ALDConnect

Committed to Connections

ANNUAL REPORT 2021

MISSION

We aim to improve health outcomes for patients with adrenoleukodystrophy (ALD) by empowering patients, raising awareness, and accelerating the translation of scientific advances into better clinical care.

VISION

We strive to revolutionize care, end suffering from ALD, and find a cure.

Dear ALD community,

On behalf of the Board of Directors, we are honored to present ALD Connect's first ever impact report. We have accomplished so much since our organization was founded in 2013. We have been successful in filling the gaps and connecting stakeholders to empower patients, facilitate early diagnosis, and develop better treatments. We are proud of what we've been able to achieve together, and we are excited to highlight some of our transformative work from fiscal year 2021, which was January 1st to December 31st.

In 2021, we focused on the specific needs of various phenotypes: newborn screening and monitoring phase families, boys with cerebral adrenoleukodystrophy (ALD), men with adrenomyeloneuropathy (AMN), and symptomatic women with ALD.

We continued our monthly Community Calls and held Bootcamps for each of these patient populations. We also launched our ALD Landscape Project, which has provided us with valuable information regarding where our patients are located, how they are being diagnosed, and who is involved in their care.

Our community continues to rise to the challenge and care for each other despite the uncertainty of the world in which we live. In June of 2021, we hosted an FDA Listening Session, which was our first official engagement with the Agency. Seven patients and one caregiver bravely shared their stories and experiences. Our donors enabled us to provide financial support to several ALD families through The Myelin Project Patient and Family Support Program and the Michael Benton Travel Scholarship Program. We also matched several patients and caregivers with mentors in the ALD community through our Peer Mentor Program.

In 2022, we plan to host an Externally-Led Patient-Focused Drug Development meeting, focusing on adult ALD and AMN patients. This is an opportunity to educate FDA staff about ALD and amplify patient perspectives, which are critical to help provide context when FDA makes regulatory decisions for new drugs. Our goal is to publish a report that documents the severe disease burden and unmet medical need of ALD and AMN in patients' own voices. Our hope is that it will influence every stage of drug development.

Thank you for your commitment, dedication, and perseverance. We can't wait to see what we can accomplish for ALD families in brighter days ahead.

With gratitude,

Kathleen O'Sullivan-Fortin
Co-Founder, Treasurer

Florian Eichler, MD
Co-Founder, President

Sharing our Stories

FRANI'S STORY:
NAVIGATING THE UNKNOWN

Then and now. Before and after. Pre-diagnosis, post-diagnosis. My mind's automatic cataloging system for memories. Everything before the summer of 2018, and everything after the ground seemingly fell out from under me.

As many of you know, six people in my family, including me, were diagnosed with ALD in the summer of 2018, all within a few weeks of each other. A mere three years ago but a lifetime lived in them. Nearly three years with an answer following a lifetime of questions. With this diagnosis, our family finally had a name, a disease to put an end to the vague guesses, the half-attempted explanations, and a nearly thirty-year diagnostic odyssey. It was a defining marker that had been defining our lives unwittingly.

Shortly after we were diagnosed, a fuzzy "before" memory resurfaced. In it, I'm about the age of twelve, not sure why I'm not at school, but rather, am in an elevator with both of my parents, having just left a doctor's appointment for my mom. I don't remember the doctor. I don't remember the office. I only remember standing in the back of the elevator with my parents in front of me. My mom and dad are hugging, and my mom is crying. No, my mom is sobbing. I can't remember the sound. I can't remember her face. But I remember her sobbing, and somehow, I knew that her sobs were those of relief. Relief for a negative multiple sclerosis diagnosis.

Relief, short-lived, a catch-22. Because if not MS, then what? What was the cause of these mysterious symptoms that found her — at about the age of 38 — losing her balance and having numb feet, unable to walk across a baseball field to watch her sons play, unable to keep up in her exercise class? Symptoms that over the years required the assistance of a cane, then a walker, now sometimes a scooter. There were seemingly endless appointments, and her case baffled doctors. At some point, hereditary spastic paraplegia was suggested. But nothing definitive. Then in the summer of 2018, her odyssey abruptly came to an end. At a cocktail party in Arizona.

It was there, at the grand opening party for my youngest brother's new business, that my mother met another guest who happened to be a VERY knowledgeable doctor. Noticing my mother's walker and gait, the doctor asked her if she had adrenoleukodystrophy. Of course, her reply was no, but her interest was piqued. Upon returning to Texas from her trip, my mom requested "the ALD test" (for both herself and my middle brother Patrick, who was exhibiting similar, but more aggressive symptoms) from her neurologist. He ordered the tests, and low and behold, the diagnosis was adrenoleukodystrophy.

"ALD always will be a defining part of their lives — all of our lives. But as we are slowly learning, the fear of ALD doesn't have to define us, but rather, the brief moments of joy found in spite of it."

