CURE MITO FOUNDATION

Registration is still open 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

Join us for a community event during Mitochondrial Disease Awareness Week! Hear from leading LS researchers from around the world, doctors, data experts, and patient families! Agenda can be found here: https://www.curemito.org/conference

REGISTER NOW

Cure ATP6 (a Rare Village Foundation Fund) and Cure Mito Foundation are excited to announce the collaboration between the Dr. Michal Minczuk (University of Cambridge), Dr. Alessandro Prigione (University of Dusseldorf), and Dr. Steven Gray (University of Texas Southwestern) on a 2-year $400,000 MT-ATP6 research grant. This grant will allow researchers to continue their work on creating an ATP6 animal model, creating neurons from Induced pluripotent stem cells (iPSC) lines, and exploring base editing for AAV9 gene therapy for mtDNA Leigh syndrome.


2 YEAR $400,000
MT-ATP6 LEIGH SYNDROME
RESEARCH GRANT AWARDED
Michal Minczuk, PhD
Group Leader, University of Cambridge
Alessandro Prigione, MD, PhD
Professor, University of Düsseldorf, Germany
Steven Gray, PhD
Associate Professor, University of Texas Southwestern Medical Center
CURE ATP6
rare village FOUNDATION
CURE MITO FOUNDATION

Congratulations to our Scientific Advisor, Dr. Qinglan Ling on receiving a 1-million-dollar award from the National Institutes of Health (NIH) for MT-APT6 Leigh syndrome research!

Dr. Ling said: " I have been involved in the gene therapy research for the SURF1 Leigh syndrome project, initiated by a family foundation, Cure Mito Foundation, for over four years while being a postdoctoral trainee at Dr. Steven Gray's lab at UT Southwestern in Dallas, TX. Being inspired by the families, especially their passion and dedication, I wanted to make my efforts to further fulfill their mission. I am honored to receive this opportunity from the NIH to be able to continue contributing to the mitochondrial disease community while assembling my own research team."

Cure Mito Foundation is extremely proud and excited for Dr. Ling and the research she is conducting for Leigh syndrome.

Help Our Children Grow!
September 18th-24th is Mitochondrial Disease Awareness Week! Our goal is to raise awareness and funds for Leigh syndrome research.

We need YOUR help to reach our $20,000 goal by the end of September:
• $50 covers lab reagents for one experiment
• $500 Pays for a postdoc for one day
• $5,000 Supports an entire lab for 3 days
• Any amount will help us get closer to therapies and cures for Leigh syndrome

Please donate through our Facebook fundraiser:
https://www.facebook.com/donate/829940254670817/

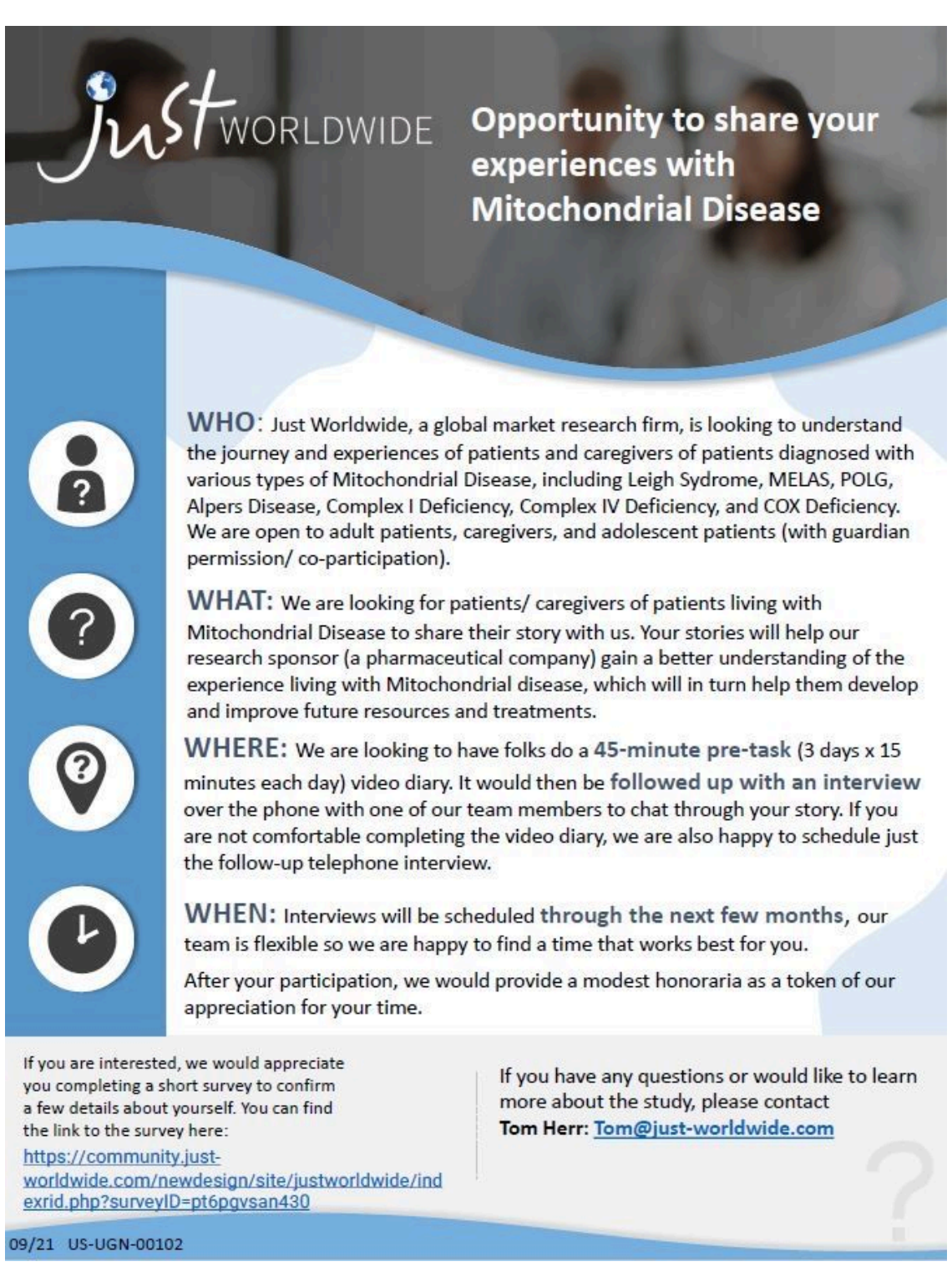
Want to donate another way? More ways to give are here: https://www.curemito.org/ways-to-give

