

2023 Annual Report



#TogetherWeCan

Our Mission, Vision & Values

As part of our strategic planning process that culminated in July 2023, United MSD Foundation affirmed our mission as stated, revised our vision statement, and created new organizational values.

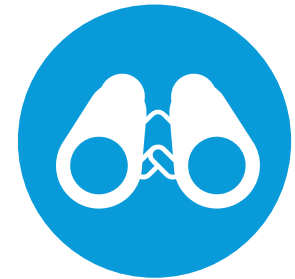


Our Mission

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.

Honoring Our Founder

After leading the Foundation since its inception, Amber Olsen formally stepped away in May 2023.

I reached out to Amber directly on May 22, 2016, after she was added to our Facebook family group. This was when her daughter was diagnosed with MSD. I had arranged a meeting with a group of Lysosomal Storage Disease (LSD) experts at an LSD conference in Cambridge, UK, in July 2016, and I convinced Amber to come to this meeting also. I am very grateful that Amber and Tom came to that meeting. Turning up was a superb show of commitment. When my wife Michelle and I met with them, I was sure that they believed we were on the road to doing something huge for MSD patients. I was delighted when they indicated they were going to set up a charity in the USA to advocate for MSD research. Also, finally, we were not alone in the pursuit of research. Amber made huge strides in getting conversations going with researchers and keeping the chain of communication open, along with travelling to many conferences and university hospitals to meet researchers. I encouraged Amber to attend WORLD Symposium with me in February 2017 in San Diego. This was where Dr. Sean Ekins told me and Amber that he felt that Dr. Steven Gray was the best gene therapy scientist in the USA and that we should get him in the boat for MSD. Amber followed this advice to the point of travelling to North Carolina to meet Dr. Gray at his (then) university, and, after much persuasion she, managed to get him to agree to help in the fight against MSD. This was one of Amber's greatest achievements. The other great achievements are that she drove UMDF to raise a lot of funds for MSD research and that she developed a very sustainable model of a charity that not only supports research but that provides family support, also. This family support did not exist, to this degree, before Amber joined the fight against MSD. I will be forever grateful that Amber gave it her all to try to make a difference for MSD patient families by providing hope for a better future, as research is hope—hope that research delivers clinical trials where we hope patients can benefit and those trials can be successful and deliver treatment options for patient families across the world.

~ Alan Finglas, MSD Action Foundation / SavingDylan.com
Dublin, Ireland



We are tremendously grateful for Amber's dedication and service to the worldwide MSD community!

Path to Clinical Trial



Natural History Study Visits

The Children's Hospital of Philadelphia and the University of Göttingen are working together to address gaps identified in the previously completed meta-analysis and retrospective natural history study. This current natural history study, including patient travel, was funded collaboratively by the United MSD Foundation and the five sister foundations around the world. In total, at least 10 patients will be seen two times in either the U.S. or Germany. The two visits, 12 months apart, will allow the researchers to monitor how the disease progresses. Throughout 2023, 9 patients made their first visits (3 in Philadelphia; 6 in Germany), with second visits scheduled for 2024.



Meeting doctors who had suggestions was the best part of the trip. When discussing Baylor's bad seizure control, Dr. Adang had a med recommendation. I've never had a specialist know what to do with Baylor. It was a relief to just be his mom.

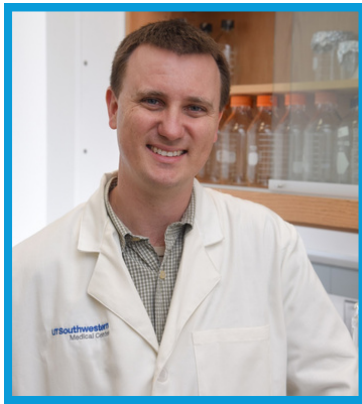
Any bit of research we can help with we will. We're committed to making sure Baylor's life helps science. When other families get this diagnosis, there should be hope, even if it's just 1%.

~ Stormi Scotting, MSD mom



Toxicology Study

In order to show safety of the AAV9 gene therapy prior to clinical trials, a toxicology study is required to test the therapy at various doses. Charles Rivers Laboratories completed the toxicology study in July 2023. The completed work on the AAV9 *SUMF1* gene therapy in rats showed acceptable safety at the therapeutic dosages. This study was funded by UMDSF and Grant Us Grace.



These favorable safety results paved a clear path to move the gene therapy treatment towards a human trial through the Bespoke Gene Therapy Consortium.

