

WE CAN'T WAIT: Action Meeting

Perspectives from People Living with ALS

To help the FDA better understand the importance people with ALS place on access to therapies with potential to provide benefit, individuals from across the ALS community generously shared their personal perspectives on the matter with The ALS Association. Their testimonials are compelling evidence of how extra months of life, slowed disease progression, and other seemingly modest benefits of an experimental therapy are truly meaningful to people with ALS and their loved ones.

What follows is a compilation of more than 150 responses to a questionnaire that was opened to the ALS community in advance of the May 25, 2021 "We Can't Wait" Action meeting with FDA and industry officials. These perspectives provide a clear picture of the urgency action the community is seeking FDA and industry to accelerate the community's access to potentially beneficial therapies.

PERSPECTIVES QUESTIONS

- How do you view the importance of a new therapy that offers the chance of a modest benefit, such as slightly slowing disease progression or providing a few months of additional life?
- How would you weigh a new therapy that offers the chance of a modest benefit in balance with:
 - a) the risk that the therapy ends up having no benefit at all; or b) the therapy also has significant side effects?
- How important is it for you that the clinical trial process is complete, and the FDA has approved it before you take a new drug?
- How has being diagnosed with ALS impacted your life?
- How has being diagnosed with ALS impacted those close to you?
- What do you wish people understood about ALS?
- Is there anything else you'd like the FDA [and pharmaceutical companies?] to know?

Please Note:

Some submissions have submitted a video or audio message, please click to play in the top right corner.

A United States Flag symbol  indicates the individual is a military veteran.

Kerry G. | Georgia

Age: 55

Year of Diagnosis: 2015

Type: Limb Onset, Sporadic



One more day! **When your life is put on a time clock every second matters.** Any treatment or therapy that extends your life makes it well worth the risk. It's been over 75 years since Lou Gehrig farewell speak to baseball and we're still no closer to a cure. Riuzole is still the same medication we had for the last 50 years, which is unacceptable. **We need your help to find a cure.** We have a terminal disease and are willing to participate in finding a cure but we're being handcuffed from even trying.

Nothing from nothing leaves nothing. At the present time we on a course with death. All I want is an opportunity to fight for my life.

The entire world has accepted vaccinations for Covid 19 in record timing. I have received the vaccine myself, yet it feels like a punch in the gut to see everyone rushing to find the Corona vaccine. While we restricted from taking our lives in our hands and trying new drugs. I'd rather die trying than wait for death to knock on my door.

My family and I have gone from an upper middle family to living below the poverty line. **ALS has financially destroyed my family.** With me being paralyzed and completely dependent on others for everything, my wife has also had to leave her job to care for me.

My children have missed out on a lot of things that kids normally experience. Either we can't afford it or do to me being unable to do things that a healthy father does. The level of care needed for each patient is incredibly expensive and critical.

Get us a fighting chance. Allow us to be involved in the decision to choose. Take a chance on the use of drugs. **I assumed the risk of playing football for 20 years. Now I'm being benched by not allowing me to fight for my life. Let me go down trying every reasonable opportunity available.**

Mark W. | Ashburn

Age: 59

Year of Diagnosis: 2021

Type: Limb Onset

Click
Here to
Play
Video

Having the chance to try medicine is essential.

I would take the chance. Not trying has the side effects of continued deterioration.

I don't believe I necessarily have the time to wait and again side effects are less terrifying to me than the inevitable of the disease

I never felt frail before in my life and psychologically is weighing on me while physically exhausting me.

My family is afraid.

You don't need to pity us. Given the chance we are very strong people and just want a little more time.

There are limits to our ability to make income so pricing the medicines so high is a burden.

Katherine B. | Virginia

Age: 52

Year of Diagnosis: 2020

Type: Limb Onset, Genetic

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It is imperative that ALS patients be given the opportunity to try new drugs that have been proven safe. Any benefit, including the slightest slowing of progression is beneficial to us. **We should be given the opportunity to work with our physicians to determine if a protocol or drug is appropriate for us to try.**

Any therapy that may be beneficial is worth trying. **Patients, not the FDA, should decide is the risk of any drug is worth the potential benefit.**

The FDA is dragging its feet. There are two drugs that have been proven helpful for some patients. ALS is not a one drug fits all disease. Different patients will benefit from different therapies. The FDA should allow us to make these decisions. Once a drug has been proven safe and shown any potential benefit, such as NurOwn and AMX0078, they should be approved by the FDA for use. The fast progression of this disease does not give us time to wait.

My disease is considered slow progressing, yet it has changed my life, impacted my work and relationships and altered my day-to-day activities. **In the past 6 months I have lost strength in both arms and all fine motor skills in my left.** I cannot type, have difficulty prepping meals and performing some self care functions. I get tired and have experienced cognitive deficits. I pray that does not mean I will end up with frontal lobe dementia which is prevalent in my form of genetic ALS (C9ORF72). **My 16-year-old daughter is now in therapy and is dealing with the mortality of her mom** and mom's inability to do so many of the activities that were a part of our lives just a year ago. **This is affecting decisions that will impact her future** (i.e. she no longer wants to go away for college). I worry about how much longer I can work - I am the primary income in our home.

It has impacted all of my family and close friends.

That our lives are worth fighting for!

Our lives are worth fighting for! We deserve the opportunity to try ANY therapy that may slow or halt this disease. Every extra day in our lives is a gift that we deserve to claim!

Craig P. | Wisconsin

Age: 63

Year of Diagnosis: 2020

Type: Limb Onset

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Extremely Important, I wake up every day hoping that this is the day I get a phone call that a new drug is ready, and I can start immediately.

Score of 1-10 this is a 10 all day long, I don't have months or years to wait its now or never I need HOPE! any drug or the promise of a new drug gives me that HOPE.

If a trial for a new drug is showing to be safe with few side effects like AMX0035 has shown to have promising benefits, then it should be available immediately. **As a person living with ALS, I do not want to wait for stage 5-6-7 trials time is most important!!!**

I wish everyone at the FDA could live for One month with ALS or sit at there kitchen table and look at their spouse who has ALS and tell them there is a new drug that's showing great results but I'm sorry honey we have to run more trials it should be approved in 3-5 years. After a lifetime of sports A love of the outdoors, a cabin in the north woods of Wisconsin I'm left with a window to look out of a TV remote and a lot of books to read that's how ALS has impacted my LIFE !!

Devastating, to watch a loved one die slowly before there eyes, To try and keep their spirits up let them know that there is hope for new drugs and maybe to slow the progression down, its sometimes harder to give loved ones hope then yourself.

That time is critical !! This does not go away when you turn your back, we need to speed the process of making new drugs available ASAP. Please give us HOPE !

Think for one minute how different a person living with ALS is compared to someone working for the FDA or a pharmaceutical company. when Friday night comes and you go home for the weekend you make plans with your family camping trips, zoo, swimming, you shut down your work with ALS. A person living with ALS doesn't know the difference from Friday night to any other night we can't shut down and start back on Monday with our symptoms. You have the luxury to wait till next week or next month or a year to make decision for approving the use of new drugs, **Guess what we don't have that luxury we can't wait !!! We don't care if the new drug makes us glow in the dark as long as it slows down the progression, we are all in PLEASE !!**

Karen R. | South Dakota (1 of 2 pages)

Age: 63

Year of Diagnosis: 2017

Type: Limb Onset



I am anxious to have a chance to try any therapy that shows even a modest benefit such as slowing of disease progression or additional survival time, even if it showed such benefit in just a subset of patients. Due to the heterogeneity of the disease, it should not be expected that every therapy will work for all patients. I need to slow or stop my progression NOW to give me more time as we await a definitive cure and give me time to make more memories with my family. What you might think of as a modest benefit, may be of critical importance to me in maintaining my quality of life. For instance, even a 1-point drop on the ALSFRS-R scale in the area of ambulation would mean that I am no longer able to walk at all. A 1-point drop on the scale in the area of dressing myself would mean that I can no longer independently dress myself and would need to rely on the assistance of someone. One more point lost on the handwriting portion will mean my handwriting has gone from slow and sloppy to impossible to perform. **These are huge quality of life issues for me.** I do think a cure for ALS will be found someday and I want the time to live to see that day.

Right now, I know my disease is 100% fatal and I am on the trajectory of becoming more and more disabled. I would gladly accept the risks to try a new therapy - I would be no worse off than I am now and **that chance to TRY SOMETHING** would definitely give me hope. If the therapy has significant side effects, I would be able to weigh at that time whether I can continue the treatment or not. **I JUST WANT SOMETHING TO TRY** as I am dying waiting.

I feel the FDA and manufacturers should allow those of us who do not qualify for the clinical trial to have access to the drug before final approval. That expanded access can continue to provide scientific information about the drug, and I do not have 2 years or more to wait for another trial to be completed. By the time a drug has reached Phase 2 or Phase 3, the safety data should be known enough so that I can feel confident from the safety standpoint at least.

Karen R. | South Dakota (2 of 2 pages)

Age: 63

Year of Diagnosis: 2017

Type: Limb Onset



At the time in my life when I was looking forward to retirement and spending more time with grandchildren, volunteering more, and enjoying life, I find myself grieving loss after loss. Once a very independent person, now I have to rely on my spouse for the most simple of things. **Although I try to remain optimistic and hopeful, it is hard not to be afraid of what is ahead of me.** A future where I am totally paralyzed and a burden to others is very scary. There is also a great financial impact to the disease and care involved, and I worry that once I am gone, my husband will not have enough of our retirement savings left for himself.

I know that this is very hard on my daughter and family but especially hard on my husband. Instead of being partners and teammates, I feel I am becoming more and more of a burden to him, and I know this is very difficult for him but also very hard for him to communicate to anyone else who understands.

I wish everyone understood the urgency of finding treatments and ultimately a cure for ALS. **Many people don't realize that it is 100% fatal** and that not much has really changed since Lou Gehrig's diagnosis. If the resources that were put into the AIDS fight or the coronavirus fight, there would be real progress. In the time that it takes to bring one drug from the lab to patients, the majority of ALS patients will have died.

Please give me a chance to try anything that has been shown to help even 20% of patients in a trial. I want the chance to see if I might be in that subset of patients who is helped. I don't have time to wait for one drug to hit a home run - I just want more time now as I am dying a little more every day.

Bill W. | Georgia

Age: 74

Year of Diagnosis: 2019

Type: Limb Onset



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I am a pediatric oncologist and clinical trialist. I have been heavily involved in clinical trials my entire professional career, including at the national level as a leader, and appreciate both the advantages and potential challenges of using innovative drugs, like serious side effects. BUT childhood cancer is mostly LIFE THREATENING and deserves every chance for cure. **This is not dissimilar to ALS!** Through NCI sponsored clinical trials, started in the 1960s, we have been able to take the overall cure rate of all childhood cancers from 25% at that time to 80% today. For most of the trials we did not wait for confirmatory trials to move forward, with arms of trials shown to be superior to the past 'standard' now becoming the NEW 'standard' [and I should note that for the vast majority of the drugs used, there was NO pediatric indication for most important agents, many of which are still used today without a peds indication!]. Since Richard Pazdur's leadership in ODAC, in charge of oncological new agents in the cancer world, many new molecular inhibitors have been introduced, with some **VERY SMALL** trials then showing efficacy in adult cancers. Many have then received 'Accelerated Approval', with the FDA following further use carefully through the drug companies, with subsequent analysis. Some drugs have been stopped for certain indications because of lack of efficacy in subsequent analysis, with more patients and longer follow up.

I HAVE ALS, DIAGNOSED IN 2019. WHY CANNOT THE FDA HAVE A SIMILAR APPROACH WITH DRUGS AIMED AT ALS? A great example: the two-drug combination called AMX0035, with a very convincing report in the NEJM of early efficacy. **Why not give the drug(s) Accelerated Approval?!** Then conduct a Phase III trial, single agent, or just collect data on ALS patients and see how they do? Most of us would want to go on such a drug, even if potential side effects ultimately are found. **Our disease is LIFE THREATENING, big time!** Those who don't want the drug do not have to get it, of course . But the rest of us will line up for it, with excitement. Similar other drugs should be evaluated like cancer meds, studied quickly, approved by the accelerated process if they show any efficacy, with careful follow up by the drug company and the FDA. **In my humble opinion it is time for the FDA to increase access to novel agents for patients with a disease like ALS, using a approach similar to the ODAC Accelerated Approval process. Clinical trial yes; full FDA approval NO, see above.**

ALS has 1] Led to retirement; 2] Now physically limited, cannot swim or bike, 2 passions. 3] Pain, not fun 4]sedentary lifestyle: I used to be very athletic and physically fit

My wife now does the vast majority of daily chores, very 'unfair' to her.

First, I bet the half the folks in the country don't even know what ALS stands for, and what it means.

I feel very passionate about this!

Steve K. | Massachusetts

Age: 57

Year of Diagnosis: 2017

Type: Limb Onset, Sporadic



With a diagnosis of 3-5 years left to live a few months is a huge benefit to me.

I would consult my neurologists to weigh the risk/benefits but would be willing to accept more risk of side effects.

Since it is taking years and years of FDA red tape, I strongly feel after Phase 2 completion, ALS patients should be given the opportunity to have access to the treatment if it is proven to be effective.

It has impacted my life in every way. My families' lives have changed for ever! It is a very debilitating disease that progresses every day and there is no cure. Symptoms and progression time are different for each ALS patient.

ALS patients are grateful for the all the ongoing effort to find treatments and a cure, but we need regulatory urgency.

ALS cannot be viewed as a livable disease but ticking clock that results in people losing their life while waiting for approvals. Trial design should include innovative early access contingency plans.

Judith M. | Arizona

Age: 65

Year of Diagnosis:

Type: Limb Onset

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When my husband, Alex, suffered with the disease of ALS, he was willing to try anything that would possibly help with the progression of the disease until he got to the point of requiring a trach tube for breathing. **He wanted to live even in a wheel chair, but when he lost his ability to move his hands and breathing was so strained he decided it was time to end his battle.** He had 18 months prior to advancing so quickly that he would have tried anything. He took the one drug, Rilusol, I believe that possibly extended life for 3 months.

It would have depended on his side effects.

If the clinical trial is complete with good results then try it. **These patients only have a short time to live - what can possibly be worse than the effects of ALS.** The covid vaccine happened quickly without FDA approval. Just don't make the cure so expensive that no one can afford to take it!

My husband died at the age of 61! 2 years with ALS. His personality was bigger than life itself and he continued that spirit thru the disease. He was a hard working family man and was missed by the huge number of people he impacted. Take the time to view the YouTube video - "Alex Massey Farewell" and you will understand what a loss he was to his family, church family and employer.

What a good spirit these patients have during the battle.

Get going on a cure.

Roberta M. | Clarksburg

Age: 65

Year of Diagnosis: 2019

Type: Bulbar Onset



I view new therapies as highly important to the ALS community. Anything to give hope, strength and potentially change how ALS is treated is something that we (patients and caregivers) have needed for years.

I believe that any treatment, whether it is effective or not, is one step forward in finding a treatment or cure for ALS.

Not important at all to wait for FDA approval; my mother would have tried anything to treat her ALS, whether approved or not. She HATED the suffering she was facing, to the point she attempted suicide. Her exact thoughts on ALS treatments were that she was already dying from an incurable disease, so nothing worse could happen to her.

My mother went from working fulltime to being disabled within a month of diagnosis. She was miserable, sat in a chair all day watching tv and lost her will to live. She pulled away from family, friends, she lost who she was with her diagnosis. Witnessing this turn around, seeing my mother go from a hard working, funny, vibrant person to an empty shell broke my heart in two and has forever changed me. I was thankful she passed before she became bedridden and immobile, because I knew how miserable she was.

My mother's diagnosis was very hard on our family. She was the rock, the one-person who held what family we have left together and without her we are all lost.

That everyone impacted suffers, not just the patient. I also wish people understood more about the financial difficulties and hardships and that medical companies and insurance providers would change their relationship with hospice care facilities. My mother could not get proper care and equipment due to restraints placed on hospice care.

Stop being greedy, **we need treatments and cures now!** We needed them 100 years ago!! Every second you drag your feet, that is another person you allow to die needlessly!!

Richard E. | Connecticut

Age: 62

Year of Diagnosis: 2020

Type: Limb Onset, Sporadic

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It's important because "slowing" is better than nothing

Define "modest benefit". No one likes bad side effects. However, ALS is a death sentence with a life expectancy of 2 to 5 years. And a quality of life significantly less than that. So the risk is minimal

NOT important at all.

Again, ALS is a death sentence with a life expectancy of 2 to 5 years. And a quality of life significantly less than that. The FDA moves way too slowly for pALS. Again, ALS is a death sentence with a life expectancy of 2 to 5 years. And a quality-of-life significantly less than that. **Quit my job, started making final arrangements**

Wife is distressed and depressed.

Mention ALS and only two names are ever mentioned; Lou Gehrig (who died 80 years ago) and Steven Hawking (a longevity anomaly). This tells me there is a publicity problem. 1. No cure. 2. No effective treatment. 3. Life expectancy 2 to 5 years. 4. Quality of life much less than 2 to 5 years. 5. Under funded research. 6. only two approved treatments both having very little positive results

Forget about Steven Hawking living 50 years with the disease, he was an anomaly. Quality of life much less than 2 to 5 years. **Pharmaceutical companies need to think out-of-the-box for new effective treatments and lobby US Congress for increased funding.** Two quotes come to mind; **Albert Einstein, "The definition of insanity is doing the same thing over and over again and expecting a different result."** Also, James Ling, "Don't tell me how hard you work. Tell me how much you get done."

Lou Gehrig died 80 years ago and since that time only two drugs have been approved which only extend life for about 3 months, not a very impressive track record of accomplishment. I'm suggesting a different, perhaps more radical approach be brainstormed and implemented in accomplishing ALS objectives. Unfortunately, I don't have the answers. Hopefully people smarter than I can come up with more efficient, out-of-the-box ways to achieve the goals needed by ALS patients, because with the average lifespan of an ALS individual to be just between 2 to 5 years and their quality-of-life years considerably less than that, **four years to achieve an accomplishment is a lifetime for an ALS patient."**

Paul K. | Alabama

Age: 79

Year of Diagnosis: 2009

Type: Bulbar Onset



Highly anticipated particularly Neuron stem cell therapy. ALS patients should be put on a diaphragm Pacer as soon as diagnosed. I was and it has been my life saver treatment. Why does the ALS and MDA not recommend that instead of Cpap or Trilogy.? It works 24-7 without the inconvenience of carrying a machine and mask every where you go.

Not at all worried about side effects of a new drug as long as it is shown to be tolerated and not kill the patient.

Cannot walk, talk very poorly and have trouble communicating with my Love ones , friends and fellow workers.

Horribly! I cannot help at all do the things I use to do, and my wife has to do them or hire them out.

The devastation it brings on a family and the person that has ALS.

Create a drug or vaccine that stops ALS and cures a patient. Just like you did for Covid-19 in operation warp speed. You have the brain power and funding and do it now.

Karen R. | South Dakota

Age: 63

Year of Diagnosis: 2017

Type: Limb Onset

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I am anxious to have a chance to try any therapy that shows even a modest benefit such as slowing of disease progression or additional survival time, even if it showed such benefit in just a subset of patients. Due to the heterogeneity of the disease, it should not be expected that every therapy will work for all patients. I need to slow or stop my progression NOW to give me more time as we await a definitive cure and give me time to make more memories with my family. What you might think of as a modest benefit, may be of critical importance to me in maintaining my quality of life. For instance, even a 1-point drop on the ALSFRS-R scale in the area of ambulation would mean that I am no longer able to walk at all. A 1-point drop on the scale in the area of dressing myself would mean that I can no longer independently dress myself and would need to rely on the assistance of someone. One more point lost on the handwriting portion will mean my handwriting has gone from slow and sloppy to impossible to perform. These are huge quality of life issues for me. **I do think a cure for ALS will be found someday and I want the time to live to see that day.**

Right now, I know my disease is 100% fatal and I am on the trajectory of becoming more and more disabled. I would gladly accept the risks to try a new therapy - I would be no worse off than I am now and that chance to TRY SOMETHING would definitely give me hope. If the therapy has significant side effects, I would be able to weigh at that time whether I can continue the treatment or not. **I JUST WANT SOMETHING TO TRY** as I am dying waiting.

I feel the FDA and manufacturers should allow those of us who do not qualify for the clinical trial to have access to the drug before final approval. That **expanded access can continue to provide scientific information about the drug, and I do not have 2 years or more to wait for another trial to be completed.** By the time a drug has reached Phase 2 or Phase 3, the safety data should be known enough so that I can feel confident from the safety standpoint at least.

At the time in my life when I was looking forward to retirement and spending more time with grandchildren, volunteering more, and enjoying life, I find myself grieving loss after loss. Once a very independent person, now I have to rely on my spouse for the most simple of things. Although I try to remain optimistic and hopeful, it is hard not to be afraid of what is ahead of me. A future where I am totally paralyzed and a burden to others is very scary. There is also a great financial impact to the disease and care involved, and I worry that once I am gone, my husband will not have enough of our retirement savings left for himself.

I know that this is very hard on my daughter and family but especially hard on my husband. Instead of being partners and teammates, **I feel I am becoming more and more of a burden** to him and I know this is very difficult for him but also very hard for him to communicate to anyone else who understands.

