

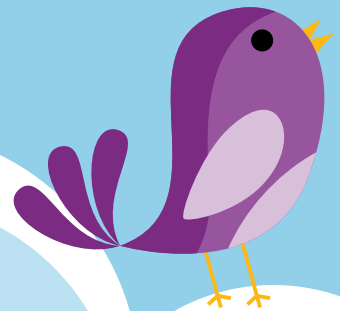
# International MPS Awareness Day

MAY 15, 2019



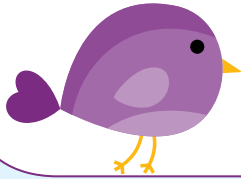
National  
MPS  
Society

Support for Families. Research for a Cure.



## On International MPS Awareness Day, we...

- **honor** everyone in the MPS and ML community.
- **recognize** all the children and adults diagnosed with MPS and ML.
- **think about** loved ones we have lost.
- **thank** the doctors and scientists who are dedicated to finding cures for MPS and ML.
- **remember** one another, and are thankful for the strength and support we both give and receive.
- **acknowledge** the parents, siblings, and caregivers who work tirelessly to support those with MPS and ML.
- **promote** efforts of siblings to raise awareness and share their stories.



The National MPS Society exists to find cures, support and advocate for MPS and ML. To help celebrate International MPS Awareness Day on May 15, we ask you to do the same!

While every day is an opportunity to grow awareness, we are creating initiatives that we hope you will share with others.

- **Sharing Your Own “Day in the Life” Story:**

**May 1–May 15**—Join the Society on social media to share your story

- **A Day of Awareness and Sharing:**

**May 15**—Join families and individuals as they share their stories and honor those that have passed. Help us drive awareness around the world

- **The National MPS Society Goes to Washington:**

**May 18**—The Dare to Be Different Gala and Auction at the Mansion on O Street

**May 20**—Capitol Hill visits to advocate for MPS and ML awareness and funding

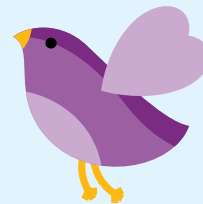
- **Create awareness on social media:**

**#mpsawareness**—Share the hashtag and photos on your social media posts

**@mpssociety**—Tag the National MPS Society!

- **Follow the calendar!**

We have provided awareness ideas with the enclosed calendar!



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The National MPS Society exists to cure, support, and advocate for MPS and ML. Their mission serves individuals, families, and friends affected by Mucopolysaccharidoses and Mucopolipidosis through supporting research, supporting families, and increasing public and professional awareness. The Society advocates nationwide for critical legislation that will impact our rare disease community.

For more information on MPS and ML, please visit [www.mpssociety.org](http://www.mpssociety.org).

## 1974-1976

- "Parents for MPS" formed (1974)
- The Society was incorporated (1975)
- First seminar held and attended by 33 people (1975)
- Membership of 34 families (1976)

## 1980

- Brochure explaining MPS/ML published
- First bone marrow transplant performed on 9-month-old boy with MPS I

## 1983-1984

- Dr. Haskins reports to BOD research with MPS I and MPS VI cats and MPS VII dog (1983)
- Amniotic Membrane Transplantation in MPS I, MPS II, MPS III A&B announced (1983)
- Annual fundraiser money used to fund Research Student (1984)

## 1986

- First major national family conference held
- Brochures, logo wear and a membership directory are created
- Membership of 300 families and mailing list of 600



## 1988

- Hosted 1st International Congress on MPS and related diseases, MN

## 1990

- First Disney family conference



## 1977-1979

- Society headquarters moved from Washington, DC, to New York (1977)
- Dr. Neufeld summarizes MPS/ML research for newsletter (1978)
- First annual fundraiser—a raffle raising almost \$1,000

## 1981-1982

- \$2,557.03 reported in treasury (1981)
- First conference devoted to MPS/ML Diseases at North Shore University Hospital, NY (1982)

## 1985

- Society refiled for incorporation as the National MPS Society, Inc. in New York
- **Newsletter renamed *Courage***



## 1987

- **President, Marie Capobianco, testifies before Congress**
- Ballot nominations for board member appears for the first time in *Courage*



## 1989

- Two student researchers selected by the SAB for summer research fellowships

## 1991

- **National MPS Society meeting—UCLA includes scientific, family and joint meeting**
- Syndrome-specific booklets published



## 2008

- Awarded \$528,000 in research grants
- **Updated all the syndrome specific booklets and developed and distributed comprehensive MPS II and MPS III Resource Guides**
- **Provided 37 stipends for member families to attend the 10th International Symposium on MPS and Related Diseases in Canada**
- Membership of 800, mailing list of 6,000



## 2010

- MPS I, MPS II and MPS III are added to the Compassionate Allowance Program under the Social Security Administration
- **Held first affected adult conference (SPIRIT) in CA**
- Created the Governance Committee
- **Celebrated 10 year walk/run anniversary**
- Created National MPS Society Facebook page



