THE LOST ENZYME PROJECT

Annual Report 2025

Our Mission

We are a patient advocacy group dedicated to supporting those affected with Beta-Mannosidosis and the development of new life-saving treatments.

A Message from the Founders

This year has been one of both progress and urgency.

What started as a mission rooted in a single diagnosis has grown into something much bigger—a collaborative effort between families, researchers, clinicians, and partners all working toward the same goal: a treatment.

We have seen meaningful scientific progress. We have built critical partnerships. And we have continued to push forward, even when the path is uncertain.

But the reality remains the same: our children are still waiting.

Everything we are building is with them in mind—and we will not stop until a treatment is within reach.

— TLEP Team

Founders & Board Development

TLEP is currently assembling and expanding its Board of Directors to support the next phase of organizational growth and treatment development efforts.

Name	Role
Erin Hubbard	Co-founder • Board Member, ISMRD
Nathan Hubbard	Co-founder • Secretary, ISMRD
Laurel Gregier	Co-founder • Board Member, ISMRD
Nathan Gregier	Co-founder
Lorena Lomelin	Co-founder
Fernando Fernandez	Co-founder & Development Officer • Board Member, ISMRD

Scientific Advisory Board

Member	Role
Dr. Virginia Kimonis	Lead Researcher
Samantha Behunin	Science Liaison / Patient Identification Specialist
Jenny Jackson	Scientific Advisor
Liz Worthy	Scientific Advisor

Contributing Members of TLEP

Emma Luptak — Program Manager / Patient Advocacy Specialist
Alex Ju — Podcast Producer
Trevor Hubbard — Chief Information Security Officer
Ivy Wang — Website Designer
Phoebe Wang — Research / Patient Identification Specialist
Arina Danilina — Fundraising & Advocacy Specialist
Elena Hernandez — Design & Marketing Specialist
Edwin Ventura — Graphic Design & Marketing Specialist
Arman Memarzadeh — Communications Specialist & Fundraising
Olivia Buckel — Communications Specialist
Ann Davis-Williams — Newborn Screening Specialist
Marcella Pietrzyk — Grant Writer

Year in Review 2025

Scientific Progress

- Advanced preclinical research toward a potential treatment for Beta-Mannosidosis
- Continued collaboration with leading researchers at UC Irvine
- Progress toward a Natural History Study, a critical step in clinical development
- The Worthey group is conducting a pilot study to examine the broad phenotypic and genotypic heterogeneity of beta-mannosidosis using specialized bioinformatics tools developed by the Worthey group. The study aims to establish proof of concept for developing personalized interventions and characterizing disease phenotypes at the individual level.

Partnerships & Collaboration

- Continued support from the Kimonis Lab at UC Irvine
- Engaged with academic and industry partners to support long-term development
- Built a growing network of clinicians, researchers, and advocates

Building the Foundation

- Raised over $500,000 to support early-stage research
- Expanded outreach to families affected by rare diseases
- Formed a Scientific Advisory Board to guide research and development
- Built a Patient Identification Team to connect and support affected families

Why This Work Matters

Rare diseases affect millions of children worldwide, yet most still have no approved treatment.

For families like ours, time is not theoretical—it is daily, urgent, and real.

A Natural History Study is one of the most critical steps in bringing treatment to clinical trials. It helps define how a disease progresses and provides the data needed for regulatory approval.

Without it, treatment cannot move forward.

Financial Overview

$500,000+ was raised during 2025 to support preclinical research and treatment development efforts. Please refer to our publicly available Form 990 for our 2025 financial information.

Primary Use of Funds

- Preclinical research
- Scientific collaboration
- Early planning for Natural History Study
- Operational and administrative support

Every dollar raised is directed toward advancing treatment development efforts for Beta-Mannosidosis.

What Comes Next

- Launching a Natural History Study
- Securing funding for GMP manufacturing
- Preparing for clinical trial readiness
- Expanding patient identification efforts

These steps are essential—and time-sensitive.

Our Community

This work would not be possible without the support of families who share their stories, researchers committed to progress, donors who believe in what's possible, and advocates helping us move faster.

Together, we are building something that did not exist before.

Donors, Sponsors & Partners

Please refer to our website for a list of donors and partners. Thank you for your continued support.

Looking Ahead

We are closer than we have ever been—but there is still work to do. Science is moving forward, but time and funding remain the biggest barriers between children like ours and a treatment for Beta-Mannosidosis. With continued support, we can keep pushing this work forward and bring hope to families still waiting.

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