August 17th, 2026

Submitted via drugs@finance.senate.gov

The Honorable Ron Wyden
Ranking Member
Senate Finance Committee
United States Senate
219 Dirksen Senate Office Building
Washington, DC 20510

RE: Comments on the Request for Information: Commonsense Policy Options to Lower Drug Prices for Patients

Dear Ranking Member Wyden,

Thank you for the opportunity to submit comments on "Request for Information Commonsense Options to Lower Drug Prices for Patients" (RFI). We appreciate this opportunity to provide commentary on the ideas presented in the RFI as the Medicare model continues to evolve in the aftermath of the Inflation Reduction Act.

The Muscular Dystrophy Association (MDA) is the #1 voluntary health organization in the United States for people living with muscular dystrophy, ALS, and related neuromuscular diseases. For over 75 years, MDA has led the way in accelerating research, advancing care, and advocating for the support of our community. MDA's mission is to empower the people we serve to live longer, more independent lives.

While each disease varies in its progression, affected muscles, and symptoms, and every individual's journey is unique, Neuromuscular diseases (NMDs) generally cause muscle weakness, fatigue, and are progressive, meaning symptoms grow worse over time. Some are present at birth, while others manifest in childhood or adulthood. NMDs are rare, with most caused by changes in one or more genes.

From the outset, we largely echo the comments of our colleagues at the Maprx coalition, however there are some things contemplated by the RFI that have specific impacts on the NMD community that we'd like to comment on.

International Pricing:

While relative pricing between OECD countries and the U.S. is certainly disparate, we have two major concerns when using international models. First, these countries often use value

assessment measures including Quality-Adjusted Life Year (QALY).[1] Too often we see the false dichotomy put forward that a non-discriminatory value assessment will inherently disallow quality-of-life improvements from being considered. This is reductive and false. We have observed value assessment tools using both the QALY and the Equal-Value Life-Years Gained (evLYG) used discriminatorily to justify non-coverage of treatments for muscular dystrophies and other neuromuscular conditions. The QALY discounts an extended life for an individual with a disability compared to a "healthy" individual. While the evLYG does not discriminate in the same manner, it also obtusely ignores quality-of-life improvements altogether. As a practical matter, value assessment measures were banned in the 2024 update to section 504 of the Rehabilitation Act.[2] Even so, given the attacks on this iteration of the 504 Rule,[3] we think it's best to raise these concerns. On a broader level, given international use of these discriminatory measures, it's important to factor these in while considering international pricing models as they relate to U.S. pricing measures.

Second, bother international models and the U.S. have considerable problems with utilization management and delays in access to care.[4] It is important that future policies look to access concerns driven by utilization management which also in and of themselves have an impact on price when considering international models.

Eliminating Blockbuster Bailouts:

The RFI contemplates ending the orphan drug exclusivity in pricing negotiations. While the RFI is correct the orphan drugs are, more often than not, incredibly expensive and that cost should not be passed on to patients. However, we would not want to jeopardize the future research and production of orphan drugs, therefore we would only support changing how orphan drugs are contemplated if other protections are put in its place.

First, if exclusivity is removed it is incredibly important that the loophole closed by the *Orphan Cures Act* (as eventually passed in H.R. 1) remains closed. As a primer, when Catalyst Pharmaceuticals successfully retained exclusivity for Firdapse, an FDA-approved treatment for Lambert-Eaton Myasthenic Syndrome (also a disease that falls under MDA's umbrella), a loophole was opened within the Orphan Drug Act that counters FDA's long-term interpretation and implementation of the statute.[5] Under this decision, the FDA must more strictly interpret the Orphan Drug Act's "same disease" definition, thus handcuffing FDA in designating and subsequently approving therapies for subpopulations of rare diseases.[6] This is problematic for several reasons. First, under the current interpretation, approved orphan products would retain orphan drug exclusivity for the "same disease or condition" no matter the specificity within the approved label. Consequently, under this paradigm, an approved product could only be approved

[1] https://www.ohe.org/insights/how-widely-are-qalys-used-in-oecd-countries-a-snapshot-of-international-practices/
[2] 45 CFR Part 84 § 84.57
[3] *Texas v Kennedy* 5:24-cv-00225-H N. Texas
[4] https://www.kff.org/global-health-policy/health-policy-101-international-comparison-of-health-systems/?entry=table-of-contents-access-to-care
[5] *Catalyst Pharmaceuticals, Inc. v. Becerra*, No. 20-13922 (11th Cir. 2021). This also kicked off a host of other similar cases. *See also, Jazz Pharma v Kennedy 23-cv-01819 DC District and Nurelles v Sara Brenner and AQUESTIVE, 24-cv-1576 DC* as two of the most recent examples.
[6] *Id.*

for a narrow subset of a rare disease, but retain orphan exclusivity for the entire population regardless of the label. This would prevent innovative products that treat subsets of rare disease communities from reaching the market because the FDA has lost the authority to offer approvals and exclusivity to subpopulations of a disease community. As you can see, this exclusivity where the label is concerned is *incredibly* important both in terms of future development and research as well as access.

