Public Witness Testimony Submissions from Those Impacted by ALS

Christy Alexander

I started experiencing my first ALS symptoms in 2002, when I was 36, about 6 months after my second child was born. I was finally officially diagnosed with ALS in 2005. I was 39, my children were 3 and 6. It was devastating. I was informed about a clinical trial by a doctor at the clinic I visited in San Francisco. I said I was interested but there was no follow up from the clinic. I called several times to inquire about it. Nobody had answers, nobody called me back. Overwhelmed by my diagnosis and trying to raise two young children, I gave up pursuing the clinical trial. In fact I never went back to the clinic. Later on I heard of a clinical trial going on in Italy. There were promising results that lithium was helping improve people with ALS. I persuaded my neurologist to let me try it. It gave me horrible headaches so I discontinued use. In the end lithium was not a successful drug for ALS. Most clinical trials exclude people who have had ALS for 3 years or longer. Many exclude people after 3 years since the onset of symptoms. Perhaps that was why I never heard back about my first attempt to get into a trial. I have not attempted to get into a trial since. Every time I read the requirements I see that I am automatically excluded. My neurologist has called me a slow progresser. He says I'd skew the results of a clinical trial if I was allowed to participate. Now it's 2021, there are real treatments, like NurOwn, that I'd love to have access to. There are clinical trials that I'd like to have a chance to participate in, but I'm excluded. I would like to have a chance. Things need to change at the FDA, so all ALS patients get a chance to try new treatments. My ALS story is long but this is my story as relates to clinical trials.

Jennifer Baringhaus

My husband was diagnosed in late 2017 and he went through dozens, maybe hundreds of tests during the time he declined through the first several months after his diagnosis. Jeff was still able bodied at the time and eager to try all treatments available, however he was ineligible for the limited trials in our area because the right gene wasn't impacted and the more promising trials in Boston or through the Mayo Clinic in MN put too much of a strain on our family to get involved with. Having young children at home and one working adult made the possibility of participating in a trial a dream but not reality. Eventually Jeff was able to convince his doctor to try a therapy for another neurological disorder which significantly slowed the decline and in fact corrected the atrophy in his hands, but because there were not significant improvements in the functional rating scale, the doctor stopped the treatment which was humiliating.

Sheri Baum

My son has been in the Healy platform trial.

Mark Bedwell

In mid 2018 it was getting hard to walk and talk and I had to use both hands to brush my teeth and brush my hair. When I ate, I spilled food everywhere but my dog did like that part. At the end of 2018 I was able to enroll in the NurOwn trial and after a few months I was able to walk better and even run and my customers at work noticed a big difference in the way I spoke. Whether I've got the real treatment or the placebo I don't know but something changed in my body for the better. I just can't wait until everybody can have a chance to get NurOwn in their bodies. It's been 2 1/2 years since my last treatment and I am beginning to decline again. Could you please help save my life and 30,000 American lives also?

Brenten Blakeman

I lost my grandpa and mom to ALS. It is a cruel and brutal disease. No new drugs have been approved for use despite numerous showing promising hope. A new drug could take 2-5 years for approval at current pace, 99% of people living with ALS today will be dead by then. If it shows hope, let the patients take a chance at improving their quality of life or extending it. ALS leaves patients, their families and friends hopeless and powerless. Have a heart and give them access to desperately needed hope. Open up your eyes and your heart.

Caroline Brautigam

My husband, Bill Brautigam was diagnosed with ALS in March, 2018 at 54 years old. He was fortunate to be able to get into a drug trial 4 months after his diagnosis. He was part of the Fortitude study by Cytokinetics. From start to finish his strength stayed steady the whole 4 months of the study. Within 2 months after the completion of the study he was losing his strength rapidly. It was later disclosed he had the mid dosage of the drug, Reldesemtiv, not placebo. I aggressively sought to get Bill access to this drug. I sent Cytokinetics an email and they had said outside of a clinical trial they could not give access to this drug, apply next time IF and WHEN there would be a phase three. They did not offer expanded access or compassionate use, NOTHING. When you are faced with death and NO HOPE of survival the PALS should have a say in what they are willing to put in their bodies. For this disease one drug will NOT fit all and FDA guidelines do not work for this very complex disease. The FDA was established 115 years ago and for ALS only 2 non effective drugs are available. Clearly traditional FDA guidelines DO NOT work for ALS. The new guidance document was signed and I actually cried because I felt like there was HOPE for my husband but

that was crushed when NurOwn was denied. They are denying drugs that are efficacious and safe. This is unacceptable!!! It is inhumane to not allow a dying person access to ANY treatment that has been deemed safe. I hope this Congressional hearing will give PALS the HOPE and CHANCE to access investigational therapies.

Sunny Brous

I've been part of many different trials and ultimately if the drug or therapy is deemed safe by DFA, trying it is better than the alternative of just waiting to die.

James Brunton

I've contacted companies and governments for trial access to try to get access to any promising treatment for my mother.

