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

Volume 34 | Number 4 | Winter 2010

COURAGE

Conference Recap
24th annual conference offers something for everyone: families, affected adults and those remembering a loved one

We hear you!
Membership survey results reported

Super Siblings
Society honors brothers and sisters of MPS children

"Our family's experience of intrathecal ERT"
Steve Holland speaks about his family's chosen treatment path

MPS III Expert Meeting Review
Researchers from around the world provide insight, knowledge and renewed hope

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.

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National MPS Society
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Courage *reserves the right to edit content as necessary.*

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Pictured on the cover:
Andre Andrews (ML)
Brooklyn Boyce (MPS III)
Sergio Torres (MPS II)

Membership & Subscription Form

Name

Affected Individual's Name

Date of Birth

Diagnosis

Relationship

Address

City, State, ZIP

Telephone

E-mail

Family	☐ $50.00
Foreign	☐ $80.00
Professionals	☐ $75.00

Corporate Memberships Available

Would you like your name to appear in our directory? ☐ YES ☐ NO

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

Would you like our publications in
☐ electronic (e-mailed) format or
☐ hardcopy (mailed) format

Please send your membership form and check to:

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

A Tribute

A tribute to Ernie and Debbie Dummann

In 1998 Ernie and Debbie Dummann began their tenure on the National MPS Society board of directors (BOD). During that time they initiated many changes to move the Society forward, served on committees and held officer positions. Early in 2010 they made the difficult decision to not run for another two years, with the ending of their current tenure coinciding with the completion of Ernie's four-year presidency. During the awards banquet at the 2010 family conference in Buena Park, CA, they were recognized for their years of service. Ernie was awarded the Crystal Diamond Award, a scrapbook with photos and correspondence was presented to Ernie and Debbie, and Debbie was recognized for her dedication to the Society with an artisan amber necklace and earrings.

"It is my distinct honor and privilege to present an award to an individual who will be retiring from our BOD at the end of this year," said board member Steve Holland. "He has truly distinguished himself in every way. His loyalty and dedication to the MPS cause has been unwavering ever since I have known him.

"Let me take you back in time to 1997. Amy and I had lost our first attempt to run for the BOD after attending conferences for several years. (Back then you could only run every two years.) So we were running again and I read the background info about this guy who was running for the BOD—a guy who had not yet set foot in an MPS conference. It said that he was appointed by the governor of Alaska to a state-wide disability committee. I thought—how can I compete with that?

"He and his wife and my wife and I were both elected, and we started our board service together in 1998. One of our first major changes on the board was to revise the Society's bylaws—a process that had not occurred since the Society's creation many years earlier. Ernie quickly volunteered to spearhead the effort—which turned out to be a total rewrite. I recall wondering how in the world he was going to pull this off. (This was before Microsoft Word made these tasks easy.) He did it by hiring a legal secretary out of his own pocket who created redlines for us throughout the drafting process. I even recall Ernie sending a draft out on Christmas Eve and the then president Linda Shine sending me an e-mail saying, "I think this guy might be for real!"

"So, he has served on the BOD for the past 13 years and attended approx 40 in-person BOD meetings, spending tens of thousands of unreimbursed travel dollars and participated in hundreds of committee teleconferences. He has served on the Education Committee and as an officer for the past 11 years—three years as vice president, four years as treasurer and four years as president—the only person to serve in three officer positions—and the only president to also chair a standing committee, the Legislative Committee.

"Over the years as he took on each new challenge —being treasurer and then legislative chair, I learned to quit wondering how he would do it and just take comfort in the fact that it would get done. I can't begin to describe how much I have personally learned from him—his can-do attitude, his ability to make tough decisions when necessary, and his commitment to finish a job and do it in style.

"It is my honor to bestow the Crystal Diamond Award to Ernie Dummann."

Accepting the award, Ernie said:

"As the dad of a young man who was disabled due to MPS disease, I am honored to receive this award, allowing me to thank all those involved who have made it possible for me to be an advocate for all who have MPS and related disease in their lives.

"To be a good advocate you need a lot of help and support from our MPS families and people all around you.

"You need pharmaceutical companies dedicated to our Society like BioMarin, Genzyme and Shire to help you learn about the difficulty bringing treatments to the patient and the ways we as advocates can help speed delivery.

continued

"You need visionaries like Mary Coture, Marie Capobianco and Linda Shine who dreamed of a better life for MPS children and were willing to make sacrifices.

"You also need the help of commissioners like Michael J. Astrue, with the Social Security Administration.

"You need policy makers at the National Institutes of Health like Dr. Tagle, Dr. McKeon and Dr. Groft.

"You need senators such as Arlen Specter and Lindsey Graham.

"You need change agents like Dr. Emil Kakkis and MPS member Mark Dant.

"You need powerful insiders like Barbara Wedehase.

"You need great staff like Laurie Turner and Terri Klein.

"You need dedicated researchers like Dr. Neufeld, Dr. Muenzer, Dr. Haskins and Dr. Ellinwood, just to name a few, to seek out and find treatments and cures.

"You need international MPS leaders like Christine Lavery of the United Kingdom.

"You also need good friends who are powerful advocates in their own right like Karin Adams, Sue Rattman, Steve Holland, Larry Kirch, Les Sheaffer, Sissi Langford, Kris Klenke, Angela Guajardo, Jennifer Restemayer and Kim Frye to help mold your policies for the betterment of all.

"You need a network of organizations and caregivers to provide quality of life needs like respite, home modifications, palliative care and in-home services.

"Then you need true partners. Partners who will share your pain, your disappointments and your vision, but most of all your successes. One of my partners, my wife Debbie, is here with me tonight. She first started advocating for the disabled when she was just a teenager, testifying on a state and local level to policy makers molding a better quality of life for all who suffer terrible illnesses. She's been with me all the way. A confidant in all my deliberations, a partner in all decisions and most of all a trusted friend who is there any time I ask.

"Lastly you need inspiration, like a child who was born with an MPS disease, a disease that has disabled him and caused him to cry out many times. A disease that isolated him from the community and caused him to lay helpless at the mercy of all around him. My inspiration was and will always be our son Sean who passed from us three months ago.

"Although Sean could not speak and required full-time care, with his smile, a look and the touch of his hand he did more to inspire people to support, care for and protect all people who experience disabilities more than anyone I have ever known. He continues to motivate me and others like me to improve the lives of MPS individuals and their families.

"My wife and I are proud to have served on your board of directors for the past 13 years, and I am honored to accept and dedicate this award to Sean and all these people who deserve this recognition, along with my thanks.

"After all, I am just a dad."

Executive Director's Letter

This being the holiday season, I have been thinking about the many things for which I give thanks. In addition to my family and friends, I am thankful for the friends I have made among our National MPS Society members. We are an organization comprised of people throughout the country who share a special bond of caring for one another and working toward the common goal of finding a cure for MPS and related disease. It is such a pleasure for me to work with our board of directors and our Scientific Advisory Committee, dedicated groups of people who selflessly give of their time and expertise.

So many of you devote your time to fundraising, allowing us to fund research and move forward in providing support services to our members. At the time of this writing, $217,000 has been raised for research through the 2010 walk/runs, with money from the October runs coming to the office daily. In addition, this year's Annual Fund appeal has generated $57,000. The second Annual Fund letters have been sent to all our members and supporters, so please make your donation before Dec. 31.

We will be saying goodbye in December to board members Ernie and Debbie Dummann, Klane and Amy White, and Tami Slawson. The tribute to the Dummanns on page 2 notes some of their many contributions to the Society during their tenure on the board. During Klane and Amy's six years on the board, they moved the family support programs forward and provided a wealth of educational and medical information. Tami Slawson and Amy White were amongst the developers of the Governance Committee and advocates for strengthening the board. Tami works tirelessly for all our families to ensure they receive the services and information they need. Interestingly, all of these board members have indicated they want to continue working for the Society – although they won't be official board members. We are so grateful for all they have done and will continue to do.

The following incumbents were elected for a two-year term: Anne and Tom Gniazdowski, Kris Klenke and Kim Whitecotton. We welcome our new board members elected by the membership, Jeff Bardsley, and Hope and Dave Madsen. MaryEllen Pendleton and Stephanie Bozarth have each been appointed for a two-year term on the board of directors. You'll be hearing much more about our new board members in the next issue of *Courage*.

During this holiday season, please join me not only in giving thanks to everyone in our MPS family, but also in remembering all the very special children and adults who are no longer with us.

Barbara Hudson

Fundraising: road to a cure. This has been our theme throughout last year. Each event, large and small, set its GPS to CURE. Whether the road was smooth at high speeds or bumpy moving a bit slower, fundraising in 2010 has once again been successful. Our families truly embraced the challenges of hosting events throughout the year, determined to make a difference on behalf of a loved one or friend affected by MPS or ML. How exhilarating it has been to watch our families set goals and reach them.

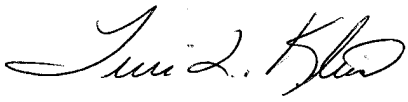
In 2010 fundraising and walk/run events included school plays, lemonade stands, hot dog stands, auctions, jewelry parties, golf tournaments, baseball scrambles, cornhole tournaments, charity dinners, inaugural events, Sponsor a Child for a Cure and much more! Highlights from some events hosted this year can be found in the fundraising section of *Courage* (page 22). Please take a moment to read about these extraordinary families and their efforts.

On behalf of the Fundraising Committee, thank you donors for contributions to the 2010 Annual Fund Campaign. The Annual Fund focuses directly on National MPS Society programs and our operational budget. This campaign provides funds to meet the goals of our mission, support for families and research for a cure.

Each and every event set its GPS on a course for the road to a cure. Still, we need your help. Last year we had fewer events that celebrated the 11th annual walk/runs for research; we need to increase our efforts. These events support our general research funding. For more details on hosting an event, corporate gift matching programs, the United Way, combined federal campaign or planned giving, please contact me directly at terri@mpssociety.org.

Be sure to visit our Web site (**www.mpssociety.org**) frequently for upcoming events and our Giving pages. There are many helpful ideas and tools available for you to help the Society now and in the future.

Happy New Year!



Wow, what an amazing conference in California! It is always energizing and refreshing to be able to spend time with all of you. It is wonderful to visit with old friends and get to know so many new friends. As we move into 2011, we are getting excited for the St. Louis conference, and I hope that its central location will allow many of you to attend; it promises to be a fantastic conference.

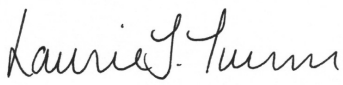
Our first SPIRIT conference for adults affected with MPS and related disease was an amazing experience and we are looking forward to hosting this adult-only conference again in 2012. We also held a CYCLE conference for those families who have lost a child to MPS; once again this conference allowed for families to share memories and provides strength to help move forward.

I also would like to thank our retiring board of directors. Thank you for your countless hours, unending dedication and expertise in helping the Society continue to move forward. I am looking forward to working with our new board of directors in 2011!

Thank you for the well wishes on baby Amelia's arrival, and thanks to Barb and Terri for taking over my duties during my maternity leave.

You will notice a new section in this issue of *Courage*, OSCAR awards. This section honors outstanding siblings and children in our amazing MPS families. We have honored our super siblings for many years, and thanks to Dorothy Mask these individuals will now be formally recognized. If you wish to nominate someone in your family, please see page 11 for more information.

We would love to hear how your family is doing—send us an update, let us know what is happening and don't forget we love photos!



New Members

Lourdes Arellano
Fresno, CA, mother of Gabriela and Vincent Arellano, MPS III A

Bertha Avendano
Hawthorne, CA, mother of Adan Avendano, MPS II

Chris and Allison Bingham
Raleigh, NC, parents of Riley Bingham, MPS I

Jennifer Bishop
Scottsdale, AZ, mother of C.J. Bishop, MPS I

Julie Boos
Lees Summit, MO, mother of Landon Boos, MPS III

Rebecca Castillo
Uvalde, TX, mother of Adam Nathan Vasquez, MPS III A

Vince Clemens
Martinez, CA, adult with MPS VI

Diane Dalton
Lake Forest, CA, mother of John Dalton, MPS III A

Felicia Espinosa
Fresno, CA, sister, MPS III A

Delina Frenette
Morris, CT, legal guardian of Jonquasia Frenette, MPS IV

Safiyyah Karriem
Fresno, CA, mother of Marquis Roberts, MPS II

Veronica Landeros
Hayward, CA, mother of Javier Landeros, MPS I

Salvador and Adriana Maciel
Irvine, CA, parents of Andrea Maciel, MPS III

Alison MacKay
Weare, NH, mother of Hunter and Jack MacKay, MPS IV

Cynthia McCollum
Kansas City, MO, mother of Corey Mitchell, MPS II

Nancy Mondry
Saint Paul, MN, mother of Jaeda Robson, MPS III A

Emily Nichols
North Richland Hills, TX, mother of Austin Jay Nichols, MPS II

Danielle O'Connor
Ronkonkoma, NY, mother of Emily O'Connor, MPS III C

Eileen O'Connor
New Hyde Park, NY, grandmother of Emily O'Connor, MPS III C

Eileen O'Connor Duffy
West Hempstead, NY, aunt of Emily O'Connor, MPS III C

Daniela Samantha Padilla Cerda
Quito, Ecuador, mother of Daniela Padilla, MPS IV

Anna Phillips
Orange, CA, adult with MPS II

Scott and Lori Phillips
Orange, CA, parents of Alex and Anna Phillips, MPS II

Floreimy Rodriguez
Lawrence, MA, mother of Floreimy Rodriguez, MPS III B

Geraldine Stephens
Lebanon, MO, grandmother of Christian and Isaiah Brown, MPS III A

Sara Stutes
Ventura, CA, mother of Quinn Stutes, MPS II

Kari Tarvin
Shelby, OH, mother of Garrett, Grant and Jonathan Hickman, MPS III A

Mich and Brooke Thomas
Texarkana, AR, parents of Zachary Thomas, MPS I

David Whiteman, MD
Cambridge, MA, professional, Shire Pharmaceutical

Donations In Honor of

Cecilia Bien
Rosemarie and Jennifer Houser

Matthew Caldwell
Robert and Marjorie Austin

Blair Chapin
Linda Chapin

Jack Frye
Denise Taylor

Sabrina Gabriel
Ed Kozlowski
Laura McCann
Cindy Preuss

Zachie Haggett
Carol Sharpe

Mary and John Hoffman's 50th wedding anniversary
Anita Mancini
Charles and Catherine Viviano

Ryan Hunt
Jeff Kramer

Sue Klein-Bagdade's birthday
Ellen Krech

Jack Miller's 81st birthday and in honor of his grandson Danny Miller
Ray and Amy Miller

