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## We are Drs. Jeremy Shefner and Seward Rutkove here to answer your questions about bringing research into the homes of ALS patients!

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### CORRESPONDENCE:

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God bless you and the work you are doing. My brother died from this disease at the age of 30 in 2008. Anything that can be done to help in finding treatment options, and eventually a cure, is worth doing. Keep up the great work!

[bradrodstrom](#)

Thanks for sharing. (SR)

Thanks for doing this.

I was only 16 when my father was diagnosed with ALS and the memories of the following year are forever burned into me. It started with a backache that was lingering. A few weeks later he went to the doctor to get it checked out but by then everyone had noticed his voice was changing too.

My parents were separated at the time and one cold fall day without any notice my mother drove me there parked the car and told me to stay inside. 15 minutes later she came back outside with her face covered in tears and said I needed to go in and talk to my dad. He could barely choke out the words through the tears. Eventually he just said "I'm dying". It was so strange at first watching a man who was never the most emotional become so overwhelmed with sadness. He was a science teacher and was already well familiar with all that was about to happen to him. My brother had agreed to set up home hospice care in their house and we were going to take care of him the best we could as a family until the end.

The next months screamed by. I remember being so angry in high school knowing that while my friends and classmates concerns were things like homework and the girls they thought were cute, mine were understanding how to use the feeding tube or worrying which seemingly unexciting physical ability he would lose next. I tried to be able to laugh at the fact that I was now changing diapers on the man who changed mine, but everything felt so horrible back then.

By the end of winter he couldn't leave bed, he could barely speak. We were watching someone become a prisoner in their own body. We tried to make the most of it and spend the little time we had together but it felt like everyday something more was taken from him.

Months and months later he finally asked us all together to say he couldn't stand it any more. It hurt too much to go on so he was asking the nurses to put him in a morphine coma and pull the feeding tube. We said our goodbyes and spent the last 2 weeks of his life destroyed by the splash damage of this awful disease. When the call finally came in that he was about to go, I stood next to him holding his hand and could see the tiny flicker of light left in his eyes go out once and for all.

Several years later the ice bucket challenge became a thing and I remember being so happy that this awful disease would finally get some extra research funding. Those feelings dried up quite a bit as people started complaining about the trend or arguing that it didn't deserve the money because there were other more common diseases out there. In the time since then I've lost several other family members to this disease.

I guess my question would be having seen/heard of some off label use of medicines like Zolpidem (Ambien) in people with neurological ailments that some patients actually temporarily regain some functionality as a side effect of the medicine increases activity/shifts functions in the brain. Has there been any promising results like that with ALS or is the neurological damage too widespread? I've also heard there are suspicions that there may be an environmental factor as well as a genetic one as there's a high prevalence of the disease near certain waterways and lakes. Is there any evidence to back this up or is just speculation?

As hard as it was losing a loved one watching the whole process was honestly worse. There are lots of deadly illnesses out there but this one is particularly nefarious to me as it strips all of the joy out of your life along the way. Sorry for the small novel here, but thanks again for what you do from all the people who've had experiences like mine.

[kingrichard336](#)

Thanks for your note; we know that ALS is tough from all perspectives. Our study is focused on finding ways to more efficiently perform trials, and to empower patients to be part of important clinical research even if they are not close to a study center. We are not aware of particular off label uses of drugs at this time.

My grandmother passed away early 2016.

First of all I would like to thank you for your efforts in helping to fight ALS. My question though is how much has really progressed in the research of ALS? Have there been any breakthroughs recently?

My father knows much more about ALS since my grandmother stayed with him once she was diagnosed.

Is there something we should be more aware of about ALS that isn't really talked about much?

[ShadowCory1101](#)

There has been a great deal of progress in ALS research, but much of it is on the basic science side. It's hard to say there have been any true "breakthroughs," but there is a new FDA-approved medication (Radicava). So things are advancing....slowly but surely. (SR)

Hi dr shefner and rutkove,

My aunt has been diagnosed with als for over a year and it progressed rather fast. We live in Canada and she has recently chose to begin the process of ending her life. My question is, what can I do to help set her at ease? She isn't able to communicate and has a full time caregiver. I don't see her that often but as I don't have any experience with people in her condition I am curious to know from you

experts what people like my aunt are going through and how her family can help make her comfortable and let her know we support her in other ways than just talking at her?

