NEWBORN SCREENING RESOURCES
Tools for advocacy, educational and family support services, webinars and more!
PAGE 40

2022 RARE DISEASE WEEK: FEB. 22–MARCH 3
Join the Advocacy Committee on Capitol Hill or virtually to help create policy change
PAGE 39

MPS MUSIC CITY
36th Annual Family Conference to be held in Nashville, TN
PAGE 33
# Table of Contents

**BOD Election Results** ......................................................3
**Nord Summit Recap** .......................................................4
**Science & Research** ......................................................7
**Clinical Trials & Emerging Therapies** .........................10
**Fundraising** .................................................................15
**Pathways** ...............................................................29
**Family Support** ..........................................................30
**Adult Resource Committee** ...........................................36
**Advocacy** .................................................................38
**Resources** .................................................................40
**Donations** .................................................................45
**Bereavement Support** ..................................................47
**Classifications** ..........................................................48
**Board of Directors** .......................................................49

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**ON THE COVER:**
Main photo: Taytem White (MPS I); Inset photos: Makayla Hayes (MPS VI), Dominic Henriquez (MPS II), Jesse Taormino (MPS IIIA)
LETTER FROM THE CHAIRMAN

I wish you all **peace and joy** this holiday season!

It has been a very busy time at the National MPS Society. As the holiday season approaches, I want to take this opportunity to say thank you to our staff and all the volunteers at the Society who continue to push forward our mission. You will see their accomplishments clearly throughout these pages. I am very grateful for their incredible efforts.

This year the board of directors and various committee members collaborated to continue the outstanding work of the Society. I am amazed at the number of projects completed each year. Our community is so fortunate to have such outstanding individuals working toward a common goal.

As the year ends, so do our board of directors’ terms. I want to personally thank outgoing board members Larry Kirch and Amber Mongan. They have both greatly impacted the Society’s mission and their volunteer hours have made a huge difference. They will be missed as members of the board, but I look forward to their continued involvement as exceptional members of the Society.

We are looking forward to resuming in-person meetings in 2022, including the Annual Family Conference in Nashville, TN.

Although our virtual format provided unprecedented access and reach to our families, we dearly miss the personal connection that can only come from time spent face to face. For me, this time together over the years has been very meaningful and I cannot wait to see you in August! For those unable to join us, we are working on a hybrid delivery model so that all members will continue to have access to critical educational sessions and research updates.

Thanks to the entire Society staff for their dedication to our mission. It has been another year of many challenges and exciting opportunities. As an MPS mom (Jack, 19, MPS II), I am so grateful for having this amazing support system within this community. I wish you all peace and joy this holiday season!

Sincerely,

Lisa P. Todd, chairman, board of directors
We continue to develop relationships with companies that are in clinical trials and have developed treatments for MPS and ML diseases.

In late September, BioMarin hosted its Annual North America Summit, and we were fortunate once again to participate. Leslie Urdanet presented on the Society’s invaluable Pathways program and how we assist families that are newly diagnosed with MPS or ML. We have now supported more than 250 families directly through the program. Following this conference, I participated in the BioMarin Latin America Achondroplasia Patient Advocacy Summit. Our presentation was directed toward advocacy efforts for newborn screening in the United States. Other countries, like Latin America, hope to gather best practices and materials and grow their legislative efforts.

I also was honored to participate in two additional conferences and present on newborn screening. The first was the Simons Foundation Autism Research Initiative conference. This conference invited two patient advocacy groups to discuss pilot design challenges that need additional research. Some of the most credible newborn screening clinicians, researchers, and health departments were stakeholders and included in this meeting. We look forward to reconvening in the future and addressing obstacles in the robust newborn screening program and how we can improve outcomes for the patient community. Finally, I participated in the 17th International Congress on Neuronal Ceroid Lipofuscinosis workshop in St. Louis, MO. We provided a presentation on lessons learned and are learning, and on the MPS I and MPS II Recommended Uniform Screening Panel (RUSP) nominations to assist the Batten disease community in preparing a nomination for CLN2. The workshop provided us with a full lysosomal disease perspective and demonstrated the need to help one another on difficult policy initiatives.

We are saying goodbye to board members who are retiring. Larry Kirch has served a total of four years this term and had served eight years in an earlier board term. We are grateful for his expert skills in philanthropy, fundraising, and strategic planning. He has guided development at the Society to ensure we maintained key programs, the Annual Fund, and Planned Giving. We are also saying goodbye to Amber Mongan who has been a member of the board since 2014. Amber was the secretary, Fundraising Subcommittee chair, and Family Support chair at various times during her board service. Her abilities to help the Society through identification of new and management of existing programs were just some of her contributions. Returning to the board are Stephanie Cozine, Steve and Amy Holland, Scott and Lynn Hopkins, Austin Noll, and Lisa Todd. In addition, we welcome Tamara Cullere as a new board member for the 2022–2023 term.

We continue to work with the Advisory Council on Heritable Disorders in Newborns and Children on the technical review for evidence of the MPS II RUSP nomination. In February 2022, the advisory council secretary will provide a recommendation to the Health Resources & Services Administration. In the interim, we have been working at the state level on boots-on-the-ground advocacy for MPS I newborn screening programs in North Carolina and Texas. If you have interest in newborn screening efforts in your state, please contact us directly.

We continue to develop relationships with companies that are in clinical trials and have developed treatments for MPS and ML diseases. There are several clinical trials continuing and new ones starting in 2022. You can read more about these on page 10.

Excitement is building at the office for our in-person family and science conference in Nashville, TN, in August—we can’t wait to see everyone! Updates and registration information will be posted to our website in January. Wishing you a joyous holiday and happy new year.

Terri L. Klein, NPGC, president and CEO
The National MPS Society is pleased to announce the 2022–2023 board of directors election results. The new term commences on Jan. 1, 2022, for the following electors:

Stephanie Cozine  
Steve and Amy Holland  
Austin Noll III  
Lisa P. Todd

In November, the Governance Committee appointed Scott and Lynn Hopkins to the board. Lynn currently serves as treasurer. We appreciate all of their continued service.

The Society thanks Larry Kirch and Amber Mongan who are retiring from the board on Dec. 31, 2021. We are grateful for their years of services and many contributions. Many of our programs exist today because of decisions that Larry and Amber participated in. They will be missed.

**WELCOME NEW BOARD MEMBER**

Tamara Cullere is a special education teacher who lives with her family in New Jersey. Her son, Benji, was diagnosed with MPS I in 2017. Her family quickly became members of the Society after diagnosis. Since then, Tamara has attended every family conference and became engaged in the Family Support and Education/Publicity committees. She also is an advocate and has shared Benji’s story on Capitol Hill during Rare Disease Week with both the Society and the Rare Disease Legislative Advocates group. In addition, Tamara has honed her writing skills and is now a frequent blogger and children’s book author.

“I want to be part of creating a community and working toward finding more treatments,” said Tamara. “My heart is with the families. I am looking for ways to help the entire family, especially young siblings, and would love to share that support with the whole community.

“I plan to engage a wider variety of members to join and actively participate in committees and Society events. I will create supports for siblings too young to participate in the sibling committee and transition them to that committee when they are old enough to independently join. I will increase support and access to information from other organizations to provide assistance that would benefit families. The connections between those organizations and the Society would allow more people to learn about MPS and ML.”

**RECOGNIZING LARRY KIRCH AND AMBER MONGAN**

Thank you, Larry and Amber, for your years of service on the board of directors. Larry and Amber served as chairs of the Fundraising and Family Support committees and have contributed countless hours to further the mission and goals of the National MPS Society. We appreciate you!
On Oct. 18 and 19, the National Organization of Rare Diseases (NORD) held a virtual conference for key stakeholders. This year, diverse topics covered public health challenges; impact of COVID-19 on rare disease research; clinical trials; drug development; designing clinical trials incorporating diversity, equity, and inclusion; drug pricing; and the continued evolution of gene therapy and gene editing.

NORD is always a valuable conference to attend. MPS families hear directly from senior CDC, FDA, and NIH leaders. It continues to be both an exciting and heartbreaking time for science breakthroughs for our patient community. Here are summaries of a few of the panel discussions that were held and have an impact on our MPS and ML community.

**RARE DISEASES AS A PUBLIC HEALTH CHALLENGE—PANEL WITH QUESTIONS**

*Christopher Austin, MD*

*Gail Jarvik, MD, PhD*

*Brian Smith, JD, MPH*

**KEY TAKEAWAYS:**

- We have a moment for rare disease—we need to embrace what we know how to do and demand prioritization. We need to get everyone involved and show that rare diseases have a face and have value.

- We need to use our power as advocates by sharing our stories and working collectively to solve problems, which are rapidly becoming solvable as science advances.

**QUESTION ONE: Provide perspective on rare diseases on public health challenge.**

Christopher Austin, MD, asked how we change the public perception to understand rare diseases, so they have knowledge of the burden and costs to change the future dialogue and policy. Intervention to care for patients is crucial. We know how to diagnosis and treat, but we have a “scientific opportunity and medical obligation” to change the perception of the general public and policy makers for rare diseases.

Brian Smith, JD, MPH, from the CDC, challenges the public to think of rare diseases in the aggregate to create a full impact change. Answers on perspective change might be helped at the state level if more research is completed. Public health priority in the aggregate. A better understanding of the epidemiology and what tools can be used to leverage the policymaking spectrum to assist rare diseases.

**QUESTION TWO: Discuss the importance of accurate and timely diagnosis of rare diseases and the impact for public health.**

Today, there are many new tools for diagnosis, including whole genome sequencing and exome genome sequencing. It is possible to diagnose many genetic diseases by looking at the genomes of even newborns. A diagnosis can be made prenatally or at birth. Most of these children go out into the world without a screening or diagnosis and it can take decades for a family to determine what is wrong with their child. Children are suffering from potential irreversible damage from a disease. Current health systems do not reimburse most of the diagnostic technologies. Advocates need to make clear that the costs are about $1,000/child, and we need to do more to raise awareness. The value is evident in the importance for public health. —Chris Austin, MD

**QUESTION THREE: What levers can we push to create public policy alignment for rare diseases?**

Rare disease is a significant burden on the entire population. If current systems are not working well, then we need to be prudent and tinker with systems and continued
make small changes. We need big policies that have not been done before and determine if we can model a new system using creative problem solving and possible drug pricing model changes. The demand/supply curve for a rare disease is warped and cannot work well with the market forces. Increase communication with regulators and legislators and industry to solve problems. Advocates can create a coalition and band together with stakeholders to raise pricing awareness and create equitable access to a viable, life-saving treatment. —Brian Smith, JD, MPH

QUESTION FOUR: Public health is having a moment during these past 18 months. What are some lessons learned from the COVID-19 experience to help rare diseases?

• Ecosystem of collaboration changed overnight with all key stakeholders.
• Remarkable timeframes were introduced. The health system, which can be burdensome, performed. The United States showed we can collaborate to save lives.
• If we could develop therapies to treat patients with COVID-19, then we can do the same for rare diseases. We do not want to live in a society where we value persons with COVID-19 more than those with a rare disease. We need to remove excuses to save lives beyond COVID-19.