Attention to all families in US and Canada: Your stories can help improve understanding patient experiences with mitochondrial disease and in turn can help develop better treatments

You can help by participating in 3 15-minute video diaries followed by a phone interview - please see details in the flyers below. If you are interested in participating please fill out short form below:

https://community.just-worldwide.com/newdesign/site/justworldwide/index.php?surveyID=pt6pgvsan430&published&id=68096b360f0e630be967cfbe3

For any questions please contact Tom@just-worldwide.com. You can also reach out to us at info@curemito.org.


Just WORLDWIDE
Opportunity to share your experiences with Mitochondrial Disease
WHO: Just Worldwide, a global market research firm, is looking to understand the journey and experiences of patients and caregivers of patients diagnosed with various types of Mitochondrial Disease, including Leigh Sydrome, MELAS, POLG, Alpers Disease, Complex I Deficiency, Complex IV Deficiency, and COX Deficiency. We are open to adult patients, caregivers, and adolescent patients (with guardian permission/ co-participation).
WHAT: We are looking for patients/ caregivers of patients living with Mitochondrial Disease to share their story with us. Your stories will help our research sponsor (a pharmaceutical company) gain a better understanding of the experience living with Mitochondrial disease, which will in turn help them develop and improve future resources and treatments.
WHERE: We are looking to have folks do a 45-minute pre-task (3 days x 15 minutes each day) video diary. It would then be followed up with an interview over the phone with one of our team members to chat through your story. If you are not comfortable completing the video diary, we are also happy to schedule just the follow-up telephone interview.
WHEN: Interviews will be scheduled through the next few months, our team is flexible so we are happy to find a time that works best for you.
After your participation, we would provide a modest honoraria as a token of our appreciation for your time.
If you are interested, we would appreciate you completing a short survey to confirm a few details about yourself. You can find the link to the survey here: https://community.just-worldwide.com/newdesign/site/justworldwide/index.php?surveyID=pt6pgvsan430
If you have any questions or would like to learn more about the study, please contact Tom Herr: Tom@just-worldwide.com
09/21 US-UGN-00102

Leigh Syndrome Families - Have You Joined the Patient Registry?

Largest LS patient registry with nearly 200 participants from at least 30 countries

