



**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**

**REGISTER NOW**

More information at:

<https://www.curemito.org/conference>

The detailed agenda will be available soon - please continue to check the link above for updates!

**THANK YOU TO OUR CONFERENCE SPONSORS**



**Leigh Syndrome Global Registry Updates**

We are excited to share our LS Global Registry poster with our patient results will be presented at mitoNice conference in Nice, France, in September 2022!



We are proud to share a new collaboration:

**Cure Mito Foundation and Sumptuous Data Sciences, LLC partner on alignment and interoperability of Leigh Syndrome Patient Registry Data with Regulatory Submission Standards**

Please read a full press release here: <https://www.curemito.org/cdpsc>



The results of this project will be shared at the PHUSE/FDA Computation Science Symposium in September 2022!



If you or your loved one has Leigh syndrome or if your loved one has passed, [please join the registry](#) - each patient helps to understand the disease better, raise awareness, create additional research opportunities, find treatments, and build a stronger community!

Results and findings are always shared and available.

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!

Sophia Zilber, Cure Mito patient registry director and board member shared tips and best practices regarding working with data at the Sanford CoRDS 12th Annual Great Plains Rare Disease Summit.

The recording can be viewed here: <https://www.youtube.com/watch?v=Im200ZwaHC8>

Slides can be seen here: <https://www.slideshare.net/SophiaZilber/data-management-and-analysis-example-from-leigh-disease-global-patient-registry>

**Data Management and Analysis – Example from Leigh Disease Global Patient Registry**

Sophia Zilber, Cure Mito Foundation

Sanford CoRDS 12th Annual Great Plains Rare Disease Summit  
May 21, 2022



We attended the UMDF conference in Phoenix, AZ early June. We shared our patient registry poster and connected with patients, researchers, and industry. Thank you to all who came up to the Cure Mito booth to say hello, learn about our programs, and connect!



**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](http://www.themit-estudy.com) or

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

With any questions please contact [MitoStudy@ptcbio.com](mailto: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](https://clinicaltrials.gov/ct2/show/study/NCT04378075)

To search 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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