—Chris Austin, MD

QUESTION FIVE: Can you share your downstream impact of COVID-19 or we can adapt to the infrastructure that we can build out to help rare disease?

• People don’t live near specialists. Nearest center near state lines.
• Telemedicine was not reimbursed but is now.
• States granted licensure to meet with patients during the pandemic and insurance paid to support patients. We need to extend this philosophy further for healthcare. More telemedicine across state lines and accessible to patients. Perfect example of what can change overnight to benefit everyone.

—Gail Jarvik, MD, PhD, University of Washington

QUESTION SIX: How do we reframe private healthcare prioritization?

Key themes included all the underlying issues on how healthcare works on a broad sense and how they are threaded together. Became readily available to public at large. Used to be niche issues like patient access and value-added services. All these questions came up during the pandemic. Patients need to insert themselves in these conversations to create policy change. —Brian Smith, JD, MPH

QUESTION SEVEN: What are some important health works that are effective that we want to do more of?

• Undiagnosed disease program seen by region. Look at unsolved gene sequencing disorders. Provide access to whole genome sequencing, exome sequencing, and using functional systems to help in the diagnostic process.
• Collaborations and coalitions—combining resources; expand across other diseases. Building out accessibility for patients and clinical research. Getting more patients and families involved in clinic process and help them participate in additional policy changes.
• There has been disruptive change with COVID-19. We have gone from waiting until a patient is ill and “jury rigging” a system post-hoc, to thinking about it from a screening perspective. We didn’t wait for COVID-19 patients to be sick. We began screening and creating a vaccine. Commonality of rare diseases is key. Find out what they have in common vs. what separates them. We could find answers and solutions more quickly.
• Screen the genomes and find commonalities for treatment. We can create a population prevention screening program—preventing systemic issues and halting the diagnostic journey.

DESIGNING TRIALS FOR INCLUSIVITY, EQUITY, AND ENGAGEMENT

Phillip Pearl, MD, Children’s Hospital of Boston
Richardae Araojo, RAD, PharmD, MS, FDA, Office of Minority Health
Meghan McKenzie, MA, Genetech
Michael Poku, MD, MBA, Oak Street Health

KEY TAKEAWAYS:

• Diverse materials for communities to understand, translational and early access.
• Build local patient navigators and create a blueprint to walk a family through a clinical trial.
• Introduce the personal patient advocator and local navigator support concepts.

continued
• Partner with community health organizations to promote diversity and reach communities.
• Build a grassroots-level effort and reach stakeholders through trusted organizations.

This presentation provided an excellent panel of experts to help create an environment where systemically we are doing a better job of reaching the minority population for clinical trials. Currently the U.S. population is more than 40% nonwhite. This percentage does not match clinical trial participation across the board. Through identification of minority patients, education, and clinical trial sites, we need to amplify best practices and achieve inclusivity, equity, and engagement. We have a social responsibility to give to long-term healthcare infrastructure funding and provide health equity and social equity grants across the country. These grants need to reach underserved populations or those with low economic status. Presenters shared that the FDA is building foundation efforts to address this shortfall.

In a guidance dated November 2020, enrollment practices in trial design include how to broaden methodological approaches. The guidance also challenges how industry can improve recruitment, most likely to use the drug they are creating for patients. The guidance gives recommendations for potential growth metrics and provides threaded layers of accountability for all stakeholders.

The panel discussed how to create a more geographical reach in communities of color and minority. Through education there could be more of a referral outcome. Suggestions included looking at best practices in minority centers and how they engaged with the community and then leveraging those ideas. Also, building cultural competency materials and training for families in social equity giving funds. We need to build competencies and translational materials. We no longer can have funding excuses.

It is noted that we have trust and distrust as a bad legacy in the current health system. The FDA has changed that with improved consent processes, monitoring safety, and being central in the clinical trial process. Consent is crucial and patient families need to understand what they are consenting to in a clinical trial. Finally, the panel addresses better educational materials for clinical trial engagement.

With science exponentially changing the landscape of clinical trials, it is hard for families and patients to keep up. It is important to build materials that are easy to understand, and provide a road map for care management and enrolling into a trial. Drug companies need to help with education, authenticity, and cultural competency.

Society Receives Multi-Year Gift of $100,000 for Family Support Programs

The Pennies for Nicoll Foundation, dedicated to fighting MPS, has gifted the National MPS Society $100,000 for family support programs. This multi-year gift allows us to continue to serve our families in profound and meaningful ways. We will continue to provide medical durable goods, scholarships, assistance to medical appointments, and bereavement support for our MPS and ML families.

Pennies for Nicoll was formed after a diagnosis of Sanfilippo syndrome impacted the Nicoll family. During the ongoing health crisis, Pennies for Nicoll has met a gap for an unmet need.

We are grateful to the Pennies for Nicoll board of directors and Tracie Nicoll, PhD, for identifying the Society to receive this extraordinary gift. Thanks to them, dozens of families will be provided support during their difficult journey.
As my first year as chief scientific officer of the Society concludes, I am mindful of the many roles I try to fulfill. I stay focused on being an active advocate of science and public policy as they impact our disorders. In our community, that means issues around newborn screening and rare disease drug development and approval. I also am conscious of the need to provide vision to our research program so that it addresses the manifold and current needs of our community as well as those that may emerge in the future. As part of this overarching effort, I am pleased to share that I have been asked to serve on the External Advisory Board for the NIH-funded Lysosomal Disease Network Program Project, thus ensuring that MPS and ML diseases have a strong voice in one of our country’s most important research networks.

As a generation of individuals move forward in life equipped with life-saving therapies, the existence of those same therapies means that we will encounter varied responses that uncover further medical and therapeutic challenges. Such emerging aspects of disease will be based on residual disease due to severity associated with a given mutation, innate individual biological variation in response to disease, age at initiation of therapy and type of therapy, and the interactions of all these elements. A challenge ahead will be continuing to track these aspects of a rare disorder so that meaningful action can be taken to benefit patients.

We remain deeply mindful of those in the community whose lives have gone on without a therapy and who, with their families, struggle every day for an improved quality of life. These individuals are every bit as deserving of our research efforts to improve their quality of life, even if we can’t reverse their disease. My experience, both as a researcher of Sanfilippo syndrome and as the primary caretaker for a parent with a neurodegenerative disease, means that the human needs of this group call to me every day.

These are the broad areas that make me so deeply grateful to now be devoting my efforts to the MPS and ML community. This issue of Courage highlights aspects of therapeutic developments and clinical trials, our work in advocacy in the area of newborn screening, and research efforts of the Society as part of its extramural research program.

Since our last issue, travel has opened up to some degree. In August, I attended the World Orphan Drug Congress 2021 in Washington, DC, where I worked with advocacy and industry stakeholders on issues critical to our community. I want to highlight the importance of having an MPS leader at these events. While in DC, I visited one of our industry leaders (REGENXBIO) at their new facility in Maryland. They are a great group committed to delivering a gene therapy to MPS I and MPS II patients in need of a neuro-targeted therapy. Virtual meetings and seminars still abound and as this September was Newborn Screening Month, there were many productive meetings and symposiums on this process.

Here at the Society, we realize that if therapeutics don’t get to patients at the optimal time, there are profound consequences. That makes newborn screening for our disorders with therapies a priority. The MPS II nomination to be included on the Recommended Uniform Screening Panel (RUSP) for newborn screening, which Terri Klein and I put forth last fall with the efforts of a tremendous expert panel, was accepted this past May, and forwarded to the Technical Expert Panel, to which both Terri and I have been appointed. Reviews and meetings continue, so that we can make the strongest case for the science and clinical practice to move this nomination to a successful conclusion. To

continued
that end, many of us are working on the rapid turnaround of scholarly contributions to the peer reviewed literature to document the benefit and need for MPS II to be included on the RUSP. The report from the Technical Expert Panel will be shared this winter with the Advisory Committee on Heritable Disorders in Newborns and Children, which will vote on whether to forward the nomination to the secretary of Health and Human Services, with a recommendation for inclusion of MPS II on the RUSP.

In addition, Terri and I recently visited the North Carolina State Laboratory of Public Health as guests of Dr. Scott Shone, laboratory director. Dr. Shone provided us with a terrific tour of the laboratory, highlighting the newborn screening program in North Carolina, including their exciting plans to begin newborn screening for MPS I.

We were busy here at the Society at the end of September with the submission of abstracts for WORLDSymposium™ 2022. Terri and I have co-authored no less than four abstracts, including case studies, advocacy efforts, and basic science in support of our disorders. Look for a full recap of this coming February’s WORLDSymposium™ in the next issue of Courage.

The extramural grants program continues to move forward productively. The first round of grants (Cycle I) have been awarded and were reported in the fall issue of Courage. Cycle II grants are detailed on the next page. Cycle III letters of intent are currently being reviewed.

As we approach the end of 2021 in hopeful expectation of a post-pandemic life with COVID-19 as a manageable presence, I am working to put together a meaningful science program for our in-person 36th Annual Family Conference to be held in Nashville, TN, Aug. 4–6.

I will continue to bring about as many of your hopes and wishes for research, science, and policy for our community as I am able.

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

MPS IVA Natural History Study

Researchers at Nemours/Alfred I. duPont Hospital for Children in Delaware have received funding from the NIH to complete a study to evaluate the natural history of Morquio A syndrome

Study aims and objectives are as follows:

MPS IVA is a devastating systemic skeletal disease in which detailed progression and pathogenesis remain unknown. The proposed project aims to establish a non-invasive objective assessment that can be applicable to all ages of patients to better understand the progress of their disease and the most serious clinical problems (cervical instability and stenosis, tracheal obstruction, hyperlaxity of joints, hip dysplasia, and small lung capacity). The outcome of this project will lead to a more precise understanding of the skeletal/pulmonary compromise and defining clinical endpoints in this disease for future clinical trials of current or developing therapies.

Three nights lodging and travel costs for two people (patient and caregiver) are included, along with the costs of all tests, with participants receiving results from assessments completed.

Contact the study coordinator with questions at Somto.Jigide@nemours.org.

Scan the QR code for more information.
The National MPS Society’s board of directors is pleased to announce the results from Cycle II of the Society’s 2021 Innovative Research Grants Initiative. The second cycle of grant proposal submissions, review, and awards identified four proposals totaling $200,000 in award funds. The reviews of Cycle III proposals have begun; full proposals are due Jan. 15, 2022, with a funding target date of April 1, 2022, based on the assessments of letters of intent received.

**COMPETITIVE RENEWAL AWARDS (TIER II)**

The Society’s new grant program permits researchers one funded competitive renewal of a previous award. Researchers with awards from earlier years have had their awards grandfathered into eligibility for a competitive renewal. Competitive renewal means the same rigorous review process is used as with first-time awardees. We are excited to announce that Drs. Enrico Moro and Jodi Smith are the recipients of the first such competitive renewal award.

**“Investigation of the Retinotectal and Neurobehavioral Defects in a Zebrafish Model of Hunter Syndrome”**

_Dr. Enrico Moro_  
_University of Padua, Italy_

Dr. Moro has developed a novel MPS II zebrafish model that used an improved approach relative to past approaches to studying genetic diseases in zebrafish. He is continuing his neurodevelopmental characterization of this model with important findings of how we understand neuropathic MPS II disease. His presentation of his current work in this area at the Society’s 2021 Annual Family Conference can be seen on the Society’s YouTube channel.

