National MPS Society
Support for Families. Research for a Cure.

Volume 35 | Number 2 | Summer 2011

COURAGE

2011 Family Conference July 28–30
Don't miss it!

2010 Fundraising Recap
44 events, one goal: Make a difference!

Legislative Update
MPS research gains $1 million increase in 2010

Family Support Committee Announces New Program
Medical Travel Assistance Program helps families with long-distance medical appointments

MPS III Research News
Physician statement on use of genistein

Do you have a personal story or an article idea for a future issue of *Courage*? Please write to us and remember to send photos!

MISSION STATEMENT

The National MPS Society exists to find cures for MPS and related diseases. We provide hope and support for affected individuals and their families through research, advocacy and awareness of these devastating diseases.

The National MPS Society's office (ground floor on left).

Submission Cutoff Date	Issue
Jan. 1	Spring
April 1	Summer
July 1	Fall
Oct. 1	Winter

To submit information to *Courage*, please send text (preferably via e-mail) to the address at right. Photos should be labeled whenever possible. Please note cutoff dates. Any information received after these dates will be included in the subsequent issue.

The articles in this newsletter are for informational purposes only, and do not necessarily reflect the opinions of the National MPS Society and its board of directors. We do not endorse any of the medications, treatments or products reported in this newsletter, and strongly advise that you check any drugs or treatments mentioned with your physician.

National MPS Society
PO Box 14686
Durham, NC 27709-4686
t: 877.**MPS**.1001
p: 919.806.0101
f: 919.806.2055
E-mail:
info@mpssociety.org
www.mpssociety.org

National MPS Society
Support for Families. Research for a Cure.

Courage *reserves the right to edit content as necessary.*

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Pictured on the cover:
Erin Peters (MPS III)
Mulder family (MPS III)

Membership & Subscription Form

Name

Affected Individual's Name

Date of Birth

Diagnosis

Relationship

Address

City, State, ZIP

Telephone

E-mail

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Would you like to receive *Courage*, the Society's newsletter? ☐ YES ☐ NO

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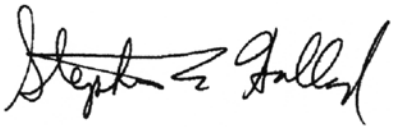
President's Letter

I hope you and your families have begun to enjoy the traditional activities of summer as this issue of *Courage* arrives in your mailbox. My children were always so thrilled to complete another school year and be released to the less structured days of summer. This year is even more significant than most as my youngest, Laynie, graduates from high school. While this rite of passage is celebrated across the land by parents and students as the conclusion of at least 12 years of dedicated schoolwork, often accompanied by many additional hours spent in various extracurricular activities, the thought that their children would not reach this milestone probably never occurred to them. It was expected—more or less guaranteed that it would happen. However, as MPS parents, we see things differently. We realize we must cherish the accomplishments of today because we cannot be assured of what tomorrow will bring. As a result, graduation is a milestone that was very hard to imagine 17 years ago when my children were diagnosed with MPS. It was hard to imagine because many children with MPS did not live that long; nothing was guaranteed.

Fortunately, more and more of our collective MPS children are now living to experience this rite of passage to adulthood. This can be attributed to improved treatments and therapies that have benefited some of the MPS syndromes as well as improved medical care in general. So while we celebrate these successes and milestones, we are soon faced with the question of "What comes next?" While the traditional education system for special needs kids tends to provide unlimited amounts of frustrations, the one thing it excelled at was providing structure. As our MPS kids are now moving out of these structured environments, it can be daunting to assist them in figuring out what comes next, what to do with their lives. The Society is responding to these needs through the development of targeted educational resources for these young adults and the commitment to hold conferences for MPS adults every other year, the first of which was held in 2010.

So while we celebrate having these new problems to deal with, we are acutely aware that not all of our MPS children have access to these life-sustaining therapies. Some are still waiting and still hoping. While the waiting is so difficult, comfort can be taken in the great strides that have been made. This year may indeed be a pivotal year in proving the technology that brings treatments to so many who are waiting—especially in treating the brain disease that ravages so many of our children. One of the best things we can do as parents is to ensure that adequate funding is available to conduct these research efforts. We've recently seen a decrease in fundraising for research, most of which comes from the Society's walk/run program. I encourage all of you to participate in this program. The Society provides much support to assist you in having a successful event. My family has hosted a run for the past 10 years and my children really treasure the attention and awareness it brings to their lives in addition to the funds that are raised.

However, the run/walk program is not for everyone. On pages 16–19 we focus on the many other family fundraisers held during 2010. They are all equally important in supporting the Society's efforts of supporting our families and curing our kids. Please join me in a dream for a moment. If each of our 800 families committed to holding a small fundraiser in 2011 that raised a modest $500 per event, our fundraising would increase by $400,000 for the year. An average of $1,000 per event would raise $800,000. These increased funds would allow us to stop the waiting and reach our goals so much quicker. Please consider it. Now let's join together and turn this dream into reality!



Executive Director's Letter

We are reminded by the media and card companies every spring about Mother's Day and Father's Day. For many families they are a time for bitter-sweet celebration. That is one of the reasons International MPS Awareness Day was established, a day of togetherness for our MPS family. If you have not yet done so, please let the office know how you celebrated International MPS Awareness Day so we can share your stories and photos. We are grateful to your commitment to people helping people, allowing the MPS and related diseases togetherness to exist every day.

We've had some questions about the changes you saw in our 2011 budget that was printed in the Spring issue of *Courage*. In December 2010, we received a very generous donation from Shire HGT which allows us to proceed with the video project and significantly increased the education outflow number. This project had been on hold for several years due to lack of funding, so we are delighted to move this forward. Beth Karas, who developed our 2003 video, is the lead on this project. Beth is a senior reporter with truTV and had two brothers with ML III. Working with Beth is Peter Shaplen of Peter Shaplen Productions.

The other change in the budget is a slight decrease in research funding. The board carefully assessed these numbers at our first board meeting in 2011, the reasons behind the decrease and means to increase the numbers for 2011. To boost the number of grants offered, the board allocated funds from the Shire donation for a general grant. Many of our families face extraordinary expenses when necessity requires expert medical evaluations far from home. To help with those expenses the Family Support Committee initiated a Medical Travel Assistance Program this year, providing up to $500 in travel costs for medical appointments 200 or more miles from home. We also increased funding to our most successful and well-used program, the education scholarships. We are proud to know that, through our financial assistance, so many from our MPS family are achieving their career goals, many in special education or allied health professions.

I will be representing the Society this spring and summer at a number of conferences. The Genzyme Global Summit for patient organization gathers leaders from around the world who support rare diseases and lysosomal storage disorders. This two-day session of interactive discussions provides an opportunity to engage with Genzyme about current events and topics of interest and concern. Following that I will be a co-leader of a breakout session titled, "Resources to prepare for clinical research/Patient registries/Patient recruitment" at the Partnering to Advance Therapeutics for Neurological Disorders: the NINDS Nonprofit Forum at the NIH on June 1. In addition, I have been asked to again speak at the Multidisciplinary Approach to the Treatment of MPS VI Center of Excellence Program about the role of support groups. The program was initially held in Oakland, CA, in January for Asian geneticists and will be repeated in June for geneticists from Turkey.

As always—thanks for all your calls, e-mails and letters. I look forward to seeing you in St. Louis in July.

Barbara Gelbman

Summer is here and we are gearing up for the walk/run season and preparing for the annual conference in St. Louis, MO. The Fundraising Committee has been preparing for the launch of our newest program—Planned Giving. This program will be unveiled during the annual conference and information will be available on our Web site under "Fundraising."

This will be the third year for our Sponsor a Child for a Cure program. Last year 30 children, young adults and families participated. Together they raised more than $11,000. We hope this year more families will be able to participate in the joy of raising awareness and money for research. This program is embraced by runners nationwide and we are thrilled that families unable to attend a walk/run in person can still make a difference.

Take a moment and read through the wonderful fundraising events of 2010 on page 16. We are thankful to everyone who contacts the Society with creative and worthwhile fundraising events. Our strength is gained from those who reach beyond their expectations in honor or in memory of a loved one and raise awareness to help eradicate these diseases.

When you have time, visit the National MPS Society Facebook page or follow us on Twitter! For more information about Planned Giving, Sponsor a Child for a Cure or any other fundraising programs, please contact me at terri@mpssociety.org. Happy summer everyone!

The last few months have been a whirlwind of activity. I have had the opportunity to speak with a lot of our members and even had the privilege to spend time with some of you. I spent International MPS Day at Shire HGT with the Noll family. They shared their story of little Austin and his journey with MPS III with the Shire employees, and I was able to inform them of the Society and our programs. It was a lovely afternoon, made better by some hugs from little Austin! In June I attended Action for Aiden—a walk fundraiser in Exeter, NH. Yes, this is the first time in almost 10 years that we have had an event in New England. Thank you Carter family. It was wonderful to be able to participate in this event!

I am very excited to see many of you in St. Louis. Conferences are always the highlight of the year for me; it reminds me of the reasons I love my work with the Society. Being able to spend time with those of you I speak with on a regular basis, being able to finally put a face to the voice and, best of all, having the opportunity to spend time with your children. The families I serve are the best; I am amazed by your courage, strength, love, passion and determination. You inspire me daily, and for that I thank you.

I am pleased to be able to let you know that the Family Support Committee approved travel scholarships to help 18 families attend the conference. For 12 of these families and affected individuals this will be their first conference! We hope your family also will be able to attend this wonderful event!

New Members

Keith Bakken
Park Ridge, IL, uncle of Brooklyn and Jayden Boyce, MPS III A

Shawn Barkley
Louisville, KY, father of Davis Barkley, MPS II

Maria Bermudez
Miami, FL, aunt of Alejandro Padilla, MPS III C

Joan Boyce
Waukegan, IL, grandmother of Brooklyn and Jayden Boyce, MPS III A

Amy Cherrstrom
Chicago, IL, mother of Alex and Nicholas Cherrstrom, MPS II

Amanda Fults
Lincoln, IL, mother of Kenton Fults, MPS I

Janelle Girod
Albuquerque, NM, sister of Heather Marie Dettmer, MPS III A

Jane Hancock
Liberty Township, OH, grandmother of Case Hogan, MPS II

Whitney Hayes
Pasadena, CA, sister of Kimberly Lisle

Judy Heitman
Glenview, IL, aunt of Declan Mitchell, MPS I

Abe and Veronica Jones
Mahomet, IL, parents of Gavin and Wyatt Jones, MPS II

Dessinna Kirkpatrick
Perryville, MO, mother of Kaylee and Wendy Kirkpatrick, MPS I

Jeffrey and Deena Leider
Elmwood Park, NJ, parents of Jason and Justin Leider, MPS II

Laurie and Mike Leiva
Spring Lake, NC, parents of Olivia Leiva, MPS III B

Curtis and Michelle Lewis
Fuquay-Varina, NC, parents of Michael Lewis, MPS IV

Michele McGehee
Olympia, WA, mother of David Downing, MPS II

Tim and Sarah Mitchell
Westfield, IN, parents of Declan Mitchell, MPS II

Dawn Nelson
Lawton, OK, grandmother of Kenny and Ricky Nelson, MPS II

Julia and Marcos Ramirez
Norwalk, CA, parents of Jazmin Ramirez, MPS II

Jimmy and Willow Rice
Tucson, AZ, parents of Amelia Marie Rice, MPS III A

Joel and Becky Roman
Plainfield, IN, parents of Hudson Patrick Roman, MPS II

Barbara Rutledge
Bloomington, IL, aunt of Isabella and Makenzie Hardesty, MPS I

Brian and Angela Sawyer
Waterford, CA, parents of Emily Sawyer, MPS III

Joie Ann and Robert Steffen
Clark, SD, parents of Hudson Steffen, MPS VI

Marla Stevens
Lockport, LA, grandmother of Meekel Claire Stevens, MPS VI

Janet Swink
Fredericksburg, VA, mother of Rachel Lee Swink, MPS III B

Barbara Wiersma
Byron Center, MI, relative of Case Hogan, MPS II

Teresa Wininger
Odon, IN, mother of Jordan Wininger, MPS II

Jim and Amy Yard
Cranberry Township, PA, parents of Christian Yard, MPS II

Heidi Yaskus
Stevensville, MT, mother of Lauren Yaskus, MPS III A

Roy and Zezee Zeighami,
McKinney, TX, parents of Reed Zeighami, MPS III A

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Mel and Millie Anhalt

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Carol Ruhnke*

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Megan Weisenbach
Tom and Theresa Weisenbach*

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Estelle Culpepper*

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Billy and Diane Hardison*

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Susan Woodward*

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Arlene Miller's son, brother and uncle
Michael and Marcia Geller

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IBM Employee Charitable Contribution Campaign
Kimberly Clark Foundation
McGraw-Hill Companies
Morgan Stanley
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Thomson Reuters

Fundraisers

Laff-A-Thon hosted by Indyprov, in honor of Lucas Montgomery

Macy's birthday fundraiser hosted by Tim, Tina and Macy McDonough in honor of Blake and Morgan McDermott

Pi Kappa Alpha bike-a-thon held by Theta Sigma Chapter of Pi Kappa Alpha

Play for Taylor hosted by Matt and Rachel Wojnarowski in honor of their daughter Taylor

Post Office Café Great Case Race held by Post Office Café in honor of Casey Lessing and in memory of Mark Lessing Jr.

