HR 7071: Accelerating Access to Critical Therapies for ALS (ACT for ALS) Act:
A bill to speed patient access to promising therapies.

Summary
HR 7071 increases the opportunity for patients to access investigational therapies outside of clinical trials, patients who may not live long enough to benefit from a drug eventually being approved, and creates a new center of excellence at the U.S. Food and Drug Administration (FDA) to streamline review and approval of effective therapies to treat neurodegenerative diseases.

- ACT for ALS will provide resources through the National Institutes of Health (NIH) to fund expanded access programs for therapies in development from small biopharmaceutical companies, increasing the opportunity for people with terminal, currently untreatable, diseases to access promising therapies.
- ACT for ALS establishes a Center of Excellence for Neurodegenerative Diseases within the FDA to accelerate the development and approval of therapies for devastating neurodegenerative diseases.

Rationale
There are people living in the United States today who have been diagnosed with a terminal disease and told that there is nothing anyone can do to help. That is the case for those living with amyotrophic lateral sclerosis (ALS). Yet, there is a rich pipeline of possibly effective treatments under preclinical or clinical study that exist out of reach for the vast majority of ALS patients who don’t know about, make it into, or are excluded from a clinical trial. For people living with a terminal disease with no FDA-approved treatment that provides hope for a longer life, there are few options and potential great opportunities in investigational therapies that are a lifetime, for them, away from being approved.

1 in 15 adults in the developed world will be diagnosed with a neurological disease in their lifetimes. In the coming decades, with a growing aging population, this healthcare crisis will only continue to worsen. The impact of neurodegenerative diseases will be felt broadly, if we don’t find answers. The science of a number of neurodegenerative diseases is linked and, likely, so are their cures. By consolidating the relevant expertise and processes at the FDA for the development and evaluation of therapies for neurodegenerative diseases, we can expedite their review and approval and advance our broad understanding of how to develop treatments and cures, as has been done with the Oncology Center of Excellence.
Legislative Components

Section 2: Grants for Rapid Development of Therapies for ALS and Other Rapidly Progressing Neurodegenerative Diseases

- The Secretary of Health and Human Services (HHS) shall award grants to eligible entities for the provision of investigational drugs through an expanded access program for individuals for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis or another rapidly progressing neurodegenerative disease.

- The Secretary of HHS through the NIH Director and FDA Administrator must make a determination on a submitted grant application and on the legitimacy of an expanded access program, respectively, within 60 days of a grant submission to this program. The Secretary may also vest the authority to determine the validity of an expanded access program with a clinical trial site or sites.

- Eligible entities include drug sponsors that meet the small business concern criteria as defined in the Small Business Act or a phase 2 or phase 3 clinical trial site.

- There is $75,000,000 authorized for appropriations for each of fiscal years 2021 and 2022; and $150,000,000 for each of fiscal years 2023 and 2024.

- The Foundation for the National Institutes of Health (FNIH) may solicit and accept gifts, grants, and other donations, establish accounts, and invest and expend funds in support of this program.

- Not later than 18 months after the date of the enactment the Secretary of HHS shall convene an independent review panel that includes representatives of patients, researchers, drug sponsors, and government agencies; and the independent review panel shall submit to Congress a report on their findings and conclusions with respect to the design and implementation of the program for 2023 and 2024.

Section 3: FDA Center of Excellence for Neurodegenerative Diseases

- By September 2021, the Secretary shall establish within the FDA a center of excellence, to be known as the Center of Excellence for Neurodegenerative Diseases.

- The Center of Excellence for Neurodegenerative Diseases will have duties and authorities similar to those of the Center of Excellence for Oncology as defined in the 21st Century Cures Act, including the duties and authorities with respect to Project Facilitate, which provides a single point of contact where FDA oncology staff will help physicians and their healthcare team through the process to submit an Expanded Access request for an individual patient with cancer.

About ALS

Amyotrophic lateral sclerosis, or ALS, is a progressive neurodegenerative disease. ALS attacks cells in the brain and spinal cord that are needed to keep muscles moving, leading to muscle weakness and paralysis. It makes the brain stop talking to the muscles, causing increased paralysis over time. Ultimately, ALS patients become prisoners within their own bodies, unable to eat, breathe, or move on their own and most people don’t live more than 2-5 years from diagnosis.