












National Ataxia Foundation
COMMUNITY RESPONSE:
FDA Review of Troriluzole NDA

NAF
National Ataxia Foundation

SEPTEMBER 2023

Our Mission

To accelerate the development of treatments and a cure while working to improve the lives of those living with Ataxia.

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Contents

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Spinocerebellar Ataxia (SCA)

Spinocerebellar Ataxia (SCA) is a group of progressive neurodegenerative diseases with no approved FDA treatment and multiple subtypes. Spinocerebellar Ataxia Type 3 (SCA3) is the most common subtype, representing 20–50% of the SCA population worldwide.

SCA3 or Machado-Joseph Disease (MJD) is caused by an autosomal dominantly inherited genetic mutation that is passed on from parents to their children.

In SCA3, the impairment of nerve cells and nerve fibers causes degeneration of the cerebellum (the coordination center of the brain) and related brain regions.

Impaired balance is usually the first symptom of SCA3, followed later by vision impairments, incoordination of the hands, and slurring of speech.

As the disease progresses, it is common to experience spasticity, rigidity, loss of muscle bulk and strength, and slowness of movement.

SCA3 is characterized by progressive deterioration and an intensification of symptoms over time. Disease characteristics vary greatly between individuals and between juvenile and adult-onset forms of the disease.

SCA3 has a heartbreaking prognosis as symptoms eventually become very severe, profoundly impacting the lives of individuals affected as well as their families and caregivers.

Caregivers and family members of individuals affected with SCA3 are forced to live with the fear and anticipatory grief of watching the slow decline of loved ones.

The psychological burden of SCA greatly impacts functional status and quality of life, with one study finding that 65% of SCA3 patients experienced suicidal ideation.

SCA3 is ultimately fatal with death typically occurring 10–15 years after symptom onset.

SCA3 is characterized by a massive unmet treatment need. There is currently no curative, disease altering, or neuroprotective treatment for SCA3.

See Appendix D for sources.

NAF Community Response Letter to FDA

Andrew Rosen
Executive Director
National Ataxia Foundation

September 14, 2023

Emily Freilich, MD, Director
Division of Neurology 1
Teresa Buracchio, MD, Director
Office of Neuroscience
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993-0002

Dear Drs. Freilich and Buracchio,

Spinocerebellar Ataxia Type 3 (SCA3) is a rare neurodegenerative disease with only ~5,000 people affected in the United States. The condition irreversibly and uniformly deprives people of the capacity to carry out everyday activities like walking and other activities on their feet, and eventually talking and swallowing. There are no approved treatments to slow or stop progression, meaning that people with SCA3 have a serious unmet medical need as they face the possibility of a permanent disability and premature death because of their condition. Today we ask that you reconsider the decision to review the NDA for troriluzole.

It has been thirty years since the first specific genetic mutation was discovered for SCA, yet our community continues to wait for the first treatment to be available. **People with SCA need a treatment — anything that has the potential to even incrementally slow the relentless progression of this condition or lessen symptoms.** We heard this loud and clear at our September 2020 Externally-Led Patient-Focused Drug Development Meeting on Ataxia (the Voice of the Patient report can be found here: https://www.ataxia.org/ataxiapfdd/).

We ask that the FDA consider these perspectives from those who, today, have the most to lose by having a potentially effective treatment be sidelined prematurely. Consider their serious unmet needs. Consider the irreversible progression that they will experience over the several years it would take to conduct another study and reapply to the FDA. Consider that people with SCA3 live with absolute certainty about their future, and so are willing to accept greater uncertainty when it comes to new drug treatments.

People with SCA3 deserve to have the full data on troriluzole reviewed, and with this patient input in mind. **NAF is concerned that the FDA's decision to Refuse to File the application may amount to a false negative conclusion, denying patients access to a drug that works.** Only a full review of the data would give us confidence that this is not the case.

NAF believes that FDA regulations and policies, as well as previous reviews of other drugs for rare diseases, support reviewing the troriluzole NDA.

It is well-established in FDA regulation and policy that rare diseases face unique challenges in drug development that make it harder to design and conduct clinical trials that definitely confirm a drug benefit. Some of these common challenges are present in SCA3: (1) there is a limited patient population who can enroll in clinical trials; (2) there is often a slow progression of the disease which makes it harder to show a slowing of progression; and (3) there is a lack of well-established clinical endpoints or biomarkers, adding variability or noise to the assessments. The presence

of these challenges demands a more flexible approach. Rare diseases must be considered differently than more prevalent conditions.

Especially when combined with the serious unmet medical need, **diseases like SCA3 deserve the maximum regulatory flexibility in the review of data from clinical trials.**

FDA's own policies say so. FDA's 2019 *Substantial Evidence of Effectiveness Draft Guidance* says that "certain situations, such as when a disease is rare **or** the disease is life-threatening **or** severely debilitating with an unmet medical need, may warrant additional flexibility." SCA3 certainly meets **all three** of these criteria.

There is ample precedent set by the FDA for reviewing applications for drugs that did not meet their primary clinical endpoint. Most recently, in April of this year, FDA approved a drug, tofersen, for a type of amyotrophic lateral sclerosis (ALS), even though the primary endpoint did not come close to being achieved. Yet, in this case, FDA chose to review the NDA, during which they considered other endpoints and subgroup analyses that ultimately supported approval.

We are asking for the same consideration in regulatory flexibility that has been given to other rare neurodegenerative and genetic conditions, such as ALS, Duchenne muscular dystrophy (DMD), Batten disease, and mucopolysaccharidoses (MPS).

The SCA3 community's need for a treatment is both urgent and great, so careful consideration of the totality of the data, taking into account patient experiences and preferences, is deserved. We urge the FDA to apply appropriate regulatory flexibility and to accept and review the NDA for troriluzole.

NAF gathered input from the Ataxia community to ensure that the FDA has the opportunity to hear the voice of the patient when considering an NDA for a potential treatment for SCA3. **Nearly 3,000 individuals stepped forward to make their voices heard by supporting this request.** This packet contains their feedback, including more than 1,200 comments that our community wanted to share directly with the FDA review team (see Appendix B). Here are several quotes that are representative of the community's response:

> "As a patient who suffers with this debilitating disease and knowing the hopeful findings for possible help from "troriluzole", I plead for the FDA to review the application for use of this drug along with regulatory flexibility to give a chance for some relief from this devastating disorder. Thank you so much for your consideration."
>
> "As a spouse to someone with SCA3, this issue is very personal. It is imperative that the FDA at least review the application so that the potential treatment can be explored and the conversation can continue."
>
> "My son is currently living with SCA3. His father passed away at the age of 50 with SCA3. It's a helpless feeling as a parent to realize there is nothing you can do to prevent or help the rapidly developing symptoms of this disease. Please reconsider your decision regarding the application for troriluzole as a potential treatment for SCA3. Thank you."
>
> "As someone who has been living with Ataxia with no treatment available, I would like to strongly urge you to review this drug because researchers have dedicated their entire lives to find treatments."

In addition, members of NAF's Medical and Research Advisory Board, a consortium of world-leading Ataxia clinicians and researchers, urge the FDA to review the full clinical data from the trial. This packet includes a letter from them (see Appendix A).

Thank you for taking the time to consider your position at this seminal moment in Ataxia drug development. The National Ataxia Foundation offers its SCA patient and caregiver community to the FDA as a resource in consideration of these issues, as well as its world-leading Ataxia clinicians and researchers, should the FDA seek expert consultation. We also welcome a meeting to discuss these issues, including patients' needs and preferences.

Sincerely,

Andrew Rosen
Executive Director
National Ataxia Foundation

CC: Patrizia Cavazzoni, MD, Director, Center for Drug Evaluation and Research; Peter Stein, MD, Director, Office of New Drugs

"As someone who has been living with Ataxia with no treatment available, I would like to strongly urge you to review this drug because researchers have dedicated their entire lives to find treatments."

— Denise H.

Summary of Community Feedback

3,137 RESPONSES

SURVEY QUESTION 2

Affiliation with the Ataxia Community*

*1% Other

37%
Living with Ataxia

31%
Parent or family member

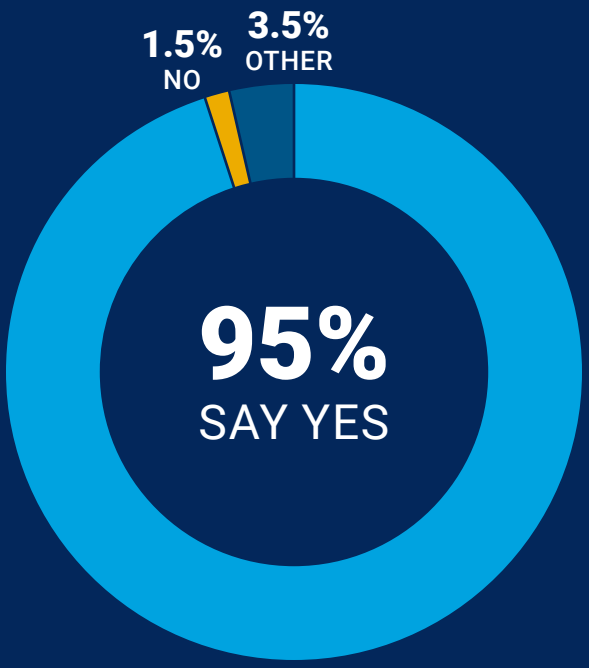
24%
Friend of community, advocate, or volunteer

3%
Healthcare provider

2%
Caregiver

1%
Researcher/ scientist

1%
Advocacy organization

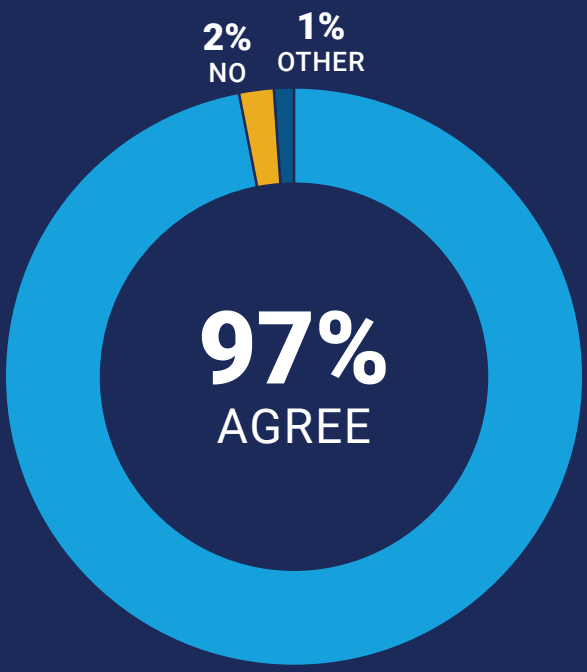


SURVEY QUESTION 3

Would like the option (for themselves or a loved one) to try a drug even though it may have a somewhat reduced chance of benefiting them.

SURVEY QUESTION 4

Agree that the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review.



RESPONSES FROM 47 STATES

Alaska	Hawaii	Massachusetts	New York	Texas
Alabama	Idaho	Michigan	North Carolina	Utah
Arizona	Illinois	Minnesota	North Dakota	Vermont
California	Indiana	Mississippi	Ohio	Virginia
Colorado	Iowa	Missouri	Oklahoma	Washington
Connecticut	Kansas	Nebraska	Oregon	West Virginia
Delaware	Kentucky	Nevada	Pennsylvania	Wisconsin
District of Columbia	Louisiana	New Hampshire	South Carolina	
Florida	Maine	New Jersey	South Dakota	
Georgia	Maryland	New Mexico	Tennessee	

RESPONSES FROM 52 COUNTRIES

Albania	Costa Rica	Ireland	Peru	Sweden
Angola	Croatia	Israel	Puerto Rico	Switzerland
Argentina	Czechia	Italy	Malaysia	Taiwan
Australia	Denmark	Japan	Mexico	Turkey
Belgium	Dominican Republic	Lebanon	Philippines	Uganda
Bermuda	France	Netherlands	Portugal	United Kingdom
Brazil	Germany	New Zealand	Qatar	United States
Canada	Ghana	Nigeria	Somalia	Uruguay
Chile	Greece	Norway	South Africa	Venezuela
China	Guernsey	Panama	Spain	Yemen
Colombia	India			

Appendices

APPENDIX A:

Letter from NAF's Medical and Research Advisory Board

Jeremy D. Schmahmann, MD
Professor of Neurology
Harvard Medical School

September 1, 2023

Dr. Emily Freilich, Director
Division of Neurology 1
Dr. Teresa Buracchio, Director
Office of Neuroscience
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993-0002

Dear Drs. Freilich and Buracchio,

We, the members of the National Ataxia Foundation's Medical and Research Advisory Board, have had the opportunity to review data from the Phase 3 48-week, double-blind, placebo-controlled study (Study BHV-4157) that was provided in Biohaven's New Drug Application (NDA) for troriluzole. Spinocerebellar Ataxia Type 3 is a devastating, irreversibly progressive rare disease with large unmet medical need and no current treatments available. After careful consideration and discussion amongst leading ataxia researchers and clinicians, we strongly encourage the FDA to reconsider its position and accept the NDA for thorough review.

Sincerely,

Jeremy D. Schmahmann, MD, FAAN, FANA, FANPA
Professor of Neurology, Harvard Medical School
Founding Director, Massachusetts General Hospital Ataxia Center
Director, Laboratory for Neuroanatomy and Cerebellar Neurobiology
Cognitive Behavioral Neurology Unit
Senior Clinical Neurologist
Department of Neurology, Massachusetts General Hospital

Matthew R. Burns, MD, PhD
University of Florida

Sheng-Han Kuo, MD
Columbia University

Gülin Öz, PhD
University of Minnesota

Susan Perlman, MD
UCLA Medical Center

Laura P.W. Ranum, PhD
University of Florida

Liana S. Rosenthall, MD, PhD
Johns Hopkins University

Lawrence J. Schut, MD
University of Minnesota

Vikram Shakkottai, MD, PhD

George Wilmot, MD, PhD
Emory University

Robert B. Wilson, MD, PhD
University of Pennsylvania

APPENDIX B:

Community Comments to FDA

NAME	COMMENT
Deborah G.	As a clinical study manager and also a family member of someone living with SCA I understand both the need for high data standards for pivotal trials but also the immense need of therapeutics for people living with rare diseases. The refusal to review by FDA was crushing news for my family. We felt like the door was shut in our face with little to no regard for the lives being impacted by this disease. Patients, at the very least, should be given the choice to make informed decisions on available therapies. By refusing to review the NDA, the FDA is essentially stripping patients of the only hope they have and option for potential treatment. As a member of the SCA community we have every right to demand a thorough review of the NDA. After all it is our loved one's life that is on the line here. It was even more frustrating because of the excellent safety profile of troriluzole. There should be pathways for flexible reviews especially for drugs that have good safety profiles and pose very little to no risk to patients. Even if the proven efficacy thus far does not meet FDA standards the data so far is promising and patients should not have to suffer while waiting for additional data collections. Time is the one thing patients with SCA don't have. Refusing to review this NDA is stealing precious time from patients and their family. I urge FDA to review the NDA and allow for a flexible pathway to approval that both maintains the integrity of the drug application process and puts patients' health and rights at the forefront.
Adrian H.	I have a son with Friedreich's ataxia. As there is so little that can alleviate or help with all Ataxias, it is important that the fullest consideration is given to all drugs that may help before any clinical conclusion is reached.
Melissa S.	I personally participate in trials in hopes of improving quality of life for myself and future generations. Rare diseases are difficult to live with because there is not enough education and options available. I would appreciate the support of the FDA by allowing regulatory flexibility for drugs being a potential treatment for SCA. Thank you.
Lisa B.	As a friend of someone with SCA3, I strongly believe there needs to be improvements and advancement in the study of medications and new drugs to treat the disease. Without research, there is no way to make these advances. Please reconsider.
Jerry T.	I've been in this drug trial at UCLA, for almost 5 years. I definitely feel it's helping to slow the process of my disease.
Ilija T.	This is the first step towards a drug that could benefit me. Any risk would be worth it. The trials so far look promising and I would definitely try it. What have I to lose? My life is spiraling down at this stage — I can't walk, I fall when sitting, I have trouble eating, my speech is becoming unintelligible, I cough and choke much of the time, and I have severe jolting pains that leave me groaning. It distresses both me and especially my family to the extent that I limit my time with them. What quality of life is there? It can't get much worse. Give me a chance please.
Rhonda J.	I have SCA3 and participated in the final phase of the clinical trial. While I didn't improve while taking "the real thing" I DID NOT get worse. I have seen a decline since I stopped taking it. (I withdrew from the study in 1/23 because of an unrelated illness.). It did help me! I hope the FDA will reconsider.
Silvania L.	I am very disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory process from FDA for rare diseases like ataxias should be more flexible!
Carole L.	I have watched my dear friend go from one of the most energetic, dynamic people I've ever known to not being able to get out of a chair and walk without assistance because of Ataxia. I know that she would do anything, try any medical trial that might help with this horrible disease. I hope the FDA will reconsider their decision on the drug troriluzole.
Ana C.	I am very disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory process from FDA for rare diseases like ataxias should be more flexible.
BethAnn M.	It has been difficult watching my husband's physical and functional decline due to SCA3. We are trying desperately to do what we can to keep him at his optimal level of function. But nothing is working. We need something to give us hope. Even if it's not perfect, even if it just slows the progression of the disease a little, it's worth it. Every day is a blessing and every day with slower disease progression would be miraculous for him.
Keith B.	If I understand correctly, the FDA reviewed an incredibly expensive Alzheimer drug. To not review a drug for Ataxia, a disease with no successful drugs, seems an extreme oversight.

NAME	COMMENT
Jayda G.	I am writing to express my deep concern regarding the recent decision to turn down the new drug application for Ataxia treatment. As someone who cares deeply about the well-being of individuals like my dear friend, who battles with this challenging condition, I implore you to reconsider this decision and open the door to renewed hope for countless lives. My friend is a woman whose resilience and courage inspire everyone who has the privilege to know her. She has been living with Ataxia for years, facing daily challenges that most of us can only imagine. Despite the hurdles this condition presents, she remains a loving mother to her young son. My friend's greatest wish is to be granted more time with her child, to witness his growth, and to create cherished memories that will carry him through life. The potential of the drug under consideration to improve the lives of individuals like my friend cannot be understated. By granting access to this treatment, you have the opportunity to offer hope and brighter prospects to people who have been living in the shadows of a relentless condition. Your decision carries the power to strengthen families, cultivate connections, and provide individuals like my friend the chance to live more fulfilling lives. I urge you to take into consideration the countless stories, dreams, and aspirations that are intertwined with the fate of this drug application. **Please remember that behind every statistic is a real person with loved ones, dreams, and a desire to contribute positively to society.** By approving this drug, you can contribute to the creation of a future where people are empowered to live their lives to the fullest. Thank you for your time and consideration. **Your agency's commitment to public health and well-being is a beacon of hope for so many, and I believe that this reconsideration can bring light to those who need it most.**
Marjorie W.	You're taking away an opportunity for someone with a debilitating disease without giving it a chance. That's just unfair. Please reconsider.
Patrick M.	Being diagnosed with SCA3 I was glad to hear of such a drug. I am progressively getting worse so if there is such a drug that would slow down the progress I urge the FDA to reconsider their decision. I don't want to end up in a wheelchair.
Sky	We would like it if the FDA could approve this drug. Thank you.
Michelle B.	I am extremely disappointed that FDA won't review all the research information from the trials. There is currently no potential treatment or treatment for any of the SCAs. My dad has SCA and I have SCA. I'm hoping my son will not have it but if he does I would like to know a treatment is available.
Lauren A.	I was a coordinator on the trial and saw the attenuating effect of the drug in our patients with SCA3, and potentially other genotypes. This is backed by natural history studies of our patients not on the drug. Our Ataxia Center specializes in the clinical treatment and presentation of these patients, and therefore our providers are experts in this disorder. Flexibility should be given in this case as we did not have the breadth of research or subject numbers to determine which subtype would specifically respond, or how long this may take in subtypes that progress at a much slower pace but have similar pathology. We have found positive outcomes in SCA3 — this should not be understated for these patients.
Justina	Look at the data!! Please! Our disease is showing up and progressing in family members 20–30 years earlier than the generation before. We deserve a chance at some relief / help just as everyone else with a disease! Especially when ours so closely resembles so many that have more funds and research.
Greg R.	As an individual living with ataxia I feel the urgency of an approved treatment. I have been a participant in the clinical trial of troriluzole. It is safe and deserves a full review.
Donna B.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Cherilyn M.	I want the FDA to reconsider their position on troriluzole because we desperately need HOPE. After being an Ataxia support group leader for many years, I am crushed every time a member of my group ends their life because they see no HOPE. I am crushed right now and I know I will be again and again. HOPE.
Seth M.	I have seen two generations of my family die from ataxia. My grandpa and aunt lived agonizing lives in their latter decades and died in pain. My dad is currently suffering from it, as are my cousins, my sister, and me. We are begging for any option we can take, even if it ultimately isn't fruitful. The only way to know if it works is to allow its use. Ataxia is a miserable disease. Please review the NDA for troriluzole in treating ataxia.
Nicole V.	This needs to be reconsidered to help support those with Ataxia.
LeRoy K.	I have been diagnosed with SCA#3 since 2011 and the progression is worsening as we speak. I feel the FDA should consider this treatment as a beneficial upgrade to science. Thank you.
Stacey S.	I am a patient living with SCA3. I feel if there's any opportunity at making people live longer or with less symptom progression, they should be offered that opportunity.

NAME	COMMENT
Gail R.	Because Ataxia is a disease with many possible symptoms, it is unfortunate that the research didn't look at just the expected outcome, which was on falls. They did find a SIGNIFICANT reduction in falls. This alone can be a lifesaving difference that this drug could make for these patients. In the future, be very careful about looking at the SCA 30 point assessment. There have been times when my spouse has been very dependent and sick and yet scored very high on this test. Unfortunately, the test needs to be weighted. There are times when a person cannot walk at all and yet all of the other tests are OK, leading to a very good overall score.
Karen S.	This disease has been life altering for me and heartbreaking for my family to watch the relentless progression. Please help the ataxia community by reconsidering your review of this NDA. Thank you.
Adrienne S.	My niece is only 22, and her disease is moving so fast that, despite graduating with an excellent GPA, she is no longer as verbal as she once was. Her communication is becoming shorter, more limited, even truncated, and she goes to physical therapy on a regular basis to be able to walk. She avoids stairs. She should be able to access this medication given that there are no other options. Making it available does not mean patients have to choose it. But to block possible improvements for those in such need is to make the FDA's unwillingness to take appropriate risks more important than the experiences of those patients who have no medications now, need hope and a chance now, and may provide insight for future patients. My niece's symptoms are going to steadily worsen. With this med, she might be able to enjoy a good year or two. Please reconsider.
Marcus T.	I am 48 years old and am currently living with SCA3. I have seen the harmful effects of this disease in my mother (who died at age 69), my maternal grandfather (who also died at age 69), my maternal aunt (currently living with SCA3), and my younger brother (43 years old and currently surviving SCA3 but confined to a wheelchair). It is extremely difficult to maintain a positive view of the future with the inevitable formalities that this disease presents. Please FDA reconsider your stance on this disease, and allow the flexibility needed for the approval of drugs such as troriluzole. If there is any chance of it slowing down this dreadful disease, I would welcome it with open arms. Sincerely, Marcus T.
Jennie M.	Hello. I am 1 of 4 siblings currently living with this horrible, fatal and hopeless disease. I cared for my mother up until she passed away with this, and my aunt is currently in the tail end of this, so my experience with this fatal disease runs deep. I am asking, no, begging the FDA to at least review Biohaven's NDA before making such a detrimental decision. I realize that healthy people are not concerned with anything that does not effect them, but we have nothing. Nothing to look forward to. Nothing to hold on to. Nothing to hope for. To be able to be a part of a future that we haven't even had the opportunity to dream about would mean everything. I know how bad it gets, and I remember thinking that if my mother could have done one thing independently, held a spoon, moved herself from a chair, uttered a word, it would have made all the difference, to her and us. Now, all 4 of my siblings and I are faced with the same reality, and all I can do is just watch us die, slowly. So again, I am asking for a chance. Something to give us hope where there is none. Something for us to look forward to and the ability to at least TRY and save ourselves. Thank you.
Walt K.	Please accept the NDA filing for troriluzole for a full review. I have benefited from drugs that have failed their broad objective but have shown to significantly benefit some study participants. Because it is relatively rare, Ataxia does not get the Pharma support it desperately needs. To deny opportunities for benefit is cruel.
Tee A.	The Biohaven trial was the first legitimate phase-3 clinical trials for SCAs. The postdoc analysis suggested efficacy. FDA should review the data because there was a precedence of NDA granted in a similar situation.
Robert C.	Any drug which may have potential to aid a person living with ataxia should be given serious consideration and examined in all aspects.
Mark H.	Let's take a further look at troriluzole. The FDA's 2019 Substantial Evidence of Effectiveness Draft Guidance says that "certain situations, such as when a disease is rare or the disease is life-threatening or severely debilitating with an unmet medical need, may warrant additional flexibility." SCA3 certainly meets all three of these criteria.
Jane K.	While I do not personally have SCA3, I have numerous aunts and uncles and cousins that do. Watching their condition deteriorate over the years has been truly heartbreaking. I see the fear in my cousins who are my age or younger (43) and have watched their parents struggle with the disease, knowing their fate will be inevitably similar or worse. The fact that the FDA decided not to review the NDA for troriluzole is beyond frustrating. The people suffering from SCA3 deserve the chance to try this medication. The FDA should apply appropriate regulatory flexibility to accept and review the NDA for troriluzole.
Cornell B.	I feel the FDA should give this drug a chance. Give it a chance to help people with this illness.

NAME	COMMENT
Susan A.	The FDA needs to realize that the different forms of ataxia are very rare and there aren't many options that work to improve the condition or help patients function better. This drug should be reviewed because of the potential to help a person who is living with debilitating symptoms and limitations that have a form of ataxia that could respond to this drug.
Rachel L.	I am living with Ataxia which is a very debilitating disease. As yet there are no cures or even treatment. All medications as a result should be given a full review.
Marni M.	I'm angry. Really angry. I'm angry at a disease that has turned my husband from a marathon runner into a man who can barely walk. I'm angry that the intelligent, determined, and stubborn man I married has lost the ability to solve problems, take care of finances or help me make decisions. I'm angry that I was forced into early retirement because assisted living and memory care cost $9,000 a month. I'm angry that his three siblings all have the same debilitating disease. I'm angry that two of them don't have the financial resources to properly meet their needs. I'm angry that another generation will endure the pain that we're going through. This disease is striking earlier and earlier with each generation. There are young people who will see their spouses and children deal with this disease. The children we see today may not live to see their grandchildren grow up. I'm angry that my mother in law has had to witness the deterioration of her husband and all four of her children. Above all, I am angry that the FDA refuses to apply appropriate regulatory flexibility and to accept and review the NDA for troriluzole. While I hold little hope that my husband's generation will have a meaningful treatment for ataxia, I have hope that his nieces, nephews, grandniece and grandnephews will.
Fanny W.	My grandmother, my mother and uncle are the patients who have been suffered from the disease for a few decades. It was both a strike for themselves and the family members. We have been tried so hard to live a normal life like ordinary families. We were thrilled to know that there will be a new drug for the disease so I strongly hope that it would be accepted by FDA. Please help us!
Dr. H. Brent C.	While troriluzole is not a 'cure" for SCA3, it may have some functional efficacy in what is currently an untreatable condition. The initial trials were run on multiple types of spinocerebellar ataxia, many of which have a similar genetic mechanism, but also can have widely different pathologies and clinical phenotypes. SCA3 itself has a very wide phenotypic variability and was once considered to a distinct entity from Machado-Joseph disease, which shares the same genetic mutation. Many patients with SCA3/ MJD have a motor neuron component. Riluzole, which is the metabolic product of Proriluzole has already been approved for motor neuron disease, so it seems that a meta-analysis of the SCA3 component of the original trial would be worth doing.
Isaac M.	At this time there are barely any drugs available for us to, at least, give us a glint of hope in treating our condition. We need any and all efforts in this direction to, at least, give us a fighting chance.
Jamilah M.	As with many treatments for diseases or disorders, in order to know if the drug will be beneficial trials have to be done. My best friend of over 20 years whom is just in her 30s has ataxia. She had to watch her grandfather deteriorate and pass because there was no known treatment or anything to slow down the disorder. She recently had to watch her father's rapid debilitating journey that also led to his death due to ataxia. If there is possibly a treatment out there that can give her and others some sort of hope to slow down the progression so they can continue to live their lives without that added stress, I don't understand why the FDA would block such a thing. You spend millions and billions on drugs that may or may not actually do what is claimed. Here is a drug that seems to show that there could be some benefit. So I ask the question, why are you blocking people from being able to live a fulfilled life? Troriluzole may not be a drug to eliminate the disorder but if it does anything to benefit people's lives for the better, why block them from having that opportunity. Please reconsider your decision to not review troriluzole and any other treatment that may provide benefit to those living with ataxia.
Martha T.	As a rare disease, SCA does not receive the funding that other "big name" diseases (i.e. MS, Parkinson's, etc.) receive. The population of individuals with SCA is very small, but the disease is positively devastating for the individual with the disease and family members. I urge the FDA to apply regulatory flexibility with regard to new treatment trials.
Stephanie P.	I have been part of the trial for about 3 years now. No side affects whatsoever. I would think in comparison to my older brothers progression who IS NOT in the trial, that my progression is slower than his and our CAG repeaters are 2 away from each other, which is the bloodlines that determined SCA. I thought Biohaven had an excellent chance of having their drug approved, and now not approving AND not willing to review the NDA file, goes against the guidelines that you, the FDA, have set. I hope you reconsider for all people suffering from the horrible disease. There is not one day that passes that I don't look at someone, on a leisurely walk, in a store or just walking from their car and wish I could do that so effortlessly…I hope you would ponder that on the way to your next meeting.

NAME	COMMENT
Rita C.	As a carrier of SCA3 and suffering the terrible consequences that this rare disease generates, I would like the FDA to reassess the decision they took not to review the new drug application form for the troriluzole from Biohaven Pharmaceuticals.
Madalyn G.	My 3 siblings with SCA3 have been taking troriluzole for years now. They all feel that the medication has brought some help to them and that without it, they would be worse off. I do believe that you should allow people with rare diseases take a medication even if it's only giving them minimal affects.
Theodore P.	Due to the lack of available treatments for SCA3 and the lack of well developed and recognized endpoints for clinical efficacy, in addition to the long term development of the disease, the FDA should review the complete Phase III data set to ascertain if there is potential clinical benefit from troriluzole.
Jeandrei W.	I believe that the drug troriluzole is effective in slowing down the progression of SCA3 ataxia. I would like that the FDA could be more flexible and agree to evaluate Biohaven's data for troriluzole. SCA3 is a serious, degenerative disease and for which there is not even a medicine to stop the progression of the disease.
Marian S.	As a person living with ataxia, it is disappointing that the FDA will not give troriluzole a full review, taking away the right to try some kind of treatment for ataxia. Any little progress is better than what is available now, not a thing every day every year just more progression of the disease.
Maryellen T.	I and many members of my family are or have been affected by SCA-3 -- my sister, mother, aunts, uncles, cousins. It is frustrating to live with a disease which has no medical treatment, despite the potential for drugs that may be available to help the slow degeneration of the body. Please consider reviewing the New Drug Application for troriluzole as potential treatment for SCA3.

"I cannot understand why the FDA would not review the full data. It is ALWAYS worth reviewing the full data, especially when it comes to developing drugs for rare diseases, which are at a huge disadvantage for doing so."

— Katelyn L.

NAME	COMMENT
Odinachi O.	I have several patients on the study drug (of note I am not a PI on this study, but one who sees and treats Ataxia patients). I do agree that the endpoint was not made but there were clinically significant improvements in the function of my Ataxia patients on the study drug troriluzole with an improvement and stabilization of their HRQoL. I would strongly reconsider your status on reviewing the new drug application for the sake of the many patients who would benefit from this drug. Thank you.
Wendy E.	Living with this fatal neurological disorder is horrible. I lost my husband two years ago from this horrible disease. My son, 30 years old, also has it. I've been a caregiver for over 20 years. I too am affected. SCA research and trials to help slow the progress are essential and just as important as other fatal disorders, i.e., Parkinson's, MS, Alzheimer's. It is only right for the FDA to review the data. To decline without reviewing is not acceptable. It seems like discrimination. First seek to understand and take all data into account. Thank you.
Sharon K.	All investigation is worthwhile. Please review the application!
Paco M.	I have witnessed various family members suffer with a lack of treatment options. I'm submitting this on behalf of my cousin who has thoroughly investigated her options. Given the lack of treatment available for many of these patients, this should be considered for further review.
Maggie	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Karen V.	This disease has affected our family for over 5 generations. We are doing our part with spreading awareness, fundraising and brain donations for research. This disease deserves a full review of the drug troriluzole as a potential treatment for SCA3. We deserve a full review!! Troriluzole showed promising and meaningful improvement in ataxia symptoms for SCA3 patients. Don't give up on us!! We're a resilient group of people willing to help end this disease and we support promising drugs that have the potential to improve symptoms even if means taking risks!
Gregory K.	I suffer from SCA3. I want to be able to try any med to alleviate my symptoms — if the drug is safe PLEASE let me decide its efficacy.
Beth M.	As a person living with ataxia, I count on the FDA to take a flexible approach to approving potential treatments. The data is there for troriluzole as a treatment for SCA3. The FDA should file the Biohaven application and get this drug to people who are suffering today.
Claudine R.	All information should be completely reviewed prior to making a decision. People with ataxia likely feel like there is nothing to lose by trying something that has the potential to slow the progression of the illness. My dad died 25 years ago from ataxia and had nothing to slow his progression. My sister is starting to show symptoms at 53 and I think something is better than nothing.
Georgia L.	As of date, there is no known cure or treatment for Ataxia. Those of us suffering with it deserve the option of trying troriluzole.
Jeremy H.	My mom has SCA3. She has fallen and broke her jaw in 5 places. She fell another time and cracked her skull. She has fallen numerous other times and received bumps, bruises and stitches. This is a terrible disease. Please review any drug that could help this terrible disease.
Sergio C.	I strongly support the full review of the NDA for troriluzole as a potential treatment for SCA3, as first step. Second, there must be regulatory mechanisms to allow for flexibility. For example, the European OD regulation allows for conditional Marketing Authorization, typically under exceptional circumstances i.e. for products/diseases with limited scientific info available at the time of MA. The MA of such products is subject to annual reassessment as to the risk-benefit ratio upon the periodic scientific info collected, elaborated and provided by the MA Holder. Specific additional risk mitigation activities can be laid down in the Risk Management Plan agreed with Reg Authorities. Today technology offers powerful tools for therapy monitoring, early sign detection and timely intervention if needed.
Alicia E.	Please let any drug that could help be available, even if the benefit is unknown or could be small. To those living with SCA3, the hope of a new drug and even small improvements can mean a world of difference.
Dana C.	In the world of chronic, progressive diseases, when a promising lead is exposed, that could lead to a cure, enhancement of one's quality of life, or slowing the progression of a disease, it should not be ignored. While the efficacy of the drug may not be 100% without side effects, or possible adverse reactions, other medications have those same risks. Without even trying, how would the FDA be able to put their stamp of denial on a drug before the data is brought forth for review? If there is an ability to help even a portion of the population that is dealing with a chronic condition, it should be reviewed as an option — it may have the potential to better many individual's lives.