_Funded ($50,000) with Society support from MPS II research funds._

**“Efficacy of Gene Therapy and PPS Therapy in a Large Animal Model of MPS IIIB”**

_Dr. Jodi Smith_  
_Iowa State University_

Dr. Smith and her team recently identified significant quantitative improvements in brain inflammation and storage markers in response to pentosan polysulfate (PPS) treatment in the MPS IIIB canine model. Her extension study will both complete a proposed study of synergistic effects of gene therapy and PPS in the canine model, as well as evaluate a number of biomarkers using archival samples with the goal of developing an approach to monitoring central nervous system response to therapy via easily accessed sampling. Dr. Smith’s report on her first year of results presented at the Society’s 2021 Annual Family Conference can be viewed on the Society’s YouTube channel.

_Funded ($50,000) with Society support from MPS III research funds._

**NEW AWARDS (TIER II)**

**“A Clinically Relevant MPS IVA Animal Model for Investigation of New Therapies”**

_Dr. Nicola Brunetti-Pierri_  
_TIGEM, Naples, Italy_

This is a particularly exciting award as the MPS IVA field has struggled to find an animal model that will help drive better therapeutic development. The proposed rat model was created using novel CRISPR-based genome editing and has the prospect to significantly move the needle on MPS IVA therapeutic development.

_Funded ($50,000) with Society support from MPS IVA research funds._

**“Role of Polyamines in Disease Progression in Mucopolysaccharidosis IIIA”**

_Dr. Elvira de Leonibus_  
_TIGEM, Naples, Italy_

Over the last decade, our understanding of the cellular and molecular mechanisms underlying the neurodegeneration seen in the neuropathic MPS disorders has greatly improved. Dr. de Leonibus and her team have helped drive this understanding forward and using the current award she continues this effort by examining polyamines, a group of cellular metabolites that hold potential to both help monitor neuropathic disease progressing as well as being a potential therapeutic ameliorating the consequences of neuropathic disease in MPS IIIA model systems.

_Funded ($50,000) with Society support from general research funds._
Emerging Treatments, Clinical Trials, and Therapies
Visit clinicaltrials.gov for more details.

Abeona
MPS IIIA (ABO-102)
MPS IIIB (ABO-101)
An IV AAV-2/9 based one-time IV gene therapy
The positive phase I/II interim data on neurocognitive development of young MPS IIIA patients presented last winter at the 2021 WORLD Symposium™ was updated Oct. 21 at this year’s European Society of Gene and Cell Therapy’s virtual congress. Earlier reports detailed neurocognition preserved up to three years following treatment with ABO-102 gene therapy. The Transpher A study will be the subject of a presentation titled “Interim Results of Transpher A, a Multicentre, Single-dose, Phase 1/2 Clinical Trial of ABO-102 Investigational Gene Therapy for Sanfilippo Syndrome Type A (Mucopolysaccharidosis IIIA).”
In addition to encouraging clinical trial results, Abeona also reported a successful Type B meeting with the FDA for ABO-102 this past July. For additional information, visit abeonatherapeutics.com.

NIH, U.S. National Library of Medicine, Clinical Trials
MPS IIIA
Phase I/II Gene Transfer Clinical Trial of scAAV9.U1a.hSGSH: NCT02716246
A Long-term Follow-up Study of Patients with MPS IIIA Treated with ABO-102: NCT04360265
Gene Transfer Study of ABO-102 in Patients with Middle and Advanced Phases of MPS IIIA Disease: NCT04088734

MPS IIIB
A Long-term Follow-up Study of Patients with MPS IIIB Treated with ABO-101: NCT04655911

Allievex
MPS IIIB (Tralesinidase alfa (AX 250))
An ICV ERT with a GILT-tagged NAGLU
Allievex continues the phase I/II clinical extension trial of tralesinidase alfa (AX 250), an investigational enzyme replacement therapy, using a novel fusion of recombinant human alpha-N-acetylg glucosaminidase (NAGLU) with a glycosylation independent lysosomal targeting (GILT) peptide tag derived from insulin-like growth factor 2 (IGF2) for the treatment of MPS IIIB. Designed to restore NAGLU activity in the brain, AX 250 is delivered directly to the fluid surrounding the brain (cerebrospinal fluid) by an intracerebroventricular (ICV) port infusion.
The most current report was at the WORLD Symposium™ 2021, and featured some of the most encouraging data to date of an MPS III clinical trial. WORLD Symposium™ 2021 presentation details can be found at allievex.com/post/worldsymposium-2021.

NIH, U.S. National Library of Medicine, Clinical Trials
MPS IIIB
A Study of Mucopolysaccharidosis Type IIIB (MPS IIIB) Completed. Also referred to as AX 250-901, a natural history study of MPS IIIB. NCT02493998
A Prospective Natural History Study of Mucopolysaccharidosis Type IIIB (MPS IIIB) Active, not recruiting. Also referred to as AX 250-902, a natural history study of the progression of MPS IIIB over time in children 0–18 years of age with all levels of cognitive function. NCT03227042
A Treatment Extension Study of Mucopolysaccharidosis Type IIIB Active, not recruiting. Also referred to as AX 250-202, a treatment extension study. NCT03784287
An extension of the completed AX 250-201, which enrolled and treated patients from AX 250-901. NCT02754076

Denali Therapeutics
MPS II (DNL310)
An IV IDS ERT designed to transit the blood brain barrier (BBB)
Denali Therapeutics has continued development of DNL310, a BBB transiting enzyme replacement therapy for MPS II, designated DNL310. The therapy employs an Enzyme Transport Vehicle for IDS (ETV:IDS). Interim data from a phase I/II study of ETV:IDS (DNL310) for the potential treatment of Hunter syndrome was presented in July. The presentation, called “Interim 24-week Results of Cohort A in a Phase I/II Study of Intravenous DNL310 (brain-penetrant enzyme replacement therapy) in MPS II,” is available on
Denali’s website. Highlights of the presentation show positive six-month data from DNL310 phase I/II study in MPS II. The hallmark biomarker glycosaminoglycan normalized in the cerebrospinal fluid in all patients; global impression scales show improved cognition, behavior, and physical function; exploratory lipid biomarkers consistently improved; and the safety and tolerability profile is consistent with current standard of care. To view the full presentation, visit denalitherapeutics.com/investors.

The U.S. Food and Drug Administration (FDA) granted Fast Track designation to ETV:IDS (DNL310) for the treatment of patients with MPS II in March 2021. Fast Track is an FDA process, designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. This FDA designation usually increases frequency of information with the FDA, could allow earlier development of DNL310 for the treatment of MPS II, enables a rolling review of the research and potential priority review of the application. For more information, visit denalitherapeutics.com.

**NIH, U.S. National Library of Medicine, Clinical Trials**

**MPS II**
A Study of DNL310 in Pediatric Subjects with Hunter Syndrome
   NCT04251026
A Study of Potential Treatment-Responsive Biomarkers in Hunter Syndrome
   Recruiting: NCT04007536

**ESTEVE**

**MPS IIIA (EGT-101), phase I/II**
**MPS IIIB (EGT-201), preclinical**
**MPS II (EGT 301), preclinical**

An AAV-2/9 based one-time ICV gene therapy

ESTEVE, a biotechnology company based in Spain, employs a viral vector (AAV-2/9) to deliver the correct copy of the defective gene. The vector delivery is via a one-time intracerebroventricular (ICV) injection. The MPS IIIA phase I/II trial is fully enrolled, and the MPS IIIB and MPS II programs are in the preclinical stage. MPS IIIA is in phase II and MPS IIIB is in phase I. Clinical trials are outside the United States.

Additional information about the MPS IIIA clinical trial can be found at [https://reec.aemps.es/reec/estudio/2015-000359-26](https://reec.aemps.es/reec/estudio/2015-000359-26).

**Homology Medicines, Inc.**

**MPS II (HMI-203)**

A human hematopoietic stem cell derived AAV (AAVHSC)-based vector one-time gene therapy

Homology Medicine, Inc., a biotechnology and gene therapy company based in Bedford, MA, has announced plans for an MPS II gene therapy clinical trial. Although a new name for many in the MPS and ML community, the company features a group of human hematopoietic stem cell derived AAV (AAVHSC)-based vectors, with blood-brain barrier transiting properties. The leadership also features scientists formerly employed by Shire, meaning despite the newness of the company, the knowledge and abilities of this organization have significant contributions to make to the MPS and ML field. This October, the FDA approved the Investigational New Drug application for HMI-203, clearing the way for the juMPStart trial which targets MPS II. This trial, expected to launch in 2022, will be a dose-escalation trial evaluating the safety and efficacy of a one-time single IV infusion of the gene therapy product (HMI-203). For details, visit homologymedicines.com/news.

**Inventiva Pharma**

**MPS VI (odiparcil)**

An oral GAG clearance therapeutic

Inventiva Pharma, a French pharmaceutical company specializing in small molecules for unmet medical need, is developing odiparcil for the treatment of several MPS subtypes with a current concentration on evaluation in MPS VI. Odiparcil is a small, orally available molecule that is well distributed in the body, including in cartilage and eyes, which are tissues that are poorly penetrated by enzyme replacement therapy. Odiparcil’s ability to produce two forms of soluble glycosaminoglycans (GAGs) [dermatan sulfates (DS); chondroitin sulfates (CS)], which can be excreted via urine, makes it potentially suited to treating MPS I, II, IVA, VI, and VII patients, where these types of GAGs accumulate. Odiparcil changes the way DS and CS are synthesized, thereby facilitating the production of soluble DS and CS GAGs, which can be excreted in the urine, rather than accumulating in cells.

In the recently completed iMProveS phase IIA study, odiparcil confirmed its favorable safety profile and demonstrated efficacy in adult MPS VI patients, especially in tissue types not reached by standard of care. Most recently, Inventiva received the Fast Track designation in MPS VI for its clinical stage asset for odiparcil. Despite these promising results, the company is pausing further development of odiparcil while it develops other front line products for non-alcoholic hepatitis. For complete details, visit [https://inventivapharma.com/wp-content/uploads/2020/11/Inventiva-PR-EOP2-Meeting-EN-10-11-2020-1.pdf](https://inventivapharma.com/wp-content/uploads/2020/11/Inventiva-PR-EOP2-Meeting-EN-10-11-2020-1.pdf).

**NIH, U.S. National Library of Medicine, Clinical Trial**

**MPS VI**
A Study in MPS VI to Assess Safety and Efficacy of Odiparcil (completed)
   NCT03370653

continued
JCR Pharmaceuticals

**MPS II** *(Pabinafusp alfa, JR-141)*

**MPS I** *(JR-171)*

**An IV ERT designed to transit the blood brain barrier (BBB)**

JCR employs a BBB transiting technology involving the transferrin receptor on the vasculature of the central nervous system to shuttle enzyme into the brain, a tissue otherwise resistant to enzyme penetration via normal enzyme replacement therapy (ERT). Earlier this year, JCR secured approval of IZCARGO® for the treatment of MPS II in Japan, the first-ever approved ERT to penetrate the BBB via intravenous administration. Pabinafusp alfa is the IDS enzyme fused with anti-human transferrin receptor antibody, and thus targets the neurodegeneration in MPS II. JCR plans to initiate a phase III clinical trial for MPS II patients in the United States.

