



July 13, 2021

The Honorable Nancy Pelosi
Speaker
United States House of Representatives
Washington, DC 20515

The Honorable Kevin McCarthy
Minority Leader
United States House of Representatives
Washington, DC 20515

The Honorable Frank Pallone
Chairman
House Committee on Energy and Commerce
Washington, DC 20515

The Honorable Cathy McMorris-Rodgers
Ranking Member
House Committee on Energy and Commerce
Washington, DC 20515

Re: Neuromuscular Disease Community Calls for Passage of ACT for ALS (H.R. 3537)

Dear Speaker Pelosi, Leader McCarthy, Chairman Pallone, and Ranking Member McMorris-Rodgers:

In service of the approximately 300,000 Americans living with a neuromuscular disease, the undersigned twenty patient advocacy organizations call on the House of Representatives to pass the ACT for ALS Act (H.R.3537) as soon as possible, and no later than the end of this year. The ACT for ALS could substantially accelerate therapeutic development for the neuromuscular disease community, a community that far too often lacks an FDA-approved treatment to change the course of the disease. With over 270 Congressmembers cosponsoring the legislation, we call on the Energy and Commerce Committee to move the legislation as soon as possible.

The ACT for ALS would create the “HHS Public-Private Partnership for Rare Neurodegenerative Diseases” with the intention of convening drug development stakeholders in rare neurodegenerative diseases to better coordinate efforts. Such coordination is sorely needed

to better develop validated biomarkers and surrogate endpoints, innovative clinical outcome assessments and trial endpoints, and progressive designs for clinical trials in heterogeneous neuromuscular diseases.

This legislation also commissions an action plan from the Food and Drug Administration (FDA) on the ways in which the Agency will advance therapeutic development, regulatory science, and policy dissemination all to accelerate drug development in rare neurodegenerative diseases. This blueprint will not only chart the way forward for FDA, but also for the community in how best to further advance rare neurodegenerative disease drug development.

Finally, the ACT for ALS would create two new grants program both with the goal of accelerating development and access to promising new therapies. The “FDA Rare Neurodegenerative Disease Grant Program” would contribute tens of millions of dollars per year to innovative therapeutic development ongoing within rare neurodegenerative diseases, funding that is often lacking due to the small size of rare neuromuscular disease populations. The legislation would also create a grants program to fund expanded access programs from small biopharmaceutical companies developing therapies for ALS.

In conclusion, the ACT for ALS could substantially accelerate neuromuscular disease drug development through a combination of greater coordination, collaboration, and funding. We stand firmly behind the ACT for ALS and ask that you pass this legislation out of Committee, and then out of the full House, as soon as possible. For questions, please contact Paul Melmeyer of the Muscular Dystrophy Association at pmelmeyer@mdausa.org.

Sincerely,

ALS Association
Answer ALS
Charcot Marie Tooth Association
CMT Research Foundation
Coalition to Cure Calpain 3
CureLGMD2i Foundation
Cure VCP Disease, Inc.
Friedreich's Ataxia Research Alliance (FARA)
FSHD Society
Hereditary Neuropathy Foundation
I AM ALS
Les Turner ALS Foundation
LGMD Awareness Foundation
Muscular Dystrophy Association
The Myositis Association
Myotonic Dystrophy Foundation
National Ataxia Foundation
The Speak Foundation
Team Gleason
United Mitochondrial Disease Foundation

CC: The Honorable Steny Hoyer, Majority Leader, House of Representatives
The Honorable Steve Scalise, Minority Whip, House of Representatives
The Honorable Anna Eshoo, Chairwoman, House Committee on Energy and Commerce,
Subcommittee on Health
The Honorable Brett Guthrie, Ranking Member, House Committee on Energy and
Commerce, Subcommittee on Health