NAME	COMMENT
Steve M.	By refusing to review the new drug application the FDA is eliminating Hope from individuals living with this horrible disease. Watching this disease progress in a dear friend is heartbreaking. The Hope of any form of relief from the unmerciful progression of this disease is not only being taken away from her, but also from her family and all of us that love her. Help, and Hope, is needed.
Cath T.	I am a 70 year old woman. I am so very grateful to NAF for providing Ataxians with the opportunity to make our voices heard re: the maximum regulatory flexibility in the review of data from clinical trials. I am so desperately disappointed in FDA's decision to refuse to file the application for troriluzole. I was diagnosed with SCA 16 years ago, 16 years that have felt like a robbery in slow motion. My wonderful Spouse has been a victim of the same robbery. My hard-working, capable independent self is quickly becoming more and more dependent. This puts a strain on the dynamics of our relationship, which we both struggle to adjust too. I live on the edge of anxiety because of the vulnerability that has crept into my being. My defenses are weakened and dull. Unlike a specific traumatic event, the degenerative process is not a one time issue. It is unrelenting, incurable and requires immense emotional strength and endurance. Walking is exhausting, talking is rehearsed mentally before speaking. A clear passage through a room is mentally determined before approach. Level ground is mandatory. Exertion leaves me with very slurred speech and exaggerated imbalance. My pride is deeply bruised when using my walker in public, resulting in a self imposed confinement. My weight gain is excruciating for me. A sedentary lifestyle is completely against my nature. I have learned over the years how to modify and adjust, but it surprises me how easily I slip backward into resentment and frustration after I have a setback. Knowledge of this disease is needed to promote the support of the public to assist with research to find a cure for this horrible condition. Ataxia is never dormant. It grows like fungus. No matter how much you will it to slow down, nothing to date will arrest its march of war on the destruction of cells in my cerebellum. The vacant space in my brain becomes larger with each year that passes. Sadness comes with loss. Each day I experience loss; loss of coordination, loss of balance, loss of independence, loss of ability to do my favorite activities. Perhaps the greatest loss I experience is the loss of self-esteem. I don't like the person that I have become. For the most part I have strutted through life with my head held high, confident in my appearance, my self expression, my accomplishments and my successes. I have been fiercely independent and have taken great pride in my mental and physical abilities. Guilt consumes me for having to burden my spouse with so many extra chores now. The grip of depression can strangle your spirit and destroy the precious union of loving partners. Some days my infliction is overwhelming. Ataxians need for a treatment has gone unmet for too long. Our numbers are small but our lives are important and deserving of any chance we can get to improve them. Generally, people don't understand the pain that we go through every day of our lives. Our entire essence suffers with this deplorable disease. Please, don't turn your backs on us.
Robert A.	Seeing my dear friend struggle as her quality of life deteriorates with Ataxia, and the pain and sorrow all her loved ones are also experiencing with the helplessness of the situation makes me deeply believe that the hope making the Biohaven drug available to people with SCA3 at this stage is worth it for those willing to take it. And also could provide valuable information and research data to find a cure for those who have Ataxia in the future.
Joel J.	I have had a mother-in-law die from this disease. To watch her grandkids be robbed of critical time with their grandparent when there is the possibility of a drug that prolongs their life would be immense for the entire family. Yes we understand there are risks but as there are with any drugs people take, I would ask you to reconsider and take this under further guidance and review
Chunying M.	As the wife and mother of SCA3 patients, I deeply feel the pain and despair that the disease has brought to my family. I watched as my husband was completely unable to walk, work, and have no medicine to cure. SCA3 brings insurmountable difficulties and fears to me and my family. We can't wait any longer! I strongly recommend that the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review.
Elisabet S.	I implore you to change your mind! I have a dear friend with this disease. I have seen the many changes and decline with her these last few years. We have had many talks and she so wishes for a chance to get better and at the very least have options. This especially hits home because my beloved husband recently passed from cancer. We were hopeful until the cancer spread and little could be done. But he did everything and tried everything the doctor's suggested. We have had discussions with our friend about quality of life. Everyone deserves a chance at their best life. Please reconsider and give my friend that chance. I promise you, she makes the world better and we need her here! Thank you
Joy H.	I have been friends with a person who has ataxia for nearly 20 years. I've watched her go from an active, athletic person, to one who can barely move her feet and is wheelchair bound. Swallowing has become a challenge. This disease is taking her before our eyes. Please apply regulatory flexibility to this drug and accept the NDA filing for troriluzole for a full review. We NEED this. Without it there is no hope.

NAME	COMMENT
David B.	It is imperative that governmental agencies follow their own policies and guidelines. If for some reason Biohaven has not met the criteria for review, the Ataxia community, and indeed, the entire Rare Disease Community, should be made aware of it.
Francisco U.	1. Please, this is the nearest chance we have seen in our lives in order to have a medicine that will benefit us. 2. We are hopeless right now, it is a catastrophic disease which the only thing you know is that each day you wake up, you are worse than yesterday. 3. We see that this medicine has a potential benefit to relentless, for us to win one day to relent the disease is a new opportunity. 4. The only chance that we see in the short term is this medicine, if you revise the pipeline of medicines for SCA3, there are not good signs of having results in the short and mid term.
Kim U.	Please give us all hope by starting with this drug for SCA3. All with ataxia believe this could be a beginning for having an improved extended life. Many cancer drugs that are experimental are approved without knowing for sure if they will be successful to give patients a chance for life - this is same situation. By not even reviewing you are giving them a death sentence for sure. Please reconsider giving the drug trial a chance with a full review. Thank you for your consideration.
Soorya	For people suffering with the disease, trust me completely that they would rather have some hope and treatment than nothing. Please let people try it.
Shakti R.	To whom ever is reading this, my father passed away last year from SCA after battling the disease for 15 long excruciating years. Little did we know that the worst part was not that there was no cure or treatment but that as he lost his ability to speak to see so point that he would lose his ability to communicate and be trapped in his own mind. Because although his body was failing him his mind wasn't and he knew all along what was happening to him. It was the most excruciating most torturous way for someone who used to love life to have passed. Up until his very last I prayed so hard for a miracle for something anything that would slow this illness or at least stop what had been done and nothing came. Given that SCA is a autosomal dominant mutation meaning it is inherited /passed down from parent to child I do not know if I will face the same demise or if my children will suffer in seeing how I end up suffering. I understand the standard you want to hold drugs to, but please I beg of you even if this troriluzole could benefit in even the smallest way, you have no idea what it would mean to families suffering from this. I beg of you give some flexibility.
Jessica P.	I am 36 years old with a 6 and an 8 year old daughter. I have SCA3 and would love to try the drug that could potentially extend my time with my daughters. I feel like without any form of treatment I would be lucky to still be able to see them graduate from high school, which scares me to admit. Please consider fast tracking this medication so I would be able to try it while I still can!
Gary S.	There is no cure and very little treatment for SCA sufferers, so every drug that has a positive effect must be investigated thoroughly.
Élodie H.	I am struggling daily, from morning to evening, with the disease of SCA3 and I would like to try the troriluzole drug because I have life threatening. Thank you.
Cynthia P.	Please review this new drug application for troriluzole to treat SCA3. This will help so many with this disease and their families. We need you to reconsider.
Hirut M.	Dear FDA: It was devastating news to learn that there were no treatment options for my suffering relative. However, I set my hope high when i heard that in a clinical trial the new medicine showed potential benefits to patients. I have been eagerly waiting to hear about its availability as the condition of my relative continues to get worse by the day. Thus, I am earnestly asking you to reconsider the application for its review as soon as possible. It feels only fair to give my relative the chance to try it. Thus far it is the only hope for him. Thank you much.
Kui H.	As an SCA3 patient, I deeply feel the pain and despair brought by the disease. After more than 10 years of illness, I am now completely unable to walk, work, and have no medicine to treat. SCA3 brings insurmountable difficulties and fears to me and my family. I strongly recommend the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review.
Judy M.	I have Ataxia and there is no hope for a cure so I would like to have a chance to try this drug.
Michael C.	I have direct experience with someone who suffers from SCA3. It is devastating to watch, and I beg you to allow someone to take whatever risks might come with such a promising drug. The FDA simply MUST look at this differently.
Sandra M.	I am very disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory process from FDA for rare diseases like ataxias should be more flexible.

NAME	COMMENT
Mollie U.	Please reconsider your recently issues refusal to file letter to Biohaven regarding their NDA for troriluzole as a potential treatment for SCA3. I work with the Ataxia community daily and witness firsthand the disease's impact on individuals and their families. The hope and anticipation surrounding troriluzole have given this community a dream of what could be. The potential benefits of this treatment could provide much-needed relief to those who have been tirelessly battling SCA3. However, we will not know that without your full review of the drug. I believe your policies and previous reviews of other drugs for rare diseases, support your reviewing the troriluzole NDA. Your thoughtful reconsideration could make an immense difference in the lives of those living with SCA3 and offer renewed hope and a chance at a better future for all those in the Ataxia community.
Therese F.	The ultimate approval decision on troriluzole should come after a thorough review of the clinical trial results and other information submitted by Biohaven in the NDA. The SCA community's need for a treatment is both urgent and great, so careful consideration of the totality of the data, taking into account patient experiences and preferences, is deserved. Please look at this through the eyes of Ataxia sufferers, their family, caregivers, friends and doctors.
Patricia W.	As the Granddaughter, Daughter, Sister, and Aunt of loved ones suffering with SCA3, aka (Machado-Joseph) disease, I find it unconscionable that the FDA refuses to review the New Drug Application for troriluzole as a potential treatment for SCA3. My Grandmother on my Father's side was deceased before I was born. As the youngest of 3, I watched my Father's terrible struggle (I always felt as though he was a beautiful person trapped inside his deteriorating body). In the 1950's when he had this disease, there was very little known about it, no name given to it. Also, genetic testing was not available. First he lost the ability to walk , then he lost the ability to be understood when he tried to talk, eventually after being a complete invalid for at least 4 years, he was unable to swallow or eat. He died at the age of 46. (I was 10 years old.) My sister, Mother of 8 died at the age of 58 from the effects of SCA3. Now 3 of her 8 children have succumbed to SCA3, with a 4th sibling also having it. I can't believe that any of them would not or would not have wanted to try a drug showing possible benefits in the alleviation of their conditions. As a believer in the Sanctity of Life, it is amazing in today's culture that the FDA can be very quick to approve drugs that cause the intrauterine death of unborn babies, but don't want to consider the approval of a drug, such as troriluzole, for the benefit of the living. I urge the FDA to please review this New Drug Application for troriluzole as a potential treatment for SCA3.
Kyle B.	I believe that newly developed drugs for rare diseases, especially those with no known treatments, must be given more flexibility to be approved. As someone with a brother who has a very advanced disease of the central nervous system, and who has no options for drugs, it is devastating to see the disease take away his ability to walk, talk, use a phone or computer, and even eat food on his own. He applies for every clinical trial in hopes of something that might slow the disease. Many of the people in his support group are like him in that they would try a new drug if it had any chance of slowing their disease progression. Many of them have no other option.
Kia R.	The NDA needs to be reviewed absolutely!
Julie T.	I don't understand why the FDA is hesitant to allow this to proceed. If there is no cure currently, it can only be beneficial to the continued research that is necessary. Those who are suffering deserve to have this decision reversed.
Zachary D.	FDA, Please review the new drug application for troriluzole. My wife has ataxia. Please help us!
Girma M.	It was extremely frustrating to learn that FDA would not review the NDA for troriluzole. The data so far is very promising for our family. Even if all this drug achieves for our family is slowing the progression of the disease by even a small percentage that would be time reclaimed with our loved ones that has been stolen by this disease. Please afford us a chance to get a fair and thorough review of the NDA.
Jo Ann K.	I am living with Ataxia that is caused by an unknown genetic mutation. However, I believe that the FDA should review troriluzole to give hope to people with SCA3, because everyone with Ataxia deserves a chance to improve his or her life. My Ataxia, and all other types, have the same symptoms, and the symptoms do diminish quality of life. The FDA has the opportunity to improve quality of life for a portion of the Ataxia community. The FDA should take advantage of that opportunity.
Mary B.	Spinocerebellar ataxia affects every aspect of my life — personal and professional. With each incremental deterioration of movement, speech, and vision I am reminded that progression of disease is inevitable and that there is NO treatment available. My world is shrinking. Please review the data for troriluzole as it applies to SCA3 and determine risk/benefit for patients with this progressive, incurable, and to date, untreatable condition. Thank you.

NAME	COMMENT
Stephanie P.	Please grant approval for troriluzole. Both my mother and brother suffer from SCA3 and I care for them both. We've traveled over 8 hours to a clinical trial site that was shut down, attended numerous webinars, changed eating habits and participate in as much physical therapy as possible. My brother has twice as many falls as my mom and it's humanly impossible for me to be there to break all their falls. I don't sleep listening out for them both. My mom suffers from a great deal of depression due to this illness and seeing my brother suffer too. We are willing to do what it takes to obtain access to this medication. A little improvement will mean a lot to them both along with my family. No drug is perfect but having "an option" for SCA3 is huge. Please help my family by giving them hope and reducing the fear of falls and rapid progression of SCA3.
Regina B.	Please review this drug as it is showing promise with SCA3 patients. The impact this could have on those suffering from this debilitating condition is enormous and could change the lives of generations.
Peter K.	Having witnessed my friend of thirty years bravely face her Ataxia diagnosis, along with the onset of its symptoms, I've seen her cope valiantly with the tiny number of good clinical options she has. There aren't many, and what is available has done very little. She has participated in studies and research, and is quite a self-advocate, but, because of the nature of her diagnosis particularly given the population at large, it is an ongoing struggle to fit into a medical system not meant to accommodate the exception to the rules, which Ataxia is. This seems to be the case with the FDA decision to not review the application for troriluzole. One certainly understands policy and procedures, and also liability and risk management; however, mercy and common sense should also be employed as well. I think back to the AIDS epidemic when drug trials were revolutionized due to the pressure of a minority group who needed these life-saving drugs, or even during the COVID pandemic, when policies were altered. The rules can be changed if a situation demands that they be changed. And that is the case here, given all of the complexities at play. I would ask that FDA reconsider its policies here since changing them will only help a population who has few good options available, a population who can make an informed decision about what risk they are willing to assume in their own care, a population who is trying to stave off a debilitating degenerative condition which is already harming their lives. My friend watched her mother become paralyzed and die from Ataxia, and now she and her sister are both looking at the same progression for themselves. It is cruel to deny them the one possibility of help when there is no other possibility available. And it may even help future generations facing this terrible disease. Please, use some common sense and mercy here, and reconsider your decision in light of the unique circumstances which Ataxia creates. The FDA has made plenty of exceptions to their rules in the past in order to save lives, and there is no reason why they cannot do so now. Thank you.
Lori S.	I have watched over the last 18 years the devastating impact that SCA has on patients and families. People with SCA need a treatment – anything that has the potential to even incrementally slow the relentless progression of this condition or lessen symptoms. People with SCA3 deserve to have the full data on troriluzole reviewed to have confidence in the drug development process and the FDA's role in that process.
Joyce F.	My mother had SCA for 10+ years until it contributed to her recent death. It is an extremely debilitating disease with no treatment or cure. It was extremely tough on her and our family, having her lose her motor control and independence as well as having to change our lifestyles because of it. Any potential treatment that could slow down the disease progression and help maintain motor control is worth exploring. Please reconsider reviewing the NDA filing for troriluzole in order to help those with ataxia and their loved ones.
Michael Y.	It is a bad decision not to include regulatory flexibility to the research of the new drug application troriluzole for SCA3. I can attest to the personal experience of being a prime working age tax payer. But living with SCA3 in a assisted living facility with old people who are beyond their prime and not able to complain about pain... I am not contributing anything to society! I can also attest to being a physician and yet cannot work because of my physical limitation! Where I can be so much assistance if I did not have this life-devastating illness.
Mary G.	Ataxia is a relentlessly progressive disease so a medication with rare side effects with the potential to stop or slow progression is HUGE. And, as I sit here recovering from a broken wrist requiring a $60,000 surgery from what seemed like a 'minor' fall on Christmas, the reduced fall risk is also HUGE. I have read a lot about Glutamate, and I do believe the reduction of extra-cellular Glutamate is important. I am heartened by the participants of the Biohaven trial who have taken troriluzole for years, and they do not want to stop because they believe the medication helps them. Please help us. People have been waiting for decades for a therapy to help, and the FDA decision to not even review the Biohaven NDA is a painful slap in the face, and it appears to be bureaucratic and petty.
Lisa C.	If you or your loved one were told "there's no known cure", what would YOU do? The FDA must look at this differently and give those that are afflicted a chance at a brighter future.

NAME	COMMENT
Karen W.	My son is currently living with SCA3. His father passed away at the age of 50 with SCA3. It's a helpless feeling as a parent to realize there is nothing you can do to prevent or help the rapidly developing symptoms of this disease. Please reconsider your decision regarding the application for troriluzole as a potential treatment for SCA3. Thank you.
Dr. Jeremy S.	I have been treating patients with cerebellar ataxia for 35 years. I have extensive experience in the assessment and management of patients with spinocerebellar ataxia, and in particular, spinocerebellar ataxia type 3, Machado Joseph disease. There is a large population of patients with this disorder in the New England area, and at the MGH Ataxia Center we are the premier and largest site for the care of these patients. I have witnessed firsthand the debilitating nature of this disease, the devastation it causes to patients and their families, and the early death that results from the condition. It is untreatable, and our management has been entirely symptomatic, working to improve the quality of life on a day-to-day basis. I have been involved in the troriluzole study with Biohaven, and I have been impressed by the fact that patients on the drug appear to be relatively stable compared to how they would have otherwise performed. This has been borne out by the data analysis by Biohaven. The decision by the FDA to refuse to consider the data from the sponsor of this trial is inexplicable, and a devastating blow to patients and their families. I urge you to reconsider, to give the data an opportunity to be heard and dissected, and for those who are knowledgeable in this matter to be able to make an informed decision about whether in SCA3 in particular, there is enough reason to go ahead and approve the use of this drug. In the absence of anything available at all that can slow down progression of SCA3, the availability of troriluzole as would be an entirely new direction and an opportunity for optimism and hope in the disorder. Please reconsider your decision, and hear the data. Patients deserve it, and I hope that you will respect that.
Hayley M.	I currently lead a competitive research program at the University of Michigan studying mechanistic and translational research for the most common dominantly inherited ataxia, Spinocerebellar ataxia type 3 (SCA3). I have studied this polyglutamine expansion disease for nearly 10 years. SCA3 is a progressive neurodegenerative disease that usually leads to death within 10–15 years of symptom onset and for which there is currently no treatment. I believe, any and all opportunities for SCA3 therapeutic interventions should be provided full regulatory flexibility (i.e., full review).
Diana A.	It is devastating to deal with a disease to which little or no treatment shows any significant benefit. The difficulties with running clinical trials on drugs targeted at rare diseases are many and a more flexible and creative approach will have to be applied in order to expedite testing of drugs targeted at rare diseases. I know many who have had significant morbidity and ultimately died from rare diseases and they would have voluntarily tried a drug that may have alleviated their symptoms and improved their quality of life.
Michael N.	The FDA should review the New Drug Application for troriluzole. I have lived with Ataxia for fifteen years. It is time for the FDA to recognize Ataxia as rare disease for funding.
Judson J.	As you know, each SCA is unique. Biohaven probably hoped to get positive, statistically significant results for many SCAs, but instead, they got such a result for one. SCA3. Biologically, it's unsurprising; each SCA is unique. As a person with SCA3, I strongly believe the FDA should at least agree to review the results for SCA3. Biohaven thinks it has a potential treatment for SCA3, there are no others, and opportunities to evaluate SCA3 drugs are extremely rare. We want to be absolutely sure we don't drop the ball on this one.
Tammie H.	I have Spinocerebellar Ataxia Recessive 7-Batten Disease, CLN2 which is the only CLN that has a treatment. I will start the treatment in 2 weeks. At 51, I was told I am the oldest person in the United States with CLN2. Having this genetic disease is DEVASTATING to say the least but I feel fortunate that there is a treatment that will (hopefully) slow the progression of this debilitating disorder. I have gone from walking with a very noticeable limp to a walking stick to a walker to a wheelchair within the last three years. I was misdiagnosed and can't help but wonder if I had the correct diagnosis in the beginning what my life would look like today because I could have started the enzyme treatments sooner. Please reconsider your decision and review the New Drug Application for troriluzole as a potential treatment for SCA3. We who are RARE already feel alone and that no one cares about us — YOU are proving us right. Please reconsider.
Macey G.	Please reconsider your decision. I have family that has died from SCA and my mother currently is suffering the effects. Her symptoms have drastically changed her quality of life in a quick two years she now is bound to a wheelchair. We have been watching closely for the passing of this medication through the FDA in the hopes that something would become available for my mother before her symptoms go too far, like all the mothers in my family before her. She is so close and the medication is so close to being available. Even if there are negative side effects or an unknowing going into taking the medicine, the potential benefits far outweigh these in our opinion. I do not want her to die simply waiting for the chance to make a life-saving change. This is a very rare disease but the small amount of people that could be helped will be worth a start with this medication. Please reconsider your decision. Thank you from those living with Ataxia SCA.

NAME	COMMENT
Marcia K.	My son passed away from SCA2, very similar to SCA3. Time ran out for him as we waited, wished and prayed for a cure/treatment or anything to slow this disease down. This disease is horrible. It takes one ability after another. Time matters. Everyday the disease worsens until there are no more days. Let us choose what treatments we want. Maybe this drug won't work or the benefits be too small to scientifically measure. Let the families choose. I would give anything to have my son here with me for just one more day. We already know what doing nothing will do. It will lead to death. There are no side effects worse than that. But maybe we could get another day, week or year. It's too late for my son. But there are so many other families that are still struggling with ataxia. Please don't stand in the way, help us fight together!
Louise L.	Please reconsider reviewing troriluzole. I was beginning to participate in trials for this drug in 2020 but activity stopped due to COVID. I was prepared to accept all risks and remain so. My diagnosis of SCA3 was 2018. I appreciate that FDA has limited resources and the drug addresses a small community with a rare disease but a 30 year wait seems excessive. Please don't continue to set us back. This progressive disease is debilitating and robs those afflicted of quality of life. We need to start taking action to treat this disease. Please, please reconsider the review!
Julie G.	I am in the Clinical Trial for troriluzole for the past 3 years. It has helped significantly and I would like for this to become available to other with SCA3. It is my understanding that a sister drug Riluzole has already been approved and this one is safer — I believe. This disease is very is not for everyone and I have witnessed the decline and death of my father whom I inherited this from. I would like a chance to continue to slow the process down as I am early stages. If you were diagnosed with this disease and knew this drug would be helpful why would you not approve it?

"Living with SCA3 is a nightmare and a walking death. A drug that presents hope for any kind of Ataxia it is worth review, and that review can change thousands of lives for the families that have this disease."

— Ignacio U.

NAME	COMMENT
Julie W.	I think the drug should be fully reviewed. My cause is still unknown although neurologist strongly feels it is genetic. It is extremely stressful having a condition with no treatment that is destroying you. Any potential treatment should be fully investigated
Graeme S.	I have watched my father-in-law going from using a walker occasionally when I met him to being completely dependent on full-time care for basic needs like feeding and using the toilet. I know the same is going to happen to my wife. I know she would take any chance to slow the progression of this horrible condition. It could mean the difference between our daughter having to care for her mother instead of being cared for by her mother. Please consider all the data before refusing this application. The potential benefits are life-changing if it can slow the disease even slightly.
David K.	I have two adult children with AOA2 and I have witnessed the unrelenting disease progression in each of them for the past 15 or more years. If a drug like troriluzole was available for them that could stymie the disease progression like it appears to do for those afflicted with SCA3, it would be a godsend. What each of my kids are dealing with in regard to lost motor skills is devastating but to keep symptoms from becoming worse would be a magnificent gift. Please consider a full review for troriluzole as a potential treatment for SCA3. Thank you
Dr. Lucie S.	As a pediatric neurologist trainee from the Centre of Hereditary Ataxias at the Motol University Hospital and the Second Faculty of Medicine at Charles University in Prague, I am deeply invested in advancing the understanding and treatment of ataxic disorders. My PhD candidacy focuses on the intricacies of these disorders, and being a member of both the Ataxia Global Initiative and eFACTS further cements my commitment. Troriluzole, as a modulator of glutamate, presents a promising therapeutic approach. Glutamate is the most abundant excitatory neurotransmitter, and its deregulation has been linked to a host of neurological disorders. By modulating this neurotransmitter, troriluzole potentially offers neuroprotective effects, filling a critical therapeutic gap in conditions like SCA3. It's imperative for us, in the field, to have access to all potential therapeutic options, especially when the conditions we are dealing with have limited treatments available. While I respect the rigorous evaluation process the FDA upholds for the benefit of patient safety, I am deeply concerned about the recent refusal to even review the NDA for this promising drug. For families affected by SCA3 and for healthcare professionals like myself, who aim to provide the best care possible, it's a significant setback when potential treatments are not given a comprehensive review. Moreover, the possibility of new treatments and the trust in their thorough evaluation by agencies like the FDA play a crucial role in improving patient adherence to check-up routines and participation in clinical trials. When patients see that their efforts and hopes are valued and that someone genuinely cares about their well-being, it motivates them to be more proactive and committed to their health journey. I implore the FDA to reconsider its stance, in light of the desperate need for more therapeutic options for SCA3, the promising science behind troriluzole, and the cascading benefits it can bring in terms of patient trust and participation. The global ataxia community, of which I am an active member, is closely watching the developments. A second chance at reviewing troriluzole might pave the way for hope, increased cooperation from patients, and potentially improved quality of life for countless individuals. Warm regards.
Family member	Please reverse your decision concerning this potential treatment for SCA3. Our family and I watch my daughter suffer everyday and its heartbreaking. I always give support and try to be positive to keep her upbeat but to her its hopeless as she has been told that there is no cure so some days she wants to give up. But after receiving news of a potential treatment she has brightened up somewhat. So please rethink.
Jeff A.	Currently there is no treatment or cure for Ataxia. Every day she slowly deteriorates. There is currently no hope for improvement. I just have to sit back and watch her struggle to continue to not be a burden on her loved ones. I would do anything to slow or miraculously stop the slow disintegration of her motor skills and allow a quality of life. The alternative right now is to be a vegetable. (FDA) please rethink your position and allow hope for those who currently have NO hope.
John W.	I believe that the FDA ought to review the New Drug Application for troriluzole as a possible treatment for SCA3. I'd like to know if the FDA concurs!
Serena H.	There have been recent examples where the FDA has applied regulatory flexibility in the review of drugs that, in their pivotal trials, did not meet the primary objective(s), and yet upon further analyses, evidence of efficacy emerged for either a subgroup or with longer term follow up. I don't believe drugs should be approved if there is no evidence that they work. However, in the current situation, there may be evidence to support the use of troriluzole in a subgroup of patients with genetic ataxia where no cure or treatment is available. I would ask the FDA to reconsider their decision and grant a review for troriluzole. Members of the community are asking for a thorough review and determination by the FDA and will be more than happy to provide feedback in the setting of an advisory committee.

NAME	COMMENT
Talia O.	The FDA should review the NDA for troriluzole as a potential treatment. SCAs do not have very effective treatments to date and do not have a cure. I also think that SCAs are not heavily explored in terms of treatments. People suffering from SCAs deserve to get treatments and access to new treatments, regardless of if the treatment in question appears not be very effective. As a person with SCA, I have not had many treatments and I'm willing to try anything that may work or help manage my symptoms. The SCA community deserves to not have potential treatments denied to them because the FDA decides to not review the NDA.
Stephanie A.	Any person who has the chance to approve a possible drug that might possibly benefit us should do it. I have zero doubt that if any one of them had to suffer with SCA3, they would. This disease takes your life away…very slowly. I can no longer drive, walk, paint, sew, write, eat correctly, walk the dog, go shopping, go antiquing. Should I go on? Because I could list a hundred more things that have been taken away from me slowly by this disease. I fall constantly when just trying to move around. I can't believe if there's a drug that might help me, someone in charge would deny help.
Michael L.	FDA should review the data regardless how many people have this disease, even though it's small relative to COVID-19. FDA are not doing their duties as to assess and review the data and make a decision. No confidence in FDA nowadays.
Deborah H.	PLEASE reverse your decision and review the FULL results of the Biohaven NDA for troriluzole to focus on the audience of participants with SCA Type 3 (SCA3). There are 5,000 people depending on this first ever rare disease treatment and the results are clearly positive. Importantly, there are tens of thousands of us with related Ataxias that will benefit long-term from the science that led to this novel medicine. I have had Ataxia symptoms for 15 years. It took 4 years before my doctors could even ascribe those symptoms to Ataxia. No one knows what form of Ataxia I have, knows its genetic cause or can offer any treatments for the progressive symptoms that I suffer. This totally disrupts the quality of my daily life and makes every day dangerous. Approving a treatment for SCA3 will give me hope and create a pathway to identify the cause and treatment for my Ataxia. Ten of my family members been identified with Ataxia and this genetic threat to my children and grandchildren must start to be addressed NOW! 1. The data presented do show benefit among those with SCA Type 3 (SCA3). 2. As scientists, you realize that FDA's decision to "Refuse to File" without further investigation is setting up a false negative conclusion, denying patients access to a drug that works. 3. Rare diseases must be considered differently than more prevalent conditions. 4. FDA's 2019 Substantial Evidence of Effectiveness Draft Guidance says that "certain situations, such as when a disease is rare or the disease is life-threatening or severely debilitating with an unmet medical need, may warrant additional flexibility." SCA3 certainly meets all of these criteria, where: • there is a limited patient population who can enroll in clinical trials; • there is a slow disease progression of the disease making it harder to demonstrate slowing; • there is a lack of well-established clinical endpoints or biomarkers, adding variability or noise to the assessments. 5. FDA precedent for reviewing applications for drugs that did not meet their primary clinical endpoint already exists for Tofersen, a drug for amyotrophic lateral sclerosis. Why should ALS sufferers be more important to FDA than Ataxia sufferers? Please grant the same regulatory flexibility for my disease that you have already given to other rare neurodegenerative and genetic conditions such as Duchenne muscular dystrophy (DMD), Batten disease, MPS and ALS. 15 years of waiting is more than enough!
Erika L.	I'm an occupational therapist, so I work with patients with ataxia. These drugs could benefit their progress in therapy. Additionally and closer to my heart, a friend and coworker of mine has ataxia and would like to have access to these possibly life elongating meds before it's too late. Thank you for considering this input.
Catherine M.	I believe that FDA regulations should be flexible enough to support reviewing the data from troriluzole trials. One of my dearest friends inherited SCA from her mother and is now facing life in a wheelchair. She clings desperately to the limited muscular function and speech she still has. I am intimately familiar with her limitations because I take her to a Pilates class twice a week with her wheelchair where she attempts to use her diminishing strength to the best of her ability. This breaks my heart. In addition she has two adult children who have not been tested for the genetic abnormality. Her neurologist suggested that they not be tested unless there is some potential treatment that could help them avoid the condition if they are diagnosed as carriers. If there is ANY chance that this or similar drug treatments could work to diminish the impact of this progressive disease, it would be too late for my friend but could dramatically impact the future lives of her children.
Naomi F.	Living with SCA3 for the past 12+ years, I was discouraged reading about the FDA's decision not to review the NDA for TRORILUZOLE as a potential treatment for SCA3.
Robert H.	Please review troriluzole. It's definitely worth a chance. All you would need to do is come into my house and see what it's like all day caring for my dad.

NAME	COMMENT
Rebecca G.	I am currently a pharmacy graduate intern who is currently living with my family at home. My mother was diagnosed with SCA3 almost 10 years ago and she is currently enrolled in the study by Biohaven regarding troriluzole. Once my parents realized that my mother had SCA3, my fraternal twin sister and I were also tested. While I was negative for the disease, my twin sister was not. With there being no treatment or cure for SCA, I do not understand why a "Refuse to File" was issued by the FDA without reviewing the full data from the study. Any medication or treatment should be looked at and completely analyzed if it has any potential at all to slow down the progression of this rare disease. I understand that medications do need to go through a comprehensive process before being approved, and that is for the safety for all patients, but in a group of patients that do not have any other options and will only continue to feel the symptoms and progression of their disease as time goes on, I believe that this subgroup of patients should at least be given the chance or choice to take the medication. If the medication truly is not effective, then let the NDA be denied. But if there is any, even minuscule chance that it can help these patients who have such an unmet need, I implore the FDA to please review the NDA and research submitted. Thank you.
Mary C.	Since there is no current approved medication for SCA, I have found a medication approved for MS to be helpful. I would encourage you to allow Biohaven to continue with their clinical trial of troriluzole so that I can see if there is more positive affect on my SCA than the medication I am currently prescribed off label. I am willing to accept responsibility and plead with you to review the NDA for troriluzole.
John O.	I'm 56. My symptoms started when I was 27. So I've been dealing with this disease for 29 years. Over the years, I've been to 3 neurologists and the 3rd one at 49 years of age, finally diagnosed me…I didn't like the debilitating diagnosis, but at least I new what I was up against. Please FDA, review the NDA in its entirety. If I was 27 again and if this troriluzole, I definitely believe my life would be different for the better.
Karen W.	I watched my grandfather, father, aunt and several people in my family struggle to live with SCA3; and in the cases of my grandfather, aunt and father, this rare debilitating condition did not yet even have a name. Now, it's my turn and my brother's turn. We struggle daily, trying to stay somewhat independent. To those making this decision of refusal, maybe you should experience what little hope you have being ripped away, after decades of watching others in your family and even yourself struggle for decades. It is a perfect example of bureaucratic inflexibility. In doing so, you have reduced all SCA3 patients to nothing but insignificant statistics. But, keep in mind, we are, in reality, living and breathing sons, daughters, aunties, uncles, mothers and fathers, who struggle every day and now know no hope of potential treatment.
Michael M.	I am fortunate that the FDA recently approved a drug to treat Friedreich's Ataxia and I hope the FDA gives flexibility in all drug trials if there is a glimmer of hope that the drug may provide some benefit to patients, even though those improvements may be slight.
Amanda D.	As someone who has lost a dear friend to a rare disease, I saw firsthand how her and her family had to deal with there being little they could do as the disease slowly took away her physical, mental, and basic living abilities, until it ultimately took her life. They searched far and wide for anything that could slow or stall the progression of the disease and would've gladly participated in any proven or experimental treatment had there been one available. I believe that anything with the potential to even incrementally slow the progression of a rare disease or lessen symptoms should at least be reviewed and assessed. I implore the FDA to at least consider implementing the maximum regulatory flexibility when determining whether or not to file Biohaven's New Drug Application. Given the challenges involved with developing a treatment for a rare disease, I feel that thoroughly reviewing and carefully considering the totality of the data, not just the trial outcomes but also patient experiences and preferences, is necessary in this particular case. Please reconsider filing the NDA request, on behalf of those living with SCA3 and their loved ones who are avidly seeking out a treatment that could potentially help alleviate, stall, or reverse the disease or symptoms in any way. Respectfully submitted.
Dawn N.	We are overdue for some treatment for SCA3. Troriluzole provided hope for a community that has had no leads for a treatment for 30 years! Three generations of my family have suffered so far with no treatment. Please consider reviewing this drug so that we may have some relief to this debilitating degenerative condition! It is cruel to allow thousands of people and their families to continue to suffer.
Rachel S.	I am a 51 year old woman living with Spinocerebellar Ataxia 3, a rare disease. In the last few years I have had two major falls resulting in shattered bones, surgery and pain for the rest of my life. My mom, my cousin and I deserve a chance to try a drug that could help with our balance, vision loss, swallowing, choking and memory loss among others. Please reconsider your refusal of troriluzole. Thank you.
Susan A.	The FDA needs to realize that the different forms of ataxia are very rare and there aren't many options that work to improve the condition or help patients function better. This drug should be reviewed because of the potential to help a person who is living with debilitating symptoms and limitations that have a form of ataxia that could respond to this drug.

