December 6, 2022

The Honorable Nancy Pelosi
1236 Longworth House Office Building
Washington, D.C. 20515

The Honorable Kevin McCarthy
2468 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Chuck Schumer
322 Hart Senate Office Building
Washington, D.C. 20510

The Honorable Mitch McConnell
317 Russell Senate Office Building
Washington, D.C. 20510

Re: NMD Advocacy Groups Call for FDA & Clinical Trial Reforms in End-of-Year Bill

Dear Speaker Pelosi, Minority Leader McCarthy, Majority Leader Schumer, and Minority Leader McConnell,

In service of the neuromuscular disease (NMD) community, the undersigned 23 patient advocacy organizations urge Congress to enact the critical bipartisan reauthorizations and reforms to the Food and Drug Administration (FDA) and clinical trials included in the Food and Drug Amendments of 2022 (H.R.7667) and the FDASLA Act of 2022 (S.4348) but were not enacted

as part of the user fee reauthorizations signed into law in September 2022. These include provisions to make clinical trials more diverse, reform the Accelerated Approval pathway, clarify orphan drug exclusivity, reauthorize the Orphan Product Grants Program and the Humanitarian Device Exemption program, and more.

Neuromuscular diseases are a group of rare diseases defined by progressive muscle weakening. Each NMD is unique in rate of progression, the muscles effected, the onset and severity of symptoms, and the genetic underpinnings (if any). Only a handful of neuromuscular diseases have an FDA-approved treatment, and for those that do, often the treatments are targeted to a narrow subpopulation of the condition, and/or the treatments may not substantially alter the course of the disease. Innovative treatment development through clinical trials and eventual FDA-approval are critically important to the community. Consequently, Congress's ability improve the process by which clinical trials are conducted and FDA regulatorily considers the approval of new products is salient and of great interest to the NMD community.

First, we ask that Congress enact legislation to make clinical trials more diverse and representative of the patient populations hoping to subsequently access the treatment upon FDA-approval. Such reforms are included in Title V of the House-passed H.R.7667. All individuals with a neuromuscular disease, no matter their gender, race, age, or ability should have the opportunity to participate in clinical trials, and we hope these reforms will lead to greater participation of individuals historically locked out of participating in clinical trials. Within the NMD community, not only have clinical trials historically inequitably included minority populations and women, but to this day, older individuals with NMDs and non-ambulatory patients continue to often be excluded. We join the call for reforming clinical trials to make them more diverse and inclusive.

Second, several therapies for neuromuscular diseases have been approved by the FDA via the accelerated approval pathway, and we anticipate many more in the future. We support the accelerated approval pathway reforms included in section 506 of S.4348 and section 804 of H.R.7667 as the refinements to the pathway, including the timing and conduct of post-approval confirmatory studies, are warranted. Moreover, we do not anticipate these provisions to deleteriously effect the utility of the accelerated approval pathway as other policy proposals would have achieved. These reforms strike the right balance, and we support their passage.

Third, clarifying which therapies qualify for orphan drug exclusivity under the Orphan Drug Act, as outlined in section 812 of H.R.7667 and section 510 of S.4348, is crucial to ensuring incentives remain for the development of innovative new treatments for rare neuromuscular diseases. The need for this language stems from a recently decided court case involving therapies for Lambert-Eaton Myasthenic Syndrome (LEMS), a rare neuromuscular disease. Clearly resolving this issue is of great importance to our community.

Finally, the authorization for several FDA programs, including the Orphan Product Grants Program, the Humanitarian Device Exemption program, and the Pediatric Device Consortium, is set to expire this December if not extended by Congress. We ask that Congress include a full reauthorization of these programs to align with the user fee reauthorization dates to ensure these FDA efforts continue to support the rare neuromuscular disease community.

Thank you for your consideration of our requests. If you have any questions or desire additional information, please do not hesitate to contact Paul Melmeyer, Vice President, Public Policy and Advocacy at the Muscular Dystrophy Association at pmelmeyer@mdausa.org or 202-253-2980.

Sincerely,

Acid Maltase Deficiency Association
ALS Association
Answer ALS Foundation
Charcot-Marie-Tooth Association
CMT Research Foundation
CureDuchenne
CureLGMD2i
Cure CMD
Cure VCP Disease
Friedreich's Ataxia Research Alliance (FARA)
FSHD Society
Hereditary Neuropathy Foundation
Les Turner ALS Foundation
LGMD2i Research Fund
LGMD Awareness Foundation
Muscular Dystrophy Association
The Myositis Association
National Ataxia Foundation
Neuromuscular Disease Foundation
The Speak Foundation
Team Gleason
Team Titin, Inc
United Mitochondrial Disease Foundation

cc: Congressman Frank Pallone, Chairman, House Committee on Energy and Commerce
Congresswoman Cathy McMorris-Rodgers, Ranking Member, House Committee on Energy and Commerce
Senator Patty Murray, Chairwoman, Senate Committee on Health, Education, Labor, and Pensions
Senator Richard Burr, Ranking Member, Senate Committee on Health, Education, Labor, and Pensions