Lucas Montgomery
Chris Boerm

Kassi Offenbacker
Cathy Kerns

Andrew Perry
Karen Kroll

Erin Peters
Alan Dilla
Littia Royal

The Peters family
Linda Ziemnik

Tiffany Sanders
Rod Sanders

Samuel Santos
Rosemarie Galang

Luke Sarantinos
Colleen Stromatt

Sasha Segal's 12th birthday
Norman Blanchette
Linda Burtt
Barbara Clark
Alicia, Justin and Margo Dearbhail
Carole and Donald Elliott
Gary Finkle
Jill and Elmer Haley
Betty Huff
Jim, Sandy and Jon Huff
Mike and Joanne Huff
Intellivest Financial Services, LLC
Tanya Josie
Sarah Lester
Sally Luce
Gregory Markis
Matthew and Sally Mulcahy
Kristine and Jeffrey Pettee

Ethan Sowden's 5th birthday
Thomas and Elizabeth Sowden

Rev. Tim and Linda Whittington
Karen, Lindsey and Hannah Efird

Andrea Wood's birthday
Duncan and Joan McInnis

Donations In Memory of

Joey Angiolino
Laurina Savattieri

Mark Bernbaum
David Bernbaum

David Downing
Jeffrey and Elizabeth Goodwin

Eddie Ducham
Richard Jenkins
Michael and Kathryn Snyder

Sean Dummann
Steven and Jennifer Clarke
R.M. Dummann
Karen, Lindsey and Hannah Efird
Timothy and Roslynn Hanchett
Phyllis Kiehl
Susan and Larry Kirch
Brian and Kris Klenke
Carol Sue Poulsen
David and Marcia Secrest
Tom and Kim Whitecotton
Susan and Brad Wilson

Rishi Garg
Morris County Orthopedic Group, PA
St. Andrew's Philoptochos Society

Danny Gniazdowski
Lisa Albitz

Morgan Klessens
Joel Klessens

Billy Kohls
Roy Plana

Polly, Duke and Robb Murray
Jacquie and John Harrell

Terry Romo
Melanie Brannan

Hannah Salcher
Meta Mueller

Natalie Talcott
Noel and Nancy Talcott

Andrew Watkins
Laura Jachetti

Matching Gifts

Robert R. McCormick Foundation
Xcel Energy Foundation Matching Gift Program

Fundraisers

5K marathon, hosted by Michael Fisher in honor of Allison and Lacey Lukondi
Bicycle ride, hosted by Adam Williams in honor of Riley Muller
Blue Grass concert, hosted by Sharon Gillham in honor of Riley Osterhoudt
Chuck E. Cheese fundraiser, hosted by Robb and Lisa Muller in honor of Riley Muller
Courage bracelet fundraiser, hosted by Kathy Cavanaugh in honor of Allison Kirch
Dance-a-thon, hosted by Griffin Lee
Jewelry show, hosted by Robb and Lisa Muller in honor of Riley Muller
Links for Lucas golf fundraiser, hosted by Lew and Stacey Montgomery in honor of Lucas Montgomery
Purple lollipop fundraiser, hosted by Ashley Restemayer in honor of Allison Restemayer
Shots for Sean golf fundraiser, hosted by Ernie and Debbie Dummann in honor of Sean Dummann
Soccer camp fundraiser, hosted by Stephanie Bozarth in honor of Annabelle Bozarth
Softball tournament, hosted by Kristi Abel in honor of Evan Abel
Tricky Tray fundraiser, hosted by St. Andrew Greek Orthodox Church in memory of Rishi Garg

Donations

Angel's Hands Foundation, Inc.
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Julio Martin and Leticia Borges
Vince Clemens
Jason Finkle
Xiao Ling Jiang
Joan Kroll
Carol Kuhn
Douglas Macleod
Lawrence Pacl
Rhoda Pashkowitz
Thomas and Vickie Patterson
Samuel Ramsey
Mike Rohner
Mike and Barbara Smith
Jack and Barbara Sorter
Andrew Spaziani
The Inner Circle, Inc.
Maureen Williams

2010 Annual Fund Campaign Raises $57,000 by Oct. 1, 2010

The National MPS Society's Annual Fund is a valuable tool that helps to strengthen the vision, purpose and mission of the Society by committing resources to projects that provide direct benefit to members and their families. Please help us by sending your donation today.

Please accept our sincere thanks to everyone who has already donated to the 2010 Annual Fund. With your ongoing support we can continue our very important work, supporting children and adults with MPS and related diseases and their families.

Philantropist
$1000 or more

Raymond and Barbara Alpert Foundation
Mel and Millie Anhalt *in honor of Conner Anhalt*
Wayne and Catherine Bardsley
Joe and Kelley Capone
Joan Cook *in honor of Jacob and Samantha Slawson*
Eileen Gideon
Scott and Lynn Hopkins *in honor of Michelle Hopkins*
Wayne and Lori Hummel
Kevin and Tracey Litwin
Jan and Joe Melnyk
Mark and MaryEllen Pendleton *in honor of Allison Kirch*
Pool Tool, Inc.
Tracy Szemanski *in memory of Clinton and Zachary Szemanski*

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Diane and Rob Cassil *in honor of Matthew Cassil*
Stephen and Kimberly Frye *in honor of Jack Frye*
Steve and Amy Holland *in honor of Spencer, Maddie and Laynie Holland*
Mark Hopkinson *in memory of Mark Bernbaum*
Joseph McCarthy *in honor of Jack Mahony*
Larry and Mary Sweeney *in honor of Denise Dengel*
Donald and Lisa Wells *in honor of Matt Wells*
Gordon Wingate

Volunteer $250–$499

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Scott and Peggy Dionne
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Bart and Sally Finzel *in memory of Eddie Ducham*
Stephen and Betsy Fowler *in memory of Robb, Polly and Emmett Murray*
Wallis, Monica, Natalie and Nicole Hampton *in honor of Jake Hampton*
Peggy Holland *in honor of Spencer, Maddie and Laynie Holland*
John Hancock Foundation *in honor of Bryce Chesser*
John and Janet Kappel *in honor of Debbie Kappel*
Terri Klein
Brian and Kris Klenke
Lori LeDoux *in honor of Matthew Caldwell*
Sunni and Gary Markowitz *in honor of Conner Anhalt*
Maria Meconi *in honor of Denise Dengel*
Joseph Orendain *in honor of Ganesh Shrestha*
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Naureen Sayani
James and Sheila Slawson
Terra Properties, Inc. *in honor of Kraig Klenke*
Jon and Eleanor Totz
Kenneth and Barbara Velten *in honor of Steve and Bryce Chesser*
Barbara Wedehase
Stuart Swiedler and Judy Weiss
Hubert and Elaine Willman

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Chuck and Nancy Lukondi
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Kathy Ward *in honor of Bryce Chesser*
Lynn Ann Witt
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Donor $26–$99

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Robert and Marguerite Iannacone *in memory of Kristofer Arnold*
Heidi Kanealy

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Jennifer Klein
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Joanne and Bill Lennon *in memory of Martin Elliott Jr.*
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Tom and Sheila Morley *in honor of Payton Morley*
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Anthony and Pamela Paladino *in honor of Caden Mitchell*
Florence and Mario Paolilli *in honor of Austin Nace*
Elizabeth Binkley and Robert Park
Ronda Paullin
Teresa Peecook *in honor of Lynn Peecook*
Stephanie and Edmund Rabuse *in memory of Paul Adams*
Stella Reeve *in honor of Kristen Reeve*
Lindsey and Karen Rice *in memory of Christopher Migliozzi*
Shawna Robson *in honor of Jaeda Robson*
Rosemary Rotelli *in honor of Richard Rotelli*
Mark Schlafer
Judy Shafiee *in honor of Sharon Browne*
Burt and Eileen Skolnik
Robert Slowey
Donald and Rachel Swicker *in memory of Jon and Joe Karas*
Allen and Jackie Theriot *in honor of Blake and Lance Dutile*
Fern Titus *in honor of Waverly and Oliver McNeil*
Ruth and Matt Travis
James and Bonnie Tully *in memory of Paul Adams*
Rosemarie and Edmundo Veliz *in honor of Chase Veliz*
Ruth and James Watkins *in memory of Andrew Watkins*
Raymond Young *in memory of Kaylee Kacer*

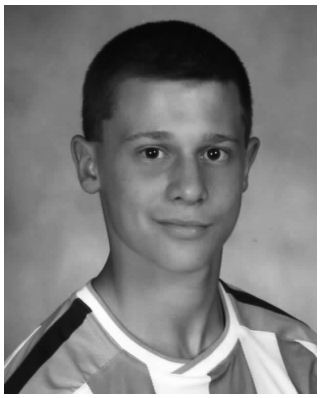
Patron $5–$25

Kim Armstrong *in honor of Bryce Chesser*
Rose Ann and Dominic Bencivenga
Todd and Nicole Blancheri *in honor of Wyatt Blancheri*
Marjorie Ditmore
Darlene and John Duall *in memory of Aaron Jackson*
Geri Edwards *in honor of Danny Miller*
Darla Elswick
Peter and E.F. Fairchild
David and Leesa Galloway *in memory of Danny Gniazdowski*
John Gladysz *in memory of Danny Gniazdowski*
Jennie Gladysz *in memory of Danny Gniazdowski*
Dennis and Christine Goggins *in memory of Sam Anthony*
Roy Hedrick *in honor of Hannah Taulton*
Marlys Herring
Janice Inman *in honor of Jim and Tim Oliger*
Janice Inman *in honor of Bill Sellmeyer*
Lisa and Chris Jones
Gloria and Irwin Katz *in honor of Jack Frye*
Charles and Sandra Kegel *in memory of Kylie and Kendall Moran*
Milliicent and Charles Kennedy *in memory of Russell and Dougie Kennedy*
Gordon and Nancy Kinley
Janet and Gary Kirch
Louise and Edward Kofron
Clint and Nikki Kremer *in honor of Ava Kremer*
Norine and Thomas Lippens *in memory of Cade Morrissey*
Edward and Suzette Ludloff
Larry Michael *in honor of Lauren Pope*
Erna Mlekusch *in memory of Paul Adams*
Merritt and Madeline Moseley *in memory of Edie Burke*
Billie Sue Nutter *in honor of Davis Barkley*
Eugene and Elizabeth O'Shea *in memory of Patrick Trainor*
Larry and Eileen O'Steen *in memory of Ryan O'Steen*
Sandra Page *in honor of Marcus Garvin*
Patti Pair *in memory of Audrey Lawson*
Shirley Pelletier *in memory of Amanda Keith*
Ambrose and Patricia Perreault *in honor of Logan Marcotte*
Floyd Reed *in memory of Evan Reed*
Donna and Thomas Schidlmeier *in memory of Jaret Schidlmeier*
Lowell and Janet Schmidt
Donald and Janet Schmidt
Shirley Smith *in honor of Kyle Lingo*
Myrna Stelman *in honor of Jack Frye*
Deborah Toga *in memory of Chad Pyper*
Jean Jacobsen and Richard Underwood
Lewis Whitaker Jr. *in honor of Logan Piefer*

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. We begin by honoring 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*.

Jeremy Mask, brother of Ryan, MPS III



For those who may not know me, my name is Jeremy Ryan Mask and I am 15 years old. I am currently in 9th grade at Pennridge High School and I live in Sellersville, PA. My favorite things to do are drawing and listening to music. This past year I have started to draw portraits of MPS kids or siblings with MPS, so if you want me to draw something for you contact me. Just hanging with my friends and family is what makes me happy, cherish every moment of it.

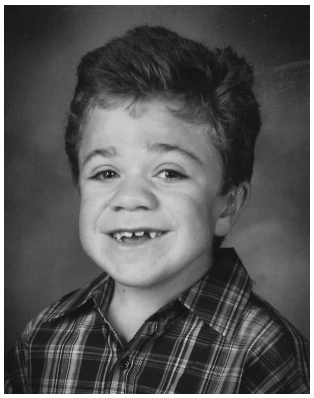
At my school I am involved with football, wrestling and baseball, and baseball outside of school.

Having an MPS sibling was always hard for me, I guess because he was older than me. When I was young I didn't recognize it, then I was a little older and started to ask questions. Now I understand MPS. I really think school has helped me understand the scientific point of view of it; MPS families have helped me understand the emotional and physical parts. MPS has opened my eyes and I am planning on doing something with MPS after high school.

Having an older brother with MPS was good for me because I could learn a lot. But my favorite thing about having an MPS sibling, specifically my brother, was whenever he would laugh. If you ever got just one laugh…you know how special one of their laughs are. Because you don't know how special it is until you lose it. My brother, Ryan Taylor Mask, passed away at the age of 17 in January 2008 and the thing I miss most is his laugh.

If you want to contact me my cell phone is 215.859.2344 or my e-mail is lilbrojmask@gmail.com.

Nicole Frye, sister of Jack, MPS II



I am 11 years old and in the 6th grade. I enjoy basketball, swimming (not competitive), baseball and soccer. Some activities I like to do outside of school are bowling, going to the movies with my family and hanging out with my friends.

My favorite thing to do is to have play dates with my friends and play with my guinea pig Caramel. The things that make me happy are helping my brother Jack when he wakes up in the morning to get dressed and helping him with his homework. My mom, dad, grandparents and playing with my friends also make me very happy.

Last year for Halloween my dad told my brother he wasn't going to dress up, but he secretly bought an Iron Man costume and didn't tell anyone. He hid it in the trunk of his car and on Halloween night he went outside and changed into it. When Jack was just about to open the door dad walked in and said, "I am Iron Man!" Jack excitedly said, "Iron Man?" and then dad took off the costume and they both started laughing.

It has been rough and challenging having a sibling with MPS, but also fun and a definite learning experience because I always learn something new. My favorite thing about my MPS sibling is that I get a lot of attention not only from him and my family but from the people around me. Thank you, Jack, for being my brother. I love you!

Our daughter Sydnee's 10-year bone marrow transplant anniversary was Sept. 20! Hard to believe it was that long ago that we spent months in Cooks Children's Hospital in Ft. Worth trying to save her life. This was a great day to celebrate her life and thank God for all that He has done. So many children are affected by the disease that it's nice to have a celebration of some kind.

We also wanted to share this letter with you that Sydnee's teacher wrote about her to read to her class. Sydnee has been having some difficulty lately with her shyness and making new friends. Other kids are really starting to see the differences in her.

God Bless and thanks for all the support and prayers over the years.

