UNITED MITOCHONDRIAL DISEASE FOUNDATION®

ANNUAL REPORT 2025

A message from the **CEO**

Kristen **Clifford**
President and CEO

When I arrived at UMDF in January of 2025, I spoke with many of the leading minds in mitochondrial medicine and science. I heard the same phrases again and again -- "on the cusp of great things," "momentum," and "breakthroughs."

With affected families, it was different. Hope was there, but memories were long.

For years, progress has come in shades of gray -- better diagnostic tools, promising research, therapies described as coming soon. Meaningful steps forward, yet little that felt life changing for those living with mitochondrial disease.

Until this year.

After enduring year after year of anticipation, 2025 marked a turning point. In early September, following a 20-month dialogue with regulators and the tireless efforts of advocates across the country, Forzinity (elamipretide) was approved for individuals living with Barth syndrome. Just months later, Kygevvi (doxecitine and doxribtimine) was approved for patients with thymidine kinase 2 deficiency (TK2d).

For the first time in decades, progress no longer feels theoretical. There is now something tangible to point to -- two FDA-approved therapies with the potential to reshape the lives of hundreds, if not thousands, of patients.

There is no mistaking the reality that mitochondrial disease includes hundreds of genetic variants, and these therapies address only two. But momentum matters. The snowball is rolling. Finally, the focus isn't on the approval of a therapy; it's on the approval of a therapy for every single patient.

UMDF's work moving forward must be guided by ensuring a timely diagnosis, world-class care, and effective therapies for every person affected by mitochondrial disease. In the year ahead, a new strategic plan will outline how UMDF will continue advancing this work.

We look forward to showing you the future of UMDF.

Sincerely,

Kristen Clifford
UMDF President and CEO

A message from the **Chair**

I cannot imagine a more exciting time to serve as UMDF's new Chair of the Board of Trustees.

For years, progress was incremental. I think we all could sense the change the last few years. The science was getting better. And now, finally, therapeutic development is catching up.

As UMDF marks its 30th year of service to the community, you'll see us reflecting on how far we've come. But even in moments of pride, the work ahead never strays far from our mind. Until every patient has access to the care and therapies they deserve, our work continues.

Despite that focus, I would be remiss if I didn't take a moment to thank everyone who got us to this point -- and those who will do even more in the future.

To the patients who selflessly participate in trials; to the caregivers who give up so much of themselves for others; to the researchers who dedicate their lives to unlocking the secrets of this disease; to the clinicians who work tirelessly to help us live and thrive; to the UMDF staff that keeps us laser focused on our mission; and to the army of volunteers and donors who fuel the UMDF mission. On behalf of the entire mito community, thank you.

Todd and daughter Maddie Lacey at Mito Med Conference

Todd **Lacey**
Chair, Board of Directors

Todd Lacey
Chair, Board of Directors

2025 YEAR IN REVIEW

Kristen Clifford Named UMDF President and CEO

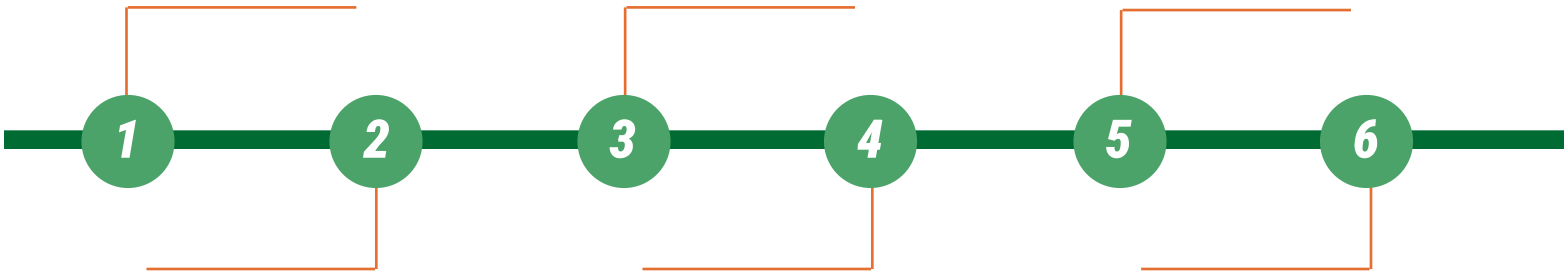
In January, Kristen Clifford was named UMDF President and Chief Executive Officer. Kristen came to UMDF with decades of non-profit leadership experience, including her most recent work as Chief Program Officer at the Alzheimer's Association where she oversaw strategic community programming that focused on increasing quality and equitable access to care for patients. "When it comes to charting a course toward treatments and cures, it's clear this community is on the precipice of incredible things. It's an honor to step into this role at such a pivotal moment. In UMDF, this community has built something extraordinary, and I look forward to working alongside the many dedicated volunteers, supporters, and staff members to carry the mission forward," she said in a statement to the community.

