August 17, 2026

The Honorable Ron Wyden
Ranking Member
Senate Finance Committee
United States Senate
Washington, DC 20510

Re: Request for Information: Commonsense Policy Options to Lower Drug Prices for Patients

Dear Ranking Member Wyden:

On behalf of the more than 30 million Americans living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD®) appreciates the opportunity to respond to the Senate Finance Committee Minority's Request for Information (RFI) on "Commonsense Policy Options to Lower Drug Prices for Patients." NORD applauds the Committee's efforts to ensure that patients have access to affordable rare disease treatments.

NORD was founded by people living with rare diseases and family members whose voices spurred action by Congress to enact the Orphan Drug Act (ODA). Throughout our history, patient access to life-changing interventions has been our north star. The hope these treatments provide, and the promise of new science is built on the foundation of the ODA. Since then, more than 6,000 orphan drug designations have been granted for more than 1,000 rare diseases.[1] The Orphan Drug Act has been, and continues to be, a tremendous success measured in breakthrough innovations, safe treatments, and improved lives.

With more than 40 years of history supporting patients, caregivers, and advocates, NORD is the longest-standing umbrella group for the more than 30 million Americans living with a rare disease. An independent 501(c)(3) nonprofit, NORD is dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 355 patient organization members, is committed to improving the health

[1] Lewis J. Fermaglich, Kathleen L. Miller, *A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act*, ORPHANET J RARE DIS. 18(1):163 (June 23, 2023) doi: 10.1186/s13023-023-02790-7.

and well-being of people with rare diseases by driving advances in care, research, and policy.

Ensuring America's continued leadership in the biopharmaceutical industry is imperative not only for national security,[2] but for persons living with a rare disease to maintain access to treatments.

NORD urges the Committee to advance reforms that meaningfully lower costs and improve timely, equitable access to therapies for people living with rare diseases. For people living with a rare disease, many of whom still have no FDA-approved treatment, affordability and access must be addressed in ways that also preserve incentives for continued research and development of new therapies. NORD is concerned that government price-setting approaches, including international reference pricing policies, could have unintended consequences for the rare disease community by discouraging investment in treatments for small and medically underserved patient populations. NORD encourages the Committee to pursue patient-centered solutions that reduce patients' financial burden and improve access to existing treatments while sustaining the necessary innovation for new therapies and potential cures for people living with rare diseases.

Healthcare costs and affordability are front and center for Americans, and whatever policy solutions are put forward to address these issues must be balanced with the need to provide persons living with a rare disease with hope that comes from the investment in research and the right incentives.

I. Lowering Drug Costs

Incorporating foreign price data into the Medicare Drug Price Negotiation Program (DPNP) could have unintended consequences for patients, particularly when those prices are derived from health systems that use methodologies that may undervalue treatments for people with rare, chronic conditions. **For people living with a rare disease, the value of a treatment cannot always be adequately captured by traditional population-based measures of cost-effectiveness, particularly when a therapy addresses a serious condition with significant unmet medical need and few or no alternative treatments.**

[2] *Modernizing Medical Biotechnology Regulation*, NSCEB (January 2026) https://www.biotech.senate.gov/wp-content/uploads/2026/02/NSCEB-Future-of-Regs-Medical-FINAL.pdf.

Of particular concern is the use of the quality-adjusted life year (QALY) in some foreign health technology assessment and pricing systems.[3] QALY-based assessments can disadvantage people with disabilities and chronic or serious illnesses by assigning greater value to health improvements for individuals who can be restored to a state of "perfect health."[4] For people living with a rare disease, many of whom live with lifelong, progressive conditions, such methodologies can fail to capture the full value of treatments that extend life, slow disease progression, preserve independence, improve daily functioning, or reduce the burden on patients and families.

We encourage policymakers to ensure that Medicare's approach to drug negotiation does not directly or indirectly import valuation methodologies that could disadvantage people living with rare diseases. Decisions affecting access to rare disease therapies should reflect the experiences and priorities of patients and families, the severity of the disease, unmet medical need, and the full range of benefits a treatment may provide. Affordability is critically important to the rare disease community, but efforts to lower costs should not rely on methodologies that risk undervaluing the lives or health improvements of the very patients our healthcare system is intended to serve.

