H.R.8662 and S.4867: Accelerating Access to Critical Therapies for ALS (ACT for ALS) Act:

A bill to speed patient access to promising therapies.

Summary

H.R.8662 and S.4867 increase 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 takes efforts to enrich the development and streamline the review and approval of effective therapies to treat neurodegenerative diseases. Specifically, the bill:

- Creates a grant program that funds research on and access to investigational ALS treatments being tested from small biopharmaceutical companies to patients not participating in the clinical trial.
- Establishes an HHS Collaborative for Neurodegenerative Diseases jointly led by the FDA and the National Institutes of Health (NIH), the first federal effort explicitly charged with the responsibility to speed the development and approval of therapies for neurodegenerative diseases.
- Develops an FDA grant program which funds research on and development of interventions to prevent, diagnose, treat or cure ALS and other life-threatening or severely debilitating 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 streamlining the relevant expertise and processes at the FDA and NIH for the development and evaluation of therapies for neurodegenerative diseases, we can expedite their review and approval and advance our understanding of how to deliver effective treatments and cures.

Bill Components

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 purposes of supporting research on, and expanded access for individuals to investigational drugs for the prevention, diagnosis, mitigation, treatment or cure of ALS.
- Eligible entities include participating clinical trial site or sites conducting a phase 3 study sponsored by a small business concern as defined in the Small Business Act.

HHS Collaborative for Neurodegenerative Diseases

The Collaborative is charged with coordinating efforts across the public and private sector to accelerate therapeutic development and regulatory review of treatments and cures for ALS and neurodegenerative diseases, including:

- Coordination among the centers of the Food and Drug Administration to achieve the goals specified in the draft guidance entitled 'Amyotrophic Lateral Sclerosis: Developing Drugs for Treatment Guidance for Industry' published September 2019;
- Facilitation of access to investigational drugs for ALS;
- Definition or development of the regulatory and translational pathway for emerging therapeutic categories; and
- Development and implementation of an ongoing mechanism to share feedback and information and develop strategies with the neurodegenerative disease community, including patients, treating physicians, national organizations that facilitate provision of care services, access, research, researchers, drug sponsors, drug manufacturers and federal agencies.

Rare and Neurodegenerative Disease Grant Program

The Commissioner of Food and Drugs shall carry out a program of awarding grants to, and contracts entered into with, public and private entities to cover the costs of research on and development of interventions intended to prevent, diagnose, mitigate, treat or cure ALS and other life-threatening or severely debilitating neurodegenerative diseases.

Appropriations

For purposes of carrying out this Act, there are authorized to be appropriated $100,000,000 for each of fiscal year 2022 through 2026.

About ALS

ALS is a disease that attacks cells in the body that control movement. 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, talk, breathe or move on their own. Their mind, however, often remains sharp so they are aware of what's happening to them. ALS will affect 1 in 300 people in our lifetimes, and patients usually have no more than 2-5 years to live following diagnosis.

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