Data is freely available to interested researchers and industry

Results are always reported back to the community

JOIN THE PATIENT REGISTRY

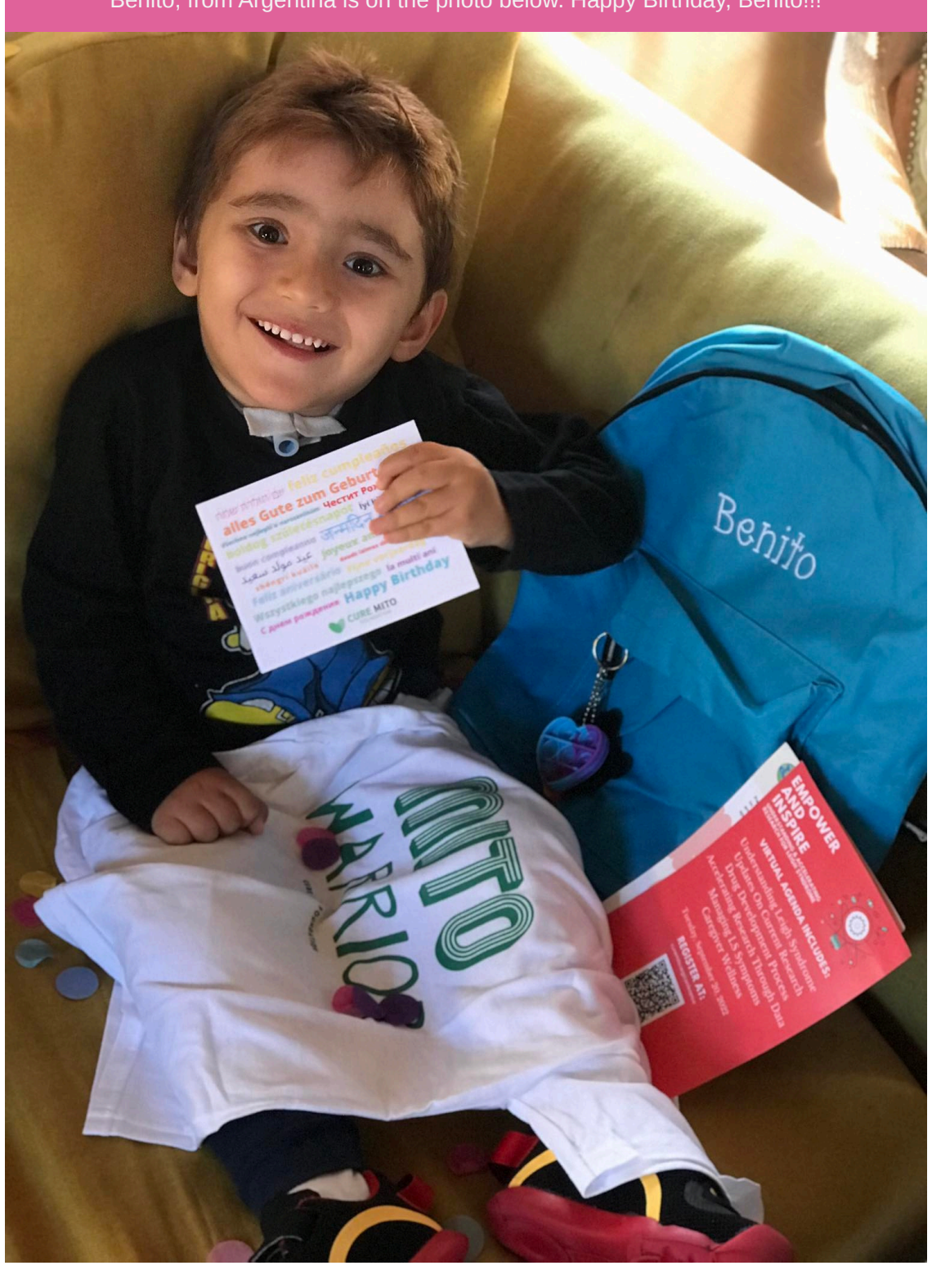
Patient registry timeline, news, and activities:

- Poster "Building a Worldwide Community - Leigh Syndrome Global Patient Registry" will be presented at the 2022 NORD Rare Diseases and Orphan Products Breakthrough Summit on October 17-18, 2022.
- 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/leighsyndromeregistry

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!

Did you know that more than 80 Leigh Syndrome families are a part of our Birthday Club? If you or your loved one have Leigh Syndrome, please join to get a special gift from us: https://www.curemito.org/birthdays
Benito, from Argentina is on the photo below. Happy Birthday, Benito!!!



Asset-Based Health Care for Children With Severe Neurologic Impairment

Liz Morris, a mom to Colson, who passed away from mitochondrial disease, a blogger in residence for Courageous Parents Network and one of the Cure Mito partner family members has co-authored this important paper in journal, Pediatrics, along with leading experts in the field of palliative medicine.

"Although it is certainly the clinician's duty to prepare families for the medical and developmental challenges that children with SNI (severe neurologic impairment) often face, it is equally important that these concerns are shared in conjunction with the triumphs that are possible and the hopes that always exist, even as they change form. Building a shared, holistic understanding of a child's baseline health and quality of life provides important points of consideration and reflection that can guide care for the child, even as that baseline may decline. Studies suggest that families want to share these aspects of their child's life with clinicians so that there is alignment with the health care system that supports their family."

You can read the full paper here: Asset-Based Health Care for Children With Severe Neurologic Impairment | Pediatrics | American Academy of Pediatrics (aap.org)

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

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

DONATE