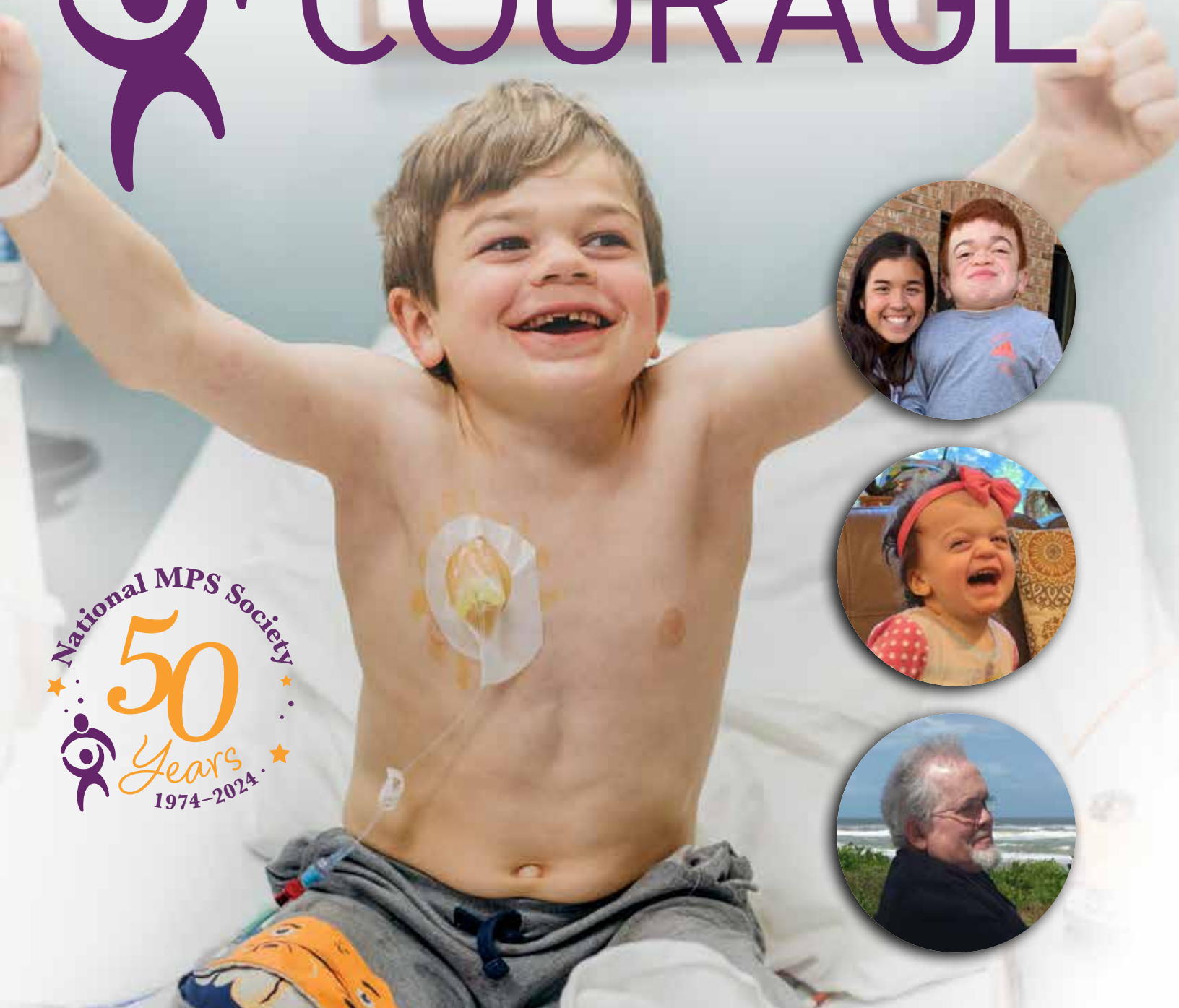




COURAGE



REAGAN-UDALL WORKSHOP

MPS biomarker significant to rare disease drug development

SAVE THE DATE

38th Annual Family & Scientific Conference
Dec. 19-21, 2024
Orlando, FL

ADVOCACY EFFORTS

Current legislation aims to facilitate access to treatments

TABLE OF Contents

- SCIENCE & RESEARCH 3
- PATHWAYS7
- FUNDRAISING 9
- FAMILY SUPPORT 14
- ADULT RESOURCE COMMITTEE 21
- ADVOCACY 22
- RESOURCES 24
- DONATIONS..... 28
- CLASSIFICATIONS..... 36
- BOARD OF DIRECTORS.....37



10 ANNUAL FUND CHAIR IS CLIMBING TOWARD HOPE
 Carl Kapes climbs to new heights to raise funds and awareness for MPS and ML



14 CROSSING PATHS PROGRAM RECOGNIZED
 The Society's travels to underserved communities receives recognition by Sanofi



16 FAMILY SUPPORT GRANT RECIPIENTS
 Families receive grants to help purchase necessary items



22 MPS AWARENESS DAY
 Two states recognize May 15, 2024, as MPS Awareness Day



MISSION
 The National MPS Society exists to cure, support, and advocate for MPS and ML.

P.O. Box 14686, Durham, NC 27709-4686
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MPSSOCIETY.ORG

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ON THE COVER:

Main photo: Rowan Musick (MPS II); Inset photos: Dominic Henriquez (MPS II), Maddie Todd (MPS I), Robin Wright (MPS IVA)

LETTER FROM THE CHAIRMAN

As the leaves begin to change and we embrace the beauty of the fall season, I am filled with immense gratitude as I introduce this edition of *Courage*. This year has been one of remarkable milestones and continued progress for the National MPS Society, and none of it would have been possible without your unwavering support and dedication.

This year, we celebrate the Society's 50th anniversary—a half-century of advocacy, research, and community building that has impacted countless lives. Together, we have made strides in advancing treatments, supporting families, and raising awareness for MPS and ML diseases. It is your commitment that fuels our mission and drives us forward, and for that we are deeply grateful.

As we reflect on the past 50 years, we are also looking ahead with great excitement. In December, we will come together for the 39th Annual Family and SPIRIT Conferences in Orlando, FL. These events are always filled with inspiration, learning, and connection. They provide us with unique opportunities to strengthen our bonds, share our experiences, and continue our fight for a better future for all those affected by MPS and ML.

In this edition of *Courage*, you will find stories of resilience, updates on our research efforts, and important information about upcoming events. I hope these pages inspire you as much as they inspire me.

Thank you for being a part of this journey with us. Here's to the next 50 years of progress, hope, and solidarity.

Lisa P. Todd, *chairman, board of directors*



save the date

38th Annual Family and Scientific Conference

Orlando, FL
Dec. 19–21, 2024



LETTER FROM THE PRESIDENT AND CEO

The photo below represents some of the global collaboration at the 17th International MPS Symposium, held this spring in Wurzburg, Germany. The symposium hosted more than 600 professionals and families from around the world. Lisa and I spent the first two days at the International MPS Network meeting with colleagues from Africa, Asia, North America, South America, and Europe. These meetings emphasized education, collaboration, and inspiration, focusing on gene and intrathecal therapies that cross the blood-brain barriers.



Our MPS and ML adult community stole the show by openly sharing glimpses into their lives. We learned of unique challenges and were brought to tears with laughter and joy as they shared their accomplishments.

One of the highlights of the symposium was the convergence of three countries—Switzerland, Austria, and Germany—that hosted the event together, demonstrating to the world that all things are possible through collaboration. We were very proud of the turnout. Also of note was Dr. Maurizio Scarpa's provisional bid for the 18th International Symposium to be held in Florence, Italy, in June 2026.

We learned of unique challenges and were brought to tears with laughter and joy as they shared their accomplishments.

Research grants comprise the most significant percentage of program funding of the Society's budget, illustrating the commitment of our members and donors to our mission to find therapies and eventual cures. Our grants are critical for moving MPS research forward.

This fall, we are introducing three new challenge grants to award funding for three submitted proposals. These include MPS IVA, MPS VI, and ML II/III. Each challenge grant will be awarded up to \$500,000. More information will be provided regarding fund development for these challenge grants.

While attending the 2024 Annual Symposium of the Society for the Study of Inborn Errors of Metabolism, Dr. Ellinwood and I were able to network globally for research and registries and talk with families and sponsor companies. From the United States, Denali Therapeutics announced a successful meeting with the FDA and plans to file for accelerated approval of tildenafil alfa (DNL310) for the treatment of MPS II, Hunter syndrome. Following the Reagan-Udall Foundation meeting, the Center for Drug Evaluation and Research had a positive and collaborative discussion and received guidance on cerebrospinal fluid heparan sulfate as a surrogate biomarker. This is a significant step forward, not only for MPS II, but for other rare diseases as well.

REGENXBIO announced positive data from their pivotal dose level of RGX-121 for MPS II, demonstrating long-term systemic effects. This illustrates sustained reductions in cerebrospinal fluid levels of heparan sulfate D2S6, a key biomarker of brain disease in Hunter syndrome. Eighty percent of patients in this trial have stopped using enzyme replacement therapy. RGX-121 is moving forward with a biologics licensing application for the accelerated approval pathway by the end of 2024. There is excitement around this gene therapy, and a one-time application for approval.

Lastly, the Annual Family Conference will be held Dec. 19–20 at Disney World. A fantastic program has been lined up, as well as many fun extras, including the National MPS Society's 50th anniversary party, visits from Santa and Disney characters, and more. We are also excited to debut our new childcare program, led by Brooke Thomas (MPS I mother).

Please join us for this unique celebration and take advantage of the opportunity to meet families from around the country.

Terri L. Klein, *president and CEO*

SCIENCE & RESEARCH

In the past year, there have been many notable accomplishments in the MPS and ML research realm, but the winter and spring of 2024 hold several of great distinction that have been in development for some time. Some of these will be highlighted in this issue of *Courage*, but all are focused on transforming the lives of our communities by improving the timely access to and the effectiveness of therapies for the MPS and ML II/III disorders.

The first step to equitable and timely access to any of the approved and accepted therapies for our community is a diagnosis at birth via newborn screening (NBS). I hope you are inspired by our maps of projected MPS NBS status in the United States for MPS I, II, and IVA on page 5. Progress is always too slow, but we are looking at near universal U.S. coverage for MPS I in the not-too-distant future. This will be such a milestone!

Before we can fulfill the promise of an early diagnosis, we must have an effective therapy, and that just doesn't yet exist for our community who live with neuropathic MPS II, IIIA, IIIB, IIIC, IIID, and VII. Increasingly, science has developed the knowledge to deliver therapies, but the

I am ever mindful of the need for all of our community to both rejoice in our past accomplishments, and with solidarity and resolve keep ourselves focused on accomplishing our many future goals.

FDA and the federal agencies responsible for delivering safe food and proven medications struggle to understand how to approach rare and ultra-rare diseases.



This January, I was honored to be published by the widely read Salon.com platform, which has a monthly readership of up to 15 million unique visitors.

