Ask FDA to Finalize Guidance for ALS and Right to Try

Sending Office: Honorable Mike Coffman
Sent By: Jeremy.Lippert@mail.house.gov

Request for Signature(s)

Dear Colleague:

Deadline: COB November 29th.

We have made great strides in treating many life-threatening diseases, but treatment for ALS (Lou Gehrig's disease) has lagged behind. Unfortunately, this rare but devastating disease is fast-acting once diagnosed and affords little opportunity for study. Families are often left scrambling to adapt and have little time to advocate for federal policy changes. Yet I, and many of my colleagues, have had the opportunity to get to know victims and their families, and it's up to us to advocate on their behalf. Fortunately, there are opportunities to make a difference.

Please join me in sending this letter to the Food and Drug Administration. It urges FDA to quickly finalize guidance for ALS drug development, a project it has been working on for several years. The ALS Association has participated in developing the proposed guidance using proceeds from the ice bucket challenge. The letter also urges FDA to create guidance for drug sponsors and physicians under the Right to Try Act. At present, drug manufacturers have some concerns about how adverse reactions among Right to Try patients might affect approvals of the drug; FDA should clarify how it will look at these developments so that it's easier for drug sponsors to participate. Finally, the letter asks that FDA appoint a specific point person to serve as a liaison with the ALS community.

It is my hope that, with renewed attention, we can start making strides to find effective treatments, and eventually cures, for this terrible disease. Please contact Jeremy Lippert in my office with any questions or if you would like to sign on.

Sincerely,

Mike Coffman

Date

Scott Gottlieb, M.D.
Commissioner Food and Drug Administration
U.S. Department of Health and Human Services
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Commissioner Gottlieb:

We write to you on behalf of those who suffer from Amyotrophic Lateral Sclerosis (ALS) to request three simple, yet critical, actions by the Food and Drug Administration. During our service in Congress, we have had the opportunity to get to know many of our constituents, who are among the thousands in the U.S. suffering from ALS. As you know, there is no cure for ALS. There are, however, an increasing number of drugs in the developmental pipeline that provide these patients hope where none previously existed.

Earlier this year the Right to Try Act was signed into law. We note that the FDA recently created an informational website about this law and has made repeated statements that it is working on providing guidance. We appreciate both; however, it is now nearly six months after the act passed, and no guidance has been promulgated. In addition, the FDA does not appear to have made any significant progress on finalizing updated guidelines for ALS drug development. This apparent lack of urgency for a disease that kills 50% of those diagnosed within 15 months baffles us and our constituents.

Earlier this year, we received a letter signed by members of the ALS community outlining a range of concerns with current FDA policy as it pertains to ALS trials and patient access to treatments in the trial phase. The writers challenge the current timelines and protocols the FDA has in place that create barriers to potential treatments for ALS. Specific concerns expressed in the letter include: the overuse of placebos, lengthy and unnecessary observation periods, and the disparity between the design of ALS clinical trials and other terminal disease drug trials. Crucially, the authors point out that, despite the enactment of the Right to Try Act, they still largely lack access to experimental drugs outside the clinical trial process through Expanded Access or Right to Try.

In light of this concerning reality, we write you with three requests:

1. We ask that you expeditiously finalize updated guidance for ALS drug development. It is our understanding that a draft of the guidance document was released in February 2018. Thousands of

comments were submitted, and an extensive review period has already occurred. It is time to complete this document; such guidance would help industry prioritize research and streamline the drug development process for ALS. Ideally, it would be published in final form prior to the end of 2018.

2. We ask that that you expedite the creation of guidelines for drug manufacturers and physicians under the new Right to Try Act, that provide a roadmap to encourage companies to provide their experimental drugs to terminal patients. Such guidance should address concerns including the extent of documentation required from patients and physicians, and whether, or to what extent, adverse reactions to drugs under Right to Try may be considered before FDA approves a drug.
3. We ask that you appoint an FDA representative to work exclusively and directly with the ALS community and their representatives to build a strong partnership and aggressive plan to ensure our goals are met.

For all three of these asks, we request that you reply with a clear timeline for completion of these initiatives, an explanation of why the proposed timeline is appropriate, and the ways in which you have incorporated patients, their feedback, and their lived experiences into the guidance that the FDA is drafting. We also ask that you provide us with the appropriate points of contact at the FDA who are leading the effort to promulgate the requested guidance.

We cannot adequately stress to you the need and importance for further action in combatting ALS. This disease is torturous to those who suffer from it and a nightmare for family members who must watch their loved ones atrophy. We appreciate your prompt attention to this request and the favor of your reply by December 28, 2018.

Sincerely,

Related Legislative Issues

Selected legislative information: Family Issues, HealthCare, Veterans