2025

Annual Report

UNITEDMSD FOUNDATION
MULTIPLE SULFATASE DEFICIENCY

#RelentlessHope

Our Mission, Vision & Values

Our Mission

To cure Multiple Sulfatase Deficiency.

Our Vision

We aspire for all children with MSD to lead a life without limitations.

Our Values

- **Compassion—**We lead with care, respect, and empathy.
- **Inclusion—**We endeavor to foster a culture of belonging while actively working to remove barriers, biases, and discrimination that prevent all individuals from fully participating and benefiting from our services.
- **Community**—We strive to create a sense of belonging, support, and empowerment by providing an environment for MSD families to build connections, alleviate isolation, amplify their voices, and share knowledge.
- **Collaboration**—We foster partnerships between families, researchers, organizations, and funders to advance our mission and goals.
- **Responsibility**—We recognize it is a privilege and duty to represent our families to the larger medical and policy communities, to share our families' stories with dignity and accuracy, and to steward philanthropic resources with integrity.

From the Board Chair & Executive Director

Dear Friends and Partners,

Reflecting on 2025, we are struck by how far we have traveled together. What began as collective hope has matured into a global movement defined by unprecedented growth. We share this report with deep gratitude for the community that makes our progress possible.

In 2025, the United MSD Foundation became more inclusive than ever. We now support 87 patients across 23 countries, representing half of all known MSD cases worldwide. By welcoming 13 new families and expanding our support for adult patients, we have ensured that geography is never a barrier to advocacy. Whether through monthly meetings or shared milestones, our community's spirit has never been more resilient.

Our hope is now fueled by a thrilling sense of urgency. We are no longer speaking of "someday"—we are preparing for now. With our first gene therapy clinical trials targeted for Fall 2026, every milestone reached this year has brought us to the threshold of a new era in treatment.

While we celebrate this growth, we remain grounded in the legacy of the lives that have shaped our mission. Their strength fuels our determination to move faster. A cure is no longer a distant dream; it is a destination we are approaching with confidence.

Thank you for your unwavering commitment.

Whether you are a donor, researcher, or family member, you are vital to this journey. Together, we enter 2026 with the collective power to change the future for everyone living with MSD.

With hope, gratitude, and determination,

Rich Schega
Board Chair, 2025

Sarah Cortell Vandersypen
Executive Director

Bespoke Gene Therapy Consortium

2025 Accomplishments: Bridging Science and Hope

Building on the momentum of previous years, 2025 has been a transformative chapter for the MSD project and the Bespoke Gene Therapy Consortium (BGTC). This year, the collaboration between the National Insittutes of Health (NIH), Foundation for the NIH, research partners, and the United MSD Foundation moved us closer than ever to the clinic as years of preparation began to take tangible form. In a massive coordinated effort, the consortium finished manufacturing our AAV9 gene therapy while simultaneously finalizing the clinical trial protocol and drafting the Investigational New Drug (IND) application. Throughout this technical process, the Foundation worked alongside these experts to ensure the patient voice remains the guiding force, elevating the hopes of our families within this groundbreaking public-private partnership.

The energy within the consortium is palpable as we stand on the threshold of our most significant milestones. Every batch manufactured and every document drafted represents a promise kept, signaling our steady approach toward the start of clinical trials. We are moving closer to the day this therapy reaches our patients. We remain profoundly grateful for the expertise of our NIH and industry colleagues and the unwavering support of our donors. Together, we are no longer just preparing for the future— we are bringing it to life.