Silge lemonade fundraiser hosted by Allie and Emily Silge in honor of Blake and Morgan McDermott

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** Annual Fund donor*

12th International Symposium on MPS and Related Diseases

Noordwijkerhout, The Netherlands
June 28–July 1, 2012

The 12th International Symposium on MPS and related diseases will take place in Noordwijkerhout, The Netherlands, June 28 to July 1, 2012. The goal of the symposium is to bring together patients, their parents and professionals, thereby setting the stage for free sharing of information from around the globe on all aspects of MPS and related disorders, in order to advance quality of care and treatment.

The theme of the 2012 symposium is ***"Patients, Doctors and Specialized Networks for Optimal Care and Innovative Treatment."***

The program will focus on subjects that underline the need for the development of sustainable networks for expertise on MPS and related diseases. Special attention will be given to those disorders still lacking curative treatment. Cross-cutting themes could be orthopedic problems in MPS and neurosurgery in MPS.

Some of the topics that will be covered at the family level are:

- Living with MPS: patients, doctors, scientists and industry as partners
- Special needs for the adult patient with MPS
- Quality of life and continuous care for patients with Sanfilippo and Morquio disease
- New emerging therapies for MPS diseases (including late-breaking news)

Some of the topics that will be covered at the professional level are:

- Expectations regarding quality of life research: Do we need disease-specific scales?
- The importance of physician-driven registries and patient-reported outcome surveys
- New insights in tissue targeting and treatment development
- (Long-term) clinical effects of ERT, HSCT and other emerging therapies

For more information, visit **www.MPS2012.eu**.

National MPS Society 2011 Family Conference

The 25th Annual National MPS Society Family Conference will be held July 28–30, 2011, in St. Louis, MO. The beautiful Sheraton Westport Chalet Hotel is nestled in St. Louis' impressive West Port Plaza area, just minutes from some of the city's most popular attractions. West Port Plaza offers access to more than 30 restaurants, exclusive shops and entertainment. Save the date to attend this conference that promises to offer all the updates on research, medical care and topics that you've requested.

Registration materials are available on our Web site.

Outstanding Sibling, Children and Relatives (OSCAR) Award

We are excited to present a new section of *Courage* to honor the brothers, sisters, children and relatives of our children with MPS and related disease. This special group of individuals provides support, humor, direction and most of all unconditional love. In each issue of *Courage* we will honor two super siblings who shine each and every day. To nominate someone in your family for an OSCAR award, please send an e-mail to Laurie Turner at laurie@mpssociety.org. Names will be selected randomly to be featured in each issue of *Courage*.

Kelly and Ben Peters

Kelly and Ben Peters recently attended their sister Erin's (MPS III) final high school Special Olympics.

Kelly, 19, drove in from college, missing two morning classes; and Ben, 16, missed four high school classes to be there with Erin. Over the years that Erin has participated in Cherokee County Special Olympics, one or the other or both of her siblings have been there for her. They do everything from participate in the games (softball throw, hurdles, assisted meter walks, etc.), to get her bottles of water, take her to the bathroom and, most importantly, cheer her on and be there for her at the finish line.

The Peters family at the Cherokee County Special Olympics (from l. to r. Tom, Kelly, Erin [MPS III], Ben and Stacy).

Kelly and Ben have lived with MPS for 14 years now. They are compassionate, protective and proud of all Erin has accomplished in her nearly 22 years. In addition, they have volunteered, participated and/or coordinated the *Run for Erin* for 11 years. Since 2000, they have helped raise in excess of $225,000 for MPS III research.

In 2010, Kelly missed her first sorority "date night" to be home for the *Run for Erin*. Ben has recruited both his football and baseball teammates to participate, donate and/or volunteer.

As parents of an MPS child, we could not be more proud of Kelly and Ben. They learned early how to live with the difficulties that a sibling with MPS brings, and come away with empathy, kindness and caring that few know.

Tom and Stacy Peters

Kate Adams

Paul (MPS II) and Kate Adams

Only an MPS family knows the meaning of true love and devotion. My grandson, Paul Adams (MPS II), during his lifetime was so lucky to have Kate for a sister. She held a unique role in the family. She loved and looked up to her brother—holding his hand walking on a sandy beach in Florida, kayaking on a lake in the Adirondacks, going on a ride together at Quassy Amusement Park, finding meaning in his artwork, family gatherings and holidays shared, weekends home to spend time with him and blowing out his birthday candles. During Kate's junior year at college, Paul was hospitalized and she was always there to sit with him, give hugs and also to embrace her parents. At Paul's memorial service, Kate spoke about her brother; her words brought smiles and tears to the overflowing congregation. She is a special, mature, caring person, best sister, and Paul gave back his love for her. I could not be more proud of her. Kate and I often speak of Paul and she keeps him close in her heart.

Mary Starr Adams (Kate and Paul's grandmother)

One of the goals of the National MPS Society is to increase awareness of MPS diseases. With the assistance and persistence of our members, we are making great strides. Don't forget to let the MPS Society know when you are featured in a media story!

Following is an excerpt from an article printed in The Peoples Defender*, West Union, OH, April 20, 2011. Written by Carleta Weyrich/OCM News Service. To read the complete story go to* ***http://peoplesdefender.com/main.asp?SectionID=13&SubSectionID=83&ArticleID=133241****.*

Megan's wish list complete!

It was the final wish on her list for 8-year-old Megan Rust—to go horseback riding with a couple of friends. A second grade student in Paula McIntosh's class at Peebles Elementary, Megan wrote five wishes as part of a classroom assignment. For those who know Megan, those wishes were extra important to accomplish. She was diagnosed with I-cell disease (mucolipidosis, Type II), which prevents normal growth and life expectancy.

Photo by Rachael Hamilton/OCM News Service.

On Saturday, Megan visited Unity Stables on Bailey Road with her friends Jacey Justice and Hannah Sturgill, her sister Rebecca, and her parents, Melissa and Matthew Rust. Megan rode Peaches, a flaxen maned chestnut, while the other girls rode their own mounts.

McIntosh had already helped Megan accomplish her other wishes. In order, her wishes were to have an ice cream party for the whole school, be teacher for a day, have five outfits to wear just like her friend Jacey's, and to meet someone famous and be on TV just once.

Peggy Shipley's son, James, and her parents, Marge and Leroy Shipley, were featured in a story in the *Baxter Bulletin* of Mountain Home, AR, with a United States flag presented in Peggy's memory by Sen. Pryor. The flag was flown over the U.S. Capitol and gifted to Peggy's family at the request of the National MPS Society as part of its White Rose Bereavement Program. Peggy passed away on Oct. 10, 2010, at the age of 53. She had MPS I.

Sam Caswell (MPS I) with Heather (a volunteer) and David, his best friend.

After a long seven-week practice with his Bobcats team for "skills" in basketball, Sam (MPS I) won the gold medal in his division at the New Hampshire State Finals!

Sam would love to be on the school basketball team, but because he can't play "contact" sports (spine surgery, c1/c2 neck surgery, size, etc.) he got involved with the Bedford Special Olympics team. He does the Basketball Skills competitions only. There are five areas of skill: shooting 12 baskets from various lines marked off, running dribble, passing the ball and hitting it into a square on the floor six times and never hitting the line, throwing in a square against the wall and then catching it, and Sam's BEST which is the minute-long dribbling while staying in this particular circle. You need to get 71 dribbles to get the highest score possible on that particular part of the event, and Sam gets well over 250 every time!

Heidi Caswell (mother of Sam, MPS I)

YOU ARE IMPORTANT TO US, PLEASE KEEP IN TOUCH.
Please remember to let the Society know if you are moving. In addition to helping keep printing and postage costs down, you'll help us keep our database up-to-date. Keep us informed of new addresses, telephone numbers, e-mail addresses, and any interesting news about your child.

Hope

I wake up every morning with hope,
Hope that today will be different.
Today all his problems will be gone, like they were never there,
Today he will be a normal kid like there was never anything wrong.

It never happens though.
Each day goes by and things only get worse.
Slowly he loses what he once had.
Slowly my hopes fade away with his abilities.

My dreams of a normal family life seem harder and harder to reach.
Time is running out with each day that goes by.
Dreading the day when it will be too late,
Too late for an answer, too late for a cure.

I feel so helpless.
What is the reason for this?
Why would you make me feel this pain?
There is nothing left for me to do, but hope for a change.

Madison Noll (sister of Austin, MPS III)

Jason and Randi Osterhoudt with sons Alex and Reiley (MPS II)

Alex and Reiley (MPS II) Osterhoudt

A Father's Love

If you look up the definition of **"father"** and **"love"** in the dictionary, you should also find a picture of Lee Louden. The compassion he displayed for his daughter, Alyssa Brooke Louden, from the day she was born through her fight of MPS I was nothing short of heroic. How a parent should react or maintain themselves through such a tragedy is not outlined for anyone, and yet he managed to stay focused and hopeful for a positive outcome for his precious little girl. Lee's passion for MPS awareness is still very much alive today although Alyssa passed more than two years ago.

Everyone who knows Lee will agree that his world revolved around Alyssa. After her diagnosis he made the bold decision to move to a different state and seek treatment at Duke. He bragged about how well she did on the car ride from Kentucky to North Carolina, how great she handled surgeries and treatments, reported her progress constantly and, in October, when things seemed grim, he said, "It's so hard knowing me, her dad, is Alyssa's hero and can't do anything to save her." But how mistaken was he! A hero is there for you when you MOST need them, and Lee was for Alyssa. He was there from the beginning until the end, never leaving her side. Alyssa had a real live super hero to swoop her up and put her in the hands of some of the best doctors in the nation. A parent who was at Duke at the same time shared with me that she had "never seen anyone who you can tell loves their child with all their heart just by looking at them like Lee did when he looked at Alyssa." I think we all would love to have such a brave and courageous person looking out for us in our time of need. I think we all would love to have a hero like Lee Louden!

Lee and Alyssa (MPS I) Louden

He will never forget his little angel and he continues to share her story with others, wanting people to be educated about MPS I. He wears two purple bracelets all day, every day in remembrance of her, along with the hope of making more people aware of MPS. Lee started a Web site after Alyssa's diagnosis and continues to update that site to this day. The site allows people to follow Alyssa's journey, gives readers diagnosis and genetic information, as well as donation information. The Web site is **www.alyssaloudenfund.com** and it's filled with pictures and personal day-to-day stories of Alyssa's story. In the future Lee plans to organize an MPS walk in his hometown in honor of Alyssa, as well as forming a scholarship in her name.