Robb, Trisha, Sydnee and Andrew Jensen

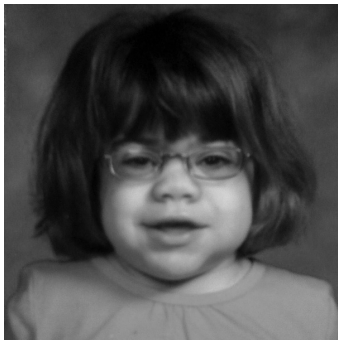
YOU ARE IMPORTANT TO US, PLEASE KEEP IN TOUCH.
Please remember to let the Society know if you are moving. In addition to helping keep printing and postage costs down, you'll help us keep our database up-to-date. Keep us informed of new addresses, telephone numbers, e-mail addresses, and any interesting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

My Friend

Boys and girls, I want to tell you a story about my friend.
I was blessed to meet my friend when I was 41 years old and my friend was 11 years old.
My friend changed my life from that first day on.
My friend has some amazing gifts.
My friend can sing the most beautiful melodies.
My friend can smile and giggle at the kindest of words.
My friend has two dogs, Miley and Taz, who give my friend lots of wet kisses on the ear.
My friend rides and cares for a horse named Powder.
My friend has a fabulous family who love my friend abundantly.
My friend has a dad who teaches math and coaches baseball at Jersey Village High School.
My friend has a mom who works for CFISD food services. She buys the food that school children eat each day.
My friend has a younger brother who plays football and keeps a protective eye on his sibling.
My friend has a grandmother who lives in Pearland and gives her extra-tender-loving care.
My friend started 5th grade without her best friend who is attending another school.
My friend is beautifully and wonderfully made just like each of you.
I hope that you make the effort to get to know my friend, Sydnee, this year.
She may look a little different, but she is a fun-loving, caring young person just like you.
Sydnee wears a listening device to help her hear better. Imagine trying to learn and not hearing your teacher and classmates clearly.
Sydnee wrote with her right hand until she could no longer grasp a pencil. She then changed to her left hand. Imagine having to do all your daily activities with your other hand.
Sydnee has Hurler's syndrome which is a genetic disorder that affects sight, language, hearing, internal organs and joint movement. Imagine having many doctor appointments and surgeries during your lifetime.
Sydnee is an awesome girl.
I ask you to get to know her, ask her a question, eat lunch with her, play at recess with her, be a friend. She has so much kindness to offer.
She may appear somewhat shy; however, all that melts away when she feels comfortable.

Livia Hubert (MPS III) on school picture day.

Sydney Vanhook (MPS I)



Autumn Cooper (MPS I)

Scotty Whitecotton (MPS II) at Halloween with his dad Tom.

Julie Thorsrud (MPS III) and mom Lauren on cover of catalog.

Michael Sheridan (MPS I) makes his weightlifting debut in Eastview High School's news program. Go to **www.district196.org/evhs/academics/flash**, select the 9/23 archive. Michael appears at the 5:45 minute mark.

On Sept. 18, 2010, Society members from the Illinois area met at the Miller Park Zoo in Bloomington, IL. Eight families had lunch, played bingo and held drawings for gifts. Thanks to everyone who donated time and gifts, and most of all to the Society for offering funds to host this event.

Brooklyn (MPS III) and Stefanie Boyce

Hardesty/Souza family

Tyler turned 6 on Sept. 13 and I entered this in my Caring Bridge journal. The pictures show Tyler's regression in just five years, which we all know speaks 1,000 more words.

Donna Kay Langan

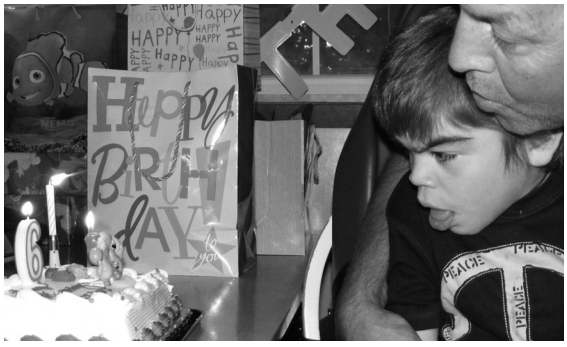
Birthdays are special, individual and unique. To me it's that one day where you can truly celebrate being you—just as you are. MPS childrens' birthdays are bittersweet. On one hand we want to celebrate such a courageous milestone for our children, but as parents of MPS children we are also reminded of the lack of time we may have left.

Birthdays with Tyler become more difficult as I try to plan a celebration and select a gift. Age appropriate toys cannot even be considered. There are no remote controlled monsters or cars, no Legos to wedge under my big toe and no silly putty to scrape off the carpet. There also is no ceremonial hand-down of the GI Joes or the Rescue Heroes that Matt so lovingly saved so one day his little brother could share the joy of pretend play. Instead, we select baby toys that ring, ding and sing as we guide Tyler's fingers toward the buttons.

MPS birthdays aren't pretend, they are as real as we are human and it's only fair to say that I struggle with this day. I wrestle with the guilt because I don't throw elaborate themed parties for my youngest son and reluctantly pay $5.99 for Elmo gift wrap that I know Tyler cannot open. I will buy a specialty cake with whipped cream icing and put illuminating candles on it. We will dim the lights while Tyler begins to panic in the dark and sing to my son who would rather eat cheerios and freeze-dried peas under the fluorescents. And when it comes time to blow out the candles and make a wish, Tyler, who can no longer blow, will swipe at the bright flaming lights on the cake with his hand trying to grab at the flames having no fear of the danger or pain he could inflict upon himself.

I feel the danger and the pain though, the danger that if I don't perform these acts, one day I will look back and realize we didn't make the most of every minute. I will continue to feel the pain of looking through the years and seeing my child's smile fade and his abilities regress from one birthday to the next. But what choice do I have? The only choice I know is to continue to move forward. Time waits for no one. As Tyler regresses and I search for new ways to cope, as painful as that is, I am learning and living. I will adjust, adapt and continue to seek alternative ways to celebrate the life I am in. I can generously give this one gift to my son. Happy birthday to my sweet Tyler. Six years ago I thought you had completed my life, but now I realize you have only made me begin to grow. I love you. Love, Mommy.

Tyler's 1st birthday



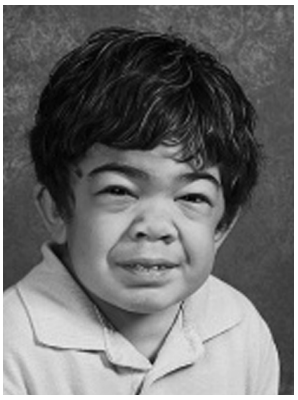
Tyler's 6th birthday

First day of school:
Emma, Julia (MPS III) and Olivia Dopheide

Following is a letter written about Ryan that highlights children with disabilities, written by his ESE teacher Mrs. Caravelli. Ryan's "success story" will be displayed in the Turlington Building which is the Florida State Education Building in our State Capitol, Tallahassee.

We are honored that others recognize Ryan for his strength, determination and good character. We are very proud of him. He also is a Florida Scholar, honor roll student, singer/songwriter, and hopefully he will be elected vice president of his 8th grade class this November. We all love him dearly!

Stephanie and Joe Duffy



Ryan Duffy (MPS II)

Ryan Duffy's Success Story

Ryan Duffy is an 8th grade student at Palm Pointe Educational Research School at Tradition in Port St. Lucie, FL. Ryan has a rare condition called Hunter's syndrome where his body does not produce an enzyme needed to break down certain molecules. The molecules build up in his joints and he has severe stiffness and immobility. However, Ryan does NOT let his condition hold him back. Ryan has a band in which he plays the drums. He produces music and participates in the school band. Even though Ryan gets intravenous medications each week, he still manages to maintain A's and B's in his classes. Ryan also is currently running for vice president of his class. Ryan is a true success story in that he does not let his condition hold him back from realizing his dreams.

Madison Nareski (MPS IV) received a Standing Ovation award for the fall issue of Courage *and is pleased to be able to share a little about herself with you now:*

My name is Madison, I also go by Madi. I am 12 years old and in the 7th grade. I live in Hagerstown, MD, located 45 minutes outside of Washington, DC. I currently am home schooled as I recover from my first surgery, but I look forward to returning to school to be part of the drama with my friends. I have many fantastic friends!

When I was younger, I performed in ballet and jazz dance recitals, and also baton twirling parades and competitions. My current hobbies include horseback riding, swimming, singing in the school chorus and theatre camp. I am extremely environmentally aware and my newest desire is to explore cooking and baking. I think about attending culinary school in the future.

During the past couple of years I was able to attend family conferences, fundraisers and clinical trials where I met other friends with MPS IV. I enjoy keeping in touch with them. I look forward to meeting many more. A future goal is to increase awareness of MPS and become very involved with many areas of the MPS Society and all the families.

My teachers love me and tell my mom they can't imagine that I will let anything physical stand in my way of what I want to achieve.

Madison Nareski

Enter the Annual EveryLife Art Contest!

RareArtist.org invites you to participate in the Second Annual EveryLife Art Contest. This contest provides a showcase for artists affected by rare diseases to express their talent and unique stories.

Two Grand Prizes will be awarded in each age group:

Children age 5–11: $100 Visa gift card

Teens age 12–17: $250 Visa gift card

Adults age 18+: $500 Visa gift card

Each Grand Prize winner also will receive a flip video camera, which can be used to record their story about being an artist affected by a rare disease. Artists' video stories will be posted on **www.RareArtist.org**.

The EveryLife Art Competition is open to everyone affected by a rare disease age 5 and older. Artists may have a rare or undiagnosed disease or may be close friends, family members or caretakers of those with a rare or undiagnosed disease. Work must be accompanied by a brief statement by the artist (250 words or less) offering insight into the work and their experience as an artist affected by a rare disease. For children 11 and under, parents or guardians may submit the statement.

Limit of three pieces of art may be submitted per person, and must be original artwork created exclusively by the entrant. Maximum height of work is 48 inches, maximum width is 72 inches and maximum weight, including framing, is 50 lbs. Two-dimensional artwork in the following media will be considered: paintings in oil, acrylic, watercolor, gouache, encaustic; drawings in pencil, pastels, pen and ink, etc.; traditional and digital photography and; printmaking, etchings, mixed media and digitally created works.

Entries must be received by 5:00 p.m. PST on Friday, Jan. 7, 2011. Entrants are encouraged to submit images of their artwork via **www.RareArtist.org**. Entries also will be accepted by e-mail (info@rareartist.org) and by regular mail at:

Kakkis EveryLife Foundation
77 Digital Drive • Suite 210 • Novato, CA 94949

The Kakkis EveryLife Foundation's board of directors will select the winning entries. Grand prize works of art will be displayed in the Kakkis EveryLife Foundation's collection for up to one year.

For more information and to download an application, visit **www.RareArtist.org/contest**.

24th Annual National MPS Society Family Conference

On Sept. 30, 2010, more than 200 people gathered at Knott's Berry Farm in Buena Park, CA, for the start of the 24th Annual Family Conference with dinner and syndrome breakout sessions. Earlier that day 12 adults with MPS and related diseases held their inaugural conference, Finding Our SPIRIT (**S**trength, **P**urpose, **I**ndependence, **R**esilience and **I**nitiative **T**ogether), led by Nicole Shannon. This was a closed conference, to allow the participants the freedom and confidentiality to discuss such issues as life goals and independence.

"Nicole Shannon was fun and had a lot of information about setting goals for oneself," said Brian Madeux (MPS II). "From my experience with past conferences, the MPS Society has focused solely on children and parents. This is the first ever adult conference – long overdue! The Medieval Times dinner show was a great group activity. The location was very convenient, and we appreciate the Society covering our hotel accommodations – it was very helpful to us."

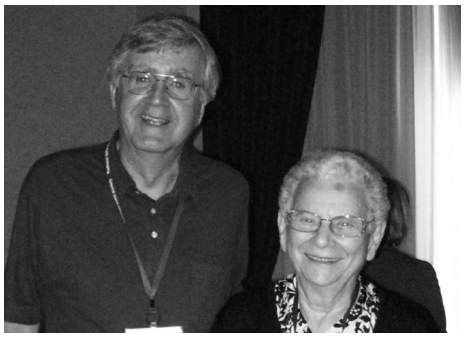
The third CYCLE (Celebrating Your Child's Life Experience) conference also was held on Sept. 30, attended by 11 parents. Speakers presented on topics of sane parenting, finding meaning, redefining the family unit and making a memory stepping stone. The day ended with a remembrance ceremony and butterfly release.

"The family continues to change after a death. Time for these changes varies and evolution takes time."

Dr. Lorne Clarke from British Columbia Children's Hospital in Vancouver, Canada, opened the educational sessions Friday, Oct. 1, with a clear overview of the disease pathophysiology of MPS and related diseases, followed by Dr. Joseph Muenzer from UNC in Chapel Hill, NC, reviewing the management of these diseases. Dr. Klane White, an orthopedic surgeon at Seattle Children's Hospital and Society board member, reviewed orthopedic issues and surgical interventions. Ending the morning were the fundraising and legislative talks, where the Madsens inspired everyone with their video about hosting an MPS Cup in Minnesota. In addition, Tami Slawson shared her 10 years coordinating the LA 5k Walk/Run. Jennifer Restemayer enthralled the group talking about her legislative advocacy efforts locally, state-wide and nationally, showing photos of her with President Obama during her recent trip to the White House.

CYCLE conference attendees

Before families walked next door to enjoy an afternoon with Snoopy and friends at Knott's Berry Farm Park, Steve Holland spoke about remembrance. "By definition, a remembrance ceremony means we have lost something—something that needs to be remembered," said Steve. "For some of us we have lost a precious child to this terrible disease. Several of us who have lost a child were fortunate to come together yesterday in a similar manner and participate in a butterfly release to remember our children. Others here have lost friends to MPS and are coming together to remember those many MPS faces and those earthly friendships that are now missing. Still others have come together to remember that loss of innocence you once had—the innocence of not knowing that your child had MPS and the loss of dreams that you once held for your child: graduation from high school, driving a car, first kiss, or marriage and grandkids."



Drs. Muenzer and Neufeld

Saturday morning Dr. Kendra Bjoraker from Children's Hospital Denver informed parents about navigating the

continued

educational system and individual education plans (see the Special Education fact sheet in the library section of our Web site), followed by Dr. Joan Keutzer from Genzyme talking about newborn screening for MPS diseases. After 20 years of research, MPS assays for newborn screening are being tested in pilot programs, beginning with MPS I in Washington. The following updates on clinical trials were presented and can be viewed on our Web site:

- Dr. Elizabeth Neufeld: *Sanfilippo Syndrome: What Happens in the Brain?*
- Dr. Celeste Decker: *MPS IV Clinical Trial*
- Dr. Joseph Muenzer: *MPS II Intrathecal Clinical Trial*
- Dr. Patrick Haslett: *Intrathecal ERT for MPS III A*
- Dr. Elsa Shapio: *Overview of the Lysosomal Disease Network*
  Longitudinal Studies of Brain Structure and Function in MPS
  Characterizing the Neurobehavioral Phenotype in MPS III A
- Dr. Sara Cathey: *Longitudinal Studies of Mucolipidoses II and III*
- Dr. Patricia Dickson: *Intrathecal ERT for Cognition in MPS I*

Drs. Chris and Leslie Miller gathered all the siblings together in the afternoon for a two-hour session, divided between younger and older children. Through sharing of stories and experiences, siblings realized they aren't alone and gained a renewed sense of the gifts their brothers and sisters offer them.

