FALL 2012

courage

Volume 36 | No.3

National MPS Society

Support for Families. Research for a Cure.

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

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!

ISSUE
SPRING
SUBMISSION CUTOFF DATE
January 1

ISSUE
SUMMER
SUBMISSION CUTOFF DATE
April 1

ISSUE
FALL
SUBMISSION CUTOFF DATE
July 1

ISSUE
WINTER
SUBMISSION CUTOFF DATE
October 1

To submit information to *Courage*, please send text (preferably via e-mail) to the address below. 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.

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

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

TABLE OF
contents

ON THE COVER

Anyssa, Karina (MPS III), Luis and Angela Guajardo

Paige (MPS I), Carla and Blake (MPS I) Halk

Dominic (MPS II) and Freddy Henriquez

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.

LETTER FROM THE

president

IT WAS WONDERFUL to see all of you at our recent family conference in Boston! The energy generated by bringing all of our families together in one place is palpable.

Following are my remarks to the federal advisory committee regarding adding MPS I to newborn screening panels in each state. The committee approved moving MPS I to the next and final step of evidentiary review where a final vote will be taken. While these remarks are specific to MPS I, they easily apply to all of our MPS syndromes as more treatments become available:

"Once your child receives a diagnosis like MPS I, a parent feels an overwhelming desire to make things right by that child—to create as equal of a playing field in life as possible for that child—who obviously was born with a huge disadvantage of having a terminal genetic syndrome through no fault of their own. One of the most important ways of doing that is by providing them with a medical treatment that will help prevent further damage by their condition and help sustain their life, whether that be through a stem cell transplant or weekly enzyme replacement therapy.

The problem is we cannot begin these treatments until we know they have the disease. It often takes many months and sometimes years between noticing there is a problem to getting a diagnosis. During this time, irreparable harm is being done to our children that a future treatment will not be able to reverse. This delay in diagnosis and treatment often creates parental guilt and regret for not following up sooner on these problems, or for not forcing their pediatricians to follow up on these early symptoms when the pediatrician dismisses the parental concerns as complaints of an overzealous parent. Once it is too late, parents realize they lost precious time when early treatments could have forever changed their children's long-term clinical outcomes.

Secretary's Advisory Committee on Heritable Disorders in Newborns and Children voted unanimously May 17, 2012, to move the MPS I nomination to evidence-based review. The goal is that MPS I will be added to the panel of diseases screened in the newborn period. Pictured: Amy Holland, Steve Holland, Maddie Holland, Laynie Holland, Barbara Wedehase, Stephanie Bozarth, Dr. Joan Keutzer.

However, with newborn screening, all of this regret, guilt and conflict with the medical community over a delayed diagnosis are eliminated. Treatment by stem cell transplant, enzyme replacement therapy or whatever new treatments are around the corner can start immediately. Evidence shows that the long-term clinical affects of MPS I can virtually be eliminated by early treatment, giving that child the level playing field that we as parents so desperately desire.

I understand there are concerns over false positives and the resulting parental anxiety that can create. However, such anxiety is short lived compared to the permanent damage caused by the untreated disease in the months or years following birth. I predict that the recipients of false positives barely remember the event a few years following birth. I know parents dealing with a delayed diagnosis and treatment remember it and live with it for a lifetime. What would my child be like if they had only received treatments since birth?

Another important benefit from newborn screening would be reducing the births of affected siblings. In my family, all three of our children were affected even though those odds were one out of four with each birth. Because our kids were born close together and had an attenuated form of the disease, we didn't realize there was a problem while we were having children. If newborn screening had indicated my son had MPS I, we would have used the benefits of genetic counseling to prevent my other children from being affected. We know many families with more than one affected child that indicate they would have done the same thing, reducing the overall prevalence of the disease and the resulting demands on society in general and on the family specifically.

So, it just comes down to time and options. We have the ability to prevent most of the permanent damage caused by MPS I by providing parents with treatment options at birth. Let's do it!" ❁

Steve Holland

LETTER FROM THE

executive director

THIS YEAR'S FAMILY CONFERENCE was filled with surprises—not just for attendees but for us, too! Our attendance of more than 400 was about one-third more than anticipated based upon past conferences. Our conference committee listened to your past comments and suggestions, and initiated changes: later and longer breakfast time, lunch with the experts, separate section for newly diagnosed families, and the social hour before the banquet, to name a few. We were thrilled to see so many of you and delighted that you embraced the changes.

It's always interesting to view our conferences from someone new to our MPS family, and that's what Erin Eaton writes about on page 33. Erin was our genetic counseling graduate student who interned with us this summer, and she succinctly describes the unique relationships among the families and the time the parents and siblings spend together. We are grateful to our board member, Gordon Wingate, who filmed the speakers and spent hours formatting their PowerPoint presentations so they can be available to all our members online.

The conferences for adults with MPS and related diseases (SPIRIT) and for our bereaved families (CYCLE) each had about two to three times more attendees than in the past, and both received high marks for the sessions and overall quality. Requests from the adults to hold their conference every year will be considered by the board of directors.

A couple weeks before the conference we received the first draft of the fundraising segment of the video you've been hearing about for last few months. We showed it at the Boston conference and everyone thought it was amazing. We'll soon be sending a blast e-mail with information on how you can access all the video segments. Beth Karas, whose brothers Jonathan and Joe had ML III, is leading this project. Beth identified a producer in Los Angeles, Jamie Witker, with whom we've been working. Jamie and his crew spent hours with families in their homes and quickly absorbed the meaning of living with MPS and ML.

Thanks to your generous donations and to the hard work and dedication of everyone who hosted a fundraiser and walk/run in 2011, we are funding three new research grants for 2012–2013. A special thanks to Team Sanfilippo who helped support the MPS III Grand Challenge Grant. The total amount of money provided in 2012 for research is $547,000, which includes the second year funding for the 2011 grants! Detailed information about the grants funded in 2012 can be found on page 44 and on our website, www.mpssociety.org. We are indebted to the members of our Scientific Advisory Board for their reviews of the grants. These scientists and clinicians, whose names are listed on the back page of *Courage*, provide their expertise not only to grant reviews but to the overall focus of our research program. We also request their advice for our legislative issues.

SEATTLE CHILDREN'S HOSPITAL AT CAMP KOREY

Denise Dengel, Dr. Paul Harmatz, Barbara Wedehase

Dr. Klane White, Barbara Wedehase

Don't forget to update us about the happenings in your lives along with photos.

Barbara Wedehase

LETTER FROM THE **DEVELOPMENT DIRECTOR**

THIS YEAR IS MOVING SO QUICKLY and we have a lot of excitement to share with you! During the 26th Annual Family Conference in Boston we were honored by two new philanthropists, Brooke Carter and Lisa Todd, as they spoke with attendees and shared their stories about inspirational fundraising. Little did any of us know just how much we would be touched by their active role in fundraising and the family members they cherish who suffer from MPS.

For those in attendance, we were extremely fortunate to preview our new video created by Beth Karas and Jamie Whitker of Outland Films in Los Angeles. This new video gently shares the story of MPS and ML while asking for help and illustrating hope. This piece is to be used to help educate and fundraise exclusively for National MPS Society programs. Throughout the remainder of the conference many families approached me about how this video had moved them. We will let you know when the final compilation will be available for your family.

We are pleased to announce the launch of ***Donor Pages***. This new and exciting way for families to share individual stories, and raise awareness and funds for MPS and related diseases will be available to members on our website. Each family will have the ability to share a photo and tell a small story about their loved one while raising funds. This new program will be hosted directly under www.mpssociety.org. Technology is ever changing and the Society is working hard to bring you tools that will make fundraising easy and fun! Contact me directly to sign up for the fall launch of ***Donor Pages***.

Currently we are in our 13th walk/run season. Please review a list of this year's events on page 16. If you can't attend an event in person, consider becoming a part of the Sponsor A Child For A Cure program. There are many opportunities for families to contribute to Society programs and we need your help now more than ever this season.

The 11th Annual Fund campaign is under way; materials have been mailed. We are honored to have Kim Whitecotton and her mother Lennie Forkas as our chairs for the 2012 Annual Fund. The ability for the Society to continue valuable programs would suffer without your contributions. Please make a donation and recruit friends, family and colleagues to our cause. Start an Annual Fund awareness event and host family and friends for a day or night of fun.

Members and friends of the Society recognize that while others raise awareness for our cause, we have the ability to raise money for research and family support, moving the mission forward for treatments and cures. We are always trying to find creative ways to spotlight our affected loved ones and raise money to eradicate these diseases.

It was a pleasure meeting so many new faces at the Boston conference and talking with you about raising needed funds for Society programs at our fundraising booth. If you have the courage to step out and help us raise funds for family support programs or valuable research that could provide treatments and help save lives, contact me at terri@mpssociety.org or 919.806.0101. ❁

Terri Klein

LETTER FROM THE **PROGRAM DIRECTOR**

THE FAMILY SUPPORT COMMITTEE is as busy as ever. We helped families attend the family conference with travel scholarships, as well as scholarships for newly diagnosed families. The Continuing Education Scholarship Program funded $25,000 in college scholarships. This year we received a record number of applications and wish we were able to fund everyone. The Medical Travel Assistance Program has helped families make trips to see specialists such as Dr. Muenzer, post bone marrow transplant follow-up evaluations at UMN and Duke, and other medical institutes. The Emergency Relief Program has helped families facing a financial crisis by covering costs such as bereavement expenses, utility bills, gas for medical appointments and rent/mortgage payment, just to mention a few. The Family Assistance Program has funded hearing aids, adaptive chairs, an adaptive trike and a pulse oximeter. We have helped fund regional social events. There is still time to plan an event in your area. What could be better than an afternoon with your extended MPS family? The changes made to the Extraordinary Experiences program have allowed us to fund some amazing special trips for young individuals with MPS and related diseases. We also have funded church camp expenses, airfare to attend the International MPS Symposium and a MacBook.

All programs still have funding available for 2012. Please contact me if you have any questions. Stay tuned in early 2013 for information on Continuing Education Scholarship applications and in the spring for conference scholarships.

The Family Support Committee is looking for individuals interested in serving on the committee. We also want to hear any ideas you might have on new programs for the future and suggestions for current programs. We want the programs to be the best they can be, but we need your help to make this a reality.

Don't forget to send us an update —we want to hear how your family is doing and we love pictures. I know many of you are in daily communication via Facebook and blogs, but let's not forget the importance of sharing in *Courage*.

Laurie Turner

NATIONAL MPS SOCIETY **MEMBERSHIP FORM**

YOUR NAME

AFFECTED INDIVIDUAL'S NAME

DATE OF BIRTH

DIAGNOSIS

RELATIONSHIP

ADDRESS

CITY / STATE / ZIP

PHONE

E-MAIL

Please send your membership form and check to:
National MPS Society / PO Box 14686 / Durham, NC 27709-4686

Join or renew your membership online at www.mpssociety.org/become-a-member.

☐ $50 Family
☐ $80 Foreign
☐ $75 Professionals
Corporate Memberships Available

Would you like your name to appear in our online 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

congrats grads!

Morgan Paige Lewis, 18, sister of Madison Lewis (MPS III)

Scotty Whitecotton (MPS II), 14, graduated 8th grade on May 25 with special recognition for making the Honor Roll the entire year.

Autumn Mortensen (MPS VI), graduated from Sprague High School, Salem, OR, on June 8, 2012. Autumn graduated with a 3.9 GPA including advanced placement courses. She was awarded a two-year scholarship at Chemekata Community College.

Ensuring a Successful University Transition

by Jennifer Klein (ML III)

When I was accepted this past spring to North Carolina State University I was overjoyed at the opportunity to continue my academic pursuits. Like many other students I worked hard and am proud of myself for making the grade and their acceptance criteria. After the initial jubilation wore off, I began to wonder, can I actually do this?

My studies in human biology and psychology were not my concern. I questioned if I could physically do this. I began to make a list of questions for myself and the university. Most of my concerns were about physical logistics of apartment and classroom location. I also questioned my weight-carrying restrictions and would I be able to use motorized assistance easily on campus.

NC State University and their Disabilities Service Office (DSO) were more than accommodating. They provided me with an individual plan for a successful university career. Their philosophy is teamwork. We work as a team to foster equal access to programs, services and the work environment for persons with disabilities. They embrace people first, teach advocacy, encourage the spirit of equal opportunity and strive to achieve a diverse, inclusive university. I was thrilled when they shared with me, "We embrace and celebrate that 'disability' is just another great way to be alive."

Following are some suggestions from the DSO. I have happily implemented all of these, and I hope they might benefit you or your child if they have the opportunity to attend a university.

- Rent an iPad from the university to download text books and have portable, light-weight access to online functionality.
- Break all the bindings on text books and have them scanned directly to the iPad by the DSO staff.
- Buy large binders and three-hole punch text books and carry only those chapters necessary for class that day. (This will eliminate having to carry heavy textbooks throughout the day.)

continued >>

>> • Use a Smart Pen. The university gave me this to record notes I take in class. The Smart Pen's recording will match up with your note taking by simply touching the Smart Pen to your paper.

• The DSO gave me carbon paper so someone can assist me with note taking in class.

• Take a personal tour of the bus transportation system. The DSO provided me with a guided tour of my fall schedule and matched it up to the bus transportation. This was extremely helpful!

• Review all your choices for disability housing and accommodations. My apartment is located on campus on one floor with handicap accessible openings and an elevator next to my apartment door.

• Get multiple copies of your campus map, it will become an essential tool.

• Have stepstools and meet the maintenance staff. This will allow you to reach high places and have direct access to someone close by in case of an emergency.

• Get a list of all the group activities on your campus and get involved, it is a great way to meet people right away!

Haley Miller (sister of Danny, MPS II) graduated from Avon Grove High School in West Grove, PA, and is on the National Honor Society. She received the Student of the Year award at the technical high school where she studied education. Haley will be attending Millersville University in Lancaster, PA, with a major in early childhood and special education. She would someday like to be a special education teacher for preschool students.

Madison Noll, sister of Austin (MPS III), graduated maxima cum laude from St. Francis High School in Mountain View, CA. She will be attending the University of Michigan in the fall.

Michael Sheridan (MPS I) recently graduated from Eastview High School, Apple Valley, MN.

Jason Vanderpool (MPS III) graduated from Mary Cariola Children's Center on June 15.

Justin White (MPS IV) graduated from Highland High School in Gilbert, AZ.

SOCIETY ANNOUNCES **CONTINUING EDUCATION SCHOLARSHIP RECIPIENTS**

Congratulations to all those who applied for the 2012 Continuing Education Scholarship Program. The Family Support Committee is pleased to offer 25, $1,000 scholarships for individuals affected with MPS and related disease, their parents, children and siblings. The scholarships will help the following individuals as they continue their secondary education. Congratulations and best wishes to all of the applicants.

Nicholas Boyce is a graduate of East Providence High School and is in his third year at College of Providence, where he is majoring in sociology with a minor in black studies. Nick's goal is to work in law enforcement, specifically either the Federal Bureau of Investigation or the Drug Enforcement Administration. Nick does not allow the fact that he has MPS I slow him down, and is proud that he is able to live on his own. While at college Nick works as a park ranger and grounds keeper for the Rhode Island Department of Environmental Management.

Kyle Brockman is attending Lebanon Valley Technical College where he is majoring in biology with plans to continue on to medical school and become a doctor. Kyle is able to balance his college life and his life with MPS II, making adjustments in his schedule to allow for doctor appointments and his weekly Elaprase infusions. Kyle refuses to allow MPS to put a limit on what he can do, but he does allow it to make him a better person.

Jenna Caswell is in her fourth year at Keene State College, where she is majoring in special education. Jenna plans to join the Special Education Masters Program upon receiving her undergraduate degree. Jenna's brother, Sam, has been an inspiration as he faces the daily struggles with MPS I. Jenna hopes to be able to focus on the abilities of the children she will educate, not their disabilities.

Ryan Dant attends Brookhaven Community College with plans to graduate in 2014. He will continue on to a four-year university to complete his studies in sports administration and wants to work in sport administration/equipment management at a major university. Ryan currently is working as the equipment manager at Southern Methodist University for the football team. He is responsible for getting game-day uniforms ready and the helmets looking nice. Ryan has MPS I, and loves living like a normal young adult.

Nicholas DeVolder is a graduate of Olivet High School. Nicholas plans to major in economics or engineering, and hopes to make a positive impact in his community and world. Nicholas was able to donate his bone marrow to his brother, Luke, who has MPS I. He hopes to be able to continue to educate his peers about the importance of bone marrow donations and also encourage his college peers to be tested for the bone marrow registry.