Even though Alyssa is now an angel in heaven, her fight will never be forgotten, nor will the love of her father. I have learned so much from Lee the last few years; we went from being friends to becoming engaged to being married. You should never give up hope, no matter what. Never take a day for granted, and when you are faced with a mighty obstacle, you FIGHT like there is no tomorrow, because none of us are promised another day. And, most importantly, have no regrets—life is too short! Lee's strength has inspired me to become a better person, a better parent, a better friend to all those around me. I hope in sharing some of his and Alyssa's story, you too will be inspired to check out his great Web site and to feel free to reach out to him for anything. He loves to help anyone, especially those families dealing with MPS.

Sherri Martin

Trevor Ramsey (MPS II)

Happy birthday to Lindsey Efird (MPS III) who turned 13 on May 17! (Pictured with mom Karen and sister Hannah)

3rd Annual Mad Hatter Tea Party

On June 5, I will "hop down the rabbit hole" to our 3rd Annual Mad Hatter Tea Fundraiser—a bright, cheerful and fun garden party honoring my 4-year-old daughter Annabelle, who has MPS IV. Along with 40 hostess volunteers, we create and plan a 2 ½ hour event to include entertainment for the young and young at heart. Our goals: raise $40,000 in research funds and a whole lot of MPS awareness!

As the guests stroll down the "paint your roses red" garden lined with silent auction items, the children play flamingo croquet and dance at the "Queen of Hearts court" entertained by the local kid favorite, Mr. Knick Knack. There are plenty of sweets to tempt your tummy and crafts to create a masterpiece hat. The Mad Hatter, White Rabbit, Alice in Wonderland and Queen of Hearts mingle among the guests offering delightful conversation and whimsical treats. The day would not be complete without the entire party wearing their maddest hat attire for all to admire.

Attendance has grown each year for our fundraiser and we are looking forward to even more participation this year. I am very excited, as this event will continue to raise awareness for Morquio syndrome and the MPS Society, and provide generous funds to support MPS research in hopes that it will lead to treatment for all those affected by Morquio syndrome. I believe the key to our success has been to keep it fun, share our story, be inclusive, stay organized, and ask for lots of donations within our community. Not only has the event accomplished our goals for awareness and fundraising, it also has created a community for Annabelle and our family to grow in which people are aware and supportive.

If you have an interest in hosting the same or similar event, I encourage you to get started. I will always be thrilled to share our secrets to fundraising success and creating a fun-filled event. Don't hesitate to contact me at stephbozarth@yahoo.com.

Stephanie Bozarth

Fundraising Committee:
MaryEllen Pendleton, chair
Stephanie Bozarth
Jennifer Clarke
Ernie Dummann
Anne Gniazdowski
Tom Gniazdowski
Angela Guajardo
Steve Holland
Larry Kirch
Terri Klein
Dave Madsen
Hope Madsen
Laurie Turner
Barbara Wedehase

Fundraising Reminders

- Don't forget to submit a brief article for *Courage* about your fundraising success stories and suggestions—they are terrific resources for other families planning events.
- Check out the fundraising section on the Web site for more information or to post your event.
- For free MPS Society brochures and donor envelopes, or to submit information for the Web site or *Courage*, send an e-mail to Terri Klein at terri@mpssociety.org.

Keep in mind—the Annual 5K Walk/Run and the Annual Fund are great ways to raise money for the National MPS Society.

National MPS Society Receives 4-Star Charity Rating

"On behalf of Charity Navigator, I wish to congratulate the National MPS Society on achieving our coveted 4-star rating for sound fiscal management. As the nonprofit sector continues to grow at an unprecedented pace, savvy donors are demanding more accountability, transparency and quantifiable results from the charities they choose to support with their hard-earned dollars. In this competitive philanthropic marketplace, Charity Navigator, America's premier charity evaluator, highlights the fine work of efficient charities such as your own, and provides donors with essential information needed to give them greater confidence in the charitable choices they make.

"Based on the most recent financial information available, we have calculated a new rating for your organization. **We are proud to announce the National MPS Society has earned our 4-star rating for its ability to efficiently manage and grow its finances.** Approximately a quarter of the charities we evaluate have received our highest rating, indicating that **the National MPS Society executes its mission in a fiscally responsible way and outperforms most other charities in America. This "exceptional" designation from Charity Navigator differentiates National MPS Society from its peers and demonstrates to the public it is worthy of their trust.**"

Ken Berger, President and Chief Executive Officer, Charity Navigator

Sponsor a Child for a Cure 2011—Don't get left behind!

If your family has wanted to participate in a walk/run event to raise money for research we have created that opportunity. This program reaches out to families and members of the Society that want to sponsor an affected loved one or a child who has passed away, and help find cures for MPS and related diseases. Walk/runs across the country will be participating in this program in 2011.

All you need to do is:

- Submit a photo of your loved one to the race you wish to participate
- Include the name of your child and address
- Get sponsorship and send to the National MPS Society

In turn, the event will:

- Assign a runner to participate on behalf of your loved one
- Send you a courage medallion and photo with your assigned participant

The assigned runners are inspired by our heroes of MPS. Together they pave the path of continued hope. The photos and amount raised will be published in an upcoming *Courage* magazine.

For more information on the Sponsor a Child for a Cure program, contact Jennifer Clarke at jenniferclarke@mpssociety.org.

Ways to GIVE

- Renew your membership or sponsor another family
- Gifts in honor of a special person
- Gifts in memory of a special person
- Matching gifts through your employer (check with your human resource office)
  1. Request a matching gift form from your employer
  2. Complete the employee section of the form
  3. Mail to the Society and we'll do the rest
- 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)
- Annual Fund donation
- Major gift (usually 10 times that of your Annual Fund gift)
- Planned gift
  1. Bequest in your will
  2. Charitable remainder trust or charitable gift annuity
  3. Charitable lead trust
  4. Life insurance policy
  5. 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 designated programs.

CONTACT: terri@mpssociety.org or 877.**MPS**.1001

National MPS Society

2010 Fundraising Recap

44 events, one goal: *Make a difference!*

The National MPS Society is honored and proud of families who have found creative ways to raise money for our organization. Since 2004 the Society has raised more than $1 million for research, family assistance and other Society programs through fundraising events. Those efforts have provided funding for research grants, durable medical goods, scholarships for education and conference registration, regional family gatherings, extraordinary experiences and support toward the CYCLE program for bereaved families.

We extend our gratitude and recognition to our 2010 fundraising hosts. Your efforts strengthen the foundation of the National MPS Society. Whether large or small, each fundraising event succeeded at increasing awareness in communities nationwide. These families set out to make a difference—individually they succeeded, collectively we soared!

1st Annual Corn Hole Toss

Hosted by Hank & Carolyn Hinton

Carolyn Hinton was motivated to fundraise in honor of her son Danny, who has MPS II. Carolyn and her family wanted to make a difference. Together they hosted their first corn hole toss on May 15, MPS Awareness Day.

More than 200 people registered, formed teams and tossed bean bags into corn hole boxes 30 feet away. They raised more than $3,000. The community, along with other MPS family support, inspired the Hinton family to host another event in 2011.

1st Annual Minnesota MPS Cup

Hosted by Dave & Hope Madsen

Inspired by the Vancouver MPS Cup and Gala, the Madsen family decided they wanted to contribute to the Society. Their daughter Fran has MPS I. With their enjoyment of ice hockey, they spoke with the Minnesota NHL Alumni and other nearby MPS families.

With everyone on board the turnout was spectacular and they raised more than $26,000 for family assistance. Fans paid to play with NHL Alumni and Brad Maxwell, the celebrity game host.

Afterward, 150 donors, including the alumni, gathered for a charity gala event with a live band and auction.

A Wish for Evan Softball Tournament

Hosted by John & Kristi Abel

The Abel family hosted a weekend softball tournament and spaghetti dinner in Hewitt, TX, in honor of Evan who has MPS III. Teams and the community showed up to support the first A Wish for Evan Softball Tournament.

The event held an auction, issued prizes and raised more than $10,000.

This also was a unique opportunity for the Abel family to meet and share stories with other MPS families in their area.

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Bowl for Caden
Hosted by Chris & Clarissa Bartlett

The 1st Annual Bowl for Caden was held in Woodstock, IL, in honor of Caden Mitchell who has MPS II. More than 200 families participated in this team event. Through bowling, raffles, a silent auction and bumper bowling for the kids, this event raised more than $1,000.

Cameron's Car Show
Hosted by Brian & Julie Mollett

This event was held in honor of Cameron Mollett who has MPS II. The community of Paintsville, KY, got their motors running for a cure and raised approximately $2,000. Thirty cars entered the competition for a fee. The event presented an overall winner for Best in Show and trophies for other category entries. This event also included a 50/50 raffle and a DJ.

Clara's Courage
Hosted by Shane & Jenifer Gibson

The Gibson family hosted their 2nd annual Clara's Courage hot dog stand in honor of Clara who has MPS III. This event was held outside the local grocery in Wilimington, OH, and brought the community together for food and awareness. Clara's Courage hot dog stand has raised more than $4,000. Each year the Gibson family hosts charity dinners and finds creative ways to help fund Society programs.

Chapin's Lemonade Stand
Hosted by Grey Chapin

Grey Chapin, daughter of Roger and Susan Chapin, hosted a lemonade stand in honor of her sister Blair who has MPS III. They event was held on May 16 for MPS Awareness Day with overwhelming support from the community.

Grey's lemonade stand raised more than $1,500. The lemonade stand was decorated with bright colors and provided beverages, cookies and many other treats.

Delaware Inaugural Walk/Run
Hosted by Carl & Jennifer Kapes

This walk/run was held in honor of Ryan and Brayden Kapes who have MPS III. More than 400 people traveled to the Delaware River to support the Kapes in this extraordinary event that raised more than $28,000 for MPS III research.

If you would like more information on fundraising, contact Terri Klein at terri@mpssociety.org.

Dopheide "Oklahoma" Fundraiser
Hosted by Emma Dopheide

Emma Dopheide, along with her middle school, presented "Oklahoma" for their school play. The school selected the proceeds to benefit the National MPS Society in honor of Emma's sister, Julia, who has MPS III. This wonderful gift and charming invitation was then followed with a matching gift from the Sanfilippo Syndrome Medical Research Foundation. Together the event raised approximately $6,000 for MPS III research.

Fowler Family Charity Dinners
Hosted by Jason & Jamie Fowler

Each year the Fowler family gathers around their home in Colorado or with family in Arizona. They raised more than $1,500 through charity dinners in honor of their son Jack, who has MPS II.

Klenke Bowl, 12th Anniversary
Hosted by the Klenke family

The Klenke family hosted its 12th Annual Bowl-a-Thon in Edwardsville, IL, in memory of their son Kraig who had MPS II. Family, friends and the community came together to bowl a game for Kraig and help raise more than $10,000 for the Family Assistance Program.

continued

This event included a raffle, food and prizes for the bowlers. Each year they develop a theme for participant T-shirts.

Everyone celebrated the end of this event with a special cake.

Links for Lucas
Hosted by Lew & Stacey Montgomery

Every year the Montgomery family hosts an event to raise money in honor of their son Lucas, who has MPS III. Links for Lucas was a terrific golf tournament that brought family, friends and the community together. The event raised more than $10,000. The Montgomery family has hosted other events such as walk/runs and charity dinners, and even Lucas' sisters have been involved with hosting a dance-a-thon.

Lukondi Fisher Walk/Run 5K
Hosted by Rob Lukondi

Robert Lukondi and his stepson, Michael Fisher, who is autistic, trained for and ran their first 5K.

Robert and Michael completed their event in honor of Allison and Lacey Lukondi, who have MPS III, and raised $250. Michael trained for months while working through his autism in this inspiring tribute for his cousins.

Muller Family Fundraisers
Hosted by Robb & Lisa Muller

For the second year in a row the Muller family hosted the Riley Corn Hole Tournament. More than 60 teams participated in the event to honor Riley Muller who has MPS II.

The Muller family had special boards made for the event with purple ribbons painted on each of them. Teams registered and participated on this sunny day. They raised more than $5,000 in the past two years. Along with this event the Muller family likes to host fun charity dinners throughout their hometown of Marysville, OH.

Play for Taylor
Hosted by the Wojnarowski family

For many years the Wojnarowski family has hosted Play for Taylor, a musical recital in honor of Taylor, who has MPS III. The family hosted a number of other events throughout the year and raised more than $900. In 2010 they held a T-shirt sale for Run 13 Miles for 13 Years and included family in the 2010 Annual Fund drive.