Writing a commentary as the Society's chief scientific officer, I put forward the case for our Sanfilippo and allied communities who are not being as well served as they could be by the FDA. I hope you will take a moment to search for and read the commentary titled *"The FDA is failing children with a rare, neglected disease sometimes called 'childhood dementia.'"*

Finally, I was especially pleased to represent the Society as a participant at the February workshop hosted by the Reagan-Udall Foundation for the FDA. The workshop, titled *"Qualifying Biomarkers to Support Rare Disease Regulatory Pathways: Case example: heparan sulfate in neuronopathic lysosomal storage diseases,"* represented a coordinated effort of more than a year by numerous allied advocacy and stakeholder groups. I feel it was perhaps the most important scientific presentation I have ever made. Read more about this on page 4.

There are so many other exciting developments for our community, many of which are highlighted in this issue of *Courage*. Despite this, I am ever mindful of the need for all of our community to both rejoice in our past accomplishments, and with solidarity and resolve keep ourselves focused on accomplishing our many future goals.

N. Matthew Ellinwood, DVM, PhD, chief scientific officer

Are You Expecting a Baby?

For families expecting a baby after having a child with MPS, there may be opportunities for specialized care and treatment immediately after birth. Please contact leslie@mpssociety.org to discuss your unique situation. Some of the following options are available, and we can help connect you with additional information:

- prenatal testing to determine if a child has MPS while in utero
- clinical trial opportunities (prior to birth and/or setting up connections for trials that may be available immediately after birth)
- connection to approved therapies immediately after birth and coordination of care
- free cord blood banking for siblings without MPS

Reagan-Udall Foundation Workshop

This past February, the National MPS Society was honored to join a one-day assembly of research scientists and clinicians from across the globe. We gathered in Washington, DC, to take part in a workshop at the Reagan-Udall Foundation for the U.S. Food and Drug Administration (FDA).

The Reagan-Udall Foundation is an independent, private 501(c)(3) nonprofit whose mission is to enhance the work of the FDA to ensure safe and effective product development and approval. Mandated by Congress in 2007 and named for President Ronald Reagan and Arizona State Representative Mo Udall, the foundation works as a public-private partnership of stakeholders to dive into important questions, many of which involve particularly complex issues around the drug approval process.

The workshop, *“Qualifying Biomarkers to Support Rare Disease Regulatory Pathways: Case example: heparan sulfate in neuronopathic lysosomal storage diseases,”* was the result of a more than yearlong alliance of industry stakeholders, allied advocacy organizations, and the National MPS Society. At stake is the continued viability of drug development for ultra-rare neuropathic genetic metabolic disorders. The discussion was to form a “case example” using heparan sulfate as a biomarker in the MPS disorders, especially those that affect the central nervous system, such as MPS I, II, IIIA, IIIB, IIIC, IIID, and VII. All of the proceedings are available online.

WHY WAS THIS EVENT SO MOMENTOUS?

Ultra-rare neuropathic disorders such as the neuropathic form of MPS II and MPS IIIA have faced difficulties in the drug development arena. Because of the rarity of these diseases, the number of patients who are able to enroll in studies is very low, often requiring the added expense of international recruitment and international trial sites. Added to this is the fact that those who do enroll often have some level of disease because they were diagnosed years after clinical signs began. This complicates their clinical response to therapy based on the already existing burden of disease. Because of this complex population and the difficulty of showing cognitive improvement in treated children over a short period of time (for example, more than two years) that also corresponds to an already complex period of childhood development that happens to span the age when different cognitive tests are used. This leads to tremendous complexity and variability, such that, even though parents may note a tremendous improvement in the disease course, conclusive and statistically robust results are usually lacking, leading drug development programs to be abandoned or require extensions that make continuation of clinical trials impossible.

Fortunately, the FDA has at its disposal an important regulatory tool that allows for product approval using the Accelerated Approval Program. This innovative and far-reaching regulatory pathway was pioneered to solve the drug development impasse that the country faced with the HIV/AIDS crisis. Adopted in 1992, the Accelerated Approval Program allowed drugs to be approved based on the measurement of a biomarker, a value that could be measured, that served as a surrogate that is reasonably likely to predict a positive clinical response to therapy. In the early years of HIV/AIDS, this biomarker involved T-cell counts, which made it a much more efficient process of approval. Since its adoption, the Accelerated Approval Program has led to tremendous improvements in the number and speed of such approvals. A requirement of the application of this pathway is a disease where there is a particularly significant unmet need.

Because of all we know about the MPS disorders, encompassing decades of basic biochemical and animal model research as well as our deep clinical understanding, we know that the common and inciting cause of disease is always the intracellular and intralysosomal accumulation of glycosaminoglycans (GAGs). Furthermore, in all seven of the MPS disorders that affect the brain, the GAG that is stored is heparan sulfate. Heparan sulfate can be measured in cerebrospinal fluid, and would serve as an excellent biomarker to use as a surrogate biomarker that would likely predict clinical benefit in these diseases. The workshop at the Reagan-Udall Foundation was designed to bring together experts from across the globe to demonstrate the utility of heparan sulfate as such a biomarker.

Those experts included industry representatives from Denali Therapeutics, Orchard Therapeutics, REGENXBIO, and Ultragenyx, all of which helped support this workshop. Additional presentations were provided by allied advocacy organizations including the National MPS Society, Cure Sanfilippo Foundation, and the Ryan Foundation. Academic researchers and clinicians also presented from Washington University in Saint Louis, the University of North Carolina, Adelaide Hospital in South Australia, and Manchester University in the UK. Since this important workshop, the proceedings summary has been published online at the Reagan-Udall Foundation website, along with a peer reviewed article authored by the presenters. Two drug programs have since announced plans to move forward with accelerated approval using central nervous system heparan

continued

sulfate (Ultragenyx for MPS IIIA, and REGENXBIO for MPS II), with a third program having been granted a new FDA pilot program designation (START for Denali’s MPS IIIA program).

<https://reaganudall.org/news-and-events/events/qualifying-biomarkers-support-rare-disease-regulatory-pathways>

<https://ir.ultragenyx.com/news-releases/news-release-details/ultragenyx-announces-plans-file-accelerated-approval-ux111>

www.biopharminternational.com/view/fda-selects-participants-for-start-pilot-program

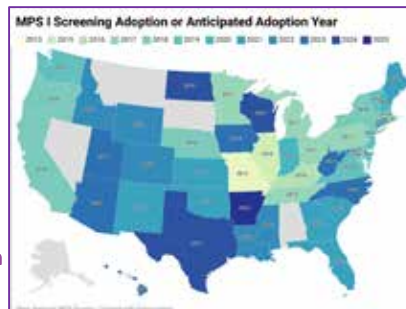
<https://regenxbio.gcs-web.com/news-releases/news-release-details/regenxbio-announces-successful-pre-bla-meeting-fda-support>

www.sciencedirect.com/science/article/pii/S1096719224004190?via%3Dihub

Newborn Screening for MPS Where does your state stand?

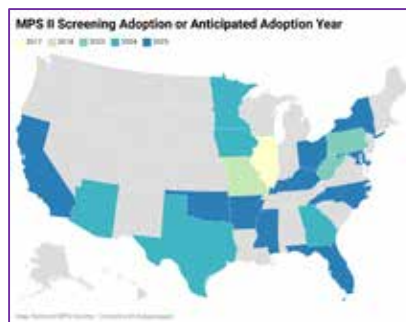
MPS I

Predicted by 2025
At least 45 States and the District of Columbia & ~96% of the US birth population



MPS II

Predicted by 2025
At least 19 States and ~57% of the US birth population



MPS IVA



Your State Can Make History

Be the first to start screening for the other MPS conditions with approved therapy including MPS VI or VII

Update on Allievex and AX 250

It is with deep sadness that we inform you of the impending liquidation of Allievex. The MPS IIIB enzyme replacement therapy developed by Allievex delivered traletsinidase alfa weekly directly to the brain via a catheter. The therapy reduced heparan sulfate levels in the brain and spinal cord in preclinical studies, and levels were lowered in the cerebrospinal fluid of treated patients. Especially in the cohort of trial patients who began treatment at a young age, therapy halted the neurodegenerative process and preserved neurological function in patients.

Despite these promising results, the collective efforts of Allievex's team, and the support of rare disease advocates, researchers, clinicians, families, and allies around the world, the desired regulatory flexibility from the FDA around Accelerated Approval Pathway was not forthcoming during their early discussions with the FDA. At the end of July 2024, Allievex announced that they had entered an assignment for the benefit of creditors marking the formal liquidation of their assets.

We share in your deep despair over this outcome. The families and patients who have benefited from this therapy and who now have nothing to look for in the future are an abiding concern to us. While we are grateful that the Allievex program did play a very positive role in the efforts of other MPS treatment developers such as Denali, Ultragenyx, and REGENXBIO to access improved regulatory paths with the FDA, we are also aware this news leaves our MPS IIIB community bereft.

The Society remains committed to advocating for and supporting research and treatments for all MPS disorders, and we will continue to communicate any news regarding the future of Allievex as it develops. Together, we hope to find a path forward that ensures the best possible outcomes for everyone affected by MPS.



As we celebrate the National MPS Society's 50th anniversary this year, let's remember that the sky is the limit for the next 50 years and beyond. Together, we can continue to make a profound difference in the lives of those affected by MPS and ML.

50 FACES OF MPS CAMPAIGN

In recognition of the National MPS Society's 50th anniversary, the Society is looking to share the stories of affected individuals, families, researchers, and supporters who play an active part in our mission to cure, support, and advocate for people affected by MPS and ML. If you would like to share your story, please scan the QR code and fill out the submission form to the best of your ability.



PATHWAYS



Pathways Committee:

Steve Holland, *chair*
Lisa Berry
Stephanie Bozarth
Carol Bryant
Maureen Cote
Tamara Cullere
Evelyn Fisher
Amy Holland
Terri Klein
Stacey Montgomery
Leslie Urdaneta

There is so much happening in our community. More children are getting diagnosed earlier thanks to newborn screening, new clinical trials are promising better treatments, and there is a renewed push to advocate for advancements in research and a more expeditious process to get treatments to the individuals who need them. The momentum is strong and there is so much to do.

At the beginning of the year, we welcomed Evelyn Fisher to the Pathways program as care coordinator. Evelyn brings to the program work experience in both social work and genetic counseling. She has been diligently learning all things MPS and ML and stands at the ready to meet and support those newly diagnosed.

The Pathways Committee has been strategically and thoughtfully examining the program to see where we can increase quality in the delivery of the program to newly diagnosed families. The committee is also brainstorming areas where the program may expand, always looking for a new means to service our families better.

The Pathways support group provides a great opportunity for individuals and families to come together, learn about a relevant topic, and support one another virtually. The Pathways Committee identified that the MPS and ML community at large also may benefit from this support group and we have recently opened the group to general membership in addition to Pathways families. Be on the lookout for postings about the group each month.

Last year we launched the Crossing Paths program as a means of outreach to families not known to the Society. With the success of last year's events and after thoughtful research to identify locations as areas of highest need, more events were held this year in Sacramento, Houston, Boston, Detroit, and Rock Hill. We will also meet in Phoenix in October. For more information on these or future events, follow us on social media, watch your inbox, or reach out.

