

2023 ANNUAL REPORT



Genevie Echols, SADS Family Support Director, at our 2023 EL-PFDD to inform the FDA about ARVC.

Groundbreaking SADS Meetings to Inform FDA

In June of 2023, we held an EL-PFDD (Externally-Led Patient Focused Drug Development) meeting to inform the FDA, researchers, and biopharma companies about the experience of living with ARVC (arrhythmogenic right ventricular cardiomyopathy), one of our SADS conditions.

"We would like to hear what patients look for in an ideal treatment to manage their condition," Chinwe Okoro, MD, FDA said. "This will help inform the focus of new medical products, development, and future clinical trials."

An Incredible Turnout & An Incredible Impact

As a community, we broke a record; according to our consultants, this was one of their most attended meetings. We had almost 100 testimonials submitted during the day of the EL-PFDD - written by those with ARVC - providing the FDA and researchers with information about their daily lives with the condition, and what they would like to see in future treatments.

This was such an impactful event for everyone involved - from the ARVC community to the sponsors and FDA members who attended.

"Current therapies for SADS don't target the disease itself, are sometimes invasive, and have negative side-effects," says

Genevie Echols, Family Support. "We are thrilled that for the first time, the FDA and medical product developers, clinicians, and academic researchers will hear directly from patients and their families. By centering real-life experiences with the disease, more effective therapies can be developed that help people live their lives to the fullest."

Expert Voices Weigh In

In our second major meeting for our EL-PFDD for ARVC - our Scientific Session - a panel of international experts gave insights on how to shape future treatments so they reflect what's most meaningful to those with ARVC.

Sam Sears, PhD, talked about the importance of mental health for those with ARVC, and how we can address those needs now and while developing future therapies; Dr. Hugh Calkins discussed the desperate need for better therapies - including gene therapy - and how exciting the state of current research is; and Dr. Mario Delmar talked about the importance of developing treatment for all the ARVC communities - including those with no identified genetic mutation, and how to help kids and teens with ARVC.

In September of 2023, all of the data from both EL-PFDD meetings, including all of the polls and testimonials from participants with ARVC, were summarized in a Voice of

"On behalf of the Rejuvenate Bio team, I'd like to congratulate you on a fantastic EL-PFDD meeting. It made me incredibly proud to be working on therapies for this disease and we look forward to incorporating all that was shared today in our program moving forward. Congratulations to you on this incredible feat of advocacy that will move the field forward toward meaningful treatments."

- Jodi Wolff, Vice President,
Patient Advocacy & Engagement at
Rejuvenate Bio

the Patient Report, which continues to be available to the FDA, biopharma, researchers and physicians so they can learn more about ARVC and what's needed in new therapies (It's also available to our community to read).

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TOP STORIES IN THIS ISSUE

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New resources on mental health, exercise, pregnancy - page 5

Our Next Next Steps - for Long QT and CPVT

This meeting emphasized the critical need for newer and better treatments for SADS conditions. While ARVC needs a treatment that will stop disease progression, all SADS conditions need newer, better therapies that target the disease process itself.

That's why we are holding another EL-PFDD meeting in 2024 to inform the FDA and stakeholders about CPVT and LQTS. We are currently collecting testimonials and registrations for those

"This has been such a striking and inspiring day. Thank you to SADS for leading this important effort and my sincere gratitude to the patients with ARVC who bravely shared their stories today,"

- Megan, ARVC



affected, and their families, to attend on the day of the EL-PFDD and make sure their voices are heard by those shaping and approving new, groundbreaking treatments for SADS conditions. You can register at the QR code or by visiting sads.org/annualreport24.

Alice Lara, SADS CEO & President, and Genevieve Echols, SADS Family Support Director, with Christy and Jeff, who told their stories for the FDA.



Genetic Testing - What You Need to Know as Research Progresses

Knowing the name of your condition is not enough.

Through our genetic testing campaign aimed at helping families and their doctors understand the importance of genetic testing we reached 112,137 people in 2023 with this critical message.

Genetic testing is considered the standard of care - in other words, a critical part of being properly evaluated - for anyone diagnosed with a SADS condition. Here at SADS, we recommend that ALL families with a SADS diagnosis get genetic testing - and that doctors treating people with SADS also learn about how to connect families with this vital resource.

Not only can getting genetic testing help your doctors decide what kind of treatment is best for you - it's necessary to know your family gene in order to participate in upcoming research. To learn more about genetic testing and its importance in participating in upcoming research, and access resources to help your family get genetic testing, visit sads.org/annualreport24 or scan the QR code on this page.