Eliminating Blockbuster Bailouts

H.R. 1 expanded the Orphan Drug Exclusion (ODE), excluding medicines treating one or more rare diseases from Medicare price negotiation while they are used exclusively for rare disease indications.[5] The legislation also clarified that the timeline for potential negotiation begins once a medicine loses that exclusion.[6] This approach aligns Medicare policy with the original patient-centered purpose of the Orphan Drug Act: to encourage continued research and development for populations that are too small to benefit reliably from conventional market incentives.

NORD supports preserving such protections. At the same time, protecting orphan drug incentives should not mean insulating manufacturers from appropriate accountability or overlooking the affordability challenges facing patients and families. Policymakers can, and should, address excessive patient out-of-pocket costs, insurance barriers, and affordability while maintaining incentives for rare disease research and treatment development.

Rolling back the expanded ODE could revert to a policy that discourages researchers and industry from pursuing additional rare disease indications precisely when

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[3] Henry Fong & Martina Garau, *How Widely Are QALYs Used in OECD Countries? A Snapshot of International Practices*, OHE (June 11, 2025).
[4] *What Is a QALY? Meaning, Calculation, and Debate,* ScienceInsights (March 12, 2026).
[5] Section 71203.
[6] *Id.*

patients need more research, not less. Congress should instead preserve incentives that encourage developers to investigate the full clinical potential of medicines for rare disease populations, and pair those incentives with policies that promote meaningful patient access and affordability.

For patients living with one of the thousands of rare diseases for which no approved treatment exists, the stakes are not theoretical. A decision not to pursue a second or third rare disease indication can mean that an entire patient community loses the opportunity to learn whether an existing medicine could treat their disease. **Federal policy should encourage—not penalize—the continued pursuit of scientifically promising treatments for people living with a rare disease.**

II. Bolstering Biopharmaceutical Innovation

NORD strongly supports efforts to bolster biopharmaceutical innovation. Only five percent of rare diseases have an FDA-approved therapy.[7] We are encouraged by the Senate's interest in this topic in the hope that it will lead to more treatment options for people living with a rare disease.

Basic Research

For the rare disease community, sustained federal investment in basic research is essential to advancing our understanding of rare diseases and creating pathways to treatments and cures. With more than 10,000 known rare diseases and the vast majority still lacking an FDA-approved treatment, patients and families depend on a strong and sustained federal commitment to scientific discovery. Basic research is particularly important in rare diseases, where limited understanding of disease biology, small patient populations, and significant scientific uncertainty can make early-stage research especially challenging.

Scientific breakthroughs often take years, and sometimes decades, to translate into therapies. For persons living with a rare disease, federally funded basic research can provide the scientific foundation necessary to understand the underlying causes and mechanisms of disease, identify potential therapeutic targets, develop biomarkers and other research tools, and ultimately make clinical development possible. Because rare diseases affect very small populations, there may be limited commercial incentives to undertake this foundational work without public investment.

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

The National Institutes of Health (NIH) plays a particularly important role in supporting research that may otherwise never occur. Federal funding enables academic researchers and other investigators to pursue promising scientific questions despite uncertain outcomes and long development timelines. A stronger scientific foundation can create opportunities for companies and other private-sector partners to invest in translating basic science into potential therapies. For patients with rare diseases, this public-private research continuum can mean the difference between a poorly understood disease and an emerging viable pathway toward treatment.

Federal research investment also strengthens the broader rare disease ecosystem. NIH-supported research contributes to scientific discovery, development of the next generation of researchers and clinicians, translational science, natural history studies, and new therapeutic opportunities. It can also provide the scientific knowledge necessary for patient organizations, researchers, and industry to collaborate more effectively in advancing treatments.

NORD urges Congress to provide robust, stable, and predictable federal funding for NIH and other agencies conducting and supporting rare disease research that remains free from political interference. Interruptions or uncertainty in research funding can be particularly damaging for rare disease programs, where there may be a small number of investigators studying a particular condition and limited alternative sources of support. Sustained investment helps preserve scientific expertise, maintain research infrastructure, attract new investigators to rare diseases, and ensure that promising discoveries continue moving toward patients.

Orphan Drug Act and Incentives

NORD has long supported the incentives in the Orphan Drug Act (ODA), including the Orphan Drug Tax Credit and provisions that incentivize innovation by assuring market exclusivity. Incentives that support pioneering research and development that may otherwise not garner commercial commitment are critical. Exclusivity protection helps make development more economically feasible for sponsors and creates some investment certainty, encouraging innovation. By all accounts, the ODA has been a resounding success at spurring the development of rare disease drugs.