UMDF Launches New Clinical Trial Search Tool

As part of the organization's commitment to a "culture of clinical trials," in March UMDF announced a partnership with Carebox to launch a new clinical trials finder tool focused on mitochondrial disease. Beyond the online portal, for the first time ever, a "concierge service" – guides known as clinical trial navigators – became available to assist mitochondrial disease patients, families, and clinicians via phone. "We continue to hear from families that interpreting eligibility criteria is a major barrier to clinical trial enrollment. We're extremely excited to launch this new platform, which we think will ease many of the problem points when families look for trials," said UMDF's President and CEO Kristen Clifford. The tool had logged more than 2,000 search sessions by the end of 2025.

New UMDF TikTok Channel Debuts

In the spring, UMDF launched a new TikTok channel – @mitotok_umdf. Tricia Melland (@tmelly231), a prominent TikTok influencer and affected patient who boasts nearly 350,000 followers, helped UMDF announce this new launch at Mito Med's Evening of Energy.



UMDF Partners with MitoWorld on #BeyondTheDisease

In February, in an effort to keep the community updated on the latest in mitochondrial science, UMDF began partnering with MitoWorld, a non-profit organization dedicated to "bringing focus to the latest research and emerging fields associated with mitochondrial research," for a new feature called #BeyondTheDisease. The monthly #BeyondTheDisease series recaps the latest mitochondria research and news, along with analyzing how it could impact mitochondrial disease research.

UMDF Starts Mito Biorepository

In April, UMDF announced a partnership with nonprofit COMBINEDBrain to develop a mitochondrial disease biorepository hosted on mitoSHARE, UMDF's patient registry. Patients with any form of a genetically confirmed mitochondrial disease could participate in the biorepository by donating a blood, urine, or nasal swab sample at any of ten COMBINEDBrain roadshows across the United States, including Mitochondrial Medicine Conference 2025 in St. Louis. "A biorepository is a crucial tool to better understand a disease, develop new treatments, and work toward potential cures. Partnering with COMBINEDBrain allows UMDF to focus on patient participation and marketing, while they work on collecting, housing and distributing samples," said UMDF's Science and Alliance Officer, Philip Yeske, PhD.

Todd Lacey Named New Board Chair

At Mitochondrial Medicine Conference, Todd Lacey was voted UMDF's new Chair of the UMDF Board of Trustees. An accomplished business leader with nearly 30 years of experience leading high-growth companies, Todd serves as President of Financial Finesse, an Atlanta-based company that provides personalized financial coaching solutions. Todd joined the UMDF Board of Trustees in April 2019. "This mission is deeply personal, and I believe fiercely in the strength of this community — in what we've built together and where we're headed. The momentum in mitochondrial research, awareness, and advocacy is real. And it's growing. What once felt like distant hope is now within reach," he said at the Evening of Energy opening remarks at Conference.

Highlights of the Year

A **month-by-month** snapshot of the progress, initiatives, and major milestones that defined 2025

UMDF Genetic Testing Offerings Surpass 700 No-Cost Tests

As part of Genetic Testing Action Day in late July, UMDF announced the launch of a new Patient-Enabled No-Cost Genetic Testing Program – the organization's fourth testing program – in partnership with Probably Genetic to help shorten the diagnostic journey for patients with suspected mitochondrial disease. For the first time, the test was offered on UMDF's patient registry, mitoSHARE, enabling a seamless transition for genetic testing participants to receive curated results, connect their data to share with a clinician, participate in surveys and studies, and more. By the end of the year, the program had helped almost 100 patients further their genetic understanding via free testing, bringing UMDF's total free test total to more than 700 over four years. The program was supported in part by an unrestricted grant from UCB.

Forzinity Approved by FDA
See story on page 7

UMDF on the Road

UMDF's Support and Education team hit the road in the fall, including stops at Child Neurology Foundation for transition of care courses, the Child Neurology Society Annual Meeting, and NORD Breakthrough Summit.

