

ALS Association Thanks Community Following FDA Release of Final Guidance Document

On Monday, the Food & Drug Administration (FDA) issued a finalized ALS Drug Development Guidance for Industry. This is one important step in comprehensive efforts to bring therapies to people with ALS more quickly. Five years ago, there was no clarity around what the FDA expected from companies pursuing ALS treatments. The ALS Association recognized this problem, galvanized the broader community, and engaged the FDA to provide a clear roadmap that also can respond to new science as it emerges. The FDA Guidance is one part of a commitment that will not be complete until we have a cure for all people with ALS.

The ALS Association is in the process of carefully reviewing the final guidance document. We are grateful to people with ALS and their caregivers who have contributed to this process and we thank the FDA for incorporating this input, as well as recognizing the devastating impact of ALS.

We look forward to working with the FDA and industry to reduce the number of people on placebos and to expedite therapy development in areas such as master protocols, adaptive designs, and enrichment strategies. We also note the language in the FDA guidance around considering innovative approaches that meet the Agency's requirement for approval and the call to industry to engage the Agency early and often to help get therapies to patients as efficiently as possible.

The ALS Association's review of the final FDA guidance document will be informed by our earlier comments on the draft version of the Draft FDA Guidance, the ALS Community Workshop: Therapy Development and Regulatory Pathways, hosted by The ALS Association in conjunction with the FDA, and the recently-released ALS Association Principles for Urgent, Patient-Centered Clinical Trials.
We thank people with ALS, their caregivers and families, as well as our partners in industry, at the FDA, and the entire community for bringing their experiences and expertise to the FDA guidance process. We look forward to working with all these stakeholders to bring treatments to people with ALS as quickly as possible.

Background:
The ALS Association, in coordination with the FDA, academic experts, clinicians, industry, and most importantly, people with ALS and caregivers, sent the FDA draft ALS drug development guidance,

delivered in 2017. This product jumpstarted and informed the FDA's creation of the first ALS-specific FDA drug development guidance for industry.

The community-developed guidance (not to be confused with the FDA's guidance) is part of a larger effort by The ALS Association to improve the efficiency, reliability, and speed of the ALS therapy development process. It includes content that goes well beyond what is found in an FDA guidance document and serves as reference for scientists, drug developers, and FDA reviewers. The additional detail provides much broader and deeper input on ALS science, as well as the community's views on key topics not typically found in an FDA guidance.

The FDA guidance documents are the Agency's views on ALS therapy development. The final guidance provides industry with the FDA's current scientific thinking so that effective treatments with a favorable benefit to risk profile can be most efficiently developed and made available to people with ALS. The FDA's final guidance are recommendations, not requirements, and they can be updated over time as our understanding of ALS improves