2024
Annual Report

UNITEDMSD FOUNDATION
MULTIPLE SULFATASE DEFICIENCY

#TogetherWeCan

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,

As we reflect on the past year, we are filled with both gratitude and hope. 2024 has been a year of significant strides in our mission to cure Multiple Sulfatase Deficiency. From groundbreaking advancements in gene therapy to the heartwarming connections forged at our International MSD Scientific and Family Conference, we have witnessed the power of community and the relentless pursuit of a cure.

We are particularly excited about the progress of the Bespoke Gene Therapy Consortium. The execution of manufacturing contracts for the plasmid and vector is a monumental achievement, bringing us closer than ever to a potential treatment for MSD. This progress, coupled with the vital research shared at our international conference and the expansion of our biobank, fuels our optimism for the future.

While we celebrate these accomplishments, we also remember the precious lives we've lost. Their memories inspire us to continue our work with renewed determination. We are deeply grateful for the unwavering support of our families, researchers, donors, and volunteers. Together, we are building a brighter future for those living with MSD. We are hopeful that the clinical trials on the horizon will bring us closer to our ultimate goal: a cure for MSD.

With hope and gratitude,

Renee Goodin Elliott
Board Chair

Sarah Cortell Vandersypen
Executive Director

Bespoke Gene Therapy Consortium

2024 Accomplishments

Since the Bespoke Gene Therapy Consortium awards were announced in May 2023, the MSD project team has been hard at work on this groundbreaking project, and the progress is truly inspiring! We've laid a solid foundation for our clinical trial, with crucial toxicology studies wrapped up and a deeper understanding of the disease gained through our ongoing natural history study.

Looking ahead, we're actively engaging with the FDA to explore ways to expedite manufacturing and get this life-changing therapy to patients sooner. Every milestone achieved is a testament to the dedication and collaborative spirit of the team, and we're filled with optimism for the future.

We are excited to continue to advance our efforts to launch the MSD AAV clinical trial. While the vector is being produced, we are hard at work finalizing the clinical trial design in preparation for asking the FDA for permission to launch a trial.

Rebecca Ahrens-Nicklas, MD, PhD
Director, Gene Therapy for Inherited Metabolic Disorders Frontier Program
The Children's Hospital of Philadelphia

IND Preparation Milestones

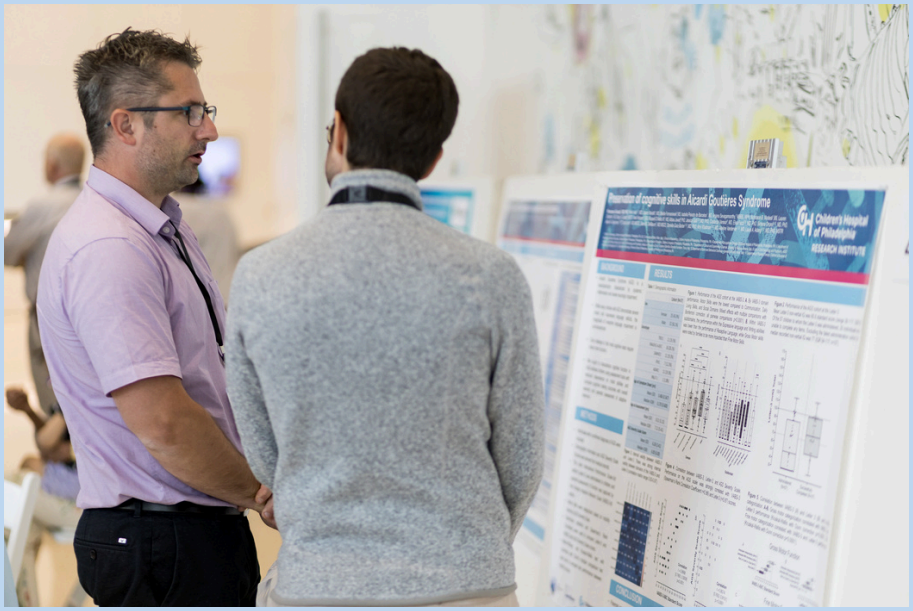
- Pivotal Animal Toxicology Study concluded – remaining laboratory work completed
- Continued Prospective Natural History Study in collaboration with UMSDF
  - Allowed better understanding of systemic burden of the disease
  - Will be used to inform clinical trial safety monitoring
- Clinical protocols being finalized
- Rare Pediatric Disease and Orphan Drug Designations granted August/September 2024
- December 2024, requested meeting with FDA to discuss allowances to accelerate drug manufacturing and release timeline to start clinical trial sooner