I wish everyone understood the urgency of finding treatments and ultimately a cure for ALS. Many people don't realize that it is 100% fatal and that not much has really changed since Lou Gehrig's diagnosis. If the resources that were put into the AIDS fight or the coronavirus fight, there would be real progress. **In the time that it takes to bring one drug from the lab to patients, the majority of ALS patients will have died.**

Please give me a chance to try anything that has been shown to help even 20% of patients in a trial. I want the chance to see if I might be in that subset of patients who is helped. **I don't have time to wait for one drug to hit a home run - I just want more time now as I am dying a little more every day.**

Julie S. | Middletown

Age: 28

Year of Diagnosis: Bobby- 2014

Type: Limb Onset, Sporadic

**Click
Here to
Play
Video**



Jan P. | Alabama

Age: 69

Year of Diagnosis: 2019

Type: Limb Onset, Sporadic

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Any chance to slow Progression with the chance of living longer would be worth taking

If we don't try we will never know!

If there is positive improvement after 1st or 2nd trial I am in favor of letting us try it

If you consider that I have spent my life's work helping others in the community to not being able to walk or use arms or hands, very significant. Our whole family has been impacted!

Grandchildren and children are heartbroken, we had so many plans

There is no treatment!

Find a treatment that will give us some hope!! **There have been many trials that have shown promise and yet we are denied.** Fund the research like many other diseases and make it a priority, not another hundred years of waiting!!

Norene B. | Iowa

Age: 64

Year of Diagnosis:

Type:

**We
Can't
Wait**

Very important to be giving ALS patients hope and more time to live is vital!

People with ALS know that the disease is terminal. They deserve to have every chance to extend their lives, especially if they choose to do so despite possible side effects or no effect.

The family of a person with ALS is highly impacted. The spouse and children see their loved one lose mobility and the ability to live a normal life, which is very traumatic.

People with ALS are willing to do most anything that they can to be able to live longer.

Because ALS is basically a death sentence, ALS patients should be able to have any and all treatments that could work to extend their lives. They should be allowed to try treatments that may not be fully tested or may have side effects because that is their only hope. I'm sure that they would be willing to sign a liability waiver in order to receive experimental treatments.

Chris M. | California

Age: 58

Year of Diagnosis: 2013

Type: Bulbar Onset



My brother had bulbar onset ALS and died but he wanted desperately to find ANY new therapy to offer ANY chance of ANY benefit, however small.

My brother had bulbar onset ALS and died but he did not care AT ALL if the trial process was complete or if the FDA had approved a drug before he could start to give it a chance.

My brother had ALS and died but he would say it was devastating, very time consuming and very expensive. He pursued any type of therapy he could possibly find, however remote a chance it had of helping, including many sessions of stem cells which were very expensive and made him travel to remote places, including Mexico where therapies are further along than here in the US.

My brother had bulbar onset ALS and died but he would say that those around him were devastated and distraught. They tried to make life as comfortable and as fun as possible, but it meant they had to take off work a LOT and pay people to come help with his daily life.

My brother had bulbar onset ALS and died but he would say that he wished people understood that ALS is the most cruel, horrific disease that exists.

PLEASE, PLEASE, PLEASE find a cure - soon!!!! Or, even better still - find a vaccine or some way to avoid ALS in the first place!!!

Age: 60

Year of Diagnosis: Brother: 2018

Type: Bulbar Onset

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Imperative!! It is imperative to have a chance to try something that will help my quality of life.

Current therapy has side effects and current therapy has limited duration too but better to have it than not have some options.

Death sentence and frustration that a cure cannot be found. My brother's life expectancy is nearing the end of a torturous 3-year period

Devastated our family. My Brother is currently 60 years young ALS is also a very expensive disease and often people always need to wait for insurance approval which is very frustrating. Waiting for approval for breathing equipment can take weeks !!

It is a devastating generally short-lived disease that therapies cannot stop the progression. **People with ALS are the most determined and strong fighters I have ever witnessed. They deserve any consideration for any new medication therapies to give hope to those fighting this monster.**

Find a cure.

FDA IS KILLING ME

Mary C. | Texas

Age: 61

Year of Diagnosis: Texas

Type: Bulbar Onset



I feel that the therapies used now that only “slow” the progression are useless in most aspects. Some people don’t want to suffer longer than necessary when there are therapies that the FDA is sandbagging that actually showed positive results for patients. ALS doesn’t affect everyone the same way. So they need to stop searching for one size fits all therapies. All this is doing is letting more and more people die while the FDA & ALSA get to decide if people get these therapies. It shouldn’t be up to you. Let the patients decide, They are the ones dying.

The ones now have significant side affects and don’t really extend or improve quality of life. What’s the difference? You want more drugs that barely help and have side effects?

The FDA hasn’t even approved this so called “covid vaccine” and people are getting it. Why not NurOwn? Or other therapies held up in trials? So it’s not important to me that the FDA has any say in it. You and they have dropped the ball so far. Let the patients decide if they want to take therapies like NurOwn.

How do you think it has impacted our lives? My mom has a terminal illness and the FEA is holding out on therapies like NurOwn!

It has devastated everyone in my family.

That it’s different for everyone. 100% fatal and the FDA is letting people die for profit. Stop looking for one size fits all therapies!

That we all know what you’re up to and we’re spreading awareness. Things WILL change one day for patients. We don’t trust you!

Edwin B. | Wisconsin

Age: 59

Year of Diagnosis: 2019

Type: Limb Onset

**We
Can't
Wait**

Extremely important to have the chance to take any medicine that will help.

Highly important to have any opportunity, when no chance exists doesn't make a difference

If it shows potential their decisions are meaningless

In every way it is important to find a cure.

They are my caretakers 24/7. It seems no gives a shit.

Big Pharma has one Concern \$\$\$ People with ALS are statistically moved from moral equity to nothing profitable and forgotten. The FDA problem 1 first word.

Linda R. | Texas

Age: 72

Year of Diagnosis: 2017

Type: Bulbar Onset, Sporadic



I believe it is critical to have access to all new therapy. ALS is terminal therefore the chance to live a few months longer means an opportunity to experience more family and loved one's special occasions; a birth, a graduation, a school event with the grandchildren. Life is now measured in days; one day at a time.

I am willing to risk any side effect in order to have more days. I feel the risk may not only benefit me but may lead to a cure which will benefit all those with ALS.

I have enrolled in every trial I have access to; I do believe the new drug needs to complete the approval process. The alternative is death coming sooner.

ALS has changed the lives of my family, colleagues, friends and myself in countless ways. Having a death notice makes you appreciate every day you have. My life changed in everyday. I am active, independent, had a meaningful career, physically fit and healthy. Now I live with my daughter's family and depend on others.

My family has been impacted by now having to care for me. I never wanted my children to have to care for me. I don't think anyone wants that.

ALS strips you of hope. From the initial diagnosis to every doctors visit you are told of the dismal future.

I don't understand why the FDA would make an ALS patient wait for therapy or treatment. I life expectancy is 3-5 years, there really isn't time to wait. There is urgency in finding a cure and each therapy deserves to have to chance to providing answers.

Teri D. | Colorado

Age: 40

Year of Diagnosis: 2016

Type: Limb Onset, Sporadic

We
Can't
Wait

Important to have access to all medicine.

No side effect is worse than ALS.

Not important.

You're kidding, right? Is this a serious question?

It could happen to you, too.

Restricting access to therapeutic agents for ALS is unconscionable.

Mary Ann W. | California (1 of 2 pages)

Age: 66

Year of Diagnosis: 2018

Type: Limb Onset, Sporadic



I am speaking for my husband, Harry who passed away from ALS in October 2020. Harry was a man who lived his life with extreme optimism. He wanted to believe that a therapy would be developed in his lifetime that would significantly increase his life span or possibly cure ALS. As much as scientists know about ALS and other motor neuron disease, it is clear there is more that is unknown. Without trials that may move the dial even slightly in the right direction how can we even hope to find a therapy that could significantly impact progression or cure ALS. This is a disease without hope and without any good outcomes. We have no choice but to try and rejoice in the slightest progress.



The only way we will come close to learning more about ALS will be if we engage in aggressive trials. The risks are more than worth it if it will bring us closer to finding out why this disease occurs, how we can slow its progression and potentially find a cure. ALS victims have no other choice, and they have no other hopes.

It is of no importance to me

For Harry, it altered all his life plans and expectations. It took away his future. He went from independent to dependent, from one who prided himself as giving person to one who needed to accept from others, from one who loved to give hugs to one who had to request a hug. He could have easily lost his dignity, many do. However, he did have to fight hard to maintain that dignity. He lost so much control over his life and had to patiently accept what that meant. We were fortunate to have financial resources and strong community to help us through. Many ALS victims do not. **It can be a very lonely journey with devastating financial consequences for the family**

Mary Ann W. | California (2 of 2 pages)

Age: 66

Year of Diagnosis: 2018

Type: Limb Onset, Sporadic



Watching Harry's disease progress over three years was devastating.

The stress involved in his care was overwhelming. There were so many things that needed to be attended to, medicines, medical equipment, caretakers, insurance, doctors. There was the stress of something going wrong and knowing if you didn't do it right, it could kill him. His disease made him a moving target. No sooner did you solve one problem, when suddenly that solution no longer worked. Everyday ended in exhaustion, only to know that in the morning you would have to do it all over again. The sadness could be all consuming. It changed us and it changed our relationships with Harry and each other. As I said, we had financial resources to help with Harry's care. But many do not. In these cases, it requires family members to assume the full burden of care, often resulting in loss of income. Our children are adults. I can not imagine what it must be like for a family with young children and a parent with ALS. What this disease takes away from them will impact them for the rest of their lives.

Harry's biggest challenge was teaching others that even though his body did not work, he was still Harry. He needed to put himself out there and help people become comfortable being with him.

I watched Harry be repeatedly disappointed when he was rejected from one trial after another. He was willing to do anything that may have helped him or helped others in the future. The availability of trials and being part of one should not be so difficult. ALS is only one of many motor neuron diseases afflicting people all over the world. More needs to be done to learn about these diseases and help discover viable therapies. It should not be about the money. **Real people are being impacted and one day it may be one of your family members.**

Melanie B. | Colorado

Age: 70

Year of Diagnosis: My father was diagnosed in 1958.

Type: Genetic

We
Can't
Wait

Critically important like water in the desert.

I would want to know the significant side effects but would most assuredly go forward with the new therapy. **Just as Chemotherapy has significant risks to Cancer patients it saves lives.**

It's a devastating disease that takes and takes until you have nothing left to give. The toll on caregivers is exhausting and emotionally devastating too.

Please hurry!

Jerry M. | Kansas

Age: 66

Year of Diagnosis: 2020

Type: Bulbar Onset



We deserve the right to try promising treatments

I'm living with a death sentence. What do I have to lose? The "side effects" of ALS are not pretty. I don't want someone telling I can't get access to a treatment that could improve my quality of life. If you were dying of thirst and came across a puddle of nasty looking water. Would you drink it to save your life not knowing for sure what it might do to you? I sure would and wife and kids and grandkids would want me to.

Not important at all to wait on FDA. Who are they to make life and death decisions for me. Don't take my hope away from me and my family. Right my chances of survival is 0%. **If a treatment would have a 10% chance of improving my condition, I want to take that chance and don't want someone telling what's good for me. I'm dying for heavens sake.**

What kind of question is this. Someone that asks that question, hasn't had it or had loved with it. My gawd yes, it's impacted my life. **I can't talk. I can't eat food. I can't walk unassisted. I can't get on the floor and play with my 2 yr. old granddaughter. I'm having to sell our home and move into a home that's more wheelchair accessible. I could go but hopefully you get the picture and I ccx sure hope you already know how it impacts one's life.**

My wife is my caregiver and her dreams of traveling in our retirement years. She has to wipe me and clean up after my accidents. She has to drive me everywhere. My 2 children are watching their father wither away.

The things I've just explained about how it affects the PALS and their loved ones. I was a once proud and independent man. I now have to look to others to do things for me. My wife has had to call the fire department twice to get me off the floor, after I had fallen.

Let us make the decisions about therapies. Stop holding us hostage and letting us die. This is AMERICA and THIS IS INHUMANE!

Gerry E. | Washington

Age: 76

Year of Diagnosis: Neighbor

Type:

We
Can't
Wait

I view any effort to slow the disease progression is important if a patient is willing to use it.

If it were me, I would feel I have nothing to lose and everything to gain.

If I had ALS (a death sentence) I wouldn't care.

There is a person in that trapped body

Why so slow? ALS is horrible.

Mary T. | New Jersey

Age: 68

Year of Diagnosis: 2020

Type: Limb Onset, Sporadic



**We
Can't
Wait**

Very important to have a chance at making life better.

Significant side effects would keep me from trying it

I think if something is working with minimal side effects it should be approved quickly

Totally changed cannot work or walk long distances My hands are weak I cannot lift things or open bottles, or jars am not driving any more used to be the caretaker of my family. Now my family has to take care of me

Very upset that there is no cure. It is underfunded.

Shawnee B. | Florida

Age: 58

Year of Diagnosis:

Type:



Even the slightest bit of hope is an improvement.

Anything would be better than nothing.

With the alternative being death, not important at all.

My Daddy was unmercifully attacked by the cruel unrelenting disease of ALS. After an otherwise relatively healthy 83 years, being an avid runner since the 70's, active in his community, this disease erased a lifelong of responsible choices in the matter of mere months.

No one in our family has ever had this disease, and even if we could have predicted it, there are ZERO options to defend against its ravages. Be warned, it can and does strike anyone as it pleases. It's awful for one to be told not only you face a deadly disease that will slowly lock you into your own body. But to add there are no choices or hope and to be forced to just take it, is devastating for the victim and their loved ones.

Please use your good will, position and influence to shine a light on the need for a cure before it rips and savages another family. **Dad gave his life, spine and brain in his fight.** I carry the torch in raising awareness and pass it to you.

Brian L. | Nebraska

Age: 55

Year of Diagnosis: 2019

Type: Sporadic



We
Can't
Wait

Extremely high. It's all about slowing the train down.

Better to have the opportunity than not

Moderate importance. Better to have opportunity for new treatments before the FDA is involved. They take way too long.

It's Impacted every corner of my life. Put it to you this way, I was able to walk with assistance a year ago and now not so much.

I'm humbled and lucky by all the support and help received by everyone around me

People need to understand once the diagnosis is given a clock really starts. Besides the physical things, **we don't have time to mess around with Traditional slow track medical research**

People with ALS and similar time sensitive diseases are more prone to take on higher risk scenarios than the general population. **Therefore, the FDA process should be fast tracked in a completely different way than other medication**

Patrick R. | Michigan

Age: 50

Year of Diagnosis: 2014

Type: Bulbar Onset, Genetic



Each new therapy that is identified safe and effective in extending life to any degree should be pursued at the behest of leading scientists given the leaps and bounds we can take in development today with a new maps and tools that can be game-changers for research, for families impacted by ALS, and pursuit of solutions for the wide set of currently fatal neurodegenerative diseases.

I am more concerned with potential side effects, and not at all with the potential for what results might return. Even a program that fails to reach or is counter to expectations is valuable for future endeavors. Weighing the benefit of extended life versus types of potential side effects from the new therapy that provides is naturally a unique prospect each time. The most important thing is that a person who wants to answer "yes" to those potentially life changing, or life-saving therapies has the opportunity participate.

It's not important to me. I understand why it is for many people, but I've been educating myself on the disease that's now killed my mother, and three of her sibling, and is a fight to be had for me and potentially my children. I don't want to wait for FDA approval, I want to be a part of the process that makes lifesaving therapies easy to approve for the FDA and easy to access for people who need them in clinical settings.

My aunts', uncle's, and my mother's diagnosis and demise were brutal. Sad. My C9 status has both put me on alert and allowed me to be a resource for finding answers. So, in the end, I feel empowered by the charge to prevent ALS and other neurodegenerative diseases.

The people around me have learned a lot about the disease. My wife has been very strong throughout, but probably worries the most, about me and our kids. I don't worry about me because I don't want to, and I no longer worry about my kids because I know the work being done today will solve the problem for future generations -- If we keep our resolve and continue pressing to find answers.

My goal is for every person who ever heard of the Ice Bucket Challenge to understand that the campaign raised millions of dollars that are vital to the fight against ALS every single day. The person you tell, the dollar you send, and the research program you support WILL make a difference for people with ALS.

Thank you for all of your work in ensuring the safety and efficacy of the things we put in our body. We know that regulatory requirements and safety standards, like medical research and drug development, are complicated matters that require an immense amount of time, patience, funding, intellect. Thank you for what you do and know that families everywhere are grateful for the work being done to improve the well beings of the ones we love.

Theresa J. | Illinois

Age: 80

Year of Diagnosis: 2007

Type: Limb Onset



**We
Can't
Wait**

My Loved One is deceased as of 3 years ago. **We would have welcomed any new therapy, any opportunity to have hope and time.**

I could not accept adverse side effects added to the already existing torture of ALS but if therapy had no benefit or modest benefit, we could not have been worse off.

FDA approval is ideal but traveling to clinical trials is next to impossible and the disease does not allow the luxury of time. Apply "Warp Speed" money please and end this disease!

100% horrible

The worst experience of our lives, the worst pain and suffering. I can't even respond to this without sobbing. **We are shattered, broken, irreparably.**

I ask God to send wisdom and insight to the researchers and scientists for a treatment. I wish people understood this disease has chosen some of the most gallant of our families, the healthy athletes, the community and religious leaders, the parents and our beloveds, it is merciless and offers no hope for remediation.

Don't look at the incidence, look at the impact and the torture

Larry P. | Indiana

Age: 62

Year of Diagnosis: 2020

Type: Limb Onset, Sporadic



I'm not interested in extending this living hell on earth. I need a treatment that leads to curing it or at least controlling it.

I'm only interested in a treatment that leads to curing it or at least controlling it.

Not important whatsoever, if the new drug puts me in a position to be cured.

ALS has and is destroying what used to be our happy little family in our happy little home on a big hill on 15 acres way out in the beautiful country. We are devastated. I feel more sorry for my wife and daughter than I do for myself. Not only do I suffer physically, but mentally too while I watch them. I feel like I'm stranded on the planet Mars, all alone and slowly dying by a thousand paper cuts while strapped in a chair in front of a mirror with my eyelids propped open with toothpicks.

My 19-year-old daughter can't hardly process it. It's like she pretends it's not happening. My wife is being destroyed, one day at a time. I'm quite sure all three of us need therapy.

That it can strike ANYONE at ANYTIME.

Please help us. Please...

Greta L.

Age: 59

Year of Diagnosis: 0

Type: 0

We
Can't
Wait

Extremely important

100% worth it and necessary.

ALS patients have NO other hope, it is a death sentence.

Not at all important to risk everything – it will be worth it.

Robert S. | Ohio

Age: 62

Year of Diagnosis: 2017

Type: Limb Onset, Sporadic



I am advocating for this. I want to continue to be a part of my family. I would be in favor if the benefits outweigh the side effects. If the drug won't kill me, I don't care if they have approved it.

It was devastating. I tried to take the drug on tap, but it made my liver angry. I tried to keep active. My family didn't expect me to be there two Christmases ago. But with my vent helping me breathe I am still here, and I got to meet my 4 grandkids

My daughter doesn't like to visit, she doesn't like to see me degrade, my wife has to take care of me, totally. I am now a quadriplegic I am really sorry she has to do everything for me, my Son convinced me to write stories and I have.

There is no cure, it is devastating to a family and friends.

Every moment counts.



Melanie F. | Texas

Age: 53

Year of Diagnosis: 2020

Type: Limb Onset

We
Can't
Wait

If a new medicine will only work for a few months, it's not worth for a few months.

If it offers more than a few months, worth it.

I am a Caregiver for my mom

There is no cure

How can you come up with a vaccine in a matter of months and no treatment for ALS in years.

Gail K. | Utah

Age: 68

Year of Diagnosis: 2020

Type: Bulbar Onset



New Therapy is vital. I read each day about NurOwn, AMX0035 and other very promising new treatments. **Every ALS patient that meets the criteria should be allowed the opportunity to participate.** Every month and year that passes and we are not given this opportunity it feels very cruel. We should be able to sign waivers knowing that we are taking a risk. I am desperate to live each day the best I can, as long as I can. **Please seriously consider what ALS patients are facing. We are desperate for anything that will give us the "Gift of Time"**

When a person has a terminal illness, this should be your Right to make this decision, not the Government.

I realize the FDA serves a purpose in society; however, we are educated people and we should be allowed to make these decisions and accept the consequences.

It is very difficult to know each day that you live in fear of the next day and have nothing to cling to for some sense of hope. It is extremely difficult to read about new medications that are showing promise and not being allowed the opportunity to try.

It is very difficult for all my family and dear friends to know there is nothing that they can do but pray and show support.

People know very little about ALS. People say "Can't you exercise in order to not lose muscle? Speech therapy will help you."

I know that I did not know much myself about ALS before my diagnosis. However, I knew it was a terminal illness. It almost seems as though until recent years ALS was not a disease that was given much research or concern. **I know that now this is not the case, therefore please give ALS patients the dignity and hope we deserve.**

Jennifer B. | Ohio (1 of 2 pages)

Age: 52

Year of Diagnosis: Mom: 2020

Type: Sporadic



**We
Can't
Wait**

I helped take care of my Mom, who was diagnosed in late October/early November of 2020 and sadly passed away in April of 2021. She had been diligent in her health concerns, spending years going to doctor appointments, etc.. to see what was causing her foot drop. **When she was finally diagnosed with an advanced stage of sporadic ALS at age 79, she was determined to fight. I tried to get her enrolled in clinical trials, but she was denied based on the advanced stage of the disease, unfortunately.** We continued researching therapies and drugs/medications to slow down the progression, as she had so much more to share with us and so many more lives to impact. Providing her with a choice in her battle is benefit enough, in my mind. So, a modest benefit such as slowing the disease or providing a few months of additional life was not offered to her, the choice in what she wanted was a luxury that was abruptly taken away upon receiving the diagnosis. This choice should be given to those that are battling this disease, as I believe that any person in my Mom's position would welcome an alternative to what they are given with this diagnosis, such as a new therapy.