~ Dr. Steven Gray, UT Southwestern Medical Center

UMSDF Biobank

In partnership with Genetic Alliance, the United MSD Foundation houses biological specimens (blood and urine) in Fredrick, Maryland, at the Precision for Medicine Biobank. Through our self-funded biobank, UMDSF has collected and processed samples from around the world and provided them to researchers interested in identifying crucial biomarkers.

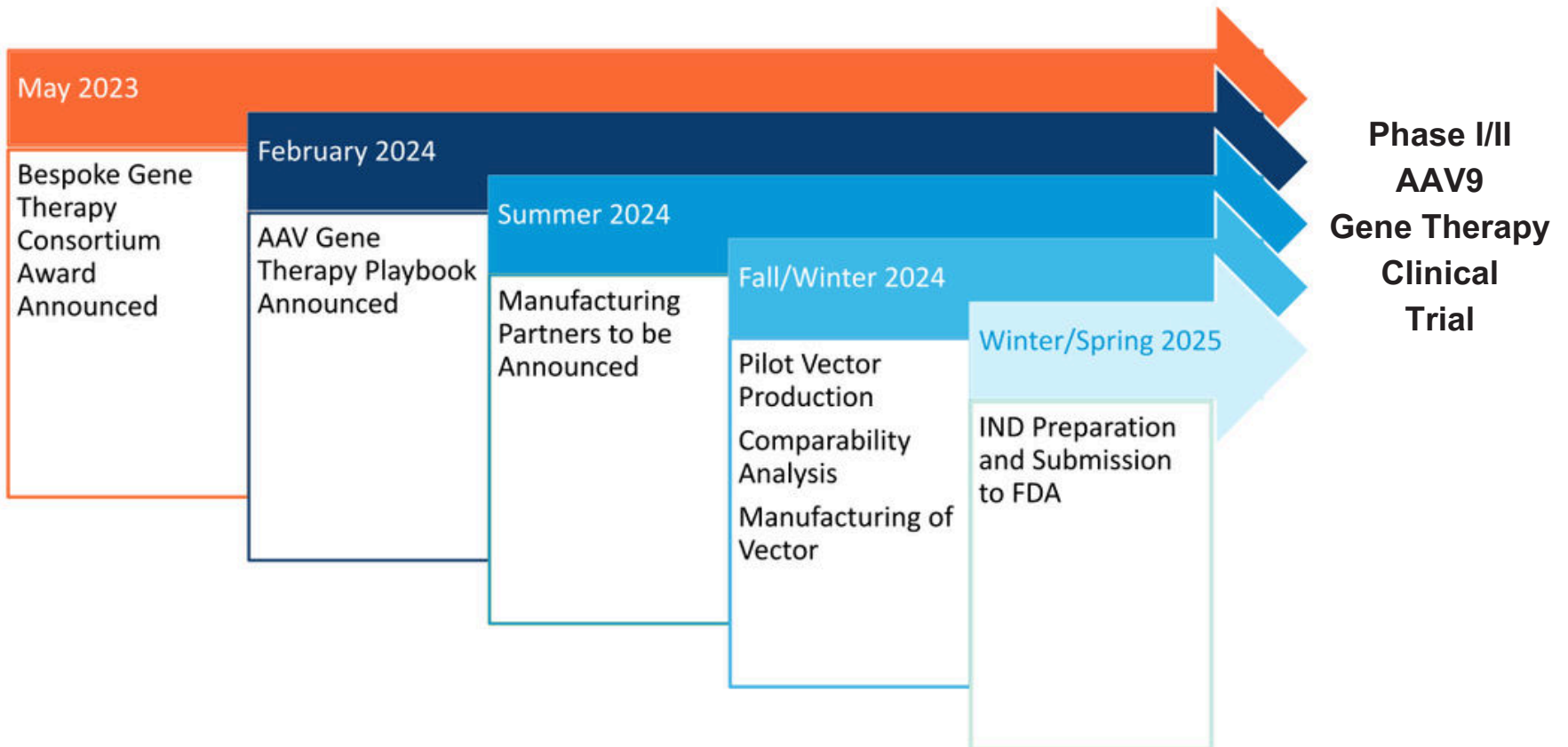
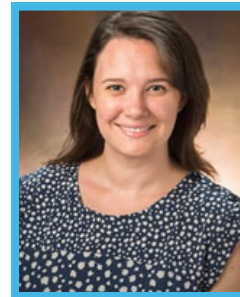
In October 2023, the MSD research team released a publication on the investigation of the patterns of glycosaminoglycans non-reducing (GAG-NREs) species in MSD patients, identifying a promising biomarker for disease severity in MSD. You can read the publication [here](#).