Traveling to visit our new families is an integral component of the Pathways program. Visiting families allows us to further assess and support needs, continue to provide education, and connect the individual or family to the larger community. Evelyn, Leslie, and I have the privilege of being welcomed into homes, hospital rooms, family moments at a park, and wherever suits the families best. There is such value to these interactions with our MPS and ML family.

We look forward to seeing and supporting each and every one of you, whether online, in person, at a Crossing Paths event, or at our family conference in December. There is much work to do, but let's remember to take moments to celebrate each other as well as our advancements and successes along the way.

Carol Bryant, *Pathways program director*



TRAVELING THE JOURNEY TOGETHER

The National MPS Society's Pathways program provides families with education and comprehensive support throughout the first year of diagnosis. By connecting newly diagnosed members with services, both locally and collectively, as well as trained Society professionals, we equip and strengthen families to manage care and establish resources needed for their MPS or ML journey.

Visit mpsociety.org/pathways to learn more or email pathways@mpsociety.org.

THE TODD FAMILY



MADDIE TODD (MPS I)



REBECCA, MADDIE (MPS I), AND JASON TODD

Maddie is a strong-willed and joyful child with an irrepressible spirit.

Every night, we ask Maddie who she wants to pray for, and she enthusiastically begins listing all the people and pets in our family. When we read books that involve giving hugs or kisses, she reaches out to us for hugs or leans in for kisses. Maddie also behaves like a director at times. She tells us where to sit next to her to play and read books. She warms up to new people very quickly, and we believe that this is in large part due to the vast amount of exposure she has had with clinicians within the past year.

Maddie was diagnosed with Hurler's syndrome (MPS I) at 20 months of age. This was a daunting diagnosis for us to receive, but we were fortunate that she had a number of strong donor matches for a bone marrow transplant (BMT) and would be able to have the transplant two weeks prior to her 2nd birthday when treatment outcomes were the most beneficial. We decided to have Maddie's BMT performed at the University of Minnesota's Masonic Children's Hospital due to the high level of experience this institution has with transplant services for MPS patients.

In the months between receiving her diagnosis and her BMT, Maddie received weekly enzyme replacement therapy (ERT) infusions at our local hospital. She continues to receive ERT infusions each week at our home. Each week, we also go to the clinic for blood testing and meet with her local clinicians who monitor her progress and adjust her medications as needed.

Maddie is the only pediatric patient in Arkansas with MPS I, so we quickly realized we would need to broaden our community to include the National MPS Society and families who were active in the MPS I Facebook group. Carol Bryant, Pathways program director with the National MPS Society, quickly introduced us by email to other families with children who had just gone through a BMT at Masonic or were about to do so. We formed lasting connections with these and other families during our time in Minnesota.

There are times in life when we all must go through things alone, but the entire process of understanding and responding to an MPS I diagnosis for your beloved child is not a time to stay isolated. The other families we met helped lift us up and continue to provide valuable information. Some of our most grounding times in Minnesota were the group walks that we took with other families, pushing our kids around in strollers and wagons, and chatting about our respective experiences. Even though all of our kids had the same diagnosis, we were facing unique struggles. It was comforting to also talk about more lighthearted topics, such as what foods our kids currently enjoyed or how they liked to read books and play games.

Maddie, now 30 months old, is currently being treated with the approved therapies for MPS I, but we hope new and better therapies emerge that can treat neurological and musculoskeletal pathologies of the disease. We were very excited to learn what BioStrategies (a startup located in Arkansas) is doing to improve outcomes for patients with this disease through new therapeutic options. Due to the rarity of MPS I and similar diseases, it is challenging to mobilize new products to the clinic as the potential patient population available for new therapeutics makes funding for commercialization activities scarce.

Maddie is a strong-willed and joyful child with an irrepressible spirit. She is teaching us daily to accept each day as it comes, whether it is filled with clinic visits or we are able to stay home and play for hours on end. She has completely enriched our lives and our world is better for having her as our child.

FUNDRAISING

The incredible passion, talents, and courageous hearts that define our membership are truly inspiring. Whether it's brainstorming new ideas for family fundraisers, flexing your creative muscles with digital designs and art, or participating in our National Runs in Raleigh and Napa, your dedication, creativity, and unwavering support make a tangible difference in the lives of those affected by MPS and ML.

One of our proudest achievements has been the collective effort to refine our vision and bring it to life through unified action. By harnessing our passions, leveraging the expertise of our team, and bolstering support for one another, we've elevated the MPS and ML family to new heights. Our Fundraising Committee, in particular, has been thriving, brimming with energy and talent. Monthly meetings are buzzing with brainstorming sessions, updates from subcommittees, and innovative ideas that are taking fundraising at the National MPS Society to unprecedented levels.

A special thanks to Lynn Hopkins, esteemed board member and treasurer, for her outstanding leadership as chairperson of the Fundraising Committee for nearly three years. Lynn, along with her dedicated family, has been a driving force behind multiple fundraisers each year, generously lending their time and effort to support our cause. Lynn, Scott, Christopher, and Michelle (MPS I), we are profoundly grateful for your ongoing dedication.

Stepping into the role of Fundraising Committee chairperson is Stacey Montgomery. With years of fundraising experience as an MPS IIIB parent and a longstanding member of the Society since 2006, Stacey brings invaluable knowledge and passion to the table, ensuring continued success for our fundraising initiatives.

We have an exciting lineup of upcoming events, including the final two of four national runs in Long Beach and Orlando. Whether you join in person or virtually, your participation makes a significant impact, showcasing the power of unity within our community. If you're interested in contributing to the planning of these events, please reach out to a member of the development team or Fundraising Committee – your involvement is invaluable.

The 2024 Annual Fund campaign is under way. The funds raised through this initiative play a vital role in providing essential support to families, advancing research for a cure, and strengthening our advocacy efforts on state and national levels. Look for more information about the Annual Fund to be mailed and emailed soon.

As we celebrate the National MPS Society's 50th anniversary this year, let's remember that the sky is the limit for the next 50 years and beyond. Together, we can continue to make a profound difference in the lives of those affected by MPS and ML.

Fundraising Committee:

| | |
|------------------------------------|---------------------------------|
| Stacey Montgomery, <i>chair</i> | Malisa Hernandez Amy Holland |
| Alicia Bohley | Steve Holland |
| Mary Beth Brennan | Lynn Hopkins |
| Stephanie Cozine | Scott Hopkins |
| Tamara Cullere | Mike Hu |
| Rebecca Dopheide | Larry Kirch |
| Sarah Gniadzowski | Charlotte Tate |
| Chris Hernandez | |



Thank you to the members of the National MPS Society for your unwavering dedication and tireless efforts in organizing fundraisers within your communities. Your commitment to our mission is truly inspiring, and the time and energy you invest in these events make a significant impact on our Society. Each fundraiser not only raises essential funds for research, family support, and advocacy, but also strengthens our community bonds and raises awareness about our cause. Your hard work demonstrates the power of collective action and the difference we can make together.

The most successful fundraisers are those that reflect your passions and interests. Whether it's a community bake sale, a charity run, or a local art auction, every event can be transformed into a meaningful fundraiser. Remember, nothing is too small; even the simplest activities can create a ripple effect of support and engagement. By channeling what you love into fundraising efforts, we can inspire others and cultivate a deeper connection to our cause. Let's continue to explore creative ways to unite our passions and make a lasting impact together.

Bethany Greene, *development and program support*

2024 Annual Fund: Climbing Toward Hope

In a world where hope often feels elusive, the story of National MPS Society board member Carl Kapes and his family's journey with MPS IIIA reignites our belief in the power of community and compassion. Carl's resilience and determination, coupled with the unwavering support of the Society, highlight the incredible impact we can have on lives affected by this rare disease. As we launch the 2024 Annual Fund campaign and celebrate the 50th anniversary of the National MPS Society, we are honored to share Carl's inspiring story and invite you to join us in climbing to new heights for countless families facing similar challenges.

Facing unimaginable grief after his sons' (Ryan and Brayden) diagnoses, Carl turned his despair into action, seeking solace and support through the National MPS Society. As he delved into medical information and connected with other parents, Carl's determination to find answers and raise awareness grew stronger. His quest led him to undertake extraordinary challenges, including climbing Mt. Kilimanjaro to raise funds for MPS research. Each step he took mirrored the relentless struggle of living with Sanfilippo syndrome, yet his purpose remained clear: to find treatments and cures. His efforts continued with summits of Mt. Rainier and Pico de Orizaba, each climb symbolizing the ongoing fight against MPS.

Tragically, Carl's son Ryan lost his battle with MPS in 2021, but Brayden continues to fight. Carl joined the board of directors of the National MPS Society in 2022, driven by the belief in the power of raising awareness and funds. The Society's support, advocacy, and funding for crucial research offer hope for better treatments and accurate testing.

The National MPS Society's Annual Fund provides direct assistance for guiding families through their journey, funding research, educating stakeholders, and providing resources and support. Contributions to the Annual Fund enable this vital work to continue, making strides toward curing MPS and ML diseases.

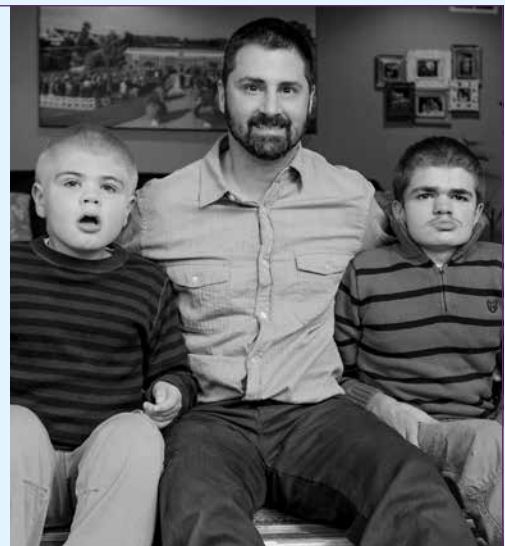
As we embark on this year's campaign, we humbly ask for your support. Together, we can reach the summit and find cures for MPS and ML. Join us in honoring Carl's climb and the countless families fighting these diseases.

Read part one of Carl's story by visiting mpssociety.org/wp-content/uploads/2024/07/2024-AF-Letter-one-Final.pdf or scanning the following QR code:



The National MPS Society is thrilled to announce Carl Kapes as chair of the 2024 Annual Fund. Carl has been an integral member of the board of directors at the Society since 2022. He lives with his family in Delaware. He is an engineering manager for Burns Engineering, dad to Ryan (forever 16, MPS IIIA), Brayden (MPS IIIA), Bryce, and Brooklyn, and an experienced advocate and fundraiser, having summited Mt. Kilimanjaro in 2012, Mt. Rainier in 2016, and Pico de Orizaba in 2018 to raise money and awareness for MPS III.