NAME	COMMENT
Stephanie A.	As a SCA sufferer the development of troriluzole offers a glimmer of hope for the future. I watched my own mother wither away in a wheelchair in her 60s. Now 25 years on I'm the one withering away with no promise of a drug that will halt progression or eradicate completely. It would be good to know that there could be a drug that improves the lives of my own children. Please reconsider your decision and review the full data.
Marilyn P.	My father was diagnosed almost 30 years ago with Ataxia now I am newly diagnosed with the same disease at 53!! There has not been hope for a cure or at least a drug for the slow down of the progression until recently. This glimmer of hope is what keeps us in the Ataxia community going! We can only do so much exercise, eating right, watching our stress levels, going to PT, doing anything to help ourselves holistically! We need the help of the FDA to revisit the new drug application for troriluzole now!! It takes years for another clinical trial to start up again and we are running out of time!! What is it going to take for the FDA to see that this could help to change many lives!! There could be crossover with troriluzole for other diseases!! Why wait? Quality of life and dignity are all that we have left. I beg and plead with the FDA to revisit the application for approval for troriluzole. I want to still be independent and enjoy life while I can. I dare think if my niece or nephew inherit this awful disease. We have to act fast to save the next generation. Please help us and stop ignoring these rare diseases. We don't need another arthritis drug! Please please reconsider and re-evaluate your decision for the ataxia community. This could be life changing. Thank you!! Marilyn
David P.	My son has Ataxia and there is no cure. If anything can be done to ease his suffering, it is something that I will do. I am a vet, a former US Air Force fighter pilot, his mother is a former US Air Force nurse. We gave the best, most production years of our lives to the service of the US Government. I think your consideration in this matter is needed and I hope that you feel incentivized to pursue it. People with Ataxia have little hope, this is something, please don't deny it.
Brigit P.	I would like this drug reviewed as it gives some hope to those of us living with this disease today.
Connie M.	Ataxia is an extremely debilitating disease/condition that deserves every chance to be cured OR treated like any other disease would be. Please help us. What I am living with you certainly would not want. This is hereditary. I had no choice. You DO have a choice to look into this and you CAN APPROVE this to go forward for those of us that need it. Please!!!
Laura Elizabeth A.	I have had to watch countless family members and my own mother deteriorate past the point of even being able to communicate with, and I have tested positive for the gene repeats that cause SCA3. It is incredibly scary knowing what's coming for me and extremely frustrating knowing that my own mother can't get the treatment she needs so that I can have her in my life for a reasonable amount of time. My mother and I would be among the first to reach out for treatment with this drug, and we are willing to accept the consequences of that, if it means even a chance that we can live normally. Thank you for your time, and I hope this encourages you to reconsider your stance.
Dr. Surinder A.	SCA3 is a slowly progressive and debilitating condition. Any new treatment will be slow to show any benefits. I urge the FDA to review their recent decision regarding troriluzole especially if it fits under my professions 'prime directive' : primum non nocere.
Stephanie W.	My little brother is one of the individuals with this rare disease (Ataxia). Seeing his body and physicality change before my eyes everyday that i see him brings me pain and sadness and even though we don't know what the cure is or what will work, i want him to have a chance to try anything and everything he possibly can before his progression takes over his entire life and changes him forever. I beg of the FDA to please allow him and many others a fighting chance, by giving them the ability to trial whatever might be out there - i.e., troriluzole.
Dr. Dinesh S.	My wife suffers from SCA3. It is a debilitating disease and so depressing to see such a vibrant person slowly go downhill in front of our eyes for no fault of hers. My wife was a research subject of the troriluzole study. As a physician and her husband, I noticed subtle improvements in her condition and we knew she got the drug both in the randomized phase and the open label phase. I strongly believe it slowed the progression of her condition somewhat. Subsequently, she participated in the Phase I Biogen study for the intrathecal injection of an ASO. As a physician and a PI on several trials, I understand the risks of a Phase 1 study and that too for an agent being injected intrathecally. The fact that she participated in the study on my encouragement, should convey to you the desperation we have to find a solution to slow the progression of her disease that we are willing to take any risks and accept uncertainties as we have no other options. I hope the FDA makes their decisions by placing themselves in our shoes and understand the desperation we have to find solutions, no matter how small they may appear.
Andre B.	FDA needs to respect the desire of patients try to test new drugs and try new methods.

NAME	COMMENT
Amy K.	We have watched my husband's grandmother, uncle, aunt, mother, and 2 cousins die horribly slow deaths from this disease. Losing the ability to walk, coordinating simple movements such as writing, eating, bathing independently and finally the ability to communicate- all while trapped in their bodies. Any medical innovations and treatments NEED to be seriously looked into and in my opinion, be given access to patients and families of SCA3. This group of individuals deserves the opportunity and access to be part of the 40% improvement group. SCA3 takes people down slowly, without any hope. The FDA needs to carefully consider of the totality of the data, taking into account patient experiences and preferences, which is deserved to this group of individuals with this rare disease- along with their families and friends, who also suffer with them. If the FDA allowed this drug to be available to this group, it would not only have a positive impact on at least 40% of the 5,000 individuals currently with SCA3, it could also lead to more medical finds to help those who have yet to be diagnosed. If this drug really does positively impact 40% of the current population- that would improve at least 2,000 peoples quality of life. How can you deny that many people the opportunity to have a better life and future, and live longer with dignity. The FDA's job is to look into this, and in my opinion allow this drug to be available. The FDA's own policies say so. FDA's 2019 Substantial Evidence of Effectiveness Draft Guidance says that "certain situations, such as when a disease is rare or the disease is life-threatening or severely debilitating with an unmet medical need, may warrant additional flexibility." SCA3 certainly meets all three of these criteria. Please consider the patients, families and future generations. SCA3 families need hope that the FDA sees us and is on board to helping, we are asking for the same consideration in regulatory flexibility that has been given to other rare neurodegenerative and genetic conditions, such as ALS, Duchenne muscular dystrophy (DMD), Batten disease, and MPS.
Karen H.	My father has been waiting more than 25 years for something — anything — that may mitigate some of the impacts of SCA3. I have been waiting 15 years since my own diagnosis. I face more risks associated with the daily management of this disease than I would face taking troriluzole. Please do not let perfection here be the enemy of good — even a marginal treatment benefit is better than having no treatment options at all. As a community, we are frustrated at the lack of progress in drug therapies. The FDA must recognize the needs of orphan disease sufferers and help us. Other companies will see this set back and be dis-incentivized to research drug therapies for ataxia. This drug shows promise to SCA3 sufferers — no it is not perfect, but it is something! I urge you to reconsider Biohaven's application.
Sandra S.	I would like FDA to reconsider allowing a new review of this new drug that brings hope to people who suffer with this terrific condition. It is to sad to see our beloved friend evolving Ataxia with no hope new efforts are done to address it.
Roman H.	Well since I was diagnosed with SCA3 back in January 2021 it has caused my military career to be cut short. I mean I absolutely love what I do but since my diagnosis I have become more isolated and very depressed. I feel that the FDA should consider reviewing the NDA as the troriluzole for a potential treatment for SCA3. My mother passed away from causes related to SCA3 in 2017 and my older brother Jeremiah is currently in a wheelchair fighting SCA3. I beg the FDA review the NDA because it's only fair to the ones living and fighting this life-changing disease!
Jermaine F.	I think that FDA should consider that a rare disease has just as a profound effect on an individual an their family as any well known debilitating disease. They would give anything to have a chance at a cure or a treatment. It's a helpless feeling seeing someone deteriorate and know there's nothing that can be done. Let people have a chance, have some hope. Let us have this drug.
Celia M.	All SCA victims, of every type, deserve the chance for every possible treatment available to persons with this dreaded disease.
Carmen M.	I watched my grandmother, aunt, uncle, and mother all suffer from this disease. I have watched them deal with the varying symptoms to the best of their ability all the while getting progressively worse and actively trying to still the lives they were used to leading. As we all know from this disease, that is not possible. Major life altering accommodations have to be made, sacrifices must be given, and acceptance must be had. Of course, I would do it all over again to have my loved ones in my life. It pains me when my mother apologizes for being a "burden," when we take her to the grocery store. We reiterate that her spending time with us, makes it worth it, never is an apology needed. I'm not looking at this for my own benefit, although SCA3, I am sure is my future as well, I am hoping and praying the FDA is willing to review the New Drug Application for people like my mom, my uncle, my aunt, my grandmother. People who want to be a part of their families in a way that they don't feel is a "burden," to anyone. Seeing my 3 loved ones go from fully able bodied individuals to completely wheelchair bound over time is heartbreaking for me, but I can only imagine for them. It is most likely my future, which I am hopeful that I can be half as strong as them to continue on despite the challenges. It seems so simple to me, if there is something out there, that may do some good for people who have experienced so much pain, why wouldn't we consider it?

NAME	COMMENT
Sofia V.	Generations are passing and we assist to so many of our relatives being affected, suffering and dying at young ages because of this terrible disease… my dad was so healthy and strong until the moment he started with symptoms…everything changed and he suffered so much… you cannot imagine the pain… if we had a therapy/drugs for this disease he would had lived so much more years, so many things he planned to do and instead he just suffered until he passed away. Please, please, please review the new drug application as a potential treatment for SCA3. Our future is in your hands, PLEASE HELP US!!!
Leonard N.	The devastating effects of living with the disease is indisputable. The FDA must exercise flexibility. If there is any hope that SCA3 can be treated with a newly developed drug and provide sufferers with a glimmer of hope and relief, then it must be considered — simple!
Jamie W.	Please consider reviewing the decision. There is so little research and hope for people living with this illness. The chance to try this would make such a difference to peoples lives.
David S.	Being in the 6th generation of a family which has been plagued by this horribly debilitating disease, whose Mom (2014) and Brother (2017) passed away, think the FDA has to be open to new drugs for not only SCA3, but all the related ataxias. Them both having donated their brains and organs to the research by Dr. Vik Kuhrana in Boston. I currently have been taking Riluzole for at least 6 years, a drug founded in Parkinson's research, can only profess how slowly my disease has progressed since being officially diagnosed in Jan . 2014 at age 51. (Prior signs at age 47/48). Presently at age 61, I'm still able to care for myself and maintain a relatively normal life. Not saying I haven't progressed but at such a slow rate I currently only use a walking stick to get around and still able to spend my winters in Mexico living alone. Please reconsider for all of us with Hope. Enough is Enough !! Silva Ataxia Foundation's mantra. Thank you.
Fred P.	I have been living with Ataxia for more than 13 years and I would love to have a drug to try. Each year that passes the Ataxia gets worse and worse.
John I.	I have Acquired Ataxia and am therefore dubious of any benefits.
Rita C.	In my opinion, the research should continue and the tests should be more flexible. And even if the results are different from the original aim and only part of the individuals with ataxia are benefited, it would be a huge improvement in their condition and it would mean a fairly considerably number of people being benefited.
Laraine M.	Rare diseases like SCA3 should be given additional considerations or flexibility regarding trials. Because it is a rare disease, the number of participants in any trial will be much more limited compared to more common diseases. Ataxia patients have the right to be heard and treated fairly. By issuing a Refuse to File letter to Biohaven, you have taken away the only hope for a drug that may help with the progression of this relentless disease. I ask that you reconsider your decision and review the full data presented in the Biohaven NDA filing for troriluzole.
Janice C.	I am not a medical expert but I believe Ataxia patient should have the opportunity to see if these drugs might help them! (Knowing that the drug didn't reach the level of approval) It should be given the same flexibility that are assigned to other drugs. I respect findings of the FDA but would like to see them reconsider!
Charles W.	A dear friend has been suffering from SCA8 for past few years and is progressively declining in her motor skills, balance and no longer able to live a life that is fulfilling. She and her husband are acutely aware of the risks entailed and would administer troriluzole in a heartbeat. Please speed the process of approval, understanding the risks and that the drug may or may not be of help.
Ahna J.	As a 56 year old Woman diagnosed in 2017 with SCA3 I would like the opportunity to have the choice of trying troriluzole as a potential treatment. If it is able to help with my day to day struggles with my symptoms. Since there has been limited medical help or knowledge to deal with this disorder, I am anxious to have the opportunity to try this new treatment. Nothing could be worse than the hell I have to face daily. I have seen my mother, aunts, and uncle die due to this debilitating disease. I believe in my heart that there would be some sort of advancement, in finding a cure or some relief discovered in my lifetime. As a mother and a grandmother I hope to see advancement in research for a cure. I would hate to see the approval of the FDA, put this in jeopardy. I am positive that I speak for myself and many people with advanced SCA3, that we would happily be the treatment trial group for such positive advancement for a cure.
Connie B.	As a patient who suffers with this debilitating disease and knowing the hopeful findings for possible help from troriluzole, I plead for the FDA to review the application for use of this drug along with regulatory flexibility to give a chance for some relief from this devastating disorder. Thank you so much for your consideration.

NAME	COMMENT
Charmaine N.	I urge you to reconsider your decision and approve the drug for the treatment of SCA3. This drug has the potential to give SCA3 patients a chance in life, and I believe that it is the right thing to do.
Karen L.	By not reviewing the NDA, the FDA is taking away hope from those affected with SCA3. As an individual with Friedreich's Ataxia, I can't emphasize enough how important hope is to mental well-being. I have been living with FA for almost 30 years. Within the last six months, the first treatment for FA received FDA approval. SKYCLARYS is not a cure but any treatment to mitigate the symptoms and/or halt or slow progression is welcome. The drug has renewed my hope that a cure may be found in my lifetime. When I was first diagnosed I was very optimistic that a cure would be found. But as the years passed and the disease progressed, FA relentlessly robbed me of the ability to stand, walk, to write legibly and caused crippling fatigue, my hope for a cure dissipated. Bringing a new drug to market for the treatment of a rare disease is extremely challenging as the NAF has discussed in its statement. To deny a full review of troriluzole, robs those individuals afflicted with SCA3 of hope.
Rita G.	While I am a person living Ataxia, I have not been diagnosed with SCA3 but I have many friends who do have this particular type of Ataxia. I feel that they could possibly benefit from this research and deserve a chance of taking troriluzole as a possible treatment. It is URGENT that the FDA accept the NDA filing for a full review of this drug.
Richard L.	I have already written the FDA with my comments. When people are willing to take a chance with their lives for the long term benefit of others, the FDA should surely approve this new drug application.
Mark M.	SCA3 runs in my family. I saw my grandmother's decline, and saw very closely my father's more acute decline that led to an early death. Now two of my uncles are struggling with the disease and myself, my sisters, and several of my cousins are living with the difficulty of watching our loved ones decline while being fearful of that 50% chance we each have that we will also have to live with this progressive disease with no available treatment. With the certainty of this decline, my family is searching for any hope of treatment and is more than willing to accept some uncertainty in available treatment. It is deeply disheartening that the FDA will not review this drug that provides some meaningful hope for people living with SCA3 and I urge the FDA to reconsider.
Holly	The FDA must not have make decisions based on need and suffering. This disease is debilitating for the person who has it and those who know them. There is nothing that can be done as these people continue to suffer. Please help them by pursuing this drug that has a chance to help.
Jenna P.	All avenues should absolutely be explored to treat this debilitating disease. I have seen firsthand what this disease can do to someone and the effects it has on family and loved ones. From the look of it, there is no reasonable explanation not to review the NDA for this potential treatment.
Cinda L.	Each day, my brother-in-law faces more difficulty living with SCA3. This is the future that my niece faces. Please reconsider your decision regarding troriluzole urgently, while it still has the potential to help people who may never have the opportunity to try ANY treatment.
Stephanie L.	People with SCA3 have been told for too long that "there is nothing you can do." We need the FDA to be certain that this potential treatment option is not effective and offers no improvements to the lives of people living with SCA3 before it is rejected completely. The ONLY way that the FDA can be confident in their decision to take this potential treatment option away from patients is to accept the NDA filing for troriluzole and offer a complete review of the scientific data. The SCA3 community has no alternative options. We cannot accept outright rejection of our FIRST potential treatment without reviewing the entirety of its potential merits. We NEED the expertise of the FDA to weigh in on this treatment so that we know for sure if this drug could be of benefit to some.
Pedro A.	My mother has been living with SCA3 for the past 20 years. Knowing that there's a therapy that has shown that works in slowing down the progress of the disease, it gives me and my sister hope that our lives won't be as hard as our mother's live has been so far. It's all about giving us and countless others hope of living a normal and healthy life. Please take a look at the data regarding this new therapy for SCA3.
Holger W.	A former member of my team is suffering from SCA3 and it has dramatically impacted her life. I do think she should have the ability to choose if the risk of more experimental drugs is something she wants to take vs. having that decision made for her.
Kathryn C.	Troriluzole was found to have promise for treatment of persons with SCA3. Further assessment is needed and there is no good reason to forbid this. Please reconsider.
Rinaldo O.	I'm disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory process from FDA for rare diseases like ataxias should be more flexible.

NAME	COMMENT
Robert C.	Any new medicines that can help SCA should be available to the patient.
Mary S.	Those of us with SCA are driven to find any possible solutions to prolong our quality of life - and to delay the ever-worsening symptoms of this disease! I have SCA6, not SCA3, but the symptoms are similar. My father had SCA (deceased 2019), and I have five current relatives diagnosed with SCA. We are all seeking ways to preserve our abilities so we can live as fully as possible. In our subset group alone, we have business owners, consultants, teachers, musicians, and all love our families and communities. We still have life to live and contribute, and this disease slowly robs us of our abilities. Please apply regulatory flexibility and accept the NDA filing for troriluzole for a full review. Our community deserves this consideration which may be able to help some of us improve our quality of life. My personal sincere hope is that research will continue, and at some point a cure will be found. Thank you.
Martin C.	My wife was part of this trial. There was improvement for a short time for her and no side effects. More importantly it gave her some hope. Those with SCA Type3 deserve the chance for any help. Senator's Casey and Rand petitioned the FDA to expedite drug designation for certain medications to no avail. Please reconsider your decision. Our loved ones deserve better.
Cherlyn W.	Having seen how it affected my friend's life from having their parent go through this AND knowing that they will also be going through it AND that it will be passed on to their children (who will watch their parent suffer, just as my friend watched their parent go through this) with no treatments, cure or new technologies or medical breakthroughs and when there is one, the abrupt nature in stopping any trials or further groundbreaking treatments to come to fruition is not only heartbreaking it's shameful.
Darshan N.	It's my humble request to FDA to review the medicine for SCA3. My health is getting deteriorated day by day. I am getting scared of my body becoming immobile, everyday. It's my sincere request to FDA to review the medicine for SCA3 immediately. Please...
Patricia L.	My husband was diagnosed with SCA3 in the spring of 2010, a few months before our daughter was born. This disease is relentless! The progression makes daily life so hard for my husband now, and our daughter is showing very early symptoms (nystagmus). She's only 13 years old. It saddens me so incredibly much to read that the FDA won't consider this treatment. Imagine if the president of the FDA had this disease. Would he/she not want to have a treatment? It feels really unkind to not consider something that might make a difference to families like mine. I'm extremely disappointed. I hope that a full review and consideration will be made.
Lorna M.	It is my belief that the FDA needs to do a full review on everything leading up to the application for a new drug as a potential treatment for spinocerebellar ataxia 3. Even though it's very rare disease, it should not discount the FDA's weight of importance placed upon it. As it does affect severely those who have it. I have a 50% chance of contracting this, as it is genetic. My mother has suffered greatly, and she is so interested in finding a cure that she is donating her brain to research once she passes. Please consider all avenues.
Darlene T.	As a member of a family with four members affected by Cerebellar Ataxia, I feel we not only want, but NEED to have some treatments that look hopeful in clinical trial to SCA3 specially be made available through the FDA Regulatory Flexibility. The individuals affected by Cerebellar Ataxia's deserve consideration in the decision.
Amy M.	As someone who works in regulatory affairs in the medical device industry and often reads news on regulatory approvals of drugs and devices, it is not uncommon for a product to be approved that did not meet it's primary endpoint. Often times, post-hoc analyses and other subgroup analyses are submitted if the data is found to support a particular subgroup outside of the original intended patient population. Additionally, FDA can and does work with the sponsor to determine the appropriate indication for the product based on the data analyses and what the data best supports. Rare diseases and small patient populations can take many years to collect data on a product. Due to the difficulty of this and the long duration of getting this data, it would be prudent to review a reasonable proposal for the data supporting a different intended patient population.
Maria Christine C.	This has to be reviewed! Close family friend with ataxia and need this drug to be reviewed! Please. This disease is dreadful!
Stephan N.	The impact of SCA3 on the quality of life of the patient and their caregiver/family is tremendous. The individual variability of responses to the few existing molecules to control for some symptoms used for other disease is also very large. Any new drug can have a significant impact on the quality of life and flexibility in reviewing new ones is critical to advance our comprehension of SCA3.
Annette E.	I have SCA3 and am noticing progressively worsening symptoms. I would like the FDA to thoroughly review data regarding potential treatments.

NAME	COMMENT
Anne M.	I have SCA3 and have been monitoring very closely this drug trial. I believe that this drug has demonstrated sufficient proof that it can be effective for patients. Troriluzole is an improved uptake version of a drug that can already be prescribed — riluzole. Please consider those who can benefit from this treatment and review the New Drug Application for troriluzole and a potential treatment for SCA3.
Dr. Patricia G.	In all of the clinical trials that I have led for treatment of SCA, I have witnessed stability in the natural history of this inexorable disease with troriluzole. That stability has continued for at least 2–3 years. I have patients still walking, (on troriluzole) when by all predictors, they should be in wheelchairs. We did not see this with Chantix or TMS in prior trials. I do not understand the logic of the FDA who are quick to recommend a treatment for Alzheimer's disease with a risky side-effect profile and little benefit vs. a safe drug and stability. No, troriluzole does not reverse the cerebellar injury, but it does keep patients very stable. The drug has an outstanding safety profile.
Jonathan M.	I have been diagnosed with SCA6. While not as aggressive as SCA3, the prospect of not being able to treat the ataxia I've been diagnosed with can be frustrating. I know personally, I'd be willing to subject myself to riskier treatments if there was a good chance I'd be able to be there for my family better. Please reconsider your decision.
Robert N.	This is such an aggressive, debilitating and mentally depressing disease to go through. There is no cure and its effects on the individual, family members and those providing care are numerous. It would be so wonderful to have this drug available if even a portion of the patients reacted positive to this treatment. In the situation that this disease creates it couldn't possible make things any worse. Currently people affected by this have no hope or relief from the effects of SCA3. Please reconsider your decision for this new drug application for troriluzole as a potential treatment for SCA3. Thank you.

"There currently is no form of treatment for my son's rare ataxia. AOA2 has robbed him of balance, motor skills and the ability to live independently. He and others deserve for the FDA to fully review this first possible treatment and glimmer of hope for improved quality of life. Please give the drug your full review."

— Rita A.

NAME	COMMENT
Barbara S.	Recently I have been diagnosed with a genetic form of ataxia. My oldest brother and 4 cousins have also been diagnosed with this rare genetic disease. For the past years I have been watching my brother decline. He can no longer walk, his speech is very difficult to understand, he needs assistance with daily personal care and eating, and swallowing is becoming difficult. To know I have this disease and not have a medicine that can be helpful to address this disease is distressing. Now when research is finding a medicine that may be helpful and the FDA is possibly going to make a decision to stop ongoing clinical trials is immensely frustrating and greatly discouraging. As a person who has ataxia I join the National Ataxia Foundation in requesting that the FDA gives maximum regulatory flexibility in the review of data from clinical trials. Please make decisions that make it possible to continue working with troriluzole as a potential treatment for SCA3.
David P.	Strongly demands a comprehensive review.
Robert S.	I was diagnosed with SCA1 about 18 months ago through genetic testing after experiencing symptoms. My father and uncle died of SCA1 about 20 years ago. Currently I have a sister and cousin also with the disease. I also have 3 sons who are at risk of the disease. Any attention by the FDA to further the process of getting troriluzole approved would be greatly appreciated to say the least.
Teresa C.	I was on the drug trial for two full rounds and I wasn't sure if it was doing anything, but when I went off it I could tell that it was doing something! I was wanting to go back on it! It definitely slowed my progression down! We need this drug available! It gave me hope!
Maria Carolina T.	Please, review the New Drug Application for troriluzole as a potential treatment for SCA3! Having this disease is so sad and troriluzole might be a light at the end of the tunnel. Please, help!
Derrick W.	As a person living with ataxia, please consider submitting it. Truthfully having all your future plans for life, retirement and celebrating birthdays with future grand kids. When all that is wiped out, knowing I'll never get to do those things is hard. If this drug would help slow the symptoms, why not approve it for review. It's not your quality of life that sucks it's others.
Elaine D.	Please reconsider to a full review of troriluzole. Those with SCA3 are suffering with a progressive disease of the brain. They lose the balance and coordination to walk and talk among other things. Thanks for listening!
Janetta M.	I am a participant of the clinical study. I have SCA6. I knew going into this that it may not make a difference to me but hopefully it would help someone. I can't accept that the FDA would not take another look at the results of the trial. Even if it only helps part of the ataxia family, we all are so proud to have been a participant in trials. Every day or two, we have a new challenge. You might not be able to roll over in bed. You can have a never ending cough. We can choke on air. Lack of balance is something we all deal with. We go from furniture walking to a cane to a cane or walker to a wheelchair. That is only part of the story. The remaining part is about how isolated we become. Because we have speech that progressively is taken from us. We don't answer the phone because people can't understand what we're saying. Our writing is illegible. We are isolated because of our inability to do so many things. If this drug helps one person feel a little bit better, I will be so happy for them. Please give this drug a chance. It gives us hope.
Ron L.	This review should be done. It will open the door for more research and assistance to those affected by ataxia. It is not a high profile condition, but the opportunity to improve the life of those with ataxia needs to be considered. Please review your decision.
Rachel K.	This is a horrible debilitating disease. I think the time to go back to starting would be devastating. Please FDA review this with regulatory flexibility!
Matt S.	Why would you not review anything in this world including a drug that may or may not be a treatment for SCA3? Ataxia is a life ruiner for me as a father of young children I can't do the things I always drempt of doing with my children. Any chance that could help me achieve that I would.
Charles D.	Living with Ataxia is very frustrating. The FDA needs to look into this new drug now and hopefully approve it.
Ann C.	My sister-in-law has SCA3 and was very disappointed to hear the FDA's decision. As a mom she has two children she needs to look after and would want to stay healthy as they grow into adults. Taking this choice away from her and the community is the wrong thing to do. I strongly recommend that the FDA reviews this new drug as every solution must be explored, even though there may be risk the reward is much greater.
Antoine D.	The SCA3 community deserves troriluzole to be considered for a path forward even if the primary endpoint has not been reached in the trial. At this point in time, without a disease-modifying treatment available, any benefit is substantial.

NAME	COMMENT
Denis D.	I am stunned at the decision of the FDA not to review the New Drug Application for troriluzole as a potential treatment for SCA3. We need the ability to go forward with a treatment for SCAs. Living with Ataxia that has no treatment or way forward is unacceptable. We've prayed for anything for over 30 years, please reconsider the decision and give us a possibility.
Joanne H.	The FDA's refusal to accept the NDA filing for full review of the potential treatment for SCA3 wipes out the hope that people like my son hold onto in believing that the possibility of a better quality of life could be. Living with Ataxia is hard enough. The thought of having no hope for so many is unacceptable especially when the possibility of what troriluzole as a potential treatment means. Having lost two grandchildren to SCA7 and having a son who is on the deteriorating decline of this horrific disease I find it appalling and heartbreaking that consideration of the review is being dismissed. Give people with SCA3 the hope they deserve and the other Ataxia's a glimmer of hope of what a treatment could be for them also.
Jamie H.	I'm currently taking troriluzole from Biohaven Pharmaceuticals in my Clinical Trial. Doing something is better than doing nothing! I'm so very thankful for research. The FDA needs to get on board!
Anonymous	I write today not only as a clinician but as an individual with a confirmed diagnosis of SCA3. As both a patient and clinician, I was dismayed to learn of FDA's recent decision not to give troriluzole any regulatory review due to clinical data showing it failed to meet primary endpoints among all the SCA types included in the study despite the post hoc review showing efficacy for SCA3 patients. I write not only in support of a full regulatory review for troriluzole but to address very important issues with FDA concerning the agency's review of drugs for rare neurological diseases to ensure that FDA establishes helpful and prudent practices concerning future drug review for SCA3 or any other SCA. The fact that the clinical study failed to meet primary endpoints for all SCAs is not a good reason to decline review for many reasons. First, it failed to meet primary endpoints for all SCAs included in Phase I of the clinical study, an outcome that is not necessarily determinative with respect to efficacy for SCA3 as there are important distinctions between all the SCAs. I'm sure Biohaven can provide important education to regulatory reviewers on this point much better than I can. This is not a sufficient basis not to sit down with the data at all to determine whether approval of the drug is appropriate. Second, this drug has been proven to be absolutely safe. This is also the first drug ever submitted for approval for a condition that has no other approved therapy. Therefore, we have a drug that a pharmaceutical company claims to have clinical data as to its efficacy for a condition which has no other treatment and it is known to be safe. What good reason could there be not to give it it's due regulatory review? FDA cannot ignore its duty to provide swift, diligent and thorough regulatory review of proposed therapies for serious and progressive conditions such as SCA3 without any available treatment. Perhaps if the therapeutic options for SCA3 were ample, the FDA could take this position. But to the contrary, the opposite is true. Again, my comments are not aimed at influencing FDA's decision regarding whether approval of troriluzole is appropriate. I trust the talented and hardworking public servants at the agency have the requisite skill, knowledge and experience to undertake a thorough and fair review of the clinical data. Instead, my comments are aimed at ensuring that FDA gives proper consideration of a drug that aims to treat a condition with unmet medical need. It is also about setting the landscape for future drug reviews. It is for these reasons that I wholeheartedly support NAF's statement that the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review. Frankly, it is FDA's duty to do so.
Jodi R.	I have seen firsthand the debilitating nature of SCA3 among several extended family members, and it is heartbreaking that more research is not being done to help those who deal with this affliction. Please consider reviewing the New Drug Application for troriluzole, so that those who live with SCA3 may have hope for treatment in the future.
Owen H.	My mother has been living with SCA3 for over 20 years. We have been looking forward to a medication that could slow down progression and possibly reverse it. There was great hope for troriluzole but we were disappointed by the news that the FDA declined the NDA. We hope the FDA will reconsider and look to see if this drug can be used for SCA3. We hope it wasn't thrown out due to the lack of results found for other SCA types, since we heard it did seem to help with patients with the SCA3 subtype. We hope that at a minimum the FDA can approve troriluzole for SCA3.
Dr. Ebrahim A.	I wish for the FDA team to be more logical and explain to NAF members why they did not accept the application before reviewing the data. Thank you.
Kenneth H.	I have watched my mother and other near relatives succumb to the ravages of SCA3 and am fully aware of what I am facing. It is unfair of the FDA to not give a promising treatment, even one that may not deliver all that is hoped, a chance for approval. Please reconsider your decision to not file.
Samuel D.	This new drug application for troriluzole is an urgent issue for people who are experiencing this slow debilitating disease.

NAME	COMMENT
Patrick M.	I am a care provider for my spouse who has SCA3. I feel the FDA decision to not review the New Drug Application is a travesty to individuals suffering with SCA3. If medical research, identified possible medications and treatments that can delay or cure this disease the FDA should review it and continue the vetting process. As I watch my spouse lose functionality in her limbs, I can only be amazed and feel sadden that the FDA would withhold medication that could improve her quality of life.
Sharon R.	I have cerebellar ataxia and I know how frightening the uncertainty of this disease is. I have a friend with SCA3; two of her family members have died from it. She is fighting hard but is terrified of what lies ahead and wants desperately to try a medicine to stop disease progression. Please give her and our community this chance.
Barbara M.	My husband was diagnosed in 2001. The disease has slowly progressed to where he is now housebound and in a wheelchair. This year my daughter has been diagnosed. Seeing her father's decline has made her willing to try all remedies available to her. These treatments provide hope — please reconsider your decision.
Donna M.	I urge you to reconsider your decision and review the application for the new drug as it could be so beneficial and improve the lives of those suffering now and for those in the future.
Jill H.	We ARE rare and we deserve to live the best life we possibly can regardless of physical ability. I'm more than willing to give any treatment the good old college try!!!!! This disease strips individuals of their ability to perform even the once simplest of tasks. I remain hopeful for a treatment for the next generation……my daughter is 13 and god forbid she has inherited this awful disease from me, I can only hope that there is something available to at the very least slow progression should she begin to exhibit symptoms. Please hear us!!!!!
Ana V.	I saw my father suffering so much with this terrible disease….and he was so young and with so much pain… he had plenty of years ahead of him if he was not affected by this neurologic disease (Machado Joseph). Unfortunately my father passed away 4 years ago :((now my brother is starting to have the same symptoms, you cannot imagine what we are all suffering assisting to the same process as my father had, without any drug that could help them… for so many years we are waiting for a drug…. Please, please, please support us bringing to life a drug that will bring life to many, many families!!! With love and hope that you will review your decision and support these people in pain…
Stephen P.	At the moment I suffer from a relentlessly progressive disease with no effective treatment. Your decision not to even review the application just removes any hope of any therapeutic options becoming available. Hereditary ataxias are rare diseases and any potential treatments need to be encouraged not dismissed.
Patrick A. Jr.	I served in the US Army for 30+ years retiring in 2017. In 2020 I started experiencing symptoms associated with SCA3. On 4 Aug 23, it was confirmed that I had SCA3 by Veterans Affairs. I now have grandchildren, want to see them grow to productive citizens in our society. I ask the FDA to review the full data of the new drug, troriluzole, to give me and others like me a chance to a better quality of life and the opportunity to see our grandchildren group.
Constanza P.	My husband was diagnosed with SCA3, we have two little girls and for us has been very tough to receive the news. My husband is fearing he will be worse every day and this medicine is the only solution we see in the short term. Please help!! This is our only hope
Duncan Y.	Living with the unabated progression of SCA3 robs an individual of not only the normal functionality of their body, but also of the DIGNITY that they had when they were physically able to perform those seemingly oh-so-simple functions-memories that I relive every day. As one who suffers from this disease, I implore the FDA to show the flexibility of at least reviewing the data included in the troriluzole filing: It is not hyperbole to say that, given the rare nature of this disease and thus the limited pools of study specimens, that troriluzole may for some represent their last opportunity to arrest progression — and thus regain some of that dignity.
Susan B.	SCA is all encompassing. We need help! Do your part and let this application through!!
Linda W.	My first husband, my daughter passed away from Ataxia and my grandson currently has Ataxia. I would surely like a full review done on this drug. We were so hopeful that a drug would be found for my grandson to try. Any little bit of help is better than nothing. For over 50 years we have been praying for a drug to be approved to help this terrible disease. Please be sure this review gets done. If a drug can be found for one type of Ataxia, it will then be easier to find a drug for other Ataxia's. We are desperate for some help!
Melanie P.	There is NOTHING that stops or even seems to help with this horrible disease! No hope! Why not give someone a chance to see if it would benefit their life in any way?!

