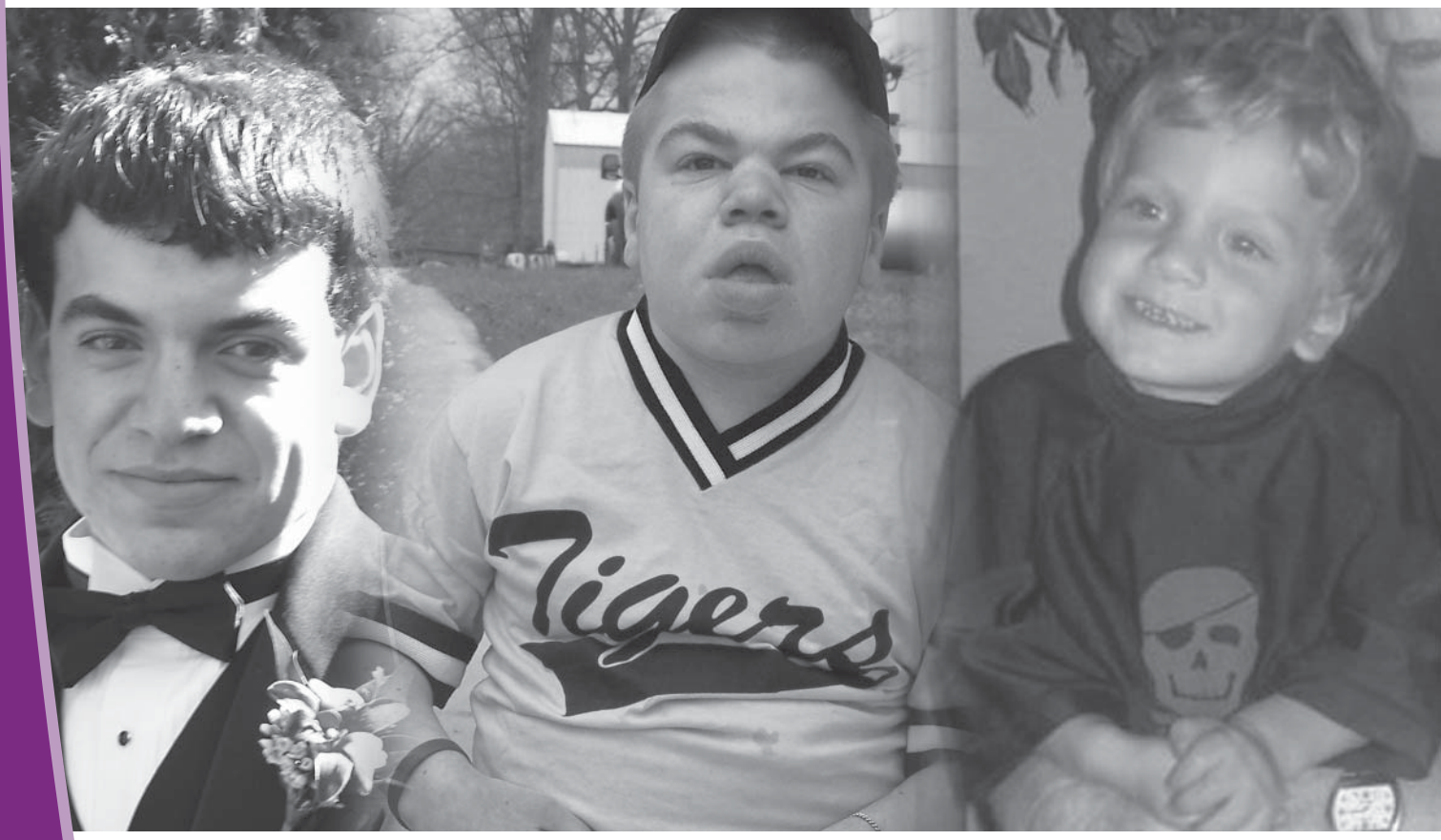
National MPS Society
Support for Families. Research for a Cure.

Volume 33 | Number 2 | Summer 2009

COURAGE



Big Plans for the Future

National MPS Society announces Continuing Education Scholarship winners

Research News

BioMarin announces new clinical trial for MPS IV A

Rediscover Your Resources

This issue packed with helpful information and unique opportunities

Focused and Moving Forward

Board of directors tackled wide array of issues in 2008

Legislative Update

Obama administration names new leadership at FDA, removes stem cell research barriers

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 new 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
Support for Families. Research for a Cure.

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

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Pictured on the cover:
Nick Boyce, Kraig Klenke, Trey Lane

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

President's Letter

It is summer once again. During a time when all things become new and there is a strong life force present all around us, your board has been diligently working on the new year's tasks and duties as always, but this year it seems somewhat different. This year I have seen our committee chairs, committee members and our MPS staff working more as a finely tuned team than ever before. There seems to be a renewed trust and your board is feeling very engaged. Your working committees have taken on new responsibilities and have rekindled all tasks. All chairs and committee members have put forth many hours of devoted work and it shows in the products created.

For example, this December will be our seventh Disney conference, and for the second time in a row we will be staying at a Disney resort with great prices and better access for our families. We are proceeding with a makeover of our Web site, new MPS fact sheets and booklet updates, advocacy trips to Washington, DC, along with collaboration meetings with the National Institutes of Health and your Scientific Advisory Board. Even though we are industriously working on speakers, childcare issues, etc. for Disney, work is also underway on the 2010 family conference in California. We are reviewing research grants, attending MPS international meetings and partnering with other lysosomal storage disease organizations. Enlightening others about National MPS Awareness Day, creating our New Members Spotlight, Standing Ovation Program, Continuing Education Scholarships, along with our new U.S. flag program for deceased children, all came about from ideas and suggestions from our members.

In February we welcomed Austin Noll to our board of directors to fill the spot open after Kym Wigglesworth resigned. Austin and his wife, Cheryl, are parents of Austin with MPS III and reside in California. Austin was defeated in his first run for the board, yet immediately picked up the phone and said he was willing and able to volunteer his time to our Society. We thank the Noll family for their commitment.

You, as members, also have had a very busy launch into summer by planning family fundraisers, MPS walk/runs, attending special needs camps, and preparing and participating in MPS social gatherings. We are thrilled that you continue to submit your wonderful articles for *Courage*. Sharing your stories, pictures, ideas and networking helps all of us manage our lives with MPS. You, our members, continue to be creative and educate others when it comes to life with MPS. You have shown us several ways you enjoy celebrating National MPS Awareness Day, from participating in local family and school events to making contact with local and national news media, and communicating with our congressional leaders to affect change for policies which will improve the quality of life for all.

With all of this you still have time to devote to your daily activities of caring for a loved one with MPS or reaching out to friends affected with MPS. I am not sure how you do it, but you carry on every day with unrelenting energy and zeal. The commitment and enthusiasm of our membership is overwhelming and that is why you achieve so much. You never give up. You never quit. You don't just plop down on the sofa even if life is not going your way. You are willing to assist, support and extend a hand in friendship. We are all bound by a common bond and I am thankful for it. As always, I am honored to serve this motivated membership.

The importance of the National MPS Society's research program was highlighted this year by the number of researchers submitting letters of intent, a total of 33. The Society has received global recognition as an organization that continually supports quality research, and we receive letters of intent from researchers around the world. During this period of reduced funding for this critical research, the money supplied by the Society ensures that these innovative research studies continue. Our research money also financially supports young researchers, in hopes MPS research will be a lifelong pursuit. Our Scientific Advisory Board Research Review Committee will read the 12 proposals selected for final consideration of funding for the five grants the Society is offering this year. Those five grants will be announced in our next newsletter.

We have developed a strong research funding program because of the tremendous support of you, our members. You hear our thanks often, but let me say it again—thank you! And that thanks is echoed by everyone who has been and will be recipients of the funding.

The National MPS Society is a leader nationally and internationally. This year we have interacted with many of the lysosomal disease patient groups in support of the National Institutes of Health (NIH) grant submission of the Lysosomal Disease Network (LDN). We met while attending the LDN conference in San Diego and have participated in conference calls. Our letters of support are not only welcome, but required as part of the grant submission process. Collaboration among patient groups, researchers and the NIH is crucial.

Plans for the 2010 International MPS Symposium in Australia are under way, and I have been asked to serve on the program committee. Leaders from many international MPS organizations will attend the Disney conference. The National MPS Society is hosting the yearly meeting of the International MPS Network on Dec. 19. This year we are increasing our outreach efforts to MPS organizations in South America and in the Asia-Pacific region.

Rarely a day goes by without one of us in the office talking about and planning for some aspect of the Disney conference. We are hearing from so many families about your plans to travel to Orlando in December, and we are thrilled. There are many people working on this conference, and there will be surprises for everyone!

Barbara Hudson

It has been a very busy spring here at the MPS Society. I am excited to be working with Terri Klein, our new development director, and look forward to moving forward with her leading our development efforts. Welcome Terri.

The Family Support Committee (FSC) has been very busy in the recent months with some of our larger programs—the Continuing Education Scholarship Program and the Conference Travel Scholarship Program. The committee reviews each application and evaluates for funding. Congratulations to all those who applied; we wish we were able to fund all of the applicants. The committee determines who will receive the awards (for more information see page 26). We wish all applicants the best of luck as they continue their education.

The FSC also spent time reviewing and evaluating the 43 conference scholarship applications received. Disney scholarships will financially help families attend the Disney conference to be held in December.

Special thanks to the committee for undertaking these projects. It truly was an amazing experience to read through the applications, and it is heartwarming to hear the wonderful words used to describe the special person with MPS or related disease and how he or she has impacted the life of the applicant. All of the applicants submitted wonderful essays. You can imagine the heartfelt responses we received.

We hope you and your family will be able to attend the Disney conference, it promises to be the best ever!

Laurie J. Tuma

Development Director's Letter

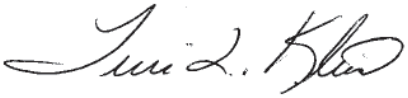
It is a privilege to begin a new chapter with the National MPS Society in fund development. I have been a member of the Society since the diagnosis of my daughter with ML III in 1999. In my new role as the Society's development director, I bring compassion, conviction and support for fundraising events that will benefit our children and adults affected by MPS and related diseases.

"Back to the basics," working harder and stronger to develop funds is a goal for 2009. Our supporters will soon be given the opportunity to contribute to the 8th Annual Fund to support the Society in research, programs and operations. Many of our families and supporters appreciate giving to the National MPS Society through this annual contribution. It also is a wonderful time to share information about our Annual Fund with family, friends and co-workers. If your company participates in a corporate match program, explore raising awareness through the Annual Fund at your workplace.

The walk/run season will soon be here as well. Several new families are excited to host an event this year. I am here for support and to answer questions, and a number of previous event coordinators are available to mentor new organizers. We also have established online resources and a guide to make the planning of an event easier.

Driving from Michigan to my new home in North Carolina, I reflected on ways the National MPS Society continuously supports our families. The Society is a leading authority on MPS and related diseases and provides support to our families at the time of diagnosis and beyond. We are a working community of families, gathering knowledge and sharing experiences, while providing support with numerous publications and annual conferences. Our family support programs provide essential assistance for scholarships and medical equipment. And lastly, as we strive to attain the goals in our mission to find cures for these diseases, are the advocacy work of the Society's Legislative Committee and the research funding.

It is important to stay encouraged, motivated and compassionate even in the most difficult of times. The Society counts on the continued support of our families and friends. With each fundraising event, increased awareness about MPS and related diseases escalates! Call and let me know how I can help make your event more successful.



New Members

Alta School
Alta, WY, professional membership

Annette Arevalos
McCamey, TX, mother of Shawn Damien Walton, MPS IV

Sara Aswegan
Lexington, MA, Shire HGT

Cari Aufman
Wayland, MA, mother of Jessica Aufman, MPS III A

Barr Memorial Chapel, Inc.
Fort Madison, IA, friends of Lucas Montgomery, MPS III B

Misty Bonner
Quitman, MS, mother of Nicholas Bonner, MPS II

Jeff and Krista Burkett
Fort Riley, KS, parents of Jarin Burkett, MPS II

Amanda Craig
Milton, KY, mother of Brinley Craig, MPS I

Shannon and Andrew D'Agostino
Newmarket, NH, aunt and uncle of Elliot Matheson, MPS I

Dianna Delacoeur
Nevada City, CA, mother of Kristopher Delacoeur, MPS III

Tara and Jack Elston
Springfield, IL, parents of Jake Elston, MPS III A

Jason and Jamie Fowler
Denver, CO, parents of Jack Fowler, MPS II

Stephanie and Ben Gaswint
Everett, WA, aunt and uncle of Jacob Irish, MPS I

Arthur and Karen Gibson
New Vienna, OH, grandparents of Clara Gibson, MPS III

Alicia Marie Hemingway
Shelburn Falls, MA, adult with MPS I

Debbie Hernandez and Gilbert Torres
Fort Myers, FL, parents of Adriana Torres, MPS I

Rene and Chris Irish
Everett, WA, aunt and uncle of Jacob Irish, MPS I

Aaron and Ann Johnston
Center Point, IA, parents of Oren Johnston, MPS II

Russell Kacer
El Campo, TX, brother of Kaylee Kacer, MPS III A

Joan Kelleher
Chicago, IL, aunt of Molly Birmingham, MPS I

Aura Leal
Miami, FL, mother of Andy Rodriguez, MPS II

Andrew Loudermilk
Cincinnatti, OH, adult with MPS II

Linda and Charles Meador
Williamsburg, VA, aunt and uncle of Stacy and Matthew Wain, MPS I

Jill McDermott
Monticello, IA, mother of Morgan and Blake McDermott, MPS III A

Troy Mitchell
Woodstock, IL, father of Caden Troy Mitchell, MPS II

Soraida Munoz
Homestead, FL, mother of Edward Munoz Jr., MPS II

Kindall Nelson
Galesburg, IL, mother of Lauren DeJaynes, MPS I

Austin and Dorothy Noll Jr.
Randolph, NJ, grandparents of Austin Noll IV, MPS III A

Anthony and Pamela Paladino
Lake in the Hills, IL, grandparents of Caden Mitchell, MPS II

William Peterson
Fishers, IN, adult with MPS VI

Darrell Saylor
Wilmington, OH, grandfather of Clara Gibson, MPS III A

Karli Schellenberg
Hummelstown, PA, professional membership

Marilyn Stephan
Tamarac, FL, aunt of Zachery Townsley, MPS II

Brooke Story
Post Falls, ID, mother of Elijah Story, MPS II

Sheila Thornton
Davis, CA, mother of John Thornton, MPS III A

Hal and Laura Thorsrud
Decatur, GA, parents of Juliana Katheryn Thorsrud, MPS III B

Stephen Weaver
Blomington, IL, adult with MPS II

Monique Whitaker
St. Petersburg, FL, mother of Eddie Whitaker-Russell, MPS II

Phyllis Wilson
Tomball, TX, grandmother of Michael Trahan, MPS II

Donations In Honor of

Mel and Millie Anhalt in honor of their birthdays
Caroline Masur

Davis Barkley
John and Sue Scott

Carter Brotherton
Chris Roussy

Matthew Caldwell in honor of his 15th birthday
Marjorie and Robert Austin Jr.
Mark and Stephanie Caldwell
Lori Ledoux

T.J. Catanzarite
Wooster Elks Lodge #1346

Jon Deutser
Mel and Millie Anhalt

Jake Elston
Heather Grund

Michelle Hopkins
Mike DeLisle
Dana DeVore

Alex Jacob
Julia Jacob

Bridget Joyce
Friends from FCCAA

Aiden Lopez
Ronald Weil

Caroline Masur in honor of her birthday
Mel and Millie Anhalt

Maxwell Mingo
Diana Potts
Lisa Rajakovich
Melissa Woodward

Roger Mingo
Becky Mingo and
Dutch MacDonald

Toby Mingo in honor of his birthday
Laura Vinchesi

Amy Shapiro
Becky Mingo and
Dutch MacDonald

John Thornton
Stephanie Rubinstein

Carly Trockeck
Vickie McFarlan

Donations In Memory of

Eric Conner
Jeff and Julia Conner

Kelley Crompton
Patricia Watson

Karly Davis
Mary Beth Barrack
Ira Glant
Joan Kroll
Milden Presbyterian Church
Evening Circle
Robert and Deborah Oder