I would weigh the benefit of any therapy that might end up having no benefit at all or has a significant side effect to my ability to have a choice in my approach to my fight. My Mom was given the choice of taking Radicava or Riluzole, both drugs used with ALS patients. She chose Riluzole, understanding that the drug might not have any benefit or cause side effects for her. She made this choice to aid in the fight for her life.

The clinical trial process and the FDA have rules and regulations in place for keeping people safe. However, if given a diagnosis, such as ALS, with no known cure or cause, my Mom, and possibly others, would welcome the advancement of determining if medications could prevent their outcome of a death sentence in their fight. Suffice to say, my Mom was interested in clinical trials, if they could offer some insight, breakthrough, or an alternative to what ALS did to her.

I can only answer for how this diagnosis impacted her based on my time with her. Upon hearing the diagnosis, she stated that this diagnosis was a death sentence, and she knew what her future held for her. I recall sitting with her one day and she looked at me and said, "**I miss my life.**" **ALS is cruel, taking away her independence, dignity, and her voice. Your mind and soul remained unchanged, so you are keenly aware of all of the abilities you find are no longer part of your routine. You miss conversing, expressing your joy, feeding and bathing yourself, walking into the room and so much more. In essence, ALS impacts the person and all of those who love them, as we are all left to watch helplessly as the disease progresses.**

Jennifer B. | Ohio (2 of 2 pages)

Age: 52

Year of Diagnosis: Mom: 2020

Type: Sporadic



**We
Can't
Wait**

Since my Mom is no longer with me, I know how being diagnosed with ALS impacted our family. We all began to fight with her to help her, advocating for physical therapy to maintain what strength she had left, finding ways to raise money to help with the 24 hour care that soon became the norm, and spending 4 hours driving in the car to provide assistance and time with her for encouragement and aide in her care. Due to the pandemic, this created a whole new hurdle for us, as our family knew the importance of keeping her safe, but also how important it was to spend time with her. ALS did not define who she was, but instead proved the value of her legacy.

My wish for what people understand about ALS is that it is not curable, there are no treatments available to stop the progression. Those with ALS will not get better. ALS is more commonly sporadic than hereditary, anyone can be diagnosed, at anytime during their lives, as this affects old and young alike. ALS is cruel, as it takes away what so many of us take for granted, while you sit and recall in your mind the things you were doing a year before the diagnosis. My wish is that those with ALS are given more, in terms of studies, money for research and funding to find a cause, they deserve better than what they are given. Those diagnosed are so much more than what others see in pictures, they are full of life, with many adventures left to explore, and many more holidays and family celebrations to enjoy, and so much love to share with others.

I would like the FDA and pharmaceutical companies to know that my Mom was more than a statistic, she was more than one of the many that pass away from ALS. More than the Member ID number on her Medicare account. She was in the fight for her life and was not given the adequate supplies to wage her battle. The cost of her one and only medication, Riluzole, was astronomical. She and her husband are retired educators and cannot afford the cost of the many medications she required with her diagnosis. I would like them to know that waiting on approval for a new therapy or medication costs lives, ALS patients do not have the luxury of time on their hands. **I appreciate your job and the responsibilities that come with your line of work, but please remember your impact on those fighting for their life and discover ways to improve the process of approval or costs for those that may need it.**

Mariano A. | Argentina

Age: 39

Year of Diagnosis: 2021

Type: Limb Onset



Time is life, every minute count, all trials is hope"

With ALS only Want time until the cure be available

I am filled with...

Depression.

Sadness and depression.

The important time to find a cure. Every minute is life.

All patients of ALS in the world needs gift us time for enjoy our family.

Robert P. | Pennsylvania

Age: 68

Year of Diagnosis: 2019

Type: Limb Onset, Sporadic



We
Can't
Wait

Very important to have the chance to try something that will help. I would look at the significant side effects and make a determination.

I would assume a risk if the drug has shown positive effects and is not FDA approved. It is a very scary diagnosis, but I try to live day to day.

It has affected my wife the most since she is my caretaker. The devastation of the disease. **Those of us diagnosed with ALS need help quickly.**

Angela F. | Montana

Age: 47

Year of Diagnosis: 2013

Type: Sporadic



Very important to me to get on the list for any drug regardless of any side effects.

Has limited what I can do and my quality of life has diminished

How it takes away your independence is awful.

Please let us try new medications, it takes too long for the FDA to approve

Lynn M. | Wisconsin

Age: 70

Year of Diagnosis: 2017

Type: Limb Onset

We
Can't
Wait

I think that all new therapy is promising. While not curative, new therapy provides hope to those who have ALS. In my husband's case, **he was very interested in starting therapy that only slowed disease progression and continues to take Rilutek even though he is currently in the end stage of his disease.**

All new therapies are relatively untested in the general population and for each person, their response to the therapy, whether new or still in clinical trials, is unique. The responses to therapies under study can range from no benefit in some people to a significant benefit in others. Side effects are to be expected. **If a person has significant side effects, they and their doctors can decide - on a case-by-case basis - whether it is worth stopping the new therapy.**

It is not important at all for me, although I have been a cancer research coordinator in my career. It is very important for my husband that the FDA has weighed in on the new therapy. He is not interested in therapies in clinical trials (he is the patient) so we have not pursued any of these.

Our entire life has been impacted. Currently my husband, who moved to Colorado to hike in the mountains, is totally unable to move at all, and needs total assistance with all of his ADLs, including turning in bed at night. He uses a ventilator (without a trach) as assist breathing sporadically during the day and all night long. We have moved to Wisconsin now to be close to family, although he never wanted to move back to Wisconsin.

I am his caregiver. Although I am an RN with 43 years of experience and am very able to deal with all of his ADLs and therapies, I am with him 24/7 and have to get up with him several times during the night to turn and use the urinal (which I have to place). **This is the hardest thing I've ever done in my life.** Currently, my grown children are helping with his care. My adult son has basically moved in to help and works from here on his computer. My daughter in law comes at least 2 days a week to help with cares although she works full time and has 2 teenagers at home. My husband's sister who lives in Florida is very upset that he is doing so poorly.

That it is worse than cancer. No effective therapy and no cure. More research is needed desperately to develop effective therapies and a cure. **ALS is a death sentence.**

This disease needs more funding and new therapies need to be approved quickly. If you get ALS, currently, it always ends in death and pain for the family.

Kent D. | Indiana

Age: 58

Year of Diagnosis: 2014

Type: Bulbar Onset

We
Can't
Wait

Anything that gives hope is worth it!

Anything is better than nothing

As long as it can't do more damage, I'm willing to try anything.

Still run a company but cannot communicate, tire easily, in a powerchair, can't play with grandchildren

Taken a toll on them, while I'm blessed to be able to do some things, always dependent on family and friends around me

Very little trials available and not enough money going into research. **One of few diseases since 1939 that really made no significant progress in slowing down**

People are praying for a breakthrough, in a cure, therapy or something to significantly slow this disease down. Don't you?

Rick M. | Florida

Age: 67

Year of Diagnosis: 2019

Type: Bulbar Onset



Any treatment that can give me more time with my family would be a godsend. However, if that treatment requires me to sit in a hospital for weeks every month, then I would not be interested. Traveling to hospitals or being confined to one isn't beneficial to me or my family, not for the "possibility" of only a few months.

That may be just what I need. Yes, even with the risk, that might be just the ticket. Look at the risks and rewards and side effects of the two "approved" drugs currently available to us. **The "risks" may well be worth it.**

Not important at all. **After all the dealing with COVID, and previous ALS trials, I have very little faith in the FDA even caring about ALS patients.**

My diagnosis was devastating, but nothing compared to actually having this dreaded disease. My wife and I had just retired and had extensive travel plans, most of which was canceled. **I have gone from walking and talking to mumbling and spending 16 hours in a power wheelchair.** It's been hardest on my wife, knowing that the man she married and planned on getting old with won't be there for her.

I would wish that everyone would know that ALS is always fatal and nothing can change that. That funding for AIDS and erectile dysfunction took precedence over research for ALS. That not enough people die from ALS to make it worthwhile for Big Pharmacy to care about.

Just one question... when all the other diseases have a treatment, will you then decide to bother helping the 6000 people who get ALS every year? We all know why there is no cure...Money. It just isn't profitable.

Scott M. | Pennsylvania

Age: 52

Year of Diagnosis: 2019

Type: Limb Onset; Sporadic

We
Can't
Wait

It's very important! I for one don't like trying to figure out what's the next thing to stop working and when. It's better than just waiting to die.

If it shows any sort of benefit, I would take it. **I'm not ready to die.**

Too many ways to list here.

How horrible this disease is. It takes your ability to do everything away from you, and you're fully aware of what's happening to you and the FDA sits on their hands while people die.

I'm not ready to die. I think most people with ALS feel the same way.

Get drugs in bodies as soon as possible!

Christine Ann H. | Colorado

Age: 55

Year of Diagnosis: 2019

Type: Limb Onset; Sporadic

We
Can't
Wait

I would be grateful to have medication that would slow progression, but **I want more than a few months.**

I think it's important to be allowed to try any medication that might have even a modest benefit.

It is not important at all.

ALS has taken so much away. **I was a nurse for 26 years.** I worked in Oncology and Hospice. **I administered life saving treatments to cancer patients, now I have a disease without a cure.** I am now unable to work. I moved to Colorado 15 years ago because I love the mountains and thoroughly enjoyed hiking. That has been stripped away. Independence was always very important to me. **Now everything is a struggle.** Shower days wipe me out. Getting dressed takes a long time and sometimes I need help. The fatigue that goes along with ALS is maddening. I am often too tired to socialize. If I have plans, I have to ensure that I get 9-10 hours the night before, then do very little leading up to the activity. An example: I can't clean the kitchen and go shopping the same day. It's just too much. **I think the shorter answer would be to tell you how has ALS NOT affected my life.**

My fiancé lost his hiking partner. That was one activity that brought us together. **We had planned on hiking together for the rest of our lives.** He now has to do much more. He does all of the cooking. He has to do a lot of the cleaning. He has to help me, at times, with getting dressed. Eventually I will be completely dependent on him for all of my care. ALS has had an emotional toll on him. He worries about me if I don't answer the phone, thinking I may have fallen again. He worries about how we will pay for all of the expenses coming up. **He worries about how much time we have left together.**

I wish people understood that it is terminal. I wish they could see a day in the life of someone, through every stage of the disease. I want people to know how cruel this disease is.

I would like them to know that we are going to die and we will try anything. Most people with ALS are trying every supplement that has been rumored to have a benefit.

Hossein O. | Kentucky

Age: 70

Year of Diagnosis: 2016

Type: Limb Onset, Sporadic



Very important especially young pals with young spouse and children. It's empowering to most pals to know that they are doing something to fight back and not give up.

ALS is 100% fatal is not just a slogan for fundraising. Most pals are willing to take a chance with side effects and more complications just to have a chance specially if some of phase 3 trials show some benefits like NurOwn.

Totally unimportant. **By the time a drug is approved by the FDA, I would long be dead and buried.** I rather have access to AM0035 and NurOwn NOW while still here to potentially benefit from. How many drugs have been approved by the FDA in the last 50 years for treating ALS?

I no longer have a longer view of living my life. **I can only think of living on day at a time.** Working with my arms is challenging. Bathing and dressing is hard without assistance. I used to run long distances but I can't even run 200' due to weakened neck and upper body. I can't do routine home maintenance, cook and help my spouse with chores.

I have an amazing wife/CALS. Very mature individual. I am still enjoying an FRA-R of 35 but she still ends up doing most of the work around house and when traveling. Intimacy is a challenge. Our kids are grown and so far have managed life without their help.

I am not concerned with people's understanding of ALS. What can they do with a better understanding of ALS when the providers, insurance companies fail addressing our needs? FDA, Congress, ALSA lobbying efforts, all fall short.

Find ways to remove the obstacles so the RTT act can be better utilized to make promising drugs more accessible. Addressing the funding shortfalls that prevents pals to seek treatment specially if drugs not yet approved.

Lisa M. | Pennsylvania

Age: 53

Year of Diagnosis: 2021

Type: Sporadic

We
Can't
Wait

Very Urgent that All ALS patients be provided with ALL options to all and any Trial Medications

As previously stated, the **person living with ALS should have the option to decide** Not the Doctors or the drug companies .As we know each person with ALS can react differently

Very important to be included and complete a clinical trial process.

For me this was life altering . At 53, I am now waiting to see if I will live to see my grandchildren grow up with No understanding of how and why I now have ALS

My children are suffering not knowing what will happen to their mother

That All Person with ALS can progress differently . I would wish that the Neurologist would do more than give a diagnoses then say return in 3-4 months when this is precious time of a patient's life

My life is on my hands ,The drug companies and Neurologist are playing God with our Lives

Nina W. | Kentucky

Age: 67

Year of Diagnosis: 2016

Type: Limb Onset



I would participate in such therapy if it was not a major inconvenience such as daily injections or travel requirements

I would definitely take an untested therapy if it possibly had no benefit at all. I would have to waive the benefit against their significant side effects if that were the case.

Not at all

Why would you ask that? It has completely ruined my life and taken away many of the things I value. Also, it's going to kill me.

Most of my family and children feel helpless and sad. My husband who is my caretaker is depressed and completely worn out.

That it's a horrible disease and there is no cure

Barry T. | Temperance

Age: 66

Year of Diagnosis: 2020

Type: Bulbar Onset

We
Can't
Wait

I no longer can drive a car, dress myself, or reach for anything. My food has to be cut up like for a 3-year-old. My balance is off. I have only been diagnosed with ALS for 6 months.

My wife must do most things for me now

How insidious this disease is and how anybody can get it.

Get off your ass. I should not have to die because of your ineffectiveness.

Please help me!!

Michael O. | New York

Age: 59

Year of Diagnosis: 2015

Type: Limb Onset, Sporadic

We
Can't
Wait

ALS has Changed everything. I cannot walk and I am in a wheelchair. **Any medicine is important.**

It has made my caregiver life more difficult

That it is terminal, and we need therapies to slow it down

Give me access to experimental drugs now

Cathy S. | Colorado

Age: 64

Year of Diagnosis: 1954, 1990, 2017 (Family)

Type: Genetic



**We
Can't
Wait**

Life changing, any help for slowing progression until a cure is found.

The risk is worth the outcome, family members are already dying.

Not important- acceleration needs to happen and **risk is worth it all.**

LOSS! A FUNERAL everyday of a LOSS of physical function- grieving each and every physical loss daily- a daily funeral of loss.

FEAR, SCARED, TRAGEDY, LOSS, We are in a club we do not want to be in.. ALS

That outcome is HOPELESS and has been HOPELESS for 150 years with NO CHANGE is treatment or cure.

That we are ALONE! Only help for us is talking with other patients. Neurologists say- get your affairs in order, you have 2-5 years to live. That is the only treatment.

Alfred A. | Bullhead City

Age: 77

Year of Diagnosis: 2009

Type:



It is important to have access to medicine. ALS is devastating.

I am filled with sadness that they couldn't do more to improve my quality of life.

The ability to do the normal things of life leave you. Like walking, swallowing, picking up a spoon, bathing yourself, etc.

David H. | Michigan

Age: 44

Year of Diagnosis: 2020

Type: Limb Onset, Genetic

We
Can't
Wait

Very important. It could buy time to for the next therapy that might be better.

The alternative is nothing. Side effects would be a matter of how much one can tolerate them.

If there is sufficient evidence to show some efficacy, I would take it. I can always stop if side effects are too bad, or the FDA later finds a problem.

I am devastated. We are devastated.

ALS is the most complex disease and the least funded. A breakthrough for ALS could lead to breakthroughs in other neurodegenerative diseases.

How would you handle watching a parent or your child die from ALS?
Imagine total paralysis, a feeding tube, loss of speech, ventilator and finally death by suffocation.

Tara B. | Washington

Age: 51

Year of Diagnosis: 2020

Type: Limb Onset



We
Can't
Wait

This would give a quality and quantity of function and independence that would make a real difference

Not sure but willing to try if recommended by neurologist team

Very important

Fear, anxiety, sadness, insomnia, nausea and anticipating needs of family and response of employer and friends

Fear, anxiety, sadness, insomnia, nausea and anticipating needs of family and **will the wife and very young adult kids be "okay" when my brother is gone**

Its hard, its real and it needs effective treatment options in a timely manner funded by the government

Do not stop development, please...

Lisa M. | Pennsylvania

Age: 71

Year of Diagnosis: 2017

Type: Limb Onset



Give ALS patients ALL ACCESS to all therapy options! VERY IMPORTANT for ALS patients to have HOPE, CHOICES and OPTIONS, even if modest benefit. When my mom had ALS, she was barely even offered any clinical trials for participation!

EACH patient needs to be able to make their OWN CHOICE how they feel about the risk but ALS IS A TERMINAL ILLNESS....Any therapy offering time and hope is important

OF NO IMPORTANCE at ALL to wait on the FDA! The FDA operates at snail speed.....ALS is a terminal illness. Give patients the options to try therapies/and/drugs. **I bet 95 out of 100 ALS patients would agree- SPEED this UP ASAP!** People are literally DYING everyday while someone at the FDA is slowly pushing papers in a clinical process. TIME is of the ESSENCE! Forget the FDA process -- if a COVID Vaccine could be done and available in 6-7mos, the ALS options are possible too!!!

My mom had ALS and she passed in 2018 at the age of 75. She survived just shy of 4 years after diagnosis. **ALS impacted EVERY aspect of our family's life for many, many years.** It was devastating, sad, caused financial hardship and took away my mom's chance to enjoy life, retirement and her 50+ year marriage with my dad. We must find a cure NOW.

My dad, my brother, myself, our spouses, grandchildren and my mom's siblings were all impacted and lost a wonderful person to an awful cruel disease.

ALS takes away your life, your breath, your dignity, your happiness and everything you care about. It is a cruel and awful illness that is not understood by most people. **I implore you to toss the clinical trial process, the FDA "rules" and FIND A CURE NOW!**

This past pandemic year has been especially cruel to ALS patients/families because ALS is SO SO SO SO SO SO much worse than COVID but no one seems to care in finding a cure. Again, if science can figure out SEVERAL Covid vaccines, Covid infusions, etc. in only 6-7 months time, we can solve the ALS mystery and find a cure!

Michiel H. | Oregon

Age: 70

Year of Diagnosis: 2014

Type: Limb Onset



We
Can't
Wait

Extra months is not really what is worth the effort. Early detection with treatment is more important

ONLY AT ONSET OF DISEASE.

In the cases of ALS, promises of one to six months is not worth the effort

It has ripped our life's apart and the lives of those of the family members to the extent that all joy of life is stripped from us

It has destroyed our way of life and taken away all hope for the future and taken away basically all friends and intra actions with anyone outside the immediate family

They need to understand what a devastating disease that it is and what an impact it has on the lives of those that have been diagnosed and the need for support from extended family. **They will never know what that means the to the person the has the disease and the main caregiver**

They need to understand the disease and what it does to all involved and stop trying to make their fortunes on the backs of the people that have the disease. They need to live day in and day out with those that have the disease so they can get the full impact of the disease. **God forbid they be diagnosed themselves.**

Candice P. | Michigan

Age: 37

Year of Diagnosis: 2019

Type: Limb Onset, Genetic



New medicine? Anything is good. Anything to slow this monster disease. **I have 3 children under 8 years old and I only have a few more months with them.**

Depends on severity of side effects whether I am willing to risk the worst. Modest benefit is good. Not important at all to have FDA approval. If it shows promise..... put it in my body.

I can't live a normal life. I can't play with my kids. I cry over not being able to see them grow. I am sad all the time.

Everyone is sad. Everyone has more work. Everything "normal" is curtailed.

It's a monster. Leaves you helpless while your mind dwells on what could have been, what you're losing

You have given us nothing. No hope. There must be a new and faster pathway for patients with ALS. We've seen you pull out all the stops for Covid. While not apples to apples , we deserve to live. Throw us a bone.

Eudonna L. | Pennsylvania

Age: 63

Year of Diagnosis: 2019

Type: Genetic

We
Can't
Wait

Very important to have medicine. Any medicine.

There is no hope, only hope is you start trying anything you can!!
Very important to try anything and everything.

It has ruined life for him, 48 years old, you must be kidding

They only have hope to cling to

It's awful effects, A death sentence, you need to start helping!

These questions are an embarrassment, It is sad you would ask these, when you know how bad it is

Age: 20

Year of Diagnosis: Dad: 2020

Type: Bulbar Onset

We
Can't
Wait

The ALS community is desperate to place their hope on a drug that can potentially offer modest benefits. As of right now the available ALS medication that my dad was given, Riluzole, is just a band aid. There have been zero significant findings that Riluzole helps an ALS patient in any way. There is no guarantee the medication will give you an additional three weeks or three months of life. When my dad was first diagnosed with ALS in December of 2020 the Bulbar onset ALS specialist with Kaiser Permanente said they did not believe in it because it is not an effective medication. Every ALS patient responds differently to medication and the medications that are available to ALS patients today are not promising. ALS patients need a variety of medications, treatments, and therapies available to them in order to prove the effectiveness of each one. What do ALS patients have to lose? Their disease is 100% fatal anyway.