FRANI BROUSSARD

To say the following weeks were a blur is an understatement. With negligible support or counseling from my mom's neurologist or his office, we navigated the scary waters of Google for any information we could find. Within a few clicks, it was clear that what little information we had was wrong, and what we did find was clearly conflicting (i.e. women are only carriers or have mild symptoms). I finally stumbled upon a website with a very handy chart showing the inheritance pattern for ALD. And fear set in.

I often wonder what memories my young children will have from that summer — the "after" memories that are seared crystal clear in my mind. Will they remember the long car ride to the specialty testing lab, with me frantically reciting the Rosary and fighting back tears? Will my daughter remember comforting me as I laid sobbing on the living room floor, minutes after receiving Oliver's diagnosis? Will they remember our trips to the beach, where I sat inside the house the entire time, researching ALD on my laptop? I suppose, to sum it up, I wonder if they will know that fear replaced my joy for nearly an entire year of our lives.

And maybe they will. But maybe they also will remember the moments when joy crept back in. Maybe they'll remember playing with the children of one of our ALD specialists at an outdoor restaurant in sunny California? Or apple picking in Massachusetts after appointments with another ALD specialist? Maybe they'll remember my excitement on the day we received our 501(c)3 status for Arrivederci ALD? And pony rides and opera singing with all of their friends and family at our first fundraiser?

And though many of the memories from that time are mine exclusively, I will make certain they know of them. Memories of doctors and nurse practitioners responding within minutes to my frantic emails with words like "enjoy your son this weekend" and "ALD doesn't have to define your life". Stories of physician scientists excitedly sharing research findings using the funds we raised. Memories of shared meals, drinks, and tears with fellow parents at conferences. The story of a childhood friend calling to propose a partnership between his newly-founded business and our newly-founded charity to "round up" to the nearest dollar every transaction at every one of his retail stores for ALD research funds.

And maybe one day, the memories will no longer be bifurcated. No emotionally-charged before, then, after, post. Simply a memory. Because ALD always will be a defining part of their lives, all of our lives. But as we are slowly learning, the FEAR of ALD doesn't have to define us, but rather, the brief moments of joy found in spite of it.

REX'S STORY:
LEAVING A LEGACY

I hope by telling the story of the love of my life, Rex Akins, and our journey with Adrenoleukodystrophy ending with his death due to Cerebral ALD, that the ALD Connect community can use our grief to ignite a passion for research that leads to a cure. Tragic stories like ours have been written too many times, and our daily prayer is that in our lifetime, a new ending is written where ALD becomes extinct and those affected by ALD will live out their destiny without these losses.

Rex and I met 52 years ago when our parents lived across the street from one another. I was 5 and he was 10. We grew up together, attending the same church, and I have very few memories without him.

Rex was very athletic and enjoyed water skiing, snow skiing, all sports for every season. He enjoyed weightlifting and worked out every day. He blueprinted rebuilt engines, restored cars, and loved driving sports cars. He had a beautiful tenor voice, sang in Barbershop quartets, and led singing at church. He was a computer programmer with a BS in Computer Science and a Master's in Education, but his passion for aviation caused him to join Army to become a Cobra and Apache helicopter pilot and, later, a maintenance and instructor pilot.

At the age of 37, Rex noticed his feet would become numb when running for physical fitness tests, which made running difficult. He also noticed occasional stress incontinence during daily activities. He saw his PCP and urologist who could not find anything physically wrong, so they told Rex he was getting older and that things don't always work the same as you age.

At the age of 40, while deployed, Rex developed a foot drop causing the side of his boot to be worn so badly that a physician noticed and did a thorough neurological exam. An MRI of his brain and spine revealed a bone spur growing on his spinal cord. That became the "red herring" that

explained all the neurological deficits. He had decompression surgery with a slight improvement in symptoms, and a Military Medical Review Board declared he was able to remain on active duty.

At age 45, Rex grounded himself because of his deteriorating health, feeling as though he wouldn't be able help crew members in an emergency. He remained in the Army for the next three years in a non-flying role and retired from the service at age 48.

At age 51, he began to use a cane due to frequent falls. He walked our middle daughter down the aisle with a cane. He began using a scooter for long distance walking, such as for our son's graduation from Air Force training.

By age 60, Rex had lost bladder function and was using a scooter and eventually a wheelchair 100% of the time. Our oldest daughter was married this same year, and he used a scooter to walk her down the aisle. He worked so hard to be able to walk her part of the way down but could not. This picture was taken right after the lighthearted moment Alyssa's dress was caught in the wheel of his scooter and an 80-year-old Alabama farmer quickly wrangled it out so they could continue down the aisle.

At this point, his neurologist could no longer explain the fast decline and increased symptoms. He did more tests that were negative, then referred us to Mayo Clinic so genetic testing could be done. The Hereditary Spastic Paraplegia panel was ordered but the ABCD1 gene defect was NOT identified.

March 2019, due to fatigue and physical limitations, he could no longer work and retired again.

Rex's mother passed away June 2019 at the age of 87. She had dementia, possible strokes, and was in a memory unit unable to walk or move independently for almost 5 years. She was never tested for ALD, but looking back, her symptoms supported that diagnosis.