Emily Durcholz is a second year student at Indiana University, where she is studying business administration and minoring in Spanish. Emily plans to work in the marketing or advertising fields. She is inspired by her brothers, Ben and Zach, who have MPS III. Zach passed away in 1998. Emily's proudest accomplishment is having hosted "Beat it For Benny," a 2010 walk fundraiser for the MPS Society. Emily finds tremendous joy in knowing that she helped put MPS families a little closer to a cure.

Mitchell Finzel is a graduate of Morris Area High School and is attending the University of Minnesota-Morris. He plans to study genetics and become a researcher or genetic counselor. Mitchell would like to one day cure diseases like MPS, and also spread awareness and understanding of rare diseases to the rest of society.

Taylor Harvey is a graduate of Sperry High School and is attending Oklahoma Wesleyan University. She plans to make a difference in the lives of children as she pursues a career in the pediatric medical field. Taylor is inspired by her little brother, Tad, who has MPS II and is her best friend.

continued >>

>> **Jennifer Klein** is attending North Carolina State University majoring in biology and psychology. She plans to continue on to medical school and eventually work with children with rare diseases, either in a doctor's office or a clinical lab. Jenny has ML III which has taught her to be strong and to not take people, things, days or even her life for granted. Each day Jenny finds a way to laugh and smile.

Chelsey Klenke is a second year student at Missouri Baptist University where she is majoring in psychology. Chelsey is looking forward to working with children and their families as they deal with rare genetic diseases and special needs. Her brother, Kraig, had MPS II and passed away in 2010. Kraig continues to show Chelsey how to appreciate and respect life and other people.

Kyle Lingo is in his second year at Baldwin Wallace College where he is studying sports management/ communications. Kyle hopes to coach and scout at the high school, college and professional levels, with a goal to coach in the NBA. Kyle has MPS IV, however he will not allow it to stand in his way. He credits MPS IV as making him a more mature, determined and positive individual.

Frances Madsen is attending Michigan Technological University majoring in electrical engineering and will graduate in 2013. Frances has MPS I and is proud of being able to find a balance between her health needs and academic needs, all while attending college six hours away from home. Living with MPS has given Fran the opportunity to become a stronger person. She has learned valuable lessons from every obstacle she has had to overcome due to MPS I.

Joscelyne Mason is in the nursing program at North Carolina Central University with plans to graduate in 2014 and to continue her education to become a nurse practitioner specializing in pediatrics. Joscelyne has passion to help others and will be able to help many in her career path. Her brother, Khye, has MPS II and is her inspiration to pursue medicine.

Charlena Melnyk is a graduate of The Woodlands High School and is attending Texas A&M University, where she is majoring in pre-medicine. Charlena hopes to work in genetic or cancer research. Her brother, Nick, has MPS II, and she hopes to be able to help others in her career path.

Nicholas Melnyk is in his third year at Texas A&M University where he is majoring in computer science. Nick has MPS II and wants to learn programming skills so he can touch people's lives through the Internet developing applications or through video games. Nick is successfully able to balance college life with his infusions and appointments. He hopes to use his talents and potential to better society.

Haley Miller is a graduate of Avon Grove High School and is attending Millersville University majoring in early childhood/special education. After college, Haley hopes to be able to teach at The Child and Career and Development Center where her brother, Danny (MPS II), attends. She is proud to say Danny is different and special because if he wasn't she would never have become the person she is today.

Heather Millington attends Huntington University, where she is majoring in psychology with plans to become a counselor after she completes graduate school. She hopes to open a ranch which will help serve children in need as well as abused or neglected horses. Heather is inspired by her sister, Hope, who had MPS VI and passed away in 2008.

Joseph Morris is a graduate of Chanute High School and will be attending Kansas State University majoring in pre-medicine. Joseph, who has MPS II, plans to attend medical school after obtaining his undergraduate degree and eventually become a radiologist. Living with MPS has inspired him to go above and beyond while continuing his education, and know he can do what other kids do.

Autumn Mortensen is a graduate of Sprague High School and is attending Chemeketa Community College where she is studying English with some business and computer studies. Autumn has MPS VI and plans to work in the publishing/editing field. Autumn strives to always look on the bright side and focuses on the power of her mind, creativity and her ability to absorb knowledge. People often tell her she is a strong person, but she thinks of herself as optimistic.

Edward Ruhnke is a graduate of Moorpark College and is attending Sacramento State University with plans to graduate in 2015. He is majoring in special education, and plans to continue his education to obtain his master's degree. He hopes to eventually teach his own methods at the college level. He credits his sister, Carly (MPS IV), with altering his life for the better. She has taught him that nothing is impossible; you can find a way to make it work.

continued >>

>> **Megan Sheridan** is a graduate of Vermilion Community College and is continuing her education at Augsburg College with plans to become a physician's assistant, with the ultimate goal of working in a children's hospital. Her brother, Michael, has MPS I and she is honored to be his sister. She admires his irrepressible and unwavering zest for life, and his ability to do absolutely anything he sets his mind to.

Katherine Slavik is in her second year at Cleveland State University in the College of Engineering, with a major in mechanical engineering. Kat plans to develop products to help people with illnesses and handicaps. Her brother, William, has MPS II and has given her the gift of caring and sharing.

Jacob Todd is a graduate of La Cueva High School and is attending the New Mexico Military Academy. He hopes to work for the CIA, FBI or another government agency. His little brother, Jack, has MPS II and has taught him to take advantage of life, appreciate your loved ones and to be the best you can be every day.

Becky Von Handorf is a graduate of Newport Central Catholic High School and is attending Northern Kentucky University where she is majoring in criminal justice with plans to continue on to law school and become an attorney. She hopes to make a difference by helping people receive their justice. Her sister, Aly, has MPS III and has taught her to not sweat the small things, and that life is not always what you wanted or expected, but you have to make the best of what you have been given.

Cameron Watkins is in his second year at Dalton State College, majoring in English, and hopes to pursue his dream of becoming an author. Cameron has MPS II and is proud that he has succeeded despite the obstacles he faces. He is happy with the person he has become and would not change anything.

OUTSTANDING SIBLING, CHILDREN AND RELATIVES

OSCAR award

The National MPS Society's OSCAR awards honor the brothers, sisters, children and relatives of our children with MPS and related diseases. This special group of individuals provides support, humor, direction and most of all unconditional love. In each issue of *Courage* we will honor super siblings who shine each and every day. To nominate someone in your family for an OSCAR award, please send an e-mail to Laurie Turner at laurie@mpssociety.org.

In this issue, we honor Anyssa Guajardo, sister of Karina (MPS III).

Anyssa was nominated by her mother, Angela, who would like to recognize her work with organizing an MPS Day fundraiser where she designed purple T-shirts and sold them at her school. Anyssa also made an announcement about MPS and her sister, and asked everyone to wear purple for MPS Awareness Day.

International MPS Day, May 15, is a way to honor everyone in the MPS community. It offers an opportunity to remember all the children and adults who suffer from MPS and related diseases; to think about the children we have lost; to recognize the doctors and scientists who are dedicated to finding a cure; and to remember each other and be thankful for the strength and support we both give and receive.

international mps day

Caitlin and Tom Thornton supported their brother John (MPS III) on MPS Day by wearing Team John shirts to school, along with many of their teachers and friends. The siblings handed out purple bracelets that said "Faith, Hope, Cure," purple M&Ms that said "Help us cure MPS," and packets of information about MPS that contained a "Text to donate" card.

Kemp, TX, showed its support of nearby resident Luke Chambers (MPS I) by including mentions of MPS Day in the First Baptist Church Kemp bulletin and displaying an MPS Day awareness message on the Kemp Pharmacy sign.

Jill, Chris and Lucas (MPS III) Valdez celebrate MPS Day.

MPS III friends Levi Omeroid, Lucas Montgomery and Lucas Valdez celebrated MPS Day in Chicago at the Brookfield Zoo.

Sam (MPS I) and Jenna Caswell on MPS Day.

What started as a simple Facebook post by Cameron Watkins to raise awareness turned into a full-fledged MPS Day event. After receiving many questions regarding his post, Cameron decided to distribute purple courage ribbons and a card with MPS information at Dalton High School, Christian Heritage School and Northwest High School, where a segment on MPS will be added to its health class curriculum. Employees at several local businesses also wore ribbons that day.

Thanks to Cameron's friends Kala Barrett and Mikaela Lamm, and his cousin Savannah Pierce, 5,000 ribbons were assembled in less than a month.

Valley Christian High School girls soccer team in Oshkosh, WI, honored Lucas Valdez (MPS III) by wearing purple for warm ups at their soccer game on MPS Day.

fundraising news

Donor Pages – A New and Exciting Tool for Fundraising Frenzy!

The National MPS Society continues to bring innovative fundraising tools to its members by launching Donor Pages.

Donor Pages allows you to create a personalized web page under the National MPS Society website. Once created, invite everyone you know via e-mail or social media networking sites, such as Facebook or Twitter, to visit your site and honor your cause. Your Donor Page can tell your story, share a photo and include your fundraising event and/or the goal amount you are trying to raise. You can share a donor recognition list and include additional links for an upcoming event.

Contact Terri Klein at terri@mpssociety.org or 919.806.0101 to receive your signup sheet and template to begin building your Donor Page. The Society will input all data and upload photos. We want this to be as easy as possible to spread the word about MPS and related diseases and bring a dynamic, individual approach to raising funds. Whether you have a goal for individual fundraising or event fundraising, Donor Pages is a great companion tool.

How Many FANs Do You Have?

by Tom and Anne Gniazdowski

If you are not a famous actor, musician or professional athlete, you probably think you don't have any fans—you may have more than you think. Your **F**amily, **F**riends, **A**nd **N**eighbors are your personal fans and they can help you raise money to find a cure for MPS and related disorders.

The National MPS Society's FAN Program is designed for the family that wants to participate in fundraising but does not have the time to host a major event like a walk/run, golf outing or bowl-a-thon. You can reach out to your network of family, friends and neighbors by using a simple mailer that you create to tell your story about your child. It is an opportunity to educate people about your child, MPS, the work of the Society, and to ask for a donation to support the Society's mission to find a cure for all MPS and related disorders.

Contact Terri Klein at at terri@mpssociety.org or 919.806.0101 if you are interested in utilizing this easy fundraising tool to reach out to your FANs.

WAYS TO GIVE

- Renew your membership or sponsor another family
- Gifts in honor of a special person
- Gifts in memory of a special person
- Matching gifts through your employer (check with your human resource office)
  1. Request a matching gift form from your employer
  2. Complete the employee section of the form
  3. Mail to the Society and we'll do the rest
- Contribute through the Combined Federal Campaign if you are employed by the federal government—CFC #10943
- Designate the Society as a member of your local United Way. You will need to supply them with the Society's name, address and Federal ID number (FEIN #11-2734849)
- Annual Fund donation
- Major gift (usually 10 times that of your Annual Fund gift)
- Planned gift
  1. Bequest in your will
  2. Charitable remainder trust or charitable gift annuity
  3. Charitable lead trust
  4. Life insurance policy
  5. Gift of appreciated assets (stocks, mutual funds and bonds)
- Gifts may be applied to the Society's general operating purposes or restricted to one of our designated programs.

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

Concert for a Cure

On June 24, Jeremy Mask held his 2nd Annual Concert for a Cure for MPS at the Sellersville Firegrove. More than 400 people attended the all-day event, with 11 local bands performing. Local vendors sold goods, a children's area offered face painting, sand art, balloon animals and string art, food was available for purchase and gifts were raffled off from local businesses. For the second year, Haley Miller, sister of Danny (MPS II), baked nearly 650 cupcakes for the event.

The event raised nearly $5,500 for MPS. Besides raising money and awareness to so many new people, the number of MPS families that showed up was wonderful! The Millers and Langans from PA, the Kapes' from DE, the Taorminos from MD, the O'Connors and Vanderpools from NY, the Rodrigues' from CT and the Espinolas from VA were all there to enjoy the day.

Sponsor A Child For A Cure 2012— Don't Get Left Behind!

If your family has wanted to participate in a walk/run event to raise money for research, we have created that opportunity. This program reaches out to families and members of the Society that want to sponsor an affected loved one or a child who has passed away, and help find cures for MPS and related diseases. Walk/runs across the country will be participating in this program in 2012.

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

In turn, the event will:
- Assign a runner to participate on behalf of your loved one
- Send you a courage medallion and photo with your assigned participant

The assigned runners are inspired by our heroes of MPS. Together they pave the path of continued hope. The photos and amount raised will be published in an upcoming *Courage* magazine. For more information on the Sponsor A Child For A Cure program, contact Angela Guajardo at angela@mpssociety.org.

Inspire to Give

National MPS Society 11th Annual Fund Campaign

The Annual Fund is a mailing campaign, sent out to members, friends and families each summer with a reminder in the fall. Giving to the National MPS Society's 2012 Annual Fund campaign is a partnership opportunity. By donating, your family is supporting the National MPS Society's work of providing support for families. Donate through the mail or online—together, we can make a difference.

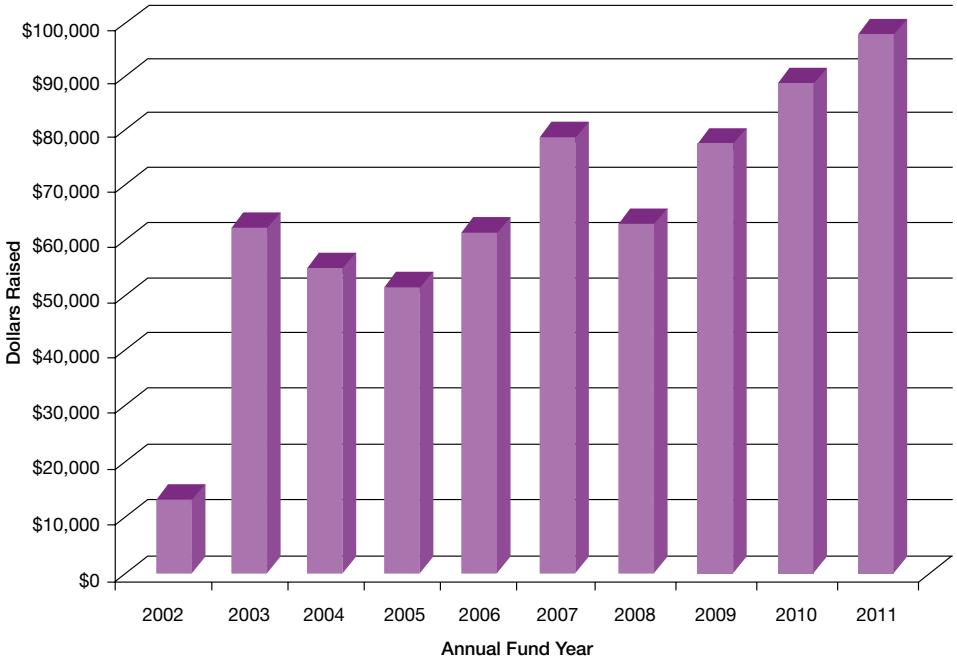
This year's Annual Fund chairs are Kim Whitecotton, a current board member of the National MPS Society, and her mother Lennie Forkas. Their touching and extraordinary experience with MPS has transcended generations within their family.

Since its inception, the Annual Fund has raised more than $615,000 and supports critical programs such as:

- family support
- continuing education scholarships
- medical equipment
- conference scholarships
- legislative advocacy
- member services
- educational materials
- website
- special projects

The Annual Fund continues through the end of the year. Make a donation and let us recognize your gift in our Annual Report.

Annual Fund Donations



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 website for more information or to post your event.
- For free MPS Society brochures and donor envelopes, or to submit information for the website or *Courage*, send an e-mail to Terri Klein at terri@mpssociety.org.

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

Consider hosting your own fundraising event. Whether large or small, the rewards are endless.

Fundraising Committee:

Stephanie Bozarth
Ernie Dummann
Toni Ellard
Anne Gniazdowski
Tom Gniazdowski
Steve Holland
Larry Kirch
Terri Klein
Austin Noll
MaryEllen Pendleton, *chair*
Kelly Rose
Lisa Todd
Laurie Turner
Barbara Wedehase

Anne Schnare, grandmother to Logan Piefer (MPS II), with the Piefer family

From One Generation to the Next

Logan Piefer (MPS II) has tremendous support behind his fight with MPS. His family have been fundraising and raising awareness for MPS since 2005.