Jeff Cade

My name is Jeff Cade. I'm an athlete and a coach. I played semi-pro soccer, coached women's college soccer leagues, and was Director of a 2,000 kids soccer league. In February, 2019 I lost my ability to speak one night. Shortly after, my legs began to feel "heavy" at times. This resulted in a dragging of my feet while walking. My prognosis has gone from minor stroke, to MS, to aggressive MS, to ALSP, to unspecified with CSF1R gene. Today, just 2 years later, I'm in a wheelchair and my hands are beginning to stiffen. I have weekly physical therapy sessions, but I continue to decline. I no longer have the physical ability to cry. My emotions are sporadic at times and my medications don't do much to help, if at all. I'm desperately seeking solutions with the help of doctors at UCSF. However, the overall response is mass confusion. I hear a lot of, "I don't know...we'll run more tests...this is rare...we don't have treatments for this type of disease" and nothing about what can help me...NOW! This disease impacts me. But, it also impacts my family—my 75 year-old mom who is my caretaker, and my sister and brother. And, since it impacts my sister and brother, it impacts their spouses, their jobs, their surroundings. This disease is a domino effect of frustration, sadness, and confusion. All that we want are treatments that will work!

Martha Carr

I was so shocked when I was diagnosed because there were no 'weapons' (drugs) to fight this disease. I have been on a waiting list for a clinical trial for over 6 months. I've read everything I can and take so many supplements because that's all I can do.

Nicole Cimbura

My husband, Mike Cimbura, was one of the 36 participants who received the drug NurOwn in a Phase 2 clinical trial for amyotrophic lateral sclerosis. Mike regained some function, but he was able to get only one dose before the trial ended. We

quickly sprang into action and contacted FDA, Congress, the company who makes NurOwn, its board of directors pleading for help. We shared Mike's results far and wide. Mike and I fought for continued access to treatment and to improve an archaic regulatory pathway. He died waiting for change in 2019. Promising therapies are on the horizon for ALS, but most people with the disease won't live to see them. Most patients die within two to five years of diagnosis. These patients need more access to promising therapies and a more flexible regulatory process that would accommodate the moderate advances needed to get closer to a cure.

Tara Collazo

I was diagnosed in April 2019. My ALS diagnosis was earlier in the course of the disease than most, thanks to a highly skilled group of neurologists here in Texas at UT Southwestern with experience diagnosing ALS. Three months later, in July of 2019, I began the screening process for Brainstorm's NurOwn trial in San Francisco. After four months of medical testing, our hopes for a cure were shattered. The slope of my decline was not considered 'fast enough' to qualify - in other words I was not yet sick enough - and I was excluded from the trial. I believe if I were accepted, or if I were able to access the treatment, I would have been among the subset of people that would have done well and improved function. Instead today, I have continued to decline. It is not possible or safe for me to live independently. I am unable to dress, feed or clean myself. My hands have failed, walking is difficult, and my breathing is rapidly declining. I don't want to end up paralyzed from the neck down for years. I want a chance to live.

M. C. Collet

Everyone reading this letter already knows the urgency that ALS demands. ALS has become an orphaned orphan disease. That's the worst. Ever since we lost Mom to ALS in 1997 I've written letters and testified to the FDA and written more letters and visited legislators. We're still an orphan disease. FDA, we want the same FDA that oncology has. They have Project Facilitate to help with Expanded Access. We don't. Why not? You can find 106 enrolling interventional trials for ALS at clinicaltrials.gov. How many Expanded Access Programs are there? Two. And one of them is highly restricted. Just two. Something is not right here. Other diseases have this Expanded Access path that also allows rigorous science to proceed while dying people can get access to therapies. They even have an 800 number to help. We in the world of ALS seem to get bogged down in opaque processes that move at the speed of concrete. NIH, we want the same NIH that other diseases have, too. Sure, we appreciate the grants that the NIH makes toward a patchwork of ALS research. But what about the expertise of the NIH that can make trials smarter and more accessible? What about the knowledge of the NIH on how to do biorepositories better and more cost effectively than anyone else? We need the NIH to become engaged with ALS for more than passing out grant money. Please, we need the NIH that is known for the science that goes on at its headquarters for so many other diseases. Is there any question as

to why those dealing with ALS and their families feel that they have been orphaned? We need for our federal agencies simply to do for ALS what they have done for other diseases. That's all. Thank you.

Paula Corey

My brother was diagnosed with ALS at the age of 35. This is a terrifying diagnosis not only for the patient, but for the whole family. At the time he was diagnosed in 1995, there were two drugs under investigation but he was unable to obtain access to either due to the research protocols. It is amazing to me that in 25 years, this has not changed. My brother passed away in December of 2020 and we often had conversations about access to drugs that could help an ALS patient. He consistently wondered why, since this disease is terminal, why wouldn't you learn from the patients who are willing to take the risk?

Robert Daniels

After being denied access to the Healy platform trials I began taking sodium phenylbutyrate and Tudca. It was costing me $2400.00 every 3 weeks, after appealing to the state insurance commission and the positive article in The NEJM my insurance company decided to cover the sodium phenylbutyrate. This was recommended by my neurologist.