NAME	COMMENT
Marcia L.	Having SCA3, I believe it is of utmost importance for the FDA to review their findings. This is important for me, my children, and grandchildren. PLEASE HELP US!
Cynthia D.	As a daughter to a SCA3 affected mother, a sister to 3 SCA3 affected siblings and relative to several other affected others, I definitely think the new drug troriluzole should have it's drug application reviewed. It is a horrible, sad ordeal to watch all aspects of their health slowly deteriorate with no help or cure in sight. SCA3 deserves the FDA's review!
Brenda C.	Ataxia can strike anyone. It currently has no treatment and no cure. Although it is rare, you never know when and where it will strike. As a retired special education teacher, I never gave it a thought that I would one day have a disability. Because it is rare, SCA needs to have more flexibility, when deciding to market a possible treatment. I feel it is very warranted to review the drug application for troriluzole for SCA3. Ataxia is a rare and all too forgotten disease
Erika L.	My husband/son's family have many members with ataxia. My very compassionate and giving mother-in-law is doing her very best to maintain a functioning life with this disease, as are her siblings. She deserves as much help with this as possible. On top of that there are many children in the family who may have to deal with the same fate. If there is a chance with those who have it and those who may get it to have a medication that could help them, why wouldn't this be a priority! I feel this definitely should be addressed. Thank you for your consideration.
Kazuharu S.	For rare diseases such as SCA, more care in decision making and full read and understanding of the data, leaving no stone unturned would be appreciated by those suffering from this degenerating disease. Please reconsider.
Rodney T.	I was diagnosed with SCA3 in 2020. Since then my symptoms have went from bad to worst in just 3 years! I can no longer work any job. I haven't worked since December 8th 2021 because of this disease. I am in the process of filling for disability and I am only 43 years of age. I am very aware of this horrific disease and how quickly the symptoms take over. My grandfather died, my mother just passed recently in August 2021. My auntie has SCA3. My brother also suffers from SCA3. I am in a wheelchair, my auntie is in a wheelchair my brother is in wheelchair. If I go on this way I will certainly have no quality of life in the near future. I am in constant pain and I cannot walk. Any drug that might have the possibility to slow down or help with my day-to-day needs would not only be a blessing for people who suffer from SCA3, but it would be a literal life changer for the better. I beg you, please reconsider the application to view the data of troriluzole in its entirety for the betterment of people such as myself that suffers from this unfortunate rare disease. Thank you in advance for your consideration. I have full confidence that you will make the right decision.
John E.	The FDA [at a minimum] should approve use of Biohaven's drug troriluzole for SCA Type 3 patients while simultaneously allowing Biohaven to continue its research. Also, we fail to see any reported negative side effects on the use of troriluzole. If this is the case, then the entire population of Ataxia patients should be allowed to take this drug with the understanding that it remains experimental.
Colleen Z.	A close friend has a degenerative disease that fits into this realm. It is robbing her of function at an age when she should still be enjoying quality of life. It is heartbreaking to see her suffering and the effect it has on her spouse, children and friends. She has told us there is little known about the disease and no treatment currently available. They are willing to try anything at this point and some flexibility in the drug approval process (with careful monitoring) would be so helpful. They need to still have hope and a way to provide data for future trials.
Amalia M.	For people with ataxia, urgency means life. Such a disease doesn't give a break to patient and family and FDA should be aware of that.
Andrew D.	The FDA, as I learned it, should allow any possible treatment to a potentially fatal medical condition. It's a disservice not to allow it. Of course, with a label saying potentially harmful. And, to avoid lawsuits, have the client sign a contract saying he or she is responsible if anything bad happens.
Juliana P.	This should be urgently reviewed for the benefits to lots of patients to improve their life quality and also live better and longer with their jobs and family. My husband´s family has many members with SCA3 and it's sad to see that nothing has been done for them, and they struggle a lot with the limitations this disease imposes on them each year not treated.
Soorya G.	I think it's not right to hold a drug from the public that can potentially help treat this disease. Just because it's not perfect yet doesn't mean people aren't willing to try it out and hopefully treat their symptoms. We are desperate as there is no treatment/cure released and need something to try and help us lead better lives. Please be responsible and help those affected by SCA to help live better lives through treatments and medications.

NAME	COMMENT
Barrett S.	As a hereditary SCA3 patient, as well as watched my mother and grandmother lose so much of their lives, and living with this, I am willing to try something that has had success. As I age, am 64, my progression increases, and as mentioned, am losing valuable time, and function that is irreversible I respectfully would ask to let the patients and Dr that have lived and researched this, and let us make our decision. This is a very cruel disease, and as we see more cases, we need your help. Please consider reviewing the study and allow our team to make the call base on our conditions. Thank you.
Ed G.	I have a friend, a co-worker, who lives with this and I see daily how much he struggles. I hope and pray the FDA takes the appropriate steps in this matter
Linda B.	I watched my mother live and die with SCA3, my brother's life is horribly compromised by SCA3, and my other brother and I are facing the possibility of having this genetic disorder as well. ANY possible medication that could slow down the progress, improve quality of life, or even stop this disease would be so incredible. PLEASE allow the review of this New Drug Application for troriluzole as a potential treatment for SCA3.
Jacob T.	There are few things more frustrating than living with a rare disease and knowing the science for a treatment is out there but not being able to benefit from it with all the red tape and slow movement to pass FDA protocols.
Betina M.	For people living with ataxia SCA3 it"s very important to try new medication in order to slow down the progress of that degenerative disease so FDA MUST review the new drug application for troriluzole as a potential treatment for SCA3.
Corey W.	There is nothing out there to treat or help slow the progression of SCA3, this gives people a chance at least...come on already.
Emily C.	We believe that people with SCA3 deserve the opportunity to have the FDA review the data in full to avoid a false negative conclusion. The well known challenges of running clinical trials for rare conditions, combined with the serious unmet medical need, lead us to believe that the FDA should apply the maximum regulatory flexibility when considering whether to review the NDA.
Jeff W.	Why not review the drug? It could help people.
James G. Sr.	Please reconsider your decision as people such as my wife need hope that something will be found to help them regain a normal life.
Rose K.	Please do a full review of this drug. Anything helps for this disease right now!
Angela J.	I watched my father deteriorate without a diagnosis of his disability. It was emotional torture. My family did not know what SCA3 was until I was diagnosed. I do not want to look forward to following in my dad's steps if there is any chance that a drug could slow progression of this disease. SCA patients deserve grace and assertive measures to find treatment for this horrible disease. Please do a full review of troriluzole for treatment for SCA3. The quality of my life and the lives of others depends on it.
Marcia N.	My husband died of SCA3 and both our children inherited and are now suffering with it. I think it's worth taking a chance to see if the drug does or doesn't help them.
Andrew M.	People: my wife has Ataxia type SCA6. As such, she is not directly affected by your decision. But as a caregiver watching my loved one rapidly deteriorate with a rare condition, I think the broader implications of your decision to refuse to review a previous broader study to assess its potential to benefit people with Ataxia SCA3 is wrong. The people who are affected by SCA3 deserve the chance to have their condition considered by itself in relation to the potential benefits of this drug.
Chenlei	I strongly demand a comprehensive review.
Richard U.	The NAF request is reasonable and fits within the FDA's guidelines. Timing is very important for those SCA3 patients.
Joan E.	I am a retired pharmacist and clinical research regulatory professional with certification in clinical research and IRB regulations. I believe the FDA should follow their own policies on regulatory flexibility for patients with rare diseases. My loved one with Ataxia feels hopeless and helpless and that there isn't enough research support or attention to his debilitating progressive disease.
Sandra M.	Flexibility in FDA evaluation should be utmost in rare and debilitating diseases. If there is any chance it could work please give it a through evaluation.
Jose Luiz R.	They should change their decision.

NAME	COMMENT
Melissa M.	As a person with SCA, everyday is a challenge. Please give us hope for the future by reviewing the application!
Lisa D.	I have a close friend who lost 5 family members to ataxia and is currently battling this disease herself. Please consider reviewing this drug NOW, before it's too late for her and others.
Kathleen M.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Grace K.	I think the FDA should review the NDA. As someone who suffers from SCA, I truly think the FDA should reconsider the thought of reviewing the NDA from the biotech company, Biohaven. Since the FDA did say that the unmet medical need, rare diseases, and disability may warrant additional flexibility, I strongly think that the FDA should review and try to add on to the "cure" or hope that the Biohaven has come up with.
Carol M.	I am afflicted with SCA6 and have participated in Biohaven's trial of riluzole. If any drug would provide any relief from even one or more of my symptoms, I would jump at the chance to try it. Please give the SCA3 sufferers the opportunity by reviewing the data.
Anderson D.	I have SCA3, and it is horrible, I can try new methods as long as we have hope, please FDA, don't take our hope away
Brian T.	I, and my brother have both been diagnosed with SCA. Due to genetic testing costs, we haven't yet been able to determine which type of SCA we have. It would be terrible to think that when we are able to afford to nail down our SCA type, that the FDA hasn't taken advantage of all the info gathered, to decide if a drug MAY help us. In my opinion, the FDA's refusal to review the NDA filing for troriluzole is further wasting time, that many SCA3 patients have already had to forfeit, due to their condition. PLEASE reconsider reviewing the NDA for troriluzole.
Joan M.	Please review the drug. Every opportunity for any drug should be given because there is no cure for SCA.
Márcio G.	There is NO CURE for SCA3. FDA should be more flexible and apply fast tracks to ataxia drugs. SCA3 is degenerative and we have no time.
Robert W.	I am 73 years old. Further delay will reduce the possibility that troriluzole can help me.
Raquel	SCA3 is an invalidating diagnosis which changes your life. 4 persons already affected in my family. We are rare, I think we don't cost millions. Please help us find a cure! If troriluzole doesn't harm, flexibilize regulation, at least have a look at it!
Angela B.	I am very disappointed in the FDA. I expected better. I am a person living with Ataxia. I also am a special education teacher. I have worked with students and many families of students with progressive degenerative diseases. All those families, students, and I want is a chance (a hope).
Charles H.	FDA should review the drug troriluzole!!
Denise K.	My dear Mother, highly intelligent, funny and engaged throughout her life, struggled with Ataxia for 20 years and died in May 2021. She was 80 and Ataxia robbed her of the ability to talk, walk and at the end, eat. The quality of life from a progressive disease like Ataxia is gut-wrenching for the afflicted and the family and friends. PLEASE evaluate all data and do not slam the door on this important opportunity.
Kristy T.	I am in this study and it has helped my speech.
Nancy G.	Everyday I live with the fear of when I will permanently have to live in a wheelchair. If there is a drug out there that would even give me one more day of freedom from that nightmare, I would take it in a heartbeat. It pisses me off that the FDA has made this is decision.
Sandra L.	To not even review the drug is negligent behavior. We have this disease now!
Ed D.	Ataxia patients and their care partners oftentimes feel hopeless because there is no cure and because it is an "orphan" disease, it does not get the same attention as more prominent conditions. The FDA should give us hope by allowing for the drug's use in SCA3 patients.
Carla G.	Please consider the debilitating, progressive, and deadly nature of SCA when you make your decision. If you were to watch a family member decline, I believe you would want access to any and all possible interventions that may stall progression.
Edward S.	My brother's slow but steady decline currently has no hope of a treatment. I implore (beg) the FDA to reconsider the NDA for troriluzole and conduct a full review. Thank you.

NAME	COMMENT
Nancy B.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Michael L.	My wife and I have been caregivers for over 20 years for a daughter who has declined due to Ataxia from a fully functioning high school student to a totally dependent person with profound physical, emotional and intellectual deficits. We believe that the FDA owes those with SCA3, as well as all those with some form of Ataxia, the benefit of reviewing the data filed on this drug. We need FDA's thorough review and guidance to get to the next level in attacking this rare disease.
Kathy L.	Please give those suffering from SCA a chance and review the NDA.
John Y.	FDA fails to fulfill its obligation to the public by rigid application of regulations. Drugs used to treat Ataxia in its various forms are mostly not habit forming or dangerous when used carefully and monitored properly.
Fernanda S.	I have a family member with ataxia and I believe the FDA should review the application for the drug.
Betsy H.	My daughter suffers from Spinocerebellar Ataxia. There is presently no drug that can help her. Shame on the FDA for not reviewing this potential drug that could help in any way.
Cory G.	Living with this condition requires one thing everyday, and that is hope. I ask you to please accept this NDA for this medicine as it provides people dealing with this condition the hope that there are drugs out there available for them. Without that, we feel like we must deal with our suffering alone. Thanks
Robert E.	SCA is a terrible disease. FDA, please be open minded for the use of troriluzole for patients with SCA.

"Would you accept, falling a lot, walking as if you were drunk, speech and swallowing reducing and unable to do ordinary tasks? If not please make sure this drug is given a fair trial."

— Beryl D.

NAME	COMMENT
Earl S.	For these orphan conditions ultimate flexibility is needed to assess treatment efficacy. In this type of situation a focused phase 3 trial should be carefully evaluated for any potential benefit.
Andre L.	Seen first hand the effects of Ataxia in otherwise young and healthy people is extremely sad. I trust and appreciate FDA's process and the safety it brings to the American people. But insist that, given the severity of this disease, an exception is made and the full evaluation of the application is performed.
Howard S.	It is important that the FDA approve the filing of this drug. The data shows it is effective for SCA3. We as the Ataxia Community need to have this drug approved for use by people with SCA3. It will make their lives better.
Ann S.	My husband is participating in the trial and has experienced very little progression. In fairness to the circumstances of rare diseases I would request that the FDA give full hearing on the medication.
Louise E.	I've had SCA3 for over 10 years with an increasingly rapid progression. I need whatever I can use to fight this relentless disease NOW. I do not expect to be independent for much longer and if there is something that might help then please release it and don't tie it up with red tape. Time is imperative.
Stephen E.	Dear FDA representative, please consider helping advance drug trials against ataxia. My mom has this and it has really debilitated her life. We would like some help to see if other victims and her can help address this terrible disease. Thank you for the consideration.
Jeannie B.	I believe that the FDA should review the findings of a possible new drug that has a potential to stop the disease.
George S.	Our dear friend has just reached retirement age with the promise of years of travel, mobility and bouncing grandchildren on her knee ahead. Tragically, she has been diagnosed with Ataxia after a year of tests for other diseases. She would gladly participate in a trial knowing the uncertainty of the outcome. I am pleading with the FDA to please reconsider their refusal to review the New Drug Application.
Brandon H.	I feel there should be more flexibility in these experimental drugs for SCA3 and other Ataxias. My insurance won't accept medications not approved for ataxia and it's difficult for my neurologist to help figure out how to treat my symptoms. I am currently undergoing genetic testing and have not yet been diagnosed with SCA3, but I would be open to try the medication and others that become available to see if they help my worse symptoms to improve my quality of life.
Andrea C.	It is very difficult for a rare disease patient to understand why a drug that could potentially offer benefit would not at least be reviewed. On behalf of all rare disease patients, I urge you to reconsider the refusal to review troriluzole.
Jayden B.	I have SCA3 been on the study for almost 3 years. I'm not progressing as many of my friends have or even died. Please give the drug the FDA OK. I'm afraid I'll progress immediately. Thank you
Maryum B.	As there is currently not a cure for SCA3, the FDA should be willing to review/explore all potential safe treatments.
Joanne G.	We are too strict and blinded to people who would benefit from new drugs. To not even review the New Drug Application is unconscionable. Please do the right thing and review the application.
Courtney Q.	I am a healthcare provider and my best friend suffers from SCA. I know she would be willing to TRY something if given the opportunity, since the progressive nature of this disease is slowing her life anyway. Do it for her child who needs his mom, if she has even the slightest chance of benefiting then why not just try.
Robyn P.	The FDA should apply regulatory flexibility for rare diseases such as SCA.
Meghan T.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Jeannette V.	I'm suffering with SCA3 and I'm watching as my aunts, uncles, and some cousins are deteriorating from this rare disease. It gives me hope when I see troriluzole may help even in the slightest; which is why I believe the FDA should take the time to at least consider this an option.
Denise H.	As someone who has been living with ataxia almost 40 years with no treatment available, I would like to strongly urge you to review the drug because researchers have dedicated their entire lives to find treatment and eventually a cure.
Richard B.	Every option must be explored fully, this is not about profit, it is about people's lives.

NAME	COMMENT
Joe T.	As a spouse to someone with SCA3, this issue is very personal. It is imperative that the FDA at least review the application so that the potential treatment can be explored and the conversation can continue.
Samuel D.	This new drug application for troriluzole is an urgent issue for people who are experiencing this slow debilitating disease.
Kathy S.	My husband's entire family has been affect by SCA and we have tried several medications which were developed specifically for either Parkinson's or ALS with only limited success. To have the FDA refuse to review this medication and results for troriluzole is quite discouraging. I hope they will reconsider. :(
Bruno C.	Hi, my name is Bruno, I have SCA3 and have been showing symptoms for over 2 years now. My mother also has it, I have family members, dead and alive, with this condition. I believe firmly that the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review. There are no medications available for this disease, absolutely none. It is only logical that it's process of review is made differently than the others.
D. W.	Why not review? Doesn't make sense.
James J.	I have witnessed Ataxia progressively steal my dear friend's movement, balance, thought process and overall zest for a fulfilled life be taken away. I ask for your help and plead that you review the new Drug application for troriluzole so that my friend can have the potential treatment for SCA3. With prayerful hope.
Sharon B.	SCA3 meets all 3 requirements of the exception. As a widow from SCA3 individual and a parent of a SCA3 individual, I beg the FDA reconsider. I want my grandchildren to have their father and they themselves to have hope in case they too have the gene.
Dana M.	Ataxia is a serious, often fatal disease. Currently there is no treatment or medication for those with ataxia. This drug represents hope in the quest for a treatment and at the very least, deserves a review by the FDA.
Cathy L.	It is so disappointing that the FDA is not going to review the New Drug Application for troriluzole as a potential treatment for SCA3. As a patient with SCA, this decision is going to affect me as well as my children who will also inherit this condition. So little is know about SCA, and because of that I feel that the FDA is making a snap decision. If there is a potential for the drug to alleviate the symptoms, I feel it's worth further review. It would mean a lot to SCA3 patents as well as other types of Ataxia. Potential treatment could change the course of my life, and that gives me hope. Please reconsider.
Scott F.	The debilitating nature of ataxia means sufferers are eager for any chink of light in the medication field. I am sure flexibility would be possible, supported by sufficient disclaimer language, so urge the FDA to give additional consideration.
Ernie S.	Although there is no cure for Ataxia. I believe medications provide a promising avenue for symptom alleviation. Any medication that shows any evidence to that effect needs to be considered seriously, providing no significantly negative side effects are undocumented.
Lisa C.	Please change your decision. I may not have SCA3 but wouldn't want a possible treatment be denied.
John M.	We would just like the data to be reviewed just like so many other drugs. This impacts many who are already taking it. If anything it has reduced falls or the same.
Gary W.	My mother had Ataxia, and passed it to me. I hope my two daughters don't learn that they also have it. Although there is no cure for any variant of Ataxia, any possible lessening of any symptom should be explored. To not explore any possible improvement would be cruel. I'm currently seeing a speech therapist as well as a Neurologist and I would gladly take anything that might help reduce any symptom of my Ataxia.
Andrea S.	I am very disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory process from FDA for rare diseases like ataxias should be more flexible!
Daniel M.	If there's a chance it may do something, review it. I haven't had a med do anything to help. Need more med to try
Guttenberg G.	I am very disappointed with FDA'S decision to not review the new drug application for troriluzole from Biohaven. The regulatory process for FDA for rare diseases like ataxias should be more flexible.
Rosemary W.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Claire S.	I have SCA3. My mom also had it. We have absolutely no treatment options. Please consider this drug!

NAME	COMMENT
Ed F.	I have a different type of Spinocerebellar Ataxia (SCA). I'm 32 years old with mild symptoms currently, but the disease is progressive and I have an uncertain future. Hearing that a drug works for another type of SCA is great news, but hearing that it will not be accessible for those with SCA3 is terrible news. If the drug is approved for use, this may also lead to similar drugs/treatments, for other SCAs, and hopefully ataxias in general. The research and data has to be reviewed.
Connie B.	A dear friend was diagnosed with this rare debilitating genetic disease. She needs help NOW. Additionally, her children and grandchildren may have this same disease one day. Please help accelerate the FDA review and please provide some leniency to help those who are suffering today.
Andrew G.	I am not looking forward to the slow death that is SCA3. I would very much like to be able to have access to a drug that can slow or stop the symptoms. I watched my grandmother die from this and am watching my mother suffer now.
Linda W.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Michael C.	I have lost my wife Phyllis and my eldest son Christopher to SCA-3 and am now caring for my son Matthew. I am seeing my son Matthew go down hill with no treatment options. I implore the FDA to have more flexibility with approving trials for troriluzole.
Gary S.	Myself + thousands of other sufferers of this debilitating condition need every chance given to a possible drug that has potential positive effects.
Celina M.	I have been living with SCA3 for 20 yrs. I have 3 other sisters with SCA3 which the oldest one already passed away. I am so afraid for my children and grandchildren. We need something that will stop the progression. Please help myself and my family. We need some hope. Please review troriluzole again. Thank you.
Hannah M.	I think that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Raj K.	It is imperative for FDA to apply regulatory flexibility for rare disease such as SCA3 when there is no other known cure.
Heather D.	All drugs that may help any type of ataxia should be looked into as there needs to be some help for a condition which currently has no widely available drug or help.
Charles L.	I am watching my friend bravely dealing with the debilitating effects of ataxia. She is working hard on maintaining her balance and movements. It helps, but the future is not good. She needs access to the promising drug troriluzole, so that she can see for herself if it helps.
Dianne P.	Living with Spinocerebellar Ataxia is horrendous! We need help and that is only made possible by research and trials for us to participate in. Please, FDA, don't hinder the ability for those with Ataxia to be able to try a potential treatment!
Claudia A.	I am very disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory process from FDA for rare diseases like ataxias should be more flexible!
Bhushan B.	We are struggling to get treatment in Ataxia. It will be really helpful for everyone if FDA will consider this for the approval. Lot of hope will be there from this treatment for the Ataxia patients.
Pat J.	The FDA should review and reconsider their decision. Why not approve the use of this drug in SCA3 for compassionate use.
Emily M.	Not reviewing this medication for SCA3 is ludicrous! My husband has SCA3 and struggles with falls daily. Anything that could cut down on his falls should be an option for him to try! The biggest morbidity/ mortality risk for him currently is falls.
Christian M.	Please give everything due process.
Levi B.	I have already lost a father and uncle to this disease in last 25 years of studying this disease. Please tell me I might have an option before I also die.
Betty R.	My husband developed Ataxia in 2000. He passed away in 2010 at the age of 64. Having a drug that might have helped him would have been a miracle. Please, please accept the NDA filing for troriluzole.
Mary Law	I do not agree with your decision, every measure should be taken to try to find a cure / treatment for SCA3.

NAME	COMMENT
Angela B.	This is a horrible disease that has many of the same side effects of other high profile illnesses. It deserves the same consideration for treatments as all debilitating diseases and the decision should be reconsidered.
Judy B.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Celeste S.	Rare conditions like SCA3 deserve to have potential therapeutics receive a full review of the data from the FDA. Without a full review, we could be missing out on treatments that work. Until there is a full review of the data, all that the ataxia community is left with is uncertainty.
Janet S.	Watching someone you love slowly deteriorating is a horrible and frustrating experience. Any possible relief should be given a fair trial.
Annalisa D.	Please review the NDA to give families dealing with SCA3 hope and a fighting chance for survival.
Alan H.	As a person with SCA, I have waited patiently for help with this rare disease. Much of the money for research understandably goes toward more common diseases such as cancer, heart disease, diabetes, etc. When I ask what treatments are available for patients like me, the response is crutches, wheel chairs, therapy for talking and swallowing, and so on. Finally, more attention and dollars were given to rare diseases. We have been watching the progress of this drug for several years, hoping for a breakthrough. Before it's back to the drawing board, and waiting untold years or decades for another trial, I ask that this drug be given a thorough review before being discarded. We simply do not have years to wait. SCA is a crippling killer that robs one of his/her decency and quality of life. I deal with my disease as part of my daily life, but I and many, many more would greatly appreciate the FDA reconsidering its position on this drug and conducting a thorough evaluation. Thank you.
Amanda C.	Please allow this.
Bobby	It's very simple. The FDA needs to come inside my house and see what it's like for 24 hours. At least it's worth a try.
Janet Z.	A drug to slow the progress of this disease that could prolong my mobility is worth exploring.
Kate C.	Knowing one person's journey living with Ataxia is too many...this is a cruel way to live and those battling the daily fights should have a right to try something, anything that may help them live a more quality filled life!
Mary B.	My friend and his sister and their mother (who has passed from it) would probably take advantage of any drugs available for SCA3.
Zack S.	I believe the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review for a few reasons: SCA3 while considered a rare disease by affected number standards, is different in the wide spread effect that it has on entire families and those that are close to them. Drugs like troriluzole, or any other in the pipeline that can prolong the effects of this disease, can provide relief and hope to not only those effected, but the widespread families of those. SCA3 has taken the life of my grandmother, my father, four of my aunts, my brother, and several of my cousins, including myself now have it.
Aurizangela P.	Peço aos que fazem a FDA, que revisem a decisão de não aceitar revisar o remédio troriluzole, pois há muitas pessoas com o diagnóstico de SCA3 esperançosos por um remédio que ao menos retarde os sintomas, nos ajudem!!!
Liz B.	I live with SCA3. Although I am an optimistic person who participates in a series of group activities (pilates, chair yoga, tai chi), I still suffer from neuropathy and am losing the ability to use a walker. I will have to use a wheelchair full-time soon. Any medicine that would help relieve the daily pain or slow the progression of this disease would be beneficial. There is currently nothing available.
Chris C.	I have SCA3, as did my mother, and I may have a child who has SCA3. The thought that there could be a treatment through a drug is powerfully hopeful. My life is a balance between reality and hope.
Karen H.	The devastation to quality of life and downward spiral of debilitation can drive people with SCA into states of hopelessness and depression in addition to the relentless negating of ability after ability due to SCA. Any treatment that might offer even the slightest slowing of the progression is worthy of a try — without anything to try, this plunging tunnel to indignity and inhumanity may be unbearable.
Lillian D.	I have friends that have several members of their family with this disease. They would very much like to try and benefit from this new drug. They realize it isn't a cure, but would greatly benefit if it stops the disease's progression.

NAME	COMMENT
Anonymous	As someone recently diagnosed with Cerebella Ataxia, it is vital that the FDA review all applications for the treatment of all Ataxia so people with the condition can lead the lives they were used to. I had to leave the job I had for almost 30 years because I could no longer write legibly or speak clearly and I don't know how much longer I will be able to walk or drive. Ataxia is a frustrating, depressing, and debilitating condition that you can't always see from the outside. Ataxia can cause many other difficult symptoms as well. We need the FDA to review all applications that address treatments for this life-changing condition.
Lesley S.	My daughter has had cerebellum ataxia following a near death illness of encephalitis when she was four years old. She is now 45 years old and struggles every day just to get by. If new drugs have been found to be safe then they should be considered as a potential treatment. Provided they are safe then if not tested no progress will be made.
Frances P.	If you had a family member or friend with this disease, would you want to be able to try to lessen the effects of the disease?
Jennifer D.	To watch my mother-in-law deteriorate and pass from complications of the disease, understanding the inheritability and repercussions of the disease I fully support the use and research of new drugs and therapies.
Josephine L.	Ataxia is not just someone walking as if having a few drinks, it is devastation beyond belief. In the early stages, yes the gait is noticeably off. With progression the eyes can't stay still so of course vision is impaired. This of course means that you can't drive. The throat changes and causes choking and speech problems. At this stage there is constant falling with the inability to help yourself to get up, at this point 911 calls become necessary. There is not enough movement stability to get dressed on your own, it is even too difficult to pull the covers up on your body. The ataxia we must deal with is still called Degeneration of the Cerebellum since the gene has not been identified. Hopefully when this gene is identified there will be a medicine to at least eliminate the symptoms.
Karina K.	We, who live with ataxia with no hope of cure, believe that a medicine that brings some benefit is valid.
Renata R.	People with SCA3 ataxia deserve this chance to have a treatment.
Ashlynn B.	This drug has the potential to make a real difference in the lives of people with SCA3. I believe that it is worth further investigation, and I hope that you will give it the opportunity to prove itself in a larger clinical trial.
Ross D.	I think this is being done for commercial gain. I have no personal experience of this.
David W.	This medication may help on an individual basis and it should have the right to try it provided I accept all risks associated with the medication
Kenia V.	I lived with my husband's diagnosis of MJD for 30 years, our eldest son is a carrier. And we don't know if the other children are carriers, and grandchildren. Living a life without expectations and seeing the person die drop by drop is not living a life, it is suffering. Why not make available what could be a light at the end of the tunnel? How many more will suffer to death with no quality of life... they didn't choose to be carriers of a cruel disease. My husband died at the age of 56, of which he lived for 30 years due to SCA3. I hope this decision is reviewed, you can make a difference in many lives! SCA3 is no longer an orphan disease. There are thousands of people waiting to see that light at the end of the tunnel.
Mark T.	I have seen my mom suffer with Ataxia. Now the disease is passed on to me, any glimmer of hope will help future generations. Thank you.
Vicky C.	Hereditary SCAs are rare diseases affecting a small population making it difficult to generate studies with sufficient power. Currently there is no cure but individuals suffering from the conditions may experience debilitating symptoms that interfere with their independence. Any medication that may slow down the disease progression or reduce symptoms will be beneficial to those with any SCAs. Please re consider and review the troriluzole application. I would like to not progress to wheelchair bound. Thanks.
Lenore H.	In living daily with this movement disorder, if a drug could even just reduce the intensity of the symptoms experienced, it would be wonderful! Don't rob the afflicted of some optimism/hopefulness by NOT reviewing the NDA for troriluzole!
Carey B.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease. It's heartbreaking to watch it cripple generations in my husband's family. Even worse, they know what's coming as they watched their father deteriorate over the years in front of them. Please do something.
Ana P.	A FDA deve analisar os estudos do medicamento troriluzole para os portadores de SCA3, dando-lhes o direito a um tratamento, tendo em vista que não existe nenhum medicamento para a doença.

NAME	COMMENT
Fred B.	SCA6 has been in my family as long as we have records. It is devastating to think that my children may be impacted as well. I watched my grandmother, mother, and two sisters decline and I am losing ability at a disturbing rate. Any possible treatments should be tried. New treatments will probably not help me, but if they are available for my children and grandchildren I am more than willing to try almost anything.
Kristin H.	Ataxia is a devastating illness affecting not only the sufferer with unimaginable fear as well as the progressively worsening symptoms but also having a huge impact over many years on family life, children growing up, children who always have a fear overhanging their lives and partners who have to give up so much emotionally, physically and financially to care for their loved one without much support or understanding from the outside world. Please do everything in your power to ensure that a drug that could help an Ataxia sufferer is reviewed and given every chance to be prescribed as soon as possible. When there is no hope and someone is prepared to try a new drug then what is there to lose? Please review troriluzole as a potential treatment for SCA3 which we hope will lead to breakthrough treatments for other SCAs. Thank you!
Wong Chee Y.	Give the drug a review, and if the data shows no efficacy then reject the drug. But at least review it, the community and other drug company need to know that FDA cares.
Keith S.	I was diagnosed with SCA7 in July 2020 and have found my life has changed since then, as I am also registered blind, and my consultant told us I could not go out on my own anymore, which means I lost my independence when walking out with my guide dog. It would be great to know there may be help in the future with a new drug, so I support the NAF in asking the FDA to review troriluzole as a potential treatment, as I do not want to lose any more of my capabilities.
Katie T.	The patient should have the choice.
Chloe H.	I would love something that would help.
Frances M.	If there was even a remote chance of a drug being able to help my brother, I'm pretty sure he would chance it. The alternative is living with something that is slowly robbing him of life. The hardest part is that he is mentally OK but physically dependent for everything. He can hardly even communicate now. So, so cruel yet he remains positive. Why deny anyone in such a situation even a small ray of hope?
Phillip B.	To be honest, I can't believe this decision. Knowing how debilitating having SCA is, anything that even has the potential to help needs to be properly investigated surely.
Kirah S.	I have watched my friend's mobility degenerate for 23 years and only learned it was due to SCA3 in 2021. She is only middle-aged; she's not ready to be in a wheelchair or lose all mobility so soon in her life. A new drug could give her hope and a new set of abilities to continue into the second half of her life.
Cheryl D.	There is no treatment for ataxia. We are desperate to find something that helps. Make this available! It will help someone.
Dr. Kevin O.	My first wife suffered from SCA and I watched helplessly as she slowly lost her ability to stand, walk, feed herself, and even sit unassisted. It was absolutely devastating to my family. I am now faced with repeating this experience with one of my children. Having seen the progression firsthand, I understand how important it is to even slow the progress of this horrible disease so that my daughter can have a normal life for as long as possible, and perhaps buy her some time until an actual cure can be developed. Given the low risk and apparent safety of troriluzole, I would absolutely consider using this therapy even if it provided only a modest benefit. It represents the only real hope she has for even living with this condition.
Solange C.	Please, FDA, review the New Drug Application for troriluzole. I' m sure this medicine will help us a lot.
Karla M.	My mother, aunt, grandfather, and maternal relatives have lived with this unfortunate disease. I am unaware if I carry SCA3 but I have a 50% chance of having it. It is important for us to feel like there is a drug to help. Seeing first hand the effects of SCA3 is very difficult and anything would be better than nothing to better quality of life.
Cassandra M.	I have seen the effects of SCA3 on my father-in-law and former husband who are now deceased from complications and no available treatments. Now I am now watching my daughter deal with SCA3. It's time families had a fair chance at drug treatments that may provide an improved and better quality of life.
Craig O.	The current drugs are basically no help, as far as I can see. A reduced chance of helping, is still better than the current state, in my opinion.
Jennifer K.	This disease is affecting my husband and has the potential to affect my two teenage children. They all deserve a chance!

NAME	COMMENT
Joan M.	I have several family members alive and deceased, that have been severely affected by two different types of ataxia that being episodic ataxia type 2 and also SCA6. I was blessed to have dodged the bullet. If there was a treatment that would help my family who suffer greatly even though they do not have a SCA3, it would be a dream come true as I'm sure it would be for those with a SCA3. What a shame that the FDA does not have the wherewithal to approve furthering the troriluzole next steps. My 32 year old nephew just passed away 3 weeks ago from the psychological trauma of having ataxia. His mother who also suffers from this is devastated and blames herself for his condition that he inherited from her. Please FDA, get to work on this drug as soon as possible. Lives are at stake!!!
Claire D.	I have a close friend who is currently loosing her mobility and independence due to SCA3. She also recently suffered a life changing fall as she is no longer living independently. I suppose if the research is accurate and it is able to reduce falls then I think this is enough to allow more trials. This is a small community and there are few options available so this might be one.
Aline N.	Every life matters and every attempt at new chances is valid.
John W.	I have SCA6. Not the same as SCA3 but not dissimilar. I feel that any possible benefit should be offered to sufferers.
Peter J.	It is emotionally draining to see my Father, whose mind is sharp and muscles strong, slowly lose his ability to walk, talk or do anything because Ataxia is atrophying his nerves. And he now faces decisions on whether or not to use medical devices to stay alive (ex - feeding tub), or just give up. Even more depressing is knowing Ataxia primarily attacks younger people, and seeing it do so. A few years back, a friend of mine learned his teenage daughter has Ataxia, she was married in a wheel chair, and they predict she will be dead before she reaches 30. My same friend just learned his teenage son has Ataxia too. Through support groups, everyone tries to stay positive, and ultimately life is not fair, but we all still reach for ANY kind of HOPE ..., but it feels like we are grasping at straws. And so, to have a treatment with positive results, and learn that BUREAUCRACY is denying a chance for people to try this treatment, is beyond frustrating. I ask every member of the FDA board involved, to imagine if their child was diagnosed, and imagine the rapid, unrelenting nature of the condition ..., would they not want the ability to choose to try an experimental treatment with positive results?!? I am 100% certain this treatment will not work for everyone, but please allow HOPE, and allow those for whom it will work, to receive the benefit! Thank you!!!
Jacki C.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Stephanie O.	They need to know how wrong it is and how people are suffering and dying while they are refusing to review this application.
Leon A.	I have been suffering with SCA3 for nearly 7 years now and I have been changed by the condition completely and I have not been able to meet anyone because of my communication issues and I cannot work or do activities like I used to be able to do because of my motor function issues both gross and fine, so I struggle with basic things like doing a button up on my shirt front right up to having severe difficulties with being able to walk and my right leg is aching as it hasn't been able to fully recover from an injury suffered during the start of the condition.
Jenna D.	SCA3 took my nana and uncle and is going to take 2 more uncles in the not too distant future. I've watched them struggle and deteriorate in ways that you never want to see a loved one go through. Even something, anything, would be better than nothing.
Alexandre V.	In my opinion I do think that the FDA should be flexible because there is not even one medicine to try to stop this disease. I would like to try this medicine.
Caroline S.	Please review your decision.
Neema	If you're listening to the people who say that they're benefiting from troriluzole and it's helping them in their daily struggle, why would you want to limit that? We are desperately trying to hold on to hope and the promise of research Please move forward and review the new drug application for troriluzole as a potential treatment for SCA3. Please consider how your decision could hold back human progress for our American community and beyond. We are counting on you right now. We need this consideration yesterday!
Anonymous	As a person living with SCA3, I would like to be given the chance to try troriluzole, which may help alleviate some of the symptoms of this debilitating disease.
Dr. Lawrence S.	I have done research which led to the discovery of the SCA1 gene as well as SCA5 and SCA8. All people afflicted with ataxias feel there is an urgency to develop a drug such as troriluzole.

