

Empower and Inspire: 2nd Annual Leigh Syndrome symposium was held virtually on September 19th 2023, during Mitochondrial Disease Awareness week.

The conference has been organized by the Cure Mito Foundation in partnership with the integrative Cardiovascular Metabolism and Pathophysiology Laboratory (iCaMP) at Boston University, USA.

This event successfully convened patient families, industry professionals, academic researchers, and clinicians across the globe. Overall, 240 participants have attended, and the live language translation that we have provided was used to translate the symposium to 13 different languages.

Highlights of the conference included:

- Kasey Woleben gave an overview of the Cure Mito achievements in the past year, such as a launch of a new information website AboutLeighSyndrome.com and publication of a paper on Leigh Syndrome global patient registry. Throughout the presentation, she underscored the message that Cure Mito is led by families, for families.
- Dr. Steven Gray, PhD gave an uplifting keynote describing the current state-of-the art for using AAV vectors to deliver genes to the nervous system.
- Patient story: Diagnosed with Leigh's disease at just nine months old, Abby defied the initial prognosis that predicted a life expectancy of merely two years. Now 12 years old, Abby and her father Greg have become familiar and participated in numerous local road races, including multiple Boston Marathons.
- Research talks covered multiple topics, including gene therapy, gene editing, drug repurposing, and the immune system's role in Leigh syndrome.
- Dr. Shamima Rahman, FRCP FRCPCH PhD shared about resources designed to deepen our understanding of Leigh syndrome genes, such as the ClinVar gene curation and Leigh map.
- Dr. Ibrahim Elsharkawi, MD shared insights into how and why different therapies impact and benefit patients with mitochondrial disease, including physical therapy, occupational therapy, and speech therapy.
- Data-focused presentations included an update on Leigh syndrome global patient registry, and an update on Cure Mito collaboration with Critical Path Institute (C-Path) to launch a mitochondrial disease task force which would include multiple stakeholders to advance data sharing and accelerate development of treatments.
- Industry updates included presentations from Abliva, Astells, MytoLyfe Biotherapeutics, Saol Therapeutics, and Standigm.

Conference was sponsored by PTC Therapeutics, Saol Therapeutics, Sumptuous Data Sciences, and Society for the Study of Inborn Errors of Metabolism (SSIEM).

Additionally, the conference was supported by 17 community organizations serving as media partners, actively disseminating information and raising awareness.

Cure Mito Foundation has received outstanding feedback from the participants such as:

"This was incredibly well organized and executed. 10/10 worth my time. I'm so glad resources like this exist for our family!"

"This was fantastic! I had no idea how much was going on for Leigh's community. Very eye opening. I had gotten discouraged several years ago and just focused on caring for our daughter. This has re-energized me to do more and try to stay current on research and drug opportunities. Thank you for all the work that went into this symposium and to all the presenters!"

To view conference agenda and details please visit: <https://www.curemito.org/conference>

To view recordings of the presentations please visit:
<https://www.youtube.com/playlist?list=PLfcCshCQuODfClbPMAIQR1QgezXjgy1pS>

We extend our heartfelt gratitude to Society for the Study of Inborn Errors of Metabolism (SSIEM) for your invaluable support, which was instrumental in the success of this event. We look forward to future collaborations and the continued advancement of our field.

Sincerely,
Cure Mito Foundation