UMDF Hosts First-Ever "Ask the Mito Doc" Focused on an Approved Therapy

In late November, UMDF hosted a special Ask the Mito Doc webinar with Michio Hirano, MD, of Columbia University focused on questions related to TK2d and Kygevvi (doxecitine and doxribtimine), marking UMDF's first ever webinar focused on an FDA-approved therapy.

7 8 9 10 11 12

UMDF Leads Mito Collaborative in Fight for DCA Approval

In August, Saol Therapeutics announced the U.S. Food and Drug Administration issued a Complete Response Letter (CRL) regarding the company's application for dichloroacetate (DCA) to treat PDCD. In this CRL, the FDA declined to approve DCA at this time and requested that Saol initiate a new clinical trial to address remaining questions. Four other mito-focused patient advocacy groups joined UMDF in a statement: "While we fully support rigorous scientific standards, regulatory flexibility is essential for rare disease populations like PDCD, where delays in access to potentially life-saving therapies can lead to irreversible damage – or even death. Make no mistake, that is the risk when it comes to DCA not being available for PDCD patients." UMDF would go on to support a letter from more than 80 leading scientific and medical leaders to the FDA, a change.org petition, more than 2,000 letters to Congress, and led a briefing with the U.S. House of Representatives' Mitochondrial Disease Caucus.

"Bench to Bedside" Explores Mitochondrial Replacement Therapy

Over the summer, researchers in the United Kingdom published two papers sharing promising results from eight births utilizing mitochondrial replacement therapy, a fertility technique that helps prevent the transmission of mitochondrial disease to future generations. UMDF hosted their first Bench-to-Bedside focused on the topic in September, bringing together non-profit leaders and clinicians across the globe who have personal experience with the science and political components of MRT. The technique is legal to use in clinical research in the UK and Australia, but not in the United States.

Kygevvi Approved by FDA
See story on page 7

New York Jets Player and Coach Team Up with UMDF to Support Mito Community

New York Jets Safety Malachi Moore and Jets Coaching Operations Coordinator Maddie Johnson choose UMDF as part of the NFL's "My Cause, My Cleats" campaign. As part of his support, Malachi took the field during Week 13 wearing customized UMDF cleats.

Carrie Fordyce, Patrick Mullin and family

Patrick's Story

"Patrick is hope to me," his mom, Carrie, says.

When Patrick Mullin was a baby, his parents noticed something felt off. He wasn't hitting milestones. "We just thought it was developmental delays," Carrie recalls.

Then, at age four, everything changed. One morning, Patrick woke up dragging his arm and leg. "It looked like he was having a stroke," Carrie says. They rushed him to the hospital, desperate for answers.

Until then, Patrick's tests – MRI and EEGs – had all been inconclusive. It was during this hospital stay that the family finally received answers from genetic testing: Patrick has Alpers syndrome, a mitochondrial disease caused by mutations in the POLG gene. His mitochondria, the body's energy source, run, as Carrie explains, "like a battery that's always at 18%."

Following that metabolic stroke, Patrick lost all of his motor skills. "I remember telling the physical therapist that I wanted Patrick to walk again," Carrie said. "She thought I was crazy. He couldn't even hold his head up."

And yet, Patrick walked again.

Today, Patrick is 18. He still battles seizures that strike without warning and requires constant care, medication, and monitoring. "There's always this undercurrent of what's going to happen next," Carrie admits. But even through it all, Patrick's laughter fills their home. His room is bright and colorful, reflecting the joy he brings to those around him.

He loves chocolate, slapstick comedy, and watching The Lorax, a story that holds special meaning for Carrie. Its message hangs on a canvas in their home, a daily reminder of why they keep fighting:

"Unless someone like you cares a whole awful lot, nothing is going to get better. It's not."

As Carrie says, "We haven't found a cure. We don't have a lot of treatment options. But we've done all the interventions along the way that I think have really led him to this place – and really a joyful life."

Breakthroughs in Treatments

In September, following a 20-month review with the FDA, Forzinity (elamipretide) became the first FDA-approved therapy for a form of primary mitochondrial disease – Barth syndrome. Only a few months later, Kygevvi (Doxecitine and Doxribtimine) joined the ranks of approved therapies, gaining FDA clearance to treat thymidine kinase 2 deficiency (TK2d). With two approvals now on the book, the end of 2025 saw UMDF moving into access conversations for the first time, launching website pages on medications and insurance assistance in December.

Forzinity

Forzinity (elamipretide) is a therapy administered via subcutaneous injection, typically given once daily. It is a mitochondria-targeting peptide designed to improve the function of mitochondria – the energy-producing structures within cells.