More serious health issues followed, and in November 2019, tests showed Rex did in fact have ALD, despite the earlier negative test results. With no doctors in our area familiar with ALD, we soon found Dr. Eichler in Boston, and in the midst of COVID, we saw him through telehealth visits.

July 2020, at the age of 62, an MRI found brain lesions consistent with Cerebral ALD. We tried several treatments to slow the progress of the brain inflammation to no avail. By October, he could no longer work a TV remote, turn on his computer, use his cell phone or be safely left alone for more than a few minutes. December 13, 2020, Rex spoke his last words to me, which were "I love you." January 23, 2021, he died at home under the care of hospice. He lost the ability to fight infections, to move, to swallow, and he did not wish to have a feeding tube placed.

One thing that ALD could not dampen was Rex's spirit of determination. He fought so hard to overcome the many obstacles, but there were many losses our entire family endured as his quality of life diminished. Our kids were not sad because their dad couldn't take them to Disney World. They were sad because they watched their dad suffer with pain and muscle spasticity. They were sad because they watched Rex lose the ability to do the things that he loved doing. They were sad to lose their father who loved them so much. Our kids were his biggest cheerleaders.

Continued on next page

"One thing that ALD could not dampen was Rex's spirit of determination."

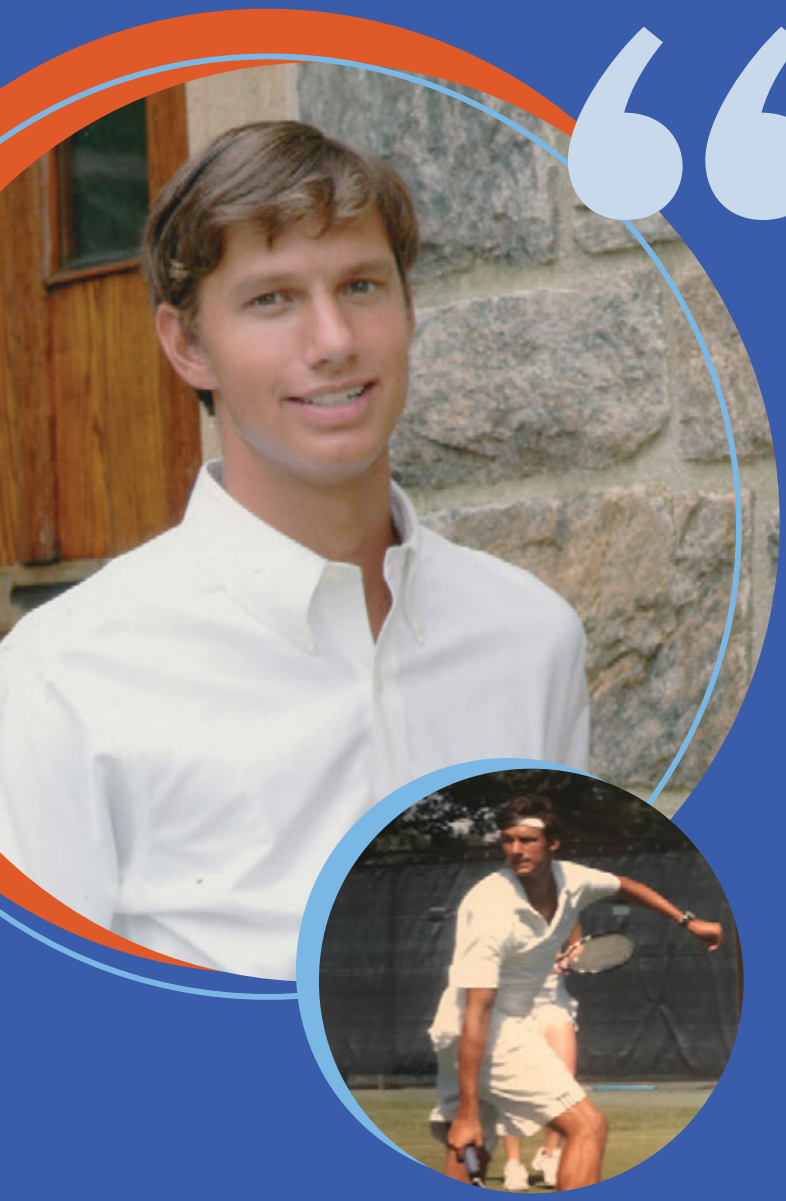
CONNIE AKINS

REX'S STORY *Continued*

No longer able to walk, Rex was enduring immense nerve pain, unable to regulate temperature (cold or heat), suffering from bladder infections (to the point of sepsis), spending hours of each day on a bowel program, losing his cognitive abilities, and having to depend on me to help him with every aspect of daily activities. Every ability he lost meant we would have to fill in the gap and do those things for him. He hated that the most. He loved us well and we were grateful to be able to care for him, but there were some really hard days.

Our middle daughter found out she was pregnant with a boy shortly after Rex was diagnosed. It was a scary time knowing he could inherit ALD and also worrying how ALD could affect her someday.