Post Office Café Charity Events
Hosted by Mark Lessing & Kerri Rose

The Lessing family hosts charity events at their Post Office Café each year to benefit Society programs. Events are held in honor of Casey Lessing and in memory of Mark Lessing Jr., both with MPS III.

The Post Office Café hosted the Great Case Race and raised $600 while extending hospitality to patrons and raising awareness for MPS and related diseases. In 2010 the Post Office Café also celebrated their fifth walk/run event for MPS.

Shots for Sean
Hosted by Ernie & Debbie Dummann

Shots for Sean is an annual golf tournament hosted by the Dummann family. In 2010, this event was held in memory of their son Sean who had MPS III.

The event was held in Anchorage, AK, and brought family, friends and colleagues out to swing for Sean.

The Dummanns included a raffle, prizes, plaques and giveaway bags for the participants and food as part of the registration fee. Shots for Sean raised more than $11,000 in 2010.

continued

Wynn & Dorian Birthday Fundraiser
Hosted by Chris & Mercedes Johnson

The Johnson family held their second birthday bash in honor of twin sons Wynn and Dorian who have ML II.

The family combines Wynn and Dorian's birthday with a Halloween theme and invites family and friends. They have raised more than $7,000 for ML research.

Someone I LOVE NEEDS A CURE
mpssociety.org

Other 2010 Fundraising Events

Allison and Ashley's fundraising event, *hosted by Ashley Restemayer*

Anderson harvest fundraiser, *hosted by Dave and Cynthia Anderson*

Angel Aurora Laorenza's birthday event, *hosted by Providence Schools*

Bluegrass concert for MPS, *hosted by Sharon Gilham*

Booster Arbonne fundraiser in honor of Rebekah, *hosted by Dawn Booster*

Bosch garage sale neighborhood fundraiser, *in honor of Daniel "Squeek" Bosch*

Bozarth soccer camp fundraiser, *hosted by Stephanie Bozarth*

Camelot Preschool MPS Day, *in honor of Oliver McNeil*

Cavanaugh bracelet show, *hosted by the Cavanaugh family in honor of Allison Kirch*

Chesser lemonade stand, *hosted by Bryn Chesser*

Ciroli jewelry show, *hosted by Lisa Muller in honor of Riley Muller*

Holmes Elementary bake sale, *in honor of Clara Gibson*

Ice Cream Corner fundraiser, *hosted by the Restemayer family in honor of Allison Restemayer*

Jean for Genes, *hosted by BioMarin*

Kassi's Kause charity dinners, *hosted by the Offenbacker family*

Life Skills walk-a-thon, *hosted by Kathy Greenberg in honor of Logan Piefer*

Lee dance-a-thon, *hosted by Griffen Lee*

Malone garage sale, *hosted by James and Joan Malone in memory of Louis Butts V*

NH Eye Associates fundraiser, *in honor of Sasha Segal*

Sowden's Walk for a Cure, *hosted by Josh and Sheri Sowden*

St. Anne's bake sale and craft show, *hosted by St. Anne's school in honor of the Sarantinos family*

Tricky Tray fundraiser, *hosted by St. Andrews Church in memory of Rishi Garg*

Williams' bicycle race, *hosted by Adam Williams in honor of Riley Muller*

Inspired by the fun and success of last year's fundraising events? Consider hosting your own!

The National MPS Society provides a comprehensive how-to packet with checklists and details for hosting events, including instructions for securing donations, building a volunteer team, obtaining liability releases, gathering sponsorships, publicity and much more. To receive your packet, contact the Society at 919.806.0101 or send an e-mail to Terri Klein, development director, at terri@mpssociety.org.

A Warm Welcome

A Warm Welcome introduces new Society members/families and offers members yet another chance to connect with one another. If you have a moment, please contact the new family to say hello and welcome them into our MPS family. If you have been a member for a longer period of time, but would like to introduce your family to the rest of the Society, please e-mail Laurie Turner at laurie@mpssociety.org.

The National MPS Society welcomes the **Mulder family** from Michigan.

I have to be honest, this is all a bit surreal to me. Just this past December we were leading our lives, preparing for Christmas and trying to get to the root of our 10-year-old son's enlarged liver. Then, in March of this year, he was diagnosed with MPS III A and I am now introducing our family to the MPS community. Surreal.

My name is Tina. My husband is Mark, and we have two sons: Jarod, 10, and Caleb, 5. We live in Hudsonville, MI, where Mark is a high school history teacher and also the school's varsity soccer coach for both boys and girls. For six years now I have been running a part-time home daycare so I can spend as much time with my boys as I can.

Our boys keep us on our toes. Jarod is a fun-loving boy with a huge heart. We call him our "neighborhood greeter" as we live on a corner lot and he will try to chat with anyone walking or riding by. He knows every child at his school and will greet each by name and ask for a "high-five" as he walks the halls. He has the best belly laugh in the world, and the most amazing green eyes. He plays AYSO soccer, and baseball with our local Little League's Challenger Division. He is a huge fan of the Detroit Red Wings and the Detroit Tigers. He also loves our lower-level local teams like the Grand Rapids Griffins and the West Michigan Whitecaps. He loves to bring home programs from games or get yearbooks for the teams so he can memorize all the players by name, face and number. When he is awake, he is non-stop—riding his bike, shooting hockey pucks, dribbling a soccer ball or hitting whiffle balls in the yard, getting into the pantry for snacks, playing with cars and toys in the house, or watching his favorite movies like The Mighty Ducks, The Big Green or Miracle. He does have his behavioral issues and he tires us out, but we wouldn't change a thing.

Caleb is a funny, active and sometimes sassy 5-year-old. He plays AYSO soccer and t-ball. He attends preschool and absolutely loves it. He enjoys going to the movies or the zoo, reading books, doing puzzles and playing games like Toy Story Yahtzee or Uno Moo. He and Jarod absolutely adore one another, but they have a typical brotherly relationship: sometimes hugging, sometimes fighting. I love it when they are getting along and Jarod will throw an arm around Caleb's shoulder and say, "Caleb, we're brothers, right?" with a big smile on his face.

As a family we are active in our church, love to go to the beach in the summer, go camping in our pop-up or hiking in the woods. We like to attend a few hockey or baseball games each year. We love to spend time together, whether that is going to the zoo, playing at our neighborhood park or just playing baseball or basketball at home. We typically have to pick activities that are "Jarod friendly," as I'm sure most of you can understand. Mark and I are trying to savor every moment now more than ever.

As far as Jarod's diagnosis, I'll begin our story on a snowy Christmas morning in 2000 when we were given the best gift ever. Jarod made his entrance to the world, a little peanut at 6 lbs. 13 oz., with soft brown hair and big, dark eyes. He stole my heart that day and has had it ever since. He was happy and healthy and ate like a champ. He was our first child and we cherished every little smile, laugh and sigh. His growth and development seemed normal and he hit every physical milestone just when he should. He had many ear infections between the ages of 1 and 2 and subsequently had tubes placed.

continued

I began noticing when he was a toddler that I didn't think his speech was where it should be, but when I mentioned it to our pediatrician, he told me not to worry. "Every child develops at their own pace…boys typically develop speech slower than girls…don't compare him to other children." But we parents know, don't we? I kept pestering and he finally sent me to our county's early childhood program where we learned he was indeed behind. He started speech therapy at the age of 3. Things seemed to be progressing well, but Jarod always seemed to be a little "different." He never really played with other children, but more alongside them. He would have huge temper tantrums if he didn't get his way.

Kindergarten was a big year. We got a call from his teacher telling us they thought Jarod should have some testing done and had we ever heard of Asperger's? Although he showed some characteristics of Autism he didn't fall on the spectrum and it was ruled out. We also learned that year that he is hearing impaired (moderate high-frequency loss) and got him hearing aids. The school system proceeded with IQ testing and Jarod's score was 68. We complained that they tested him before he had the aids and wondered how much of his score reflected the fact that he had been hearing-impaired his whole life. Once he got his hearing aids I think we all expected his development to catch up, but it never did. The school psychologist started using the words "cognitively impaired," but we resisted. We wanted to give him time.

Due to Jarod needing more one-on-one time than was possible in his current resource setting, and his frequent behavioral outbursts and tantrums, we reluctantly placed him in our school system's cognitively impaired program when he entered second grade. It was a huge hurdle for us to overcome and it broke our hearts. But, his teachers there are wonderful and love him to pieces. They go above and beyond to get him what he needs and help him to be and feel as successful as he can. He currently reads at a kindergarten level and can do simple addition, etc. He loves the social aspect of school and enjoys being with all of his friends.

So we continued life for the next year trying to give him all of the love and resources we could to help him learn, express himself and control his behavior, hoping to usher him into adulthood someday, ready to be the best he can. And then, last June, our pediatrician (a new one since his preschool days) noticed that his liver was enlarged at a routine physical exam. She tried not to scare us and ordered an ultrasound and some urine/blood tests. His liver was moderately enlarged, and so was his spleen. More tests. No answers. October of 2010 brought a liver biopsy and still no answers. Then, in December, we met with a metabolic specialist from the University of Michigan. That was the first time we ever heard the word "mucopolysaccharidosis."

In March we were given Jarod's diagnosis of MPS III and were completely devastated. What a paradigm shift to go from wondering what kind of future our son will have to realizing that our son is slowly dying and that we will be fighting forever for any future for him. But we will fight. We pray. We hope. We are trying to plug ourselves into the right places with the right doctors. We've learned so much in the last few months. I'll admit the future is frightening, but in the midst of all of this we are realizing how blessed we are. Blessed that Jarod is doing as well as he is for his age. Blessed with family and friends who surround us with love and support. Blessed that Jarod's teachers and the staff at his school absolutely love him and will do anything for him. Blessed that so many of you have already reached out to us with a helping hand.

We look forward to meeting so many of you to connect, learn, share joy and even grieve with you, if necessary. We are working on a Web site for Jarod that should be up soon. You can find it at **www.teamjarod.org**. You also can reach me by e-mail at tmulder@sbcglobal.net.

Thanks, and God bless.

Tina Mulder

MPS Research Gains $1 Million Increase in 2010

During the last several years, the Legislative Committee concentrated heavily on increasing research dollars through the National Institute of Health (NIH) for MPS and related diseases. With research funding by the NIH stagnant at $7 million for the last few years, we feared the loss of research jobs, and the delay of new treatments or therapies being brought to the marketplace for MPS and related diseases.

However, a $1 million increase was given due to the many excellent research opportunities available for funding. Much of this credit needs to go to President Obama and our MPS researchers for their superior grants and to our Policy with Partners members who advocated with Congress in Washington, DC, and at home. The NIH also is reflecting this $8 million amount for MPS research in 2011 and 2012.

Legislative Committee Travels to DC

May 16, 17 and 18 were busy days in Washington, DC, spent calling on members of Congress and their health liaisons to thank them for the $1 million increase in research funding while advocating for more research dollars in 2011 and 2012. At this time we also discussed the Society's priorities. It was very moving to once again have the resolution brought by Sen. Lindsey Graham (R-SC) declaring May 15, 2011, as National MPS Awareness Day. We thank Sen. Graham and his health liaisons, Colin Allen and Leigh Ellen Gray.

You will read more about our trip, National MPS Awareness Day and detailed talking points in the next issue of *Courage*. Thanks to board members Jeffrey Bardsley and Stephanie Bozarth, and MPS parent Amy Barkley for volunteering their time and expertise to help MPS families, and for accompanying Ernie and Debbie Dummann through the halls of Congress.