Forzinity received accelerated approval for treating Barth syndrome, an ultra-rare form of mitochondrial disease caused by mutations in the TAZ gene associated with heart problems, muscle weakness, delayed growth, chronic fatigue, and low levels of white blood cells that fight off infection. It is thought to work by selectively targeting and stabilizing a key mitochondrial lipid called cardiolipin, which plays a crucial role in maintaining mitochondrial structure and function.

"This approval represents a historic breakthrough for the Barth syndrome community and for every family affected by mitochondrial disease. For decades, families have been told there is little that can be done. Now, for the first time, we have an FDA-approved therapy that addresses the root cause of a mitochondrial disease," UMDF President and CEO Kristen Clifford said after the announcement.

Kygevvi

Kygevvi (Doxecitine and Doxribtimine) is an FDA-approved therapy for the treatment of thymidine kinase 2 deficiency (TK2d) in adult and pediatric patients with an age of symptom onset on or before 12 years.

TK2d is an ultra-rare mitochondrial disease and enzyme deficiency defined by muscle weakness, breathing difficulty, limb weakness that impairs gait or causes loss of ability to walk, droopy or saggy eyelids, and trouble chewing and swallowing. While estimating TK2d population size is challenging, the most recent literature puts the disease's prevalence at 1.64 patients per million people.

TK2d is caused by inherited mutations in TK2, a nuclear gene responsible for making a mitochondrial enzyme called thymidine kinase 2. These mutations reduce the amount and quality of mitochondrial DNA inside cells. "Too many families have had to endure the terrible burden of this disease. The approval of the first-ever therapy for TK2d is more than a medical milestone – it's a moment of hope, validation, and possibility for every patient and caregiver who has never stopped believing," said UMDF President and CEO Kristen Clifford.

Advocacy & Awareness

Advocacy News

UMDF's advocacy work on Capitol Hill was never more crucial than in 2025.

- UMDF joined the Barth Syndrome Foundation and fellow mito patient advocacy groups in appealing the FDA's decision against elamipretide approval, which included coordinating letters from the Mitochondrial Disease House Caucus, Congressional meetings with UMDF leadership, multiple letters of support from UMDF and UMDF's patient advisory council, and more than 200 emails to congressional offices from UMDF advocates. In the end, the campaign worked – leading to an approval for what is now known as Forzinity, to treat Barth syndrome in those above 30 kilograms.
- Despite political headwinds, UMDF was able to secure mitochondrial disease as an eligible topic in the Department of Defense's Congressionally Directed Medical Research Program, making mito researchers eligible for up to $150 million in federal funding. The program is estimated to have generated more than $80 million in mito research funding since inception.
- UMDF stepped up to support families in the battle over Medicaid cuts, producing a Medicaid survey and delivering results to congressional offices demonstrating how drastic changes could impact the mitochondrial disease community.
- Following the rejection of Saol Therapeutics' New Drug Application by the FDA, UMDF leapt into action, leading a mito patient advocacy group coalition that shared messaging, coordinated letters from scientists and clinicians to the FDA, issued press releases and statements, wrote op-eds, and led to over 2,300 contacts with Congressional offices.

Nearly
$80,000,000
Federal Mito Research Funding Unlocked Since 2012

8,066
Letters sent to Congressional Office by 2,197 advocates

UMDF Social Media for 2025

1,900,000+
Facebook Views

336,000+
Instagram Views

250,000+
LinkedIn Views

Discussing MRT on BBC

On July 17, UMDF Science and Alliance Officer Philip Yeske, PhD, joined the BBC's world radio news program to talk about Mitochondrial Replacement Therapy. The interview would kickstart discussions around the process stateside, with UMDF hosting a Bench-to-Bedside focused on the MRT in the fall.

Viewpoint Public Television

A Viewpoint production highlighting mitochondrial disease and UMDF debuted in August on public television channels in different markets across the country. The six-minute production featured Bonnie Kallaos, mother to Olivia, who lives with Leigh syndrome; as well as Dr. Philip Yeske, UMDF Science and Alliance Officer; and Kristen Clifford, UMDF President and CEO. This project was made possible in part by the Melissa Kieffer Family Impact Fund.

United Mitochondrial Disease Foundation

Research

Collaboration is the Key

On the research front, collaboration helped UMDF once again pass the million-dollar mark for research dollars awarded in 2025.

In January, UMDF announced it was pooling funds with the Australia-based **The Mito Foundation** to award up to $500,000 in grants focused on research and clinical work that advances cures and treatments for primary mitochondrial disease.

