



DIRECTIONS

FAMILIES OF SPINAL MUSCULAR ATROPHY

ANNUAL SMA CONFERENCE 2013
 The Disneyland Hotel - Anaheim, CA | June 13-16



Families of SMA
Research. Support. Hope.

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ANNUAL SMA CONFERENCE 2013

The Disneyland Hotel – Anaheim, CA | June 13-16

Registration for the 2013 Annual SMA Conference, held in Anaheim, California June 13th–16th, at The Disneyland Hotel, is now available

For more information
and to register, please visit
www.fsma.org



We are a non-profit, 501(c)3 tax exempt organization. Funds will be specifically directed to scientific, educational or literary purposes in keeping with a charitable organization. The organization is proud to provide funding to the FSMA newsletter and website, which provide information and networking opportunities to its members.

Submissions

To submit articles or make other contributions to our newsletter, please contact us at: newsletter@fsma.org.

Digital images are encouraged! Send your digital pictures to: newsletter@fsma.org.

Change of address

Send changes, including ZIP code to: info@fsma.org or call 1-800-886-1762 or mail to: FSMA Membership, 925 Busse Road, Elk Grove Village, IL 60007

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FSMA does not support or endorse any particular treatment or therapy. Information contained in this newsletter should not be used as a substitute for consultation with a qualified healthcare professional.

On the Cover: Nora Gooden

Mission Statement



Families of Spinal Muscular Atrophy is dedicated to creating a treatment and cure by:

- Funding and advancing a comprehensive research program
- Supporting SMA families through networking, information and services
- Improving care for all SMA patients
- Educating health professionals and the public about SMA
- Enlisting government support for SMA
- *Embracing all touched by SMA in a caring community*

Our vision is a world where Spinal Muscular Atrophy is treatable and curable.

June 13th–June 16th, 2013
The Disneyland Hotel, Anaheim, CA

2013 ANNUAL SMA CONFERENCE

Every year, Families of SMA sponsors a conference to bring together the leading SMA researchers, clinicians, and families living with SMA. Families of SMA has been hosting the Annual SMA Conference since 1989. The weekend is filled with a wide variety of workshops, a memorable children's program, a family fun fest and carnival, many opportunities to connect and interact with families and receive first hand updates from the researchers.



Let the Adventure, Fantasy and Fun Begin

Every year we look forward to reuniting as a community at this conference and showing our support for others. As always, the Family and the Research Conferences run alongside each other. This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it. The interactions between the researchers and families at this one conference are extremely special. The annual conference also provides the children an opportunity to make new friends and have a great time. We are expecting another great attendance of over 1300 attendees.

The Disneyland Hotel and Paradise Pier Hotel have been carefully selected to meet the many needs of Families of SMA as the 2013 destination for the Annual SMA Conference. The Disneyland® Hotel first opened in 1955 as the place to be and be seen. More than 50 years later, the hotel that started it all is back, re-imagined for a new millennium. You'll enjoy the hotel's 975 newly renovated guest rooms which are non-smoking and offer great features – like



refrigerators, coffee makers, flat-screen TVs and wall safes—as well as, complimentary Internet (wired and wireless) in

every room, and complimentary weekday newspapers available on every hotel floor. After a day of meetings, indulge in outstanding restaurants and lounges (like the one-of-a kind Trader Sam's—Enchanted Tiki Bar), relax in new pools and spas, and network in the Downtown Disney® District—all just steps from your room.

You must first register for the conference before you can reserve your discounted hotel room rate of \$144 at the Disneyland or Paradise Pier Hotels.

SMA Conference Goals

- 1) To allow networking between researchers, medical providers, patients and families
- 2) To educate researchers on the latest research advancements
- 3) To attract the best researchers to the SMA field and encourage collaborations
- 4) To educate medical providers, patients and families on the basics of SMA
- 5) To update medical providers, patients and families on the latest research and medical progress

To register for the
2013 Annual SMA Conference,
please visit www.fsma.org

Highlights from the 16th Annual Spinal Muscular Atrophy Research Group Meeting

An important way Families of SMA advances Spinal Muscular Atrophy research is The Annual SMA Research Group Meeting. The International SMA Research Group Meeting is the largest SMA research conference in the world. For 16 years, FSMA has organized the conference, and has financially supported the meeting by covering hotel, travel, and registration for all research presenters for a total cost of about \$200,000 each year. The meeting this year was held at the DoubleTree Hotel in Bloomington, Minnesota from June 21 to June 23.

Overall two hundred researchers attended the conference to learn about the latest developments in SMA research. The researchers attending the conference represented over fifty academic organizations and thirteen companies, from thirteen countries around the world. Ninety-eight research presentations were given at the conference this year. They included seven invited talks during the special session on "Lessons Learned in Pediatric and Neurological Drug Development", twenty-nine talks selected by the FSMA Scientific Advisory Board from submitted abstracts, and sixty-two poster presentations.

The conference, which is the only open venue for annual communication between international SMA researchers, has many benefits for the entire SMA community:

- Enables open communication of early, unpublished scientific data among researchers - a key component in accelerating the pace of research.
- Creates a vital sense of community among SMA researchers, creating a collaborative spirit that results in many productive research partnerships.

- Allows cross-disciplinary dialog among basic researchers, clinicians, and industry representatives, which is vital in creating effective therapies.
- Motivates SMA researchers by allowing for direct interaction with families and patients living with SMA.
- Allows young researchers to interact with experienced leaders in the field, which helps build the future of the SMA research community.

One SMA researcher said: *"SMA Research Group Meeting is of great importance to get a feeling for main trends in SMA research. Due to the poster sessions also minor-fields of research get the chance to be recognized. In these few days I learned more about SMA than in half a year of literature research—in the scientific way by integration of different insights into a more complete picture of SMA pathology and in a personal way by meeting SMA patients."*

Special Session: Lessons Learned in Pediatric and Neurological Drug Development, moderated by Dr. Douglas Kerr of Biogen Idec

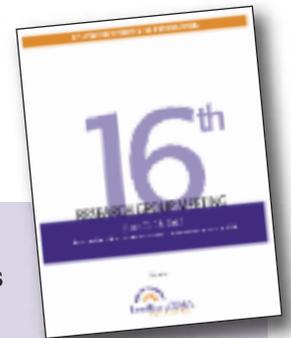
The SMA research community is primed to begin human clinical trials on the first novel drugs specifically designed for SMA. By focusing on successful drug programs for other diseases that are paving the way, the session goal was to highlight areas of importance for future and current SMA drug development. The topics included special considerations in pediatric drug development, the use of biomarkers in early drug development,

Summary of Research Findings Presented at the 2012 Conference:

The research meeting is organized into sessions focusing on major unanswered topics in the field. This year there were seven sessions with an expert moderator leading each. These included sessions on:

- 1) Lessons Learned in Pediatric and Neurological Drug Development
- 2) Regulation of SMN Expression with Therapeutic Implications
- 3) New SMA Animal Models
- 4) SMN Functions and Phenotypes in Motor Neurons
- 5) Temporal and Spatial Regulation of SMN
- 6) Clinical Research
- 7) SMA Drug Development

clinical trial design issues, the use of complex outcome measures in drug trials, and development of combination therapies. Dr. John Kissel, Professor of Neurology at The Ohio State University gave the opening address. His talk was titled, "SMA Therapy Development: Challenges and Opportunities." Dr. Kissel introduced the session by giving an overview of the state of SMA drug development, by specifically addressing: 1) the current SMA drug pipeline, 2) clinical trial readiness, 3) SMA trial design, 4) SMA outcomes and biomarkers, 5) patient recruitment efforts, and 6) inclusion of multiple SMA patient types into trials. His opening address was followed by six invited talks focused on drug programs that have already started human clinical trials. Speakers discussed the importance





of conducting pre-clinical studies in a very careful way that is mindful of FDA requirements and the importance of engaging with the FDA early. They also discussed that obtaining FDA approval for human studies takes time and includes long-term and expensive studies, assessing both safety of your product and safety of your delivery / surgical method. Dr. Eva Feldman gave a very nice example of this in her talk on intraspinal stem cell transplant for ALS, where the safety studies around the surgical technique were paramount for entering human trials. Also, discussion occurred about the fact that early clinical trials will be small and focused on safety, and that drug development and clinical trials advance in a step-wise manner. In addition, all the speakers mentioned the fact it is critical to have sensitive test measures for clinical trials, other wise good drugs can be thrown away. These include long-term endpoints that must measure clinically meaningful benefit, as well as biomarkers assessing molecular response to the drug to be used for early go / no-go decisions. Specifically, Dr. Shefner discussed the use of electrophysiological measures as endpoints in ALS and SMA, and the complexities of implementing them across trial sites. There was much discussion on whether SMA currently has a useful biomarker for early trials, and how critical it is to develop one soon, whether SMN based or not. Biomarkers in the CSF should be of greater focus in SMA. Finally, Dr. Kerr discussed how critical patient retention is to the success of clinical trials, and how difficult trial retention is in fragile medical populations, like in infants with SMA Type I. He provided an example from an ALS clinical trial being conducted by Biogen Idec on how home visits can greatly improve retention, allowing trials to more effectively test whether drugs work or not. Invited talks included:

- Importance of Biomarkers in Early Drug Development, Ajay Verma, MD, PhD, Vice President,

Neurodegeneration and Experimental Medicine, Biogen Idec

- Gene Therapy And Exon Skipping Strategies For Treatment of Muscular Dystrophy, Jerry Mendell, MD, Professor of Pediatrics and Neurology, Nationwide Children's Hospital
- ASO Drug Development for ALS, Timothy Miller, MD, PhD, Assistant Professor of Neurology, Washington University
- Outcomes and Biomarkers in Neuromuscular Disease Drug Development, with Focus on Electrophysiology, Jeremy Shefner, MD, PhD, Professor and Chair of Neurology, Upstate Medical University
- Intraspinal Stem Cell Transplantation in ALS Stem Cell Therapy for CNS Indications, Eva Feldman, MD, PhD, Professor of Neurology, University of Michigan
- Small Molecule Development for ALS, Douglas Kerr, MD, PhD, Director of Experimental Biology, Biogen Idec

Regulation of SMN Expression & Function with Therapeutic Implications and New Animal Models of SMA, moderated by Dr. Adrian Krainer of Cold Spring Harbor Laboratory

The first session addressed ways in which regulation of SMN expression can be modulated for therapeutic benefit. First, Natalia Singh from Iowa State University presented data on the identification of a new antisense oligonucleotide sequence that can be targeted to effectively promote exon 7 inclusion. Then Faraz Farooq from the McKenzie lab showed that modulating the p38 pathway by pharmacological means stabilizes SMN mRNA leading to more SMN protein. This resulted in modest but significantly increased survival in the Delta 7 mouse model of SMA.

The next three talks were presented on new animal models of SMA. Kavita Praveen from the Matera lab at UNC Chapel Hill presented her findings from a new *Drosophila* model of SMA. SMN mutants without any endogenously made SMN showed no apparent change in the splicing of most mRNAs. However, a sub-

stantial defect in locomotion and survival occurred suggesting uncoupling of survival and movement from snRNP biogenesis in flies. Kentaro Sahashi from Adrian Krainer's laboratory reported on the use of inhibitory antisense oligonucleotides (ASOs) to lower SMN protein levels and generate a model of SMA, which arises post-developmentally. Inhibitory ASO was delivered by ICV injection into a mouse model of SMA Type III at 60 days of age, and disease manifestations were exacerbated, with motor dysfunction, shortened lifespan, motor neuron loss, and abnormal neuromuscular junctions. Sandra Duque from the Burghes Lab at OSU presented data on generating a pig model of SMA using AAV9 vectors and inhibitory ASOs to disrupt SMN expression. ICV injection into 5-day old piglets resulted in abnormal gait, ataxia, and abnormal neuromuscular junction electrophysiology and function. Pig models will aid in therapy development for SMA due to more similar physiology and morphology with humans than found in rodents. Another pig model is also being made in the Lorson lab at the University of Missouri, using a different strategy.

SMN Functions and Phenotypes in Motor Neurons, moderated by Dr. Samuel Pfaff, Salk Institute for Biological Studies

This session focused on possible new functional targets of the SMN protein, which may eventually provide new points of therapeutic intervention for SMA. First in the session, Jocelyn Cote from the University of Ottawa presented data on CARM1 protein in SMA. CARM1 protein levels are increased in SMA cells. In normal cells, SMN protein represses CARM1 translational via the Tudor domain of SMN. This data suggests that SMN could control how much of some proteins are made. Yong-Chao Ma of Northwestern University presented data that the protein histone deacetylase 5 is mis-regulated in SMA motor neurons. Modulation of this pathway can partially rescue motor neuron defects in culture and in zebrafish, suggesting this protein as a potential drug target in SMA. Next, Min Jeong Kye of Harvard University showed data suggesting the SMN complex regulates the expression

miRNA-183, which in turn controls mTOR activity, providing evidence that this pathway may also be of therapeutic interest in SMA. Next, Sara Custer from the Androphy Lab, presented data on the role of SMN in motor axons. She showed that SMN interacts with the alpha-COP protein through the 2b domain of SMN. This interaction may prove to be important to SMN functionality in neuronal processes, and thus provide a point of therapeutic intervention in SMA. Finally, Marcus Riessland presented a possible new protective gene modifier of SMA currently being called MOD2. It could eventually prove to be a novel drug target too. In zebra fish, SMN mutants have truncated motor neurons, while a MOD2 and SMN double mutant has rescued motor axons that are functionally restored too.

Temporal and Spatial Regulation of SMN, moderated by Dr. Arthur Burghes of Ohio State University

The next five talks focused on when and where SMN protein is needed. Christine Beattie from OSU showed that removing SMN early during motor neuron development causes axon outgrowth defects in zebrafish. Adding SMN protein back at different times in these fish indicated that SMN is needed in motor neurons at the time they are born or soon after. Lingling Kong from the Sumner Lab at Johns Hopkins University presented data that SMN is needed directly in motor neurons themselves to preserve synaptic integrity. Providing back SMN protein to just motor neurons in the Delta7 mouse model of SMA almost fully rescued neuromuscular physiology and morphology. It also improved defective spinal cord circuitry onto motor neurons, although survival benefit was modest. Providing SMN back to muscles did not improve neuromuscular or motor neuronal defects, but did increase muscle size and survival modestly.

