Accelerating Access to Critical Therapies for ALS (ACT for ALS) Act: H.R.3537 + S.1813

A bill to speed patient access to promising therapies.

Summary

ACT for ALS 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 takes efforts to enrich the development and streamline the review and approval of effective therapies to treat neurodegenerative diseases.

The Accelerating Access to Critical Therapies for ALS Act will make $100,000,000 available each fiscal year from 2022-2026 to build new pathways to fund early access to ALS investigational therapies, accelerate ALS and neurodegenerative disease therapy development through a public-private partnership, and increase research on and development of interventions for rare neurodegenerative diseases through a new Food and Drug Administration (FDA) research grants program.

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 proven safe 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 HHS, 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.

Contact to Support

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Bill Components

- **GRANTS FOR RESEARCH ON THERAPIES FOR ALS** - creates a new grant program that funds access to investigational ALS treatments currently in development from small biotechnology companies for those patients who cannot participate in the clinical trial, while supporting research on how these investigational treatments impact the disease;
- **HHS PUBLIC-PRIVATE PARTNERSHIP FOR RARE NEURODEGENERATIVE DISEASES** - establishes an HHS Public-Private Partnership for Rare Neurodegenerative Diseases to advance the understanding of rare neurodegenerative diseases and foster the effective development and evaluation of treatments;
- **ALS AND OTHER RARE NEURODEGENERATIVE DISEASE ACTION PLAN** - commissions the publication of an FDA Action Plan to support drugs that improve and extend the lives of people as quickly as possible and facilitate access to investigational drugs for those living with amyotrophic lateral sclerosis and other rare neurodegenerative diseases.; and
- **FDA RARE NEURODEGENERATIVE DISEASE GRANT PROGRAM** - implements an FDA grant program to fund research and therapy development for ALS and other life-threatening or severely debilitating rare neurodegenerative diseases.

Appropriations

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