CURE MITO FOUNDATION

Dear Friends, Partners, and Followers of the Cure Mito Foundation,

Thank you for reading our November 2023 Newsletter!

In this newsletter we are sharing about our new published paper, new collaborations, and more news. With only a few weeks left of the year, we are reflecting on the past months.

This year, with the support of our community, we have raised funds towards gene therapy and drug repurposing research projects, launched a corporate advisory council, gathered nearly 250 participants at the 2nd Annual Leigh syndrome symposium, launched aboutleighsyndrome.com website, created family planning guide and many other new educational and support resources for patients and healthcare providers, built Leigh syndrome medical provider directory (to be announced soon!), published 2 papers (with one on the way!), worked on launching a mito taskforce with C-Path (watch out for news soon!), got recognized as the Top-Rated Nonprofit by GreatNonprofits and so much more.

As you reflect on your end-of-year charitable contributions, we hope you will consider a gift to the Cure Mito Foundation, so that we can continue to do more for our community. As always, we hope this newsletter will leave you feeling a little bit more hopeful and a little more inspired.

The Cure Mito team

"The difference between ordinary and extraordinary is that little extra."

— Jimmy Johnson

We have a new published paper!

In 2022, we held a first Leigh syndrome symposium, which brought together patients, industry, academic researchers, and clinicians globally. We co-hosted it with integrative Cardiovascular Metabolism and Pathophysiology Laboratory (iCaMP) at Boston University.

Our paper, "Teamwork makes the dream work: functional collaborations between families, scientists, and healthcare providers to drive progress in the treatment of Leigh Syndrome" can be found HERE.


Moreira et al. Orphanet Journal of Rare Diseases (2023) 18:355
https://doi.org/10.1186/s13023-023-02871-7

Orphanet Journal of Rare Diseases

LETTER TO THE EDITOR — Open Access

Teamwork makes the dream work: functional collaborations between families, scientists, and healthcare providers to drive progress in the treatment of Leigh Syndrome

Jesse D. Moreira[1†], Karan K. Smith[1†], Sophia Zilber[2], Kasey Woleben[2*†] and Jessica L. Fetterman[1*†]

Abstract

Background Leigh syndrome, an inherited neurometabolic disorder, is estimated to be the most common pediatric manifestation of mitochondrial disease. No treatments are currently available for Leigh syndrome due to many hurdles in drug discovery efforts. Leigh syndrome causal variants span over 110 different genes and likely lead to both unique and shared biochemical alterations, often resulting in overlapping phenotypic features. The mechanisms by which pathogenic variants in mitochondrial genes alter cellular phenotype to promote disease remain poorly understood. The rarity of cases of specific causal variants creates barriers to drug discovery and adequately sized clinical trials.

Body To address the current challenges in drug discovery and facilitate communication between researchers, healthcare providers, patients, and families, the Boston University integrative Cardiovascular Metabolism and Pathophysiology (iCAMP) Lab and Cure Mito Foundation hosted a Leigh Syndrome Symposium. This symposium brought together expert scientists and providers to highlight the current successes in drug discovery and novel models of mitochondrial disease, and to connect patients to providers and scientists to foster community and communication.

