Congress of the United States

Washington, DC 20510

March 6, 2020

Stephen M. Hahn, M.D.
Commissioner
United States Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, Maryland 20903

Dear Commissioner Hahn:

Congratulations on your recent swearing in as Commissioner of the Food and Drug Administration (FDA). We look forward to working with you.

In your Senate confirmation hearing, you made a commitment to improve the clinical trial process for those with terminal illnesses such as Amyotrophic Lateral Sclerosis (ALS). Our constituents diagnosed with ALS are eager to see action in this regard. We take this opportunity to reiterate our interest in working with you to ensure that those with terminal and rare diseases have trials designed with them in mind and are provided a clear and actionable pathway to access safe, promising therapies.

As you know, in the last decade we have seen tremendous advancements in potential treatments and therapies for neurological diseases. The treatment pipeline for ALS, in particular, is more diverse and robust than it has ever been. This gives us and the ALS community great hope. The FDA recognized that traditional processes do not adequately meet the needs of people living with this disease and issued final guidance on ALS clinical trial development. The FDA even approved the launch of the first-of-its-kind ALS platform trial. While these steps reflect an evolution in how the FDA addresses the devastating nature of the disease, the reality is that patients diagnosed with ALS today are still facing a death sentence as there is no known cure. The disease robs patients of their ability to speak, eat, and ultimately breathe. To date, the FDA has only approved two treatments for ALS that merely slow down the progress of the disease. With few meaningful options for treatment, any hope held by ALS patients and their families can quickly turn to desperation and despair.

Just last month, you received a letter signed by over 15,000 people impacted by the disease asking you to take steps to protect American lives by ensuring expeditious clinical trials and a well-functioning expanded access program. We share the belief that, under your leadership, the FDA can meet the needs of the ALS community. In our view, that means that the FDA will work to ensure that all future ALS clinical trial designs incorporate the provisions included in the recently finalized guidance on ALS treatment development. Further, all future ALS clinical trial designs sanctioned by the FDA should examine the appropriateness of participants who receive placebo alone. In addition, future trials should consider whether to involve mechanisms to ensure that expanded access is easily available, as appropriate, to patients seeking treatment.

Congress has made many efforts to create a better system for those fighting rare and terminal diseases, yet, barriers like the aforementioned still exist. There are many ways for Congress and the FDA to continue to work together to ensure people with ALS, and other rare or terminal diseases, have access to safe, promising therapies, while maintaining the foundational and necessary safety and efficacy standards for approval at the FDA. To assist Congress in this continued effort, we respectfully request that you provide, in your reply, specific recommendations for congressional action that will help the FDA facilitate faster access to safe, promising therapies for those Americans struggling with this deadly disease.

Thank you for your commitment to solving these problems for those living with ALS. Again, we look forward to working with you to ensure Americans diagnosed with terminal illnesses are allowed the opportunity to fight for their lives.

Regards,

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United States Senator

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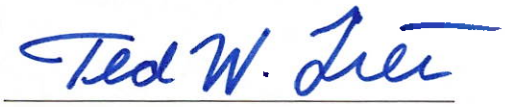

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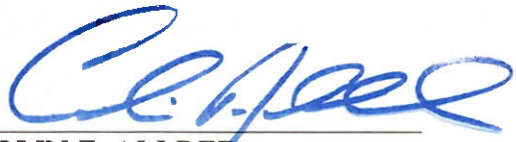

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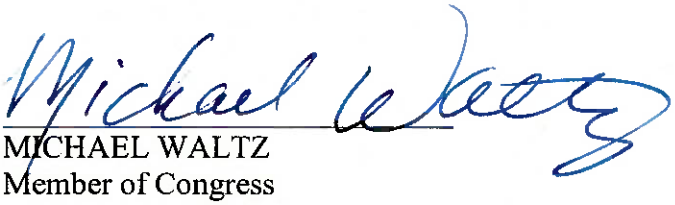

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cc: The Honorable Alex Azar
Secretary
Department of Health & Human Services

The Honorable Janet Woodcock
Director, Center for Drug Evaluation and Research
U.S. Food & Drug Administration

The Honorable Lamar Alexander
Chairman
U.S. Senate Committee on Health, Education, Labor, and Pensions

The Honorable Patty Murray
Ranking Member
U.S. Senate Committee on Health, Education, Labor, and Pensions

The Honorable Frank Pallone, Jr.
Chairman
U.S. House of Representatives Committee on Energy and Commerce

The Honorable Greg Walden
Ranking Member
U.S. House of Representatives Committee on Energy and Commerce