> ***Throughout the years of careful lab work and getting ready for a trial, we have prioritized scientific rigor, patient safety, and patient-informed trial design. As we ask for permission to move forward, it’s important that the next steps are carried out with strong oversight, close monitoring, and a focus on doing right by patients.***
>
> *Rebecca Ahrens-Nicklas, MD, PhD*
> *Director, Gene Therapy for Inherited Metabolic Disorders*
> *Frontier Program*
> *The Children's Hospital of Philadelphia (CHOP)*

BGTC 2025 Milestones

Major Manufacturing Milestones Achieved

- Pilot batch executed (process validation)
- Reliability & safety insights generated
- GMP clinical batch completed (December)
- Entered quality control testing

Finalizing Trial Infrastructure

- Clinical Trial Agreement adopted
- Trial activation framework established
- TORO finalized (regulatory oversight + safety governance)

Patient-Centered Clinical Trial Design

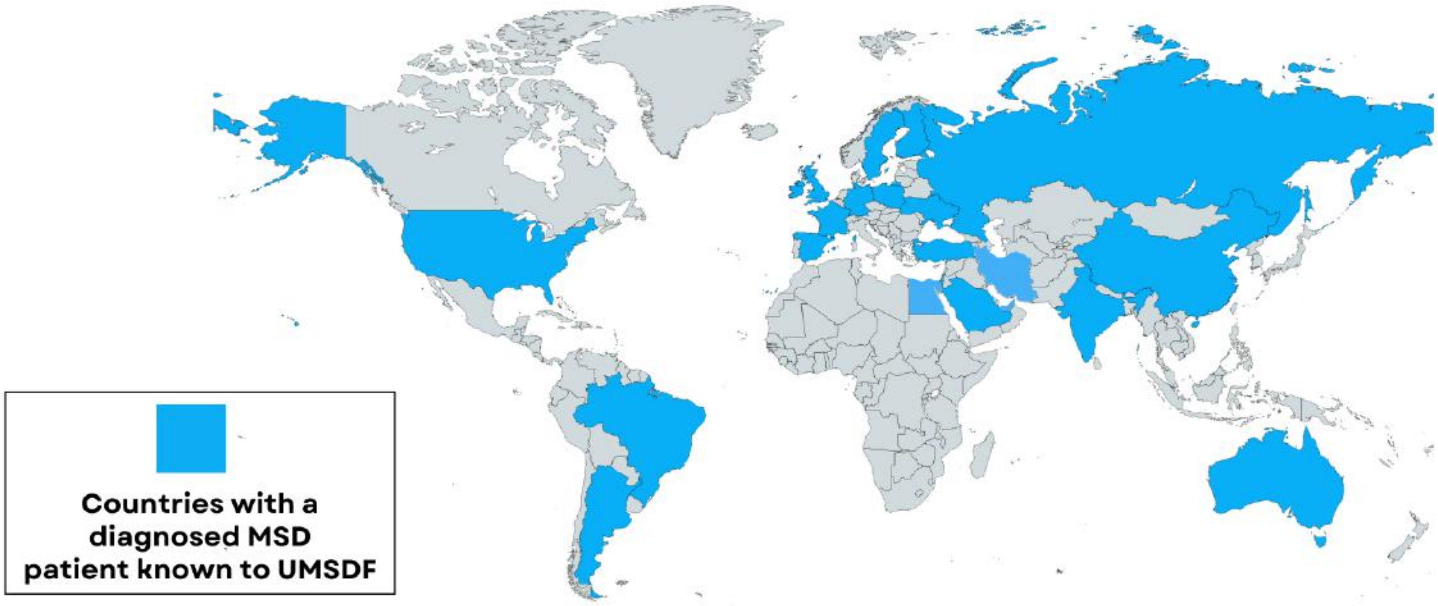
- Protocol finalized with patient input
- Patient & family perspectives integrated
- Designed to reduce patient burden

Expanding Our Global Family Support

Supporting families remains the heartbeat of the United MSD Foundation's mission. In 2025, that heartbeat grew stronger as the Foundation expanded its reach to support **87 patients** across **23 countries**, representing **12 primary languages**. Since its inception, the Foundation has now served half of all known MSD cases worldwide, a milestone that underscores our role as a global anchor for the community. This year also marked a significant diversification of our patient population with the inclusion of three adult cases, including one attenuated patient and two individuals living with *SUMF1*-associated non-syndromic retinal dystrophy.