Vicki McGovern from the Burghes Lab at OSU also presented extensive data on a similar topic. SMN was specifically removed from certain tissues with two copies of SMN2 remaining in the background. Mice with SMN removed from muscle or from many neurons but not motor neurons lived to 200 days.

Mice with SMN removed from neurons, including motor neurons, lived for only about 20 days. Conversely, replacement of SMN into each of these three tissues: 1) just muscle, 2) just into motor neurons, or 3) into some neurons but not motor neurons resulted in 10-day to 22-day survival for each group. However, replacement of SMN in both motor neurons and other neurons resulted in mice that lived for over 200 days. This suggests that SMN is sufficient in only neuronal tissue for survival, but it is required in both motor neurons and other types of neurons too.

In the next talk, Lyndsay Murray from the Kothary lab in Ottawa showed the intermediate (2b/-) model of SMA is capable of neuromuscular remodeling. However, it is reduced compared to what is typically observed after injury or paralysis. The reduction may be due to a loss of terminal Schwann cells, which are known to be integral to neuromuscular remodeling. Finally, Hans Rindt from the Lorson lab at the University of Missouri presented data that SMN delivery specifically into astrocytes and microglia can modestly increase survival in the Delta7 mouse model of SMA, although not nearly as long, more widespread expression. These two talks explored the role of SMN in other cell types, associated with motor neurons

Human Clinical Research, moderated by Dr. Kathryn Swoboda of University of Utah

This session included discussion of new outcome measures and biomarkers for use in SMA clinical trials, as well as updates on SMA clinical trials. The session started with a talk from Jacqueline Montes from Columbia University on the use of fatigue as measured by the six-minute walk test as a clinical endpoint in patients with SMA Type III. Incorporating electrophysiological measure into the six-walk test provided real time evaluation of the specific muscle groups responsible for fatigue. Aga Lewelt from the University of Utah showed data on a pilot exercise study in children with SMA Type II and III. She showed that a 12-week, 3 days/week, supervised, home-based, strength-training program appears feasible, safe, and well tolerated. A larger

scale study may now be warranted. David Jacoby of Repligen Corporation reported on their Phase I study of RG3039 in healthy volunteers. RG3039 was safe and well tolerated to the highest dose tested of 3 mg/kg. Drug exposure analysis demonstrated a dose dependent relationship of drug levels and the inhibition of the molecular target DcpS. The session ended with updates from three clinical trials conducted in SMA patients. These include the CARNIVAL Type I trial in SMA infants, the VALIANT Trial in Ambulatory Adults, and a Phase II Trial of Salbutamol in SMA Type II and III patients. None of these trials showed a clinical benefit across the study population. However, they allowed important information to be obtained on 1) how to run multi-center trials, 2) how to manage retention and clinical management of fragile SMA populations during trials, 3) validation of clinical endpoints and outcome measures, 4) the use of consensus clinical care across sites, and 5) the assessment and optimization of SMN related biomarkers from peripheral blood.

SMA Therapeutic Development, moderated by Dr. Elliot Androphy of Indiana University

During this session talks were given on several ongoing drug programs. Monica Siegenthaler of California Stem Cell presented data on cervical spinal cord transplants of human motor neuron progenitor cells in to the Delta7 mouse model of SMA. Transplanted mouse pups showed improvements in respiratory function measured by pulse oximetry and in neuromuscular pathology at the intercostal muscles. This was due to trophic support from the transplanted cells into the spinal cord, not re-innervation. Stefania Corti from the Comi Lab at the University of Milan presented data on the transplantation of human motor neurons derived from iPS cells. This enhanced survival in the Delta7 mouse model of SMA by 50%. Johnathan Cherry from the Androphy lab at Indiana University presented data on small molecule enhancers of SMN protein levels. The compounds increased SMN survival up to 25 days, although the molecules are currently insoluble and must be delivered in DMSO, which is itself toxic to neonatal

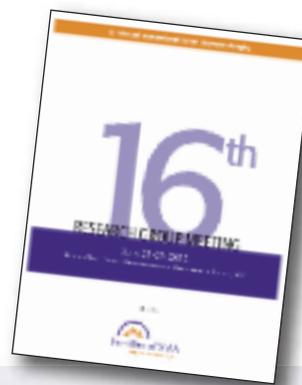


mice and may mitigate responsiveness. Chien-Ping Ko from USC presented data on RG3039 in the Delta7 mouse model of SMA.

He showed that compound treatment resulted in an increase in cross-sectional area of muscle, significantly increased the percentage of fully innervated muscles, improved synaptic transmission at the neuromuscular junction, and increased the number of interneuron synapses onto motor neurons in the lumbar spinal cord. Nikolai Naryshkin of PTC Therapeutics and Friedrich Metzger of Hoffman La Roche presented data on their series of small molecule SMN protein enhancers. The compounds work by correcting SMN2 splicing, increasing survival by more than 100 days, protecting motor neuron numbers, and enhancing the number of fully innervated muscles.

The session ended with three talks on gene therapy for SMA. Martine Barkat from the Institute of Myology in Paris showed that injection of scAAV9-SMN into a single hindlimb muscle resulted in dramatic increases in survival in the Delta7 mouse model. ICV injections and IV injections also both resulted in dramatic survival, but ICV injections into the CNS were more effective. At P100, 80% of the ICV injected mice were alive while 60% of systemic or IV treated mice were already dead. Somewhat surprisingly, early ICV injection allowed for gene expression outside of the nervous system in the peripheral tissues too. Marco Passini at Genzyme discussed his work to determine the minimally required percentage of spinal cord motor neurons that need to be targeted for significant survival benefit in Delta7 SMA mice. This appears to be about 30% of motor neurons. The group at Genzyme is now using normal juvenile pigs, whose spinal cords are more similar to humans, to optimize their intrathecal delivery methods directly into the CSF, using 30% targeting of motor neurons as the desired therapeutic benchmark. Brian Kaspar from Nationwide Children's hospital closed the meeting by discussing his work on translating scAAV9-SMN gene therapy towards the clinic. His group has recently started a 6-month GLP safety study required by the FDA for human trials. In addition, the Kaspar group is exploring the use of CSF delivered gene therapy because it would allow for lowered doses of drug to be used and reduce the manufacturing burden significantly. Importantly, CSF delivery will allow for older and bigger patients to be treated, rather than just infants.

Many of the programs discussed during the therapy session at the research meeting were also presented during the closing session of the Annual SMA Family Conference. Here representatives of the leading drug programs answered questions from SMA families.



Families of Spinal Muscular Atrophy Released Summary of the Researcher Question and Answer Session at The 2012 Annual SMA Conference

The 2012 Annual SMA Conference brought together 1,200 families and researchers from around the world to Minnesota this June. At the conclusion of the conference the families gathered for a special and unique opportunity to hear the very latest research announcements along with a Question and Answer session on the leading drug programs for SMA.

Panel 1: Representatives of Drug Programs in Preclinical Development.

- Omar Khwaja, M.D., Translational Medicine Leader, F. Hoffmann-La Roche AG
- Todd E. Bowser, Ph.D., Associate Director, Research and Development, Paratek Pharmaceuticals
- Monica M. Siegenthaler, Ph.D., Manager of Preclinical Development, California Stem Cell, Inc
- Brian Kaspar, Ph.D., Assistant Professor, The Research Institute at Nationwide Children's Hospital
- Louise Simard, Ph.D., Professor & Head, Biochemistry and Medical Genetics, University of Manitoba

Panel 2: Representatives of Drug Program in Clinical Development or of Human Research Studies.

- Stephen J. Kolb, M.D., Ph.D., Assistant Professor of Neurology, The Ohio State University
- Eric Dessaud, Ph.D., Clinical Manager, Trophos
- David Jacoby, M.D., Ph.D., Medical Director, Repligen Corporation
- Kathie M. Bishop, Ph.D., Director of Clinical Development, Isis Pharmaceuticals
- Douglas Kerr, M.D., Ph.D., Medical Director, Biogen Idec

Please visit the Families of SMA website to view the presentations, at www.FSMA.org.

The 2012 Annual Spinal Muscular Atrophy Conference in MN was the Most Successful Midwest Conference Yet

The 2012 Annual SMA Conference in Minnesota was by far the largest Midwest conference that has ever been hosted. The final attendance number was almost 1,200, which is a wonderful showing of families, researchers and professionals looking to connect with each other in the SMA Community.

The 2012 Annual Spinal Muscular Atrophy Conference was a fantastic success with around 1,200 families, researchers and professionals in attendance! This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it for SMA families. The interactions between the researchers and families at one conference are so special. The annual conference also provided our children an opportunity to make new friends and have a great time. There were so many wonderful events that made this conference the most memorable and successful conference to date.

The Family Conference began Thursday, June 21st with the **Newly Diagnosed Program**, where families, who were diagnosed from 2011 to present, were able to attend this special session prior to all of the other families arrival. It was a wonderful way to slowly introduce new families to the conference as well as other families and professionals on a much smaller scale. This program included:

- An Introduction to the Conference and SMA Community
- Understanding Genetics and the Disease
- Choices, Evidence, Hype and Hope
- Life after Diagnosis - Parents Share Their Journey
- A Meet and Mingle Session with Families, Doctors, Chapter Officers and Board Members
- Optimal Care for SMA Type I
- Optimal Care for SMA Type II and Type III
- A Grieving Parents Session

While the conference was underway for the families, The **16th Annual International SMA Research Group Meeting** was being held in the same hotel, which is the biggest SMA research conference in the world. Families of Spinal Muscular Atrophy organizes the conference, and financially underwrites the meeting by covering hotel, travel and registration for all research presenters. There were 200 researchers that attended from around the world. These researchers represent 50 institutions in total, 13 biotech and pharmaceutical companies, and 13 countries worldwide. The personal connections made between families and researchers meant so much to everyone who attended.



Continuing Medical Education

ANNUAL SMA CONFERENCE 2012

Families of SMA began the conference a little different this year as we introduced a new component for medical professionals. Families of SMA offered a Continuing Medical Education Conference on Wednesday, June 20th, prior to the start of the 2012 Annual SMA Conference and Researcher Conference.

The CME Conference, titled *"Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role"*, for medical professionals, is the first of its kind for Families of SMA. The conferences have previously covered two components of SMA; Research and Support. The Research aspect covered scientific, research & clinical updates, while the Support aspect included the family support workshops and family networking. The addition of this conference provides an exciting new third component of Care to the Families of SMA Conferences by educating medical professionals on SMA. Families of SMA partnered up with Gillette Children's Specialty Healthcare, who are accredited to provide continuing medical education credit for medical professionals, and offered a full-day of lectures and educational materials to all attendees.



Videos and Presentations from the Families of SMA Continuing Medical Education Conference for Spinal Muscular Atrophy Now Available

The videos and presentations from the Continuing Medical Education Conference for medical professionals, titled *"Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role"*, are now available on the FSMA website.

Families of SMA has posted videos and presentations from the 2012 Continuing Medical Education Conference held this past June in Bloomington, MN. This year's Annual SMA Conference introduced this new component for medical professionals by offering a Continuing Medical Education Conference on Wednesday, June 20th, prior to the start of the 2012 Annual SMA Conference and Researcher Conference.



To view the video presentations please visit

<http://www.fsma.org/FSMACommunity/Conference/ContinuingMedicalEducationConference/>

Details on the 2013 CME Conference at Disneyland, in Anaheim, CA, on Wednesday June 12th, are available on the Families of SMA website.

The 2012 Annual Spinal Muscular Atrophy Conference Family Fun Fest

Ice cream was offered to all attendees as the most exciting part of the evening began – **The Family Fun Fest!** The Family Fun Fest was an absolute huge hit! Games were adapted so that every child could participate and win great prizes. The children and adults all had a great time playing games, socializing, eating many treats, and every child left with a large bag of goodies!

There was a wonderful atmosphere at this year's conference! The Conference kicked off on Thursday evening with an amazing **Meet and Greet** to introduce and bring everyone together to celebrate the start of a great weekend! Attendees enjoyed a dinner, as well as many snacks such as, Popcorn, Cotton Candy, and Snow Cones. The Minnesota Delegation showed tremendous support as Senator Klobuchar helped to welcome everyone to the conference and then participated in the first Relay Race against Isabella Andrade, who has SMA Type II. The incredibly popular **Researcher Relay Race** always starts off the conference in such a positive way! All of the children and SMA researchers had fun racing each other while conference attendees cheered them on.



ANNUAL SMA CONFERENCE 2012



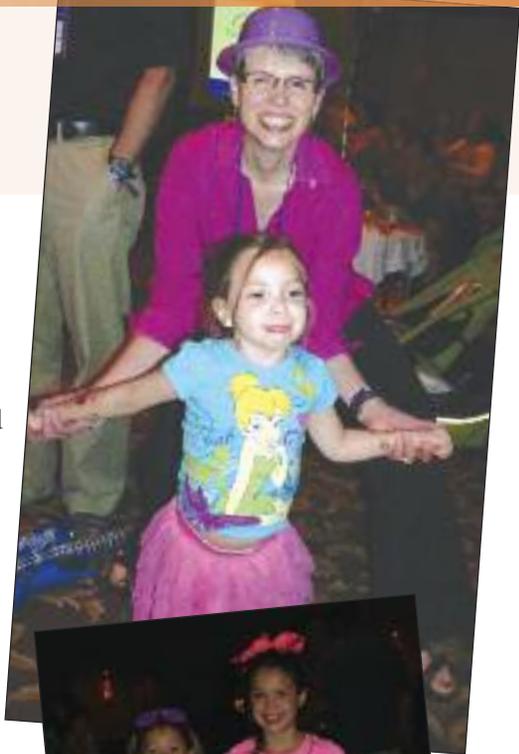
The 2012 Annual Spinal Muscular Atrophy Conference Researcher Relay Race



The 2012 Annual Spinal Muscular Atrophy Conference SMA Dance Party

Friday evening was the first ever **SMA Family and Researcher Dance Party**, and it was a huge hit with parents, children and researchers alike! The theme was a Disco Party, with lots of disco balls, lights, blow-up guitars and microphones that put everyone into a “groovy” state of mind. We are so thankful to Mike O’Dea who sponsored the amazing DJ who helped keep the dance floor full throughout the duration of the evening with his great music choices. Our wonderful volunteers were a huge help as well, as they showcased their many dance skills and encouraged all of the families to “boogie” on the dance floor.