In December, UMDF teamed up with the **Muscular Dystrophy Association (MDA)** to co-fund more than $500,000 for two research projects to accelerate the development of promising therapies.

"Collaboration is at the heart of everything we do at UMDF, so working alongside groups like MDA and the Mito Foundation aligns perfectly with our mission," said UMDF's Science and Alliance Officer Philip Yeske, PhD.

mitoSHARE Growth Explodes

2025 also brought increased participation in **mitoSHARE**, UMDF's Global Patient Registry. More than 1,100 new patients and caregiver accounts were added for the year, an increase of 52-percent versus the previous year. Similarly, genetic test curation more than doubled and continues to rise. UMDF now has 58 different countries represented in the registry.

The platform also hosted multiple new studies, including cognition-focused MitoARCH study, and the fatigue-related Merlin study, along with all of UMDF's no-cost genetic testing offerings for the year.

mitoSHARE
WORLDWIDE PATIENT-POPULATED REGISTRY

Clinical Research Pavilion Returns

UMDF's Clinical Research Pavilion returned to Mitochondrial Medicine Conference, giving patients, caregivers, and healthy controls the opportunity to participate in research. This year, nearly 75 people participated, including 25 participants in the new UMDF Biorepository with **COMBINEDBrain**.

Funded Grants in 2025

Since 1996, UMDF has provided more than $18 million in research grants and clinical study awards, making it the leader in non-governmental funding for research aimed at discovering a faster diagnosis and effective treatments and potential cures for mitochondrial disease.

In 2025, UMDF partnered with Australia-based **Mito Foundation,** the **Muscular Dystrophy Association (MDA)** and UK-based **The Lily Foundation** and pooled resources to award over $1.1 million, which was split between ten projects focused on research and clinical work that advances cures and treatments for primary mitochondrial disease.

Investigator Grant

Caterina Garone, MD, PhD *

University of Bologna
Bologna, Italy
$100,000.00
"NanoMDS – Nanomedicines nucleotides or RNA therapeutics delivery for treating mitochondrial DNA depletion syndromes"

Investigator Grant

Christian Bergamini, PhD *

University of Bologna
Bologna, Italy
$100,000.00
"Elucidating Mitochondrial Impairment in Troyer Syndrome - EMITS"

Investigator Grant

Nan-Kai Wong, MD, PhD *

Columbia University
Columbia, NY
$100,000.00
"Unraveling the Effects of SSBP1 Mutation on Vision"

Investigator Grant

Norma Frizzell, PhD *

University of South Carolina Columbia, SC
$100,000.00
"Anaplerotic odd-chain fatty acid therapy for the treatment of Leigh Syndrome"

Clinical Trial Readiness Grant

Nikita Konstantinoskiy *

Jellyfish Bio, Inc.
Dover, DE
$50,000.00
"Validating Digital Functional Assessments in Mitochondrial Diseases: A Multinational Smartphone and Wearable Validation Study"

Clinical Trial Readiness Grant

Florence Van Tienen, PhD *

Maastricht University
Maastricht, Netherlands
$32,531.76
"Optimizing cryopreservation of muscle stem cell medicinal product for direct clinical administrationin Mitochondrial Myopathy patients"

Postdoctoral Grant

Anastasia Dimitriou, PhD *#

University of Northumbria at Newcastle
Newcastle upon Tyne, UK
$50,000.00
"Monoclonal antibody therapy for Leigh Syndrome"

Postdoctoral Grant - Family Funded

Keri-Lyn Kozul, PhD

Washington University
St. Louis, MO
$50,000.00
"Therapeutic degrader molecules targeting excessive mitophagy in FBXL4- and PPTC7-associated Mitochondrial Disease"

Over
$1,100,000
Research Grants Funded or Co-Funded in 2025

More than
$18,000,000
Research Funds Awarded since 1996

10
Funded or Co-Funded Research Grant in 2025

(*) denotes grants co-funded by Mito Foundation

(+) denotes grants co-funded by MDA

(#) denotes grants co-funded by The Lily Foundation

Funded Grants in 2025

Mariena D'Aurelio, PhD +
Weill Medical College of Cornell University
$299,973.00
"Modulating ER stress-induced lipotoxicity to ameliorate mitochondrial myopathy"

Alba Pesini Martin, PhD +
Columbia University Irving Medical Center
$210,000.00
"Investigating MAM domains and neuronal function in Coenzyme Q10 deficiency"

Pretzel Therapeutics Added to the Mito Fund

In June, The Mito Fund added to its portfolio by investing in **Pretzel Therapeutics**, a Massachusetts-based company with research facilities in Mölndal , Sweden, focused on harnessing cellular energetics to modulate disease processes and improve survival, function and quality of life for a breadth of conditions.