This growth was matched by the deepening of our ongoing family support, led by Patient and Family Advocate Brenna Bentley. Throughout 2025, we welcomed **13 new families** into the fold, immediately bridging the gap between diagnosis and action by connecting them with medical experts and providing resources tailored to their specific needs. Our commitment to the community extended far beyond the initial onboarding process; we continued to walk alongside every family through monthly support meetings, birthday celebrations, and heartfelt remembrances of those we have lost.

To ensure that no barrier—geographic or linguistic—could hinder access to care, we integrated professional interpreters into every onboarding session and event for our non-English speaking families. By translating complex research opportunities and providing quarterly check-ins in a family's native tongue, we have moved closer to a world where every MSD family has a seat at the table and the support they deserve.



Connecting and Empowering Families Online

AMGEN Supports Launch of Family Portal

This year marked the launch of the Foundation's new Family Portal, made possible through a #RAREis Global Advocate Grant from AMGEN. This secure, dedicated online space provides families with access to trusted resources, educational materials, and personalized updates related to MSD. It also fosters meaningful connection by enabling families to share experiences and support one another within a private, global network.

Stay Connected

Connect with other MSD families and get access to valuable information from medical researchers and doctors.

MSD Families now have access to this exclusive section of the Foundation's website (main page shown above), designed to be a source of connection and support, hosting a wealth of information including care guidelines and research updates.

Family Support Meetings: Navigating the MSD Journey Together

In addition to the Family Portal, the Foundation continues to provide ongoing family support through educational programming, research updates, and community-building initiatives designed to reduce isolation and empower caregivers. These efforts ensure that families have access to the information, resources, and connections they need throughout their journey.

The 2025 Family Support Meetings included topics such as "Bespoke Gene Therapy Update," "Navigating Educational Supports," and "Leukodystrophy and Vision." The calendar is evenly divided between medical care talks, research updates, psychosocial support, and social sessions, allowing families to pick and choose what they are most interested in.

2025 Impact Numbers

11
Family Support Group meetings

23
Countries where supported families live

35
Metabolic clinics contacted with MSD resources

13
New connected and engaged families

12
MSD World Day proclamations

18
Student Ambassadors

125
Volunteers

> *My sole goal now is to make sure Max has the best life he can possibly live. MSD has taken so much from us, but there is one thing it cannot touch: our love.... No matter the progression of Max's symptoms, he will always happily hold your hand, or sometimes, even give a smile. In a world where there is no cure for MSD yet, let endless love be our cure for now.*
>
> -Valerie Daniels, Mother of Max (age 14)

Supporting Adult Patients

Meet Breanna: First Attenuated Adult Patient, Identified in 2025

Breanna is a thoughtful, resilient young woman whose circuitous path to an MSD diagnosis reflects both the complexity of this ultra-rare disease and the strength of families who persist in searching for answers. Raised by her adoptive mother, who spent years advocating tirelessly on her behalf, Breanna grew up experiencing a wide range of unexplained medical challenges that affected nearly every aspect of her daily life. In 2022, after progressive vision changes led to advanced ophthalmologic evaluations and genetic testing, Breanna was finally diagnosed with MSD through the identification of variants in the *SUMF1* gene.

Although MSD is more often associated with childhood presentations, Breanna is among a small but growing number of individuals diagnosed in adulthood, underscoring the likelihood that additional attenuated patients may remain undiagnosed or misdiagnosed for years.

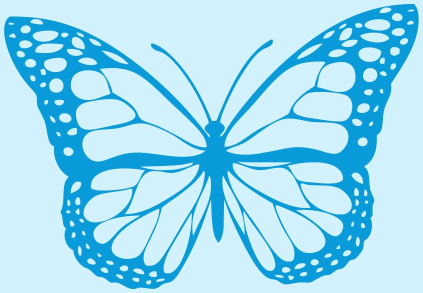
While attenuated adult MSD cases like Breanna’s remain exceptionally rare, stories like hers are helping expand understanding of the disease’s variability and progression.

Breanna’s journey, which you can read more about here, reinforces the urgent need for greater physician awareness and earlier diagnosis to help families avoid prolonged diagnostic odysseys, and receive support sooner.

