nnpdf
NATIONAL NIEMANN-PICK DISEASE FOUNDATION, INC.

COMMUNITY RESPONSE:
FDA Review of Arimoclomol

FEBRUARY 2024

nnpdf NATIONAL NIEMANN-PICK DISEASE FOUNDATION, INC.
ARA PARSEGHIAN MEDICAL RESEARCH FUND UNIVERSITY OF NOTRE DAME
DART DANA'S ANGELS RESEARCH TRUST ...aiming to cure Niemann-Pick Type C
Firefly FUND SHINING A LIGHT ON NPC1
HIDE & SEEK FOUNDATION for lysosomal disease
Hope for Marian
SOAR Support Of Accelerated Research for Niemann-Pick Type C

Our Mission

The National Niemann-Pick Disease Foundation, Inc. (NNPDF) is a non-profit, patient advocacy and family support organization dedicated to supporting and empowering patients and families affected by Niemann-Pick disease, through education, collaboration, and research. Founded in 1993, NNPDF serves families throughout the nation at all stages of their Niemann-Pick journey. The NNPDF is the US member organization of the International Niemann-Pick Disease Alliance (INPDA).

In September of 2020 we were told we needed to start making end of life plans for our daughter, Willow, as they assumed she only had a few months left to live. In October of 2020 she started Arimoclomol. Not only is she still alive to this day, but she is happy and has an amazing quality of life. Arimoclomol slowed down the progression of her disease and even helped make it possible for her to get some of her lost abilities back. Willow would not be here today without Arimoclomol.

Krystal S, Utah

Arimoclomol is the only medication that is giving my family a chance to love our girl a little longer. It's giving us a fighting chance. It's saving future generations of feeling the same pain.

Cassandra S, MI

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I have access to Arimoclomol through EAP, which has enabled me to continue to work full-time and be as independent as possible. I cannot tolerate Miglustat and have not experienced any adverse side effects with Arimoclomol. I attribute my delayed disease progression to this drug and it gives me the hope and motivation to keep trying my best every day!

Cara G, PA

There is so much to manage and cope with as a caregiver of someone (especially a child) with a disease like NPC. Knowing that the disease is degenerative and fatal is a constant worry that gnaws at you every day and taints every experience. Trying to coordinate the medical care required-doctors, therapists, health insurance, equipment providers-is a full-time job. Let's NOT add "fighting to maintain access to a treatment that helps your loved one" to the long list of worries and responsibilities.

Taylor S, MA

NNPDF Community Response Letter to FDA

February 8, 2024

Catherine Pilgrim-Grayson, MD, MPH, Acting Director
Division of Rare Diseases and Medical Genetics
Janet Maynard, MD, MHS, Director
Office of Rare Diseases, Pediatric, Urologic and Reproductive Medicine
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993-0002

Dear Drs. Pilgrim-Grayson and Maynard,

Since the founding of the National Niemann-Pick Disease Foundation (NNPDF) in 1993, a core part of our mission has been to work to improve the lives of those living with Niemann-Pick disease by supporting and empowering patients and families affected by this condition. In the past several years, implementing this mission, in concert with our partner organizations and advocates, has focused on accelerating the development of treatments and a cure for this devastating disease.

We have now come to a seminal moment for many in our community who suffer from Niemann-Pick disease. It has now been nearly 110 years since this condition was first described by Albert Niemann and its etiology characterized by Ludwick Pick, and over six decades since the genetic disease subtypes were elucidated, yet our community continues to wait for the first treatment to be available for Niemann-Pick type C (NPC) in the United States. The hallmark hypotonia, delayed fine and gross motor development, speech impairment, and dysphagia deprive patients of the capacity to carry out everyday activities. However, NPC continues to take a toll, with often rapid neurodegeneration and progression of the disease, causing patients to lose what function and abilities they do have. Eventually NPC takes their lives prematurely. The stark reality is that, without treatment, the prognosis for patients with NPC is dismal today, particularly for those with early-onset disease. People with NPC simply cannot wait any longer.

People with NPC need a treatment – anything that has a reasonable chance to even incrementally improve the known and relentless course of this disease. This was clearly heard by participants in the discussion at the March 2019 Externally Led Patient-Focused Drug Development Meeting on NPC, and subsequent Listening Sessions, as we have many times before and since these meetings.

We ask that FDA consider these perspectives from those who have the most to lose by having a potentially effective treatment be sidelined yet again. Consider their serious unmet needs. **Consider the irreversible progression that they will experience if the current data is deemed inadequate and if Zevra Therapeutics is asked to identify and gather additional new data.** Consider that people with NPC live with absolute certainty about their future, and so are willing to accept greater uncertainty and even risk when it comes to new drugs treatments.

People with NPC deserve to have the existing data on arimoclomol reviewed, and with this patient input in mind. **NNPDF and its partners hope that the collective and iterative patient input we and others in our community have provided in recent years provide a framework for maximum regulatory flexibility, minimizing the chance that patients will not gain access to a drug that works due to any possibility of false negative conclusion.**

We believe that FDA regulations and policies, as well as previous approvals of other drugs for rare diseases, support applying maximum regulatory flexibility when reviewing the arimoclomol NDA resubmission. This is supported by Congress, where the 2023 Agriculture Appropriations Bill, Senate Appropriations Committee Report "encourages FDA to use its existing authorities and pathways to meet the urgent unmet medical need of the current generation of NPC patients…" (S. REP. NO. 118-44, at 127 (2023).

NPC is an ultra-rare condition with approximately 300 people known in the United States. 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 definitively confirm a drug benefit. The presence of these challenges demands a more flexible approach. Rare diseases must be considered differently than more prevalent conditions, and ultra-rare and highly heterogenetic diseases even more so.

Especially when combined with the serious unmet medical needs (as discussed above), **diseases like NPC deserve the maximum regulatory flexibility in the review of data from clinical trials and accompanying confirmatory evidence.**

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." NPC certainly meets **all three** of these criteria.

There is ample precedent set by FDA for approving applications for drugs that have a wide range of efficacy data, everything from pivotal studies not meeting their primary endpoint or not even having a randomized, placebo-controlled arm (or sometimes even a prespecified protocol). Recently, in April of this year, FDA approved a drug, Qalsody, for a type of amyotrophic lateral sclerosis (ALS), even though the primary endpoint did not come close to being achieved. Yet, in its review, FDA chose to approve the NDA, during which they considered other analyses that ultimately supported approval. We believe that any new analyses of the NPCCSS results should be considered even if not deemed a "perfect solution" to concerns that FDA had raised, as perfect should not be the enemy of good. The same goes for any new confirmatory evidence provided by Zevra Therapeutics.

We are asking for the same consideration in regulatory flexibility that has been given to other rare and ultra-rare, progressive, and genetic conditions, such as ALS, Duchenne muscular dystrophy, Batten disease, and MPS.

The NPC community in the United States calls on the FDA to apply appropriate and maximal regulatory flexibility in its review of the NDA resubmission for arimoclomol.

NNPDF gathered input from the NPC community in the United States to ensure that FDA has the opportunity to hear the voice of the patient when considering an NDA for a potential treatment for NPC. **Nearly 1,000 individuals stepped forward to make their voices heard by supporting this request.** This packet contains their feedback including over 300 comments that our community wanted to share directly with the FDA review team (see Appendices A, B C, D). Here are several quotes that are representative of the community's response:

Individuals with access to arimoclomol today:

When my son Cole was diagnosed with NPC he slowed cognitively, his balance was unstable, his speech slowed, he had a hand tremor, and he was very quiet. When he started arimoclomol months after his diagnosis, we noticed a positive difference right away. Much of his symptoms were better, especially communicating. Arimoclomol allows him a better quality of life. Without this medicine he will digress fast. It's more than extremely important to our son that arimoclomol continues to be available, it is life saving! - Chip S, Florida

"I am 1 of 6 kids, and three of my siblings were diagnosed in 2019. One is nonverbal and cannot walk. The other 2 not only have to manage their disease but live with a picture of what their future might look like if they are not able to continue treatment. All 3 take arimoclomol, and while my sister's disease was already advanced, I believe she is still with us because of the drug, and that my brothers have declined less. My family calls it "the miracle pill". Losing access would be devastating." - Kelly M, Pennsylvania

"I am a senior in high school, and I have been just recently diagnosed with NPC. I have been on arimoclomol for just a few months. I think that it helps me think clearer and I'm not as tired as I used to be. I used to come home from school and take naps, but now I feel I don't need to sleep as much. During my last visit with the neurologist the doctor mentioned that my symptoms seemed to have stabilize and don't seem to be progressing as much." - Morgan H, Utah

Individuals without access to arimoclomol today:

"Arimoclomol could very well be the only thing that stands between a young soul's bright future and her eventual total incapacitation." - Felix C, MA

"Currently there is no approved treatment for NPC. Without treatment NPC will kill my wife. Arimoclomol may be a treatment that extends her life and provides an improvement in her quality of life. Having an approved treatment may allow for additional trials wherein a cocktail of drugs could be tested, providing an even more effective treatment." - Daniel R, Virginia

"My other son Daniel, age 36, does not have access to arimoclomol as he receives a different trial drug. After seeing the positive effects on David, we are eager to add arimoclomol to Daniel's protocol. This would enable him to benefit as his brother has and provide important information on the effects of combined drugs. It is widely felt by clinicians, patients, and families that the management of NPC will be best achieved through a combination of approved treatments." - Barbara L, Connecticut

NPC researcher/clinician:

"As a clinical trial principal investigator, I have witnessed firsthand the benefits study patients and families have experienced while taking arimoclomol. Subjects who were unable to sit upright due to truncal hypotonia, were ataxic, or overtly encephalopathic, were able to sit upright, improve their ambulatory abilities, and be awake and alert enough to attend school and pay attention to tasks and schooling. We've noted deterioration when drug was stopped, and regained abilities when restarting." – Raymond Wang, MD, California

We have the utmost respect for FDA's role in ensuring that drugs approved in the United States are both safe and effective, including that the benefits outweigh the potential risks. We share this goal with the FDA. The ultimate approval decision on arimoclomol should be considered in the context of this serious and life-threatening unmet medical need that is ultra-rare. The NPC 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.