PX578, the lead development program in Pretzel's bioenergetics portfolio, recently entered Phase 1 clinical development in the U.S. The therapy's focus is restoring function to mutant mitochondrial DNA polymerases, allowing repopulation of depleted mtDNA, and offering a promising approach for the treatment of mitochondrial DNA polymerase disorders (commonly known as POLG disorders) and other severe conditions linked to mtDNA depletion.

The Mito Fund now has holdings in four companies: **Khondrion**, **Napigen**, **Pierrepont Therapeutics** and **Pretzel Therapeutics.**

Aneesa Licorish, Jeremiah Gracen and family

Dr. Iyer's Story

Meet Dr. Shilpa Iyer, a mitochondrial disease researcher with the University of Arkansas and member of the United Mitochondrial Disease Foundation's (UMDF) Scientific and Medical Advisory Board.

"The future is very, very bright when it comes to mitochondrial disease science," said Dr. Iyer. "We've come so far. We're finally at a stage where we can develop diagnostics and therapies."

That Dr. Iyer is working in the mitochondrial disease space, let alone known as one of the world's leading researchers for diseases like Leigh syndrome and Leber's hereditary optic neuropathy, she chalks up to a chance -- but life-changing -- conversation.

As a PhD student at the University of Georgia, Dr. Iyer's studies focused largely on telomeres, the caps on chromosomes whose dysfunction are often associated with cancer. Those efforts would eventually land her a postdoctoral research position at the University of Virginia (UVa), where she worked on gene therapy for Parkinson's. Her then mentor, Dr. James Bennett, suggested she learn more about the connection between mitochondria and telomeres and encouraged her to attend UMDF's Mitochondrial Medicine Conference.

It was there, at breakfast more than 15 years ago, she found herself sitting next to a young patient living with mitochondrial disease and his parents. "My heart turned that morning. I wanted to bring a smile to the parents' faces," she said.

That introduction became a pivotal moment in her career. She would go on to complete postdoctoral training in mitochondrial genetics at UVa and subsequently serve as a research faculty at the Center for the Study of Biological Complexity at Virginia Commonwealth University. There, she became actively engaged with the mito patient community.

During several patient meetings, advocates asked if she would develop stem cell models. The proposal she crafted in response would make her one of the first recipients of mitochondrial disease research funding from the Department of Defense's (DOD) Congressionally Directed Medical Research Program. The program had only added mitochondrial disease to its list of priorities the year prior, largely due to UMDF advocacy work on Capitol Hill.

The $1.4 million award from DOD would kickstart an illustrious career in mitochondrial disease research – and the creation of a newly formed Laboratory of Cellular Energetics and Mitochondrial Disorders at the University of Arkansas. All from a happenchance conversation at breakfast.

At another UMDF Conference, she recalled having a conversation with the mother of a son affected by Leigh syndrome saying, 'When my son Milo closes his eyes, I'm afraid he might not open them again.' So I began to think, how can I help Milo? How can I bring this child energy?"

"We started looking for biomarkers across the multi-organ system impacted in mitochondrial disease in order to develop targeted therapies," she said, that led to another proposal which would bring in millions more in federal funds.

Today, she is known as one of the world's leading scientists in the mitochondrial disease space, with more than 40 published studies – including eight pieces of research focused on Milo's disease, Leigh syndrome. With recent funding from the Eunice Kennedy Shriver National Institute of Child Health and Human Development, her work is focused on better understanding the complexities of mitochondrial diseases in different organ systems using stem cell models.

Her work continues today, still as patient-centric as ever. Her office wall is full of photographs of patients she has connected with through the years.

"It's exciting," said Dr. Iyer. "My biggest hope is we can move the field forward. With all the enthusiasm, together we can make a difference."

Education & Support

The year 2025 was unlike anything UMDF's Director of Support and Education Kara Strittmatter had ever seen.

As UMDF's first employee, she has unique perspective. "In the early years, it was a shoestring budget, doing whatever we could to move the ball forward. It's incredible to see all the progress we've made over the years," she said.

From those early beginnings, Kara has seen UMDF's Support and Education team grow to three full-time employees – and an army of 110-plus Support Ambassadors -- who run hundreds of events, ranging from small disease specific groups all the way up the 700-person Mitochondrial Medicine Conference.