A fun addition to the dance party was a photo booth opportunity for families and researchers to dress up with boas, big sunglasses, blow-up instruments and pose in front of a disco scene. We are so thankful that Mike Graney was able to take photos of each group which allowed us to showcase these entertaining photos on our Families of SMA Facebook page.



The 2012 Annual Spinal Muscular Atrophy Conference

Photo Booth



ANNUAL SMA
CONFERENCE 2012

On Saturday evening, we held the **Movie and PJ Party** for families. Each person was greeted with a bag full of treats, along with water bottles and a table with assorted candies for them to choose from.

Families brought their own pillows and blankets to lie on the floor and wore their snazziest PJs for an official pajama movie night! Popcorn popped all night for families to eat as they watched the movie, "The Muppets" which was a huge hit for the children, while parents caught up with old friends and met new ones.



21
countries

We were honored to have SMA Families and Researchers attend this conference from all over the world. There were individuals attending this conference from over 21 countries including: Argentina, Australia, Austria, Brazil, Bulgaria, Canada, Chile, Denmark, France, Germany, India, Italy, Netherlands, Philippines, Romania, Slovakia, Spain, Sweden, Switzerland, United Kingdom and the United States.



We were so privileged to have Jennifer Miller Smith and Aaron Smith of Expo Convention Contractors from Miami, FL donate all of the signs that were displayed throughout the conference. All of these signs donated to Families of SMA were worth over \$30,000 and they were absolutely magnificent and we cannot thank them enough for their generosity. There were spectacular signs and displays for the Family Conference, the Researcher Conference and the Continuing Medical Education Conference. Aaron worked so hard all weekend putting up each sign, banner and drape, just to then take them down and move them for the next event. He helped for countless hours all weekend to ensure this conference was so wonderful. We cannot thank Aaron and Jennifer enough for their continuous efforts throughout the conference, which was such an important part of making this conference a success!



The Wyatt Kyle Sutker Foundation and The Jacob Isaac Rappoport Foundation hosted another wonderful luncheon this year inviting all Type I families to attend. Families of SMA would like to extend our most sincere gratitude and appreciation towards Julie & Steve Sutker as this year could be the last year that The Wyatt Kyle Sutker Foundation hosts the Type I Luncheon. It was always a great way to provide Type I families with the opportunity to meet and spend time together in a relaxed and friendly social setting. There were around 200 attendees at this special luncheon, who had a wonderful time interacting and supporting one another.

The 2012 Annual Spinal Muscular Atrophy Conference Children's Program

This year's **Children's Program** was extraordinary and another huge success! Thanks to so many of our incredible volunteers, all of the children had a fabulous time. Children stayed busy with countless arts and crafts projects, many fun toys, exciting movies, fun activities, bubbles, 3 Wii stations, a dance session and so much more.

There was fantastic entertainment for the children to enjoy such as a face painter, a magic show, an adorable tea party where children were welcomed to bring their dolls, as well as a costume story teller who told stories of Airplane Annie and The Rainbow Lady and a balloon twister.



ANNUAL SMA CONFERENCE 2012



As always, the Annual SMA Conference would never be possible without the assistance of our fantastic volunteers! Whether they helped with registration set up, working the incredibly chaotic Children's Program, setting up and running carnival games, blowing up thousands of balloons, moving boxes, or any of the other countless jobs that needed to be done, our volunteers were absolutely amazing.

The 2012 Annual Spinal Muscular Atrophy Conference

Friday and Saturday offered families informative workshops and sessions. There were

30 workshop sessions

over **30 workshops** offered during the conference covering the latest information on critical topics in SMA Care including:

- Sharing your SMA Type experiences, as well as a session for Adults with SMA, Grandparents of SMA children and a session for grieving parents.
- Life Care Planning
- Genetics and Reproductive Options for SMA Families
- Hands on Physical Therapy
- Toy Adaptation
- Becoming College Ready
- It's a Wonderful Life
- SMA Kids Talk it Out, Sessions I and II
- Siblings Talk it Out Workshop Sessions I and II
- Orthopedic Management
- Healing the Grieving Heart Parts I & II
- Augmentative Communication
- Good Nutrition for All People with SMA Parts I & II
- Occupational Therapy: Optimizing Function & Fun
- Medical Management for Adults with SMA
- Breathing Basics and Care Choices for SMA Type I
- Breathing Basics for Type II and Type III
- What it Means to be a Teen on Wheels
- Fundraising Event Operations Best Practices & Revenue Strategies
- Family Dynamics: How a SMA Family Copes Living Life with a Family Member that has SMA
- Transition to Adulthood
- Tapping into Creativity: Tools for Personal Healing
- The Power of Mobility – A Guide to Power Mobility in SMA
- Optimizing Quality of Life: Making the Choices that Matter to you Most
- Yoga Therapy
- Aquatic Therapy
- Anticipatory Grief: Preparing to Expect the Unexpected

Some of the presentations from the 2012 Annual SMA Conference are available online at www.CureSMA.org.

Families of SMA is so honored to receive funding from the **Jacob Isaac Rappoport Foundation**, in memory of Jacob Rappoport, which supports the "Newly Diagnosed Program". Last year, their funding allowed nearly 200 individuals, all of whom had a family member recently diagnosed with SMA, to attend this important conference. We are thrilled that they have again supported this program to allow more newly diagnosed families to attend the 2012 Annual SMA Conference and experience the benefits of this amazing conference.

Families of SMA would like to thank Barbara & Gene Trainor and the **Erin Trainor Memorial Fund**. This mission of the Erin Trainor Memorial Fund (ETMF) is to provide Conference Scholarships allowing newly diagnosed SMA Families/individuals the opportunity to attend the Annual SMA Conference. The ETMF will generate substantial funds which will be used for Conference Scholarships allowing newly diagnosed SMA families and individuals the opportunity to attend The Annual SMA Conference. In addition, ETMF will provide increased awareness of Conference benefits to individuals affected by SMA, corporate partners and the medical community. If the endowment gets large enough then funds may also be used to help support other needs of newly diagnosed families at the conference, and additional general conference expenses. Over \$225,000 has been raised so far, illustrating forward progress toward achieving the initial \$1 million dollar goal. In February of 1994, Barb and her husband Gene lost their daughter Erin to SMA Type I. It is absolutely incredible that 18 years later, Erin's impact is felt by thousands of SMA families and researchers.

Information on the 2013 SMA Annual Conference at Disneyland in Anaheim, California, is available at www.CureSMA.org

We hope to see you there!



IPAD DRAWING WINNERS

Families of SMA was able to offer a great incentive to all attendees who completed a conference survey this year. Any conference attendee who submitted a conference evaluation form before the Researcher Q&A on Sunday morning, had their name entered into a raffle to win a new Apple iPad. The first three winners that were announced at the conference were: Valerie McPherson of New York, grandmother to Oscar Merulla – Bonn who has SMA Type II; Cristian Neira of Chile, father to Antonia who has SMA Type III; and Debbie Duggan of Canada, grandmother to Zoe Duggan, who has SMA Type II.

A fourth drawing for an iPad was held after the conference so that any attendees who wanted to submit their surveys online could also enter to win. All surveys were to be completed by Thursday, July 12th at 11:59pm Central Standard Time to be eligible for the iPad drawing.

The 4th iPad winner was Rio Landa of Georgia, mother to Mateo, who has SMA Type II.

Everyone at Families of SMA would like to thank **The Charles Rosenbaum Foundation**, as well as an anonymous donor, who provided us with these four Apple iPad's. Their generosity has made these SMA families extremely happy and grateful! Thank you to all conference attendees who completed a conference survey, this has helped provide us with great feedback to consider for improving next year's conference.



Sunday wrapped up the conference with a family buffet breakfast for all attendees. Then, the **Sunday Closing Session** began where families could learn the latest research developments from researchers which were given to families at Sunday's Closing Session and included a Research Update and Researcher Q & A. Leading experts in the SMA Research Community answered questions from SMA families. Presentations from this informative session are available on-line at www.CureSMA.org.

We would like to take this opportunity to thank all of the amazing companies and organizations for their generous support of the 2012 Annual SMA Conference. These sponsors and vendors are partners in our community who are critical to success in the battle against SMA. Many of these partners contributed to scholarships and family assistance programs for the 2012 Conference and for travel and lodging expenses for the International SMA Research Meeting. Without their support many patients, families and researchers would never be able to attend and join together in this fantastic meeting.

This year, we had an all time record of 40 vendors represented at the conference and it was an amazing way to provide another service to families attending the conference. We had a great variety of vendors displaying their products which offered families the ability to test out and learn about what each company had to offer.

Submitted Photos from the Conference Now Posted on the Families of SMA Facebook Page

- Many photos taken of children, families and researchers at the 2012 Annual SMA Conference in Bloomington, MN are posted on the Families of SMA Facebook Page at this link: www.facebook.com/familiesofsma

- These wonderful photos have been submitted by many families and friends who attended or volunteered at the Annual Conference. This year's conference was such an amazing and memorable conference for all attendees. The final attendance number was around 1,200, which is a remarkable record number of attendees for a Mid-West SMA Conference.

- There were so many incredible moments and special events that made this conference extremely special. Thank you to all of the friends and families who have submitted photos of these special conference moments. If you have any photos from the 2012 Annual SMA Conference that you would like to submit, please email newsletter@fsma.org or post them on the Families of SMA Facebook page at: www.facebook.com/familiesofsma.

Families of SMA Awarded Grant from the National Institute of Health for The Annual SMA Research Group Meeting

Families of SMA has received a grant from the National Institute of Neurological Disorders and Disease (NINDS) at the National Institutes for Health (NIH). We thank them for the fantastic support that helps bring together the SMA research community along with our families.

The SMA Research Group Meeting is the largest research conference in the world for SMA. It was held June 21, 22, 23 in Minneapolis, MN. There were over 200 researchers that gave 100 different updates on the latest breakthroughs in SMA research.

The research conference is held together with The Annual SMA Family Conference, which is the largest conference in the world for families affected by SMA and for medical professionals involved in providing support and care for SMA patients

Running the two conferences simultaneously gives the unique opportunity for SMA families, researchers, and clinicians to interact and meet each other.

Quest Diagnostics Participates in the Annual SMA Conference

Thanks again for inviting Quest Diagnostics to participate in the Annual SMA convention. “To say it was an eye opener is an understatement,” says Mary Jo Tague, a Women’s Health Specialist from Philadelphia. “Meeting the families with SMA and hearing their heartfelt stories on how this devastating disease has affected their lives, helped us realize how important our role will be this year in creating physician awareness.”

Rita Cacini, a Women’s Health Specialist in Tampa, Florida said “The FSMA event was four days filled with fun for the children and education for the parents. The staff members of FSMA truly care and helped to make a difference in the lives of these families. It was a wonderful experience to meet the staff, families and special children of FSMA.” In addition to attending the Annual Conference, the Women’s Health Specialist at Quest Diagnostics have participated in many of the local Walk and Roll events and fundraising activities. These community events allow us the opportunity to let people know Quest Diagnostics can provide carrier testing to help identify couples who may be affected.

It was truly an honor for our team to partner with FSMA who continue to provide real hope to the families and patients impacted by the disease.



BAYADA Pediatrics Proud to Sponsor FSMA Annual Conference

BAYADA Pediatrics, a specialty of BAYADA Home Health Care, is proud to be a premier sponsor of two major, national events hosted by FSMA

- The national FSMA conference, held recently in Minneapolis
- The Cure SMA Across America Walk-n-Roll fund raising program

“BAYADA has been supporting FSMA for over eight years at the local level through sponsorship of the Annual PA Chapter Walk-n-Roll in Philadelphia, PA and the Annual Gray’s Gang Walk in Charlotte, NC,” said BAYADA Pediatric Practice Leader Karen Buttler. “Now, we have an exciting opportunity to make a positive impact on a national scale.”

BAYADA President Mark Baiada agrees. “I became involved with FSMA through my colleague, friend, and psychologist Dr. Al Freedman, whose son Jack was diagnosed with SMA at the age of six months. Jack is now 17 and over the years I have come to understand the challenges his family faces on a daily basis. Getting involved with the local walk for a cure was one way to give back to help Jack. Now, we can work with FSMA to make a difference in the lives of more and more families across the country.”

The national conference proved to be a valuable experience for over two dozen BAYADA employees who attended. They had the opportunity to set up an exhibitor display, network with SMA-affected families, develop relationships with physician experts, and attend the continuing medical education program.

Moving forward, our clinicians are taking what they have learned at the conference and are developing a training program for the nurses who care for children with SMA, and an educational program for parents.

“The conference provided a myriad of rich educational resources that will support our ongoing efforts to enhance our expert knowledge in diseases that affect many of the children who receive our care,” said Buttler.

We look forward to partnering with the wonderful team at FSMA on future initiatives that may benefit the families we service.



BAYADA Pediatrics offers a broad range of services and a team of pediatric professionals who are committed to keeping children safe at home. The nurses receive advanced training to care for children with complex care needs, which includes specialization in tracheostomy and ventilator care. The nurses provide care at home and at school, and they are committed to ongoing communication and collaboration with each child’s parents and physicians.

To find a BAYADA Pediatrics office near you, call 888-910-PEDS.