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

Sincerely,

Joslyn Crowe

Joslyn Crowe, MSW, MA
NNPDF Executive Director

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

On behalf of the those living with NPC and their direct caregivers in the United States:

Summary of Community Feedback

988 Responses

Affiliation with the NPC Community

51
NPC Diagnosed Individuals

345
NPC Immediate and Extended Family Members

576
Advocates or Friends of the NPC Community

16
Reseachers or Clinicians

Responses from **47 States**

Alabama
Alaska
Arizona
Arkansas
California
Connecticut
Florida
Georgia
Illinois
Indiana
Iowa
Kansas
Kentucky
Louisiana
Maine
Maryland
Massachusetts
Michigan
Minnesota
Mississippi
Missouri
Montana
Nevada
New Hampshire
New Jersey
New Mexico
New York
North Carolina
Ohio
Oregon
Pennsylvania
Rhode Island
South Carolina
Tennessee
Texas
Utah
Virginia
Washington
West Virginia
Wisconsin
Wyoming

APPENDIX A:

Community Comments to FDA

If you or a loved one has had access to arimoclomol in a clinical trial or through expanded access, please describe why continued access to arimoclomol would be important to your loved one with NPC

Casey M.	Pennsylvania	3 of my siblings have NPC and are taking arimoclomol, it is beneficial to them and their well-being to continue taking it. It allows them peace of mind to have something to help them endure this disorder
Marla L.	Utah	A child in my community has had 3 more years with her family that they did not think they would have. The drug has worked for their child. I have been following their story and love this little girl and I don't even know her!
Alex C.	Ohio	A cousin of mine has been receiving this drug for 5 years and it has performed wonderfully for them. Continued access to this drug means that they can continue to live with the quality of life they currently have, and I want them to continue to have this quality of life.
Diana C.	Connecticut	A dear friend's daughter is doing well with arimoclomol. More focused, happier. Continued access will improve her quality of life.
John P.	Pennsylvania	A family friend has a child with NPC. Their child has received treatment for 4.5 years and continued access is required to assure the quality of their child's life.
Marci W.	Utah	A friend in our community has a sweet granddaughter who was told by doctors she would not live long. Thanks to this medication she has lived beyond her life expectancy.
Matt S.		A friend of mine through work has a daughter with NPC. Since starting this medication, she has seen promising improvement in her daughter.
Jessica V.	Indiana	A friend's daughter has NPC. The family was told the daughter was too far along for this to do much for her, but they tried it with the hope of slowing her disease progression. Not only has it slowed, but her speech is much clearer and the family has much better communication with her now.
Kathy H.	South Carolina	A friend's daughter is currently taking these meds. She's showing progress with no side effects. It's valuable to continue such treatments to help people like her to move forward. I plead with you to continue these trials.
Christina R.		A friend's daughter has NPC. Arimoclomol has helped her so far. Please approve this drug so it can continue to help Gracie and other people who are left with no hope for treatment.
Karen M.	Virginia	A friend's daughter was diagnosed with NPC in Dec 2022. This devastating disease has NO approved medication - that is hard to swallow and needs to change. Her daughter enrolled in the EAP in October 2023. Data has shown arimoclomol is safe and effective. Her daughter is tolerating arimoclomol well. Her cells need this medication to help with protein misfolding. Approval will mean it becomes standard of care and could work synergistically with additional medications to stabilize this disease.
Chelsy H.	Utah	A little girl in my community has been given 3 more years of life at this point, 3 more years than her family ever thought they'd have thanks to this drug.
Nellie B.	California	A loved one is taking this drug and it is saving him. Please do not interrupt this access to this life-saving drug for my cousin.
Tara M.	Texas	A member of our community has been diagnosed with NPC. She is responding well to medical treatment, but the family is advocating for better standard of care based on what has been working for individuals with NPC.
Kathu S.	Florida	A son takes this through Boston Children's. This medication helped to decrease his hand tremors, bring back our social children after he had withdrawn into himself during the time of his diagnosis, and has helped to give us hope when we weren't initially given any. Right now it's the only medication available to give him time. The thought of not having it is devastating. This is why. Families are slowly losing their children. There's a chance for more time? How could someone steal that time?
Julie P.	Georgia	A very dear friend of mine has a nephew currently taking arimoclomol with NPC and he deserves the best chance at fighting this disease possible.
Anne A.	North Carolina	A young woman I know has been taking arimoclomol and has seen improvement in her symptoms of NPC disease with no negative side effects. She and others who share this illness deserve the opportunity for an improved quality of life and relief from their symptoms of NPC disease.

Elizabeth F.	California	Access is important in order to provide my niece with a comfortable life, what little she has left.
Stefanie P.	Ohio	Access to any drug no matter what it is should be maintained as long at its benefiting patients and their families.
Roberto B.	Florida	Any medication or treatment that will slow NPC's progression until a cure is found, is critical.
Judy M.	Virginia	Anything that can help improve the life of my grandson is important.
Danielle Y.	Florida	Arimoclomol has allowed our athletic and friendly teenage neighbor, who lives across the street from us, to emerge from his retreat from the world. In 2018 we used to always see him playing ball with his father in the front yard & gradually we no longer saw him. I visited his mother & him when he was hospitalized from multiple seizures. It was heartbreaking seeing him not able to move. Using arimoclomol has enabled him to go to sporting events, visit with friends, and give his family hope.
Sreekanth P.	Texas	Arimoclomol has been helping my friend considerably. Continuing it is important for him to lead a better life.
Lauryn E.	Florida	Arimoclomol has helped slow the progression and allow my cousin to lead a better quality of life than we were originally led to expect.
Srinivas P.	California	Arimoclomol has shown remarkable efficacy and helped improve my nephew, Mr. Sanjay's condition in many endpoints and stabilize in others according to his physician. With no other approved treatments for the indication, it is of utmost importance that arimoclomol be made available to patients suffering from this debilitating disease. It is my earnest appeal to the FDA to approve arimoclomol by outweighing the benefits seen in real patients over any perceived risks.
Madi C.	Ohio	Arimoclomol is saving my loved ones' life. He is thriving and doing so well through this medical trial. My loved one is in need of continued arimoclomol to save his live and give him the continued chance of a semi-normal life.
Raymond W.	Mississippi	Arimoclomol is the best treatment available today.
Cassandra S.	Michigan	Arimoclomol is the only medication that is giving my family a chance to love our girl a little longer. It's giving us a fighting chance. It's saving future generations of feeling the same pain.
Julie Hargette A.	Virginia	Arimoclomol, the clinical trial medication Cole takes through Boston Children's. This medication helped to decrease Cole's hand tremors, bring back our social child after he had withdrawn into himself during the time of his diagnosis, and has helped to give us hope when we weren't initially given any. Right now it's the only medication available to give him time. The thought of it not being available is devastating
Ronda J.	Massachusetts	As a friend to a family fighting NPC, I feel strongly that any path forward that results in a gap in treatment or no treatment at all for a person facing significant loss of function is an unacceptable result. Arimoclomol could very well be the only thing that stands between a young soul's bright future and her eventual total incapacitation. My friend has reacted to this medication favorably. Please approve it.
Muslum C.	Florida	As my son's condition declines arimoclomol keeps his condition stable. He is able to somewhat function by himself.
David L.	Connecticut	At 34 I have been taking arimoclomol for 3 years. I feel this has helped me with my eating and swallowing skills. Before taking arimoclomol, I would cough and gag when drinking or eating but after taking the drug those things have greatly improved and I feel safer. I also used to drool regularly but since starting the drug, that has stopped. I feel very strongly that this drug is essential in treating NPC and want to continue to receive the benefits I have been getting. Please approve this drug.
Jennifer R.	Alaska	Because Everett deserves a fighting chance at tackling this disease.
Jackie B.	Virginia	Because everyone deserves a fighting chance
Donna H.	Pennsylvania	Because he is so young and needs to live a full life. It is working to save and extend his young life now. Let others receive this same benefit.
Denise S.	Pennsylvania	Because it's keeping him alive and able to thrive as a toddler.
Kris W.	Utah	Because it's there only thing that has worked for her.
Jennifer G.	Virginia	Before my daughter who is now 22, started taking arimoclomol she was beginning to have a weakening in her swallowing muscles. We had to have her use a sippy cup that limited how much she could drink at a time. As a young adult this made her feel as though she was being treated like a baby. She began taking arimoclomol 3 years ago, as a result of her taking this drug her swallowing and choking on food and liquids has ceased. The longer we can keep her from needing a feeding tube the better for her.
Jolie V.	South Carolina	Better quality of life.

Madeline Richardson M.	Pennsylvania	Brody needs it to survive.
Whitney S.	Florida	Continued assistance for his health and well-being as he currently lives with NPC.
Lenette F.	Michigan	Continued treatment is necessary to prevent the disease from progressing. Multiple administration methods or in other words a cocktail approach in needed and this can't continue unless we have approved treatments.
Mailynn E.	Utah	Dear friends' granddaughter has been taking this, it's gave them 3 years longer than thought with their precious little girl, please approve this, when something helps families keep their love ones around especially children, they need these meds.
Katie G.		Everett and his family deserve the best chance at fighting this disease. I couldn't imagine not having the option for my own child.
Ashley G.		Everett Brutocao is my cousin and has had access to arimoclomol. I was at church when Everett was there with his mom Sara. We were at the part of mass when we say the Our Father. And despite all Everett has gone through and all the noise in the room I could hear Everett reciting the Our Father. It was amazing. I think this was possible because of the treatments he has been able to have. Please approve this drug so that we can have more moments like this with him. We love him so much!
Brittany W.	Alabama	Everett is a part of our family and that he deserves the best chance at fighting this disease.
Kimberly M.		Extended Willow's life for the past 3 years when her parents were told to start making end-of-life arrangements. Continued access to this medicine will extend her life even longer.
Daniel Z.	Washington	FDA needs to approve this…Life is precious, and time is running out for the people who need this…
Elicia M.	Indiana	For Gracie Lee and all others to continue having access to arimoclomol.
Rianna B.	California	Friend of friend's daughter seems happier and able to follow directions better with no side effects.
Toni B.	Texas	Grace Lee was diagnosed with NPC in Dec 2022. She enrolled in EAP in Oct 2023. Gracie Lee is tolerating arimoclomol well and data has shown it is safe and effective. She needs this medication to help her cells with protein misfolding. Approval will mean this becomes standard of care for a devastating disease that has NO approved medication.
Jacqueline K.	South Carolina	Grace, a delightful child, has shown improvement using this medication. She can follow directions much better, with additional improvements. Please give her a chance. She, and many others, deserve a chance at a better life. We can help, you can help. Please make it happen.
Tori H.	Texas	Gracie is a young woman that needs a chance to live she needs this medication, and her family wants this for her, they want her to have a chance of life!
Shannon B.		Gracie is responding to arimoclomol - she is able to complete tasks and maintain focus.
Joy C.	South Carolina	Gracie is responding to medication and showing improvement in severity of symptoms.
April E.	Texas	Gracie was diagnosed with NPC in Dec 2022. She is in the EAP and needs this medicine. Please make it available so she can continue to get better!
Judy B.	California	Our great granddaughter has been on this drug. It is helping. Also have grandson with same disease. Research is very important to us.
Tyler B.	Pennsylvania	Great improvements from original diagnosis. Would like to see him continue to improve.
David S.	Massachusetts	Gwenyth is a 15 year old high school sophomore and 2 sport athlete. She is vibrant, active, intelligent, considering plans for post-secondary education and fortunate to remain largely unscathed by a disease that takes so much from many. Her participation in the arimoclomol trial soon after diagnosis has a lot to do with these positive results. To be forced to witness her decline in the absence of a treatment that has been effective for her is an unconscionable outcome for her & our family.
Amie W.	South Carolina	Has shown arimoclomol is safe and effective.
Jennifer M.	Nevada	Having continued access to arimoclomol is incredibly important to us and for our young child. Liam has been on arimoclomol since December 2022, taking his first dose at 17 months of age. We have had no negative side effects from the drug and have not seen any significant increase of symptoms from his NPC. Arimoclomol is the only drug being researched for NPC that rescues the NPC protein that is produced. With no negative implications this drug should be available for all NPC kids.