Lindsay Dewis

A few months after I was born, my grandfather Dr. Edwin Grafing Dewis took his life. He had been diagnosed with Amyotrophic Lateral Sclerosis and since he was a physician, he knew what that diagnosis meant. He took his life to spare his family the heartbreak of watching him die in such a dire way. I don't have an ALS treatment story to share for the FDA congressional hearing, but I truly believe that my grandfather taking his life adversely affected my father's life, which in turn adversely affected my life. My Dad was a Princeton graduate, a naval pilot World War II hero, and a successful businessman. After my grandfather took his life my father's world fell apart. He lost everything, died alone and penniless, and as you can imagine this had a profound effect on my life. So you may ask why I am writing this sixty three years after my grandfather took his life ..... well because not much has changed as far as getting ALS patients and their families the help and support needed from the United States government. The government has put a concerted effort into research and finding cures for cancer, aids, heart disease, COVID-19 and the list goes on but have yet to give ALS the attention it deserves. My story is just one of countless other stories of people who have lost family members to this disease. The impact of a lost life in this manner can affect generations. I implore you to make this a healthcare priority.

Jane Dignam

Since 2018 I have watched my son from walking to now in a wheelchair, from a TV announcer voice to a whisper; we've had 5 different diagnoses, continuous spinal taps. Doctors are aware of possible medications available but because of no FDA approval they must try other avenues in case something might work. Our family has spent endless time reading familiar stories trying over the counter medications. To read stories of those able to take experimental medication and it worked, I can't help wondering what's the problem!

Michelle Dupree

Being a volunteer advocate for people with ALS over the past 15 years, I have only known 3 people given the option to try an expanded access program. This should be an option for any people who are disqualified, especially due to the fast proceeding nature of ALS.

Jenny Gore Dwyer

In 2005, my handsome husband Pat was diagnosed with ALS. He was 44. It hit us out of the blue, as most sporadic ALS does. We had no idea what we were in for. You can read about ALS all you want, but until you live the minutes of the days, where your husband cries because he has lost the ability to hug his children, you have NO IDEA how horrible ALS can be. Pat had a slow progression of ALS. He lived with his disease for 8 years. He always said he didn't know if that was a blessing or a curse. A blessing because he had more time on this earth than the standard 2-5 year ALS life expectancy. A curse because he hated every little thing about ALS. He was a commercial fisherman and master diagnostic mechanic. During his fishing career he worked the fierce waters of Alaska. That included moving heavy crab pots, pulling gear, and heaving huge and heavy lines. It took every bit of muscle he had. It is hard physical work. For someone who has worked hard physically all of his life, losing that ability was devastating. It was part of who he was, and without it, he felt he wasn't "Pat" anymore. That's one of the horrible things you never read about when you are googling ALS, after you've been diagnosed. The mental "horrible" of losing your abilities, can be harder than the physical "horrible" of ALS. For Pat, that was definitely true. Because of Pat's slow progression, he didn't qualify for any drug clinical trials. So right from the beginning, he knew there wasn't going to be anything that was going to slow his progression down, stop it, or reverse it. He knew that because there weren't any promising drugs in clinical trials. Even if there had been a "promising" drug discovered the day he was diagnosed, the clinical trial process takes at least 10 years or more. He only lived 8 years with his ALS. Again, because of his slow progression and declining breathing ability, he wouldn't have qualified for the parameters of the clinical trials anyway. EAP might have been a possibility, but we will never know. For Pat, his bottom line of being done with ALS was when he couldn't speak in real time any more. For us, we were lucky in that respect, his speech was one of the last

abilities ALS took from him. When he got to that point, not being able to speak and be understood, he looked around the ALS drug landscape one last time. He realized that there was nothing in the near or even far future that would help him. He realized that it was time and he was done. We scheduled his palliative sedation through Hospice. On a bright sunny Seattle day, 8 years after he was diagnosed with ALS, Pat's undying spirit flew out the doors of our bedroom and to his new place in the universe. The nature of the disease and the incredibly slow pace of research does not bode well for people with ALS. It is a disease where people decide what their bottom line is that they will live with. ALS is a disease where someone will choose to die rather than continue to live with ALS. ALS is a disease where the one of the choices left is being able to donate your brain and spinal cord to ALS research. It is a disease where you look around the landscape and say, yes, the best I can do now is donate my brain and spinal cord to science. To be clear. Pat's choice of treatment for his ALS was to choose to die. In this day and age, how do we still think that is acceptable? Change the process so that choice is at the bottom of the treatment list, instead of the top. There shouldn't have to be those choices in ALS. You shouldn't have to pick a bottom line of what you will live with. People with ALS need to have more than hope for treatments. They need to have better choices than choosing to die and donating their brain and spinal cord. They need to have hope in the form of real viable treatments. Years ago, when I was writing testimony for an ALS FDA hearing in 2013, a very wise friend asked me what I wanted to come out of this hearing. Now, 8 years later, it seems insane that I am repeating what I said then. What do I want the FDA to do? Anything and everything to speed processes up on your end to move ALS treatments through the process. Don't make the decisions based on "the way things are always done." Think of my children and the horrible reality they lived with as they watched their father melt away. Think of my soon to be born granddaughter, who will never ever know her grandfather, and know the look of unconditional love from his twinkling blue eyes. For drug developers: Be creative, do things differently, open up the doors to innovation, be bold in your vision of shaking up clinical trial processes. Design clinical trials that run an Expanded Access arm. But the most important thing you can do is listen to the real stakeholders in ALS, people who live with ALS. Include them in your innovation process. Have people with ALS on your speed dial, or email list, consult with them, work with them, include include include them. People with ALS are your most valuable resource. They are the real, brilliant experts in the field of ALS. They can give you clear practical advice, they can give you details on risk versus benefit, and most importantly they can remind you of the urgency in which things must be done. The longer things take, more people with ALS will have to look around the landscape, realize that things are still moving too slow, and make the choice to die. Be innovative, be creative. Do it now. ALS can't wait.