Helping Educate Healthcare Professionals

Accessing genetic testing does not have to be a major lift for physicians or their practices. Here at the SADS Foundation, we have created a toolkit for physicians so they can easily access the information, tools, and specialists they need. We've reached over

Genetic Testing by the Numbers

- **Less than half of families** had extended family members tested - that's a lot of family members who haven't been tested yet!
- Almost half of participants were **tested over 6 years ago** - it might be time to consider getting retested with all the new discoveries in genetics.

3,000 physicians so far with this toolkit. Scan the QR on this page for more information.

"If cardiologists are diagnosing someone with a genetic heart condition and are not doing genetic testing for them, they are not practicing standard of care."

- Dr. Michael Ackerman, chair of the SADS board of directors and genetic cardiologist at Mayo Clinic

Resource Spotlight: SADS & Exercise

We've just updated our website with the latest updates and guidelines around exercise, movement, and sports participation with a SADS condition. From stories to up-to-date webinars with guidelines for all our conditions, you can learn the latest on moving in a safe, healthy way.



Bella, our Family Support Director Genevieve's dog, participated in our recent Exercise & Movement Challenge at SADS.

Drs. Michael Ackerman and Dr. Hari Tandri stressed that exercise with a SADS condition is safe and possible (including for ARVC) and that joint decision making is an important part of the process of finding a safe level of exercise. Learn more by scanning the QR on this page.



Gene Therapy: A New Horizon for SADS Conditions

You may have recently heard more about gene therapy - a new type of potential treatment for SADS conditions. Several companies are getting closer to having clinical trials open for specific gene mutations - and a few trials are already beginning recruitment for ACM (arrhythmogenic cardiomyopathy).

"The energy committed to finding new, improved treatments for SADS conditions is like it's never been before in my career."

- Dr. Michael Ackerman, chair of the SADS board of directors and genetic cardiologist at Mayo Clinic

This is a great time to start educating yourself about gene therapy - what it is, the potential risks of participating in a clinical trial, and the impacts the therapy might have for SADS conditions. We've put together FAQs and webinars, and will also be spending the month of May focusing on educating our community about gene therapy. Visit sads.org/annualreport24 or scan the QR code on this page for more.

Gene Therapy by the Numbers

Learn more by watching our Gene Therapy 101 Webinar with Dr. Andrew Landstrom, Duke University School of Medicine.

- The first patient was successfully treated with a gene therapy (for an immune condition) in 1989.
- There are currently 30 FDA approved gene therapies. Nearly all these therapies are for cancer, skeletal myopathies, and metabolic diseases.
- There are 3,951 gene, cell, and RNA therapies in the pre-clinical trial through pre-registration phase of development.

"I Have Hope for My Daughter:" Melanie's Research & ARVC Story

Melanie was diagnosed with ARVC (Arrhythmogenic Right Ventricular Cardiomyopathy) after experiencing ventricular tachycardia - a fast, irregular, and dangerous heart rhythm - in 2021. But that was just the beginning of Melanie's heart journey.

She had an ICD (implantable cardiac defibrillator) implanted ten years after experiencing her first symptom, and underwent genetic testing; she tested positive for the PKP2 mutation for ARVC.

"I'd heard that ARVC is progressive but I didn't recognize how much loss I would face, because everyone's journey is so different. No one can tell me what comes next and that can be blindsiding," says Melanie.

In the future, Melanie hopes new, improved treatments will be available for her eldest daughter - who is also PKP2 positive. "I have hope that in five or ten years, we may see new options that could save her from all of this," she says. "There's been a lot of progress in research so far, and because of that, I can hold onto hope for my daughter."



Marcia Baker, SADS Program Director, and Dr. Sam Sears, PhD, East Carolina University

Mental Health: An Important Piece of the Journey for SADS

We know that living with a SADS condition isn't always easy. You may find yourself struggling with anxiety, PTSD, depression, or a sense of isolation following diagnosis; or experience these symptoms as a result of treatment for your SADS condition, like a device.

We want you to know that you're not alone. During a meeting with the FDA in June of 2023, we learned that people living with the SADS condition ARVC rated anxiety and depression as one of the most burdensome health effects that they experienced on a daily basis.

We've revamped our resources on our website to have a rapidly-growing section dedicated to mental health and SADS, with the latest resources and up-to-date webinars from Sam Sears, PhD on coping with SADS and mental health. Scan the QR code to learn more.

Five Ways of Developing Your Safety Net

- Start small
- Get help from loved ones
- Focus on sustainable change
- Expect barriers
- Every little step counts



SADS Corporate Roundtable 2023

In 2023, six companies joined as members of SADS Corporate Roundtable: **ARMGO**, **BioMarin**, **Bristol Myers Squibb**, **Cardurion**, **Rejuvenate Bio** and **Tenaya Therapeutics**. These biotech companies are engaged in developing therapeutics and devices for genetic channelopathies and cardiomyopathies. The SADS Corporate Roundtable met twice in 2023 - during Heart Rhythm Society and during American Heart Association Scientific Sessions - to address shared challenges and opportunities in genetic channelopathy and cardiomyopathy therapy development.

