June 4, 2021

Dear Dr. Cavazzoni,

On behalf of the people living with ALS who presented at the May 25 "We Can't Wait" meeting, I write to thank you and your FDA colleagues for participating. While we are heartened by your words at the close of the meeting, those words need to be followed by action on behalf of the ALS community. As you heard clearly from our speakers, people living with ALS and their caregivers want access to novel therapies that have shown effect, even if that benefit is modest. You heard from speaker after speaker, as well as the over 150 people that sent in testimonials, that "modest" can mean the opportunity for more time with loved ones, more time to walk, to talk or to feed oneself, more time to potentially benefit from a next therapy.

During the May 25 meeting you also heard from members of our community about their hopes and fears. They hope those entrusted with accelerating development beneficial ALS therapies will do everything within their power to bring new treatments forward. They fear additional rounds of data collection and delays to this process, taking months that most people with ALS simply do not have.

In recent months, we have become concerned the Agency is not adhering to the commitments made to our community in the 2019 Guidance. We all need to better understand your approach. As people living with ALS bravely fight their disease while waiting for access to novel therapies, our community is demanding accountability from all of us.

We call on the FDA to publicly recommit to exercising the regulatory flexibility described in the Guidance and make therapies that have demonstrated potential effect as widely accessible as soon as possible. We request you issue a clear statement about your willingness to apply the flexibility described within the Guidance and commit to providing our community a detailed report about how you have been implementing the Guidance since it was issued.

Next week, hundreds of people living with ALS and their caregivers from around the country will participate in the ALS Association's annual Advocacy Conference. As part of this event, we will be advocating for a series of policy actions to accelerate development and delivery of effective therapies. In our appropriations request to Congress, we are asking for $50 million for the FDA to fund clinical research to benefit people with ALS. We also are supporting the Promising Pathways Act to give the FDA additional tools to work faster. In addition, the newly released ACT for ALS legislation expands access for

experimental ALS treatments and provides the FDA additional research capacity and public private partnerships to speed development and approval of new ALS treatments. Finally, we are asking Congress to fund a study by the National Academies of Science and Medicine to help coordinate the entire ALS research agenda.

These efforts support the FDA to do its part in bringing forward better treatments that extend and improve the lives of people living with ALS. While we all work for a cure, our near-term objective is to make ALS a livable disease. We cannot achieve this result without the active involvement of the FDA. Once again, please send us your strong commitment to aggressively apply your Guidance to help people living with ALS gain access to potentially beneficial therapies, along with your commitment to provide to us a detailed report on how the Guidance has been implemented since it was issued.

We can't wait.

Sincerely,

Calaneet Balas
President & CEO
The ALS Association