Brooke S.	Florida	He has access to arimoclomol now thanks to his participation in a clinical trial. That trial could end at any time and he would lose all the progress he has made.
Danyelle K.	Pennsylvania	He is thriving on this medication. He started kindergarten!
Mary L.	Pennsylvania	He needs it to live.
Julie S.		Helps my niece manage her disease.
Ana R.	Utah	Her quality of life has been improved since diagnosis! Her mother continues to advocate for these efforts because it has given her more time with her child.
Parna B.	Illinois	Hi. My kid is 2 years old. We have him enrolled in an underage trial for arimoclomol under Dr. Patterson when he was around 18 months or so. He doesn't have any Neurological symptoms so far and is not eligible for most of the trial drugs. Having taken arimoclomol will increase his chance of delaying the onset even later. And I believe taking some medicine is better than taking nothing. Also, he has not faced with any side effects after taking arimoclomol.
Kelly M.	Pennsylvania	I am 1 of 6 kids, and three of my siblings were diagnosed in 2019.One is nonverbal and cannot walk. The other 2 not only have to manage their disease but live with a picture of what their future might look like, if they are not able to continue treatment. All 3 take arimoclomol, and while my sister's disease was already advanced, I believe she is still with us because of the drug, and that my brothers have declined less. My family calls it "the miracle pill". Losing access would be devastating.
Kelly S.	Minnesota	I am a 45 year old woman with NPC, who was diagnosed 2-3 years ago at the Mayo Clinic in Rochester, MN. I have taken arimoclomol for 2 years, prescribed by Dr. Mark Patterson, through expanded access. I have found that arimoclomol and miglustat together have kept my health stable, BUT ARIMOCLOMOL DOES IT WITHOUT THE DIGESTIVE ISSUES!
Morgan H.	Utah	I am a senior in high school and I have been just recently diagnosed with NPC. I have been on arimoclomol for just a few months. I think that it helps me think clearer and I'm not as tired as I used to be. I used to come home from school and take naps, but now I feel I don't need to sleep as much. During my last visit with the neurologist the doctor mentioned that my symptoms seemed to have stabilize and don't seem to be progressing as much.
Ashley R.	Utah	I am friends with an amazing mother who has a child diagnosed with NPC. On this medication, the mother (and medical professionals) have seen positive changes in the child - in development as well as general health (fewer ER visits). This child has been able to participate in school more fully and is thriving despite the diagnosis. Her mother has had some sense of peace for the first time in years and the family is enjoying the time they have with their child NOT being in a hospital.
Sanjay K.	Oregon	I am thankful to have a medication like arimoclomol which is in the form of a pill unlike spinal infusion. I feel very confident about my health and managing my symptoms due to this medication. Please approve the drug and save my life.
Kristine F.	Indiana	I believe that arimoclomol has slowed the progress of NPC for Kailey without the side effects of being sedated every two weeks with Cyclodextrin LP injections. We need to continue studying arimoclomol at different doses, and in combination with other treatments for NPC. We have not researched enough for it to be taken away.
Stephanie W.	Washington	I believe that continued access to arimoclomol should be granted. Right now, anything that could potentially help my brother get better or help the medical community find a cure for future cases would be beneficial. My brother’s life has drastically changed in the last 4 years and we don't know how much time we have with him. There's no viable reason for there to be a limit on the resources he can use to help him fight this disease. He is 25 and hasn't even started life. Let him live!
Matthew M.	New Jersey	I feel this drug has kept my daughter stable for the last few years. We have not seen any significant decline. Who knows where we would be if she had not taken this drug or if she would still be alive. She has NPC infantile onset, never walked, and barely talked. Suffered from seizures at the age of 3. We did not think she would live past 5 years old. But she just turned 11 in September. So obviously this is helping her.
Autum H.	Utah	I have a friend whose daughter has NPC and am aware that this drug has helped improve her quality of life but also her chances to live a longer one.
Kelli V.	Texas	I have a friend whose daughter is having success with arimoclomol. It has improved her quality of life.
Cara G.	Pennsylvania	I have access to arimoclomol through EAP, which has enabled me to continue to work full-time and be as independent as possible. I cannot tolerate miglustat and have not experienced any adverse side effects with arimoclomol. I attribute my delayed disease progression to this drug and it gives me the hope and motivation to keep trying my best every day!

Christopher F.	Rhode Island	I have been taking arimoclomol and have been tolerating it very well. There are no side effects! This drug allows me to do activities that I would not be able to do without it. I feel this drug has allowed me to continue to be me and do the things I love to do, like work and socialize. I am very lucky to be in this clinical trial and have access to a drug that can actually help me live a good life. I feel like the NPC has not progressed since I have been taking arimoclomol.
Gauri M.	Oregon	I have seen great improvements in my brother's symptoms. He deserves the medication and FDA should approve this medication and save my brother's life and others like him suffering this disease.
Virginia M.	Connecticut	I have seen the lessening of the progression of this disease through the use of arimoclomol in the trial over the past three years.
Shane H.	Utah	I have watched my daughter regain abilities she had lost. I know it has added time to her life she otherwise would have not had.
Cortny P.	Utah	I have watched my friend's daughter on this drug for the last 3 years. It has given her the life that the doctors didn't think she would have. It has been amazing to watch her progress and be able to see her family get more time with her than anyone thought possible.
April C.	Pennsylvania	I know a little boy who receives this and he's thriving on the drug
Stormy S.	Utah	I know a little girl that had loved 3 extra years of life because of this drug. It has given her precious time with her living family.
Adrian L.	Washington	I need the FDA to approve arimoclomol. There isn't any alternative and our loved ones need care.
Callie W.	Montana	I was diagnosed in January 2020 with NPC1. I started in the expanded access program in October 2020. Yearly testing of my oxysterols have shown improvement in lowering those levels because I am taking arimoclomol. I need to continue using this medication to continue to improve my chances of surviving NPC1.
Tim S.	Utah	I work with the mother and stepfather of a child with NPC and witnessed the impact that this drug had on the quality of life for their daughter. They don't have another option that would match this impact. Losing access to this drug would mean losing her is a very short time frame.
Andrea G.	Utah	I'm aware of a 3 year old baby girl that's depending on this trial for her life.
Elsa B.	Florida	Improvement to quality of life, have her with us for a longer time. My daughter's swallowing and fine motor skills have improved tremendously since starting this drug in 2020.
Krystal S.	Utah	In September of 2020 we were told we needed to start making end of life plans for our daughter, Willow, as they assumed she only had a few months left to live. In October of 2020 she started arimoclomol. Not only is she still alive to this day, but she is happy and has had an amazing quality of life. Arimoclomol slowed down the progression of her disease and even helped make it possible for her to get some of her lost abilities back. Willow would not be here today without arimoclomol.
Thomas K.	Massachusetts	In the four years since her diagnosis, there has been minimal (although certainly present) effects from this terrible and thus far unstoppable, genetic disorder.
Janice Y.	Texas	It has been a benefit to my friend and continuing the medication is crucial
Callie W.		It has helped slow the regression of sweet Willow. Without it she wouldn't be here
Teresa G.	Pennsylvania	It has prolonged his life.
Mj V.		It has saved my nephew's life!
Jessica D.	Alaska	It has slowed the progression of Everett's disease
Leslie M.	Pennsylvania	It has worked for our friend's grandson and has been giving him a chance to live like a little boy should.
Peter F.	Florida	It helped my neighbor's child who had such a promising start until his diagnosis. The medication really helped him deal with the symptoms.
Nic B.	California	It is crucial that no time is wasted in approving medications that have the possibility of assisting in any way possible to reduce the terrible outcomes of this illness. Arimoclomol has seemed to help my nephew's condition and being able to explore the use of this medication with others is a crucial next step to making progress in fighting NPC.
Amanda R.	Pennsylvania	It is saving my nephews life

Amanda W.	Indiana	It is the only treatment we qualify for right now and I do not want to lose access to something that is helping her!
Sharon C.	New York	It is very important for is quality of life.
John W.	North Carolina	It is working for their daughter without side effects!
Amber B.	Pennsylvania	It literally is saving his life.
Alex T.		It saves lives. Rare or not, lives matter, and this saves them. Do the right thing.
Lee L.	South Carolina	It seems to help slow down the progression of the disease and also help her mood swings. Please help save my granddaughter!
Richard L.	South Carolina	It seems to slow down my granddaughter's deterioration. We have observed this since she started taken arimoclomol.
Whitney C.	Utah	It slowed down the progression of the disease and gave our girl back some quality of life. As a 7 year old with NPC she deserves to have a medication that works!
Judy D.	Ohio	It would help her live a happier life, be less frustrated and able to complete tasks.
Marta I.	Florida	It's helping my friend's daughter to have an almost normal life. It's the only med that has helped her so far.
Dean G.	Texas	It's working! Less frustrated, not bothering GI.
Summre S.	Pennsylvania	I've seen how well he's done on the medication. He would not be alive without or doing the things they said were impossible. He's 5 years old and we'd like him live a long life but without access to this medication that won't be possible.
Leslie K.	Pennsylvania	Keep my little cousin alive.
Jeannine H.	Utah	My 18 year old daughter has just recently been diagnosed with NPC. In the last 3 years she has been rapidly losing her ability to walk, talk and eat, due to this disease. Since her diagnosis earlier this year she was put on the EAP of arimoclomol the neurologist stated that her symptoms of her disease began to stabilize somewhat. I hope that with continued studying of this disease and medicines like arimoclomol, that she can get the help that she needs to avoid any more declining.
Denise S.	California	My 20 year old son has been taking arimoclomol for 3 years. Prior to taking this medication he slept most of the time and rarely responded to external stimuli. He is now more awake and alert, sits up, uses his hands to lift his bottom and shift his weight, responds to us either with eye contact or with 1-3 word phrases, sings, and participates in school utilizing an eye gaze device. I sincerely believe that he would no longer be with us if he were not taking arimoclomol.
Wallisa M.	North Carolina	My 24-year-old son, Justin, has been taking arimoclomol for the past three years. It is not only important it is critical that he continues to have access to arimoclomol. Caring for a child with a life-altering condition, particularly one without a known cure, what becomes increasingly important is ensuring that your child is safe, well cared for, and has an optimal quality of life. Due to the deficits NPC has left Justin with access to this drug is essential for dramatically slowing progression.
Nikki H.	Pennsylvania	My 6 year old daughter was diagnosed with NPC at 5 months old. She is currently in a clinical trial, the only way for her to receive arimoclomol so having the FDA approve this medical would be beneficial to her and all the other children with NPC. She is making tremendous improvement by being on arimoclomol. The FDA wants to approve medication that doesn't need to be approved but won't approve medication for children with a deadly disease. Please make this make sense.
Brian S.	Connecticut	My 9 year old daughter has been diagnosed with NPC and has been on arimoclomol for over a year now. Continued access is extremely important to provide the best quality of life for as long as possible. We haven't seen any signs of the disease progressing in a quick manner since on arimoclomol. Arimoclomol gives my family and I the hope that in time a cure is found, until then arimoclomol is all we have.
Catherine M.	Pennsylvania	My absolute best friend of many years has a son with NPC, the drastic change of being excited for her new baby, to the scare of finding out his condition, to finding this amazing clinical trial with this drug, has been a ball of emotions. This trial bringing out the best ones. This drug has allowed my friend's son to live a life every child deserves. He goes to school, he has friends, he does activities. Without this drug none of this would be possible. With this approval, many families stay whole.