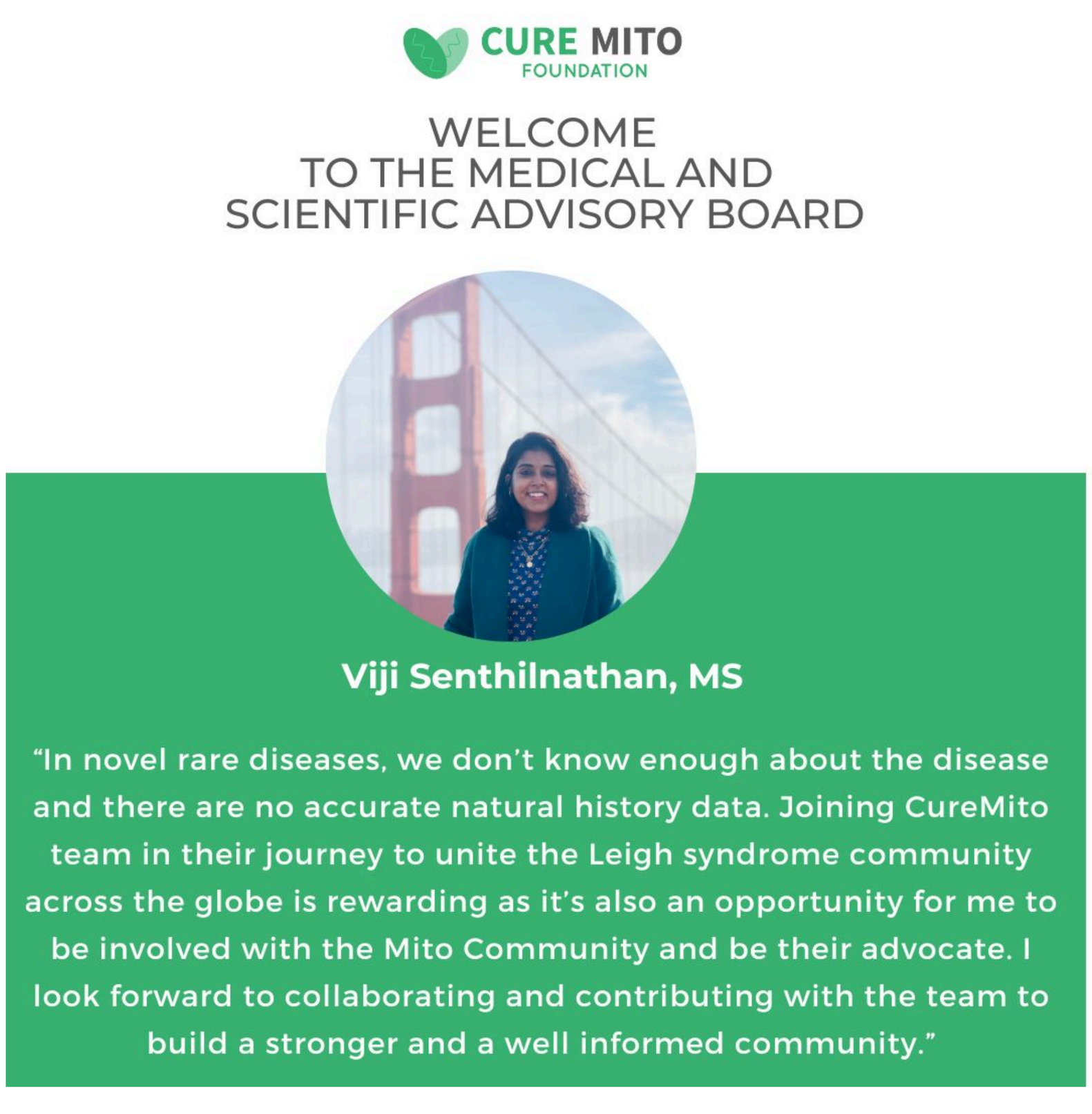
Conclusion In this symposium review, we describe the research presented, the hurdles ahead, and strategies to better connect the Leigh syndrome community members to advance treatments for Leigh syndrome.

Keywords Leigh syndrome, Mitochondria, Mitochondrial genetics, Mitochondrial disease, Community, Patient registries, Symposium

We are excited to share that we have joined Defense Health Research Consortium in order to be able to better advocate for funding for Leigh syndrome and mitochondrial disease.

The Defense Health Research Consortium was formally established in 2014 to bring together the diverse community of patient advocacy organizations, medical provider groups, veterans' organizations, research advocacy groups, and private sector interests — all with the single purpose of protecting and preserving funding for the Congressionally Directed Medical Research Programs (CDMRPs).

We are honored and excited to welcome Viji Senthilnathan, MS to our medical and scientific advisory board! Vij is a leader in clinical operations working on small molecule therapy. Her involvement in rare diseases has been since 2014 starting with her role in running global clinical trials for Leigh Syndrome, MELAS, MERFF, RARS2, Pearson Syndrome, Friedreich Ataxia, Amyotrophic lateral sclerosis (ALS), Parkinson Disease and Rett Syndrome. She works with various patient advocacy groups to understand patient's perspectives for a clinical trial and ensures that the patient-centric approach is incorporated into the study design.


CURE MITO FOUNDATION
WELCOME
TO THE MEDICAL AND
SCIENTIFIC ADVISORY BOARD
Viji Senthilnathan, MS
"In novel rare diseases, we don't know enough about the disease and there are no accurate natural history data. Joining CureMito team in their journey to unite the Leigh syndrome community across the globe is rewarding as it's also an opportunity for me to be involved with the Mito Community and be their advocate. I look forward to collaborating and contributing with the team to build a stronger and a well informed community."