Quinn Stutes (MPS II)

The afternoon concurrent sessions offered attendees opportunities to deal with persistent stress with Dr. Linda Bortell. Dr. Kimberly Frye helped parents work through the multitude of variables associated with making thoughtful and collaborative treatment decisions, while Dr. Elana Evan shared the benefits of palliative care. Living with loss is not just the loss of a child, but the multiple losses parents face from diagnosis on, as presented by Dr. Robyn Westbrook.

Tami Slawson arranged for her annual 5k walk/run to be held at Knott's Berry Farm Park Sunday morning, so all the families attending the conference could be a part of this amazing experience. With more than 800 participants, it was an inspirational ending for an inspiring conference—seeing so many people supporting research for our diseases.

The following people were recognized at the Awards Banquet:

10-Year Walk/Run:
Tami Slawson

Outstanding Member Awards:
Kimberly and Stephen Frye
Angela Guajardo

Director's Awards:
Dave and Hope Madsen
Cynthia Miller

President's Awards:
Kimberly Frye
Steve Holland
Kris Klenke

Friendraising Award:
Shane and Jenifer Gibson
Chelsea Montgomery

Crystal Diamond Award:
Ernie Dummann

SPIRIT Conference: attendees' perspective

I was excited to find out about the first adult conference, SPIRIT, hosted by the National MPS Society and sponsored by the pharmaceutical companies. The conference was attended by adults 18 and older who are affected by MPS or ML. Though the conference was during my college curriculum, I decided it was important for me to attend so that I could begin to share my concerns for an independent life and how I could achieve this. I also was very excited to meet other affected adults around the country and hear their stories.

SPIRIT conference attendees

continued

The speaker, Nicole Shannon, was great at gathering all of our information on life goals prior to the day of conference so we could discuss these openly throughout our discussion periods. I learned that many of us are at different points in our life, but each of us is learning daily how to go after our goals with strength and determination. It may take us a bit longer, we might need to face pain on a daily basis, but we all want to strive and move forward with set goals.

I enjoyed speaking about real issues and sympathized with others as we expressed our opinions and felt raw emotions over difficult subjects. It was an extraordinary opportunity to meet others face to face and build a new connection.

I really enjoyed our evening out together at the Medieval Times dinner. This was a great way to relax the group before we met all day at a conference. The atmosphere ironically was fun and challenging!

I hope the National MPS Society will continue to host adult conferences in the future; there were so many topics that need to be explored further. One of my suggestions for future conferences includes creating group classroom tables so we can work together more. It also seemed that a major concern among the adults focused more around the social aspect of life rather than the medical. It was good to discuss medical issues but I feel the adult conference would be more beneficial if we talked about our social problems as well. For example, how to interact and share our stories with people who are not affected by MPS/ML. We discussed our hopes and dreams; I would like to see a follow up conference that will show us how to take steps and make those dreams come true!

Families enjoying an afternoon at Knott's Berry Farm.

Thank you National MPS Society. My parents and family have benefited from conferences over the years to help treat my disease. This year was the first time I felt strong and walked away with new friendships and tools that will help me in the future. I am looking forward to the next invite!

Jenny Klein (ML III)


Bailey and Noah (MPS II) Woodcock

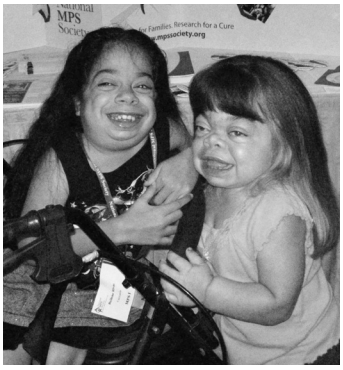
Snoopy and LA Walk/Run participant


Amy and Maddie (MPS I) Holland

As an adult with MPS I who has been diagnosed for just six years now, I always appreciate the chance to attend conferences and meetings about MPS to learn about potential new research for my disorder, but also so that I can take this information and use it to continue educating my specialists who represent a very diverse set of medical problems, as most of the specialists I see either have very few other lysosomal patients or I am their very first. I try to learn from younger families who have had a diagnosis much longer than I have and I try to question the MPS specialists who are giving talks at conferences as there is not always a lot of information available to us adults on what we can do for ourselves to empower our care and to best advocate for ourselves. I had the pleasure to attend the inaugural adult SPIRIT conference with 10 other adults with MPS and ML, ranging from very young adults to individuals in their late 30s. This conference provided all involved a chance to work together, share ideas and brainstorm on how to continue to help ourselves and others affected with MPS and ML, while also having a life not defined just by the disorder and not losing sight of the simple fact that we have MPS disorders but MPS does not have us and does not have to define our lives. The idea was discussed that adults with these disorders can be anything they want to be in life; sometimes this just requires taking a slightly different path to get there and that sometimes because of the difficulties these disorders present (medical, time, insurance, etc.) we give up one dream but find another in a different but similar field which provides an equal passion. My goal here on out with the MPS Society is to continue to reach out to other adults with these disorders and find ways to help affected adults feel like they are more involved and thus want be more involved. As adults with these disorders we are the voices of tomorrow's MPS generation. I encourage affected adults to share their ideas and make your voice heard to help pave a better way for tomorrow's kids. Thank you for the chance to attend!

Erica Thiel (MPS I)

Team Brown participants at the LA Walk/Run.



Reilene Rivas (MPS I) and
Allison Restemayer (MPS I)

Marcie and I had a nice time at Knott's Berry Farm, the conference, meeting new families, reuniting with old friends, etc. I thought the speaker for the adult conference, Nicole Shannon, was fun and had a lot of information about setting goals for oneself. I thought there should have been more time spent on everyday life issues like marriage, having children, careers, suicide, death, etc.

I would also recommend for future adult conferences that a representative from the Social Security Administration (not just a social worker) be included to talk about benefits, entitlement, rights, etc.

It would be nice to discuss together how many and what surgeries we all have had, how to deal with pain management, etc. It would be inspiring for a famous disabled celebrity or Olympian to discuss how to deal with challenges.

Other issues I would like to learn more about are how to speak to old and new families about their children with MPS. For example, how much to say to them about ERT, BMT, surgeries, treatment options, school, etc. It would also be beneficial for family members to participate in a portion of the adult conference, to discuss their caregiver, spouse/partner/parent issues, needs, questions, etc.

From my experiences with past conferences, the MPS Society has focused solely on children and parents. This is the first ever adult conference—long overdue!

I thought the Medieval Times dinner show was a great group activity. We really appreciated the hard work Barbara, Laurie and others did to make this conference happen. The location was very convenient. We also appreciated the Society covering our hotel accommodations—it was very helpful to us. We are always open and available to talk or e-mail any of our MPS family. You can also find us on Facebook. Thanks for a great conference; we look forward to the next one!

Brian Madeux and Marci Humphrey

Rachel Adams (MPS III)

Sergio Torres (MPS II)

John Dalton (MPS III)

Sponsor a Child for a Cure

Fall 2010 went forth with a burst and the 2nd annual Sponsor a Child for a Cure program exceeded our expectations! Five runs participated:

- BioMarin "Run for Your Life 5K," *Kathy Ward, race coordinator*
- Mackenzie's 5K for MPS, *Steven and Jennifer Clarke, race coordinators*
- National MPS Society Walk/Run LA, *Tami Slawson, race coordinator*
- Miles for MPS, *Laurel Radius, race coordinator*
- 11th Annual Run for Erin, *Stacy Peters, race coordinator*

Thirty families signed up to be included in these events and raised thousands of dollars for research. After the events were finished, families were sent a letter, photograph and medallion of courage in honor or in memory of their loved one affected by MPS or related disease.

Our goal is to have families participate from every state. It is an opportunity to reach out to members who wish they could do something more that contributes to awareness and research but are not able to attend an event themselves.

There is a way your family can help and provide hope for the future. Contact Terri Klein at terri@mpssociety.org for details on 2011 Sponsor a Child for a Cure.

Amy West, a student in Poquoson, VA, finished the race with a time of 26:10 and ran for Taylor Geary (MPS III) of Hilliard, OH. Amy participated in the Mackenzie 5K Run for MPS.

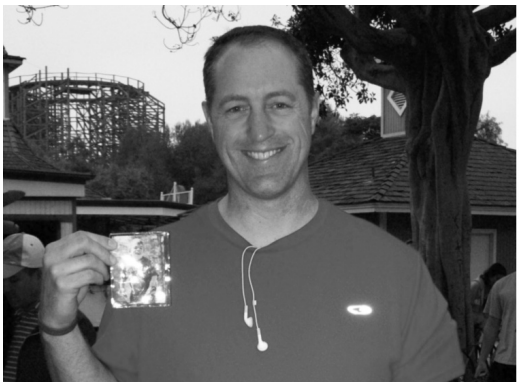
"Thank you for this new program! How precious to receive a picture of Amy West from Virginia."

Rachel Wojnarowski, mom to Taylor Geary (MPS III), who participated in the 11th Annual Run for Erin

Runners gathered from across the Los Angeles region at Knott's Berry Farms to participate in Sponsor A Child for A Cure at the 10th Annual Walk/Run LA.

"The Sponsor a Child for a Cure program lets John's presence be felt at runs when he cannot be there in person. There is power in numbers; the more people see those afflicted with MPS the more powerful the message is that we need help to find a cure. A runner wearing a picture of John is the next best thing to John being there in person."

Sheila Thornton, mom to John (MPS III), who participated in the 10th National MPS Society Walk/Run LA



Klane White, MD, runs for Peter Wehrle (MPS II) of Issaquah, WA. Dr. White finished in 25th place overall with a finish time of 24:39 at the 10th Anniversary of the National MPS Society Walk/Run LA at Knott's Berry Farms.

"Russell and Dougie Kennedy were sponsored at the National MPS Society Walk/Run at Knott's Berry Farms. Dougie was 11 when he died in 1990; Russell was 13 and died in 1991. They both had MPS II. My son, Chuck Kennedy, their father, would like to walk for them and has already raised $2,000."

Millicent Kennedy, grandmother of Russell and Dougie Kennedy

It's amazing who you meet. While attendance to Buena Park was not possible for my family, we made sure to take full advantage of the fund-raising opportunity. And unexpectedly we made a friend.

I took this opportunity to participate in the Sponsor a Child for a Cure program by posting fliers at work, where I received an overwhelming response to the donation request. Not only did this flyer help educate those who were not aware of my son's condition, it helped me speak openly about our situation and MPS in general. This was quite a relief as since December 2009, when Owen was diagnosed with MPS II, I only shared our situation with a select few individuals. I wanted to scream out loud about Owen, but until the fundraiser I never had the appropriate venue.

Here's what really blew me away: A gracious gentleman by the name of Scott Mangini of Benicia, CA, took the flyer home to share with his family. His oldest son, Tony, said, "Dad, I would like to do something to help." Tony, as it turns out, happens to be a very successful semi-pro Legends car racer. Tony decided to raise awareness for MPS. He asked if we minded if he put Owen's picture on his race car, along with the MPS Society's logo. The MPS Society gave their blessing. Owen loved the idea. Tony easily could have had a paying sponsor taking up this precious space on his car. During the last few months Tony has received many questions about MPS and he is doing all he can to educate people.

Tony will continue his mission to raise awareness for MPS next season. If you happen to live in Northern California, please come out and show your support for Mangini Racing.

Not only did the Buena Park event raise money for research, but it helped my family open up about our situation. And, most strikingly, the MPS Society and our family gained an advocate and friend in Tony.

Jason McKee, father of Owen (MPS II)

Diedra Walker of Grand Rapids, MI, walked in the Miles for MPS Run in Grand Rapids, MI, in memory of Cade Morrissey (MPS I).

Tom Lester ran the BioMarin 5K in a finish time of 19:12. Tom ran for Rachel Adams (MPS III) of Napa, CA.

Bobby Singer of Woodstock, GA, ran in honor of Nathan Bivens (MPS II) of Banner Elk, NC, at the 11th Annual Run for Erin. Bobby ran with a finish time of 23:50.

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
- Publish everyone and all the money raised in an upcoming *Courage* magazine

The assigned runners are inspired by our heroes of MPS. Together they pave the path of continued hope.

For more information on the Sponsor a Child for a Cure program, contact National MPS Society Development Director Terri Klein at 919.806.0101 or terri@mpssociety.org.

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—Casual Dress for MPS, the Annual 5K Walk/Run and the Annual Fund are great ways to raise money for the National MPS Society.

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 #0845
- 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

Bryn Chesser, sister of Bryce (MPS II), and Renee Congdon have formed the B&R Baking Company and sold their homemade cookies to raise funds for the MPS Society. They collected $75.

A friend of the Muller family, Adam Williams, road races with a bicycle team every year. This year he decided to donate his winnings to the National MPS Society in Riley Muller's (MPS II) name. Adam wrapped the handles of his bike with purple tape to show his support and raised awareness with his teammates. Next year he hopes to get the whole team to race for MPS awareness.

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:

Adam Podesky, MPS I

Tucker Lanier, MPS II

Tucker is 9 years old and the most active little boy you will ever meet. He lives in Statesboro, GA, with his little brother Dylan, his stepfather Jason, and his mommy Casey. Tucker is in the 4th grade. He loves to go on field trips; the bus ride is his favorite part!

Tucker loves to play sports outside with his family. He really enjoys playing baseball, basketball and soccer. Usually he ends up kicking all the balls. He runs all over the place, and climbs on to things he shouldn't. He is hard to keep up with! Tucker loves to go to the playground and slide and kick sand at all of the other kids.

At home, Tucker enjoys watching Dora, Blue's Clues, Barney and The Fairly Odd Parents. If he's not watching TV, he is usually kicking the couch, the door, or one of us! We definitely have to Tucker-proof the house!

Tucker is a very happy little boy. He laughs a lot, smiles a lot, talks a lot and sings the cutest little songs. His favorite is "Sun, Sun, Mr. Golden Sun, Please Shine Down on Me," but in Tucker's words, "Sun, Sun, Down on me." He keeps a smile on everyone's face who has the pleasure to meet him.

Thank you to the MPS Society for this award. It means a lot to our family.

Luke Sarantinos, MPS III B

Our family was exceptionally blessed when Luke was born in January 2000. Luke is the third of three beautiful boys. They are the best brothers and the best of friends! There is no doubt that Luke brings the joy and laughter to our home and is the glue that keeps our family moving in the same direction.

Now, almost 11 years old, Luke wakes up happy every day with smiles and hugs for all of us. He attends Wedgwood Elementary where he enjoys spending time with his friends and teachers. Luke's favorite activities at school include calendar time (he will recite the days of the week and months of the year to anyone willing to listen), computer games, playing basketball at recess, and riding the bus home with his buddies. At home, Luke likes to take walks with his dog, Kefi, play on the computer, ride his scooter to the park, have a fire in the backyard fireplace, and watch his favorite shows—Dora, Special Agent Oso, Mickey Mouse, Diego and Blue's Clues. He has memorized every song from every show and isn't afraid to belt out a tune at any given moment. Luke loves family dinners and says the prayer before every meal. Luke makes sure that we make it to church every Sunday, where he loves to show off the tie he is wearing.