Crystal Edie
R.M. Marc and C.R. Weal

Carolyn Faust
Larry Lomax and
Barb Wendt
Susan and Larry Kirch

Petey Feibelman in honor of his birthday
Phyllis Feibelman

Fannye Gottsegen
Mel and Millie Anhalt

Aaron Jackson
Debbie Hanford
Kathryn Hanford
Jennifer Schmidt

Joe and Maggie Langford
Valerie Barnett
Andrew and Joyce Nielsen
Brian and Rosemary
Rosendale

Alyssa Leonard
Patti Haney

Charles Nathan Page
Mary Jo Page

Evan Reed
Gary and Lana Marble

Hannah Salcher
Monica Eberhardt
Mona Fulghum
Rebecca Weber
Chuck and Judie Whitley
Marla Whitley
Sarah Whitley

Noah Scott
Erinn Colin
Rebecca Grinstead
Kathryn Huff
Steven Jongewaard

Katie Shine
Mary Shine and
Thomas Cleaver

Brian Stanley
Judith Cloward
Paul and Beverly Delucchi
Laura Gaytan
Mr. and Mrs. C.B. Glass
Livermore-Amador Valley
Legal Secretaries Association
Karen Ratto
Valerie Silva
Beverley Struthers
Abramson and Thaete, LLP

Clinton Szemanski
Gail Finney

Clinton and Zachary Szemanski
John and Cheryl Hakola

Jennifer Wingate
Donna Booker
Brookwoods Group
Don Canada
Theresa Drago
Kevin and Sheila George
Steve Guidry
Sherri Heim
Stephen and Katherine
Herbst
Wayne Howser
Ingram Micro
INSGROUP INC.
Ritesh Kapadia
Susan and Larry Kirch
Myron F. Steves and Co.
Howard and Sallie Namkin
Jeff Namkin
Nicole Parker
A.R. and Katherine Pintsch
Eric Thomas
The Uson Company
Constance Van
Jason Waldrop
Stephen White
Joan Wingate

Matching Gifts

Allstate Giving Campaign
Bristol-Myers Squibb
Company
Exxon Mobile Foundation
The GE Foundation
Giving Express Online from
American Express
ITW Foundation
Microsoft Matching Gifts
Program
Pfizer Foundation Matching
Gifts Program
Scitor Corporation
Wells Fargo Community
Support Program

Fundraisers

Eliza Burke cookie sale in
memory of her sister Edie
Jonathan Formica
Courage Gala
Montgomery family Links
for Lucas fundraiser
Rachel Wojnarowski Play for
Taylor fundraiser

Donations

Mary Starr Adams
Dean and Kathy Aker
W. Andrew and Lauren G. Jack
Frederick and Nancy Andrews
Cynthia and Randy Anhalt
Gerald and Susan Anhalt
Dwight and Margaret Barkley
Valerie Barnett
Frank and Lorraine Bien*
Michael and Grace Bodura
Marie Bonville
Susan Booker
Daniel and Debra Brockman
Bryson Foundation Ltd.*
Earl and Pat Chambers
Patrik Claussen
Stephen and Denise Clayton
Margaret and Larry Cohen
Estelle Culpepper*
The Daniel Molinaro Foundation
Dianna Delacoeur
Bonnie and William Doster
Sean Dummann
The Elizabeth Goldberg Family Educational Fund of the Jewish Communal Fund
Toni Ellard
Amy and Patrick Fitzgerald*
Lennie Forkas
Kim and Stephen Frye*
Kevin and Andrea Gates
Michael and Marcia Geller
Vincent and Shirley Giles
John and Jennie Gladysz*
Tom and Anne Gniazdowski*
Blake and Ashley Graeber
Sheryl Hall
Monica and Wallis Hampton
Walter and Judi Hauck III
Martin and Mary Heikel*
Susan and John Herzig
Scott and Lynn Hopkins
Todd and Jennifer Howard
Steve and Pat Irish
James Kalteux
Rick and Beth Kania*
Ruth Kimberley
Kevin and Mary Kimmet
Bill and Kelly King
Gary Kirch
Terri Klein
Brian and Kris Klenke*
Fred and Joyce Koehler
John and Joyce Lane
William and Lynda Langin
Steven and Camille Lanier
Ron and Monica Leone
Joan and Mark Lessing
Kathryn and Hai Lieu
Rigo and Imelda Luna
Misty Luthcke
Jason Madison
Stephen and Dorothy Mask
Patricia and Richard McCann
Stephen and Marie McClurg
Susan McDonough
Janine and Jeff McGuire
Mr. and Mrs. Arthur McLendon
William and Karen McNeil
Michael and Anita Muonio
Austin and Dorothy Noll Jr.
Thomas and Vickie Patterson
Sherrie Roman
Sourchi and Thavy Sasry
David and Rebecca Silkey*
Tami Slawson
Mike and Barbara Smith
Chad South
Thomas and Elizabeth Sowden
Larry and Tracy Szemanski
Noel and Nancy Talcott
Robert and Anita Taulton
Janet Thomas, MD
USA Couriers
Veronica and Michael Vacca
Helen and Bill Vespe*
Sent and Mary Visser
Charles Vite
Kathryn and James Ward*
Marie Wegener
Donald and Lisa Wells*
Elaine and Hubert Willman
Gordon Wingate
Donna Sue Woods
Raymond Zechender

** Annual Fund donor*

Upcoming Events

Disney Family Conference

Dec. 17–20, 2009

The National MPS Society will celebrate its 35th anniversary during the opening evening of the Disney conference, Dec. 17. A wide range of topics and speakers are planned for Friday, Dec. 18, ending with the awards banquet. The remainder of the weekend will be free for families to enjoy the parks, including evening fireworks. Thanks for your ideas about how we can improve the conference. Registration materials have been sent and are posted on the Web site. Contact Laurie Turner at laurie@mpssociety.org with questions.

11th International Symposium on Mucopolysaccharide and Related Diseases

June 23–27, 2010
Adelaide, South Australia
"Translating Research into Clinical Reality"

Hosted by Lysosomal Diseases Australia, Mucopolysaccharide & Related Diseases Society Aust. Ltd. and Lysosomal Diseases New Zealand, this international conference will include exciting scientific and family programs that will focus on the areas of newborn screening, prognostics, understanding pathology and therapeutic options. Genuine opportunities for thorough discussion and debate, for both academics and families, will be featured. For more information, visit **www.mps2010.com.au**.

The **Ohio 2009 family gathering**, "Fantastic Mexican Fiesta," will be held from noon to 4:00 p.m., July 25, 2009, at Northwest Bible Church, 6639 Scioto Darby Road, Hilliard, OH 43026.

If you plan to attend, please RSVP to mwojnaro@columbus.rr.com or by phone to Rachel Wojnarowski at 614.529.3775.

Board of directors tackled wide array of issues

The board of directors of the National MPS Society meets three times a year, as stated in the bylaws. This diligent group of dedicated members strives to fulfill the Society's mission of finding a cure for MPS and related diseases. The following overview of the 2008 meetings summarizes how your board is directing resources to efficiently and effectively support research and support individuals and families affected by MPS. A complete copy of the minutes can be obtained by contacting the office.

Seated: Alice Martin, Debbie Dummann, Kim Frye

Standing: Amy Holland, Steve Holland, Kim Wigglesworth, Klane White, Amy White, Ernie Dummann, Angela Guajardo, Sharon Cochenour, Anne Gniazdowski, Kris Klenke, Steve Chesser, Tom Gniazdowski, Larry Kirch

Not pictured: Luis Guajardo, Stephen Frye, Sissi Langford

The first meeting of the year was held in Las Vegas, NV, Feb. 15–16, 2008. The meeting followed the Lysosomal Disease Network meeting which several of the board members attended.

- Discussed development of a formal structure to quantify staff evaluations.
- Preliminary plans reviewed for International MPS Day include several live events throughout the country.
- Development director presented list of funding proposals that have been approved and long-term funding goals.
- Approved funding for a new Family Support program.
- Approved development of a partnership MPS III grant with the Society and MPS III family foundations.
- Approved policy to offer a one-year complimentary membership to newly diagnosed families.
- Approved allocating funding for the September 2008 CYCLE conference and additional funds for scholarships.
- Approved scholarship for a MPS IV family to attend the U.K. Morquio conference in August.
- Approved 2008 budget.

The second board meeting was held June 29, 2008, following the International MPS Symposium in Vancouver, Canada.

- Issue raised/discussed about donor reports of not receiving thank you letters, and necessary office quality control measures that include timely deposits and communication of financial information.
- Development director assured that funding for updating the MPS video will be approved.

continued

- Strategic plan was reviewed and committee chairs will meet with their committees to make updates and changes to their specific sections.
- Board pledge form was presented and discussed.
- The following syndrome booklets were updated and printed: MPS I, MPS II, MPS III, MPS IV and MPS VI.
- Presented information about the two companies, Vivendy and BioMarin, developing enzyme replacement therapy for MPS IV.
- Family Support Committee received 24 applications for the 20 available education scholarships and provided 34 stipends to families attending the International MPS Symposium.
- Approved Office Procedure Policy.
- Approved $4,000 funding for Genistein research project, a collaborative funding through the International MPS Network.
- Approved Scientific Advisory Board review committee funding recommendations for 2008 research grants.
- Approved Los Angeles, CA, as the location for the 2010 family conference.
- Approved the executive director evaluation forms.
- Approved the new Extraordinary Experiences program.
- Approved the slate of officers for the term beginning 1/09 and ending 12/10: Ernie Dummann, president; Kris Klenke, vice president; Angela Guajardo, treasurer; Kim Frye, secretary.

The final board meeting in 2008 was held Oct. 24–25 in Raleigh, NC.

- Welcomed new board members whose terms began Jan. 1, 2009: Mary Ellen Pendleton, Tami Slawson and Kim Whitecotton.
- Decision was made to change payroll companies and to obtain a VISA credit card. Currently the Society uses American Express, which some vendors do not accept.
- Intellectual property rights between the Society and University of Iowa (Society funded research of Dr. Ellinwood) is being reviewed by our non-profit attorney.
- The MPS III resource guide developed by the Society is completed.
- Presented option for the 2008 Form 990 to be completed by the same CPA firm that does our audit.
- Plans for International MPS Day 2009 include sending branded luggage tags to all members.
- Dr. Klane White and Kim Wigglesworth attended the National Institutes of Health-sponsored meeting on advances of rare bone diseases.
- Mixed feedback was received from the CYCLE conference as to whether the next conference should be a stand-alone conference or in conjunction with a family conference.
- Lengthy discussion held about the Disney conference and the issue of families joining or renewing their membership just for the year with the Disney conference.
- Development director presented that the Society is on target for 2008 development goals, specifically grants that have been approved.
- Farewells and thank yous for their service as board members to Larry Kirch, Alice Martin and Sissi Langford, all of whom did not run for reelection.
- Approved registration fees for Disney 2009 conference and early bird fees.
- Approved moving some Society funds to Merrill Lynch (from the current CD holdings in Bank of America) for investment CDs in order to diversify.
- Approved increasing funds to the local bank account since the executive director pays the majority of invoices. This resulted in an amended Financial Policy which was approved.
- Approved two new policies: Whistleblower and Executive Compensation, both of which are required by the new 990 form.

National MPS Society Financial Report Summary

The Society annually obtains an audit of its financial activity and files a Form 990 with the IRS. For additional information, contact the Society's office.

	2008 Actual*	2009 Budget
BEGINNING CASH BALANCES		
Restricted for Endowment, Research and Family Assistance	$ 1,616,700	$ 1,524,380
Unrestricted	736,770	668,206
INFLOWS		
Annual Fund	68,868	70,000
Conference Income	138,257	130,000
Donations/Fundraisers — General	125,777	111,500
Donations/Fundraisers — Family Assistance	19,048	18,000
Donations/Fundraisers — Research	155,201	70,000
Dues	37,829	40,000
Interest	62,291	38,000
Operating Grant/Sponsored Revenue	149,500	87,500
Corp Gifts/Foundation Grants/Major Gifts/Endowment	209,000	275,000
Run/Walk Research Fundraiser	310,861	300,000
Sale Items	1,744	3,000
TOTAL INFLOWS	**$ 1,277,776**	**$ 1,143,000**
OUTFLOWS		
Administrative	$ 34,641	$ 41,500
Bereavement	17,719	5,000
Conference	308,886	348,500
Education — Newsletters, Booklets, Web Page	144,693	82,000
Family Assistance — Direct	31,263	48,500
Fundraising	60,781	53,500
Legislative	6,772	10,000
Membership Database and Directory	14,900	14,500
Office and Equipment	47,126	46,000
Personnel	244,122	238,637
Research	527,757	477,000
TOTAL OUTFLOWS	**$ 1,438,660**	**$ 1,365,137**
ENDING CASH BALANCES		
Restricted for Endowment, Research and Family Assistance	$ 1,524,380	$ 1,462,381
Unrestricted	668,206	508,069

* 2008 Unaudited data

Calling all artists!

If you have artistic talent and an interest in donating your time to the Education & Publicity Committee, we need you. We are looking for talented individuals who can collaborate on various projects and publications currently in progress. If you are a painter, illustrator or photographer with a desire to help compliment our words with images, please submit a work sample to kim.frye@mpssociety.org. Please include a statement of how you can help, your availability and your artistic method (painter, illustrator, photographer). We look forward to hearing from you!

We are so grateful to the Family Assistance program for helping us get a three-wheel bike for Jerry (MPS I)! He is really enjoying it (see photo on page 16). He was a bit nervous about trying the bike because he has had bad experiences with two-wheel bikes, but he was so happy to see that he could start pedaling by himself and that he did not have to worry about falling off when he stops. We recently rode one mile to the grocery store, then to McDonald's for lunch. He is feeling much more independent. Thank you!

Katherine Klemm

This is a photo of Kasey and her service dog, Odie. Odie was trained to serve Kasey and her guardian in 2001. We always thought Kasey's disability was organic in nature; she was diagnosed with MPS III in June 2008. She is now 16 and has returned to school after a long year of problems.

Ted and Susan McCannon
(Kasey's grandparents and guardians)

Fourteen-year-old Chelsey Montgomery, sister of Lucas (MPS III), was recognized as volunteer of the year at her school. She will travel to Washington, DC, in May for the national ceremony where she could win $5,000 for the charity of her choice. To see Chelsey's video about why she volunteers that was submitted for the ceremony, go to **www.youtube.com/watch?v=94fEN5ZmMX0**.

Our Little Angel

You don't know her pain because of the way she sings and plays.
She is always singing a song.
She can't say the words but she knows the tunes,
In the nursery rhymes.
She is like a breath of fresh air when she awakes each day.
When you look into her eyes, you can feel the love in her heart.
If the world could know the love and strength of this little girl,
They would know what happiness really is!

The Dula family and Maw-Maw Price,
written for Madison Dula (MPS III)

A Tale of Two Parents

Let's pretend there are two kinds of disease: Blue disease and Red disease. Both are made up in my mind to create an example and are not intended to resemble any real-life childhood afflictions but can resemble many different types of anomalies that afflict our kids. I am making generalizations here with the consideration that we are talking about imaginary sick children and their parents and the establishments that deal with them. Just as everyone's experience can be unique and personal to them, this one is my own.

Let's pretend that Blue disease is a childhood illness that everyone is aware of. Blue disease isn't rare, but it's uncommon. The child suffers relentlessly, but is able to keep about them their cognitive aspects. They can play games with their parents, draw pictures to express their feelings, and even on very good days live an almost idealist childhood experience like attending school, making friends or visiting a theme park. The Blue child has a 50/50 chance of surviving this disease, so plans are made for their future in case the sun does shine upon them.

Everyone knows of at least one child affected by Blue disease and those people banded together and worked together and formed projects, foundations and coalitions to fight Blue disease. Because the disease is so well known, there is more money being offered for research, and the research itself is promising. Many agencies know that half of the children survive and go on to lead normal, productive lives, so there are always survivor stories to present and share with the affected families to give them hope.

Take this a bit further and imagine there is no actual medicine to cure Blue disease per se, but the researchers, doctors and foundations that work with kids who have Blue disease support and recommend various remedies and therapies that might help improve the chances of recovery. As the parent of the child with Blue disease you would get right down to business and you would try anything to save your child's life or at least enhance your quality of time together. You would exhaust yourself trying everything that was offered in clinical trials, magazines, newspaper articles and word of mouth.

It would be hard to not know of some hospital or doctor who doesn't oversee a patient with some form of Blue disease. The parents of the Blue diseased children can seek knowledgeable medical personnel easily, because, in most cases, the medical gurus are the gatekeepers to a kingdom of medical services that can lie ahead of you if you know all the right passwords. Along the way you could easily call one of many local agencies and explain your circumstances. Help would be on the way, because everyone would already know what a horrible state Blue disease could be to live with. Therapies would be offered, recreational facilities would be listed, and many other parents of Blue children could be contacted.