Assuming the protection of this solution continues, we would offer a few potential guardrails to both lower the financial burden on NMD and rare disease patients while also protecting broad interests in research and development for rare disease therapies. Future policies (or versions of the No Big Blockbuster Bailouts Act, whatever the case may be) should draw the distinction between small-population products such as gene therapies or various small molecule and exon skipping therapies, and products with broad uses or multiple indications. Similarly, research and access could be protected by ensuring that where there is a severe unmet need or there is not an alternative therapy on the market that there is protection in place for those instances.

Two other protective mechanisms the committee should consider are 1) requiring an analysis of likely effects on rare and ultra-rare disease development, and 2) including ongoing monitoring and a mechanism to modify a future policy, if it's found that the policy is disrupting or discouraging research in underserved or rare disease development and research.

Finally, future policy should consider post-market evidence costs. Many therapies for rare diseases need to move through FDA's approval process via accelerated approval which requires study and monitoring post-approval which adds to the cost of therapies and should be considered with regard to negotiation availability.

Part A and B cost caps:

We absolutely support capping costs in Medicare Parts A and B, we would recommend that you take note of the various learnings from the implementation of Medicare Payment Plan Enhancements (see MapRx's response to this RFI) to ensure that access remains robust when applied to these programs.[7] Also we would urge the committee to remember that in Parts A and B negotiations will need to factor in the existence of inpatient costs that exist both separate from and are intertwined with the cost of therapies here.

Biomedical innovation and clinical trials:

Biomedical Innovation: We absolutely support further efforts in public private partnerships and biomedical innovation generally. One need look no further than the ACT for ALS to note the impact of programs like these for those with NMDs. The ACT for ALS, enacted in December 2021, ushered in the exact kind of change the ALS and rare neurodegenerative disease community deserves. Notably, the law successfully expanded access to investigational therapies for those with ALS. According to the Government Accountability Office (GAO), approximately 750 people with ALS have received an investigational therapy due to ACT for ALS funding,

[7] *See also,* MapRx's report on unintended consequences https://maprx.info/wp-content/uploads/2024/08/MAPRx_whitepaper_2024-08-15_Final.pdf

individuals who were otherwise ineligible to participate in clinical trials and other available treatments were inadequate to alter the progression of their disease. This funding also unlocked the research potential of clinics across the United States, including the very first clinics to participate in ALS expanded access efforts in states like Idaho and Iowa. The law also has funded groundbreaking natural history data collection and analysis through the Access for All in ALS (ALL ALS) Consortium, a crucial endeavor for better understanding the risk factors, etiology, and progression of the disease. The ACT for ALS also created and continues to fund the Accelerating Medicines Partnership for Amyotrophic Lateral Sclerosis (AMP ALS) as well as the Critical Path for Rare Neurodegenerative Diseases, two key facets of the HHS Public Private Partnership for Rare Neurodegenerative Diseases created under the law. Both programs seek to speed therapeutic development in ALS and other rare neurodegenerative diseases by accelerating the creation of drug development tools and other approaches to bringing new treatments through clinical trials and to our community.

To continue to see benefits like these future policy should sustain predictable and robust funding for the NIH, provide dedicated and robust support for rare and neuromuscular disease research, expand funding for translational research, support natural history and biomarkers as points of trial (see below), and require patient engagement throughout these processes.

Clinical trials: Access to clinical trials is an incredibly complex issue for the NMD community members. Community members *want* to participate in clinical trials, but there are substantial barriers to participation including financial hardships, travel, caregiving (including their financial hardships to travel with loved ones), and clinical trial designs. To address these concerns (and others), future policy should:

- Require accessible facilities, equipment and communications (again per the updated 504 rule)[8].
- Support transportation, lodging, meals and caregiver reimbursement (which has been supported by OIG changes in stature with regard to sponsors assisting with these costs)[9].
- Permit home health visits and remote assessments where scientifically appropriate.
- Encourage decentralized or hybrid trial models.
- Use local laboratories and health care providers when feasible.
- Provide accessible electronic platforms and plain-language informed-consent materials.
- Include patients in protocol design and selection of outcome measures.
- Use broader, scientifically justified eligibility criteria.
- Support post-trial access policies, shared control groups, platform trials and rare-disease trial networks.
- Ensure trial-related assistance does not jeopardize eligibility for means-tested programs.

[8] https://d3dkdvqff0zqx.cloudfront.net/groups/mda/attachments/11.13.23%20MDA%20Comments%20to%20HHS%20on%20Sec.%20504%20Update.pdf

[9] Prescription settings, https://oig.hhs.gov/documents/advisory-opinions/765/AO-20-02.pdf. Clinical Trials, https://oig.hhs.gov/documents/advisory-opinions/823/AO-21-08.pdf

We are grateful for the opportunity to provide information on this RFI. Rising costs and access are a huge issue for the NMD community, and it is important to note that this response is not exhaustive. For questions regarding MDA or the above comments, please contact me at jcartner@mdausa.org.

Sincerely,

Joel Cartner

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Muscular Dystrophy Association