Earlier this month, Green Family held a walk in honor of Bradlee who passed away on June 20th. Bradlee's family raised $1000 for Leigh syndrome research! She is missed by all but will not be forgotten! Our hearts are with Bradlee's family. Thank you so much for your generous contribution to fund research for this devastating disease. Fly high, Bradlee


Meet Will! A McKinney Resident!
Communities for a Cure
How far would you go to save your child's life?
These children have a life-limiting rare disease called Leigh syndrome that is robbing them of their abilities. We need your help to fund a cure!
Donations ARE tax-deductible! No amount is too small!
$2.5M is needed to complete our gene replacement therapy project. Thank you!
CURE MITO FOUNDATION
for more information: curemito.org

Communities for a Cure

Giving Tuesday and End of Year are approaching! Support Leigh Syndrome research by joining our "Communities for a Cure" campaign. We can personalize flyers to your city and child, making them suitable for display in various locations such as downtown areas and near cash registers. Please email us your information at info@curemito.org to get a customized flyer!

Leigh Syndrome Families - Please Join the Patient Registry and Be Counted

Largest LS patient registry in the world
Accessible and available data
Results are always reported back to the community

LEARN MORE

A therapist's perspective: Supporting your child with a rare and fatal disease.

"During my son Lucas's life, I often struggled with how many therapy sessions to schedule. There was so much we could do for Lucas. Physical, occupational, feeding, and speech therapies were just the start. Yet nothing we did would fundamentally alter the course of his disease. What should we do?"

Kim interviews her son Lucas's therapist after his passing. Please read the full interview HERE.

Meet us at upcoming conferences

Sophia will speak at a PHUSE one-day event dedicated to rare diseases and hosted by UCB. She will share about the work of her working group, "Best Data Practices for Rare Disease Patient Foundations and Researchers" and about our patient registry for Leigh syndrome. Learn more HERE.

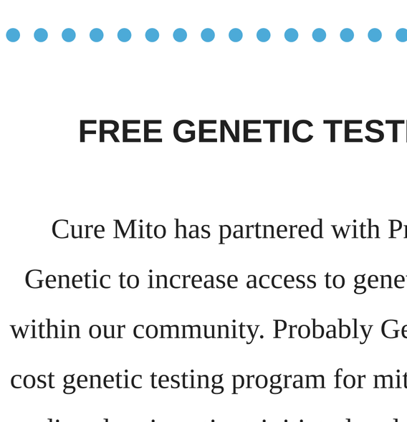

CURE MITO FOUNDATION
Get to Know Us!
We are a nonprofit patient advocacy organization led by parents who volunteer their time to change the future for families affected by Leigh syndrome.
Our mission is to unite the global Leigh syndrome community to accelerate patient-centered research, treatments, and cures.

Remember to visit our website to download and print flyers and bring to your own event, fundraiser, share with your medical team, and more! Learn more HERE.

About Leigh Syndrome
Cure Mito Foundation

WANT TO LEARN MORE ABOUT LEIGH SYNDROME?

AboutLeighSyndrome.com is a first of its kind informational website about Leigh syndrome with resources and support for both families and healthcare professionals.

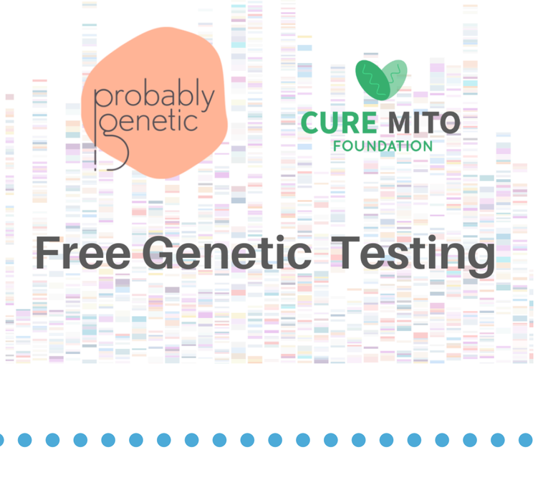


LEIGH SYNDROME CLINICAL NETWORK UPDATE

If you see a medical provider of any specialty who sees patients with Leigh syndrome OR if you are yourself such medical provider, please complete the form by clicking HERE

FREE GENETIC TESTING

Cure Mito has partnered with Probably Genetic to increase access to genetic testing within our community. Probably Genetic's no-cost genetic testing program for mitochondrial disorders is patient-initiated and includes genetic counseling to explain any questions or findings. Please learn more HERE.



Cure Mito Foundation is a 501(c)(3) nonprofit organization led by parents who volunteer their time to search for a cure. 100% of your donations are tax-deductible and will go directly to research dedicated to mitochondrial diseases.

Your donation matters!

For many ways to give please visit:
https://www.curemito.org/ways-to-give

Many volunteer opportunities are also available!

Skills and expertise currently needed: fundraising, marketing, writing (scientific writing, grant writing, blog writing). Please contact us at info@curemito.org to learn more.

DONATE NOW

Follow Us:

Platinum Transparency 2023 Candid.

GREATNONPROFITS 2023 TOP-RATED NONPROFIT