Make a difference by supporting the 2024 Annual Fund. Your gift will not only change lives, advance research, and provide care for those affected by MPS and ML, but will also drive the growth of the support network so many families rely on. Your contributions help execute our mission to cure, support, and advocate for those coping with MPS and ML.



**HAVE A FUNDRAISING IDEA?
EMAIL [BETHANY@MPSSOCIETY.ORG](mailto:bethany@mpssociety.org) TO GET IN TOUCH!**

RALEIGH RUN FOR RARE



More than 200 smiling faces participated in person (and nearly 130 more took part virtually!) on March 23 for the 14th Annual Raleigh Run for Rare 5K and One-Mile Walk. Families, friends, volunteers, donors, and sponsors made their way to Lake Crabtree County Park to rally in support of the National MPS Society and its mission. The run took place on a cool and rainy morning, but that didn't stop anyone from having a blast! Following the run and awards ceremony, participants gathered for a picnic lunch, DJ, face painter, and a special visit from the Easter Bunny. We are immensely grateful for everyone who supported this event through their presence, gifts, sharing information, and time given to make it a huge success. With your support more than \$35,000 was raised for our mission. Thanks to everyone involved in any way.

2024 FUNDRAISING EVENTS

FIRST AND SECOND QUARTER

Adam Brennan's Birthday Party

hosted by Mary Beth Brennan in honor of Adam Brennan

Aunt Joan's Pearl City Popcorn Purple Bow Sales

hosted by Wayne and Joan Eppehimer

Avery's Army T-Shirt Fundraiser

hosted by Madison Neuendorf in honor of Avery Neuendorf

Blood Drive

hosted by Johnston Pain Management in memory of Carter McGraw

Coin Drive - Aleyah

hosted by LE White Middle School in honor of Aleyah Smith

Crop for Rachel

hosted by Jim and Michelle Dodson in memory of Rachel Dodson

Garabito Fundraiser

hosted by Katherine Garabito in honor of Emilio Garabito

Jaela's Courage

hosted by Sydnee Rios in honor of Jaela Hernandez

Jammin' for Julia

hosted by Andrea Felten in honor of Julia Donahue

Keller's 5K

hosted by Clint and Lindsey in honor of Keller Blakeley

Lilah's Lemonade

hosted by Kimber Heiling in honor of Lilah Mueller

Million Dollar Bike Ride

hosted by National MPS Society

MPS Awareness Day

hosted by National MPS Society

Napa Race for a Cure

hosted by National MPS Society

Raleigh Run for Rare 5k

hosted by National MPS Society

Rare Aware Shop

hosted by Adult Resource Committee

Ride for Jack

hosted by Dirty Weasels in memory of Jack "The Beast" Bennett

Shoe Fundraiser

hosted by Alicia Bohley in honor of Jacob Bohley

Stevens Fundraiser

hosted by Marla and Randy Stevens in honor of Meekel Stevens

Super Bowl Fundraiser

hosted by MaryBeth Brennan in honor of Adam Brennan

Super Bowl Fundraiser

hosted by Amy Downen

Team Nora

hosted by Leanne and Trevor in honor of Nora Spring

T-Shirt Fundraiser

hosted by Breanne Busterud in honor of Garrett

T-Shirt Fundraiser

hosted by Katarina Nelson in honor of Wesley

SAVE THE DATE

2024 National MPS Society Events

OCT. 12

Long Beach Race for Rare 5K

Long Beach, CA

OCTOBER

Kramer Chili Cook Off

Strausstown, PA

Do It 'Fore' Dan

Atglen, PA

Post Office Cafe 5K

Babylon, NY

Halloween Spooktacular

Lewisville, TX



50 STATES 5K

DEC. 3

Giving Tuesday

(virtual)

DEC. 19-21

Annual Family Conference

Orlando, FL

DEC. 20

Jingle Bell 5K

Orlando, FL

DECEMBER

Holland Sharing and Caring

River Oaks, TX

NATIONAL MPS SOCIETY MAINTAINS FOUR-STAR CHARITY RATING

The National MPS Society continues to be recognized with an “exceptional” rating from Charity Navigator. This four-star rating and distinction differentiates the National MPS Society from its peers and displays a badge of trustworthiness and stewardship to the public. Receiving four out of a possible four stars signifies that the Society adheres to good governance and other best practices that minimize the chance of unethical activities and consistently executes its mission in a fiscally responsible manner. Only approximately one tenth of charities evaluated have received the highest rating, indicating that the National MPS Society outperforms most other charities in America.



MPS CHAMPION CIRCLE

Join the Champions Circle by making a monthly recurring donation. The impact of a recurring gift is felt for years to come. Families often use this method to distribute their Annual Fund gift, or another form of annual giving, on a monthly basis.

50 years ago, the National MPS Society began the fight of a lifetime. Together we can finish that fight. Become a member of the MPS Champions Circle today. Contact Bethany Greene at bethany@mpssociety.org or 919.806.0101. You can also register your monthly contribution online at mpssociety.org/recurringdonation/ or by scanning the QR code.

Facebook Fundraising

Have you ever considered hosting a Facebook/Instagram fundraiser? All it takes is for you to have an account. The average donation through a social media fundraiser is \$34 per person!

Create a birthday fundraiser for your big day, or consider starting a campaign to help raise additional funds to coincide with another event. Follow the steps below to start fundraising on Facebook today.

DO IT YOURSELF *Facebook Fundraiser*

IMPORTANT STEPS:

1. **Go to your Home page.**
 - Look at the left side of your Home page.
2. **Find Explore.**
 - Under explore click on FUNDRAISERS.
3. **Click on Raise Money.**
 - It is a green bar.
4. **Who are you raising money for?**
 - Click Non-profit.
 - Type in National MPS Society. Select.
5. **Determine how much money you want to raise.**
 - Fill in amount in the box.
6. **Choose an end date.**
 - Click next.
7. **Name your fundraiser.**
8. **Explain why are you raising money.**
 - The Society has information that automatically populates in that box.
 - Click next.
9. **Pick a cover photo for your event.**
10. **Click Create.**
11. **Share your fundraiser with friends, family, etc!**



TIPS:

- Share with as many people as possible.
- Ask friends and families to share as well.
- Write a personal story in the WHY box.
- Make sure to share your fundraiser multiple times during the timeframe.



COURAGE PAGES

Looking for a place to share your story, fundraise, and honor or celebrate the memory of someone you love with MPS or ML? Create a custom Courage Page! You decide what to include and how you want your story to be told.

Get started by registering a new Courage Page on our website (mpssociety.org/give/courage-pages) or by updating your existing page. Feel free to include pictures, stories, and current happenings with your family and friends. Your page can be used to direct those who would like to give, and you choose where the funds are applied.

Our members have raised more than \$100,000 through Courage Page donations. Create your Courage Page today, or refresh your existing page now!

WAYS TO GIVE AND INSPIRE HOPE

- Gifts in honor or in memory of a special person.
- Matching gifts through your employer (check with your human resources office).
 - Request a matching gift form from your employer.
 - Complete the employee section of the form.
 - Mail to the Society and we will process the gift.
- Courage Pages—share your family's story with your own web page to raise awareness and funds.
- Contribute through the Combined Federal Campaign if you are employed by the federal government (CFC #10943).
- Designate the Society as a member of your local United Way. You will need to supply them with the Society's name, address and Federal ID number (FEIN #11-2734849).
- Give to the Annual Fund.
- Major gift (usually around 10 times that of your Annual Fund gift).
- Planned gift (visit our website and search Planned Giving).
 - Wills or bequests
 - Charitable remainder trust or charitable gift annuity
 - Charitable lead trust
 - Life insurance policy or 401(k) retirement funds
 - Gift of appreciated assets (stocks, mutual funds, and bonds)
- Gifts may be applied to the Society's general operating purposes or restricted to one of our research, family support, or legislative programs.

Contact Bethany Greene at bethany@mpssociety.org or 919.806.0101 with questions.

FAMILY SUPPORT

The goal of family support is to continuously improve services and provide comprehensive care for families. In listening to our community's needs, we are learning more about how to reach those who may be underserved, have less access to information, and have needs extending beyond those directly associated with an MPS or ML diagnosis. We are committed to offering relevant programs and resources to our members, beginning with our Pathways program for those newly diagnosed and continuing to provide assistance throughout the MPS journey.

We invite you to attend the Crossing Paths events we host nationwide. Invitations will be emailed for local events and are open to those with MPS or ML and their families. They are designed to introduce people to the National MPS Society who want to learn more about resources, information, clinical studies, treatments, and more. Crossing Paths gatherings are free, fun, have lots of food, and time to be together with other families with plenty of kid-friendly activities. Think of those you may have met in waiting rooms, during treatments, or others with whom you have connected and tell them to come along.

In September, we hosted our bereaved families at a special Celebrating Your Cherished Life Experiences (CYCLE) retreat in Charlotte, NC. This retreat united families in times of reflection and honor and provides the opportunity to hear from trained professionals to help with grief and loss. All members are encouraged to join us for the 38th Annual Family and Scientific

Conference in Orlando, FL, Dec. 19–21, where we will celebrate the Society's 50th anniversary while learning about the latest in science, advocacy, and care management. There will be a SPIRIT conference for adults with MPS or ML and special activities, including an event for siblings.

Our updated website, mpssociety.org, has family support program guidelines and links to apply for grants and scholarships. Scholarship applications for the conference are prioritized for those newly diagnosed or who have not attended a conference before.

We welcome new members on our committees and invite you to share your talents with us! Please email me at leslie@mpssociety.org if we can provide any resources, connections, or support.

Thank you for being a part of the National MPS Society!

Leslie Urdaneta, *director, family support and communication*



Family Support Committee:

| | |
|---------------------------------|------------------|
| Tamara Cullere, <i>chair</i> | Terri Klein |
| Ginger Beverly | Katherine Klemm |
| Carol Bryant | Kris Klenke |
| Patricia Espinal | Sheri Lueb |
| Amy Fadden | Jason Madison |
| Bethany Greene | Amber Mongan |
| Angela Guajardo | Carolina Montoya |
| Amy Holland | Melissa Thompson |
| Steve Holland | Leslie Urdaneta |
| Scott Hopkins | Rebecca Walker |
| Jerry Kanney | Amy White |

NATIONAL MPS SOCIETY HONORED AS HEALTH EQUITY HERO

In January, Sanofi recognized the National MPS Society as a Health Equity Hero because of our family support, Crossing Paths, and Pathways programs. This award and recognition brought awareness to MPS and ML and highlighted efforts to reach those in need. These programs are designed to reach families and members and offer resources and connections in various ways. The Crossing Paths program received specific recognition for the Society's efforts to travel to communities that may be underserved by existing programs and reach new populations.



Family Support Programs

mpssociety.org/support

EXTENDED HOSPITALIZATION RELIEF

The Extended Hospitalization Relief grant provides financial support to an individual with MPS or ML who has experienced inpatient hospitalization for a minimum of 30 days.