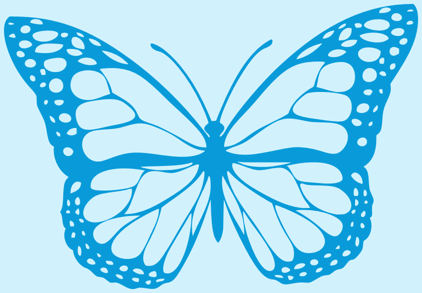
> ***Meeting with other MSD families, I find myself thankful for where I am in life. My journey has been long and difficult, full of searching for answers, but finally, we have answers. While my experience is unique, I am still connected by the same hunger for more information and the hope of a future treatment. My hope is that my story can generate awareness of the full spectrum of MSD and prevent other patients and families from spending years asking questions with never any answers. I hope something positive comes of my case and that it helps others, too.***
>
> -Shelly Breanna Rose

In Memoriam

"And we wept that one so lovely should have a life so brief."
-William Cullen Bryant



March 20
Dylan Finglas
Age 13



July 12
Alma Ballauz
Age 11

October 3
Baylor Scotting
Age 6

Student Ambassadors: Turning Awareness into Action

In 2025, the United MSD Foundation's Student Ambassador Program continued to grow as a powerful force for awareness, education, and community-building. Through both collaborative initiatives and individual projects expanding the Foundation's capacity, these students extended the Foundation's reach into new communities, on campuses, in clinics, and across the globe.

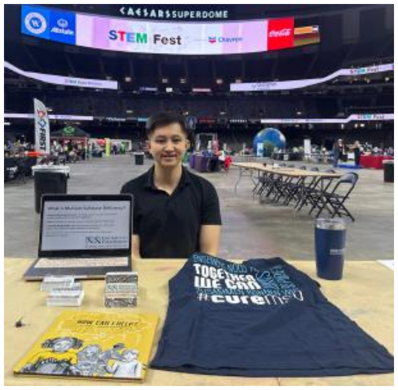
- ✓ 18 Ambassadors • 12 States • 900 volunteer hours
- ✓ First cohort to complete a record 18 unique personal projects: community presentations; fundraisers; tabling at events; & writing a children's book!
- ✓ Research + provider outreach support
- ✓ 170 pediatric neurology clinics mapped
- ✓ 145 metabolic clinics reached
- ✓ Family outreach projects including Rare Connections cards
- ✓ 7 country-specific flyers
- ✓ 3 building lightings • 10 proclamations
- ✓ & 6 new Family Stories shared!

Personal Projects with Purpose

At the heart of the Student Ambassador program were 18 personal projects, each reflecting the creativity and dedication of the students behind them. Below are some highlights of what the Student Ambassadors were able to accomplish in their year-long commitment working with the Foundation.

Michael Yang (first photo) tabled at Caesar's Superdome in New Orleans, as part of STEM Fest 2025. He gave a presentation on MSD to raise awareness of this ultra-rare disease. **Emily Sandlin** (second photo) reached some of the largest audiences all year, presenting on MSD and genetic counseling to ~200 high school and college students, to raise awareness and inspire future advocates. **Lianna Schubert** held a Zebra Run Pop-Up (pictured in the third photo) at her college, the New Jersey Institute of Technology, with 15 runners and even more donations, raising $360!

In 2025, **Malaika Vaz** wrote and began work with an illustrator on her newly published (2026) children's book (pictured at far right). Designed for siblings of those with rare diseases, ***Baby Brother: A Rare Sibling Adventure***, offers families a powerful tool to communicate, connect, and navigate complex emotions together. You can now order your own copy **here**!



A Global Blue Wave: MSD World Day

Every July 30, the world unites for MSD World Day, creating a powerful "Blue Wave of Hope" to accelerate the path toward a cure. In 2025, this movement reached new heights, combining record-breaking advocacy with the deep local roots that sustain our mission.

