



June 15, 2021

Calaneet Balas  
President & CEO  
The ALS Association

Dear Ms. Balas,

Thank you for your email of June 4, 2021, following the “We Can’t Wait” meeting. My colleagues and I would like to extend a heartfelt thank you, to you and the speakers for providing testimonials and sharing your views on regulatory flexibility.

The Food and Drug Administration (FDA) recognizes the burden of ALS for patients, their families and caregivers, and we remain committed in all efforts to advance drug development. We recognize the continued unmet need for treatments for patients living with ALS and are committed to engaging with companies and the patient community to facilitate the development of treatments for this disease. We can also assure you that we are exercising the regulatory flexibility described in FDA’s 2019 guidance for industry titled *Amyotrophic Lateral Sclerosis: Developing Drugs for Treatment* (ALS Guidance) and we continue to commit to doing so.

Despite years of research and the availability of some approved therapies, we know the lack of new treatments for ALS is deeply frustrating for patients and their families and caregivers. Gaps in disease characterization, the heterogeneity of the ALS patient population, and a lack of fit-for-purpose biomarkers, among other things, all create significant scientific challenges across all clinical phases of ALS drug development. It is critical that we work with all stakeholders to further advance the scientific understanding of ALS, encourage the development of treatments for ALS, support patient involvement in clinical trials for ALS treatments, and facilitate patient access to investigational treatments when appropriate.

We would like to highlight that the Robert J. Margolis, MD, Center for Health Policy at Duke University, through a cooperative agreement with FDA, is exploring the state of scientific advancement for ALS. They are facilitating the development of a research roadmap, to increase collaboration and coordination among all ALS stakeholders to tackle specific challenges across the product development lifecycle.

Regarding your request for a detailed report about how we have been implementing our ALS Guidance, we note that, as a general matter, FDA is limited in the amount of information we can



provide about specific development programs<sup>1</sup>. However, as we noted above, we continue to stand behind and support the availability of the regulatory flexibility described in our ALS Guidance and are committed to exercising it when appropriate.

FDA continuously strives to fulfill our mission of protecting and promoting public health by ensuring safe and effective drugs are available to patients. We are committed to working with the community at large to continue to find ways to facilitate the advancement of ALS treatments. We look forward to continued engagement with ALS stakeholders, including patients and their families, pharmaceutical companies, academic researchers, and other government agencies.

Thank you for contacting FDA concerning this important matter.

Sincerely,

Patrizia Cavazzoni, M.D.  
Director  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

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<sup>1</sup> Relevant laws include the Freedom of Information Act (FOIA) (5 U.S.C. 552); the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 331(j)); and FDA regulations (e.g. 21 CFR 20.61(c), 21 CFR 312.130(b), 21 CFR 314.430(c), and (d)(l)).