Those with ALD desperately need treatments to improve their quality of life, and we must find a way to keep this condition from becoming CALD and ending lives too soon. Our grandson was born September 29, three months before Rex passed away. Our family agrees that our grandson won the genetic lottery; he has my perfect, non-mutated ABCD1 gene. He had a 50/50 chance. We thank God for this joyous news in the midst of our sorrow and for the many people who blessed us on this journey.



Through Neddy, we learned what it means to be a truly loyal friend, what it takes to roll with life's punches with grace and without self-pity, and what it looks like to fight like hell even in the hardest circumstances."

JOAN CHASE

NED'S STORY:
A RESILIENT MIND

Neddy graduated from Bronxville HS and Princeton '10 (proud Tower Club member). A first-rate mind, he obtained a patent soon after. As a youth, he bravely and successfully fought cerebral palsy becoming captain of his high school tennis team.

At age 34, on January 1, 2021 he finally succumbed to cerebral adrenoleukodystrophy, a devastating genetic disorder, with his loving family, Joan, Ned and Lila at home in Bronxville, NY.

Through Neddy, we learned what it means to be a truly loyal friend, what it takes to roll with life's punches with grace and without self-pity, and what it looks like to fight like hell even in the hardest circumstances. Neddy lit up every room he entered and made all of us laugh. His family and vast network of friends will miss him terribly, especially his wicked sense of humor.

> **I was walking in a dark place, all alone, trying to do what was best for my son."**
>
> SYDNE PANTALEON

PEYTON'S STORY:
FINDING A CALLING

My name is Sydne, and in 2017 my beautiful boy Peyton was diagnosed with Adrenoleukodystrophy at the age of 2 and half years old. Unfortunately, at that time, our state of Illinois was not a newborn screening (NBS) state yet, but thankfully someone from a side of our family whom we've never had close contact with overheard that I had a son recently and reached out to let me know the importance of getting him tested. I was a young, single mom at the time, navigating motherhood, and when this came about, my world was in shambles. Because we were not a NBS state, there were no protocols set in place for this type of rare disease. I struggled for weeks to find someone to test him. When we went to our pediatrician she said because he was meeting all milestones, on track with vaccinations and meeting all the growth requirements, there was no reason to test him.

Finally, I found a specialist out of state who ordered us a VLCFA, and a week later we got the call. I remember that call like it was yesterday, scariest moment of my life. I was given no direction, until I started connecting with other families on social media. We were then referred to University of Chicago Medicine, where I basically had to tell them what kind of team I was going to need to monitor my son. I was still struggling, especially, when I decided to get my son on a 504 plan at school. There was lack of communication between his school and his teacher, his pediatrician (who had never heard of ALD), and his care team at UIC. I was walking in a dark place, all alone, trying to do what was best for my son (because of course I did the worst thing possible and spent countless hours on google!).

After a few months of trial and error, I decided to move our care over to Lurie's Children's Hospital of Chicago where they have a leukodystrophy clinic. I had finally felt that it was safe to take a breath. We have had a wonderful experience here, and it wasn't until this move that I was offered a Social Worker. I remember I was sitting in traffic when she reached out for the first time over the phone. She introduced herself and listed all the ways she was going to assist us in getting the best care for Peyton. Calling insurance, talking to the school, monitoring his 504 plan, helping him talk through his MRI and blood draw anxieties, and checking in with our family to make sure our mental health was strong while we are on this journey. She was the biggest blessing for us.

After two universities, a degree in sociology, and a beautiful son in-between in addition to our strong bond with our social worker, I decided that my calling was to be that person for other families like ours. In the summer of 2021, I was accepted into the Masters of Social Work program at Aurora University here in Illinois. My goal is to be a school social worker and inspire students just like Peyton to love who they are, be proud of who they are, and be successful no matter the circumstances. If it wasn't for our ALD journey, I don't think I would have found my calling. Although I wish no one had to endure this awful disease, I am grateful every day for the people that we have and will continue to meet, and for the life lessons it has taught me along the way. I look forward to being just a little ray of light in students' lives, just like our social worker has been in ours.

TIM'S STORY:
FIGHTING FOR A CURE

My name is Tim Mathwig. I am 44 years old and currently live in Dallas, TX. I have been married for 17 years and I have a son and two stepchildren — who are all adults now. My wife and I made the decision almost immediately not to have any more children together as we didn't want to pass this disease on to a daughter. I work for a construction company and am the Director of Purchasing and National Accounts. When I started with my current company, I was the General Manager of our Dallas branch, but I couldn't continue with the physical demands of that job. My current role is mostly a desk job.

Growing up, I loved the outdoors and playing sports like baseball and basketball. I enjoyed fishing and hunting of all kinds. I rode my bike and skateboard everywhere. When I was in my early 20's I fell in love with playing golf and actually got pretty good at it. I've had to give up almost all of these now, but I still have a deer stand I can drive to and climb in occasionally.