Thanks to your overwhelming generosity, we raised over $10,500 for life-changing research and family support programs. A highlight of the day was our high-energy "Power Hour" social media campaign; your momentum generated nearly $3,000 in just sixty minutes—an impact instantly doubled through a generous matching gift.

Rooted in Community: Ocean Springs Giveback

This year featured a meaningful homecoming with the launch of the Ocean Springs Giveback Day. Celebrating the Foundation's origins, local businesses and residents rallied in a powerful show of solidarity.

This spirit of partnership was further exemplified when Executive Director Sarah Cortell Vandersypen addressed the Rotary Club of Ocean Springs (pictured above). Building on the Club's longtime support, Sarah shared our latest clinical progress with these steadfast advocates, reinforcing that while our reach is global, our strength remains anchored in the Ocean Springs community.

A Growing Movement

Advocacy reached a new milestone as **12 states** officially recognized July 30 as MSD World Day. To mark the occasion, landmarks worldwide were bathed in blue light, including **Coral Gables City Hall** (FL), the **Peace Bridge** on the U.S.-Canada border, the **Mary C. O'Keefe Cultural Arts Center** in Ocean Springs, and **Tarleton State University** (TX), all shown below. This movement continues to bridge the gap between complex science and the families who inspire our work.

Jackson Labs decorated their office window with handprints from each team member.

Blue Wave Laboratory Door Decorating Contest

The inaugural **Blue Wave Door Decorating Contest** added a vibrant new dimension to our 2025 MSD World Day celebrations, transforming laboratory halls across the globe into canvases of hope. This initiative bridged the distance between our families and the front lines of science, reminding us that behind every breakthrough are dedicated individuals pouring their hearts into a cure. Our international research partners competed for the most inspiring "Blue Wave" display, with the global MSD community casting their votes via social media to crown a champion.

We extend our deepest gratitude to the following teams for their incredible artistry and unwavering commitment to the MSD community:

- Children’s Hospital of Philadelphia (CHOP) Leukodystrophy Center, led by Dr. Laura Adang
- Children’s Hospital of Philadelphia (CHOP) Metabolic Disease Program, led by Dr. Rebecca Ahrens-Nicklas
- The Jackson Laboratory, led by Dr. Maximiliano Presa
- UMC-Göttingen University, led by Dr. Lars Schlotawa
- UT Southwestern Viral Vector Facility, led by Dr. Steven Gray

Congratulations to our 2025 Winner! Dr. Laura Adang and her team at the CHOP Leukodystrophy Center took home top honors for their exceptional creativity. They are pictured above with their “Fairy Door Trophy,” a bespoke award designed by our Patient and Family Advocate, Brenna Bentley, who envisioned and coordinated this inspired new tradition.

Zebra Run for Rare Disease

On March 29, 2025, a record-breaking **445 participants** gathered for the 8th Annual Zebra Run, our cornerstone event for community and impact. This year's run reached a historic milestone, raising over **$116,000** to directly fund gene therapy research and vital family programs. From the chip-timed 5K to the all-ages fun run, the day was fueled by the dedication of sponsors, families, and advocates committed to racing toward a cure for MSD.

Expanding the Herd

The momentum extended beyond our local tracks in 2025 with the first Zebra Run Pop-Up in Overland Park, Kansas. Hosted by Board Member Brandy Brockus, this regional expansion introduced a new community to our mission. Brandy shared her personal connection to the Foundation's founding story and provided updates on our progress toward clinical trials, proving that the "Zebra" spirit of rare disease advocacy continues to gain speed across the country.