Legislative Committee:
Ernie Dummann, Chair
Debbie Dummann
Jeff Bardsley
Stephanie Bozarth
Chip Brady
Steve Holland
Austin Noll
MaryEllen Pendleton
Kim Whitecotton
Roger Chapin
Terri Klein
Kelly Rose
Laurie Turner
Barbara Wedehase

International Rare Disease Research Consortium Launched

In April, regulatory agency stakeholders, researchers, patient group representatives, members of the biopharmaceutical industry, and health professionals gathered for the second meeting and official launch of the International Rare Disease Research Consortium. Following a successful preparatory meeting in Iceland in October 2010, the second reunion picked up the pace with the endorsement by members to fulfill certain goals, including, notably, a commitment to the development of 200 new rare disease treatments by the year 2020 and the development of diagnostics for all rare disorders. Related challenges identified include the need to establish and provide access to harmonized data and samples, perform molecular and clinical characterization of rare diseases, boost translational, preclinical and clinical research, and streamline ethical and regulatory procedures.

Formed under the auspices of the U.S. NIH and the European Commission, the consortium will involve "an unprecedented cooperation at the international level" in order to fulfill the ambitious goals defined. As such, public and private stakeholders from all over the world are invited to join the fight to alleviate the suffering of rare disease patients and their loved ones. Participants included representatives from Canada and individual European countries (France, Germany, Italy, Spain and the UK, among others). Genetic Alliance President and CEO Sharon Terry, who participated in the three-day meeting, commented, "The energy generated by having concrete goals, with the commitment and leadership of major international funders throughout the entire meeting, was palpable. Now it is time to roll up our sleeves as a community, and focus our energy on getting the business plan in place to succeed."

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The loaded agenda began with an analysis of ongoing research efforts and future challenges, including a breakdown of country-specific activities. The conference then gave over to several breakout sessions, including Understanding of Pathophysiology of Rare Diseases (including genomic analysis by next generation sequencing, and animal and iPS cells models of rare diseases); Ontologies/ Disease Classification/Natural History; Biomarkers; Patient Registries and Biospecimen Repositories; Preclinical Research and Clinical Trials; and Communication/Publication/Information/PR/Data Policy. The meeting wound down with a report and conclusions from the breakout sessions.

Next Steps

A scientific and policy framework must be developed in order to "guide the research activities and foster collaboration among the stakeholders to systematically explore all the opportunities to accelerate the development of diagnostics and therapies for rare diseases." Funding and budget considerations also need addressing. It is anticipated that the governance of the project will be modeled after the Human Genome Project—and thus be open to research bodies from around the world. It is probable that a minimum financial contribution will be required by participants, although no figure has yet been determined. Participants also would have to share all relevant data, a process that would help harmonize terminology between countries.

Court Okays Federal Funds for Embryonic Stem Cell Research

A federal appeals court has sided with the Obama administration, overturning a judge's ruling last summer that NIH funding of embryonic stem cell research violates a law against destroying human embryos. A panel of three judges on the U.S. Court of Appeals for the District of Columbia Circuit —who were all appointed by Republican presidents—ruled 2-1 in April 2011 to strike down the injunction issued by federal judge Royce Lamberth that barred the NIH from paying for research on embryonic stem cells (ESC).

Lamberth ruled that ESC research violates a federal law known as the Dickey-Wicker amendment that bars use of government money to destroy human embryos.

After the prospect of losing funding put the ESC research community into a panic, the same court that issued the ruling ordered that the NIH could continue to fund ESC research until a formal decision was made on Lamberth's injunction.

The Obama administration argued that millions of dollars worth of research would be put in peril if the injunction was allowed. Soon after he came to office, President Obama lifted a ban on stem cell research imposed by President George W. Bush and expanded federal funding for ESC research.

The NIH came up with ethics guidelines in 2009 to govern ESC research that stated federally funded embryonic research can only involve leftover embryos from in vitro fertilization procedures with the parents' consent, and that there be a "clear separation" between the decision to create embryos for reproductive purposes and for research purposes.

But critics say that ESC research destroys human life and is not something the government should be paying for.

The case was originally brought by two researchers who work with adult stem cells, which are found in various tissues throughout the body, who argued the NIH's guidelines violate the Dickey-Wicker amendment, and Lamberth agreed.

"Responsible stem cell research has the potential to treat some of our most devastating diseases and conditions and offers hope to families across the country and around the world," said White House

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spokesman Nick Pappas. “Today’s ruling is a victory for our scientists and patients around the world who stand to benefit from the groundbreaking medical research they’re pursuing.”

NIH Director Francis Collins, MD, PhD, said he was happy and relieved with the ruling. “I am delighted and relieved to learn of the decision of the Court of Appeals,” he said in a prepared statement. “This is a momentous day—not only for science, but for the hopes of thousands of patients and their families who are relying on NIH-funded scientists to pursue life-saving discoveries and therapies that could come from stem cell research.”

NORD Gala

On March 17, 2011, the National Organization for Rare Disorders (NORD) honored two members of the U.S. Congress for improving the lives of people with rare diseases. The awards were presented at the annual NORD Partners in Progress Celebration held at the Andrew W. Mellon Auditorium in Washington, DC. Each year, NORD celebrates pioneering achievements of individuals, organizations, and companies in public policy, patient advocacy, medical research and product development.

Actress Patricia Richardson of TV’s Home Improvement, Strong Medicine and The West Wing emceed the event with more than 500 guests from patient organizations, government agencies including the NIH and Food and Drug Administration, academic research centers, and pharmaceutical companies in attendance.

Among others honored were the Honorable Joseph Crowley (NY-7) and the Honorable Leonard Lance (NJ-7) who received the National Health Leadership Award. Representatives Crowley and Lance are the co-chairs in the U.S. House of Representatives of the new Rare Disease Congressional Caucus.

The caucus was formed to bring Congressional attention to the nearly 7,000 known rare diseases that currently have no approved therapies; ensure sufficient funding for research and orphan product development; explore ways to incentivize companies to create new drugs, biologics and humanitarian use devices; and provide an opportunity for members of Congress, families and advocacy groups to exchange ideas and policy concerns. Rare and neglected diseases affect nearly 30 million Americans.

“The Rare Disease Congressional Caucus will provide an important forum in Washington for the exchange of ideas and information related to rare diseases,” Rep. Lance said. “Congressman Joe Crowley and I are committed to working together in a bipartisan capacity with like-minded members, policy advocates and families across the nation to increase awareness and education of rare diseases.”

Stephanie Bozarth, Jeffrey Bardsley, and Ernie and Debbie Dummann were guests of BioMarin Pharmaceutical and would like to thank them for their hospitality.

Let's Give a Standing Ovation To…

The Standing Ovation Award is intended to honor amazing people in our MPS family for their resilience, courage, tenacity and passion for life while facing the many challenges of having MPS. This award was created by Denise Dengel, an adult with MPS I, who knows the daily struggles of living with MPS and envisioned an award to honor the individuals who also battle MPS each and every day.

We give a standing ovation to:

Hannah Gosey, MPS I

My name is Hannah Gosey and I will be 15 in July. I had a bone marrow transplant when I was only 6 months old. I have been through a lot and life is definitely challenging, but I am determined to make the best out of everything!

I have always loved to read, and when my eyesight got really bad reading became a huge challenge. I have had both corneas transplanted. After I had the first eye done, I remember sitting in the doctor's office reading the newspaper without my glasses. That was really cool! Now that I have had both eyes done, I am able to function without my glasses for the first time since I was 2. That's even cooler!

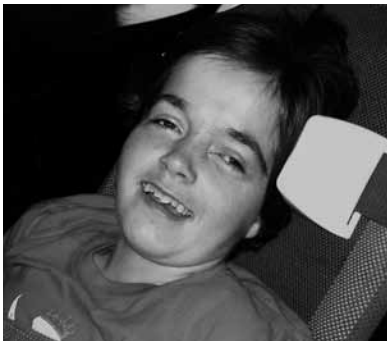
I love music and dancing. I took ballet and hip-hop dance classes for nine years. I had to stop last year because of my knees, but I still like to dance around the house. I love watching videos and listening to music on my iPod. My favorite videos to watch are Kennan Cahill's YouTube videos. He reminds me so much of me. My favorite TV show right now is the Big Bang Theory—Bazinga! I like doing things with my family too. We like hiking, biking and swimming—pretty much anything outside.

I have been able to do a lot over the years and I have a lot of great memories. I think one of my favorite memories was having dinner with Bill Elliott at a Ronald McDonald House event. We got to sit with him and he was really nice. When I get older, I either want to be a vet tech or work in special education. I love all kinds of animals (except snakes!). I also like helping others. It would be nice to be able to help other children like me, the way that I have been helped.

Case Hogan, MPS II

Case is now 4 years old and he was diagnosed with MPS II just after his second birthday. Case's grandma is an RN and saw the episode of Mystery Diagnosis with Zachary Townsley (MPS II); she recognized the symptoms, prompting his diagnosis. Since then, Case has been getting Elaprase® at Vanderbilt Children's Hospital in Nashville, TN, each week where he knows many of the volunteers and employees. He loves to make new friends there, watch Barney and eat special snacks. Case is a huge Barney fan and got to meet Barney at Universal Studios in May of this year. He also enjoyed many rides at Disney World with his two brothers, Tyson (7) and Brock (5). Case loves to wear an MPS awareness shirt we made and give out bracelets and cards that tell about his condition. He also has been participating in the MPS II intrathecal clinic trial since December 2010 and has begun learning many new things! He loves using apps on his iPad and showing off for videos we put on his blog (**www.savingcase.com**). We don't know where things will go from here, but right now we rejoice in Case's health and learning, and in his participation in furthering treatments for our kids.

Kirby Wilson, MPS III



Kirby celebrated her 20th birthday on April 15. Reflection on the many blessings Kirby has brought to our family filled the day. We remain in awe of Kirby's perseverance and find the spring season a truly fitting time of year for her special day. Many of us think of spring as a time of rejuvenation and uplifting of our spirits from the cold bleakness of a long winter. The warmth of sunny days and the bright emerging colors of the season remind us of the power of nature and its resilient splendor year after year—seemingly so simple.

We now realize that Kirby's journey and gift to us are really quite similar. Year after year, she has shown us her perseverance against the disease that afflicts her and her quiet resolve to be happy. Her resilience from within to thrive for 20 years reminds us of the power of the sun's warmth. Her smile is as beautiful to us as any flower nature has created and brightens our days like no other—seemingly so simple. Like spring, we see her power to revitalize and enliven those around her. She is a remarkable child whose amazing grace continues to nurture her family so that we may flourish. She is our hero.

Chris Fitzgerald, MPS IV

My name is Chris Fitzgerald, and I'm a junior at Scarborough High School in Scarborough, ME. I am an active member in Boy Scout Troop 47 and I recently received my Eagle Scout badge. Only about 2 percent of people who join scouts earn this badge, so I am very proud of it. I am quite fond of animals, so for my Eagle Service Project I constructed two wooden cat shelters for a local Animal Refuge League.

Vince Clemens, MPS VI

Megan Lich, ML

My name is Megan Lich and I have ML III. I currently am living in Castle Rock, CO, having moved here three years ago for my dad's new job. It was a stressful sophomore year getting used to a new school and environment, but it made me a stronger person having to rely on myself more and not having the security of friends that I made growing up. I am looking forward to starting at Arapahoe Community College in the fall. My goal in life is to become an elementary school teacher. So, I will be pursuing an Associate of Arts degree in elementary education.

My most current goal is to get my driver's license. I recently got my permit and started driving around the community. It has been a two-year process in the making with getting both my hips replaced the past two summers. It was worth all the grueling physical therapy to once again be walking independently again after six years. I want to be as independent as possible by the time I start college in the fall.

One of the proudest moments in my life was walking across the stage at graduation and receiving my diploma. I had family come from Ohio, California and Arizona to cheer me on, along with hundreds of peers and teachers who were so supportive throughout my high school years. As I look back, I realize how blessed I am in life. The party after graduation was amazing! This summer is going to be busy. I am going to spend about four weeks with my grandparents in Tucson. Then in July my mom and I will be taking a road trip back to Kentucky and Ohio to visit my good friends who I grew up with and haven't seen in three years. They have not seen me walk again and it will be a pleasant surprise. We have a lot of catching up to do that can't be done over the phone or by texting.

I will keep busy this summer shopping, shopping and more shopping. Can you tell that I like shopping? I also will be first in line to see the last Harry Potter movie coming out in July. I am and always will be a Harry Potter fanatic. I enjoy going out to dinner with friends and family and love to swim at our local community pool. I love to scrapbook and have recently taken up calligraphy. It is challenging getting those letters just right. I like to try new things, some I stick with and some I don't.