Thank you.

[hashtag\\_fart](#)

Hello, and thanks for the note. This is a terribly hard process. As physicians, I think we sometimes know less about what people go through at home than family and caregivers. Just being there, being attentive, and trying to understand her needs may be the best you can do.

Hello,

I have what may be a strange question regarding ALS. I have a friend who was diagnosed in 2002 with ALS. He's still alive 16 years later. He attributes his longevity to the use of marijuana. A treatment he didn't start until after he was diagnosed. He still has the ability to speak and move his head and his fingers... Of course with very limited mobility.

Clearly he isn't the first person with ALS to outlive the short 5 year prognosis (Stephen Hawking comes to mind.) but what do you think it is that almost flips the switch on the disease after a certain period of time? And do you believe drugs like Marijuana do have an effect on ALS prognosis?

Such a terrible terrible disease... So thankful for people like you who use their knowledge to find treatment for ALS patients.

I would be happy to get you in touch with my friend if that is something you guys even do? He is still able to communicate, especially by way of text... He's very active on social media haha. He would love to help in any way I'm sure.

[skinnymidwest](#)

For most people, ALS advances at given rate--for some people it is fast and for others, fortunately, it can be slow. It is hard to say whether a given drug like marijuana is having a positive effect in just one person. But it is also interesting to know of these connections.

It would be great to have your friend participate, but he's actually outside the window for us to be able to enroll him in our study. (SR)

Hello Drs. How do you ensure the data you are collecting remotely was done so correctly/in line with the study protocol? I see that you are using a number of consumer-medical devices for this study, which I assume helps with usability.

[MattD](#)

Hi, and thanks for the question, which is a great one. This is in fact one of our main questions- we will see from the reliability of the data sent to us how much trouble people are having using these devices. Simply by comparing our results to those obtained in traditional clinical trials should help understand the variability as well. (JS)

I lost my grandfather in 1988 to ALS when very little was known about it, or most people even knew what it was.

I was quite young but my mother and grandmother tell me there really wasn't much treatment at all for

him, just a machine to help him breathe at night.

How much change has there been since then in regards to treatment for people suffering with ALS?

[RodneyRuxin18](#)

Though no miracle drugs have been discovered, there has been lots of progress. Multidisciplinary ALS clinics are important to maintain function and quality of life, riluzole and radicava both slow progression, and we use a variety of other treatments to address specific symptoms. Life expectancy has increased, and I hope quality of life has as well. However, there is much to be done, and we hope our study will help new drugs to be tested more quickly and efficiently. (JS)

Are you seeing a change in the incidence of comorbidity with FTD or other dementia-related diagnoses? How are you measuring these kinds of cognitive disabilities that may arise with ALS but aren't often talked about?

[robbiedenali](#)

It is now more clear that some ALS patients have abnormalities in thinking, with some people having frank dementia of the FTD type, as you note. The more closely we assess mental status, the more abnormalities we find. However, it remains true that most ALS patients remain in control of their decision making abilities throughout the disease. (JS)

Is ALS similar to MS in terms of our understanding of etiology? Is there suspicion or discussion in ALS research community that it may not be a single condition, but rather may belong to the same group of poly neuropathic diseases as MS, CMT etc?

Is there sufficient variability in symptoms progression and treatment outcomes to warrant this conclusion?

Apologies if this is a simple question but my quick scan of the inter webs on this issue turned up what appears to be a lack of a disease vector.

[manic\\_panic](#)

Hi-- ALS is different in underlying cause than MS, although some MS treatments are being studied. We don't really know whether ALS is truly one disease-- this is a very important question. There is certainly great variability in how rapidly people progress with the disease. (JS)

I've read articles about water quality and algae bloom possibly being a factor in ALS. Do you notice you visit more patients in areas with a closer proximity of water?

[rya556](#)

Yes, these factors may be related to ALS, but we don't actually know. There are a number of people studying this, but the jury is definitely still out (SR).

