trial participation risk disrupting access to care and affect trial enrollment, which fuels our country's innovation pipeline.

Patients participating in clinical trials may seek to avail themselves of the optional short-term hardship exemption for travel to receive medical care. The rule permits states to offer an exemption when an individual or their dependent must travel outside their community of residence for an extended period of time to receive care for a serious or complex medical condition not available locally. However, two structural features of the rule limit the utility of this pathway for rare disease patients.

First, the rule requires states to offer all short-term hardship exemptions or none of them.[10] This all-or-nothing structure may have a chilling effect on state uptake. A state that is willing to offer the travel exemption may decline to do so rather than take on the full package of optional exemptions. The result is that patients who would benefit from the travel pathway lose access to it not because the state objects to it, but because the rule's structure makes adoption more costly than it would otherwise be.

Second, the rule limits the travel exemption to travel for care related to a serious or complex medical condition as defined at § 435.554(c)(5)(i)(E). This definition ties the travel exemption to conditions that appear on the state's qualifying medically frail list. As NORD has documented above, rare diseases are at risk of being systematically underrepresented on those lists due to ICD-10 coding gaps and eligibility reviewer unfamiliarity. A patient with a rare disease that does not appear on their state's qualifying list who must travel to a clinical trial site may therefore be unable to access the very exemption designed to protect them.

NORD requests that CMS address clinical trial participation explicitly.

- The most durable and protective approach would be for CMS to recognize clinical trial participation as an indicator of a serious or complex medical condition, potentially qualifying an individual as medically frail under the specified excluded individual framework. This would provide permanent protection — individuals participating in clinical trials as their only therapeutic option would be fully exempt from the community engagement requirement without needing to make repeated requests or rely on temporary exemptions subject to state

[9] Minn. Stat. § 62Q.451, subd. 1(b), https://www.revisor.mn.gov/statutes/cite/62Q.451.

[10] Section 1902(xx)(3)(B)(ii) "An applicable individual receives certain institutional (or comparable) services; an applicable individual resides in an area in which an emergency or disaster under certain Federal authorities has been declared or in an area of comparatively high unemployment; or an applicable individual, or the dependent of the applicable individual, must travel outside of their community for an extended period of time for necessary medical care for certain conditions."

adoption decisions.

- If CMS does not take that step, NORD requests at minimum that CMS recognize clinical trial enrollment and travel to clinical trial sites as qualifying activities under the short-term hardship exemption for travel to receive medical care — and that this recognition not be conditioned on the underlying condition appearing on the state's medically frail qualifying list.

D. Permanent and Stable Exemptions for Permanent and Stable Conditions

NORD requests that CMS direct states to grant permanent medical frailty exemptions for individuals whose conditions are lifelong, degenerative, terminal, or otherwise permanent and unlikely to improve. Once a permanent condition has been established, CMS should not require re-documentation.

Additionally, NORD notes the establishment of a precedent that a condition in stable management still qualifies for an exemption. Individuals with a Substance Use Disorder (SUD) in active recovery, including those in early or sustained recovery for up to five years, are recognized under a medically frail exclusion.[11] NORD requests that CMS apply this logic broadly. For individuals whose rare or complex condition is clinically managed to stability, the stability of the condition should not be used to deny medical frailty status. Stable management is an outcome of successful treatment, not evidence of the absence of a serious condition. Similarly, degenerative diseases, where clinically predictable outcomes indicate normal disease progression, should not require re-documentation. Doing so adds an unnecessary administrative burden to sick patients or their caregivers.

II. State Implementation and Operability

A. Caregiving as Community Engagement Activity

NORD appreciates that CMS has recognized caregiving hours as a qualifying community engagement activity. This is especially meaningful for community members who reorganize their lives around the needs of family members with complex diseases.

B. Good Faith Effort Exemption

Implementing the community engagement requirements will require significant fiscal appropriations and authorizations, system redesign, staff training, data integration, and beneficiary outreach. Adequate time is critical to ensure these systems function correctly and do not result in unnecessary coverage loss. In its "Collection of Information Requirements" section, the IFC notes that CMS expects to only approve good faith effort exemptions for two states. It also specifies that CMS expects to approve initial good faith effort exemptions for no longer than six months. NORD is concerned that this information may discourage states from seeking additional time, even when needed to implement the requirements successfully.

States that do not have adequate time to plan for and implement these changes will face an increased risk of administrative errors, enrollee confusion, and wrongful disenrollments. NORD respectfully

[11] 91 FR 33348 §II(E)5b.

requests that CMS consider applying the good faith effort exemption as flexibly as H.R.1 permits.

C. Operability of the Two-Part Medically Frail Test

As stated above, the proposed two-part test for exemption under medical frailty does not comport with congressional language and intent and will have the effect of forcing clinicians to be the arbiter of someone's ability to work, something they were never trained to do. CMS should return to Congressional intent and withdraw the second test. Furthermore, diagnostic codes are not designed to capture this information, and the ability to work or participate in other community engagement activities requires an individualized determination. The IFC does not define what constitutes a "significant impairment." States will have to re-work and expand on existing plans for medical frailty to allow them to make more complex and individualized determinations, and further incorporate medical providers into these determinations, who may need to now document an individual's inability to work.

As a result, NORD is concerned that many states will not be able to solidify such complex plans in time for January 1, 2027, without unintentionally disenrolling people with rare and other serious and complex conditions that should be eligible.

D. Self-Attestation Limits

NORD believes that the IFC's limit on states' ability to accept self-attestation for the medical frailty exclusion only once during an individual's period of enrollment beginning on January 1, 2028, is not consistent with congressional language. H.R.1 provides no specific limit on states' ability to rely on self-attestation when reliable data is unavailable to verify exclusions and exceptions.

For people with rare diseases, self-attestation is the most reliable way to establish eligibility for medical frailty or any other relevant exclusions. Producing frequent documentation is a substantial challenge for many with a rare disease diagnosis who require access to a small number of highly specialized providers and often face long waits for appointments. The IFC also indicates that it expects states to rely primarily on documentation in the form of diagnostic codes to identify medical frailty. This approach risks excluding individuals whose conditions are serious or complex but not fully captured in diagnostic codes. As noted, approximately 9,500 rare diseases do not have a unique ICD-10-CM code and are instead grouped under broad categories such as metabolic disorders, genetic syndromes, and inflammatory conditions. As a result, people with rare diseases would be indistinguishable from those with milder conditions and would likely be disenrolled despite being eligible for the exclusion.

NORD also notes that the self-attestation limitation will fall most heavily on individuals' mid-diagnostic odyssey. Individuals go undiagnosed for years due to a scarcity of physicians familiar with rare diseases, making it challenging, if not impossible, to produce definitive documentation that would meet the current criteria.

Conclusion

Medicaid is a lifeline for the rare disease community. NORD stands ready to work with the patient community and CMS to ensure that Medicaid coverage is not denied due to arbitrary definitions or

measures.

NORD, again, appreciates the opportunity to provide our comments to CMS on this critical issue. We look forward to further opportunities to continue engaging with CMS and are prepared to serve as a resource for CMS and state Medicaid agencies.

For questions regarding NORD or the above comments, please contact Michael Beard, Vice President of Federal and Global Public Policy, at mbeard@rarediseases.org or me at klowell@rarediseases.org.

Sincerely,

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