Alena B.	Indiana	My child Olive is 9 years old and will turn 10 in March 2024; she has received this treatment since she was 2.5 years old and has been able to survive beyond the life expectancy of children with early-onset infantile NPC. Access to this treatment has given her life: time with everyone she loves and everyone who loves her.
Lauren G.	Pennsylvania	My cousin Brody has been getting arimoclomol, is thriving in school and doing so well! It has helped him tremendously!
Shivani P.	Arizona	My cousin Sanjay Kambhatla is on the clinical trial and has seen significant improvements in his condition and I would like to see him have continued access to arimoclomol. It is my earnest appeal to the FDA to approve arimoclomol by outweighing the benefits seen in real patients over any perceived risks. As there are no other approved treatments for this debilitating condition, I sincerely hope Sanjay will have continued access to arimoclomol.
Mandy B.	Colorado	My daughter Amaria has Niemann-Pick C. No cure, no FDA approved treatments. She currently receives arimoclomol through the expanded access program and we would like to continue having access. The prognosis for NPC is death. Delaying access to any drug that could potentially slow the progression of this disease is inhumane. The only thing worse than knowing your child will die is being denied the ability to try everything you can to save them. Arimoclomol is hope. I need that!
Kelly L.	Texas	My daughter Gracie was diagnosed with NPC in Dec 2022. This devastating disease has NO approved medication - that is hard to swallow and needs to change. Gracie enrolled in the EAP in October 2023. Data has shown arimoclomol is safe and effective. Gracie is tolerating arimoclomol well. Her cells need this medication to help with protein misfolding. Approval will mean it becomes standard of care and could work synergistically with additional medications to stabilize this disease.
Keri S.	Massachusetts	My daughter Gwen has had access to arimoclomol for two plus years now. Because of the medication she has had little to no regression of her NPC symptoms. She is a sophomore in high school and can participate in High School soccer and basketball teams due to having access to arimoclomol.
Robert G.	Virginia	My daughter has had an improvement with swallowing since she started on arimoclomol, otherwise she would probably be on liquid thickening agents or a feeding tube. She loves her food and would be devasted and depressed if she was no longer able to eat the foods she loves.
Cindy R.	Oregon	My daughter just started on the expanded access program last month. She was diagnosed a year ago at age 23 when she was just moving into the adult phase of her life and now the future is dark and questionable. Arimoclomol brings us light and hope. We hope other NPC individuals and their families will be able to share in that in 2024.
Lisa T.	Illinois	My daughter luckily has access to arimoclomol. It is a pill, which makes administration very easy (as opposed to an infusion). My daughter has early infantile NPC and is now 9 years old. Without our drugs, she had a life expectancy of 4-5 years.
Tayva T.	Oregon	My daughter was diagnosed with NPC February 12, 2021. She had no access to medication until I was able to get her into a clinical trial at the Mayo Clinic. I worry every day that the trial will end and she will no longer be able to receive the medication she needs. On the meds I have seen her tremors lesson, she has been able to understand schoolwork better which helps with her confidence. She isn't as wobbly while she walks and is just an all-around happier kiddo.
Susan W.	Montana	My daughter was diagnosed with NPC1 January 2020 and has been in the Expanded Access Program since October 2020. After three years of yearly monitoring oxysterol levels, there is significant improvement. The continuation of arimoclomol is paramount in my daughter's treatment for NPC1
Roger F.	Indiana	My daughter, Kailey has NPC. She has been receiving arimoclomol after transitioning from the Cyclodextrin trial. The 3x daily pills have allowed her an improved quality of life vs. the Cyclodextrin infusions which caused balance issues for days after treatment. The capsules are much less invasive, carry less risk than LP's, have shown no balance issues, still reduced disease progression, and she maintains her ability to swallow compared to the period after diagnosis and before any treatment.
Mary-Brooke D.	Texas	My dear friend and neighbor's daughter was diagnosed in December 2022, and she is being helped by taking this medicine. She is a beautiful young and vibrant teenage girl and I pray she is able to continue this medication.
Heather M.	Illinois	My dear friend's daughter is benefiting from this medication. Her mother sees her happier and able to process and respond to directions with more ease.

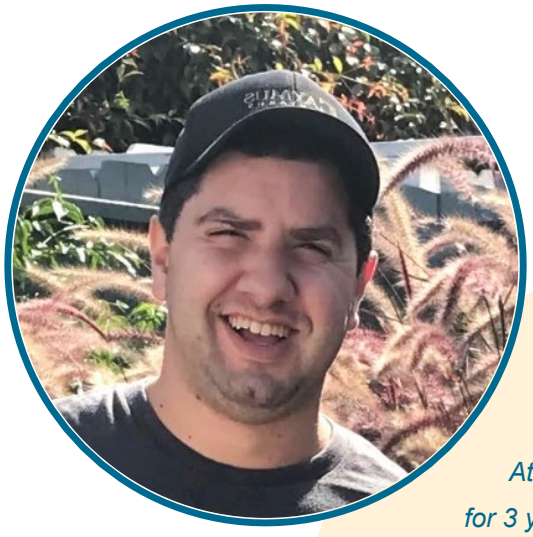
Fatima C.		My friend has been using arimoclomol for his NPC. I met him in his early twenties, and he was predicted to have at least three years more before his NPC made him unable to walk. I have come to know him well these past three years while taking this drug, and he has been able to delay the process significantly. He's still able to walk and drive and enjoy activities he loves.
Travis M.		My friend's daughter has been taking the drug and has had great improvement in many ways. Hoping this will be made available to others that can benefit from it. Thank you so much.
Alecea P.	Utah	My friend's granddaughter was diagnosed with NPC. They were told at that time to start making end of life plans. She has been on this drug for 3 years. That's 3 years that my friend and her daughter have had to take sweet Willow to Disneyland with her cousins, to see Willow take modified dance classes with her cousins, to celebrate birthdays, Christmas, Thanksgiving, and so many other events this family did t think they would get with Willow. This drug has given my friend the gift of her grandbaby.
Patty D.	Utah	My granddaughter was declining quickly and we knew we were going to lose her in months when she started this. 2 years later we still have her and she is doing well. We want to keep creating memories with this beautiful child.
Louis R.	California	My granddaughter has NPC disease. When she was a little less than two years old, I noticed she was forgetting information she had known. She no longer knew my name. After starting treatment knowledge and skills started growing again.
Brad D.	Utah	My granddaughter was diagnosed with NPC at about age 2, we were told she had a life expectancy of 5 years old. Due to arimoclomol we just celebrated her 7th birthday! Without this life saving drug we fear that she will decline very rapidly, and we will lose her.
Carmen N B.	Florida	My granddaughter will have a better quality of life. This is a very cruel disease.
Bobbi G.	Washington	My grandson, Charles Wong was diagnosed with NPC 5 years ago, just before he turned 21 years old. He was an incredible athlete from a very early age. He played softball as a pitcher, and then went to State for his high school tennis team. Due to his diagnosis, he has not been able to do all these physical activities. However, thanks to being part of the arimoclomol, drug trial program, he has successfully graduated from Washington State University on Dec 9, 2023, with honors in. Computer Science.
Linda K.	South Carolina	My great-niece with NPC has recently been on arimoclomol and has been happier with more emotional stability and has been able to complete more tasks with greater facility on the drug.
Tatiana B.	California	My nephew deserves the opportunity to grow up alongside his many cousins, celebrate milestones and live a happy life. If this medication can give him more meaningful time with his family, it should be approved. This is his only hope.
Gregory M.	California	My nephew Everett has NPC and has been on arimoclomol. It has seemed to make a significant difference in his course and thankfully he is still able to walk and speak to us. I love Everett very much and since this medicine is improving his life, I respectfully ask that you make it available to all children with NPC. We hold our hope for a cure, but in the meantime, this is one of the only things giving him a chance to survive until then.
Phillip C.	Virginia	My nephew is a person living with NPC and has had access to arimoclomol and it has improved his well-being and allowed him to accomplish daily tasks he previously could not. If he were to lose access to this medication it would prove detrimental to his well-being.
Carolyn S.	North Carolina	My niece in TX is on arimoclomol & it seems to be helping. She is happier than before taking it.
Jeff A.		My niece was diagnosed with NPC and it appears to be making a difference for her in which she is happier and able to take better direction when completing tasks.
Victoria P.	Utah	My niece Willow has expanded her life expectancy farther than any doctor ever expected before arimoclomol. My sister was told to start making end of life plans with her then four year old daughter. My niece just celebrated her 7th birthday. Willow has been able to stop regression and gained back some limited motor skills such as holding her own cup. She needs this medicine to continue to sustain her life that is already sentenced too short."
Susan G.	Washington	My oldest son has been taking arimoclomol for a few years and I have seen very little progression of the disease.
Meaghan B.	Minnesota	My sister has been fortunate enough to have access to arimoclomol. While on this medication, we have seen minimal progression of her disease. This has allowed us innumerable priceless moments to share as a family that would otherwise have been impossible had this devastating disease been allowed to run rampant. My sister is a loving and warm person who I cannot imagine being without. Arimoclomol helps to keep her herself and the time to experience life's joys which can be stolen by this disease.