Luke is a "people person" who loves to be out amongst the public. He greets everyone who passes by with "hello friends" or "this is Athena" and fully expects everyone, including complete strangers, to shake our hands or give us a "big hug"! Luke loves all his cousins, aunts, uncles and especially his grandparents. If he understood the concept of a family compound, that is where he would want us all to live. His favorite activity of all time is having dinner in a restaurant. He loves his rice, beans, chips and salsa. Salt and pepper, a candle and flowers on the table are a must. Luke is never one to miss a party and looks forward to each and every holiday. He lets every family member know exactly what costumes we will wear for Halloween and insists we be among the first on the block to have our Christmas lights up.

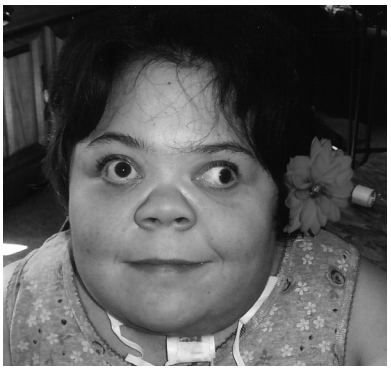
Our family is extremely grateful for Luke's continued health. We don't take one day for granted. Luke's greatest gift is the perspective his life has brought to our family and friends. His presence reminds us that it is easy and good to laugh, sing and be kind to people every day.

Jake Kreul, MPS IV A

Aloha, my name is Jake and I am 10 years old. I was born in Hawaii and have lived here my entire life. I like playing video games on Wii and Nintendo DS (and "old school" Gamecube). I also have a Web site, **www.Jakekreul.com**. My favorite class in school is computers. When I grow up I want to be a scientist or a spy. Seeing my friends makes me happy. Sometimes I play miniature golf with my dad and the last time we played I got four hole-in-ones.

Thank you for giving me this award.

Tempra Hauskens, MPS VI



Tempra is our social butterfly, shining light, jokester and strength all in one. She is 13 years old and in the 7th grade. She would rather spend her days talking and visiting with everyone around her, or completely engrossed in a good book, than doing anything else. She loves to make people laugh and if she can play a joke on a good friend or family member, she's all for it. Her compassion for everyone around her puts her in the hearts of the whole community. Her heart and soul are the biggest parts of Tempra.

She believes in hard work and attention to details, to do the best job she can. She shows us all how to live life to the fullest and how every moment should be enjoyed. If she comes upon a roadblock in her life, she turns it around and uses it as a stepping stone, rather than a stumbling block. She doesn't know the meaning of defeat and when she sets her mind to something, that's just the way it's going to be. Tempra is very assertive and doesn't let people tell her she can't do something because of MPS.

Tempra is very involved in many aspects of school, community and family life. She leads the school band in drums and is a "must have" team member if there is a fundraiser. She is forever challenging her teachers to keep up and find new ways to challenge her. She has a booth at the local craft fair and loves to meet and visit with everyone who stops by. Her winter months are spent working on weaving, beading and soap making, just so she can get out in the public in the summer. She loves to raise money for the local food shelf or any other project the community may be doing. She has even started volunteering several hours a week at our local library.

The things Tempra enjoys most are singing, whistling like a bird, playing with babies (two- or four-legged), crafts, baking, writing silly stories and making funny faces! Everyone needs a little brightness and laughter in their life and Tempra sure can bring it!

Andre Andrews, ML

Andre is 16 years old and lives in Washington, DC, with his mother, Jane Andrews. He was diagnosed with ML II in December 1997. Andre is in 10th grade at Phelps Architecture, Construction and Engineering Career High School with aspirations of being an architect so he can build homes for people with disabilities. He has always been a math scholar. As part of a Leap Award this year he received a new laptop computer with Skype and he spends a great deal of time using it. At school, Andre maintains a 3.95 GPA, has been a consistent honor roll student and his parents are extremely proud of him. His aspirations are to maintain a 4.0. He participates with the school robotics team.

Andre is an active member of Shiloh Baptist Church. He attends the Jonathan Sunday School class which is geared for children with special needs, and is a church usher.

Andre participates in a local Saturday bowling league, and travels throughout Maryland and Virginia to compete in tournament play for scholarships.

Andre has participated in various events for children with special needs, like the Starlight Foundation, Dreams for Kids and Kids Enjoy Exercise Now. This year he attended many Nationals baseball games, shows and other holiday activities. He enjoys playing video games and has a Facebook page.

Andre doesn't complain much and realizes that it's others who need to be able to overcome the types of challenges he has in spite of his disability. He gives unselfishly and has a heart of gold.

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 **Nichols family** from North Richland Hill, TX.

The story of this family begins with the birth of my youngest brother. Born Sept. 28, 1989, Evan Jay Ludiker was a beautiful little boy. He always had this unique smile. But my mother felt something wasn't right. Having already had a daughter and son, she knew something was very different with Evan. My mother claims it was a gut instinct. I understand that feeling. She searched for answers to various issues. Her search ended with the words "Your son has mucoploysaccharidoses." MPS II to be exact. I was a very small child when my brother received the diagnosis. But I do remember that it was so rare, it was completely unknown. He received the diagnosis in the early 90s.

My brother lived a perfect life. Well, as perfect as you can have under the circumstances. I never had much to do with the doctors. My memories are of him watching Walt Disney movies, going to school on the school bus every morning, except for when he would stay home for mommy/Evan time. He was always jumping on the bed. My memories are of good times that siblings share with each other. Yes, we had our fights, and let me tell you, he won every time! He was a true angel. The love I felt for him is more than words can describe. It wasn't until I was an adult did I realize this.

Evan Jay passed away peacefully in March of 1997. He fought for weeks to stay with us, but it was time to let go. Leaving every person he touched with a sense of pride for knowing, loving him, touching him. Shortly after he passed, I just went on as if he had not been there. I was only 12 when he left me. The family as a whole dealt with his death in a different way. I would though, when I was alone, think about his smile.

I grew up, left my parent's home, and eventually started a family of my own. I had my daughter, Samantha Michelle Nichols, in December 2003. I thought that would pretty much be it for me. Just Sammy. A few months into 2008, I found out I was pregnant again. I did everything the same as I did when I was pregnant with Samantha. I saw a geneticist, always went to the checkups, did everything right. We found out it was a boy and that put a little more need for the geneticist. I didn't really think about it during my pregnancy. I mean, what would be the odds of having another "Evan Jay"? I had testing done and it showed I was not a carrier for most genetic disorders, the only thing not tested, for whatever reason, was MPS.

Evan Jay

In December we had a beautiful little guy come into this world, Austin Jay Nichols, who was named after his uncle Evan Jay. He was the most beautiful little baby I have ever seen, except for Samantha. There were issues right away, though. He had a traumatic birth, due to his broad shoulders. Then he had a hard time passing his hearing test. I figured every birth was different. But deep down inside my heart I believe I was blocking out that there could be a chance he had MPS. As an adult, having babies, going to geneticists, I had done plenty of research on MPS. I also had the greatest resource you could have, my mom. Austin grew as the months went on. I eventually assumed his birth was just that, difficult. Austin was right on developing, crawling, then walking. He was as happy as could be. Sure, he didn't sleep, he ate tons and had

Austin Jay

continued

constipation issues, but every child is different. He smiled all the time. A smile no one could match. My gut was always telling me something. I just ignored it.

Then came the haircut. He had the thickest hair I had ever seen on a baby. He already needed a haircut at six months! Shortly after the haircut I put him to bed. Being the only one awake in the house, I realized that he looked just like his uncle Evan. But, how can that be? No one looked like my brother. After looking at Austin's hair the next day, my mother asked me if I knew what needed to be done. Of course I knew. That brings us to today.

Austin Jay received his diagnosis in October 2009. It has been a roller coaster of emotions to say the least. Knowing what the future holds, trying to make it through the day. It still has not completely hit me. I try not to let it hit me. I remember the night we got his diagnosis, my mother said, "Be sad today, cry today, get it out of your system, because tomorrow we fight." I think about those words at least once a day. That is what we do, we fight. God has graced me with the honor of taking care of the most handsome little man there could be. I am honored, I am proud, and I would not change what my mission in life is. As I write these words, yes I have tears, but I know when I am done, I am going to sit down with Austin, watch a little Sponge Bob, eat a snack or five, and hold the most amazing gift from God.

Emily Riannon Nichols

Upcoming Events

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 will be available in February 2011.

Samuel Anthony
19, ML II, 9/11/10

Anthony Bulkhak
5, MPS II, 11/7/10

Joshua Burt
11, MPS II, 9/26/10

Devon Clark
17, MPS II, 9/4/10

Kraig Klenke
18, MPS II, 10/26/10

Mark Lessing Jr.
20, MPS III, 11/1/10

Kraig Klenke, MPS II

While driving one day a few weeks ago I found myself deep in thought. On occasion my mind goes to a place where I begin to wonder about the future. We work hard to live most days in the present but sometimes you have to go "there" because in part it is natural and in part it is healthy to prepare. We try to make our visits brief, but on this little trip I began to think about a time when our boy would no longer be able to tell us what he is thinking. During that trip to the future I began to imagine or hope what it was he might want to be able to say to us. About half way into this imaginary conversation I found myself on the freeway in tears and perhaps this is another reason why I make my trips to the future brief. Los Angeles freeways are dangerous enough without an MPS mother driving around, lost in the future, in tears on the road. I forced myself back into the reality of the present and continued to work to find a way to assist my patients in their work of recovery, support and healing.

A week later I received a call with the news about Kraig Klenke. I returned to my imaginary future to continue my conversation that I had since realized was a dialogue of hope and assurance to any parent who wonders the very same thing I will wonder, but this conversation was not for me, at least not today. It was, however, for me to share with my dear friends the Klenke's. This is what I shared with them at Kraiggy's service.

Kim Frye, mom of Jack (MPS II)

If you could hear what I am thinking…

you would hear me say I love you every morning and every night.

you would hear me say I always knew what you were doing for me and what you were not doing for you.

you would hear me say I always knew that you were there for me.

you would hear me say I know you always did all you could and even then you did a little more.

you would hear me say I know you left no stone unturned as you searched for answers, for relief, for fairness, for a cure.

you would hear me say how much I was in awe of your drive, your energy, and your unwillingness to give up.

you would hear me say how desperately I wanted my body, my feet, my skin to be the way it was designed to be so you could rest and we could just be.

you would hear me say how loved and important I felt to you and how good you always made me feel even when others did not.

you would hear me say how much it meant to me that regardless of the pain you felt at times, your love for me kept you going.

you would hear how much I love you and how lucky I feel to have you as a mother, a father, a sister, grandparent, cousin and friend.

you would hear me laughing out loud with you when you tell another story about poop.

you would hear me say there is no way I could thank you enough for what you have given to me.

you would hear me say, we won, we will walk together in June and always.

you would hear me say over and over how much I love you and how I will always be with you watching and smiling.

Mid-Term Election Results 2010

November's mid-term elections are said to bring many changes in the nation's capital. Congress returned on Nov. 15, 2010, for what is known as a lame-duck session, lasting just a few weeks. (A "lame-duck session" is one that occurs after an election, but before the new Congress is sworn in.) Many of the substantive legislation, such as funding for government agencies, expiring tax cuts, stem cells and other important issues are being taken up in 2011.

Further delay of final congressional decisions on government funding could be a mixed bag at best for research. It is possible that Congress could decide to fund the entire Fiscal Year 2011 through a "continuing resolution" at 2010 levels. Unfortunately, that would mean the National Institutes of Health (NIH) would not receive its proposed $1 billion increase and the new Cures Acceleration Network would not get its planned $50 million to get the program started.

In regards to the appropriations committees, both the House and Senate will need to appoint several new members in the coming weeks. Sen. Tom Harkin (D-IA), a friend of NIH research, will remain as chairman of the Senate Appropriations Subcommittee.

With Republicans taking control of the House, one of the Rare Disease Caucus co-chairs, Rep. Fred Upton (R-MI), is in line to become chairman of the House Energy and Commerce Committee, which has jurisdiction over a great deal of health legislation. Sen. Harkin will remain as chairman of the Senate Health, Education, Labor & Pensions Committee, which handles health issues in the Senate.

We will be saying goodbye to Sen. Arlen Specter (D-PA) who lost in the May 2010 election primary from Pennsylvania. Sen. Specter, his Legislative Health Liaison Regina Campbell, and his staff will be missed. We look forward to meeting newly elected Sen. Pat Toomey (R-PA) in 2011.

Alabama	Shelby	R	Iowa	Grassley	R	Ohio	Portman	R
Alaska	Murkowski	R	Kansas	Moran	R	Oklahoma	Coburn	R
Arizona	McCain	R	Kentucky	Paul	R	Oregon	Wyden	D
Arkansas	Boozman	R	Louisiana	Vitter	R	Pennsylvania	Toomey	R
California	Boxer	D	Maryland	Mikulski	D	South Carolina	DeMint	R
Colorado	Bennet	D	Missouri	Blunt	R	South Dakota	Thune	R
Connecticut	Blumenthal	D	Nevada	Reid	D	Utah	Lee	R
Delaware	Coons	D	New Hampshire	Ayotte	R	Vermont	Leahy	D
Florida	Rubio	R	New York	Schumer	D	Washington	Murray	D
Georgia	Isakson	R	New York *Special Election*	Gillibrand	D	West Virginia	Manchin	D
Hawaii	Inouye	D	North Carolina	Burr	R	Wisconsin	Johnson	R
Idaho	Crapo	R	North Dakota	Hoeven	R			
Illinois	Kirk	R						
Indiana	Coats	R						

President Obama Signs Improving Access to Clinical Trials Act

On Oct. 5, President Obama signed legislation to enable people with rare diseases to participate in clinical trials without losing eligibility for public healthcare benefits. The Improving Access to Clinical Trials Act had passed the Senate on Aug. 5 and the House on Sept. 23.

Rare Disease Investigators Training Course Draws More Than 100 Participants

National Organization for Rare Disorders (NORD) co-sponsored a successful three-day course for rare disease investigators Oct. 18–20 in Rockville, MD. More than 100 researchers attended. The course was organized by the U.S. Food and Drug Administration (FDA) Center for Drug Evaluation and Research, the NIH Office of Rare Diseases Research, the NIH National Institute for Neurological Disorders and Stroke, and Duke University Medical Center.

Janet Woodcock, MD, director of the Center for Drug Evaluation and Research (CDER), opened the conference by stating the FDA's commitment to advancing orphan products through the regulatory system. The same commitment to orphan product development was voiced by other FDA and NIH speakers, including Christopher Austin, MD, director of the Clinical Genomics Center at NIH and NIH Therapeutics for Rare and Neglected Diseases.