Now on the flip side, you thank your lucky stars that your child doesn't have Red disease. Because Red disease is unknown clinically, most of the time there is nothing to offer the families that have Red diseased children. The disease itself is difficult to recognize and many times may go undiagnosed or misdiagnosed. The parents of course are just as desperate as the parents of the Blue diseased children but they are offered nothing, because there is nothing. Being as desperate as the Red parents, they too search, but in vain: on the Internet, through shelves and shelves of medical journals and calling specialists all over the world.

Parents of children with Red disease are ready to work just as hard as Blue parents. They form coalitions, foundations and raise huge amounts of money for research. They meet families and connect over the phone or the Internet and occasionally through social or personal events. It is more difficult because Red families are spread further apart due to the incidence of the disease. They feel lonely by themselves with only electronic devices to purge their feelings into. In the world of Red there tends to be more heroes lost in war than battle scars worn in victory over Red disease.

The Red parent searches through local and state agencies and pulls information they think may be useful and begins to share. No one has ever heard of Red disease…the burden of proof and explanation relies then on the parent. So parents

continued

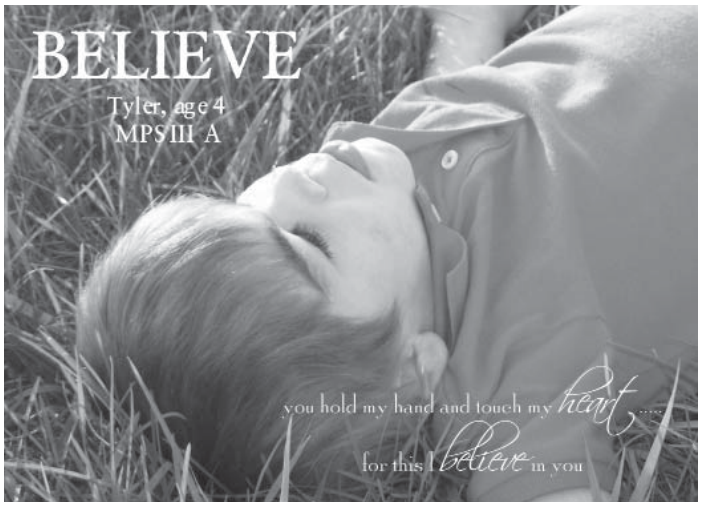
YOU ARE IMPORTANT TO US, PLEASE KEEP IN TOUCH.
Please remember to let the Society know if you are moving. In addition to helping keep printing and postage costs down, you'll help us keep our database up-to-date. Keep us informed of new addresses, telephone numbers, e-mail addresses, and any interesting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

with Red children spend innumerable hours explaining the disease and copying medical records. They send booklets of information, along with laundry lists of specialists and phone numbers, to anyone who will listen. Many times when asking for services or equipment for the Red child after numerous piles of proof have been submitted, the request is denied because, frankly the agencies have no idea what they are dealing with.

Living solely inside a Red world, albeit comforting, is not always an acceptable means of existing for most families. They usually have outside commitments like work, possibly other children, maybe to their communities, but especially to each other and to themselves. Red parents have no choice then but to demand more of themselves and create extensions of themselves to reach out to others. Red parents are constantly choosing between the responsibility to their child and to others. Fluctuating between humility, gratitude and guilt, they are driven by circumstance fueled by emotion. They are forced to shed layers of themselves; letting go of relationships with friends, family, each other and even within themselves to maintain the delicate dance of obligation and longing.

Yet while the Red parents set about to live in sync between their Red world and the typically colored world, their children cannot be ignored! Red children cannot be easily set in front of a TV or a video game as mommy and daddy set about to work and strategize. Most Red children can not be left alone, if even for a second. The Red child demands the attention of the most patient and positive of caregivers. No longer can a Red parent employ the services and the affordability of the 15-year-old neighborhood babysitter. New demands are constantly arising as the Red child is losing touch with their world around them. Safety awareness for them is gone as well as reasoning and understanding of the family life that once was. Red children barely sleep and are plagued with seizures and episodes of screaming and crying as well as violent behavior. Many Red children do not perform on command; neither do their toileting habits. Everything within reach of the Red child is chewed or thrown, pulled or bit through, including themselves and others. There is a never-ending trail of pain, destruction and debris along their path.

Red parents wait in desperation, sometimes for months, for appointments for any medical doctor who has even seen a child like theirs. They travel hundreds of miles with their Red children who are uncomfortable being confined in their outgrown



Tyler Langan (MPS III)

car seats, only to see the famous wizards of Red disease for but a moment. When they arrive at the appointments, the medical personnel look at the families and their Red infected children and shake their heads; there is pity in their eyes as they reiterate the facts that the child with Red disease will progressively degenerate in every way possible before your very eyes, losing speech and ways to communicate with you, losing sensations and feelings so they can only bite, kick, scratch and scream. Eventually the Red kids lose the ability to walk, run and play, along with the ability to eat, drink and swallow, all while growing up in front of you. There is sympathy in their voice as they instruct the parents to go home and deal with the tragedy as best they can, for there is nothing they can do for you today. But they assure the parents and siblings that they are doing a marvelous job along the way—they are to be commended for their efforts—keep up the good work!

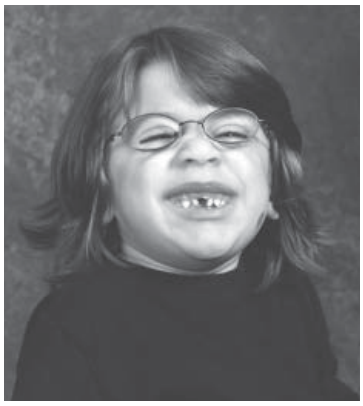
But the parents plead that there has to be something! Diet, behavior modification, prophylactic antibiotics —anything! The parents promise to be good, to give up their lives if only they had something to go on. But the gurus of Red just shake their

continued

heads, the reports, if any, are misleading…nothing has been proven scientifically. There is no research working on this that they know of. I'm sorry, there is no known remedy to stop it, reverse it or even slow it down. The Red parents are disheartened and feel like failures; they smile through the tears and thank the medical professionals for their time.

Red parents sit in silence as they embark on their long journey home with their sick child. They are educated parents; they should be able to find something, but their inner thoughts conflict with the doctor's spoken words that there is nothing to be done. The parents stare at their Red child asleep in the backseat. So sweet, so innocent, so beautiful…this child never even had a chance. It is such a cruel fate, how can this be happening to us, they think. There is no fairy tale ending in this story they suddenly realize; it is only the very beginning of their untimely end.

Donna Kay Langan (mom to Tyler, MPS III)



Zach Haggett (ML)

Carmelia Panghulan with her daughter, Erica (MPS III), who celebrated her 18th birthday surrounded by other MPS families including the Nolls, Bennetts, Gabriels and Offenbackers.

Nick Boyce (MPS I) going to his junior prom

Trevor Ramsey (MPS II)

My story: How I feel about my illness

by *Eddie Bell Jr.* (MPS I)

I feel I was deprived; an innocent bystander. Sometimes I get sick, depressed, frustrated and angry. I want to do things that my friends and family members can do. I get real ticked off because I used to have good vision and now I can't see or read like I used to. I can't drive anymore, I couldn't see well enough to pass the driver's eye exam. It frustrates me to see my parents look at me and see what I go through on a daily basis by me just being me.

I try to hide that I'm really not happy, but I try to make myself happy so that I won't be a burden on anybody. It's a lot of things that comes with having a critical illness that you've been faced with all your life. It's my life and it's something that I've adjusted to. After years of growing up, I didn't understand what was going on with me. It didn't look like anything was wrong with me, but now that I've gotten older and I broke my hip, while going through all kinds of disappointment, I notice the change in me. It's not good for me to get depressed. It seems like my illness is getting worse, so I try to keep a positive mind and keep my head up.

Eddie Bell (MPS I)

It's a lot of things I want, it's a lot of things I want to do, and it's a lot of things I need. It's a struggle trying to do just the simple things such as putting your hands behind your head. I try to encourage people, letting them know that everything is going to be alright not matter what you are faced with. Just keep your head up, take the good with the bad, all in stride.

For me to get through what I'm going through I always pray. I surround myself with friends because I don't get to be around most of my family members. I don't feel accepted by them. The people I grew up with are the ones who accept me and they make sure I'm alright. They watch out for me and they come and get me out of the house.

Dealing with this illness is on a whole different level, like a whole different world for someone to adapt to. I was raised as if I was normal just like everybody else. You don't know how you look until you look into the mirror. So when I walk into the world without looking into the mirror, I don't see what I have. I just see me being me. I see myself like everybody else. I just keep on praying and hoping that my life will get better.

MPS I is a hard pill to swallow, but I have to swallow it. I ask myself, "Why me? Why do I have to be the one dealing with all this suffering, this burden?"

I would like to say, for those with this disease or any other life-threatening illness, one day things will get better. I feel that if my illness is not going to get any better, then something good has to happen for me. I don't like going through this. I don't like to be looked at as being different or being judged by my outward appearance. That's the way I feel about my illness.

Kraig Klenke (MPS II) and mother, Kris, playing Challenger baseball

Zain Semones (MPS II) and his sister Star

Jerry Klemm (MPS I)

Joey Naporano (MPS II)

Allison Kirch (MPS III)

Morgan and Blake McDermott (MPS III)

A Warm Welcome

Take a moment to learn about new Society families. **A Warm Welcome** introduces new members and offers yet another chance for members to connect with one another. If you have the time, 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 Mike and Cami Lane who live in Chandler, AZ, with their three sons—Drake (8), Broc (7) and Trey (MPS IV A) who turned 4 this past April.

Mike has been a police officer for the City of Tempe for the past 12 years. Cami is a flight attendant for Southwest Airlines.

Drake is a second grader who enjoys art, music and sports. He participates in soccer, wrestling, flag football and swimming. Swimming starts the week after school lets out where Drake will defend his second-place state record in the breaststroke. As the oldest of three brothers, Drake has proven to be the brightest and best big brother around. He is so protective of his "baby" Trey that most times mom calls him "brother hen." Drake wants to be a professional football player when he grows up and you better believe we will nurture that aspiration…maybe the payout for such a lucrative career could fund a cure for his "baby"?!

Broc just completed the first grade. Being the middle brother, Broc enjoys the best of both worlds—having a big and little brother. Broc always plays so sweet with his little brother Trey and forever includes him in all his fun. Broc is a gifted athlete who holds his own, "playing up" in age on his big brother's athletic teams. In addition to being one of the fastest kids in the neighborhood in all age groups, Broc enjoys the outdoors, always with his shoes off. When asked what he wants to be when he grows up, Broc will answer a veterinarian every time.

And then came "Mr. Busy" Michael "Trey" Lane. Misdiagnosed at the age of 2 with MPS VI, Trey has gone through more than most adults will in a lifetime. After enduring 43 infusions of Naglazyme™ and a multitude of tests, just days before his 4th birthday Trey was correctly diagnosed with MPS IV A.

Trey is your typical preschooler who loves his peanut butter and jelly sandwiches, riding on his electric "racer," Mickey Mouse, the Arizona Cardinals, his "Cardinal birdies" and his new love…swim lessons. Aside from the usual life most 4-year-olds live, Trey also attends physical therapy twice a week, and a new approach to brain/hand coordination type therapy once a week. Trey's brothers, mommy and daddy also incorporate physical therapy into his everyday playtime which is the "sly" approach to "work" for a child with such a bone disorder. Trey also is a member of HopeKids, which affords our entire family local events free of charge as a way of instilling hope to children with life-threatening illnesses.

The Lane family

Although Trey stopped growing at about 2 1/2 years old, he is head and shoulders above the rest. Trey is such a kind and compassionate soul. We believe God knew exactly what he was doing when he blessed this family with Michael Trey. Trey makes our world go around, he brings a smile to everyone's face he encounters and is the best baby brother two little guys could ever, ever ask for. According to daddy, "He is my little bit of somethin' and a lotta bit of everything."

Together with our family and friends we started a non-profit for Trey known as "Trey's Treasures." With our family, friends and the community behind us we have accomplished grand things. Our fundraisers have included our very own "Trey's Treasures" golf tournament, "Vegas Nights" with Spark of Hope, watermelon slices on the 4th of July, Usborne books sale, mini pumpkins for a mega cause, Trey's Treasure Chest for Hope dinner auction, Take Down for

continued

Trey, a neighborhood garage sale, Silpada jewelry party, a swim-a-thon and car wash, WAMU grand opening dedication and fundraiser, Carebear preschool fundraiser, a golf tournament in honor of my sister-in-law's late father, a chili cook-off, Tempe Police Citizens Academy donations, Avon sales fundraiser, the Hard Chargers motorcycle ride, children who have raised money for Trey in lieu of birthday presents, selling lemonade and popcorn, along with giving their winnings in contests to Trey's medical fund, and last but not least a kindergarten class that had 25 trees planted in a national forest in honor of Trey! This year we are gearing up for another "Trey's Treasure Chest for Hope" second annual dinner auction and "Dunkin Cops" dunk booth at the Tempe Town Lakes Oktoberfest. Due to the head- and heartache that came with the misdiagnosis, we weren't up to the 2nd Annual Trey's Treasures golf tournament but have high hopes for next year.

Along with all the fundraisers, we also have been blessed to have Trey's journey catch the attention of the media. Trey's story was told in numerous newspapers and many local television stations. Is it those eyes or those curls? We don't know, but what we do know is that whatever it is, Trey's sweet looks have helped us spread the word and raise awareness of MPS (Trey's blog alone gets an average of 20 hits a day from North America as well as countries far and wide). After just one article in the state newspaper, we were blessed to be brought together with Taylor (MPS VI) and his family as well as the honor of meeting one of the MPS Society board of directors, MaryEllen Pendleton and her family. This media attention also bestowed us with the generosity of a few local gentlemen. One of these men provided Trey with free full-body massages at every infusion he underwent in the hospital, the next provides us free photo processing until a cure is found and the other graces our family with free car repairs and service indefinitely. These blessings come full circle and make myself as well as my husband, who as a police officer doesn't often get to see the good in society, by opening our eyes to the greatness in people and the love and concern they have for their family member, friend and child who is a complete stranger to them but who, just the same, is a part of their community.

Our story wouldn't be complete without mentioning our own "Patch Adams," Dr. Chet Whitley at the University of Minnesota. We would still be spinning if it weren't for him and his team of astounding colleagues. The level of care and concern, the attention to detail, the infinite amount of time he gives his patients are without question a representation of the best of the best. The MPS family is so lucky to have such a brilliant man on their team and we are so privileged to call him Trey's doctor.

My strength not only comes from those near to me, but although far, oh so close to my heart—my new and dear friends who are the parents of children with MPS. Without Margo (Nathan MPS VI), Kaylene (Taylor MPS VI), Jenny (Holden MPS VI), Ellen (Isaac MPS VI), Jen (Eddie MPS IV), Cassandra (Payton MPS II), Darla (Kikki and Justin MPS IV), and Mark (Ryan MPS I) to bounce thoughts, cares, concerns and tears off of, I don't know how strong of a mom I could be for my baby Trey. I have come to recognize that the families of MPS are beyond an amazing group.

Our "normal" will never be the same. In order for our family to survive, we must find a new normal, whatever "normal" is anymore. In Don Piper's words, "The new normal is about living to bless. We can be a victim or a victor. It's a decision; it won't come naturally. Take your tragedy and turn it into triumph. Take your test and turn it into testimony. Take your disappointments and turn them into divine appointments. It's a decision. It's a choice. It's not what you go through but what you do with it that matters." Life is never problem-free. But when the bottom falls out, when tragedy strikes, then healing requires a change of perspective. Healing means you've got to find a new normal.

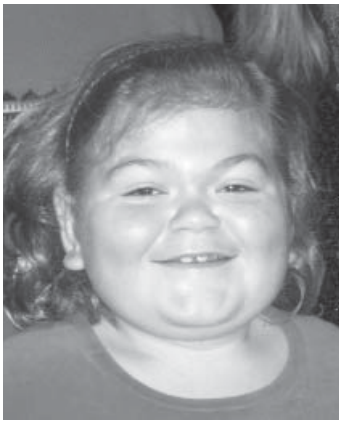
Thank you MPS Society for hearing our story and for all you do for each and every family fighting this fight.

To read more about Trey's journey, go to **www.ourtreystreasures.blogspot.com**.