## 2012

- Awarded \$547,000 in research grants
- Provided \$114,000 in family support and scholarship grants
- **Began three film video series for fundraising and awareness with Beth Karas, TV correspondent for fundraising and awareness**
- **Introduced Mobile Pledging and Family Courage Pages for fundraising**



## 2009

- Met with Social Security Administration about adding MPS to the Compassionate Allowance Program
- **Provided a record \$72,500 through the family support programs**
- Annual Fund raised \$73,500
- Raised \$470,000 in fundraising events
- Hired Terri Klein, Development Director



## 2011

- Provided first training webinar for Federal Legislative committee and Society members, *"How Advocacy Moves Government"*
- **Launched Planned Giving Program and the Rising Sun Legacy Circle**
- Launched improved website with increased membership interaction
- Received four-star charity rating with *Charity Navigator*



## 1992

- *Ethan's Feeling Switch* published



## 1994

- Dr. Neufeld awarded National Medal of Science award for her MPS research presented by President Clinton
- Budget increases to \$106,450



## 1996

- National MPS Society website launched
- MPS Day held in Chapel Hill, NC with UNC hospital



## 1999

- BioMarin/Genzyme LLC provided first operational grant to Society allowing hiring of first employee
- Bylaws of Society amended and restated for first time

## 2001-2002

- MPS II phase I/II ERT clinical trial began (2001)
- Second year of 5K walk/run program raises \$170,000 for research with 11 events (2001)
- Awarded \$100,000 in grants for research (2001)
- Participated in the formation of Global Organization for Lysosomal Diseases (GOLD) (2002)
- Annual Fund program established, raising \$12,600 (2002)
- Part time office assistant hired, Laurie Turner (2002)



## 1993

- Mark Dant family begin a large fundraising campaign in Texas to fund Dr. Kakkis lab and the 1994 Disney family conference
- Board votes to waive conference fees for affected individuals



## 1995

- Calendar and note cards created for fundraising

## 1997-1998

- MPS I phase I/II enzyme replacement therapy (ERT) clinical trial began (1997)
- First clinical trial of gene therapy on a human conducted on adults with milder MPS II (1998)
- Creation of the Society's Committee on Federal Legislation
- Steve and Amy Holland elected to Board of Directors



## 2000

- MPS VI phase I/II ERT clinical trial began
- Co-hosted international MPS and related disease conference in MN
- National MPS Society 5K Walk/Run Program launches with seven events, raising \$100,000 for research
- The Society completed its first strategic planning process
- Barbara Wedehase becomes Executive Director



## 2014

- Vimizim®, ERT approved by FDA for MPS IVA
- First patient with MPS VII treated with ERT under compassionate use
- Society holds its 1st fundraising gala honoring its 40th anniversary



## 2016

- Awarded \$485,000 in research grants
- Board of Directors created a new Strategic Plan and rewrote the mission statement
- Mark Dant is hired as CEO
- Introduced *e-Courage*, new digital publications
- Joined the MDBR efforts and raised more than \$150,000
- Hired the first Communication Director
- Hosted the CYCLE conference and provided funding for MPS IV patients to the LPA meeting
- Introduced advocacy webinars to members
- Added the physician database to mpsociety.org
- Annual Fund raises more than \$185,000



## 2013

- Families testified before FDA on patient results in clinical trials for MPS IVA; FDA recommends approval
- Board travels to Washington, DC for large advocacy effort; Sissi Langford presents Senator Graham with Legislative Achievement award
- Award of Courage signed by President Steve Holland given to all of our affected individuals with MPS and related diseases
- Annual Fund raised \$135,000
- MPS VII phase I/II ERT clinical trial began



## 2015

- Barbara Wedehase retired in October 2015; Society established the Research Fellowship Fund in her name
- Adult Resource Committee begins with diversified MPS and ML diseases represented
- National Run Program launches hosting the first two events raising more than \$100,000
- Created MPS IV Resource Guide and revised the Daily Living Booklet with Canada
- National media tactics provide reach on TV shows and game shows
- Awarded \$455,500 in research grants
- Awarded the first Fundraiser Directed Research Grant for \$94,000
- Created the Capitol Hill Advocacy video to champion legislative efforts
- Raised \$478,500 in fundraising events



## 2004

- \$1 million dollar budget
- \$410,000 awarded in research grants
- Hired part-time Development Director
- Membership of 800, mailing list of 3,500
- **Society leases first paid office space in Bangor, ME**



## 2006

- **Elaprase<sup>®</sup>, ERT approved by FDA for MPS II**
- **Launched Join the Search campaign**
- **Participated in the NASDAQ closing ceremonies, ringing the bell**
- Received a four-star charity rating and raised \$423,000 in fundraising events
- Published *Daily Living with MPS and Related Diseases* in conjunction with Canadian MPS Society
- Held the inaugural CYCLE (Celebrating Your Child's Life Experience) conference for bereaved families