Pictured during the FSMA national conference are (from l to r) BAYADA Minnesota Pediatrics Director, Krista O’Connor, Dr. Al Freedman, Rocco Arizzi, BAYADA client, BAYADA Director of Pediatric Clinical Operations, Kathy Pfeiffer, and BAYADA Minnesota Clinical Manager, Lynn LaMott.

Leading Researchers Give Talks on SMA Research and Drug Development at the Annual SMA Conference

The 2012 Annual SMA Conference brought together 1,200 families and researchers from around the world to Minnesota this June. At the conclusion of the conference the families gathered for a special and unique opportunity to hear the very latest research announcements along with a Question & Answer session on the leading drug programs in SMA. Summaries of the talks given during this session are below.

For slide presentations about each talk below see:
<http://www.fsma.org/Research/News/>

Update on Families of SMA Research Activities: Jill Jarecki, Ph.D., Families of SMA Research Director

In her talk, Dr. Jarecki focused on the FSMA research strategy and recent investments. She discussed that FSMA is dedicated to creating a treatment and cure for Spinal Muscular Atrophy (SMA) by funding and advancing a comprehensive research program. This has included \$53 million in research to date in three distinct research funding areas:

- 1) Basic Research to understand the disease and provide seed ideas for drug making
- 2) Drug Discovery to find and develop new SMA drugs
- 3) Clinical Trials to provide the means to test new drugs effectively

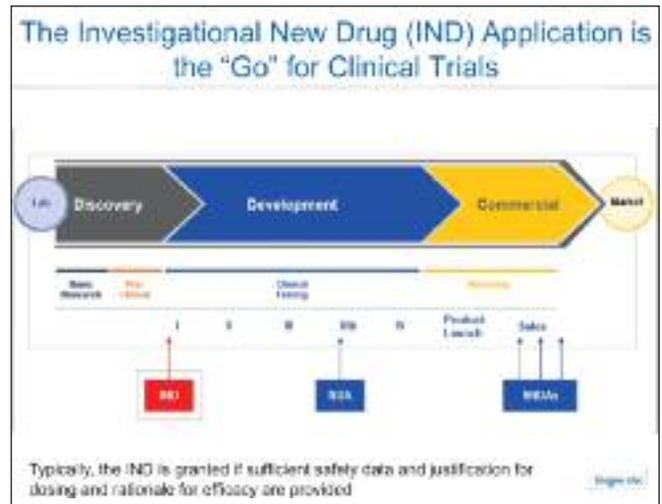
Please see our funding facts page on the web for details.

All research at FSMA is based on expert and independent prioritization and oversight of research projects. This approach ensures that FSMA funds only the most promising research, and that funded projects are run in a professional and efficient manner under the guidance of world-class experts.

Next she continued to explain details about each of the three FSMA funding areas. She began with basic research and the fact that she is often asked why our community still needs to invest in basic research, when we have drugs about to enter clinical trials. The answer lies in the fact that basic research allows us to better understand SMA biology, which reveals new and more effective ways of making SMA drugs by providing seed ideas for new drug programs. Also FSMA is committed to finding ways of treating every type of SMA at every stage of the disease. This could require several different approaches, some yet to be discovered by basic research studies. FSMA is committed to funding basic research, and on July 5, 2012 FSMA released a Request for Proposals for new basic research projects.

FSMA believes it is critical to continue to build a larger, more diverse drug pipeline for SMA, which simply means increasing the number of viable drug candidates and using multiple different approaches for them. This is key because only 10 to 20% of drugs that begin in human clinical trials ever get FDA marketing approval. Moreover, only about 20 to 25 new drugs total receive FDA approval each year across all diseases, even with many very large companies focused on making new drugs. Drug development is hard, and drugs often fail in the clinic for reasons that are hard to predict ahead of time.

In order to plan for this, FSMA is helping to build a drug pipeline for SMA. We have funded 6 programs for \$18.5 million



to date. This included two new awards in Spring 2012:

- 1) \$750,000 to Dr. Brian Kaspar for Gene Therapy
- 2) \$700,000 to Dr. Peter G. Schultz at CALIBER

FSMA plans to fund two new programs in 2012. A Requests for Proposals for preclinical drug discovery programs in SMA was announced on June 28, 2012. Currently, there are about a dozen drug programs ongoing in SMA. They are at many different stages of development, from the earliest stages to more advanced stages. For example, the Trophos program in France is now in Phase II human trials, having completed recruitment for a 2 year 165 patient trial. Repligen has completed a Phase I trial in healthy adult volunteers, and ISIS has started a Phase I safety trial in children with SMA. Typically, as time goes on, some of the drug candidates in the current pipeline will continue to advance, some will fail, and other new candidates will replace them.

One major goal for FSMA funded drug programs is to provide early seed money that can be leveraged to obtain industry or government involvement / funding. We have been quite successful in this. Two

Please see www.fsma.org for a detailed summary of the findings from the 16th Annual SMA Research Group Meeting.

good examples from our direct drug discovery funding are from the Repligen and Paratek drug projects that FSMA funded. FSMA started the quinazoline project in 2000, the first ever industrial drug program for SMA. It was out-licensed to Repligen in 2009, and the company is now responsible for funding and executing all clinical development. Also, FSMA provided \$2 Million in early funding to Paratek Pharmaceuticals for tetracycline compounds to correct SMN2 splicing. Paratek scientists used this preliminary data to obtain a multi-million dollar award from NINDS.

Once our community has drug candidates in hand we need a means to test them effectively. The clinical trial network Project Cure SMA was started by FSMA in 2001 as a collaborative initiative between our organization and clinical investigators. It was designed to facilitate the progress of promising new therapies toward clinical trials. Overall, this effort was intended to develop the needed infrastructure to conduct pivotal FDA clinical trials on novel SMA drugs, while initially conducting repurposed drug trials as novel therapies were being developed. The Project Cure SMA network has conducted five clinical trials and helped develop multiple clinical trial endpoints, with over \$6 million in funding from FSMA.

Our current goals in this area are to help develop the needed infrastructure to conduct pivotal FDA clinical trials on novel SMA drugs, rather than to test repurposed drugs. Our community needs to be ready to conduct FDA registration trials by having validated trial testing measures, established means for patient recruitment, and experienced clinical trial sites. We also want to add value to the 2 major government initiatives: 1) The NeuroNEXT SMA trial at NINDS (see

most recent issue of our newsletter Compass for more details) and, 2)

Statewide New Born Screening Pilots funded by NICHD. NeuroNEXT will help identify and validate early biomarkers for SMA clinical trials, which will make testing drugs more efficient. Newborn screening will allow patients to be treated as early as possible.

Major Findings from the 16th Annual SMA Research Group: Louise Simard, Ph.D., Professor and Head of Biochemistry and Medical Genetics, University of Manitoba

Dr. Simard started her talk discussing that SMA is a disease with a spectrum of severities including SMA Types 0, I, II, III and IV. It affects approximately 1 of 6,000 births. It is an autosomal recessive disease, meaning an affected individual must inherit a mutated SMN1 copy from each parent, both of whom are SMA carriers. SMA is thought of as a motor neuron disease. Motor neurons are very special cells with long axons that grow meters to reach their muscle targets. Motor neurons are the cells most sensitive to lowered SMN levels. However, other tissues may be affected in at least some types of SMA too.

Lowered levels of SMN protein cause SMA. This is due to the loss of the SMN1 gene on both chromosomes, as discussed above. The varying clinical presentation in SMA (Type 0, I, II, III, IV) is caused by SMA individuals having different numbers of a low functioning back-up gene called SMN2. Patients with more severe SMA typically have fewer SMN2 gene copies, while those with more mild forms have more.

The SMN2 back-up gene provides a unique opportunity for therapy development. SMN2 primarily makes a deleted

SMN protein that is not very functional. However, it does make some functional SMN protein. Therapeutic opportunities can come from boosting the amount of functional SMN protein made from SMN2: by activating gene expression; by correcting the underlying defect in SMN2 that results in incorrect mRNA splicing; or by stabilizing the SMN protein. It is important to recognize that understanding the basic biology of SMA is what leads to effective therapy development and the strategies used in the SMA drug pipeline.

Drug development is a long process lasting 10 to 15 years. It contains many steps. One of the most critical aspects of developing drugs is to “do no harm”. Drugs must be shown to have an acceptable risk to benefit profile.

During the research meeting, we heard updates from three clinical trials conducted in SMA patients. These include the CARNIVAL Type I trial in SMA infants, the VALIANT Trial in Ambulatory Adults, and a Phase II Trial of Salbutamol in SMA Type II and Type III patients. None of these trials showed a clinical benefit across the study population. However, they allowed important information to be obtained on 1) how to run multi-center trials, 2) how to manage retention and clinical management of fragile SMA populations during trials, 3) validation of clinical endpoints and outcome measures (testing measurements), 4) the use of consensus clinical care across sites, 5) the assessment and optimization of SMN related biomarkers from peripheral blood, 6) the need for better natural history data, and 7) the likely benefits of newborn screening.

One of the most important factors in the success and current pace of SMA research is COLLABORATION. Collaboration is very evident at the research meeting, and actually has been actively fostered by

Leading Researchers Give Talks on SMA Research and Drug Development at the Annual SMA Conference (*cont.*)

having the meeting itself for the past 16 years. During the course of the research meeting, it is very evident that we have cross-disciplinary interaction between basic, researchers, clinicians, industry, and advocacy groups. In addition, there is an impressive shared toolbox in both SMA and other neurodegenerative disorders, as we heard in the “Lessons Learned” session. The shared toolbox included: many mouse models with different disease severities, first presentations of a SMA pig model, and use of normal non-human primates to study drug distribution. Having all of these tools allows for:

- 1) Proof-of-principle testing
- 2) Toxicology & biodistribution needed by the FDA
- 3) Identification of barriers & finding solutions
- 4) How to best delivery drug for maximal efficacy (who/what/where/when/why)
- 5) REPLICATION/VALIDATION of results

Multiple drug candidates for SMA were presented at the meeting. First we will discuss small molecule drugs, and several small molecule drug candidates were presented, including RG3039 from Repligen and PTC/Roche compounds. In SMA mice, RG3039 increases survival, improves neuromuscular physiology and morphology, and enhances motor function. A Phase I study has been completed, and it appears safe in single dose. In a second class of small molecule, PTC-SSN, SSQ, and SSX correct SMN2 splicing, improve motor function, greatly enhance survival, and improve neuromuscular physiology and morphology.

SMN gene therapy using AAV9 vectors are showing a very robust benefit in mice, as presented by Marco Passini of Genzyme, Martine Barkats in Paris, and Brian Kaspar at Nationwide Children’s

Hospital at the meeting. Tests of delivery, dose, tissue distribution, and safety are currently ongoing. These groups are working towards Phase I Clinical Trials now. The remaining challenges are focused on making sufficient virus for human use and optimizing delivery methods, such as those focused on direct CSF delivery.

Talks were also given on alternative therapies that address motor neuron deficiency or lack of functionality, such as work on motor neurons progenitors derived from stem cells. Intraspinial transplant in mice does show modest survival benefit and benefit to respiration. The current challenges include differentiating cells to the needed cell type, preparing sufficient cells for human use, optimizing surgical delivery, and achieving sufficient range of action within the spinal cord. These methods are currently providing benefit by neuroprotection rather than motor neuron replacement.

There are many questions we need to answer to make the best drugs. These include understanding:

- 1) Where SMN protein is used
- 2) What the protein does
- 3) When it is needed
- 4) How the reduction of SMN makes motor neurons sick

Obtaining a greater understanding of this through basic research will inform us on how to improve drug strategies (which may include a combination of therapies) and also identify new drug targets.

The Drug Development Process: Douglas Kerr M.D., Ph.D., Director of Medical Research, Biogen Idec, Member FSMA Scientific Advisory Board

During his talk, Dr. Kerr described the

general drug development process from an industry perspective. He started by telling us that his first FSMA conference was in 1999, and how heartening it is to see the growth of the conference, the many critical research advances leading to actual drug candidates, and the growth of industry involvement in SMA

He began describing drug development by describing that developing a new medicine takes an average of 10–15 years and cost \$1.3B. Moreover, the Congressional Budget Office reports that “relatively few drugs survive the clinical trial process”. Then Dr. Kerr summarized each step in the overall drug development process. It starts at:

- 1) The earliest discovery stages looking for drug candidates
- 2) Moves to studies that enable filing of an Investigational New Drug (IND) application with the FDA
- 3) Then to FDA approval of the IND application allowing the start of human trials
- 4) To first human safety studies often in healthy adult volunteers
- 5) To Phase II proof of concept studies in patients
- 6) Finally to larger Phase III pivotal FDA registration trials

Next, Dr. Kerr described each of these areas in more details. For instance, key questions for the early preclinical discovery stages are the following:

- Does the drug work in cells and in SMA animals
- What are the potential adverse drug effects
- Where does the drug go and how does it behave
- What does the drug do to organs and tissues

- What is the safe dose to start in human clinical studies
- What monitoring do we need to do in human clinical studies

Once these questions have been answered, the sponsor can submit all the collected data to the FDA in the form of an IND application, which if approved gives the green light for human clinical studies.

Now the clinical development stage of the process begins. Dr. Kerr stresses that the goals of the first human studies called a “Phase I study” are safety and tolerability. The goal is not to see if the drug works or not, which is the goal of later staged Phase II and III trials. The specific goals of Phase I studies are to answer the following questions:

- What are the drug levels in blood at various dosages
- What is the half-life of the drug or how long does it stay in the body
- Does it alter metabolic pathways
- Is there evidence for pharmacologic activity (biomarkers)

In Phase II studies, proof of concept data is sought to show some preliminary evidence that the drug is working in humans. Often this is in the form of some biochemical change (biomarker) indicating “you’re on the right track”. It is desirable to see a dose response here in a biomarker or clinical outcome, meaning there is a linear response to drug levels versus biological response being measured. Also, it is important to determine the best dosing schedule to be used during the Phase III studies that will prove the drug works.

