

Committee on Energy and Commerce
Opening Statement as Prepared for Delivery
of
Chairman Frank Pallone, Jr.

Hearing on “The Path Forward: Advancing Treatments and Cures for Neurodegenerative Diseases”

July 29, 2021

Our understanding of the human brain has rapidly increased in recent years thanks to advancements in science and research. Yet neurodegenerative diseases and their causes continue to be a mystery in many ways. Few treatments for the symptoms of neurodegenerative diseases exist, and there are no known cures that significantly slow or eliminate disease progression. As a result, millions of Americans and their families face the heartbreaking daily challenges that come with a neurodegenerative disease.

Congress and this Committee in recent years have supported substantial investments in neurodegenerative disease research and flexibilities for clinical research.

In 2016, we passed the 21st Century Cures Act, which authorized over \$1.5 billion to support the National Institutes of Health’s (NIH) Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative or the BRAIN Initiative. This Initiative’s mission is to revolutionize our understanding of the human brain and discover new ways to treat, cure, and prevent brain disorders, including neurodegenerative diseases. By accelerating the development of novel technologies to map a new picture and understanding of the brain, the BRAIN Initiative is providing a revolutionary foundation for future research and clinical development. This work will be augmented by the Advanced Research Projects Agency for Health, or ARPA-H, proposed by President Biden.

The Food and Drug Administration (FDA) also plays a key role. The agency is responsible for the safety and efficacy of all drugs and treatments in development, including those to treat brain disorders. FDA also provides guidance to industry on clinical trial design, meaningful endpoint considerations to determine whether a treatment is beneficial, and market approval. It also works with physicians and patients when treatment options may be unavailable.

Through both 21st Century Cures and the FDA Reauthorization Act, this Committee has encouraged greater guidance on the use of novel clinical trials and the inclusion of patients in the drug development process.

These are all promising steps, but it is clear that more must be done to support the discovery and development of safe and effective treatments and cures, and to provide quality, affordable, and equitable care to patients and their families.

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In order to protect patients, caretakers, and the American public, it is important that we understand the current state of science for neurodegenerative diseases and how we can further improve access to clinical trials and the development of potential treatments or cures.

It is our responsibility to provide federal agencies with the necessary resources.

On our first panel, we will hear from government representatives at the FDA, National Institute on Aging, and National Institutes of Neurological Disorders and Stroke. I am particularly interested in hearing about the progress that has been made since the passage of both the 21st Century Cures Act and the FDA Reauthorization Act in 2017, how these programs are affecting clinical research and drug development, and what more needs to be done.

On our second panel, we will hear from researchers, industry, patients, and caretakers. Their experiences are critical to our work here today.

Patients and their caretakers live the physical and emotional symptoms of these diseases every day, including the arduous process of enrolling and participating in a clinical trial and searching for available treatments. These are our neighbors, friends, and for many of us, family.

I also look forward to hearing from those on the cutting edge of research into ALS and neurodegenerative diseases, who have conducted clinical trials and treated patients with the disease. We will also hear from industry about therapies in the pipeline and the challenges manufacturers face in developing treatments for neurodegenerative diseases.

I thank all of the witnesses for appearing here today, and I yield back the balance of my time.