Are you doing any research involving cannabis? I see stories of people regaining function and living for decades longer than expected. It would be great to hear more research about cannabis oil for ALS.

[thats-what-we-need](#)

We are not doing any specific research into cannabis. There are many stories out there about cannabis and its potential therapeutic benefit. There is no question some people with ALS do feel better, but whether or not it slows progression remains to be determined. I think it will continue to be a topic of study and interest for a long time to come (SR).

This might seem a bit basic, but what does ALS actually do to a person?

[Bomb787](#)

ALS is a disease of the motor system, making people progressively weaker in their arms, legs, face, and breathing muscles. Some people have modest changes in thinking as well. (JS)

Hi Dr. Shefner & Rutkove, Thanks for doing this AMA. My father died of ALS in June of 2015 with only a year long duration of the disease. It's uplifting to hear about all the different kinds of ways great people like you are trying to research this terrible disease.

I have two questions: -As a child of someone who had ALS, would I be able to provide and kind of tests results you would need for this study? I have a very small chance of getting the type he had, but was wondering if research on and ALS patients' descendants could help anyway

-What made you want to pursue ALS research?

Thanks again!

[Delpy294](#)

So sorry to hear about your dad.

As for your questions, if you are interested in participating in our study as a healthy control, that would be great! Please go to our website: [als-at-home.org](http://als-at-home.org) and join! There is nothing specific about your being a family member of someone with ALS relevant to our study, but we need more healthy people with which to compare the patients with ALS. Thanks so much. (SR)

I'm interested to hear some about how much this "at home" approach helps to reduce study attrition. Do you think this approach could be generalized to other populations as well? Do you suspect you will have to deal with noisier data because of the self collection?

[divvyflax](#)

Hi-- this is an important question; we don't know yet. Our hopes are that frequent data collection will reduce variability of our outcome measures allowing for shorter and smaller trials. Given that many patients drop out of trials because travel gets too difficult, we hope that at home measurement will reduce attrition. We will see.

Any extra noise should be more than cancelled out by frequent data collection, but again, we will see.

I think this idea is relevant to many other conditions; there are studies collecting data at home in Parkinsons disease and others as well. (JS)

What are your thoughts on the visuALS technology created by a group of college engineering students?

[DawsonHelms](#)

I think it is great that people are trying to come up with better approaches for helping people with ALS communicate more effectively. I can't comment specifically on this technology, but kudos to them for working on this! (SR)

My uncle died at 48 from ALS. He was a big football running back thru highschool and college, getting a lot of carries every game. Is there evidence brain trauma connects ALS? What about marijuana in helping with the effects?

[hockey44456](#)

This is a hot research area, but currently most scientists think that chronic traumatic encephalopathy, the condition seen in football players and other athletes, is different than ALS. Some studies have suggested that physical trauma may be a risk for ALS, but this is not certain. Marijuana has not been shown to affect disease progression; clearly the effects of the drug on mental state are pleasurable to some people. (js)

Why has Steven Hawking lived with ALS for so long but other people getting diagnosed only have a few years of life expectancy? I had a friend recently pass from ALS that seemed to be so sudden, maybe 5 years from diagnosis and symptoms to death.

[KaptainKrondre](#)

As I mentioned in an earlier post, some people just have a more rapid disease course and others a slower one. In Steven Hawking's case, he's had a very slow one...fortunately for him and for science! (SR).

Have you looked into the ALS and Chronic Lyme connection?

[liketosee](#)

Hi- this has been looked at extensively. Lyme disease does not cause ALS. While Lyme can cause some symptoms similar to ALS, this never occurs in isolation, so that an experienced ALS clinician should be able to tell the difference. (JS)

The ice bucket challenge was a huge hit in regards to raising money for ALS. How has this money impacted ALS research? Have these donations enabled researchers to come closer to understanding why ALS occurs? Has this challenge brought us any closer to more effective treatments? Do ALS patients receive funds to assist with home care, purchase equipment they might need, and make their homes ALS friendly?