Phase III trials are the pivotal trials needed for FDA registration and marketing approval. Here the sponsor needs to show evidence the drug works and is safe, using a validated and clinically meaningful endpoint (i.e. death or

function). The FDA also typically requires statistical significance (i.e. 95% chance that the observed benefit is not likely due to chance) over the entire study population, not just a response in a subset of participants. Most often the FDA will require the use of placebo. A successful “New Drug Application (NDA)” will allow the sponsor to market the drug for a very specific purpose and patient population only.

Drug development is hard. This is evidenced by the increasing costs and time to reach the market. Also, new drug approvals are not keeping pace with R&D spending in recent years.

Therefore, the government has put incentives in place to encourage companies to invest in drug development. The Orphan Drug Act (ODA) was passed in 1983 to encourage the research and development of medicines to treat rare diseases. Because of this, drug approvals for rare diseases have increased from less than 10 total in the 1970’s to over 200 in the last decade.

Given how hard drug development is, how can we stack the deck in our favor? There are number of things that can be done to improve the efficacy of drug development. These include:

- 1) better understanding disease mechanism from the onset (basic research investments)
- 2) develop more specific and potent drugs
- 3) better use of biomarkers to make informed decisions early in the process
- 4) personalized medicine
- 5) better use of natural history data from clinical databases
- 6) better consortia of clinical sites
- 7) bring the trial to the patient instead of the patient to the trial

Finally in closing, Dr. Kerr discussed that it is really important for patients to understand what a clinical trial is and is not. Clinical trials are research, not therapy! During clinical trials, we do not know the drug is safe and we do not know the drug is effective. The goal is to learn, not to make the patient better.

Also, a clinical program that does not answer the question of whether the drug is safe and effective will fail to be approved and will not be available for patients at all. Thus, it is critical to design all trials with this in mind. What does this mean for patients?

- Placebo groups are almost always required in clinical trials
- Clinical trials have strict entry criteria
- Clinical trials are hard on patients and families
- Most drugs tested will not work or are found to be insufficiently safe to justify approval
- The most likely beneficiary is the community and future patients

Drug development is hard, but on the other hand the amount of academic research, clinical research, drug company investment, advocacy and patient involvement is unprecedented right now in SMA. It is likely SMA is poised for a major breakthrough in the next 5-10 years. Working together, we CAN and WILL make huge strides in SMA.

Please see www.fsma.org for a detailed summary of the findings from the 16th Annual SMA Research Group Meeting.



Marvin and Laney Stulley with their grandchildren at their 50th Anniversary Party



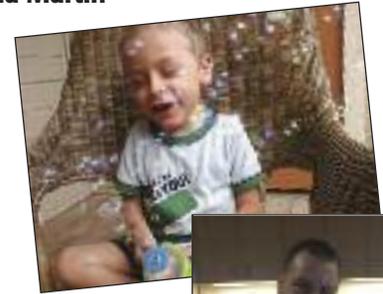
Maxwell Peppers



Mateo Medina, Peyton Elsner and Arianna Martin



Mateo Medina



Matthew Williams



Melissa Milinovich



Natalie Shuler



Madi Ramirez



Madison Smith with star of So You Think You Can Dance Tiffany Maher



Nadia Rodriguez

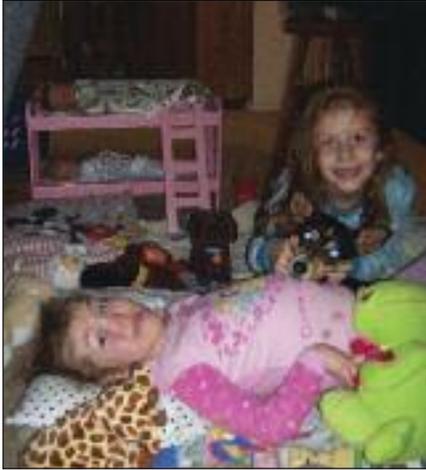


Mia Ehorn



Malena Daya

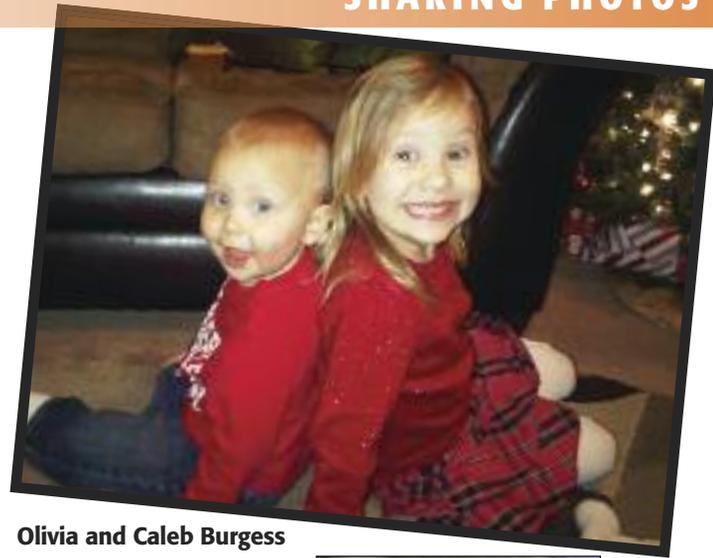




Tambryn and Braelyn Campbell



Stella Bartlett



Olivia and Caleb Burgess



Tianna Rivera



Vincenzo Schabach Fedrigo



Scarlette Ricotta



Savanna Williams



Tilly McRoberts



Shaekia Sterling-Black



Nadia Rodriguez



Nadine Natcho



Philip Struble



NINDS Launches First Trial, A Biomarker Study for Spinal Muscular Atrophy, with Innovative, New National Clinical Trial Network

The Network for Excellence in Neuroscience Clinical Trials (NeuroNext) is a twenty-five site national clinical trial network created by NINDS of the NIH to test promising new therapies for both pediatric and adult patients with neurological diseases. By creating shared infrastructure, NINDS hopes to maximize efficacy and minimize the cost and time associated with running clinical trials. The first study to be conducted by NeuroNext is a Biomarker and Natural History Study in infants with Type I SMA. Families of SMA will be collaborating with NeuroNext and funding patient travel for the study.

Spinal Muscular Atrophy (SMA) is a inherited disease that results in loss of nerves in the spinal cord and weakness of the muscles connected with those nerves. The muscles most frequently affected are those of the neck and trunk that control posture, those of the legs and arms that control movement, and those in the area of the ribs that help breathing.

There is no known treatment for SMA; historically, nearly half of babies born with the most severe form of the disease have died before two years of age. All people with SMA have a higher than normal risk for progressive disability. The most severely affected are at risk for breathing complications and early death.

Stephen Kolb, MD, PhD is the Protocol Principal Investigator for the study “Spinal Muscular Atrophy (SMA) Biomarkers in the Immediate Postnatal Period of Development”. Dr. Kolb is located at the Ohio State University, in Columbus, Ohio.



“Families of Spinal Muscular Atrophy (FSMA) is very pleased that NINDS has established the NeuroNEXT Clinical Trial Network. We believe it will help streamline and improve the efficiency of the clinical trial process for SMA, paving the way for future FDA registration trials of novel SMA drug candidates, particularly in the SMA Type I population. At FSMA, we have dedicated a significant portion of our legislative strategy over the last five years to achieving NIH commitment to the SMA clinical trial process, and we are excited to see this come to fruition. Moreover, FSMA is proud to be currently collaborating with Dr. Kolb and NINDS on the first ever NeuroNext trial by providing travel funding for patients participating in the study,” said Kenneth Hobby, president of FSMA.

NINDS is conducting this research study because there is strong scientific evidence in animal models of SMA that treatment of SMA may be successful if delivered in the first 3-6 months of age in humans and preferably before symptoms develop. At this time, however, researchers who are developing therapies for SMA find that there is little to no information about how to study infants with SMA during the course of a clinical trial. That is why this study is important. The subjects in this study will teach us how to perform the critical SMA therapeutic clinical trials of the future.

“We are very excited to begin enrollment in this SMA Biomarker study that will focus in on infants with SMA. This work is designed to pave the way towards efficient and successful SMA clinical trials and will inform the design of future clinical trials. We are thrilled to have the support of FSMA who are a critical partner in our efforts to inform the SMA community about this study and have provided essential funding support that will have a positive impact on the quality of data that will be generated by this study. We are also honored that this study is the very first clinical study to occur through the NINDS' NeuroNEXT Clinical Trial Network, and we intend for our study to serve as a positive model for NeuroNEXT studies of the future”, says Stephen Kolb, Protocol PI for the study.

The study will be conducted by the NeuroNEXT Network for Excellence in Neuroscience Clinical Trials (NeuroNEXT) in 15 sites around the United States. Up to 54 volunteers will take part in this research study. Information will be collected from two groups: infants diagnosed with SMA and infants without a neurological disease (we will call this group the control group). All infants will be between 0-6 months of age at the time of enrollment. Parents or guardians of the enrolled infants must sign an informed consent form prior to any study procedure being performed. Other inclusion and exclusion criteria may apply.

For additional information, please contact: Amy.Barlett@osumc.edu or call 1-855-SMA-BIOM (1-855-762-2466).



Participating clinical study sites are:

East

Boston Children's Hospital, Boston, MA

Principal Investigator: Basil T. Darras, MD
 Coordinator: Virginia Trainor, BS
 Email: Virginia.Trainor@childrens.harvard.edu

Children's National Medical Center, Washington, DC

Principal Investigator: Carolina Tesi-Rocha, MD
 Coordinator: Meenal Pathak, MB, BS
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Families of SMA Adds Dr. Elliot J. Androphy to Scientific Advisory Board



Dr. Elliot J. Androphy is Kampen-Norins Professor and Chair of the Department of Dermatology of Indiana University School of Medicine and was formerly Vice Chair of the Dept. of Medicine at UMass Medical School. His laboratory has studied the genetics of SMA,

discovered the role of exon 7 splicing in the SMA back-up gene SMN2, and currently examines axonal transport in motor neurons. Dr. Androphy has been a participant in multiple translational programs including antisense oligonucleotides, tetracyclines, and presently leads a multi-center drug discovery program for SMA that focuses on small molecule SMN enhancers.

Our Scientific Advisory Board (SAB) assesses all research grant applications to ensure that we fund only the best ones. Since 2004, the FSMA SAB has awarded 66 grants for over \$8 Million in funding, including 7 new awards in 2011. These grants have been awarded to 47 different principal investigators at 33 different institutions.

The FSMA SAB also organizes the scientific content of the Annual SMA Research Group Meeting. This is the largest SMA focused scientific conference worldwide, which included over 100 scientific presentations in 2011.

The SAB plays a key role developing the FSMA research strategy. Continued investment in basic research, leading to a greater understanding of the exact nature, causes, and consequences of SMA, is key to ensuring the most effective SMA treatments can be identified and developed as quickly as possible.

Over the last 25 years FSMA basic research funding has contributed to many critical SMA breakthroughs, including the following:

- Mapping and cloning of the SMA gene, SMN1.
- Identification of the SMN protein and its roles in the cell.
- Discovery of the back-up SMA gene, SMN2 that provides a unique and straightforward approach to developing a treatment.
- Development of animal models to better understand SMA disease pathology and to test SMA drug candidates.
- Determination that HDAC inhibitors can enhance SMN2 gene expression.
- Identification of the nucleic acid sequence being used to correct SMN2 splicing by ISIS Pharmaceuticals.

Abstracts Presented on Spinal Muscular Atrophy at the 2012 American Academy of Neurology (AAN) Meeting

The 64th Academy of Neurology (AAN) Meeting was held April 21 to 28, 2012. SMA abstracts were presented to 10,000 clinicians and researchers in attendance. These included presentations by Project Cure SMA on their clinical trial results, by Repligen on the SMA drug candidate RG3039 currently in clinical trials, and by Dr. Charlotte Sumner's group at Johns Hopkins on human pathology of SMA.

Dr. John Kissel, a Project Cure SMA Principle Investigator and member of the FSMA Medical Advisory Council, gave a platform presentation on "A Prospective, Randomized Controlled Trial of Valproic Acid in Ambulant Adults with SMA: The VALIANT Trial", which was funded in its entirety by Families of SMA.

Repligen Corporation gave a presentation on their drug candidate for SMA called RG3039 now in clinical trials. Families of SMA out-licensed RG3039 to Repligen in 2009.

The Comi group from the University of Milan gave a presentation on Stem Cell Therapy for SMA. Families of SMA funded earlier work on this project in 2009.

Please see the titles and authors of the SMA presentations below.

- **Motor Unit Pathology in SMA Patients.** Melissa E. Crowder, Michelle A. Polley, Lingling Kong, James Van Meerbeke, Baltimore, MD, Kelley Murphy, Kathryn J. Swoboda, Salt Lake City, UT, Thomas O. Crawford, Charlotte J. Sumner, Baltimore, MD
- **A Prospective, Randomized Controlled Trial of Valproic Acid in Ambulant Adults with SMA: The VALIANT Trial.** Bakri Elsheikh, Stephen Kolb, Columbus, OH, Wendy King, Worthington, OH, Sharon Chelnick, Columbus, OH, Charles Scott, Fort Washington, PA, Bernard LaSalle, Salt Lake City, UT, Kristin Krosschell, Chicago, IL, Sandra Reyna, Kathryn Swoboda, Salt Lake City, UT, John Kissel, Columbus, OH
- **Falls and Spinal Muscular Atrophy (SMA): Exploring Cause and Prevention.** Jacqueline Montes, Tara McIsaac, Sally Dunaway, Shirir Kamil-Rosenberg, Douglas Sproule, Carol Garber, Darryl De Vivo, Ashwini Rao, New York, NY
- **Gene Corrected Spinal Muscular Atrophy-Induced Pluripotent Stem Cells and Motoneuron as a Model and Cell Source for Transplantation.** Stefania Corti, Monica Nizzardo, Chiara Simone, Marianna Falcone, Martina Nardini, Dario Ronchi, Chiara Donadoni, Sabrina Salani, Giulietta Riboldi, Milan, Italy, Giorgia Menozzi, Clara Bonaglia, Bosisio Parini, Italy, Francesca Magri, Nereo Bresolin, Milan, Italy, Giacomo Comi, Milano, Italy
- **The Therapeutic Effects of RG3039 in Severe Spinal Muscular Atrophy Mice and Normal Human Volunteers.** James P. Van Meerbeke, Baltimore, MD, Rebecca Gibbs, Los Angeles, CA, Heather Plasterer, Waltham, Zihua Feng, Ming-Yi Lin, Los Angeles, CA, Claribel Wee, North Chicago, IL, Bing Xia, Vincent Jacques, James Rusche, Waltham, MA, Chien-Ping Ko, Los Angeles, CA, Charlotte Sumner, Baltimore, MD

Hope for Eliza Willow!