Anne Schnare, Logan's grandmother, has been running marathons to raise awareness about MPS for seven years! This year she participated in the Vermont City Marathon in Burlington, VT, and raised $2,230. Since 2005 total contributions received from the community have been more than $22,750.

"When I look at Logan and see all he has had to endure over the years and think of all he still has to face, I am determined to keep running until a cure is found," said Anne. "Logan is almost 9 and has lost the ability to talk, eat and play, but still has his sweet smile and disposition. We are thankful for him every day."

Logan's sister, Avery, made bracelets and sold them with her friends at a fundraising event at school last year. Together they raised more than $400 in honor of her brother.

The love for Logan as well as making a difference for MPS awareness has passed from generation to the next.

Upcoming Walk/Run Events

It Works 2nd Annual MPS 5K & Family Fun Walk
Hosted by Kate Martin
Bradenton, FL
Sept. 15

Henry's Walk and Roll for a Cure
Hosted by Melissa Koker
McPherson, KS
Sept. 15

BioMarin MPS 5K Run For Your Life
Hosted by Kathie Ward
Novato, CA
Sept. 23

6th Annual Laps for Lucas
Hosted by Lew and Stacey Montgomery
Cedar Rapids, IA
Sept. 23

13th Annual Run for Erin
Hosted by Stacy Peters
Woodstock, GA
Sept. 29

Post Office Café 21st Annual 5K Run & 1K Fun Run
Hosted by Kerri Rose
Babylon, NY
Oct. 20

11th Annual MPS Run for Their Lives 5K
Hosted by Steve Holland
Fort Worth, TX
Oct. 27

The Gamache family of Geneva, OH, hosted their first fundraising event in honor of their daughter Bethany (MPS III). Inspired to help fund research, the family held a fun-filled auction with gift baskets, food and music. Several MPS families from across the area attended, raising more than $3,000.

National MPS Society Earns 4-Star Rating

The National MPS Society has received four out of a possible four stars from Charity Navigator, America's premier charity evaluator. The four-star rating indicates that the Society adheres to good governance and other best practices that minimize the chance of unethical activities and consistently executes its mission in a fiscally responsible way. Approximately one quarter of the charities evaluated by Charity Navigator have received the four-star, or highest rating, indicating that the National MPS Society outperforms most other charities in America. This exceptional designation differentiates the Society from its peers and demonstrates to the public it is worthy of their trust.

North Carolina 5K Walk/Run for MPS & ML

Mike Schleter with volunteer Chris McEachen at the North Carolina 5K Walk/Run for MPS & ML

After a year of planning we hosted our first walk/run event in our new home of North Carolina! Leslie Phillips, along with my family and a number of volunteers, worked hard to make this event a success. I have attended events in the past and watched as my wife hosted events in the Ann Arbor area, but I really wanted to do something to help my step-daughter, Jennifer Klein (ML III), and raise awareness for MPS and ML.

It is heartbreaking for me to watch this superb young woman struggle throughout different points of her day with the disease that gives such physical pain. She is truly inspiring and has shown me these past five years that "No isn't an option." I needed to do something to help raise money for research to help Jennifer. She and all the other children deserve a treatment.

This past May we hosted the North Carolina 5K Walk/Run for MPS & ML. We created a website (www.mpsrunnc.com) with the help of Jessica Wellman and Wellman Designs. Friends and coworkers volunteered and more than 320 people were at the start line.

It is incredible to witness a community coming together to help our family fight for research and a treatment. The staff, volunteers and sponsors were amazing. Together we raised $15,000 for research and, more importantly, we made new friends throughout the Raleigh area. Following the event, we shared lunch and a balloon release. One of our local families provided music, and it was a great opportunity to get people together to share stories, spend time together and spread awareness. We look forward to the next event on May 18, 2013. Mark your calendars and come join us.

Mike Schleter

Text to Give Embraced in Boston

During the 26th Annual Family Conference in Boston, the Fundraising Committee surprised banquet attendees with a fun and creative way to give to Society programs. The committee has signed on with Mobile Causes to increase giving opportunities and brought the live link functionality to the conference dinner.

A $5,000 dollar goal was set, however members and friends were determined to top that and did so successfully with a total raised of more than $6,000. There were many positive comments and in those brief few hours families and friends worked as a team to reach a goal and fund needed program services.

Contact Terri Klein at terri@mpssociety.org to find out how to bring this interactive tool to your next fundraising event.

There is no amount too small or too large. Donate today by texting "COURAGE" to 41444 on your mobile device. You will be notified by phone by Mobile Causes how to provide your credit card information.

Or quickly donate $10* by texting "CUREMPS" to 80888. Your phone bill will be charged this amount.

**A one-time donation of $10 will be added to your mobile phone bill or be deducted from your prepaid balance. Message and data rates may apply. All charges are billed by and payable to your mobile service provider. Service is available on Verizon Wireless, AT&T, Sprint and T-Mobile. All purchases must be authorized by account holder. By participating you certify that you are 18 years or older and/or have parental permission. Donations are collected for the benefit of MPS Society by Innogive Foundation and subject to the terms found at igfn.org/t. Privacy policy: igfn.org/p. Text STOP to 80888 to stop; Text HELP to 80888 for help.*

family news

Mia Pruett (MPS III) with her service dog Breno.

Luke Sarantinos (MPS III) skiing with his brothers George and Evan.

THERE ARE NO WORDS TO DESCRIBE some people's kindness and giving hearts, especially when this generosity comes in the midst of one family's own personal battles and everyday struggles with a special needs child. We are overjoyed and truly thankful for Casey and Jason Hurst for bringing their wonderful family to Kentucky from Georgia to attend our wedding in April.

We met Casey, Jason and Tucker (MPS II) last year at the MPS family conference in St. Louis and never predicted that nearly a year later our families would come together again. After simple conversations online, many nights at the kitchen table watching Tucker's videos and offering encouraging words to this precious family, we often talked about visiting them. Their willingness to travel all that way with their two sons for a surprise visit was such a meaningful gesture to us, and added more MPS awareness to our local area and to the already purple wedding in memory of Alyssa Louden (MPS I).

Sherri and Lee Louden, with Tucker (MPS II), Casey, Jason and Dylan Hurst

We wanted to share this with our MPS family and friends and let Casey, Jason, Dylan and Tucker know we will NEVER forget one of the priceless memories of our big day that they so graciously made happen. You guys are the BEST!

Sherri and Lee Louden

The National MPS Society funded a Chicago area pizza party held June 12. Four families attended. "We are fortunate that the Society has a program which allows us to make these connections," said Valerie Barnett.

Stevie Barnett (MPS III)

Jake Elston (MPS III) and Christopher Dutcher (MPS III)

Thank you MPS Society for approving the Extraordinary Experience grant to allow us a trip to Sea World! We really needed it and it helped boost the kids' spirits. Things were getting so hard and it seems like we get bad news after bad news, but for those three days I watched (with a smile, pride and so much love) my kids smile, laugh and enjoy life. I see the faith and hope back in our lives and we are back to our everyday fight. Things seem a little lighter since the trip—I think just getting away from it all for a couple of days and remembering the good and fun in life and getting to experience it with family was just what the doctor ordered.

The Halk family

Paige (MPS I), Carla and Blake (MPS I) Halk

Carnival of Courage Brings Fun and Joy

Twenty one MPS families met to celebrate life and enjoy one another at the annual Ohio Regional Gathering. This year's theme was Carnival of Courage. More than 200 attendees enjoyed lunch, including sides and desserts brought by the families to share. Each family introduced their child, the type of MPS by which they are affected, family members present, and how many years they've attended the gathering. Carnival games were played and prizes were chosen. Cotton candy and root beer floats were deliciously consumed.

A professional photographer volunteered her time to take a photo of each family and a group picture, as well as many others. A caricature artist captured many great memories. In addition, a massage therapist was on hand to massage away neck and shoulder aches.

Canines for Companions was present again this year with five dogs to be petted and loved by all the children.

Nathan Roma (MPS VI) with his younger brother and sister at the district swim meet.

Karsyn Wallace (MPS III) enjoyed her first trip to the beach in Clearwater, FL.

Sarantino family at Camp Korey

Rob's Story

Rob and Joy Grandi

"God works in mysterious ways," people have been heard to say. But what they truly mean is that God brings about unexpected results, results they didn't anticipate in their framework of thinking. When God works, it is most often quite rational, but the end result can be quite surprising! That is the truth of our story.

Philip Yancey gave this definition of faith: "Faith is believing in advance what will only make sense in reverse." For the Christian, faith is trusting God's loving heart in advance when surrounding circumstances are exciting or exhausting, delightful or dismal, heart-lifting or heart-breaking, whether they do or don't make sense.

From 1976 until 1998, I was a pastor in what I called a "mobile ministry." I spent many hours driving from my home in Kentucky to various churches and church camps, retreats and revivals, where I would sing and teach or preach the gospel. Many times I visited a small church in southwestern Pennsylvania, and while ministering there, I was a guest in the home of Bob and Joy Jackson and their four children.

In October 2004 I was shockingly grieved to hear that Bob Jackson had lost his life in a tractor accident. I sent my condolences and offered Joy some materials that had helped me in my beginning stages of grief when my wife passed away. Between 2004 and 2008, Joy's and my paths crossed once, when I was in Pittsburgh to do a concert at a church. We spoke briefly afterward. An occasional e-mail and holiday card kept our friendship loosely tethered.

In the fall of 2008, I received an e-mail from Joy relating the disturbing news that her son Aaron was not doing well and that the doctors had given a short-term negative prognosis for his survival. I phoned Joy, and from that phone call on we connected each night, often talking into the early morning hours. Multiple e-mails were exchanged during the day. I do not know how, with all that was swirling around her, Joy made the time to talk with me. I suddenly found affection growing in my heart that was based on deep heart-to-heart conversation, and I wanted more of it.

In January 2009, I traveled to Pittsburgh to visit Joy and her family. The moment Joy greeted me I was "hooked." When I returned home, I told my son-in-law that if Joy would ever have me, I was going to marry her. I was lost in a love that had grown in my heart and soul through deep communication and respect. Phone calls were now not enough.

In March of 2009, Aaron was taken back to the heart of his heavenly Father who had entrusted him to a wonderful loving family, and back to the heart of his earthly father who left this Earth so suddenly. Joy entrusted me with the honor of performing Aaron's funeral. After the funeral, I wondered, would I have any part in Joy's life?

continued >>

>> Joy and Aaron's Story

Aaron Joseph Jackson was born May 2, 1996. He joined our family of five (the Jackson Five!) which included two older sisters and one older brother. He was diagnosed with MPS II the same month he turned 3. I felt like a death sentence was handed to my son when I researched this condition. My husband, Bob, a dairy farmer in southwestern Pennsylvania, and I committed to support and care for Aaron, knowing the benefits for the entire family, when parents strengthen one another in raising a special needs child.

But then tragedy struck our family on a fall October day in 2004, when my husband lost his life in a farming accident. Overnight I became a widowed mother of four children, one of which had MPS. My oldest daughter, Andrea, was a junior in college and my youngest daughter, 16-year-old Amber, was a junior in high school. Adam was 10 and Aaron, 8, was already showing signs of decreasing abilities.

Often, when the death of a spouse occurs, stability is provided by limiting other drastic changes in a family's life. But the aftershocks of Bob's death would continue to rumble beneath the Jackson family's feet. I was employed as a high school teacher and did not have the knowledge or the ability to operate a dairy farm of 500 acres. The process of selling the farm and finding a new location for my family took two years. This was another major adjustment in a very short time for me, but especially for Adam and Aaron as they moved from the country to the suburbs and began attending new schools. I was doing the best I could, but often felt like I was walking in the shadows. I was not walking in the dark, however, because God's word is a light to my path and a lamp to my feet. For each step He gave enough light and hope, while remaining faithful to us.

Being a single mom with an 8-year-old MPS child was demanding. Aaron only slept three hours a night at the time, so sleep depravity wore me down quickly. I was working full time and keeping the house while striving to keep my children's activities as normal as possible. I was fortunate to have "Wrap Around Services and Behavioral Health," but that was only 20 hours per week, primarily during school hours. Another blessing came through Mental Health Services when our social worker recommended during an annual visit that we qualified for nursing aides and in-home assistance due to Aaron's safety. Thus we began a schedule of night-time professional aides guarding Aaron's safety and saving my sanity! Finally I was able to sleep.

Having these aides was beneficial, but it also brought another major adjustment to our home and family. The workers were present when we ate dinner, had family conversations, watched television at night or carried on "normal" family activities. So basically, we surrendered our privacy for assistance for Aaron. But there was not a cost too great to pay for this gift of life. We were fortunate to have two regular aides who were wonderful. It was a good experience and provided a much needed respite.

Witnessing the declining health of my youngest child was overwhelming at times. My girls provided care for Aaron so I could run errands or have those few-and-far-between free moments with friends. Their support, along with my family, was great, much appreciated and needed. My social life was minimal, with my connections limited to work and family. Attending church as a family became a mourned distant memory.

Shortly after our move in 2006, Aaron lost fully his ability to speak or even make noise when he laughed, which also did not occur

often. The day of never hearing, "I love you, mom" was here and sad to all of us. His mobility remained strong enough to traverse around our home, turn light switches on and off and, every once in a while, even become an uninvited guest at a neighbor's home! Aaron liked to wander, and our new neighbors got quite a surprise the few times he wandered in to their homes or sat in their cars. A new school was a new adventure, but Aaron had a great young teacher who adored him. Still, MPS continued to eat away Aaron's abilities.

continued >>

>> 2008 became the year of major changes in Aaron. Aaron was having difficulty drinking, causing him to choke. Swallowing tests were ordered, and the results required that Aaron's food be pureed and his liquids thickened for easier swallowing. Aaron began to walk with a lean to one side and would, at times, lose his balance. The walking difficulties brought a limit to visiting places. Pain in his joints increased to the point that the only sounds he made were cries of pain. Those cries were heart-breaking, and the steady shrinking of his world brought grievous thoughts of

where we were headed. Changes were made to accommodate Aaron's needs. Since Aaron could no longer trek up and down the steps independently, a hospital bed was ordered and placed in our dining room. The times of services provided by aides increased from 12 hours a day to 18. Aaron needed 24-hour care, but I needed to work, so my wonderfully loving and unselfish friend, Linda, volunteered to take care of Aaron while I worked. Aaron had already become an "adopted" part of Linda and her husband Gary's family three years earlier when Linda and I met at work and, as Linda put it, she fell in love with Aaron. Linda, who just retired, willingly learned and provided the much needed daily care for Aaron. My daughter Amber also lovingly decided to suspend her college education and return home to help with Aaron's care. Andrea was still living at home. These personal resources provided me with the support and assistance I desperately needed.

On Halloween of 2008, I sat in my dining room and signed papers to begin hospice care. Aaron was gifted with two wonderful and caring aides—a nurse named Steve and Flo, an aide who visited Aaron daily from family hospice. Within a week of the onset of Steve's care, I was informed that Aaron had just a few short weeks to live. That news hit me like a brick wall and devastated me as did the MPS diagnosis five years before. The original "death sentence" had now become a much heavier reality staring me directly in my face with its malevolent eyes. I wanted so desperately to stop time, then wind it backward!

Also in the latter months of 2008, I had become reacquainted with an old friend, Rob Grandi. Initial e-mails turned into numerous phone calls long into the night, when we spent hours in conversation. Rob had spent many years traveling as a preacher/musician in the late 70s through the late 90s. He led revivals and sang numerous times at the church my family had attended, and during those times Rob became acquainted with Aaron and my family when we provided lodging for him. In between those visits and after Bob's death, Rob and I maintained distant contact through occasional e-mails and Christmas cards. Rob had lost his wife due to cancer five years before Bob died. We had a lot in common and confided in each other. He was both a comfort for my present pain and a voice from outside my pressing situation. Rob lived six hours away in Louisville, KY, but a relationship was developing through our resurrected contact.

The holidays of 2008 passed, and each week to follow brought ups and downs. I took extended time from work to care for and be with my son. Rob came to visit in January; we continued talking daily and he began visiting monthly. Aaron's condition worsened in March of 2009, when digesting food turned from a monthly aspiration issue to a weekly one. Aspiration pneumonia set in for the final time during the third week of March. Aaron could no longer eat or drink, so medications were provided for rest and pain. It was a difficult week to say the least. My friend Linda remained by Aaron's and my sides, providing breaks for me and care for Aaron. Aides continued their care and Steve, Aaron's favorite aide, requested to be in our home daily, even when he was not scheduled to work. With a pattern of inconsistencies with medication, pain relief was not consistent and Aaron's cries out of pain were excruciating for all of us. At 11:00 p.m., March 19, 2008, Aaron went home to be with the Lord and his father. He was surrounded by his family, Linda and Steve.