NAME	COMMENT
Mark R.	Having watched my mother, two sisters and a brother, a nephew and niece suffer for decades from this disease with little hope of any intervention or treatment they need some sliver of hope, no matter how small to make them feel they are in some way fighting back. Even if the medication offers minimal improvement or prolonging progression of the disease, it is something. Something they don't have at this point. They need hope and a chance to participate in the development of treatments even if the effect of such medication is not fully known at this time.
Vanessa D.	I believe the only explanation is that the FDA nor their loved ones (luckily) suffer from this devastating disease that is SCA3.
Abdi A.	I suggest that we trust the scientific drug application for SCA3. Sabotaging efforts by FDA on the use of the drug by SCA3 patients just means the US government is not having a duty of care for its citizens. As a family we lost many of our family members and still other young ones by the age of 40 are using wheelchairs.
Frank S.	To put it bluntly, without trials Ataxia sufferers have a high mortality. With trials there may be a percentage chance of improvement. So from little if any chance to a percentage chance is a leap forward. Here in the UK my wife has been written off by doctors and we are not even told the type of Ataxia she has or if she actually even has it. She hasn't received so much as an analysis or even an aspirin for the condition.
Lewis B.	I have had Ataxia for 8 years now and have received no treatment at all. I am happy to try this medication as anything would be better than nothing
Jomita P.	I live with SCA3. My father and sister both suffered for many years with this illness until it finally killed them. I have two nephews currently suffering with this debilitating disease navigating falls, struggling with speech. Each of them are only in their 40s, so this medication could possibly help them and be a game changer in their lives. I am 66 and experiencing all the negative impact this illness has to throw at you, extreme pain, wheelchair bound, needing help to do the most basic things in life like eating, hygiene, etc. I also have spasms.
Lynnette P.	Since Ataxia, particularly SCA3, has no medication that helps to date, the refusal by the FDA to review is puzzling. Sufferers need hope that something can be developed that can help us.
Kathy V.	As a parent with 2 children with Ataxia, I am heartbroken, and feel helpless in providing hope for these children. This medication has a chance to make their life a little bit easier, and they deserve every bit of a chance to have a fulfilling life. Please, please, please consider this medication for those suffering.
Nami L.	I am a person who is battling this relentless disease, EVERYDAY. There is nothing currently available to treat or even slow the progression. Every moment this disease progresses and I wait and hope for anything to help. I have two kids and one grandchild who are at risk. I hope and pray there will be a therapy for them and I would be willing to try a new drug that may or may not work, just so they don't have to. PLEASE review the NDA submitted by Biohaven and give us a chance to live and to spend a little for quality time with the people we so dearly love.
Steven A.	As a person living with this disease and having watched several family members, including my mother and three aunts, have to live with this highly debilitating disease, I believe that any assistance in treatment should be reviewed. This is genetic and my children, grandchildren and niece and nephews may have to face this same fight. The thought of that is horrifying. The drug and disease meet the criteria for flexibility and therefore should be granted. The slowing down of this disease would be life changing.
Kiara B.	My personal experience that led to my opinion is my grandmother had Ataxia and seeing her deteriorate over the last 8 years was hard to watch. And knowing her death has affected my family makes my opinion on helping other with this disease a no-brainer.
Fei Z.	Strongly demands a comprehensive review
Anne H.	It is so disappointing to hear about this decision. It feels like there is no hope for people suffering with SCA, that they need to accept their fate without any possibility of medication to help them live and manage this condition.
Deonna L.	I cared for someone with SCA3 for 12 years and treatment wasn't available. She went to UCLA to a neurologist and waited for years for any news of some form of treatment, sadly to say she died before anything was available for her. I have a 30 year old niece with SCA3 who falls often. I feel that the individuals who are with SCA3 should have a medication that may help.
Kalyn G.	Medications offer hope to those living with ataxia. There are very few options. By denying this review you are denying the possibility of a possible lifeline for those who have progressed closer to the end.

"Even if it partially works for some people, it is far better than nothing. When there are no side effects, even a 1% improvement for 1% of people should be offered."

— James S.

NAME	COMMENT
Julie G.	My 13 year old nephew has a new diagnosis of Ataxia. He uses a wheeled walker at the age of 13. He has never been able to run like the other kids and has felt the sting of being different. He is a determined, brave, and courageous young man who is willing to try different medical treatments in order to help him live his best life. Please consider that this SCA3 causes severe disabilities, and patients are willing to try clinical trials and treatments. Give them the right to their bodily autonomy to make their own decisions if they want to engage in research.
Evelyn V.	We are living with an "orphan disease" that does not get enough funding to treat and cure those suffering with Ataxia. We are desperate for any chance to relieve the effects and progression of this disease. I am now watching my 2 adult children suffer after watching my husband suffer and die from the effects of this disease. All we ever have is " off label drugs", so we would be more than willing to try this drug.
Ana M.	I think any experimental drug is valid. Living with Ataxia is very difficult and any medicine that gives some kind of hope to these individuals should be evaluated with care, but with more flexibility than other medications due to the gravity of the illness.
Kristine W.	Ataxias are highly debilitating. They impact both quality and length of life. My husband suffers from both a genetic ataxia and Multiple System Atrophy. He looks down a long lens of progressive disability, all while doing everything he can to help himself. He exercises his mind and body, and does as much as he can for himself. At the same time, he is wheelchair-bound, has a feeding tube, and has limited use of his arms, hands, legs and feet. We have tried every available drug, and given the prognosis would try anything else, even if the results were uncertain.

NAME	COMMENT
Krishna	This disease has upended my mother-in-law's life. Now my husband is positive, and potentially my two kids too. Having a treatment could give our family another chance at life.
Joseph A.	My wife died a horrible death from ataxia, and we tried various drugs that had potential. Although none helped we need the freedom to try any new drug that may have a possibility of some improvement. After all what is the alternative, sure death in the most disturbing, dreadful, and frightful way. Please consider the request to try any means to help and give some glimmer of hope to these most unfortunate sick people.
Antonio O.	Please FDA help us to use this drug. My 2 brothers and 1 nephew 25 years old just died because of this non curable disease. We want a cure doesn't matter the cost. Let us use this drug please FDA.
Erica S.	I struggle terribly even doing daily activities. My balance has deteriorated so terribly that I have fallen even though I use a walker full time now. I would be so appreciative to try any medication that might be beneficial for me.
Pete S.	Right now there is no pharmaceutical treatment for SCAs. Even marginal effectiveness is a step forward. Pushing aside any drug with promise leaves me feeling abandoned. Life is hard enough with gait, swallowing and balance issues. We need some assurance that our life just might improve with a drug that slows down or maybe even stops progression.
Lawrence H.	We need to find a medication/drug that slows down/cures/reverses the symptoms of ataxia. The impact of ataxia on an individual and their family is tragic and makes one feel helpless. Young people are faced with tremendous challenges that are mentally and physically challenging and draining, robbing them of their youth and placing them under pressures that are soul destroying. Please help us to find a cure, a solution, something that will slow the relentlessness of the illness down and provide hope and happiness for the future for those impacted upon by ataxia.
Karen W.	I am living with this dreadful disease and have watched approximately 8 close family members DIE from this untreated condition that progresses rapidly. With every step that I take, I am reminded that it will be a short time frame until I cannot walk at all. Try to go to the bathroom, take a shower, make a meal, walk with a baby in your arms, go to Walmart. Go swimming with your friends and try to get out of a pool or off a floor, picking up a spilled mess. While everyone watches, you have to figure out how to stand without sticking your butt up in the air like a 2 year old. ALL DIGNITY LOST! I was a dancer my whole life and now cannot even get my legs to walk a straight line… Go to a neurologist and be told "oh, you have Ataxia, there is NOTHING WE CAN DO FOR YOU" Go home and deal with it. I have watched my Mother, Grandmother, several Aunts, Uncles, cousins and most of all my Brother at age 45 Die from this disease. Leaving behind 3 loving children and a beautiful wife… Just wait until you Choke to death on water! I have 2 children and my close cousins have children who have a 50/50 chance at getting this disease. And then my grandchildren…. We are all just waiting to see who gets it and how bad will it get. This disease it debilitating! No one asked for it! Just let me give you an example of how this disease STARTS with the beginning symptoms.. Imagine you are standing on a Rocking chair in the middle of a room. Now try to change a light bulb with your left hand 4 feet above your head. That is what the beginning symptoms are like… it just gets worse from there! PLEASE ALLOW US TO TRY SOMETHING AND GET A LITTLE BIT OF DIGNITY BACK!
Brooke	Give the community with ataxia a fighting chance.
Brenda S.	I think that the time has come for the FDA to give those with Ataxia some kind of hope. At the Very least, a shot it's slowing down SCA disorders. But they don't. It's very disappointing because the only alternative is disability and death and that seems to be what they are peddling. Not hope. My son is now 33 years old, and I have been looking for a cure for his disability Sca8. He has been on coenzyme Q and Buspar for 28 years to relieve his symptoms. But the FDA has never once given any cringed any therapy. I have followed this extensively. Hopefully they will come to their senses and offer those to suffer. At least a chance.
Alun P.	Should have access to this drug.
Carlos M.	FDA should approve to use the new drug regarding the treatment SCA3, there are many people with this disease dying, it must be now to approve the drug.
Pravin K.	I believe this drug has helped individuals with SCA3 in the trial having participated in the trials from the start. A full review of the NDA is prudent. Thank you!
Season H.	I am affected by this decision because I am newly diagnosed with SCA3, and I'm only 30 years old. My mom had this disease, and she only made it to 43. I'd like to double my mom's life span, or even triple it, but I won't even get a chance unless I can try these potential treatments. Please reconsider. My life depends on it.

NAME	COMMENT
Samuel K.	The FDA has placed its organization in the position of a God by not following its established policies for review. I have lost three step-siblings to SCA3 and have a clear understanding of the impact of this disease. The lack of a cure or effective therapy robs each patient of the ability to live. The FDA short-cutting its own policy does not bode well with the ataxia or rare disease community. The ataxia community is only seeking the same consideration given other rare diseases such as ALS, Duchenne muscular dystrophy (DMD), Batten disease, and MPS.
Karen S.	Would try anything that might improve wellbeing, even a trade-off to accept something that might have a minor negative effect.
Kellie-Jo D.	If you knew the real daily struggle to live with this disease you would do anything you could to help. To not even entertain the possibility is a gross misuse of your power. Please know that when you are afflicted by SCA3 your mind is fully functioning it's your body that doesn't respond as it should and that's it's own special torture. It's lingering, people survive for years and years having their body slowly deteriorating. There are NO options for us at this point, we need the studies and research to help us without it is cruel and unusual punishment!
José Eduardo S.	Because the lack of solutions and the short number of studies in this area, all credible solutions may be considered.
Jeremy B.	We had very few options if any that work. All we have is a future where we knows everything gets harder. Anything that could provide any respite would be useful. Please make these drugs available at prices we can work with, the option we do not have the is an early death. Any extension is worth it no matter any personal negative personal effects
Barbara K.	After many years of having symptoms of ataxia (speech/motor problems) I finally received a diagnosis of a spinocerebellar ataxia from NIH, resulting in several efforts to stave-off the inevitable decline. FDA can ameliorate or possible cure some/many of these neurological disorders, but only if they try. Please consider relaxing clinical attempts to prevent family and social tragedies of these horrific disorders. Neglecting to authorize a trial which may potentially postpone or minimize symptoms of people afflicted with a spinocerebellar ataxia is archaic and sentences us to a known path to destruction of an otherwise valuable human.
Alex B.	I have watched my mother's quality of life decline as she deals with balance and other issues resulting from SCA3. This is such a rare disease that it makes sense to me to review the data for this treatment.
Christine B.	Ataxia makes life very difficult, affecting entire generations of families and there is currently no cure or treatment. Any possibility of treatment should be made available, out of compassion for those affected.
Kathleen G.	Out of 6 siblings, 4 of us were victims of SCA3 — two oldest passed at 70 years old — I believe prematurely partly due to ravages of ataxia. A younger sibling has much more advanced symptoms than me. I have to believe that the trial drug, troriluzole, is responsible for holding back the progression of my ataxia symptoms. Please move forward on approval of troriluzole!!!
Renee B.	I am writing this comment regarding the FDA's decision not to review the New Drug Application for troriluzole. Both mom and aunt suffer from ataxia (SCA3). I have witnessed the deterioration of their health and mobility due to this devastating disease. An experimental drug like troriluzole could provide an option for treatment for my mom and aunt. There are currently no drug options or treatments. An experimental drug offers hope for a lessening of side effects or prolongation of lifespan for family members afflicted with SCA3. Due to the relentless and progressive nature of SCA3, it would still be great to have an option to try even if the drug doesn't ultimately benefit their disease progression. Thank you for your consideration.
Kathy S.	I believe that the drug TRORILUZOLE may help some people and has been tested long enough to be deemed safe.
John T.	I have SCA18 and my two children and 2 of my grandchildren also have SCA18. This dreaded disease is robbing us all of lives that might otherwise have hope for stopping or even reversing the effects of this disease. At this time this drug is our only hope!! As an Ataxia patient who lives with the life ending effects of this disease; I beg you to let the research of this drug continue!! Without it, we have no hope!
Jessica J.	For years I watched my big, strong, healthy father decline to the point where he was in a wheelchair, couldn't use his hand, and could barely talk. Both my sister and I have been recently diagnosed with SCA3, and it is TERRIFYING because I know what the future could hold. Every moment of the day I think about it, I worry with every step and am thinking about how, or if I even should, continue my life. The possibility of treatment gave me something that I had lost...HOPE. PLEASE don't take that away from me.

NAME	COMMENT
Jason G.	Biohaven should definitely request the Type A meeting. Good communication is extremely important. FDA may simply want more data. If the phase 3 troriluzole study only had 57 participants with SCA3 (41% of the total 141 participants), it might not be enough data to warrant drug approval. Type A meetings are intended to determine the path forward. Doctors are still able to prescribe troriluzole to patients if they believe there is a benefit and patients understand the risks. If more data are warranted, then there would be opportunity for people with SCA3 to enroll in future trials as well.
John J.	As I am living with Ataxia, any advancement in treatment is to be desired especially for my descendants.
Karen V.	My Father, his siblings, my cousins and my sisters have all been impacted by SCA3. As both a carer and a relative to individuals affected I have observed that the one thing the affected patient clings to is hope. This drug gives hope and will help them keep going. They really need to feel and see that the medical community has not forgotten, or discarded them as unsuitable for help. They do realize on a rational basis, that this drug may not be a permanent cure, or provide much relief in the long term, but they do want the opportunity to test it.
Renata N.	I am very disappointed with the FDA's decision not to review the data on TRORILUZOLE submitted by Biohaven. The regulatory process for rare and serious diseases such as ATAXIAS should be more flexible.
Mason R.	Any hope for those living, or around those, with SCA3 to have any chance of improvement or halt in symptoms or changes would be a true gift.
Jaskiren S.	Due to the rarity of the condition there doesn't seem to be many options for people with ataxia, and from personal experience speaking with someone with the condition I know they are open to trying anything that might help, even experimental and drugs with uncertainty.
Edward R.	My son died 3.5 years ago. He had SCA1. He was willing to try any new medication in hopes that it would help him. Really what's the alternative other than misery and eventual death. Any new drug should be available to patients with a terminal disease such as this.
Farzana I.	Living with ATAXIA is quite painful and research must go on to find treatment.
Hailey	As a 30 year old presymptomatic woman who has been diagnosed with SCA3, the fear that this disease will overtake my life like it has for so many of my family members is crushing. "There's no cure" renders my family completely helpless. The prospect that there could be a treatment that could potentially lessen the effects of the disease gives me hope for myself as well as the next generation. I'd be willing to try anything given how few options I have.
Joyce B.	Ataxia I know from personal experience with three family members IS VERY DEBILITATING AND CREATES EXTREME HARDSHIP TO LIVE AND TO LIVE INDEPENDENTLY. PLEASE be flexible and look at ALLLLLL the data of new drugs like troriluzole to help my family and the many others. These folks don't loose their capacity to contribute intellectually and emotionally to our lives. They deserve all possible sources for healing and treatment and prevention.
Alex B.	I have watched my mother's quality of life decline as she deals with balance and other issues resulting from SCA3. This is such a rare disease that it makes sense to me to review the data for this treatment.
Anna B.	Living with SCA3 and knowing there is no hope of any cure at all is extremely worrying. Any sort of improvement that a drug could provide would give a feeling of hope and positivity.
Carole G.	Ataxia is degenerative and I started with it approximately 10 years ago (although I didn't realize it at the time). Now it has become so I may have to give up work, worry about leaving the house as I don't know for sure when I will have what I call a full blown attack when I can't see properly (Nystagmas) slurred speech and inability to balance. On other days my walking is limited, although I try every day to go further after approximately 500 yards my balance goes off. Ataxia is RARE (MY GP had never heard of it) and degenerative; getting people to understand how you feel is nigh on impossible. I also have epilepsy so the both may well be linked genetically as my grandson who is three and a half, cannot walk or talk. All new drugs need to be tested. People with Ataxia need to be given the chance/option/opportunity of testing any drug which will aid their day to day living. If the opportunity is not given for this drug to reviewed then the FDA and all involved are sentencing people with Ataxia to a life of discomfort and inconvenience with no signs of it improving
Kristen B.	As a person living with SCA we need a treatment — anything that has the potential to even incrementally slow the relentless progression of this condition or lessen symptoms is a step in the right direction.
Laurie H.	When you're facing a situation with no hope of survival, any possibility is better than none. Even if a cure were found, we cannot recover what is lost in the meantime. Please reconsider. We are desperate.

NAME	COMMENT
Renae S.	I am writing in regard to the possibility of an NDA for ataxia. I have two siblings — a brother and a sister — who are living with this disease. I also have several cousins living with ataxia. I see their lives slowly being stripped away due to this disease. And it is not just their lives, but the lives of their children and grandchildren is being greatly impacted by the fallout this disease has. My brother and sister still have sharp minds, but their bodies are daily being weakened. They are trying to be proactive in their daily lives with PT and speech therapy, but we all know that is not the final answer, but it has helped them cope with the resources that are available. I ask that the FDA please continue the research of the drug troriluzole and eventually its approval. This disease will not go away as it is hereditary — it will keep impacting future generations in their personal and family quality of life. Thank you for your time.
Penny H.	I wish the people who are in control of reviewing this drug would have some empathy and compassion to understand the this disease is never going to get better only worse. What is the harm of reviewing this application? Please review.
Josh	If you've lived with someone who has this disease, this is a very obvious and easy choice. The FDA should absolutely review the application for troriluzole.
John S.	Ataxia is a slow and relentless disease that impacts life both mentally, physically and crushes dignity. Any help or potential medical relief should be encouraged for this often forgotten disease.
Ali O.	It is so tough to watch day by day the progressive of this disease that affects the life of everybody in this family. Any chance of a safe drug treatment is a hope, that at least the patient recover in part his normal life. I think the door should not be closed and the Lab petition for a full review, should be granted by the FDA.
Dennis N.	I'm concerned that new studies and techniques that are being developed almost daily might be ignored or not given the consideration they deserve due to the FDA not keeping pace with these discoveries that are moving at a faster rate than in the past. The result is that needy patients may not get the chance that they choose before time runs out to make their life more comfortable.
Cindy	I have three sons with Ataxia! This is the worst disease no one has ever heard of we need your help!!! Please reconsider and give us hope
Karina M.	It's very difficult to live with SCA3. It's very important for all people who has this illness to try the drug because it can reduce symptoms and allow more quality of life.
Sue M.	It is critical that we find new drugs to treat SCA3. There are currently NO drugs to treat ataxia. We ask for flexibility and leniency when approving drugs. We accept any risks involved.
Jennifer H.	Any chance of hope would be life changing for my dad. If he could try something in his lifetime that would be amazing.
Adriana A.	Essa medicação precisa urgentemente ser liberada para uso e dar esperanças aos portadores e familiares.
Craig	People need this, please help them.
Dr. Peter W.	Ataxia as a group are a rare disease, composed of several syndromes. They are usually progressive and disabling, and drugs which slow that progression are desperately needed. My ataxia syndrome, from a mutation of the POLG gene, is unrelated, but progress for one type of ataxia might help treatment for other types of ataxia. Even though the number of patients may be small, please at least view the application.
Naomi R.	I'm a part of the clinical trial and was forced to go off the drug between phases, at which time my symptoms took a nose dive. I am terrified to find out what will happen if I am forced to stop taking it permanently.
Hilary J.	I have been living with ataxia for the majority of my life (50+ years) and the effects it has had have been so debilitating that any chance of slowing the progress should be looked into and I would welcome.
Kathy D.	Although I have SCA6, I would welcome the chance to have a treatment even with some degree of risk. My maternal grandfather, mother and mother's 2 sisters all suffered with and died from SCA6. It is a horrible illness that only those affected by it and those closest to them will know. There is a high probability that my children and their offspring may inherit it. I would give anything to give them the chance to avoid the suffering this illness can cause.
Carol C.	I feel that because of the rarity of the different forms of Ataxia, it is important to have options which may offer relief available to the community. And for the patient and family to make the decision if the option is appropriate for their circumstances,
Dawid R.	Get the drug to the people, it has already been proven to work

NAME	COMMENT
Catiana S.	If it is a matter of assessing the objectives, a reassessment of the study is necessary and not its complete annulment. SCA3 has a large number of patients worldwide.
Celma G.	Eu gostaria de tentar o tratamento com troriluzole. Ainda não existe nenhum tratamento para a doença de SCA3 e essa pesquisa com o trorizole como tratamento é uma esperança para mim e todos que tem a doença e está esperando um tratamento pra poder viver melhor. É muito triste viver como estamos, a cada dia que passa nossos movimentos e equilíbrio vão diminuindo. A doença só piora, ter a possibilidade de melhoria é tudo que busco nessa vida.
Eric W.	Please look into this matter.
Dongguiqin	I strongly demand a comprehensive review.
Lisa A.	My son who is 34 years old has SCA3. He is starting to feel symptoms and is in desperate need for a drug to slow his progression. He watched his father die with this disease and is very scared of his future. PLEASE!!
Sandra Regina P.	I am very disappointed with FDA's decision to not reviews the new drug application for troriluzole from Biohaven the regulatory process from FDA for rare diseases like Ataxias should be more flexible!
Candice S.	Having seen what Ataxia, in all its forms, does to a person and subsequently a family, I firmly believe any and all drugs should be put to the test. If relief cam be brought to even 1 person and family, it is worth the cost and time.
Alcina T.	I have a very close friend that has SCA3, she is 53 and seeing the difficulties becoming bigger and her limitations rising isn't easy. Having the possibility of a drug that would be able to slow down the pace of the limitations is very much comforting. The review should be done.
Deborah J.	Any help is better than no help!
Jenny P.	I have been encouraged by the passage of legislation such as the Accelerating Access to Critical Therapies for ALS and hopeful that the FDA can see the need for making available potential treatments for other debilitating and incurable disease.
Martha H.	Ataxia and specifically SCA3 is such a debilitating condition. Seeing if this treatment would slow the progression of disability is so worth a trial for those suffering from SCA. Very little research is done for SCA compared to major problems like cancer but SCA patients suffer and incur life changing disability that often gets ignored. Not allowing a trial would be greatly tragic.
Peter M.	If you suffer, like me, from ataxia you want every treatment possibility to be explored.
Linda C.	As a person living with advanced SCA3, I am excited that finally we see on the horizon a potential treatment. It may come rather late for me, but it is a ray of hope for my two daughters who have tested positive.
Alan T.	This will enhance the quality of life of those with Ataxia.
Annie K.	Please do all you can to explore treatment for a condition that effects generations upon generations. This is incredibly important.
Diane M.	My condition has worsened since being diagnosed with SCA3 in 2013 and I feel that I have become somewhat of a burden to my family. Even if this medication doesn't help me, hopefully it will help future generations.
Rebeca R.	I lost my father, my uncles, my cousins... and I still have a lot of family members with SCA3. Look carefully to this drug could be a hope for us.
Maria	Feel that this drug should strongly be considered!!
K. Isaac K.	I served as one of my mother's caregivers until 2013 when this horrific rare disease took her from here exactly 30 days after it took her sister from this life. Now the disease has stricken me. I have lived with this ailment for approximately 14 years. This disease runs in my family and it really is horrible. My decline has been steady I went from a strapping 220 lb. combat vet who served his country to a 175 lb. man who can no longer walk. I implore you to please approve the medication to make my life a little bit better.
Andrea F.	É importante revisar o formulário de aplicação de novos medicamentos pois pode ser que ele traga benefícios futuros.
Al H.	Let the patient decide whether to take the drug while knowing all the possible side effects.
Analva J.	I am a doctor, child neurologist, and I have SCA3. Its a frequent disease in my family. I'm a 3rd generation with Machado Joseph and we are looking for a treatment soon.

NAME	COMMENT
Brad W.	This rare condition is debilitating to the physical. It also very tough emotionally to accept the fact that you can no longer do some of the things that made you happy. The doctors just tell you to keep moving. When it is very difficult to move at all! This drug will, at least, allow an individual to feel like they are taking some control back of their physical and emotional health!
Denise C.	As a person living with ataxia a potential drug that could help the progression of this hereditary disease is crucial. I beg of you to approve this drug
Kimberly F.	I've watched my 85 year old mother live with progressive SCA3-MJ for 25 years with zero treatment options. My sister and I gave both recently been diagnosed with this hereditary disease. So of course, we would think in 25 years there would be some treatment options, even if experimental or if possibly ineffective for us. We still deserve the opportunity to make that choice. That the FDA thinks how many are affected in determining to move forward on possible medications is unconscionable and very lacking in vision. As we know millions of times where a drug developed for one particular issue ends up helping many other types of health issues. Science and progress should not be governed in such a way that it's only developed for a large demographic.
Renee W.	Please accept this. The SCA community needs to move forward with hope.
Bryce S.	From a commercial perspective this is by no means expected to be a blockbuster. The incentives are not for Biohaven to profiteer on a compound that has perceived equivocal data. Due to it's proven safety record, it's not only appropriate but ethical to approve this medicine for use in patients with ataxia. They have NOTHING currently available to them and even marginal benefits matter. Again I repeat this is not a profiteering motive. Please put the patient first in your decision making.
Amanda S.	Though I suffer with SCA6, I know many people in my community with SCA3 and anything that might help them even a little should absolutely be available to them.
Linda N.	This is the first time that people living with ataxia have had any chance to have any drug targeting ataxia. Living with ataxia is grueling. We deserve the right to have some choice it this debilitating and degenerative disease.
Phyllis C.	We urge FDA to approve troriluzole!!!
Mary Jo G.	Our son-in-law is one of sufferers of this rare disease that took the life of his mother. When there's no hope, any possibility is better than none. Even if a cure is someday found, any degeneration that has occurred is not recoverable. Please reconsider
Christy	As I see my dear friend progressing through this horrible disease, I would wish she would have the opportunity to try a potentially helpful drug to give her and her family hope. Even small gains from a trial drug would be very promising.
Adriano D.	é muito importante, para aumentar minha esperança de vida, ou uma simples melhora
Betty M.	My wife has SCA2. I would like to see the FDA approve this.
Joseph K. Jr.	Please help me find a treatment to protect my children from this genetic disease.
Mary Ellen R.	Watching my family members deteriorate from this awful disease is heartbreaking and I worry for the future generations.
Mari F.	Watching a vibrant young woman go from being a runner and pianist to struggling to put one foot in front of the other is devastating. If she could improve her quality of life by even a fraction, it would be worth it. Please help citizens with ataxia be able to improve their quality of life.
Kathleen S.	My family has SCA1 which is similar to SCA3. If there is a opportunity to receive a drug which may make a difference in one's life considering how the disease progresses, I feel it is imperative to be an opportunity to try it.
Cari V.	Anyone with SCA, and certainly my wonderful friend, will happily take whatever risks might come with a promising drug. The FDA simply must look at this differently.
Jaime S.	People with progressive and incurable diseases need to be free to decide to take more risks in search of medicines and treatments.
Henricus S.	The disease is too serious and progressive for waiting on decisions in the future.
Lee D.	A family member has this and should be given every opportunity to live.

NAME	COMMENT
lipeng	My wife was diagnosed SCA3 last year and my son also have the big risk for this, but by now no one drug for SCA3. I hope FDA can consider the NDA for SCA3 based on the data. For my family, I can see a glimmer of light and hope. Thanks again from one SCA3 husband and father.
Lesa M.	I am lucky to have a late onset, slowly progressing form of ataxia. I know that there are other forms that are life altering at an early age and I support their decisions to use their best judgment when seeking treatment. If there is an experimental drug that may help, so be it.
Kimberly S.	This study offers the rare opportunity to examine outcomes. Even if they are negative it's a beginning. I have had ataxia since 1998 when a viral infection left me with episodes of dizziness and nausea. It took five years to get a diagnosis, which did not include treatment plan. There was none. I was prescribed Neurontin, an anti seizure med which made me sleep 14 hours a night. It did lessen the symptoms, but made my life impossible. I closed my law practice and began a search for others with a similar disorder. That led me to NAF, but not to anyone with the same symptoms. Now I have been genetically tested and know why. SCA42 is very rare and fortunately slow progressing, so I may live to see a treatment developed. But I'm 81 and the clock is ticking, so I support any potential treatment. Thank you.
Amanda O.	Precisamos dessa oportunidade para termos esse medicamento.
Cara G.	The FDA needs to consider neurodegenerative diseases are progressive and our communities are willing to try treatments and accept risks that it may be less effective but willingness to try without placebo control.

"Please provide this community with hope. A diagnosis is a death sentence and they would jump at any chance to relieve symptoms or live longer."

— Dustin L.

NAME	COMMENT
Bridget G.	I have a rare disease, SCA7. It is slowly progressing and there is a small population. I know it will be difficult to gather the strongest data on drug performance, but it is critical to make an improvement in options and choices for people like me and my children with no treatment options available today.
James K.	Dealing with Ataxia has opened my eyes to many options to deal with the affliction. I think as many options as reasonably possible should be available.
Karen	As I watch my dear friend deal with the limitations this disease has caused, I can't help but want every possible avenue researched fully, and opportunities made available for medication trials if patients so choose.
Natália A.	I am very disappointed with FDA's decision to not review the new drug application for troriluzole from Biohaven. The regulatory processes from FDA for rare diseases like ataxia should be more flexible.
Rene	If you don't test it how will you make a difference.
Marlene T.	I'm 73 and living with SCA3. My life is progressively more restricted. The thought of trying a drug that might help me is beyond wonderful. I thought at my age there wouldn't be any hope for a medication. Just the thought of it gives me hope.
Amy A.	My son has ataxia. Any drug or therapy which may improve his ataxia without any serious adverse effects would be welcome.
Annmarie A.	Please strongly reconsider to review troriluzole. Living with SCA3 is torturous and there are currently no treatments!
Mary E.	I see my brother suffer every day with ataxia. If he can gain any benefit from troriluzole what a gift; if not, he would be back where he started and dealing with any negative side-effects if any. Please let him try to gain back some of his normal life!!
Irene H.	I don't think I would benefit from this drug. Anything that would help SCA3 should be tried.
Kelly F.	Not only do people with Ataxia suffer, but so do their family members. We all understand the situation we are in. And we all want resolution. Some of us sign up for clinical trials just for to CHANCE to find a resolution. We have an opportunity to be a part of the solution, And even though there's a chance that it might not reach our destination, it at least gives us some form of a pathway to the ultimate goal. Please don't get in the way of that. Those that sign up are well aware of the risks, and will choose to TRY anyway. We deserve the chance. Thank you.
Mary Ellen V.	Imagine your parent having cancer and a potential drug inaccessible to you. This is how ataxia patients feel.
Joana D.	I've been treating patients with riluzole for 6 years, and have a good experience. Still the main problem are the side effects, as liver function impairment or anemia, which would be overpassed with troriluzole. In an area without much therapeutic options, troriluzole would be of much benefit to MJD/SCA3 patients.
Bettina S.	The people with the disease have no time to lose. Therefore, immediate action should be taken and the drug released. Everyday counts….
André S.	No one deserves to be sentenced to a life of suffering and limitations, including for their relatives, if there is even a small chance of having a normal life. Anyone would choose the uncertainty of healing then a certainty of a lifetime of suffering.
Brad H.	Newly diagnosed with Ataxia and I don't wish any type of Ataxia on anybody, if there's a new drug to try we should give it a shot.
John H.	If it has the potential to help then it should be approved
Mary S.	If any available medical advances can help relieve any symptoms..... we need to try it
Liliane S.	It is hard to see healthy and productive people losing their autonomy and confidence so early. We have to try to find some hope. There is no other way.
Katie C.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Andreas	The disease is so devastating that every drug with prospects to heal it, should be studied.
Elois S.	Seeing the pain and suffering my niece is going through we are prepared to try any drug that will give hope.
Martin A.	I have SCA6 and would welcome any progress in medical treatments for any form of Ataxia.

NAME	COMMENT
Andrei N.	Hi there, my name is Andrei, 31 old, from Brazil with SCA3. And my family have been hurts for this disease a long time ago, its so difficult see your mother having difficult day by day. I hope that formulary to press FDA to approve this.
John L.	Both my son and daughter have SCA3 and every day I witness their gradual decline. Since the present end is inevitable any possible drug will intervention is worth trying
Leslie V.	Not allowing the solution for SOME sufferers of Ataxia to be approved is unethical and inhumane.
Kelsey	This drug would provide necessary data on how it interacts with SCA3 and it would potentially help individuals struggling with SCA3. There are only upsides to having people use troriluzole.
Beatriz A.	I believe that the troriluzole will help a lot in delaying the progression of SCA3, providing more quality of life for all patients.
Andreza V.	In the face of a serious, debilitating and fatal illness, troriluzole represents hope. You don't take away a person's only hope.
Kathleen H.	My progression on SCA3 is happening if ever there's a chance to stop progression or being able to walk I would agree in a second to take the drug on trial. We need to come to a solution of this disease by far any drugs would be a blessing, more trials is a step forward also.
Matt B.	This possible treatment could be life changing for many people, as this disease is devastating to family and unknown future generations
John L.	I watched this disease affect my mother most of my life and take her life earlier than expected. I now see my sister and brother affected in similar ways. The worst was and is feeling like there is nothing to do about it. This drug and study data at least provides hope and a chance for those fighting this disease. To deny them the choice or any applicable information is the incorrect decision.
Amy D.	Debilitating disease. My daughter suffers everyday and there is no medication to help. Please, please approve.
Andrea C.	To improve the people's lives with Ataxia every scientific analysis has to be implemented and reviewed over and over as it is meant to be as a Science object.
Jacqlyn H.	As a mom of two boys at a 50% risk for acquiring ataxia after their father was recently diagnosed, I beg of you to please extend some faith and trust into the process of scientific experimentation that may help advance treatments for this devastating disease. Our family depends on you.
Karen K.	People don't have time to wait.
Kemi U.	My daughter's condition is deteriorating every day and she is only 22 years old and just recently graduated from college. Right now she feels completely out of hope and options. With this drug, she may actually have a shot at life.
Matt S.	Please review the application for troriluzole!
Joe M.	The New Drug Application for troriluzole needs to be reviewed as a potential treatment for SCA3 because of the hope it gives and the eventual potential for finding a cure.
Andrea G.	I feel that ANYTHING is worth reaching out to for assistance. We already live with unknowns uncertainty.
Dick M.	SCA3 is an hereditary form of Ataxia that has affected my wife, her father, and here grandmother (that we know of). Our daughter also carries the gene, although she is not yet symptomatic. It is vital that we find a cure, or at least a treatment for this devastating condition. Even a partial treatment carries the potential for a first step to finding that cure. It is vitally important for this research to go on.
Marlee	It seems unfair to me to not give it a shot, one may never know what it could do to benefit and give hopes to those with SCA3.
Veronika I.	Anything what could help to increase the chance of a treatment should be considered carefully.
Anonymous	As my disease is prognosis — death , I feel that I should have the opportunity to decide whether I take a drug experimental or not.
Amy F.	As someone living with Ataxia, we'll take all the help we can get. We'll try anything that might improve our lives! Thank you for your consideration.
Ben B.	Let's try it, because even it helps only a little bit it is better then nothing.

