Congressman Jeff Fortenberry, First District of Nebraska

Hearing Testimony on Amyotrophic Lateral Sclerosis

House Energy and Commerce Subcommittee on Health

July 29, 2021

The US Government's approach to Amyotrophic Lateral Sclerosis (ALS) is not working for patients dying from the disease.

Our regulatory process is not matching the urgency of these diseases, which, in the case of ALS, takes about two to five years to destroy a body and exhaust a family. Drug trials can drag on a decade or more. Most ALS sufferers do not live nearly that long.

This insight was validated by University of Massachusetts' Dr. Robert Brown, an internationally recognized ALS clinician and researcher: He stated, "the FDA is far more restrictive with treatments for neurodegenerative diseases like ALS than those for HIV or cancer."

The Food and Drug Administration (FDA) needs to recognize this fact. It also must be more consistent in its regulatory action and communications related to drugs for neurodegenerative disease. For one illness a drug with limited benefit is approved. For another drug for ALS that seems to benefit a subset of patients, the FDA will not even consider it. The FDA has also made statements criticizing the right of scientists and a company to more fully analyze its data to determine which patients might uniquely benefit from the drug.

The FDA recently commented on criticism of its approval of a drug for Alzheimer's Disease. They are quoted as saying that it matters that patients are willing to accept risk, specifically, "Their willingness to accept some uncertainty about clinical benefit to get earlier access to a potentially valuable drug" must be part of the standard. Why not the same standard for ALS?

ACT for ALS calls on FDA to build a partnership with others to create consistency across neurodegenerative diseases. It also funds the FDA to accelerate research through a Rare Neurodegenerative Disease Grants Program. This is a chance at real progress to align FDA guidance with FDA action.

The second part of ACT for ALS is to assure that patients in a clinical trial have access to a drug that may be saving the lives of some, but not the ones in the placebo arm of the study. Many new drugs for ALS are made by small biopharmaceutical companies. These companies are stretched just to cover the demands of a trial. If a new treatment is showing promise, ACT for ALS allows the National Institutes of Health to work with FDA to assure that patients in a trial

and also ones not in the trial can have access to the drugs that are showing promise. This is what Joe wanted for himself and he wanted it for other patients.

We have a chance to transform the relationship that our US government has with patients with ALS. It is a chance to be scientifically sound and humane. Through an expanded access program between FDA and NIH defined by ACT for ALS we can make it right for ALS patients who volunteer for trials and for those who never have a chance to enter a trial. We have a chance to learn more about benefits to patients who don't qualify for a trial and we have a chance to ask questions not required for a drug to gain approval. This path will accelerate knowledge, give patients today hope, and assure that each patient has the best chance to benefit from what science can offer today. Act for ALS is an important step forward.

The FDA must exercise regulatory flexibility to accommodate incremental progress which can be monumental for diseases like ALS. The current risk-averse and inflexible regulatory system sometimes fails to understand complex diseases like ALS.

In September 2019, FDA released the Amyotrophic Lateral Sclerosis: Developing Drugs for Treatment Guidance for Industry | FDA. We believed this to be a step forward in modernizing the development of ALS therapies. Within this guidance, FDA reiterated their support for exercising regulatory flexibility when applying statutory standards to drugs for diseases with serious unmet medical needs. Yet today it is hard to see what has changed in the FDA actions. Some ALS clinical trial designs remain a problem. Trials continue to be designed with placebo only arms without open label extension, so patients do not get a chance to try the drug. They are left in despair without hope.

I am particularly moved by this outpouring of Congressional support. ACT for ALS now has 306 cosponsors. My own family was cast into the same trauma suffered by so many Americans. At 37, with 4 young children, my brother-in-law, Joe Gregory died waiting for access to a promising ALS treatment in development.

Thank you for holding this important hearing.