- **348 in-person participants!**
- **34 virtual participants in 20 states!**
- **59 event sponsors!**

2025 SPONSORS *($500 and Above)*

Allergy, Asthma, & Immunology
AlphaCare Urgent Care
Angela Wolf Financial
Anonymous
Armand Place LLC
Butch Oustalet Foundation
Cadence Bank
Chevron
Chuck Kelly Salon Spa
Citizens Bank
Coast Aesthetics
Community Bank
Descher McDonalds
Don Blaize
Douglas and Pamela Roll
Elliott Homes/New Star Homes
EXIT Realty Heart Properties
Eye Associates of the South
Gulf Coast Human Resources Association
Gulf Coast Silk Screening
Holcomb Wealth Management
Ingalls Shipbuilding
In Loving Memory of Lola Rose
Keesler Federal Credit Union
Marcus Lee MD
McMurphy Family Dentistry
Memorial Health System
Mississippi Power Company
Mosaic Ministries
PT Solutions
SAAD HealthCare
SCI, Inc.
Singing River Federal Credit Union
Singing River Health System Foundation
Stein LTC
The First Bank
To The Rescue Bookkeeping, LLC
Dr. Van Wurm
Victory Behavior Center

Meet Remy: Rare, *but Wrapped in Hope*

> *When you find out how rare [MSD] is, you feel alone. But finding the United MSD Foundation showed us what's possible. There's a big, supportive community striving for progress. With every year, we hope this will be the year of the clinical trial.*

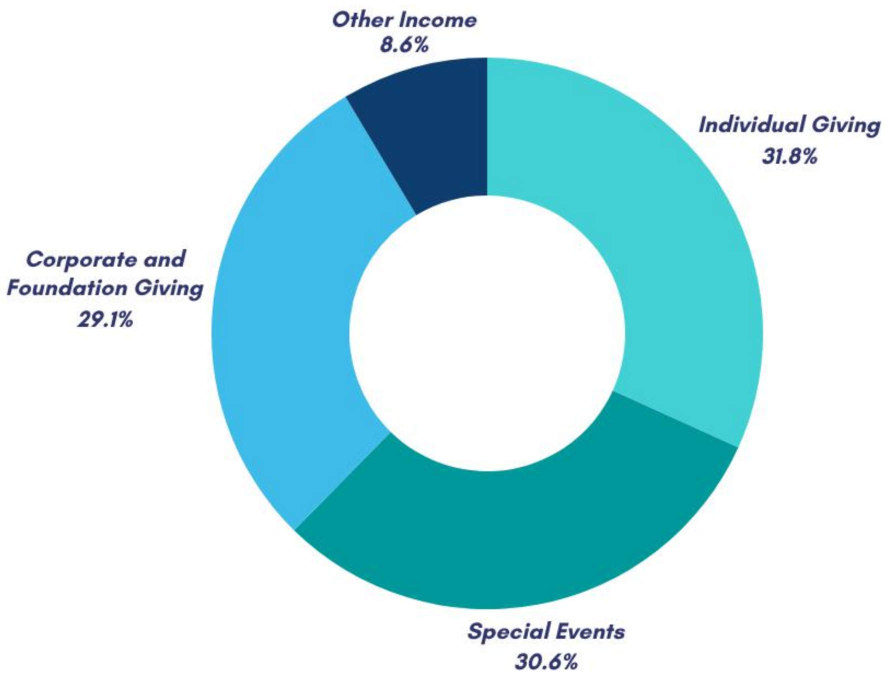
Natalie Sheppard, Mother of Remy Levine (age 4)

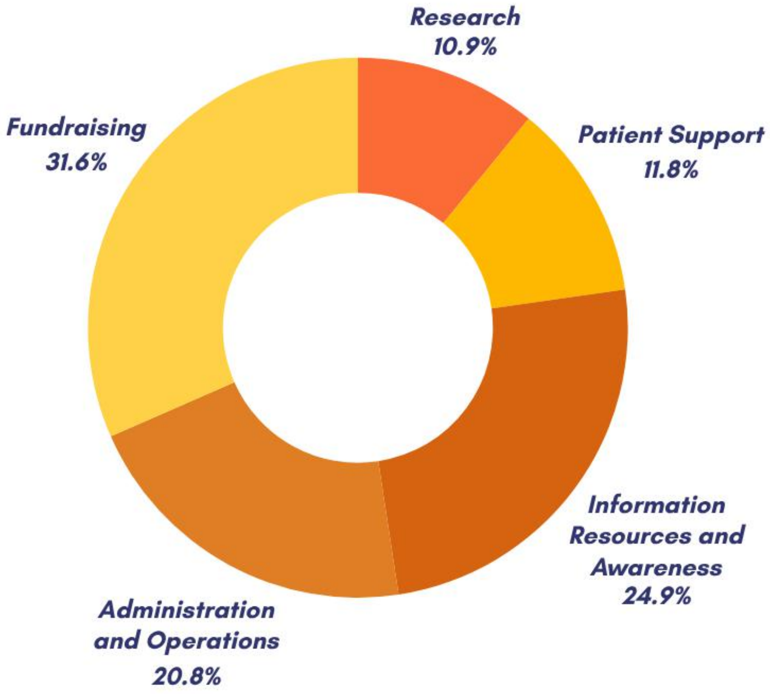
Read more about Remy here!