Jennifer Emerick

My doc put me on riluzole which I think sped up my progression. Doc also wants me to try oral Edaravone. Doc told me absolutely nothing about trials. Reached out a few days ago to a Healey location. I'm hoping to participate.

Jose Fajardo

Living in Hawaii makes it difficult to participate in trials. Hawaii is not a big enough market for trials, so if we manage to be accepted, we have the additional burden of travel expenses. I have also found the system to be overwhelming. I wish there was one database for PALS to enter their information for clinics to find us, or share information one time rather than having to submit it to various clinics. It is time-consuming and very stressful.

Kerry Falzone

My father has bulbar ALS and is on a clinical trial at Columbia University in NYC. He decided to go on this trial because he had hope. He believes in science and that it would help him. Not knowing that within 3 months his symptoms would rapidly decline. While he is on 2 ALS drugs that were approved by the FDA, this trial limits him to taking anything else. He would be disqualified if he took something like TUDCA (a component of the much needed Amylyx). I have been communicating via email with Dr. Janet Woodcock for 6 months about getting people like my dad access to drugs that show promise like Amylyx. After 6 months of persistence I was declined a meeting. If my father is still alive when Amylyx gets to Canada hopefully this year, we will be driving to another country to get access to this drug.

Brian Foderaro

In late 2019, I agreed to travel from northern VA to southern CA 14 times over 12 months just for a 50% chance of receiving NurOwn as part of the phase 3 trial. I was later accepted into the NurOwn expanded access program, but this opportunity was taken away from me earlier this year after the FDA released their inaccurate public statement on the effectiveness of the therapy. Now, I have no options for treatments.

Lisa Garasic

The first doctor told me that there was only one drug that might help me. He said the side effects might not be worth it. Especially because I was still breastfeeding. My son was 10 months old when I was first diagnosed. Second doctor said to start Radicava and stop breastfeeding. I didn't. My third doctor ordered genetic tests done immediately. He knew of several clinical trials involving certain ALS mutations. I was warned that they cross reference white European samples so, they might not come up with any significant findings. Well, they did not find anything of significance. They did however give me hope that my slow progression would give me decades to live. Maybe I'll get to see a treatment that works for me.

Monique Green

Immediately after diagnosis, I participated in 2 drug trials through my ALS clinic. Now, 8 years after being diagnosed, I am no longer eligible for most ALS clinical trials.

Brenda Guevara

I am not afflicted with ALS, by the grace of God. or does any loved one of mine have ALS. So why am I writing? Because of profound empathy for those afflicted. Because I can't turn away. Because the more I hear the facts, the more I want to be involved: 1 in 300 are diagnosed; 90% of those diagnosed are NOT genetically predisposed; veterans have a high incidence of getting this disease. I want those ALS patients and their loved ones to know that they are seen and that they have MANY allies who support their collective efforts. As an American, I want to know that FDA guidelines and restrictions, designed with good intentions - to protect the well being of those taking medications - can be responsive and flexible enough to accommodate those who have a death sentence. If I were to have ALS, knowing there is no cure, I would absolutely be willing to get involved personally in clinical trials, and would be willing to take medications not yet FDA approved. It's a no brainer. Allowing access to those drugs is the right thing to do, and the humane path for people who are living with a cruel affliction. Time is of the essence, and ALS patients have the right to 'put up a fight' against ALS.

Sam Hamilton

I don't have ALS, I have too many friends with it. It started with one, Pat Dwyer, then my advocacy started. I've stood on a bench in Boston begging for money to help find a cure. I've dumped countless buckets of ice on myself and others. I've fought tears when my college daughter called me & told me one of her buddies had been diagnosed. Unfortunately I have talked about ALS and it's truths; she knew it was a death sentence for her 22 year old friend. I spent an evening with a friend, who had ALS and was in a wheelchair, trying to figure out how we could break into a lab to steal drugs for him. I'm a middle aged woman, no history of criminal activity and I was going to commit a burglary. This is how important this is.

Patrick Harmon

Our doctor Roos at U of C tells us NurOwn therapy has not been approved by the FDA. It has a high success rate, why does my husband need to die waiting for approval? I love him, please do something. Please!

Kathy Mulkeen Jezioro

I don’t qualify for access to clinical trials because my symptoms are over 3 years. Takes too long to get diagnosed and even longer for an appointment at the ALS clinic.

Patricia Joyce

My husband was forced to go to South Korea for stem cell treatments. They helped slow his progression, but he was unable to get them here in the US because of his breathing. The devastation and depression of knowing that there was treatment out there, but it wasn't available to him was heart wrenching. Words can't express the despair we felt. He would've signed/agreed to anything to be allowed access to those treatments.

Dave LaForest

I was just diagnosed and have yet to talk to my new ALS doctor. So everything that I have learned has been from google or social media. I was diagnosed on June 23rd and my first meeting with my ALS doctor is Aug 5th. So I have had to wait 6 weeks to actually talk to a doctor about my new ALS diagnosis.

