CURE MITO FOUNDATION

Have you registered for the conference?

EMPOWER AND INSPIRE:

Understanding and Accelerating Research for Leigh Syndrome

Co-hosted by Cure Mito Foundation and integrative Cardiovascular Metabolism and Pathophysiology Laboratory (iCaMP) at Boston University

Please join us Virtually on

Tuesday, September 20, 2022

The agenda is now ready! Join nearly 250 people who are already registered and join us for a community event during Mito Awareness Week! Hear from leading LS researchers from around the world, doctors, data experts, and patient families!

VIEW AGENDA AND REGISTER

THANK YOU TO OUR CONFERENCE SPONSORS

PTC THERAPEUTICS

SUMPTUOUS

THANK YOU TO OUR MEDIA PARTNERS

Thank you to the mito and rare disease organizations around the world who help us share this event with the community!

AEPOW www.aepow.org

mitoACTION

indousrare

mitocanada

Leigh Syndrome Families

Our patient registry has been open for less than a year. It has grown to be the largest LS patient registry with over 190 patients currently enrolled from at least 28 countries. Data is freely available to interested researchers and industry. Results are always reported back to the community. If you have not yet joined - please join, your participation is making a difference!

JOIN THE PATIENT REGISTRY

Please learn more about the patient registry timeline, news, and activities:

- We want to thank the peer-reviewed open-access journal, *Biomolecules*, for an invitation to submit a paper describing comprehensive results from our Leigh Syndrome Global Patient registry in the special issue, "Mitochondrial Genetic Variation in Health and Disease". You can read the announcement of our planned paper here: Biomolecules | Special Issue: Mitochondrial Genetic Variation in Health and Disease (mdpi.com)
- Poster with our patient results will be presented at mitoNice conference in Nice, France, in September 2022
- Poster with the results of our collaboration on alignment and interoperability of Leigh Syndrome Patient Registry Data with Regulatory Submission Standards will be presented at PHUSE/FDA Computational Science Symposium in September 2022. After this, results will be shared with the entire community. The press release about this project can be found here: https://www.curemito.org/cdisc
- Our registry has been listed on Orphanet, with other international registries and biobanks - June, 2022
- Poster has been presented at UMDF symposium - June 2022
- Poster has been presented at Mitochondria-Targeted Drug Development Summit, February 22-24, 2022. The poster can be seen here: https://www.curemito.org/results/
- Leigh Syndrome Global Patient Registry opened - September 2021

If you see a Mito specialist, please download and share our printable IRB-approved flyer with your medical team: https://www.curemito.org/registry

Surveys in Spanish and Portuguese are available. All translations are certified, and IRB approved. If you have an interest in additional languages, please reach out to us!

We were interviewed by Khondrion and had an opportunity to share out stories and our efforts to help patients. Thank you, Khondrion, for helping our voices be heard! To read the full interview please visit: https://www.khondrion.com/patient-perspective-a-conversation-with-kasey-wolfeben-co-founder-and-sophia-zilber-board-member-patient-registry-director-cure-mito-foundation/

Helpful links

Newly diagnosed patients guide

https://www.curemito.org/newly-diagnosed

Birthday club

https://www.curemito.org/birthdays

Support

https://www.curemito.org/support

Books about finding resilience, hope, and courage

https://www.curemito.org/recommended-books

Just for kids

https://www.curemito.org/for-kids

Clinical trial updates

If you or your child has confirmed mitochondrial disease diagnosis with associated epilepsy you may qualify for MIT-E study by PTC Therapeutics, Inc. The study is currently open and enrolling patients. Please note that the study inclusion criteria have been expanded to include men and women up to 20 years of age.

More information can be found at:

www.themit-estudy.com or

https://clinicaltrials.gov/ct2/show/NCT04378075?term=NCT04378075&rank=1

With any questions please contact MitoStudy@ptcbio.com.

Children 6 months-17 years old with genetically confirmed pyruvate dehydrogenase complex (PDC) Deficiency may qualify for a Trial of Dichloroacetate in Pyruvate Dehydrogenase Complex Deficiency: (DCA/PDCD).

More information can be found at:

Trial of Dichloroacetate in Pyruvate Dehydrogenase Complex Deficiency: - Full-Text View - ClinicalTrials.gov

To search for a complete list of clinical trials please visit: https://clinicaltrials.gov/

Get in touch and join us!

Please visit our website to learn about our research projects, resources, and more:

https://www.curemito.org/

We invite you to join us in our efforts! For ways to get involved please visit:

https://www.curemito.org/get-involved

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