The days after losing Aaron were agonizing. After loving and caring for a child for 12 years, not only was I achingly empty within, on the

continued >>

>> outside it was difficult to know what to do with myself, my empty lap and my time. Also disappearing was the constant ins and outs of Aaron's healthcare workers. I never thought I would miss the night-time aides, and now found myself feeling alone and afraid for the first time during the long evenings. I had depleted my allotted sick days from school, but my request for additional time from work was approved, because compassionate and generous staff members donated their sick days for me. I was surprised that almost every faculty member gave me one of their sick days. Tears rolled down my face when I was told, for it helped to know that others cared so deeply. It took months for me to begin my life again. Grief counseling had been provided by family hospice prior to Aaron's death and followed for a year after.

As I write, the third anniversary of Aaron's death has passed a week and a half ago, which made this writing bittersweet and difficult. Looking back, I can see so many circumstances in my life that prepared me to be Aaron's mom—from a community member who convinced my mom to permit me at the age of 16 to work with special needs children and adults, to my God-planted desire to pursue a career in special education. After Aaron's diagnosis, the paths of many warm, tender-hearted people who were sensitive to our needs crossed our family's path, making our road easier to walk. This gift of loving helpers was from God, I believe, and not by accident. God had a purpose through Aaron's life to affect the lives of others in positive respects, and it happened just as He planned!

Many would say that 12-year-old Aaron missed out on a full and wonderful life. That may be true if you look only through the lens of time, but I believe Aaron accomplished more in his short life than many people do after living for decades. Aaron had a gift for affecting people in a positive way, even when his negative behaviors complicated situations. He lost his voice to communicate at a young age, but his smile, his little hands and eyes said more than the best orator. He brought smiles to the faces of the most negative individuals. People our family did not know remembered Aaron from various encounters, and it wasn't unusual to be out with Aaron and have strangers approach, smile and talk to him, even when he could no longer participate in the conversation! Aaron brought a positive touch to the lives of many, including his family. That was part of God's purpose, and I was privileged to be chosen by God to be his mom.

Through the privilege of having Aaron in my life, I also learned several things, including acceptance, patience and a stronger faith in God. Even though Aaron did not comprehend his condition, he was accepting of his life, unknowing of his fate. He enjoyed what life and others gave him, and the smallest things made him happy. I also watched my other children accept Aaron and his condition within our family. Never once were they negative or shameful of their brother, but always protective, caring and supportive. I had to build up a "boatload" of patience to care for and share life with Aaron. He didn't always make situations easy or work on my schedule. My faith grew stronger through the life of my son. I saw the love of God through so many situations and people who showed comfort and love to our family. Without my faith and God's care and love, my children and I would have never made it through the last few years.

Rob and I fondly view Aaron's final gift to us as the gift of bringing us together.

Rob and Joy's Story

On May 19, 2010, the anniversary of Aaron's "homegoing," on the Indiana side of the Ohio River with a view of the Louisville skyline, I asked Joy to marry me. I told her I wanted to redeem May 19 for her; to hopefully allow the remembered joy of our engagement to cushion the sorrow of such a notorious anniversary while providing a reason to celebrate through years to come. Nothing will totally remove the sorrow of Aaron's death, but the joy and celebration of love can bring powerful comfort. On May 14, 2010, with our families present, Joy and I were united in marriage.

continued >>

>> God's Unexpected Results

Faith is believing in advance what will only make sense in reverse. As Joy and I look back at our lives and the paths we took separately, the words of a Rascall Flats country song speak a truth of God about us and for us: "God blessed the broken road that led me straight to you." We both walked paths we never would have chosen for ourselves. We both still have unanswered questions that may never be answered this side of the grave as to God's purpose and His timing. But through the rear-view mirrors of our lives, we can see the hand of God leading us to our present reality. God knew the tragedy that would happen in Joy's life. He knew the heartbreak and pain I would experience at the death of my first wife. Why did a loving God allow such sorrowful events? Only God knows. In the Bible, God never promises a safe and painless life. But God does promise to be faithful through all circumstances, and he was true to His word, within us and beside us as a strong shepherd through the valley of the shadow of death.

Years before either of us lost our spouses, our paths had crossed and a friendship was in place. Coincidence? Not to us who know a God of great love and timeless preparation. God used Aaron's life and death to unite us. Were God's ways mysterious? No, not really, but how absolutely wonderful His unexpected results.

Jackson-Grandi family

upcoming events

National MPS Society 2013 Family Conference

The 27th Annual National MPS Society Family Conference will be held Oct. 24–26, 2013, in San Antonio, TX. The Omni San Antonio Hotel at the Colonnade is alive with the spirit of Texas. This four-diamond, luxury hotel captures the essence of the city with impeccable service and charm. Overlooking the rolling Texas Hill Country, this 19-story high-rise is close to the area's most exciting sites and attractions such as the historic Alamo, Sea World San Antonio and the enchanting Riverwalk. Saturday has been reserved for families to enjoy Morgan's Wonderland, the world's first ultra-accessible family fun park designed with special-needs individuals in mind and built to be enjoyed by everyone. Transportation to the famous San Antonio Riverwalk will be provided as an alternative to Morgan's Wonderland. Save the date to attend this conference which promises to offer all the updates on research, medical care and topics that you've requested.

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!

making headlines

Twins with rare disorder recovering after bone marrow transplants

Baen and Bryce Hurst are slowly recovering after undergoing life-saving bone marrow transplants at the University of Minnesota Amplatz Children's Hospital in Minneapolis, but they still have a long road of recovery ahead of them.

The identical twin sons of Brian and Casey Hurst, of LaGrange, were diagnosed with Hurler syndrome in November. The twins are the only documented identical twins on record who have this rare disease.

A leader in successful bone marrow transplants for Hurler syndrome patients, the University of Minnesota Amplatz Children's Hospital is involved with a research trial that is the first of its kind to inject enzyme replacement into the spinal cord in an effort to stop the neurologic deterioration that comes with having Hurler syndrome.

Baen and Bryce were accepted into the clinical trial, where they received an unusual bone marrow transplant and enzyme replacement therapy.

Excerpt from an article featured by The Chronicle-Telegram, *Elyria, OH, May 29, 2012. Written by Melissa Linebrink. To read the complete article, go to http://chronicle.northcoastnow.com/2012/05/29/twins-with-rare-disorder-recovering-after-bone-marrow-transplants/.*

Baen Hurst (MPS I)

Raising MPS awareness in the Latino community

WFDC TV recently featured Dominic Henriquez (MPS II) in a story regarding new legislation in Congress that would help raise funds for treatment. To view the story, go to **www.tvwfdc.com/noticia/2012/07/03/389759-ley-congreso-podria-ayudar-enfermos-graves.html**.

Sixteen-year-old James Oliver (MPS II) was featured in a news story by WFLA, Tampa Bay, FL. James was recently photographed by world-renowned fashion photographer Rick Guidotti who has traveled all over the world to photograph children with Hunter's syndrome. To see the story, go to **http://video.tbo.com/v/61269473/raising-awareness-for-hunter-syndrome.htm?q=hunter+syndrome**.

NATIONAL MPS SOCIETY 26TH ANNUAL

family conference

Conference presentations are available on the Society's website

We loved having all the families together. Camp Courage is the most amazing thing!

This year's family conference was held July 26–28 in Boston with more than 400 attendees, all of whom had fun being together, and sharing and hearing new information. The conference began Thursday evening with registration and dinner. Families whose children had been diagnosed in the last year attended a session with Drs. Muenzer and Frye where they could ask questions about their diseases and discuss coping issues. The syndrome breakout sessions held Thursday evening are always a favorite part of the conference and a great way for families to personally meet their Facebook friends and learn from one another.

A presentation given by Lillian Wong, JD, an attorney who advocates for special education rights, representing parents and children, focused on educational issues. Ms. Wong discussed effectively advocating within the special education system and provided practical advice about communicating with the school. Lisa Muller, a special education teacher and mother of Riley (MPS II), talked about how being "on the other side of the table" is both challenging and terrifying. Melissa Hogan, lawyer and mother of Case (MPS II), led an excellent workshop on Individual Education Plans where she provided a tremendous amount of helpful information.

Dymon Hardy (MPS II) and Chelsey Klenke

Thanks to the work of Beth Karas, many families, researchers and clinicians, a series of new MPS educational and fundraising videos is now available. The Fundraising Committee showed the fundraising video and everyone raved about how amazing it is. Jamie Witker from Outland Films in LA produced a magical video that gently educates while telling the families' stories. We will let you know when the final products are completed and available to you—at no cost. Lisa Todd and Brooke Carter spoke passionately about fundraisers they hosted, very different fundraisers, and the mechanics behind each. We will let you know when these presentations are available online.

Dr. Roberto Pineda, an ophthalmic surgeon, spoke about eye-related problems in individuals with MPS diseases. Two of his patients, Nick Boyce and Sam Caswell, both of whom have MPS I, shared their positive experiences with eye surgery. Stephanie Bozarth talked about the orthopaedic surgeries her daughter Annabelle (MPS IV) has undergone with Dr. William Mackenzie. Dr. Mackenzie, an internationally renowned orthopaedic surgeon, had lunch with parents Friday during the Expert Lunch session, along with several other speakers.

continued >>

>> M+R Strategic Services has been contracted to help with the Society's expanding legislative work. Lori Fresina and Diane Pickles from M+R hosted a concurrent session to show parents how to meet with federal lawmakers in their home state and share their story. This was a personal presentation of the webinar the Legislative Committee presented this spring. During the Legislative Committee presentation, Roy Zeighami showed the steps of contacting your legislators when "action alerts" are sent to members, and how incredibly easy it is.

On the research side, Dr. Calogera Simonaro presented her work with anti-TNF-alpha therapy to reduce inflammation leading to improved bone growth and joint tissue in MPS VI rats. Dr. Steven Walkley, chair of the Society's Scientific Advisory Committee and leading researcher on lysosomal diseases affecting the brain, explained the many events ensuing when the lysosomal system is not functioning normally and where therapeutic intervention may occur.

Coach Jerry Bennett, Camp Courage director, explained the use of Behavioral Sensory Integration, using personal examples presented by parents attending his concurrent session. Dr. Kim Frye presented the compelling data why we must take care of ourselves in order to care for our children, and gave examples how to do so. Nicole Shannon provided tools to help solve those problems in marriage that seem to get stuck in gridlock, and helpful information about ancillary therapies was provided by Amy Holland (art therapy), Lisa Muller (adaptive swimming) and Julie Reneer (essential oils).

Before the social hour and awards banquet the conference ended with concurrent sessions hosted by Monica Miller from BioMarin, Dr. Gerald Cox from Genzyme and Dr. Ann Barbier from Shire. They presented updates on research, clinical trials and natural history studies, and answered questions from attendees. As the banquet speaker, Matt McNeil talked about writing *The Strange Tale of Ben Beesley*, proceeds of which are going to the Society (**www.benbeesleybook.com**).

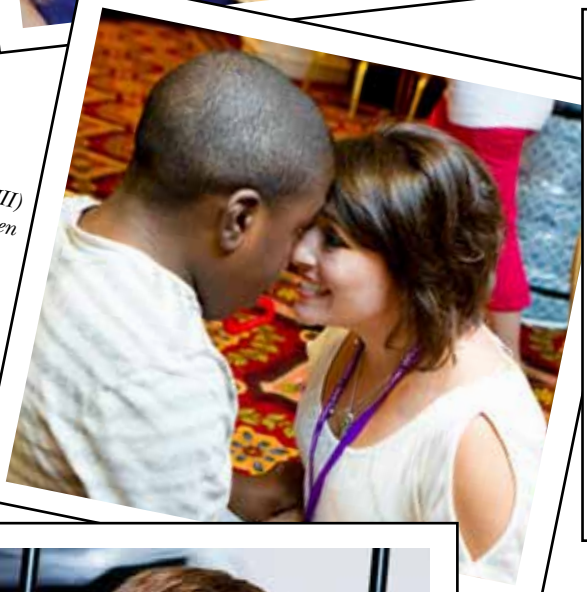
Next year's conference will be held Oct. 24–26, 2013, in San Antonio, TX, with plans for a day at Morgan's Wonderland. Registration materials will be available in early 2013.

As a first-time adult attendee, I was very impressed with the range of topics covered by the sessions and the overall organization of the Society.

Loved the syndrome breakout sessions; I found those to be the best source of information.

I love the opportunity to speak with other families and hear their advice/situations.

Elliot Mattheson (MPS I)



Kevin Carter (MPS III) and Monica Green

Annabelle Bozarth (MPS IV)

Caleb Waddell (MPS I)

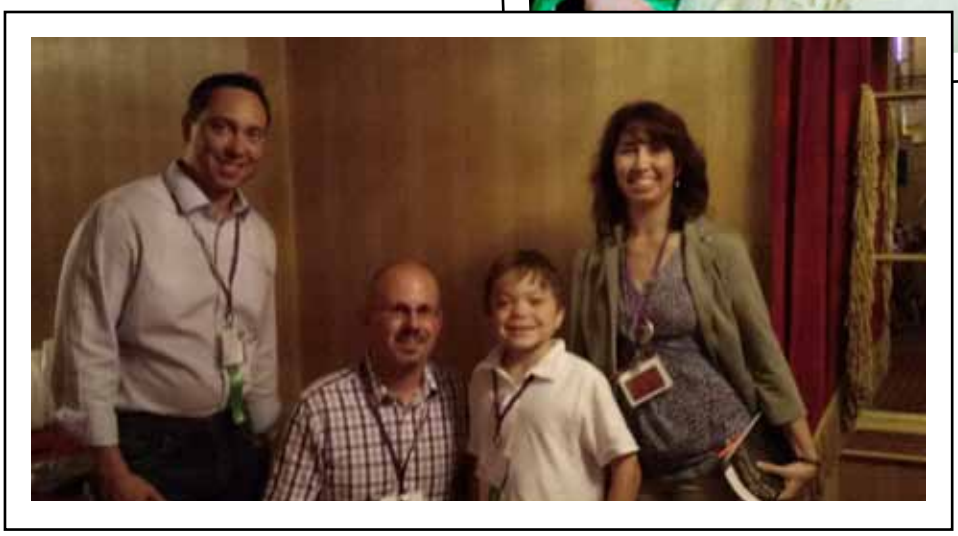
Zahar Beg (MPS III)

Haley Miller and Austin Noll (MPS III)

Jack Frye (MPS II) and Alan Charest (MPS III)

Isabella Clendenny (MPS I) and Case Hogan (MPS II)

Dominic (MPS II) and Freddy Henriquez



Jerry Todd, Matt McNeil, Jack Todd (MPS II) and Angela Guajardo

2012 National MPS Society Member Awards

Presented at the Boston Conference Awards Banquet

Millie and Mel Anhalt

Friendraising Award: Millie and Mel Anhalt

The Friendraising Award recognizes individuals whose contributions to the Society include not just fundraising, but raising awareness about MPS and related diseases.

Millie and Mel Anhalt are the grandparents of Conner Anhalt. When Conner was diagnosed with MPS II in 2000 at the age of 2, they became members of the Society. Their journey with MPS as grandparents began as most of ours, with learning as much as possible about the disease and treatments. Along the way they realized how important it is to increase awareness about MPS and to provide support to the National MPS Society. Over the last 12 years the Anhalts have averaged six donations a year to the Society, and each donation comes with a list—sometimes a long list—of friends they want to acknowledge for birthdays, anniversaries, get well wishes, etc. Many of these friends, in turn, have become members of the Society and have also donated. In the last two years, we received 16 donations from those friends!

What began as a small gesture of giving to a charity in lieu of gifts has created an extraordinary momentum of giving and awareness. Millie and Mel exemplify the intention of the Society's Friendraising Award.

Jerry Bennett

Directors' Award: Jerry Bennett

The Directors' Award is given on behalf of the board of directors to a Society member who volunteers their time and expertise to the Society.

This year's Directors' Award recipient, Coach Jerry Bennett, became involved with the Society two years ago when one of his clients asked for his help with our childcare program. Prior to that time local childcare agencies were contracted to assist, and the services they provided were not consistent. The reality is no childcare agency has an understanding of MPS and related diseases, despite attempts at education. Jerry assured us that he could handle all the children with just a couple members of his staff, which seemed unbelievable. He was right, and all the children immediately flocked to him when he walked in the room. If there was any question of his abilities, he won us over when the hotel lost power and the childcare rooms were totally black. He gathered the children together and they set up a pretend campfire using cell phone lights, telling stories and singing songs. Parents were quickly reassured that their children were fine—better than fine—they were having a fantastic time.