Philip Leavy

New advocate for a friend battling disease.

Manuel Lopez

Found out was too old or too far along since being diagnosed. Was hoping having service related disease would be eligible for something.

Karina Malmquist

I have a family member with ALS.

Scott Matthews

I honestly haven't even attempted to gain access to any treatments, because from everything I've heard and read about the process, it's like banging your head against a brick wall. The FDA seems to be content to let us die.

Thurman Maynard

I was a phase 3 trial participant in the Brainstorm trial. I started the trial in January 2019. May 30th 2019 I received my first injection. I could barely walk before May. July 24th 2019 I received my second injection and noticed I could walk better and had strength in my left hand. I couldn’t open water bottles before and in July I noticed I could again. July 28th 2019 ABC Nightline News showed up at my house to do a story about the treatment I have been doing. They filmed me for the first time in 2-1/2 years able to ride a dirt bike again. I wasn’t able to ride a dirt bike for 2-1/2 years. September 19th 2019 I received my 3rd and final injection for the trial. My breathing

went from 93 to 101. I could open water bottles/Gatorade bottles with my left hand. September 24/25 2019 I walked miles in Washington DC advocating for ALS and the treatment I had been doing. ABC Nightline News has all that footage recorded. I don't know to this day what I received but I'd love to be able to receive it again. Today is July 20th 2021 and it's been almost 2 years since my second injection. Today I'm in better shape than I was in May of 2019. Please consider and help get drugs in bodies now.

Nancy McGee

My mother in law began having odd symptoms in June 2019. In October of 2019 she was diagnosed with ALS. We got very little information from the doctors. We called the closest ALS Association and their first appointment was one year away. We had so many questions and needed info and the best we could do was google. Her experience happened so quickly. By November she lost the ability to walk and she died in December 2019. We were so lost and had nowhere to turn. Nowhere to get info.

Anne McIntosh

After Mark began experiencing symptoms in 2016, it took 15 months to receive a diagnosis of ALS in November 2017. By that time, he was losing function in his limbs so rapidly that he was told he did not qualify for any clinical trials. Like most ALS patients, his progression timeline did not fit neatly into the pattern required for access to clinical trials. Although his progression has slowed since then, sparing his ability to speak at least for now, the path to treatments was effectively never an option for him.

Osiel Mendoza

In 2018, about a year and a half after my diagnosis, I was screened to enroll in the Brainstorm Cell Therapeutics Clinical Trial. When doing the physical assessment and ALS FRS evaluation, I was one point lower than the approved ALS FRS score and was found not eligible to participate in the trial. One point was the reason for me not being able to participate in a promising trial that could have led to me receiving expanded access to the drug that has helped slow down progression significantly for many ALS patients. I was 23 years old at the time. And now, I'm completely paralyzed and on a ventilator just a few years later. One point on a not so very accurate functional rating scale is the reason that I missed out on gaining access to a potential life changing treatment.

Shahriar Minokadeh

Over 90% of the people diagnosed at the same time as me are gone. I was a double board certified pain management anesthesiologist with a thriving practice, but am

now confined to a wheelchair with 24 hour caregivers and a ventilator to survive. My life hangs in the balance every day. My family, friends and I have corresponded with the FDA, pharmaceutical companies, and neurologists countless times. Despite my medical background, knowledge and connections in medicine, I was unable to access a single investigational therapy for 7 years.

Christine Moretti

A few months ago, my ALS neurologist prescribed Amylyx's AMX0035 but I was denied coverage by my insurance, Federal BCBS, because it is not an FDA approved drug. I am already taking TUDCA, which is one of the drugs, a supplement, in this regimen. The other part of AMX0035 is an already approved drug, sodium phenylbutyrate, yet the FDA is requiring another lengthy trial on this drug. Meanwhile, people are dying of ALS. Simply put, this is cruel and heartless. ALS patients deserve better. This drug has proved to extend life. Why are we being denied the opportunity to live longer? We demand better of our government. Please help us. We are desperate. Thank you.

Sandy Morris

I was in the phase 3 NurOwn Brainstorm trial and stabilized for a year. With no ability to access the therapy after the trial, my family and I flew to South Korea 4 times for Corestem (closest thing to NurOwn). When COVID hit, there was no ability to continue stem cell therapy and after only falling 3 ALS-FRS-R points over 14 months, I then fell 10 points over a 7 month period. I am now on a Bipap 24/7 and only have the use of my left hand. It is easier for people living with ALS to schedule their assisted suicide than it is for us to access investigational therapies. This way of thinking is upside-down. We should be allowed to reach for therapies so we can stay here longer. We want to live.

Mike Mulhern

I have participated in 2 trials since getting diagnosed 1 year ago.

Sarah Olsen

At the young age of 29, I received my ALS diagnosis. I was a bodybuilder. I was a police officer. I will always be a fighter! At the time of my diagnosis I was already excluded from nearly every trial because of my low lung function. I have never even had a chance with this disease but I have not let it defeat me. I have traveled to DC to speak with many of my legislators. Traveled to China just to receive some treatment. I have been a part of national TV shows for als. It is time we turn these tragic diagnoses into success stories.