National MPS Society Announces New 'Standing Ovation' Award

The new 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:



Molly Birmingham, 9, MPS I

Molly is a bright, personable 9-year-old girl. She has an infectious laugh and a silly sense of humor. Molly's must-see TV shows are Hannah Montana and iCarly. She also loves to watch the Food Network, especially Paula Deen. Her favorite foods are mac 'n cheese, grilled cheese and anything chocolate.

Molly sings in a children's church choir and has participated in local children's theater productions of Annie, High School Musical 2, Seussical and Mary Poppins…from Barney to Camp Rock, singing has always been one of Molly's favorite things!

A special quality Molly has is her concern for others—whenever anyone is sad or hurt, Molly is first in line to help comfort them. She is very sensitive and kind to everyone. The best thing about Molly is her hugs—she shares them generously and lovingly.

Nicholas Zubia, 12, MPS II

Nicholas John Kenneth Zubia was born in Albuquerque, NM, on May 4, 1997. He was diagnosed at the age of 5 $^{1}/_{2}$ after multiple ear infections and other health issues. He has four sisters and two brothers. His 8-year-old brother also has MPS II. We moved to Las Vegas in 2003 to be closer to family. In January 2004, Nicholas became a participant of the clinical trials in Oakland, CA. He enjoyed weekly flights and staying at the hotel, but most of all his time with his new friend, Samuel. This was one of his greatest accomplishments. To him, it meant his brother could now receive weekly enzyme replacement therapy. But now he sees it as everyone can receive the drug. We moved to the Bay Area in 2006 to be closer to Children's Hospital Oakland and the doctors. Nicholas is a sweet and very intelligent young man and has lots of friends. He's a Boy Scout and is learning a lot from his scout master and his troop. He's experiencing camping and other fun things. He's the type of child who isn't going to let MPS take over his life. He is strong and happy and loves everything and everyone around him. Thank you for this award. It's an honor.

Kasandra Belle Offenbacker, 7, MPS III

Kasandra, better known as Kassi Belle, was born Nov. 10, 2001, and we knew right away we had our hands full. Kassi was overflowing with a curious free spirit ready to change the world. Her first steps were her first run and once she started it was almost impossible to stop her. When she got something in her sights it was as good as done. Kassi gives so much to everyone who meets her and her inspiration has been undeniable.

Kassi enjoys a wide range of activities from eating to sitting outdoors and just enjoys being there. She loves all kinds of animals but dogs and horses are her favorites. She loves her dogs so much that she wanted to be one as a toddler. It was a constant struggle to keep her from eating from the dog bowls or to stop her from crawling out the doggy door. One of our favorite memories of Kassi was when she was 4 we went to visit her uncle Dan's horses in Phoenix. After a day of petting and feeding the horses we went inside but Kassi decided she wasn't done visiting. She had found her way out the doggy door and when we found her she was in the horse corral with a young horse who was being abnormally gentle.

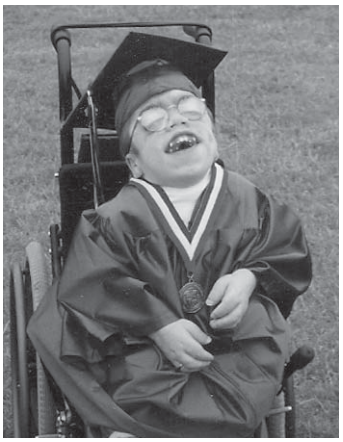
She also loves to watch movies and even though she doesn't talk as much or as well as she used to she can still recite a great deal of "Ice Age," which is her favorite of all time. Some of Kassi's favorite foods are pizza, rice and spaghetti.

Kassi has given so much to our family and taught us so much as parents. She has been nothing short of an inspiration and has changed our lives in every aspect. Her absolute honesty and ability to forgive are always there to remind us of their importance. Even as mean as other children have been and even sometimes adults, Kassi still wants to share with them. Kassi is always herself—you never have to second guess her mood and she never cares what other people think. There is so much beauty in her simplicity that it has shaped our priorities both as a family and personally. We get caught up in having something fun to do and having somewhere to go, but when we get there Kassi is happy just being in the sun sitting on the beach, in the grass, or on a snow bank taking in the beauty and being with her family. She reminds us to enjoy the simpler side of life and to place more value on the beauty of where you are and the company you share it with rather than what you have and what you're doing.

You can't spend time with Kassi without her affecting you in some way. If you happen to see us in the bay area of northern California come tell her hi. She'll probably want to hold your hand and share a drink with you, but I can guarantee when she starts laughing you'll find yourself laughing right along with her. As a parent you want to teach your children all the lessons you have learned and your values. Kassi has taught me so much and has changed my values to make me a better person. All of this is why we share Kassi with the world; not because we want to share but because she has so much to give.

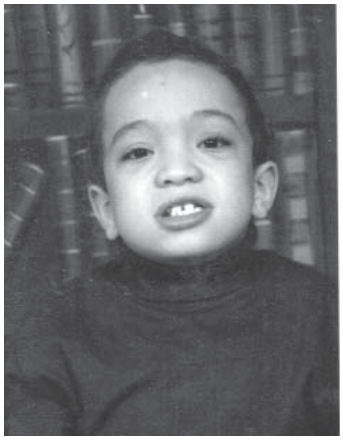
John Scott, 19, MPS IV

My name is John Scott and I am a college freshman at Christopher Newport University in Newport News, VA. I have Morquio syndrome but that is not who I am. It has affected me but has not defined me. I have felt the thrill of success both in overcoming my disability and succeeding in other areas of my life. I love the challenge that school and independent living in college brings. I have been stretched and changed beyond anything I have ever experienced before. I find joy in being able to live at college and succeed in college even though before I thought it might be impossible. When I was younger I remember not worrying much about the future and what it held. I enjoyed swimming and playing video games with my friends. I still enjoy both those activities today and cannot wait until the semester is over and I can swim once more in my pool. I gain much strength from the relationships I have with my family and friends. My strength also comes from my relationship with God. I have found much joy in community in my church and Christian fellowship on campus. It has been a great means of strength knowing that God is in control of my life and that He has a direction and plan for my life. My greatest achievement in life so far has been using my struggles and difficulties to inspire my friends around me. It has been great to hear how my disability is not going unnoticed in a positive way and to show people how to have a joyful outlook on life in the midst of hardship.



Kristofer Arnold, 27, MPS VI

I love sports and my favorite teams are the New York Yankees and the Dallas Cowboys. I also enjoy watching WWE Wrestling and have since I was 3 years old. I used to love going to Yankee games and traveling. My mom and my "bonus" dad Tony have taken me to all of the United States except five. I graduated high school with honors and also was going to community college. But three years ago I got very sick and it is difficult to travel now because I'm home on a ventilator. I still like to go to the Mohegan Sun Casino once in a great while to play the slot machines. I have been very fortunate and have gone to my senior prom, and I met the New York Yankees and the Dallas Cowboys a few years back. I also got to ride in a NASCAR race car. My greatest accomplishment was winning my fifth grade spelling bee. Everyone thought one of the girls in my class who was very intelligent would win. But my name is on the plaque as the 1993 winner. Thank you for honoring me with this Standing Ovation award.



Peter Martinez Jr., 9, MPS VII

Ever since Peter was 2 years old he loved to play with his action figures. He also enjoys watching cartoons and he loves to sing. Peter is happy when he is close to his parents and big brothers. Peter is a very strong, brave, happy little boy. Whenever sick, down, or weak and in pain he keeps himself energized and full of joy. When Peter sees someone sad he has a way of taking that sad feeling away. As his mother I believe Peter is a messenger with a gift and a purpose.

Peter's biggest accomplishment is remembering his brother's name and stepdad's name. At school he learns to say his numbers and alphabet. Peter also is very smart using the remote control.

Anna James, 13, ML

I was diagnosed with ML when I was 4 years old. I don't remember this very well but I do remember that I used to be able to walk and run and I miss not being able to do that anymore. My favorite thing to do is to read. I love reading adventure stories that are exciting. I also go to equine-assisted therapy and I love riding Levi, a brown and white pony. He is very old but he is always happy to see me and is very gentle. My dog Tess makes me very happy. I got her two years ago and she is a support dog. She makes me laugh a lot and she is my friend. I love my family very much. They make me so happy and I love them with all my heart.

From Anna's mom: Anna's teachers at school call her Little Miss Sunshine. No matter the pain, no matter the circumstance, Anna always has the biggest, sunniest smile all over her face. Anna loves life and she loves everyone around her; those around her have been truly affected by her spirit. Anna has accomplished so much in her short life. I don't know if you could say there was one accomplishment better than another, but she has tackled her disease with extraordinary grace and strength. When she sees others sick or struggling she is moved to tears and tries to help in any way she can. We love her so much and could not be prouder of her.

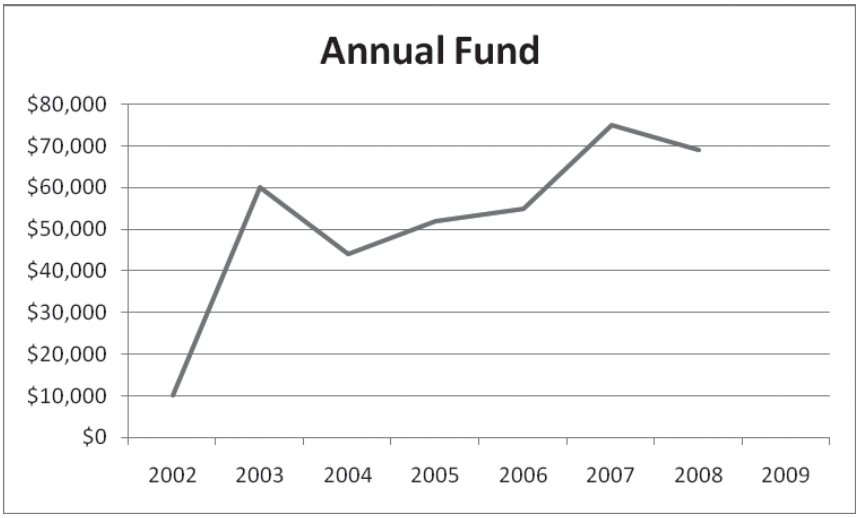
2008 Annual Fund Results

In 2008, the Society completed its seventh Annual Fund campaign raising $69,000. Over the past seven years you helped raise more than $365,000 to sustain the mission of the National MPS Society. In 2002, the first year of the Annual Fund, a total of $10,000 was raised; now the Annual Fund consistently raises more than $60,000 yearly. With your help and through the leadership of the board of directors and the Fundraising Committee, it is hoped that this amount continues to increase each and every year. The success of the campaign is primarily because of the generosity of members and special donors who best know the wonderful work of the National MPS Society. As we kick off the 2009 Annual Fund later this summer, please consider making a gift to this important campaign.

The Annual Fund is a significant source of funds for our Family Assistance Program, legislative program and our general operating expenses. Much of the fundraising that takes place by members includes family fundraisers and walk/run events. Giving by individuals outside of these events also is important. The original intent of the Annual Fund remains the same, it gives those of us who might not be able to host a fundraiser the opportunity to make a personal gift on an annual basis to these important aspects of the mission of the Society.

A sincere thank you to all who contributed to the 2008 Annual Fund!

Larry Kirch
Chair, 2009 Annual Fund



Fundraising Committee:
Steve Holland, chair
Steve Chesser
Ernie Dummann
Steven Frye
Anne Gniazdowski
Angela Guajardo
Larry Kirch
Terri Klein
MaryEllen Pendleton
Naureen Sayani
Laurie Turner
Barbara Wedehase
Amy White

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.

Hosting a golf outing as easy as 1-2-3

Hosting a golf outing can be an excellent way to raise funds and awareness of MPS. Family and friends usually rally around this type of event, but some fundraising organizers feel that the event should involve something you have an interest in as well as be compatible with the interests of family and friends.

1. **Select the date and location.** These are the most important first steps. Be aware of off- and on-seasons for golf in your area. The best deals may be found during off-season but courses are not always in the best shape. However, being a fundraiser, a better price may outweigh this. Look at how often you feel you can tap into your friend support network. You can easily make $10,000–$25,000 a tournament, but not if you expect the backing of the same supporters. Events held every 18 months is a good timeframe.

2. **Build a committee of organizers.** The first year is the most difficult to organize. Breaking down tasks into committees headed by one or two people is most efficient. Suggested committees include:

   - **Golf course negotiator/communications with golf course.** The person handling this should try to negotiate for as many extras as possible. This is especially important in today's economy. Golf courses should be willing to supply practice balls, free rounds of golf (for prizes), etc. This also holds true when negotiating with catering staff for a luncheon. Feel free to tell courses that you are negotiating with other courses.

   - **Person(s) to set up spread sheets and maintain lists of golfers and donors.** This person should have the best computer skills of the group. Accuracy in maintaining lists of golfers and donors will make sending thank you notes a much easier task.

   - **Silent auction/raffle.** Everyone can help with obtaining items for an auction and raffle, but keeping items in a central location allows for better organization. It is important to have items in every price range. It can be a stretch for some friends to pay the greens fees, so keeping some auction items in the $10–$50 range allows them to bid comfortably. Grouping like items together can generate more funds.

   - **Collecting money and making deposits.** Set up a separate checking account with the name of the tournament; this makes depositing checks very easy. Make photo copies of all checks. Addresses and phone numbers from checks are important for recordkeeping and thank you notes.

   - **Hole sponsors/extras.** This is the best money making aspect of a tournament. You can have many levels of sponsors. Each hole can have a separate sign listing the level and names of sponsors. (i.e., gold, silver, etc.) Sponsors should be listed prominently on posters, flyers, etc. Additional extra games are a source of fun and funding. Sell mulligans, and have a closest-to-the-pin and longest drive contest.

It is useful to have monthly meetings for four to six months prior to your event, then switch to bi-weekly, then weekly. Meetings can be done by phone or e-mail; communication is essential so you are not duplicating efforts.

3. **Relax and have fun the day of the tournament.**

The National MPS Society has more detailed information available about hosting a golf tournament. Contact Terri Klein at terri@mpssociety.org or 909.806.0101.

National MPS Society Receives Three-Star Charity Rating

Having received the prestigious four-star Charity Navigator rating for the last three years, the National MPS Society learned in December 2008 that we were awarded a three-star rating, indicating that we "exceed or meet industry standards and perform as well or better than most charities in its cause." The board of directors has reviewed Charity Navigator's detailed ratings table and scoring system in order to identify ways to earn a few more points to regain the four-star rating.

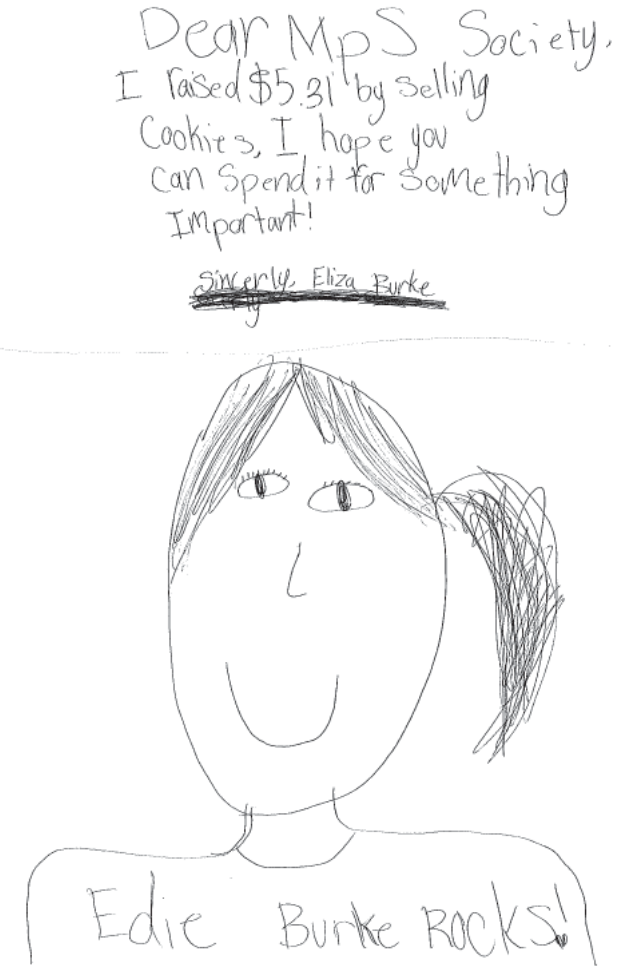
Ways to GIVE

- Renew your membership or sponsor another family
- Gifts in honor of a special person
- Gifts in memory of a special person
- Gifts of cash or marketable securities
- Matching gifts through your employer (check with your human resource office)
  - Request a matching gift form from your employer
  - Complete the employee section of the form
  - Mail to the Society and we'll do the rest
- Contribute through the Combined Federal Campaign if you are employed by the federal government
- 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.
- Annual Fund donation
- Major gift (usually 10 times that of your Annual Fund gift)
- Planned gift
  - Bequest in your will
  - Charitable remainder trust or charitable gift annuity
  - Charitable lead trust
  - Life insurance policy
  - 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: info@mpssociety.org or 877.**MPS**.1001

Special thanks to Eliza Burke for her fundraising efforts in memory of her sister, Edie (MPS I)



The 5th annual Play for Taylor piano recital was held in honor of Taylor Geary (MPS III) in Hilliard, OH, on March 14, 2009. Taylor's mother, Rachel Wojnarowski, teaches piano and every year her students hold a fundraiser concert in honor of Taylor. This year's event raised nearly $500.

