February 28th, 2024

The Honorable Brett Guthrie, Chairman
House Energy and Commerce Committee
Subcommittee on Health
2434 Rayburn House Office Building
Washington, DC 20515

The Honorable Anna Eshoo, Ranking Member
House Energy and Commerce Committee
Subcommittee on Health
272 Cannon House Office Building
Washington, DC 20515

Dear Chairman Guthrie and Ranking Member Eshoo:

In service of the neuromuscular disease (NMD) patient community, the Muscular Dystrophy Association (MDA) thanks the Energy and Commerce Committee's Subcommittee on Health for convening an impactful hearing on access to care for those with rare diseases. We appreciate the opportunity to provide our viewpoints.

We are grateful for the subcommittee's commitment to improving access to therapies and care for those living with rare diseases. Many of the neuromuscular diseases MDA represents are progressive, and all are rare, meaning access to relatively few effective interventions is vital to allow members of the NMD community to live longer, more independent lives. We ask that members of the subcommittee remember these perspectives as they consider the bills before them today.

MDA is the #1 voluntary health organization in the United States for people living with muscular dystrophy, ALS, and related neuromuscular diseases. For over 70 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.

Below are the specific bills under consideration for which we ask Subcommittee members to support:

H.R. 1092, Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act: MDA is supportive of the BENEFIT Act as it holds the potential to increase the collection, submission, and Food and Drug Administration (FDA or Agency) consideration of patient experience data within neuromuscular disease clinical trials. Too often blunt and archaic functional endpoints are solely considered by the Agency when evaluating the safety and effectiveness of a new product. This legislation, which has advanced in previous Congresses, would require FDA to consider our community's viewpoints when evaluating the risk/benefit ratio of our community and report on what patient experience data was considered in the approval of a therapy.

MDA strongly supports both of these goals that would make clinical trials and the resulting FDA-approved therapy more patient friendly and better targeted to our community, and we ask Subcommittee members to support the legislation.

H.R. 4758, Accelerating Kids Access to Care Act: MDA stands in strong support of the Accelerating Kids Access to Care Act. The bill serves as a common-sense solution to the needs of the many children living with rare diseases who experience delays in receiving care caused by traveling out of state. Children account for, roughly, half of total Medicaid enrollment,[1] and nearly one third of those children have complex medical needs.[2] Unfortunately, there are often an incredibly limited number of clinicians who specialize in a given rare disease. Additionally, as the number of FDA approvals for novel gene therapies grows, and administration of these therapies is incredibly complex and specialized, traveling out of state is often necessary for appropriate care. A 2019 study by the National Organization for Rare Disorders (NORD) found that 39% of respondents traveled more than 60 miles to receive care, which often means crossing state lines and utilizing an out-of-state Medicaid agency or Managed Care Organization.[3]

Given that there is no federal pathway for out-of-state providers to be screened by a child's home Medicaid program, providers are often required to be screened *every* time they see that child. This process can cause delays in treatment, which, given the progressive nature of many NMDs, means the disease progression experienced while waiting for treatment cannot be reversed. MDA supports the Accelerating Kids Access to Care Act's creation of a voluntary pathway to expeditiously enroll providers in out-of-state plans when needed, all without interfering with state Medicaid plans' authority to authorize out-of-state care or negotiate payment.

We thank the Subcommittee for its consideration of this legislation and support is continued progress through the Committee.

H.R. 5547, Maintaining Investments in New Innovation (MINI) Act: We support Congress addressing this technical fix at the intersection of the 21st Century Cures Act and the Inflation Reduction Act (IRA), two bills with provisions that can be transformative for our community. In particular, the IRA used a 21st Century Cures Act definition of anti-sense oligonucleotides (ASOs) (among others) that result in these incredibly complex products being considered small molecule therapies under the IRA.

There are several FDA-approved ASOs for neuromuscular diseases, including Spinraza for spinal muscular atrophy (SMA), Qalsody for an ultra-rare form of hereditary ALS, and more. These are incredibly complex products that have much more in common with complex biologics and gene therapies than small molecules.

Consequently, we appreciate the Subcommittee's consideration of this legislation and support its continued progress through the Committee.

H.R. 5663, **ALS Better Care Act:** MDA stands in support of the ALS Better Care Act. Amyotrophic lateral sclerosis (ALS) is a rapidly progressing neurodegenerative disease that is typically fatal within two to five years after diagnosis. Recognized as a Quality Care Measure as defined by the American Academy of Neurology, the optimal way to care for those living with ALS is through a multidisciplinary approach (e.g. an approach combining neurology, physical and occupational therapy, respiratory, and speech therapy, among other modalities). While this

approach leads to a longer and higher quality of life for those living with ALS, the delivery of this care remains a challenge.

Currently, only the treating physician can bill Medicare for reimbursement; this means that the treating institution eats the cost of the rest of the *vital* care provided by the multidisciplinary care team. This financial paradigm means that fewer hospital systems and providers are willing to take a multidisciplinary approach, which leads to longer wait times and farther to travel for those living with ALS seeking care.

If multidisciplinary care were reimbursed appropriately, not only would it result in lower preventable healthcare costs for patients who could avoid emergency room visits with better access to clinics, shorten wait times for care, and reduce disparities between rural and urban populations by incentivizing more clinics to open and provide a fuller range of services, but also it would allow clinics more time for research to improve multidisciplinary care and offer access to more clinical trials accelerating the timeline to a transformative treatment for ALS.

For these reasons and more, MDA strongly supports the ALS Better Care Act, and we look forward to further Committee consideration of the bill.

H.R. 7383, Retaining Access and Restoring Exclusivity (RARE): 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.[4] 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.

MDA joins many in the rare disease community in believing that this decision not only counters the intent of the Orphan Drug Act, but also is counter to the interests of the public health of the rare disease community. Consequently, we support the RARE Act, and welcome the Subcommittee's attention to it.

H.R. 7384, Creating Hope Reauthorization Act of 2024: The Rare Pediatric Disease Priority Review Voucher (RPD PRV) program has been instrumental in encouraging therapeutic development in challenging pediatric neuromuscular diseases that otherwise may not receive biopharmaceutical attention. Already, several FDA-approved rare neuromuscular disease treatments have received a PRV upon approval, including treatments for Duchenne muscular dystrophy (DMD) and spinal muscular atrophy (SMA). Both these areas, as well as several other ultra-rare pediatric neuromuscular diseases, have seen increases in biopharmaceutical attention since the creation of the RPD PRV program.

MDA has also heard from several small biotechnology companies developing treatments for ultra-rare pediatric neuromuscular diseases that the presence of the voucher upon approval, which they can then sell, is a major incentive for the continued activity in the space. Otherwise, the incentives are still far too inadequate to outweigh the risks of development in ultra-rare

pediatric rare diseases where commercialization, even with high prices, will not be lucrative whatsoever.

We strongly urge the Committee to reauthorize the RPD PRV program prior to its expiration at the end of September, and we are grateful that the Subcommittee is considering this legislation within this hearing.

MDA is committed to ensuring that individuals with neuromuscular diseases and other rare diseases have access to safe and effective therapies and robust access to care. We appreciate this opportunity to provide the Subcommittee with the perspectives of the NMD community. For questions regarding MDA or the above comments, please contact either Paul Melmeyer, Vice President, Public Policy and Advocacy, at pmelmeyer@mdausa.org or Joel Cartner, Director of Access Policy, at jcartner@mdausa.org.

Sincerely,

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