Coach Jerry is a part of our MPS family, having assumed responsibility for Camp Courage and even planning a huge fall fundraiser. He and his wife, Tanya, developed the Behavioral Sensory Integration technique that is the basis of the programs offered at BMod Fitness in LA which he uses with our children in Camp Courage—all on a volunteer basis.

continued >>

>> Outstanding Member Award: Klane and Amy White

The Outstanding Member Award is the highest achievement awarded to a member of the Society for the devotion of their time, expertise and valuable contributions to the Society.

Klane and Amy White with Susannah, MPS I (1999-2008), and Madeline

Klane and Amy White were elected to the Society's board of directors several years after their daughter, Susannah, was diagnosed with MPS I. When they retired from the board six years later, Amy continued her work on the Family Support, Education, and Publicity and Governance committees. Klane utilized his professional expertise as a pediatric orthopaedic surgeon, chairing the Grant Review Committee. But their work in the greater MPS community is far more reaching. As a faculty member at Seattle Children's Hospital, Klane has advocated for children with MPS to be included at Camp Korey, a camp outside Seattle that focuses on children with special needs, and he lectures throughout the world about orthopaedic problems associated with MPS. He is readily available to families outside the Seattle area who have questions about their children's orthopaedic problems, and refers them to qualified physicians in their area. Amy spent a week with Klane this summer at Camp Korey where she was a behind-the-scenes helper, including driving children to the airport and ensuring they were on their way home. The hospital staff know to call her if a family has special needs, and she'll reach out to the Society for support of those families. The mission of Klane and Amy has been to support families touched by MPS, as well as the Society's programs, and we are very grateful of their dedication of time and expertise.

2012 SPIRIT Conference

The adult conference, *Finding our **SPIRIT** – **S**trength, **P**urpose, **I**ndependence, **R**esilience and **I**nitiative **T**ogether*, also was held in Boston. A social gathering, including a Duck Tour of Boston and dinner at Fire and Ice, provided an opportunity to make new friends, catch up with old friends and prepare for the conference.

The conference began with a discussion about key figures in attendees' lives—their parents. One of the talking points was the role MPS adults want parents to play in their lives. Whether it is choosing a career path or deciding when to leave the nest, parents have to understand and respect decisions.

Living independently seemed to be a goal most people in the room wanted to accomplish, and a goal some had already achieved, despite the financial obstacle which seemed to be a concern for many. How to improve daily activities was discussed, including tools that can be used to overcome small obstacles that are sometimes the hardest to understand. For instance, how to tie shoe laces if you can't reach or tie a tie if you have carpel tunnel. Trying to focus on accomplishing small tasks can help you see the bigger picture and prepare you for increased independence.

After lunch, attendees participated in ice breakers to stimulate the group. During the course of this full-day conference, this was the most successful activity for group participation. These activities provided an opportunity for interaction in an informal setting and achieved the goal of increased talking and sharing of ideas.

An Inspirational Fundraising Session

How Did They Do It? was the topic of the fundraising session at the 2012 National MPS Society Family Conference. MaryEllen Pendleton, chair of the Fundraising Committee, opened the session by highlighting the many fundraising events held in 2011. There were a total of 70 fundraising events that raised in excess of $550,000. She spoke of the importance of every fundraising event, large or small. Every dollar raised puts us closer to a cure. Small fundraisers bundled together can support a research grant, a scholarship opportunity or a family assistance item. The Society also is using social media such as Facebook and First Giving for additional fundraising opportunities.

Lisa and Jack (MPS II) Todd

Terri Klein, National MPS Society development director, discussed the need families have to make a difference once they have dealt with a diagnosis. Society families are eager to raise funds with a variety of strategies. Terri spoke of three individual fundraising opportunities: The **Planned Giving** program which enables families to plan long-term gifts to the National MPS Society; **Donor Pages**, a new web-based program; and the **Sponsor A Child For A Cure** program which enables families unable to attend a walk/run event to raise money in honor of a child, adult or someone who has passed away and was affected by MPS or a related disease.

The highlight of the fundraising session was sharing the newly released National MPS Society video that will be available for families and fundraising events this fall, along with presentations from two speakers who shared their inspirational stories about fundraising for someone they loved.

The Society also has begun filming a new video compilation that will help MPS families tell their story. This four-part compilation will benefit healthcare workers and researchers, promote awareness, educate school professionals with the understanding of your child's diagnosis, and help raise awareness and funds nationwide to help find treatments and cures for MPS and related diseases. The videographer, Jaime Whitaker from Los Angeles, has been working seven months filming and editing families, physicians, researchers and those affected to compose a compelling product for the Society to share. There was hardly a dry eye during the session while this heartfelt video brought awareness to the viewers.

The Legislative Committee and Fundraising Committee presentations were great and gave me lots of tips. I love the new video!

Brooke Carter hosted a run/walk event in honor of her nephew, Aidan, who recently was diagnosed with MPS II. Aidan is their family's first grandchild and her first nephew, and they were devastated by the news. Brooke and her sister-in-law, Jennifer, mother of Aidan, hosted a fundraising event in Exeter, NH. Her event was a lot of hard work but she received tremendous support from her family and the community and raised more than $100,000. Her advice was to tell yourself to remember why you were doing this, stay motivated and ask for help. Remember to thank everyone along the way!

Lisa Todd hosted a fundraising event, Keep Hope Alive, in honor of her son Jack (MPS II). Her event was an informative "Ask Event" on a Sunday afternoon where she gathered attendees at a family home in Albuquerque, NM, and hosted talk sessions and a video presentation about MPS and related diseases. The event raised $50,000! Some of these donations will be recurring donations through a new program she began, Circle of Hope. Her key to success was to have a great support group, stay organized and be passionate about your goals.

Perspective from a Genetic Counseling Student

by Erin Eaton

As I reflect upon my time in Boston, I am listening to the song "Awake My Soul" by Mumford and Sons. For those of you who are unfamiliar, they are a folksy group of talented musicians from Great Britain. The lyric that speaks to me the most from this song and embodies the spirit of the family and SPIRIT conferences is this one: *"Where you invest your love, you invest your life."* You, families and members of the MPS Society, appreciate and embody this sentiment more than most.

As a genetic counseling student, I have studied genetics, biology, chemistry and psychology. I've been taught how to interpret lab results, how to break bad news to patients, and how to be sensitive to individuals from other cultures. I've been taught how different diseases are inherited and the best ways to explain them to clients. I've also been taught how families cope with new diagnoses and how to help them navigate the medical system. However, as is true of most things in life, book and classroom knowledge can only provide me with a good base of information. I will be forever grateful for the chance to interact with your members and families at the conferences in Boston, for this experience provided me with knowledge that cannot be attained in the classroom.

I came to the conference with book knowledge about MPS and ML and I knew that both fell into the category of lysosomal storage diseases, both were genetic and both were rare. I knew there were different subtypes of the diseases and different ranges of severity. I also knew there were limited treatments available and that most of these were ancillary. I also knew that ultimately, those with the disease are betrayed by their bodies and often lose their fight much too young. However, to know about something is vastly different from seeing it in person.

I was able to see first-hand how this debilitating disease impacts individuals and their families because you so openly shared your stories with me and included me in your day-to-day lives at the conference. For example, I watched as you ate with your children. Some of you encouraged them to eat only from their own plates (I really don't mind sharing my Cheerios), while others fed your children by hand or through a feeding tube, or simply helped them cut up their food. Through presentations and observation I was able to learn more about the physical manifestations of the disease—orthopedic, ophthalmological, neurological, etc. I watched as some of your children ran, playing with each other. I also watched as some were pushed by their siblings, or took walks around the room and down the hall. Over the course of the week, you taught me the differences between Hunter, Sanfilippo, Morquio, Hurler and other syndromes. Through all of this, I was able to see the heartbreaking and sobering reality of the disease—there is no cure. But most importantly, I saw how much you understood the value of time. You acutely appreciate life and living and I fiercely admire how much you appreciate every moment you're given.

What most impacted me was the amount of time, love, and dedication your families pour into this Society and one another. You invest your time to raise money. I watched in awe as the purple thermometer hit its target and you raised $5,000 in less than 30 minutes at the banquet dinner! I listened

to stories of various fundraising events and how you traveled (sometimes cross-country) to support each other. I was so impressed by the multitude of various fundraising events you've created to generate funding for research—and by the dedication of the siblings who raised money through everything from lemonade stands to benefit concerts.

And oh, how you *"invest your love"* into one another! The conference was like a huge family reunion. I watched as old friends hugged and greeted one another, and as newly diagnosed families bonded as they talked about their struggles with diagnosis. I could visibly see parents relax as the conference progressed because they were finally around people who understood, who "got it." You took notes and asked insightful questions throughout the breakout sessions, and listened with hope to the updates about the ongoing and upcoming clinical trials. I was so impacted by members who have lost a loved one, yet continue to be advocates for the Society. The remembrance ceremony was one of the most touching events I've ever had the privilege to attend. As we listened to music composed by a father of a child with MPS, we each lit a candle with tears streaming down our faces. At the adult SPIRIT conference, I watched and listened to shared struggles, hopes and dreams. All of these examples are just a few of the ways you invest your love and your lives.

The love you show toward your children, your families and each other is amazing. I am eternally grateful for the opportunity provided to me by the National MPS Society to be privy to such an amazing constellation of families, friends and members of the Society. Thank you for letting me share in your tears and your laughter.

Wayne Eppehimer (MPS II, left) with his nephew Jason Madison (MPS II)

REMEMBERING

our children

Lila Beth Hughes
14 months, MPS I, 6/19/12

Ashtyn Spangler
17, MPS III, 7/16/12

The following was written by Sarah Gniazdowski (sister of Danny, MPS II, 9/10/97–8/16/05) and read at the remembrance ceremony at the MPS Society family conference.

My favorite author, Nicholas Sparks, once said, "In life, there are seldom clear-cut beginnings, those moments when we can, in looking back, say that everything started. Yet there are moments when fate intersects our daily lives, setting in motion a sequence of events whose outcome we could never have foreseen."

Fate intersected my life on Sept. 10, 1997. It delivered to me a baby brother who was hand-crafted in the hands of an angel. Every sibling here has their own story, their own joy and their own pain of having a sibling with MPS. We share a bond that would have never been formed had it not been for MPS.

We stand here today as a family whose kindness softens the hearts of the crude, whose patience represses the tempers of the restless, whose hope inspires the discouraged, and whose lives exemplify the importance of love. Fate led me to a family that makes every member feel loved and accepted. People spend their whole lives searching for bonds that resemble the ones we share today. We are truly lucky to be members of a family whose strength, dedication and compassion are so admirable. Though all of us have faced the struggles of MPS, we can be comforted by the fact that we are not alone. We are a family, and as a family we will fight to the end. One day when our journeys come to a close, our legacies will be carried out through the lives of our family standing amongst us. And each one of us will be looked upon with the utmost admiration and honor as people say, "There goes a fighter."

A Warm Welcome introduces new Society members/families and offers members yet another chance to connect with one another. If you have a moment, please contact the new family or member 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.

a warm welcome

The National MPS Society welcomes the Beam family from Kingwood, WV.

Tammy, Nathan and Hunter (MPS II) Beam

Our names are Nathan and Tammy Beam. We met while working at a Federal Prison in New Jersey. In 2007, we decided to leave big city living and transferred to a federal prison in the middle of nowhere, West Virginia. We have an adorable, very active little boy named Hunter, who will be turning 3 on Nov. 25. Hunter has three dog sisters and one dog brother that he loves to boss around.

Hunter was born a couple of weeks early and spent the first 10 days in the NICU with fluid in his lungs. When he came home we thought everything was going to be okay. For the last two and a half years Hunter was constantly sick with a cold, runny nose and lots of ear infections. Finally, after being referred to an ENT doctor, the answers to why Hunter was always sick would come to light. It took a month to talk the doctor into placing tubes in his ears. When Hunter was in the recovery room the doctor said, "I think your son might have cystic fibrosis." I looked at him like he was crazy. Here was my son having a fit, screaming and crying, and I did not even know what cystic fibrosis was. The nurse printed out some information which I read, but did not believe anything was wrong with my son. Before leaving the hospital I called my pediatrician and informed her of what the ENT said. She agreed to send Hunter for a sweat test just to put my mind at ease. Two weeks later the results came back negative. I thought this would be the end but it was only the beginning.

After testing negative for any allergies we were sent to a geneticist. After tons of questions from the doctor, he informed us that Hunter was going to need to give a urine sample, have X-rays taken, blood work and an ultrasound. He informed us that he thought Hunter might have MPS. Two weeks later we received a call telling us he needed to see us in his office right away. It was hard to listen to the doctor with Hunter running all around; I was numb when he told us the test results. I had tons of questions and a range of emotions going through my body. The doctor told us he was going to have to do more testing before he would be able to tell us which type of MPS Hunter had.

continued >>

>> On June 14 Hunter was diagnosed with MPS II. We left the doctor's office unsure of what to do next. I turned to the computer and found the National MPS Society. Here was a place I found answers to my questions. We learned about the Annual MPS Society Family Conference, which would be happening the next month. We knew we had to go; we even received a scholarship for newly diagnosed families. We were so nervous, but upon arriving we met the most amazing, wonderful and caring families. We felt normal again. Here were families that knew exactly what we were going through and how we were feeling. Hunter loved the play room so much, after dropping him off he would push me to the door and tell me to go. We are already looking forward to attending the conference next year.

Hunter loves anything Diego. We had to start taping Diego since Hunter believes every time he wants to watch TV, Diego will be on. He loves playing with his cousins, dancing and playing on his Kindle. He is always on the go and recently learned how to open doors—now he helps himself to a snack. Every weekend we try to go somewhere fun, even if we have to drive an hour or two. He loves going to the store and saying hi to everyone.

The last few months have been crazy to say the least. We have lost some old dreams but also have made new ones. We are now living life in the moment, treasuring what we do have, and not thinking about what we don't have or how things are going to change. With the support and love of our family and friends, we are going to give Hunter the best life possible. We will be praying for a cure and will be doing whatever we can to raise awareness and money so in the future another family will not follow our journey.

www.caringbridge.org/visit/hunterbeam

Beam family

NEW MEMBERS

Craig and Dawn Abbott
Grand Rapids, MI, aunt and uncle of Jarod and Caleb Mulder, MPS III A

Alia Ahmed
Minneapolis, MN, professional

Tammylee and Nathan Beam
Kingwood, WV, parents of Hunter Dylan Beam, MPS II

Joshua Burgos
Vernon Rockville, CT, adult with MPS II

Elizabeth Clendenny
Mineral, VA, mother of Isabella Clendenny, MPS I

Kevin and Michelle Collins
Prospect, KY, parents of Alexandra Collins, MPS I

Ashley Cotton
Jacksonville, FL, mother of Ashden Cotton, MPS II

Kathleen Delaney
Minneapolis, MN, professional

Tamara Haas
Hainesport, NJ, BioMarin

Michelle Hughes
Marion, IN, mother of Kara Stoeb, MPS III

Sheri Kizer
Placitas, NM, aunt of Jack Todd, MPS II

Lacye Lemke
Mansfield, TX, mother of Jeff Bowman, MPS IV

Dana Martin
Cambridge, MA, Synageva

Amelia McHugh
Central Islip, NY, relative of Michael Nappi, Frank McHugh and Frank Tirelli, MPS II

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

Rachel Starnes
Lincoln, MO, friend

Phuong Vuong
Rosemead, CA, adult with MPS VI

Chad and Kathleen Wallace
New Albany, IN, parents of Karsyn Wallace, MPS III A

Doris Waymire
Ballwin, MO, grandmother of Eliza and Emma Weaver, MPS IV B

Antoinette Weisse
Pequannock, NJ, friend of Jason and Justin Leider, MPS II

David and Denise Welch
Magee, MS, grandparents of Koby Walker, ML II

Tasha and Craig White
McKinleyville, CA, parents of Makynna White, MPS I

Frank and Kimberly Willman
Yorkville, IL, aunt and uncle of Kasey McCannon, MPS III A

legislative update

Policy with Partners: Time to Take Action!