Taylor Geary (MPS III) and Clara Gibson (MPS III)

MPS Society's Continuing Education Scholarship winners have big plans for the future

Congratulations to all those who applied for the 2009 Continuing Education Scholarship Program. The Family Support Committee is pleased to be able to offer 20, $1,000 scholarships for individuals affected with MPS and related disease, their parents, children and siblings. The scholarship awards will help the following as they continue their secondary education. Congratulations and best wishes to all of the applicants.

Kenny Ahles, an adult with MPS I, is attending Brevard Community College and will obtain his associate's degree. His plans include attending one of the Florida state colleges to earn a business degree.

Jenna Caswell will graduate from Manchester High School West in 2009. Jenna has always been proud to be able to make a difference in the lives of children, especially her brother Sam, who has MPS I. Jenna will attend Keene State College majoring in early childhood education.

Drew Delvaux will graduate from Yankton High School, and plans to attend Minnesota State University with plans to major in social work or psychology. Drew's younger brother Joseph has MPS II. Drew enjoys his involvement in the theater department at school.

Chelsea Hauck will be entering her fourth year at Goucher College in the fall, where she is majoring in peace studies with a minor in biology. Her sister, Megan, had MPS III and passed away in 2001. Megan continues to inspire and motivate Chelsea each day.

Stephanie Hauck will be entering her third year at Elmira College in the fall. She is majoring in elementary education with plans to obtain a master's degree in education, with an emphasis on special education. Stephanie's sister, Megan, had MPS III. Stephanie was inspired by Megan's teachers, who were so dedicated to her big sister that she decided to become a teacher with hopes to impact her students' lives.

Amanda Hedrick will be entering her fourth year at Wittenberg University in the fall, where she is studying biology with plans to attend medical school; she would like to become a pediatrician. Amanda would like to be able to help children with special needs. Amanda's brother, David, who had MPS III and passed away in 2004, is her inspiration to lead her to being the best pediatrician.

Joanne Huff, mother of 10-year-old Sasha who was diagnosed one year ago with MPS III, will attend training to become a licensed nurse's assistant. She will then continue on to become a registered nurse. In the short time Sasha has been diagnosed, Joanne has found a new capacity to love and appreciate every little thing that Sasha is able to give her—the smiles and laughs are treasured each and every day.

Jeffrey Fowler will be starting his second year at Albion College in the fall. Jeffrey is majoring in economics and management/accounting, and plans on a career in accounting. Jeffrey enjoys spending time with his sister Kim who has MPS III. He especially enjoys all of the love, smiles and giggles she shares with him. Kim has taught Jeff how to live, laugh and love. She is his best friend.

Jenifer Gibson is mother of Clara, 7, who has MPS III. Jenifer is switching careers and is working toward her master's in special needs education with plans to teach children with multiple handicaps. Her greatest personal satisfaction has come from being a mom.

continued

Megan Leighton is obtaining her degree in nursing at Point Loma Nazarene University. She was motivated to become a nurse due to the wonderful nurses who helped her sister, Christina, who had MPS I and passed away in 2002. Megan hopes to be able to help her patients as her sister was helped during the many hospital visits she endured.

Heather Millington has graduated from a home school program and plans to attend Huntington University in the fall, where she will major in psychology to become a counselor after she completes graduate school. She will become a child life specialist in honor of her sister, Hope, who had MPS VI and passed away in 2008.

Mariah Montgomery will graduate from Prairie High School and will attend Iowa Wesleyan College in the fall where she will study special education. Mariah defines inspiration as Lucas, her 11-year-old brother who has MPS III. Mariah plans to teach children with special needs as she enjoys making a difference in their lives.

Stephen Melnyk will begin his second year at Texas A & M University where he is majoring in petroleum engineering. Stephen enjoys spending time with his brother Nick who has MPS II. Because of Nick, Stephen is able to look at individuals and see what they are able to do, not what they are not able to do, on a daily basis.

Cristol Barrett O'Loughlin is attending College of Marin, where she is working toward a master's degree in preschool teaching, with an emphasis on special needs. Cristol has been inspired by her three older brothers, Jared, David and Randy, all of whom had MPS II. Cristol was inspired by her daughter, Chloe, to pursue her preschool teaching credentials. Chloe also is a carrier of MPS II.

Jennifer Prince is an adult with MPS I. She attends Georgia Gwinnett College where she is studying marketing/business. Upon graduation, Jennifer hopes to work for Genzyme, marketing Aldurazyme®, the enzyme replacement therapy for MPS I patients. Jennifer is proud of all of her life accomplishments, and is trying hard to make all her dreams a reality.

Caleb Reed will graduate from Mountain Grove High School, and hopes to attend College of the Ozarks to study wildlife management. Caleb misses his brother, Evan, who had MPS II and passed away in 2006.

Ian Smith is a student at Massachusetts Institute of Technology, where he studies electrical engineering/computer science. Ian has MPS IV, and has been working to improve accessibility at MIT for all people with special needs.

Tierney Veliz is a student at Rosemont College where she is studying English. She plans to continue her education after receiving her undergraduate degree by going to law school. She plans to work as an attorney in criminal law. Her brother, Chase, has MPS II and is 18 years old.

Maggie Wilson will begin her senior year at Marquette University in the fall. She is majoring in advertising and graphic design with plans to become a creative director with an advertising agency. Maggie's biggest inspiration and role model is Kirby, her younger sister who has MPS III. Kirby continues to teach Maggie about every aspect of her life.

Tiffany Wojnarowski will graduate from Grove City Christian School and will attend Miami University in the fall. She will be in the pre med program with a major in chemistry. Tiffany plans to become a cardiothoracic surgeon. Taylor, her step-sister who has MPS III, provides her with comfort and strength to become the person she is.

Our first trip to Washington, DC, for 2009 was very eventful and productive. Ernie Dummann, Kim Whitecotton, Barbara Wedehase and Debbie Dummann divided this trip into four main areas of attack. Visiting the Senate, House, National Institutes of Health (NIH) and Social Security Administration (SSA), focusing our advocacy efforts on National MPS Awareness Day, increased funding to the NIH, language submission to the Labor Health and Human Service subcommittee and increased funding for the Life Span Respite Care Act. Debbie and Kim did a tremendous job and had many productive meetings including our old friends in the Senate offices of Specter (D-PA), Feinstein (D-CA), Kennedy (D-MA) and Murkowski (R-AK). This team also began dialog with the House of Representatives to seek support and cosponsors for H.R. 1441, the Ryan Dant Healthcare Opportunity Act of 2009.

Debbie Dummann, Taryn Morrissey, legislative health aid to Sen. Ted Kennedy, and Kim Whitecotton

Ernie Dummann and Barbara concentrated their efforts on the NIH and SSA focusing on partnering, collaboration and access to services. It was extremely gratifying meeting with the directors of the National Institute of Neurological Disorders and Stroke, National Institute of Diabetes and Digestive and Kidney Diseases, National Center for Research Resources and Office of Rare Diseases. This group has been very supportive and helpful in our interest to increase research funding to help find cures and effective treatments for all our member who are affected with this devastating disease. We also had a very productive meeting with Nancy Schoenberg, the acting program director of the Social Security Administration Compassionate Allowance Program, who was very supportive of our MPS families that need to apply for Supplemental Security Income (SSI) benefits. In many states this is the first step in receiving Medicaid services.

Thanks to Les Sheaffer, Sissi Langford and Kym Wigglesworth whose past efforts made our jobs so much easier.

As your Legislative Committee continues to support and advocate for your families, we welcome our newest committee members: Kim Whitecotton, MaryEllen Pendleton, Austin Noll, Steve Holland, Steve Chesser and Stephen Frye. They all bring a wealth of knowledge from their families' MPS experiences and will take their advocating skills on a national level.

Legislative Committee:
Ernie Dummann, chair
Steve Chesser
Debbie Dummann
Stephen Frye
Tom Gniazdowski
Steve Holland
Terri Klein
Austin Noll
MaryEllen Pendleton
Laurie Turner
Barbara Wedehase
Kim Whitecotton

The 111 Congress

Legislations we continue to follow and support:

H.R. 1441 The Ryan Dant Healthcare Opportunity Act of 2009

This proposed legislation will amend Title XIX of the Social Security Act to allow states to permit certain Medicaid-eligible individuals who have extremely high annual lifelong orphan drug costs to continue on Medicaid notwithstanding increased income. The state will have options to waive the annual earnings restrictions on individuals like Ryan who will have already qualified for Medicaid due to their disability. The objective is to allow these individuals the opportunity to become employed, pay taxes and become whatever their abilities allow them. To qualify for H.R. 1441, an individual must have expired an insurance lifetime cap of at least $1 million and have a prescription orphan drug cost of at least $200,000 annually. This legislation is being sponsored by Rep. Kenny Marchant (R-TX-24). Thank you to Mark Dant, Steve Holland, Eric and Vicki Merrell, Melissa Bryant and Dawn Checrallah who secured co-sponsors during their trip to Washington in May.

continued

S.726 & H.R. 1427 Legislation Introduced to Promote Development of Generic Biologics

Henry Waxman (D-CA), with Health Subcommittee Chair Frank Pallone (D-NJ) and Ranking Member Nathan Deal (R-GA), introduced the "Promoting Innovation and Access to Life-Saving Medicine Act," H.R. 1427. The bill creates a regulatory pathway for the U.S. Food and Drug Administration (FDA) to approve generic biologics, or biotechnology products, given that there are "no clinically meaningful differences" between the generic and an already approved biotech drug. The legislation provides a five-year exclusivity period to the manufacturer of the original biologic, or reference product, and also grants six months of market exclusivity to the first maker of the generic biologic. The bill was referred to the House Committee on Energy and Commerce.

Following are excerpts from a letter the National MPS Society, along with 46 other non-profits, sent on May 1, 2009, requesting further funding of the Lifespan Respite Care Act of 2006 for FY 2010:

The Honorable David Obey
Chair
Subcommittee on Labor, HHS and Education
Committee on Appropriations
U.S. House

The Honorable Todd Tiahrt
Ranking Member
Subcommittee on Labor, HHS and Education
Committee on Appropriations
U.S. House

Dear Chairman Obey and Ranking Member Tiahrt:

We, the undersigned national organizations, are writing to request that the Subcommittee include funding for the Lifespan Respite Care Act (PL 109-442) in the FY 10 Labor, HHS and Education Appropriations bill at its modest authorized level of $71.1 million. Despite its widely supported enactment in 2006, the program received only $2.5 million in FY09. The Lifespan Respite Care Act is the only federal law which mandates respite services, the only federal program which could help ensure respite quality or choice, and the only federal program which allows funds for respite start-up, training and coordination. As the only federal program to address basic accessibility and affordability issues for families regardless of age or disability, it serves a critical and unique purpose.

In these times of serious budget constraints, the economic value that accrues from respite is exceptional. Respite, the most frequently requested family support service, has been shown to provide family caregivers with the relief necessary to maintain their own health, bolster family stability, keep marriages intact, and avoid or delay more costly nursing home or foster care placements. Delaying nursing home, institutional or foster care placement of just one individual for several months can save Medicaid, child welfare or other government programs tens of thousands of dollars.

For families and individuals who may not qualify for any public or private respite funding, including children with severe disabilities, and many other chronic physical and mental health conditions, these programs may be holding out the only helping hand.

We urge you to fully fund the program at $71.1 million. Please join us in sending a message to our nation's family caregivers that we value their health and well-being and that help is on the way in these economically challenging times.

Sincerely,

National MPS Society

HOW TO CONTACT YOUR CONGRESSIONAL LEADERS

For senators go to **www.senate.gov**, in the upper right-hand corner, click on your state.

For representatives go to **www.house.gov**, in the upper left-hand corner put in your ZIP code.

American Recovery and Reinvestment Act of 2009: IDEA Recovery Funds for Services to Children and Youths with Disabilities

The American Recovery and Reinvestment Act of 2009 (ARRA) appropriates significant new funding for programs under Parts B and C of the Individuals with Disabilities Education Act (IDEA). Part B of the IDEA provides funds to state educational agencies and local educational agencies (LEAs) to help them ensure that children with disabilities, including children ages 3 through 5, have access to a free appropriate public education to meet each child's unique needs and prepare him or her for further education, employment and independent living.

The IDEA recovery funds under ARRA will provide an unprecedented opportunity for states, LEAs, and early intervention service providers to implement innovative strategies to improve outcomes for infants, toddlers, children and youths with disabilities while stimulating the economy. Under the ARRA, the IDEA recovery funds are provided under three authorities: $11.3 billion is available under Part B Grants to States; $400 million is available under Part B Preschool Grants; and $500 million is available under Part C Grants for Infants and Families. Preliminary information about each state's allocation is available at **www.ed.gov/about/overview/budget/statetables/recovery.html**. This Web site also provides information about the State Fiscal Stabilization Fund under the ARRA, which is separate from the IDEA recovery funds described in this fact sheet. This document focuses on Part B; additional information on Part C will be available shortly.

Overview of ARRA

PRINCIPLES: The overall goals of the ARRA are to stimulate the economy in the short term and invest in education and other essential public services to ensure the long-term economic health of our nation. The success of the education part of the ARRA will depend on the shared commitment and responsibility of students, parents, teachers, principals, superintendents, education boards, college presidents, state school chiefs, governors, local officials and federal officials. Collectively, we must advance ARRA's short-term economic goals by investing quickly, and we must support ARRA's long-term economic goals by investing wisely, using these funds to strengthen education, drive reforms, and improve results for students from early learning through college. Four principles guide the distribution and use of ARRA funds:

a. **Spend funds quickly to save and create jobs.**
b. **Improve student achievement through school improvement and reform.**
c. **Ensure transparency, reporting and accountability.**
d. **Invest one-time ARRA funds thoughtfully to minimize the "funding cliff."**

ADDITIONAL INFORMATION

The Department will provide updates as additional information becomes available regarding the details of the IDEA recovery funds.

The Department also will provide further information on the government-wide data collection and reporting requirements as this information becomes available.

Send an e-mail to IDEARecoveryComments@ed.gov with questions or concerns.

Kathleen Sebelius Sworn in as Health and Human Services Secretary

Kathleen Sebelius was sworn in as the 21st secretary of the Department of Health and Human Services (HHS) on April 29, 2009. The secretary governs one of the largest civilian departments in the federal government with more than 67,000 employees. HHS is the principal agency for protecting the health of all Americans by providing effective health and human services, especially for those who are least able to help themselves.

While her first focus will be responding to the H1N1 (swine) flu emergency, Secretary Sebelius will soon turn to the larger effort of helping to pass an overhaul of the U.S. healthcare system.

Sen. Edward Kennedy Working with HELP Committee Members to Introduce, Mark up Healthcare System Overhaul Legislation before August Recess

Senate Health, Education, Labor and Pensions (HELP) Committee Chair Edward Kennedy (D-MA) and a group of five other committee members hope to unveil their universal healthcare legislation for early summer. Kennedy's group includes Senate HELP Committee ranking member Mike Enzi (R-WY) and committee members Sens. Christopher Dodd (D-CT), Orrin Hatch (R-Utah), Judd Gregg (R-NH) and one of three other senators—Sens. Jeff Bingaman (D-NM), Tom Harkin (D-IA) or Barbara Mikulski (D-MD), who previously were named to working groups focusing on insurance coverage, prevention and quality improvements, respectively.

Kennedy's staff has been holding stakeholder meetings, which include 20 interest groups, and members and aides from the Senate HELP Committee and the Senate Finance Committee have been holding joint and separate meetings to discuss reform. However, nothing from those meetings has been made available to the public. Kennedy's drafting group is scheduled to have legislation ready by early summer and to the floor before the August recess.