CONTINUING EDUCATION SCHOLARSHIPS

The Continuing Education Scholarship Program awards post-secondary education scholarships for individuals with MPS or ML and their siblings, children, and parents. Scholarships are awarded to full-time students at \$1,000 each and \$500 for part-time students. The Jeffrey Bardsley scholarship is available to individuals diagnosed with MPS or ML and awards up to \$5,000. The Klenke-Kirch Sibling Scholarship awards an additional \$500 to siblings who assist with programs at the annual family conference and support sibling efforts. Scholarships are paid directly to the school or institution. Applications will reopen in January for the 2025-2026 academic year.

FAMILY ASSISTANCE PROGRAM

The Family Assistance Program offers grants of up to \$3,000 per individual with MPS or ML per 12-month period to help fund special equipment or medical aids not covered by insurance. Members must submit an insurance denial and medical order as part of the application process. Families and individuals are asked to cover 10% of the item's cost. Grants are awarded directly to a vendor.

MEDICAL TRAVEL ASSISTANCE PROGRAM

The Medical Travel Assistance Program assists families and individuals who need to travel to consult with medical professionals knowledgeable about MPS and ML by reimbursing travel expenses for appointments 125 or more miles away from home. This program may reimburse up to \$550 for travel and lodging expenses per individual with MPS or ML per 12-month period. Appointments must be approved in advance to ensure adequate funding is available for reimbursement.

JOURNEY ASSISTANCE PROGRAM

Journey Assistance Program grants are available to help purchase items needed to make life easier or more enjoyable. Items covered under this program are often what families pay out-of-pocket. Grants through this program will be awarded for 40% of the total purchase price, not to exceed \$500 per individual with MPS or ML. Grants cannot exceed \$500 in a 12-month period per individual.

EXTRAORDINARY EXPERIENCES

Extraordinary Experiences grants are available to individuals diagnosed with MPS or ML to provide a life-enriching extraordinary experience. This program is for individuals aged 13 and older to help achieve a personal goal. Grants can be used to support a special trip for education purposes or personal growth, such as a camp, program, or other sponsored activity that would offer the individual a chance to enhance his or her life. The focus of this program is to provide an experience that will build peer relationships, increase interactions with others, and provide an enriching opportunity for an individual. It does not fund wish-granting requests. Grants are available for up to \$1,000.

BEREAVEMENT EXPENSE PROGRAM

Assistance with final expenses for an individual with MPS or ML is available through the Bereavement Expense Program.

REGIONAL SOCIAL EVENTS

Families interested in hosting a gathering in their area can request up to \$750 in funding. Regional Social Events are open to anyone interested in attending, and the Society will provide support by marketing and sending information out to members.

Contact Leslie Urdaneta at 919.806.0101 or leslie@mpssociety.org with questions or more information about Family Support programs.

FAMILY SUPPORT GRANT RECIPIENTS

FAMILY ASSISTANCE PROGRAM



DOMINIC HENRIQUEZ (MPS II)

“Dominic is 14, is about 4 feet tall and will likely not grow much taller. He is extremely social and loves to interact with others. Having the activity chair provided by the Family Assistance Program significantly improves his quality of life as he is able to sit at a height similar to that of other adults. Before having an activity chair, Dominic would sit at a smaller/shorter table, usually alone, or he would sit at the regular-sized table, but was uncomfortable because his feet would dangle. With the activity chair, he can sit comfortably (and properly) with his peers, adults, and family members.”

— **Jeanette Henriquez**, mother of Dominic (MPS II)



AALONNA FISCHER (MPS IIIA)

“The mobile stairlift obtained through the Family Assistance Program for my daughter, Aalonna, helps her go up and down steps throughout the house. It also makes traveling easier when there are steps involved due to her increased challenges with climbing stairs.”

— **Candice Fischer**, mother of Aalonna (MPS IIIA)

JOURNEY ASSISTANCE PROGRAM



ASHLYN AND EMBER REA (MPS IVA)

“As a family of seven, three of whom have MPS IVA, we have quite a bit of difficulty going places with everyone as we cannot fit a wheelchair and stroller in our vehicle. Thanks to a new quad stroller wagon from the Journey Assistance Program, we can now take the entire family out, take the kids to their appointments, and provide an overall better quality of life.”

— **Carrie Rea**, mother of Christopher, Ashlyn, and Ember (MPS IVA)

National MPS Society NEW MEMBERS

Malik Ghulam Abbas, parent, Muhammad Jarrar Haider, MPS II, Pakistan

Jessica Acle, parent, Owen and Arlo Acle, MPS VI, FL

Callie Barcellos, parent, Malachi Barcellos, MPS II, WA

Bryan and Blair Begbie, parents, Rowan Begbie, MPS III, TN

Sarah Burnett, parent, Mason Burnett, MPS IIIA, OH

Breanne Busterud, parent, Garrett Busterud, MPS II, WI

Dana Caggiano, MPS IV, PA

Yabo Cao, MPS VI, MA

Jesus Castillo, MPS I, CO

Nisha Chaturvedi, parent, Shivansh Chaturvedi, MPS III, India

Ricky Chu, parent, Skyler and Oliver Chu, MPS II, CA

Taryn Cluff, parent, Daxton Cluff, MPS II, CO

Rebecca Cornell, parent, Daniel Cornell, MPS II, PA

Jill Cristich, friend, Spencer Gates, ML II/III, CA

Cassandra David, grandparent, Axel Garver, MPS I, CO

Ashley Davis, parent, Cameron and Jordan Davis, MPS II, MA

Catherine De Leon, parent, Maria and Greyci De Leon, MPS VI, MA

Laura DeChelbor, parent, Carson DeChelbor, MPS II, MI

Severin Andrieu Delille, parent, Oliver Delille, MPS I, CA

Cody Duncan, parent, Kasey Duncan, MPS II, MO

Jacqueline Mendoza Eichelberger, parent, Angelica Eichelberger, MPS IVA, CA

Nasrin Faqery, parent, Aysel Faqery, MPS I, MD

Nicole Feltner, parent, Garrett and Alayna Feltner, MPS IVA, IN

Rebecca Finn, parent, Freya Finn, MPS IV, GA

Katie Finney, parent, Abraham Finney, MPS I, IN

Ashley Funke, parent, Alivia Funke, MPS IIIA, TX

Sydney Gingrow, grandparent, Scout Scoggins, MPS I, NC

Brianne Green, parent, Braylee Green, MPS II, AR

Eric Griffith, parent, Scarlett Griffith, MPS IV, NJ

Cynthia Hernandez, parent, Emily Gomez, MPS I, IL

Tami Hetzer, parent, Kayden Hetzer, MPS VI, IL

Isabella Hutson, parent, Francesca Martin, MPS I, WI

Courtney and John Jamison, parents, Harper Jamison, MPS III, IA

Emily Layman, parent, John David Layman, MPS I, WV

Lauren Kennedy, parent, Everhett Rogers, MPS II, TX

Shelby Kizer, parent, Kit Kizer, MPS I, MS

Emily Layman, parent, William Layman, MPS I, WV

Roberta Lister, parent, Skylar Lister, MPS IIIB, MI

Alvaro Lopez, parent, Lorenzo and Alvaro Lopez, MPS IVA, MA

Amanda Marquart, parent, Rachel Marquart, MPS VI, OH

Laura Marquez, parent, Sergio Marquez, MPS IV, OR

Robbie McDonald, parent, Ross McDonald, MPS IIIA, MN

Erin Meixel, parent, Penelope Meixel, ML II/III, PA

Dinia Miranda, caregiver, Kimberly Bautista Martinez, MPS IVA, LA

Amina Mizerek, parent, Zeyad Mizerek, MPS II, CT

Katy Modesto, parent, Sebastian and Samson Modesto, MPS II, PA

Sharon Modesto, grandparent, Sebastian and Samson Modesto, MPS II, PA

Sajin Mohamad, parent, Sarah and Maryam Mohamad, MPS IVA, CA

Ishrat Mohammed, parent, Yazdaan Mohammed, ML III, IL

Betzaida Montes, parent, Selena Montes, MPS I, CA

Ashley Muth, parent, Kane Muth, MPS IIIB, FL

Brandon Nash, parent, Kinley Nash, MPS IIIA, IL

Jennifer Neuerburg, parent, Atlas Neuerburg, MPS II, KS

Alden Nipar, MPS VI, AZ

Natalie Overstreet, parent, Flora Overstreet, MPS I, TX

Noelia Santos, parent, Jasnely Santos Argueta, MPS IV, TX

Ashley Parrish, aunt, Titus Barrett-Weber, MPS I, KY

Annika Pedersen, MPS IVB, Denmark

Ryan Petri, parent, Lily Petri, MPS I, MA

Courtney Radakovitz, parent, Matthew Radakovitz, MPS II, CA

Brad and Glenda Ray, grandparents, Bonnie Ray, MPS I, NC

John Rodriguez, MPS IVA, CA

Michelle Rogers, parent, Lily Rogers, MPS IIIA, UT

Jerry Rogers, parent, Everhett Rogers, MPS II, TX

Sherrie Rojas, grandparent, Melania Rojas, MPS IV, NM

Rebecca Scott, grandparent, Noah Michael Scott, MPS I, CA

Megan Selser, parent, Benjamin Selser, MPS II, NC

Angela Sharp, parent, Aaron Sharp, MPS I, MI

Katrice Sharp, parent, Ty'Rai Sharp, MPS II, NE

Aleksandra Checinska Sielaff, parent, Filip Sielaff, MPS I, WA

Emily Smith, parent, Ellaura Smith, MPS I, NV

Ary Spilkin, parent, Elsie Muir, MPS I, CA

Denise Stultz, sibling, Douglas and Duane Stultz, MPS II, FL

Jennifer Sweatman, grandparent, Sebastian and Samson Modesto, MPS II, PA

Barbara Swilley, grandparent, Kit Kizer, MPS I

Kylin Teo, parent, Aureus Teo, MPS II, Singapore

Ras Turner, parent, Taheisha Turner, MPS III, PA

Terrence Tyrrell, parent, Mara Tyrrell, MPS I, MI

Azlan Ulhaq, parent, Rabia Ulhaq, MPS IV, NY

Marissa Valenzuela, parent, Mason Valenzuela, MPS IIIA, TX

Joanna Velasquez, MPS IVA, FL

Jaky Vergara, MPS IVA, CA

Teanna Warren, parent, Jayanna Warren, MPS IIIB, IN

Cindy Weaver, grandparent, Lydia Rachal, MPS III, LA

Brooke Wehbe, parent, Maximus Dannen, MPS IV, IA

Tracie Wiegert, parent, A.J. Wiegert, MPS III, IA

Tiffany Witherington, aunt, Jaxon Clark, MPS II, SC

Courtney Woodard, parent, Josiah Woodard, MPS II, TN

Jinmei Zheng, parent, Changyan Zheng, MPS III, CO

FAMILY SUPPORT

Christopher and Robin Wright are long-time members of the National MPS Society, both diagnosed with MPS IVA. They have submitted stories of their experiences and successes, hoping to encourage others with rare diseases to see beyond timelines and limitations. Since submitting these pieces, Christopher passed away at age 75, leaving his brother, Robin, to continue to share his legacy with the world.