By: Christy Lynn Miller, Aunt of Eliza Willow who has SMA Type I

Photo by Sara Faris

Eliza Willow Smith was born on June 26, 2010, first daughter of the baby of the family, Julia Miller. Eliza Willow came into this world with adorable big cheeks and a cute little mouth. For the first five months of her life, everything appeared to be normal. Looking back there were signs of something being different, but it was not dramatically noticeable. In December 2010, our whole family would get a shock none of us ever could have imagined; Julia would hear words that no parent would ever want to wrap their thoughts around. Eliza had caught the RSV virus and upon the difficulty for her to breathe, she was rushed to Egleston Children's Hospital in Atlanta, Georgia. She had to be intubated to help with her breathing. Doctors noticed the floppy muscles and ran some genetic tests. The results came back as Spinal Muscular Atrophy. They explained the condition to Julia and told her it was Type I according to Eliza's age at the time of diagnosis. Eliza spent months in the hospital on breathing tubes. Every time they would try to remove them, her stats would crash. The doctors then explained to Julia that she should probably make no more attempts to remove them; that she could have a trach put in place, but that they did not advise this and that Julia should make a choice to let her go. They said that with Type I that we may not have her very long. Julia decided that any time that God would give us with Eliza was enough and decided to have the trach installed. A g-tube had been installed in order to feed Eliza early on and due to the SMA causing swallowing issues, this tube would have to stay also.



Eliza was allowed to come home when she stabilized, which was a great day for the family and made Eliza happy herself. A nursing company provided around the clock nurses in the beginning days with the shifts tapering off to so many hours a day. We all had learned to care for Eliza. Eliza began forming her favorite things to do, such as watching *Dora the Explorer* DVDs over and over again, learning to make faces at us, and playing peek-a-boo in her own special way. She was the best baby, so very content and appeared to handle her condition with stride. Before we knew it, it was time for her first birthday. Such a big milestone! She had many people praying for her, physical therapy every Tuesday, a great pediatrician with a positive attitude, and so much love from everyone who met her.

Days and months continued to pass with a few setbacks now and then, but we toughed out a whole year more. On June 26, 2012, Eliza turned 2 years old! She celebrated this milestone with a *Dora Fiesta* birthday party. Eliza has shown that she will fight SMA with all she has. She has continued to show strength and contentment. She will now move muscles on command that she had never moved before. As we sit and patiently wait for results from the clinical trials, Eliza shows us she's a trooper and will prove those doctors from Egleston wrong and so far she has done just that. I am Eliza's Aunt CeCe, Julia's older sister, and I'm a nursing student getting educated by my niece. I have Hope for Eliza Willow because she has hope in herself!

"You Can do Anything You Want, as Long as You Try!"

By: Kate Vogedes, mom to Leah Vogedes, SMA Type II

It all started out with a simple email from my friend, Laura Murphy. "Liam is doing the Pewaukee Kids Triathlon and wants his princess to run it with him". I immediately clicked on the link and read the information about the triathlon. Run, bike and swim; seems easy enough. I replied back, "Let's figure out a way for Leah to do this!". I figured that Leah could do the run/bike part in her power chair. We just needed to figure out the swim part. Our problem was quickly solved by Laura generously offering to carry Leah through the water. After emailing some other families Laura was able to assemble ten of Liam and Leah's friends and Team Mud Dogs was born.

When I broke the news to Leah she was so excited and could not wait to start her training. I read the list of instructions to Leah. She was so worried because all participants needed a bicycle helmet. Fortunately for us the City of Franklin was having its annual bike safety rodeo and was handing out free helmets to all the kids that participated. I signed Leah up for the bicycle safety course figuring that the rules of the road also applied to her. It was a great learning experience for her and she also got her helmet for the triathlon. Then we hit our local trail for some running/power chair driving. Learning how to stay to the right and pay attention to the other people on the trail was all part of Leah's training plan that she created.

The week prior to the race Wisconsin got hit by a heat wave. We had a week of weather that was over 100 degrees (unheard of for us!). We were worried the race would be cancelled due to the heat. God was looking out for us because the night before the race a cold front came through and the morning of the race was perfect, 80 degrees and sunshine. We got to the race at 7:00am for registration. They marked Leah's arms and legs with her bib number and she was ready to go. First they were swimming, then biking and finishing it with the run. The sponsors of the race were very accommodating. They let me park Leah's chair at the front of the bike racks so that we could easily get her strapped in. Laura picked her up and walked down to the water with her and waited for the starting gun to go off. They quickly finished the swim and we got Leah strapped into her chair and put her all important helmet on. Our original plan was for me to run the bike/run portion with her (parents were allowed to join their children if needed). However, once Leah got moving she told me she did not need me to join her. She zoomed off and started the bike race and breezed through the course. For the final run portion we needed to move a gate out of the way because part of the race was on a sidewalk that she could not access. The sponsors let her "run" along side the sidewalk and our problem was quickly solved.

Leah zoomed to the finish line and waited in line for her medal. She was grinning ear to ear and was so happy. While we were in the line another mom came up to me and asked how Leah did the swimming portion. The woman explained to me that her son was handicapped and would love to do a race. They never thought it was possible and the water portion was particularly challenging. She was so happy to hear we figured it out and told me she would see us next year with her son.



It was a great moment when they hung Leah's medal around her neck. I have always tried in every way to include Leah in "normal" kid activities. Leah and her friends were all so excited about the race and it did not faze anyone that she was doing it in a wheelchair. Laura told me she will never forget the day Liam came home and came up with the idea. He told her that Leah can do anything any other kid can do. The quote from his mom that he has heard a million times resonating in his head "you can do anything you want, as long as you TRY." Leah cannot wait to do her next race with her friends. Next up is figuring out how she can do a mud run!



Matt Swinton's Declaration of Independence

By: Beth Grisoli '87, '90 M.A. Beth Grisoli is the director of multimedia services at Notre Dame.

Published: Summer 2012 Posted In: Campus & Community



One day in May, the videographer in the Multimedia Department at Notre Dame affixed a GoPro camera to the wheelchair of Matt Swinton '12, a finance major from Dallas. The GoPro is the Navy Seal of cameras. It's designed to ride on surfboards, motocross helmets and extreme skiers.

Riding a wheelchair across campus may not seem adventurous, but when Matt moved to Notre Dame as a freshman, it was the bungee jump of his life. The camera was a window to campus most of us never peer through — a view from three feet off the ground, moving up and down ramps and through handicapped-accessible doors. In our video about Matt and the campus community that has embraced him the past four years, the University looked the same but felt different.

Doctors diagnosed Matt with Spinal Muscular Atrophy when he was a baby and gave him a year to live. Later, when a company in New England offered his father, Mike, an executive position, he turned it down because young Matt was too fragile to survive in a cold climate. The company arranged for Mike to remain in Texas and commute every week. So Matt grew up in Dallas, visiting doctors, undergoing hours of therapy and going to school.

Mike and DeAnn Swinton now confess that when their son was little, one of their private jokes was about saving for college. When adding a candle to a cake each year is never assumed and tasks as small as using a water fountain don't seem attainable, how could college be realistic? But Matt excelled in school. He was accepted at a number of top-ranked universities. Naturally DeAnn pushed for the ones in warmer climates. She feared the frosty months in South Bend might isolate him because he'd have to stay inside so much.

Spinal Muscular Atrophy affects the voluntary muscles, leaving Matt extraordinarily weak. While he can use his hands, he cannot raise his arms; he cannot roll over so must be turned periodically. Because respiratory muscles are also voluntary, Matt cannot take a full breath, cough or clear his lungs — so any infection becomes life-threatening. South Bend didn't seem like the place for him.

But Matt loved Notre Dame, where he had attended some football games. And he wasn't the only one who persuaded his parents. "Notre Dame did the majority of the convincing," he says. Before he even wheeled onto campus, the University architect's office had devised a plan to renovate a dorm room to meet his needs.

Roommates were one of the aspects of college Matt wanted most. Stand-up comedy is his hobby, and he needed people to critique his jokes. Other schools had offered single rooms and couldn't promise the same residence hall each year. But just off the second-floor lounge of O'Neill Hall, architects created a suite of rooms that connects to a private bath. Matt had one bedroom for himself; his two roommates shared the other. Instant community.

After Matt arrived on campus, the health attendants hired to assist him proved unreliable. Overruling his parents' concerns, he convinced them the students he'd met at Notre Dame were far more capable than the professionals. He circulated an ad in the dorm, and 11 students in O'Neill stepped up that day. So for the past four years, several young men have helped Matt with every daily task imaginable — washing, dressing, compassionately carrying him from wheelchair to sofa to bed. Every night, including weekends, they took turns sleeping on the cot beside his bed and waking four to five times to turn him to keep him comfortable and prevent pressure sores.

Members of this humble group have become Matt's dearest friends. Interviewing them one day, I thought of a priest who once asked me, "When is the last time you saw the face of Christ?" In my head I was shouting, "Right now! Right now!"

Matt found academic support through Notre Dame's Office of Disability Services. It provided note-takers for his classes. The Swintons were amazed at the accessibility of all the buildings. "There's really nowhere on campus I've wanted to go that I haven't been able to," Matt says. Even after the deepest snow, the grounds crews cleared the sidewalks before dawn. The only time he wasn't able to make it to class was when a friend forgot to plug in the charger to his power chair the night before. Matt called the professor and said, "I'll bet you've never heard this excuse for missing a class before. . . ."

At football games, the University assigned him two seats in the handicapped section so a friend could sit with him. At pep rallies, the men of O'Neill would announce their arrival with the giant hall flag attached to Matt's electric wheelchair. After every game, Matt joined the students in the alma mater, swaying his chair side to side. Win or lose, a community.

As Matt's family gathered for commencement, they spoke of the Notre Dame family as something real, not just as a trite metaphor. Countless people had supported and mentored him — Mendoza faculty like Bill Brennan and Carl Ackermann, rector Ed Mack, Scott Howland of Disability Services and roommates like Nathan Albertson, Michael Landron, Eric Vaughn and Manan Dhingra.

Matt is now employed as a financial analyst in Dallas with Sabre Holdings, a global travel-technology company. He says of his ND experience, "Before coming here, I had a very big definition of what independence was. I thought it was doing everything by myself. Notre Dame taught me you can achieve what you want and be independent, but you can ask for help. That's not a bad thing, and there are people willing to help. I learned you don't have to go it alone."



Families of SMA is so thankful to have received a special delivery of handmade positioning pillows for our Newly Diagnosed Care Package Program! These items will be sent to all newly diagnosed families when they first contact FSMA in memory of Jacob Slaymaker. Thank you to the special donor for these wonderful additions to our care packages!



60 beautiful handmade blankets were donated to Families of SMA, from Caroline Kurz Ritter for our Newly Diagnosed Care Package Program! These gorgeous blankets will be put into the Type I Care Packages, in memory of Zane Schmid. Caroline is the owner and designer of Lina Bean and we are all so touched by her thoughts and generosity to help our families.

Families of SMA is ecstatic to be awarded 150 stuffed animal bears from Build-A-Bear as a take home gift for the children who attended the 2012 Annual SMA Conference! Thank you Build-A-Bear for these amazing gifts that helps make the Annual SMA Conference such a success!



Christian Ministry is a senior theology class at Pius XI of Wisconsin. Throughout the course, seniors act as 'Big Buddies' to freshmen by mentoring a group weekly and also take on service opportunities. Each semester the seniors choose a charity to focus their fundraising efforts on and amidst many worthy causes, FSMA was voted on particularly because one of their very own classmates, Jackie Hoffmann, who has SMA. The class shut down the cafeteria on Ash Wednesday to sell only water, Ramen and bread, or beans and a tortilla with all of the proceeds going to FSMA. They also sold 'Cure SMA' bracelets to get the word out to their community. With over \$800 raised, the class then went out to purchase toys and supplies for the care packages FSMA sends to newly diagnosed families.

Thank you for all of your efforts that provided many newly diagnosed families with an extra special gift inside their care package!

Good Afternoon,

I want to say a huge THANK YOU to the families of FSMA. You guys are amazing. I received a wonderful package and I just want to say that I was floored. I could not be more grateful to you all. My son Zane loves all of the toys and generous gifts. I think I cried for an hour. The home made blanket and the sheepskin blanket for him to lie on is wonderful. I am so grateful, thank you so very much. Thank you for everything.

Sincerely

Rebecca, Edward, Alexis and Zane Dick of Franklinville, New Jersey

Families of SMA,

We recently received a care package and literature from Families of SMA and I wanted to say thank you! We loved the gifts for baby Gemma and the literature was very helpful for us to gain knowledge on SMA.

We appreciate the work that you all do from research, to providing info and care packages, etc., and we will be donating to your organization on a regular basis.

Thanks again for everything!
Cindy Eckstein of New Windsor,
New York

Hi FSMA,

I want to thank you for the info packet that I received today, can't wait to educate myself on my niece's newly diagnosed disease. And my sister-in-law, Erica, also wants to thank you, she was overwhelmed with the care package she received. She wasn't expecting all the wonderful gifts that were sent to her! Thank you so much again for everything!

Take Care,

Darlene Amby of Las Vegas, Nevada



Hi Everyone at FSMA,
I can't thank you enough for the wonderful "care" package you sent for Jordan. It was like Christmas all over again. All he could keep saying was "wow, wow". I never expected that much. My son and daughter-in-law were overwhelmed. I'm

so glad I found FSMA on-line. You have been so helpful. I just wanted to let you know that Jordan was so happy. I can't wait for you to meet him. We just love him so much.