NAME	COMMENT
Burton S.	I would ask the FDA to accept troriluzole as possibly beneficial in SCA3.
Celeste B.	My grandson has ataxia but we don't know how or why he has it. I do know that a bit of his cerebellum is smaller... causing a bit of shaking. I digress.. I know it's important for the FDA to approve this drug for the benefit of those suffering from this disease. My grandson is almost 20 and he's been diagnosed since he was 12. It is stable, but so many others aren't. Please, FDA members, please allow this drug to go through for all who will benefit from it. Thank you.
Don M.	I can't imagine what the FDA was thinking when it is a up to a patient and his Neurologist when contemplating taking the drug.
Leanna S.	This needs to be reviewed at least. It is life altering
Gilson José R.	My wife has SCA3. She is 51 years old and is very interested in all kind of researches that could reduce the symptoms of her disease. She is a fighter but I can see on my own eyes that her health is getting worse week after week, sometimes day after day. Nowadays the people that have neurodegenarative disease don´t have nothing, just their own strength to hold themselves. Any movement on the direction of a cure is a hope that helps them to continue fighting.
Denise B.	Ataxia sufferers need medicine to increase their quality of life.
Arthur R.	I have seen my wife go from a vibrant, happy enthusiastic person to a person who can't tend for themselves, any relief would be welcomed.
Kassia C.	My family has always been suffering with this ataxia, many members have died, and now my father is on wheelchair because of it, I'm already gelling the symptoms too, and it'd be so good, to have the hope the a medicine is coming to treat all these people. We need something to hold to.
Louise W.	I have a rare and debilitating ataxia, because it is degenerative I have no choice but to loose my dignity and suffer and slowly die. If any one can be saved from this, I support it.
Karina T.	It is very frustrating for us patients who wait anxiously and full of hope for treatment. Our disease progresses without pause and we do not have a treatment. From Uruguay, a 47-year-old SCA patient, I am very attentive to news. I have two daughters and I long for a treatment that gives us a promising future. Please review this. From a remote city in Uruguay, Nueva Palmira.
Angee B.	I feel this drug must get a trial, It may help family members who may get diagnosed in the future.
Elvecio C.	Having ataxia is affecting my life in every way, I can't get around well, I have difficulty speaking and it's getting worse quickly, this medicine would be hope.
Aluizio C.	Esse remédio é uma luz no fim do túnel para nós,tem que haver o máximo de facilidades para aumentar as chances de ele ser testado.
Dipti G.	My child had infantile onset Ataxia and treatments and availability of drugs that makes life of person with Ataxia is far to none so if there is studies showing to help FDA has a responsibility to at least read the studies.
Anita V.	I watched seven family members suffer and die from SCA. I believe they would have taken any drug available that showed promise of slowing the progression of this disease.
Joe K.	Systems need to be flexible to account for and reflect human empathy — we want to celebrate dynamism in our systems, and to do that: The decision makers need to treat each case with the empathy they would apply if their own child were suffering with Ataxia. Citizens want to have faith that we are uplifting and hiring decision makers that value accountability from a standpoint of empathy, before it affects those decision makers personally. If we truly champion the value of every life, we have to care: always.
Linda V.	I lost my brother at 48 to this awful disease. He had no hope whatsoever. Let's give those fighting this disease some hope.
Kate F.	All of us living with Ataxia would welcome further research - it can only help! If you find yourself with a degenerative condition and you are struggling to cope on a good day, just imagine the worry that you have with how you will manage in the future, as things get even worse...
Denise N.	It often takes risks to advance treatments and patients need your bravery now!
David M.	Any drug which benefits ataxia treatments should be used.
Janis S.	I am a friend of someone who has SCA3 and know she would be willing to try this drug.

NAME	COMMENT
Jennifer K.	With the terminal diagnosis of SCA3 it is imperative to find a solution quickly. Anything would be an acceptable trial treatment to an SCA patient such as myself. I would be pleased to try anything as a mother of 3, that could change this death sentence. Please reconsider a second look.
Linda A.	Please allow us the opportunity to slow or stop the progression of this debilitating condition.
Ashok K.	Please see from patient view you will realize need of treatment.
Barbara M.	I believe that anything which could possibly relieve Ataxia symptoms should be made available without delay.
Gilberto Adriano R.	All efforts of science, for the benefit of humanity, must be encouraged. There is no reason to create more difficulties for people with the disease known as ataxia.
Julie G.	I can no longer walk without pain nor carry on a conversation. And I am terribly off balance. And to think I used to be a super salesman. If a product comes on the market, what can it hurt? I am dead anyways!
Elton Antônio N.	This is very important. Hope to all people who have SCA3.
Joan M.	There is so much work to be done on neuroscience. Please take this very important first step. It is very frightening knowing you have a death threatening problem and no one can help.
Kim S.	I strongly believe the FDA should pass this new drug that has the possibility of helping people with SCA3. This horrible disease has struck my family for the 3rd time. My mother, brother both was taking at an early age and now my oldest nephew has been diagnosed almost 2 years ago. This can give many people a hopeful outlook on their ongoing struggle with daily living.
Adauane B.	Estacionar a doença é primordial na vida de minha amiga, e de outras pessoas com a doença.
Carol	To have Ataxia and the life changing implications that comes with this,myself I would try or trial anything or any medication that would possibly help to halt or make myself better!!! Living with ataxia is a hopeless life that is only understood fully by those who suffer with it.
Devon B.	All patients should have the opportunity to try any drug in this rare disorder land. I have a SCA1 and would most certainly try a trial drug.
Kathy M.	I don't understand why you would not approve every drug for such devastating neurodegenerative diseases. Imagine how you would feel in our shoes watching a disease slowly destroy your child over time.
Anthony L.	Denying people an opportunity is wrong.
Bryton S.	This condition has been incredibly fast and debilitating. Anything that we can do or try to slow or recover from this would be supported and appreciated
Amanda M.	Any treatment for this awful condition is so very badly needed, it's living in hell all the time.
Cindy H.	Since my mother had ataxia, it greatly impacted her way of life. She became depressed because ataxia made life unrecognizable. Any chance someone with ataxia can get to try something that can help is immensely helpful.
Jolene F.	My opinion is, we are all dying anyways. We would like some options to lessen the symptoms. Anything that we can try to help slow down the process would be greatly appreciated because everyone is so Desperate to get better. Even slightly better will do!
Geraldine W.	As a mother of 4 outstanding young children who has recently been made aware of this hereditary disease. Imagine my fear and concern for the other 3 siblings and their families. Only through my son's diligence has he been diagnosed and made aware of what the future holds. Any possible drug help is absolutely meaningful as well as the physical therapy he diligently uses to maintain a normal life. His family remains in severe damage if hereditacy pursues. Please commit any means possible to contain this life stealer.
K. S.	In my opinion every possible, non dangerous, drug should be permitted. My Ataxia is permanent. I am having no medication because there is no cure. But I will try ANYTHING to try to ease my discomfort and disability
José C.	Why the FDA don't want to review, the best for the patients!
Kim S.	I strongly believe the FDA should pass this new drug that has the possibility of helping people with SCA3. This horrible disease has struck my family for the 3rd time. My mother, brother both was taking at an early age and now my oldest nephew has been diagnosed almost 2 years ago. This can give many people a hopeful outlook on their ongoing struggle with daily living.

NAME	COMMENT
Derrick M.	I have multiple family members with SCA3 and want to see every possible resource utilized to help this and future generations of individuals with SCA3 to benefit from modern medicine.
Adélia F.	My son needs this medicina urgente please.
Angélica C.	Meu pai, faleceu em decorrência da doença (SCA3), em 1992, aos 52 anos. Meus 2 irmãos homens, faleceram em 2006 e 2008, aos 40 e 41 anos, respectivamente. Em 16 de julho último, minha irmã faleceu aos 56 anos. Todos, portadores da DMJ. Eu e minha irmã caçula, ainda não desenvolvemos sintomas e nem tivemos coragem em realizar o exame. Nós temos pressa!
Jose O.	At this moment, we tried everything and nothing works for my ataxia. I would rather try something and fail than live with no hope. The lack of hope is worse than the disease.
Carol M.	Anything that has a possibility of improving life is wonderful, and to make it hard to obtain is torture.
Brad S.	My friend has ataxia and would like to have access to these potentially life elongating drugs before it's too late.
Erin H.	SCA6 Involved in the Biohaven trial for troriluzole. I truly believe it helped me. I am currently taking Riluzole twice a day. If this med is not going to kill us, we (Ataxia Warriors) have NOTHING to loose! Please reconsider.
Brian B.	It's worthwhile trying a new drug properly, if people are willing.
LaVerne T.	I have a friend who has Ataxia and I know she is willing to try anything to help her live a longer life. She says that she is not in pain, but if it helps her mobility than give her the option to use it.
Xia Y.	I am an SCA3 patient with a severe condition and there is no effective medication available on the market. Previously, we have been paying attention to the research trends of troriluzole, hoping that the FDA can review this drug and give patients a hope and opportunity.
Carol B.	I think anyone with Ataxia would love the chance to have a drug like troriluzole, only find out it was turned down by the FDA.
Bruno	Sou portador da Ataxia tipo 3 e tenho um filho que também é. E tudo aquilo que tiver alguma chance (por mais que mínima) de trazer alguma melhora, serei sempre a favor.
Ellen J.	If this drug could extend the time my father uses a walker before he needs a wheelchair like his older sister, I would be really happy. It would be less scary for the next generation of our family if there was the possibility of a drug to slow symptoms.
Joan S.	I'm seeing the relentless progression of this disease in a vibrant friend. He is worth taking a chance. To do nothing will be fatal.
Fauzan A.	FDA should consider the single medicine till now which made for ataxia. At least FDAs, people would consider the griefness of our type group SCA3 patients
Deborah C.	It is a severely debilitating condition that affects the quality of life and mental health. Any chance, no matter how small, of any improvement could mean a lot to the sufferer as well as those caring for the patient who witness the effects and any decline.
Brian H.	Similar to flexibility with early medications for AIDS, there should be flexibility for other devastating diseases with no know cure at present.
Kevin L.	My wife and many of her relatives have SCA. Having witnessed the effects on the people I love, I believe its in their best interest to have the choice to participate in medical treatment
John Rafael C.	Please, we need flexibility to try to find the cure. That's the way science always worked.
Carolina T.	I suffer from SCA3 and I pray for a medicine that might cure or, at least, relieve the symptoms.
Helen H.	People affected with Ataxia are finding life incredibly difficult and it's important to work together to give them a chance for a treatment to give them hope for a better life!!!
Lawrence L.	Please reconsider your decision to not review the NDA for troriluzole. I am watching FA ravage my twin granddaughters and we need every tool available to fight the progression of this rare disease.
Ellie E.	The advances with the FDA for us living with ataxia is critical to review potential new drug treatments that could drastically improve our lives.

NAME	COMMENT
Joan O.	I am a woman of 69 years and have had Ataxia since my early 20s anything that can possibly make life easier we should be given the chance to try it out.
Lex K.	For suffering patient's sake, you should be more flexible to review the full data presented for the NDA filing for troriluzole.
J. C.	Any SCA3 patient will tell you that ANY hope offers them a better chance than nothing. Hope also allows for improved mental health and an overall improved quality of life SCA3 patients deserve.
Bridget P.	Why is Ataxia always on the back burner? Please, help
Caroline E.	My progressive neurodegenrative disease still has no drugs that successfully treat it. There has to be more flexibility so that we can build on what little we have in the way of medication.
Janice H.	Our family has experienced 3 generations of SCA2 and would be supportive of regulatory flexibility in our case as well as for those suffering with the other ataxias. Hope is much needed in these situations.
Doug B.	I want Hope. The FDA took some of that away.
Julie K.	My friend has ataxia and would like to have access to these potentially life elongating drugs before it's too late.
Ken J.	I have SCA2. It's progressive. My ability to walk is gone. Any drug that could possibly delay the continuing progressive nature of this disease would be beneficial.
Cecily J.	My son's father suffered for many years the effects of SCA3 before he passed away. And now my son has been diagnosed with this horrible illness. We can use as much help as there is available.
George G.	A friend has this debilitating, deadly, rare disease and wants the right to try this drug.
Joy P.	Please give the people living with SCA3 access to any shred of hope that is available. Otherwise there is nothing to live for.
Coll D.	Watching how a healthy body deteriorates with this disease is frustrating when all options to fight aren't provided.
Jonathan W.	I currently manage each symptom of cerebella ataxia individually, which is both costly and takes time! I feel that all potential treatments should be investigated that would improve my quality of life and allow me to make a more dynamic contribution to society.
Kelly P.	People with SCA3 deserve a chance. Please consider helping people diagnosed an opportunity to better their own lives and be there for the lives of their families and loved ones.
Camila O.	Acredito que esse medicamento seja muito importante para as pessoas diagnosticada com ataxia, visto que não tem medicamento para tratamento da mesma. Vivo isso em casa, minha mãe descobriu recentemente que tem ataxia e tem sido muito difícil dela e nós entender esse diagnóstico que a princípio não tem tratamento, e provavelmente e hereditário o que causa ainda mas medo, desejo sinceramente que aprovem a única esperança que temos.
Josh L.	I live with SCA3. I'm trying to still work but it especially is getting hard. I would like to try something to keep me on my feet even for a bit longer.
Elissa F.	We are suffering!! Please help. We understand that one drug works for some and not others.
Gabriely C.	I'm girlfriend of a person who has Ataxia. And he unfortunately lost his mom, his grandfather and two aunts to the same pathology; and part of his normal life too. The least of hope is an opportunity for the Ataxia community living.
Dr. Joshua R.	Every small bit can help. its parental (cruel in fact) to make this decision without consulting stakeholders.
Kelly S.	My family member has expressed and a need and desire for this drug. Her life has significant changed and been impacted by her illness.
Emily C.	I am living with a degenerative progressive disease. We need help. Some of us are just slowly dying. We need all the help we can get.
Ferdinand	Please review.
Judith G.	What's the harm in trying everything to stop this awful disease!

NAME	COMMENT
Jillian C.	Reviewing the drug for treatment for SCA3 may tremendously effect the disease and assist in saving lives.
Ernest L.	I've used riluzole and want to be able to use troriluzole.
Hrithik K.	I totally agree with the NAF, there is a ray of hope after several of years for ataxians for treatment, so kindly please do the helpful needs
Debbie G.	Please help ataxia patients as possible. They deserve to be heard and live their best lives. We need your help.
Girard P.	My wife died of Spinocerebellar Ataxia 10 years ago. We would have given anything to have a chance for some type of treatment.
Fernanda B.	Unbelievable that we are still trying to review... This is an urgent cause!!!! Please, review and accept troriluzole now!
Karla G.	I have ataxia and would like to have option to try medical treatment.
Nichole M.	My family member doesn't have this specific type of ataxia, but if there was a medication that could potentially slow the progression of her form I know she would want to try it. And even though this wouldn't help her, if it has the chance to help even a few people I think it is worth taking a closer look at.
Patsy G.	With no cure, any options to help mitigate this are invaluable.
Anonymous	Troriluzole works by the same mechanism as the already FDA approved (and no longer on patent) drug Riluzole. If it impacts SCA3 patients, its effects are modest and only impact the symptoms in patients (without impacting the natural history of the condition). While I am desperate to see FDA approved medications for SCA3 and all other ataxias, I do not think that this is the place to by fighting with the FDA. The drug (based on recent ALS and FA approvals) is likely to be very expensive (more than $100K/year) and if the benefits are not clear then I think continuing to trial Riluzole in SCA3 patients as an off-label indication is the right path forward until the efficacy of troriluzole is established.
Toni H.	I have been living with SCA for 15 years and there's, literally, no help from the FDA or government to help those inflicted with this condition. In layman's terms, it's like Parkinson's Disease and Multiple Sclerosis had a baby. My quality of life has been severely affected since I was 46. I cannot work because both my speech and motor skills have deteriorated.
Shalini G.	Ataxia is a disease that not only affects the patients but their entire family because how limited a patient is. FDA should approve all potential medications.
Celma G.	Esse medicamento vai nus ajudar a retardar a doença.
Nicole W.	I have family members that are high risk for hereditary SCA3 and through this family members with SCA3. Due to the progressive nature and severity of this disease I know they would take any opportunity to move forward with treatment regardless how strong the effects. Any thing is a move in the right direction- the direction of hope and some progress.
Alessandra	O remédio é importante pra qualquer enfermidade. Principalmente para doenças raras e degenativas.
Cibele A.	I live with my weakened mother at home, with depression for not being able to do anything, so the least chance of the medicine taking effect would be enough for our family. We agree to try a new medicine!
Jorge L.	Any chance to live better will be our option.
Nancy M.	My husband was on riluzole for 6 months and he would definitely try an advanced drug to help reduce the progression of this disease and help future patients. He is 50 now and is fighting this with exercise, supplements and diet and wants to be around as long as possible for me and our 17 year old son. Also he has other cousins with the disease that would also benefit and they could do a familial research study if they all took it. We do not know if my son has the gene. Anything new medication should be offered to people with this illness. Also knowing its efficacy is only determined by people taking it and it may lead to further advancement in the medication.
Luiz Alberto V.	Ataxia is a disease that slowly takes away hope from the patient. It affects your entire circle of friends and family. The slightest possibility of a drug for the disease to remain stable brings back that hope.
Daphne O.	My friend and coworker has ataxia and would like to have access to these potentially life elongating drugs before it's too late. I have watched, in just the few short years I have known her, her body become more and more affected by this disease. Her ability to move freely and her vision have increasingly become worse over such a short period of time. She spends her days as a Pediatric Occupational Therapist helping so many and deserves to have someone help her in return.

NAME	COMMENT
Diana L.	My nephew is 23 and lives in Canada and has been diagnosed with Friedrichs ataxia. If a trial medication would be available for him, he would feel hopeful at the chance of slowing or curing this debilitating disease as he often becomes depressed because he feels there's no hope of cure. I wish this medication could be available in Canada. I keep praying for a cure for him. He and many others need this hope to beat this disease and have the chance and hope of a healthy normal life.
Anonymous	Surely everyone has the right to be considered for a treatment that could possibly make a difference to someone's life when living with Ataxia. I'm sure anyone would appreciate the opportunity to try anything in the hope of living a normal life. What right do the FDA have to take this choice away!
Richard M.	Solutions from through DNA, measure of relief to Ataxia patients would be helpful. While the numbers are small, the effects on a persons life are very high. I heard the FDA to allow continued development of these drugs.
Troy K.	I'm 48 years old and living with SCA3 ataxia. I would love the opportunity to take a experimental drug to slow down the progression. As of now, I'm able to walk and move around, but my balance is effecting daily activities. I no long can play sports or much physical activity, because of my Ataxia. I know it's going to get worse as the years go on. I really would like the opportunity to take any type of drug that might help. Thank you.
Alexandra D.	Pela esperança de dar uma qualidade de vida e um possível início para cura.
Craig V.	Without trial and research of potential drugs there is no hope of ever getting cures or means of stopping this horrible affliction. Full review of the drug potential should be pursued before writing off its possibilities.
Jarred H.	My mother has SCA3 and currently the medication she takes is not specifically for ataxia and isn't effective. Her day to day like is difficult and lonely. She would be interested in taking medication as a trial as she is desperate to find medication that will help her and will hopefully help get her life back as she now suffers with depression because there is no hope for cure or reduction of effects.
Paola Z.	Any improvement in the clinical picture of an individual with a rare and progressive disease, brings immense happiness to the patient and/ or family besides providing the development of future treatments/medicines.
Monalisa S.	I have ataxia 3 and would like to try a remedy for this degenerative disease.
Robert L.	I am the last surviving member of our family that is diagnosed with ataxia. Six others have carried the curse of ataxia and passed. Several younger family members show early symptoms but prefer to believe they will escape the inheritance. I have little time left and nothing currently available will alter the course of my fate. My children have a 50/50 chance of inheriting ataxia from me. No one should expect a single silver bullet to stop the many version of ataxias dead in its' tracks. Incremental successes and occasional failures are to be expected even embraced. If creditable scientists believe they have developed a formula that will lighten my load as I transition to total incapacitation; I want it. If it so happens that the formula fails its' purpose; so be it. The failures bring us closer to success. The failures will do little to change my life but may make it possible for my children, grand children to escape my fate. Troriluzole is but one step; maybe backwards but hopefully forward. At the very least we will learn from it. I accept all the risk involved and I accept the results.
Melissa M.	What can it hurt to allow this? Why do you get to make that decision for others? Let us try what WE want to try to help us as you have no cure but we might find 1 without you.
Alessandro R.	Please this is a terrible disease and unfortunately it is hereditary. Why try to stop it?
Elizabeth M.	We, my family, have what I call the family curse, "Machado's Joseph's Disease." A child from a parent that has this has a 50/50 chance of having the curse. I've seen many loved ones struggle, with no hope for treatment. I know, if given a chance, even if small, to get relief and manageability, God willing, maybe even a cure. They would not have thought twice. If this treatment isn't allowed to be used, these people have no hope.
Aline G.	Tenho 37 anos de idade, faço 38 daqui a 2 meses, sou portadora da Sca3. Eu tenho uma filha de 14 anos de idade. Só quem tem essa doença sabe o quanto é difícil. Tá certo que todas as doenças não são fáceis, mas a ataxia vc depende de outras pessoas para tudo. #chóataxia.
Lynnette P.	Since Ataxia, particularly SCA3, has no medication that helps to date, the refusal by the FDA to review is puzzling. Sufferers need hope that something can be developed that can help us.
Melissa R.	My husband and I are thinking of starting a new family but it is terrifying to think that he may be hugely debilitated by the time we start one. We need medication and we need the FDA to be flexible with rare neurogenic diseases. We beg for mercy!

"The FDA has guidance that supports their flexibility for drug approvals in matters such as this. As a friend of NAF and those with SCA, we encourage the FDA to exercise that flexibility so people can access treatment. People are waiting."

— Hilary R.

NAME	COMMENT
Anonymous	My daughter has SCA and our lives for the past 10 plus years have been horrific. I've watched her deteriorate and it's been horrific for us. I wish more than anything that she could be given treatment. Until someone lives like this you cannot understand how awful life is. She should be married, have children and live independently. Instead she spends her days with her parents. Every day I read the papers and the internet to see if there's any breakthroughs. We can never be happy about anything because this illness has taken everything from us. We cannot bear to think of the future. Please give the SCA community some hope.
Shana F.	Please reconsider your decision and help us find treatment. To stop now would be cruel and sentencing us to a miserable life that could have had a chance of slowing the progression down. Though not the full effect, slowing it down means I may get to see my daughter get married, help with my grandchildren and live a full life.
Alberto R.	Porque me daría una nueva oportunidad para poder vivir y a todas mis dependencias.
Dan D.	Due to our experience with the disease, I support full exploration of any new drugs or therapies. My wife died from this disease at age 64 with a perfect mind. Anything that could prolong this terrible disease would be very helpful. Thanks so much.
Michael H.	As a very rare and progress disease that is almost completely debilitating, Ataxia research is chronically underfunded and needs as much support in new medicines as possible.
Nicola Topham	My Son was diagnosed with hereditary Ataxia when he was 16. He's 26 now. We would do anything to bring us nearer to a cure or even a drug to slow the progress. We get involved in surveys and would be happy to try a drug as this is a helpless situation.

NAME	COMMENT
Thomas M.	As someone who was diagnosed with spinocerebellar ataxia about 23 years ago, I would like to encourage opening the doors to all forms of legitimate research.
Sandra E.	I was first diagnosed with Ataxia, then a movement disorder neurologist diagnosed me with MSA-P, which is even more rare. Ataxia is part of my MSA. Anyone that is diagnosed with a rare disease is trying desperately to find anything that will help with progression or a possible cure.
Geovana C.	When I was a child, I always fell very easily and after I had my first period, other symptoms started to appear, little by little I lost balance and motor coordination, today I have a lot of difficulty walking and I can't stand alone, I'm always accompanied by someone and I use support from the walls and furniture in the house, in addition to shaking in the head.
Hailey L	My friend has ataxia and would like the opportunity to access these drugs before it's too late.
Altair L.	Estamos desesperados por qualquer remédio não tenho defeitos laterais será bem-vindo e ficamos com a esperança de pelo menos frear a doença.
David M.	Progressive Spinocerebellar Ataxias are unremitting handicaps to daily living. One is constantly adjusting to new levels of difficulty with balance and then coordination. Surely it can't get worse? Then it does. Though a rare disease the impact on its thousands of sufferers deserves the utmost care and research in pursuit of a cure. My Ataxia is hereditary. Though a cure seems unlikely in my lifetime, ongoing research is needed for the benefit of my children, and their children.
Suzanne H.	My sister, Edith Hayes is a 2 yr. victim of SCA3. She is 68 yrs. young. Because of SCA3 she is now fearful, reclusive. She would desperately like to try troriluzole, however experimental. I'm told children are afflicted also... Mr/s FDA denier: would you want to deny your 6 yr child any hope for treatment, however tentative?
Sandy R.	There is currently no possible hope of a drug that could make positive life altering changes for people with Ataxia. The FDA needs to review the New Drug Application for troriluzole as a potential treatment for SCA3.
Anonymous	When you live with the debilitating effects of a neurodegenerative condition your choices become starker. People should be allowed to consider the facts themselves and draw their own conclusions.
Nicholas H.	My 7 year old daughter has Friedreich's Ataxia, a rare progressive disease with no cure and no treatment for children. This decision indicates an unwillingness to have the flexibility and understanding necessary to progress towards a cure for disease like the one she struggles with daily. Please reconsider this rigid stance and try to understand the need for nuance with these rare diseases.
Bruce D.	Recently diagnosed with SCA27b, a disease not even known until 4 months ago. SCA3 has been identified for a while, and still no progress on treatment. Please help.
Jack B.	We have been living with ATAXIA since before they even had a name for it. My mother in law and members of her family had mobility issues and no Doctor could tell them what it was. The Doctors told them that they thought they had some kind of MS. My wife and I got married in 1980, she no signs of having any health problems. A couple years later we decided to start our own family. Shortly after our third child did my wife start to show signs of ATAXIA … and it wasn't until 1993-4 was there a proper test for ATAXIA. Long story short my wife passed away at the age of 52, we had three children of which two of our children also have ATAXIA. I am a father, husband who has been living with this condition by myself for the last 40 years. I've watched my wife and several of her aunts and uncles deteriorate until their body was no longer able to keep up with the fight. Now I am a caregiver to my two adult children, all these people would like is a chance … they like their family members before them would try anything even if it just slowed the progression of ATAXIA
Nancy V.	ANYTHING that slows down progression of this horrible disease would be great. And probably allow some to become contributing members of our society.
Margaret S.	I take Rilutek for SCA7 (since 2009) even though it is for ALS. Here it is 2023 and Im fairly healthy, despite my mobility and vision losses. Grant the same flexibility for any drug that may help - we have NO treatment for SCAs!
Fernando L.	Since the disease is degenerative, the person is already condemned to suffering and anguish, what's the problem in letting him test new drugs, letting him look for a cure?
Amarildo C.	Because there are many patients with ataxia...and they urgently need an effective treatment and the medicine is the only way to solve this problem.
Alice J.	If patients with SCA want to be considered and try a new drug trial then its up to them to take responsibility

NAME	COMMENT
Alfred M.	I am a 4th generation person living with SCA6. When I first started to notice something, I was 26. I eventually went to see Dr. Susan Bressman in NYC when I was 30. I was "asymptomatic," but I knew something was not right. I was officially diagnosed with SCA6 in the spring of 2023 at 58. I am also a Biomedical Engineer, so I discussed as a matter-of-fact manner that the men in my family who get SCA6 start to see the full effects in their late 50s to early 60s, so at 30, I have 30 years' worth of the Human Genome Project and advancements in research to develop a drug that either slows down the progression or cures the disease in its entirety. Obviously, that has not happened, and to pour salt in an open wound, a solution for COVID was developed in 10 months which clearly states if the medical/scientific world came together, what other diseases could they cure? SCA is a horrible disease in the sense that you live through a slow degeneration of your life, and it usually doesn't kill you but robs you of everything. My grandmother who also had this from 26-93 when she just gave up living to die in her sleep would always say "twice a child, once an adult." Any drug that can slow down the progression and or cure this disease is one where EVERY person living with an "INCURABLE" disease would take whatever, to live as "normal" a life they can and NOT feel like they are a burden by needing a family member, aide or assisted living location to live. Our lives are ones where we're not asking for a handout, we're asking for a chance to live. The fact that the FDA is preventing this is criminal!
Francine F.	I lost my father for this disease, my brother passed away last week, and now I know that I am sick. I truly believe that people can create a medicine for this, and I am super hopeful for that.
John A.	I was diagnosed in July 2023. I was told there is 'no cure'. I would enjoy a cure.
Robert B. Sr.	Ataxia patients experience unique challenges in obtaining early, accurate diagnosis. Having a medication when options are limited is absolutely critical. Having the disease is difficult enough. Denying them the CHOICE only makes it worse for patients suffering from the disease. Give these patients and their caregivers what they need to survive and have the quality of life others without the disease enjoy. It's their life. Let them live it.
Susan J.	My friend with this disease needs hope for herself and her children.
Xin J.	So disappointed.
Anonymous	I suffer from SCA1. Any opportunity to fully explore (and possibly alleviate the symptoms of) any drug that may be of use to fellow sufferers should be fully explored rather than sidelined for (probably) bureaucratic reasons that are easy to apply by those that don't have the condition.
Simon B.	It has taken a long time to diagnose my ataxia and until she was 15, it was not recognized that it is a (potentially) genetic condition. It has taken my entire lifetime to recognize, accept and deal with my symptoms and I feel that any 'official' activity is a step in the right direction.
Mary P.	I believe the rare disease Ataxia deserves the attention of other diseases like ALS, Parkinson's, etc.
Edward S.	I am living with Ataxia, as are 2 brothers and likely several of my children and grandchildren. Do everything you can to further potential treatments.
John H.	My grandson is physically collapsing in front of my eyes. It's heartbreaking.
Guilherme M.	I have a friend diagnosed with SCA3. She is a strong inspiration in my life, young, strong, and joyful despite the daily challenges. I have faith and hope that with the advancement of this medication, she will be able to enhance the vitality that is already a trademark in her life and further explore her own existence, reducing the risks and limitations imposed by this condition. With faith in God and trust in medicine, this medication could be the beginning of many happy stories!
Carole M.	How come the covid vaccine was rushed out regardless of all the adverse effects yet big pharma are stalling over meds that could actually make a difference to people's lives as opposed to killing them?
Sue R.	People living with the Syndrome are the Experts and their opinions should be respected.
Amanda M.	O remédio é importante para uma qualidade de vida dos portadores de Ataxia.
Fiona S.	All avenues should be explored. There are many types of ataxia, I have SCA Type 6, it is genetic, so if research was done into gene therapy it could help lots of sufferers of many diseases including Ataxia.
Maria Clara R.	I'm Brazilian and my grandmother died a few mouths ago because of the Ataxia, I'm 20 years old and I don't remember seeing her healthy. The ataxia is slow and cruel and I wish she could tried a medicine to get any better. I think other people with this illness would want to try it too, so I support this cause. But please, be careful so as not to worsen the living conditions of these people with those medicines.

NAME	COMMENT
Yuzalinda Mohd Y.	Please approve. I have seen my grandmother, my father, my 2 uncles and my sister suffer from this chronic SCA. I also being diagnosed with SCA3. — from Malaysia with love. Looks like there is never ending story.
Sonya K.	I am a psychological researcher with SCA3, diagnosed in 2020 and progressed substantially over the past three years. Even a slim chance that this drug will slow the insidious progression of SCA3 is priceless for the insider: it provides hope and bolsters the belief that the country is aware of the urgency and cares. "Change is greatly dependent on the willingness of the nonhandicapped to institute change." (Dembo et al., 1973)
Sally Gardner	I have SCA6 and my younger family members are still asymptomatic but genetically tested positive. We need help, please help!!!!
Pamela G.	My son has Ataxia. Specifically Niemann Pick Type C. Like those living with a loved one with Ataxia, we are very keen to try new potential / trial drugs. Our children don't live long, so we are very willing to try anything in the hope it may either cure, or at least slow down the progression if the disease. Please think outside the box, and treat the rare disease community differently. Thank you.
Parna M.	I am waiting for FDA to approve the drug. I've been on this trial since October 2020. Currently this is our only hope.
Meron G.	I have a family member who has SCA3 and I want any an all benefits of a new drug that could potentially work for my brother. You have no idea what life is for my brother his family and specially for his children to each him day in and say out! I beg you to do let this to hands of those who can use this drug ASAP. Time is running and we need help.
Nick B.	Ataxia is a blight on my life and I am prepared to try any potential drugs which might aid or alleviate symptoms.
Melvin E.	The drug might help me walk.
Marcello B.	Need this urgently.
Patrice L.	The individual's who refused leniency as to making the drug available to individuals without any options is clear that these i individuals do not know the consequences. Declining motor skill function, declining ability to speak, swallow. Being confined to a motorized wheelchair, use of a lift built in the ceiling to get in and out of bed and even shower or use the toilette. But the brutal reality of individuals who do not care to understand urgency in seeking help even with risks is painful.
Anonymous	I beg the FDA to accept the application for troriluzole. Any chance of treatment my daughter with ataxia cerebellum is necessary. I know that without a treatment soon she will be in a wheelchair. The decease is progressing really fast. With this decease time is crucial. Please don't take her chance of having a somewhat normal life.
Yolanda R.	Good day I am from Bermuda and I believe I am the first person diagnosed with SCA3. I would love to be able to try this medication as there is no cure or treatment for this devastating disease!
Tim S.	The FDA seem quick to dismiss the potential benefits of troriluzole and should allow for further testing.
Stephen E.	My wonderful and formerly very active sister-in-law is suffering from this debilitating disease. It is robbing her of everything she enjoys doing. Please allow the use of troriluzole for trials. There is so much to gain for future patients from these trials and some potential relief for those now suffering.
Anonymous	Please FDA we need to try this drug is our only chance!!!
Olbrich	This is such a debilitating disease and it seems to be growing in the amount of people becoming afflicted by it. I would think working on it before it gets to gigantic proportions would be in the interest of the civilization's advancement.
Mauro Henryque C.	I have SCA3, I lost my mother, two aunts and my grandfather to the same disease, and all these people wanted was the opportunity to have an alternative for them, and this medicine could be an alternative for those of us who never had any.
Judy A.	Patients with this debilitating condition should have every option available to them as doing nothing only insures a long and painful existence.
Dr. Best	I have Spinocerebellar Ataxia and know the difficulties in treating my own pains, etc. so to ensure adequate medication can benefit others, both mentally as physically. Since our FDA feels beneficial, others need to have access.