Before the Orphan Drug Act was enacted in 1983, fewer than 40 FDA-approved therapies were available to treat rare diseases.[8] Thanks to the ODA, rare disease therapies now consistently account for a substantial number of FDA approvals.[9] Still,

[8] *Orphan Drug Report*, EvaluatePharma 3 (2015) EvaluatePharma+Orphan+Drug+Report+2015.pdf.
[9] *Orphan Drugs in the United States: An Examination of Patents and Orphan Drug Exclusivity*, NORD 5 (2021) NORD-Avalere-Report-2021_FNL-1.pdf.

more than 90% of rare diseases do not have an FDA approved treatment, making continued investment in rare disease research and innovation critical to the rare disease community.[10]

Therefore, any policies that hinder innovation will be detrimental to the millions of patients living with rare diseases.

Clinical Trials

NORD supports efforts to increase clinical trial participation. Designing trials for rare diseases can be challenging due to small patient populations. Incentives to participate in clinical trials may improve the diversity of participants. One incentive, particularly for people living with a rare disease, could be an exemption from H.R. 1 Medicaid community engagement requirements for clinical trial participants. This exemption could preserve access to Medicaid coverage while the beneficiary is participating in a clinical trial.

The CMS Interim Final Rule (IFR) for Medicaid Community Engagement Requirements 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,[11] 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. Community engagement frameworks that fail to account for clinical trial participation risk disrupting access to care and affecting trial enrollment. Patients participating in clinical trials may 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.[12] However, two structural features of the rule limit the utility of this pathway for persons living with a rare disease.

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[10] *Rare Disease Drugs: FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development*, GAO-25-106774 (Nov. 18, 2024).
[11] *Rare Disease Drugs: FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development*, GAO-25-106774 (Nov. 18, 2024).
[12] Section 1902(xx)(3)(B)(ii).

First, the current interim final rule requires states to offer all short-term hardship exemptions or none of them.[13] 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.

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, rare diseases are at risk of being systematically underrepresented on those lists due to ICD-10 coding gaps and eligibility reviewer unfamiliarity.[14] A person with a rare disease that does not appear on their state qualifying list who must travel to a clinical trial site may therefore be unable to access the very exemption designed to protect them.

The most protective approach would be 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 adoption decisions.

Improve Efficiency

NORD supports efforts to make clinical trials more efficient. Systems that enable and recognize data collection from disparate functional settings can expediate the use of real-world clinical research data. For rare diseases, this is particularly important. Equally important to data infrastructure is for FDA to ensure that real-world data, natural history data, and other fit for-purpose methods are considered through a disease-context lens. Furthermore, new technology tools are becoming increasingly available that need further exploration in specific use cases, such as rare disease clinical trials.

NORD believes Congress should support Patient-Focused Drug Design (PFDD). In a recent request for information from FDA, NORD highlighted PFDD as a critical

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[13] 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."
[14] *NORD Rare Disease Database*, NORD List of Rare Diseases | A-Z Database | NORD (last visited August 2026).

framework for ensuring that the perspectives, experiences, and priorities of patients with rare diseases are integrated into drug development and regulatory decision-making. Systematically incorporating patient-reported outcomes, patient preference studies, and insights from PFDD meetings into clinical trial design, endpoint selection, and benefit-risk assessments will strengthen clinical trials and innovations for rare diseases. NORD emphasizes that engaging patients early and consistently helps ensure therapies address what matters most to the rare disease community, improving both the relevance and impact of new treatments.

Expand Access

NORD supports efforts to expand access to clinical trials. Persons living with a rare disease may need access to specialists in other cities or states and, similarly, may need access to clinical trials outside their home communities. NORD also supports efforts to bring clinical trials closer to patients, including those who do not live near large academic institutions. Reimbursement and coverage for remote monitoring and other innovative tools can help reduce the burden of trial participation. Before they are deployed, however, the tools should be validated in an effective and expedient manner.

Thank you for your attention to these important matters. NORD stands ready to work with Congress on behalf of the rare disease community to improve the health and well-being of people living with rare diseases by driving advances in care, research, and policy.

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

Sincerely,

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