The Legislative Committee reviews and tracks legislation important to our community, which we then forward to the Society's Policy with Partners (PwP) group members. Previously you would need to sign up for PwP e-mails, however, due to the impressive number of members participating in action alerts and the improved ease of advocating, the Legislative Committee will be including all Society members in alerts and updates in the future. We will continue to track legislation and only ask for member advocacy participation when it would greatly benefit or significantly affect the MPS community.

THIS SUMMER THE LEGISLATIVE COMMITTEE has continued to gain speed in our advocacy efforts with great promise for the future. We were successful with advocating for the MPS Awareness Day Senate Resolution, changes in legislation that will allow for more innovation and opportunities in drug development for the rare disease community, inclusion of MPS language in the Senate version of the Appropriations Bill, providing a toolkit and webinar for our members to engage in advocacy within their own district, and providing such ease in advocating with the "click and send" action alerts that anyone can do it in just a few spare minutes. The "click and send" action items have been extraordinarily valuable to our Society due to the fact that our families our juggling so much every day. Simply, we needed to make it easier so more people could participate. Additionally, I have been so grateful for engaging M+R Consulting because it has given our committee the tools to be successful, passing that along to the entire MPS community. As a mom of three young children and one having MPS IV, I know the value of keeping it succinct and with a clear purpose. If any members would like to join the Legislative Committee, please do not hesitate to contact me to see if it would be a good fit for you.

Sincerely,

Stephanie Bozarth
Chair, Committee of Federal Legislation
Stephanie.Bozarth@mpssociety.org

After The Ruling: A Consumer's Guide

by Mary Agnes Carey, Kaiser Health News

On June 28, 2012, the Supreme Court upheld the 2010 federal healthcare law, dismissing the challenge by states to the law's requirement that individuals get insurance. The justices, however, did give states the right to opt out of a critical provision requiring them to expand Medicaid programs for the poor and disabled. The decision will have sweeping ramifications for consumers, state officials, employers and healthcare providers, including hospitals and doctors.

While some of the key features don't kick in until 2014, the still-controversial law has already altered the healthcare industry and established a number of consumer benefits.

A Q&A about some of the law's provisions that are already up and running, as well as major features of what's to come, can be found at **www.kaiser-healthnews.org/Stories/2012/March/22/consumer-guide-health-law.aspx**.

Current Legisltaive Priorities and Action Items

– Ask your Congressman to Join the Rare Disease Caucus

With the caucus now introduced in the 112th Congress, we need your help to drive membership. The caucus will help to further educate our legislators about the special needs of our MPS community and other rare disease communities with similar issues. This is where we start our search for advocacy champions who greatly influence legislation important to us. You will be able to determine if your congressman is in the caucus at **www.rarediseaseadvocates.org**.

– Develop a relationship with your representative and let your voice be heard!

We asked our consultants at M+R Strategic Services to conduct an online training session for our members. The goal was to help our members learn how to meet with their members of Congress to advocate for research funding and other important policies related to MPS diseases. There's no better way for us to fight for funding and policies to advance new treatments, and ultimately a cure for MPS than meeting with lawmakers and telling our stories.

A previously recorded training session is posted on the Society's website. It takes less than an hour to view. In addition there are lots of great handouts on the website that provide a step-by-step guide to meeting with your lawmakers in your home state. Go to our website (Members Only - Legislative Toolkit). Under "How to Webinar," click on "Guide to in-district Legislative Visits."

We aren't a large organization, but we are powerful—because of YOU! You are all fantastic advocates. Our strength as an organization isn't defined by our size but by the involvement of our members.

Obama Signs FDA User Fee Legislation Bringing Hope to Rare Disease Patients

On July 9, 2012, President Obama signed into law *The Food and Drug Administration Safety and Innovation Act (FDASIA), S. 3187*, bipartisan legislation that will spur the development of lifesaving treatments for 30 million Americans suffering from rare diseases.

"We are thrilled the language to improve access to the FDA's Accelerated Approval pathway for rare diseases has been included in *FDASIA*," said Emil Kakkis, MD, president, EveryLife Foundation for Rare Diseases. "We thank Representatives Cliff Stearns (R-FL) and Ed Towns (D-NY) for being champions for the rare disease community."

Stearns and Towns first introduced the *Unlocking Lifesaving Treatments for Rare Diseases Act* to empower the FDA to use all the science available for allowing surrogate endpoints in clinical trials for rare diseases to determine whether a drug is working, significantly decreasing the development time and cost. Stearns and Towns later introduced the *Faster Access to Specialized Treatments Act* that improved Accelerated Approval for life-threatening diseases while maintaining high safety and efficacy standards.

Legislative Committee:

Stephanie Bozarth, *chair*
Amy Barkley
Jeff Bardsley
Austin Bozarth
Dawn Checrallah
Ernie Dummann
Steve Holland
Terri Klein
MaryEllen Pendleton
Kelly Rose
Laurie Turner
Kim Whitecotton
Roy Zeighami
Barbara Wedehase

FDASIA is the culmination of more than a year of negotiations between industry and FDA and includes the reauthorization of the drug and device user fees.

The FDA's Accelerated Approval has been successful in getting treatments approved for cancer and AIDS patients, but has been essentially unavailable for rare disease treatments. There currently are fewer than 400 FDA-approved treatments for nearly 7,000 rare diseases. Investment and interest in development will surge for rare diseases if there is access to the Accelerated Approval pathway.

"We would not have been successful if it were not for the great work of Energy and Commerce Chairman Fred Upton (R-MI), Biotechnology Industry Organization, and more than 300 patient organizations that advocated for improving the FDA's regulatory process," said Kakkis.

Congress Recognizes National MPS Awareness Day

"I would like to recognize the National MPS Society for their 37 years of supporting families while searching for cures for this genetic disease," said Rep. Kenny Marchant (R-TX) while addressing Congress on May 16, 2012.

"Mucopolysaccharidosis or MPS is a group of genetically determined lysosomal storage diseases that render the human body incapable of producing certain enzymes needed to break down complex carbohydrates. The damage caused by MPS on a cellular level adversely affects the body and damages the heart, respiratory system, bones, internal organs and central nervous system. MPS often results in intellectual disabilities, short stature, corneal damage, joint stiffness, loss of mobility, speech and hearing impairment, heart disease, hyperactivity, chronic respiratory problems and, most importantly, a drastically shortened life span. Symptoms of MPS are usually not apparent at birth and without treatment the life expectancy of an individual affected begins to decrease at a very early stage in their life. Research toward combating MPS has resulted in the development of limited treatments for some of the MPS diseases."

"I ask my colleagues and their staff to join me in recognizing May 15, 2012, as National MPS Awareness Day. This is an important time during which the MPS disease community will help increase the awareness of this devastating disease, as well as supporting research to improve treatments, find cures and receive early diagnosis. MPS families are encouraged to reflect and support each other and to reach out to those families who have lost loved ones to MPS. By wearing their purple ribbons and sharing these ribbons within their community, they are increasing public awareness about this disease. This date is also the start of the National MPS walk/run season along with other local community activities to raise awareness, along with money for research and family assistance programs. I commend the National MPS Society and their many volunteers for an unwavering commitment to bring about awareness of this disease, and to continue to advocate for federal legislation to streamline the regulatory processes and to speed effective treatments and cures for their loved ones. More must be done to find cures and effective treatments, but let us reflect on the importance of this day. I ask that all of my colleagues join me in commemorating National MPS Awareness Day."

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 offer!

In this issue, we interview **Flossie Pryor**. If you know of someone you would like to have featured in a future issue contact us at info@mpssociety.org.

volunteer spotlight

Andrew Cochenour (MPS II) is my 14-year-old grandson. He lives in Clarksburg, OH, and just finished 8th grade at The Pioneer Center School. Andrew plays on the school baseball team, is in the Special Olympics and enjoys horseback riding.

Andrew Cochenour (MPS II) with his grandmother Flossie Pryor

Andrew was diagnosed in 2001. Soon after his diagnosis, I became a member of the National MPS Society and attended my first conference. I have attended all except two conferences since. In 2002 my daughter, Sharon, had her first walk/run fundraiser for MPS. I have helped with them each year since. At one of the conferences I attended there were bibs for sale. Sharon bought one for Andrew. I thought I could improve on the design and made some for Andrew. Sharon asked if I could make one for each MPS child attending Andrew's walk. Soon after, parents of MPS children called asking if I could make them more bibs. When Sharon was on the board of directors, she asked if I could make some as a fundraiser for the Society. I made 65 bibs, sent them to MaryEllen Pendleton who put the MPS Society logo on them and they were sold at the Disney conference. How wonderful the feeling of walking into the awards banquet and seeing the bibs everywhere.

Soon after, my daughter, Barbara, said people on Facebook were asking who made the bibs. One mother asked if I could make some for her child and she would pay me. I told her I would make them but I would not accept any money for them. The expense for the necessary things MPS kids need is enormous and this is my contribution to the families. I can make them, mail them and it doesn't cost me much money.

Volunteering for the MPS Society does not have to involve a lot of time or money; small things mean a lot. My daughters and I volunteered to make the family gift bags at the Kentucky conference and we helped in childcare. I am the mother of five, grandmother of 14 and great-grandmother of nine. Andrew is a special grandson, the one who has taken me all over the country to places I would never have been able to go. I have met the most wonderful people from all over the world. These bibs are a small thing to give but what I receive back is so great—the love and thanks of grateful parents.

standing ovation

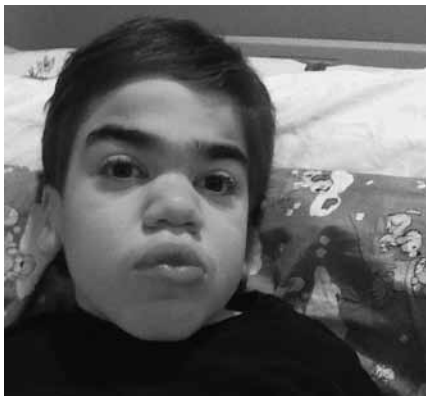
The Standing Ovation Award is intended to honor amazing people in our MPS family for their resilience, courage, tenacity and passion for life while facing the many challenges of having MPS.

We give a standing ovation to:

Leonel Yoque, MPS I

Ever since I was diagnosed with MPS at age 8 my life has been an adventure. I think positive and try not to worry about my illness. I am now 15, and I plan to study law enforcement. I have been involved with the LAPD/USC Cadet Program for nearly two years. This program teaches teens about law enforcement and the duties police do in the field. It also teaches that character counts, including such traits as responsibility. One mental phrase we use when an obstacle gets in our path is, "Avoid it and keep on going, never let your team down" and this is what I do, especially when a difficult time comes. I will never let an obstacle get in my path of life.

Every morning I tell myself that any conflict I deal with during the day, let that conflict be a positive view of what life is about. My team is myself, family and friends. I will never let my team down and I will strive in life with any type of dream.



Conner Anhalt, MPS II

Conner has brought us so much joy and happiness. When he was younger and still had a voice, he was the funniest little guy! He could light up a room with his infectious laugh and his big smile. His imagination was so grand. Conner loved to dress up in costumes and never left the house without a funny hat or bow tie on. He wanted to be a mailman or a "McDonald's Man." He often practiced these trades by having a pen and paper in hand to take everyone's orders, hungry or not! As a young child, Conner could do most anything that other kids his age could, only with much more difficulty. At age 9 Conner lost his speech. That did not stop him from lighting up a room. He still delights when his cousins and "sissy" walk in the room. Conner is 14 now and although he doesn't have the ability to do much anymore, he has taught us all so much about love, patience and compassion.

Sasha Segal, MPS III

Sasha has traits so many of us strive for in our daily lives. She is warm, sweet, funny, non-judgmental and full of good-humored mischief. Most impressive of all, she does not complain. She is almost always upbeat, even during some of her most difficult days when we know she is experiencing the utmost discomfort.

Despite the limitations presented by MPS, Sasha has a rich world. She is enthralled with vibrant flowers and the rustling of trees, is happiest when singing loudly with the car radio, and has clearly let us know she is left handed. Never leave Sasha in the position of only having a right hand available to manipulate her chewy toy as this will lead to disgruntlement. She has always loved bubbles, swing sets, books and ice cream (and we hope

continued >>

>> she always will). Sasha is an adventurous spirit who is as happy on the road as she is at home. She has a very positive impact on everyone she meets.

As Sasha's parents, our focus has been to celebrate the smaller moments of life which can actually be quite huge. We are thankful for every day that we see Sasha walk, smile and laugh.

Emma Weaver, MPS IV

Emma is 10 years old and lives in St. Louis, MO, with her parents, two younger sisters and brother. Her youngest sister Eliza has MPS IV as well. Emma is excited to start fifth grade this year at her school, Shining Rivers, the St. Louis Waldorf School. She is anxiously awaiting the opportunity to study myths and history of ancient civilizations, and compete with children from other schools in the pentathalon. She also studies Spanish and German; math is her least favorite subject.

Emma has a beautiful voice and loves to sing. She performed with a local children's choir last year, and will be auditioning to perform with the Variety Club Children's Choir this fall. Our home and car are often filled with the sound of her beautiful voice. Emma is an avid reader. She also enjoys knitting, form drawing, swimming, gardening and spending time with her friends. She aspires to be a farmer in Hawaii one day, but first would like to compete at the Summer Olympics in beach volleyball. It doesn't seem to deter her dream that we live in the Midwest where there are no beaches, or that she has never played volleyball. There is an Oscar Pistorius quote on a poster where Emma attends physical therapy that she loves. It states, "You are not disabled by the disabilities you have, you are able by the abilities you have." We encourage Emma to explore her gifts and talents, grow them and share them with others. Emma is a kind, loving girl who loves life, which is always evident by her big beautiful smile.

Noah Wiest, MPS VI

Noah is truly a blessing from God. He is the sweetest, happiest boy ever. He loves life and lives every day to the fullest. Noah's favorite food is cheese pizza. He won an award for Student of the Month in first grade. His favorite thing to do is workbooks and he wants to teach others when he grows up. Noah is happy when he can spend time with mom, dad and his younger sister, Emily. He loves attending church and doesn't know a stranger. When Noah was diagnosed, his uncle created a website, www.noahwiest.com, to bring awareness to the disease.

Our family strives to be in contact with other MPS families, especially those newly diagnosed.

Mark DeFilippis, ML III

Mark is a vivacious, fun-loving, 8-year-old boy from Old Bridge, NJ. He enjoys swimming, playing baseball and wiffle ball, as well as video games and watching Nickelodeon, but mostly he loves spending time with his family. Mark is an exceptional student with an unbelievable memory and is adored by all of his teachers and classmates.

Every year he looks forward to taking vacation with his family, usually in Virginia at the end of the summer and Aruba in November.

Mark was diagnosed in July of 2010 and has undergone major surgery at a very young age. Despite being challenged physically, Mark tries his hardest at everything he endeavors and is determined to overcome any obstacle in his way. His positive attitude and strong will are an inspiration to his friends and family and all whose life he touches. With the assistance of a loving family, Mark will strive to face all challenges in his life with the same intensity and perseverance as he has done so far.

research news

2012 Research Grants

The National MPS Society awarded $547,000 in grant funding for 2012, which includes the second year of funding for grants awarded in 2011 plus the 2012 grants. Funding the Society provides has been and continues to be critical as we move forward with our mission to find the cures. We received 16 letters of intent from researchers around the world for the three grants offered in 2012. After reviewing those letters, our Scientific Advisory Board review committee requested full grant proposals from seven researchers.

The Society also will fund $25,000 to support the Lysosomal Disease Network's National Institutes of Health grant research goals. The funding is designated for the Neuroimaging Core, which will benefit the four MPS projects. An additional $15,000 has been allocated for a mucolipidosis partnership grant with the Gandhi Foundation to Dr. Sara Cathey at Greenwood Genetics Center for her study, "PTC 124 for nonsense mutation suppression in ML II and III cultured fibroblasts." A $10,000 partnership grant with the Ryan Foundation funded the University of Minnesota project "Brain structure and function in developmentally normal children ages 4–7." The Society also provides funding for post-doctoral fellows to attend scientific meetings, such as the American Society of Gene and Cell Therapy.

MPS II – $45,000 each year for two years

Gustavo H.B. Maegawa, MD, PhD

Johns Hopkins School of Medicine, Department of Pediatrics

Baltimore, MD

"Induced-neuronal (iN) cells as tools to study the pathogenesis of neurological manifestations in MPS II"

MPS II is a genetic disease caused by the inability to break down large molecules called glycosaminoglycans. MPS II is caused by the deficiency of an enzyme located in the lysosome, essential recycling units present in each cell. In MPS II, the accumulation of undegraded material results in dysfunction of the lysosomes, compromising the entire cells and ultimately multiple organs/systems. The mechanisms of how the storage of glycosaminoglycans can severely affect the brain, causing severe mental disabilities, have not been fully elucidated. Using a new technology, we are now able to convert skin cells into brain cells, called "induced-neuronal cells."