The SADS Foundation thanks our Corporate Roundtable Members for their support:



Four Years of Live Q&A with Dr. Michael Ackerman

It's been four years since we started our twice-a-month livestreaming program, SADS Live. In the past four years we've had incredible guests from around the globe including Drs. Peter Aziz, Susan Etheridge, Christopher Semsarian, Silvia Prior (Italy), Elijah Behr (England), Georgia Sarquella Brugada (Spain), and Charlie Berul.

A few of the research episodes you might have missed in 2023 include: the team behind the ORCCA study (about pro athletes with SADS conditions) discussing their research, Drs. Prince Kannankeril and Dominic Abrahms discussing participation in clinical trials for patients, and Dr. Andrew Landstrom recapping the top research papers of 2023 - and what they mean for you. Scan the QR code to catch up and learn more.

2023 Giving Highlights

The SADS Foundation extends its most heartfelt appreciation to all the families who have supported us financially in 2023!! Through contributions of all sizes, monthly donations, Facebook Fundraisers, and Volunteer Fundraising Events - more than \$440,000 (55% of our budget) was raised by our community. Thanks so much to each of you for your dedication!!

- 75 Impact Donors (gifts of \$1,000 or higher) brought in more than \$250,000.
- Another 584 donors raised an additional \$75,000 plus.
- 33 sustainers gave more than \$25,000.

Finally, 17 different families hosted volunteer fundraising events in their local communities bringing in nearly \$100,000! Events include Brittany's Trees, runs, golf tournaments, pub crawls, benefit concerts, and so much more! [Scan the QR code to learn more about the amazing donors who powered our advances in 2023.](#)

INCOME

Program Services

Individual Contributions.....	\$336,676
Foundations.....	\$19,500
Corporate Donations.....	\$277,250
In-kind Donations.....	\$25,260
Volunteer Events.....	\$80,266
Other	\$14,215
Grand total	\$753,167

EXPENSES

Program Services

Advocacy	\$42,604
Awareness.....	\$137,134
Volunteer	\$16,980
Family Support	\$257,951
Medical Education	\$160,877

Total Program Services..... \$615,545

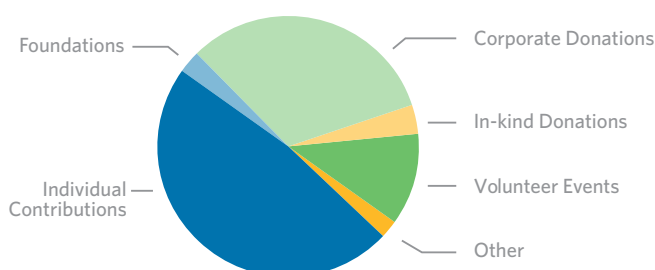
Support Activities

Management & General.....	\$42,505
Fundraising.....	\$95,379

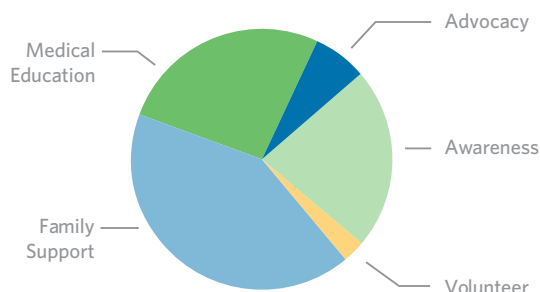
Total Support Activities..... \$137,884

Total Expenses..... \$753,429

INCOME



EXPENSES



“The Future is Looking Brighter” Alexis’s Research & LQTS Story

Alexis was diagnosed with Long QT Syndrome a year after collapsing during a pep rally in seventh grade. Her Long QT was missed by two cardiologists and two providers before she received a diagnosis.

Alexis has tried several different methods of treatment since then – including beta blockers, mexiletine, and devices – but hasn’t found one that works for her as a competitive dancer.

After returning from dance practice this spring, Alexis collapsed again – and her ICD recorded a dangerous rhythm. This means that she will need to escalate treatment.

As Alexis prepares for Worlds this spring with her dance team, she is hopeful that one day new and improved treatments will be available for LQTS. “I am excited for the potential treatments research can offer for LQTS, from medication that could treat my condition without needing several surgeries to potentially even a cure one day,” says Alexis. “The future is looking brighter!”



Alexis, a competitive dancer, is hopeful for new, improved treatments for her Long QT Syndrome.

Resource Spotlight: Pregnancy

We know that having a heart condition while considering starting a family, or while pregnant, can create extra challenges and questions for our SADS community. You may be looking for resources to help educate your doctor - or just answer questions like, what are the chances my child might have a SADS condition? And is it safe to stay on my medication while pregnant?