Other recent developments include accelerated drug status (PRIME Designation) granted from the European Medicines Agency, and a partnership with Takeda to commercialize JR-141 outside the United States, Japan, and certain other Asia-Pacific countries, with the potential to pick up U.S. rights after phase III work is completed. The trial also will be conducted in Brazil, the UK, France, and Germany.

JCR announced in October 2020 that the first MPS I patient was dosed with JR-171 in the phase I/II global clinical trial. JR-171 has been granted Orphan Drug status from the FDA (Feb. 2021). The study will be held in the United States, Brazil, and Japan with patients enrolled first in Japan and Brazil.

**NIH, U.S. National Library of Medicine, Clinical Trials**

**MPS II**

Multiple Phase I/II Trials and Their Associated Extension Studies

- All completed, or active and not recruiting, with foreign sites (Japan and Brazil)
  - NCT03128593
  - NCT03359213
  - NCT03708965
  - NCT03568175
  - NCT04348136

A Phase III Study of JR-141 in Patients with Mucopolysaccharidosis II

- Active, not yet recruiting (no sites listed): clinicaltrials.gov/ct2/show/NCT04573023

**MPS I**

An Entry Trial and an Extension Study of JR-171 (both recruiting: Japan and Brazil)

- NCT04227600
- NCT04453085

The Lundquist Institute at Harbor-UCLA Medical Center

**MPS III** *(Kineret (generic name: anakinra))*

**MPS I and MPS II** *(HUMIRA® (generic name: adalimumab))*

**Immunosuppressive therapy for aspects of somatic and CNS disease in MPS**

Dr. Lynda Polgreen, a clinician scientist at the Lundquist Institute (Torrance, CA) has two trials ongoing that are looking at repurposing drugs used in autoimmune disease. The rationale of using these drugs is based on preliminary findings in animal models and clinical knowledge. The hope is that the blockade of the immune activation and inflammation cascade induced by stored glycosaminoglycan (GAG) substrates will benefit patients. Ongoing studies target both the connective tissue aspects of disease, and pain in MPS I and MPS II, as well as the central nervous system disturbances that can make later stage MPS III difficult for patients and their caregivers. The MPS III/anakinra study has successfully been enrolling patients, with an anticipated completion of enrollment in November 2021, with plans currently in place to expand into a phase III trial.

**NIH, U.S. National Library of Medicine, Clinical Trials**

**MPS III**

Open-label Study of Anakinra in MPS III

- Active, not recruiting: NCT04018755

**MPS I and MPS II**

Study to Evaluate the Safety and Efficacy of Adalimumab in MPS I and II

- Recruiting: NCT03153319

Lysogene

**MPS IIIA (LYS-SAF302)**

**An AAVrh.10 based intraparenchymal brain gene therapy via a one-time surgery**

LYS-SAF302’s efficacy, safety, and tolerability have been assessed in a phase II/III trial in the United States and Europe, called the AAVance study. Nineteen children in the trial are in the phase III portion of the trial, which is fully enrolled, active, and due to conclude in March 2022. This therapy may help stabilize and/or repair neuronal damage; it has been given Fast Track and Rare Pediatric Disease designations.

**NIH, U.S. National Library of Medicine, Clinical Trials**

**MPS IIIA**

Natural History Study of Patients with MPS IIIA

- Competed: NCT02746341

Study of AAVrh10-h.SGSH Gene Therapy in Patients with Mucopolysaccharidosis Type IIIA

- Active, not recruiting: NCT03612869

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Continued
Orchard Therapeutics

MPS IIA (OTL-201)
MPS IIB (OTL-202)
MPS I (OTL-203)

Transplantation of ex vivo lentiviral transduced autologous hematopoietic stem cells

Orchard Therapeutics uses a patient’s own stem cells (autologous cells) and delivers the missing gene to harvested cells ex vivo. These cells are then transplanted back into the patient. This technology has been used successfully in severe immune deficiencies (a group of potentially fatal “bubble boy” diseases), and the approach is being used for neuropathic MPS diseases. By giving these gene-corrected cells to the patient, Orchard aims to permanently correct genetic disorders with a single treatment. This approach relies on the intrinsic ability of blood stem cells, also known as hematopoietic stem cells or HSCs, to self-renew in a patient’s bone marrow and produce new blood cells of all types. This approach also avoids the need for a donor-based transplant which uses cells from another person and can result in serious complications including graft-versus-host disease. The patient’s own over-expressing stem cells will have the potential to repopulate the brain with high-expressing gene therapy transduced cells.

The proof-of-concept trial for MPS IIA with OTL-201 has met its recruitment goal with the enrollment of a fifth patient. Interim data from this study was presented at the 16th International Symposium of MPS and Related Diseases; further updates are expected to be presented at medical meetings later in 2021 and 2022. Plans are in place to move into clinical work for MPS IIB with OTL-202, and MPS I with OTL-203. Orchard has received informative feedback from both the EMA and the FDA for the structuring of a revised global clinical study protocol for OTL-203, with a potential study initiation date in 2022.

NIH, U.S. National Library of Medicine, Clinical Trials

MPS IIA
Gene Therapy with Modified Autologous Hematopoietic Stem Cells for Patients with Mucopolysaccharidosis Type IIA
Active, not recruiting: NCT04201405

Paradigm Biopharma

MPS I and VI
Injectable (SC) pentosan polysulfate sodium (iPPS) for MPS pain and joint disease

The Australian biopharmaceutical company Paradigm Biopharma is developing licensed drug technology pioneered by Mt. Sinai researchers and MPS Society Scientific Advisory Board members Drs. Simonaro and Schuchman, and have launched trials in Australia (MPS I, phase I/II trial, 10 patients total) and Brazil (MPS VI, phase II). The first patients in Australia were treated in November 2020, and the first patient in Brazil was treated in June 2021. With both FDA and EMA Orphan Drug designations, Paradigm looks forward to being able to expand their clinical program. MPS I data was presented in November at the International Congress on Inborn Errors of Metabolism.

ReGenesisBio, Inc.

MPS II (RGX-121)
MPS I (RGX-111)

An AAV-2/9 based one-time intra cisterna magna CNS directed gene therapy

ReGenesisBio has active, recruiting trials for MPS I and MPS II, both employing a similar AAV vector targeted to the central nervous system via a one-time intra-cisterna magna injection of vector into the cerebrospinal fluid. Positive interim data from nine MPS II patients in escalating dose cohorts 1, 2, and 3 from the ongoing phase I/II clinical trial for RGX-121 were presented at the American Society of Gene and Cell Therapy’s 24th Annual Meeting. Recognizing the unmet need in older MPS II patients, ReGenesisBio also has begun enrolling a trial for patients age 5 and older (see Clinical Trial title and number below). The MPS I clinical trial enrolled and dosed its first patient in December of 2020.

NIH, U.S. National Library of Medicine, Clinical Trials

MPS II
RGX-121 Gene Therapy in Patients with MPS II (Hunter Syndrome)
Recruiting: NCT03566043

RGX-121 Gene Therapy in Children 5 Years of Age and Over with MPS II (Hunter Syndrome)
Recruiting: NCT04571970

MPS I
RGX-111 Gene Therapy in Patients with MPS I
Recruiting: NCT03580083

University of California, San Francisco

MPS I, II, IVA, VI, and VII
In utero ERT with approved ERT products

In what is a milestone clinical trial, Dr. Tippi Mackenzie of the UCSF has initiated with collaborators at Duke a phase I clinical trial for up to 10 patients, each with one of eight lysosomal storage diseases, including the five MPS disorders with an approved enzyme replacement therapy product. The trial is newly opened, and is recruiting MPS pregnancies at 18–34 weeks of gestation. More information on this exciting trial can be found at https://fetus.ucsf.edu/in-utero-enzyme-replacement-therapy.

NIH, U.S. National Library of Medicine, Clinical Trials

MPS I
RGX-111 Gene Therapy in Patients with MPS I
Recruiting: NCT03580083
MPS I
Aldurazyme®, administered once-weekly, is approved in more than 60 countries worldwide, including the United States and the European Union, for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of MPS I to treat the non-neurological manifestations of the disease. Aldurazyme was developed by BioMarin and Genzyme under a joint venture agreement that assigns commercial manufacturing responsibilities to BioMarin, and worldwide sales and marketing responsibilities to Sanofi Genzyme. Additional information can be obtained at aldurazyme.com or by contacting Sanofi Genzyme CareConnectPPS® at 800.745.4447.

MPS II
Elaprase® is a long-term ERT for patients with a confirmed diagnosis of MPS II, approved for use in the United States, Canada, and many countries in Europe. Elaprase was developed and is produced by Takeda (formerly Shire Human Genetic Therapies), and is given as weekly infusions to replace the missing enzyme that Hunter syndrome patients fail to produce in sufficient quantities. Additional information can be obtained at takeda.com or by contacting OnePath℠ toll-free at 866.888.0660. OnePath provides assistance with insurance, product access, treatment centers, and education about Elaprase and MPS II.

MPS III
There currently are no approved therapies for Sanfilippo syndrome. There are clinical trials and investigative research transpiring and noted earlier.

MPS IVA
Vimizim™ (elosulfase alfa), developed by BioMarin Pharmaceutical, Inc., is the ERT for individuals with MPS IVA (Morquio A syndrome). Vimizim improved endurance in clinical trials and is administered weekly via intravenous infusion. BioMarin offers support to patients through its BioMarin RareConnection™ program, from which patients receive live, personalized support by a specialized case manager who will research insurance coverage and alternative benefit options. RareConnection will help patients obtain coverage, minimize out-of-pocket expenses, and find alternative financial assistance for treatment. Call toll-free at 866.906.6100.

MPS VI
Naglazyme® is the ERT for individuals with a confirmed diagnosis of MPS VI (Maroteaux-Lamy syndrome) and has been approved for use in the United States and in many European countries. Developed and produced by BioMarin Pharmaceutical, Inc., Naglazyme® has been shown to improve walking and stair-climbing capacity. For more information, contact BioMarin Patient and Physician Support at 866.906.6100 or bpps@bmrn.com.

MPS VII
Mepsevii™ is the ERT for individuals with a confirmed diagnosis of MPS VII (Sly syndrome) and has been approved in the United States and other European countries. Developed and produced by Ultragenyx Pharmaceuticals, Mepsevii (vestronidase) has shown improvement in children living with MPS VII. It is indicated in pediatric and adult patients for the treatment of MPS VII. The effect of Mepsevii on central nervous system manifestations has not been determined. For more information contact the Ultracare™ support team at 888.756.8657 or visit ultragenyx.com/patients/access-approved.
The Society hosted two virtual runs at the beginning of the year, raising a record amount through the run/walk program. More than 1,100 participants from across the country participated in these efforts, raising $125,000 through individual contributions and corporate sponsorship alone. In May, we celebrated International MPS Awareness Day through a virtual gala featuring Broadway performances, a magician, and live and silent auctions. We were not able to gather in person, but the spirit of support prevailed, raising more than $202,000 through this outstanding event.

