Committee on Energy and Commerce

Opening Statement as Prepared for Delivery
of
Subcommittee on Health Chairwoman Anna G. Eshoo

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

July 29, 2021

I called for today's hearing to discuss the challenge of advancing treatments and cures for neurodegenerative diseases.

My constituent, Jamie Berry, was diagnosed with ALS one year ago. She wrote a letter to me saying that "With ALS, a piece of you dies every single day. We are simply asking for a fighting chance to live the lives we were meant to live."

Today, we will hear from four patients and caregivers who, like Jamie, are simply asking for a "fighting chance" against the neurodegenerative diseases that have afflicted their families. Brian and Sandra, Kala and Yvonne, thank you, especially for traveling across the country, to offer your testimony to us. It was a difficult journey that you've made and we thank you for being profiles of courage and being here to offer that testimony.

Our work today is to help create the fighting chance against these deadly diseases.

According to the National Institute of Neurological Disorders, each year 50 million Americans are affected by neurological disorders such as ALS, Alzheimer's, Huntington's, and Parkinson's. These diseases exact an enormous personal toll and cost the U.S. economy as much as $800 billion dollars annually.

Despite the prevalence, the deaths, and the heartrending impact on families across the country, there are few effective treatments for neurological disorders.

Lack of investment, difficult drug approval processes, and limited understanding of these extremely heterogenous diseases all keep effective drugs off the market.

Private companies invest one-fourth as much into neurological drugs as they do for oncology treatments. Only 7.9% of drugs for neurological disorders successfully make it from Phase 1 to approval. When they are successful, neurologic drugs take, on average, 57% longer to reach approval than drugs for other disease areas.

There have been recent breakthroughs in understanding the genetic basis of the diseases and potential biomarkers, but this has yet to translate into effective treatments. For patients, a diagnosis is still a death sentence.

I think every Member of our Committee has heard from ALS patients fed up with their lack of options. Two drugs, AMX0035 and Nurown, have captured attention and sparked a debate over whether the potential benefits of the drugs outweigh the risks.

Everyone here shares the same goal: Full approval for effective drugs. But the question before us stills stand: How do we best get there?

An obvious first choice is full funding to the FDA to ensure they're completely staffed and working at capacity. We made progress with a House appropriations bill that increases the FDA's budget by nearly $250 million, but I'm still interested in hearing from FDA about what more should be done to support their important mission.

Second, we need better multidiscipline coordination between FDA, NIH, academic researchers, private drug companies, and patients. Breakthroughs in cancer and HIV came from a better understanding of the basic science of the diseases, but also through better collaboration, data-sharing, and a coordinated strategy. These efforts will bring the breakthroughs from the bench to bedside.

That's why I'm pleased to be working with the Biden-Harris Administration to create the Advanced Research Projects Agency for Health, ARPA-H. This new, independent agency will take on projects like Alzheimer's and ALS where the market has failed to invest due to risk and bring new strategies and collaborations to our current siloed system.

Finally, there needs to be clarity and transparency about the standards for approval for deadly diseases with unmet medical needs. A promise of "flexibility" rings hollow when it is undefined.

These challenges are not insurmountable. These diseases are not incurable. We can provide, as my constituent said, the fighting chance for patients to live the lives they were meant to live.

That's our work today and for the days and years ahead.