Mick Palmesano

I have experienced a rapid progression of sporadic ALS which crippled me and had me in a wheelchair just weeks after my formal diagnosis in Mar-20. My ALS FRS-R was already too low to participate in any clinical trials available through MGH. I am however on an EAP for IC-14, a drug that will be added to the Healy platform trial at some point. I wish to obtain access to anything and everything possible to recover from this disease. However, with an FRS score of 10 I am being left for dead.

April Patelski

My husband, Brian, was diagnosed with bulbar ALS on 9/11/18, our 19th wedding anniversary. Brian was 47 years old at the time and had always been the picture of health, he ate right, exercised, didn't smoke and had never had any health issues his whole life. He was the kind of person that everyone immediately loved, he was always kind and went out of his way to help anyone that needed it. He loved his job, his friends and our 5 kids that were ages 4-18 at the time of his diagnosis. We immediately started attending clinics at the doctor for people with ALS and he started on a regimen of vitamins and medicines that would hopefully buy him more time than the 2-5 years he was given. Unfortunately every treatment out there is in vain, at this time ALS is 100% fatal. We were made aware of trials for new drugs but it was hard to get selected for them and even if you were selected there was no guarantee that you'd be given the drug and not a placebo. Brian completely lost his ability to speak by January 2019 and by March eating was becoming so difficult that he had to get a feeding tube. What a horrific nightmare we were in, watching him lose his ability to eat and speak, his hands growing weaker and him not being able to dress or shower himself anymore. He was very frustrated with the wait for approval for ALS patients to get experimental and new drugs, there was no need to make ALS patients wait for approval, they have nothing to lose and want to have access to anything that could possibly help them. Sadly Brian lost his short battle with ALS on 5/11/19, exactly 8 months from diagnosis. I am haunted by the torture he had to endure the final months of his life, he and I would've tried anything to try to defeat this monster of a disease. I became a 42 year old widow with 5 kids. This is nothing we ever thought would happen, we had no chance to fight ALS, there is very little known still about the disease and that needs to change. No one should have to feel the pain and nightmare of fighting ALS and the pain of fighting to get proper treatment. Please allow ALS patients access to anything that could potentially help them ASAP, they can not afford the time to wait for approval, again they have nothing to lose by trying a new drug or treatment. Thank you.

Deb Paust

My husband Tim was diagnosed in 2012. There were very few clinical trials at the time but he wanted to get into the mesenchymal stem cell trial at the Mayo Clinic in Rochester MN. He was 257 on the waiting list for the Phase 1 safety trial. Due to the turnstile of death of ALS and FDA inappropriate criteria for clinical trials for terminal diseases, Tim became patient number 3 in the safety trial. This means that over 250

people living with ALS became ineligible or died waiting to get into this trial. Similar to NurOwn, stem cells were injected into his cerebral spinal fluid at the lumbar region. After 7 days Tim had gained significant range of motion, improved function, safety and comfort thus improving his quality of life. This was a safety trial with no option to continue treatment. Tim was grateful for the improved quality of life and felt sure future treatments would have not only extended his life but improved his comfort and quality. Our request for another injection was denied. We were told 'if it was a home run then yes but no increased strength was achieved.' We disagreed as improved quality of life and function was everything to us. Tim required min- mod assist with sit to stand transfers and ambulation with a rolling walker. He improved to stand by assist for both with increased step height of .5 to 1.5' and more than 30 degrees of increased bilateral upper extremity range of motion. Open Label Extension would have given us a better quality of life and possibly more time. Expanded Access in this trial would have given us more data, improved the quality of life and possibly extended the lives for many more inflicted with this brutally cruel death sentence.

Corey Polen

Thank you for taking the time to read my story. It took a considerable amount of time to type with my eyes. In the past very few cared. My limited time is valuable so please generate action. I have requested EAP and RTT access a dozen times with only denials until one day ProjectALS helped me. All those previous denials are never measured by anyone. Everyone only hears about the overly inflated FDA reported success of 99%. That is a typical government spin to avoid accurate figures. The FDA would count me as a success because I was able to receive EAP. What that fails to report was the dozen denials prior. From the day I requested EA to the day of injection was 684 days. That is not a success. Every day I lost more and more of myself. I went from walking to needing 100% support for everything I need. Much of the delay sat square on the shoulders of the FDA. Originally we tried to create a 12 patient EAP, to help others with efficiency. The FDA eventually denied it because 12 patients was too many. Yes, you read that right. EA is broken, and I speak from 684 grueling days of experience.

Jess Rabourn

In 2009, my dad became the second member of my close family to be diagnosed with ALS. My mother faced the grim likelihood of losing both her dad and her husband to the same awful disease. My dad was well informed, and he made clear his interest in turning every stone for possible treatment. But Expanded Access, at the time, was poorly understood by life science companies and healthcare providers, and there was no central organization to build consensus, publish best practices, or organize collaborative sponsorship models for treatment access programs. Now that we have achieved those things, the next step is for Congress to earmark funding for Medicare to recognize investigational medicines under Expanded Access as 'reasonable and necessary' care under its coverage policies. FDA allows 'Cost Recovery' for Expanded Access, but this community needs help in covering those costs for early access.

Corey Rodriguez

My mother died in 2017. She had zero access to Radicava or NurOwn.

