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Representing Herself, The ALS Association, and Columbia University

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Committee on Energy and Commerce Hearing on The Path Forward: Advancing Treatments and Cures for Neurodegenerative Diseases

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Chairs Pallone and Eshoo, Ranking Members McMorris Rodgers and Guthrie, members of the Committee: thank you for the opportunity to testify on the challenges and opportunities of ALS research and development today. My name is Dr. Jinsy Andrews, and I am currently the Director of Neuromuscular Clinical Trials at Columbia University and a volunteer board of trustee member for the ALS Association. As part of truth in testimony, I also receive NIH funding for clinical trials via the NeuriNEXT pathway and I serve as a part-time staff physician at the James J. Peters VA hospital to care for veterans with ALS.

I am here today to ask for your help because ALS research and development is not moving fast enough. The ALS community needs your help to ensure that the FDA accelerates drug development, approval, and access to effective new ALS treatments. Congress should urge the FDA to use their existing regulatory authority to move much more quickly on ALS drugs. In addition, The ALS Association supports passage of the Accelerating Access to Critical Therapies (ACT) for ALS Act (H.R. 3537), which offers opportunities for expanded access; new funding for impactful research; and creating a new FDA conditional approval pathway modeled on the successful experience of the European Union with its conditional marketing authority.

I have been caring for people living with ALS and conducting clinical trials for over 15 years in both an academic and industry setting. In 2013, I attended a public hearing for ALS held by the FDA where many people living with ALS and their caregivers provided testimony sharing that they were

willing to take higher risks, that time was a luxury that they didn't have, that they had limited access to investigational therapies through clinical trials or expanded access. Many of those people with ALS, including those that I cared for, have since died without seeing much change.

Eight years later, on May 25, 2021, people with ALS again made the same case to the FDA in a virtual meeting on the urgent need for expedited access to experimental therapies. In addition, more than 150 people sent testimony to the FDA. For more information on this meeting, please go to the "We Can't Wait Action Meeting" on The ALS Association website.

People with ALS urgently need effective new treatments. The FDA must be fully funded, fully staffed and provided the regulatory authority they need to be fast, effective, and transparent on ALS trials and drug approvals. Making approval decisions about promising treatments is never easy, especially when it comes to fatal diseases. But year after year, people with ALS have made it clear to FDA and Congress that they are willing to accept greater risks, that anything that retains function and provides more time is meaningful, and that access to new treatments – including prior to FDA approval – is a top priority because there is no cure for ALS. In summary, if any new treatment is shown to be effective and safe, the FDA has an obligation to side with people who are suffering.

There are also opportunities for manufacturers to make ALS clinical trials more efficient, impactful, and respectful. In the ALS space, expanded access to experimental therapies is often not provided by the pharmaceutical companies because they are small and have less resources. In contrast, big pharmaceutical companies that can afford to provide expanded access do not invest as much in ALS until it is de-risked. All clinical trials should be accessible to people with ALS by including open label extensions and expanded access, which designed appropriately can collect additional data regarding safety and tolerability.

Additionally, I want to emphasize that providing expanded access to patients outside of clinical trials can be accomplished without impeding completion of clinical trials. To do this effectively, expanded access outside of trials should be 1) for non-trial-eligible patients 2) staggered to follow recruitment for the trial 3) trial participants should be provided assurance of an open label extension to the experimental drug following their participation.

People with ALS urgently need increased federal investments to find effective treatments and a cure. This means we need more ALS scientists, more ALS clinical trials, and more ALS research that can lead to effective treatments and prevent new cases of ALS. Because ALS is rare, there are few patients and trials for them to participate in. Additionally, the average diagnosis is 12 months, which results in a situation where most people with ALS are being excluded from trials due to symptom onset. That is why expanded access and open label extensions are essential.

There is a perception that ALS is rare, but it is not. There is a higher chance of getting ALS than being hit by a bus during a lifetime. The actual incidence is comparable to MS, but ALS is most the fatal and rapidly progressing neurodegenerative condition with highest unmet need. The perceived rarity of ALS leads to less funding and less federal focus on ALS, which is impacting trials and research.

To make transformational change in the ALS field, Congress should increase funding for ALS research at NIH, the DOD ALS Research Program, and the FDA Orphan Products Grants Program. Congress should also provide level funding to continue the valuable work of the CDC National ALS Registry, and commission a National Academies of Science, Engineering and Medicine study to provide policy recommendations on making ALS livable. Together, these investments will attract the next generation of researchers in ALS, increase drug discovery and development and increase clinical trials, and help identify risk factors for ALS.

Why is it important for us to act now? Thousands of people living with ALS don't have options and lack access to potential treatments. ALS can affect any one of us. Our veterans who have served this country are up to twice as likely than the general population to develop ALS. Our loved ones, our children, our grandchildren are at risk. Anyone is at risk. Let them not have to face what thousands of people living with ALS face today – lack of access to treatments. You have the power to change that.

We have seen the ability for regulatory flexibility and speed in other areas. The reality is that ALS is 100 percent fatal. The pipeline and our understanding have grown significantly in the last five years. Approving a new drug for ALS — or Alzheimer's or other diseases — can have a bigger impact than just providing people with a single new treatment. New approvals can spur innovation and investment by industry in a disease space with few available treatments available. But in cases of fatal neurological diseases without cures, when a promising drug comes along that has the potential to retain function and extend life, patients' needs are paramount.

Thank you for the opportunity to testify before you today.