## 2003

- **Aldurazyme<sup>®</sup>, the first ERT for MPS I, approved by FDA; Society members testified before FDA as part of the approval process**
- MPS II phase III ERT clinical trial began
- MPS VI phase III ERT clinical trial began
- First Society video produced
- **National MPS Awareness Day established as February 25th**
- Bereavement and regional family picnic programs implemented



## 2005

- **Naglazyme<sup>®</sup>, ERT approved by FDA for MPS VI**
- **Received a \$1 million dollar endowment from Drs. Emil Kakkis and Jenny Soriano**
- U.S. Senate unanimously passed the resolution marking February 25 National MPS Awareness Day
- **Celebrated MPS Awareness Day with NASDAQ, ringing opening bell**
- Created Continuing Education Scholarship program—\$1,000 grants
- Launched new branding for the National MPS Society with current logo and tagline



## 2007

- **Provided testimony to the Social Security Administration**
- Awarded \$459,000 in research grants
- **Moved the National MPS Society to Durham, NC**
- The global MPS advocacy organizations adopt MPS Awareness Day—the day is changed to May 15th and officially called International MPS Awareness Day



## 2018

- Awarded \$550,000 in research grants
- **Hosted the 2018 International Symposium for MPS and Related Diseases in San Diego, CA**
- Pathways provided direct visits to 84 families since inception and visited 29 states
- Provided more than \$120,000 in family support programs
- Provided a series of four Advocacy Webinars to increase awareness for rare disease legislation and newborn screening
- Raised \$530,000 in fundraising events
- Received the eighth four-star charity rating from Charity Navigator
- Increased newborn screening initiatives throughout the U.S.
- Terri Klein is promoted to President/CEO



## 2017

- Awarded \$750,000 in research grants
- Ultragenyx is granted approval by the FDA for Mepsevii<sup>™</sup> for MPS VII
- Pathways program launches, providing direct family services to newly diagnosed families
- The Society celebrates speaker bureau funding to assist with both grass roots and federal advocacy efforts
- Provided more than \$120,000 in family support and introduced the Jeff Bardsley Scholarship Program
- **Developed the MPS VII Resource Guide**



- **Adult Resource Committee develops the road kit for MPS and ML adults**
- Raised \$655,000 in fundraising events
- Hosted the Boston Gala and raised more than \$250,000
- Social media campaign—Markiplier raises more than \$100,000 in one day for the Society
- Courage Pages reaches 50 MPS family stories online

## 2019

- National MPS Society celebrates its 45th anniversary
- Increases newborn screening initiatives to assist an NIH Grant in eight states
- **Hosts the Dare to Be Different Gala in Washington, DC for MPS Awareness Day**



# 45<sup>th</sup> ANNIVERSARY

## Celebrating 45 Years of Support and Research



**National  
MPS  
Society**

Support for Families. Research for a Cure.

The National MPS Society provides me an avenue for connecting with others in the MPS community. Through the various events and programs, we all learn and support one another with compassionate sharing, advocacy, and helping increase awareness.

ADELE KARY, MPS I ADULT

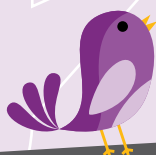


When we first joined the National MPS Society in 1990, we were so happy to be able to meet up with some wonderful people who had some understanding of what our lives were like. The growth we have seen since then—when most of the letters submitted for publication were handwritten, and the Board consisted of parents working out of their homes—has been phenomenal! The outreach to families, fundraising, and research grants by the Society today should give hope to all.

DENISE CROMPTON, ML PARENT

As researchers we usually tell the stories of our work, whether to the community or through our publications, as focused narratives that make the positive results seem self-evident and foregone conclusions. What we don't convey often enough is the skin of our teeth and nail biting close calls about sustaining our research projects, our careers, and our focus on rare diseases. Over the years, the National MPS Society has been a lynch pin that has ensured that careers and research approaches focused on MPS diseases were viable and sustainable. Now more than ever, the research community needs the resources and leadership of the Society so that we can continue to make progress for MPS patients and families.

MATTHEW ELLINWOOD, PHD, RESEARCHER



With 45 years of support of the National MPS Society and the remarkable collaborative efforts of families, researchers, laboratories, and clinicians, I am thrilled to see the progress that has been made in newborn screening, diagnostic testing, and treatment options for individuals with an MPS disorder. It has been exciting to be part of this motivated scientific community and inspiring to work alongside so many courageous patients and families worldwide. I can only imagine how quickly we will progress from here!

TIM WOOD, PHD, RESEARCHER GREENWOOD GENETIC CENTER

I will never forget the day we found out Magnolia's diagnosis of MPS I—it felt like my whole world was unraveling. Despite a stellar medical team, we were left feeling alone and uncertain about our family's future. I didn't even know what to hope for. This is where the National MPS society holds its true value—within hours, we were connected with other families that had been in our shoes, had a wealth of information on the latest and greatest in MPS research, and had an instantaneous community of supporters to guide us. It was invaluable.

TRACY ROSE, MPS I MOTHER