Thanks to the MPS Society for always being there and supporting me throughout my life. I might not need you on a day-to-day basis, but I know if I ever need help emotionally, financially or in any way possible, the Society is always there for me. Once again, THANK YOU!

Mona Lisa Brown
24, MPS III, 12/27/10

Matthew Caldwell
17, MPS II, 3/11/11

Bailey Lott
12, MPS III, 3/14/11

Chip Radius
15, MPS II, 3/29/11

Richard Rotelli
25, MPS III, 5/2/11

Nathan Vasquez
13, MPS III, 4/17/11

Steffany Barnett, MPS III

6/29/95–2/26/11

Our children are not our own, but God's most precious gift—the spirit of a child. You conceived this spirit and it grew and matured into its earthly body—named Steffany. She is a special spirit entrusted to you, her special parents, to love, nurture and care for.

In her short life, Steffany taught us much about unconditional love, patience, caring and how to live in the eternal now.

One of the most difficult tasks is giving up the physical part of Steffany and allowing the spiritual part to graduate back to God's care.

The physical may fade, but the everlasting gift we have are her memories.

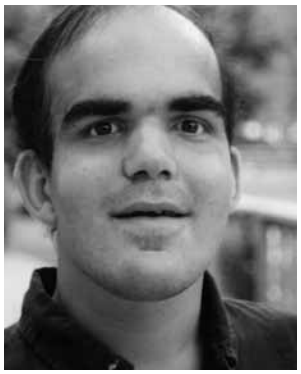
Steffany, we love and bless you and commend you back to God—your mission accomplished here on earth.

Aunt Bon

Richard Rotelli, MPS III

10/6/85–5/2/11

Following is the eulogy read at Richard's funeral by his siblings, Veronica Vacca and Christopher Rotelli, who wrote it with siblings Becca and his twin brother, Kyle.



Having Richard as a brother was the greatest gift imaginable. The lessons he taught us have shaped who we are today. Thank you, Richard, for teaching us how to recognize what's really important in life. "Don't Worry Be Happy" was one of his favorite songs as a kid, and it truly represents the way he lived.

We are all so blessed to have experienced Richard's love, and while we are sad to say goodbye today, we believe Richard would want us not to worry; not to be sad, but to be happy and celebrate his life. So with that in mind, we'd like to share some of the stories of Richard over the years.

Richard was the happiest kid you've ever met. I'm sure he is laughing as loud as he can right now…anyone who has ever been in church with Richard should know what I am talking about. He'd march down the isle with the seven of us, jumping, laughing, waving to everyone he saw, and pronouncing "amen" at the top of his lungs. It didn't matter that we were five minutes late and mass had already started; Richard would always let everyone know we had arrived.

He loved life and ate it up, often times literally. Six cloves of garlic, whole sticks of butter, beer, bolognaise…he enjoyed just about everything.

As most of you know, we spent our summers on the beach at the Dunes Club. One of our favorite stories that mom tells is a day when Richard slipped away from her. He was very quick back then. So she spent some time frantically looking for him in his usual spots: the snack bar, the bathhouses, sitting with some cute blondes on the beach. Finally, she found Richard in the formal dining room. There he was, sitting shirtless at a table in a soaking wet bathing suit. Richard had found a formal wedding reception and he was happily enjoying a piece of their wedding cake. He looked up and said, "Hi mom" as she gracefully excused them from the reception.

There is more than one woman at the beach who lost a string bikini top with one swipe of

continued

Richard's quick hand while walking by, and an older couple who in one fell swoop had their entire scrabble board scattered across the beach.

You can't talk about Richard without talking about our parents. There wasn't an activity we did that Richard was not a part of. From skiing, swimming, soccer, or just watching his favorite sitcoms and cooking fine foods with mom and dad, Richard and our parents did everything together. They were a real team.

He often rode in the cart while my parents played golf. Last spring he was finally fed up that they never let him play. Mom and dad were taking their fairway shots on the ninth hole, leaving Richard sitting in the cart critiquing their form. Richard had had enough, and decided to hijack the golf cart. He floored it, jumped two sand traps, and almost gave both of my parents a heart attack before stopping short of the water with a big smile on his face.

Richard was tough and brave. He was dealt a tough hand, but played it as well as he possibly could, and always with a smile on his face. His courage was inspiring, and his happiness was contagious.

He was loved by everyone, made us all better people, and infected us with his smile and warm embrace. The love he shared with all of us can still be felt in the room and will always be here.

We could not be more proud to be his siblings. He was the happiest, toughest, bravest and kindest person we've ever met.

Thank you Richard for teaching us these life lessons, and thank you all for surrounding him with so much love. Let's celebrate his life, and pass on the gifts he shared with us to everyone we know.

(To see a video of the eulogy, go to **http://vimeo.com/23514110**, password: Richard.)

Twenty-three years ago we received the diagnosis of MPS III for our son, Richard. I could not imagine how my family or I would survive this horrible disease. The doctors described a gut-wrenching timetable for the disease and all its horrid effects on Richard. What we did not hear at that time was the amazing blessings and gifts Richard would bring to our family and friends. So today, as I write so soon after Richard's passing, I hope to focus on some of those gifts.

Richard and his fraternal twin brother, Kyle, were the youngest of our five children. Needless to say our family was a loud and active one, which was perfect for Richard. He was one of the first to receive a bone marrow transplant in Minnesota under the direction of Dr. Chet Whitely. Richard and I spent more than three months in Minnesota while Peter manned the home front in Rhode Island and traveled back and forth. As difficult as this decision was, it was the right one for our family.

I often worried how this disease would affect my other children. As my children matured into adulthood, I became acutely aware that this had been a blessing for sure. Richard's siblings are incredible. They have qualities I definitely attribute to having Richard as a brother. They are humble, hard working, accepting of others, generous, well balanced and all have amazing friends; friends who willingly included Richard in their activities. They helped feed, lift, walk or watch Richard without any prodding. So often they amazed me; even as young children they innately knew how to love and accept Richard far better than most adults. Richard taught them lessons they would not learn elsewhere. These children, now adults, traveled from distances throughout the country last week to join in celebrating Richard, a testament to how integral a part he was to them. I too have made some amazing friendships due to Richard; people whom I would never have met without him in my life. I honestly believe there are angels walking among us.

I cannot say this was always an easy journey. The exhaustion and stress of caring for a child with MPS can be overwhelming, especially as you try to maintain a somewhat normal family life. Fighting the bureaucracy to find the needed support and help was lonely and infuriating until things finally fell into place. And the fear of what was to come caused so much anxiety. Yet, Richard's zest for life and unconditional love of everyone always made it worth the effort.

I invite you to view the multimedia presentation I compiled in honor of Richard [**http://vimeo.com/23514110** (password: richard)]. I finished it the week before Richard died, not expecting it to be shown for quite some time. I believe it shows what a full, happy life Richard experienced, surrounded by love! There have always been so many more smiles than tears in this family because of him.

Thank you, Richard.

Rosemary Rotelli

Family Support Committee Announces NEW PROGRAM

Medical Travel Assistance Program assists families with long-distance medical appointments

Families may need to travel long distances to consult with medical professionals who are knowledgeable about MPS and related diseases. The Medical Travel Assistance Program (MTAP) helps fund out-of-town travel costs for such non-recurring medical appointments. MTAP may reimburse up to $500 per affected individual, per 12-month period, in transportation costs for member families traveling to a medical appointment more than 200 miles from their home.

Reimbursable expenses include air, train or bus fares, rental car, airport parking, and transportation to and from the medical facility or appropriate mileage at the current IRS rate. Only coach airfares are eligible for reimbursement. A copy of the ticket invoice and other original travel receipts are required for reimbursement. Verification of the attended medical appointment must be included. Please read program guidelines in the Family Support section of our Web site for complete details.

The MTAP is a three-step process. In step 1, the applicant will submit an application to request funding for a future appointment. The Society will notify applicant if funding has been approved. In step 2, the approved applicant will attend the medical appointment and have the verification form signed by the medical professional. In step 3, the approved applicant will submit the completed reimbursement request and verification forms along with the travel receipts to obtain reimbursement.

To request an application or if you have any questions, contact LaurieTurner at 207.843.7040 or laurie@mpssociety.org. Applications also can be downloaded from the Family Support Section of the MPS Society Web site.

Insurance Help

BioMarin Offers Help with Insurance Questions for MPS VI

BioMarin has developed a free and confidential service designed to assist with healthcare insurance questions. The BioMarin Patient and Physician Support program (BPPS) can help families evaluate their current healthcare insurance coverage, provide information on potential healthcare insurance options that may be available in your state, and educate insurance companies about MPS VI. To contact a BPPS patient advocate, call their toll free number 1.866.906.6100.

Shire HGT Offers Support for MPS II

www.hunterpatients.com

Shire Human Genetic Therapies introduced OnePath support center to help understand the complex issues pertaining to ERT, coverage, coding and reimbursement. When you call OnePath, you'll be assigned a personal case manager who will help address any questions or concerns associated with ERT. OnePath case managers have access to many important resources; they also get to know you and can anticipate your needs. OnePath case managers can also address questions about insurance and other issues related to ERT. Toll-free number: 866.888.0660

Genzyme Treatment Support Offers MPS I Families Insurance Counseling

Genzyme Treatment Support is a free and confidential service staffed by a team of nurses, social workers, and other health care professionals act as patient advocates and provide confidential one-on-one insurance counseling to patients and their families. For more information, call 1.800.745.4447, Monday through Friday.

New NAGLAZYME.com Web Site Up and Running

The new **NAGLAZYME.com** is now live and available for you to visit. You will find expanded content about MPS VI, its diagnosis and treatment with NAGLAZYME® (galsulfase) enzyme replacement therapy. The new Web site also has expanded features and functionality.

Fishbowl Cards offers unique greeting cards for special needs

Fishbowl Cards is a unique greeting card company with the focus on children, adults, families and friends living with special needs.

This line of greeting cards, posters and figurines reflect the celebration of hope, recovery, improvement, achievement, love and support that children with special needs, their families and friends experience every day.

Visit **www.fishbowlcards.com/Default.aspx**.

Did you know there are several Family Support Programs available to help members of the National MPS Society?

- **The Family Assistance Program** can help families or affected adults purchase durable medical goods not covered by insurance or other sources. Families or affected adults can request up to $3,000 annually.
- **The Social Gathering Program** — Do you enjoy getting together with other MPS families? You can request funds up to $750 each year from this program to help with organizing a picnic or other social function.
- **Conference Scholarship Program** — MPS families or affected adults can apply for financial assistance to attend an MPS Society family conference.
- **Continuing Education Scholarship Program** — Affected individuals and their siblings, spouses and parents can apply for one of several $1,000 Continuing Education Scholarships.
- **Extraordinary Experiences** — A new program for individuals with MPS and related diseases ages 14–24 to help create an extraordinary experience. Grants of $1,000 are available.

Contact Laurie Turner at laurie@mpssociety.org for more information.

Parent Educational Advocacy Training Center: help for families and professionals

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 in order to improve opportunities for excellence in education and success in school and community life. Its focus is children with disabilities.

For more information visit **www.peatc.org**.

MPS I Web site

www.MPSIdisease.com

A Web site has been developed by Genzyme to provide parents and patients with information and resources on MPS I. This site provides valuable information on the disease, diagnosis, on-going clinical trials, and other references and services available to patients. Visit **www.MPSIdisease.com**.

MPS I Registry

Access to information is critical to providing the best care for patients with MPS I. However, information on the disease is limited because of its rarity. A resource developed by Genzyme is now available for your physician or health care professional that is dedicated to improving the understanding of MPS I. With the MPS I Registry, your physician can access your data and compare it to aggregate data from around the world. Ask your physician to call 1.800.745.4447 ext. 17021 for more information.