The hypothesis of the project is that the induced-neuronal cells from patients are research tools to study mechanisms causing the neurological problems in MPS II. This project aims to convert skin cells from MPS II patients into the induced-neuronal cells and determine how they can be used to study brain disease in MPS II. Results of this project will provide cell-model to study the brain disease in MPS II, which can subsequently result in the discovery of novel treatments for the neurological problems commonly seen in affected patients.

MPS IV – $40,000 each year for two years

Shunji Tomatsu, MD, PhD

Nemours Children's Clinic – Delaware Valley of the Nemours Foundation

Wilmington, DE

"Development of Long Circulating Enzyme Replacement Therapy for MPS IV A"

MPS IV A results from a deficiency of the enzyme *N*-acetylgalactoseamine-6-sulfate sulfatase. This enzyme is required for the break-down of complex sugars called glycosaminoglycans (GAGs). GAGs are normally found throughout the body, including bones and joints, and are constantly broken down and replaced with new GAGs. In MPS IV A, GAGs cannot be broken down properly, resulting in their abnormal accumulation. Patients with MPS IV A often present with

continued >>

>> systemic skeletal abnormalities (bone deformities, short stature and spinal cord compression).

Several treatment strategies for MPS have been tested. These treatments showed limited effect on bone and brain involvement. Recently, an enzyme replacement therapy strategy using modified long-circulating β-glucuronidase on MPS VII mice was shown to almost completely correct brain storage of GAGs and to reduce storage materials substantially in bone. Given the success of this therapy in MPS VII mice, the question of whether the modified enzyme also works to improve bone pathology is of great interest in MPS IV A mice. We have developed an assessment method to evaluate skeletal pathology quantitatively in MPS mice. The new treatment strategy is expected to improve bone lesions in MPS IV A, leading to increased mobility and a better quality of life.

MPS III Grand Challenge Grant – multi-year $235,000 with support from Team Sanfilippo

Brian Bigger, PhD

Stem Cell & Neurotherapies Group

Manchester, UK

"Evaluation of high dose genistein aglycone in the treatment of mucopolysaccharide disease types III A, B and C"

Sanfilippo diseases result from an inability of the body to break down long chain sugars (GAGs), which are stored in cells. Sanfilippo affects the brains of children, resulting in progressive behavioral and mental degeneration, and ultimately death. There are no treatments available for these devastating diseases.

Genistein is a drug derived from soya beans that is able to reduce GAG storage and improve behavior of mice with MPS III B at high doses. Although there have been unsuccessful clinical trials of genistein with MPS III, they have all used low doses of the food supplement form which we think is too low to get into the brain where it is needed.

Given the beneficial effects of pure genistein in MPS III B mice, we want to evaluate high doses of pure genistein in a placebo-controlled clinical trial in patients with Sanfilippo to see if this drug is effective. The benefits of running a clinical trial using pharmaceutical grade product include definitive assessment of high-dose genistein effects and, if beneficial, genistein could be an approved drug, distributed through healthcare providers and via health insurance. If not beneficial, patients will save on future investment in genistein by investing in other worthwhile therapies.

First year reviews, 2011 grants

The reviews of the first year of research of these grants can be found in the Research section of our website at **www.mpssociety.org/research/2011-research-grants**.

MPS II

Vito Ferro

University of Queensland

Brisbane, Queensland, Australia

"Small molecule chaperones for ERT for MPS II"

MPS III

Patricia Dickson

Los Angeles Biomedical Research Institute at Harbor-UCLA

Torrance, CA

"Choriod plexus-directed gene therapy as a source of intraventricular NAGLU-IGF2 for MPS III B"

General

Alberto Auricchio

Fondazione Telethon

Naples, Italy

"Gene therapy of MPS VI"

Adriana Montano

Department of Pediatrics, School of Medicine

St. Louis University

St. Louis, MO

"Role of inflammation in pathogenesis of MPS IV A"

Richard Steet

University of Georgia

Athens, GA

"Blockade of cathepsin activity and TGF-beta signaling as a therapeutic approach for LSDs"

Second year reviews, 2010 grants

The reviews of the second year of research of these grants can be found on our website under the Research section, 2010 grants, at **www.mpssociety.org/research/2010-research-grants**.

MPS I

Mark J. Osborn, PhD

University of Minnesota,

Minneapolis, MN

"Gene therapy for the central nervous system pathology of MPS I"

MPS II

Brett E. Crawford, PhD

Zacharon Pharmaceuticals, Inc.,

La Jolla, CA

"Glycosaminoglycan inhibitors as substrate reduction therapies for MPS II"

MPS III

Elizabeth F. Neufeld, PhD

David Geffen School of Medicine at UCLA, Los Angeles

"Making a minigene suitable for gene therapy for MPS III B"

continued >>

MPS IV A Clinical Assessment Program (MorCAP)

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

Second year reviews, 2010 grants, *continued...*

MPS IV

Calogera M. Simonaro PhD,
associate professor
Mount Sinai School of Medicine,
New York, NY
"A novel approach for the growth & expansion of bone marrow-derived mesenchymal stem cells in mucopolysaccharidoses type IV and other mucopolysaccharidoses"

ML

Dr. Katrin Kollmann, PhD
(Partnership Grant with Insieme per Gabriel)
University Medical Center Hamburg-Eppendorf, Hamburg, Germany
"Skeletal abnormalities in mucolipidosis II alpha/beta Pathomechanisms and therapeutic strategies"

General

Dr. Andrea Ballabio
(Caterina Marcus Foundation Grant)
Telethon Institute of Genetics & Medicine, Naples, Italy
"Modulating lysosomal function to treat mucopolysaccharidoses"

Dr. Alisdair B. Boraston
Department of Biochemistry and Microbiology, University of Victoria, Victoria, Canada
"Discovery and assessment of inhibitor-based chemical chaperones as potential agents for the treatment of mucopolysaccharidosis III B"

Observational Prospective Natural History Study of Patients with MPS III B

Shire HGT announced in January 2012 that they will sponsor a natural history study for individuals with MPS III B. The objective of this study is to evaluate the natural, unaltered disease progression of MPS III B; the study has been designed similarly to an interventional clinical trial. This is a longitudinal, prospective, observational, natural history study to identify endpoints that may be used for future enzyme replacement therapy trials via standardized clinical biochemical, neurocognitive, developmental, behavioral and imagining measures. Shire expects the results of this study to provide key disease insights, similar to what would be measured in an untreated control group in an interventional clinical trial.

The study is being conducted at the University of Minnesota. For additional information, contact Brenda Diethelm-Okita at 612.625.1594 or dieth001@umn.edu. Complete information about this study is available at **www.clinicaltrials.gov/ct2/show/NCT01509768?term=MPS+IIIB&rank=1**.

Clinical Trials

MPS I

MPS I Intrathecal Enzyme Replacement Clinical Trial

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

The purpose of this research study is to find out whether giving enzyme replacement therapy with Aldurazyme® as an injection directly into the cerebral spinal fluid (the fluid around the spinal cord and the brain)

continued >>

>> can stabilize (keep from getting worse) or improve cognitive decline in patients who have MPS I. The term "cognitive decline" refers to a change for the worse in the ability to think and learn. Difficulty with thinking, memory, language, concentration and decision making are some signs of cognitive decline.

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

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

Study participants will have:

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

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

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

Dr. Agnes Chen
310.222.4160 / 310.782.2999 (fax) / ahchen@ucla.edu
or
Dr. Patricia Dickson
310.781.1399 / 310.782.2999 (fax) / pdickson@ucla.edu

MPS I Intrathecal ERT for Children Being Considered for Transplantation

The University of Minnesota has recently obtained FDA approval for the delivery of laronidase into the spinal fluid of children with Hurler syndrome being considered for marrow/cord blood transplantation. The goal of these studies is to decrease the neuropsychologic decline that has been observed in children with MPS I from the time the patients are initially evaluated to the time they are one year from transplantation. The hypothesis is that there is a significant delay in achieving sufficient enzyme levels in the brain following transplantation, and that this may be overcome by giving enzyme into the spinal fluid until this occurs.

Patients with MPS I who are between 8 and 36 months of age whot 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, Dr. Paul Orchard, can be reached at 612.626.2961 or by e-mail at orcha001@umn.edu. Alternatively, Teresa Kivisto, nurse coordinator with this study, can be reached at 612.273.2924 or by e-mail at TKIVIST1@Fairview.org.

MPS II

MPS II Intrathecal Enzyme Replacement Clinical Trial

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

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

This phase I/II clinical trial is planning to enroll 16 patients with MPS II between the ages of 3 to 8 years with evidence of early neurocognitive decline using an open-label, three-dose trial design. This clinical trial will initially have both a treatment group (12 study

continued >>

>> patients) and a control group (four study patients) with the control group eligible to receive intrathecal enzyme after a six-month observational period. The monthly intrathecal administration of idursulfase-IT will be given using a Port-A-Cath® II Low Profile™ intrathecal implantable access system manufactured by Smiths Medical MD, Inc. (St. Paul, MN) that requires surgical implantation.

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

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

MPS III

Phase I/II Safety, Tolerability, Ascending Dose and Dose Frequency Study of Recombinant Human Heparan N-Sulfatase (rhHNS) Intrathecal Administration Via an Intrathecal Drug Delivery Device in Patients MPS III A

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

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

The study is ongoing but no longer recruiting patients. The estimated primary completion date is September 2012 (final data collection date for primary outcome measure).

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

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

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

Intracerebral Gene Therapy for MPS III A

A one-year, phase I/II gene therapy clinical trial for MPS III A is being conducted at Hôpital Bicêtre – Assistance Publique des Hôpitaux de Paris. This is an open-label, single-arm, monocentric, phase I/II clinical study evaluating the tolerance and the safety of intracerebral administration of adeno-associated viral vector serotype 10 carrying the human SGSH and SUMF1 cDNAs for the treatment of MPS III A. The treatment plan consists of a direct injection of the investigational medicinal product SAF-301 to both sides of the brain through six image-guided tracks, with two deposits per track, in a single neurosurgical session in four patients with MPS III A.

The primary objective is to assess the tolerance and the safety associated to the proposed treatment through a one-year follow up. The secondary objective is to collect data to define exploratory tests that could become evaluation criteria for further clinical phase III efficacy studies.

Lysogene, the biotechnology company sponsoring the clinical trial, announced June 14, 2012, that the last planned patient had been treated.

Primary Investigator Dr. Marc Tardieu, can be reached at Hôpital Bicêtre – Assistance Publique des Hôpitaux de Paris Recruiting Le Kremlin Bicêtre, France, 94275, +33 1 45 21 32 23 or marc.tardieu@bct.aphp.fr.

Additional information about the study can be found at **www.clinicaltrials.gov/ct2/show/NCT01474343?term=MPS+IIIA&rank=4**.

continued >>

>> MPS IV

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

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

Highlights from the phase I/II study:

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

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

MPS VII

On Jan. 5, 2012, Ultragenyx Pharmaceutical, Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, announced it has in-licensed an enzyme replacement therapy program from St. Louis University to treat MPS VII. The in-licensed program is a treatment for an ultra-rare genetic, metabolic disorder that results from the deficiency of the beta-glucuronidase (GUS) enzyme. Also known as Sly syndrome, the disorder was first identified in 1973 by William S. Sly, MD, a world-renowned researcher in inherited diseases, who currently is professor and chairman emeritus, Department of Biochemistry and Molecular Biology, St. Louis University School of Medicine. Dr. Sly will collaborate with Ultragenyx on the MPS VII development program.

"We are pleased to have the opportunity to develop this treatment for MPS VII which has been in the research stage for a long time and has yet to be made available to patients," said Emil D. Kakkis, MD, PhD, chief executive officer of Ultragenyx. "We look forward to working in collaboration with Dr. Sly and the MPS community on this program."

Dr. Sly noted, "After so many years of research by my laboratory and my research colleagues, I am pleased to finally have the chance to see if MPS VII patients can be successfully treated with enzyme replacement therapy. I have confidence in Ultragenyx's ability to advance the MPS VII program through the development process and fulfill our shared goal of bringing this potentially life-changing therapy to patients. We look forward to working closely with the Ultragenyx team on this program."

On Feb. 28, 2012, Ultragenyx Pharmaceutical, Inc. announced that the FDA office of Orphan Products Development granted orphan drug designation for UX003 for the treatment of MPS VII. On March 28, 2012, Ultragenyx was granted orphan drug designation for MPS VII by the European Medicines Agency.

ML II/III

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

MPS III Patient Registry

Team Sanfilippo is working with the Coordination of Rare Diseases at Sanford (CoRDS) organization to build a patient registry for MPS III (Sanfilippo). Information obtained through the registry would be available for future U.S. clinical trials, and will accelerate future research by providing researchers with access to an existing database of pre-screened individuals who are willing to participate in rare disease research. It also will notify patients of opportunities for clinical trials should they be eligible.

How can I enroll?

If you or a family member have been diagnosed with a rare genetic disease, you are invited to submit information to the registry. Patients or their parent/legally authorized representative should contact CoRDS via e-mail at CoRDS@sanfordhealth.org or by phone at 605.312.6413 and provide:

- name
- contact information (mailing and e-mail address, phone number)
- preferred method of contact and best time to contact

CoRDS personnel will contact the individual to briefly discuss the registry, answer questions and send patients a consent form and a short questionnaire.

For more information or to ask questions about the CoRDS registry, contact:

Liz Donohue
CoRDS administrator
2301 East 60th St. North
Sioux Falls, SD 57104-0589
phone: 605.312.6413
e-mail: CoRDS@sanfordhealth.org
www.sanfordresearch.org/cords

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.

Creating an ER Protocol for Your Child

When you have a child with a serious medical condition, it's important to have different plans in place to address a situation as it arises. One of those plans should be for visits to the emergency room. Creating an ER protocol for your child is an important step in streamlining your visit so that all the doctors and nurses involved in treatment are aware of your child's condition and what is necessary to treat immediately. This protocol should include:

- child's name
- birth date
- primary or coordinating physician's name and contact information
- specialist physician's name and contact information (if appropriate)
- primary or most critical diagnoses
- any allergies
- general medical considerations
- physician's signature

Following this information should be instructions for the ER staff regarding testing and care for your child, including the symptoms, vital signs that need to be monitored, necessary tests, medications to give or to avoid, and anything else to avoid in general. This will help prevent a crisis situation for your child, and help everyone involved provide the best care for your child.

The Miracle League

The Miracle League is an organization that affords children with disabilities the opportunity to play baseball. Players are paired up with a buddy and everyone has a chance to play—batting, running the bases, etc. Even children without the use of their arms or legs can participate. The Miracle League has more than 200 leagues across the country, as well as Puerto Rico, with 100 specialized rubberized fields (for safety) and 100 fields under construction. It serves more than 80,000 children and young adults with various disabilities.

For more information, visit **www.miracleleague.com**, call 770.760.1933, or send an e-mail to Johnny@MiracleLeague.com.

Mercy Flight Southeast Offers Free Flights for Those in Need

The mission of Mercy Flight Southeast is "Coordinating free air transportation for children and adults with medical or compelling humanitarian needs." For more than 25 years, Mercy Flight Southeast has coordinated these FREE missions, which are generously flown by a team of volunteer pilots who give of their aircraft, their skills and their fuel, to help those who are in need. For more information, visit **www.MercyFlightSE.org**.

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.

First Hand Foundation Strives to Change Children's Lives around the World, One Child at a Time

In 1995, Cerner Corporation, a leading supplier of healthcare information technology, founded the First Hand Foundation. A nonprofit organization, First Hand assists individual children with health-related needs when insurance and other financial resources have been exhausted.

The Foundation helps with clinical necessities (such as medication and surgeries), medical equipment and travel related to a child's care. Additionally, the Foundation implores doctors, hospitals and equipment providers to discount their services below listed prices. Funding is distributed directly to the child's immediate healthcare provider.

Since inception, the First Hand Foundation has distributed $10 million to 85,000 individuals in 66 countries.

For more information or to apply for funding, go to **www.cerner.com/firsthand**.

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.