NAME	COMMENT
Yvonne G.	I have idiopathic cerebella ataxia and 100% agree that any medication should be given the opportunity to be reviewed! I would give anything to find a medication that would help me as I'm sure those with SCA3 would be happy too! Ataxia is a horrible condition and it's a fight every day living with it.
Aline F.	Todos tem direito a tratamento, a vida,é tentar ser mais forte com a esperança da chegada de uma medicação que ajuda.
Brad Y.	My experience is providing me with knowledge that is not being expressed. Why, I don't know. The condition of SCA3 is a progressive one. Which means it gets progressively worse. But, what is not being said is that the rate of worsening abilities accelerates as time goes on. In the last few years, the FDA approved TMS (Transcranial Magnetic Stimulation) as a treatment for MDD (Major Depressive Disorder). Most places that offer TMS, do so for the treatment of MDD only. However, I have read two papers that describe major improvement for conditions like PTSD, TBI, Ataxia, MAS (Multiple Atrophied Systems) and substance abuse. The FDA is a major obstacle for receiving TMS treatment for SCA. I've been trying to get TMS for two years.
Jill	I am mom to a son (9 years old) with a rare type of ataxia. This particular drug will not help him, but if it could help anyone who has to live with ataxia daily, I think they should have that chance. Every day is a fight, but my son is so brave. I would hope with more options and more studies I pray that someday my son could benefit from a drug that could make his life simpler and easier.
Anonymous	Anything that has any bit of potential to halt the progression is so important to those of us that suffer with the debilitating effects of Ataxia. It is terrible when one cannot control their own bodily movements to do even the most simple things one could do as a toddler and young child. Just imagine having difficulty swallowing, sleeping, standing, walking, coordination, fatigue … difficulty with everything and being dependent on others for everything.
Yue W.	Hi Reviewers: I am an SCA3 patient. Currently there is no treatment of this disease, from my perspective, I strongly agree FDA could approve troriluzole as only treatment of SCA3 Thanks for reviewing
Sharon K.	I have a friend who suffers from Ataxia r/t a neurodegenerative disease. She has no hope of a cure or even treatment for her symptoms. She and her family watch her grow worse every day. Surely review of troriluzole would be the least the FDA could do for her and others like her.
Jeane G.	As a person who has a rare autoimmune disease myself, I understand how those with SCA3 think they should be allowed to try any new therapies. I agree.
Marete E.	This is a rare disease with no cure right now. People with ataxia are suffering with this awful disease, along with family members. This illness strikes during the prime of people's life's. My husband realizes he will not live a long life, and he is extremely concerned about our children that are in their early twenties and his younger brother. We need some hope from the scientific community that we are not alone in our suffering and watching our loved ones suffer from this disease. We have watched generations suffer. Please helps us put an end to our suffering or give us some hope that the scientific community is with us and we are not alone.
Jóse Roberto A.	Our ataxia patients urgently need their medicine.
Claude E.	I would be ready to try new drugs on myself even though it may have secondary effects that are not fully understood yet, if there is a chance that the drugs in question could lessen the symptoms or slow the progression of the disease
Jean F.	My sister suffers from Ataxia. This disease has stolen her life. Please continue research and trial drugs.
Lucia P.	Any type of research / trial can only help.
Joy P.	I'm very sensitive to medications. I usually have adverse reactions. Example. Diamox gave me seizures. Even vitamins or Tylenol cause problems. I was diagnosed with EA2 in 2001 after 8 years of searching for answers. At the time genetic testing was not covered by insurance and was very expensive. 2002 my episodes eventually stopped. I went back to work and life. I wasn't give much information at that time. I was extremely ill. 2016 symptoms returned. I had to close my business and am now in a wheelchair part time. I've never known anyone else who has my condition. EA2 has been studied for a long time with little treatment available. I'm excited to know that a new medication might be available. It gives hope to so many suffering with Ataxia every day.
Kyle B.	I absolutely hate having SCA3!! It has literally ruined my life!
Dinesh S.	I want to get better and if it means trying a new drug untested yet I am wiling to try.

NAME	COMMENT
Anonymous	This disease is difficult to cope with as it is not yet fully understood and challenging to live with and manage. The fact that there is possibly a medication which could minimize some of the symptoms, but not available due to reasons not made clear, is unacceptable and potentially and unnecessarily crippling someone for the rest of their life.
Trevor F.	There are so few interventions that help, and even if one person is helped to some degree that is worth having. You need to keep hope for all patients and believing something may help you will give you an effective placebo effect. Having no intervention removes any actual or placebo effect of a drug.
Susan L.	My very dearest friend of 25 years was diagnosed with SCA8 several years ago. It has progressed to the point of a scooter and a wheelchair. She is only 69 years old and one of the most active, energetic, positive person I've ever known. Her husband is only 70, just retired as President of the NCAA and we are traveling on a cruise together next week. She is brave, but this is heartbreaking. She would absolutely be willing to try a drug that could possibly help her in some way. Her SCA8 is very rare, I understand, but she is so willing to take the plunge with a new drug if there is any chance she could improve....or God, please, be cured. Thank you for hearing my plea for my dear friend.
R. C.	Anything is worth trying.
Peter W.	There are so many things that are not yet understood about the full range of conditions that are labeled 'Ataxia' that any knowledge gained about potentially useful drugs helps push our understanding. I have late onset Ataxia, progressing slowly but inexorably. My Ataxia is probably genetic but it may instead be from some defect that can be treated. We need to grow our Ataxia knowledge wherever we can!
Morgain M.	Ataxia is such a deadly degenerative disease that needs to be treated on every front.
Melissa M.	I am a person suffering from SCA1 (not even type 3), so I have nothing to benefit from this decision- as do probably all of you. Even though it is not directly related to my disease, I find it very discouraging that you choose to let individuals with this devastating disease lose any chance at ANY relief. I can guarantee that no one is expecting a cure. We just want any improvement, even in the least. Most people aren't aware of this disease and haven't seen it. If you had, I think you would think twice about denying people any hope. It affects literally every aspect of your life and we all deserve to have hope and treatment.
James C.	Despair at the limited possibilities for treatment.
Celio C.	Creio que as pessoas tem o direito de se tratarem com resultados das mais avançadas pesquisas.
Bruno	Any chance of a treatment is welcome!
Ann M.	I have had Ataxia for over 25 years. At one time, I formed a support group, Ataxia Support Group of Northeast Florida. It has been disbanded for years due to several reasons (members have died. I am not able to host a meeting any longer, etc.) My primary doctor was Dr. Subramony at University of Florida but I have not seen him for several years. I've just accepted it is what it is and It's only going to get worse.
Lorna B.	Any treatment that can ease symptoms or indeed help to cure Ataxia would be welcomed. At present my husband has little relief from the muscle spasms and weakness. He would try anything to be able to sleep peacefully.
Rachel	My friend has ataxia and would like to have access to these potentially life elongating drugs. She needs the opportunity if anything for hope and a reason to keep getting up in the morning.
Rosemary M.	I have SCA1. I believe patients should have the choice to use experimental drugs for incurable diseases.
Shirley C.	The drugs to treat the Ataxia my sister is experiencing has helped, even though her neurologist wasn't certain that it would. That was 5 years ago, and she is progressing nicely. Please allow troriluzole to be used as a potential treatment. Remember how uncertain we were with COVID vaccines at the beginning. Grateful that we have this vaccine to lessen horrible symptoms and possibly save lives!
Willa M.	Need all the help we can get.
Anonymous	I am one of three sisters, both of my sisters have been diagnosed as having an Ataxia. They were first diagnosed with MS many years ago. I believe that a chance at having a treatment or preferably a cure for this devastating illness should be pursued at any cost.
Tim K.	Although I have mild symptoms of ataxia, ataxia is in my family. I would welcome all approaches to cure and prevent ataxia.
Sandra	I want to support my cousin, her husband and daughter.

NAME	COMMENT
Regan B.	People who live Ataxia SCA3 live with so much uncertainty already and a constant dread knowing what the end will be like for them. They are incredibly courageous people and informed and I know would only make informed decisions but willing to take the chance to try out the treatment of troriluzole especially given the promising results of the trials and research so far! Please approve this drug for some really desperate people and their families and friends who live in fear and constant dread too of the outcome of ataxia on their family / friends!
Norman E.	I've suffered from this disease for about 20 years now, I've been unable to work for about 5, although I know what to say in my head, it comes out as unintelligible, slurring gibberish and who gets really tired every day. I am on bended knee (unable to get up!!) praying, hoping nee begging you FDA to please reverse your decision and grant troriluzole NDA status for sufferers of SCA3, like me.
Michelle L.	I believe if the said treatment has been refused, that only attracts improvement. My clock is ticking, this improvement is only happening when the review is in place.
Jim N.	I feel as if we MUST do everything that we are capable of to help with this dreaded disease. My dear friend has inherited Ataxia from a parent and a grandparent. And there is fear that his children may begin to show signs of having this as well! When will it end??? When can my friend, once a multi-sport athlete, get better relief and live a better quality of life that he deserves?
Dale L.	Please help!!!
Anonymous	My father was diagnosed as SCA3 four years ago, which is inherited from his father. More than tens of members in his family have died or become disabled because of this disease. He also developed many typical systems like lack of balance and coordination and trouble swallowing. We really want a treatment (and troriluzole may be the most promising one so far) to slow the relentless progression of this condition or lessen symptoms.
Sharen M.	Anything that could prolong the quality of life should be made available for the person to have the right of refusal.
Michael W.	Since first being diagnosed some twelve years ago the effects of this condition have worsened to the point where now I am unable to walk without the aid of a stroller and then only over short distances. I find visual focusing very difficult unless I am sitting and my deteriorating spatial awareness and hand tremor make very clumsy i.e., knocking things over. My handwriting is now almost unreadable and without the help and support of my partner life would be very challenging indeed and ordinary routines impossible to cope with eg carrying a cup full of liquid.
Clarice P.	My father has ataxia and his life is being weakened with time and the evolution of the disease. He has a lot of will to live, but due to the advanced degree of ataxia he cannot.
Carolina C.	Quem não autorizou ainda, provavelmente não tem um membro na família que tenha a doença, qualquer melhora e de extrema importância. Por favor, liberem essa medicação o mais rápido possível. Obrigada
Natalie B.	Please review, this condition is relentless.
Susan S.	SCA6 has took over my life. I am a nurse of 25 years I have had to retire from being house supervisor at a level 2 trauma hospital because I fall and can't speak clearly. A drug to slow the process down or better yet to prevent the symptoms is needed. My 2 sisters have SCA6 my mother had it and I have a 24 year old daughter I am afraid will have it. Help please.
Robert B.	Although I don't have SCA3 I do have Cerebellar Ataxia which is also degenerative and progressive with age. We know that as yet all Ataxias are incurable any new drug to help relieve the many varied conditions must surely be paramount to the FDA. If it was any of you or your relatives I'd bet it'd be no holds barred! Please help! Regards.
Melanie D.	I have had symptoms of SCA8 for at least 3 years. I went through numerous tests to figure out what was going on. Luckily I had to see Genetics for Inherited Retina Dystrophy. 3 tests later (all genes) we had finally got results. I have dealt with falling or near falls, (finding ways to use stairs (on my bottom)), dizziness, lightheadedness, memory issues, gait and balance issues, spasms, swallowing, jerkiness of eyes, yearly eyes getting worse, weakness in legs & arms, etc. Not being able to walk alone (used to walk 2-3 miles 3-4 times a week. SCA8 people need help, too! Thank you!
Jalpa P.	Please consider.
Danny W.	SCA is an unforgiving disease. I would try anything if it meant my legs could potentially return to their preSCA usage. We all are tired of stumbling around with weak legs. Let us try something!!

NAME	COMMENT
Edward F.	To be able to breathe, walk, see, Correctly speak, Use my computer, Is my life or The ability to have a kid without worrying about passing this disease on. These are things I wish I had back.
Anup K.	At the age of 63 I am walking like an old man of 90 age. Having a hard time climbing on even foot path.
Anonymous	There are limited medications to treat Ataxia, in fact some people have no choices. Even if the drug is experimental there may be some benefit. It is the right of the individual to choose to take an experimental drug if they wish to. It may be too late to benefit some but by continuing research on the progressive illness it allows us to move forward in finding treatment choices for future individuals diagnosed with the disease.
Paul S.	I was part of the trial of this drug. I had no side effects for the 2+ years I was on troriluzole. While the benefits seemed to be minor, now that I've been off the drug for close to a year, it was worth taking.
Jean G.	Even if one life saved its worth the effort. I totally support this.
Louis R.	I was first diagnosed with this insidious condition 10 years ago. This disease has slowly taken from me everything that I loved to do. I was fortunate to retire from the FDNY in healthy condition. I could enjoy going on vacation, I was an avid fisherman, I enjoyed planting a vegetable garden, flower garden and cutting my lawn, driving and working on my classic car. Now I rarely leave my house. A wheelchair and a nursing home is what my future looks like unless some kind of treatment or medication is available. PLEASE HELP.
Hannah X.	I agree with NAF.

"I have been taking riluzole for my SCA3 and SCA8 for 3 years now and have seen improvement. I would love to take a medication that is actually FDA approved for my condition and I would only have to take it once a day. Quality of life increase for sure!"

— Christina N.

NAME	COMMENT
Wendy G.	Living with a progressive disease is physical loss, yes, but also the loss of our lives. We can't play sports, can't do most travel, and can't even walk with a cup of coffee to the living room without half of it sloshing onto the floor. Thank goodness for books. Bottom line when Ataxia emerges: Husbands leave us, our homes become too difficult to navigate, we can't drive any longer, others can't understand us because our speech slurs. In other words, our world gets smaller and smaller and smaller with each month of progression. We've been waiting with bated breath for the troriluzole. I'm behind NAF 100 percent.
Sheree M.	My 29 year old son is living with SCA3 and we would like the chance to try therapy. Thank you
Nancy K.	My ataxia is unknown. There really isn't any treatment for these types of diseases. I had a Corporate job in Biotech and I totally understand drug development and the FDA. I volunteered for years. I also hiked for years. I am taking an off label drug, and this troriluzole does not impact the liver. Can you imagine having a disease that has no known drug? It is truly terrible. Perhaps release and study the impacts over time. It is truly difficult to get a large study group that has a rare disease. And sometimes just hope is enough.
Matthew K.	Low incidence does not mean ataxia has any less of an impact on human life. Those living with SCA desperately need a treatment.
Carolina P.	Para meu amor ter um tratamento que lhe der esperanças.
Akhenaton D.	Research on medicines brings hope to patients and families, it cannot stop.
Gail C.	I have lost a brother and sister and 4 more family to SCA8 they all received a different diagnosis. I was diagnosed by the Mayo Clinic with a DNA test. I have had 2 doctors tell me they had never heard of it and I became so frustrated along my journey. I am a retired RN. I tried to tell numerous doctors I had some sort of inherited disorder but because I have non-insulin-dependent diabetes they immediately told me all my symptoms were coming from that. I am on numerous meds at the present time and none of them are controlling my pain. We need to have the option to try new meds quicker. Please consider this it is an awful thing to go from an active retiree to someone who loses their ability to walk and do most activities.
Lorena L.	I currently don't take anything for my Ataxia, but in the future when I need to I would like the option to take it. Since it's my body, I would like to be able to choose!
Stephen L.	Every avenue for treatment should be explored for a disease that is barely understood, relentless and incurable. Please create the possibility to investigate and use new treatments.
Scott P.	Please understand that there are many of us living with this disease and all we want is a chance, however slim at living longer in our present state.
Wendy J.	I just recently found out I have SCA3, I am starting to see subtle symptoms and would love to try anything if it would help to lower my symptoms by even a small amount. My mother had SCA3 as well and recently passed away last year after being in a nursing home for 8 years because we could no longer take care of her at home. She was on 64 years of age.
Anonymous	I have watched my friend suffer and slowly lose her ability to walk, speak, swallow and to just live a somewhat normal life. I would ask the FDA to please be flexible in the approach to new treatments for this disease and help those who are suffering have some choice in the matter. Thank you.
Tom C.	Please pass this drug! My wife struggles everyday with this disease and our three young kids have to see their mother go through this. I understand this is a every rare disease and not known by most people but it is the life we live. My wife has so much more life to live and would be willing to TRY ANYTHING TREATMENT OR MEDICINE POSSIBLE EVEN IF IT IS NOT APPROVED!!! Please help us how ever you can. Thank you for your time and concern.
Douglas G.	A drug reviewed by the FDA is one step closer to a global cure for all types.
Giovana A.	In fact, the faster the medicine comes, the better, but it is better if it comes more efficiently and with certainty that it will work or bring a positive effect.
PJ H.	All possibilities to learn and examine any avenue open to research for a potential cure and or help with Ataxia should not be discouraged.
Rebecca Z.	My husband has SCA Type 43. Any ataxia patient that might benefit from troriluzole should have access to this potential treatment, given there is currently no cure for this neurological disease.
Melody B.	Due to our experience with the disease, I support full exploration of any new drugs or therapies.

NAME	COMMENT
Sherri H.	I feel if there is a minuscule chance of something working it should be tried! I believe in leaving no stone unturned. If you have lost as much as I have to Ataxia, I believe you'd feel the same. I have lost two children, a MIL and my husband is currently living with Ataxia. If a drug or therapy has the potential to help people with Ataxia it should be given a chance and not hindered by unnecessary regulations.
Meredith M.	When it comes to rare diseases like Ataxia Type 3 and Spinocerebellar Ataxia Type 8 (SCA8), statistics fail to capture the immense love and hope we feel for our dear friend with ataxia. FDA flexibility approval may pose challenges, but we cannot let numbers define our fight for effective treatments. Each individual is unique and deserving of a chance for pliancy for drug approval. When faced with the rarity of Spinocerebellar Ataxia Type 8 (SCA8)or any form of Ataxia, it can be disheartening to consider the hurdles of obtaining FDA flexibility for drug approval. However, we must remember that love for our cherished ones cannot be measured in statistics alone. Every person affected by Ataxia, no matter the type, is an individual, with their own story, their own struggles, and their own unique hopes for a better future. It is crucial that we don't compare and contrast individuals based on percentages when selecting participant drugs for clinical trials. Instead, we must recognize the true value of each person and their right to hope for a chance at flexibility with drug approval. We would like to advocate for a system that considers the needs and aspirations of those affected by this insidious disease and work towards a future where love and compassion guide medical decisions.
Jamie P.	Even if the affect might be reduced then lots of people would be willing to take a drug knowing that it may have some positive effect? I have SCA6 and would have this attitude.
João Dias P.	I see how difficult it is for my friend to live with this disease and the importance of a medication for it.
Laurel G.	Advocating for those that have limited options and are trying to prolong their deteriorating physical abilities.
Vanessa J.	By closing the door on a treatment for SCA3 you also close the door for potential treatments of all other SCA candidates. I find that the patient and family can make a decision for medication use for their own bodies and if it is helpful or not is a very private and personal experience. No two people are alike. With such debilitating experiences in the SCA family closing the door seems cruel. Any help even a little should be pursued for all that want to try it.
Anonymous	This is a rare fast progressive disease and any chance to live a relatively normal life should be given when the chance arises. The affects of the disease are not just physical but emotional also and this then needs to be treated costing more in the long run. People living with this awful disease deserve to be given a chance.
Steven T.	Knowing a friend whose husband has the condition, I firmly believe that any attempt to address the condition/illness with a new drug MUST be a good thing, and further and wider trials should go ahead.
Mark E.	This is essential given that it is nearly impossible to create truly double blind trials with such rare diseases. Living with a spouse suffering from SCA I know full well that she would happily deal with the risks of side effects.
Nina W.	So many folks, like my family are affected by these under studied and under cared about diseases. My dad and several other family members have lived and died with ataxia and would have been glad at any chance to improve. I do not even know my status but have participated in a university study in hopes of improving our chances of new drugs for ataxia.
Lucas A.	This disease is crippling, and it is agonizing to watch to watch my favorite people in the world wither away into almost nothing at such young ages. My mom was 54 when she passed away this past February, and my brother who inherited this wretched disease is only 30 years old and is not far off from needing a walker just to get around in his normal everyday life. This disease does not just affect any one single individual but it affects everyone in the persons orbit, from doing the smallest of tasks like getting ready for the day to finding transportation to and from work, to even just go out and go shopping. It requires a massive commitment and a level patience that most people/families are not blessed with. I only hope that I will one day see my bother walk normally, I would even suffice (I'm sure he would as well) with the disease just being stopped at its current progression. Thank you for your time.
Lorraine C.	Anything that has potential to help SCA3 and possibly other SCAs has to be worth a try.
Sarah W.	This is such a rare and baffling condition. Please open avenues for treatments that give us hope and that could benefit our loved ones. Any research that happens now will also benefit future sufferers of ataxia.
Adriana	Brasil need medicacion.
Bruno F.	Espero que chegue logo ao Brasil, este medicamento , para que melhore a qualidade de vida dos pacientes.

NAME	COMMENT
Anonymous	Sincerely hope you will rethink your decision on your ruling, as our lives are devastated with these rare diseases with no treatment and no cure. You have to give all of us hope, is the thread human life hangs on to hope, believe, our community of rare diseases is a hopeful faithful community. We need your support desperately please rethink your decision!
Xiaosong P.	My wife really needs this medicine to give her hope to live better. Thank you!
Teresa H.	My 21 year old son was diagnosed with a neuromuscular disorder called Friedreich's Ataxia at age 10 and it was heartbreaking to watch him lose the ability to play sports, like golfing, that he was so good at and enjoyed so much. Every year since age 10 he has lost abilities and/or mobility in one way or another due to what this disorder is doing to his body. If there was a drug that would help even just a little bit to slow the progression would be life changing.
Paul L.	Having more options Doctors need to treat is needed. Not getting government blessings for this is the appearance of a bureaucratic mess that is eroding our trust in government. Stop hindering efforts that are desperately needed.
Ruth C.	Although I have SCA8, I believe that any medication should get a full review.
Jean B.	This review can give those of us, with this progressive disease, a bit of hope for our future.
Janet G.	Now, I have no hope for myself and my children. I lost the ability to walk unassisted by equipment, I am losing the ability to feel with my hands or feet and all I have to look forward to is further decline. I want hope for myself and my children.
Sharon	Please understand that we have not much solution available to us and these medicines is for our kids that we gave this sickness? PLEASE approve this medicine PLEASE
Preetkaran R.	I have been my friend's life come to halt since they have found at that they are at risk to the disease, and plus knowing that it has no cure available makes this worse as it affects their mental health as well. I would encourage FDA to review this decision, and it can help many people like my friend who at the moment are counting days instead of believing that they can survive this disease.
Alynara M.	Significa a esperança de uma vida melhor, mesmo com essa doença tão devastadora!
Nancy K.	I have lost a mother-in-law, husband, sister-in-law, brother-in-law, and niece so far to SCA3. Another in-law and niece have been diagnosed with SCA3. All of them would have/will be willing to try anything that will be even somewhat helpful to this horrible disease. Having witnessed so many of my husband's family go through life with this disease, I also want to see some progress — ANY PROGRESS — with anything that will help in any way. I support all potential treatments for SCA3 so I do not have to witness any more family members go through this.
Danny W.	SCA is an unforgiving disease. I would try anything if it meant my legs could potentially return to their preSCA usage. We all are tired of stumbling around with weak legs. Let us try something!!
Mary N.	As an individual with a rare disease that has no cure, I find value in SOME relief/symptom improvement. Because ataxia can present differently or progress differently for each person, I find it very valuable to hear the patient voice/story/perspective. We don't have a cure, but sometimes if we can collaborate, we can find meaningful treatment or benefit.
Alberto P.	We are in a hurry.
Natasha W.	Those that live with this each and every day should be able to have the option to try.
Toni E.	My specific type has not yet been determined, however if there is even a chance that this may stop or slow progress of such a horrible disease, it must be up to the person living with it to decide if they want to take the medication.
Danrlei N.	My name is Danrlei, and I'm living with my brother and mom with SCA3, and during the life we have faced with the difficulties caused by ataxia SCA3. And our big hope is the medical develop a medication that can stop the symptoms of the diagnostic. We trusting in god, and medical for this.
James C.	My ataxia is progressing and I look forward to a cure.
Matthew L.	I've seen the impact of SCA on family members, and I am now experiencing symptoms myself. It's a brutal way to live. Any treatment that offers the possibility of improved quality of life is worth exploring.
Alice J.	Should review and comment about any new possible treatments for any of the SCA conditions

NAME	COMMENT
Susan M.	I watched the love of my life die waiting for a drug of some kind to stop progression of this terrible disease. My husband waited and fought for 20 yrs. Now I have to watch my son go through the same thing and probably my daughters too. It's not fair why does it take so long please help us at lease stop progression if not cure it please, please.
Michael W.	I have an unknown variant of SCA, I do wish for some treatment, if not for myself, but also for others affected by this. I am 61 years old, I've watched my family, whom have all passed, suffer through this & chose not to have children so as to hopefully end, at least one branch of this nightmare. It would be nice if that was an option not necessary
Paul N.	All forms of Ataxia deserve more attention and research. My ataxia was recently discovered to be SCA27B. My mobility is severely reduced. I expect to confined to using a wheelchair in the future. Hoping to start a treatment of 4-AP n the future.
Amado I.	No other drug has even shown a propensity to alleviate symptoms of SCA3.
Camila S.	A mefica cão pode ser a nossa única esperança.
Maria F.	I'm Portuguese and even though I've ataxia, I do not have SCA3. I've another type of ataxia: FA. But nevertheless, I do believe that we have to start somewhere and sooner rather than latter.
Cyrina N.	My mother has Ataxia. I am the unlucky child (50% chance) that also has it. My children can also get Ataxia. Let's give our children, on down the line, hope of not experiencing what I do every single day. This disease is debilitating. I can't walk without assistance, people look at me as if i am drunk (slurred speech, not able to balance, and a very "clunky" gate). My loved ones around, help me. However, I am only 58 and have so many limitations as to what I can do, or participate with my grandchildren. It truly breaks my heart each and every day. Knowing I just have to live with this, defeats me!
Andre P.	SCA3 is truly devastating — we need urgent help!
Rocio W.	Even though I am an Ataxia patient, I have type 6 — or SCA6 and know what living with a rare disease is, so I know this particular medication won't be the "cure" for what SCA3 is doing to their bodies (if it has manifested already) or for the symptomless ones a hope for both of them to delay further damage. So please re-consider the troriluzole as a potential treatment for SCA3, thank you.
Ellen	My mom had Ataxia and all she wanted was something to make her feel better. Anything would have helped even if it made it worse. No person should suffer like that. A little hope goes a long way. She would have loved to know someone is doing something about this horrible disease. I miss my mom.
Anonymous	The heartbreak and stress of this disease warrants more flexibility by FDA. Ataxia is a living nightmare. Any drug that might offer even the slightest relief should not be denied by people not experiencing this hell on earth.
Swarashree M.	Living with SCA3 is very difficult. Need to try something which can benefit.
Steven M.	My Father and all four of my older sisters have been diagnosed with SCA3. My wife and I currently care for two sisters while my Father and two other sisters have passed. I have watched each of their world and lives slowly shrink as the progression of SCA takes away the ability to walk with balance, stand, eat, swallow, speak, go to the bathroom without assistance and finally succumb to on of many opportunistic ailments that result in injury, infection etc. Those of us who live with the reality of SCA are eager to embrace the work of scientist who are formulating treatments to intercede on this life stealing disorder. Please extend the flexibility needed to give those with no opportunity the chance to put up a defense and ultimately fight back and win.
Cleide Regina A.	I believe that medicine is very important to ataxia treatment.
Bob J.	Due to the small number of cases reported, it is essential that those of us who suffer with this debilitating disease be offered any thread of hope.
Miguel N.	I am tired of SCA3, please think of us. Without any medicine something else with benefice are welcome..
Isabelle W.	Why can't anyone try under "the right to try" that was passed by the government. As a person with SCA50 in the US no drug company will likely look for treatments as it was recently designated in 2022. I want the right to try other Ataxia treatments.
Rodrigo Barbosa O.	I believe in FDA attention about this problem.
Amanda M.	I'm upset watching my dear friend suffer.

NAME	COMMENT
Val G.	My niece who was only 30 passed away from this horrible debilitating disease recently. I have also lost 2 other family members and have 4 more currently living with ataxia. So many years have passed by with no hope of a treatment. More delays are devastating.
Qusai A.	I believe that it is unfair for Ataxia patients to stay with progressive deadly disease when there is hope with a medication available to try.
Rebecca L.	This is extremely concerning for me and my family that drugs which could potentially help our situation are being scrapped. I have a father with Ataxia who is immobile and my sister who has the gene. My whole life has been affected by Ataxia and to abandon such a huge step for any treatment is extremely upsetting.
Dorothy H.	Patients should have right to try.
Renu S.	I was given 4 doses of the Biogen drug last year from August to November. It was administered into my spine through lumbar puncture. I can truthfully say that I feel I got the drug and not the placebo… it was a very helpful drug…. I think I really benefited from it. It seems to have some long term benefits as to this day, a year later, I do not have the relentlessness coughing associated with SCA3 and the slurred speech. It is a pity Biogen dropped the trial altogether. Before that I was enrolled in a trial for troriluzole and I think I benefited from the drug therapy and was very disappointed that it wasn't approved. I think it (my friends said) I was more stable and less ataxic in my gait… please consider the drug again…. It may have more benefits than can be helped to the SCA3 community and people like me.
İrfan Ö.	Knowing that there is a small hope instead of waiting desperately changes a lot in our lives.
Ruth P.	Would be willing to try anything to make this awful condition better.
Terri V.	I have participated in 2 drug trials the past few years. The first wasn't tolerable for me, but the current one appears to be actually slowing the progression of the disease. However, it may be too early to tell, but I'll continue until it proves otherwise, for the sake of my children and grandchildren, in the event they also carry the gene.
Susanne Z.	The disease is so debilitating that those suffering from it really need all the support they can get. Thank you.
Virginia K.	This may not be perfect but it is a chance for some.
Pamela F.	I am having to use a walker and I will eventually be in a wheelchair. My main issue is I cannot be a grandma. I have a new grand baby and I can't even hold her or play with her. I have tremors that keep me from doing most everything. I will eventually be dependent on someone to do everything and that is heart breaking. My balance is getting worse and falling is becoming more frequent. Bruises are the result but eventually the falls will be more severe and that scares me. I have had to quit my job and become disabled, dependent on the government, which is another issue. I have been participating in as many trials as I can in hopes of developing a medication to slow or maybe even stop the progression of this disease. I have had MRI's and even suffered through lumbar punctures to help provide information to doctors, nurses and researchers in hopes of developing a drug. Please don't let the many years I have dedicated to these trials be in vain.
Ana C.	We need to have more options for the people that live with SCA3.
Daniela C.	I believe that anyone who has a disease like Ataxia needs attention and care, as the symptoms are cruel and the weakness is relentless. A little bit of hope for carriers is already a great advance in their quality of life.
Patricia G.	Anything that can do should be done. This is a fate worse than death. I would rather I'd been told that I had cancer at least there's a chance of beating it.
Victor I.	I have been taking Riluzole 100mg (2x50mg tabs) bid for the past 4 years. I am a Canadian living with SCA3 (diagnosed) for the last 9 years. I believe that riluzole eliminates my bilateral knee pain thus making me mobile. I also believe the riluzole increases my daytime energy.
Ray T.	Charities should not be advocating for drug companies. If troriluzole was effective, the previous trial data would have shown that. This drug has had multiple applications for multiple diseases and was not able to meet endpoints. No one benefits from drug approval of any drug that does not work.
Anonymous	After having to deal with all the various stages of this disease while holding out hope for some type of help in this battle...I can only hope for a reversal of your decision!!
Wolney M.	First, it must be examined whether it causes harm to health (if it causes harm, it must be banned). Second, with more time and safety, complementary assessments can be carried out. We can't waste time!

NAME	COMMENT
Victoria W.	This debilitating disease slowly takes away a person's ability to care for themselves until they parish. Reviewing full data for any drug that may provide some help is imperative. We would rather have something that may help, than no options at all.
Anonymous	Any new drug or potential treatment should be reviewed as the consequences of living with the condition is terrible and the affects are life changing as anyone who suffers from this horrible condition with tell you, We owe it to futher suffers to do all we can to help.
Tamara V.	There is no drug on the market to even try for my ataxia and I would be willing to try it to help me with my symptoms! Please don't deny us this opportunity!
Nirvana R.	Any chance given to help my family member get a little relief with this disease is better than none.
Ana L.	These people deserve a chance to live or improve their life conditions.
Brenda C.	My mother had SCA12. The slow progression of watching her waste away and this was years, if there is even a small chance they there is a way to help SCA people should be given a chance to try. Nothing was able to help my mother. When she passed in 2001, we donated her brain to help with the research for SCA, and hopefully find something to help. My sister has it, she no longer can walk or use her hands, but thank God she still has her mind. Help is needed desperately. Thank you daughter of Gloria Herman.
Michelle	I watched my beloved mother wither and die from Ataxia. It was brutal and she suffered. She suffered being locked in a body that no longer worked. She was so scared and there was nothing we could do. No treatments or even hope. Even an experimental drug could have given hope. She could have helped the next generation who suffer. Maybe even me, her daughter.
Melody B.	Due to our experience with the disease, I support full exploration of any new drugs or therapies.
Fernanda S.	Any chance of cure is worth trying.
Julie P.	It would be great to have a drug on the market in the UK otherwise we are living without hope.
Matt H.	I was diagnosed with Gait Ataxia in 2022 such a rare disability should have as much research as possible put into it.
Parna M.	I have SCA2 and am in the drug trial. I think there is a mildly positive effect of the drug which an ataxian cannot forego.
Daniel C.	While I do not have SCA3, I do have spinal cerebellum ataxia. I had this problem for 42 years. I do need some relief.
Fernando O.	Hope all patients can have the medication.
Marcia R.	Hope to slow down the symptoms of Ataxia and patients can have a quality of life.
Beatriz F.	Porque es una nueva posibilidad de vida para las personas con Ataxia.
Colleen S.	It really angers me because I feel like they are putting SCA on the back burner. I have lived with this disease for quite some time. My sister also has it and I have gone everywhere to seek out a support group in this area and nobody knows anything about it. I get very, very discouraged, and very angry, because my condition is getting worse and worse. I'd swim on a daily basis or do some kind of activity physical activity every day in hopes that it may slow the progression down.
Lisa D.	This is so hopeless. Ataxia effects the whole family.
Roger L.	I have SCA3. I would like to see a treatment available because this disease has a chance to pass to the next generation, to my daughter. So, I want an end to this disease. I don't want the disease to continue through the generations, because it's such a hard disease.
Anonymous	Very confused due to illness. Having a fighting spire never giving up hope.
Tracy T.	I feel that the benefits would outweigh the risks of trialing the drug. Something is better than nothing and right now as it goes there's no hope for the people with ataxia.
Monica L.	Having read the study result and the NAF summary. I believe people living with SCA3 deserve the opportunity of a better life by making this drug available.
Fabiana N.	It's a great hope for so wonderful people, that's dying and losing quality of life cause these disease. It's so cruel and sadly. Any help is very useful and welcome.

NAME	COMMENT
Steven F.	Dear Sir/Madam, In living with idiopathic spinal cerebellar ataxia, even though I am a 70 year old senior citizen, I would welcome the opportunity to have the implementation and infusion of the drug which MAY lead to an 'eventual' reduction of symptoms and increased function of the cerebellum. Since an immediacy of vital information provided can be SLOWED by the current position of the FDA, I would HOPE there would be a 'reconsideration' of its position though SCA3 ataxia is a 'relatively' small sampling considering the number of cases in this 'orphan' disease.
Mauricio M.	THIS medication is VERY IMPORTANT for MY FRIEND with SCA3.
Igor Q.	Well, SCA3 is a terrible sickness. I've seen It for many years. My mom's friend died a terrifying death, her son died too and now her daughter and the younger son are fighting the same disease. They deserve a fighting chance, thats whats I think.
Anders L.	I suffer from SCA8, but I also have problems with walking and talking.
Margaret H.	This is such a debilitating disease the individual should be able to make the decision to take the drug or not. It should be their choice.
Nancy M.	I have Ataxia and I don't know what type I have but though support groups I know people that do. I had to relocate from my hometown, friends and family from NY to SC. I went to two Culinary schools and I know first hand how Ataxia affected EVERY aspect of my life. So, it's not fair if there is something out there that can do something! My Dad had Pulmonary Fibrosis. He was in a clinical trial. They gave him the placebo and soon after he died (which will be 10 years ago on August 19, my Moms birthday), it was approved by the FDA. This is simply INFURIATING!!!
Susan R.	Let's try anything that might work. This is a miserable disease.
Anonymous	I have a cerebellum ataxia this disease steals my life.
Carolina N.	Precisamos da medicação com urgência!!!
Barry J.	It would be up to the patients and their medical team to assess the benefits and risks. Monitoring of its use would be of assistance to making its use more effective or to be withdrawn.
Edward S.	Yes I have SCA3 I would love try this.
Melinda M.	My 10 year old doesn't have SCA3 but he does have SCA2, which currently does not have any medication to help slow the progression of symptoms. If there was a medication that could have the potential to help... I would 100% sign my son up to try it to give him a fighting chance of normalcy and a lomger life span. The potential for help outweighs the concern of his symptoms getting worse- my son will eventually pass away from this condition. If there is anything I can do to give him a fighting hope, I will!
Sanaz A.	Living with ataxia is already a challenge, we need to use our good clinical practice knowledge and try to be more helpful to this population.
Regan	As someone who recently had their grandma pass away to this disease in December of 2022. And now finding out my own mother tested positive for SCA3, I want her to be able to live a long happy life with the possibility of medication that may help her while she battles this as well as diminish her symptoms with the help of medication to further help other people like my generation or even hers.
Lisiane N.	I believe that this drug will help a lot the ataxians.
António M.	Any hope is welcome, whether slowing or stopping symptoms. I am 62, old enough to make a choice.
Carlos E.	I would love to have hope for better days. We should be given that chance.
Mike C.	Having ataxia has severely limited my confidence and ability to maintain an active lifestyle and engage in any relationship.
Susan H.	I am 55. This is absolutely necessary. My father, my aunt, my entire family has suffered with this debilitating disease. Please reconsider.
Anonymous	Anything, absolutely anything that focuses any chance (and however small that chance maybe) of a better understanding of this condition is warranted.
Mitali D.	I am looking forward for a drug which would cure SCA3 keenly
John W.	We don't understand my disease but do know it ends in a terrible end of life. Please approve virtually any drug which is safe and may (not "does") help.