Following approvals for multiple therapies, the UMDF team quickly shifted into "access" mode which saw three team members undergo RACE training – Rare Access and Coverage Education – via the Little Hercules Foundation in preparation for assisting patients with insurance coverage.

233

Total Support and Education Meetings Hosted in 2025

4,300+

Total Meetings Attendees in 2025

500,000

Cumulative Hours of Education and Support Programming *(estimated)*

733

Total Course Enrollments in Mito U ("Teachable")

Hayden and Gavin Huggans

Mitochondrial Medicine Conference

Mitochondrial Medicine 2025 was held at the Hyatt Regency At The Arch in St. Louis, MO, June 16-20, 2025.

UMDF Hosts First Ever **Mito Masterclass**

For the first time ever, the 2025 Mitochondrial Medicine Conference hosted a Masterclass for the science and medical community -- Primary Mitochondrial Myopathy with a Focus on TK2d -- with two tracks for early career investigators and up-and-coming clinicians.

The clinician track focused on sharpening clinical skills, expanding knowledge of Thymidine kinase 2 deficiency (TK2d), a rare genetic mitochondrial disease, and primary mitochondrial myopathies, and help with the tools to provide better, more personalized care for patients. The investigator track was tailored to meet the unique needs of early career investigators in mitochondrial medicine, providing expert insights, valuable networking opportunities, and practical strategies for success in academia and industry. The program was funded via a medical education grant by UCB.

To encourage attendance, UMDF provided over a dozen young science and medical professionals full scholarships to the conference.

"As a junior clinician who wants to go into the mitochondrial medicine space, I had a great time. You get to learn from clinicians, hear about ongoing research, and active clinical trials," said Burak Altintas, a Resident Physician at Washington University at St. Louis, about Masterclass.

The program is now available for continuing medical education on UMDF's MitoU platform at umdf.org/seminars.

Dr. Phil Yeske at Mito Med Conference

650+
Total Attendees

230+
Science & Medical Program Attendees

300+
Patient, Family & LHON Program Attendees

80+
Mito Med Sessions

14
Exhibitors

Conference Reactions

Voices of Family Program Attendees

"This conference, of all I have been to, had a comraderie that was beautiful. I have never felt so connected to other families."

"Hearing my daughter say it was like a summer camp for medically complex people… She felt normal and accepted and hated that it was over."

"The conference was overwhelming and emotional in all the right ways."

"As a first-time attendee, I feel more empowered. I learned that treating the symptoms is not enough – but to look for the cause."

Conference Survey Results

94%

of Scimed attendees rated 2025 Mitochondrial Medicine Conference as *Great (4/5)* or *Excellent (5/5)*

97%

of Scimed attendees said hearing patient stories and testimonials was a *Somewhat Impactful (4/5)* or *Very Impactful (5/5)* element of Conference

90%

of Scimed attendees said Conference *Helped Foster Collaboration* or *Formed New Connections* in their field

Masterclass Survey Results

100%

of clinical attendees surveyed *Felt More Confident* about diagnosing primary mitochondrial myopathy thanks to Masterclass

100%

of clinical attendees surveyed said they *Would Attend* a similar UMDF event

100%

of research attendees surveyed said they *Would Recommend* the Masterclass to a colleague

80%

of research attendees surveyed said Masterclass *Would Help Them Collaborate with Industry Partners*

Energy for Life Walks

In 2025, our dedicated volunteers and Regional Coordinators planned and hosted live and virtual Energy for Life Walkathons across the United States. Our Energy for Life Walkathons spread awareness and created a community for those affected by mitochondrial disease.Teams and individuals fundraised through a variety of ways, including social media, personal asks, letter and/or email campaigns or hosting wrap-around fundraising events.

13

Walks

1,550

Attendees

145

Total Teams

3,460

Donors

$433,000

Raised

Award Winners in 2025

Every year, UMDF honors members of the mitochondrial disease community's efforts.