BGTC Award



In May, 2023, MSD was one of eight rare diseases to receive a Bespoke Gene Therapy Consortium award. This award, the result of a public/private partnership facilitated by the Foundation for the National Institutes of Health, will total more than \$4 million to fund first-in-human Phase I/II gene therapy clinical trials. Dr. Laura Adang and Dr. Rebecca Ahrens-Nicklas, both of Children's Hospital of Philadelphia, are serving as the primary investigators for the project. The United MSD Foundation has committed \$300,000 to cover the cost of patient travel.





*In a world where there is no cure
for MSD yet, let endless love be
our cure for now.*

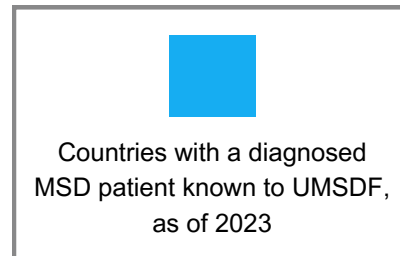
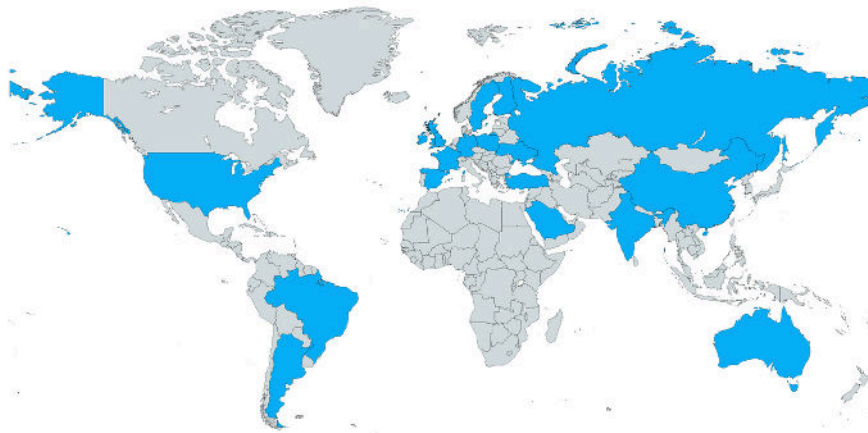
~ the Daniel family



Family Support

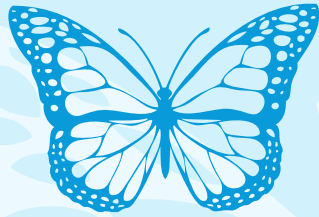
Jaxon's Story

Early in 2023, before his third birthday, Jaxon Chavez was diagnosed with MSD. His parents, Sophia Grise and Craig Chavez, were devastated after hearing what felt like the worst news in the world. After connecting with United MSD Foundation, Sophia and Craig, both educators, became actively engaged with the Foundation in order to learn more about MSD and connect with other families. In March, they created a pop-up Zebra Run event in their California community, raising more than \$30,000 toward our mission to cure MSD. *(UPDATE: Jaxon Chavez passed away on May 5, 2024.)*

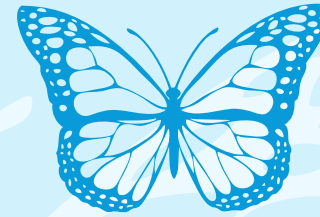


In June, we announced a new partnership with online therapy organization BetterHelp. Through this new partnership, MSD families, relatives, and caregivers around the world are eligible for 6 months of free therapy with BetterHelp credentialed professionals. We are proud to facilitate this opportunity for emotional and mental health support, as we believe that no one should carry the emotional burden of rare disease without being able to talk to and process with someone. We also hope this partnership removes any barriers to this support that might have existed.

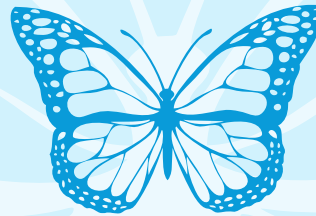
In Memorium



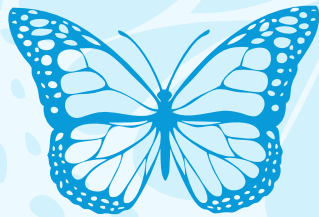
February 12
Anonymous
Age 10



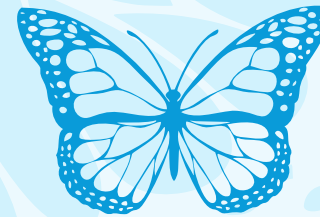
April 16
Jean-Paul Seitz
Age 16



August 4
Anonymous
Age 10



October 16
Lyric Dempsey
Age 14



December 27
Jett Burke
Age 6

#OnelsTooMany

2023 Impact Numbers

5

new connected and engaged families

124

total # of volunteers

30

student ambassadors

12

family support group meetings

countries where supported families live

18



We now feel so blessed for all that the Foundation has done for MSD. Since Anna Grace's diagnosis, there is significantly more information for families and ongoing research to one day cure MSD.