I would weigh a new therapy that offers the change of a modest benefit in balance with the risk that the therapy ends up having no benefit at all to a therapy that also has significant side effects all on the same scale. To me there is no reason to be holding medications back from ALS patients that could be risky, could have no benefit at all, or could have significant side effects. ALS patients are diagnosed with a disease that has absolutely NO HOPE IN LIFE. ALS patients are diagnosed with a disease that is 100% fatal. 100 % fatal. I would rather my dad, whom I am a caregiver for, trial with medications. If he were to pass because the medication failed him and many other ALS patients who were trialing the same drug, then the FDA can rule out that the drug they were in trial for and not give it to future ALS patients. Our ALS patients need to be tested with the medications, treatments, and therapies so that the future of ALS can have hope. Our ALS patients have nothing to lose at this point.

It is not important the clinical trial process is complete, and the FDA has approved it. As I mentioned above. Current ALS patients need to be the ones testing the medications, therapies, and treatments that are being held up so that future ALS patients can have hope. There is no reason why ALS patients who are willing to be involved in a trial, even outside of being approved, should be denied the right to test the drug on themselves. This disease is 100% fatal and they have nothing to lose.

I am my father's caregiver and am being is advocate in my responses. My dad can no longer communicate and therefore I need to be his advocate. Many ALS patients can not communicate and that is why ALS awareness is not as big as it should be. Once ALS patients can no longer be "normal" in public they stay at home and hide because they are ashamed of what their bodies have become. The public eye does not see what ALS does to individuals and unless you have known someone with ALS or have an ALS patient in your family you have no idea what ALS is like. It is something only experience can show and those who experience someone with ALS need to speak up and show even the hardest parts. No one understands.

Brenna N. | California (2 of 2 pages)

Age: 20

Year of Diagnosis: Dad: 2020

Type: Bulbar Onset

We
Can't
Wait

My dad's diagnosis has impacted my life greatly. I now have no doubt in my mind that I will forever be an advocate for the horrible disease ALS. **ALS is a nightmare that I wish I could wake up from.** I am a 20-year-old full time college student who balances my father's needs with the needs of my own. I can no longer go out and have the college and fun experience with my friends because I am needed at home. **Every little moment with my dad is so precious even if I am just talking to him while I push water through his feeding tube.** My dad's ALS diagnosis has significantly changed the way I view life and forever impact me in all that I do. Life is precious and every moment matters.

I wish people understood ALS on a deeper level. I wish I new more about ALS before my dad was diagnosed. I knew a family whose dad passed from ALS and all I knew about the disease is that it affected his muscles enough to be put into a wheelchair and nothing more. No body, including myself before my dad was diagnosed, truly knows what ALS is. People need to be exposed to all the details of ALS and not just the surface facts. I have to use the suction machine multiple times a day in order to try and clear my dad's throat of mucous. No body knows the extent of ALS and how horrible of a disease it is. Surface facts are not enough.

Medications, therapies, and treatments of ALS should not be held back if an ALS patient has a ZERO PERCENT chance to live anyway. **I am here to fight for my dad and all ALS patients present and future.**

Adam S. | New York

Age: 41

Year of Diagnosis: 2017

Type: Bulbar Onset, Limb Onset, Sporadic

We
Can't
Wait

Extremely important to have access to medicine.

**Not important to me to worry about anything else
that could happen by taking this medicine.**

It has destroyed my life

It has destroyed the life of my family

That there is no cure

Please give us a chance to try new medications that
have passed Phase 1 and 2 trials

Suzanne G. | Delaware

Age: 59

Year of Diagnosis: 2021

Type: Limb Onset

**Click
Here to
Play
Video**

A new therapy is extremely important. It is extremely important to me that the clinical trial process is complete, and the FDA has approved it before you take a new drug.

I would like to choose for myself

It has completely changed my life. I can not walk. I have no strength. I fall a lot. I am dependent on others. **I was set to retire. Now I'm set to die.**

It has caused extreme anguish and grief

That it is terminal. There is no cure. You become completely dependent on your family- when you were looking forward to your golden years.

My dad died from ALS on April 29, 2015. My husband was diagnosed with ALS on January 11, 2021. **ALS is NOT a rare disease**

Terry M. | Indiana

Age: 60

Year of Diagnosis: 2017

Type: Limb Onset

We
Can't
Wait

Totally agree. Just want the chance, **I'm going to die anyway, why can't I just have a chance to try?**

It's for me to decide. I decided I would try any and all therapies. Just give me the chance. Don't let me die waiting.

I'm am going to die anyway, I don't care if FDA had approved or not. Give me the chance to try.

It's monstrous for me and my family. No human being should have to suffer like this.

It had taken its toll on my immediate family. We are all exhausted, frustrated and just trying to get through each day. There is no time for enjoyment or tender moments.

It's ALS 24/7, no breaks for anyone involved. You and your family just endure suffering until you die. **There is absolutely NO HOPE.**

Give me a chance to try anything I want, it's my life. I have a disease that's 100 percent fatal. If ALS was contagious like COVID I know there would already be a cure.

Shelby T. | Georgia

Age: 29

Year of Diagnosis: 2021

Type: Limb Onset



My mom is hopeful, ready, and willing to receive experimental therapy. This time last year, she was in the best shape of her life. She was competing in races, excelling in workout classes, etc. Just one year later, all of that has been taken from her.

It is a risk that she is willing to take. Considering that this a terminal illness, why not experiment with something that even has the slightest potential of increasing her life here on Earth?

If the trials have proven to be safe and effective, FDA approval is not as important. Take the Covid-19 vaccine for example. It is not fully approved, yet it is used for emergency use. Is ALS not an emergency? ALS is terminal, in all cases.

I can only speak for myself and not my mother.

My mother's diagnosis has affected our entire family, friends, etc. It makes the future almost unimaginable. **The progression of the disease comes with absolutely heartbreaking situations.** Anything that could possibly slow the progression is absolutely necessary.

I wish people were more informed about the disease. I wish the masses were fighting for a cure, and not just those that are affected by it.

We need this now. ALS patients cannot wait! They simply cannot wait. Each day can come with new obstacles. My mom can talk today, but she could begin to slur her speech tomorrow. My mom can walk today, but her legs could become weak tomorrow. There is no waiting period. The time is now. **Please help this community of people that are begging for your approval and begging for added life.**



Carol L. | South Carolina

Age: 64

Year of Diagnosis: 2016

Type: Limb Onset

We
Can't
Wait

Extreme importance to have every opportunity to get medicine.

Extreme importance to find a cure.

Extremely important to try everything.

Extremely Impacted my life in a negative way

Impacted my life in a negative way

It affects every part of life for me and my loved ones

I am desperate for drugs that will slow progression and pray for a cure in my lifetime

Richard C. | Florida

Age: 57

Year of Diagnosis: 2019

Type: Sporadic



From the time of my diagnosis it is always in my mind when will I pass. The longer time passes I wonder when I close my eyes at night will they open in the morning. My feelings are we have been given a death sentence so what do we have to lose. What we could possibly gain is life.

Any medication is worth the try no matter what.

It has taken everything from me. My job my house my motorcycle and most importantly my independence.

My daughters have altered the lives to be near. And it pains me to have to see them hurt because of me.

That it's a death sentence. Before my diagnosis I new very little about ALS.

It is torture knowing your time is very limited.

Johnny F. | Florida

Age: 62

Year of Diagnosis: 2019

Type: Limb Onset, Sporadic



We
Can't
Wait

Anything to let me live longer. Who would take that choice from me? It is my choice. Just like cancer, all you can do is hope for the best.

FDA is taking my choice away from me when it restricts access to new and potentially life saving drugs. **The key part of that is "my choice", not the government, and that should be an inalienable right for all people.**

Mobility, planning for the future, more hope and more let downs

They took more time than I did to come around to the idea that I may not be around for long.

How badly it is underfunded. Research needs to get more funding to speed up finding a cure.

I am lucky the VA helps pay for my treatment. **We need to elevate the importance of the little people who are dying from these diseases, who are in poverty because it costs so much to treat.** I must have resources generate 150k per year to pay for 1 of the 2 FDA authorized treatments. **People cannot afford this, price controls please.**

Percy M. | Georgia

Age: 72

Year of Diagnosis: 2010

Type: Limb Onset

We
Can't
Wait

It would be very valuable and important to have access to medicine. **If you don't try you fail.** Important to try everything.

Lifestyle change, new habits from eating to getting dressed. They have become adjustable to my needs.

ALS is helplessness beyond your control. Research is important to our society.

Kerry P. | Georgia

Age: 69

Year of Diagnosis: 2017

Type: Bulbar Onset, Sporadic



I was willing to receive the Moderna vaccination although it was only “an emergency” vaccination. I would like to be given the opportunity to decide whether or not I take a drug for ALS.

Besides losing the ability to walk and talk? The loss of independence has been the hardest thing for me to deal with. In spite of it all, I have been able to keep a positive attitude.

My husband who is my caregiver has had to do a lot of things I used to do. In addition, he’s concerned about leaving me for any length of time, so he rarely gets time for himself.

There is no cure. **The very few drugs there are available to treat ALS really don’t help much.**

Vincent M . | Tampa

Age: 86

Year of Diagnosis: 0

Type: Limb Onset, Sporadic



**We
Can't
Wait**

We have developed a natural occurring metabolic treatment protocol, The Deanna protocol [DP], that makes patients more comfortable by suppressing incessant muscle symptoms and slows disease progression. Further in 2015 we have documented a borrelia infection as the etiological agent and treated the infection. My daughter's disease has not progressed since 2018. Other pALS have experienced similar results. Neuroscientists are reluctant to accept these results using the [DP] and have refused to fund clinical trials on the DP. Also, clinical trials should be funded at a specialty laboratory to document the presence of a bacterial infection in ALS. Then fund a trial on appropriate antibiotic Rx.

Not rewarding

The clinical trials are in the wrong direction

My daughter has overcome her disease but before we knew its cause was bacterial, she was treated only with the DP and lost muscle function until we eliminated the bacterial infection in 2018. We are now in the process in attempting to retrieve her muscle function.

Changes all our lives

It is curable and early Rx is important b/c once a nerve cell is dead it is not retrievable.

Open your minds and save millions worldwide from this devastating disease. See our results at the link below. Please help me help these patients. <https://winningthefight.org/success-stories/>

George R. | South Carolina

Age: 66

Year of Diagnosis: 2014

Type: Limb Onset

We
Can't
Wait

Please. Bring it on. Anything is worth trying.

Trying it if possible

We did not test vaccines for Covid. So, I Would try anything.

Lost my benefits for 33 years of work. Diagnosis was untimely in my case.

People assume Weakness. My opinion is that in my case. I'm tuned in to things more deeply. Very frustrating.

Adaptable vans, lifts, and wheelchairs need to work together. Someone should help. **Please help.**

Amanda C. | Arizona

Age: 36

Year of Diagnosis: 2016

Type: Genetic



My Mother could have enjoyed a longer time on earth, with more capabilities. Instead, she lasted three months from being diagnosis.

I think having that chance is so important, not only to the individual but the family. Me personally, I had tried anything to stay on this planet with my family.

If I know I'm going to pass, the FDA approval is not important to me. I would rather have access, knowing that I could help improve others' chances.

The only word I know to describe is heartbreaking. I watched my mother deteriorate in front of my eyes. Everything she once was independent at, turned into having to rely on everyone around her. I had to take family leave with no check to help my father with my mother because we could not afford to hire anyone to come help.

Not only did I watch my mother die before my eyes, I watched my father fall into pieces over the love of his life. It is a cruel disease.

Its not pretty. There is no cure. And it is a death sentence. Its not like cancer, where there can be hope. There is no hope with ALS. You just have to learn how to live with that fact, both individually and as a family.

We are suffering. It's not about the money, it's about lives.

Diana H. | Michigan

Age: 71

Year of Diagnosis: 2020

Type: Limb Onset, Genetic

**We
Can't
Wait**

Any therapy would give a PALS hope. Very willing to try any therapy.

I have not seen many of my family. Hard to not do many of the things I used to do. My husband is tired.

No cure

Speed up use for PALS

Allie S. | Tennessee

Age: 30

Year of Diagnosis: 2017

Type: Limb Onset, Sporadic



I would happily pay any amount of money that I can afford to just have a chance at receiving a medication that seems promising. ALS is a confusing disease that affects every patient differently. We will never have the chance of knowing if a medication could work for even a small subset of patients if we're never given the chance to try. I would consider taking any medication that could extend my life even for a few months.

I would try any medication as long as it didn't present side effects that would negatively affect my ALS diagnosis, such as respiratory involvement.

Absolutely do not care about FDA approval. I'm dying. I don't have the time to wait.

Everything is different. I can no longer work. It has caused stress and financial strain on our family. I just want the chance to watch my son grow up.

I wish people understood the swiftness of the progression. For many, it's a matter of a few months between recognizing your first symptom and having one of your limbs completely paralyzed. I also wish people realized that this disease isn't THAT rare - 1 in 300 people will be diagnosed with ALS during their lifetime.

Patients and families are willing to pay the money to just have a chance at extending their life. Please make it available to us.

Robert M. | Florida

Age: 76

Year of Diagnosis: 2016

Type: Limb Onset

We
Can't
Wait

Constantly looking for any breakthrough. With modest anxiety I am certain anything that provides hope is worth it.

Literal game changer, a total change in the quality of my life. I loved my job and had to give it up. I now and 95% wheelchair bound

Horrible stress on my spouse, I'm having to depend on her constantly for any movement. **Very discouraging when you can't pull up your own pants or put on your own socks.**

How debilitating and stressful it make you feel. Your helpless and all that can be done is monitor where you are with your physician

Losing muscle strength and the withering & deterioration of your body is Devastating

Linda K. | Fillmore

Age: 58

Year of Diagnosis: 2013

Type: Bulbar Onset



Any benefit no matter how small, is vital when faced with a 3–5-year prognosis. Let me try it!

The impact of the side effects on my quality of life would be a factor in my consideration of an exploratory treatment.

I would trust my doctor prior to authorization by the FDA and take it with their recommendation.

I went from being very physically active to basically quadriplegic in four years time and I have had to adapt along the way. My biggest growth was in graciously accepting help as needed.

It has created stress and sacrifice in the lives of my immediate family that they deal with on a daily basis.

ALS is a neuromuscular disease that doesn't affect the minds thinking and processing. Take the time to converse and be patient with alternate forms of communication.

Just keep in mind that small increase in time is important when your time is limited conclusively with ALS.

David C. | Sanford

Age: 65

Year of Diagnosis: 2018

Type: Limb Onset



We
Can't
Wait

A few months only extends the suffering.

My balance is compromised, and the therapy is not worth the risk.

If it is showing significant positive results in animal studies, I would definitely be interested in NU 9.

It has taken my ability to walk, speak, and have trouble swallowing. Limited income. Can't get disability. I'm a self-employed carpenter still doing what I can. The bills are overwhelming. The stress does not help the disease. It's too much for my wife to handle. I will not consider a feeding tube or breathing machine. **I do not want to extend my life because it will be too much on my wife.** If motor neuron diseases were a virus, we'd have a cure.

It's been devastating..

I wish the public had more knowledge of the disease. Then maybe there would be more funding and more support. More pressure on the FDA.

There are drugs on the market right now to help upper motor neuron disease. **But they are being held up because of the FDA. Why? I would be willing to be a part of an animal study if it shows positive results.** NU 9

People are dying with no hope. The FDA doesn't seem to care. Give us a little hope and opportunity. Any opportunity and hope is better than nothing.

Don B. | Colorado

Age: 66

Year of Diagnosis: 2017

Type: Bulbar Onset



People is a very broad group. Many do have a basic understanding. The medical community should respectfully understand that the rate of "progress" to a cure has been tortoise-like. **ALS was identified 153 years ago. Lou Gehrig was diagnosed 83 years ago. Where are real tangible results for patients?**



Most importantly, the current processes should not continue indefinitely. **Years matter.** The risks of expediting trial treatments is acceptable when you don't have the luxury of time to wait. We put a man on the moon 53 years ago because it became a priority for the scientific community. **Curing ALS could be an easier accomplishment with a high enough priority. I realize that other diseases generate higher profits in the world of medicine.**

ALS
changes people. It sculpts us into someone who understands more deeply, hurts more often, appreciates more quickly, cries more easily, hopes more desperately, loves more openly, and lives more passionately.

Bobbi A. | Ohio

Age: 67

Year of Diagnosis: 2020

Type: Bulbar Onset

We
Can't
Wait

It would give me the chance to live longer to enjoy my family & friends. It would give me hope, which is better than what I have now.

I would still want to try the therapy. Everyone is different, I may not have any reactions. It's a chance I'm willing to take.

The clinical trial is important for 2 phases. I don't need FDA approval.

It's changed my whole life. I can't talk or walk. I depend on my husband & children for getting through my day. I no longer can do what I enjoy. I miss going out to eat, gardening, & talking.

Sadness all around. Lots of tears & prayers for some kind of cure.

That it's so hard to lose your independence & having to rely on others for daily tasks. Your body experiences different symptoms like muscle spasms, mouth chattering, legs shaking & your talking is hard to understand. You can't drive anymore, you can't go to places that have steps or if the bathroom isn't on the first floor.

Please give me the chance to try different therapies. Let every ALS patient try, forget about qualifications. It shouldn't matter how far along a ALS patient is, open up access to all patients.

Marsha M. | California

Age: 66

Year of Diagnosis: 2020

Type: Limb Onset



Access to new therapies is critical for individuals with ALS. Time is not on our side. Imagine if you had the opportunity to extend your life by additional months or improve your quality of life for the remaining months with your family, friends and community. I believe everyone with ALS, a terminal disease, should have access to the benefits of these therapies. **The potential rewards outweigh the potential risks.**

I would discuss the options with my doctor, research the therapy, review results of clinical trials, and weigh each potential therapy for possible rewards and potential risks. Regarding side effects - **each person responds differently and can stop the therapy if the reward/risk equation is not positive.**

I believe that the FDA process is important but very lengthy. For ALS patients facing a terminal disease diagnosis, the timeline for completing all the clinical trial phases is too long. If a therapy has met the safety requirements, has proven efficacy after Phase 1 & 2 trials, I believe ALS patients and their doctors should be able to have access to the therapies.

My life has been radically impacted. I am fortunate to have incredible family, friends and colleagues to support me, as well as access to an excellent ALS clinic. Over the course of a few months, I have had to greatly reduce my personal and professional activities due to limited mobility and energy.

My ALS diagnosis has had significant impact on my husband in our day-to-day activities and our business together. He is now managing the lion share of our home, family and work responsibilities. My diagnosis has also been very difficult for our children, our extended family and close friends.

I wish people understood the complexity and variations of ALS. I was stunned to learn when I was diagnosed that **only 8% of cases are genetic and that 92% are from unknown causes** (mine is not genetic). The general assumption is that Lou Gehrig's Disease is a genetic disease. The research on ALS could be a window into the diverse factors in our environment that impact our bodies in profound ways. **This research could provide greater understanding and possible solutions to many related neurological and neuromuscular diseases affecting the greater population.**

I fervently request the FDA to allow for expanded access and compassionate use of available drug therapies for ALS patients. We are battling this disease on limited time and request the opportunity to extend our time and quality of life with our loved ones.

Peter S. | California

Age: 36

Year of Diagnosis: 2018

Type: Genetic

We
Can't
Wait

I feel the choice should be made available to anyone that has this mutation, and it is up to the patient advocate to make the patient fully aware of their decision before the treatment.

That sounds good, I have opted to try a new physical therapy routine I found online.

It is extremely important, as I received care and treatment prior to finding out my true diagnosis. I had a surgery on my cervical spine when I was complaining of lumbar pain that progressed to more intense pain.

I have developed new treatments, that require minimal cost. But the relief of the treatments are temporary.

I don't know to be honest what to do anymore.

The ALS disease is a lifelong disease that progresses to involve your life, those close to you and it is a difficult task to deal with.

I would like to thank you and your colleagues for giving me this opportunity to make my opinion known. Please have a wonderful day.

Ress C. | Tennessee

Age: 61

Year of Diagnosis: 2010

Type: Limb Onset

We
Can't
Wait

Honestly, no one wants to die any earlier than they have to. Yes, I am for it.

With ALS you have nothing to lose.

Not important to me at all to have it preapproved. With ALS you have nothing to lose.

It was a death sentence. No hope!

Totally crushed our family.

**Time is limited. Your trapped in your own body.
To someone that has ALS please let them try anything
you have. They have nothing to lose.**

Manuel L. | Iowa

Age: 68

Year of Diagnosis: 2017

Type: Limb Onset; Sporadic



**Click
Here to
Play
Video**

Will try anything. Been on Radicava for 3 1/2 years, and it was supposed to give me a couple months until something else came along. Well, it's here, give it to me.

I'll take that chance

Or important I took Radicava before trials were done

Horribly, just retired and found out

My wife had to quit her job and become my caretaker

It is a death sentence.

Give us a chance.

Debra O. | Murfreesboro

Age: 69

Year of Diagnosis: 2019

Type: Bulbar Onset, Sporadic

We
Can't
Wait

I think extremely important to have a chance for medication, because another more effective therapy might follow.

I am willing to take the chance because the only thing certain presently is steady decline.