I was diagnosed with AMN and Addison's Disease when I was 18 years old. My whole family was tested after my cousin was diagnosed after being hospitalized when he got hurt playing football. For almost a decade following my diagnosis, I remained healthy and symptom-free. I did start taking hydrocortisone right away for the Addison's. I started experiencing symptoms around age 30, such as problems with my gait, ED and bladder issues. Even though I had been diagnosed with AMN early in my life, my doctors didn't immediately connect these symptoms to AMN. There just wasn't enough information out there. This was before the internet and social media. I visited several doctors and had many appointments, before eventually realizing they were all part of the progression of AMN. This was frustrating as I was the only person my doctors had ever seen with AMN.

As the disease progressed into my late 30's, I needed a cane to walk. At first it was only in the morning and evening or long distances and I was able to hide it mostly from friends and coworkers. My wife bought me my first scooter around the same time to use around the house and backyard. By the time I was 40, I needed the cane full time. Then I had to start explaining to everyone the condition I had. This was extremely difficult for me emotionally. My wife had to do most of the talking and explaining to others as I couldn't do it without getting choked up. Eventually, with enough practice I got comfortable explaining it without getting emotional. The first questions I would always get were, "What's the prognosis or treatment? And is there a cure?" To which my answer was always, "not at this time but hopefully soon."

In the last 4 years, the distance I can walk has drastically diminished. I now have a wheelchair to take to places that require more than 50-100 steps. My balance is terrible and while I look like I'm going to fall a lot, I'm still capable of catching myself with the wall or a piece of furniture or my cane most of the time. I can't pick my feet up as I walk so, I trip over almost nothing or just from my toes hitting the ground before I complete my stride. The neuropathy pain is the worst part for me. I describe it to others that it feels like I'm standing in hot lava that's burning my feet up to my knees. When I walk or trip, my stumbling causes my legs to jolt my body, causing lower back and neck pain. I sit down more and more these days, and I'm sure that's not helping my back pain either. The neuropathy pain along with muscle spasms or spasticity and myoclonic foot jerks cause me to have a hard time falling and staying asleep. It's also very difficult to stand for any amount of time before the pain becomes unbearable.

In the last four years, I began using intermittent catheters. I'll use them before I go to bed or if I'm going to be sitting for long periods of time like going to a movie or getting on a plane. Not knowing where or how far the bathroom is causes a lot of anxiety for me.

I have tried so many different medications over the years and have also done physical therapy to help with my gait and posture. I've tried or currently take Gabapentin, Baclofen, Keppra, Lyrica, Nortriptyline, CBD oil, Cymbalta, Ampyra, Duloxetine, Lorazepam, Hydrocortisone, Prednisone, Biotin, Magnesium, Modafinil, Ambien, Flomax, Cialis, Viagra, Alprostadil. Many of these come

I am at the point that I will do almost anything for science and the drug manufacturers so that maybe someday, no one will have to endure what we've had to living with AMN."

TIM MATHWIG

with side effects — some serious. I want to make it clear — when you already have a poor quality of life (like we do), you're willing to accept more side effects if it also provides any relief for your symptoms.

Two and a half years ago, I participated in the first ever clinical trial called MIN-102. I live in Dallas, TX and had to travel to Boston 12 times in two years. This was very difficult for my wife and I both physically and logistically. The flight is four hours but I would do almost anything for the possibility of slowing or halting my progression — as was the hope with this trial. However, I ended up being on the placebo. I am now in the open label extension which requires another six trips over the next two years, or until the drug is approved by the FDA.

Living with AMN requires a lot of help from others. I have to carry everything with one hand as the other is for my cane. I can't lift anything heavy as my balance is so bad I would topple over. My fatigue and neuropathy pain prevent me from doing so much, and I also need to take frequent breaks just to rest. This puts an enormous responsibility on my wife, family, and friends. I can't even stay with friends or family if their guest room is on the second floor. Whenever we're traveling, my wife calls ahead to find out if there are any stairs, if they have a tub or walk-in shower, how far the room is from the parking lot, from the elevator, etc. etc. If we're staying in a hotel, I always tell my wife if there's a fire tonight, do not worry about me — just save yourself, because if we have been sitting or sleeping for a long period of time, it takes a while to get these legs ready to take the first steps.

I have hoped and prayed for a cure for this disease in my lifetime. I am at the point where I will do almost anything for science and the drug manufacturers so that maybe someday, no one will have to endure what we've had to living with AMN. In the meantime, however, I will be extremely grateful for any advancements to treat or improve any of my symptoms.

Raising Funds – and Spirits

DR. B RUNS FOR ALD

In June 2021, Dr. Josh Bonkowsky ran the Western States 100-mile endurance race in California's Sierra Nevada Mountain trails and raised $10,000 for ALD Connect.

Running was something that spoke deeply to him, and paralleled aspects of what he wanted to do in his own life. He feels that, like running, effort, persistence and chasing a goal are worthwhile. Treating and researching adrenoleukodystrophy, and taking care of patients and families with leukodystrophies, are hard but important.