Twist 'n Write™ Penagain Pencil Makes Writing Easier for Kids

The Twist 'n Write pencil is a new model specifically designed to fit smaller hands and bring back the fun in writing. A thick 2 mm pencil lead never needs sharpening. The wishbone shaped design forces the school-taught "tripod" position. It is great for those with special needs (carpal tunnel, arthritis, ADHD and many more). It is being sold at Staples, Walgreens, Office Depot, CVS, Office Max and Amazon for less than $2.00. For more information, visit www.penagain.com/twistnwrite.html.

Tissue Bank Aims to Improve Treatment of Developmental Disorders

The National Institute of Child Health and Human Development (NICHD) Brain and Tissue Bank for Developmental Disorder at the University of Maryland in Baltimore is a tissue resource center designed to further research aimed at improving the understanding, care and treatment of developmental disorders.

The NICHD Brain and Tissue Bank serves as an intermediary between the research community and people who wish to donate tissue for research upon the time of their death. The Bank safely stores the tissue until qualified researchers request the tissue for research which has been approved by their Institutional Review Board. Both people with developmental disorders and people free of disorders are encouraged to register and donate tissue. Often times it is the comparison of the unaffected with the affected which unlocks the medical mystery of a disorder.

The availability of tissue from donors with MPS and other storage diseases is especially limited. As more tissue becomes available and more researchers dedicate their life's work to this disorder, new discoveries can lead to new treatments and, perhaps one day, to a cure. It is only through the study of donated tissue that important answers will be found.

If you are interested in becoming a registered donor, or if you have questions regarding the donation process, contact Melissa Davis, project coordinator, at 800.847.1539.

Morgan's Wonderland: Finally, a place where everyone can play!

Located in San Antonio, TX, Morgan's Wonderland is the world's first ultra accessible family fun park designed specifically for children and adults with special needs, their family members, caregivers, friends and the entire community. This 25-acre park is a unique oasis which, through the spirit of inclusion, allows people of all abilities to play, learn and share life-changing experiences together, in a fun and safe environment.

For more information, go to **www.morganswonderland.com/index.html**.

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.

Brave Community Offers Customized Information about Rare Diseases

BraveCommunity.com provides a dynamic source of news, information and personal insights about certain rare diseases for patients and their families, friends and healthcare professionals. The Web site searches the Internet and posts Web content and news stories relevant to certain rare genetic diseases to help you stay informed. BraveCommunity.com also provides disease summaries, information about the clinical trial process and relevant publications. Users can sign up to personalize content and share own stories.

Learn more at **www.BraveCommunity.com**.

Healthcare 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.

donations

in memory of…

Earl Adams
Karen Dupke*

Michael Bodura Sr. and in honor of his grandson Michael Bodura
Joseph and Jacqueline Balukin
Michael and Grace Bodura
Helen Fallon
Garth family
James Heinrich
Kelsesky family
William and Alayne Lowenberger
John Richard and Amy Semk
Tracy Szemanski
The Newspaper Guild of Pittsburgh, Local 38061 of the Communications Workers of America

Kirk Bregman and in honor of Conner Anhalt
Gerald and Susan Anhalt
Mel and Millie Anhalt
Randy and Cynthia Anhalt
Linda Atzil
Mr. and Mrs. Steve Berdinis
Brad and Jill Deutser
Jim and Janice Falick
Marla and Stewart Feldman
Aaron and Perri Fink
George and Celia Fontaine
Howard and Debbie Freed
Laura Graber
Barney, Pam, David and Halley Harris
Allen and Laverne Hoffman
Melissa Houser
Barry and Ellen Hyman
Mr. and Mrs. Barry Kaufman
David and Helaine Lane
Mindy Levinson
Barry and Barbara Lewis
Sandy Lundy
Dickie and Joy Pulaski
Diana Weil
Aileen Weycer
Michael and Carol Wilk

Raymond Author Bryan IV
Paul Baker

Edie Burke
Thomas Burke
Beverly Nivens*

Matthew Caldwell
Marjorie Austin

Jean Gala in honor of Annabelle Bozarth
Felissa Lashley

Danny Gniazdowski
Susan Mikaloff

Harry Gordon Jr.
Mel and Millie Anhalt

Samantha Haynes
Jeff Haynes

Eli Holt
Sandra Greenlee

Jon Houston
Jennifer Throneburg

Lila Beth Hughes
Jill Davis

Dr. George Jackson
Mel and Millie Anhalt

Andrea Johnson in honor of her nephews Wynn and Dorian Johnson
Josephine Albrecht
James and Barbara Ballard
Ronald and Sharon Bonnett
Kathryn Collier
David and Susan Dawson
Jeanne Drevo
Charles and Janet Duchek
Marty and Pamela Fye
Thomas and Pamela Hammond
Arthur and Judith Henning
David and Sara Hughes
Sandra Karl
Alberta and Jennifer Koerner
Chad and Karla Lechtenberg
John and Connie Lentell
Patrick and Eunice McArdle
Dennis and Marilyn McElravy
Gary and Sandra McFarland
Jutta Stuart and Janis Nollendorfs
Catherine Oberg
Patricia Ohmberger
John and Joy Patten
Dr. Robert and Mrs. Carole Quick
Peggy Siedhoff
Sheryl Skala
Karen Smejdir
Lloyd and Trudy Reeves
Thomas Reeves
Lynne Rustad
Stephen and Michele Van Dyke
Mary Welty

Richard Joppa
Meredith Joppa

Kevin Keith
Jack and Mary Ann Campbell

Mary Kirch in honor of her granddaughter Allison Kirch
John and Kim Brady
City Hall Employees
Patricia Clarke
Alyce Cochran
Margaret Cohen
Michael Cohen and Jane Schramka-Cohen
Frank and Loretta Gagliardi
Barb Peshon
Bill and Ruth Rother

Tom Koss and in honor of Michael Lewandowski
Andrew Koss
David and Joann Lewandowski
Mike and Candy Sullivan
Hank and Cheri Verwohlt

Audrey Lawson
Brent Davenport

Lee Matthews
Sam Cluggish
James Deluca
Leo and Elaine Walsh
Yuan Zhang and Jie Huang

Ruth McKenna
William and Margaret Cook

Brian Neveu and in memory of his daughter Desiree
Nevin Cavusoglu

Diane Oakvik
Nancy Mondry

Jim Oliger Jr.
Mary Anne Oliger

Nicole Pagliero
Karina Guajardo

Irene Sellmeyer
Jan Inman and family
Thomas Stader

Doris Smith
Dr. William and Peggy Cook

Mikey Nappi, Frankie McHugh and Uncle Frankie Tirelli
Amelia McHugh

Megan Weisenbach
AmWINS Motorsports Group
Joe Barbieri
Shannon Burnett
Susan Devlin
Michael Fleming
Fultz Racing
Barbara Heskett
IMA board, officers, staff and members
Institute for Defense & Business
Keller Macaluso, LLC
Linda Mansfield
Sidney Moore
Sandra Perry
Paul and Carol Redmond
Ann Shutters
Danielle Trumbull
John Weted and Amanda Rosner
Elaine Whitesides

in honor of…

Cynthia Anhalt's birthday
Mel and Millie Anhalt

Cecilia Rose Bien
Rose Marie Houser

Tyler Bransford
Cortney Bransford

Mr. Joe Brown
Mel and Millie Anhalt

Jared Connor
Belmont Hill School

Amanda Crawford
Catherine Hill

Mark DeFilippis
Gina Golombos
John and Rosemarie Golombos

Mr. and Mrs. Jerry Deutser's anniversary
Mel and Millie Anhalt
Mr. and Mrs. Marvin Kaplan
Nancy Landa

Jackson Dunn-Kraus
Jeanine Stapleton

Barbara Eaker's birthday
Mel and Millie Anhalt

Mitchell Finzel
David and Stacey Aronson*

Jack Fowler
John and Judith Brooks
Kristin DeBolt
Rachel Strom

**Annual Fund donor*

continued >>

>> **Norman Glosserman's recovery**
Mel and Millie Anhalt

David Gorewitz's 65th birthday and in honor of his nephew Ricky Hodgkins
Sarah Glass
Lawrence and Amy Kornetsky
Sheldon and Felice Lieberman
Anthony Martiniello
Rhonda Pare
Jaime Roberts

Quinn Gregory
Sherry Goodin

Karina Guajardo
Rene and Nelda Rodrigues*

Zach Haggett
Dave and Rosie Battaglia

Michelle Hopkins
Mr. and Mrs. Roger Drinkwalter

Dorian and Wynn Johnson
Kacy Zeeck

Jarrett Koblitz
Stacy Black

Mr. and Mrs. Joel Levy's anniversary
Mel and Millie Anhalt

Christian Lon
Ying Guo

Mr. and Mrs. Kenneth Margolis' anniversary
Mel and Millie Anhalt

Michael McGuinniss
Morrison and Foerster Foundation

Waverly and Oliver McNeil
Kale, Chloe and Karissa Merillat

Cameron Mollette
Pamela Greene

Matthew Montano
Amber Snow

Emily O'Connor
Eileen O'Connor

Logan Piefer
Olivet United Methodist Church

Raymond Pinchak
Meredith Pinchak

Cheryl Sorter
Jack and Barbara Sorter

John Thornton
Zamora Elementary

Jack Todd
Jerry and Lisa Todd

Taylor Wojnarowski
Connie Smithson

Jerry and Marilyn Berning's 50th anniversary
Ross Berning

fundraisers

3rd Annual Clara's Courage Hotdog Stand
held by Shane and Jenifer Gibson in honor of Clara Gibson

Bethel Elementary School Fundraiser
held by teachers in honor of Oliver (MPS III) and Waverly (MPS III) McNeil and Aiden Spaeth (MPS II)

Birthday fundraiser
held by Jen Bernstein in honor of the Zeighami family

Christian Lon charity paintball event
hosted by Destiny Paintball Team

Dress Down Fridays
held by Asa Messer Elementary in memory of Aurora Laorenza

Gold party for MPS
held by Elizabeth Tuck and Margo Anderson in honor of Nathan Roma

Homecoming trunk show party
hosted by Michelle McPherson in honor of Nathan Roma

Ice Cream Corner Awareness fundraiser
hosted by Janelle Kunellis in honor of Allison Restemayer

Lacrosse game
held by Kevin Connolly in memory of Mark Lessing Jr.

Madison Lewis birthday fundraiser
held by Wayne, Paige, Morgan and Madison Lewis

McNeil lemonade stand
held by Tracy Leonard in honor of Waverly and Oliver McNeil

Mission Slimpossible
by MPS Facebook moms

MPS Bootcamp
hosted by Mercedes Johnson and family and LiveWire Fitness

Play for Taylor concert
by the piano students of Rachel Wojnarowski

Schnare marathon fundraiser
held by Ann Schnare in honor of Logan Piefer

Thomson High School MPS Awareness Week
held by Shelby Key in honor of Chloe Key

Unity Project fundraiser
held by Briarcliff Middle School in honor of Alena Galan

donations

Ross and Hilary Berning
B.L. and Anne Bickham
Claudina Bonetti Marcus
Richard Bosse*
Meg Cooper
Bob and Laura Cowin
Davids Project
Nancy DePeal
Almir Djokovic
Dan Dunn
Blythe Evans
Lou Fabian
Dianne Frazier
Agnes Greene
Wallis and Monica Hampton
Scott and Lynn Hopkins
Jennifer Hutcheson
Gary and Paula Kacer
John and Janet Kappel
Paul and Nga Keith
Saranne and Livingston Kosberg
Thomas and Carol Kuhn
Gerry Kyle
Steve and Cam Lanier
Lori LeDoux
Mark and Joan Lessing
Chris and Cathy Maat
Mitchell and Cherylynne Moore
Rene and Patricia M'Sadoques
Austin Noll*
Mary Ann Olsen
Operation United Change
Thomas and Vickie Patterson
Samuel Ramsey
Ralph and Bonnie Rennaker
Carl and Donna Rose
George and Athena Sarantinos
Tracy Szemanski*
Kevin Sheridan
Mike and Barbara Smith
Isaac and Brooke Story
James Strohecker*
Bill and Helen Vespe
R.M. Marc and C.R. Weal
Philip Werner
Rebecca Winek
Roy and Zezee Zeighami

matching gifts

AIG Matching Gifts Program
Allstate Giving Campaign
Bank of America
CA Technologies
Hewlett-Packard
IBM Employee Charitable Contribution Campaign
Microsoft Matching Gifts Program
The GE Foundation
VMWare Foundation Matching Gift Program

*Annual Fund donor

Mucopolysaccharidoses (MPS) and related diseases are genetic lysosomal storage diseases caused by the body's inability to produce specific enzymes.

classifications

SYNDROME
MPS I
EPONYM
Hurler, Scheie, Hurler-Scheie
ENZYME DEFICIENCY
α-L-Iduronidase

SYNDROME
MPS II
EPONYM
Hunter
ENZYME DEFICIENCY
Iduronate sulfatase

SYNDROME
MPS III A
EPONYM
Sanfilippo A
ENZYME DEFICIENCY
Heparan *N*-sulfatase

SYNDROME
MPS III B
EPONYM
Sanfilippo B
ENZYME DEFICIENCY
α-*N*-Acetylglucosaminidase

SYNDROME
MPS III C
EPONYM
Sanfilippo C
ENZYME DEFICIENCY
Acetyl CoA: α-glycosaminide acetyltransferase

SYNDROME
MPS III D
EPONYM
Sanfilippo D
ENZYME DEFICIENCY
N-Acetylglucosamine 6-sulfatase

SYNDROME
MPS IV A
EPONYM
Morquio A
ENZYME DEFICIENCY
Galactose 6-sulfatase

SYNDROME
MPS IV B
EPONYM
Morquio B
ENZYME DEFICIENCY
β Galactosidase

SYNDROME
MPS VI
EPONYM
Maroteaux-Lamy
ENZYME DEFICIENCY
N-Acetylgalactosamine 4-sulfatase (arylsulfatase B)

SYNDROME
MPS VII
EPONYM
Sly
ENZYME DEFICIENCY
β-Glucuronidase

SYNDROME
MPS IX
ENZYME DEFICIENCY
Hyaluronidase

SYNDROME
ML II/III
EPONYM
I-Cell, Pseudo-Hurler polydystrophy
ENZYME DEFICIENCY
N-acetylglucosamine-1-phosphotransferase

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.

BOARD OF directors

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

Stephanie Bozarth, VICE PRESIDENT
6203 Larstan Drive
Alexandria, VA 22312
703.256.1980
stephanie.bozarth@mpssociety.org
MPS IV parent

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

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

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

Dawn Checrallah
58 Leroy Drive
Riverside, RI 02915
401.639.2689
dawn.checrallah@mpssociety.org
MPS I parent

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

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

Lisa Todd
11111 Jordan NE
Albuquerque, NM 87122
505.797.3603
lisa.todd@mpssociety.org
MPS II parent

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

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

Roy Zeighami
6420 Diamond Drive
McKinney, TX 75070
972.965.5253
roy.zeighami@mpssociety.org
MPS III parent

PRESIDENT EMERITA
Marie Capobianco
Ernie Dummann
Steve Holland
Mary Majure Couture
Linda K. Shine

STAFF

EXECUTIVE DIRECTOR
Barbara Wedehase
barbara@mpssociety.org

DEVELOPMENT DIRECTOR
Terri Klein
terri@mpssociety.org

PROGRAM DIRECTOR
Laurie Turner
laurie@mpssociety.org

ADMINISTRATIVE ASSISTANT
Kelly Rose
kelly@mpssociety.org

BOOKKEEPER
Angela Guajardo
angela@mpssociety.org

SCIENTIFIC ADVISORY BOARD

Alessandra D'Azzo, Ph.D.
Gideon Bach, Ph.D.
Lorne A. Clarke, M.D.
Robert Desnick, M.D., Ph.D.
Patti Dickson, M.D.
Matthew Ellinwood, D.V.M., Ph.D.
Mark Haskins, Ph.D., V.M.D.
John Hopwood, Ph.D.
Joseph Muenzer, M.D., Ph.D.
Elizabeth Neufeld, Ph.D.
Beth A. Pletcher, M.D.
Kathy Ponder, M.D.
Mark Sands, Ph.D.
Edward Schuchman, Ph.D.
Calogera Simonaro, Ph.D.
William Sly, M.D.
Charles H. Vite, D.V.M., Ph.D.
Steven Walkley, D.V.M., Ph.D.
David Wenger, Ph.D.
Chester Whitley, M.D., Ph.D.
John H. Wolfe, V.M.D., Ph.D.
Ed Wraith, M.D.

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