



MDA Is Encouraged by Recently-Introduced Legislation, Will Work to Ensure Passage of Meaningful Reforms and Programs

June 18, 2020, Washington, DC --- Within the last month, two bills conceived and championed by patient organizations and patient leaders in the amyotrophic lateral sclerosis (ALS) community were introduced: the Accelerating Access to Critical Therapies for ALS Act (H.R.7071, also known as the ACT for ALS Act), and the Promising Pathway Act (PPA) (S.3872). Both bills are intended to accelerate access to products currently in clinical trials through an expanded access program (EAP) or Food and Drug Administration (FDA) provisional approval, among other goals.

First, we must start with our gratitude to everyone in the community who has worked tirelessly to push for innovative policies to accelerate access to promising therapies for the ALS and neuromuscular disease community. Without the many determined advocates working with our partner advocacy organizations, or simply from their kitchen table, we would not be as hopeful for a better future for the ALS community as we are today. We are also grateful for Senator Braun, Congressmen Fortenberry and Quigley, and all co-sponsors for prioritizing the ALS and neuromuscular community by leading the Congressional effort on these bills.

We are excited by the direction and goals of the ACT for ALS Act as this legislation aims to tackle one of the most pervasive problems in accessing promising pre-approval therapies. In our [May 27th statement on ALS clinical trial design](#), we asked for, “Innovative financing mechanisms to overcome the financial disincentives associated with including expanded access programs and open label extensions within ALS clinical trials, including ongoing late-stage or Phase III clinical trials.” By authorizing \$450 million over four years for a funded expanded access and open-label extension grant program, this is precisely what this bill aims to do.

Additionally, the ACT for ALS legislation will create a Center of Excellence for Neurodegenerative Diseases at the FDA modeled after the existing FDA Oncology Center of Excellence. If constructed in the right way, and with the FDA on board, this Center of Excellence could better coordinate efforts to develop and approve medical products for the neuromuscular disease community. Furthermore, this Center of Excellence could build upon the success of the [Oncology Center of Excellence’s Project Facilitate](#) in [better coordinating expanded access efforts for those in the community](#).

The Muscular Dystrophy Association (MDA) is committed to ensuring the very best version of the ACT for ALS Act is enacted into law as soon as possible, including ensuring the legislation maintains our momentum towards a cure for ALS, as well as ensuring the Center of Excellence provides impactful change within the FDA to better coordinate neurodegenerative disease efforts. We will work with patient leaders, patient advocacy group partners, Congressional champions, and the FDA to achieve this goal.

We are similarly committed to ensuring the Promising Pathway Act, a bill that constructs a provisional approval pathway at the FDA for promising therapies, including those for the neuromuscular disease community, will provide a meaningful acceleration of access to promising therapies. Advocates rightly argue that current expedited approval pathways at FDA have not accelerated access to innovative therapies for neuromuscular diseases, but particularly ALS, nearly quickly enough. The accelerated approval pathway has not been utilized for ALS due to the lack of an established biomarker for use as a surrogate endpoint, and the remaining pathways have still resulted in development timelines stretching over many years. This is clearly inadequate for those living on the ALS clock.

The current pace of ALS therapeutic development is unacceptably slow, and MDA is committed to working with the community to pursue solutions that would definitively accelerate this process. Constructing a brand new FDA approval pathway is incredibly complex, and we will continue to partner closely with Senator Braun's office and partnering Congressmembers to ensure such a pathway will succeed in accelerating access to promising therapies for those with ALS and other neuromuscular diseases.

It is a privilege to partner with so many amazing advocates in the ALS and neuromuscular disease communities, and we are eager to make a difference together.

For questions on this statement, please contact MDA's Advocacy Department at advocacy@mdausa.org.