

ALS Community Responds to FDA Guidance Document with Call for More Urgency, Flexibility

The ALS Association and its partners submitted comments to the Food and Drug Administration to further inform the agency's implementation of the final guidance on the development of drugs and treatments for ALS. The comments underscore the need for urgency and commend the FDA for including voices from our community in its rule making process. Click here to view the comments.

The ALS Association has been working closely with members of the ALS community and the FDA to create a landmark, patient-led guidance initiative that will help drive ALS drug innovation more quickly from the laboratory to the patient, providing feedback to the agency, hosting a national workshop, and creating a report that informed finalization of the guidance.

In our latest comments, the Association and our partners recognize the Agency's efforts to address community concerns raised after the draft guidance was published in 2018, as reflected in changes made to the final version of the guidance. We express appreciation to the FDA for recognizing the central role people with ALS must play in the development of treatments.

Our comments also emphasize the need for FDA to exercise its regulatory flexibility to approve drugs as quickly as possible and to work with drug manufacturers to increase flexibility in trial design. We commend the agency for incorporating language in its final guidance document that will encourage more rapid trial enrollment and broad inclusion criteria for trial enrollment. In addition, we agree with the agency that alternative trial designs, including those that incorporate the use of mobile technology, will encourage greater participation in clinical trials.

We stress the need for creativity in minimizing exposure to placebos and are pleased the agency supports trial sponsors increasing access to experimental therapies.

We also point to the willingness of ALS patients to accept significant risks for potential treatment benefits that needs to be taken into consideration as part of the agency's flexibility. We further express concern with the agency's decision to remove language that could be used to slow the approval process and encourage vigilance and urgency that the approval process be conducted expeditiously.

FDA guidance documents explain the agency's interpretation of specific policies. Guidance documents are not laws or regulations but can provide helpful recommendations in the drug development and approval process. With the encouragement of the agency, the community's contribution is intended to provide much broader and deeper input to the FDA on topics that inform their understanding of ALS and the community consensus on key therapy development topics.

Click here to view the FDA guidance document.