Darren Rose

I have been turned down for 3 different trials. Now I am over the 3 year mark so I am just out of options.

Anthony Rosello

My Vietnam War Veteran father was denied all potential trials. Even in going through the VA after unsuccessfully trying to navigate it ourselves, we were denied. My father is one of many I know, and sure of hundreds of not thousands I do not know that were denied. Serve your country and we got your back, until you don't.

Bruce Rosenblum

I participated in the Brainstorm phase 3 clinical trial, and I am currently in EAP at MGH. I consider myself extremely lucky that I live less than 3 miles from MGH and have been able to participate in 2 different trials. However many patients are unable to participate in trials due to travel challenges or not enough trial spots.

Glen Rouse

I was diagnosed with ALS in September, 2020. I had my first ALS clinic visit about one month later. At my first clinic visit I was surprised no one mentioned clinical trials or promising therapies in development until I brought them up. In the discussion I was frustrated because I seemed to know more about current ALS research than my caregivers. I find that unacceptable. Nevertheless I was finally able, working with my original neurologist, to be accepted into a clinical trial. It is very important that ALS patients have access to clinical trials. It is also important that all Phase 3 clinical trials offer Expanded Access Programs to these treatments for patients who can't or don't qualify for trials anymore. I also feel very strongly that Nurown, a promising drug that showed promising results in a subset of ALS patients, should be approved. We don't have all the options cancer has. We have two lousy drugs that do little. We are dying! Nurown and AMX0035 showed promising results in their last Phase 3 trial. Requiring the companies that make these drugs to do another Phase 3 trial is dooming 5,000 ALS patients a year to premature death. How is that right, equitable or just? It is not!

Eugenia Rozenberg

Our friend, Jacob Gamer, was diagnosed with ALS in October 2020 at the age of 65. Before his diagnosis last year, Jacob was a very active husband, father and grandfather. He owned an auto-body repair business and relished spending free time with his two grandkids, Sammy and Ben, who are his pride and joy. ALS relentlessly robbed Jacob of his ability to walk, and he is now losing ability to use his hands and voice. We are devastated to see Jacob's rapid decline and shocked to learn that after 150 years of ALS awareness, there are only 2 approved treatments that may extend life expectancy by 2-3 months. Jacob's family has been working relentlessly to get him admitted into a clinical trial. Unfortunately, he is automatically excluded from most trials just because he had a stage 1 malignancy, which was successfully treated. The exclusion criteria for trials is incredibly stringent. They were finally able to identify a trial for which Jacob meets enrollment criteria: the RAPA 501 therapy for ALS at Hackensack University Medical Center. Unfortunately, due to lack of experience/urgency, enrollment for this trial has been plagued with never-ending delays. Jacob's family is left to wait and pray that he does not progress to a point of disqualification by the time he finally gets called in for a screening. Clearly, the framework for ALS therapy development and approval is broken. Patients are desperate to get into trials, but the vast majority don't qualify. Research done on the remaining fraction of the patient population shows clear benefit to some, but apparently not enough to get additional treatments approved.

Sandra

My Bulbar ALS diagnosis came 3/30/2020, just as Covid-19 shut down all ALS Clinics and Clinical Trials. I had to wait four months just for my first appointment at an ALS Clinic to be seen by a neurologist and ancillary ALS healthcare providers. I was told about the Healey Platform Trial when I was diagnosed, and I wanted to try to get in it. The sites hosting the trial close to me kept delaying opening. I finally reached out to one 1000 miles away, in my hometown. I was able to be seen in late November, 8 months after my diagnosis of a rapidly progressing, fatal disease. I received either placebo or medication that I gave myself daily by an injection in the abdomen. After I completed 6 months in the Healey Trial, I was able to go onto active medication through 'Open Label Extension' that is a part of the trial design. This is an option that VERY few ALS patients ever get. So now my daily injections contain the active medication for certain. But travel is getting more difficult as my symptoms progress and my limitations increase. The only way that I can receive the boxes of active medication is to go to the trial site in person for regular visits.

Bruce Santora

I was diagnosed with ALS in October 2018. I was offered the chance to participate in the Orphazyme clinical trial. At the same time I had the opportunity to get tested for Brainstorm's Nurown trial, and there was a chance of getting on Biogen's C9orf72 trial. Issues impacting my decision were: Location - I live in South Jersey, so my local ALS clinic's are in Philadelphia. Only the Orphazyme trial was available there. Other two

require travel to either Boston or Montreal. Symptom onset - each of the trials had something like an 18 month symptom onset limitation. At the time I started the Orphazyme trial, I was already at 16 months since my reported symptom onset of November 2017. My concern was the uncertainty of being accepted for either of the other two trials any chance that I would be rejected outside of that 18 month window, and that would disqualify me for the Orphazyme trial as well. Rolling the dice, I chose the most likely and certain opportunity which was the Orphazyme trial. In phase 1, which lasted 18 months, I was unsure whether I was getting the drug or a placebo. During this period, I progressed from walking with a cane, to requiring the wheelchair for most activities. I had no issues with breathing or speaking as that remained unaffected. After the phase 1 trial was completed, I participated in the phase 2 trial. This lasted until May 2021, and the trial was abruptly canceled as results showed no benefit from the drug. At this point I had progressed to requiring assistance with everyday activities such as transferring from bed to chair, bathing, dressing, eating, etc. As I write this today, I am trying to find new trials to participate in but now I am nearly 4 years since symptom onset. As I watch the news, regarding early access programs being debated in Congress and challenged at the FDA, I can't help but be disheartened by the lack of empathy from people who have not had to deal with the horrors brought on by this disease. If they or a loved one were afflicted with ALS, they would truly understand the pain and suffering that PALS and their families go through as they watch us slowly lose all abilities.