I think if preliminary trial shows promise, would take without FDA approval. **Every day ALS takes a part of me away.** It is physically and emotionally exhausting because they do so much for me physically, and desperately want to find a solution for me.

ALS is presented as a death sentence; and it is very hard to fight something when hope is being dashed daily.

I know ALS is a small community, but please realize we deserve the same zeal to find a cure, or at least a slowing of progression, as any other more notable disease.

William M. | Minnesota

Age: 64

Year of Diagnosis: 2020

Type: Bulbar Onset

We
Can't
Wait

Important but willing to consider non approved trial.
It is also important to have the chance to try medicine
that could help.

Can no longer do anything I would like. Very limiting.

My wife has now become my caregiver

Don't pity me just treat me like I was before. I'm still
the same person inside.

**I have nothing to lose so would welcome trials to
possibly help this disease.**

Christie G. | Alabama

Age:

Year of Diagnosis: 2021

Type: Bulbar Onset



ALS progresses rapidly, my dad was diagnosed and passed away all within the same week. If we could have slowed down this disease even by weeks, I think we could have had proper research and treatment set up without my dad spending his last days in the hospital. **If someone said " hey we think this therapy or new medicine might ease your symptoms but here are the potential side effects..." we would have tried it, what would be the negative side of that? The benefits would far outweigh the decision to not to.**

We just want to make sure it's safe before anyone with ALS starts a trial stage there's a difference between wanting a experimental treatment not approved yet and one that's been tested. It's a peace of mind moving forward.

My dad lived with symptoms for about a year and was never properly diagnosed. The tremors, weight loss and not being able to eat he never knew why. Little by little day to day it was always something debilitating, he loved planting trees, working out in the garden but that was all stripped away rather quickly with his muscle loss. He stopped going out, missed family dinners, holidays and most importantly doing things that he was passionate about. My dad would say...but we had one last Christmas together and I was able to spend that day with my grandchildren before the final stages. This disease is like no other, to be trapped in a body with no way to live your life not even fully but just with good quality and good health day to day. It's a scary thing to go through.

Seeing someone you love suffer is heartbreaking, I felt helpless I couldn't do anything to ease my dads' symptoms. I can say it's mentally and emotionally draining. For my mom especially.

How rapid this disease progresses and how hard it is to get a proper diagnosis in some places. My dad shouldn't have suffered as long as he did with no answers from multiple doctors, hospitals and tests. Awareness is detrimental.

We need treatments, we need a way to help people living with a debilitating disease live their best most comfortable life. If you could imagine being trapped in your body but you mind was aware of mostly everything going on the pain, the tremors, not being to eat, wasting away physically, no one should have to suffer through that. Treatment or therapy to ease symptoms to help those living with this disease that should be the steps moving forward.

Ann C. | Louisiana

Age: 42

Year of Diagnosis: 2015

Type: Limb Onset, Genetic

We
Can't
Wait

I lost my mother, grandfather, cousin and uncle to familial ALS. Because of the poor prognosis and lack of options for treating/curing ALS, I think it is extremely important to at least give ALS patients the OPTION to try experimental drugs that can potentially help. After all, what is there to lose when you have basically been given a death sentence with an average of 3-5yrs left of your life?

Again, due to the very poor prognosis of ALS, I think it would be up to the patient to decide if that is a risk he/she is willing to take.

Normally, very important. In the case of ALS, there is not enough time for that to happen and that's a risk I would be willing to take.

When my Mom was diagnosed with ALS, it was the most devastating moment of my life. To hear that it was familial was then the 2nd most devastating moment in my life. My siblings, uncles, cousins and I all live in constant fear that we may have the gene and one day start developing symptoms. We worry about the gene getting passed on to our children and possibly not being around to take care of them. It is one of the most awful diseases on Earth, and it is very disappointing that since my grandfather died in 1997, there are still no significant treatment options available. Something needs to change!!

As I stated in the previous question, everyone in my family has been greatly affected emotionally, mentally, and financially by those we have lost to ALS. We are constantly worried about who will be diagnosed or develop symptoms next. **We each have a 50% chance of having the gene and getting ALS in our lifetime.** Those aren't good odds, and our family could be wiped out. We worry about the financial impacts and the impact it will have on those who become our caregivers.

Although it is a "rare" disease and most cases are sporadic, when it is Familial, it is all-consuming for that family emotionally, physically, mentally and financially. I don't think people realize the cost of caring for an ALS patient for even just one year. It takes a very specialized team of caregivers and doctors, equipment, medical devices, handicapped vehicles and even home renovations to make sure an ALS patient is taken care of. Even then, many doctors don't have the knowledge to know how to help these patients-medications don't do much to keep them comfortable. Research is critically important and time-sensitive for these patients. Of course we would love a cure, but at this point, even a moderately successful treatment would be wonderful.

There is no time to waste trying to get these drugs for ALS patients approved by the normal standards-there needs to be a "fast track" if possible, with the understanding that there may be unforeseen side effects and/or no improvement.

Meg D. | Pennsylvania (1 of 2 pages)

Age: 40

Year of Diagnosis: 2020

Type: Limb Onset

**We
Can't
Wait**

Having access to ANY new therapy that offers ANY benefit or chance of improvement for this disease is of utmost importance to my family and me. ALS is one of the only diseases where there is no chance of a cure....it is a death sentence. Chemo may not help or cure a cancer patient, but there is a chance it will. People with ALS don't have that hope. There is not enough time to wait for FDA approval on these new therapies. Let people suffering with this vicious disease make the determination if the risk of a new therapy is worth it to them. It is their only hope! It is barbaric to live with this disease knowing there is nothing you can do but wait until you no longer have use of any part of your body! We need this law to pass now!

As I mentioned above, having access to ANY new therapy that offers ANY benefit or chance of improvement for this disease is of utmost importance to my family and me. ALS is one of the only diseases where there is no chance of a cure....it is a death sentence.. Let people suffering with this vicious disease make the determination if the risk of a new therapy is worth it to them. We would rather try a new therapy and not have success than to sit idle and watch loved ones waste away from this disease with nothing to help them. It is a no lose situation. Let us decide what we can handle.

For diseases like ALS where there is no FDA approved drug/therapy or any drug which helps mitigate the disease, it is not important. The effects of ALS are barbaric....The FDA should absolutely due it's due diligence for any drug or therapy brought to its organization, but for ALS, it should not be the ONLY way to be able to try a therapy when it's a mere life or death situation. So in other words, continue to utilize the FDA as new therapies emerge, but let the ALS patient try therapies as they emerge at their own risk.

It has virtually stopped our life. My dad is no longer able to move or feel his legs, so all he can do is stay home. Our cars can't support his scooter/wheel chair, he can no longer take showers or do ADL's on his own. For now he still has his upper body strength, but that is weakening as well. My parents will need to move or build on to accommodate his lack of ability to move, but struggle with the financial aspects of the disease. My dad was diagnosed the year he retired, so my parents never got to enjoy all the things they had planned. They cannot travel, see their grandchildren play sports, go out to dinner, go to weddings/family functions. Everyone has to come to him. He is morally defeated to know we have to help him with literally everything from going the bathroom, to cutting his hair. The absolute worst part of this is that there is no hope for him. Not one med approved to help him so it's a anxiety ridden waiting game....like what's going to happen next to my body. There is no upside....it's all downhill. We need to be able to try therapies...if it even helps one person, it will be worth it!

Meg D. | Pennsylvania (2 of 2 pages)

Age: 40

Year of Diagnosis: 2020

Type: Limb Onset

We
Can't
Wait

I have 4 of my own children but my mom cannot care for my dad on her own. They rely on my siblings and I for everything. She is getting too weak to help him...I'm scared at what the future holds. Even the simplest tasks such as outside chores are too much physically for her to do on her own as well as take care of my ailing dad. We visit and help as much as we can, but we miss their visits to our house, vacations, they attendance at kid's games, etc. My children are heartbroken to see their grandfather who was so physically fit and healthy slowly deteriorate.

The despair in knowing there is no hope at the moment. That feeling keeps me awake every single night of my life. My dad needs a chance at life. We can't wait years for therapies to be tested and approved. We need help now!

I did the Ice Bucket Challenge years ago but didn't think too much about it because no one I have known ever had ALS. I never dreamed just a few years later this would be my life. This is not a normal disease. We need hope and we need help! We cannot treat it like a normal disease and we have to get creative in an effort to save lives and bring hope to a ravaging and barbaric disease. Please put yourself in our shoes!

Nancy P. | Rhode Island

Age: 64

Year of Diagnosis: 2018

Type: Limb Onset, Sporadic



**We
Can't
Wait**

Extremely important to have a chance.. Presently I am taking the 2 drugs that are available for treatment for ALS. I am also taking Zilucoplan as an open-label drug because I was in the Healy Platform Trial. I have nothing to lose, but so much to gain if my life could be extended by even a few months. It is my hope that these modest gains will buy time. **The additional time May lead to breakthroughs in the treatment for ALS.** I have 2 adult children and a loving husband that I am not and will not be ready to say goodbye to in the near future. Like every parent, I would love to witness the joy of my children getting married and having children of their own. At this stage in my disease, **I am willing to take any risks that may have the slightest chance for slowing the progress of this monstrous disease.** I don't feel that I should be denied access to AMX0035, especially since after phase 2 it is safe, tolerable and hit its targets. I don't understand how the FDA can deny patients with ALS access to this drug. The FDA is not only denying ALS patients and caregivers access to potential treatments, but they are also stripping us of Hope. Please reconsider your decision that a phase 3 trial is necessary for AMX0035. I probably won't be alive by the time the phase 3 trial ends. How can the FDA do this to me and so many others?

I would choose a new therapy regardless of the side effects or immeasurable benefits. I am looking a horrible death straight in the eye. I will try any new drugs.

It has completely changed my life from one of great independence to complete reliance on my caregivers. I was actively engaged in life and its bounties, now I am an observer in a wheelchair, unable to move.

I was diagnosed 1 year after my retirement. My husband had to retire early to care for me. **We were never able to realize our retirement dreams, my husband' life has changed dramatically.** We have had to move to a more accessible home, leaving a home we loved and many friends behind. My adult son and daughter have lost some of their wanderlust and joie d've. Of course, they are very concerned about me and their father. They have put lifelong plans on hold so they can be available to help with the caregiving.

I wish the general public knew how debilitating this disease is. I also wish they understood how swiftly it impacts the lives of those living with ALS and their caregivers. I wish those unaware of the financial burdens could understand these hardships and support legislation that would provide government funding to help the patients and caregivers.

I have never begged for anything in my life before today. I am begging you, on behalf of all of us living with ALS and their caregivers, to give us access to AMX0035 before it is too late.

Christine W. | South Dakota

Age: 63

Year of Diagnosis: My husband, Burt diagnosed in 2010

Type: Bulbar Onset

We
Can't
Wait

As we were traveling to Burt's Dr. to hear the confirmation of his diagnosis after several tests, he told me he hoped that he would be diagnosed with cancer instead of ALS. At least I will have options for treatment if I have cancer. That was not to be. **If he had had an opportunity to use a new therapy, he would have instantly wanted to try it.**

Burt would have tried it. ALS is a death sentence. He felt he would nothing to lose if a new treatment would have been available.

No importance at all to have a drug approved first.

How did ALS impact his life? Burt was diagnosed with ALS in June of 2010. He was a farmer and the wheat harvest that summer was the last work he was able to do. By Christmas he needed a walker, his ability to eat and talk were increasingly challenging. By April he was wheelchair bound and I needed to feed him. We had to hire help to help him get him our of bed in the morning and dressed and then again in the evening to get him back in bed. Once in bed he did not have the ability to change position. **He was quite claustrophobic, so this was torture for him.** We bought a special bed that was good for protecting against bed sores. We tried a hospital bed, but his voice was so weak that I could not hear if he needed me during the night unless I could lay next to him. **The ALS association provided us with a Hoyer lift so I could get him in and out of his wheelchair. In December of 2011 he passed away almost 18 months to the day of diagnosis.**

Significantly. It was so hard for our family to watch a very independent, hard working, capable man change so quickly to being dependent on everyone for all his needs. **I was asked why I didn't put him in a nursing home. I replied that he had no strength to use a call light when he needed something, and his speech was so affected that only those of us who were with him all the time could understand him.** I would have to live at the nursing home too if he were to go there. Plus, I felt he needed to be home where he had lived all his life. To have that comfort of a familiar place in which to spend his final days. It was the hardest thing I have ever done, and for his children to do.... to watch someone, you love slowly lose the ability to breathe will never leave us.

It is one of the cruellest diseases that exist. It completely destroys the body's ability to do anything, but the intelligence remains intact so essentially you are trapped in a body that will not work. Burt told me he felt like he was in prison.

I appreciate their efforts towards new meds, but I urge them to give those stricken with ALS the chance to decide if they are willing to take the risk of trying a new med that hasn't gone through all the hoops yet to get approved. As I said before, ALS is a death sentence so let them decide what level of risk they are willing to take.

Michael L. | Connecticut

Age: 66

Year of Diagnosis: 2018

Type: Limb Onset



Any therapy that can produce positive effects to slow disease progression is welcome

I just completed a drug trial which I was on for 18 months and open label for 8 months and the drug manufacturer did not meet their end goals and has now cancelled the open label so yes, I would take the risk again.

I am a firm believer that all drugs should go through all phases of the clinical trial's before being fast tracked by the FDA. The trial process is in place for a reason.

To date, had to sell our house, change positions at work, lost the primary use of my legs, had to buy a handicap van, had to stop running and much more.

My wife is my primary caregiver so her daily life has been impacted although I am still self sufficient there are things I cannot do on my own. My relationship with my children and especially my grandchildren has changed although they do like riding on my power chair!

ALS affects everyone differently and that it is a very complex disease with no true path. My progression has thankfully been slow, but I know others who have not been so lucky

Nothing specific as I think the drug companies are doing more today than anytime in the past and hopefully there will be some breakthrough in the near future.

Dean C. | Michigan

Age: 61

Year of Diagnosis: 2005

Type: Sporadic



We
Can't
Wait

Let me have a choice. Let me decide what is best.

Very important to have a chance.

It has made it clear to me what is important.

They are all gone, they could not handle it. I came all along.

That it is a death sentenced. it may take a long time or be fast, but it process.

Prasanna A. | Maryland

Age: 30

Year of Diagnosis: 2018

Type: Sporadic



**Click
Here to
Play
Video**

We need access to NurOwn and MX0035 now. Even if it shows 1% improvement, we need it. We do not have time to wait. **I am waiting dying.**

I will happily try any potential treatments even if they are detrimental to my health. There are NurOwn and AMx0035 I want to try.

For me, it is not important. I want to get access to potential treatments that are currently stuck in FDA trials. **I do not have time to wait for a decade.**

I am not able to bath, eat, drive, speak and breathe!

My caregivers are tired of FDA red tape.

I wish FDA understood the importance of giving access to Patients with ALS. There are AMx0035 and NUROWN

ALS patients need access to experimental treatments right now!
We do not have time. Please provide access to NUROWN and AMx0035 now.

Gary G. | Pennsylvania

Age: 67

Year of Diagnosis: 2010

Type: Limb Onset, Genetic

We
Can't
Wait

Every discovery is a step in the right direction. Of course, we want to "cure" found yesterday, but each small step builds to finding the cure.

Would not be as eager to promote a therapy with significant side effects, but we have to take the risk to find the cure.

The average life span for an ALS patient is far shorter than the time to take the FDA to approve a new drug. Therefore, we cannot wait for final approval before taking a new drug. We understand the risk, but we also understand we are dying within 2 to 5 years.

My wife lived 3 1/2 years after being diagnosed. About 1 year into her journey, she had to quit working and driving. About 2 1/2 years into her journey , she became bedridden, lost her ability to speak and couldn't eat any longer. Luckily, we were in a position to keep her at home until the last week, so she continued to be around friends and family. That helped immensely.

It was very difficult on us. After she became unable to walk, she was no longer able to take care of herself. At that point, I quit working to take care of her full time. She was most uncomfortable sitting up so being in bed was most comfortable. That meant we spent all our time staying home. Socializing with our friends was difficult.

While it is a debilitating disease, and greatly affects her speech and voice, she was still the same smart person. She sounded like she may have been mentally challenged, but she was not. People also kept asking about her, thinking she had a chance for improvement.

We need to find a way to diagnose ALS much sooner. **Today, by the time a patient is diagnosed, they have already suffered irreversible damage and life with those challenges in no life.** We need to get ahead of the curve, not just find meds that will extend a patients compromised life.

Earl C. | Roscommon

Age: 63

Year of Diagnosis: 2018

Type: Bulbar Onset, Limb Onset



I am sorry but I cannot ask for more month of agony and suffering. **There come a point when your prayers change; and you ask for mercy instead of prolonging.**

I would not have asked my wife to risk more side effects and suffer for really no real benefit. They had a drug they said might prolong her life 4 months.. We asked when do you start counting ?? Are we adding to the "misery" or to the better day up front ?? (Still not good). Also, the range of life varies so much saying four months was a laugh ! No one recommended it, not even her doctors.

Depends fully on what the expectation are; You don't prolong a walk-through hell ! If you expect something that is real, then of course take the risk. You have nothing to lose!

My wife died ! So it was a great impact.

The death of my wife ! The death of a lady that brought joy to many, her kids included, was quite devastating.. But we expected no miracles from the beginning and in the treatment of 2016 none were forthcoming.

Very few caregivers have any experience. I nearly blasted a few people for their ignorance and lack of empathy!! Your walk will be very alone... Like Deanna Gage (Deanna's protocol) her father was a surgeon and tried to help with his knowledge of the medical world, because there simply was no alternative.

I think the illness is over whelming! and the benefits are not for the millions but for a much smaller number of people. So time and money is absorbed by bigger issues.. It might work, if a large compensation was offered to anyone or group that offered real medical breakthroughs in this horrible disease.

Jan S. | Oregon

Age: 61

Year of Diagnosis: 2014

Type: Sporadic



**We
Can't
Wait**

Very important to have access to medicine.

My pALS and I would research these risks and rewards and decide how they would affect my pALS. I am a spouse caregiver.

Not super important to worry about the risks.

He is a quadriplegic, in a wheelchair, 100% dependent on a ventilator. Can still talk. Uses computer with his eyes.

My entire life is consumed with keeping my husband alive.

How much reform is needed in care models. These patients and families need better support that would also help ALL families with devastating diagnoses.

He was respiratory onset and excluded for trials because of low FVC and using BIPAP. Please remove FVC from trial criteria since those don't always correlate to longevity. Diagnosed 2014.

Evgeny E. | Russia

Age: 42

Year of Diagnosis: 2019

Type: Bulbar Onset, Limb Onset, Sporadic



It is necessary that people have access to medicine.

I will try anything; it doesn't matter what the consequences are.

Everything around me has faded and my family is very tired.

No one is protected from this.

I would like to receive any effective medicine I can.

Henry C. | New York

Age: 73

Year of Diagnosis: 2003

Type: Bulbar Onset, Sporadic



We
Can't
Wait

Every improvement is a blessing, and I would prefer no advanced side effects.

I would like to try before full FDA approval

ALS has devastating changes in Lifestyle and perspectives. It is simply daunting. It is torture.

Give us an option to try new medicines.

Craig K. | Kentucky (1 of 2 pages)

Age: 56

Year of Diagnosis: 2019

Type: Bulbar Onset



I believe it is extremely important to explore every avenue that may help slow the progression of ALS. Adding a few months to a person's life expectancy may not seem like a significant amount of time from the perspective of a healthy person who potentially has a long life ahead of them. However, from the eyes of a person with ALS whose life expectancy is only a few years, those few added months are equivalent to adding years to another person's life. In more personal terms it means more time with loved ones and participating in life's joys such as being there when a son graduates high school, or a daughter gets married or the birth of the first grandchild. The added months shouldn't be looked at in a dry statistical manner. It needs to be viewed from the eyes of the person with ALS and their family. **A few more months are a few more joyful memories.**

In my opinion those who have been diagnosed with ALS know what their outcome will be so if a therapy has no benefit all they've lost is the time they've put in. **The bigger question is the potential significant side effects. Everyone would have to weigh the choice of modest benefit with side effects versus not trying the therapy and knowing they wouldn't reap any benefit.** It is a hard choice however I would hope enough ALS sufferers would participate in new therapy research even if there are side effects so that medical advances can be made that could help others with ALS down the road.

Certainly, it is extremely important that any new drug or therapy go through a stringent clinical trial process and equally stringent FDA approval. However, the issue with ALS is somewhat unique. Because ALS is rare there are very few people worldwide who can participate in the clinical trial process. In addition, the overall clinical trial process, including the time spent performing non-human clinical trials before it can be tested on humans, can take years. People with ALS don't live long. 80 percent die within 5 years of diagnosis. Consequently, those with ALS who are accepted in a clinical trial could potentially pass away before the clinical trial is completed. I believe that if a promising drug or therapy has shown to be safe and effective in clinical trials then the **FDA should take a hard look at allowing people with ALS to take that drug even though it is not fully approved yet.** Consider the recent example of emergency FDA approval for the COVID vaccines. These vaccines have helped hundreds of millions of Americans. If the FDA can issue emergency approval for vaccines, it should consider that same strategy for other beneficial drugs and therapies that are critical to a person's health and longevity.