We’ve updated our resources on our website to reflect the latest guidelines and newest research on pregnancy and SADS conditions. From webinars to stories to FAQs, check it out at sads.org/annualreport24 or scan the QR code on this page for more



Kellie shared her pregnancy story as part of our awareness month – plus, took over our Instagram for an Instagram Takeover and Q&A.

New Research Published in 2023

2023 was an important year in the publication of research into SADS conditions as researchers worked toward further understanding and better treatments for these conditions. These are just a few of the amazing papers that were published.

A paper published in European Society of Cardiology looked at the last 25 years of research in channelopathies and cardiomyopathies, finding extensive developments in the technologies used for genetic testing, understanding human genetics and how SADS conditions work, leading to better patient care and new potential therapies - including gene therapies.

In February, a paper in Heart Rhythm Journal looked at the effects of SGK1 inhibitors on heart cells that were re-engineered to have congenital Long QT Syndrome. This research showed the SGK1 inhibitor shortened the action potential duration in LQTS, and this potential treatment began enrolling for human clinical trials in July 2023.

Dr. Arthur Wilde published an article in Heart Rhythm Journal looking at the Top Ten Stories on Brugada Syndrome (BrS). These articles covered structural abnormalities and progression of these abnormalities, ablation, new disease prediction scores, and the role of sodium channel blocker test in BrS.

A paper published in Circulation showed that adding flecainide to beta blockers in patients with catecholaminergic polymorphic ventricular tachycardia (CPVT) resulted in fewer arrhythmic events. This reduction was most notable in patients who continued to have symptoms even while on beta blockers. Scan the QR code to read these and other research papers.

Currently Enrolling Research

Right now is a very exciting time for those living with SADS conditions because of advancements in research and the development of potential new therapies. There are currently multiple opportunities to enroll in research studies and clinical trials for SADS conditions. Scan the QR code to find out more.





Program Director Marcia at the booth at one of our in-person events in 2023.

Meeting Our Community In-Person

As research enters this new, exciting phase, we know that one of the biggest needs of our SADS community is to gather together in-person so you can talk with others who've been through a similar journey and ask experts your questions about your condition - and upcoming research.

In 2023, we held three in-person family seminars in Iowa, Baltimore and Nashville. We made sure to discuss upcoming research and what it looks like to participate in a clinical trial at each seminar.

And in 2024, we will be holding our first in-person International SADS Family Conference since COVID in Chicago, Illinois. Join us at the 2024 International SADS Foundation Conference on Friday November 8 - Saturday November 9, 2024 at Lurie Children's Hospital, Chicago, Illinois. Visit sads.org/annualreport24 or scan the QR code on this page to learn more.

Be the First to Know About Cutting-Edge Research

There's new, exciting, and groundbreaking research on the horizon for SADS conditions. At the SADS Foundation, we're working with companies who are almost ready to recruit participants for first-of-their-kind clinical trials for these new therapies.

But first, we need to learn more about our community. Take our ten-minute survey - it helps us learn more about you so we can help connect you to research studies you might be eligible to participate in. Your participation also helps researchers learn more about our condition communities as research progresses. Scan the QR code to get started.



Genevieve, Family Support Director, with board member Meredith and her daughter Alexis at our Nashville Family Seminar.

Your Stories Make an Impact on the FDA

Courtney West didn't know that she had a potentially fatal heart condition until her brother, Jacob, died suddenly during a football practice from sudden cardiac arrest in 2013.

After Jacob's death, doctors suspected it was due to a genetic heart condition. Courtney began an extensive testing process. She had multiple arrhythmias (abnormal heart rhythms) during a stress test, which led to a diagnosis of ARVC (arrhythmogenic right ventricular cardiomyopathy), a progressive genetic disease of the heart muscle.

Courtney participated in our EL-PFDD for ARVC to inform the FDA about what it's like to live with ARVC as a young woman. Visit sads.org/annualreport24 or scan the QR code on this page to learn more about her story.



FAMILY WEBINAR SERIES

Highlights from Living with SADS Webinars

Gene Therapy 101,	Dr. Andrew Landstrom
Supplements and Drugs to Avoid	Drs. Michael Ackerman and Will Heise
Sports and Exercise for SADS	Drs. Harikrishna Tandri and Michael Ackerman
Clinical Trials - Learn the Basics	Dr. Dominic Abrams
Mental Health and PTSD for SADS	Dr. Sam Sears

Upcoming Events at SADS (Virtual and In-Person)

May 2024	Gene Therapy Awareness Month	Join us VIRTUALLY
May 16-19	Heart Rhythm Society	Boston, MA
June 11	EL-PFDD for LQTS and CPVT	Join us VIRTUALLY
November 8-9	2024 International SADS Foundation Conference	Chicago, IL