Matthew Schotte

Hello members of Congress and associated members of the FDA. My name is Matthew Schotte, currently residing in Cape Coral, Florida and I am speaking on behalf of 30,000 Americans with ALS. On May 24th 2019, not only my life, but the life of my family and friends, became defined by 3 letters, A-L-S. These 3 letters changed my life completely. ALS robbed me of my ability to do simple daily tasks and now this wheelchair is a part of me. It can happen to anyone. I was given 2 to 3 years to live and as I pass 2 years, I am not done fighting for a change. All this, while losing my ability to speak and breathe. I'm trying to stay positive, but in all honesty, I get very frustrated when I think about all the time and money spent by ALS patients seeking a potential treatment. We are spending our life savings and precious time traveling the world, searching for some hope to battle this disease. There are several drugs and/or treatments in existence, but due to the strict FDA guidelines, they will not be available for ten or more years to those struggling with ALS. We have a promising drug/treatment on the horizon called Nurown that completed a phase 3 trial but was cited as failed. How is a drug/treatment that helped one out of three people considered a failure? This should be considered a monumental success. Out of all the drugs approved by you, the FDA, none of them are worth fighting for, except Nurown. As a proud American, I feel as though the system is broken. We should be leading the world with treatments and drugs to combat ALS. We should be fighting ALS for the benefit of humanity, not for profits. I choose to fight for not only me, but for future patients to have hope while battling ALS. I believe if there is a drug or treatment that

would slow, stop, or reverse the progression of ALS on just one person, it would be worth allowing public access to try. Isn't that the humane thing to do? Speaking for myself and the thousands of ALS patients, knowing Nurown would give us a 1 in 3 chance to slow, stop, or even reverse the progression of this disease, we would gladly take those odds. Wouldn't you do the same? We don't have time to wait. If we don't act now, Lou Gehrig's Disease will claim the lives of thousands more. At this point I would like to thank you for your time and ask you to please reconsider allowing Nurown to enter a phase 4 post marketing study. The 3 letters that you represent, F-D-A, are the ones that could change the future of my 3 letters, A-L-S and benefit our 3 letters, U-S-A. Thank you again. Respectfully, Matthew Schotte

Garrett Smith

I'm a Veteran diagnosed with bulbar ALS June 2017. I was denied being in a clinical trial as my breathing score dropped by 1% below the qualifying percentage rate. With having ALS, your breathing rates will change daily which is completely unfair to be denied with no exceptions. The clinical trial I was denied has now been shown to slow progression.

Jonathan Stewart

I sent out multiple emails to sites in the HEALEY Platform. I go for a screening on August 5th at Temple University in Philadelphia. It will be costly as Philadelphia is 5.5 hours away and I will need to stay one night plus pay tolls on the PA Turnpike which are ridiculously high.

Linda Tolisano

I was a healthy individual working full-time, eating well, and exercising. Or so I thought I was healthy and doing all the right things. I was diagnosed with ALS in 2018 after having seen many doctors with multiple diagnoses. I was in shock when I was told I had ALS and am on the two current standard of care drugs. Expanded access is my only hope as I can no longer get into any clinical trials.

William Walton

I am a retired civil servant with 38 years. I worked for the Dept of Defense since I was 17 years old and retired at 55 from the Coastal Systems Station Panama City, Fl. I traveled all around the US and foreign countries doing work on mine sweepers and other important equipment for the Navy. I had all the same shots as the military had but am not entitled to any VA benefits because I was Civilian Navy. A year after I retired which was July 2018 I noticed weakness in hands and legs. I went to my family internist and he suspected ALS. He sent me to a neurologist and Oct 15, 2019 I called my parents in MD with my diagnosis. Being as I had never married, my parents knew all my insurance info. My mom immediately called Mayo in Jacksonville to get a

second opinion. By my Mama Bear and the Grace of God we were able to get an appt a month later where they confirmed the diagnosis. They would not put me in stem cell study as I had Learning Disabilities in school. I was approved by Medicare. Medicare and Blue Cross would not pay for the lift part of electric chair. Recently due to inability to get on and off of the toilet and wipe myself I had to purchase a lift toilet seat and bidet. Medicare and Blue Cross do not cover anything to do with lift. The lift toilet seat with bidet was $2,300 which is what my monthly disability check is. I can no longer shower myself and have a friend that comes to shower me. Not ready for my Mom who is almost 80 shower me. My parents who are retired Dept of Defense civilian Navy employees had to move from MD to take care of me. My father has built ramps for my door, refit the bathroom after Hurricane Michael to make it accessible with a rollator in the shower. My parents bought a handicap van so they can transport me to Mayo in Jax (5 hour from my home outside of Panama City) to local Dr appts., etc. I don't know what I would do if I didn't have my parents to take care of me as I couldn't afford around the clock care.