Manufacturing Milestones

- Plasmid Manufacturer contract fully executed March 29, 2024
- AAV Vector Manufacturer contract fully executed August 1, 2024
- Plasmid production in-progress – a critical component to allow initiation of AAV Vector manufacturing
- AAV pilot batch production to initiate February 2025 – to be used for lot-to-lot comparability testing needed for IND

International MSD Scientific & Family Conference

From August 1-3, 2024, we had the pleasure of co-hosting the **2024 International MSD Scientific & Family Conference** at the Children's Hospital of Philadelphia (CHOP). We were incredibly fortunate to have researchers from around the world in attendance to share MSD-related research progress. With 16 platform presentations and 14 posters, attendees were able to learn more in-depth about the progress our research community has made and open up discussions on the future of research and collaboration.



Thank you to our generous conference sponsors

Grant Us Grace
McMullan/O'Connor Family Fund
Kathleen and John Ewen
Victoria Anderson
The Wawa Foundation
Ultragenyx

Dr. Laura Adang of the Children's Hospital of Philadelphia received the 2024 Thomas Dierks Award for Outstanding Contribution to MSD Research.

MSD families had the opportunity to speak directly with researchers, doctors, and other experts on several topics, including new research, the current status of various clinical trials, and care management. On the last day of the conference, we held a remembrance event that honored the lives of our MSD Angels with a moment of silence, sharing of memories, and hope and encouragement for the future. During the event, pinwheels with the names of the children that we had lost were placed into the ground in the garden area at the Children's Hospital of Philadelphia. This was our first official remembrance event and was live-streamed on Facebook.

We enrolled our first carriers into the UMSDF Biobank. A total of nine individual carriers joined the Biobank, contributing blood and urine samples to advance our understanding of MSD. The Carrier Campaign is focused on identifying and studying carriers within affected families, which will enhance our knowledge of the genetic basis of MSD and support early detection, genetic counseling, and family planning.

"We flew to the US for the conference full of fear and anxiety. We returned full of hope that not all was lost, that there is a chance for an effective drug that can change the fate of children afflicted with this cruel disease."

~ Robert Ryczek, MSD Father

Click the YouTube icon to watch Highlights Video

The Zebra & The Bear

The documentary film *The Zebra & The Bear*, filmed over seven years, premiered its first in-person screening at the MSD Conference. The film chronicles a mother's determined seven-year journey to raise millions of dollars and spearhead the development of a groundbreaking gene therapy to save her daughter from a devastating ultra-rare disease.

Following the screening, attendees participated in a special panel discussion featuring UMSDF founder Amber Olsen, filmmaker Pat O'Connor, and Dr. Steven Gray, moderated by Executive Director Sarah Cortell Vandersypen.

"An eye opening film... beautifully filmed and produced ... inspiring, incredibly important, and difference-making."
- Therese, Student

