A Request from ALS Experts to Pass the *Accelerating Access to Critical Therapies (ACT) for ALS*

My name is Dr. Jinsy Andrews and I have been caring for people with Amyotrophic Lateral Sclerosis (ALS) and together with my undersigned colleagues, we have the collective experience of conducting clinical research in ALS for over 35 years. ALS is a neurodegenerative disease that causes progressive loss of voluntary muscles that help you walk, use your arms to perform activities of daily living, speak, swallow and breath. ALS is fatal, with most people living an average of 3-5 years from their first symptom. We now have treatments that improve quality of life, but no treatments that significantly slow disability or prolong survival. Clinical trials offer hope, but most people with ALS will never find a trial they can participate in. Given their grim prognosis, lack of definitive treatments, and inability to find trials, many patients "self-experiment" with alternative and off label treatments they find on the Internet. In these endeavors, they suffer physical, psychological and financial harms. Since we do not learn anything from these endeavors, our entire field suffers scientific harm.

A critical component to possible management options and furthering our knowledge of the disease that can help lead us to a cure is the passing of the ***Accelerating Access to Critical Therapies (ACT) for ALS*.**

In 2021 leaders in the House of Representatives (HR.3537) and Senate (S.1813) reintroduced ***ACT for ALS.*** **This important legislation builds new pathways to fund early access to ALS investigational therapies, accelerates ALS and rare neurodegenerative disease therapeutic development through a public-private partnership, and increases research on, and development of, interventions for rare neurodegenerative diseases through a new Food and Drug Administration (FDA) research grant program.**

Here are just some of the grim statistics as to why ***ACT for ALS*** should be passed:

- ALS is progressive, meaning the symptoms get worse over time. **Currently, there is no cure for ALS and no effective treatment to halt or reverse the progression of the disease.** (1)
- **ALS is almost 100% fatal.** Most people with ALS die from respiratory failure, usually within 3 to 5 years from when the symptoms first appear. (1)
- Approximately 5,000 people in the US are diagnosed annually, with military veterans about 1.5 to 2 times more likely to develop ALS. (1,2) Anyone is at risk of developing ALS. **This translates into a lifetime risk of 1 in every 300 people.** (3)

ACT for ALS would give people with ALS, caregivers, family, and friends much needed hope. This bill will authorize monies be spent over a five-year period to:

1. create 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 clinical trials, while concurrently supporting a research objective on how these investigational treatments impact the disease;
2. establish a Health and Human Services (HHS) Public-Private Partnership for Rare Neurodegenerative Diseases between the National Institutes of Health (NIH), the FDA, and eligible stakeholders with a connection to the patient population(s) to advance the understanding of rare neurodegenerative diseases and foster the effective development and evaluation of treatments;
3. implement a Food and Drug Administration (FDA) grant program to fund research and therapy development for ALS and other life-threatening or severely debilitating rare neurodegenerative diseases; and
4. commission 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 ALS and other rare neurodegenerative diseases.

ACT for ALS has bipartisan support in Congress. The leading ALS organizations including The ALS Association, I AM ALS, and the Muscular Dystrophy Association endorse this legislation.

The undersigned ALS providers, practitioners, scientists, researchers, administrative staff and other experts support passage of *ACT for ALS* and ask Congress to pass and enact this critical legislation in 2021.

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