Obama Names Leadership at FDA

President Obama announced his appointment of Dr. Margaret Hamburg as commissioner of the FDA and Dr. Joshua Sharfstein as principal deputy commissioner at the agency. Dr. Hamburg currently is the senior scientist at the think-tank Nuclear Threat Initiative. Her diverse background includes serving as New York City commissioner of health, assistant secretary for planning and evaluation at the Department of Health and Human Services during the Clinton Administration, and assistant director of the Institute of Allergy and Infectious Diseases at the NIH. Prior to taking her leadership post at the FDA, Dr. Hamburg must be confirmed by the full Senate.

Dr. Sharfstein currently serves as Baltimore's health commissioner, and his leadership has won the Health Department and affiliated agencies numerous national and regional awards for innovation and model practice, among others. He is a member of the Board on Population Health and Public Health Practice of the Institute of Medicine. He also served as a health policy advisor on the Democratic staff of the Government Reform Committee of the U.S. House of Representatives for Congressman Henry Waxman (D-CA).

Removing Barriers to Responsible Scientific Research Involving Human Stem Cells

President Obama signed an executive order on March 9, 2009, to remove federal funding restrictions on embryonic stem cell research, thereby expanding the NIH support for exploring the potential of human stem cell research. More specifically, the law reverses the Bush presidential statement of Aug. 9, 2001, and the Bush executive order of June 20, 2007, that prohibited federal funding of embryonic stem cell research conducted on cell lines created after Aug. 9, 2001. Under the executive order signed by President Obama, the Director of the NIH will develop guidelines for "the support and conduct of responsible, scientifically worthy human stem cell research, including human embryonic stem cell research, to the extent permitted by law." After reviewing scientific data and published best practices, the NIH will post draft guidelines for public comment, with the intention of having a final guidance ready within 120 days of passage of the Obama executive order.

Volunteer Spotlight

Volunteers have been very instrumental in making the Society what it is today. While the Society is here to support our families, our members also contribute much of their time, energy and talents. Everyone has something to contribute! In this issue, we interview **Jonathan Formica**.

Who is your child/children with MPS, tell us something about them.

While I don't have children myself, I know Russell and Alexandria Browne very well. Both Russell and Alex have MPS III. Russell has a difficult time walking on his own and Alex is down to one word, "Mommy." Though they both have lost their ability to speak, they still speak volumes with their smiles. Alex is fearless and full of life. When I went to the beach with her, she ran into the ocean before I had a chance to grab her; she's probably a professional surfer and we don't even know it. Russell, though quiet, is very stoic. When his mother sings to him, he will always stop crying and look into her eyes. While they can't communicate in the way you and I do, they sure express their love of life and warm the hearts of everyone around them.

When did you learn of their diagnosis?

I learned of the diagnoses during my eighth grade year. Alex and Russell's mother, Sharon, was my teacher at the time and was absent during many days due to the medical difficulties with Russell. While I (and the rest of the class) knew something was wrong, we weren't aware of the severity of the situation. As I matured, however, I remained friends with Sharon and she explained MPS and her story with me. It was her strength and courage that inspired me to make a documentary.

What volunteer activities have you done for the Society? How long after you joined did you begin volunteering?

For my junior film project at the Orange County High School of the Arts, I created the seven-minute documentary, "Courage." The film showed Sharon's strength in dealing with her children's disorder. Her story was so inspirational and powerful that the film was selected for the Newport Beach Film Festival. Many people expressed interest in a longer version. Throughout the next two years, I explored, developed and expanded the documentary. On Jan. 10, 2009, the 30-minute film premiered at the "Courage Premiere Gala" at Chapman University. The evening included heart-warming music performances and featured many of the people in the film, including Russell and Alex's teachers and doctor. Tami Slawson, member of the MPS Society board of directors, gave an inspirational speech on why it is so important to find a cure. The evening sold out to more than 500 people and brought in more than $9,000 to help the National MPS Society. The event was featured in the *Orange County Register* and on the FOX morning show "Good Day LA."

What do you gain from your volunteer efforts?

Following the gala, I almost felt guilty. It seemed as if I got so much more out of the event than it gave to the MPS Society. Throughout the past few years, I have met so many inspirational and extraordinary families. They have opened their hearts and shared some of their most poignant

stories with me. While I don't feel I have done much, it is my hope that the awareness raised from "Courage" will go further than the money itself. After the *Orange County Register* article was published, I received a phone call from a complete stranger. He was so touched by Sharon's story that he wanted to make a donation to the MPS Society. I have received e-mails from across the country of other people who have been touched by the story, and as such it is my hope that the story of "Courage" will spread even further.

MPS and related disorders are terrible. It is a matter of fact that it tragically shortens the lives of truly delightful children. However, from my work in support of the Society, I have become grateful. I am grateful that I have the privilege to meet such wonderful people, and I am grateful in knowing that one day we will have treatments and cures for all MPS patients.

What would you like others to know about volunteering for the Society?

Volunteering in support of the National MPS Society is truly rewarding. In addition to the wonderful people you will meet, you will have the satisfaction in knowing that you can and are making a difference. Money aside, your presence at a Society event, such as an MPS walk/run, makes a statement. It makes a statement to the public about our fight, but more importantly, it makes a statement to the thousands of other families affected by MPS. Your support enhances the already great sense of courage the MPS community exhibits on a daily basis.

Tell us about yourself and your family.

I am 18 and a freshman at Chapman University in Orange, CA. I am a television and broadcast journalism major and previously attended the Orange County High School of the Arts where I first met Sharon Browne. I am very interested in documentary production; specifically films that focus on challenging social barriers, raise awareness of powerful human-interest stories and promote social change. My family has been very supportive of my chosen career path and even more supportive of my work with the MPS Society. We always attend the Los Angeles MPS run and I couldn't have completed "Courage" without their support.

If you know of someone you would like to have featured in Volunteer Spotlight contact us at info@mpssociety.org.

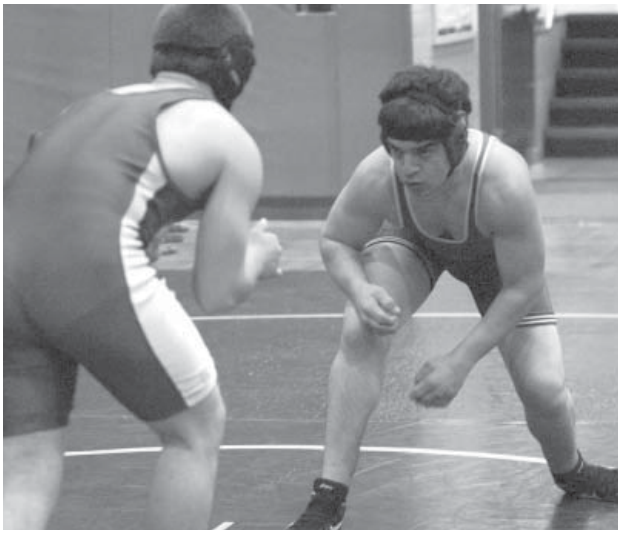
One of the goals of the National MPS Society is to increase awareness of MPS diseases. With the assistance and persistence of our members, we are making great strides. Don't forget to let the MPS Society know when you are featured in a media story!

Following is an excerpt from an article published in the East Providence Post, *East Providence, RI, Dec.19, 2008. Written by Mike Rego. Reprinted with permission.*

Townies take to the mat with an eye toward the future

A young EPHS wrestling group expects to cut its teeth this winter

With a host of unproven performers in the fold and coming off a one-win season, it's hard to imagine East Providence High School wrestling coach Tom Galligan could being anything but a little downtrodden about the prospects of his team this winter. On the contrary, the long-time Townie mat mentor was quite effusive in his praise of the squad on the eve of the 2008–09 season, believing the group will someday soon return East Providence to the top of the state wrestling heap.



Nick Boyce (MPS I)

"This group reminds me of some of the younger teams I've had in the past that have turned into good, solid teams down the line," said Galligan. "They've been working their fannies off in the room. They show up every day, on time. They're just working their tails off. I get a really good feeling from these kids."

In the upper classes, juniors Nick Boyce (MPS I) and Malcolm DosSantos wrestle at 189 along with senior John Costa. Sophomore Frank Allen is a 215-pounder. Senior Justin Perry and junior Adam Aurelio are heavyweights.

"I'm really excited about this young team," said Galligan. "It's great. The enthusiasm in the team is just fantastic. Last year, things didn't work out for us, which is rare at East Providence. This year, we're already getting great leadership from our sophomores and freshmen. It's going to be a fun season. We're going to be fine."

Dylan Bontems
13, MPS II, 4/25/09

Louis "L.B." Butts
12 months, MPS I, 5/3/09

Brinley Craig
15 months, MPS I, 4/27/09

Kelley Crompton
45, ML III, 3/17/09

Karly Elizabeth Davis
8, MPS I, 3/8/09

Carolyn Faust
16, MPS III, 3/14/09

Aaron Jackson
12, MPS II, 3/19/09

Andrew Jump
11, MPS III, 5/19/09

Alyssa Leonard
8, MPS I, 3/11/09

ShaeAnna Nelson
20, MPS III, 2/3/09

Brigham Reneer
14, MPS II, 5/15/09

Samantha Sasry
10, MPS IV, 8/7/08

Noah Scott
10, MPS I, 3/14/09

Brian Stanley
38, MPS I, 3/7/09

Nathan Thomas
12, MPS II, 4/4/09

David Wadle
30, MPS II, 4/22/09

Jenny Wingate
11, MPS III, 2/9/09

Edie Burke, MPS I
7/27/98 – 5/2/08

It is hard to believe that a year has passed since Edie's death. As I sit here attempting to write another thank you note I am moved to write to you who share a similar path and bond. I write to tell you of the amazing hours, days and weeks that followed Edie's death and make those of you who will travel that road aware of alternatives to traditional funeral arrangements.

Edie came down with a cold on a Sunday. It was nothing more than the usual and progressed normally. However on Thursday when we would have expected her to begin recovering she woke up with pneumonia. Another 24 hours later, she passed away sleeping on the sofa on which she had spent countless hours watching her favorite cartoons.

What was most remarkable about her death was all that followed. Her brother, Jack, and sister, Liza, were both awake at 6:00 a.m. which never happened. They came to my room and we chatted a bit before I headed downstairs to check on Edie. Tom met me halfway up the stairs and told me he thought we had lost her. I got to her side and, although she looked as though she was asleep, she was not breathing. Tom quickly rounded up the kids and each spent time holding her and brushing out her mane of wild blond hair. The EMTs arrived and offered to take her to a funeral home. I declined and later called a friend, Caroline, who had experience post-death. Edie's doctor came over and said his goodbyes to his friend and patient.

Caroline arrived and we began preparations for a "home funeral."* I bathed Edie as I had done for almost 10 years and as I was drying her hair, Edie's CNA arrived and helped finish. Caroline and I cleaned up Edie's room and removed all the no-longer needed medical equipment. I selected Edie's final outfit, dressed her and laid her on her bed on the quilt which neighbors had made for her a few short months earlier. Soon Edie's MeMa and MePa (Susan and Loy McKeithen) arrived from Charlotte and each had time to hold her. Susan, who often painted Edie's nails, did her fingernails one last time. Caroline made arrangements for Edie's cremation and brought us the necessary paperwork. By midday my mother delivered a draft of Edie's eulogy. I edited it and included an invitation to an intimate visitation in our home the next night. We had many visitors, mostly from the neighborhood, who were delighted to spend one-on-one time

continued

with Edie that afternoon. Many wanted to help and, upon hearing of the visitation the following day at our home, began preparing food.

The next day more people came over requesting to spend time with Edie. Family arrived from out of town, cleaned the house, fed us and kept Jack and Liza busy. As the visitation hour came, neighbors swarmed in with plates of food. A line quickly formed up the stairs of friends and neighbors, both old and young, waiting patiently to have their turn by Edie's side. We transformed our dining room into the cremation box room. Children and adults drew colorful pictures and glued them to the box. Others wrote personal messages to Edie that went in the box. When the evening was over, we had a beautiful box full of love, full bellies and a clean house. When I went to bed Saturday night I told Edie good night and gave her sweet face a kiss. But I was beginning to know that she was gone and only her body was with us.

Sunday was quieter, and I was able to pass Edie resting in her room without stopping in every time. Most of our out-of-town family headed home and would return in two weeks for Edie's memorial service. MeMa and MePa stayed with Edie while our family went to church. It was Ascension Sunday. Evidently Friday had been, according the Biblical calendar, the day Jesus rose to heaven.

Monday morning Tom and I, along with Caroline, took Edie's body to be cremated. We braided her hair into two thick pigtails and then cut them off to send to Locks of Love. We laid her body in the box; she looked peaceful and loved. This moment that I had dreaded felt so natural and even beautiful. As I reflected on the happenings of the past three days I realized that Edie's spirit had been alive and well. In death she had accomplished what she did in life… bringing people together, supporting others in time of need and spreading love.

If you would like to know more about home funerals please contact me at 828.779.3639.

Laura M. Burke

** Home funerals are not legal in all states and may require certain legal provisions.*

Family of Alyssa Louden (MPS I), 3/16/07–10/8/08, celebrated her 2nd birthday with a balloon release.

1st Advances in Rare Bone Diseases Scientific Conference

Oct. 22–24, 2008
National Institutes of Health (NIH)

by *Klane K. White, MD*
Chair, Technical Committee

Kym Wigglesworth and I had the pleasure of attending the 1st Advances in Rare Bone Diseases Scientific Conference last fall in Bethesda, MD. Because of the prevalence of musculoskeletal disease in MPS and related diseases, the National MPS Society was asked to send representatives. This meeting was organized by the NIH, the United States Bone and Joint Decade (a nonprofit organization dedicated to promoting scientific and medical advances in musculoskeletal health), and the Rare Bone Disease Patient Network. The Rare Bone Disease Patient Network consists of nine member groups including the Fibrous Dysplasia Foundation, the International Fibrodysplasia Ossificans Progressiva Association, the International Osteopetrosis Association, the Lymphangiomatosis & Gorham's Disease Alliance, the Multiple Hereditary Exostoses Research Foundation, the Melorheostosis Association, the Osteogenesis Inperfecta Foundation, the Paget Foundation for Paget's Disease of Bone and Related Disorders, and the XLH (x-linked hypophosphotemic rickets) Network.

The goal of the meeting was to bring together scientific investigators who study various rare bone diseases in order to provide up-to-date information on the etiology, pathogenesis, therapies and ultimately cures for rare diseases with primarily skeletal manifestations. Our goal in attending was twofold: 1) to make contacts with other rare disease patient groups and 2) to glean new insights about research in other rare diseases that might be translatable to MPS diseases.

The seminar was a two-day series, in which the first day was fairly dense in basic science. The second day consisted of clinically oriented talks. Both days were comprised of state-of-the-art information related to the treatment of these rare bone disorders. From the basic science side, Dr. Lila Simonaro presented her research on bone and joint disease in MPS animal models. An evening lecture was given by a medical representative from Genzyme, Dr. Edward Kaye, providing an industry link to the conference. Dr. Kaye discussed the difficulties encountered by pharmaceutical companies in their attempts to bring therapies to market for rare diseases. Aldurazyme®, the enzyme replacement therapy for MPS I, was spotlighted as an example of how this can be successfully achieved.

The meeting also included "fireside chats" in which attendees were able to meet for small group discussions with specialists in different fields. I took advantage of this opportunity to meet with Dr. Francis Glorieux, the world's expert on osteogenesis imperfecta. While individuals with MPS diseases do not necessarily have problems with frequent fractures, there is much interest in the study of low bone mineral density for MPS and mucolipidosis (ML). Dr. Glorieux offered excellent insight into the treatment of low bone mineral density with bisphosphonates. These drugs have shown great promise with bone pain in children with ML. There may be a future use for these drugs in individuals with other MPS disorders as we learn more about their use over time.

While I focused on pursuing the scientific sessions, Kym spent time making contacts with leaders from the NIH and other patient groups. It is important to further strengthen our relationship with these groups as part of our efforts to promote MPS awareness and scientific advances in the study of skeletal disease in MPS and related diseases.

The meeting was a wonderful opportunity for the National MPS Society. We look forward to attending future conferences of this nature, and expanding our relationships with other patient advocacy groups and the NIH.

University of Minnesota Study: Learning Difficulties in MPS II and MPS VI

The Center for Neurobehavioral Development at the University of Minnesota is beginning a research study seeking to better understand the brain basis for the learning difficulties sometimes found in individuals with MPS II and VI. Researchers are studying the central nervous system to better understand brain changes in MPS disorders to find better ways of treating these problems. To participate, you or your child must be over the age of 6 and under 35 and able to cooperate in an MRI imaging study without sedation.