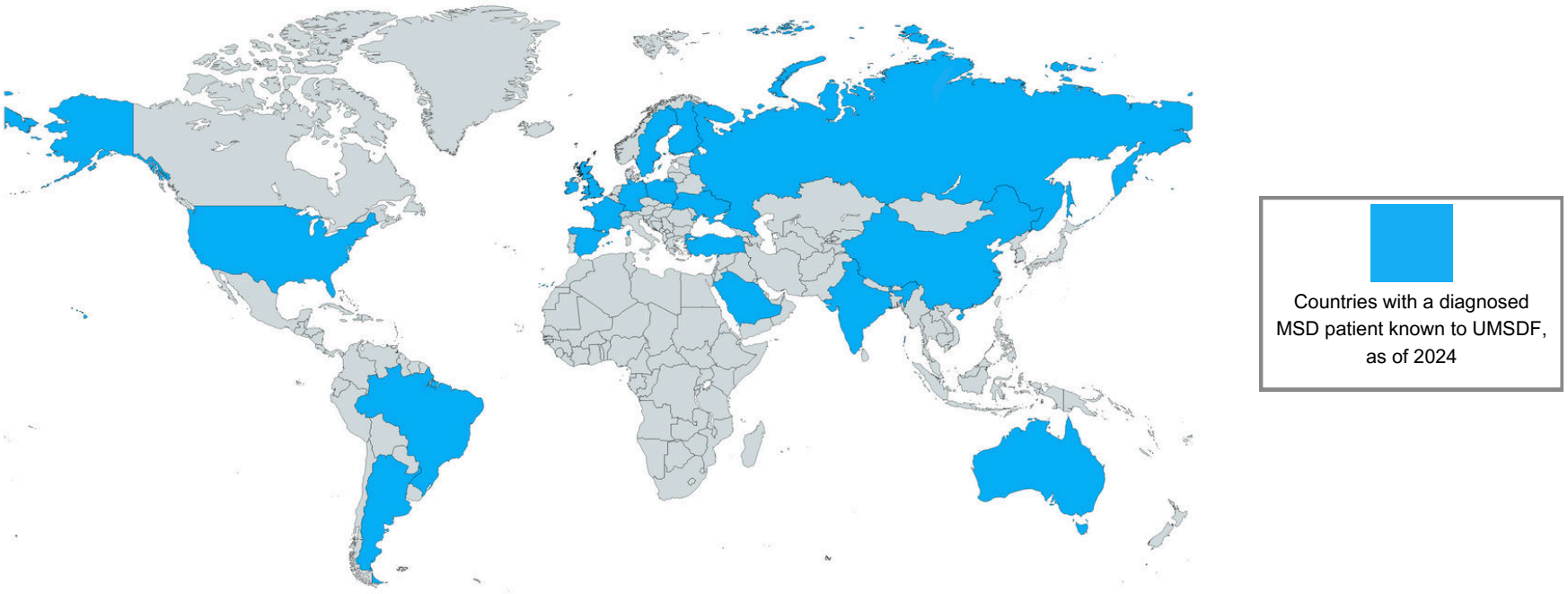
Family Support

In 2024, the United MSD Foundation supported roughly 70 families across 19 countries. This includes onboarding new families to the greater MSD community, connecting them with medical experts and local resources, pairing them with a mentor family, and providing tailored resources.

All families are supported through monthly family support meetings, birthday celebrations, remembering those we have lost, providing research opportunities, and quarterly check-ins. All non-English speaking families were provided with interpreters for onboarding and events, breaking down barriers and increasing accessibility to materials.

The 2024 Family Support Meetings included topics such as “Self-Care as a Caregiver,” “Gastrointestinal Considerations,” and “Introduction to the UMSDF Patient Registry.” The calendar includes medical care talks, research updates, psychosocial support, and social sessions, enabling families to choose relevant topics.

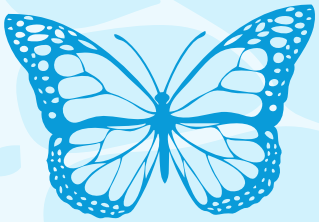
In response to a request by the Family Outreach Committee, the Foundation launched a new monthly newsletter for MSD families. This newsletter has important updates and valuable resources.



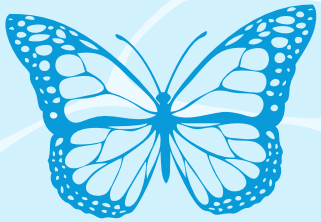
In Memorium

And we wept that one so lovely should have a life so brief.

-William Cullen Bryant



April 22
Ivet Perés
Age 8



May 5
Jaxon Chavez
Age 4

October 5
Willow Cannan
Age 11

December 7
Kenzie Masters
Age 29

Willow Abigail Cannan

August 21, 2013 - October 5, 2024

Willow Cannan, the then two-year-old daughter of Tom Cannan and Amber Olsen, was diagnosed with a terminal disease on May 9, 2016, called Multiple Sulfatase Deficiency (MSD).

After Willow's diagnosis, Tom and Amber were determined to fight this disease that had no treatment or cure. In 2016, after meeting with researchers, doctors, and other parents with children with MSD from all over the world, Amber established the United MSD Foundation with a mission that was close to her heart: cure Multiple Sulfatase Deficiency.