[Goldie\\_1738](#)

The Ice Bucket Challenge was indeed a huge hit and incredibly important for raising money for ALS research. It has funded a number of investigations and I do believe has helped advance the field. As far as I know, the money did not go to assist patients, but rather was earmarked for research purposes. (SR)

I live in an area where there is a neurocare center (residential and outpatient) for people with ALS and Huntington's Disease. I feel like the services they provide could greatly improve the lives of those in Multiple Sclerosis community. Is there any hope for Multiple Sclerosis and it's degenerative properties to be recognized in the same light as ALS?

[pug\\_mom](#)

We are not MS experts. However, there is a large MS physician community, and active MS SOciety, and clinics that are certified as comprehensive MS centers, just as is the case for ALS. They are different diseases, however, with different research communities and, for the most part, different treatments. (js)

Hello Doctor Shefner and Rutkove.

My question is, realistically, what do you hope to acheive with your data, and how do you think it could benefit a patient?

Secondly, my grandmother was young when she was diagnosed with ALS and when it started getting really bad she refused to use her feeding tube. She actually chose to die from starvation rather suffering the disease any longer. I truly believe that neurological diseases like ALS and Huntington's deserve more funding and more attention. Do you have any recommendations on where to donate, and who needs it most?

[pnumonicstalagmite](#)

That's a great question. The study itself is not going to benefit someone who is participating in it. But the data we are collecting will give us new insights into short-term fluctuations in the disease course and also the possibility of doing data collection at home in future studies. This means potentially studying more drugs, more quickly, more conveniently, and more economically.

As for where to donate, there are lots of good causes, but I would generally say the ALS Association is a good bet. (SR).

What are your opinions on the ALS ice bucket challenge, and any impact it had upon ALS and research performed on it?

[InnaruFurry](#)

The Ice Bucket challenge brought in more than \$100,000,000, and has stimulated much new research. Research is expensive, and there are more good ideas than funds, so all help is important. (JS)

Can you comment on ketogenic diets and ALS?

[hazeFL](#)

I don't believe there is any data that supports it has any effect in the disease. It is an interesting idea, however. What is clear is that eating more is generally a good bet, regardless of the specifics of what you eat (SR).

What's your opinion on BMAA being the cause of ALS?

[Animum\\_Rege](#)

This is an active area of research; I think it is clear that BMAA is not THE cause of ALS, but it may be a risk factor. At this point, it is hard to say more. (JS)

Can people still be admitted to your study? I have a science background myself and my mom was diagnosed in may.

Thank you for looking into this disease. It's likely too late for my mom for any research to make any difference for her, but this disease is such a shit diagnosis, I'm grateful for anyone fighting it.

[Girafferra](#)

YES!!! We are recruiting both patients and normal volunteers, so I encourage you, your family, and anyone else to get involved. The website is ALS-AT-HOME.org

Thanks for your interest. (JS)

Is ALS really a disease, or is it more of a common set of symptoms that result when motor neurons die off (for some reason)? It seems like anything that results in motor neuron death would result in "ALS symptoms".

I ask this knowing that there is both sporadic and genetic variations of ALS, and very different progression speeds.

[DepecheModFlanders](#)

That's a terrific question. ALS probably represents a "final common pathway" for a group of disorders--so I agree with your take. It is possible that several different problems all cause a similar loss of motor neurons that we call ALS.

Hi there. My brother has ALS and recently began Radicava. Can you tell me if/how this treatment figures into your research?

[RazzleThemAll](#)

Hi. This treatment does not particularly figure into the research that we are doing. But we will probably want to know if people are starting it as it could potentially affect our results. (SR)

As a member of a family that has been greatly impacted by ALS (multiple diagnosis) I am very grateful for people like you. Some questions:

What made you decide to focus on ALS in particular?

Are there any interesting and or exciting treatments coming in that are showing hope and promise?

What are the best organizations, in your opinions, for donations to help with ALS, now that more continue to appear?

I don't really have a lot of great questions at the moment just extremely glad for people like you. I will try to come up with more intelligent and meaningful questions before your return.



[Oh\\_lion-o](#)

Thanks for the note. I became passionate about ALS and ALS patients as a trainee, when I met my first ALS patient. The gravity of the disease and lack of treatment were both compelling reasons. There are many great organizations, but the two with the most clinical and research resources are the ALS Association and the MDA. ALS Finding a Cure is funding our study as well as other great work. (JS)