Participants will be seen for two sessions in one day at the Center for Neurobehavioral Development at the University of Minnesota. One session for neuropsychological testing will last two to three hours; the other session for brain imaging will last one hour. Neuropsychological tests will include a brief test of cognitive ability and attention, and several tests of memory. Some testing will be done on a computer.

Hotel accommodations for one night, and air and ground to the University of Minnesota, Minneapolis campus, will be provided for two persons. An honorarium of $100 also will be provided.

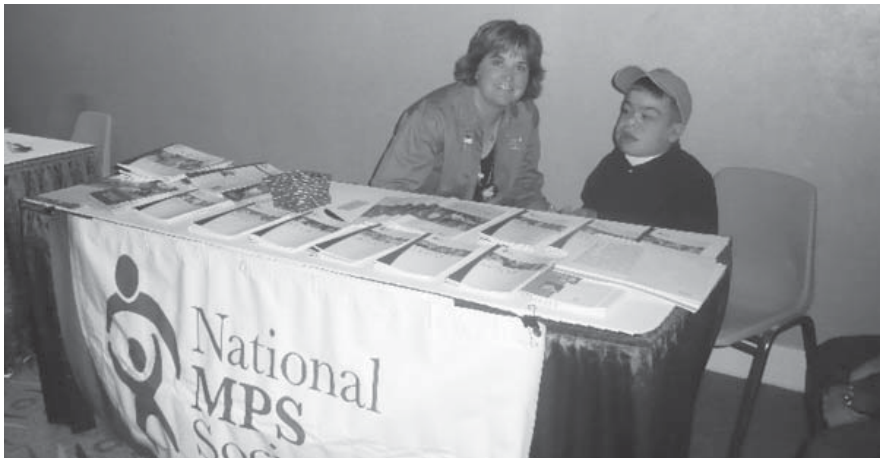
Contact Elsa Shapiro at 612.625.1618 or shapi004@umn.edu, or Kate Delaney at 612.625.1143 or delan011@umn.edu, for more information.

University of Minnesota Children's Hospital Offers Clinical Trial of Human Growth Hormone

A clinical trial of human growth hormone (HGH) is being conducted at the University of Minnesota Children's Hospital. Children with MPS I, II or VI with short stature are invited to participate. HGH is a U.S. Food and Drug Administration-approved treatment for short stature, however there is no data at this time on using this treatment specifically in children with MPS. The goal of this clinical trial is to determine what, if any, effect HGH has on growth velocity, bones, and cognitive functioning of children with MPS I, II and VI.

For additional information contact:

Lynda Polgreen, MD
Assistant Professor
University of Minnesota
Pediatric Endocrinology
516 Delaware St. SE
PWB 13-124, MMC 8404
Minneapolis, MN 55455
612.624.4459
polgr001@umn.edu



MaryEllen Pendleton and Scotty Whitecotton (MPS II), along with his mother, Kim (not pictured), staffed the National MPS Society's booth at the Lysosomal Disease Network conference in San Diego.

Kakkis EveryLife Foundation

On Feb. 27, 2009, Dr. Emil Kakkis retired from his position as chief medical officer of BioMarin Pharmaceutical to allow him time to devote to personal endeavors related to rare diseases. Dr. Kakkis will continue to be a consultant for the company for the foreseeable future.

Dr. Kakkis joined BioMarin in September 1998. Prior to joining BioMarin, he was an assistant professor at the Harbor-UCLA Medical Center, Division of Genetics, Department of Pediatrics. Together with his colleague Elizabeth F. Neufeld, PhD, of the University of California at Los Angeles (UCLA), Dr. Kakkis discovered how to produce a recombinant form of alpha-L-iduronidase (later to become known as Aldurazyme® [laronidase]), the enzyme that people with MPS I are lacking. While at BioMarin, Dr. Kakkis was instrumental in guiding Aldurazyme, Naglazyme™ and Kuvan® through development and regulatory approval. He also drove initial phases of development of PEG-PAL pre-clinical, clinical, regulatory and research organizations.

Dr. Kakkis formally announced on May 14, 2009, at the National Organization for Rare Disorders Partners in Progress Summit in Washington, DC, the Kakkis EveryLife Foundation. The Kakkis EveryLife Foundation is dedicated to improving the treatment of patients with very rare disorders through education and changes in the process regulating the development of therapies. Information about this foundation can be found at **www.curetheprocess.org**.

Clinical Trials

MPS I

New study of intrathecal enzyme replacement therapy for cognitive decline in MPS I patients

The Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center in Torrance, CA, in collaboration with the University of Minnesota, will begin a 24-month study to examine whether intrathecal enzyme replacement therapy with Aldurazyme® will help to stabilize or reverse memory and intelligence deficits in individuals with MPS I. To qualify for the study, the minimum age is 6 years and the individual must not have been transplanted.

For more information, contact The MPS Research Laboratory at the Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center, 1124 Carson St., Bldg. E-4, Torrance, CA, 90502. Telephone: 310.222.4145.

MPS I Intrathecal ERT for Spinal Cord Compression

One-Year Extension Study Approved

Enzyme replacement therapy (ERT) has been developed for MPS I. ERT helps many physical ailments due to the disease, but does not treat the central nervous system due to inability to cross the blood brain barrier. The purpose of this study is to test delivery of ERT to the spinal fluid

continued

via intrathecal injection in patients with MPS I. In this pilot study, recombinant human α-L-iduronidase will be administered intrathecally once per month for four months to individuals age 8 and older with the Hurler-Scheie and Scheie forms of MPS I and spinal cord compression. Contact Dr. Patricia Dickson, principal investigator, with questions regarding age at 310.222.4145 or pdickson@ucla.edu. If successful, intrathecal delivery could represent a practical, straightforward method of treating central nervous system disease due to lysosomal storage.

Primary Outcomes: Safety of intrathecal enzyme treatment by blood and spinal fluid tests each month; improvement in neurologic signs related to spinal cord compression, by neurologic examination and Japanese Orthopedic Association Scale each month; improvement in neurologic symptoms related to spinal cord compression, by subjective assessments and independence of functioning scale each month; improvement in mobility, by six-minute walk test each month; improvement in spinal cord compression by MRI imaging and somatosensory evoked potentials at baseline and four months; improvement in lysosomal storage by spinal fluid glycosaminoglycan levels at each treatment.

Secondary Outcomes: Improvement in spinal fluid pressure, by opening pressure measurements at each intrathecal treatment; improvement in hydrocephalus and other brain lesions by MRI at baseline and four months.

Expected Total Enrollment: 10

Additional information can be obtained at **www.clinicaltrials.gov/ct/show/NCT00215527?order=1** or by contacting Principal Investigator Dr. Patricia Dickson at 310.222.4145 or pdickson@ucla.edu.

MPS I Intrathecal ERT for Children Being Considered for Transplantation

The University of Minnesota recently has obtained U.S. Food and Drug Administration 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 Hurler 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 Hurler syndrome who are between 8 and 36 months of age who have not previously received enzyme therapy and are being considered for transplantation at the University of Minnesota are eligible. Patients receiving laronidase in the spinal fluid also will be on intravenous laronidase prior to transplant. The study will involve four doses of laronidase given during a lumbar puncture (spinal tap) approximately three months before transplantation, at the time of admission to the hospital for the transplant, three months after the transplant and six months after the date of the transplant. The principal investigator of the study is Dr. Paul Orchard, who can be reached at 612.626.2961 or orcha001@umn.edu. Alternatively, Teresa Kivisto, nurse coordinator, can be reached at 612.273.2924 or TKIVIST1@Fairview.org.

MPS II

Shire Human Genetic Therapies is committed to conducting a clinical trial in individuals with MPS II who have neurological involvement. Currently this study is projected to be at the University of North Carolina; details regarding inclusion criteria and study design are forthcoming. For more information, contact Amy Fisher at Shire Human Genetic Therapies at 919.468.0646 or afisher@shire.com.

MPS III

Shire Pharmaceuticals Group, as part of its research to evaluate new approaches to the problem of treatment of the central nervous system, is hoping to move its MPS III A program forward. If the trial to directly administer the enzyme into the central nervous system of individuals with MPS II is successful, Shire hopes to expand its research initiatives to include MPS III A. The Shire Web site is **www.shire.com**.

continued

MPS IV

The board of directors of Vivendy Therapeutics announced in late 2008 that they will stop the natural history program as well as further medical activities in the scope of the development project for enzyme replacement therapy treatment of MPS IV A.

BioMarin Pharmaceutical Inc. announced April 21, 2009, the initiation of a phase I/II clinical 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 report initial results in the first half of 2010.

"We plan to leverage our clinical, manufacturing and regulatory expertise to bring a new therapeutic option to the significant number of untreated Morquio patients around the world," said Henry Fuchs, MD, chief medical officer of BioMarin. "GALNS has been shown in mice to reach important tissues including cartilage and different zones of the bone such as bone marrow, calcified bone and importantly, the growth plate. Our experiments have also shown that GALNS is taken up into human Morquio chondrocytes in vitro and reaches the lysosome to clear keratan sulfate."

Chris Hendriksz, MD, consultant in Metabolic Disorders, Birmingham Children's Hospital, added, "This is a very exciting announcement for our patients who have been waiting so long for a potential therapy. Hope of a therapeutic is now within reach for these patients, and Birmingham Children's Hospital is very honored to be part of this exciting development."

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. All patients to be enrolled in the study have already been identified. During the dose escalation phase of the study, subjects will receive weekly intravenous infusions of BMN-110 in three consecutive 12-week dosing intervals. The objectives of the phase I/II study will be to evaluate safety, pharmacokinetics, pharmacodynamics and to identify the optimal dose of GALNS for future studies.

BioMarin also will be conducting a Morquio Clinical Assessment Program or MorCAP that will involve about 15 centers in many countries and will evaluate the disease situation for patients globally. Finally they expect to have a phase III double-blind placebo controlled study that might include 50–100 patients from many centers. Being in the MorCAP program will improve a patient's chances of being in the phase III. Additional information can be found at **www.morquioBMRN.com**.

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.

Treatment Therapies

MPS I

Aldurazyme®, administered once-weekly, has been approved in the United States and in 15 countries of the European Union for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of MPS I, to treat the non-neurological manifestations of the disease. Aldurazyme was developed by BioMarin and Genzyme under a joint venture agreement that assigns commercial manufacturing responsibilities to BioMarin, and worldwide sales and marketing responsibilities to Genzyme.

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

MPS II

Elaprase™ is a long-term ERT for patients with a confirmed diagnosis of MPS II which has been approved for use in the United States, Canada and many countries in Europe. Elaprase was developed and is produced by Shire Human Genetic Therapies (formerly TKT), and is given as weekly infusions to replace the missing enzyme that Hunter syndrome patients fail to produce in sufficient quantities.

Additional information can be obtained at www.shire.com or by contacting OnePath℠ toll-free at 866.888.0660. OnePath provides assistance with insurance, product access, treatment centers and education about Elaprase and MPS II.

MPS VI

Naglazyme™ is the ERT for individuals with a confirmed diagnosis of MPS VI and has been approved for use in the United States and in many European countries. Developed and produced by BioMarin Pharmaceutical, Inc., Naglazyme has been shown to improve walking and stair-climbing capacity.

For more information, contact BioMarin Patient and Physician Support at 866.906.6100 or bpps@bmrn.com.

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Ladies: light pink, black, midnight heather — Sizes: XS, S, M, L, XL = $30; XXL = $32; 1X = $34; 2X = $36
Youth: black, gray heather, midnight heather, light pink — Sizes: XS (2–4), S (6–8), M (10–12), L (14–16) = $30

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Ladies — Sizes: XS, S, M, L, XL = $60; XXL = $62; 1X = $64; 2X = $64

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Understanding Clinical Trials

Choosing to participate in a clinical trial is an important personal decision. The following frequently asked questions provide detailed information about clinical trials. In addition, it is often helpful to talk to a physician, family members or friends about deciding to join a trial. After identifying some trial options, the next step is to contact the study research staff and ask questions about specific trials.

What is a clinical trial?

Although there are many definitions of clinical trials, they are generally considered to be biomedical or health-related research studies in human beings that follow a pre-defined protocol. **ClinicalTrials.gov** includes both interventional and observational types of studies. Interventional studies are those in which the research subjects are assigned by the investigator to a treatment or other intervention, and their outcomes are measured. Observational studies are those in which individuals are observed and their outcomes are measured by the investigators.

Why participate in a clinical trial?

Participants in clinical trials can play a more active role in their own healthcare, gain access to new research treatments before they are widely available, and help others by contributing to medical research.

Who can participate in a clinical trial?

All clinical trials have guidelines about who can participate. Using inclusion/exclusion criteria is an important principle of medical research that helps to produce reliable results. The factors that allow someone to participate in a clinical trial are called "inclusion criteria" and those that disallow someone from participating are called "exclusion criteria." These criteria are based on such factors as age, gender, the type and stage of a disease, previous treatment history and other medical conditions. Before joining a clinical trial, a participant must qualify for the study. Some research studies seek participants with illnesses or conditions to be studied in the clinical trial, while others need healthy participants. It is important to note that inclusion and exclusion criteria are not used to reject people personally. Instead, the criteria are used to identify appropriate participants and keep them safe. The criteria help ensure that researchers will be able to answer the questions they plan to study.

What happens during a clinical trial?

The clinical trial process depends on the kind of trial being conducted (See **"What are the different types of clinical trials?"** on page 47) The clinical trial team includes doctors and nurses as well as social workers and other healthcare professionals. They check the health of the participant at the beginning of the trial, give specific instructions for participating in the trial, monitor the participant carefully during the trial, and stay in touch after the trial is completed.

Some clinical trials involve more tests and doctor visits than the participant would normally have for an illness or condition. For all types of trials, the participant works with a research team. Clinical trial participation is most successful when the protocol is carefully followed and there is frequent contact with the research staff.

What is informed consent?

Informed consent is the process of learning the key facts about a clinical trial before deciding whether or not to participate. It also is a continuing process throughout the study to provide information for participants. To help someone decide whether or not to participate, the doctors and nurses involved in the trial explain the details of the study. If the participant's native language is not English, translation assistance can be provided. Then the research team provides an informed consent document that includes details about the study, such as its purpose, duration, required procedures and key contacts. Risks and potential benefits are explained in the informed consent document. The participant then decides whether or not to sign the document. Informed consent is not a contract, and the participant may withdraw from the trial at any time.

continued

What are the benefits and risks of participating in a clinical trial?

Benefits

Clinical trials that are well-designed and well-executed are the best approach for eligible participants to:

- play an active role in their own healthcare.
- gain access to new research treatments before they are widely available.
- obtain expert medical care at leading healthcare facilities during the trial.
- help others by contributing to medical research.

Risks

There are risks to clinical trials.

- There may be unpleasant, serious or even life-threatening side effects to experimental treatment.
- The experimental treatment may not be effective for the participant.
- The protocol may require more of their time and attention than would a non-protocol treatment, including trips to the study site, more treatments, hospital stays or complex dosage requirements.

What are side effects and adverse reactions?

Side effects are any undesired actions or effects of the experimental drug or treatment. Negative or adverse effects may include headache, nausea, hair loss, skin irritation or other physical problems. Experimental treatments must be evaluated for both immediate and long-term side effects.

How is the safety of the participant protected?

The ethical and legal codes that govern medical practice also apply to clinical trials. In addition, most clinical research is federally regulated with built-in safeguards to protect the participants. The trial follows a carefully controlled protocol, a study plan that details what researchers will do in the study. As a clinical trial progresses, researchers report the results of the trial at scientific meetings, to medical journals, and to various government agencies. Individual participants' names will remain secret and will not be mentioned in these reports.

What should people consider before participating in a trial?

People should know as much as possible about the clinical trial and feel comfortable asking the members of the healthcare team questions about it, the care expected while in a trial, and the cost of the trial. The following questions might be helpful for the participant to discuss with the healthcare team. Some of the answers to these questions are found in the informed consent document.

- What is the purpose of the study?
- Who is going to be in the study?
- Why do researchers believe the experimental treatment being tested may be effective? Has it been tested before?
- What kinds of tests and experimental treatments are involved?
- How do the possible risks, side effects and benefits in the study compare with my current treatment?
- How might this trial affect my daily life?
- How long will the trial last?
- Will hospitalization be required?
- Who will pay for the experimental treatment?
- Will I be reimbursed for other expenses?
- What type of long-term follow up care is part of this study?
- How will I know that the experimental treatment is working? Will results of the trials be provided to me?
- Who will be in charge of my care?