In every 100-mile race there is a time in the middle of the run, usually in the middle of the night, where things seem pretty grim. There is still a long, long way to keep running — like 2 marathons left! You have already been out there for 10 or 12 or 18 hours, with at least that long to keep going. It seems like a deep hole. But then — around mile 50 your friends (your "pacers") are allowed to show up and run with you. And incredibly, if you keep going, you can reach the finish.

DONORS

In 2021, we received donations from hundreds of individuals across many platforms. We truly appreciate every donation.

ALD Connect Major Donors

We are grateful for the donors who continue to support our mission with major gifts to help us support operations each year.

FDA LISTENING SESSION FOR AMN PATIENTS

ALD Connect hosted an FDA Listening Session for AMN on Friday, May 7, 2021.

FDA Listening Sessions are an important opportunity to educate FDA review staff about AMN. ALD Connect Board Member, Dr. Florian Eichler gave opening remarks. We then had seven patients and one caregiver provide testimony about disease burden, symptom progression, and what meaningful function is important to preserve. It is our hope that the patient perspectives will be considered when the FDA is deciding which clinical outcomes are relevant. We hope that this will help improve the drug development process for our industry partners who are working on AMN therapy and treatments.

We are grateful to the patients and caregiver who bravely and vulnerably shared their stories for the benefit of the broader ALD and AMN community.

> **'If you want to run far, run together.' I think we are all in this together — let's help get ourselves to the finish line!"**
>
> DR. JOSH BONKOWSKY

BLUEBIRD BIO GRANT: ALD LANDSCAPE

In 2021, we received a grant from bluebird bio to bolster our patient engagement program. We hosted two Bootcamps and launched our Landscape project. Each Bootcamp consisted of four presentations and one open Zoom session for community building. Our Landscape project is focused on reaching ALD families and compiling a comprehensive list of patient demographics, leukodystrophy centers, and physicians in the ALD community. It is essential that we understand where patients are being diagnosed and who is taking care of them to ensure that all ALD patients and families receive quality care. We are grateful for the support to help obtain patient perspectives and create better resources to fill the gaps in our ALD community.

bluebirdbio®

GLOBAL GENES GRANT

We received a Financial Advocacy RARE Patient Impact Grant to promote financial health and positive financial decisions for ALD families. Our webinar, "Navigating the Financial Aspects of ALD" was held on Saturday, November 13th at 2:00 PM EST during our 2021 Annual Meeting and Patient Learning Academy.

Our expert panelists included Debbie Jacobson, the Senior Manager of the Patient Support Center at Be the Match and Emily Hardy, a Leukodystrophy Care Coordinator. In addition, we had two mothers of boys with cerebral ALD, Kirsten Finn and Jillian Smith, share their experience. During the webinar, Kirsten and Jillian shared invaluable advice on how to advocate for yourself and your family to your insurance company, along with numerous tips that could only come from personal experience. Debbie and Emily explained the necessity of knowing your health insurance and the benefits entitled to you and how to find resources to help families who are struggling with financial burdens because of ALD.

The recording from this webinar is available on our website to support families navigating financial burdens due to ALD in the months and years to come.

RARE FOUNDATION ALLIANCE MEMBER GLOBALGENES.ORG

THE MYELIN PROJECT PATIENT AND FAMILY SUPPORT PROGRAM

ALD Connect is proud to be able to offer grants to ALD patients and families who need financial assistance. Our program is intended to help offset cost of living expenses, including mortgage/rent, car payments, and utility bills.

Our Michael Benton Travel Scholarship program enables us to assist families with travel expenses for medical appointments. We are grateful for the donors who support this fund.

In 2021, we funded several grant applications:

FEBRUARY 2021

Rent and utility bill for a family in Wisconsin.

MARCH 2021

We made a mortgage payment for a patient in Maine.

APRIL 2021

Assistive technology for an advanced cerebral ALD patient in New York.

JUNE 2021

Airbnb and airfare for a patient in Mexico.

JUNE 2021

Airfare for siblings from North Carolina to travel to be with family during transplant.

OCTOBER 2021

Airfare for family in Alabama to travel to Minnesota for appointments.

Support Through Mentoring

NEW PEER MENTORS

We are excited to introduce two new Peer Mentors: Tim Mathwig and Brad Gillepsie.

Tim Mathwig was diagnosed with AMN and Addison's Disease when he was 18 years old. He started experiencing AMN symptoms around age 30. He wants to advocate for the ALD community and do everything he can to be useful and to help others through mentoring and to continue using his own body for research through clinical trials.

Tim Mathwig

Bradley Gillespie was diagnosed with adrenomyeloneuropathy in 1996. He has decided that for many people, especially those freshly diagnosed, the only way to repatriate those comfortable, familiar normal feelings is through a better understanding of what they are experiencing. If he can help just one person normalize their personal AMN voyage, he is going to call that a win. That was his motivation to become an AMN peer mentor.

What is a Peer Mentor?