Because of Willow, thousands of children may have the chance to live a fulfilling life free from MSD. Her diagnosis led to the creation of the United MSD Foundation and a potential pathway to a cure for this terminal disease. Willow's voice will continue to speak through the work and support of all MSD parents, advocates, and researchers worldwide.

That is her legacy.

> ***We love her so much and will miss her terribly, but are so excited for her to be free of her MSD body and able to do what all kids should be able to do.***
> ***- Amber, Willow's mom***

When we received Oliver's MSD diagnosis, we didn't know where to turn. Our genetic counselor had only briefly heard of it in school, much less ever seen the diagnosis. Finding the United MSD Foundation, the resources it had for us and our doctors, the hope it provides for our family, has made all the difference in our family's fight to help Oliver. United we can #cureMSD."

- Jessica Hise, Oliver's mom

2024 Impact Numbers

12
family support group meetings

19
countries where supported families live

35
metabolic clinics contacted with MSD resources

5
new connected and engaged families

12
MSD World Day proclamations

30
student ambassadors

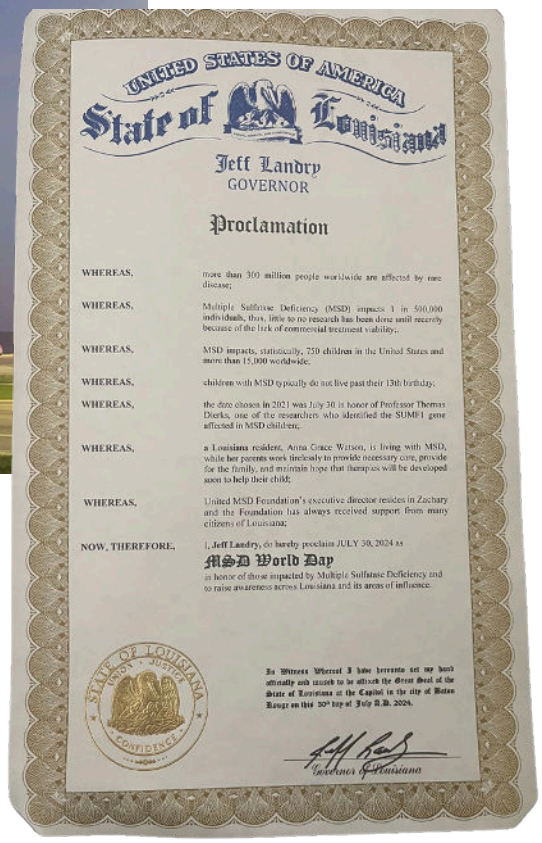
118
volunteers

The United MSD Foundation to us is a glimmer of hope amongst the turmoil.
- Kirsty McMullan

MSD World Day

MSD World Day, celebrated annually on July 30, is a day dedicated to raising awareness and support for those living with Multiple Sulfatase Deficiency. With the help of the Foundation's Student Ambassadors, we raise awareness of MSD through state proclamations and building illuminations.

This year, 12 states recognized July 30 as MSD World Day, an increase of 6 additional U.S. state proclamations from the year prior. Our sister foundations across the world participate in their awareness campaigns, which include building illuminations, events, and social media posts. Together, we strive to bring global attention to our children and their fight for a future.



UNITED STATES OF AMERICA

State of Louisiana

Jeff Landry
GOVERNOR

Proclamation

WHEREAS, more than 300 million people worldwide are affected by rare disease;

WHEREAS, Multiple Sulfatase Deficiency (MSD) impacts 1 in 500,000 individuals, thus, little to no research has been done until recently because of the lack of commercial treatment viability;

WHEREAS, MSD impacts, statistically, 750 children in the United States and more than 15,000 worldwide;

WHEREAS, children with MSD typically do not live past their 13th birthday;

WHEREAS, the date chosen in 2021 was July 30 in honor of Professor Thomas Dierks, one of the researchers who identified the SUMF1 gene affected in MSD children;

WHEREAS, a Louisiana resident, Anna Grace Watson, is living with MSD, while her parents work tirelessly to provide necessary care, provide for the family, and maintain hope that therapies will be developed soon to help their child;

WHEREAS, United MSD Foundation's executive director resides in Zachary and the Foundation has always received support from many citizens of Louisiana;

NOW, THEREFORE, I, Jeff Landry, do hereby proclaim JULY 30, 2024 as

MSD World Day

in honor of those impacted by Multiple Sulfatase Deficiency and to raise awareness across Louisiana and its areas of influence.