Craig K. | Kentucky (2 of 2 pages)

Age: 56

Year of Diagnosis: 2019

Type: Bulbar Onset



From the time I was diagnosed with ALS in the fall of 2019 until now my life and the life of my family has been completely turned upside down. At the time of diagnosis, I was working full time with a wife and three school age children to support. Because of my deteriorating health we had to make some immediate hard decisions. First, I had to stabilize my rapid decline. This required the help of a number of wonderful specialists. At the same time, we had to plan an early retirement because it quickly got to the point where I couldn't physically work anymore. That was difficult considering I was not planning to retire for another 15 years after putting my kids through college. **My wife and I spent many nights quickly making one major life choice after another. We included the kids in on many of these conversations because their lives were being drastically impacted as well. Ultimately it meant we had to sell our home and move to another home that fit our new budget in another state.** It was extremely difficult to leave behind dozens of family and friends. As you can imagine this caused a severe emotional strain for our family and our marriage. We are still adjusting to our 'new normal' but hopefully we've put the worst of this traumatic situation behind us.

First there was the shock we all go through when a traumatic event affects our lives. Then there was the anger. Each family member and friend handled it in their own way. Ultimately everyone has been very supportive and rallied around us. However, the first year from the fall of 2019 to the fall of 2020 was the most difficult. My chief concern was how my kids would adjust. It was very hard for them over that first year. Emotions ran high and were all over the board. All we as parents could do was be supportive. We also sought professional and spiritual counselling not only for ourselves individually but as a couple and as a family. Without a doubt the professional and spiritual guidance that we received helped our family get through the worst of this traumatic experience.

There is a significant amount of collateral damage caused by ALS. It not only affects a person's physical health but also their emotional health and ultimately the emotional health and stability of their family. By attacking one member of a family, ALS has the power to drastically affect the lives of the whole family.

I believe ALS research can be thought of as the tip of the spear. ALS is one of many motor neuron diseases. I believe that research and development of drugs and therapies that benefit ALS sufferers could also be the key in helping others who suffer from other similar diseases so that all of us can lead long and productive lives.

Carl M. | Colorado

Age: 57

Year of Diagnosis: 2019

Type: Limb Onset, Sporadic

We
Can't
Wait

Extremely important to have a chance to try something that will help. Especially if it is very good. But it depends on the side effects. It is not important to me if it is approved yet.

I want:

Significant reduction in mobility and quality of life

Significant reduction in freedom

We need more medications and therapies

Rosa B. | Missouri

Age: 56

Year of Diagnosis: 2020

Type: Sporadic

We
Can't
Wait

There is nothing to offer these terminal ill patients. **Anything available is HOPE, each individual should have the right to choose if they want to participate in any clinical trials or therapies. It's there only hope, nothing else.**

Any option available is better than none.

There should be a loop in the restrictions for terminal ill patients. Why wait? These patients do not have the luxury of waiting.

It has affected absolutely every aspect of my life. My mobility, independence, lifestyle 100%.

My entire family has been devastated. **My daughter has had to quit her job, she has 3 little kids and is my caregiver 100%.** Its a horrible disease to everyone involved.

I wish people to understand how devastating it is. How fast a person deteriorates. How it rips the patient from everything they were.

Let the patient decide. They are terminally ill, it's their life, it should be their choice if they want to take alternative medication, therapies, clinical trials, anything.

Philip R. | Texas

Age:

Year of Diagnosis: Wife: 2009

Type: Limb Onset



**We
Can't
Wait**

Looking for more than just a few months of additional life

She was not interested in just a few more months of life when offered a treatment known to have significant side effects

If preliminary results are promising, then compassionate use is indicated prior to trial completion and FDA approval

Totally destroyed normal living habits. My family has suffered emotionally and physically. I am also suffering.

The burden on the family is huge !!

Chip C. | Pennsylvania

Age: 61

Year of Diagnosis: 2008

Type: Limb Onset, Genetic



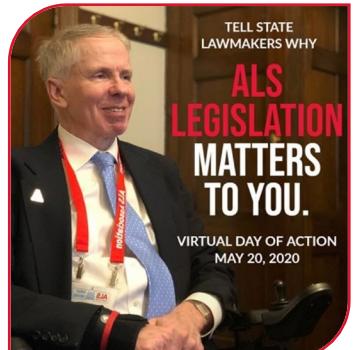
I think valuable more so to families who want as much time as possible

With great excitement

I am willing to take both if it helps people living with disease or finding cure for future generations

Well, now live in nursing home as my lower legs don't feel or work, and I have feeding tube and cpap

I am not really sure how this has affected my family, **with covid going on I have not seen any of them for almost 2 years**



The more the hear of it and know it's 100 % fatal is always an eye opener

Just that the most drugs and treatments gets us closer to our final goal of a cure



Lucyna D. | New York

Age: 58

Year of Diagnosis: 2020

Type: Bulbar Onset

**We
Can't
Wait**

A new therapy would give hope.

ALS itself is a death sentence. It is worth to try any treatment. It was not important to wait for FDA approval.

It was devastating news for the entire family. We can't take this much longer.

That anybody can get it and also that it can impact cognitive skills.

People with ALS diagnosis cannot wait long time.

Sylvania M. | Texas

Age: 64

Year of Diagnosis: Brother

Type: Bulbar Onset, Limb Onset



**We
Can't
Wait**

My brother lost his life after one year and a half of diagnosis. Any chance of an improvement would be priceless for a person losing the normal function as simple as being able to smile, to speak, to the most detrimental lost in the ability take a breath.

It is vital that they are provided with the complete information and given a chance to make that decision for themselves. We already know the outcome of ALS . In the horrifying process of physical function deterioration, one remains mentally aware of what is happening to them.

In many cases it is vital BUT let's take the Covid vaccine as an example. There would have been a greater loss of life if we had waited for the FDA to complete a 6-7 plus year process of approval. Folks with ALS don't have that luxury. Each day is so precious to that person and their family.

With respect, my brother has lost his life due to ALS

On a personal basis as his sister and as a Registered Nurse, I have never felt so helpless in watching my brother, who was physically fit and full of life, loved by his family and friends, quickly but slowly die of this disease. He had made such an impact in so many lives, including saving many lives. The pain that I and my family have words can not describe.

ALS has no discrepancy in who you are. It will ripe every fiber of your love and want of life. It will take your identity of who you are as a human being. The impact of the simple ability such as brushing your teeth will be a fantasy.

I pray to God that this does not happen to you or even worse your love one. Pharmaceutical companies, remember compassion for the patient and the families that struggle to provide the medication that will play a significant potential chance of improvement.

Kimberly B. | Pennsylvania

Age: 52

Year of Diagnosis: 2018

Type: Bulbar Onset



ALS is a death sentence. All the doctors can do is "manage" the disease and try to outsmart it....and sadly it's an insidious diseases so always has the upper hand.

My brother would have taken the new therapy as ALS is a death sentence.

My brother worked in the pharma industry a good chunk of his life and believed ALS needs to be treated like oncology or infectious disease...when products get signals in Phase 2, it should be open to all ALS patients

My brother passed away March 12, 2021 after a 3.5 year battle with ALS.
The last 12 months were awful for him...he was paralyzed, 100% reliant on his wife for care and in great pain as muscles dying cause great pain.

His one son suffers from major depression and has not been able to hold a full college class load of class the last 3 years, **his wife has PTSD from being is full time caregiver**, his 2 other sons also have PTSD from being back up caregivers. Additionally, his wife and 3 sons had to forgo their health for his health.

It's a death sentence, the current rx therapies control symptoms and don't delay progression, and it's very painful disease. In addition to impacting the patient, his family is just as impacted watching the slow, slow decline.

Treat this disease like Oncology or an Infectious disease

Dan P. | Michigan

Age: 37

Year of Diagnosis: 2019

Type: Limb Onset, Genetic



We
Can't
Wait

Extremely beneficial to have a chance. Hopefully by me time till some thing more advanced comes along. I need to stay around to see my kids grow up.

I'll take it. At least for awhile. I would weigh the severity of the side effects first though. I would want to make my life more miserable

**Not important at all to get preapproved. I have no time to waste.
This is not acne or ingrown toenail. What have I got to lose?
Drugs in my body!!**

How hasn't it impacted my life???? You name it.... wife, kids, job, home ownership, friends, activities and it's different or nonexistent.

They hurt when I hurt. And I'm always sad. They have to go so much more. Pick up the slack and more. This is a family diagnosis.

Just how awful it is. How you're never given any hope. Just go home and get your affairs in order. A devastating disease like this needs fresh , out of the box approaches. Waiting 10 years for all the t's crossed won't cut it.

Put people before profits. Do for us what you'd do for your own family. One patient touches tenfold friends and family.

Christine S. | Pennsylvania

Age: 70

Year of Diagnosis: 2021

Type: Limb Onset

We
Can't
Wait

Any drug that will slow down the disease is a blessing.

New drugs give us people suffering from ALS some hope. By the time the FDA approves of a new drug for ALS most of us will be dead.

It has totally impaired my life. I can no longer work or do the normal workload I usually did. My speech is deteriorating, and I am having difficulty breathing. Walking up the stairs has become a chore. Swallowing has also become an issue. I feel somewhat isolated because I can only talk for a few minutes to my friends.

They feel sorry that at the moment there is no cure for ALS My family wants to help me to be comfortable and content.

We need to educate the public about the disease. Most people never heard of ALS. When I told people about my diagnosis, they had to look up the disease. We also need more support from the public. More funds are needed for drug trials.

We need the drugs NOW. If a drug has any type of promise they should let us try it.

Becky M. | Massachusetts

Age: 57

Year of Diagnosis: 2020

Type: Limb Onset, Sporadic



I view new medication as something when now we essentially have nothing. I view it as a life preserver. At the very least it will buy me time while I await more effective drugs to make it through the pipeline. **I view it as Hope where there is little.**

I view a new therapy that offers the chance of modest benefit as not a risk at all. I am willing to take my chances regarding side effects.

I much prefer a drug be approved in a timely matter. It is important that it becomes standard of care so that people with ALS can continue to offer themselves to science. That being said access to any drug that has potential is better than no drug at all.

I am a musician who can no longer play her instrument in a runner who can no longer run. I am forced to process the loss every single day for the rest of my life. This started in my arms and hands, and when I lose them, I will lose my independence. And why has it not impacted my life. I consider this a silly question.

My children will be forced to witness their mother lose a piece of herself day by day and die a cruel and inhumane death. My husband becomes my caretaker. Everyone who loves me is caught up in the week of this devastating disease.

I wish they understood that if it hit me, it can hit them too. The people not directly impacted by ALS need to be our champions. To raise awareness and funds for continued research.

I would like the FDA to know that this listening session is not enough. That it is time for action. Time for a change. Time to streamline the path to approval. That it is absurd that AMX 0035 is not going forward to approval. Absurd. Is your gold standard to deny us drugs? What could be worse than dying the death I will die from this disease.

Deborah H. | Florida

Age: 70

Year of Diagnosis: Father

Type: Limb Onset



We
Can't
Wait

I believe this is of the utmost importance. My Father was dx in 1993 and died in 1995. I prayed we could have more time with him. I believe for him it was like having a ringside seat at his own demise.

I would have wanted to take the chance of making a modest benefit for my Dad but would not have wanted him to have significant side effects in addition to being trapped in his own body and choking to death.

I believe that the FDA should follow through with their clinical trial process. As we have seen with Covid vaccine side effects, it is extremely important that the FDA complete these trials.

I have not been diagnosed, but again, my father was diagnosed. Watching him slowly lose the ability to walk; breathe; feed himself; care for himself in any way was grueling and very sad. In the end, I prayed for God to take him due to the insurmountable suffering. I'll never forget seeing him on a ventilator, staring at the ceiling and a tear falling from his eye. He died the next day.

My entire family was devastated that my Dad died at age 68. He was a kind, loving, cheerful man with a heart of gold.

I wish people understood how important it is to find a cure for this horrendous disease. People are dying way to young!

Yes...I believe the pharmaceutical companies have billions of dollars and should take some of those billions to pour it into research. We must learn to give back. The greed in our Country is going to kill us.

Stephen M. | Minnesota

Age: 72

Year of Diagnosis: 2020

Type: Limb Onset

We
Can't
Wait

It would have a large affect on illness cures for the future.
Future research would better help AND WE HAVE NOT HAVE
ACCESS to this.

We're dealing w/ a GRAVE disease here. Baby steps to a cure.

Not much. FDA supported Agent Orange.....

Greatly affected myeline in terms of the physical changes.
Mentally I'm fine. I'm a recently retired Anesthesiologist.

Are you kidding??? A **GREAT IMPACT ON EVERYONE**.
There hasn't been much medical treatment or improved
therapies or a cure.

**We need expanded access through clinical trials,
compassionate release, less stringent inclusion criteria for ALS
patients.**

Janet M. | Arnold

Age: 60

Year of Diagnosis: 2014

Type: Limb Onset



It is very important I have a chance to try any medication to help me.

All I know, feel, and thinks about is death. Physical, mental and financial strain...

There is no cure

Don't be concerned with addiction until you find a cure

Mike W. | Iowa

Age: 67

Year of Diagnosis:

Type:

We
Can't
Wait

If I were given any hope in slowing down the progression of this disease would truly be a blessing. It would depend on the condition the patient at the time the therapy was offered.

It's hard to answer that question not being in that situation. I would probably opt out unless there was a good chance, I would see some improvement.

Every drug on the market has its side effects. If I was given a chance on a drug that offered a good chance of reversing the effects ALS had given me, I would take the drug.

I witnessed my cousin live nearly two years with ALS. More research needs to be done to rid this terrible disease.

Tonya F. | Texas

Age: 49

Year of Diagnosis: 2014

Type: Limb Onset

We
Can't
Wait

We will take anything. We need HOPE and more time. It is a chance I am willing to take. Give me options.

Took away my ability to work, my family had to relocate from a beautiful place we love, slowed everything down.

They have tremendous support for me as always

The clock is ticking-until this affects your family you have no idea.

The possibility of improving outweighs any risk.

Paul T. | Texas

Age: 66

Year of Diagnosis: 2009

Type: Limb Onset, Sporadic



Rilutek is a drug that fits that description and I take that now. **I am in favor of any drug that will help.** With so many variations of ALS a drug slowing progression may be more beneficial to some than the modest benefit expected

A drug that could work is better than no option. Side effect are more important when weighing whether or not to take a drug. It also depends on where you are in your progression and the rate of your progression

I think the FDA has a role to play but when a drug shows promise there needs to be a fast-track process. With a 2-to-5-year average life expectancy most PALS can't wait for the FDA to run a normal trial. Allow people the opportunity to try. You are allowing people to be in the trial so why not allow people, with the same waivers and sign offs to use the drug.

Allowed me to appreciate each day more and to do things now and not put them off. As my disease progresses, I am becoming more limited in what I can do and what I want to do.

It was rough early on because with a 2-to-5-year life expectancy we didn't know how we would deal with the coming years. As it turns out I am slow in my progression, so we have had time to adjust to my limitations

That there is little to no treatments to really manage the disease and more funding is needed to help improve and extend the quality of life for PALS. People need to know how devastating the disease is on the individual and their family

ALS is not incurable it is under funded. Time is not the friend of PALS so all efforts need to be with a sense of urgency. The way that we attacked COVID-19 was proof that with a concerted effort we can solve the most difficult problems. We have lots of disjointed efforts to find treatments for ALS if we could harness the power of all of them, we could make a difference.

David F. | Florida

Age: 73

Year of Diagnosis: 2019

Type: Limb Onset

**We
Can't
Wait**

Slowing down the progression is a yes! Anything to provide us hope and relief!

Side effects, depends on whether I am willing to risk it.
I would like to know more.

Takes too long to wait for FDA approval.

I am devastated, changed all my plans. Quit job, overwhelming

Everyday is worse with never a hope of improvement family doctor Kevorkian make sense Suddenly.

Suddenly I understand dr. Kevorkian

Heather G.

Age:

Year of Diagnosis:

Type:

We
Can't
Wait

I think this is important, especially for younger people diagnosed with ALS. **What would be wonderful is adding years of additional life or significantly slowing the progression of the disease rather than just modest benefits.** However, we must walk before we can run, and if modest benefits lead to breakthroughs that provide significant benefits, then let's do what we can. **Too many people's lives are being cut short and too many families are being robbed of time with loved ones because of the cruel, quick-moving disease.**

For most people diagnosed with ALS, it is unexpected and completely alters the course of their lives and the lives of their loved ones. Without advances in treatment that give people more time, that means more lives cut short. That means weddings and graduations not attended. The milestones of children and grandchildren missed. It means never hearing that person's voice again or giving them a hug. All this being said - when you are given a death sentence, which, to be clear, ALS is a death sentence, you will do whatever you can to have more moments with loved ones. Give people a choice and let them weigh the risks/benefits with their medical team and their loved ones about what makes sense for them.

People with ALS do NOT have the time to wait for the normal clinical trial process to proceed. Let them make the decision, along with their doctor and families, about what makes sense for them. Clinical trials, rightly so, have rigid parameters. But, because of the relentless progression of this disease many people are often ineligible for studies because they are too far along in their disease and/or access to trial sites is challenging. All trials that have passed Phase 1 for safety should be opened for compassionate use to ALS patients.

Both of my parents had ALS. No family history on either side, it was completely sporadic in both cases. They went from being healthy active people to dead in a matter of months.

How devastating the disease is on the people who have it and their families. And, how limited the treatment options are.

People with ALS do NOT have the time to wait for the normal clinical trial process to proceed. Let them make the decision, along with their doctor and families, about what makes sense for them. All trials that have passed Phase 1 for safety should have an option to be opened for compassionate use to ALS patients.

Karen R. | South Dakota

Age: 63

Year of Diagnosis: 2017

Type: Limb Onset



I am anxious to have a chance to try any therapy that shows even a modest benefit such as slowing of disease progression or additional survival time, even if it showed such benefit in just a subset of patients. Due to the heterogeneity of the disease, it should not be expected that every therapy will work for all patients. I need to slow or stop my progression NOW to give me more time as we await a definitive cure and give me time to make more memories with my family. What you might think of as a modest benefit, may be of critical importance to me in maintaining my quality of life. For instance, even a 1-point drop on the ALSFRS-R scale in the area of ambulation would mean that I am no longer able to walk at all. A 1-point drop on the scale in the area of dressing myself would mean that I can no longer independently dress myself and would need to rely on the assistance of someone. One more point lost on the handwriting portion will mean my handwriting has gone from slow and sloppy to impossible to perform. These are huge quality of life issues for me. **I do think a cure for ALS will be found someday and I want the time to live to see that day.**

Right now, I know my disease is 100% fatal and I am on the trajectory of becoming more and more disabled. I would gladly accept the risks to try a new therapy - I would be no worse off than I am now and that chance to TRY SOMETHING would definitely give me hope. If the therapy has significant side effects, I would be able to weigh at that time whether I can continue the treatment or not. **I JUST WANT SOMETHING TO TRY** as I am dying waiting.

I feel the FDA and manufacturers should allow those of us who do not qualify for the clinical trial to have access to the drug before final approval. That **expanded access can continue to provide scientific information about the drug, and I do not have 2 years or more to wait for another trial to be completed.** By the time a drug has reached Phase 2 or Phase 3, the safety data should be known enough so that I can feel confident from the safety standpoint at least.

At the time in my life when I was looking forward to retirement and spending more time with grandchildren, volunteering more, and enjoying life, I find myself grieving loss after loss. Once a very independent person, now I have to rely on my spouse for the simplest of things. Although I try to remain optimistic and hopeful, it is hard not to be afraid of what is ahead of me. A future where I am totally paralyzed and a burden to others is very scary. There is also a great financial impact to the disease and care involved, and I worry that once I am gone, my husband will not have enough of our retirement savings left for himself.

I know that this is very hard on my daughter and family but especially hard on my husband. **Instead of being partners and teammates, I feel I am becoming more and more of a burden to him, and I know this is very difficult for him but also very hard for him to communicate to anyone else who understands.**

I wish everyone understood the urgency of finding treatments and ultimately a cure for ALS. Many people don't realize that it is 100% fatal and that not much has really changed since Lou Gehrig's diagnosis. If the resources that were put into the AIDS fight or the coronavirus fight, there would be real progress. **In the time that it takes to bring one drug from the lab to patients, the majority of ALS patients will have died.**

Please give me a chance to try anything that has been shown to help even 20% of patients in a trial. I want the chance to see if I might be in that subset of patients who is helped. **I don't have time to wait for one drug to hit a home run - I just want more time now as I am dying a little more every day.**

Don N. | Nebraska

Age: 67

Year of Diagnosis: 2010

Type: Genetic



**We
Can't
Wait**

Any new therapy would be helpful and welcome.

I gladly accept no benefit or side effects. Not at all important to get prior approval from the FDA, Let's just have a chance!

It's become very difficult for others to understand my speaking to the point where my wife must speak for and make phone calls me.

My family's been extremely supportive of me, taking me to appointments and helping whenever I need it.

I went from an active, athletic father and grandfather to being wheelchair bound, unable to stand or care for myself over a short period of time. It's taken a lot of patience and Doctors visits to get me where I am today and couldn't have done it without help.

Please, please, please find anything that can help us!

Chris P. | Michigan

Age: 37

Year of Diagnosis: 2017

Type: Limb Onset, Genetic

We
Can't
Wait

Not quite what I'm looking for, but I'll take it. Perfect stopping progression.

I'd have to weigh against the severity of the side effects.

Not that important at this point. FDA seal of approval doesn't mean much to a terminal illness. Need treatments in our bodies. Now.

It has impacted every aspect of my life. Every second of every day. Work, family, kids, friends, mental health, activities, faith.

Negatively. There is no upside to ALS. Financially, emotionally, workload, family time.