Chip S.	Florida	My son Cole was diagnosed with NPC he slowed cognitively, his balance was unstable, his speech slowed, he had a hand tremor, and he was very quiet. When he started arimoclomol (A) months after his diagnosis, we noticed a positive difference right away. Many of his symptoms were better, especially communicating. (A) allows him a better quality of life. Without this med he will digress fast. It's more than extremely important to our son that Arimoclomol continue to be available, it is lifesaving!
Joseph S.	California	My son Connor was diagnosed with NPC when he was eight years old. He started taking arimoclomol at age 17. When he first started taking Arimoclomol he improved dramatically & did things he had not done in years. He is 20 now & has been taking it for the past 3 years. During this time the progression of NPC has slowed to a crawl. If we were on the old path without Arimoclomol, I am sure things would be very different. I am very thankful for the extra time we now have with Connor.
Dawn S.	Florida	My son currently takes arimoclomol through a clinical trial with Boston Children's. Each few months as we start to run low, I begin to panic that it won't be available or it won't be approved. I can't put into words the fear of not having access to this medication that has shown improvement in my son's speech and tremors.
Barbara L.	Connecticut	My son David, age 34, has been taking arimoclomol for 3 years. Before starting he exhibited numerous oral/swallowing issues including gagging, coughing, choking, drooling, and dysarthria. Since taking the drug his oral symptoms have stabilized and improved. He no longer drools, and his coughing and choking are significantly reduced; his dysarthria is minimal. This has provided David with a better quality of life as he has maintained his independence in eating and other skills.
Nadezhda K.	Illinois	My son Denis (16 years old) has been taking arimoclomol since August 2021. His swallowing and mobility improved a lot due to this medication. A huge improvement in swallowing allows him to eat any food he wants. He also can walk without support now and for much longer distances than before he started the medication.
Linda D.	Minnesota	My son died from this disease in 2021. He was taking arimoclomol for 6 weeks. If it had been available earlier, he may be alive today. It is imperative that those still struggling for survival be given this chance to live!
Amanda F.		My son has been fortunate to have access to arimoclomol. I believe this medication has slowed down his progression. He has had no side effects and tolerates it well. I encourage the FDA to finally approve a much needed medication for this fatal disease.
Elaine W.	Ohio	My son has been on arimoclomol for a couple years now and has been doing amazing. Since starting the medication, he has had straight A's in every quarter of the school year. He remains symptom free and doing very well. We need this medication to help keep him as a typical 14 year old.
Michael F.		My son has been receiving arimoclomol for two years. I Promote any medication that has the potential to slow progression and/or provide a cure for this and any Ultra Rare Disease. I encourage the FDA to become more realistic in their standards for approval of medication for Ultra Rare Disease. It is not the responsibility of the NPC community to accommodate the FDA, however, IT IS the FDA's responsibility to accommodate the Ultra Rare / NPC community in approving medications.
Clare F.	Rhode Island	My son has had access to arimoclomol through a clinical trial. This drug has been incredibly important to the wellbeing of my son. Since he has been taking arimoclomol, his health, physical and mental, has not declined. This drug has allowed him to continue to work, socialize, and lead a life of purpose. If this drug were to be taken away, his life would change drastically! His health would decline and therefor he would not be able to do the "normal" things he can now do.
Dianna W.	Ohio	My son has recently been granted access to arimoclomol through the expanded access program at Rush University. He is 9 years old and has so much more life to be lived. Any help from these medications to allow him the ability to have a somewhat normal life, with as few limitations as possible is optimal. In the short time he has been taking it he has experienced no side effects but has gained more agility and alertness. Please give my son a chance to live well.
Alexander K.	California	My son Jasper has been on arimoclomol since May of 2017. He was declining rapidly prior to beginning the arimoclomol trial but stabilized dramatically within a month of being on the drug. His dysphasia disappeared completely. What is more stunning is that his liver function tests (ALT and AST) were highly elevated prior to arimoclomol treatment, but completely reverted to normal levels within 2 months of beginning Arimoclomol. Losing access to Arimoclomol would be devastating for us.
Laura F.	Illinois	My son Joseph is living with Niemann-Pick Type C. Before this drug was offered to our son, he had such a hard time swallowing. He's been on arimoclomol for just over a year and we have seen improvements with his ability to swallow. This horrific disease has caused my son to lose so much weight and I fear without this medication he wouldn't be here today. He can at least eat and drink food without the use of a feeding tube. I pray that this life saving medication continues to be an option for him.

Srikanth K.	Oregon	My son Sanjay is in a lot better health today after almost 3 years of arimoclomol. It is a miracle drug. We see improvements and stability in all endpoints of NPC after starting the drug. Please approve the medication and save our son's life and all the other children using the drug.
Shanda H.	Alabama	My son was diagnosed with Niemann-Pick type C early February 2020 when he was just 10 years old. He was given access to arimoclomol in late November 2020. So for a little over 3 years, we have been blessed with this miracle drug. In the time that my son has been taking arimoclomol he has been able to come off of his ADHD medication. He has continued to grow into a young teenager with no further issues or complications from NPC. I strongly feel that arimoclomol is saving his life.
Pamela G.	Washington	My son was diagnosed with NPC 5 years ago, 2 days before his 21st birthday. Thanks to the arimoclomol trial drug program that our son has been a part of since his diagnosis, he was able to successfully graduate from Washington State University this December 5, 2023. He is a Computer Science Major and is now looking for employment as a full-time developer. We have seen how well this drug works for our son in slowing down the progression of Nieman-Pick type C. Thank you for this miracle drug!
Jessica M.	Minnesota	My son was diagnosed with NPC at the age of 15 in December of 2022. The moment his diagnosis was confirmed I felt like my whole life was over - and that I might never breathe again. When he started taking Arimoclomol in February of 2023 - I saw a light turn back on - and there was hope again. Connor has made improvements in his speech, in his physical coordination, swallowing and most importantly in the light that he is able to project into the world.
Anna K.	Oregon	My son's life changed with the drug. From 2016-2020 we saw symptoms with involuntary twitching in the arm, gait, speech, swallowing, cognition, and vertical palsy. All these symptoms started increasing and finally in 2020 we got the diagnosis. We started arimoclomol the life saying drug, his gait and balance improved, he climbed 2400 steps up a mountain in India last week, he rarely chokes with liquids and food, his speech is much clearer, and cognition is stable. It is a miracle drug for my son.
Stephanie C.	Texas	My special needs student has had access to arimoclomol in a clinical trial and is tolerating it well. Since she has been on the drug, I have noticed improvement in her mood as well as work stamina. The difference I have seen in her is remarkable and I believe continued access to the drug would improve her education as well as quality of life.
Rachael B.	Minnesota	My younger sister has NPC and has lived with it for over 10 years. Her life is much different than everyone else. No friends to see regularly, she can't drive, she lives with my parents while seeing everyone else get apartments, buy houses, start their own families and blossom into their careers. She gets none of that. The disease has placed boundaries on her life and taken away a normal one. Arimoclomol gives her moments of normalcy in a society that has forgotten her. Do not take that away.
Michelle J.	Florida	Our 15 yr old neighbor was diagnosed with NPC 2 years ago. Without the medication he wouldn’t be able to continue going to school, throw a baseball or be social and converse. Before this medication, there was no hope and he and his family were very depressed. This medication has given them hope and returned them to somewhat of a “normal” life. Since there is no cure for this disease, this medication can give him a much better quality of life.
Francisco B.	Florida	Our daughter has been on arimoclomol for approximately 4 years and has shown considerable improvement in many areas tracked by the CSS scale. It doesn't matter that it doesn't address every conceivable symptom of this heterogenous disease, that's not a realistic expectation. At this stage, most parents with NPC kids just want to buy time until other more targeted treatments are developed.
Beverly A.	South Carolina	Our granddaughter has NPC, and this drug appears to be beneficial for her with little or no adverse effects. We are encouraged that this drug seems to be helping her.
Paul A.	South Carolina	Our granddaughter was diagnosed with NPC and is currently participating in the arimoclomol expanded access program. This medicine appears to be slowing her disease progression and any medication that improves her quality of life is extremely important.
Donna S.	Kentucky	Our Grandson takes arimoclomol for NPC. Since using this drug we have seen a significant slowdown of the progression of this horrible disease. I know we have been fighting for a while now to please keep this medication. I wish you all on this board could actually see the difference in our kids how it had changed them given them and their families more precious time that we do not feel would have ever been possible without this medication. We are hopeful that you all will consider this.
Catherine M.	Kentucky	Our Jeffery has NPC1. He’s such a joy and blessing to everyone who comes into contact with him. He has been on arimoclomol since the summer of 2023, and we have noticed great improvement. Jeffery has not been affected much cognitively yet from NPC but has issues with his liver and spleen. That being said, he has improved his handwriting skills as well as reading since being on this medication. He’s even doing division and has mastered multiplication facts. This needs to be approved.

Ashley A.	Kentucky	Our little girl is on it, and we need to ensure we can always get her meds for her! We have seen some improvement on her symptoms
Rhonda R.	Missouri	Our loved one is having a positive response to arimoclomol with no side effects. I encourage this drug to continue to be available.
William M.	California	Our loved one is afflicted with this horrific disease. He is an otherwise healthy and good-looking 12-year-old. He has access to arimoclomol, and it is essential that he and others continue to have access because it gives them the only hope they have in an otherwise dismal outlook. Our loved one responds favorably to the treatment because of improvement in the course of his seizures.
Gwen H.	Virginia	Our son took arimoclomol until he passed away on October 26, 2023. We know that arimoclomol helped to improve his swallowing and speech. He was able to stay on a regular soft diet until the last couple of months. Please approve this medicine. This disease needs a multi-drug approach in order to prolong and sustain the lives of these precious young people. Thank you.
Jay H.	New York	Our son, JP started using this drug and has improved his strength and ability to move around on the floor on his knees. Prior to this drug he hadn't been able to do this for about a year! Talking and energy seems better. Not a cure but a miracle in hopefully stabilizing this terminal death sentence."
Elizabeth F.	California	Please allow this medication to help families! I love my niece and she needs it!
Bryan T.	California	Please approve this drug for our beloved friend Everette Brutocao. It seems to be helping him and we could do a lot more with its approval. Thank you so much, Bryan Tilbury.
Paul N.	Massachusetts	Please approve this therapy its life changing.
Joseph O.	Pennsylvania	Since starting my daughter on the medication, she has been much improved. It seems to have stabilized her since initiation of the drug. Prior to arimoclomol she was very tired all the time, and unable to perform activities of daily living. She now is interactive, and her cognition is improved
Joyce P.	Utah	So far we have 3 extra years that we wouldn't have had were it not for this drug. Please, please, please help those that need it have as much time with a loved one as possible.
Deb S.	Tennessee	So she can live a full life.
Maria D.	California	So that my granddaughter can stay alive.
Debra F.	Connecticut	The individuals I know have benefitted from the drug. I am close with the parents, and they note dramatic changes in their son's skills.
Destinee G.	Pennsylvania	The little boy in my family has been receiving this medication since he was a baby and it's literally been keeping him alive.
McKenzie R.	Utah	The most adorable and sweet girl in our community has had additional years of life with her family due to this amazing medication.
William B.	Maine	The results are positive. Symptoms of NPC are better.
Diane S.	South Carolina	The treatment so far seems to be improving the child's condition and quality of life
Sasidevi B.	Illinois	There is a significant improvement in condition for all end points. There is no approved medication, and it is critical to approve this medication to stabilize and continue to see improvements.
Katie H.	Connecticut	There is currently no medicine available to help those with NPC. A family member of mine has taken it for the last 2-3 years, and we feel as though it has slowed the progression of the disease and has improved his quality of life.
Phillip B.	California	There is nothing that has been approved to treat my 12 year old son. It seems as though all that the legislative system has to offer our small community of suffering individuals are roadblocks and excuses. My son's treatment is providing us time to be together as a loving family before his untimely demise. Restricting or limiting access would be an unconscionable action taken against us who are seeking no more than to be with our loved ones; but are afforded no treatment options.
Taylor S.	Massachusetts	There is so much to manage and cope with as a caregiver of someone (especially a child) with a disease like NPC. Knowing that the disease is degenerative and fatal is a constant worry that gnaws at you every day and taints every experience. Trying to coordinate the medical care required-doctors, therapists, health insurance, equipment providers-is a full-time job. Let's NOT add "fighting to maintain access to a treatment that helps your loved one" to the long list of worries and responsibilities.
Paul B.	California	These individuals should have access to all possibilities of care.

