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and Related Agencies
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Chairwoman DeLauro thank you for the opportunity to testify before you again today.

My name is Brian Wallach. I sat before this Committee 2 years ago and asked you to rewrite the ALS story. To see us, hear us, and to fully fund the fight to defeat ALS. At the end of my testimony, Madame Chairwoman you promised me you would fight for my survival. Fight so I have the chance to see my young daughters grow up.

And you have—for that I am eternally grateful. Thanks to you and others like Mike Quigley, Jeff Fortenberry, Jason Crow, Dick Durbin, Mike Braun, Lisa Murkowski and Chris Coons and incredible ALS advocates, we have increased federal spending on ALS research by \$83 million in just two years. And this past December, Congress overwhelmingly passed a bill to give ALS patients access to SSDI benefits upon diagnosis, averting bankruptcy for so many.

I come to you today weaker in body, but stronger in resolve and hope than two years ago. I am because you have seen us and as a result the path towards ending ALS is clearer. The question now is when do we reach the end of that path and will any of those of us living with ALS now be here to see that day?

I desperately want to be here, but my body is failing. You can hear it in my voice and see it in the videos I post on Twitter. Odds are that unless something changes, I won't be. The average patient lives 2-5 years post-diagnosis and of those diagnosed in 2017 with me, four out of five—80%—are dead.

So I come to you today with two urgent asks. Ones that if you make real will change my and millions of others' futures.

First, fund ARPA-H and include ALS among its' core disease areas. During the 2020 campaign then-candidate Joe Biden promised ALS patient Ady Barkan that he would seek to create ARPA-H, modeled after DARPA, to solve issues relating to the diagnosis and treatment of disease. He also promised that ALS—along with cancer, diabetes and Alzheimer's—would be among the first diseases it tackled.

I was elated when President Biden's administration submitted a proposal to fund ARPA-H to Congress. I was devastated when I saw that only ALS was left out of the list of identified diseases it would target.

To cure ALS, we need an ARPA-H. We need both a focus on high risk/high reward research and to break down the antiquated, bureaucratic red tape facing ALS patients seeking promising therapies. Moreover, if we cure ALS, we can help unlock cures for Alzheimer's, Parkinson's, Frontotemporal Dementia and beyond.

Today, despite the increases in funding over the last 2 years, our government still spends less than \$6,000 on ALS research per year per person in the U.S. living with ALS. You have the power to fix this by putting ALS back into ARPA-H.

Second, we need you to hold FDA accountable for failing ALS patients by denying any type of approval for two promising therapies this year.

In September 2019, FDA released an updated Guidance for ALS Clinical Trials. It stressed the need for “regulatory flexibility in applying the statutory standards to drugs for serious diseases with unmet medical needs.” The Guidance explicitly stated that “[w]hen making regulatory decisions about drugs to treat ALS, FDA will consider patient tolerance for risk and the serious and life-threatening nature of the condition in the context of statutory requirements for safety and efficacy.”

The first two tests of FDA's promise of regulatory flexibility and urgency for ALS came this year with AMX0035, an oral medication, and NurOwn, a stem cell therapy. The Phase II/III trial for AMX0035 showed that AMX0035 slowed the progression of ALS and enabled patients on average to live 6.5 months longer. NurOwn's Phase III trial did not show the same overall benefit, but did show a "clinically meaningful" slowing of progression for a subgroup of ALS patients.

FDA's response: No approval for either therapy. No regulatory flexibility. No consideration of the terminal nature of ALS. No regard for the tens of thousands of patients, caregivers and advocates who signed petitions to the FDA pleading for access to these therapies.

Instead, FDA reverted to the same inflexible position for both therapies: they asked each company to run another large, long placebo-controlled trial and then come back. Let me make crystal clear what these two decisions by FDA mean: at best these therapies won't be accessible to patients for 4 years. By then nearly every ALS patient alive today will be dead.

Why weren't these therapies approved? Both therapies showed efficacy for at least a subgroup of ALS patients. And if the concern was safety, both trials showed a strong safety profile—particularly in the context of a 100% fatal disease. Moreover, the denials deprived patients of the chance to access FDA-regulated drugs under the supervision of an ALS specialist. So, instead, patients are forced to try to replicate the formula for AMX0035 on their own and to travel abroad for risky stem cell procedures.

I've been told that FDA has claimed to members of Congress and their staff that they are doing everything they can and that there was nothing else they could do with respect to these two therapies. This is simply not true or, if FDA actually believes this, they have provided Congress a clarion call to reform how FDA regulates treatments for diseases like ALS.

I am a former federal government employee. I come from a family of former and current federal government employees. I truly believe FDA is filled with honorable, dedicated public servants. However, their actions here are impossible to square with their own Guidance. This is most clearly demonstrated by the fact that AMX0035 appears headed towards approvals in Canada and Europe based on the same data presented to FDA. FDA stands alone as an immovable obstacle.

I implore Congress to hold hearings on these denials to bring transparency and accountability to a process that has left the ALS community devastated.

In addition to hearings, I ask you to pass and fund 2 bipartisan bills to ensure this does not happen again. Over the last year, the fight against COVID-19 showed how much regulatory flexibility FDA has when it wants to use it. Since FDA appears unwilling to use it to give ALS patients a chance to live, we have worked with members of Congress to reform how FDA approaches diseases like ALS.

The first, ACT for ALS, is being reintroduced this week. It will, among other items, make a significant amount of funding available to establish expanded access programs. Programs that will make promising therapies available to ALS patients now while fueling additional research into a therapy's safety and efficacy.

The second, The Promising Pathways Act, was reintroduced in the Senate last week. It will, among other things, allow for conditional approval of promising therapies after Phase II for life-threatening diseases like ALS. This would put us on par with Europe.

Today, the science needed to cure ALS is moving faster than ever and finally producing therapies that may be able to slow or stop this disease. This reality must be matched by a new regulatory approach that speeds promising therapies to patients. As I have outlined, despite

programs aimed to do just that which have worked in other diseases, we do not have that approach for ALS today. It is our moral obligation to change this broken approach for all those facing ALS just as we did for HIV and cancer.

If we do, I will have a chance to see my daughters graduate from kindergarten, high school, and college.

You have the power to make that happen.

I thank you for having the courage to do so.

And I look forward to working with each of you to finally defeat ALS.