Eventually families began to meet in person for annual fundraising events. The Lessing family hosted the 30th Annual Post Office Café 5K Race in Babylon, NY. The Mask family celebrated the 10th Annual Concert for the Cure in Sellersville, PA, and the Hopkins family hosted the 12th Annual cabi clothing party in Long Beach, CA. Families across the country continue to support the Society through many efforts including lemonade stands, cycling events, motocross races, motorcycle rides, chili cook-offs, and various fundraising sales. The success of our fundraising program would not be possible without the support of these amazing events, large and small!

One constant fundraising effort of the Society is the Annual Fund. Gifts from the Annual Fund strengthen the Society’s capacity to carry out our mission. Please help us support families, fund newborn screening initiatives, and advocate for increased research funding by making a contribution.

There is still time to consider one last donation to the National MPS Society before the year’s end through the Annual Fund, planned giving, or unrestricted support. It is easy to donate online and select how you’d like your gift to be allocated. Use the “Donate” button on our website and help the Society serve its mission to this incredible community. The National MPS Society needs your support. Fundraisers and gifts, both large and small, help sustain our programs and the mission of our organization.

This December marks the end of Larry Kirch’s tenure as fundraising chair for the National MPS Society. Larry has been a powerful asset to the Fundraising Committee for many years, sharing his knowledge of fund development, planned giving, and strategic planning. Most of all, Larry has been an incredible advocate for the success of the Society and all of our fundraising efforts.

We are grateful for your continued support, and we can’t wait to see you in person in 2022. Happy holidays and a happy new year!

Katelyn Blackman, development director
SAVE THE DATE
JOIN US AT A 2022 RUN/WALK EVENT NEAR YOU!

MARCH 26: RALEIGH RUN FOR RARE
Raleigh, NC

MAY 1: NAPA MPS RACE FOR A CURE
Napa, CA

AUG. 6: NASHVILLE ROCK AND ROLL 5K RUN AND 1 MILE WALK
Nashville, TN

SEPT. 10: LONG BEACH 5K RUN AND 1 MILE WALK
Long Beach, CA

SEPT. 2022: ALAMO BATTLE FOR MPS
San Antonio, TX

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.

Call the National MPS Society office at 919.806.0101 with questions.
FUNDRAISING EVENTS

JULY 1 - OCT. 15, 2021

American Legion Fundraiser  in memory of Liam Johnson

cabi Clothing Party  hosted by Lynn and Scott Hopkins  in honor of Michelle Hopkins

Cincinnati Country Day School Event  hosted by Logan Beaudoin’s second grade class  in honor of Logan Beaudoin

Concert for the Cure  hosted by Dorothy and Jeremy Mask  in memory of Ryan Mask

Golf Tournament  hosted by Donald and Lynda Witte  in memory of John L. Loy

Jack “The Beast” Bennett Memorial Ride  hosted by the Bennett family  in memory of Jack Bennett

Kramer Chili Cook-off  hosted by Beth and Jayson Kramer  in honor of Marcus Kramer

Lilah’s Lemonade Stand  hosted by Kimber Heiling  in honor of Lilah Mueller

Pampered Chef Fundraiser  hosted by Jackie Mitchell  in memory of Dylan Poole

Pampered Chef Fundraiser  hosted by Kathleen and Sean Joyce  in honor of Bridget Joyce

Pearl City Popcorn Fundraiser  hosted by Wayne and Joan Eppehimer

Post Office Café 5K Run and 1K Fun Run  hosted by the Lessing family  in memory of Mark and Casey Lessing

Purple Pumpkin Smash  hosted by Emily Viti and Darryl Hohn  in honor of Christopher Hohn

Scentsy Fundraiser  hosted by Jennifer Jones  in memory of Clara Gibson

Stevens Family Fundraiser  hosted by Marla and Randy Stevens  in honor of Meekel Stevens

The Big Bake for MPS and ML  hosted by the National MPS Society

NATIONAL MPS SOCIETY RECEIVES FOUR-STAR CHARITY RATING

The National MPS Society is pleased to announce that it has received the prestigious four-star Charity Navigator rating. This “exceptional” designation from Charity Navigator differentiates the National MPS Society from its peers and demonstrates to the public that it is worthy of their trust. 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 way. Only approximately one quarter of the charities evaluated have received the highest rating, indicating that the National MPS Society outperforms most other charities in America.

COURAGE PAGES

Courage Pages are custom web pages that allow families to share their stories and honor loved ones. They are an effective way to reach donors and help enhance fundraising efforts with no expense. To create a personalized Courage Page, go to mpssociety.org/give/courage-pages.

MPS CHAMPION CIRCLE

The National MPS Society’s monthly giving program is a unique opportunity to give throughout the year. Families often utilize this method of giving to distribute their Annual Fund gift or other form of annual giving on a monthly basis.

If you would like to join this effort, call the National MPS Society office at 919.806.0101. You also can register your monthly gift online by visiting bit.ly/mpschampioncircle or scanning the QR code.
FUNDRAISING

Your Generosity Pays It Forward!

The Society's fundraising program is successful because of the hard work that began more than 20 years ago and continues today. In the late 1990s, the National MPS Society had two revenue sources: member dues and a bit of revenue from conferences. We now have more than 14 revenue sources that fund our mission.

At the start, the Society had no scientific research program, no advocacy program, and no family support program. In 1998, the Society took a giant leap forward when the board of directors began to dramatically emphasize family fundraisers, a national walk/run program, and casual dress day fundraising. The Society hired an executive director, followed by a development director and family support coordinator. We obtained partnership funds from pharmaceutical companies, received a treasured endowment gift from Dr. Emil Kakkis and Jenny Soriano, and started to receive “major” gifts from donors.

People give to successful organizations, and the Society has a proven track record of tirelessly working to change the lives of those affected by MPS or ML. Your generosity makes that happen!

Generosity takes many forms, such as when you:

• host a fundraiser or volunteer at an event;
• host or donate to a Facebook fundraiser;
• participate in a Society sponsored or nearby run/walk event;
• host a bowling event, bake sale, washer toss, concert, lemonade stand, or motocross race;
• donate to the Annual Fund;
• donate an item for our gala fundraiser;
• bake for The Big Bake for MPS and ML;
• ride your bike in the Million Dollar Bike Ride; or
• when you give through workplace giving through the United Way or as a federal employee.

The last two years have tested all of us, but it has not diminished our resolve to find additional treatments and cures for MPS and ML, advocate on Capitol Hill, or provide premier support to our families and those with MPS or ML.

Without your support and generosity, the National MPS Society cannot:

• sponsor $750,000 annually in Society and partner research;
• go to Capitol Hill and speak to federal legislators and NIH administrators;
• move the needle for newborn screening;
• conduct one-on-one support through our Pathways program;
• host master classes for physicians and social workers;
• provide outstanding publications like Courage and Angels Among Us;
• fund eight Family Support programs:
  1. Bereavement Expense
  2. Continuing Education Scholarship
  3. Extraordinary Experiences
  4. Family Assistance
  5. Family Conference Scholarship
  6. Journey Assistance
  7. Medical Travel Assistance
  8. Regional Social Events

The list of what the Society accomplishes because of your generosity is much longer. Visit our website to get the full picture. Be generous of your time, talent, and philanthropy, and you will not only pay it forward immediately to a deserving family or help fund novel research but will also help those who may walk in our shoes someday. This organization is not what it is today because we thought the task was too big, we were alone in our effort, or because we had no hope. We are making a difference and changing outcomes for affected individuals because we had a common purpose and shared vision for the future. Our work is not done. Please let’s all do just a little bit and help each other help others.

It has been my honor to serve the National MPS Society as a board member, advocate, friend, mentor, volunteer, and fundraiser since 1998.

Larry Kirch, father of Allison Rose Kirch (MPS IIIA) and Helen Kirch (co-chair, Sibling Committee); chair, Fundraising Committee (2000–2008 and 2018–2021)
The Lessing family hosted the 30th Annual Post Office Café 5K Run and 1K Fun Run in Babylon, NY, on Oct. 16. This event is held in celebration of Mark and Casey Lessing, and raises funding for MPS III research. This year more than $32,000 was raised by 650 participants, a record number for the race! There is a true sense of community in Babylon. Small businesses donate merchandise, gift certificates, and artwork for an incredible raffle. Runners gather the evening prior to the race for drinks and fellowship, and stay after the race for a meal and celebration. Thank you to the Lessing family and staff of the Post Office Café for hosting this tremendous event!

Lilah’s Lemonade Stand had a very successful summer. Social media and coverage by a local newspaper helped draw customers. “Being predictable helps us bring back returning donors,” said Kimber Helling. “They know we will be out there each week and returning every season. Each year has been better than the last, and we can’t wait to see what 2022 holds!”

“...we can’t wait to see what 2022 holds!”
CONCERT FOR THE CURE

In August, Dorothy and Jeremy Mask hosted the 10th Annual Concert for the Cure in Sellersville, PA. This event is held in memory of Ryan Mask to raise funding for MPS III research. This year featured seven hours of music from five bands, vendors, food trucks, and raffles, raising more than $3,900. The event is held in conjunction with an MPS family picnic, which provides valuable time for families to spend together. This year's picnic celebrated Jeremy Mask, his special connection with families, and his love for the children in this community.

KRAMER CHILI COOK-OFF

The Kramer family hosted the 4th Annual MPS Chili Cook-Off in Strausstown, PA, in early October. This event featured a chili competition, raffles, food trucks, an animal rescue booth, vendors, and live music by The DooDads. The cook-off raises funding for MPS II research and had an incredible turnout this year with more than $2,500 in donations in honor of Marcus Kramer.

CABI CLOTHING PARTY

In October, Scott and Lynn Hopkins hosted their annual cabi clothing party in honor of Michelle Hopkins (MPS I). This year marked the 12th anniversary of this amazing event, which has cumulatively raised more than $50,000 in direct donations to the National MPS Society. This fabulous event was filled with food, fashion, refreshments, flowers, door prizes, and incredible friends and family. Thank you to Dawn Watkins for generously co-hosting, and for donating her cabi commission to the Society in honor of Michelle.
Wayne and Joan Eppehimer spent the month of August fundraising for the National MPS Society at the Jamestown Public Market. Joan, who is known for her famous Pearl City Popcorn, has been hard at work popping corn with a purpose. The Eppehimers have spent years selling their goods at the Public Market, and donating the proceeds to various philanthropic causes. They’ve raised more than $1,000 for several initiatives including sending a child to summer camp, providing relief for Hurricane Irma, mission trips, and supporting other charitable organizations. Proceeds from August sales supported MPS II research and family support programs.

Each year Emily Viti and Darren Hohn host an incredible event in honor of their son Christopher (MPS I). Emily works hard to advocate for MPS and to spread awareness throughout her community. Over the years, the National MPS Society has received donations from several fundraisers in honor of Christopher. As a teacher and volleyball coach, Emily actively engages those around her to spread awareness for the rare disease community, and this year was no exception. Christopher’s friends and family celebrated the 6th Annual Purple Pumpkin Smash in an effort to #SMASHOUTMPS. Participants were encouraged to buy a pumpkin, paint it purple, and smash it on social media, explaining what they were doing and why. This effort, paired with a donation, raised incredible awareness and funding for the National MPS Society, raising more than $1,700 in the first half of October alone.