NORD President Peter L. Saltonstall said that NORD is seeking ways to replicate the course next year. He particularly thanked Anne Pariser, MD, associate director for Rare Diseases, CDER, for her leadership in organizing the course.

Do We Need A War on Rare Diseases?

Is it time to declare a national "war on rare diseases," comparable to the famous "war on cancer" that began in the 1970s?

The Institute of Medicine report released in October stops short of recommending a full martial assault, but it does call for the creation of an aggressive national strategy to accelerate drug development. Policymakers, Congress, the NIH, FDA and patient advocates are all taking a hard look at how to improve research and development for rare diseases, which affect about 25 million people a year.

A number of innovative approaches are already being tried, including a program at NIH to get more involved in the development of drugs for rare diseases and an effort by the FDA to encourage orphan drug filings. The report lauds these new approaches but says that the sheer number of rare diseases, the different priorities of various researchers and advocacy groups and limited available resources require a national, integrated strategy.

One key recommendation is national task force, set up by the head of U.S. Department of Health and Human Services, to make sure NIH, FDA and patient-advocate efforts are better coordinated and monitored.

Other suggestions include setting up a repository of publicly available animal models for rare disorders and another public repository of biological data on rare diseases. Both could potentially be used by patient groups as well as investigators.

Thomas Boat, chairman of the Institute of Medicine, who wrote the report, tells the Wall Street Journal Health Blog that up until now, rare disease care and research has been disorder-specific, so "efforts have really been segmented." Boat says the recommendations are designed to increase cross-fertilization of ideas and sharing of resources such as repositories and specimens. He adds that committee members briefed NIH and FDA officials on the findings and also hope to talk to legislators.

Timothy Coté, director of the FDA's orphan products development office, says that a congressionally established committee he chairs is assessing the new report. Its recommendations for how FDA ought to tackle rare diseases are due to Congress by next March, and the report's ideas will be a big part of that strategy.

"They are right. We feel we already launched a war, and now it's time to escalate," he says.

By Amy Dockser Marcus, Wall Street Journal

"Our Family's Experience of Intrathecal ERT"

Steve Holland was an invited speaker to the International MPS Symposium in Adelaide, Australia. His presentation, below, also was given at the MPS Society Family Conference.

Amy and I met and started dating in high school. I was 16; she was 14. She says I'm the only boy she ever kissed. Our genetic fate was sealed with that kiss. After college we married, then Spencer was born in 1989, followed by Maddie, then Laynie. We noticed that all three kids couldn't raise their hands above their head and had lots of ear infections, but our doctor wasn't concerned. Then in 1994 our doctor identified anisocoria (uneven pupils) in Maddie, and he referred us to a geneticist. What followed was the diagnosis of MPS I in all three children. We reached out to the National MPS Society which was having its Disney conference in six weeks. Since we'd never been to Disney, we thought, "Why not?" We were hooked, ran for the board a couple times, and were eventually elected.

What we call the "Dark Period" began in 1998. We learned Dr. Kakkis was beginning a clinical trial for enzyme replacement therapy (ERT) for MPS I, but that only one child per family would qualify.

The Holland family

While Spencer improved with his weekly infusions during the clinical trial, the girls regressed. It was heartbreaking to know there was a treatment, but it wasn't available to all the children. In 2001 the phase III clinical trial began at UNC, and both girls were accepted. Although it was a double-blind, placebo-controlled study, we knew after the first week that Laynie was receiving enzyme and Maddie was receiving placebo. Laynie refused to ride in a wheelchair when we arrived at the airport in North Carolina that second week and, in fact, insisted on pushing her sister.

Two years later our family testified before the U.S. Food and Drug Administration (FDA) Advisory Committee about the benefits of ERT: increased stamina, zest for life and a halting of the physical aspects of the disease. It was a thrilling experience, hearing the unanimous "yes" votes for this treatment, the first treatment for an MPS disease, which paved the way for future treatments.

With the FDA approval, we now had all three kids on ERT, and eventually substituted home infusions for the weekly hospital infusions. Although the physical problems lessened, all three children had cognitive decline. In 2006, at age 13, Laynie's spinal cord compression symptoms began: tingling in her legs, reduced stamina in her legs, headaches, and tight MRI with signal changes, brisk reflexes and temperature changes in her legs. We were offered the treatment options of intrathecal (IT) ERT or surgery. Given the risks of surgery and the potential benefit of treating the brain, we enrolled Laynie in the IT ERT clinical trial.

Initially we traveled monthly to Los Angeles followed by quarterly visits. Our first day in the hospital Laynie was given eye and walk tests, a physical exam, lumbar puncture to administer the ERT, then an overnight in the hospital. The next day was a day of rest to ensure there were no complications before flying home. Laynie was very tired after each visit, and often had seat pain which was managed with pain medication.

During the next two years there were conflicting MRI results, although the tingling and temperature change stopped. Following Spencer's death in 2008 after his first IT ERT, the trial was on hold for 18 months, and restarted in July 2009. While Spencer's autopsy and tissue analysis found no direct connection with the IT treatment, you can only imagine the gravity of the decision to have Laynie restart the trial—as you parents know, being an MPS parent is not for the faint of heart! But by early 2010 there was no significant documentable improvement and possible worsening of compression symptoms, such as increased leg fatigue and possible incontinence. After much thought, prayer and deliberation, we made the decision to withdraw from the trial and pursue surgical intervention.

continued

We are blessed that the outcome, through the expertise of neurosurgeon Dr. Peter Sun of Oakland Children's Hospital, was successful. With three MPS kids, we've gone through a lot of surgeries: five shunts, three carpal tunnel, 18 ports and six tubes. This surgery was, however, the most emotionally demanding for all of us.

Having had the experience of several clinical trials, there are both negatives and positives, although the positives outweigh the negatives. The negatives include an increased focus on healthcare and MPS, possibly to the exclusion of normal life activities, and disruption to family and school schedules due to frequent travel. The positives are doing something to manage the disease instead of allowing the disease to manage us, plus access to outstanding MPS experts. In addition, there are potential benefits not only to your child, but to the MPS community as a whole.

If you ask if I'm glad we participated in the IT ERT clinical trial, you may be surprised by my answer, a resounding "yes." But why, since Laynie ended up having surgery and Spencer is gone now? I would answer that with an illustration that MPS is like a battlefield with MPS as the enemy. We are all soldiers, not by choice, but because we were drafted, and we all have different positions. Some of us are in the supply line in the back, but if everyone stays in the back, then the battle will never be won. Some people have to stay in the front lines, although they may be in harm's way and have to make quick decisions, plus occasionally they are under friendly fire. But it doesn't make the battle not worth fighting. If those original 10 kids in the MPS I ERT clinical trial had not taken a risk, your children would not be benefiting today from ERT.

Someday our battle will be won. Imagine this: On a sunny day in the future, a 14-year-old boy will pop a pill after a sleepover. His buddy asks, "Why are you taking that pill?" He answers, "Oh, my mom makes me. She says I have some genetic thing—no big deal—let's go play ball."

Dr. Kakkis and patients from the MPS I clinical trial at the 2003 FDA Advisory Committee hearing.

Spencer Holland

Laynie Holland

MPS III Expert Meeting Review

The Expert Meeting on Sanfilippo syndrome (MPS III) was held Aug. 27–28 in Northampton, UK. The National MPS Society sponsored two parents of children with MPS III, Shannon McNeil and Shannon Rieg, following a random drawing of applicants. The UK generously sponsored the leaders of the International MPS Network to attend, and the group met briefly after the MPS III meeting.

Dr. Marc Tardieu from Neurologie Pediatrique Hospital in France reported on the incidence and natural history study of MPS III; this study has been submitted to Am J Med Gen for publication. The incidence of MPS III in France is .73/100,000; in the UK the incidence is 1.21/100,000. The breakdown is 70 percent MPS III A, 14 percent MPS III B, 10 percent MPS III C and no cases of MPS III D. The average age at diagnosis is 4.9 for MPS III A, with 27 being oldest age at diagnosis; 4.9 for MPS III B; 12.0 for MPS III C; and 8.2 for MPS III D. Differences were identified among countries in terms of severity of disease, incidence and mutations. Among the French patients, 61 percent had residual enzyme, based upon specific mutation.

Data from the UK MPS Society show no difference in longevity between MPS III A and III B, with average mortality of age 13; MPS III C is more attenuated. These findings have been identified in other studies.

Dr. Simon Jones from St. Mary's Hospital in Manchester had similar incidence figures from their population of MPS III patients: A: 79 percent, B: 17 percent, C: 4 percent and D: 0 percent. Dr. Jones offered some medication suggestions*:

- Prozac is beneficial for inconsolable crying, and is used as a mood leveler.
- Medications available for mycoclonus (rocking, involuntary leg shaking):
  - Risperidone: must be careful with dose and start low; if your child is over-medicated there may be facial flushing and increased salivating.
  - Mellaril and Haloperidol
- Sleep problems: melatonin, chloral hydrate, or Vallergan (anti-histamine which can have hangover effects)
- SLO drinks, **slodrinks.com**, both hot and cold can be beneficial for swallowing problems.

Presenting on Adolescence and Beyond, Dr. Chris Hendriksz from Birmingham Children's Hospital noted*:

- Sodium Valproate for mood alteration can be beneficial as the child gets older.
- Acetaminophen and ibuprofen plus anti-depressants and anti-convulsants are prescribed for chronic pain.
- There are two case reports in the literature of children receiving hip replacements, resulting in the children regaining mobility.
- Two patients have been reported with recto-vaginal fistula due to frequent use of enemas.
- Secretion control, due to difficulty swallowing, leads to infections. Medications given through a patch not as intense; Botox injections in salivary glands also have been given to control saliva.
- If child is immobile and has chronic pain, check for renal stones.
- Treat spasms and dystonia if "troublesome."

Some of the current research developments include development of a knock-out mouse model of MPS III C (currently there is no animal model for III C) in Dr. Brian Bigger's lab in Manchester. Dr. Brett Crawford from Zacharon Pharmaceutical in La Jolla, CA, showed the benefit of small molecules therapy for substrate deprivation. Heparan sulfate (HS) is critical in developing mice but can be decreased after birth without consequences.

Adeno-assoicated vector (AAV) gene therapy for MPS III A in the brain shows long-lasting gene expression without integration into the genome. Dr. Olivier Danos with Centre national de la recherche scientifique will begin a phase I clinical trial in Paris with four patients. Prior to the direct injection of AVV to two sides in brain, in six tracks, patients will receive immunosupression in order to prevent intra or extra cerebral immune response. They have filed the investigational new drug application and are waiting for approval from the regulatory agency. Date for beginning the trial is late 2010 or early 2011.

continued

** These are suggestions and do not take the place of the medical care as prescribed by your child's physician.*

Dr. Jean Michel Heard from Institut Pasteur in Paris hoped to begin an AAV gene therapy phase I clinical trial for MPS III B in 2010 with four patients, onset of disease by age 4, less than age 6. If successful they would move to a phase III study with 16 patients. This study has been delayed due to legal issues between the funding agency and the company developing the AAV vector.

Dr. Brian Bigger announced a genistein clinical trial to begin in early 2011 in Manchester, sponsored by the UK MPS Society thanks to a £500,000 from an anonymous donor, enough to get the trial started. Genistein has been shown to block GAG in vitro with 10 percent crossing the blood brain barrier. The pure form must be used, in order to get more into the blood and brain. Dr. Bigger's research in MPS III B mice has shown that genistein reduces HS in brain by a third, improves neurological function and behavior abnormalities in MPS III B mice, with no toxic side effects. He emphasized that this is a treatment, not a cure, as it slows disease progression.

Dr. Charles Richard from Shire HGT reported that the MPS III A natural history study at the University of Minnesota in Minneapolis is well underway; the estimated cost per patient is $100,000. Shire also is sponsoring the MPS II phase I/II intrathecal enzyme replacement therapy (ERT) clinical trial at the University of North Carolina. Three patients had been infused as of the date of this meeting, and the first two had significant decrease in GAG in cerebral spinal fluid after the first infusion. On Aug. 24, 2010, a child with MPS III A received the first intrathecal ERT in the University of Manchester phase I/II clinical trial. Assuming there are no serious safety issues, the phase III trial is expected to begin in 2012 with sites in the United States, Europe and South America.

We are very grateful to the UK MPS Society for hosting this superb meeting which provided not only insight and knowledge but a renewed hope for the future of all children with MPS III.

Matt McNeil, Shannon McNeill and Shannon Reig

I was fortunate enough to be one of the scholarship recipients of the first Expert Meeting on Sanfilippo Disease held in Northampton, England, this past August. It was an amazing venue where scientists, doctors, experts and parents came from all over the world to share their findings, trials, studies and experiences on MPS III. These professionals have dedicated their lives to gathering information on MPS III in hopes of finding new therapies. During the meeting, we were led through the process of bringing new therapies to life. It is evident that an enormous amount of time and money is needed to get these therapies to enter clinical trial phases. They go through many unforeseen roadblocks, red tape and politics. However, one fundamental idea was obvious: This tenacious group of people from around the globe are collaborating and sharing their information for one common goal; to find credible therapies for our children with MPS III.

They have just begun phase I of enzyme replacement therapy (ERT) clinical trials in the UK for MPS III. The purpose of phase I is safety in dosing, while phase II focuses on efficacy. These first two phases are often done together. It is hoped that phase III and phase IV will be multi-continental by 2012. Essentially, intrathecal ERT for MPS III A consists of a port being placed in the lumbar region in order to effectively deliver the enzyme. Obviously, the greatest challenge in MPS III is crossing the blood brain barrier. They believe by administering ERT intrathecally, as opposed to intraveneously, more of the enzyme will get to the brain where it is ultimately needed. We shared in the excitement with the father of the first child to receive ERT. In his words, "This is what we have been waiting for."

Of course the topic of genistein was discussed. Not surprising, it was again brought to attention that none of the findings out of Poland had been published. However, we did hear from Dr. Brian Bigger, who not only has conducted a formal trial on genistein, but has published his findings as well. I strongly encourage you to read his publication. In essence, his trial consisted of having genistein in its pure form specifically manufactured for his study. It is important to note that genistein in its pure form is not manufactured for retail, over-the-counter use. It is most commonly a blend of genistein and genistin. Dr. Bigger had to use extremely large doses in animals to see any result. He found that only about 10 percent crosses the blood brain barrier, to be non-toxic, and blocks GAG production. Even so, he was not convinced that it did a measurable amount of good even in its pure form.