In Witness Whereof I have hereunto set my hand officially and caused to be affixed the Great Seal of the State of Louisiana at the Capitol in the city of Baton Rouge on this 30th day of July A.D. 2024.

Governor of Louisiana

State of South Carolina

Governor's Proclamation

WHEREAS, multiple sulfatase deficiency (MSD) is an extremely rare genetic disorder affecting, for the most part, the brain, skeleton, and skin; and

WHEREAS, MSD is characterized by deficiencies in the activation of enzymes in the cells that break down fats and sugars, thereby inhibiting the body's ability to get rid of cellular waste in the body, resulting in toxic levels of fats and sugars in many tissue and organ systems; and

WHEREAS, according to the United MSD Foundation, an estimated one in 500,000 individuals worldwide are affected by the disorder, including approximately 750 children in the United States; and

WHEREAS, the average life expectancy for children with MSD is thirteen years; and

WHEREAS, there are no approved therapies to reverse the effects of MSD at this time, with current treatments involving a multidisciplinary team providing supportive therapies and management for specific symptoms.

NOW, THEREFORE, I, Henry Dargan McMaster, Governor of the great State of South Carolina, do hereby proclaim July 30, 2024, as

MSD WORLD DAY

throughout the state and encourage all South Carolinians to help raise awareness of this disorder and recognize the individuals and families affected by MSD, the healthcare providers providing treatment, and the organizations and researchers seeking improved treatment protocols and a possible cure.

HENRY MCMASTER
GOVERNOR
STATE OF SOUTH CAROLINA

Zebra Run for Rare Disease

The 7th annual Zebra Run for Rare Disease was held on Saturday, March 2, 2024. This annual fundraiser not only raises critical funds to support the mission of the Foundation but also serves as a powerful community gathering, uniting individuals, families, and advocates touched by rare diseases.

In 2024, former Foundation board member, GiGi Burk hosted a Zebra Run Pop-Up in New Orleans to raise awareness and funds in that community. Participants heard of GiGi's connection to the Foundation's founder and the progress toward clinical trials.

$116,311 Raised

348 in-person participants

14 New Orleans participants

34 virtual participants in 20 states

59 event sponsors

2024 SPONSORS *($500 and Above)*

Allergy, Asthma, & Immunology
AlphaCare Urgent Care
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EXIT Realty Heart Properties
Eye Associates of the South
Gulf Coast Human Resources Association
Gulf Coast Silk Screening
Holcomb Wealth Management
Ingalls Shipbuilding
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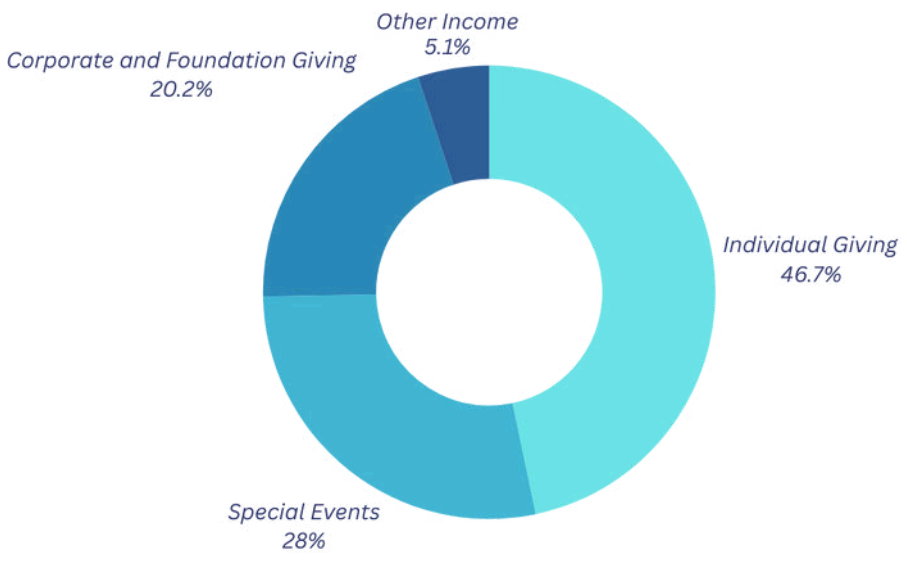
Singing River Health System Foundation
Stein LTC
The First Bank
To The Rescue Bookkeeping, LLC
Van Wurm
Victoria Anderson
Victory Behavior Center