CHRISTOPHER WRIGHT (MPS IVA)

I was born more than three quarters of a century ago with a very rare genetic connective tissue disease that was supposed to take me out by age 30. Because of an overweening sense of protection, my parents forbade me from having any friends, a restriction that remained until after I graduated from college. Consequently, there were no social skills for dealing with people who would take advantage of the infantile adult. That said, I do believe (which I didn't at the time) that I had angels watching over me, helping me when I would goof up in some pretty terrible ways.

After a serious, long-lasting concussion caused by an old barn falling on me, an accident that put me in the hospital for knee reconstruction as well as tearing off a lot of long hair and skin from my back, my battered brain reconstructed itself as a large format photographer. Prior to this, I had no interest in photography. Yet, within a very few years, my pictures were being accepted into major shows and in the Ansel Adams Workshop, something which surprised everyone, myself especially.

Since then, I have been a homesteader, carpenter, assistant home birth midwife for six of my seven children, a professional photographer for 30+ years, and a PhD student at McGill University in the history of religion. After having more than 20 major surgeries and innumerable lesser ones, many of which would seriously knock out the mental stuffing, at age 60, I was no longer able to carry my heavy antique wooden cameras.

Shortly after, thanks to severe nerve entrapment in my left elbow, I was forced to give up my beloved guitar, an instrument which I had played since age 12. At 65, I suffered three transient ischemic attacks in only six months, resulting in the near complete collapse of my mental abilities. Fortunately, my two daughters (truly angels in my life) rescued me and brought me to the little town where they live. There, after two years, neuroplasticity kicked in and my intellectual capacity returned. Although I haven't been able to walk in years, my mind is pretty functional and capable of enjoying those studies that absorbed me more than 50 years ago.

Now, at age 75, I'm one of the oldest persons in the world with Morquio syndrome. And to top it off, last year I was diagnosed with stage four myelofibrosis, a very rare blood cancer, and given only a few months to live. It's now almost a year and a half and I'm still here thanks to regular transfusions. Two very rare conditions must make me a very rare person?

It's been a long, strange, beautiful life that I will be happy to give back to the Creator when the Reaper finally calls. Advice for those struggling with disease and disability: hang in there, don't be self absorbed, but instead be thankful for every precious moment you have. No disease is greater than the spirit within.

ROBIN WRIGHT (MPS IVA)

From the very moment I could start doing physical chores and labor, I would challenge my limitations, denying that I was physically different from anybody else, despite an unusually short stature for which I received a great deal of bullying, or that I was a "slow" learner indicating some cognitive deficiency—probably the result of the isolation imposed on my life as a child. From early on, I was told that I "suffered from the anguish of inadequacy"—such an analysis by close kin instilled in me an anxiety that made me desire to achieve fame in spite of my short stature (what some call a "Napoleonic complex"). I would try every way to show that I could perform, in sports, just as good as anyone else. I played basketball, the shortest guy on the team, lying to my parents just to be able to go to practice.

My life since early adulthood has been one of enduring ordeals, putting my body through extremely difficult and

challenging situations. Sometimes dangerous and foolish situations. Sometimes exhilaratingly novel situations, but always with the desire to open new vistas. Let me give some examples in my life history.

During my senior year in college, I wrote an honors thesis on a French scholar who worked with indigenous people in Brazil. My father, a professor of French language and literature, approved the choice of topic; my French-born mother went so far as to arrange for the scholar's signed and dedicated photo, given to me as a Christmas present. That became then my intellectual quest in life—a classic hero's journey—to find meaning in distant Amazonian indigenous traditions.

First stop was Stanford University where I was accepted with a National Science Foundation fellowship, a full ride through one of the most prestigious Departments of Anthropology

in the country. The department at that time was focused in Mayan studies which interested me a great deal inspiring a master's thesis on Mayan ceremonial organization. I followed up with a field trip to northwest Guatemala, by car and bus, to learn Spanish and maybe visit the ruins like an informed tourist would do. Among other notable adventures, I scaled a volcano and slid all the way back down through its ashes. Never did my bones, especially my hips and knees, hurt so much as that night; this was my first real wake-up call of my condition as bearer of Morquio syndrome. I continued, nevertheless, to show my seriousness about studying in the Amazon. There was no Amazon specialist at Stanford nor a "fieldwork methods" course applicable to the Amazon. I was referred to specialists at other universities to which I traveled by bus to consult.

My first journey to the Amazon in 1976 involved flying in a World War II Brazilian Air Force Catalina waterplane, and walking 25 kilometers over an Indian trail through the Amazon jungle to get to where I wanted to go: a village of very powerful shamans, or spiritual specialists, and a place of exceptional natural beauty. Never had I forced my body to face such a challenge. This and numerous other adventures during the next two years hastened the breakdown of my weightbearing joints. Yet, I reached a point late in my fieldwork where I could physically withstand what I never imagined possible, like hiking the same 25 km trek in half my original time and canoeing against powerful river currents. My body had successfully adapted to living in the Amazon.

In the mid-1980s, a few years after finishing my PhD, I returned to teach in Brazil on a Fulbright fellowship; when that ended, I married a Brazilian woman who had children from a previous marriage. I decided to settle down to the life of an expat and form my own family. To pay the bills in a country plagued by skyrocketing inflation, I had to secure several jobs at the same time: as university professor rising through the scale of promotions to full; as translator of articles and book-length texts focused on indigenous rights; and as collaborator working in indigenous rights organizations in the city of Sao Paulo. I commuted to work by bus, train, and walking, which proved to be a huge strain on my joints.

I had my first and second total hip replacements in the United States, right after field research in the Amazon in 1977. Total hip replacements were then, compared to today, still a developing science, experimental and controversial. They were meant to last at most 15 years. Twenty years following my first replacements, and after two more difficult trips to the Amazon, one of my metal hip prostheses broke which launched a sequence of more than half-a-dozen surgeries on hips and knee in Brazil. Some of them successful, others were complicated due to a series of hospital infections followed by several rushed surgeries to contain the damage. Throughout all of these surgeries, my Morquio body was telling me that I had to slow down if I wanted to live and that I had to seek better medical facilities in the United States.



At the conclusion of 20 years living and teaching in Brazil, we packed up our family and moved to Gainesville, where I taught at the University of Florida for 18 years. During this time, I underwent two more revisions on both hips and for a broken femur. One revision turned out very well; the other not so good, resulting in chronic pain, increased sedentarism, struggles with legal opioids, and a series of other problems.

By the time I retired at age 73, I felt that both my body and spirit were worn out. I have therefore chosen to enjoy the remainder of my life in silence, meditating, medicating, reading, and writing. Fortunately, Morquio syndrome has affected neither my cognitive abilities nor my spiritual quest for healing and wholeness. Neither of these has deteriorated along with my body. Throughout my life, I have tried not to let my condition dominate me, or even to have a significant impact. However, over and over, my condition has imposed its limitations through chronic and debilitating pain, progressive joint deterioration, weak bones, and a series of other complications that hospital stays have revealed. It is nothing short of miraculous that my heart has survived the decades of strain, though it did want to cease beating once, and I lost consciousness.

Follow Us on Social Media!



@NationalMPSSociety on Facebook



@mpssociety on Instagram



National MPS Society on LinkedIn



Bereavement Support

The National MPS Society supports families in times of loss and during the bereavement and grief process. We understand the importance of personal connection and creating opportunities to share memories and receive direct support. Our **White Rose program** provides booklets, resources, and mementos; our **Bereavement Expense program** offers financial assistance. Staff members can speak with you and make additional connections for grief support as needed. Call 919.806.0101 or email leslie@mpsociety.org for assistance or more information about our bereavement programs. These programs are available in memory of anyone with MPS or ML.



ANGELS AMONG US

Each year, we remember individuals with MPS or ML who have passed away but remain in our thoughts and hearts. We invite families and loved ones to share their memories with others.

Through the *Angels Among Us* publication, we encourage you to join the community in lifting up, remembering, and honoring their lives as you read their names and reflect upon their memories.

To view this year's edition, visit mpsociety.org/angels-among-us.

IN MEMORIAM

The entire MPS and ML community lifts up family members and friends of those listed here, and others remembered in our hearts. We travel with you through grief and recognize this journey's beauty and joyful memories. We honor and remember the lives of those who have passed away.

Loralei Lee Bell, MPS IIIA

9/28/15–11/25/23

Trinady Bowen, MPS III

1/10/11–12/13/23

Michael Campbell, MPS II

10/24/08–12/28/23

Kasey Duncan, MPS II

8/10/10–10/18/23

Nicole Flanagan Erni, MPS IIIB

9/25/89–12/27/23

Graciela Esparza, MPS IIIA

1/27/04–11/28/23

Nathan Evans, MPS IIIA

3/20/01–5/11/24

Bethany Gamache, MPS IIIC

1/30/03–1/14/24

Madison Holland, MPS I

6/26/91–1/2/24

Tyler Langan, MPS IIIA

9/13/04–7/17/24

Francesca Martin, MPS I

7/24/22–5/3/24

Penelope Meixel, ML II/III

11/19/23–4/7/24

Gatlin Moretz, MPS II

10/24/21–4/22/24

Owen Peck, MPS IIIA

1/21/15–4/26/24

Cooper Rieg, MPS IIIA

7/26/03–4/15/24

Lily Rogers, MPS IIIA

3/9/10–3/12/24

Daymein Sanders, MPS I

8/27/06–12/1/23

Nicholas Segura, MPS II

6/11/11–3/28/24

Marwan Shaheen, MPS IIIC

12/10/90–1/17/24

Ty'Rai Davion-Cal Sharp, MPS II

7/10/03–3/25/24

Blake Stack, MPS IIIA

10/8/09–7/21/24

Luke Szabo, MPS II

2/20/14–6/11/24

Clay Taylor, MPS II

10/16/07–10/27/23

Gavin Townsend, MPS II

10/25/11–4/20/24

Harvy Vasquez, MPS VI

8/8/02–4/26/24

Noah Walters, MPS IVA

6/26/99–12/12/23

Jayanna Warren, MPS IIIB

6/29/09–2/7/24

Scotty Whitecotton, MPS II

9/29/97–5/3/24

Christopher Wright, MPS IVA

10/14/48–4/17/24

Christian Yard, MPS II

9/7/07–4/26/24

Remembrance Committee:

Kris Klenke, *chair*
 Laura Catanzarite
 Tom Catanzarite
 Debbie Dummann

Monica Green
 Steve Holland
 Larry Kirch
 Terri Klein

Noni Langford
 Dorothy Mask
 Stacey Montgomery
 Robin Piefer

Leslie Urdaneta
 Nancy Wain
 Amy White

ADULT RESOURCE COMMITTEE

The Adult Resource Committee (ARC) was formed to advocate for the unique needs of adults with MPS or ML, and provide opportunities for connection and support. The committee is composed of adults with one of these diagnoses who lead fulfilling and active lives with varied interests, while taking time to give back to their community. ARC meets each month on Zoom to plan activities and events, and advocate for resources that will benefit adults and adolescents transitioning into adulthood.