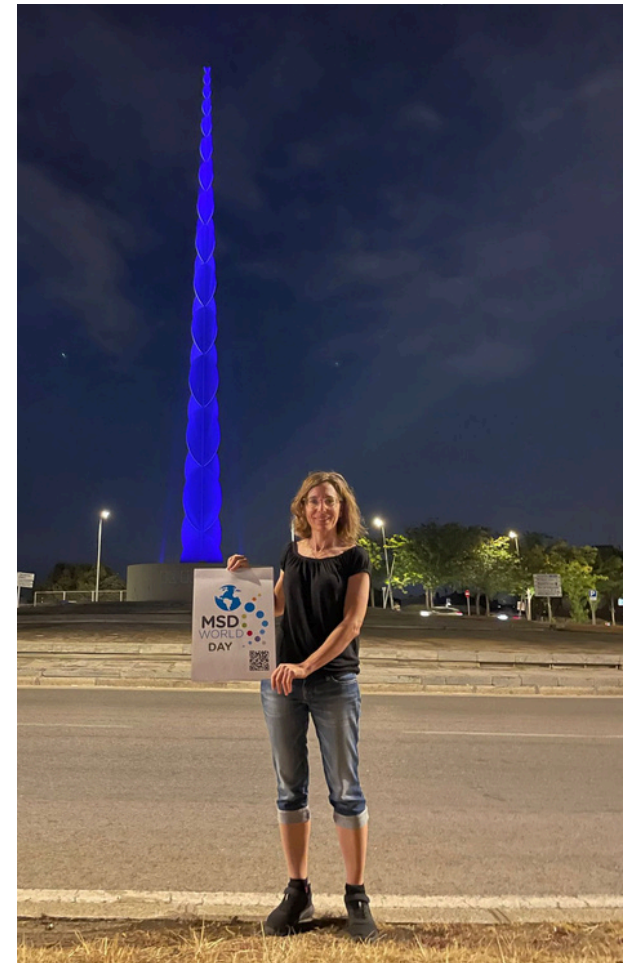
~ the Watson family

MSD World Day

When Elisabet and Fortunat learned that their youngest child, Ivet, was diagnosed with Multiple Sulfatase Deficiency (MSD) it was the worst day of their lives. The words of their physician kept echoing in their minds, “Ivet will not survive childhood.” As the youngest of five and their only daughter, the Lopez family set out to find a cure for MSD. They were elated to find they were not alone as they found three other MSD foundations around the world working toward the same goal. Since 2018, Cura MSD has worked closely with the United MSD Foundation to fight for Ivet and all children with MSD.

To raise awareness, Elisabet and Fortunat spearheaded the creation of MSD World Day. With the goals of global visibility, patient unity, and public awareness, MSD World Day now occurs annually on July 30th. In Spain, the Lopez family often raise funds through mountain hiking and yoga events. Please join us as we carry out the words of the Lopez family, “We are few, but we exist, and we will achieve the goal of curing MSD.”

United we are stronger. MSD World Day unites us, makes us visible and gives us all hope. We hope it is a happy day, where families, medical specialists and researchers think about the reason for our fight. Our joint desire is to be cured.
~ Elisabet Peres, Cura MSD



Zebra Run for Rare Disease

The 6th annual Zebra Run was a tremendous success, thanks to the Ocean Springs community, event volunteers, and our participants.

- 267 in-person participants
- 66 virtual participants in more than 40 states
- 239 Jaxon's Jog (California) participants
- Total funds raised = \$110,521

ZEBRA RUN FOR RARE DISEASE



2023 SPONSORS (\$500 and Above)

Allergy, Asthma & Immunology
Allstate - Chris Boudreaux
Alphacare Urgent Care
Angela Wolfe Financial
Armand Place LLC
Bienville Animal Clinic
Burk Brokerage
Cadence Bank
Coast Benefit Professionals
Crunch Fitness
Descher McDonalds
EXIT Realty Heart Properties

Eye Associates of the South
Gulf Coast Rehabilitation, PC
Hancock Whitney
Holcomb Financial AJH Enterprises Inc.
Huntington Ingalls Industries, Inc.
Keesler Federal Credit Union
Marcus Lee M.D.
McMurphy Family Dentistry
Memorial Hospital
Mississippi Power Community
Connection General Fund

Mosaic Church - Mosaic Ministries
Nextaff - Buffy Butler
Overwatch Supply
PT Solutions
Singing River Federal Credit Union
Singing River Health System Foundation
Stein LTC
The First Bank
The Meeting Place
To The Rescue Bookkeeping, LLC
Van Wurm
Victoria Anderson



You never know how strong you are until being strong is the only choice you have.