Thank you again for everything,
Gail Block of Glendale Heights, Illinois



Families of SMA,

I just want to say thank you for the generous care package! It was all so thoughtful. Our six month old was in the hospital for three weeks battling pneumonia/bronchitis; this was so nice to come home to! We love everything that was included. The handmade gifts and swing were our favorites, and the quilt was gorgeous. Thanks so much for the support and generosity!

Kristen & Brian Jacques of East Taunton, Massachusetts

FSMA,

Oh my goodness, we received the care package today! We are so grateful for everything that was in it! We had no clue he would even receive all of that! We thought maybe he'd get light up toys and soft things! He LOVES, LOVES, LOVES his mobile and the Baby Einstein movie! He cracks up with the feather! We just got a bath with his new float and he loved it. We are going to have a ball tomorrow with everything else (: THANK YOU ALL SO MUCH! You all are AMAZING!

With Love,
Ashley Navitskis of Hedgesville, West Virginia

Dear Families of SMA,

We would like to say thank you for the wonderful care packages that was sent to our house. We really liked the stickers that gave some helpful tips during this confusing time. We also would like to say that everyone at Families of SMA have made us feel very welcome in this new way of life and are glad for all the help we are being given.

Thanks and Love,
The Wicks of Richfield, Wisconsin

Everyone at FSMA,

Jenna received her care package today! It was wonderful and made me feel that there are people out there who really do care. We put up the swing and blew bubbles. She sat in her chair and I didn't have to worry about her falling over. She has played with the animals and loves the finger puppets! She took a nap on her sheepskin blanket. The quilt is beautiful and I will always treasure it! I already started putting papers in the binder; such a good idea. Thank you! You brightened our day and made us feel loved at a time when we really needed it!

The Family of Jenna Eichenlaub of Otway, Ohio

Families of SMA,

On behalf of the whole Rivera family we would like to thank the FSMA equipment pool for Tianna's new wheel chair. Now when we go places, Tianna's not stuck behind a grocery cart or



in a baby stroller and it's her favorite color! Shriners Hospital is going to customize Tianna's new chair. They're putting a tilt on it, new foot rest, a vest for her chest so she doesn't fall forward and sides to keep her spine straight.

Thank you so much,
The Rivera Family of North Port, Florida

FSMA,

Thank you so much for the info packet and the care package. Both were very welcomed, as I'm sure you can under-

stand. And that binder is amazing!

I really can't tell you how much we appreciate an organization like FSMA. We already think it's incredible, and we've only just started.

Erin Johnson of Kansas City, Kansas

Hi Families of Spinal Muscular Atrophy,

Both Karrie and I have received the packages. Thank you so very much. There is so much information; I cannot even begin to tell you how much we appreciate the packages. Karrie was so surprised by the package for Kolette. She has never had that type of support from people who are in her shoes. She does have family who helps, but its different coming from people who are going through or have been through this.

From the bottom of my heart, thank you, especially for sending the amazing package to Karrie for Kolette.

Kelly Unrein of Saginaw, Michigan

Families of SMA,

Thank you so much for the incredible care package and pack of information. We had never heard of SMA until a couple of months ago when our little girl was diagnosed. We were devastated to think she may not have much of a future but now we are filled with so much hope! Thank you for your efforts towards finding a treatment and for your publications. We live in St. Louis and would love to meet other families living with this diagnosis. We want to help raise money and awareness!

The McRoberts Family of St. Louis, Missouri



To all the staff and volunteers at Families of SMA,

Our family would like to take the opportunity to thank you for all your hard work, time and planning that you put into making the conference such a success year after



year. Even though this was our third conference we enjoyed it just as much as our first and second. We continue to learn new information year after year. The conference has become a highlight for our whole family and something that we have made a priority to attend. We cannot express enough how grateful we are for this organization. The time we get to spend with other SMA families is truly priceless and life changing. Thank you for giving us HOPE, LOVE, FAITH and most importantly SUPPORT.

Blessings to you all,
The Tarrence Family
Josh, Kara, Addison & Aspen of Fairfield, Iowa

FSMA Staff,

I thank you for your support of families with SMA. Your organization has been the only bright spot in our journey with my granddaughter Zoey's diagnosis. The care package and information sent to my daughter and her family was awesome! Staff she has talked to have also been very helpful and supportive. Your group has been our main source of information. Thank you again, our family members will proudly wear these bracelets and try to spread the word about SMA and the need for research dollars.

Sylvia Wheeler of Conway, Arizona
Grandmother of Zoey Dillon

To the Families of SMA

Thank you for the support, concise information and generous care package for Josephine. Your kindness and concern has warmed our hearts during this difficult time. We are focused on keeping Josie happy and comfortable while



enjoying our time together as a family during this journey.

With much appreciation,

Josephine, Tommy, Jon and Christine
Lucy of Lee, Massachusetts

Hello FSMA!

Aaron received his care package & car seat today. Thank you all SO much! I cried when I opened the care package. I had no idea there would be so much stuff! SO many wonderful, useful things. We were very pleased! I wanted to also add what a great idea it was to put together that 3 ring binder. Very helpful! I cut out all of the "donated by" labels, & put them in Aarons keepsake box. Such precious little babies.

Have a great weekend!
Nikki, Michael & Aaron Gee of
Dahlonega, Georgia

Dear FSMA,

I just wanted to thank you all again for the amazing stroller! We had the Ottobock rep out to our house today who replaced the recline mechanism that was missing and also adjusted all of the components so it fit Scarlett perfectly. We just had to pay for the new parts! We can't thank you enough for lending us this stroller. It will be great for her for many years to come!



Sincerely,
Rachel & Michael Sepe of Barrington,
Illinois

Hi, FSMA!

Thank you SO much for letting us borrow this chair from FSMA!! I received it yesterday, I opened it up and Cambria couldn't wait to get in it. She cried when I had to take her out.. Just this small thing is making a HUGE difference in her life and I can't thank you enough.



Have a wonderful day and thank you for everything.

Many thanks,
Danielle Mueller of Cypress, California

Thank you to all the staff and volunteers at FSMA!

We have been singing the praises of FSMA and sharing your web site with everyone we know since we received your wonderful care package. There is not a day that has gone by that we have not felt gratitude to FSMA and our delight with your package has been expressed to so many but not directly to YOU all.

Initially, it took us a few days to open the packages because we were reluctant to accept that this diagnosis belonged to us. Slowly the information and care packages were opened, tears came and went, the package and booklets were put away and then a few weeks later I gathered it to be opened again. To our surprise we found so many wonderful and FUN things inside. Did I say the word fun? It's been hard to remember that it is vital to have fun! We have a beautiful and delightful 9 week old baby and we have so much to celebrate and enjoy. We have much to be grateful for and we are starting to find our joy again. Watching Julius wave his feather and shake the cat toys brings delight and laughter to our daily lives, these items are favorites. Rocket is a little blue Beanie Baby bird that we love to fly to Baby Julius while changing his diaper and when Julius is on his ventilator we used the projector to show him images on the ceiling- so many wonderful and

useful items. It is like opening a treasure chest that was specifically designed for us. We used the borrowed car bed to travel to my best friends wedding in Maine and we enjoyed a wonderful 'normal' weekend away that gave us a few fun days full of summer loving with friends and family that we desperately needed. The Binder is a brilliant idea so practical and useful that helped me pull myself together and start filing and organizing the overwhelming amount of paper work we gathered in a few short weeks.

We are very grateful to FSMA for sharing so much balanced information and for being there for us.

Many Thanks,
Denise, Trimbak and Julius Karabinus of Watchung, New Jersey

Families of SMA,

We are so thankful for the information you sent to our family along with the care package and wagon. The support makes us speechless! We wanted to share a few photos of our son, Luke, with his care package items. Thank you all from the bottom of our hearts. We know we will be leaning of Families of SMA in our future as our son was recently diagnosed with SMA and we are learning what this disease is all about and what can and will come of it. As overwhelming as this has been and life changing this has been for our family, we cannot express the gratitude we have for such kindness in our time of despair. THANK YOU FROM THE BERTSCH FAMILY!

Sincerely,
Kelly Bertsch of Whiteland, Indiana



FSMA,

Thank you so much for the care package. Declan looked so excited about his new toys. We already tested out the swing and he loves it. There was so much in the box we were amazed. You guys are awesome.

Thank you again for everything. May we find a cure soon!

From,
The Fink Family, especially Declan of Syracuse, Utah



Thank You!

An extremely grateful thank you to **Brigid Collins**, who has been volunteering some of her summer days to help out the Families of SMA National Office. Brigid is a teacher and also coach's volleyball, and has been incredibly generous to spend part of her summer being a huge help around the office. We are all so happy to have you here and cannot thank you enough for all of your amazing help!

Families of Spinal Muscular Atrophy Thanks Sweet Baby Zane for Providing Car Beds and EZ-On Vests



The Families of SMA Equipment Pool recently received an additional 14 Car Beds and 30 EZ-On Vests to be sent to newly diagnosed SMA Type I families.

Families of SMA is thrilled to receive more generous funding from Keith & Hillary Schmid and Sweet Baby Zane to purchase EZ-On Vests and additional car beds for the FSMA Equipment Pool. These EZ-On Vests and car beds are vital to the well being of SMA Type I infants, who may experience possible apnea and

oxygen desaturation if they are placed in an infant car seat. These vests and car beds enable an infant with special needs to be positioned in the prone or laying down position, safely and comfortably in the car. Once a child outgrows the larger Hope Car Bed, one option is to transport them with the EZ-On Vest. Thanks to Sweet Baby Zane's generous funding, the Families of SMA Equipment pool now has these vests readily available so we can offer families a safe and alternative option when they are ready to return the car bed.

Since the passing of their daughter Zane, Keith and Hillary have been dedicated to bringing awareness to SMA. Along with their supportive community, Keith and Hillary formed Sweet Baby Zane and organized fundraisers in Zane's memory. Sweet Baby Zane raises its funds for Family Support programs at Families of SMA, such as purchasing important equipment for the FSMA Equipment pool. Since Families of SMA is now able to offer every newly diagnosed type I family that contacts Families of SMA a Hope Car Bed, we have seen a great increase in the amount of car beds lent to families. Families of SMA has now been able to purchase a grand total of 92 car beds and 30 EZ-On Vests for the FSMA Equipment Pool!

Thank you Hillary, Keith, Avery, Zane and their adorable latest additions Brennen & Braxton for allowing Families of SMA to provide support to so many newly diagnosed SMA families and for making such an impact on the lives of so many.



FSMA Receives Generous Funding to Purchase Special Bath Chairs for the FSMA Equipment Pool

Families of SMA is incredibly grateful to receive funding from a generous California donor to purchase Leckey Advance Bath Chairs.



These bath chairs are beneficial for children with all types of SMA as it allows them to bathe comfortably and safely while receiving adequate support and positioning for their bodies. Many SMA families have recommended this specific bath chair as it makes bath time a little easier and also their children enjoy taking baths in them.

The Leckey Advance Bath Chair provides postural support while ensuring the individual is safe and secure while bathing. The bath chair can be set up into various positions depending on the individual's needs, and the height can be adjusted to the appropriate height for the parent or caregiver.

The Families of SMA equipment pool consists of many used items that have been donated by other SMA families. The equipment pool also consists of new items that have been purchased with the financial support of others. All items are made available to Families of SMA members free of charge. Families of SMA would like to extend a tremendous thank you to this generous donor for allowing us to provide support to so many families across the United States. This donation will make an impact on the lives of so many.

FSMA Receives Additional Shipment of Wagons from Radio Flyer in Support of the SMA Care Package Program

Radio Flyer, Inc. donates an additional 100 Pathfinder wagons to support the mission of Families of SMA.

Families of SMA is thrilled to announce that we have received our first shipment of wagons donated from Radio Flyer, Inc. to the Newly Diagnosed Care Package Program for the year 2012. These wagons will be sent to all newly diagnosed Type I families when they first contact FSMA.

These Radio Flyer wagons are so beneficial for transporting SMA Type I infants, as it is important to keep these children horizontal. The wagons act as an easy way to transport them around the house, the yard, doctors' offices, hospitals, etc. SMA babies love to be pulled around in their wagons while moving comfortably from one location to another, as opposed to being picked up and carried frequently for feedings or diaper changes.

Families of SMA now offers these Radio Flyer Wagons to all newly diagnosed SMA Type II and SMA Type III children as well. These wagons are wonderful for families to transport their children around the neighborhood without the need for the child's stroller or wheelchair. Hundreds of SMA families across the US have already received their wagon and are enjoying the benefits of these wonderful wagons.

When Antonio Pasin started Radio Flyer in 1917, his dream was to "bring joy to every boy and every girl." Today, the Radio Flyer family continues that tradition by building safe, quality toys that spark imagination and inspire active play. From the original little red wagon to their current wagons, tricycles, scooters, and



other ride-ons, their toys bring smiles to children and families around the world and create warm memories that last a lifetime.

Everyone at Families of SMA would like to thank the Campbell Family and the Tumbleweed Wagon Fund who originally gave Families of SMA the amazing idea to send these wagons to SMA families, in memory of their "Tumbleweed" Braden. Braden's legacy lives on in every newly diagnosed SMA family that receives one of these wagons.

Thank you Radio Flyer, Inc. for making such a difference in the lives of many SMA families!

Families of SMA Awards New Funding to Advance a CNS-Delivered Gene Therapy for Spinal Muscular Atrophy

Families of SMA is pleased to announce the award of up to \$750,000 for an important new grant to Dr. Brian Kaspar at Nationwide Children's Hospital. This award will support preclinical development of a CNS-delivered Gene Therapy for Spinal Muscular Atrophy. With funding from FSMA, Dr. Kaspar's team will initiate the studies needed for an Investigational New Drug (IND) application for this therapy to the Food and Drug Administration (FDA).