The success of our fundraising program would not be possible without the support of these amazing events, large and small!
Logan Beaudoin (MPS II) is a student at Cincinnati Country Day School. On May 10, Logan and his second grade class spent the day learning about MPS II as part of the school’s Service Learning Day. Nathan Grant, a graduate of Cincinnati Country Day School and twin brother of Nik Grant who also has MPS II, spoke with the class about the condition. The students learned about the cause of MPS II, symptoms, current treatments, and ways to support people with MPS and other differences. Students then made and sold purple bracelets, flowerpots, greeting cards, and magnets to increase awareness of MPS II. From their sales, the students raised $466.82 for the National MPS Society to support other families living with MPS.

JACK “THE BEAST” BENNETT MEMORIAL RIDE

Remembering and honoring Jack’s life
12/2/02–1/28/21
PLANNED GIVING AT THE NATIONAL MPS SOCIETY
Decade of thoughtful generosity brings more than $1.7 million to Society programs

For more than a decade, the National MPS Society has been working with families and foundations wishing to give through our Planned Giving program. A planned gift is also known as a legacy gift from a trust, will, or cash gift to the Society. Since our inception of this high-impact program, we have received more than $1.7 million in planned gifts. Most of these gifts have been bestowed to the Society through trusts or wills, as well as current-day cash donations through planned giving.

Over the past two years, during the tragic health crisis, data illustrates the individual donor is the largest sector of planned gifts.* We appreciate the broad perspectives of our donors and know first hand how you have continued to support us during difficult years.

Would you consider a planned gift in a trust or will to the National MPS Society? The Rising Sun Legacy Circle comprises individuals and foundations that have pledged a gift to the National MPS Society. We encourage you to consider a planned gift and share our mission with family and friends. Spreading the word about legacy giving is an opportunity for others to make an impact that will live beyond themselves.

To discuss contributions, contact Terri Klein, president and CEO, at terri@mpssociety.org.


The Power of Facebook Fundraisers

Do you know that Facebook can be a powerful tool for fundraising? Every day more than 45 million people give birthday wishes on Facebook and many of those birthdays are associated with a Facebook fundraiser. In 2020, the National MPS Society received $101,965 in donations from Facebook fundraisers alone. Whether you are celebrating a birthday or hosting a fundraising event, Facebook makes receiving donations easy.

Consider dedicating your birthday to the National MPS Society, or using Facebook as a fundraising tool for your next event by following the simple steps at left.

“I host a Facebook fundraiser every year to raise money for research and to spread awareness. It is an easy and effective way to raise funds. I have many childhood and high school friends 50+ years later that donate because they have seen my son, Sam’s, story on Facebook. I also champion other MPS kids during my 15-day fundraiser. I point out that because MPS is so rare and unknown to the general public, you won’t see billboards, TV commercials, or envelopes in the mail asking for support, which is why awareness is so important.”

—Heidi Caswell

2020 CHARITABLE CONTRIBUTIONS BY SOURCE

- **Individuals 69%**
  - $324.10
- **Foundations 19%**
  - $88.55
- **Bequests 9%**
  - $41.91
- **Corporations 4%**
  - $16.88

(in billions of current dollars)
2021 ANNUAL FUND CAMPAIGN

The National MPS Society’s Annual Fund is a valuable tool that helps to strengthen the vision, purpose, and mission of the Society by committing resources to projects that provide direct benefit to members and their families.

This year’s Annual Fund chair is Stephanie Bozarth, mother to Annabelle (MPS IVA). Stephanie is a member of the Society’s board of directors and co-chair of the Advocacy Committee. Stephanie knows the importance of the Society’s mission and has an extensive vision for the future of the organization.

“The National MPS Society must continue to lead the way in funding innovative research, educating key stakeholders, providing supportive assistance to those in need, and setting the stage for change agents to dream big,” said Stephanie. “Our horizon includes expanded newborn screening, second-generation therapies, and breakthrough science for those diseases that do not yet have treatment.”

Contributions to the Annual Fund are necessary for this vision, and for the Society to maintain these critical initiatives:

- legislative advocacy
- conference scholarships
- continuing education scholarships
- durable medical equipment funding
- bereavement programs
- informational resources for the public, medical community, and families

We need your help. Please join us in supporting the National MPS Society’s 2021 Annual Fund campaign. Together, we can achieve our goal of raising $275,000 to support the mission of the Society.

YOUR GIFT WILL MAKE A DIRECT IMPACT ON THE FUTURE OF THE MPS AND ML COMMUNITY.

mpssociety.org/give
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Kimberly Heiling in honor of Lilah Mueller  
Steve and Amy Holland in honor of Maddie and Laynie Holland and in memory of Spencer Holland  
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Sharon Whitley in memory of Madison Lewis
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Marcus and Angela Young
Angela and Roger Zylstra in memory of Austin Neil IV
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, and to trained Society professionals, we equip and strengthen families to manage care and establish resources needed for their MPS or ML journey.

We are pleased to welcome Carol Bryant to the National MPS Society as Pathways program director. Carol looks forward to collaborating with families and individuals across the country, learning your stories, and sharing resources and support to those with new diagnoses of MPS or ML.

Visit mpssociety.org/pathways to learn more, or email pathways@mpssociety.org to get in touch.
The MPS and ML community is a family. Though we have spent much of our time apart for nearly two years, those with new diagnoses are still met with warmth, compassion, and understanding.

In family support, we began expanding conversations and asking questions to help meet greater needs. We recognize that a challenge or situation may have a deeper root cause, and we work to help identify those, providing education and guidance. If you reached out to us this year, we hope you have experienced this in practice. Our resource lists and networks are always growing; we encourage you to contact us throughout your MPS or ML journey.

Many thanks to Amber Mongan for her direction and guidance to the Family Support Committee. Amber has served as the committee chair and will transition off the board of directors at the end of the year. Under her leadership, the committee has refined programs, developed guidelines, and provided resources and support to families and individuals across the country. We are grateful for her service and dedication.

We will meet in Nashville, TN, to resume in-person family conferences in 2022! Mark your calendars and plan to join us for the 36th Annual Family Conference: MPS Music City, Aug. 4–6, 2022. The hotel is easily accessible from the airport, offers shuttle service, and is within driving distance for many of our members. Members will receive mailings with details and information on registration at the beginning of the year. Learn more through the Family Conference tab on our website, mpssociety.org.

Applications for continuing education scholarships will open on Jan. 1, 2022. This competitive scholarship program is open for individuals diagnosed with MPS or ML, their siblings, parents, and children. Scholarships award $1,000 for full-time programs and $500 for part-time. Individuals diagnosed with MPS or ML are also invited to apply for the Jeffrey Bardsley scholarship, which awards $5,000. Other family support programs also will begin accepting applications in January. Visit our website for more information.

Communication remains of utmost importance. Our booklets, resource guides, fact sheets, and other educational materials are distributed worldwide and help guide care and make informed decisions. Please contact us if you have ideas for topics or an area where you would like to learn more. You can find us on social media:

@NationalMPSSociety
@mpssociety
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National MPS Society

We are honored to travel the MPS or ML journey with you. We invite you to share your stories, pictures, and experiences with us, and reach out to connect. Email leslie@mpssociety.org or call our office at 919.806.0101. We wish you the best and happiest of holidays!

Sincerely,
Leslie Urdaneta, director, family support and communication

Family Support Committee:
Amber Mongan, chair
Ginger Beverly
Tamara Cullere
N. Matthew Ellinwood
Monica Green
Angela Guajardo
Amy Holland
Steve Holland
Scott Hopkins
Jerry Kanney
Tanya Kimbrel
Larry Kirch
Terri Klein
Kris Klenke
Rebecca Luchenbill
Jason Madison
Tami Slaughton
Leslie Urdaneta
Amy White

**National MPS Society NEW MEMBERS**

- **Navreet Bahia and Mandeep Dhillon**, parents of Amayveer and Abhiraj Singh Dhillon, MPS IIIB, CA
- **Melissa and Joseph Camacho**, parents of Hannah Camacho, MPS I, PA
- **Cecilia Chiara**, mother of Maria Donati Chiara, MPS IIIA, FL
- **Jerome Chiaro and Briana Gara**, parents of Luca Chiaro, MPS I, CA
- **Rafal Chliwinski and Justyna Mozdzierz**, parents of Nikodem Chliwinski, MPS I, PA
- **Kailey Crockett and David Boone**, parents of Nolan Boone, MPS I, NH
- **Lucy and Robert Little**, grandparents of Luna Harwood, MPS VII, MI
- **Rachel and Ryan Nickel**, parents of Shepard Nickel, MPS II, CA
- **Katia and Alfredo Parrilla**, sister and brother-in-law of Victor and Joseph Seda, MPS II, FL
- **Faviola Sandoval**, mother of Emily Enriquez, MPS IVA, AZ
- **Stacey and Cory Smith**, parents of Crew Smith, MPS II, VA
- **Kari and Justin Strong**, parents of Ryler Strong, MPS IIIA, WA
Hello friends! This is Myles. He was diagnosed with MPS I after a stressful year of many doctor appointments, hospitalizations, surgeries, and a few misdiagnoses.

Unfortunately, MPS wasn’t yet on the newborn screening panel for our state. Given the rarity of MPS, many medical professionals weren’t able to recognize or diagnose Myles until he was 16 months old. It is so nice to see babies being diagnosed early through newborn screening!

Leading up to diagnosis, Myles’ doctors (four different specialists) all had their own theories of why he had so many “normal childhood” issues, or they would write them off as no big deal and tell us to check back in six months. We would leave feeling defeated. Many doctors couldn’t give us solid answers and we were getting responses such as “He will eventually improve with time” (speaking about his spine and noisy breathing), “Surgery will fix the problem, but the cause is unclear,” or “Are you trying to find something wrong with your child?”

It was very frustrating. I wrote a list of everything Myles had been diagnosed with, issues, and symptoms, and shared it with his pediatrician who finally listened to my concerns. Myles started the testing process for MPS the very next day. Even though what we were testing him for was very scary to even think about, it was one step closer to saving his life. A few long weeks later it was confirmed that Myles indeed had MPS I.

Treatment started and we were on our way (out of state) to give our baby boy a second chance at life. He received a cord blood transplant at 19 months old. He currently is 5 years old and almost four years post transplant. He loves playing basketball, music, dancing, and anything that he can drive or has a motor. His smile and giggle light up a room.

This MPS journey has been difficult and scary, but has allowed us to meet, connect, and develop relationships with others going through similar situations. We thank the National MPS Society for all they have done for our own family and many others as well!

A new article in the American Journal of Medical Genetics – Part A reports findings from surveys of 97 siblings (age 18–61) of individuals with MPS across the United States. “The experiences and support needs of siblings of people with mucopolysaccharidosis” finds that siblings may experience both positive and negative emotions related to severity and progression of their brothers’ and sisters’ disease, producing an array of accompanying challenges. The article includes resulting implications for families and healthcare professionals. Nathan Grant, primary author, has a twin brother diagnosed with MPS II and provides support to siblings across the country. Access to the abstract, supplemental materials, and full text options to read the article are available by scanning the QR code.
CONTINUING EDUCATION SCHOLARSHIPS
The Continuing Education Scholarship Program awards post-secondary education scholarships for individuals with MPS or ML, as well as 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 an individual diagnosed with MPS or ML, and awards $5,000. The deadline to apply for continuing education scholarships is March 7, 2022. Scholarships are paid directly to the school or institution.