Sue Z.	Washington	They deserve to LIVE!
Jesi M.	Colorado	This could be a game changer for my friend's daughter. This is a fatal disease. Why not give every single option to help those with NPC!!!
Rebecca G.	Washington	This disease steals everything from sufferers in a brutal, horrific fashion. While a cure to eradicate is ideal, anything to stem the tide of progression or provide relief from symptoms is a miracle. My spouse has already lost one sibling to the disease, leaving three siblings to face it. Since being on this medication, his condition has been fairly stable. To lose this access would likely speed up progression, robbing him of years and quality of life. Please consider a path forward for others.
Lara F.		This drug gives people with NPC a longer chance at life. Every moment is important. This drug has been given to my cousin's daughter 3 years since they told her family to start making end of life preparations. That is almost half of her life.
Elisha S.	Virginia	This drug has been remarkable for slowing down the progression of my niece's deadly diagnosis. So many other children with this rare disease deserve the chance to live longer and hopefully find a cure for NPC.



At 34 I have been taking arimoclomol for 3 years. I feel this has helped me with my eating and swallowing skills. Before taking arimoclomol, I would cough and gag when drinking or eating but after taking the drug those things have greatly improved and I feel safer. I also used to drool regularly but since starting the drug, that has stopped. I feel very strongly that this drug is essential in treating NPC and want to continue to receive the benefits I have been getting. Please approve this drug.

David L, CT

APPENDIX B:

Community Comments to FDA

If you or a loved one has not had access to arimoclomol, please describe why having the option to take arimoclomol would be important to those living with NPC

Antonio G.	Washington	Access would be important so they could live a more feasible lifestyle without pain
Denise M.		Allowing my child to take arimoclomol in addition to other drug therapies could be life changing for my child and extend his life and quality of life.
Kristina A.	Alaska	Anything to keep this child alive and with his family. Do everything you can.
Felix C.	Massachusetts	Arimoclomol could very well be the only thing that stands between a young soul's bright future and her eventual total incapacitation.
Marci W.	Utah	Arimoclomol has allowed this sweet girl Willow to live past her life expectancy. Please get this important medication approved so she can continue to live a happier life.
Wallisa M.	North Carolina	As a parent with access to information about clinical trials and potential, promising treatment options for NPC, I am very aware that having access to multiple therapeutic options will result in the most optimal outcome for the disease. Given the presentation can be so variable by individual, it is imperative that those that do not have access today need a path to gain access moving forward. This is not optional, it's a necessity.
Tayva T.	Oregon	As I stated above, having access to arimoclomol changed my daughter's life in a huge way. Before she had access to the medication, she was having a hard time walking, understanding anything that was going on at school, she was choking on her food, water, and her own saliva. That has all changed, I feel like she may have a chance at a slightly functioning life if she can remain on this medication.
Kenneth W.	South Carolina	At least one patient with this disease has taken this medication and seen improvements on a consistent basis in behavior by family members.
Megan K.	Pennsylvania	Because it's saving lives.
Amanda S.		Because then everyone would have a lifesaving option!
Caitlyn C.	California	Being given the opportunity for treatment is important to the overall mental state of the patient to be able to believe they are doing everything to heal instead of mentally giving up
Amy P.	Oregon	Clara is almost 4 years old. She has not had access to this drug which could not only extend her life but improve the quality of her life. Drugs like arimoclomol have a profound impact on patients, their families, and their communities. Our entire church community is praying for Clara and her family. Over the course of the year, another drug treatment has improved Clara's motor development, her speech, her alertness and most of all, her sense of humor. She now has the chance to go to school.
Daniel R.	Virginia	Currently there is no approved treatment for NPC. Without treatment NPC will kill my wife. Arimoclomol may be a treatment that extends her life and provides an improvement in her quality of life. Having an approved treatment may allow for additional trials wherein a cocktail of drugs could be tested, providing an even more effective treatment.
Meghann F.	Virginia	Currently, there is no one-size fits all, magic pill solution for NPC. We are hopeful that gene therapy may be that solution one day but know that will take time. NPC patients don't have that kind of time. We need to be able to treat our loved ones utilizing a cocktail approach. Patients and their doctors should have access to determine which combination of treatments will be most effective. NPC patients need as many weapons as possible to fight this horrific disease.
Judy B.	California	Drug is helping great granddaughter. Would love to have grandson on same drug
Wendy M.	Michigan	Due to multiple therapies that do not allow for NPC, WE have been denied access to arimoclomol and this has saddened me that the Cures 2.0 act is not helping.
Brittany W.	Alabama	Everett is a part of our family and that he deserves the best chance at fighting this disease.
Molly S.	California	Everett deserves the chance to live and receive this care
Garland A.	Virginia	Everyday we're faced with uncertainty and doubt about Abby's future. One thing is for certain. She will die from this terminal disease and the nightmarish progression of childhood dementia. The current drug is working! We are under no illusions or delusions that the one drug she tentatively has expanded access to will cure her, but we know having access to multiple treatments will have an additive effect in the multiple ways NPC attacks this sweet girl. Let us have one treatment approved. Please.

Alicia Y.	Utah	Everyone deserves a chance to life. Knowing a little girl who is living with NPC, I have seen her regress over the last 2 years, to the extent that her family was instructed to put her on hospice. However, arimoclomol has given her 3 more years to life. If given the choice to give access to something that works, then why not accelerate the possibilities with this answered medication? She's getting that extended chance, why not others?
Nic B.	California	Families afflicted with this terrible disease need to be given the opportunity to fight for any help that may exist. The hope of this medication alone provides a benefit.
Dawn D.	Texas	Give them a chance. Give them hope.
Mike F.	New York	Have a friend who lost a child with NPC.
Gail K.	Illinois	Having combined therapies will be a life saver for my son. Every patient presents differently and each drug hits different areas of strength for the NPC patient. My daughter passed away in 2020 and I firmly believe if we had combination therapies, she would of survived. Please help our loved ones, time is not on our side.
Bryan H.	Oregon	Having access to ANY therapy for NPC is vitally important to improve the patient's quality of life and give hope in a hopeless disease. You are well aware there are NO THERAPIES available for NPC currently. Having access to Arimoclomol is extremely important, and we would start on it right away. Please consider. Blessings
Sara P.	Minnesota	Having multiple therapies is critical to fighting back the progression of NPC.
Kelly S.	Minnesota	Having the option to take Arimoclomol is important to us. It's not a cure, but it keeps us stable for longer, while we wait for the long-overdue cure for our life-altering terminal disease.
Amber B.	Pennsylvania	He's a little boy and deserves life
Murray M.	British Columbia	Our precious daughter passed away, as the result of NPC, almost 20 years ago! Mareena failed to thrive and was taken to BC Childres Hospital when she was just 6 weeks old. Without a diagnosis, we were sent home with the advice, "Enjoy your little girl while you can"! After many visits back to this hospital, hundreds of tests, and much angst, we were informed that Mareena suffered from NPC, there were NO cures and that she would probably not live to graduate from high school.
Daniel L.	Connecticut	I am 36 years old with NPC. I cannot access arimoclomol because I am taking a different trial drug. I would like to have arimoclomol in addition to my trial drug as it has helped my brother and I feel it would help me too. I am still independent in eating but have difficulty with chewing and swallowing at times and can choke if I am not careful. I have seen how this helps my brother and would like to be able to take it also. Please consider approving this drug for all of us patients.
Joseph S.	New York	I am in support of any therapy that would help these kids suffering from this.
Terri R.	Oregon	I feel anything that may extend my great niece's life or improve the quality of life should be accessible. This disease is devastating and life stealing. Anything to improve life would be a gift.
Tiffany R.	Nevada	I have 4 boys who have NPC, my oldest passed away in 2019. They are currently receiving infections of VTS 270. It would be lifesaving to add additional therapies to try to fight this terrible disease. Please help save my three remaining boys.
Brad B.	Oregon	I have a 3-yr old with NPC. While we chose to go with a different Expanded Access Program (EAP) -- adrabetadex in our case, that was due to information we received from medical professionals on how arimoclomol works versus other options and the level of proteins our daughter is making, which is almost null. That said, I've spoken with people on arimoclomol and people who have kids on arimoclomol, and those patients are alive and well. The FDA should give NPC patients more options, not less.
Christine K.	Virginia	I have a friend whose 43 old son has NPD and he is getting worse. This drug will help him
Samantha B.	Minnesota	I have been treated with Cyclodextrin for the past 10 years. However, my disease is still progressing. I need additional treatments to stop my disease or slow it down even further. Alternative approved treatments would help me live a healthier, more fulfilled life. I support the resubmission of Arimoclomol towards FDA approval.
Scott W.	Florida	I lost a child to a disease and I support parents and children who have to fight for their children's lives.
Tauni D.	Indiana	If arimoclomol were to be approved as a therapy for NPC, it could be of significant importance to those living with the condition for several reasons: Disease Management: NPC is a progressive disorder that affects various organs, including the brain, liver, and spleen. Having an approved therapy like arimoclomol could potentially slow down disease progression, helping individuals manage their symptoms more effectively.
Anah M.	Kentucky	If this medication can help people with a RARE & FATAL disease what's the harm? People need hope & that's what this medication can give! As a friend of a momma who has a baby girl with NPC, I can see that all they are looking for is a chance & hope. No mother should have to bury their child.
Jessica E.	South Carolina	If you can read, write and speak then imagine it all being taken away from you. Exactly, this is to help Gracie Lee.