MPS II Web site

www.hunterpatients.com

Shire HGT educational Web site focuses on MPS II (Hunter syndrome). The site is a resource center for the MPS II community to access information about the genetics, diagnosis, and management of MPS II, as well as information about the drug development process. In addition, the Web site provides a comprehensive overview of MPS II, including resources for patients and healthcare professionals, information on clinical trials and a patient outcomes survey, as well as the ability to stay informed as new information about MPS II becomes available on the site. Shire HGT expects to update and expand the site on a regular basis.

MPS VI Web site

www.MPSVI.com

BioMarin's Web site, **www.MPSVI.com**, is designed especially for individuals with MPS VI (Maroteaux-Lamy syndrome), their families, and for healthcare professionals who care for patients with MPS VI. This site provides education and information about MPS VI which may be helpful to share with family members, educators and healthcare providers.

Aldurazyme® Web site

www.Aldurazyme.com

A Web site has been developed by Genzyme to provide parents and patients with information on Aldurazyme. The site includes a link to ask questions regarding MPS I or anything else related to treatment. Feel free to use this mechanism to reach a healthcare professional at Genzyme who will respond to your query in a timely manner. Visit **www.Aldurazyme.com**.

MPS IV Registry

www.morquio.com

Information about MPS IV can be found at **www.morquio.com**. Also available at this Web site is the Morquio registry where adults with MPS IV can register and families can register their child with MPS IV. Once registered, it is recommended that updates be made at least yearly. This natural history information is critical for development of treatments for MPS IV, providing evidence of drug effectiveness and supporting the approval of the drug.

MPS VI Community Web site

www.MPSVI.net

Log into the first Web site devoted entirely to the MPS VI community and:

- Meet other people with MPS VI
- Tell your story
- Chat in real time
- Search postings by topic

Register for free to connect with your MPS VI community.

Resources for coping with grief

- **Hello Grief** is a place to share and learn about grief and loss. This beautiful online community includes articles, resources and forums. **www.HelloGrief.org**
- **ForeverSibs** strives to honor and recognize the unique role of brothers and sisters with rare diseases through social support and education, thereby decreasing their anxiety and isolation. **www.ForeverSibs.org**
- **Comfort Zone Camp** is a fun and safe place for grieving children. A community where kids can come year after year and obtain tools to help them cope with their daily lives. **www.ComfortZoneCamp.org**

Service dogs provide assistance, independence

As an adult with MPS IV, Julie Lintt discovered that adopting a service dog was a way of gaining more independence.

Onyx is now Julie's best friend. "She can help me in many ways," said Julie, "including picking up stuff I drop, helping me walk, helping me get off the ground if I fall, helping me to dress or undress; she can even call 911!"

Service dogs can be trained for many different needs. There are places where service dogs are available for free, however you may be on a waiting list.

Juile found Onyx through Colorado Service Dogs. There was a cost, however, a grant from the Family Assistance program at the National MPS Society helped with the cost of purchase and training. Julie also received a grant from Pennies for Nicoll's (an MPS family foundation).

For more information about service dogs, contact Lija Day, co-founder and chief executive officer of Colorado Service Dogs, at 303.669.5916 or send an e-mail to info@coservicedogs.com.

Did You Know?

The National MPS Society's Family Support Programs assisted 150 families in 2010, totaling almost $55,000.

MPS IV A Clinical Assessment Program (MorCAP)

The MorCAP has been designed to provide a better clinical understanding of the natural history of MPS IV A (Morquio A) syndrome by measuring various aspects of the disorder, including endurance and respiratory function in affected patients. These insights may help BioMarin Pharmaceutical Inc. design future clinical studies. Participation in the MorCAP study will require one or more visits yearly to a clinic or hospital for up to 10 years. Experimental drug will not be administered during these visits. MorCAP is a multinational study and includes sites located in the United States, United Kingdom, South America, Canada, Europe and Asia. For more information, call toll-free 1.866.961.8212.

Clinical Trials

MPS I

MPS I Intrathecal Enzyme Replacement Clinical Trial

The Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center in Torrance, CA, and the University of Minnesota are collaborating on a Study of Intrathecal Enzyme Replacement Therapy for cognitive decline in patients with MPS I.

The purpose of this research study is to find out whether giving enzyme replacement therapy (ERT) with Aldurazyme® as an injection directly into the cerebral spinal fluid (the fluid around the spinal cord and the brain) can stabilize (keep from getting worse) or improve cognitive decline in patients who have MPS I. The term "cognitive decline" refers to a change for the worse in our ability to think and learn. Difficulty with thinking, memory, language, concentration and decision making are some signs of cognitive decline.

To be eligible for this study, you or your child must be willing and able to comply with the study procedures and meet certain criteria:

- 6 years of age or older
- diagnosed with MPS I
- show evidence of cognitive decline on a screening evaluation

Study participants will have:

- up to 10 treatments given one to three months apart over two years (treatment group) or four treatments given three months apart beginning at month 12 (control group)
- physical examinations (general and neurologic)
- neuropsychological testing for cognitive decline and MRI of the brain
- reimbursement/payment of travel expenses

Additional details about this clinical trial can be found at **www.clinicaltrials.gov**; search under "mucopolysaccharidosis."

If you are interested in this study or would like more information, please contact:

Dr. Agnes Chen
310.222.4160 • 310.782.2999 (fax)
Email: ahchen@ucla.edu

or

Dr. Patricia Dickson
310.781.1399 • 310.782.2999 (fax)
Email: pdickson@ucla.edu

MPS I Intrathecal ERT for Children Being Considered for Transplantation

The University of Minnesota has recently obtained FDA approval for the delivery of laronidase into the spinal fluid of children with Hurler syndrome being considered for marrow/cord blood transplantation. The goal of these studies is to decrease the neuropsychologic decline that has been observed in children with MPS I from the time patients are initially evaluated to the time they are one year from transplantation. The hypothesis is that there is a significant delay in achieving sufficient enzyme levels in the brain following transplantation, and that this may be overcome by giving enzyme into the spinal fluid until this occurs. Patients with MPS I who are between 8 and 36 months of age who have not previously received enzyme therapy and are being considered for transplantation at the University of Minnesota are eligible. Patients receiving laronidase in the spinal fluid also will be on intravenous laronidase prior to transplant. The study will involve four doses of laronidase given during a lumbar puncture (spinal tap) approximately three months before transplantation, at the time of admission to the hospital for the transplant, three months after the transplant and six months after the date of the transplant. The principal investigator of the study is Dr. Paul Orchard, who can be reached at 612.626.2961 or orcha001@umn.edu. Alternatively, Teresa Kivisto, nurse coordinator with this study, can be reached at 612.273.2924 or TKIVIST1@Fairview.org.

MPS II

MPS II Intrathecal Enzyme Replacement Clinical Trial

Shire Human Genetic Therapies is sponsoring a clinical trial at the University of North Carolina at Chapel Hill to learn if direct administration of recombinant enzyme into the fluid around the brain and spinal cord is safe and a possible treatment for children with MPS II with developmental delays. The principal investigator for the clinical trial, "A phase I/II safety and ascending dose ranging study of idursulfase administration via an intrathecal drug delivery device in pediatric patients with MPS II who demonstrate evidence of central nervous system

continued

involvement and who are receiving treatment with Elaprase®" is Joseph Muenzer, MD, PhD.

Currently there is no approved therapy for treating the brain and spinal cord in patients with the severe form of MPS II. The goal of this study is to give a new preparation of iduronate-2-sulfatase (idursulfase-IT) directly into the fluid surrounding the brain and spinal cord (intrathecal administration). The new form of iduronate-2-sulfatase has not been used before in patients with MPS II and is considered investigational. It has not been approved by the FDA or any other regulatory agency.

This phase I/II clinical trial is planning to enroll 16 patients with MPS II between the ages of 3 to 8 years with evidence of early neurocognitive decline using an open-label, three-dose trial design. This clinical trial will initially have both a treatment group (12 study patients) and a control group (four study patients) with the control group eligible to receive intrathecal enzyme after a six-month observational period. The monthly intrathecal administration of idursulfase-IT will be given using a Port-A-Cath® II Low Profile™ intrathecal implantable access system manufactured by Smiths Medical MD, Inc. that requires surgical implantation.

To be eligible for the investigational intrathecal enzyme replacement clinical trial, study patients needs to have some developmental delay, but cannot be severely impaired, have received and tolerated a minimum of six months of weekly intravenous Elaprase and have adequate hearing (with or without hearing aids) to complete developmental assessments. Patients with MPS II are not eligible if they have a shunt for the treatment of hydrocephalus, have had a cord blood or bone marrow transplant, or have other medical conditions that may place the individual at an increased risk during the investigational clinical trial.

For more information about the clinical trial, contact Dr. Joseph Muenzer at 919.966.1447, or the study coordinator, Heather Preiss, RN, at 919.843.5731.

MPS III

Phase I/II Study of ERT for MPS III A

Shire Human Genetic Therapies is developing a sulfamidase enzyme replacement therapy (ERT) for patients with MPS III A. rhHNS is being administered into the cerebrospinal fluid via a surgically implanted intrathecal drug delivery device (IDDD), because when administered intravenously it does not cross the blood brain barrier.

This study is a multi-center, multiple-dose, dose escalation study designed to evaluate the safety, tolerability and clinical activity of up to three dose levels (two doses [10 and 45mg] monthly and one dose [45mg] every other week for six months) of rhHNS administered via an IDDD in patients with MPS III A ages greater than or equal to 3 years of age.

The phase I/II clinical trial is planning to enroll 15 patients, beginning June 2010. The study is expected to be completed March 2012, and the duration of the study for each patient is nine months.

Patients who have completed all study requirements in this study will be invited to participate in an open-label extension study that will be designed to evaluate long-term safety and clinical outcomes of intrathecal administration of rhHNS.

The phase I/II clinical study is being conducted at two sites: Emma Children's Hospital, Academic Medical Center in The Netherlands by Dr. Frits Wijberg; and the St. Mary's Hospital in Manchester, UK, under the direction of Drs. Simon Jones and Ed Wraith. The letter to the Society from Shire about the study can be accessed on our Web site, under the Clinical Trials section.

Additional information about the clinical trial can be obtained at **http://clinicaltrials.gov/ct2/show/NCT01155778?term=MPS+III+intrathecal&rank=1**, or by contacting Tiffany Crump at 484.595.8257 or tcrump@shire.com, or Daryll Heron at +44 1256 894572 or dheron@shire.com.

MPS IV

BioMarin Pharmaceutical Inc. announced Feb. 1, 2011, that it has initiated a pivotal phase III trial for *N*-acetylgalactosamine 6-sulfatase (GALNS or BMN 110), intended for the treatment of MPS IV A (Morquio A syndrome).

"In under two years, we have progressed the GALNS program from Clinical Trial Application to initiation of the phase III trial," said Jean-Jacques Bienaime, chief executive officer of BioMarin. "We have received FDA feedback and have finalized the design of the phase III pivotal trial. The study will be conducted at approximately 40 centers worldwide including Brazil, Japan, Taiwan, most Western European countries, Canada and the United States.

continued

The trial is expected to enroll approximately 160 subjects and will be the largest enzyme replacement therapy trial conducted. There are no therapeutic options for MPS IV A patients who have a high unmet medical need. Initiation of this well-designed pivotal study is an important milestone for both the company and the MPS IV A community."

The phase III trial is a randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of GALNS in patients with MPS IV A. The study will explore doses of 2 mg/kg/week and 2 mg/kg/every other week for a treatment period of 24 weeks. The primary endpoint is the six-minute walk test, and the secondary endpoints are the three-minute stair climb test and urine keratan sulfate concentration.

Highlights from the phase I/II study:

- Endurance improvements with GALNS were consistent with, and in some cases, better than those observed in pivotal studies of approved enzyme replacement therapies.
- Clinically meaningful improvements in two measures of endurance (six-minute walk distance and three-minute stair climb) were achieved at both 24 weeks and 36 weeks as compared to baseline.
- Clinically meaningful improvements in two measures of pulmonary function (forced vital capacity and maximum voluntary ventilation) were achieved at 36 weeks as compared to baseline.
- Keratan sulfate levels decreased shortly after the initiation of treatment and fell further as the study progressed.
- The frequency and severity of infusion reactions were comparable to those observed with Naglazyme® and Aldurazyme®.