FAMILY ASSISTANCE PROGRAM
The Family Assistance Program offers grants for 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. As part of the application process, members must submit an insurance denial and medical order. Families and individuals are asked to cover a minimum of 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 may need to travel to consult with medical professionals knowledgeable about MPS and ML by providing reimbursement for travel expenses for appointments that are 200 or more miles away from home. This program may reimburse up to $500 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 would pay out-of-pocket for an individual with MPS or ML. 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 age 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 their 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. Family members in need of support through this program should contact Leslie Urdaneta at 919.806.0101 or leslie@mpssociety.org.
MARK YOUR CALENDARS!

Join us Saturday, Aug. 6, 2022, for the National MPS Society’s Nashville Rock and Roll 5K Run and 1 Mile Walk. This event will take place before the start of the conference later that day. Transportation will be provided between the hotel and the run location. Build your teams, and get ready for music and celebration—we can’t wait to see you there!

FAMILY SUPPORT

Join together in person for the National MPS Society’s 36th Annual Family Conference in Nashville, TN, Aug. 4-6, 2022. Conference sessions will highlight current research and clinical trials, scientific information, care management, resources, and more.

Nashville is home to music of all types, restaurants, events, and activities for people of all ages. We are excited to reunite and provide opportunities for connection and fun throughout the weekend. For more information, visit the “Family Conference” tab at mpssociety.org.
We love experiencing the joy of special moments and events with our families and individuals across the country and around the world. Thank you for sharing your journeys with us!
1 Andrew Eisler (MPS II)
2 Anthony and Jaxon Clark (MPS II)
3 Kaitlyn Hart (MPS IIIA) with parents Jim and Kelly
4 Carter Kanney (MPS II)
5 Jeremy McIntosh (MPS IIIA)
6 Danica Boni (MPS I)
7 Eathan Leal (MPS II)
8 Carter Korth (MPS IIIA)
9 Abdullah Rabbani (MPS IIIC)
10 Emily O’Connor (MPS IIIA)
11 Kyle Workman (MPS II)
12 Michael Whitaker-Russell (MPS II) with cousin Makayla
13 Briana Rodriguez (MPS IVA)
14 Noah Walters (MPS IVA)
15 Adele Kary (MPS I)
16 Meekel Stevens (MPS VI)
17 Austin Reamer (MPS IIB)
18 James Oliver Jr. (MPS II)
19 Morgan, Sean, and Kylie (MPS VII) Ransom
20 Rowan Byers (MPS IVA)
21 Molly Jackson (MPS I)
22 Kasey McCannon (MPS IIIA)
ADULT RESOURCE COMMITTEE

Are you an adult or teen living with MPS or ML interested in ways to engage with this community?
Here’s how to get involved:

JOIN THE ADULT RESOURCE COMMITTEE (ARC)
ARC exists to provide support for teens and adults diagnosed with MPS or ML. The group meets the first Sunday of each month on Zoom. During this time, the group discusses upcoming conferences and other initiatives that benefit the adult community. If you are interested in becoming a member of ARC, email arc@mpssociety.org.

JOIN THE “RARELY DEFINED” PARTNERSHIP PROGRAM
This program is for individuals age 12 and older living with MPS or ML and connects partners together to gain insight, guidance, and resources through peer support. The goal of the “Rarely Defined” program is to create unique relationships to share advice and learn more about individual MPS and ML journeys. Visit bit.ly/arcpartnershipprogram to complete an interest survey and learn more.

PARTICIPATE IN A ZOOM SOCIAL HOUR
This event is held quarterly on a Friday at 8:00 p.m. EST. Social hours rotate between adults (age 18+) and teens (age 12–17). If you are interested in participating in a future Zoom social hour, visit bit.ly/arcsocialhour to sign up.

SHOP THE RARE AWARE SHOP
Designs in the Rare Aware Shop were created by ARC members; proceeds directly benefit programs for adults living with MPS or ML. Visit bit.ly/rareawareshop.

For questions about the programs listed above, call the National MPS Society office at 919.806.0101.
Adult Resource Committee Panel

On Oct. 10, six members of the Adult Resource Committee (ARC) participated in a panel on Facebook Live to answer questions about life as an adult living with MPS or ML. Committee members shared helpful insight for parents, siblings, and other members of the adult community. National MPS Society members had the opportunity to pose questions to the group prior to the panel and during the Facebook Live session. Topics included:

- transitioning from pediatric to adult care
- participating in distance learning when other students are on campus
- social isolation during distance learning
- transitioning from college to post-graduate life/entering the workforce
- using your unique perspective to find your passion
- finding a career you enjoy
- becoming involved in your local community
- driving adaptations
- living independently
- the difference between Supplemental Security Income and Social Security Disability Insurance
- Medicare and Medicaid options
- resources to cover cost gaps

To view a recording of the ARC panel discussion, visit the Society’s YouTube page at bit.ly/arcpanelvideo.

If you have questions for ARC, call the National MPS Society office at 919.806.0101.

“I encourage those who have aspirations and a real desire to make a difference in the world to use your voice. Find organizations that support your goals and who you are as a person through your career journey. As you approach post-graduate work life, seek out places that are willing to provide accommodations, ask the tough questions, and embrace your story.”
—Kyle Underwood

“We are more than just our condition. We have other interests beyond just MPS, the hospital and seeing doctors. We must use our voices and actions to show what we are capable of and to experience different things. This not only helps our confidence, it also helps parents and siblings to see that we are able to do more than what doctors or society think we can do.”
—Patricia Espinal

“Life is challenging. Help your children by talking through things. If they are struggling, let them know that is okay. Give them ideas to help them partake in activities with their friends. Help them realize that having MPS isn’t the worst thing in the world. Make life what you want it to be.”
—Kendra Gottsleben

“When you want something, go for it. Connect with people and ask for help. There are so many beautiful people out there who are willing to help you get to where you want to go, or to lead you to the next steps.”
—Fanny Zambrano

Adult Resource Committee:
Autumn Mortensen, chair
Kaitlyn Bean
Nick Boyce
Isabel Bueso
Nicholas DiTommaso
Patricia Espinal
Kendra Gottsleben
Steve Holland
Adele Kary
Jenny Klein
Jason Madison
Samantha Slawson
Julie Sykes
Michelle Teng
Erica Thiel
Kyle Underwood
Sheri Wise
Scott Wold
Jocelyn Wong
Fanny Zambrano

Winter 2021 37
Advocacy efforts are a crucial part of the National MPS Society. Every year during Rare Disease Week, Society members head to Capitol Hill to speak to senators and representatives about policies that impact change and spread awareness of the rare disease community.

In 2021, National MPS Society advocates from 23 states conducted 140 meetings covering topics including telehealth, newborn screening, and MPS Awareness Day. Due to the ongoing pandemic, these efforts also can be done from the comfort of your own home. The Advocacy Committee provides a toolkit with everything you need to prepare for meetings so you can leave a lasting impression and bring awareness to MPS and ML. If you are interested in joining the Advocacy Committee, contact Rebecca Luchenbill at rebecca@mpssociety.org. Sign up today!

**SAVE THE DATE!**

Join the National MPS Society for virtual visits on Capitol Hill on Monday, Feb. 28, 2022. Sharing your stories, experiences, and support makes a difference in the lives of those affected by MPS or ML today and those who will be diagnosed in the future.

**Advocacy Committee:**

Stephanie Bozarth, co-chair
Chris Tippett, co-chair
Jane Andrews
John Barbour
Amy Barkley
Eileen Basquill
Claudina Bonetti
Mary Cavanagh
Hannah Clark
Stephanie Cozine
Kayla DelBeverland
Nicholas DiTommaso
N. Matthew Ellinwood
Wayne and Joan Eppehimer
Coreen Gray
Angela Guajardo
Kimber Heiling
Annie and Austin Henry
Michelle Hill
Elizabeth Hoff
Andrew and Katey Hoffman
Ashley and Cody Holm
Lynn Hopkins
Mike Hu
Tanya Kimbrel
Terri Klein
Noni Langford
Rebecca Luchenbill
Chrissie McKaig
Carmen Ordaz
Mercedes Ramirez
Johnson
Suzanne and Ethan Ransom
Steve Smith
Kristin Stockin
Rebeca Stoffer
Elizabeth Stoltz
Julie Sykes
Brooke Thomas
Cara Thomas
Leslie Urdaneta
Ann Wynegar
Advocacy, Policy, and Action Alerts

SAVE THE DATE!

Rare Disease Legislative Advocates announced 2022 Rare Disease Week virtual activities will be held Feb. 22–March 3, 2022. Carve out time to join other rare disease organizations around the country by sharing your story and help create policy change. For more information, visit everylifefoundation.org.

COSPONSORS NEEDED TO SUPPORT STAT ACT, H.R. 1730/S. 670

Now that the Speeding Therapy Access Today (STAT) Act has been introduced to both the U.S. House and Senate, we ask advocates to contact their members of congress to cosponsor this important bill. One of the capstones of this act is to create an FDA Rare Disease Center of Excellence.

STAT is a bipartisan bill that was created with the input of the rare disease community aimed at improving the development of and access to therapies for the rare disease community. The STAT Act will:

- accelerate rare disease therapy development;
- optimize interagency coordination;
- advance science-based regulatory policies; and
- facilitate access to therapies.

Reach out to your senators and congressmen. Learn more at StatAct.org.

PDUFA UPDATE

A public meeting on the recommendations for the Prescription Drug User Fee Act (PDUFA) reauthorization was held by the FDA on Sept. 28. PDUFA authorizes the FDA to collect user fees to support the process for the review of human drug applications. The current legislative authority for PDUFA expires Sept. 2022, as it must be reauthorized every five years. At that time, new legislation will be required for the FDA to continue to collect prescription drug user fees from 2023–2027. The FDA will consider comments made during the Sept. 28 public meeting when revising recommendations for PDUFA reauthorization. The recorded session “Public Meeting on the Recommendations and Proposed Enhancements for PDUFA VII” can be viewed on the FDA hosting website at https://bit.ly/PDUFAVII.

ORPHAN DRUG TAX CREDIT

The Orphan Drug Act has stimulated the development of drugs for rare diseases since 1983. However, the House Ways and Means Committee’s version of the Build Back Better Act includes limiting the Orphan Drug Tax Credit (ODTC) to the first treatment approval. The legislation is removing a key incentive for investing in further clinical research to identify if a treatment is effective for other rare diseases. Current limits on the ODTC bring forth concerns that the potential savings would be at the expense of the ODTC and the potential savings would be at the expense of more than 30 million Americans living with rare diseases; most do not have therapy options. This policy change could cause irreparable harm to progress and innovation in rare disease therapeutic development. Altering the ODTC will alter and discourage companies from entering the rare disease research pipeline. In September, the EveryLife Foundation issued a statement urging Congress to remove this proposed change. To read the full statement, visit https://bit.ly/ODTCrestatement.
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?

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.
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.