Activities include quarterly social hours and virtual events, support sessions, and connecting on social media, as well as gathering in person at conferences and events. The committee works to brainstorm awareness campaigns for events such as Rare Disease Day and MPS Awareness Day, and for the Society as a whole. One of ARC's main goals is to show that adults with MPS or ML lead fulfilling lives with a rare condition.

Email arc@mpssociety.org if you are interested in joining the committee, to provide input, or with questions. Any adult with MPS or ML (ages 18+) may join ARC. We encourage you to reach out!

Adult Resource Committee:

| | |
|-----------------------------------|----------------|
| Autumn Mortensen, <i>chair</i> | Jenny Klein |
| Isabel Bueso | Jason Madison |
| Patricia Espinal | Kyle Underwood |
| Kendra Gottsleben | Sheri Wise |
| Steve Holland | Jocelyn Wong |
| Carlton Hutcheson | Fanny Zambrano |

WINTER ZOOM SOCIAL EVENT

Are you an MPS or ML affected individual age 18 or older? The Adult Resource Committee hosts Zoom social events throughout the year where affected adults from across the country have the opportunity to connect and network with other affected individuals. The next event will be Friday, Dec. 6, and will be holiday themed. Attendees will be invited to play a festive game and win a prize!



The National MPS Society invites you to attend the "Finding our SPIRIT" conference for adults (age 18 and up) diagnosed with MPS and ML. Society staff have collaborated with the Adult Resource Committee, spoken with you, and reviewed evaluations from previous conferences to determine what events are important to you. With this in mind, we have incorporated formal meeting times, as well as scheduled time to be spent with your peers on topics of interest. We hope to see you there!

SUPPORT THE SOCIETY WITH LOGO WEAR

cafepress.com/rareawareshop

Support the Adult Resource Committee by ordering from the Rare Aware Shop. Items are for anyone in the rare disease community and proceeds go directly to programs for adults diagnosed with MPS or ML. Designs in the Rare Aware Shop were created by ARC members, and proceeds directly benefit programs for adults with MPS or ML.



ADVOCACY

National MPS Society staff, board, and community members held advocacy Capitol Hill visits virtually on May 14 and 15. The Society was granted 50 meetings, a 94% confirmation rate as compared to meeting requests.

Three of these meetings were at the member level, i.e., members were able to connect directly with legislators in both the House and Senate. These meetings had 29 attendees; 17 states were represented across 28 Senate and 22 House meetings with 24 Democratic offices, 25 Republican offices, and one Independent office.

FDA CALLS FOR PATIENT AND CARE PARTNER PERSPECTIVES ON SAFETY CONSIDERATIONS FOR APPROVED GENE THERAPY TREATMENTS FOR RARE DISEASES

On Sept. 20, the FDA Center for Biologics Evaluation and Research hosted a public patient listening session in the hopes of opening a docket to better understand patient and care partner perspectives on long-term care and follow-up considerations for approved gene therapy for rare diseases. Stephanie Cozine, co-chair of the Advocacy Committee, attended the meeting as a parent advocate.

Current Legislation

ACCELERATING KIDS' ACCESS TO CARE ACT (H.R.4758)

This bill aims to improve children's access to healthcare services by addressing barriers in the Medicaid and Children's Health Insurance Program systems. The legislation focuses on streamlining enrollment processes, enhancing provider participation, and ensuring timely access to necessary care for children. By improving these systems, the act seeks to ensure that all children receive the healthcare services they need, ultimately promoting better health outcomes.

CREATING HOPE REAUTHORIZATION ACT (H.R.7384)

This bill seeks to increase access to pediatric drugs and treatments for children with serious or life-threatening conditions. The legislation reauthorizes and expands the "Creating Hope" initiative, which provides incentives for pharmaceutical companies to develop and test new therapies, specifically for pediatric populations. The act emphasizes the importance of ensuring that children have access to safe and effective medications, fostering innovation in pediatric drug development, and enhancing collaboration among stakeholders in the healthcare system.

MPS Awareness Day

The states of Oklahoma and Delaware officially recognized May 15, 2024, as MPS Awareness Day.



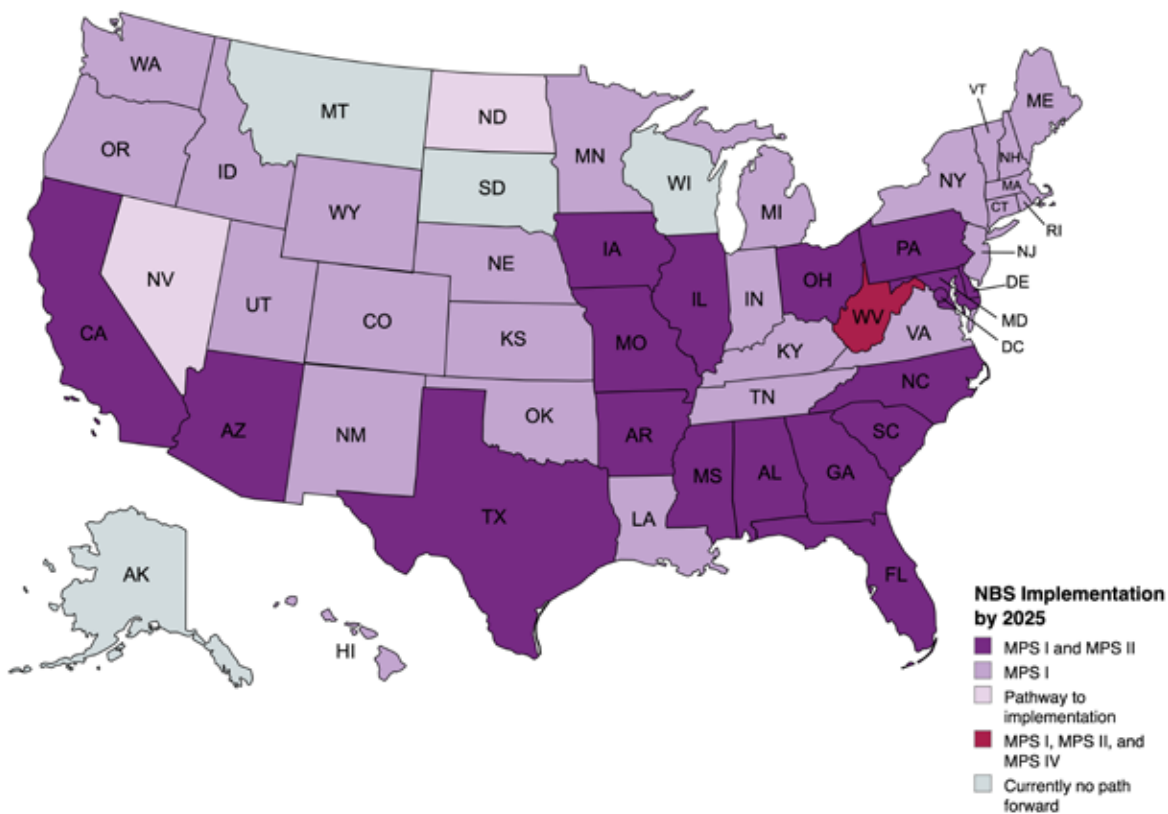
MPS SOCIETY BOARD MEMBERS SHERI LUEB (ABOVE) AND STEPHANIE COZINE (LEFT) WITH THE OFFICIAL PROCLAMATION.

Newborn Screening Updates

- Alabama’s newborn screening legislation is essentially aligned with the Recommended Uniform Screening Panel (RUSP), although MPS I and II have yet to be added. MPS I is currently being screened.
- MPS I will be added to Nevada’s newborn screening legislation from 2019. It is unclear if the bill is essentially RUSP aligned, which would include MPS II.
- Wisconsin has approved MPS I to be added to its panel.
- Minnesota has approved MPS II to be added to its panel.
- MPS II is now being screened for in Delaware, Kentucky, and Tennessee.
- MPS II was being screened for in North Carolina, although there is a gap between pilot and department of public health implementation.
- Newborn screening efforts in Montana, South Dakota, and North Dakota are pending. Alaska is likely to add conditions to its newborn screening panel when its contract expires and is renegotiated.



ZACHARY THOMAS, MPS I ADVOCATE FOR NEWBORN SCREENING



- Advocacy Committee:**
- Stephanie Cozine, *co-chair*
 - Carl Kapes, *co-chair*
 - John Barbour
 - Amy Barkley
 - Eileen Basquill
 - Claudina Bonetti
 - Stephanie Bozarth
 - Madeline Casper
 - Mary Cavanagh
 - Hannah Clark
 - Kayla DelBoverlando
 - Nicholas DiTommaso
 - Christine Dwyer
 - N. Matthew Ellinwood
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Sharing your stories, experiences, and support makes a difference in the lives of those with MPS and ML today, and those who will be diagnosed in the future. Every advocate helps further our mission to find a cure, and to support and advocate for MPS and ML causes. If you are interested in participating in future advocacy efforts, visit mpssociety.org/advocate.

RESOURCES

Newborn Screening Resources

Baby's First Test

babysfirsttest.org

The Baby's First Test website provides current educational and family support and services information, materials, and resources about newborn screening at the local, state, and national levels. This resource is dedicated to educating parents, family members, health professionals, industry representatives, and other members of the public about the newborn screening system. The site also provides many ways for people to connect and share their viewpoints and questions about the newborn screening system.

BFT NBS Month 2020 Awareness Toolkit

babysfirsttest.org/sites/default/files/%232020NBS%20Toolkit.pdf

This toolkit is for families and advocates and prepares you not only for Newborn Screening Awareness Month in September each year, but is an excellent resource for advocacy year round.

Expecting Health, Navigate Newborn Screening Modules

<https://expectinghealth.myabsorb.com>

To help families navigate their child's health during a critical period of development, Expecting Health created free online educational modules. Families can find answers to questions like, What is the process of screening? What do newborn screening results mean? What should I ask my healthcare provider about newborn screening? What are the state and federal laws around screening?

National Family Caregivers Association

caregiveraction.org

As a care provider, it is easy to become so focused on the person you are caring for that you forget to take care of yourself. The National Family Caregivers Association (NFCA) educates, supports, and empowers individuals who care for a loved one with an illness or disability. From tips and how-to guides to a story bank and pen pal program, the NFCA caregiver resource center provides a wealth of resources to support you as a caregiver.

Association of Public Health Laboratories, NewSTEPS

newsteps.org

The Newborn Screening Technical assistance and Evaluation Program (NewSTEPS) is a national newborn screening resource center that provides data, technical assistance, and training to newborn screening programs and assists states with quality improvement initiatives. It includes resources and profiles of state- and condition-specific screening. If you are interested in learning more about state lab newborn screening programs, this is a helpful resource.

Screen Baby Screen: Perspectives on Newborn Screening

youtube.com/watch?v=cCQScP9zqGQ

This webinar is a great resource for understanding why each state has its own individual screening program and why early detection of a disease is crucial. It addresses intervention and shares how newborn screening is important for babies to achieve their full potential.