It is the biggest monster around. **We need a glimmer of hope.** Something. Anything to try.

Biogen needs to release Tofersen now under compassionate care or expanded access. They need to understand their interpretation of impact on phase 3 trial results are wrong. **Something so promising doesn't need another phase 3. Do it now. Track it now. I'm dying waiting. 4-month wait could be difference of walking versus wheelchair, living versus dying.**

Jessica P. | Utah

Age: 39

Year of Diagnosis: 2020/2017

Type: Bulbar Onset



I fear ALS is all too often swept under the rug as a rare disease hardly anyone ever gets. An extremely unlikely fate, which affects very few. I believed that for many years until the neurodegenerative condition crept into my life not once, but twice.

In July of 2017, one of my closest friends was diagnosed with military service-related ALS at the age of 48. Brian was an athletic and spirited man. He was a woodworker, artist, mountain biker, and friend to many. We couldn't believe he had received this devastating diagnosis. Over the next three years, our community rallied around Brian while he gradually lost all of his physical abilities but fought hard to maintain his spirit. **Even despite Brian's veteran status, his efforts to participate in a clinical trial or experimental treatment were never fulfilled.** Brian passed away on June 8, 2020.

One month later, on July 9, 2020, my mother visited my husband and me with terrible news. She too had been diagnosed with ALS. At first, I was sickened with the thought that we would have to watch another of our closest loved ones suffer this fatal demise. Quickly, however, my sadness turned to desperation as I tried to contact every research center and neurologist, I could look for something...anything would be better than the lack of treatments Brian received.

The path was tough with absolutely no guidance or support offered by most including my mother's neurologist. Finally, thanks to the Forbes Norris Center in San Francisco, we became aware of the HEALEY Platform Trial and were able to get my mother accepted in the trial a few months after her diagnosis. This was truly a gift for which we are eternally grateful. Over the last six months of her trial participation, however, we have continued to learn about promising new therapies. **Unfortunately, each of these therapies seems to be blocked by corporate processes and bureaucracy leaving people with ALS hopeless.**

ALS is an always fatal condition that is unrelenting in its progression. It is not waiting for the red tape of our society to be removed. **This is why I request the FDA make every proven safe treatment available to ALS patients.** Doing nothing is not an option. People with ALS and their families are willing to take the risk and deserve to get a chance.

Each patient should be able to decide this for themselves. The risk of no benefit or slight side effects would be worth it in my experience.

I have lost one loved one and my mother is currently living with ALS. It is devastating and an overwhelming condition for families to support.

I want the FDA to know that it actually does happen to people.

Please put people before profit.

Paul C. | Tennessee

Age: 53

Year of Diagnosis: 2020

Type: Bulbar Onset, Limb Onset,

We
Can't
Wait

All therapy is important if it can help me maintain quality of life for a little while longer.

All drugs have risks. I should be able to decide for myself if the risks outweigh the benefits or not. I'm the one diagnosed with a terminal disease.

Not important at all. As I said, let me make the decision to assume the risks of the new therapy.

I am dependent on others every day. I've given up activities that I love and struggle with the activities I can still do.

My wife had to become my caregiver along with assume all other activities that I used to do - and work. It is stressing her out. My children have to help me get dressed, get off the toilet and eat - taking a toll on them. They all feel helpless because they can't stop my progression.

That it exists and more people than they can imagine are stricken with this hideous disease.

If you can fast track a vaccine in a matter of months, why can't you do more for us? Our disease has been around for decades. Don't we deserve more than 2 approved drugs that might slow progression?

Lisa B. | Wisconsin

Age: 55

Year of Diagnosis: Dad: 2002

Type: Bulbar Onset, Limb Onset

We
Can't
Wait

I've known several people who have been diagnosed with ALS since my dad was diagnosed. The one thing they share in common is they wanted more time with their loved ones. More time being able to feed themselves. More time with being able to walk or talk. **Any chance of slowing the disease opens them up to more quality time with their families.**

I don't view having no benefit as a risk. It may be a disappointment, but it's worth trying. We would have been willing try just about anything to help my dad. These people are living with a death sentence. As far as side effects, that's for those who are living with ALS to decide personally. As with any drug, you weigh the side effects with the benefit. Again, the mere chance that something would help outweighs the risk.

The clinical trial process is too long. I am not advocating that any drug brought to trial be widely available. There still has to be guardrails. But as I said before, people with ALS are living with a death sentence. They are trapped in their own bodies. Any promising drug should be made available.

My dad was diagnosed at the age of 60. He died 27 months later. It was heartbreaking to watch him slowly lose the ability to use his hands, then walk, then talk. He was diagnosed in 2002 and the technology available today wasn't there. **The next time you feed yourself, think about not being able to do that. The next time you have an itch, nope can't scratch it.** Want to turn over in bed to get more comfortable - sorry, no can do. Have to go to the bathroom, hold on, someone will help you shortly. These once independent people are now relying on others to provide 24/7 care for them. My father had to allow his daughters and son to help him in the bathroom and shower him. Any modesty needs to go right out the window. Don't get me wrong, **it was an honor to be able to assist my dad, but it also was very taxing and stressful.**

Our family was lucky. Our community rallied around my dad and us. We had a caregiver come and get dad up during the week to shower and feed him. His 3 children rotated putting him to bed every night and getting him up and showered during the weekends. He needed care going to that bathroom and feeding. **It's absolutely heartbreaking to see someone trapped in their own body.** To go from being active, to not being able to move. During dad's illness, we were the master of schedules. Making sure there was always someone there with him, someone who could feed him and take him to the bathroom. In addition, we were always researching new therapies and got our hopes up every time we heard of a new one. The stress put on the families of ALS patients is extremely high.

In my opinion, there is no worse diagnosis. Almost every other disease has a potential cure, or the hope to prolong life and hold the disease at bay. ALS is a death sentence where you are trapped in your own body. Slowly you lose function. Slowly you lose the ability to do things for yourself you never thought twice about. You rely entirely on others to do EVERYTHING for you. When my dad was diagnosed, I never knew someone with the disease. We participated in the first ALS walk in Appleton, WI and I think there were about 20 people there. Fast forward to today and there are thousands of people. It is so sad to see how many people in our area are affected by ALS. This disease is not "someone else's" disease. It's affecting your coworkers, your family and your friends.

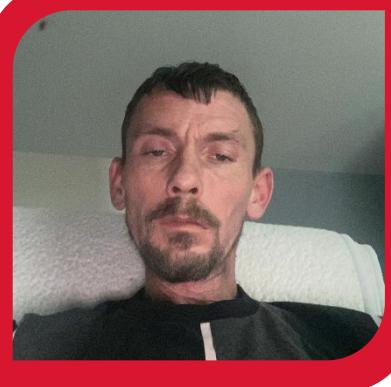
Please continue to work tirelessly on a cure. Don't give up, because we're not giving up hope. Please make new therapies available as soon as they show promise. Folks with ALS know they are going to die without new therapies. They are willing to try anything for a shot at slowing down the disease in the hopes of spending more time with their families and an eventual cure.

Justin P. | Delaware

Age: 38

Year of Diagnosis: 2019

Type: Sporadic



I think it's very important to have access to new therapy and **should be my right to decide if I want to try them**

I think it's worth it to me

I think it's important that clinical trials show that they work but I don't care if FDA approves it first

ALS has made my life very difficult I can't do stuff I enjoy doing. I loved working and providing for my family and now I can't I **struggle every day and the little bit of money disabled gives me don't let me provide for my family**

It's put a lot of stress on my wife. **It really hurts my kids because their father can't do anything with them anymore**

This disease is no joke it turns your life upside down quickly and we need better treatment or cure

I would like for them to know that **sometimes you need to think about the people living with this disease and not your pockets**

David W. | Wisconsin

Age: 68

Year of Diagnosis: 2015

Type: Bulbar Onset



**We
Can't
Wait**

Very important to have the opportunity to try medicine that could help me. It depends on the side effects. It is not important to me that it is FDA approved.

It took my life away. Has taken from my family's life as well. ALS and how it effects every aspect of your life - it's awful.

"We need to look beyond the box"

Susana R. | California

Age: 62

Year of Diagnosis: 2019

Type: Limb Onset

We
Can't
Wait

I believe new therapies are very important so that life can be extended without the horrible symptoms and disabilities.

I would be willing to try a new therapy but would need to find out the side effects.

Clinical trials are good of course, however, just like the COVID 19 vaccine I would be willing to try a new drug.

I have the slow progressive ALS but my life has been impacted as I walk assisted with a walker. I have weakness in my hands and my energy level is 50% of what it was b4 ALS diagnosis.

The diagnosis has had an emotional impact on my husband and sons.

I wish people would realize this is a terminal disease as the patient slowly deteriorates.

Please collaborate with countries such as Israel and European countries where there have been good results from their clinical trials.

Jeff O. | Arizona

Age: 50

Year of Diagnosis: 2019

Type: Sporadic



With very few treatments available currently I would welcome any new medications. I believe any medication it is still worth a try. I would be willing to try it before approved

ALS is a horrendous disease that has been known about for over 100 years. It is pathetic that there are not better treatments available or even a cure. I am no longer able to hug my family and friends, move any part of my body on my own, or even use the restroom without assistance. It is humiliating and 100% deadly.

There are not words to explain how this impacts my family and my children and friends. It is horrible for them to know that they can't save me and that I have a limited time to be with them. It is heart breaking. You become solely dependent on everyone else for everything and that it is always fatal.

Please find a cure as soon as possible!

James C. | Miami

Age: 51

Year of Diagnosis: 2017

Type: Bulbar Onset



This is important to extend quality of life.

I would try any medicine; I have nothing to lose.

I need options to try and want every opportunity to improve my quality of life.

At first, I went into a deep depression. I miss having a normal marriage with my wife as she is one of my caretakers. I miss my career. **I miss exercising and having normal, fluid conversations. I miss eating.** That being said, I have a decent quality of life using eye gaze software , I'm able to communicate and take college courses.

It has put a huge burden on my wife and children and finances.

We are still the same intelligent people we were before ALS.

Accelerate drug approval and give us access to experimental drugs.

Aqeel A. | Dubai

Age: 38

Year of Diagnosis: 2020

Type: Limb Onset

We
Can't
Wait

1% improvement is good enough to try new drugs, I have nothing to lose.

If drug shows benefits, then no need to wait for lengthy approval

Every month I lose a function, feels like will be bed bound in next 6 months

Life has gone upside down

**Approve any drug that shows 5% improvement.
Approve NurOwn asap.**

Lesley K. | Iowa

Age: 58

Year of Diagnosis: 2018

Type: Limb Onset, Sporadic



It is very important to me. Every day is important and means more time with my family and friends.

Any chance of a new treatment is worth the risk. I have done several clinical trials and would like to participate in more.

It is not important to me at all to wait for FDA approval. We don't have a lot of time. I'm willing to take the risk.

I've gone from being a healthy active fitness instructor to needing a walker to walk. **I can't lift my grandkids. It has Impacted every aspect of my life.**

My husband is impacted the most. I can't help him with our farming operation, he has to help with daily household chores. He has had to make changes to our home to help me function more easily. My kids and grandkids are impacted as well.

That anyone can get it, there is no cure. I didn't do anything to make me get ALS. I can't help that I'm tired all the time and I am getting weaker and slower.

I don't have a lot of time. I will decide for myself if a drug is worth the risk. Give me that chance.

Deborah G. | New York

Age: 53

Year of Diagnosis: 2019

Type:

**We
Can't
Wait**

We need a Cure. It doesn't matter if it's approved prior to trying.

I was diagnosed with PLS and impact on my daily life is terrible. **Life is not good.**

It's hard to live with. Please help.

Karen K. | Connecticut

Age: 67

Year of Diagnosis: 2020

Type: Limb Onset



**We
Can't
Wait**

Extremely important. As there is no cure presently, time is precious to ALS patients. Having more time makes being alive for a cure a better possibility.

If the drug ends up having no benefit then nothing was lost for trying. As for side effects, each individual would need to determine the risks vs. the benefits for themselves. For me, it would depend on what the drug did to the quality of my life to determine if it was worth continuing.

Not terribly important as ALS patients don't have the luxury of sitting back and waiting for a future decision. Each individual should be able to make a personal decision with their doctor and as many facts that are available at the time.

I am in a wheelchair as I can no longer walk. I can no longer drive and we had to buy a wheelchair accessible van so that I wouldn't be housebound. I need help with dressing, food preparation, toileting and bathing. I miss simple things like baking, shopping and especially running around with my grandchildren or even preparing a meal for them. As I was diagnosed approximately 6 months after my retirement I feel like my retirement was taken away from me and I've become a burden to my husband. I've had to give up my independence. **And I know that I am only going to become more disabled and helpless until I am totally paralyzed.** It's both physically and emotionally exhausting.

Although my husband is wonderful, he is my primary caregiver **and very overwhelmed at times with both the physical and emotional aspect of my 24 hour care.** It has changed any plans for our retirement and each day has to be planned around my care. As I am dependent on him, his independence has also been curtailed. He has a hard time emotionally as he is unable to "fix" me.

How devastating it is physically and emotionally for both the patient and families to watch the decline and know that there is no cure. It's a horrible disease and a horrible death. Why is there no cure yet?

Please work on finding a cure but in the meantime allow patients to use their own discretion as to what treatments they are allowed to try. We're dying and time is something we don't have.

Mike S. | Tennessee

Age: 78

Year of Diagnosis: 2019

Type: Sporadic

We
Can't
Wait

It would bring so much hope to have the chance to try a medicine that could help improve my life. I am not worried about the side effects or having FDA approval. I just want a chance.

Reinforced the value of every day and family.

They've circled the wagons and continue to surround me with love and support.

Their support is necessary for the quality of life.

Approve AMXOO35 for widespread use!

Ron B. | Indiana

Age: 64

Year of Diagnosis: 2019

Type: Limb Onset



It is important to receive any medication possible to help provide us with better lives.

This is devastating and horrible.

I am devastated. My family is devastated.

Everyone is different but we need to find a cure.

Bradley P. | Wyoming

Age: 56

Year of Diagnosis: 2020

Type: Sporadic



We
Can't
Wait

How will I know the difference of a few months? What will my well being be at that time

Not interested in side effects. This disease has provided plenty of those.

Completely, but not as quick as Covid approval, that is a mess

How would you think? I am deteriorating and becoming dependent. With a smile ?

People are a lot worse than me; a cure needs to be found. **Hurry up, gather funding and develop a cure**

Bobbie C. | Florida

Age: 49

Year of Diagnosis: 2020

Type: Limb Onset, Sporadic



I think anything we can get that helps, whether it's a few months or longer, we should have access to. I think we should have access and let it be our choice to deal with side affects. Considering that chemo has major side affects, why can't we have that chance too. It should go through some testing, of course, but we should still have access if it is showing improvement. We should have access to any compassionate care drugs.

I had to sell my business, give up my career, lost ability to do everything. This disease has affected every aspect of my life and my ability to make money or provide for my family. I am now completely reliant on someone else to do every single thing for me. Even something as scratching my nose, I cannot do that myself. **Every single thing, big or small, is affected and taken away from you.**

My two children have given up their careers and their lives to take care of me. Caring for an ALS patient takes so much time and energy. You have to give up every aspect of your life to be there and be an ALS caregiver.

I wish people understood that, as ALS patients, we still have our minds, and we can still make decisions. We can still hear you; you don't have to shout. What is inside is the same, but your body can't move. I wish people understood more about what this disease is because living with it is so grave, and people don't seem to understand how awful it is.

I want them to know that we need access to affordable treatment and caregivers. We are expected to quit our jobs, family members have to quit jobs, so no one can make money, but we have to pay for our own caretaking and pay out of pocket for so many medications because little is covered by insurance. **WE NEED HELP!! WE NEED HELP FROM EVERYONE!!** We need a way to be able to sustain some kind of livelihood and at the current state that this country is at with ALS, we are not able to sustain livelihood.



Baker | Texas

Age: 63

Year of Diagnosis: 2017

Type: Limb Onset



We
Can't
Wait

If the promise is for a few months, I'm really not interested. Again, if the promise is only for a few months, I'm not interested

Not very important to me if it is approved.

My life has been turned upside down because of the debilitating effects of this disease

All have been affected somewhat. My wife has been affected a great deal, particularly with her work.

Constant fatigue along with the embarrassment of extreme help necessary for everyday tasks.

I'm much more interested in pharmaceuticals that give back some of the strength in my extremities rather than extension of longevity for a few months.

Todd W. | New York

Age: 56

Year of Diagnosis: 2019

Type: Bulbar Onset



Any help would be a positive, give hope where there is none.

Anything is better than nothing, right now there is NO hope to live, née drugs may not help but there may be a chance which is something ALS patients don't have now.

Todd would have tried anything for a chance to live, he died only 14 months of being diagnosed. I don't feel the FDA has to approve something when there is no hope.

There is NO life anymore, ALS took his life.

Losing Todd has been a nightmare for myself and his children. It's a death sentence, and it doesn't have to be- whether it be through drugs or herbs

I'd like them to help people not their pockets.

Sara S. | Wisconsin

Age: 44

Year of Diagnosis: 2020

Type: Genetic

We
Can't
Wait

Having the chance to try a new medicine that may or may not help, It's better than nothing.

Incredible. Hopeful. Gives pals a fighting chance. We're dying- how is this not understood? Let us try.

**We're dying. We're volunteering to be the test subjects.
Give us the option.**

It's a freight train that has destroyed it.

It's destroyed them. There's no hope. All they can do is watch us waste away.

You die a little inside, and outside, everyday. From the neurologists that misdiagnosis due to inferior education, or pure hubris, to the crippling onslaught of debilitating symptoms. Every day you die another small death. Let us fight. It's all we ask.

"You're playing games with our lives. You've given us nothing. Then snatch away even the smallest opportunities. If this, were you, or a loved one: What would you give for a small chance of hope? The answer is Anything."

Esteban B. | Ciudad de Buenos Aires

Age: 51

Year of Diagnosis: 2021

Type: Bulbar Onset

We
Can't
Wait

I find it really important even if it implies a modest benefit

The fact that there isn't any cure makes you want to try new things, even thought that may bring no benefits at all.

For me it is essential to have the FDA approve something I would try. It makes me feel secure and positive.

At first I was devastated. I am a very active person and ALS meant the end of everything. But then I felt in peace. I am a catholic person and believe that God never asks you to carry a cross heavier than you can bare. I believe that from my public role I could help not only myself but others. And that's what I'll do. My family is my central support.

I was afraid to tell people around me that I was diagnosed with ALS because I felt they wouldn't understand that I wanted to keep on working, being active and not treated as a diminished man. I received exactly the opposite reaction. People around me went extra supportive and we immediately started working together, investigating all treatments available, and speaking to other diagnosed people. We've made a very good network and still pushing forward.

More awareness

To keep on developing this trials and medicines that gives ALS patients hope and strength to keep on going on.

Celisa M. | Nevada

Age: 55

Year of Diagnosis: 2021

Type: Limb Onset, Sporadic



A few months can mean attending your child's wedding or getting to meet a grandchild. When you have 36 months to live , 40 months is better, it's the small things that begin to matter when your life has a known end date.

When you are terminal, you have nothing else to lose and you are willing to try because not trying is giving up. There are always consequences to decisions, and I am willing to accept them for the benefit of saving my life or the next ALS patient.

I am an RN with 20 years of experience and conducted 4 clinical trials during my time. It is not important at this stage (living with ALS) to have proven drugs due to the time constraints. I'd be more than willing to start a Phase II approved drug and be tracked by whatever means.

Ended my 20-year nursing career. Ended my college studies for my advanced degree. I am not prepared to retire because I love my job and thought I'd work until I was 70. Relocating to be near my children because they are the most important now. I get to sit and watch them do what **I want to do so much!! I worked hard to be an independent strong woman but now my identify is "she is dying".**

I have 4 boys which I raised mostly alone and two of them are married. They are now having children (earlier) than they wanted so that I might get to know them. Careers are put on hold so they can spend as much time with me. Raw conversations occur about life that they weren't ready for.

There is no reason as to "why me" and it might be the most complicated, erratic and insidious disease a person can die from and those who can help with some hope, even just a little real hope, aren't or won't.

NurOwn passed phase 2 and was approved for phase 3. I have personally talked to some people who had benefit and reversed! I get that it didn't help all and they had a placebo effect. But I think the number was 34%? I know of 68 people who died last month, if they all had NurOwn, we could of saved 23 of them!! **Twenty-three lives lost because of "red tape".** If it's only producing those results for patients in early stages, then save them. You have to start somewhere, and the time is now! **Start with us, who are alive but living with a "tick tock" in our brains as we count down to our expiration.** Let us help you find a treatment or even a cure! Be humble and realize that ALS is different than other diseases and those that are suffering need to help you so that you can help us and future ALS victims.

Frances P. | Pennsylvania

Age: 67

Year of Diagnosis: Husband: 2020

Type: Limb Onset, Sporadic

**We
Can't
Wait**

My husband is willing to try any new therapy.

My husband feels he has nothing to lose by trying new therapies.

That is not important to my husband or me.

I am my husband's caregiver and I take care of him 24/7. It is hard to see him get weaker and less independent. It is hard on our children and grandchildren to see him participate less and less in their lives.

My husband can't participate in my children and grandchildren activities. My grandchildren miss him doing things with them and one always says that he wants his grandfather to be the old way.

I wish people would understand that even though the person with ALS looks different, he is still the same person. They want people to talk to them and not be uncomfortable with them.

Please help ALS patients by letting them make choices on treatment. They don't have time to wait.