Additional information can be found at **www.bmrn.com and http://clinicaltrials.gov/ct2/show/NCT00787995?term=MPS+IV&rank=1**.

Treatment Therapies

MPS I

Aldurazyme®, administered once-weekly, has been approved in the United States and in 15 countries of 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 Genzyme.

Additional information can be obtained at **www.aldurazyme.com** or by contacting Genzyme at 800.745.4447.

MPS II

Elaprase® is a long-term ERT for patients with a confirmed diagnosis of MPS II which has been approved for use in the United States, Canada and many countries in Europe. Elaprase was developed and is produced by Shire Human Genetic Therapies (formerly TKT), 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 **www.shire.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 VI

Naglazyme® is the ERT for individuals with a confirmed diagnosis of MPS VI 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.

Position Statement on the use of genistein to treat MPS types III A-D

MPS Stem Cell Research Group, Department of Biomedicine, University of Manchester Genetic Medicine, St. Mary's Hospital, Central Manchester University Hospitals NHS Foundation Trust

The Manchester team has recently published preclinical data in the mouse model of Sanfilippo disease III B (MPS III B) showing significant delay in neurodegeneration and behavioral correction following high daily doses of the drug genistein aglycone delivered over a nine-month period.

The mouse model of MPS III B is affected by increasing pathological heparin sulphate storage in the brain and in other organs, neuroinflammation, progressive neurodegenerative decline, abnormal behavior and hyperactivity with a shortened life span and as such, shows many similarities with patients with the disease.

Following nine months of high daily doses (160 mg/kg/day) of genistein aglycone, we have shown a 31–34 percent reduction in lysosomal compartment size in the brain and 37 percent reduction in brain levels of pathological heparin sulphate in the mouse model of MPS III B. Neuroinflammation was reduced by 12–19 percent while most behavioral abnormalities observed at eight months, including the lack of a sense of danger and hyperactivity, were corrected by drug treatment.

We are aware that many parents already give their affected children different forms of genistein and other supplements and thus feel that it is important to offer interim advice prior to a clinical trial due to the widespread availability of genistein as a dietary supplement.

Genistein may have several modes of action, including blocking protein tyrosine kinase receptor function and also is a mild oestrogen analogue. It has undergone significant safety testing in rodents and dogs but has only been tested in humans at low doses for its use as a food supplement or for treatment of osteopenia and menopausal flushes in the United States.

Genistein used in the preclinical study was synthetically produced and is the pure aglycone form of the drug. Genistein also can be purified from soy extract, but this is not necessarily the same product and we would advise against its use since the naturally occurring form of genistein may not be absorbed as effectively by the digestive system.

While genistein is available as a dietary supplement at lower doses, there is not yet any clear clinical data to suggest that it is effective at these doses in Sanfilippo.

Synthetic genistein aglycone is not widely available and has not been tested in a clinically controlled manner in humans at the effective and very high doses used in mice. The use of genistein in this disease at high doses is considered to be an Investigational Medicinal Product by European regulatory agencies, thus we would caution against its use by families with affected children.

We intend to run a placebo-controlled clinical trial using high doses of pharmaceutical grade genistein aglycone in patients with MPS III A, B and C in the near future in Manchester, subject to appropriate regulatory approval.

It is important that we are able to run a clinical trial comparing genistein at high doses to a placebo control as this is the only way we can be sure if it really has an effect or not. We are currently evaluating the appropriate dose for human use, as this is often relatively lower than those used in mice and, again, would advise against the use of high doses of genistein until we have concluded this work.

The preclinical genistein publication is available at **www.plosone.org/article/info%3Adoi%2F10.137%2Fjournal.pone.0014192**.

For further information, contact Dr. Simon Jones, simon.jones@dmft.nhs.uk, or Dr. Brian Bigger, brian.bigger@manchester.ac.uk.

This article was published in the UK MPS Magazine, Winter 2010.

Soya beans could hold clue to treating fatal childhood disease

Scientists from The University of Manchester say a naturally occurring chemical found in soy could prove to be an effective new treatment for a fatal genetic disease that affects children.

Dr Brian Bigger, from the University's MPS Stem Cell Research Laboratory, found that genistein—derived from soya beans and licensed in the United States as an osteoporosis drug—had a dramatic effect on mice suffering from the human childhood disease Sanfilippo.

"Sanfilippo is an untreatable mucopolysaccharide (MPS) disease affecting one in 89,000 children in the United Kingdom," said Dr Bigger, who is based in the School of Biomedicine.

"Children with Sanfilippo disease experience progressive deterioration of mental function, similar to dementia, in early childhood, with other symptoms including severe behavioral problems, hyperactivity and ultimately death in early teens."

In the study, published in the journal *Public Library of Science One*, mice with Sanfilippo disease were fed with high doses of genistein over a nine-month period. Treated mice showed a significant delay in their mental decline, including a third reduction in the amount of excess sugars found in the brain as a result of the disease, and a sixth reduction in inflammation in the brain.

Importantly, the research, carried out with colleagues at St Mary's Hospital in Manchester, also showed that the hyperactivity and other abnormal behavior normally seen in Sanfilippo mice were fully corrected by genistein treatment.

Professor Wraith, a co-author on the study and consultant pediatrician from Genetic Medicine in St. Mary's Hospital, said, "Sanfilippo is a disease where the genetic lack of an enzyme leads to a fault in the breakdown of complex sugars in the cell.

"This leads to storage of these undegraded complex sugars in cells, disturbances in brain function and ultimately to this profound mental deterioration that we see in the children with this condition. Manchester is a specialist centre for this type of genetic disease and as such we look after more than 100 patients from all over the UK and beyond."

The Manchester team, supported by the UK Society for mucopolysaccharide diseases and the Manchester Biomedical Research Centre, hope to announce a placebo-controlled clinical trial for patients with Sanfilippo disease in the near future.

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MPS III Mouse Model

During the Expert Meeting on Sanfilippo Disease held in Northampton in August 2010, the question was raised as to whether there is a mouse model for Sanfilippo Type C disease. It is recognized that these mouse models play a very important role in pre-clinical research and without this model research may be severely hampered. Upon further investigation, Professor Bryan Winchester and Dr. Brian Bigger of the University of Manchester MPS Stem Cell Group have identified a research group in Canada that has the MPS III C mouse model. Dr. Bigger is now in touch with Alexei Pcheietski from the University of Montreal to understand the nature of his mouse model and the current research initiatives.

This article was published in the UK MPS Magazine, Winter 2010.

Genistein Improves Neuropathology and Corrects Behavior in a Mouse Model of Neurodegenerative Metabolic Disease

Marcelina Malinowska, Fiona L. Wilkinson, Kia J. Langford-Smith, Alex Langford-Smith, Jillian R. Brown, Brett E. Crawford, Marie T. Vanier, Grzegorz Grynkiewicz, Rob F. Wynn, J. Ed Wraith, Grzegorz Wegrzyn, Brian W. Bigger

Abstract

Background: Neurodegenerative metabolic disorders such as MPS III B (or Sanfilippo disease) accumulate undegraded substrates in the brain and are often unresponsive to enzyme replacement treatments due to the impermeability of the blood brain barrier to enzyme. MPS III B is characterized by behavioral difficulties, cognitive and later motor decline, with death in the second decade of life. Most of these neurodegenerative lysosomal storage diseases lack effective treatments. We recently described significant reductions of accumulated heparan sulphate substrate in liver of a mouse model of MPS III B using the tyrosine kinase inhibitor genistein.

Methodology/Principal Findings: We report here that high doses of genistein aglycone, given continuously over a nine-month period to MPS III B mice, significantly reduce lysosomal storage, heparan sulphate substrate and neuroinflammation in the cerebral cortex and hippocampus, resulting in correction of the behavioural defects observed. Improvements in synaptic vesicle protein expression and secondary storage in the cerebral cortex were also observed.

Conclusions/Significance: Genistein may prove useful as a substrate reduction agent to delay clinical onset of MPS III B and, due to its multimodal action, may provide a treatment adjunct for several other neurodegenerative metabolic diseases.

Citation: Malinowska M, Wilkinson FL, Langford-Smith KJ, Langford-Smith A, Brown JR, et al. (2010) Genistein Improves Neuropathology and Corrects Behaviour in a Mouse Model of Neurodegenerative Metabolic Disease. PLoS ONE 5(12): e14192. doi:10.1371/journal.pone.0014192

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MPS Classifications

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

Syndrome	**Eponym**	**Enzyme Deficiency**
MPS I	Hurler, Scheie, Hurler-Scheie	α-L-Iduronidase
MPS II	Hunter	Iduronate sulfatase
MPS III A	Sanfilippo A	Heparan *N*-sulfatase
MPS III B	Sanfilippo B	α-*N*-Acetylglucosaminidase
MPS III C	Sanfilippo C	Acetyl CoA: α-glycosaminide acetyltransferase
MPS III D	Sanfilippo D	*N*-Acetylglucosamine 6-sulfatase
MPS IV A	Morquio A	Galactose 6-sulfatase
MPS IV B	Morquio B	β Galactosidase
MPS VI	Maroteaux-Lamy	*N*-Acetylgalactosamine 4-sulfatase (arylsulfatase B)
MPS VII	Sly	β-Glucuronidase
MPS IX		Hyaluronidase
ML II/III	I-Cell, Pseudo-Hurler polydystrophy	*N*-acetylglucosamine-1-phosphotransferase

Board of DIRECTORS

Steve Holland, president
Amy Holland
1752 Hilltop Circle
Fort Worth, TX 76114
817.625.6999
steve.holland@mpssociety.org
amyholland@mpssociety.org
MPS I H-S parents

Kim Frye, vice president
Stephen Frye
3625 E. Thousand Oaks Blvd.,
STE 217
Westlake Village, CA 91361
818.263.7420
kim.frye@mpssociety.org
stephen.frye@mpssociety.org
MPS II parents

Tom Gniazdowski, treasurer
Anne Gniazdowski
315 Meadowview Court
Springboro, OH 45066
937.748.8809
tom.gniazdowski@mpssociety.org
anne.gniazdowski@mpssociety.org
MPS II parents

Austin Noll, secretary
3735 Redwood Circle
Palo Alto, CA 94306
650.521.0089
austin.noll@mpssociety.org
MPS III parent

Jeff Bardsley
1209 Daviswood Drive
McLean, VA 22102
703.547.7087
jeff@mpssociety.org
MPS II adult

Stephanie Bozarth
6106 Larstan Drive
Alexandria, VA 22312
703.256.1980
stephanie@mpssociety.org
MPS IV parent

Jennifer Clarke
186 Odd Road
Poquoson, VA 23662
757.868.7569
jenniferclarke@mpssociety.org
MPS III parent

Angela and Luis Guajardo
1815 Post Oak Road
Edinburg, TX 78539
956.287.2887
angela@mpssociety.org
MPS III parents

Kristine Klenke
7604 Sherry Creek Road
Worden, IL 62097
618.888.2204
kris.klenke@mpssociety.org
MPS II parent

Hope and Dave Madsen
16610 North 11th Street
Lakeland, MN 55043
651.331.9625
hopemadsen@mpssociety.org
davemadsen@mpssociety.org
MPS I parents

MaryEllen Pendleton
56 E. Vinedo Lane
Tempe, AZ 85284
480.831.2157
maryellen.pendleton@mpssociety.org
MPS III aunt

Kim Whitecotton
1413 Emigrant Way
Modesto, CA 95358
209.544.2708
kim.whitecotton@mpssociety.org
MPS II parent

Gordon Wingate
16319 Jordyn Lake
Tomball, TX 77377
832.498.1724
gordonwingate@mpssociety.org
MPS III parent

President Emerita
Marie Capobianco
Ernie Dummann
Steve Holland
Mary Majure Couture
Linda K. Shine

STAFF

Barbara Wedehase, executive director
barbara@mpssociety.org

Terri Klein, development director
terri@mpssociety.org

Laurie Turner, program director
laurie@mpssociety.org

Kelly Rose, administrative assistant
kelly@mpssociety.org

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National MPS Society
PO Box 14686
Durham, NC 27709-4686