~ the Avery family

2023–2025 Strategic Plan

Throughout the first half of 2023, executive leadership and the board met to develop a new, multi-year strategic plan. While our mission remains steadfast, our vision was slightly modified, and the Foundation adopted a revamped set of organizational values.

The following overview notes our strategic priorities with related goals.



Strategic Priority #1: Research at Our Core

Goals—

- Help usher the start of AAV9 gene therapy clinical trials (tentative start Fall 2024)
- Complete Prospective Natural History Study by December 2025
- Launch and fully utilize the Patient Registry
- Create a Research Strategy Map by August 2024
- Create more opportunities for dialogue and collaboration between researchers



Strategic Priority #2: Supporting Families with Resources and Community

Goals—

- Develop and maintain a comprehensive online resource hub for our MSD families
- Find better ways to engage our Angel families and develop a transition pipeline for these families after their children have passed
- Create partnerships and resource lists for supports we don't currently offer
- Support international families through translation and interpretation services
- Create engagement opportunities throughout the organization for families
- Promote social connection between families



Strategic Priority #3: Growing Global Awareness of MSD

Goals—

- Make strides on Newborn Screening
- Host a successful 2024 International Scientific and Family Conference
- Gain more visibility in medical communities
- Connect with the hundreds of MSD families still unidentified
- Maximize organizational visibility, value and impact to stakeholders



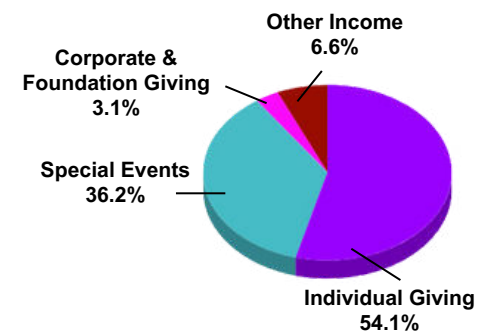
Strategic Priority #4: Setting a Foundation Through Organizational Capacity

Goals—

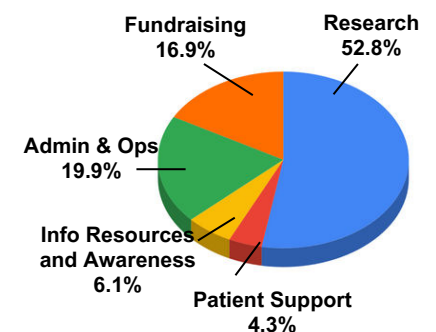
- Build an effective Board of Directors for now and in the future
- Cultivate skilled and stable personnel
- Ensure financial resources are available to support mission and strategic priorities
- Utilize volunteers to drive awareness and fundraising goals
- Optimize productivity and efficiency by focusing on effective strategies and leveraging available resources

2023 Financial Overview

Revenue	Amount	%
Individual Giving	164,963	54
Special Events	110,521	36
Corporate & Foundation Giving	9,335	3
Other Income	20,156	7
2023 Total Revenue	\$304,974	



Expenses	Amount	%
Research	353,835	53
Patient Support	28,958	4
Information Resources & Awareness	40,707	6
Administration & Operations	133,610	20
Fundraising	113,542	17
2023 Total Expenses	\$670,652*	



*surplus funds from 2022 were carried over into 2023 to pay for planned research projects

Board & Staff

Board of Directors

- John Ewen, President
- Hope Blankenship, Treasurer
- Mike Streiff, Secretary
- Brad Ball
- Sebastian Ballauz
- GiGi Burk
- Renee Goodin Elliott
- George Loukatos
- Eryn Marchiolo
- Rich Schega

UMSDF Staff

- Brenna Bentley
- Devon Byrd
- Vince Caperelli
- Sarah Cortell Vandersypen, Executive Director
- Margaret Fish
- Dawn Agar (*through June*)
- Sidney Davidson (*through February*)
- Amber Olsen (*through May*)
- Tiffany Perry (*through December*)

Parent-Led 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



MULTIPLE SULFATASE DEFICIENCY

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