Without a doubt, the most touching part of the conference was hearing experiences from siblings of MPS III kids. I was so impressed with how mature these young people were able to express what it was like growing up with Sanfilippo. There were a few common threads in both of their stories. I noted how both siblings felt a sense of guilt leaving home to attend college. Their guilt was two-fold. On one hand, they felt guilty about leaving their parents and not being around to help with the care-giving. You see, looking after their sibling had become second nature, a part of their day, they knew nothing with their brothers and sisters. They came to appreciate the relationships they had with people and not things. It was clear MPS III had an enormous impact on their lives. Whether it was finding laughter amidst the chaos, learning patience, recognizing compassion, or going on to find jobs impacting others with disabilities, they appreciated the lessons learned from their siblings. As MPS III has a way of opening your eyes to what is important in life, it was crystal clear that we have turned a corner when it comes to Sanfilippo research. This conference opened my eyes to the importance of our MPS III community working together to find new therapies for the families of today and tomorrow.

Shannon Rieg

"I hope the kids sleep well for my parents," Shannon said while waiting to board our London-bound flight. Shannon had won a scholarship from the MPS Society to attend an MPS III conference hosted by the UK MPS Society. To attend we would have to leave our children, Waverly and Oliver, for the longest we have ever been separated from them.

"Are you going to worry about them so much you won't be able to enjoy our trip?" Matt asked.

"No, I know they're in good hands."

This conversation may sound familiar to those who have a child with MPS. Most of us have a very exclusive list of people with whom we will entrust our kids. These people make possible the rare occasions when we are able to get a night or even a couple of days to ourselves, because they have demonstrated their love for our children by learning to care for them. This is our standard for deciding if someone qualifies as having "good hands."

Knowing the kids were well cared for made it possible to attend the conference, which was invaluable for the opportunity to hear from researchers. One of the difficulties in parenting a child with MPS is the enormous chasm between our desire to do something to save our children and the limits of what we can actually do. That's why hearing about developments in research mattered so much.

Dr. Brian Bigger with the University of Manchester has been collecting blood samples for his biomarker research. Identifying biomarkers for a disease is essential for a speedy, accurate diagnosis. However, newborn screening is usually reserved for diseases for which effective therapies already exist. He also has conducted experiments using a pure, commercially unavailable form of genistein called aglycone, which has shown positive effects in reducing GAG buildups in mice trials.

Dr. Simon Jones, with the help of Shire Pharmaceuticals, is fielding a clinical trial for intrathecal (injection into the spinal canal) ERT in the UK. At the time of the conference, the first patient had already received her first treatment. Intrathecal ERT has real promise, having lowered heparan sulfate levels in animal trials. Other potential therapy options in various stages of consideration include: stem cell gene therapy, adeno-associated viral gene therapy and hematopoietic stem cell therapy.

Unfortunately, the blood-brain barrier (BBB), which provides essential protection for our brains, might also be the most daunting obstacle to finding effective treatments for Sanfilippo. The enzymes produced by replacement therapy have a high molecular weight and only poor to moderate success crossing the BBB. David Begley of Kings College London has been trying to find a way to get these enzymes through the BBB, and might have found a method that will work with MPS III in "receptor-mediated transcytosis."

The pharmaceutical industry also was represented. Brett Crawford, with Zacharon, said rather than trying to improve Sanfilippo patients' ability to break down heparan sulfate, he wanted to keep them from making it in the first place. Ten years ago, scientists believed it could not be done. New research indicates heparan sulfate production can be drastically reduced. Zacharon has tested approximately 154,000 compounds trying to find one that would lead to an effective "substrate reduction therapy." They have narrowed it down to the seven that show the most potential.

We left the conference encouraged that so many capable researchers are working to give hope to MPS families. After we got home, a number of people asked, "How was the conference? Did you learn anything?"

"Yes," we would reply. "We learned our kids are in good hands."

Shannon and Matt McNeill

MPS III C natural history study

The Neurodevelopmental Function in Rare Disorders Program (NFRD) is working toward describing the natural history of Sanfilippo C (MPS III C). Given that in the near future therapies will be available to treat this devastating neurodegenerative disorder, it is of utmost importance to characterize the disease process, understand the variability in presentation and describe early markers of disease progression. Because this disease is rare and geographically dispersed it is necessary to have a concerted effort to bring families to a center of excellence where the child's functional status (behavior, communication, cognitive and motor skills) can be evaluated. In addition, markers of disease progression such as immune changes, brain imaging findings and biomarkers can be described. This information can be used as a historical control arm of future clinical trials. Families are encoured to participate in this important effort by participating in the NFRD registry and enrolling in the study.

Contact:
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MPS III patients needed for research study

This research project will focus on how the immune system is affected in patients with MPS III A or B. A blood test will be done to measure blood cell count. To participate, patients must:

- be less than or exactly 20 years of age with a confirmed diagnosis of MPS III A or B
- not use medications that suppress the immune system at time of enrollment
- not have respiratory, urinary or other infections at the time of enrollment

The study will take place at your local healthcare provider's office or at The Research Institute at Nationwide Children's Hospital, Columbus, OH. For more information and to make an appointment, contact Chelsea Rankin at 614.355.2897 or Chelsea.Rankin@nationwidechildrens.org.

Surrogate Endpoint Trial (SET) for individuals with MPS III A

Sponsored by Shire Human Genetic Therapies

SET is a one-year study designed to study the natural progression of Sanfilippo A syndrome, or MPS III A, in approximately 20 patients. During a period of 12 months participants in the study will be evaluated to assess the severity and progression of MPS III A, as measured by developmental age and milestones, central nervous system function (including cognition, speech and motor skills) and biochemical markers of the condition (levels of heparan sulfate and its breakdown products in blood urine and cerebrospinal fluid).

Additional information can be found at **www.clinicaltrials.gov** (identifier NCT01047306), or contact: Shire HGT Medical Information, 484.595.8850, HGTmedcomm@shire.com
or
Amy K. Fisher, MS, CGC, Shire HGT Global Medical Affairs, at 857.413.9553 or afisher@shire.com.

Clinical Trials

MPS I

The Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center in Torrance, CA, and the University of Minnesota are collaborating on two important studies of intrathecal enzyme replacement therapy (ERT) for patients with MPS I. A brief description of each study and eligibility criteria are listed below.

A Study of Intrathecal ERT for Spinal Cord Compression in Patients with MPS I

The purpose of this research study is to find out whether giving ERT (with Aldurazyme®) as an injection directly into the spinal canal (called intrathecal injection) can help reduce spinal cord compression due to MPS I and can provide an alternative to surgery.

Study participants will have:

- up to 16 intrathecal ERT treatments given one to three months apart over one-and-a-half years
- physical examinations (general and neurologic)
- other diagnostic tests
- reimbursement/payment of travel expenses

A Study of Intrathecal ERT for Cognitive Decline in Patients with MPS I

The purpose of this research study is to find out whether giving 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.

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 information can be obtained at **www.clinicaltrials.gov**; search under "mucopolysaccharidosis."

Study centers other than at Harbor-UCLA may be available. For more information, contact:

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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 the 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

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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 the 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 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 need 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 at the University of North Carolina at Chapel Hill, NC.

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 Sanfilippo syndrome type 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. The 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.

continued

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 on April 29, 2010, positive results for the phase I/II trial for BMN 110 or *N*-acetylgalactosamine 6-sulfatase (GALNS), intended for the treatment of MPS IV A, or Morquio A syndrome. The company expects to initiate a pivotal phase III trial in the fourth quarter of 2010.

The phase I/II study is designed as an open-label, within-patient dose escalation trial in approximately 20 patients followed by a treatment continuation phase. During the dose escalation phase of the study, subjects receive weekly intravenous infusions of GALNS in three consecutive 12-week dosing intervals: 0.1 mg/kg for 12 weeks, 1.0 mg/kg for 12 weeks and 2.0 mg/kg for 12 weeks. The objectives of the phase I/II study are to evaluate safety, pharmacokinetics, pharmacodynamics, clinical response to therapy and to identify the optimal dose of GALNS for future studies.

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®.

"We are encouraged that clinically significant improvements in endurance and pulmonary function can be detected with GALNS treatment even in a relatively short study of a heterogeneous population," said Hank Fuchs, MD, chief medical officer of BioMarin. "This gives us confidence that we can design a robust 24- to 36-week phase III clinical study with a measure of endurance as the primary endpoint and several additional supportive endpoints. We will be working to reach an agreement with regulatory authorities on a powerful and large phase III trial protocol that minimizes regulatory risk and captures the maximum amount of clinical benefit expeditiously. We are on track to initiate a phase III registration-enabling program in the fourth quarter of 2010."

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

MPS VII

A gene therapy clinical trial for MPS VII, also known as Sly syndrome, has been put on hold pending additional data.

ML II/III

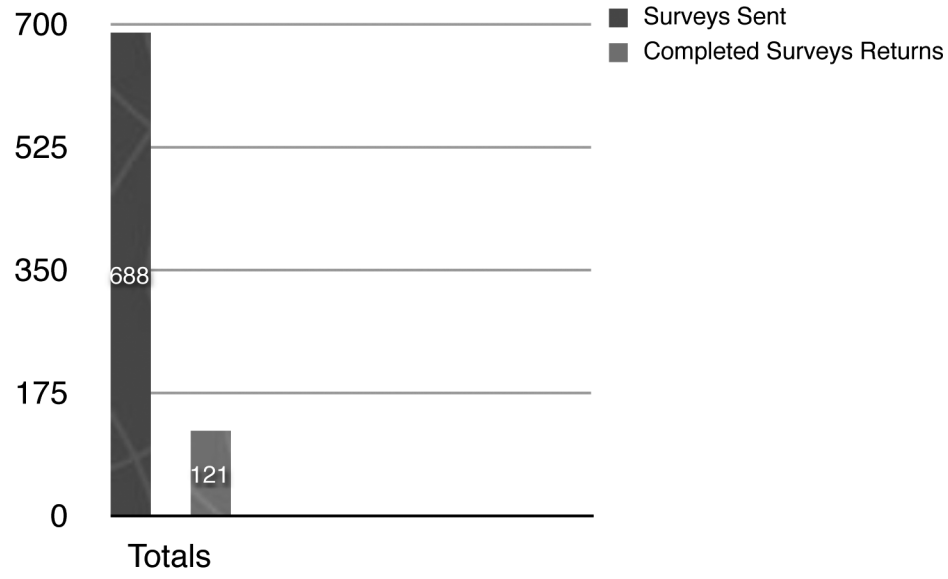
There currently are no programs in place for developing treatment options for ML II/III.

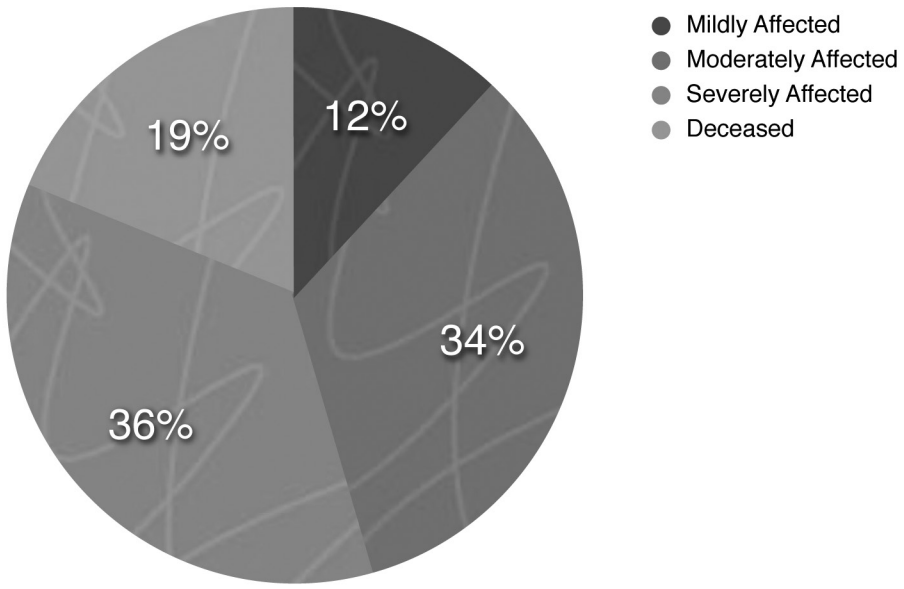
Did You Know?

In 2009, $7 million was allocated by the National Institutes of Health for MPS research.

2010 Membership Survey

This year the Education & Publicity Committee conducted a survey of the membership to explore satisfaction with communication, family support programs and publications available to the membership. Participation in this survey was made available to all members of the National MPS Society in the summer of 2010. Responses were collected through an online survey Web site. Members without Internet access were mailed a paper copy. Participation was optional and all ideas and feedback were encouraged. This was an anonymous survey designed to provide members the freedom to communicate honestly. What follows is a summary of the results of this survey. Six hundred eighty eight members were invited to participate and 121 members completed the survey.