2024 Financial Overview

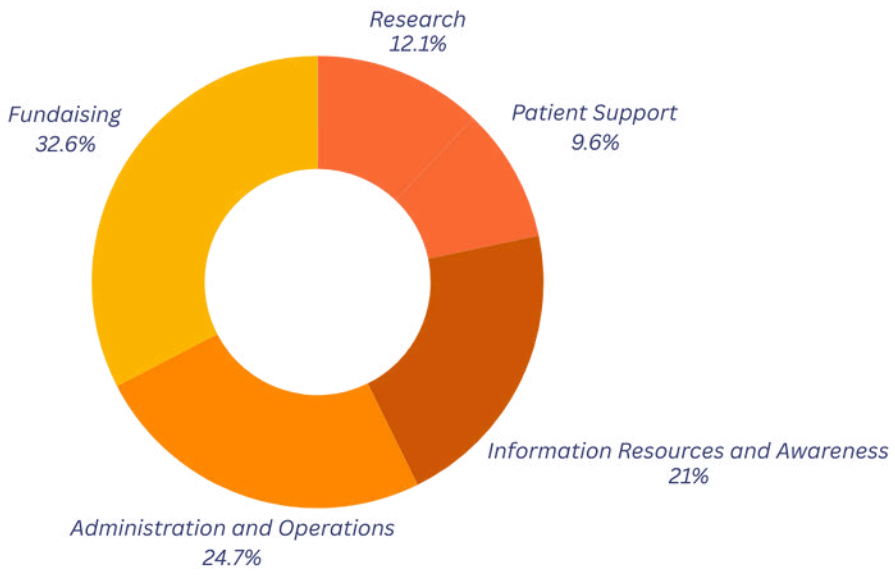
Revenue	Amount	%
Individual Giving	153,782	46.7
Special Events	92,338	28.0
Corporate & Foundation Giving	66,719	20.2
Other Income	16,674	5.1
2024 Total Revenue	**$329,515**	

Expenses	Amount	%
Research	44,651	12.1
Patient Support	35,481	9.6
Information Resources & Awareness	77,707	21.0
Administration & Operations	91,919	24.7
Fundraising	120,808	32.6
2024 Total Expenses	**$370,273**	

REVENUE



EXPENSES



Board & Staff

Board of Directors

- Renee Goodin Elliott, President
- Rich Schega, Vice President
- Hope Blankenship, Treasurer
- Eryn Marchiolo, Secretary
- Brad Ball
- Amanda Haidet-Philips
- George Loukatos
- Julie Rosner-Lengele
- Jacki Thornburg

Staff Members

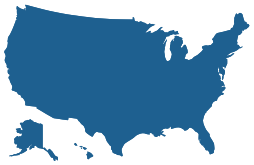
- Brenna Bentley, Patient and Family Advocate
- Devon Byrd, Patient Research Coordinator
- Vince Caperelli, Jr, Marketing and Communications Manager (through June 7)
- Sarah Cortell Vandersypen, Executive Director
- Ellery Crews, Development and Operations Specialist
- Margaret Fish, Bookkeeper

Sister Foundations

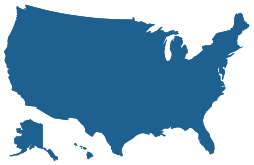
MSD Action Foundation/SavingDylan.com *(Est. 2014)*
Dublin, Ireland

Fundacion Cure MSD *(Est. 2017)*
Buenos Aires, Argentina

Cura MSD *(Est. 2019)*
Cataluna, Spain



Grant Us Grace *(Est. 2019)*
Missouri, USA



strEngTHAN *(Est. 2020)*
Miami, Florida, USA

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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 children with MSD.

Gold Transparency 2024 Candid.

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NORD 2024 MEMBER PLATINUM