NAME	COMMENT
Shivani D.	For unmet needs, like in all forms of Ataxia, the clinical efficacy hurdle should be much lower with no compromise to safety to give some treatment options to the patients.
Ian H.	From my perspective, there just seems no hope that research or testing medication will come my way in time to help halt, or at least slow down this relentless condition. I am willing and ready to participate in any research project that may help myself or others.
Anthony M.	People are suffering and in need of options.
Celeste E.	Any hope is welcome with a disease that has no cure.
Maria G.	We need drugs for SCA3!
Sally P.	My husband died of SCA3 and my 40 year old daughter has the disease. This community needs hope. To be allowed to try a drug with an uncertain outcome provides that hope.
T. C.	I believe those suffering from Ataxia SCA3 should be able to have access to the troriluzole. I have seen the devastating loss of loved ones whose families and friends have endured the long progression of this fatal illness.
Anonymous	I really think that they should review the new drug, just because I am the with the disease, they don't have a clue how this affect me and others who live with this disease everyday. So if there is a treatment for SCA3 why not review it. I have SCA3 and I would do anything to have my balance back just to play with my kids, walk my dog go anywhere where everyone not thinking I'm drunk or on any drugs. All I ask is please review troriluzole it won't affect or hurt the FDA, the FDA must don't know how many lives that they can change. If troriluzole doesn't meet the requirements send it back to the drawing board but please review it before turning it down. That's all everyone with SCA3 ask. Thanks for your time to let me express myself.
Raquel O.	Because my best friend suffers from Ataxia and I would like her to have access to a better quality of life and if this medicine can accomplish that, I support the petition.
Anthony P.	People with ataxia seem to be pushed aside probably the name ataxia isn't well known.
Deanna P.	I have SCA2. Although I would not try this drug, I firmly believe that people should be able to try any drug that might help them. Drug Companies might find a way to help me with SCA2 and I want to make sure I could try it.
Ron A.	I'm dying of SCA please give us a chance.
Stephanie B.	There is NOTHING — please give us SOMETHING!
Tina C.	Why, why, why not???? My HEALTHY WORKING 29 year old brother was told at diagnosis, THERE IS NOTHING WE CAN DO FOR YOU! NOTHING!!!!!!!!!!! YOU SHALL END UP IN A WHEELCHAIR!!! He lived with this for 14years before he starved to death. That is not an exaggeration of the outcome. He was trapped in a body that no longer functioned. His brain was well aware. People need hope.
Lara S.	Because this medicine would change a lot the quality of the person with the disease, so the medicine could slightly increase the quality of life of the person.
Daryl N.	I want my friend Shana to experience a full and fruitful life!!!
Donna D.	I have SCA7, but I as probably most of the ataxia, would be willing to at try the drug, I can't believe as much as we have to deal in life you would deny this drug. If there is anything to try, I feel like there should be an option to at least TRY.
Shelly W.	SCA3 patients should have the right to try!!
Reed H.	My mother's SCA3 has rapidly progressed even in the past few months. Any chance we have to slow its progression or halt it is important.
Byron	Give us a chance.
Donna B.	Research for all forms of ataxia needs to be funded better and the potential for new drug and other therapies realized. The progression of the disease is robbing our children of a full life.
Laryssa C.	I BELIEVE that is medicine is very important to ataxia treatment.
Jodie K.	As someone who is living with a rare disease, we need something that will give us hope. This drug definitely needs further testing anything that we can try that will help. What a struggle.

NAME	COMMENT
Weiyan X.	I'm one of the patients who suffered from SCA3 a lot. I wish FDA can pass the New Drug Application for troriluzole because this potential treatment is a dead needy for us patients and make us patients have faith in lives.
Anonymous	I have witnessed at first hand the devastation that this cruel disease has wreaked upon a whole family. We need to strive in every way possible to allow the advances in medicine and medication to help the people living a reduced life. Please help these people and allow trials to be delivered.
Susan C.	A good friend has Ataxia and she should have the right to take this drug with its known risks. Ataxia kills so this drug is a worthwhile gamble.
Omar P.	Since this rare disease doesn't have any cure so far, this drug is the closest medication we have found as a cure so my family members want to try their chances with this drug, please let us use this drug FDA.
Michael B.	Ataxia takes the lives of those who suffer from the disease but also those who care for those individuals. I watched a woman battle with the disease for 20 years and eventually she starved to death after two weeks once being removed from life support.
Bindu	Time is running out with having this progressive disease. We need science to move forward and help.
Daniel B.	This drug is important that most quality of life.
Ronald H.	I have lived with ataxia for 17 years. I know what happens to your quality of life.

"While I generally agree with the idea of stringent safeguards, I am puzzled by the FDA's decision to not perform a full review of troriluzole. Rare and debilitating diseases, with no cure or even a therapy to slow down progression, are a special case. I believe the FDA is doing a disservice to the general public by recognizing neither the lack of treatment availability, nor the prohibitive cost of developing viable therapies that would give hope to the rare disease community. I watched my mother fade into a shadow of her former self and die last year, knowing I am headed for the same fate unless policies to aggressively promote reasearch are put in place and treatments made available.

— Mark W.

NAME	COMMENT
Xiao	I believe troriluzole as a potential treatment for SCA3. Please review their applicant.
Maria Felícia R.	People with ataxia have the right to try to have a better quality of life with dignity. This medication and other studies on the subject is the hope for these family.
Donna R.	I have close friends that are afflicted with Ataxia and I see firsthand how debilitating it is and these people would try anything to stop Ataxia in it's tracks!
Miki K.	People have Ataxia and family need support due to long terminal illness similar to Parkinson. We should be deserving of equal support and finding cures.
Terry	Please help fight this horrible disease.
Raphael Farias B.	I have a relative with this disease and this is very struggling to her. I can see on day to day life that she has a lot o problems for walking due to this condition. I hope this drug will be approved so my aunt and other people can have at least a chance to be better.
Bruna V.	Bom, precisamos do remédio pra ter uma qualidade de vida melhor com menos medo do futuro.
William A.	As I read the application, there were 218 enrollees; only 41% had SCA3; and, only half of the 41% reported perceived benefit after 3 years. Ergo, I am not understanding how this could be considered a successful drug trial. Instead, I see an attempt to recoup research dollars on the back of those with a horrible disease and desperate for a cure....
Laura	My mother struggled with ataxia for 30 years and could get no answers or treatment. Please help us get treatments
Arnaldo D.	Please, give me a chance for living longer in better health.
Barbara S.	I have SCA6 and I was the caregiver for my mother who had SCA6. It has no cure or treatment and I would try any drug or treatment to improve my quality of life and any information that would be gained so that my kids do not get this disease.
Jackie S.	My mom has suffered with SCA1 with onset of symptoms 25 years ago. Something has to be done for people living with these absolutely life altering diseases!
Mukesh H.	The FDA must try different drugs to solve these rare diseases. People keep suffering and there is no solution in this day and age. Please accept me as an 'experimental patient' so that future sufferers can be medically helped.
Rhonda H.	I think they should approve the drug because anything is better than nothing. I have balance and speech issues so I use a walker. Also my hands are not coordinated so it is hard to type or write. It is terrible to live with ataxia.
Gillian W.	My husband always wanted to trial new drugs to enable others to benefit even if it was too late for his own lived condition.
Joycelyn G.	I believe that a lot of people will benefit from this drug please. Thanks.
Daniela M.	I have a friend with ataxia and every attempt is valid.
Michael W.	As a person living with spinocerebellar ataxia it's debilitating and makes it impossible to do things that I like to do like fishing, taking walks, holding my great granddaughter. I wouldn't wish this on anyone. To not allow research or allow a drug to be used to treat this is criminal.
S P.	Anything that helps.
Anonymous	I have ataxia, we need a cure or at least medication that can reduce the impact of ataxia on our lives, so we have to review carefully ALL the evidence available to us. A greater awareness of the impact of ataxia on our lives is so important when considering any new drugs.
V. K.	A disease without a cure deserves research. This decision will not only stop the current drug under investigation but will also discourage future research.
Mary W.	It's heart breaking to hear my dear friend talking about having no options for treatment. She's a lovely talented lady who has lots to give.
Bella H.	As one suffering Chinese patient, I really want to hear some news on SCA3. This disease is killing butterflies in my heart, as time goes by.

NAME	COMMENT
Trudy	Until about three years ago I biked everywhere or walked the slow progressive ataxia that I have means I now can't ride a bike due to my balance and walking is uncomfortable I drag my left leg and stamp it down have become very isolated as can't walk far or normally also falls down if path uneven if there is something that I could try would do so.
Richard C.	I am a sufferer of chronic ataxia of an unknown type. This has ruined my life right down to forcing me to take low paid employment and not being able to live a full life. I still have the same aspirations and feelings as others and to have potential opportunities removed from the environment borders on the immoral.
Becky	Help is needed for this community please help them.
Anonymous	My son-in-law is suffering with SCA3. Because he has 2 young sons, any chance he may have to halt the progression of this disease would be welcome. There is no treatment out there right now to stop this horrible disease. We need more research which may come if potential drugs would be approved and publicized.
Camila	People need to have hope. If trying a new medicine could provide some, it must be done!
Francis K.	My friend is suffering, and her diagnosis is not positive. She will continue to live to her fullest, but she is open to additional assistance from this treatment.
Saulo R.	Between the option of a relentless, inevitable decay and the (even small) hope of an improvement, let's choose the hope. Why go for the rigidness of a normativistic decision if life requires decisions under the conditions of ambiguity?
Rhonda H.	If you are living with ataxia I would try anything approved or not. I am desperate. I hate this condition. I don't want to live like this. No one understands unless they have ataxia.
Michele S.	My daughter-in-law was diagnosed with SCA3 recently. Her father suffers from SCA3 and she has watched his health decline. She is a beautiful and vibrant young woman and deserves every opportunity to stop the progression of SCA3.
Anonymous	The more drugs that is trialed the bigger chance we have of finding something that works.
T.	It would help people like us suffering from SCA3 even if drug eases our suffering to some extent.
Flavia M.	A lot of experiences I couldn't explain with no crying. This is a familiar disease. My loved one affected with ataxia is my daughter, 13 years old.
Cinthia	I believe that medicine IS Very important to ataxia treatment.
Fran	Who knows when a miracle can happen.
Julian L.	These SCA diseases are debilitating and killing people. I would accept more uncertainty in meds, if it means hope that even some symptoms get better. That would translate to people being able to work, pay taxes, and serve to society for longer, which is to the benefit of the State.
Xiongjie Y.	Please review troriluzole. I was diagnosed SCA3 for 7 years and I need troriluzole eagerly.
Terry J.	The Ataxia patients need every available resource to help them through their troubled times with this horrendous non curable disease ATAXIA
Denise B.	Please help people who need this medicine.
Darlene	Before my husband passed away last year, he was begging for any kind of experimental drug or treatment. He was 63 and passed away from cerebellum ataxia
Seth J.	Watching my father lose his independence I am sure he would try anything that may slow down the progression.
Anonymous	I am currently living with SCA3. It is a very debilitating disease. I am very disappointed that I am even not given the chance to even try a drug that may help treat symptoms. Nor will my children if God forbid they get this awful disease.
Timothy F.	All of these Neuro-degenerative/Auto-immune disorders have increased levels of cortisol...make the connection...lower the patient's STRESS level and it starts getting better. Stop blocking progress FDA. Please.
Leila Z.	People need to have some hope!
Debra O.	My daughter lives with SCA29, and I want opportunities to help her live a more independent, productive life through medical breakthroughs.

NAME	COMMENT
Yu-Wen T.	Please let the first drug troriluzole pass. Thanks a lot.
Tim K.	If there is any chance this drug has the potential of slowing progression down it should be considered and very much up to the patient that has SCA3. I have this disease and if I can help with finding a cure or any med that helps I'm in.
Molly S.	People with SCA3 need any treatment or medication that could slow the progression even though there is a chance that it will not produce results for them, there still is a chance that it will.
Luiz Fernando C.	We have waited so many years to have a light, something that will bring us quality of life, that will stop the disease, so that we can have some peace, and we have faith that this medicine will still bring us many benefits...
Berno S.	Living with Ataxia one lives in eternal hope that it can get stopped or slow down as it is irreversible. The reasons the drug is not approved has to be made public so that a wider audience can decide the outcome.
Anonymous	The review should happen. FA is such a debilitating condition. It needs a treatment now.
Valerie B.	A friend is suffering greatly and just wants a chance for something that may help!
Clayton B.	Approve the drug, unless you have an alternate provision for treatment.
Gilberto A.	With several sick people in my family, most have died. I don't want to be another number in statistics.
Rosanne B.	All the Ataxias are a rare neurological debilitating disease. We need all available research and access to possible drug treatments to help us pursue a somewhat normal existence if possible.
Wiliam D.	It makes my life harder every day. Any change would be an improvement for others.
David K.	Ataxia inhibits my ability to keep working. I want to keep working and contribute to society through paying taxes. My ability to do so depends on further research into Ataxia.
Monika M.	In order to even consider a cure or lessen symptoms in the future, the time needs to be taken in order to review any data that could potentially lead to a drug that is approved. Time is always of the Essence for those with Ataxia. It may not seem time "worthy " to the scientists, but the fact that it was presented should be considered enough evidence.
Virginia W.	Ataxia has taken away most of my active life activities. It is difficult to participate in day to day activities in every way.
Anonymous	I currently have SCA3 and would like a drug to help a little bit.
Sheila L.	I am a person living with SCA6. I have lost my independence and am now reliant on my family to help me move. I believe that any drug that offers a glimmer of hope for people living with ataxia should be reviewed. We are in desperate need of something to help us live more independent lives.
Mary W.	I feel it would help my family members who have been affected with is disease. Going forward it my help my immediate family member and future generations.
Camila R.	Acho importante tentar um novo medicamento.
Michele S.	I have a different SCA but I absolutely believe new treatments for anyone living with SCA (in the case SCA3) deserves to have access to drugs/medications/treatments that can potentially/will help/improve our quality of life.
Sérgio Alves A.	Hope, hope that one day the disease will end. I believe a lot in medicine and I believe in God that soon many people will benefit from medicines.
Daniel D.	If there is a drug for ataxia, we are struggling! Please let us use.
Julian E.	Disappointing for the thousands with this rare disorder.
Robin M.	I both live with SCA3 and am the mother of a daughter with SCA3. Knowing what my father endured due to SCA3, I would love to be spared from this pain as well possibly preventing it from happening to my daughter.
Trish W.	I have been living with Ataxia for many years and would be interested in any new drugs that might help me.
Anonymous	Any alternative other than leave without a treatment?
Francinele S.	There are very few options for these patients and the disease progression is crippling.

NAME	COMMENT
Frank E.	Unless I can find something to improve my condition, I am destined to be wheelchair bound soon.
Kerstin E.	When you have cerebellar degeneration (ataxia — mine is borderline MSA-C) and as with SCA3 there isn't a cure, you'd be willing to trial new medications just to know that 1) the MAY benefit you 2) the knowledge will benefit those patients that come after. Please support.
Sarah W.	My brother-in-law has this disease, and my husband and him lost numerous family members to it. There is a need for people with this disease to fight with any chance possible.
Whitney S.	I believe that troriluzole should be made available for anyone to use for treatment.
Daniana C.	I'm living with ataxia I need better living conditions.
Nicole P.	I am aware that a full review will be expensive but it should be done in order to give a clearer picture to patients and medical practitioners about the efficacy of this treatment.
Anonymous	In the case of rare diseases the FDA is now approving drugs that have little to no evidence of efficacy but are safe. It is getting ridiculous. This just creates false hope for patients and their families, leads biotechs to continue to push drugs that don't work because the bar has been lowered so much. What is the point of primary endpoints, randomized controlled trials, pre-specified statistical analysis (to avoid bias) if at the end of day, it doesn't really matter. The "As long as it's safe, the FDA should approve it" mentality actually makes it much more difficult for other companies to develop drugs for the same indication. It may require them to run a longer trial with more patients to offset the standard of care that is littered with approved drugs that may not work. What a paradox — approving drugs today that may not work may actually slow the approval of a drug in the future that does. If Biohaven got a refuse to file, I can only imagine how weak the dataset must have been.
Omar	FDA you don't understand how bad is this disease, is pointless live with this, please allow this drug is our only chance.
Susan B.	I have a friend with this terrible disease and they need hope for a cure!
Anonymous	Have tried several medications which have not worked and have had no negative reactions so anything that has the slightest glimpse of working would be appreciated.
Sarah T.	Everything should be done to see if it helps people with Ataxia.
Isaura	Hi there I have SCA3 for a long time and I hope to try this medicine.
Eugenia J.	Our daughter, lives with SCA, every day. Daily watching the struggle, with no hope of a treatment, is unbearable! Our daughter holds a Master's Degree, and she can't brush her teeth.
Samy K.	This treatment give the hope that the people need, the chance to come back to the normal life and do the basic and regular activities, is really important that this medicine be allowed for the public fast as possible, if proof the safety of this, each day makes difference.
Tania	If you haven't experienced ataxia you don't know how life changing it is. My daughter is only 22 and has had all her life hope and dreams stripped from her fin this debilitating disease. We would try anything to give her the chance of a more normal life.
Ruth B.	Even a small reduction in symptoms could mean a better quality of life — fewer falls, more mobility, less dependence on others. I am haunted by the memory of my mother's deteriorating health with SCA3 and dread those symptoms as my own disease progresses.
João D.	I believe that medicine is very important to ataxia treatment.
Daniela M.	My father and my sister have Ataxia. It's very important a treatment for motor independence and quality of life.
Jodie L.	Hello, Although I live with SCA18 and not SCA3, I believe this type of drug would be paving the way for all who live with any of the SCAs.
Sarah F.	It is very tough having a diagnosed condition with no medication to help with the symptoms only a constant fear that your condition is worsening. The hope that a drug can bring is a lifeline and not only potentially help the physical but mentally and emotionally offer hope.
Susan S.	I feel that this drug will help the SCA3 community and it start the process of finding a cure or at least minimize the symptoms and have a huge impact for the rest of the ataxia community.
Deniz O.	Please hurry up. I do need a medicine!!! I want to be a grandma for both my children !

NAME	COMMENT
Diane M.	My wishes are that the general public would be more educated on the disorder of ataxia. It needs to be more mainstreamed. By giving the FDA more flexibility it would give more exposure of disorder to the general public.
Anonymous	Only those living with Ataxia know what it is like to wake up everyday to a set of symptoms that is going to or is already stripping the hope out of your life. No one NOT living with Ataxia should destroy a chance of hope for sufferers.
Wilker Alexandre C.	I believe that medicine is very important to ataxia treatment.
Teresa O.	Please reconsider and review the findings.
Robin D.	We have a friend with SCA3. Until diagnosed with this affliction, she was a very active person. Now, with SCA3, she is not able to participate in her daily activities and to be a contributing member of society. People with this affliction have a right to choose to try a drug that may help them and allow them quality of life. Thank you!
Gustavo Luiz P.	It is very important for people who live with SCA3. It is a hope.
Frank M.	We have no hope currently since there is no treatment available. Please continue researching.
Dinesh W.	Anything we can do to make progress in treating this devastating condition is needed. I have seen my sister's husband suffer through this. PLEASE!
Noel H.	A very close friend has SCA3. It has devastated her, her family and her life. She and all with this disease deserve every chance they can get to treat their condition.
Rosemary K.	I believe where there is a chance a drug can help someone with SCA, then they should be offered to take that drug knowing the risks involved.
Marcus Vinicius S.	Every drug that can bring a new treatment perspective to improve the quality of life of patients is worth a try. It brings hope.
Douglas T.	Things that aren't approved by the FDA work the best.
Jeizel W.	We need an urgent treatment
Rodolfo N.	New, more effective drugs are important in the treatment. It is a very aggressive disease and all attempts must be tried.
Nick J.	We need to investigate all types of treatment, please review you're decision.
Mary M.	My son has Friedreich's ataxia. We need treatment for ataxia ASAP. If just the slightest chance of any drug etc. could help, no matter how small, we would embrace the chance of using it.
Deborah P.	My father and uncle had ataxia. Now my sister and cousin have it. My daughters worry for me and for themselves!!
Luís Carlos S.	A FDA deve analisar os estudos do medicamento troriluzole para os portadores de SCA3, dando-lhes o direito a um tratamento, tendo em vista que não existe nenhum medicamento para a doença.
Tom S.	I participated in the study in Chicago. I have SCA1 and feel the drug had some positive effects. Further research ahead be looked at further.
Anonymous	My opinion is please someone help me!! I cannot stand or walk, I fall over! Going on 4 years now! I have no life! I had to give up my career in design! I'm asking for help! Please!
Terri F.	I believe that the FDA should review the findings of a possible new drug that has the potential to stop the disease.
Hawbir F.	Hi I have SCA6. I am really eager for the new drug for SCA3; because I think it may be helpful for me also.
Dipa V.	I want this drug immediately.
Lucille B.	My son's condition gets worse every day some treatment is better than no treatment.
Marissa W.	As a child with SCA3 of a father who had SCA3… knowing there was a drug that could have helped him before he died would have been beneficial.
Denise	We are begging for the possibility only.

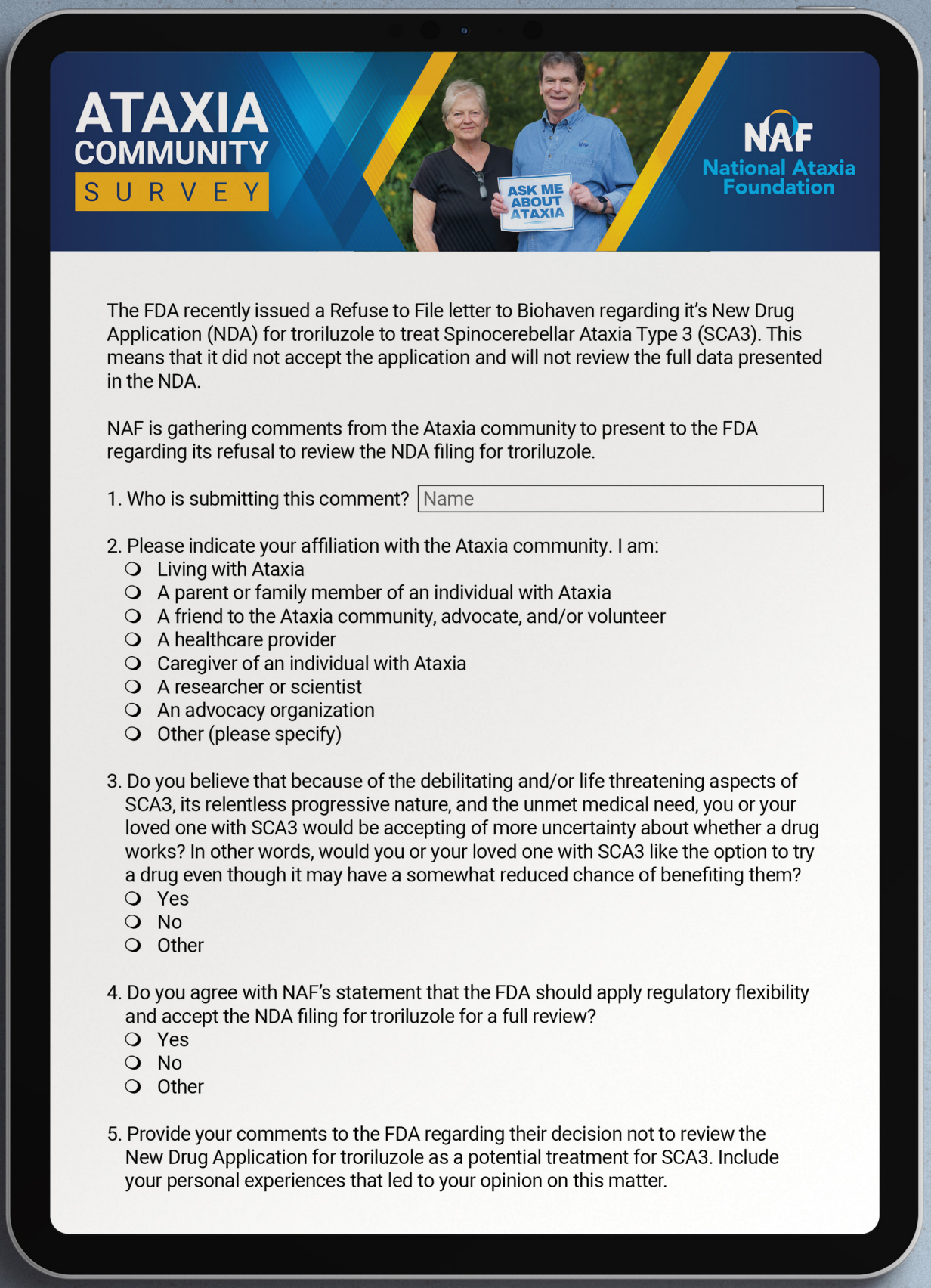
NAME	COMMENT
Anonymous	It's just not SCA3 it needs immediate attention for all Ataxia numbers! Where is 13 currently and help for those affected. Really sad since the 50's it's had little movement on treating this.
Zachary D.	FDA, Please review the new drug application for troriluzole. My wife has ataxia. Please help us!
Susan S.	Any research on ataxia should be reviewed. Ataxia is life altering.
Neil W.	If FDA won't approve, explain pros/cons in a short leaflet when drug is prescribed.
Elder O.	I live with ataxia and the flexibility of a possible medication would help me a lot to have more hope.
Deborah W.	This sounds like hope something that people with ataxia have very little of.
Melissa F.	Honestly, I'm so scared for the progression of this disease with no treatments whatsoever. For the sake of my husband and daughter, I would rather try something even if it doesn't work in the end. Better to try.
Rogério G.	My cousin lives with Ataxia. She will heal with this opportunity.
Sharon C.	My brother passed away 11 days ago after suffering for 10 years. Everyone should know about this disease.
Samir	Please approve troriluzole for SCA3, FDA, PLEASE. IT'S RARE DISEASE IT NEED TREATMENT.
Louis B.	Watching the difficulty my daughter faces every day due to Ataxia.
Glo M.	Willing to try new medication under physician supervision.
Luciana A.	We need more attention for our rare disease. Please help us to have a quality life.
Tricia M.	Any drug to help with ataxia is a blessing after living with ataxia for 27yrs and having my abilities go from pretty normal to now in a wheelchair so any drug to help is very, very VERY important!!!
Anonymous	I was diagnosed in 2020. Who knows the quality of life I would have if the medication was available. I believe it would be better than now.
Gustavo F.	I have a brother-in-law with the disease. The release would aid in your recovery.
Diogo F.	We can no longer be inert to this disease, we need a light.
Miki K.	People have Ataxia and family need support due to long terminal illness similar to Parkinson. We should be deserving of equal support and finding cures.
Gunjan N.	A drug is a necessity at the moment.
Shannon R.	My husband has SCA5. Although this may not directly affect him, it is an inroad that must be explored. Please review and reverse your decision!
Amanda R.	I don't feel patients should be given false hope. Allowing a drug to come to market will mean a very costly journey for many patients and their insurance companies for likely very little or no benefit.
Sandra P.	I am the wife of a patient with ataxia and all the research and medicines that can bring benefits linked to this disease are very important for our family and we will look for ways to have them.
Gloria T.	Please help us find some way to treat this horrible disease.
Paul C.	On the ground perspective is desperate and while it might appear to be a risk - it is better take a Risk of the benefits are real.
Sirlene	The opportunity to try to improve with the medicine is important.
Robert B.	Any help is better than none!!
Gustavo Henrique D.	This disease is cruel and we have to do our best to improve the treatment and medicine.
Ninette H.	I have a friend with SCA3 and it is heartbreaking to know she is dealing with this disorder and told there are no cures. She struggles to walk and will have to use a cane at the age of 35 years old. Her father passed away due to SCA3 in his 50s and it is gut wrenching to think she may have the same outcome as her father.
Sarah F.	This disease is absolutely horrific. Any treatment would be hugely appreciated for any relief.
Martin K.	Anything that can improve your quality of life.
Hadi G.	Living with Ataxia is difficult.

NAME	COMMENT
Marilou P.	It could save lives or give the patient a better and longer life.
Anonymous	Any drug that will relieve symptom and improve life is of obvious benefit.
Victor A.	Any medicinal drug that can, at least, reduce the symptoms of SCA3 should have more attention
Guilherme S.	I hope this treatment can save lives.
Noel B.	As someone who lives with SCA3, any and all attempts to make this affliction more bearable would be a great service.
Ron S.	Spinocerebellar Ataxia is a debilitating disease and any benefit of a drug however small is worth the chance.
Travis M.	Anything that can help lessen the burden of this disease should be done!
Cláudia Isabel D.	It´s very difficult to live with SCA3 and I have children, who may need the drug as I do now.
Laura G.	It could lead to my friend getting more help or at least moving in the right direction.
Pat F.	Ataxia patients and their families need hope. It's horrible to experience and to watch one's abilities diminish. Please reconsider this decision.
Susan R.	This is a progressive disease with no treatment or cure at present. Most sufferers would be happy to try a new treatment, including me.
Nicola H.	Any drug that can improve my quality of life should be available for an individual to make the decision. I have lived with Friedreich's ataxia for 30 years.
Zack S.	I believe the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review for a few reasons: SCA3 while considered a rare disease by affected number standards, is different in the widespread effect that it has on entire families and those that are close to them. Drugs like troriluzole, or any other in the pipeline that can prolong the effects of this disease, can provide relief and hope to not only those affected, but the widespread families of those. SCA3 has taken the life of my grandmother, my father, four of my aunts, my brother, and several of my cousins, including myself now have it.
Pam C.	Personally I feel there needs to be a better standard of measurement to prove the effectiveness of the pharmaceutical treatment. SCA in general should have a better standard for assessing the degree of change from start to endpoint. Less objective and more accurate.
Helen	We want to try using the drug to see if the result will be positive.
Douglas H.	Patients should have the choice for last hope drugs at the own discretion.
Mary Ann T.	This is such a rare disease. It deserves everything it can get.
Russell S.	This disease changes lives very quickly and we are helpless against its voracity. We would try anything that has the slightest chance of helping Joe and Taylor.
Troy M. Jr.	Ataxia has robbed me of the abilities to walk, run, dance, oil paint, sketch, and so much more. I've suffered with it for over 10 years.
Feuruo L.	Strongly demands a comprehensive review.
Luis Carlos M.	SCA3 is a scary disease and every possibility to easy people's life would be taken in account.
Helena B.	You do not know how is to live with ataxia!
Randall S.	Only those with SCA know what SCA is like in their daily lives.
Nicole L.	Any possible treatment is welcomed.
Tammy C.	I see many patients suffer from this debilitating disease. Why would one not follow through with a review?! Mind baffling
Michael W.	As a person living with SCA3. It's a tough no hope road. Please let this drug be a glimmer of hope for people like me.
Linda T.	My disease is a terrible thing to live with.
Jeffrey M.	As the only treatment available it means some hope if it helps a some…

NAME	COMMENT
Sandra T.	I was the first participant signed up in this trial. Although I have SCA type 2. My family agrees that after I started the drug I was on it for 4 years. And they said definitely I was better on it.
Marçal L.	Because it is fundamental that there is a light at the end of the tunnel, think about the topic.
Peter K.	I was diagnosed with Cerebellar Ataxia about 8 years ago. It is a very limiting condition and has severely affected my life.
Greg	I need a drug for my wife ataxia and please let our government approve it or we will seek the help of the country!
Raul L.	Me and my children need this drug please FDA.
Saurabh S.	I am approaching 40 and need the available treatments to slow the progression of this disease so I can be there for my family and maintain my quality of life.
Tomas S.	My mother, grandfather and 2 uncles died with SCA3. In the end, they had bad quality of life.
Eileen C.	If we do not try new drugs they will never be regulated.
Maria C.	I believe that medicine is very important to ataxia treatment.
Mike E.	My mother is living with SCA3 and it is hindering her quality of life. Please reconsider this, chances are my sister and I also carry the gene..
Sherri D.	Because my Ataxia is not genetic, the drug does not affect me directly, but every improvement or new development in Ataxia helps everyone, not just one specific group, dealing with this debilitating disorder.
Rhonda P.	Please. We must rattle and push every door for a cure or even a treatment to slow this disease. Please give these people a chance.
Ian C.	I am a full time career for my wife. All medicine must be tried in attempt to find a cure.
Thiago M.	If the drug presents no death risks, let us test it.
Kevin R.	It is my body let me do as I please.
Jillian C.	Reviewing the drug for treatment for SCA3 may tremendously effect the disease and assist in saving lives.
Efraim Carlos C.	New! More effective drugs are important to treatment.
Larry T.	This drug or variants may lead to helping all people living with ataxia.
Sharon	There doesn't seem to be any specific treatment for SCA3. A drug that may be beneficial for Ataxia is surely worth trials for the future.
Priscilla Farias S.	Patients need more drugs, less bureaucracies. This medication is very important.
Mohammad A.	It's not a perfect drug but an option that will help patients to some extent.
Jean G.	I long for some sort of treatment to halt or slow my ataxia — SCA6.
Seth J.	We need to work as a team in every aspect in life to meet difficult and challenging tasks, not just say "no" and not why.
Rafael N.	My father is living with SCA3 for several years, and it would be a relief to know that there is a possibility of a drug for our future generations.
Leila O.	We want quality of life.
Tammy A.	We need this desperately- I am 5 generations deep with family members having this horrible disease.
Missy P.	I have a friend who has no other options. It is a terrible disease and if this provides some hope then they should have access to it.
Ronald Z.	Anyone with this disease would like the opportunity to make their own choice with regard to their own future.

APPENDIX C:

Ataxia Community Online Survey



The FDA recently issued a Refuse to File letter to Biohaven regarding it's New Drug Application (NDA) for troriluzole to treat Spinocerebellar Ataxia Type 3 (SCA3). This means that it did not accept the application and will not review the full data presented in the NDA.

NAF is gathering comments from the Ataxia community to present to the FDA regarding its refusal to review the NDA filing for troriluzole.

1. Who is submitting this comment? Name

2. Please indicate your affiliation with the Ataxia community. I am:
   - ❍ Living with Ataxia
   - ❍ A parent or family member of an individual with Ataxia
   - ❍ A friend to the Ataxia community, advocate, and/or volunteer
   - ❍ A healthcare provider
   - ❍ Caregiver of an individual with Ataxia
   - ❍ A researcher or scientist
   - ❍ An advocacy organization
   - ❍ Other (please specify)

3. Do you believe that because of the debilitating and/or life threatening aspects of SCA3, its relentless progressive nature, and the unmet medical need, you or your loved one with SCA3 would be accepting of more uncertainty about whether a drug works? In other words, would you or your loved one with SCA3 like the option to try a drug even though it may have a somewhat reduced chance of benefiting them?
   - ❍ Yes
   - ❍ No
   - ❍ Other

4. Do you agree with NAF's statement that the FDA should apply regulatory flexibility and accept the NDA filing for troriluzole for a full review?
   - ❍ Yes
   - ❍ No
   - ❍ Other

5. Provide your comments to the FDA regarding their decision not to review the New Drug Application for troriluzole as a potential treatment for SCA3. Include your personal experiences that led to your opinion on this matter.

APPENDIX D:

Sources

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