Patricia J. | Texas

Age: 71

Year of Diagnosis: Husband – 2017, Death 2019

Type: Limb Onset

We
Can't
Wait

My husband died 2 yrs. after diagnosis. We went overseas for stem cell treatment which slowed progression for about 6 months. It was 6 months of time where he could at least function and maintain some dignity. We both were so thankful for that short respite.

Why couldn't we take this risk in our own country? Both of us would've given anything to try any drug that might have helped, no matter how short the time benefit or risk. No hope is as bad as the death sentence already handed down.

What's the difference if there's a risk? ALS is 100% terminal, not even one chance to live or beat it. Side effects are already horrific, so we would've been willing to sign any waiver at all to have a chance at something that might remotely help.

Not important at all! This is a death sentence, so why not clutch at any straw any time?? The alternative is hopelessness every single day. Let the ALS patient incur the risk. I guarantee they will sign every waiver given them if it means some chance at a better life.

My husband wanted to show my children and especially grandchildren that you fight with everything you have. He tried anything at all-going to South Korea, alternative medicine, random drugs that were suggested by almost anyone. ALS robbed him of his very existence, so all he had left was to be a role model fighter for his family.

As his caregiver, I was devastated every day in every part of our lives from emotional day to day living to potential financial ruin. ALS caused both of us so much pain. The repercussions after his death were enormous for me-I can't let go of the pain we suffered. PTSD like flashbacks hit me before I sleep and when I awake every day.

ALS ruins you bit by bit and there's nothing you can do. Lack of dignity, depression and fear are a fact of life EVERY SINGLE DAY.

I am begging you to EXPEDITE ANY AND ALL TREATMENTS-especially revisit the full denials of both AMX0035 and NurOwn. No one should suffer as we have.

Frederick A. | Pennsylvania

Age: 64

Year of Diagnosis: 2019

Type: Bulbar Onset, Sporadic



Any chance to prolong life is desirable, just to slow down progression of this horrible disease would be welcomed.

If a therapy has no harmful side effects, I see no reason to not try it in hopes it might help in some way.

I am not able to wait the time needed to complete a clinical trial and the red tape associated with them.

Disrupted everything, can no longer work, lost my recreation, can no longer speak or eat, starting to lose mobility. Trying with all my heart not to lose hope. What hurts the most is the fear in my wife's, children and grand children's eyes when they recognize what I can no longer get do.

Terrifies them. My wife had to stop working to care for me, our retirement and are now null and void.

How difficult it is to be isolated in your own body even when you're with people.

Look how quickly everyone pulled together to get covid vaccine out, I do not understand why the same protocol cannot be used with ALS therapies. ALS is always fatal, 100%, no maybe, would gladly sign a waiver to get a therapy while the experts try to find a cure.

Sybil C. | California

Age: 72

Year of Diagnosis: 2020

Type: Limb Onset

We
Can't
Wait

Guardedly – I may try some medication.

Not sure if I would take on risks; would need more information. That would be highly reassuring if it was approved by FDA first.

Would it be an understatement to say it has ruined my life? Devastatingly cruel. **It is a cruel, horrible disease.**

I beg you to hurry with a cure. What about pouring money into NU9 and speeding that along?

Pam M. | Louisiana

Age: 62

Year of Diagnosis: Brother: 2020

Type: Limb Onset



**We
Can't
Wait**

I think it is very important. I see my brother's disease progressing pretty rapidly and everyone who loves him is helpless. There is nothing to help him. If there was something that could slow this disease, it would mean the world to him and to everyone around him.

As of now, there is nothing for ALS patients. I know that my brother would gladly take a drug that may not help him or even something with side effects. What is the alternative now? Nothing!!! So if something may potentially help him, he would take it.

It isn't that important other than what the cost may be. If it isn't FDA approved, from what I understand insurance wouldn't cover it and the drug would be very expensive.

It has impacted my brother greatly. He went from being a big strong working member of society, to now being confined to a wheelchair with family members having to feed him. It has affected everyone around him. **We cry all the time. We feel helpless.** There is nothing for us to do except watch my brother's condition get worse by the day. It is heartbreaking. I beg of everyone to make medicine available to ALS patients even though not approved by the FDA.

Everyone around him has given up free time to be a care giver. His wife can't do it alone. From fixing things around the house to just being with him so he won't be alone in case he needs something.

It is not just my brother suffering but the whole family is suffering. It is an awful disease. It takes everything from you. And you can not do anything for yourself anymore. You have to rely on someone for everything.

Every day that passes is critical. My brother needed medicine a year ago and he is willing to try anything. He has nothing to lose from trying medicine even medicine not approved by the FDA.

John R. | Pennsylvania

Age: 65

Year of Diagnosis: 2013

Type: Limb Onset, Sporadic



Any new therapy that slows the progression of ALS is critical to ultimately solving the 150-year-old riddle of this disease. Meaningful treatments and ultimately a cure will be built upon a foundation of incremental improvements in the now dreary prognosis for patients with ALS.

There isn't much worse in this world then living with ALS. Currently, a patient starts out with a 2-to-5-year prognosis with zero chance of reversal or survival. The chance of modest benefit far outweighs the risk associated with participating in a drug trial.

Considering the fact that I have never been eligible for a drug trial because I had symptoms 30 months before diagnosis, and that **there are so few opportunities for ALS patients to get into any trial**, I feel that it is less critical for the process to be complete, and the drug approved by FDA prior to taking it. AMX0035 is the perfect example of a drug I would take prior to the full process being completed.

My wonderful life of movement and doing things I loved to do has not existed for the last 3 years since my diagnosis. Although I am able to function mentally, still eat real food and breathe without assistance, along with the fact that I live comfortably with my wife in a beautiful home and neighborhood, this is not a life I would choose for anyone. Grueling both mentally and physically, **ALS has destroyed what was an incredibly beautiful existence.**

My wife and I have been together for 42 years. we have no children, so she is my primary and currently only caregiver. Her life has changed courses along with mine. it has already taken a mental and physical toll on her. The other most affected person is my 93-year-old mother who still lives on her own. She has endured so much in her long life and watching her child succumb to this disease is heartbreaking. **Our entire family and long list of friends are forever changed by my ALS journey.**

ALS does not discriminate. There is zero chance of survival. Your chance of getting it is the same as multiple sclerosis which has proven to be a long term, livable disease. Anyone living on the planet should be fearful of presenting with ALS.

Personally, I understand that ALS is a very difficult puzzle to solve. **I appreciate the efforts of pharmaceutical companies willing to invest in ALS research.** I also applaud the effort of our FDA to work more closely with industry over the past 5 years. That said, ALS is not like every other disease. The history of failed therapies and the long list of deaths with no chance of survival is ridiculous in this day and age. **I would like all involved to think outside the box with regard to clinical research, clinical trials, and the rules that apply for all drugs that may not be applicable if we really want to solve the mystery of ALS.**

Dave D. | Minnesota

Age: 60

Year of Diagnosis: 2020

Type: Limb Onset, Sporadic

We
Can't
Wait

Very important to have a new therapy that offers the chance of a modest benefit. Depends on the side effects how much of a risk I will take.

I read quite often that people can't get into trials because the disease has progressed too far. It is somewhat complicated to find trials and sign up, or they take very few people around the world into trials. We have a cohort of many willing participants who want to live and will try most anything to do that. The FDA could be a leader in the promotion of new treatments and trial participants. **What is the worst thing that could happen? I am going to die, I know that. We all do.**

Not getting a chance to try these promising drugs, therapies, or treatments will not solve that issue of death, but just maybe, it can push the envelope of developing a tool that will allow people who have ALS to live longer. Or provide some hope to allow researchers to find the cure for future victims of this or any other terminal disease. **By trying treatments and new therapies, people with ALS can give back or be of some service as our bodies break down, showing if these treatments are working to prolong lives.**

ALS has slowed me down and I worry much more. ALS has made my friends and family sad and wonder how they can help.

We are not dead yet.

That I know I would like to live longer.

Leo M. | Bear Creek

Age: 72

Year of Diagnosis: 2021

Type: Bulbar Onset



It is extremely important to me to have a new therapy that offers the chance of a modest benefit even if it has no benefit or significant side effects. I would be willing to take the risk in order to have a chance of even a modest benefit.

In the case of ALS patients, if a potential drug is showing success in slowing down disease progression or improving quality of life, it should be available to patients to let them determine if they want to take the risk with only phase II complete. **Timing is crucial to NOT WAIT.**

Overwhelmingly affected by ALS. I can no longer eat or drink via the mouth and have a G-tube in place for nutrition and meds. Every day is a gift from God.

This horrible disease is heart wrenching for my family and friends, especially knowing there is no known cure at this time. All our future plans and day to day living have been affected. We pray for a cure every day.

That everything needs to be done to help find a cure and extend quality of life for those afflicted with this terrible disease.

Please hear us and take action NOW to approve use of AMX0035 for ALS patients, and any other treatments that are beneficial in Phase II trials and NOT WAIT for Phase III. Please let the ALS patients have the dignity and right to make their own choice to take accept any risks and give us the hope of more time with our families and friends.

Steven Y. | Montana

Age: 66

Year of Diagnosis: 2018

Type: Limb Onset

**We
Can't
Wait**

I believe that anything that will slow progression even in the slightest is worth trying immediately.

It is not important that the FDA approve the drug before I take it.

Any risk is outweighed by even the slightest benefit of a longer life even if it is just months.

ALS has basically ruined our life together. We had wonderful dreams of how we would spend our retirement years and now that is all gone. We are living day to day, and it is getting harder and harder to keep going.

It has put enormous strain on my wife who is my only caregiver. ALS is a death sentence unless they can come up with a new drug.

Andrea | Sonora, Mexico

Age: 35

Year of Diagnosis:

Type: Bulbar Onset

**We
Can't
Wait**

I prefer a longer life with better quality.

I would like a new therapy that offers the chance of a modest benefit even if it has significant side effects.

It is important that the FDA approve the drug before I take it.

Everything has changed, I am not independent, I feel trapped in a body.

My family has changed their lifestyle because of my illness, which makes me very sad.

ALS is a more common disease than is believed and that there is no cure.

We are waiting for a new hope to be able to live with a better quality.

Gudjon | Reykjavik, Iceland

Age: 61

Year of Diagnosis: 2004

Type: Sporadic

**Click
Here to
Play
Video**

Hope is all we live for; hope is so important.

I don't mind trying a drug that is not approved by the FDA yet. **I am waiting and dying. I'd much rather die trying.**



Safe is the only thing that matters. Please do whatever it takes to get us a drug in every way.



My family is affected, I am affected, and our social life and health are too as a family. **It is killing most of us very fast. 100% deadly.**

We choose life but do not have the time to live. But we do our best with help from our friends and family.

Please watch my video. Please help me have more days like those I experienced. Please don't make it my last.

Diane C. | Matthews

Age: 51

Year of Diagnosis: 2020

Type: Limb Onset, Genetic

We
Can't
Wait

I think this is very important to have access to drugs now. A diagnosis of ALS often means a diagnosis of a life expectancy of 3-5 year. A few months can increase the possibility for eligibility for further treatments until there is a cure or a treatment that can slow symptoms even more.

At this point I feel like I don't have much to lose. I am dying either way. Again, I am in favor of trying something rather than just letting ALS win with no fight. I took the COVID vaccine that was developed very quickly and provided emergency dosage use authorization and would easily do the same for ALS treatment.

ALS has had a dramatic influence on my life. I have had to stop working as a school social worker (a job I loved). My family has had to move because of having too many stairs. I have not been able to help with making our new house our home (unpacking, painting). I can no longer cook, clean, or walk the dog. Everything I do requires so much planning and effort that I just don't want to do them. Even typing this is as struggle as my hands and fingers are weak and clumsy.

My family has been great, but it is hard. My children are 16 and 19. I've always been the primary caregiver but now they have to help me with so much. My husband now does most everything around the house and it has been very stressful for him. **We can't wait. That's the bottom line.**

My father was diagnosed with ALS in 1998 and died in 2000. I was shocked and disappointed that there was still so little in therapies (only one new one) for treatment 20 years later when I was diagnosed with ALS. **I can't wait for the long drawn out processes the FDA uses because I am dying. My siblings and children can't wait either as they are at high risk for receiving this diagnosis too.**

Shaun K. | California

Age: 39

Year of Diagnosis: 2017

Type: Limb Onset



A few months of extra life is better than nothing. **I'd rather die trying than die waiting.**

It is not important to me to wait for FDA approval. I am diagnosed with a terminal illness. ALS has upended my entire family life.



My family all suffer as they watch me deteriorate before their eyes.

I wish the world truly knew the **devastation** it takes out on entire families.

Conditionally approve treatments so patients can at least try hope, you already know what we want.

Nancy K. | Colorado

Age:

Year of Diagnosis: 2018

Type: Limb Onset

We
Can't
Wait

I am a primary care giver for my husband. It is extremely important that ALS patients have access to any new therapies that show promise.

People impacted by ALS do not have the luxury of waiting for the usual process that the FDA requires for clinical trials.

If a therapy offers a chance of modest benefits, I think it would be acceptable as long as the side effects did not worsen the condition of the patient.

In the case of ALS, FDA approval is not important. It is much more important to make any therapy that shows promise available as soon as possible.

Our lives have been totally upended by ALS. We spend almost every waking moment trying to produce solutions for the newest symptoms that appear. Trying to plan any kind of outing is a major undertaking. Where once we spent our retirement hiking, exploring the outdoors and planning visits with our children, we now spend most of our time inside a small condo surrounded by accessibility items.

Everyone in our family has been impacted by the diagnosis.

How devastating this illness is, and how little time there is to wait for clinical trials to offer any help.

There need to be exceptions to your very stringent rules for approval. Trials need to be open to more patients and in more locations. I tried to get my husband into at least 4 trials, and he was not accepted for one reason or another.

Alok R. | Assam, India

Age: 56

Year of Diagnosis: 2020

Type: Limb Onset

**We
Can't
Wait**

No, we don't need a therapy, all we need is a cure, a permanent cure. For the end of ALS.

Please do something FAST! PLEASE? THE CURE IS NEEDED VERY VERY URGENTLY.

The situation is very bad up here. I can literally see my family tearing apart and I can't Help. Please please find a cure for ALS. PLEASE, AND FAST!

I will do whatever it takes. Can you please make the process of finding the cure faster. **It's really very urgent!**

Marc L. | Florida

Age: 64

Year of Diagnosis: 2019

Type: Limb Onset, Sporadic

**Click
Here to
Play
Video**

Since there is no cure, this is the only way to treat the disease. **I find it heartbreaking that physicians can really offer no help. But if some drug therapies can slow The disease this would be extremely beneficial for those affected by ALS.**

The effects of ALS are worse than any potential risk from a drug that could have modest benefits. I would accept Significant side effects over continued disease progression given the choice of no therapy at all.

As a physician I feel it's important for the clinical trial process to take place. The FDA approval process has always been a part of getting drugs to patient safely. However, with certain diseases such as ALS the balance of how much benefit and potential risks have to come in to play. I do not feel that phase 3 trials are needed for every ALS drug.

I was an interventional cardiologist implanting life-saving therapies daily for patient care. Now I am relegated just sitting in a wheelchair, unable to use my hands with any purpose. Obviously, my career is over, and all my years of experience are lost.

This has affected my family, friends and patients. **Patients whose lives I have saved in the past now will outlive me and are trying to help me keep going on. There are no treatments available with any significant benefits.**

We need a paradigm shift in how we handle this disease for FDA approval of drug therapies. We should not have to wait for large phase 3 trials.

Robert M. | Oregon

Age: 60

Year of Diagnosis: 2018

Type: Bulbar Onset, Sporadic

We
Can't
Wait

It's a great idea if you live in the city to get fast approvals and access to medicine. Rural communities don't stand a chance.

I'm not sure if I would be interested in something that has a moderate or significant chance of side effects. I guess that it would be what kind of side effects? Death? Worsening of my condition?

Not important at all for the clinical trial process to be complete and the drug to be FDA approved before I try taking it.

ALS had dramatically affected my life! I can no longer enjoy the things that I loved to do. It's too dangerous with high risk of having more falls. I'm pretty much trapped. The people in my life has totally changed, from threats of having me placed in a home because I refused to sell my house and take the money and move to?? I love my home.

How devastating this disease actually is... People have said, Oh have another beer because they think that you're drunk, because of my speech. Very hurtful.

We are still the same person, with the exception that we can no longer walk or have it very difficult to walk without leg braces, other aids, walkers, etc.

Linda R. | Illinois

Age: 62

Year of Diagnosis: Mother - 1994

Type: Genetic

We
Can't
Wait

Every day I would go to work wishing time would just stand still so I could have more time with her.

Everyone else was "waiting for the weekend" but I never was.

There is no risk when someone only has a short time to live anyway.

As stated before, how can there be any risk when taking the drug before it is approved.

My mother's ALS diagnose brought great sadness for everyone that was close to her.

It is a death sentence.

Any kind of hope is all you have.

MC C.

Age:
Year of Diagnosis:
Type:

We
Can't
Wait

My comments to the FDA –

<https://www.statnews.com/2021/04/30/als-groups-wont-be-played-again-fda-drug-sponsors/>

ALS groups to the FDA and drug sponsors: ‘We won’t be played again’

By Mary Catherine Collet April 30, 2021

[Reprints](#)



Matt Scuzzari holds his girlfriend Sabrina Parker's hand during a friendship ceremony in Jacksonville, N.C., in November 2010 to pledge their commitment to each other. Sabrina passed away days later from ALS, which also killed her mother and grandmother.

GERRY BROOME/AP

Robert F. | Rhode Island (1 of 2 pages)

Age: 32

Year of Diagnosis: 2014

Type: Limb Onset, Sporadic

We
Can't
Wait

"Hello, my name is Julie Sylvestre and I am here to speak on behalf of my partner, Bobby Forster whose voice was stolen by ALS.

Bobby was officially diagnosed with ALS in December 2014 at the age of 25. After his diagnosis he eagerly sought out clinical trials and research studies, anything that might help extend or improve his life. Luckily in 2015 he got into the phase 2b clinical trial for Brainstorm's Nurown.

He created a petition shortly after the trial ended to push for access and accelerated approval in which he detailed his progress after receiving one dose of Nurown. He wrote, **"After two weeks of treatment I went from barely able to stand for more than ten seconds, to being able to walk with a walker, to being able to walk unassisted. I also saw significant improvements in my forced vital capacity (FVC) and speech."**

Other participants yielded great results as well. My improvements continued for a month, then my progression plateaued for a month before I began progressing again. This therapy is one that will require multiple treatments every couple months.

Without accelerated approval thousands of ALS patients could die over the next several years waiting for the traditional process for approval to be completed. This treatment has now been in multiple trials both in the US and Israel. It has been shown to be effective and has no significant side effects.

It is time for all ALS patients to gain access to this groundbreaking treatment. That was five and a half years ago and unfortunately, we have lost thousands of pALS while we waited for a phase 3 trial to be completed.

Age: 32

Year of Diagnosis: 2014

Type: Limb Onset, Sporadic

**We
Can't
Wait**

Bobby is still waiting for his second dose of Nurown and in the years since the trial, he has lost the ability to eat, breathe, speak and move on his own. In the fall of 2016, less than 2 years after his diagnosis, he was forced to make the life altering decision of getting a trach and becoming dependent on a ventilator to breathe. He was using eye gaze technology to communicate, until about two years ago, when his eye movement deteriorated and he has been unable to use the computer to speak.

Now we communicate with yes/no questions and occasionally can spell words or phrases using what little facial expressions he has left. While we have no idea if Nurown would still work for Bobby in his current condition, I know he would do anything for the chance to try.

He quite literally has nothing left to lose. He continues to fight because he knows that this treatment has the potential to help others and prevent them from suffering the same fate as him and so many others who have come before. Yet it has been stuck in the clinical trial process for over ten years and we are still waiting. Bobby may die waiting. He has said repeatedly, **"I am sick and tired of seeing my friends die from this disease, when I know that a treatment exists that can reverse my symptoms."**

FDA approval or not, he knows there is a treatment that works, and he has not been able to access it. It is inhumane to deny dying people access to promising treatments when there are no other options. Yet that is what you are doing right now to thousands with ALS. You have the power and more importantly the ethical obligation to the ALS community to take action now and help us change this disease from one that is terminal to one that is treatable. "

Omar S. | California

Age: 39

Type: Bulbar Onset



I pray for a miracle everyday. It is the most important thing that could happen for us. If a medication doesn't have any benefit, at least we were able to try instead of being sentenced to an early death. **I don't care about side effects if I have the chance for my body to no longer be a shell with a brain.**

Clinical trial process isn't important to me, we can all be the trial. We can no longer wait on the FDA to let us continue to meet our demise waiting on trials. If we can do it for covid which people have a chance is sentenced to 100% fatal should be granted the same opportunity, we have done nothing to cause this illness.

I can no longer work or do anything for that matters that doesn't include minimal use of my left hand. I can't even get out of the house as we cannot afford transportation that my chair can get into. **I lost everything I worked for within months.**

It's a nightmare My wife isn't the same, it's affected her health I would do anything to see her smile again. My friends, coworkers and family are devastated, once not knowing about ALS to educating themselves to everything they can read about.

I would like the right to possibly delay my demise from this disease. I have 12,6m old children. We are willing to take side effects, we (I) want a chance. **I want to see my kids graduate and make more memories. I want to hold my daughter again. I haven't been able to hold her since she was 3m old. Please hear our voices please.**