EveryLife Foundation

everylifefoundation.org

The EveryLife Foundation has been providing tools for newborn screening advocacy for more than 10 years. From Capitol Hill advocacy, bootcamp webinars, downloadable toolkits, and crucial policy action alerts, this is an excellent resource to help you become a strong advocate for your family.

Hearing Aid Funding Assistance

sertoma.org

The primary focus of international service organization Sertoma is to assist the more than 50 million people with hearing health issues and educate the public on the issues surrounding hearing health. The organization offers a hearing aid recycling program, a college scholarship program for young adults with hearing loss, as well as various community support programs.

starkeyhearingfoundation.org

Hear Now is a national non-profit program sponsored by The Starkey Hearing Foundation that provides hearing aids for people with limited income.

Legal and Assistive Technology Funding Assistance

nls.org

Neighborhood Legal Services, Inc. (NLS) provides free legal services to persons with low income and persons with disabilities. It also provides a wide range of technical assistance and support services. NLS's National Assistive Technology Project supports advocacy efforts of attorneys, advocates, service agencies, persons with disabilities, and their families as they seek funding for assistive technology services and devices.

ERT Resources

| Syndrome Type | Enzyme Replacement Therapy | Website |
|---------------|------------------------------|-----------------------------------|
| MPS I | Aldurazyme® (laronidase) | aldurazyme.com |
| MPS II | Elapraxe® (idursulfase) | elapraxe.com |
| MPS IVA | Vimizim® (elosulfase alfa) | vimizim.com |
| MPS VI | Naglazyme® (galsulfase) | naglazyme.com |
| MPS VII | Mepsevi® (vestronidase alfa) | ultragenyx.com/medicines/mepsevii |

Transitioning to Adulthood

Life is full of transitions. An important transition for youth with special healthcare needs and their families is the transition to adulthood. To make this process smooth, begin early. Create a statement of needed transition services, addressing areas such as instruction, employment, community experiences, and adult living. For more information, check out these transition resources:

- Healthcare transition—resources and information focusing on a young adult's transition from pediatric to adult healthcare (**gottransition.org**).
- PACER Center—resources for parents of children with special needs, and extensive information for transitions and creating transitioning plans (**pacer.org**).

MPS I

aldurazyme.com

This website, developed by Genzyme, provides parents and patients with information on Aldurazyme®, an enzyme replacement therapy for MPS I. The site includes a link to ask questions regarding MPS I or anything else related to treatment. The site also can reach healthcare professionals at Genzyme who will respond to your query in a timely manner.

MPS IVA

morquiosity.com

This website offers a variety of information for MPS IVA patients, including a description of the disease, cause, early signs, symptoms, management, and tests and diagnosis. Learn more about the people who make up the Morquio A community, discover helpful online resources, and create a list of questions to bring to your next doctor's appointment.

morquioanswers.com

This resource for healthcare professionals provides information on pathology, systemic effects, natural history, management, and resources and publications.

MPS VI

Naglazyme.com

This site provides expanded content about MPS VI, its diagnosis, and treatment with Naglazyme® (galsulfase) enzyme replacement therapy.

National Parks Access Pass

nps.gov/subjects/accessibility/access-pass.htm

Individuals with MPS or ML are eligible for the Access Pass, part of the America the Beautiful—National Parks and Federal Recreational Lands Pass Series. This grants free access to the National Parks. Applicants must provide proof of residency or citizenship and documentation of disability.



ISABEL BUESO (MPS VI)

Miracle Flights

miracleflights.org

This travel resource provides free flights for low-income children and their families to distant, specialized care and second opinions.

Wrightslaw

wrightslaw.com

Wrightslaw offers information about education and special education law, and supports families through advocacy for children with disabilities.

Takeda OnePath®

onepath.com

Takeda provides support for eligible patients in the United States. Through the OnePath program, Takeda assists with access to treatment and provides patient support managers.

Ultragenyx UltraCare Program

ultracaresupport.com

The UltraCare program demonstrates the commitment of Ultragenyx to support patients with rare diseases, and can help with understanding coverage, determining access to assistance programs, and providing information about patient support programs for patients with MPS VII. Visit the website above or call 1.888.756.8657.

Furniture for Little People

lplibigdesign.com

Little people, BIG DESIGN is designer furniture for short people, and people with dwarfism or short stature. Created by Tracy Steele Designs, this furniture meets the ergonomic challenges of little people without sacrificing good design. Little people, BIG DESIGN furniture features:

- short seat depth and straight backs to help support the back and neck
- low seat height so legs rest comfortably on the ground
- high arms to rest on while reading
- solidly built to support the weight of adults
- steps for easy accessibility
- adjustable for the height of guests

Parent Educational Advocacy Training Center

peatc.org

The Parent Educational Advocacy Training Center (PEATC) serves families and professionals of children with disabilities in the Commonwealth of Virginia. PEATC promotes respectful, collaborative partnerships between parents, schools, professionals, and the community that increase the possibilities of success for children with disabilities. PEATC's mission is to build positive futures for Virginia's children by working collaboratively with families, schools, and communities to improve opportunities for excellence in education and success in school and community life.

Bereavement Resources

Bereavedparentsusa.org provides support groups and information for family members after the loss of a child. BPUSA hosts an annual family conference for support.

Compassionatefriends.org provides support groups and information for family members after the loss of a child. Information regarding state and local support is available.

Courageousparentsnetwork.org was created for and by parents, and provides support, information, and knowledge based around issues of parenting and caring for children with illnesses, as well as bereavement and loss support.

HealthTalker—An MPS II Online Community

hunterpatients.com

The Hunter Parents Community is an online community sponsored by Shire. The website is an exclusive forum for primary caregivers of children with MPS II to connect and share their personal stories and experiences, as well as give and receive tips for facing everyday challenges. In addition to strengthening the network of MPS II parents, the community aims to increase awareness about MPS II by encouraging primary caregivers to talk about Hunter syndrome with members of their community and to use their personal experience to help others understand this life-altering condition. The Hunter Parents Community is not a forum to discuss medical, product, or treatment options, but rather allows MPS II parents to support and learn from each other, and to raise awareness.

Support Services for Patients and Families through CareConnectPSS™

careconnectpss.com

Sanofi offers case management services to people living with genetic diseases, offering access to a dedicated team of professionals who provide disease education and help address needs, including assistance with health insurance issues. A Sanofi team works together to help resolve any issues that may arise and provides personalized support when you need it. Call 1.800.745.4447, option 3, or visit the website above to connect with a case manager online.

Frank Mobility Systems

frankmobility.com

Wheelchairs differ in many ways. Some are made for persons in need of intensive care, some are designed for very active persons using the wheelchair all day long. In order to cover the individual needs of wheelchair occupants, Frank Mobility Systems, Inc. offers a variety of add-on drives and portable wheelchair motors that enhance the benefits of a manual wheelchair. All of these power add-on drives can be retrofitted to an existing wheelchair with a special bracket. The wheelchair does not need to be modified and maintains its characteristics, such as portability. Frank Mobility also offers solutions for climbing stairs without renovations being made to the home, and recreational products such as the Duet Wheelchair Bicycle Tandem.

College Resources for Students with Disabilities

bestcolleges.com/resources/disabled-students

Prospective college students with disabilities find that many campuses are equipped with offices and services that address accessibility, accommodation, and assistive technology for a diverse range of needs. Student services offices and disability coordinators at many colleges work to make campuses inclusive environments through specialized advocacy, support, and academic services. The increased visibility of these resources makes college a very compelling option for people with disabilities. In addition to campus-based resources, students with disabilities also are protected by state, federal, and local laws prohibiting discrimination and requiring equal levels of access to academic services, environments, and resources. This guide explains the legal rights of students with disabilities—both physical and learning—and the campus resources that can provide assistive services and tools. Additionally, a number of sites, apps, and software resources are listed that are designed to aid students with specific types of disabilities, be they physical impairments or learning disabilities.

Cord Blood Banking

viacord.com/cord-banking/sibling-connection

ViaCord's Sibling Connection program is available for families who have a child with MPS or ML and are expecting another child. ViaCord offers no-cost cord blood collection, processing, and lifetime storage for full siblings (same biological parents) of a child with an eligible diagnosis.

Resources for Siblings

siblingleadership.org

The mission of the Sibling Leadership Network is to provide siblings of individuals with disabilities the information, support, and tools to advocate for their brothers and sisters, and to promote the issues important to them and their entire families.

siblingsupport.org

SibNet, the first and largest online community for adult siblings from around the world, is co-sponsored by the Sibling Support Project and the Sibling Leadership Network.

theblairconnection.org

This network for siblings of children with terminal illnesses allows opportunity to share stories and provides support.

siblingswithamission.org

Siblings with a Mission seeks to provide support internationally, raise awareness, and inspire siblings to make a difference.

Electric Scooters for Little People

assistinglittlepeople.com

Assisting Little People offers the GoGo Elite electric scooter for little people. With a shorter seat height, crutch holder and extra-large rear basket, the GoGo Elite provides a comfortable solution for those with a smaller stature.

NeedyMeds

needymeds.com

NeedyMeds is a non-profit resource devoted to making information about assistance programs available to low-income patients and their advocates at no cost. Databases such as patient assistance programs, disease-based assistance, free and low-cost clinics, government programs, special needs camps, and other types of assistance programs are just some of the resources available.

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Quarters 1 and 2

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MPS CLASSIFICATIONS

Mucopolysaccharidoses (MPS) and mucopolipidoses (ML) are genetic lysosomal storage diseases caused by the body's inability to produce specific enzymes.

Normally, the body uses enzymes to break down and recycle materials in cells. In individuals with MPS or ML, the missing or insufficient enzyme prevents the proper recycling process, resulting in the storage of materials in virtually every cell of the body. As a result, cells do not perform properly and may cause progressive damage throughout the body, including the heart, bones, joints, respiratory system and central nervous system. While the disease may not be apparent at birth, signs and symptoms develop with age as more cells become damaged by the accumulation of cell materials.

| SYNDROME | EPONYM | ENZYME DEFICIENCY |
|------------------|-------------------------------------|---|
| MPS I | Hurler, Scheie, Hurler-Scheie | α -L-Iduronidase |
| MPS II | Hunter | Iduronate sulfatase |
| MPS IIIA | Sanfilippo A | Heparan <i>N</i> -sulfatase |
| MPS IIIB | Sanfilippo B | α - <i>N</i> -Acetylglucosaminidase |
| MPS IIIC | Sanfilippo C | Acetyl CoA: α -glycosaminide acetyltransferase |
| MPS IIID | Sanfilippo D | <i>N</i> -Acetylglucosamine 6-sulfatase |
| MPS IVA | Morquio A | Galactose 6-sulfatase |
| MPS IVB | Morquio B | β Galactosidase |
| MPS VI | Maroteaux-Lamy | <i>N</i> -Acetylgalactosamine 4-sulfatase (arylsulfatase B) |
| MPS VII | Sly | β -Glucuronidase |
| MPS IX | | Hyaluronidase |
| ML II/III | I-Cell, Pseudo-Hurler polydystrophy | <i>N</i> -acetylglucosamine-1-phosphotransferase |

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