The purpose of the ALD Connect Peer Mentor Program is to connect with individuals with ALD, family members, or caregivers with an ALD Connect mentor so they can form a relationship that is maintained with encouragement, respect, and confidentiality. The main goal of this program is to provide support, easier access, and increase connections between members of the ALD community. We have more than twenty mentors who hope to help another community member who shares their circumstances and understands their specific challenges.

Bradley Gillespie

> **"ALD Connect has been my go-to resource since my son's diagnosis. I am grateful for the Community Calls as they've given me a sense of solidarity and hope along with current information relevant to the condition we face."**
>
> CHRISTIE HIGUERA

COMMUNITY CALLS

ALD Connect coordinates periodic Zoom calls to facilitate community building and provide support. Whether it's men with AMN, women with ALD, cerebral ALD caregivers, or newborn screening parents, our Community Calls are continuing to bring support and deepen our community's connections with each other. These calls are not recorded.

WEBINARS

ALD Connect hosts live, interactive webinars via Zoom that are free and can be accessed anywhere in the world. Experts from varying fields take questions and discuss a broad range of topics requested by ALD patients and their families. Be sure to subscribe to our newsletter and check out our YouTube channel to browse through past webinars.

JANUARY 20, 2021

ALD Basics and Current Treatments Q&A
Conceptos básicos de ALD y tratamientos actuales
Dr. Patricia Musolino

MARCH 5, 2021

Reproductive Options for ALD Families
Dr. Sara Vaughn
Dr. Troy Lund
Dr. Amber Salzman
Carrie Chou, MS, LCGC

BOOTCAMPS

In 2021, we hosted four phenotype-specific virtual Bootcamps. Each Bootcamp consisted of four webinars with expert presenters and an hour-long community building session.

MAY 1, 2021

Newborn Screening and Non-Cerebral ALD
- Standards of Care, Assessments
- Crucial Conversations
- Mental Health and Impact on Relationships and Families
- Looking Ahead: What comes next?

MAY 22, 2021

Cerebral ALD
- Transplant 101: BMT and Gene Therapy
- The Transplant Journey: A Mother's View
- Life After Leaving the Hospital
- Taking Care of the Caretakers

JUNE 12, 2021

Men with AMN
- Standards of Care
- Cerebral ALD
- Embarrassing Symptoms
- Looking Ahead: Industry Pipeline

AUGUST 7, 2021

Women with ALD
- Overview of Symptoms in Women with ALD
- Symptom Management
- Coping with Your ALD Diagnosis
- Looking Ahead: Future Studies

Community Collaboration

FAMILY WEEKEND: CAMP-AT-HOME

ALD Connect was a proud sponsor of Family Weekend, an opportunity to share good times, peer-support, and education, with others in the ALD community. It was held March 19–21, 2021. Campers participated in three days of online family fun, informative patient and doctor panels, campfire sing-a-longs, dance parties, arts and crafts, and community building.

Thank you to Nic and Alison Adler who co-founded ALD Family Weekend with Dr. Keith Van Haren. The wonderful event was organized by an extraordinary committee and The Painted Turtle.

Expert Insights

2021 ALD CONNECT ANNUAL MEETING AND PATIENT LEARNING ACADEMY

We hosted our 8th Annual Meeting and Patient Learning Academy from November 12–13, 2021. We had more than 350 registrants and many more watched the recordings on our YouTube channel. We were also able to offer Spanish translation during the meeting.

Thank you to our sponsors:

bluebirdbio · minoryx therapeutics · poxel · SwanBio THERAPEUTICS

GROWING OUR ALD COMMUNITY

> **We are so grateful to ALD Connect for the compassion, generosity, and support with which they carry out their mission."**
>
> KIRSTEN AND CRAIG FINN

INDUSTRY ADVISORY COUNCIL

Our Industry Advisory Council (IAC) in comprised of representatives from our ALD Connect Board of Directors and several companies that are working on ALD therapies. The purpose of the IAC is to bring together stakeholders for robust discussions about the disease, clinically meaningful outcomes, treatment approaches, and research.

MARCH 2021

Applying Research to Medical Needs: Neurofilament Light Chain Protein as a Biomarker for X-linked Adrenoleukodystrophy
Isabelle Weinhofer

Voice of the Patient: Proposal for a Patient-Focused Drug Development Meeting for X-ALD
Uwe Meya, MD

June 2021

Targeting the CNS for Small Molecule Delivery
Dr. Ali Fatemi

September 2021

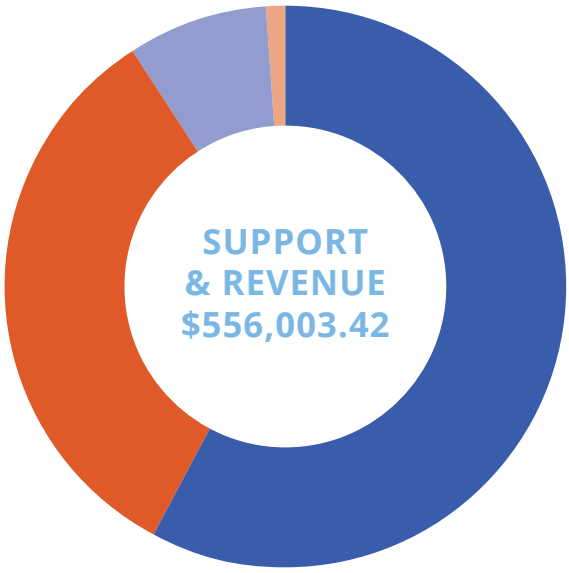
Assessing Cerebral Visual Impairment in ALD
Melissa Bambery and Dr. Lotfi Merabet