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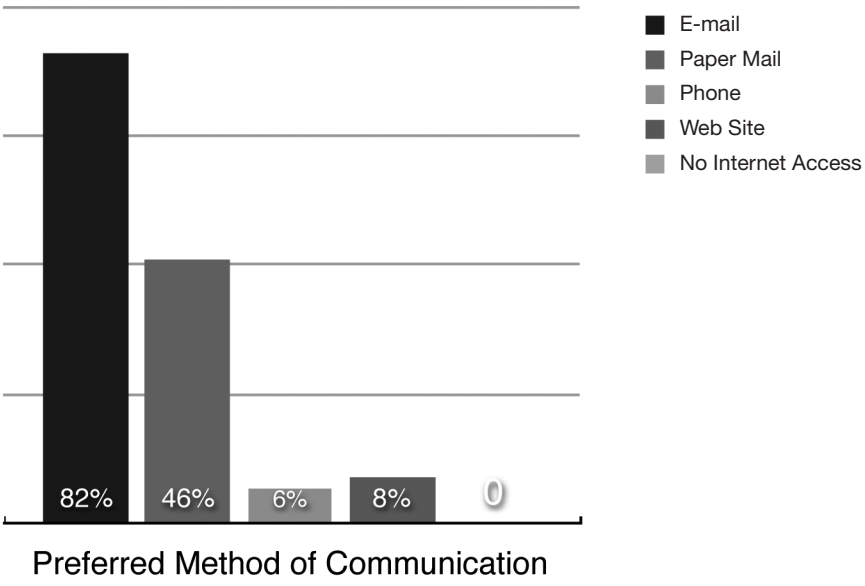
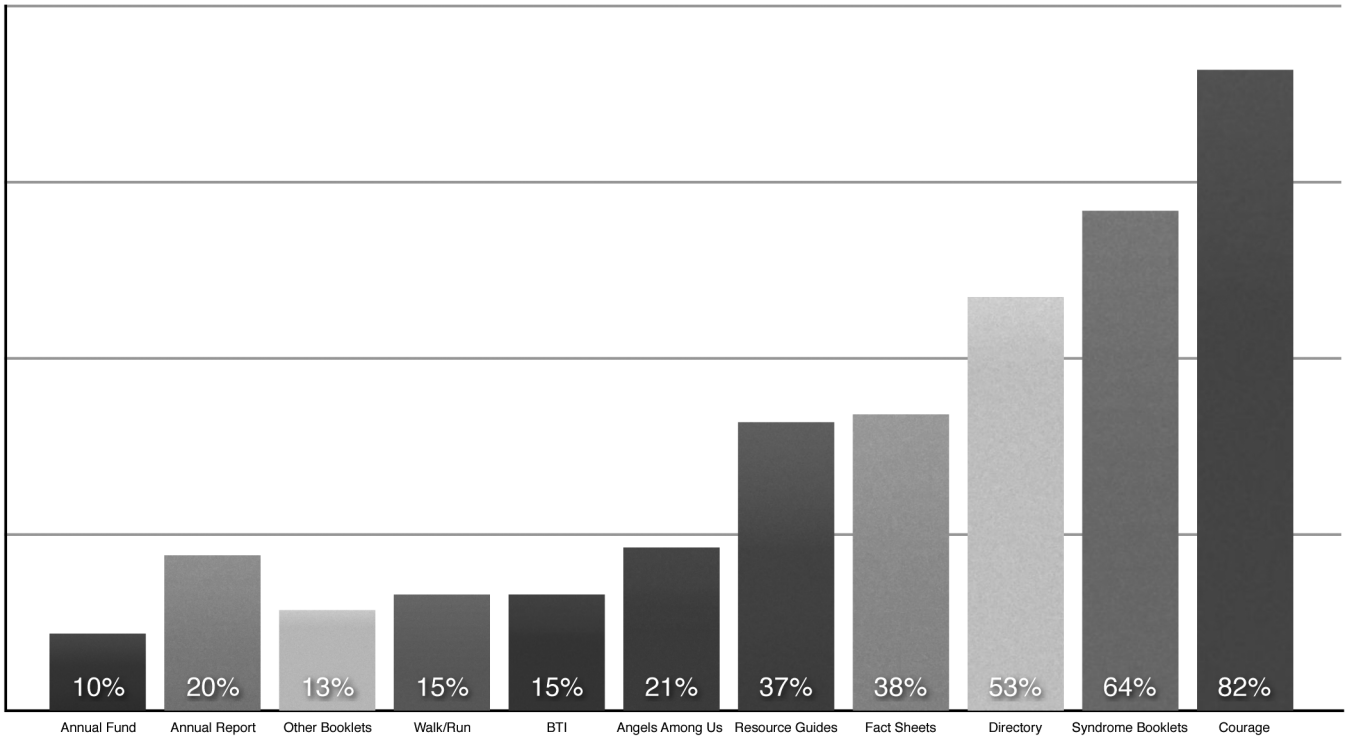
As we modify ourselves to adapt best to current trends it is important to understand exactly how our members access the Society and what preferences our membership have with regard to communication.



Preferred Method of Communication

It was of great importance to understand what publications our membership find helpful and supportive. While some publications are more costly to produce than others (*Courage* vs. fact sheets) the intended purpose is to educate families, provide support and facilitate collaboration with others. These results are presented in the chart below.



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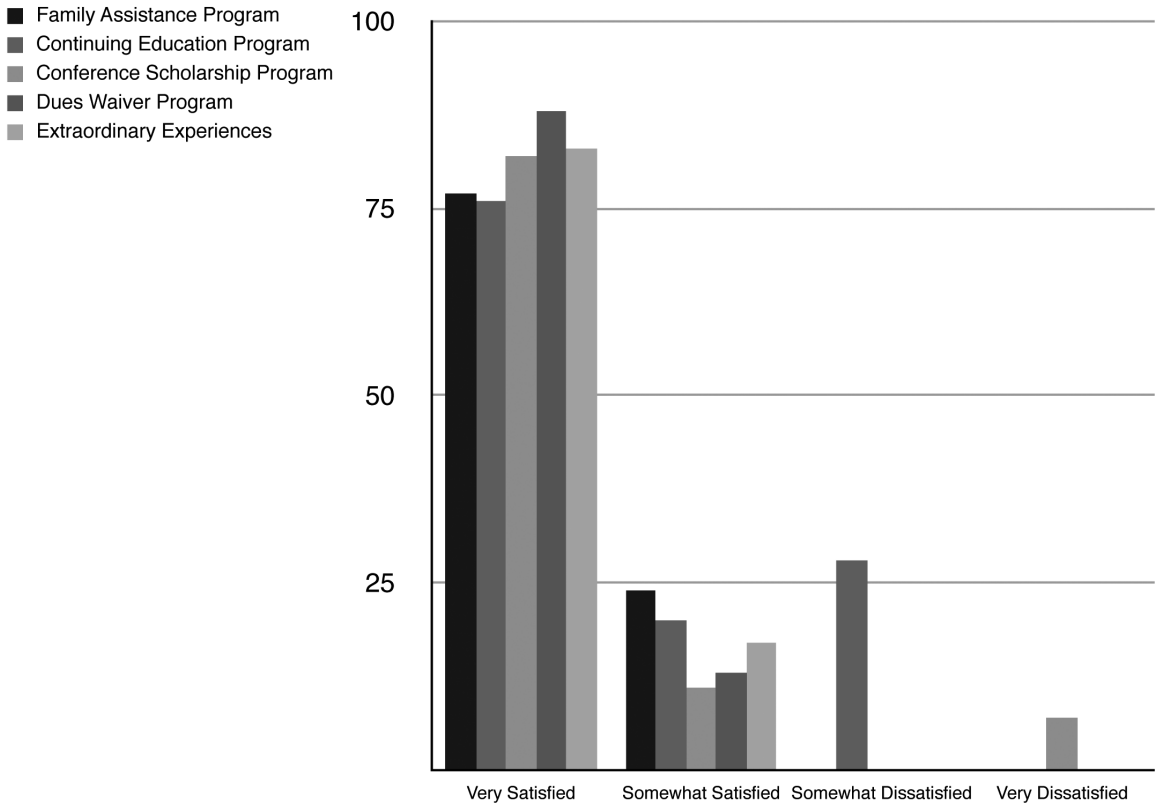
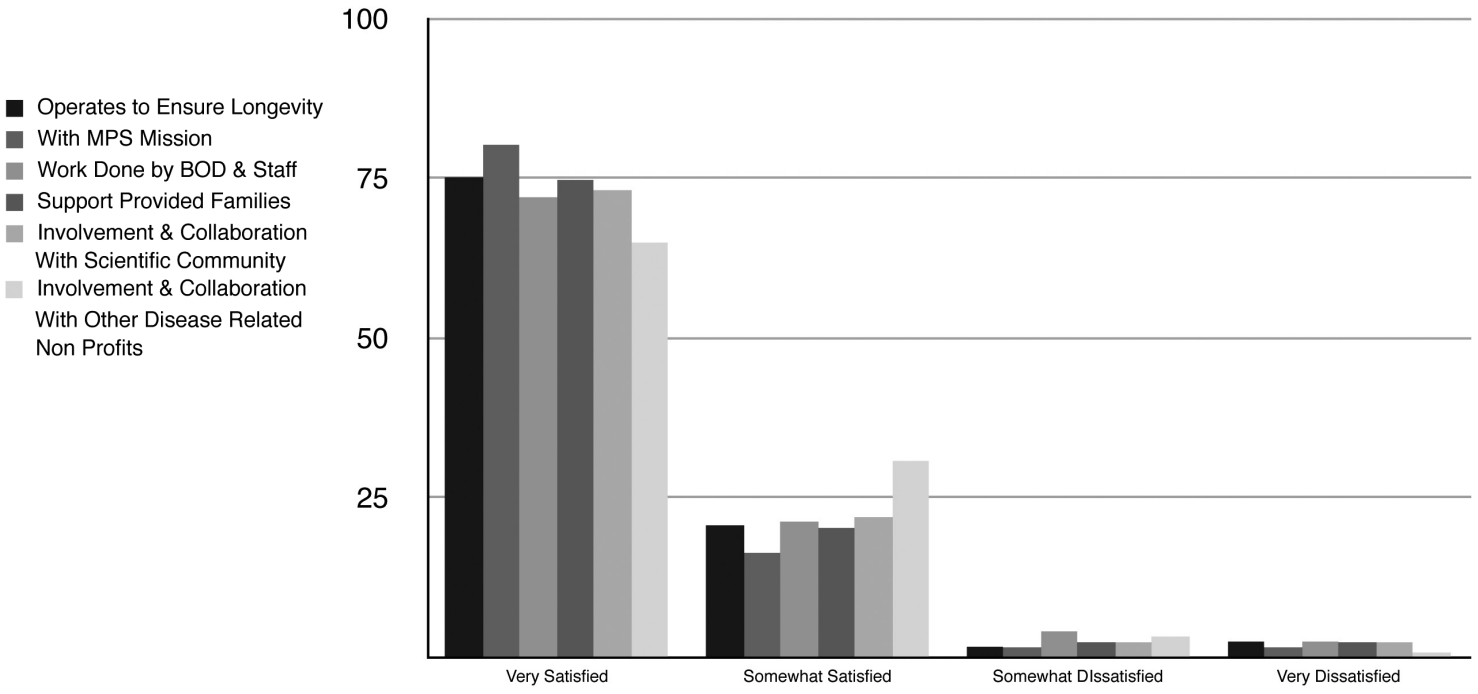
Many of our programs have been developed and designed to provide support to families. The results are presented in the chart below.



The survey included several questions exploring membership satisfaction with the work done by both the staff and the board of directors. These results are presented in the chart below.



This survey was anonymous to ensure that all who participated could respond honestly without concern for repercussion. It was our hope to hear all concerns. Following is a list of concerns communicated in this survey. These results have been reviewed and discussed by the Education & Publicity Committee at the most recent board of directors meeting and at the general membership meeting at the annual family conference held at Knott's Berry Farm.

continued

Expressed Concern	Committee/Board Discussion and Action(s)	Discussion at Annual General Membership Meeting
Board of directors needs to change more.	Issue of term limits has been discussed in the past and discussions continue.	The idea of term limits was raised as something to consider.
Appearance of selectivity based on skills and profession.	Ongoing discussion. From time to time the board has needed a member with a skill to effectively uphold the duties required of the board as stated in the mission and bylaws.	Various members agreed that there are times where this is necessary but did not feel it was done in a self-serving manner.
The new Governance Committee has taken my rights away.	Discussions ongoing. Governance has provided membership with specific goals and objectives in *Courage* and during general membership meetings at AGM Disney & Knott's.	Members discussed how difficult change can be and the existence of Governance Committee's in both public and private sectors. Members discussed the need for governance.
Too many professionals on board.	At present the professionals on the board of directors include one MD, one PhD, two CPAs and two entrepreneurs.	Discussed that board composition is representative of members who run for the board.
Staff do not have MPS children.	At present we have one staff member who does have an adult child with an MPS or related disease.	Discussed the need for this for staff effectiveness and the fact that we have a staff member with an affected adult child.
Board of directors needs to be more outgoing at conferences—too cliquey.	This was heard, discussed and acted on during our Knott's conference. Conference evaluations will be examined.	Discussion similar to stated discussion among committee and board.
Too much money going to families for items needed when other programs exist to fund items.	This is an ongoing discussion among the Family Support Committee. Access to certain programs varies from state to state. The Education & Publicity Committee is working toward creating a resource guide for families.	Discussion similar to stated discussion among committee and board.
Society is not beneficial to all MPS families.	This was heard but difficult to discuss. It was unclear as to what is not beneficial. There is great interest in development if needs are not being met.	This was heard but difficult to discuss. It was unclear as to what is not beneficial. There is great interest in development if needs are not being met.
No board members with severely affected children.	Discussed and a review of the make-up of the current board reveals that many board members have children who are severely affected by MPS.	Members discussed the lack of evidence to support this comment.

This survey provided other valuable information as well. We learned that members want booklets or fact sheets on topics not yet available in our library. However we also learned that members were unaware of certain topics that already have a fact sheet or a booklet. Perhaps this speaks to a need for this committee to arrange or organize available materials differently. We learned that our members want to hear from us more. This was a pleasant surprise. More e-mail, more contact, more communication was overwhelmingly okay with the members who completed this survey. The Education & Publicity Committee would like to extend a word of thanks to every member who took the time to complete this survey. The information you shared with us provides important direction and assurance. It also helps us see where we need to go, how we need to grow and what we can do better. Thank you.

Rare Disease Day is Feb. 28!

Rare Disease Day is an annual global event that is observed on the last day of February. The day was first celebrated in 2008 in Europe and has since grown into a worldwide initiative to raise awareness of rare diseases as a public health issue. The term "rare disease" is actually a misnomer when you stop to consider that collectively, rare diseases affect 1 in 10 people, or approximately 30 million Americans and nearly 20–30 million Europeans. That hardly seems rare at all!

Learn more about it and how you can participate at **RareDiseaseDay.org**.

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.

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.

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**.

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.

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**.

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.

Dr. Emil Kakkis Honored as a 2010 Rock Star of Science in GQ's 'Men of the Year' Edition

GEOFFREY BEENE GIVES BACK™ and *GQ Magazine* have joined forces, along with the Entertainment Industry Foundation, to bring together eight celebrity musicians and seventeen of the nation's top medical researchers. Each pairing is a tribute to "scientific heroes" in fields like translational cancer research, Alzheimer's/ neuro-imaging/prevention trials, heart disease/ integrative medicine, autism, rare diseases, stem cell research, global health and space age research.

Emil Kakkis, MD, PhD, is one of those honored physicians, paired with rock star Jay Sean. Dr. Kakkis is president of the Kakkis EveryLife Foundation and has devoted his career to finding treatments for rare diseases and fast-tracking those treatments from research phase to market. He developed a treatment for the rare disease MPS I early in his career that drove his interest in the development of many more rare disease treatments.

"It is very important for young people to know that science is cool and that these rock stars are putting their names and faces behind our need for more scientists," Kakkis notes. "I think that young people considering science should know that science is not all figured out and written in a book somewhere, and that in fact so much is still unknown, waiting for them to discover."

"There are thousands of rare diseases without treatments, and we need many new brilliant, creative and cool rule-breakers to challenge what we know and save people with an untreated disease. There is nothing more gratifying and cool than doing that. I know because it happened to me."

In 2009 Dr. Kakkis founded the Kakkis EveryLife Foundation to accelerate the process for bringing new rare disease treatments to the patients who need them. Dr. Kakkis worked with the Food and Drug Administration (FDA) and Congress to improve the regulatory process for rare diseases. His efforts and goals were recognized in Congress by the Brownback Brown Amendment to the 2010 and 2011 FDA appropriation bills. The bills require the FDA to review and improve rare disease regulatory policies.

"Scientists must venture outside their comfort zones to show the public how cool—and how important—their work really is," said Dr. Francis Collins, Director of the National Institutes of Health. "I'm thrilled to see all of these big-name musicians using their star power to shine a spotlight on science. However, it is only the beginning. I urge every scientist get into the act by telling friends, neighbors, community leaders and elected officials about his or her research and what it means for our nation's health. Imagine how powerful that would be."

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

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