What kind of preparation should a potential participant make for the meeting with the research coordinator or doctor?

- Plan ahead and write down possible questions to ask.
- Ask a friend or relative to come along for support and to hear the responses to the questions.
- Bring a tape recorder to record the discussion to replay later.

continued

Every clinical trial in the United States must be approved and monitored by an Institutional Review Board (IRB) to make sure the risks are as low as possible and are worth any potential benefits. An IRB is an independent committee of physicians, statisticians, community advocates, and others that ensures that a clinical trial is ethical and the rights of study participants are protected. All institutions that conduct or support biomedical research involving people must, by federal regulation, have an IRB that initially approves and periodically reviews the research.

Does a participant continue to work with a primary healthcare provider while in a trial?

Yes. Most clinical trials provide short-term treatments related to a designated illness or condition, but do not provide extended or complete primary healthcare. In addition, by having the healthcare provider work with the research team, the participant can ensure that other medications or treatments will not conflict with the protocol.

Can a participant leave a clinical trial after it has begun?

Yes. A participant can leave a clinical trial, at any time. When withdrawing from the trial, the participant should let the research team know about it, and the reasons for leaving the study.

Where do the ideas for trials come from?

Ideas for clinical trials usually come from researchers. After researchers test new therapies or procedures in the laboratory and in animal studies, the experimental treatments with the most promising laboratory results are moved into clinical trials. During a trial, more and more information is gained about an experimental treatment, its risks and how well it may or may not work.

Who sponsors clinical trials?

Clinical trials are sponsored or funded by a variety of organizations or individuals such as physicians, medical institutions, foundations, voluntary groups and pharmaceutical companies, in addition to federal agencies such as the National Institutes of Health, the Department of Defense, and the Department of Veteran's Affairs. Trials can take place in a variety of locations, such as hospitals, universities, doctors' offices or community clinics.

What is a protocol?

A protocol is a study plan on which all clinical trials are based. The plan is carefully designed to safeguard the health of the participants as well as answer specific research questions. A protocol describes what types of people may participate in the trial; the schedule of tests, procedures, medications and dosages; and the length of the study. While in a clinical trial, participants following a protocol are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

What is a placebo?

A placebo is an inactive pill, liquid or powder that has no treatment value. In clinical trials, experimental treatments are often compared with placebos to assess the experimental treatment's effectiveness. In some studies, the participants in the control group will receive a placebo instead of an active drug or experimental treatment.

What is a control or control group?

A control is the standard by which experimental observations are evaluated. In many clinical trials, one group of patients will be given an experimental drug or treatment, while the control group is given either a standard treatment for the illness or a placebo.

What are the different types of clinical trials?

Treatment trials test experimental treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.

Prevention trials look for better ways to prevent disease in people who have never had the disease or to prevent a disease from returning. These approaches may include medicines, vaccines, vitamins, minerals or lifestyle changes.

Diagnostic trials are conducted to find better tests or procedures for diagnosing a particular disease or condition.

Screening trials test the best way to detect certain diseases or health conditions.

continued

Quality of Life trials (or Supportive Care trials) explore ways to improve comfort and the quality of life for individuals with a chronic illness.

What are the phases of clinical trials?

Clinical trials are conducted in phases. The trials at each phase have a different purpose and help scientists answer different questions:

In **Phase I** trials, researchers test an experimental drug or treatment in a small group of people (20–80) for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.

In **Phase II** trials, the experimental study drug or treatment is given to a larger group of people (100–300) to see if it is effective and to further evaluate its safety.

In **Phase III** trials, the experimental study drug or treatment is given to large groups of people (1,000–3,000) to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.

In **Phase IV** trials, post-marketing studies delineate additional information including the drug's risks, benefits and optimal use.

What is an "expanded access" protocol?

Most human use of investigational new drugs takes place in controlled clinical trials conducted to assess safety and efficacy of new drugs. Data from the trials can serve as the basis for the drug marketing application. Sometimes, patients do not qualify for these carefully controlled trials because of other health problems, age or other factors. For patients who may benefit from the drug use but don't qualify for the trials, U.S. Food and Drug Adminstration regulations enable manufacturers of investigational new drugs to provide for "expanded access" use of the drug. For example, a treatment Investigational New Drug application (IND), or treatment protocol, is a relatively unrestricted study. The primary intent of a treatment IND/protocol is to provide for access to the new drug for people with a life-threatening or serious disease for which there is no good alternative treatment. A secondary purpose for a treatment IND/protocol is to generate additional information about the drug, especially its safety. Expanded access protocols can be undertaken only if clinical investigators are actively studying the experimental treatment in well-controlled studies, or all studies have been completed. There must be evidence that the drug may be an effective treatment in patients like those to be treated under the protocol. The drug cannot expose patients to unreasonable risks given the severity of the disease to be treated.

Some investigational drugs are available from pharmaceutical manufacturers through expanded access programs listed in **ClinicalTrials.gov**. Expanded access protocols are generally managed by the manufacturer, with the investigational treatment administered by researchers or doctors in office-based practice. If you or a loved one are interested in treatment with an investigational drug under an expanded access protocol listed in **ClinicalTrials.gov**, review the protocol eligibility criteria and location information and inquire at the contact information number.

For more information, go to **www.fda.gov/fdac/special/newdrug/speeding.html**.

Enter the SleepSafe Bed Giveaway!

Each quarter, SleepSafe Beds will be giving away a visually appealing bed (valued at $8,500) designed to prevent falls and entrapment. Go to **www.sleepsafebed.com:80/GiveAway/index.htm** to enter.

The Littlest Heroes Project

The Littlest Heroes Project is a non-profit organization comprised of professional photographers nationwide who provide free photo sessions to our nation's Littlest Heroes, those children with serious illnesses or life-altering disabilities. The organization's photographers understand each and every case, and cater it to fit and honor each child and family. Photographers will travel to homes, hospitals, clinics, fundraisers and benefits to help capture your child's journey.

"My son, Tyler, has MPS III, and I have tried numerous times to capture him in a photograph," said Donna Kay Langan. "I have spent too much money with photo places that are not tolerant of our 'special child' and even purchased my own equipment, but then we are never in the photo. It's not always about smiling at the camera, but capturing his smile and the joy in his eyes."

"This is our way of giving back and taking a stand for these children who sometimes feel forgotten because of their illnesses. We are here to let them know that they are heroes to many, and to share their inspirational stories and photos with the world."

Visit **www.thelittlestheroesproject.org** for more information.

Family Care Navigator℠

State-by-state help for family caregivers

Navigating the long-term care system can be difficult when providing care to an older or disabled family member or friend. This state-by-state resource is intended to help locate government, nonprofit and private programs in your area. It includes services for family caregivers, as well as resources for older or disabled adults living at home or in a residential facility. Information on government health and disability programs, legal resources, disease-specific organizations and more also is available.

The database includes a separate profile for each state and the District of Columbia. Profiles contain the state's background characteristics related to caregiving and aging, as well as information on publicly-funded caregiver support programs.

State-by-state data include:

- Average daily cost for adult day services
- Average hourly costs for home health care
- Average hourly costs for personal/ home care

In addition, key updates for each state include:

- Number of family caregivers and total caregiving hours
- Economic value of caregiving
- Average daily cost in a nursing home
- Average monthly cost for assisted living
- Number of home health aides in the workforce
- Median hourly wages for nursing assistants, home health aides and personal/home care aides

Visit **http://caregiver.org/caregiver/jsp/fcn_content_node.jsp?nodeid=2083**.

Hunter disease e-clinic

Hunter disease e-clinic is a virtual training clinic, designed specifically for the purpose of learning to diagnose and manage patients presenting with characteristic of rare genetic metabolic diseases. For more information, visit **www.sickkids.ca/lysosomalresearchgroup**.

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.

Video monitor offers peace of mind

Cynthia Anhalt and her husband loathed going to check their son Conner (MPS II) when he was in his bed, for fear of waking him up." They purchased a video monitor by Summer Infant, which allows them to keep a close watch on Conner without disturbing him.

"We can see and/or hear him at all times, day or night," said Cynthia. "Now we have peace of mind knowing our son is sleeping well, or know just when he's not."

The Summer Infant video monitors are sold at Target, Walmart, K-mart and Babies R US.

Expression of Hope II: Inspiration through Art

Expression of Hope is a global program featuring works of art by the community touched by lysosomal storage disorders (LSDs). People living with LSDs and their caregivers are invited to submit artwork which will be shared with the worldwide community via the Web. Selected artworks also will join the traveling Expression of Hope II art show. Our vision is that those who experience this art will be inspired and moved by the powerful expressions of the human spirit which the artworks will reveal.

Everyone who submits a photo of their art for exhibition consideration will receive a beautiful book with selected images from the program. Visit **www.expressionofhope.com** for more information.

The Simple Shirt is simply wonderful

By allowing port access for either the right or left side, the Simple Shirt offers comfort and convenience during infusions for all ages.

The Simple Shirt was created by Gina Stephenson, a home infusion nurse who was inspired by her patient, Davis Barkley (MPS II). The unisex shirts are available in fleece or cotton, and are custom made based on the measurements provided ensuring a perfect fit.

Gina is donating 10 percent of the profits of Simple Shirts to the National MPS Society. Contact Gina at 502.641.1559 or gstep@mac.com with questions.

SIMPLE SHIRT ORDER FORM

Name _____

Address _____

Telephone number (_____) _____

Please take the time to measure accurately.

1) Measure from the clavicle to the bottom of the port. _____ inches

2) Measure from the top of the shoulder to the bottom of the abdomen. _____ inches

3) Measure the width from shoulder to shoulder using the back side. _____ inches

4) Measure the circumference of the stomach (all the way around). _____ inches

5) Indicate which side the subclavian port is on. ☐ right ☐ left

6) If the port is located in a different area (e.g., on the side), measure from the clavicle or middle of the armpit to the bottom of the port and state location. _____ inches _____ location

7) Measure the head circumference. _____ inches

8) Is this shirt for a ☐ boy or ☐ girl.

SMALL AND MEDIUM SHIRTS = $30.00 plus $10.00 postage

LARGE AND EXTRA LARGE SHIRTS = $35.00 plus $10.00 postage

All shirts will be made from fleece unless 100% cotton is requested. Because shirts are custom made, specific delivery dates cannot be given. Make checks payable and send to:

Gina Stephenson
396 Floyd Fork Drive
Shepherdsville, KY 40165

Checks must clear bank before shipment.

Have you visited the Member's Only section of the National MPS Society Web site yet? If not, you are missing out!

The Member's Only site offers:

MPS Memorials — A new section recently added by the Family Support Committee where families can submit a memorial tribute and photo to be viewed online by other members of the Society. To submit a memorial, contact Laurie Turner at laurie@mpssociety.org.

Remembering Our Children — A memorial listing of those who have lost their battle with MPS and related disease. This section was created at the request of our membership. Due to timing, it is often several months after a child has passed that members receive their copy of *Courage* and find out about their friend's passing.

Membership Directory — Do you need to find another member's phone number or address and can't find your membership directory? Now you can download a copy and save it to your computer, so you will always have it ready when you want to chat with other members.

Contact Information — We know it is hard to remember to tell everyone when you have a new address, phone number or e-mail address. You can let the Society know of theses changes with just a few clicks of your mouse.

MPS Publications — Past issues of *Courage*, *Angels Among Us*, *Guide to Special Events* and annual reports are just a few of the publications available for download. In addition, *Courage* is usually posted before it is mailed, so if you can't wait for the latest issue to arrive in your mailbox, stop by to read it.

Please take a few minutes to visit the Member's Only section of our Web site if you haven't done so already. Also remember this section is constantly updated, so visit often for news and updates.

Send an e-mail to laurie@mpssociety.org if you need your member sign in and password.

UnitedHealthcare Children's Foundation to Offer Medical Assistance Grants

The UnitedHealthcare Children's Foundation (UHCCF) is offering support to meet the needs of children across the United States with assistance grants for medical services not fully covered by health insurance.

Parents and caretakers across the country will be eligible to apply for grants of up to $5,000 for healthcare services that will help improve their children's health and quality of life. Examples of the types of medical services covered by UHCCF grants include speech therapy, physical therapy, and psycho-therapy sessions; medical equipment such as wheelchairs, braces, hearing aids, and eyeglasses; and orthodontia and dental treatments.

Any child 16 years old or younger living in any UnitedHealthcare region of the United States and in need of financial assistance for healthcare services will be considered eligible for a grant. Families must meet economic guidelines, reside in the United States and be covered by a commercial health insurance plan.

For program guidelines and eligibility requirements, visit the UHCCF Web site at **www.uhccf.org**.

Have an Extraordinary Experience

The Family Support Committee is pleased to introduce Extraordinary Experiences, the newest Family Support program. Extraordinary Experiences was developed to help all of our unique and special children, regardless of their diagnosis or abilities, to create their own extraordinary experience.

This program was developed specifically for individuals ages 14–24 diagnosed with MPS and related diseases. Grants of up to $1,000 are available for special camps, events, class trips and other unique opportunities. The Society will partner with the individual and his or her family to help cover registration fees and travel expenses, and other fees associated with his or her extraordinary experience.

Extraordinary Experiences was initiated in response to a grant the National MPS Society received from BioMarin/Genzyme LLC to honor Spencer Holland (MPS I). Spencer was a pioneer and trailblazer for many individuals diagnosed with MPS. He and his sisters participated in clinical trials for enzyme replacement therapy. They openly shared their life experiences which inspired other children and adults affected with MPS. Spencer will always have a special place in our hearts.

The National MPS Society thanks you, Spencer, and everyone with MPS and related diseases for being an inspiration to many. Special thanks to BioMarin/Genzyme LLC for this wonderful gift which will allow special young adults to have an extraordinary experience.

More information about Extraordinary Experiences can be found at **www.mpssociety.org** or by contacting Laurie Turner at laurie@mpssociety.org or 207.843.7040.

Mini-Miracles

Mini-Miracles is an adaptive clothing line designed for the special needs child. Their philosophy is to promote dignity, comfort and quality of life for every child, while giving back to the community by donating to local Canadian charities.

Mini-Miracles clothing line consists of items chosen to meet the basic needs of the special needs child. They reflect the research and experience that the owner, pediatric special needs nurse Victoria Allen, has acquired working with special needs children.

The clothing is made of the highest quality Peruvian cotton and has different options available for different needs (e.g., gastro-intestinal feeding tube access pocket, cut to fit diapers, etc.).

Featured adaptations:

- Flat seams to reduce friction
- Discrete adaptations so clothing looks normal
- Easy access—snaps, Velcro, stretchy fabrics
- Roomy seat to accommodate diapers (all ages)
- Longer rise in the back to accommodate sitting in a wheelchair
- Elastic waist for ease of dressing and increased comfort
- Meets Health Canada regulations for safety and flammability

Suitable for:

- Limited range of motion and impaired dexterity
- G-tube feeding
- Wheelchair
- Cerebral Palsy
- Muscular Dystrophy
- Developmental disabilities
- Contractures
- Toilet/changing needs
- Catheters and colostomy bags
- IV tubes
- Rigidity

Visit **www.minimiracles.ca** for more information.

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.

GOLD Web site

www.goldinfo.org

The Global Organization for Lysosomal Diseases (GOLD) is an international collaboration of scientific and medical associations, patient groups and commercial organizations dedicated to improving the lives of all patients with a lysosomal disease. The GOLD Web site, **www.goldinfo.org**, has general information about lysosomal storage diseases (LSDs), symptoms and inheritance patterns, plus information about specific LSDs. To access all the discussion forums and details on the member directory, register on either the Discussions or Member page.

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.

Drug Safety Information

www.fda.gov/cder/drugSafety.htm

The U.S. Food and Drug Administration (FDA) has added a new feature to its Web site that provides consumers with specific safety information about individual drug products. The new Drug Specific Information Web page presents information on more than 200 approved drugs. Each drug has a "core page" with links to all information about the product on the FDA's Web site, including general risk information and any past alerts issued about the drug.

Health Care Coverage Options

www.nahu.org/consumer/healthcare

The National Association of Health Underwriters has created a database that provides information on health insurance options for low-income U.S. residents and people who change jobs or have pre-existing health problems. The Health Care Coverage Options Database provides a state-by-state list of public health insurance programs for low-income residents, such as Medicaid and the State Children's Health Insurance Program. It also lists 32 states that offer high-risk health insurance pools for those with pre-existing health problems.

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

Genetics Home Reference

http://ghr.nlm.nih.gov/handbook

This Web site contains a comprehensive "Guide to Understanding Genetic Conditions," with basic information about genetics in clear language and links to online resources.

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.

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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