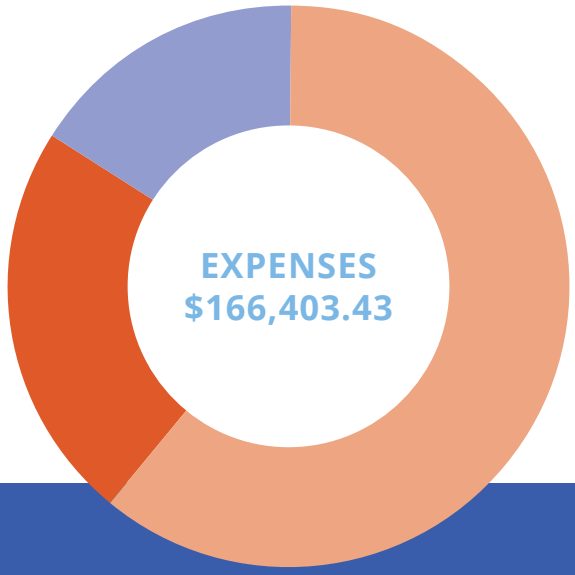
December 2021

Could ALD Connect Act as a Coordinator for Studies and Collective Industry Efforts?
Kathleen O'Sullivan-Fortin

Financials & the Future

January 1, 2021–December 31, 2021





SUPPORT & REVENUE	$556,003.42
Contributions	$324,122.68 (58%)
Securities	$183,617.88 (33%)
Sponsorships	$47,431.00 (8%)
Other	$831.86 (<1%)

EXPENSES	$166,403.43
Management & General	$101,115.69 (61%)
Events & Conferences	$38,500.00 (23%)
Programs	$26,787.74 (16%)

NET INCOME	$389,599.99

“As part of this ALD community, I’ve been able to reach out easily to the most knowledgeable people for help at various stages as we have traveled down the difficult ALD road. And it’s a privilege for me to be on the giving side as well. Thank you, ALD Connect.”

JOAN CHASE

2022 Initiatives

VOICE OF THE PATIENT

We plan to host an Externally-Led Patient-Focused Drug Development meeting on July 22, 2022. Our proposed target patient population is Adrenoleukodystrophy (ALD) in Adulthood: men with ALD (adrenomyeloneuropathy and cerebral ALD) and women with ALD.

We believe an EL-PFDD meeting will allow the FDA and other key stakeholders to obtain a wide range of patient, caregiver, and healthcare provider input on ALD. This includes their perspectives on their condition, its impacts on daily life, the landscape of current treatment options, as well as input into outcomes and patient preferences for future therapies. Given the need for better treatments for ALD, FDA's participation in such a meeting will greatly enhance current knowledge of this serious condition and show a commitment to patient-focused drug development in this unmet need. Our goal is to publish a Voice of the Patient report that summarizes the input provided by patients and patient representatives.

LIVE YOUR BEST LIFE WITH ALD

In 2022, we will focus on helping individuals with ALD live their best life. Our Board of Directors had a thoughtful discussion about how to be realistic but still remain positive and motivated. We want to be inclusive, improve the quality of life for individuals with ALD, and ensure that everyone is accepted the way they are. We know that "living your best life with ALD" will look different for everyone, depending on their phenotype and symptoms. We will also honor the memory of individuals who fought courageously against ALD.

Thank You

to all our partners and members of the ALD community for continuing to support us.

2022 FUNDING

We received two donations in December 2021 that totaled more than $200,000. We are grateful for these major donors, who wish to remain anonymous, for their commitment to our organization and mission.

Minoryx Therapeutics, SwanBio Therapeutics, Autobahn Therapeutics, and Poxel have committed to help us fund our Externally-Led Patient-Focused Drug Development meeting in 2022. We believe this is an important investment that will have a long-lasting impact on our community.

Our programs and projects would not be possible without your continued support.
To make a donation please visit aldconnect.org/donate or scan the QR code.

2021 Board Members

Greg Benton

Josh Bonkowsky, MD, PhD*

Patti Chapman

Florian Eichler, MD*

Ali Fatemi, MD*

Stephan Kemp, PhD

Ben Lenail*

Troy Lund, MSMS, PhD, MD

Timothy Maguire

Kathleen O'Sullivan-Fortin*

Amber Salzman, PhD*

Alex Sherman*

Keith Van Haren, MD*

*Co-Founders

We're proud of our board members' passion and commitment, demonstrated by the high attendance rate in our monthly meetings.

ALDConnect

ALDCONNECT.ORG