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

<table>
<thead>
<tr>
<th>Syndrome Type</th>
<th>Enzyme Replacement Therapy</th>
<th>Website</th>
</tr>
</thead>
<tbody>
<tr>
<td>MPS I</td>
<td>Aldurazyme® (laronidase)</td>
<td>aldurazyme.com</td>
</tr>
<tr>
<td>MPS II</td>
<td>Elaprase® (idursulfase)</td>
<td>elaprase.com</td>
</tr>
<tr>
<td>MPS IVA</td>
<td>Vimizim® (elosulfase alfa)</td>
<td>vimizim.com</td>
</tr>
<tr>
<td>MPS VI</td>
<td>Naglazyme® (galsulfase)</td>
<td>naglazyme.com</td>
</tr>
<tr>
<td>MPS VII</td>
<td>Mepsevii® (vestronidase alfa)</td>
<td>ultragenyx.com/medicines/mepsevii</td>
</tr>
</tbody>
</table>

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.

Miracle Flights

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

**Wrightslaw**
wrightslaw.com

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

**Takeda OnePath®**
tonpath.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.

**Support Services for Patients and Families through CareConnectPSS™**
careconnectpss.com

Sanofi Genzyme 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 Genzyme 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.

**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.
Furniture for Little People
lpbigdesign.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.

There are many ways you can help support the National MPS Society. From donations, auctions, and fundraising events, there are many ways to advocate for families and to find a cure. When you make a purchase from our logo store, you are not only spreading awareness, you are helping to support our mission.

Check out our end-of-year sale while we make room for new items in 2022. Grab your bundles and deals while supplies last!

MPSSOCIETY.ORG/SHOP

Education & Publicity Committee:
Stephanie Cozine, co-chair
Steve Holland, co-chair
Maureen Cote
Tamara Cullere
Sasha Holder
Larry Kirch
Kris Klenke
Debbie Kruse
Jason Madison
Tami Slawson
Tim Stearns
Chris Tippett
RESOURCES

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.

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).

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.

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.

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.

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.
## 2021 Donors

**Third quarter 2021 general donations**

<table>
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<th>Donor Name(s)</th>
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<tr>
<td>Amazon Smile</td>
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<td>Colleen and Shawn Arni</td>
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<td>Linda Perrell</td>
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<td>Lynda Polgreen, MD</td>
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<td>REGENXBIO, Inc.</td>
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<td>Salesforce.com Foundation</td>
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<td>Edward and Evelyn Schultz</td>
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<td>Riddhi Shah</td>
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<td>Jared Shelton</td>
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<td>Matthew Simm</td>
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<td>The Siragusa family</td>
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<td>Mike and Barbara Smith</td>
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<td>Jeremy and Rena Stearns</td>
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<td>Streblow Family Foundation</td>
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<td>Ultragenyx</td>
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<td>University of Manchester</td>
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<td>Leonel and Edith Yoque</td>
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<td>REED RIÑON (MPS IVA)</td>
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## Donations Given in Honor of

**Third quarter 2021**

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<th>Donor Name(s)</th>
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<tr>
<td>Alice Andrus</td>
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<td>Blake Barnhardt</td>
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<td>Annabelle Bozarth</td>
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<td>Francesca Tuffey</td>
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<tr>
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<tr>
<td>Brian and Rebekah Klutz</td>
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<td>Jennifer and Bryan Hutcheson</td>
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<tr>
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<tr>
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<td>The Harlan and Eunice VanVoors Charitable Fund</td>
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<tr>
<td>Rylee Noble</td>
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<td>Cooper Tippett</td>
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<td>Robert Vice</td>
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## Employee Giving

**Third quarter 2021**

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<th>Company</th>
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<tr>
<td>AbbVie</td>
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<td>Eric Wright</td>
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<td>Jim and Amy Yard</td>
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<tr>
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</tr>
<tr>
<td>Workday</td>
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<td>Steven Ausband</td>
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Donations Given in Memory of

Jack Bennett
Crystal Parks
Colin Berning
Julie and Bryan Durnen
Virginia Brennan
Jeanne Adamiec
Maci Eickman
Elizabeth and Joshua Eickman
Gary Gehrke
Martha Gehrke
Michael George
Allison Frazier
Clara Gibson
Jennifer Jones
Cameron Green
Donna Connelly
Karina Guajardo
John Bernard, PhD
Maricela Careaga
Martin Chapa
Alma Delesus
du Alaska Incorporated
Mike Duron
Tula Gonzalez
Virginia Guajardo-Chapa
John and Yvette Iannelli
Christine and Kirk Kehrley
Robert Langdon
Bayardo and Thelma Rex
Donna Hatmaker
Robert and Katie Lawson
Tennessee Valley Model A. Club
Kenneth Hockenberry
Robert and Candice Wagner
Liam Ross Johnson
Amanda and Michael Johnson
Daniel M. Jones
Daniel Marchesi Jones
Catastrophic Illness Trust
Richard Kitts
Debra and Dennis Zurek
Mark and Casey Lessing
Emily Barrett
Daniel and Christine Bergin
John Brandstetter
Laurence Campbell
Keith and Jennifer Cantin
Tess Froelich
Deven Goh
Emil Gotic
Tim Hartigan
Coleen Kane
Jim and Kathleen Keane
Lisa Khavkin
Theresa Kleine
Michael Lawrence
Joan and Mark Lessing
Melissa Lessing
Daniella May
Alyssa O’Keefe
Virginia Pugliese
Jack and Kathy Simson
Steven Staszewski
Frank and Colleen Trainer
Christopher Young
Samuel Zemet
Deena Levine
Steven and Trudy Anolik
John L. Loy
Donald and Lynda Witte
Austin Marine
Amber Booth
Ryan Mask
Bair’s Tree and Lawn Service
Matthew and Patricia Croyle
Timothy Dimnick
Karen Kiker
Plantation Candies, Inc.
Barbara Sapngler
Saturno, LLC
Sellersville Family
Smile Care P.C.
Lou and Chris Slavik
Daniel Soliday
The Perk South
Perkasie Hotel, Inc.
Timothy Schaffer
Roofing and Siding, Inc.
Women of the Moose
Jason Munoz
Gary Flores
Anna Marie Oliger
Michael and Judith Bauer
Beth and George Blough
Debbie Bossch
Deb Brown
Cindy Burchett
John and Donna Burke
Jacqueline Crouch
Donald Davison
Penny and William Fer
Steve and Valerie Frels
Carol and David Goldermann
John and Ann Harris
E.M. Heindl
John and Carole Hoeller
Alvin and Rita Holst
Mary Jo Jensen
Thomas Kerkhoven
Carolyn Kopps
Dean and Sheryl Lackey
Mark and Stacey McCreadie
Susan O’Malley
Linda and Denis Prior
Dennis and Lynn Quinn
Diana Richardson
James and Penelope Roberts
Joseph and Judy Schneider
Judith Seefeldt
Lavinda and Mark Jean Styaer
Ruth Sueverkruebbe
Tracy Wilson
Sue Price
Wilkes Regional Medical Center
Auxiliary
E. Wyatt
Annalee Perez Sanchez
Dulce Sanchez

Matching Gifts

Third quarter 2021

Anderson Corporation
Blue Cross Blue Shield of NC
Costco
Duke Energy
Liberty Mutual
Microsoft
United Health Group
Vanguard
Workday, Inc.
Pacific Gas and Electric

2021 Facebook Fundraisers

Third quarter 2021

Lana Becker
Kimberly Bergstrom
Kyle’s Birthday
Cory Blain
Rachael Bosch
Austin Bozarth
Barbara Bush
Fidencia Junior
Cisneros III
Tela Clancy
Kaiya Cotkin
Ashlee Deitche-Keeley
Wilma Dickerson
Wayne Eppehimer
Christopher Fleming
Ashley Frix
Kimberly Heiling
Jennifer Higley
Chris Hill
Judy Denney Hollington
Amanda Johnson
Gabby Miyoshi
Liz Paolone
Kim Phillips
Melissa Rachunek-Zielonka
Emily Ramirez
Shel Shampine
Neil Valentine
Allie Walls
Wendy Way
Sierra Whitaker
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, and our Bereavement Expense program offers financial assistance. Staff members are available to talk with you and make additional connections for grief support as needed. Call 919.806.0101 or email leslie@mpssociety.org for assistance or more information about our bereavement programs. These programs are available in memory of anyone with MPS or ML.

---

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 the beauty and joyful memories in this journey. We honor and remember the lives of those who have passed away.

---

Nathaniel “Thanny” Andrade, ML II
3/13/18–11/15/21

Christopher Biel, MPS II
5/14/01–7/19/21

Blaine Elliott, MPS II
10/10/02–5/19/21

Will Huskey, MPS IIIA
7/6/99–10/5/21

Keith Albert Johnson Jr., MPS IVA
1/13/93–10/29/21

Kevin Johnson, MPS II
10/12/01–8/4/21

Liam Johnson, MPS I
11/4/19–7/22/21

Atticus Judd, MPS I
1/17/20–9/6/21

Wesley Leffert, MPS II
2/11/11–11/22/21

William Louis Luthcke III, MPS IIIA
2/10/03–11/6/21

Reagan McGee, MPS IIIA
3/1/12–7/25/21

Kenny Nelson, MPS II
1/26/01–9/17/21

Jamie Parsons, MPS II
7/6/99–10/5/21

Victor Seda, MPS II
2/10/01–8/4/21

Ellis Tripp, MPS II
3/11/21–8/28/21

---

Remembrance Committee:

Kris Klenke, chair
Laura Catanzarite
Tom Catanzarite
Monica Green

Steve Holland
Larry Kirch
Terri Klein

Noni Langford
Dorothy Mask
Robin Piefer

Leslie Urdaneta
Nancy Wain
Amy White
MPS CLASSIFICATIONS

Mucopolysaccharidoses (MPS) and mucolipidoses (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.

<table>
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<tr>
<th>SYNDROME</th>
<th>EPONYM</th>
<th>ENZYME DEFICIENCY</th>
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<tr>
<td>MPS I</td>
<td>Hurler, Scheie, Hurler-Scheie</td>
<td>α-L-Iduronidase</td>
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<td>Sanfilippo A</td>
<td>Heparan N-sulfatase</td>
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<td>Sanfilippo B</td>
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<td>Sanfilippo C</td>
<td>Acetyl CoA: α-glycosaminide acetyltransferase</td>
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<td>MPS IIID</td>
<td>Sanfilippo D</td>
<td>N-Acetylgalactosamine 6-sulfatase</td>
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<td>MPS IVA</td>
<td>Morquio A</td>
<td>Galactose 6-sulfatase</td>
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<td>MPS IVB</td>
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<td>β-Galactosidase</td>
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<td>MPS VI</td>
<td>Maroteaux-Lamy</td>
<td>N-Acetylgalactosamine 4-sulfatase (arylsulfatase B)</td>
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<td>MPS VII</td>
<td>Sly</td>
<td>β-Glucuronidase</td>
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<td>MPS IX</td>
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<td>Hyaluronidase</td>
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<td>ML II/III</td>
<td>I-Cell, Pseudo-Hurler polydystrophy</td>
<td>N-acetylgalactosamine-1-phosphotransferase</td>
</tr>
</tbody>
</table>
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