2025 Financial Overview

Revenue	Amount	%
Individual Giving	103,330	31.7
Special Events	99,436	30.5
Corporate & Foundation Giving	94,932	29.2
Other Income	27,925	8.6
2025 Total Revenue	**$325,623**	

Expenses	Amount	%
Research	40,539	10.9
Patient Support	43,763	24.9
Information Resources & Awareness	92,029	11.8
Administration & Operations	77,048	31.6
Fundraising	116,930	20.8
2025 Total Expenses	**$370,308**	


REVENUE


EXPENSES

Board & Staff

Board of Directors

- Rich Schega, President
- Renee Goodin Elliott, Past President
- Eryn Marchiolo, Secretary
- Roy Taylor, Treasurer
- Brad Ball, MBA
- Brandy Brockus
- Rochelle Colburn
- Michelle Fox
- Sophia Grise
- Amanda Haidet-Philips, Ph.D.
- George Loukatos, MD
- Sue Rokaw
- Julie Rosner-Lengele
- Jacki Thornburg
- Angela Wolfe

Staff Members

- Brenna Bentley, Patient and Family Advocate
- Devon Byrd, Patient Research Coordinator
- Sarah Cortell Vandersypen, Executive Director
- Ellery Crews, Development & Operations Specialist
- Margaret Fish, Bookkeeper
- Felicity Nesham-West, Marketing & Communications Specialist (started June 16, 2025)

Sister Foundations

MSD ACTION FOUNDATION SAVINGDYLAN.COM

MSD Action Foundation/SavingDylan.com ***(Est. 2014)***

Dublin, Ireland

FUNDACIÓN CURE MSD

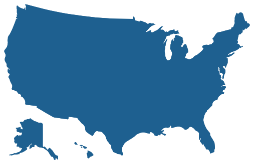
Fundacion Cure MSD ***(Est. 2017)***

Buenos Aires, Argentina

#IVET unacura MSD ivet

Cura MSD ***(Est. 2019)***

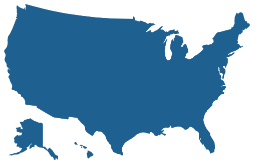
Cataluna, Spain



Grant Us Grace

Grant Us Grace ***(Est. 2019)***

Missouri, USA



STRENGTHAN A NONPROFIT FOUNDATION IN HONOR OF ETHAN

strEngTHAN ***(Est. 2020)***

Miami, Florida, USA

Stay connected

facebook.com/curemsd

instagram.com/curemsd

linkedin.com/company/cure-msd/

youtube.com/@curemsd

x.com/curemsd

Sign up for email updates, newsletter and more

Get involved

Learn more about how you can be part of our mission to #CureMSD by visiting www.CureMSD.org

Save lives

Take action and make a donation today to create a brighter future for patients with MSD.

Gold
Transparency
2025
Candid.

UNITED MSD
FOUNDATION
MULTIPLE SULFATASE DEFICIENCY

P.O. Box 806 | Biloxi, MS 39533
info@unitedmsdfoundation.org | t: (228) 327-6916 | www.curemsd.org

NORD®
2025 MEMBER
PLATINUM