Catherine M.	Pennsylvania	IT GIVES THEM LIFE!!!
Carolyn A.	California	It is essential to enhance the quality of life for those with NPC.
Vanessa M.	Texas	It is helping to better the lives of those with this disease and make a huge impact
Stefanie P.	Ohio	It saves lives isn't that enough?
Parna B.	Illinois	It will improve the chance of delaying the onset or even help him stabilize the Neurological problems.
Jeff A.		It would be important to help improve quality of life and slow the progression of NPC
JoAnn W.	New York	Justin Williamson is my nephew with NPC. His Mom Alice Williamson Bare asked me to sign and advocate for Justin on this issue. I trust her inspiration and request because she is a Registered Nurse and has been involved with Justin's NPC Treatments.
Carley S.	Pennsylvania	Life. Life is important.
Tina D.	Virginia	Local girl is responding well to treatment.
Cindy P.	Arizona	My children died before Arimoclomol was developed. As a patient advocate for 29 years and a co-founder of the Ara Parseghian Medical Research Foundation, I have come to know the disease progression of NPC. This drug has extended the lives of NPC patients and improved the quality of lives. The FDA needs to approach the rare disease drug development process to ensure patients have access to drugs that make a difference.
Chasen B.	California	My cousin, Everett, has struggled with NPC his whole entire life. I love Everett and want the best for him. This drug could help prolong his life and make it easier for him to live with NPC.
Theresa G.	Pennsylvania	My friend's grandchild has been living with this disease and surviving with this drug for 4 years. To prohibit access would severely reduce his chances of survival! I'm not sure how someone could do that with a clear conscience!
Angela W.	Utah	My friend's granddaughter has NPC and has been on this drug for three years. Three years ago, they were told to start planning for end of life. This drug has given three years they didn't think they'd have with their granddaughter (and hopefully more to come come).
Tracie C.	Oregon	My granddaughter has not had the opportunity to take arimoclomol. Getting this medication approved would give her the opportunity to have improved symptoms and quality of life.
Kimberly N.	Georgia	My granddaughter was diagnosed with NPC less than 30 days ago. We are praying for a miracle and hope this may help extend her life. She is seven years old.
Francis (Frank) M.	Virginia	My grandson Everett has Niemann-Pick type C. We need to try everything we can to fight this debilitating disease. Thank you!
Lisa L.	California	My Jessie never had access to arimoclomol. Her disease had progressed to such a degree that she did not for the profile for it. She remarkably lived till 24 years old and I believe she would have had a better chance for a longer life with treatment options. Arimoclomol is an option we would have wanted for our daughter. Please make it available to give other families hope!
Alec K.	Illinois	My name is Alec Koujaian and I have NPC. I need treatments such as arimoclomol to give me a fighting chance to live a normal life. I know other NPC patients that are on arimoclomol, and they definitely see the benefit of it. I need the same chance and urge the FDA to approve it so that I can be on it also
Belle & Abby A.	Texas	My name is Belle Andrews and my little sister, Abby Andrews, and I both have Niemann-Pick type C. Please approve arimoclomol so that my sister and I can have treatment options to treat our NPC. There are no approved medicines for NPC and I hope you will change this and approve this medication for the treatment of our condition.
Judy W.	New York	My nephew Justin has been robbed from a normal life because of this horrible disease. I truly hope those with NPC can have access to this.
Douglas P.	Louisiana	My nephew was diagnosed with NPC at age 4. He died at age 10. My niece was diagnosed almost at birth. She also died at age 10. I watched my nephew lose the ability to ride his bike, ability to walk and talk. And finally, his ability to breathe. My niece never did walk on her own. She never talked, although she was definitely able to communicate what she wanted! What a difference even a limited treatment would have made. Another day on his bike. A chance to say "love you Mom".
Barbara L.	Connecticut	My other son Daniel, age 36, does not have access to arimoclomol as he receives a different trial drug. After seeing the positive effects on David, we are eager to add arimoclomol to Daniel's protocol. This would enable him to benefit as his brother has and provide important information on the effects of combined drugs. It is widely felt by clinicians, patients, and families that the management of NPC will be best achieved through a combination of approved treatments.
Susan G.	Washington	My other son would like to take arimoclomol but has not had the opportunity to do so. We would like for him also to be able to have the opportunity to be able to receive this medication. It is important to have something that can help slow the progression of this disease.

Sherry J.	North Carolina	My sons were diagnosed with NPC in 2014. They are both in their thirties and live with their dad and I. If there is anything that can help my boys live their best life, I am on board. Watching their decline has been heartbreaking.
Adrian L.	Washington	Not having it would otherwise speed up or lead to the inevitable symptoms of NPC. There is no alternative.
Philip M.	Connecticut	NPC disease is a whole-body, neurological and systemic disease. All NPC patients, who are mostly children, will need access to multiple approved therapies in order to stop the progression of this fatal disease. Arimoclomol has demonstrated efficacy and will be an important part of an effective treatment regimen.
Haroutioun K.	Illinois	NPC is a cruel disease that robs our kids of the ability to walk, talk and eventually pass away. I have been following families that have kids who are on arimoclomol. Not only it is safe, but also it has improved the quality of life for many of the kids. We need treatments for our kids and arimoclomol is such a treatment we desperately need.
Anthony L.	California	NPC is a dreaded disease. Our daughter, Jessica, valiantly fought it for years but passed away in 2021 a month after her 24th birthday. Had arimoclomol been available for her, we would have put her on it in the hope that it would alter the trajectory of her disease progression and improve the quality of her life. Other families need this treatment to give hope to the dreams that their loved ones can live a long, full life.
Beth L.	Pennsylvania	One of my best friends has a grandson with this disease. It's heartbreaking.
Douglas B.	Minnesota	Our children need as many options as possible to stave off the horrendous effects of NPC. For those living outside of the US as well as many in the US, arimoclomol is the only option. I strongly advocate my support for the resubmission of Arimoclomol to benefit our communities need for approved treatments.
Sarah M.		Our dear friend Everett Brutucao is fighting for his life and would desperately benefit from access to arimoclomol.
Tina P.	Texas	Our friend's daughter is diagnosed with this rare disease!!! We want her to have a chance and it this drug is able to give her more time we are all for it!!!
Nancy S.	Missouri	Perhaps if arimoclomol had been available, my daughter would not have died in 2004.
Ida C.	Florida	Prolongs their life and slows progression.
Roberta M.	Massachusetts	The girl I know who has NPC is 15. I want her to have a long healthy life. If arimoclomol can help her to have that, it very important to her and all who love and know her. Let's get all the help we can!
Tara M.	Texas	The option to take arimoclomol provides access to improve abilities due to its ability to halt some of the underlying causes of NPC. When the alternative is faster degeneration of youth, there is no reason why families shouldn't be able to elect an option that will improve quality of life and increase the length of life.
Sue Z.	Washington	They deserve to LIVE!
Amy W.	Connecticut	This drug could have extended the life of my best friend Kelly. Kelly died one month before her 43rd birthday but due to NPC he was aged to that of a 9 year old. Kelly never got to meet the man if her dreams and start a family. At 27 when I had my first child we were putting Kelly into adult daycare because she couldn't be left unsupervised. We need more treatments for NPC
Amber E.	Utah	This drug works!
Alice B.	Virginia	This medication would be ideal for my son as it can be taken orally and has very few side effects. It is difficult to travel with him to early access locations. He has adult-onset NPC but has increasing difficulty with balance and walking.
Jason S.	Virginia	This treatment could significantly reduce disease progression and extend my loved one's life, allowing her a greater quality of life and happiness for her and her family.
Catherine N.	North Carolina	Those living with NPC should not be denied treatment for their disease solely because there are so few of them affected. In my opinion that is a form of discrimination against those patients. Why not give them the opportunity to take arimoclomol - either it works or it doesn't - rather than making them and their families and friends face their eventual demise with no hope whatsoever?
Michael P.	Arizona	Three of my four children died from NPC at a time when there were no drugs available to slow down or stop the disease process. This is a devastating disease! As co-founder of the Ara Parseghian Medical Research Foundation, I have had the privilege of seeing the impact arimoclomol can have on the quality of life for NPC patients. This drug is allowing patients to have fuller lives. It would be a travesty for patients not to have continued access to arimoclomol!
Wendy P.	Michigan	To live my life.
Taylor S.	Massachusetts	Watching someone you love slip away is a helpless feeling. There is immense hope in just having an option to explore.
Meredith P.	Massachusetts	We are very excited at the possibility of adding arimoclomol to Reagan's medication. With a disease as variable as NPC is it great to have multiple options. Arimoclomol is a great option given its clinical results and the fact that it doesn't have any side effects.

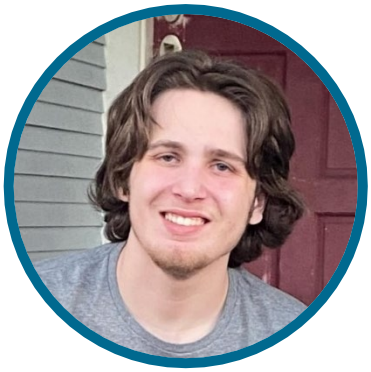
Carolyn P.	Oregon	We don't have the experience to know if arimoclomol will help my step sisters daughter (arimoclomol works by folding the NP protein, of which she barely has any), but it might help other NPC patients, and FDA approval might get other drugs currently stuck in limbo, like adrabetadex, approved. We implore the FDA to be flexible with these drug trials as having some receive only the placebo with a disease as rare and complicated as NPC is additionally devastating.
Kimberly C.	Pennsylvania	We need combined therapies. I do not have access to arimoclomol because my children are on another medication under review.
Theresa C.	Pennsylvania	We want my friend's son to live a very long and happy life
Pam & Chris A.	Texas	We would like to add Arimoclomol to the arsenal of therapies that we are currently taking.
Sarah M.	Texas	With there being no cure and no medication research for this horrible disease, why would you not offer this option to those desperate for some type of relief and/or hope?
Margaret O.	Kentucky	My son Mark Kulp II was diagnosed with NPC when he was 4.5 months old and passed away at 4.5 years old . It was so devastating to watch him go down like he did. Knowing there was nothing to cure him and I felt like a failure. But the first word he said was mama and the last word he said was mama. He was never able to walk alone, was able to hold on and walk around my coffee table which was a blessing to see since I was told he would never sit, stand, nor walk.
Cindy P.	Arizona	NPC is a devastating disease that stole the lives of my three children. Arimoclomol is a safe drug and has been shown to slow down the disease progression for patients who have been on the drug for an extended time. The primary endpoint assessing benefit on the 5-domain NPC Clinical Severity Scale (NPCCSS) was met, forming the basis of a New Drug Application (NDA) submitted. NPC is a horrific disease and deserves novel, careful review by the FDA in its drug approval process.

Before my daughter who is now 22, started taking Arimoclomol she was beginning to have a weakening in her swallowing muscles. We had to have her use a sippie cup that limited how much she could drink at a time. As a young adult this made her feel as though she was being treated like a baby. She began taking Arimoclomol 3 years ago, as a result of her taking this drug her swallowing and choking on food and liquids has ceased. The longer we can keep her from needing a feeding tube the better for her.

Jennifer G, VA

NPC is a devastating disease that stole the lives of my three children. Arimoclomol is a safe drug and has shown to slow down the disease progression for patients who have been on the drug for an extended time. NPC is a horrific disease and deserves novel, careful review by the FDA in its drug approval process.