Paul **Lore**

Stanley A. Davis Leadership Award

Paul became affected by Leber Hereditary Optic Neuropathy later in life, in his fifties. He is an LHON Support volunteer as well as a UMDF Support Ambassador – providing a listening ear and guidance in self-advocacy to patients in both the LHON and greater mitochondrial disease patient communities.
umdf.org/the-stanley-a-davis-leadership-award/

Dr. Lee-Jun **Wong**

Vanguard Award

Thie UMDF and the Scientific and Medical Advisory Board were honored to present, in memoriam, the 2025 Vanguard Award to Dr. Lee-Jun Wong for her incredible dedication to the fields of mitochondrial medicine and science. Former colleague Dr. Fernando Scaglia gave a moving tribute to Dr. Wong and her work. Dr. Wong's daughter and son, Dr. Hansie Wong and Inyork Wong, accepted the award on her behalf.
umdf.org/the-vanguard-award/

Daniel **Barsook**

LEAP Award

Daniel was diagnosed with MERRF syndrome (myoclonus epilepsy with ragged-red fibers) as a young adult. A UMDF Support Ambassador since 2019, Daniel in his words seeks to "work with individuals impacted by mitochondrial diseases, which are devastating, to help them grow personally and have a healthy life."
umdf.org/the-leap-award/

Jack **Ebert**

Heartstrings Award

Jack will be a senior next year at Oratory Prep in New Jersey. His involvement with UMDF and the mitochondrial disease community began when his little sister, Anna, was diagnosed with Alpers syndrome in 2013.
umdf.org/the-heartstrings-award/

Jody **Thompson**

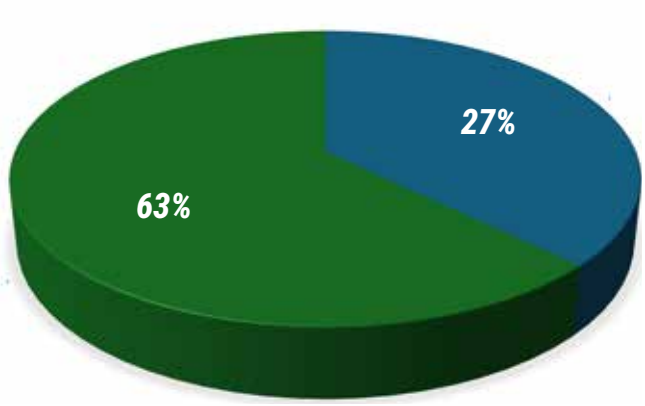
Energy Award

Jody, a mito mom from Crooksville, OH, has been a force for mitochondrial disease awareness, patient support, advocacy for mito-friendly legislation, and fundraising toward cures for over two decades. She's done it all in honor of her daughter, Baylee, who is affected by mitochondrial disease
umdf.org/the-energy-award/

Financials Overview

Unaudited, Calendar Year 2025

Elizabeth and Emma Watt

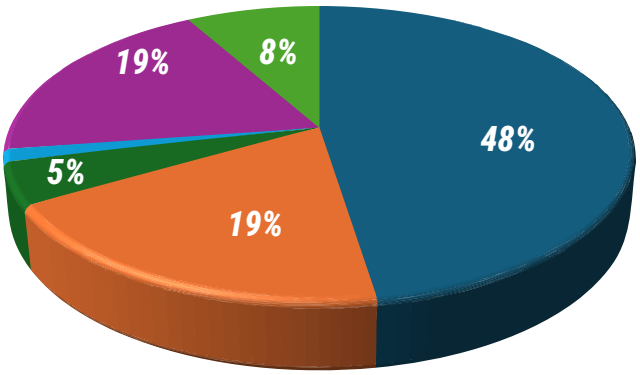


$6,646,956

Total Assets (Unaudited)

- Net Assets
- Liabilities

How **You Supported** UMDF

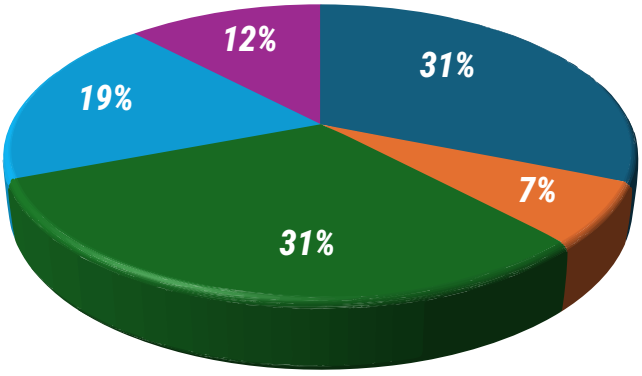


$4,277,350

Total Revenue (Unaudited)

- Contributions
- Special Events *(Net of direct costs)*
- Grants
- Grant Refunds
- Symposiums & Seminars
- Investment Income

How **UMDF Served** the Mito Community



$4,348,828

Total Support/Services Expenses (Unaudited)

- Research
- Public Awareness
- Education & Support
- Administrative & General
- Developement

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Dr. Amel Karaa, Dr. Marni Falk and Dr. Michio Hirano at Mito Med Conference

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