Cindy P, AZ



APPENDIX C:

Community Comments to FDA

If you are an NPC Researcher or Clinician, please describe why having arimoclomol as an approved therapy would be important to those living with NPC

Rudolf B.	California	Arimoclomol appears to have some genuine benefit in slowing the disease progression. More importantly perhaps, this med is all that NPC families have and they need something to help them battle on. HOPE is everything for the NPC families facing a progressive, debilitating, powerful disease. Lastly, the more this med is used, the sooner we will learn how best to use it!
Samia P.		Arimoclomol could contribute to stabilize the curse of the disease in some patients.
Jirair B.	Pennsylvania	Arimoclomol in combination with miglustat has stabilized the progression of disease in my patients with NPC. Arimoclomol is a critical IP for patients with NPC that the FDA needs to seriously consider as an additional or combination therapeutics for NPC.
Raymond W.	California	As a clinical trial principal investigator, I have witnessed firsthand the benefits study patients and families have experienced while taking arimoclomol. Subjects who were unable to sit upright due to truncal hypotonia, were ataxic, or overtly encephalopathic, were able to sit upright, improve their ambulatory abilities, and be awake and alert enough to attend school and pay attention to tasks and schooling. We've noted deterioration when drug was stopped, and regained abilities when restarting
Justin H.	New York	As an advocate, I have spoken with many families in the community about their experience with arimoclomol. This drug is extremely well tolerated and safe. The lived experience of many patients has been that NP-C symptoms including swallowing and ataxia have stabilized and even improved in many. This community has a very high tolerance for uncertain benefit and risk associated with a therapy given the unmet treatment need and the known severe outcome of neurologic decline and death.
Eugen M.		I have been treating patients with NPC for more than 25 years. Clinical and molecular biology learning have gone hand in hand. In contrast to other lysosomal diseases, the loss of NPC1 or 2 leads to restrictions in several metabolic pathways that probably cannot be treated with one drug. Arimoclomol is an intelligent addition to substrate inhibition or drugs that promote the efflux of cholesterol.
Nathalie G.		Important to reduce / stabilize dramatic neurological progression of the disease. Follow 3 patients with good safety and no neurological progression of the disease with this treatment for 2 years.
Elizabeth B.	Illinois	It is important to have all treatment options that have shown promise based on scientific and clinical data available to patients due to the severe devastating nature of the disease.
Tara M.	Texas	The option to take arimoclomol provides access to improve abilities due to its ability to halt some of the underlying causes of NPC. When the alternative is faster degeneration of youth, there is no reason why families shouldn't be able to elect an option that will improve quality of life and increase the length of life. This option brings hope as well as help to the NPC community.
Caroline H.	California	This gives an option to prevent or slow down disease progression, and when combined with arimoclomol can lead to a significant improvement in quality of life for patients as well as caregivers.
Karla R.	Texas	We have been collaborating with this research for almost 4 years and it is really incredible how much it has improved the quality of life of the participants and improving some of their lives too for some. It is great to see how much it has helped by decreasing the disease progression at some degree (very variable between participants) with minimal side effects
William G.	New Mexico	We identified over 400 NPC1 protein-coding pathogenic mutations present among six different global populations (Nature Scientific Reports 10:18787, 2020). It is hypothesized and evidence suggests that most of these NPC1 protein-coding pathogenic mutations have residual function that will respond favorably to stimulating activation of chaperones (heat shock proteins) and preventing premature degradation.
Nubia F.	California	When I joined the Early Access Program, I did not know much about arimoclomol, but it did not take long for me to realize how important it was for the NPC community. When talking to NPC patients and caregivers, I only heard positive things about it. This community does not have many options for treatment, so any help is appreciated.

As an advocate, I have spoken with many families in the community about their experience with arimoclomol. This drug is extremely well tolerated and safe. The lived experience of many patients has been that NP-C symptoms including swallowing and ataxia have stabilized and even improved in many. This community has a very high tolerance for uncertain benefit and risk associated with a therapy given the unmet treatment need and the known severe outcome of neurologic decline and death.

Justin Hopkin, MD, New York

Appendix D: Supporters Signatures

Ellen Adamosky, Oregon
Bryanna Adams, Pennsylvania
Julie Aiello, Pennsylvania
Susan Ainsworth, Virginia
Rebeca Akst, California
Aujehl Albert
Sara J. Aldridge, Ohio
Ashley Aleman, Texas
Carmen Alessi, New Jersey
Ashley Alford, Kentucky
Taylor Alford, Kentucky
Jenny Allem, Florida
Julie Hargette Allison, Virginia
Ted Aloupis, Massachusetts
Angela Alvarez, Texas
Margarita Alvarez Tabio, Florida
Garland Alvey, Virginia
Lisa Amidan, Utah
Joshua Amos, West Virginia
Kristina Anderson, Alaska
Sheena Anderson, Utah
Kathy Andre, Oregon
Beverly J. Andres, South Carolina
Brooke Andres
Carolyn Andres, California
Jeffrey M. Andres
Paul A. Andres, South Carolina
Belle & Abby Andrews, Texas
Pam & Chris Andrews, Texas
Vasudeva Angalakuditi, New Jersey
Angie Williams, Utah
Christopher Antolino, North Carolina
Kristen Antolino, North Carolina
Angele Aphaymany, Washington
Alexis Archuleta, Utah
Jacquelyn P. Arflin, South Carolina
Kalah Arsenault, Massachusetts
Todd Ashburn, Tennessee
Anne B. Atkins, North Carolina
Jenny Augerlavoie, Utah
Crystal Bailey, New York
Bryn Baisley
Dawn Baker, South Carolina
James E. Balas
Cindy Baldwin, Oregon
Justin Barabicho, Illinois
Kelli Barbera, Pennsylvania
William E. Barbour, Maine
Alice Bare, Virginia
Rani Barinepalle, New Jersey
Mindy Barnes, Utah
Ashley Barreras, Utah
Jen Barton, Minnesota
Shannon Bass
Anne E. Battles, Oregon
Brad Battles, Oregon
Gary D. Battles, Oregon
Michelle Battles, Oregon
Hollie Baum, Utah
Mandy M. Baxter, Colorado
Christina Bazzone
Lisa Beck, Oregon
Jirair K. Bedoyan, MD, PhD, FACMG, Pennsylvania
Jill Beirl, Wisconsin
Mitzi Belverstone, Utah
Ann Bennett, New Hampshire
Claire Bennett, South Carolina
Dianne Bennett, Florida
Raymond Bennett, Florida
Lucas Bentancur, California
Kasey Beresford, Pennsylvania
Kaylee Beresford, Pennsylvania
Scott B. Beresford, Pennsylvania
Tyler D. Beresford, Pennsylvania
Christine E. Berns, Minnesota
Douglas Berns, Minnesota
Meaghan Berns, Minnesota
Rachael Berns, Minnesota
Samantha J. Berns, Minnesota
Vicki Berns, Washington
Elizabeth Berry-Kravis, Illinois
Michelle Bilsky, Pennsylvania
Joseph Biotti, Massachusetts
Elsa Nazario Burgos, Florida
Natalie Black, California
Alicia Bloise, Pennsylvania
AnnMarie Bocook, Texas
Patricia Bodnar, Connecticut
Alena Bogucki, Indiana
Parna Bolel, Illinois
Barbara L. Bolsinger, Iowa
Geraldine Bonanno, Massachusetts
Mary Bosnack, New York
Toni Bowen, Texas
Amber Bowers, Pennsylvania
Suzy Bowman, Pennsylvania
Erin Boyles, Pennsylvania
Rianna Braden, California
Amy Bradshaw, Alabama
Jessica Brame, Texas
Amanda Brandaris,
Jackie Branyam, Virginia
Kimberly A. Brashear, Ohio
Maggie Brengel, Virginia
Debby Brody, South Carolina
Margaret Brogger, Texas
Tanya Brogger, Texas
Tory Brotherton, California
Taraneh Broumand, Florida
Gayemarie Brown, Massachusetts
Kimberly Brown, Texas
Erin Bruce
Blake Brutocao, California
Chasen Brutocao, California
Nellie Brutocao, California
Nic Brutocao, California
Phillip Brutocao, California
Sara Brutocao, California
Tatiana Brutocao, California
Caitlin Brutoco
Krishna Brutoco, Minnesota
Paul Brutoco, California
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Shawn Jordan
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Tonya Kain, Florida
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Wayne Palmiter, Oregon
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Lori Parent, Massachusetts
Esther Park, Virginia
Olivia Park
Amy Tollerson Parker, Oregon
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Michael Parseghian, Arizona
Keerti Parthasarathy, Oregon
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Wendy Peebles, Michigan
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Milena Pereira, Florida
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Sylvia Perschall, Florida
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Sara Peterka, Minnesota
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Michael Purrini, Massachusetts
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Dawn Stites, Florida
Whitney Stites, Florida
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Kayla Thurston, Connecticut
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Jamie Tilbury, California
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Ashley Towler, Utah
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Susan Waldrop, Pennsylvania
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Kristina Walsh
Angela Walters, Utah
Cassidy Walters, Utah
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Elaine Watkins, Ohio
Melissa Watson, Utah
Pam Watson
Callie Watterson
Debbie Watterson, Utah

Doug Webb, Utah
Kailie Wehrer, Pennsylvania
C. E. Weiland, Oregon
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Susan Weinert, Montana
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Robert Wellrr, Ohio
Erin Lee Wells
Amy Wernicki, Connecticut
Amber West, Utah
Stuart West, Virginia
Amie White, DPT OCS, South Carolina
Angela White, Kentucky
Lauren White, Texas
Marci Wicks, Utah
Justin Widener, Georgia
Dianna Williams, Ohio
Jaime Williams, South Carolina
Jared Williams, Texas
Ryan Williams, Pennsylvania
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Tracy Williamson, Virginia
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Catherine Willinger, Massachusetts
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Elizabeth Wilson, South Carolina
Jason Wilson, Alabama
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Kris Wilson, Utah
Mandy M. Wilson, Mississippi
Raymond Wilson, Mississippi
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Candy Wooten, South Carolina
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Ciara Young, Florida
Janice Young, Texas
Kevin Young, Pennsylvania
Michael Yun, Virginia
Nicole Zacarelli, Connecticut
Tara Zawodni, Pennsylvania
Gale Zilko, Pennsylvania
Daniel Zornes, Washington
Sue Zornes, Washington

My 24-year-old son, Justin, has been taking arimoclomol for the past three years. It is not only important it is critical that he continues to have access to arimoclomol. Caring for a child with a life-altering condition, particularly one without a known cure, what becomes increasingly important is ensuring that your child is safe, well cared for, and has optimal quality of life. Due to the deficits NPC has left Justin with access to this drug is essential for dramatically slowing progression.

Wallisa M, NC