"Families of SMA is excited to be awarding new goal-directed drug discovery funding for this gene therapy program. This work follows up on a 2010 grant from FSMA to test the age-dependence in primates of this gene therapy. The new funding will allow us to accomplish several key goals simultaneously", says Jill Jarecki, PhD, FSMA Research Director. "First, it will allow us to advance this very promising new therapy for SMA towards human clinical trials. Second, it will allow FSMA to fund multiple SMA drug programs concurrently, which have different approaches. Doing this will increase our community's chances of successfully finding a treatment for SMA."

"This is extremely important funding from FSMA to allow us to collect additional pre-clinical data for a CNS delivered AAV gene delivery into the cerebrospinal fluid, which will be important information to present to the FDA. It also jump starts research prior to obtaining government and commercial involvement which we are actively pursuing. We are quite hopeful for a positive funding decision on a recent NIH proposal for co-funding of this project with FSMA."

Brian Kaspar, PhD, Associate Professor, Principal Investigator The Research Institute at Nationwide Children's Hospital, The Ohio State University.

The overall project goals are:

- 1) To optimize the dosing regimen for CNS-delivered SMA gene therapy
- 2) To conduct the GLP toxicology, immune response, and bio-distribution experiments required by the FDA
- 3) To prepare and hold a pre-IND meeting with the FDA
- 4) To submit an IND to the FDA to begin human clinical trials
- 5) To produce clinical grade material for human studies

The overall timeline for this work is expected to be three years.

This program was chosen for funding by the FSMA Translational Advisory Committee (TAC), after reviewing multiple potential new drug programs. Every drug program carries risk of encountering hurdles at each of the stages described above. Therefore, a project specific Steering Committee has been put in place, which is comprised of experts in both gene therapy and in SMA biology, with representatives from academia and industry. This committee will help manage the project, ensuring it progresses in an efficient and well-run manner. In addition, project funding will be awarded upon meeting predetermined milestones, decided on by the Steering Committee.



"I am incredibly excited by FSMA's decision to support Dr. Kaspar and his team in this very important project. As a pharmaceutical scientist who works every day in drug discovery and development, I am encouraged by the quality of the science and the fact that it aims to address SMA treatment from a different vantage point from other programs in the SMA drug pipeline. This is only the first step, but it's a critically important step toward assessing whether gene therapy is a viable approach in SMA. Time will tell but I, for one, am incredibly hopeful and look forward to working with FSMA to facilitate the efforts of Dr. Kaspar and his team. I should add that as a parent of an SMA child, I am always looking for a medical breakthrough that could transform the lives of SMA patients."

Timothy P. Reilly, PhD, DABT Director, Drug Safety Evaluation, Bristol-Myers Squibb. TAC Member.

A major goal at FSMA has been to build the SMA drug pipeline, and we have been investing in drug research since 2000 towards this goal. Even with our community's current progress in adding programs to the SMA drug pipeline and advancing programs to start clinical trials, FSMA believes it is critical to do more. Statistics show that only 10% of all drugs initiating human clinical trials ultimately receive FDA approval. The new funding announced here by FSMA for this preclinical drug program will help achieve this goal. FSMA has been involved in funding half of all the ongoing novel drug programs for SMA.

See our website for an entire *Compass* Newsletter on this Project.

FSMA Announces \$700,000 Funding for Novel Drug Program at Newly Created California Institute for Biomedical Research

Steering Committee members include:

- Jerry R. Mendell, MD., Professor of Pediatrics and Neurology Director, Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital.
 - Katherine Klinger, PhD., Sr. Vice President, Genetics and Genomics, Genzyme Corporation.
 - James M. Wilson, MD. PhD., Professor of Pathology and Laboratory Medicine, University of Pennsylvania.
 - Richard S. Finkel, M.D., Professor, Division of Neurology, The Children's Hospital of Philadelphia.
 - Susan T. Iannaccone, MD, FAAN, Jimmy Elizabeth Westcott Distinguished Chair in Pediatric Neurology, UT Southwestern Medical Center.
 - Timothy P. Reilly, PhD, DABT, Director, Drug Safety Evaluation Research & Development, Bristol-Myers Squibb.
- Ex-officio member:
- Barbara C Engel, M.D., Ph.D., Vice Chair, Committees for the Protection of Human Subjects Chair, Institutional Review Board B, The Children's Hospital of Philadelphia.

This new program will focus on optimizing small molecule drug candidates which increase the SMA back-up gene in order to make them ready for human clinical trials. It is well demonstrated that increasing production from the back-up gene can lead to improvement in mouse models of SMA. Small molecule drug candidates have particular advantages: mainly ease of use by oral dosing and the ability to clear the drug quickly from the body. Dr. Peter Schultz, a renowned chemist and successful biotech leader, will be leading this new research effort.



In addition to founding the California Institute for Biomedical Research where the Families of SMA funded project will take place. Dr. Schultz also was Institute Director at the Genomics Institute of the Novartis Research Foundation (GNF) from 1999 to 2010. Dr. Schultz has founded multiple biotech companies, including Affymax Research Institute, Syrrx, Kalypsys, Phenomix, Symyx Technologies, Ilypsa, Ambrx and Wildcat Technologies. Dr. Schultz has published over 500 scientific publications, and received numerous awards for his work including: the Waterman Award of the National Science Foundation, the 1994 Wolf Prize in Chemistry, the 2003 Paul Ehrlich Prize, and the 2005 Arthur C. Cope Award of the American Chemical Society. He is a member of the National Academy of Sciences and the Institute of Medicine.

"Dr. Schultz is greatly respected in the research community, both as an academic scientist and in the biotech industry. He has successfully led many drug programs that continued on to clinical development", said Jill Jarecki, Ph.D., FSMA Research Director. "FSMA is very pleased to have a chemist and drug developer of Dr. Schultz's experience spearheading a program for Spinal Muscular Atrophy. We are also excited that Spinal Muscular Atrophy is one of the initial drug programs at the newly formed California Institute for Biomedical Research."

This new institute will focus on innovative new approaches to unmet medical needs, including neglected and rare diseases. Their expertise will be in translating basic research discoveries from bench to bedside. Therefore, this group will be ideally poised to move newly identified SMN enhancing compounds through all the preclinical steps of drug development towards first-in-human clinical trials.

"I am pleased to be collaborating with Families of SMA on a new small molecule drug discovery project for SMA. CALIBR will provide a unique opportunity to move therapies for rare diseases towards the clinic, due to our pre-clinical drug discovery infrastructure, coupled with our focused ability to move projects rapidly through early pre-clinical stages." Dr. Peter Schultz, Institute Director, CALIBR.

Please see our website for an entire Compass Newsletter on this project.

About California Institute for Biomedical Research

In early March 2012, Merck announced that it will invest \$90 million the California Institute for Biomedical Research (CALIBR, www.calibr.org), a private, nonprofit center to be led by Peter Schultz, a renowned chemist at The Scripps Research Institute. The goal of the new institute is to bridge the gap to promote translational research and early drug development. The roughly 25,000-square-foot research center will be equipped for programs in immunology, autoimmune and metabolic disorders, cardiovascular disease, regenerative medicine, cancer biology and neurodegenerative disease.

This Program was chosen for funding by the FSMA Translational Advisory Committee (TAC), after reviewing multiple potential new drug programs.

SMA and a Marine Corps Family

In 2010, I chased down a dream. At that time, I had no idea that this dream would serve as steadfast support in my greatest time of need and provide the resounding answer to my prayers.

First, let me introduce myself. My name is Stephanie Geraghty and I'm a proud Marine Corps wife. My husband Gunnery Sergeant Brian Geraghty has served his country for 14 years, deployed five times, and in my opinion is just about the best the Marine Corps has to offer! We feel called to this service as a family and are proud to serve our country. We have two wonderful boys, Caden (5 ½) and Cole (3), and a baby girl due to arrive on the Marine Corps birthday, November 10th. Our family is blessed in countless ways.

However, there's no doubt that our military life poses extra challenges. On any given day, a military family endures relentless changes, deployments, moves, and losses. And we take on those challenges without immediate family nearby. Our support network instead relies on fellow military families at each duty station.

Upon moving to Camp Lejeune, NC, in 2009, we had just given birth to Cole and Brian was slated to deploy within eight months. I needed to establish that all-important support network quickly in order to cope with his absence. As a long-time runner and athlete, I dreamed of finding a running club for Moms. I found nothing. I decided it was my time to take action and make my dream a reality.

Thus, I created Stroller Warriors™, a free running club that helps alleviate stress for military spouses. We meet several times weekly and complete running workouts as a team, supporting each other in training for races and meeting personal goals. We embrace runners of all ability levels. This club provides a wealth of camaraderie, resources, and mutual support. We welcome the inclusion of children and strive to strengthen family ties. Stroller Warriors also provides a means to give back to our surrounding military community through local outreach and fundraising projects.

Without a doubt, this club is more than just a running club. It's a massive team of women who can achieve anything together. In two-and-a-half years, the club encompasses seven chapters worldwide, 1200 members, and continues to grow daily. They are a powerful force to be reckoned with. Together, we laugh, we cry, we overcome, and we RUN.

This past spring, these inspiring military wives at Camp Lejeune decided to give back to the community by supporting my family. Several club members voiced interest in organizing a fundraiser in honor of our son Cole and his newly diagnosed disorder, Spinal Muscular Atrophy Type II/III. They wanted to



help and they felt the best way was to race in his honor, spread awareness, and raise money to support research for a cure.

Stroller Warriors selected Families of SMA as their charity of choice. I was thrilled. Families of SMA had supported us since day one of the diagnosis, sending us friendly messages, mailing us a packet of information, and giving Cole a wonderful box of toys and gifts. We were grateful for their help and anxious to give back in return.

The eager racers signed up for the Wrightsville Beach Half Marathon in Wilmington, NC. While vigorously training for this 13.1-mile race, they expended time and energy advertising the cause and generating donations. The amazing fundraiser coordinator, Elizabeth Harlow, set an ambitious goal and displayed confidence that they would not only meet but exceed that goal. Two other generous club members, Amanda Zimmerman and Rebecca Smith, designed, sold, and distributed t-shirts to support and advertise the event and bond the team.

Donations and shirt orders rolled in. Elizabeth's prediction was spot on. When the race was over, donations far exceeded any expectations! Not only had Elizabeth completed her first half marathon, she had led a team of women in supporting a wonderful cause. What an incredible accomplishment.

That race meant so much to me because I felt like I had finally legitimately done something to make a difference. The past year had not been easy and I often felt helpless. We received Cole's diagnosis while Brian was deployed to Afghanistan. One of the hardest moments of my life was relaying over the phone that his



son had an incurable genetic disease. In the days following, I shed many tears as I tried to process the news, without my husband to hug and hold. We needed each other and did our best to communicate. But we realized we were lucky to have a solid network of family and friends reaching out and helping us cope. And the day of the race, I felt that support tenfold.

I will admit that I also felt tired! I was still in the early stages of my third pregnancy and had low energy. Every step of that race felt heavy, slow, and labored. But then I thought about Cole's challenges and how he would love to be able to walk, let alone run. Despite the difficulty, he keeps trying. So I can too. I finished with flying colors even faster than my goal. I shed tears that day too but they were tears of happiness. I had completed many half marathons in the past but none of them came close to the triumph I felt that day. Those Stroller Warriors, now half marathoners, uplifted our spirits and gave my family renewed hope.

Even a year later, there is not a day that passes that I don't wish I could take away Cole's disease and relieve his extra challenges. Cole takes it all in stride though. He keeps smiling, wrestling with his brother, whizzing around on his wheelchair, and enduring whatever therapy we throw his way. In essence, he makes it easy on all of us. At only three years of age, he serves as my personal hero and inspiration. I am so incredibly proud of him and I know he will lead a long successful life, doing some truly great things. He will make a difference; he already has.

So now, I pause and reflect back on that simple dream. When I created Stroller Warriors in 2010, I never could have guessed that those amazing women would serve as steadfast support during a deployment and the surprise diagnosis, and provide the resounding answer to my prayers.

In conclusion, thank you to everyone that has helped us on this journey, especially our parents, our family, our friends, the Stroller Warriors, and Families of SMA. We will prevail and I know in my heart there will be a cure. It's a dream right now just like Stroller Warriors was in the beginning. But dreams do come true. My goal is to race and raise money for Families of SMA every year in honor of Cole and all the other families affected. I have no doubt there will be Stroller Warriors at my side.

I encourage you to find your own way to advocate research for the cure. Perhaps you could even join forces with the military wives of Stroller Warriors! Feel free to contact me if you'd like more information at strollerwarrior@gmail.com. As Military Spouse Magazine's 2012 Marine Corps Spouse of the Year, I aim to bring Stroller Warriors to every major military installation around the world. Perhaps then they can help other special needs families just like they helped us.

**HAPPY RUNNING,
The Geraghty Family
Stephanie, Brian, Caden, Cole, and Baby 3G**



Morgan Steward



Mia Israel



**Peyton Elsner, Leah Vogedes
and Arianna Martin**

Stephen Covey's 'The Seven Habits of Highly Effective People' Applied to Special Need Parents

By: Chantai Snellgrove from Parenting Special Needs Magazine

1. Be proactive

Covey's first habit is one you could apply to almost any successful leader. But the people who practice it have also been explained as those who "recognize that they are 'response-able.'" Former Secretary of Defense Robert Gates was one of those leaders. In an interview with Katie Couric during the waning days of his tenure, he said the most difficult thing about his job was that "everything that I've wanted to do to try and help the men and women in the field I've had to do outside the normal Pentagon bureaucracy."

This should speak volumes to parents with special needs children. Be proactive; recognize that you are "response-able" to help make a better life for you child. It is most definite that you will probably have to do things outside of the normal.

Translation: think outside the box.

2. Begin with the end in mind

Covey's second habit "is based on imagination," his Web site says, "or the ability to envision in your mind what you cannot at present see with your eyes." Steve Jobs' ability to imagine great, world-changing products — and then see every detail through to the end — made him one of the world's most effective innovators.

What kind of life do you envision for your child as an adult? Imagine them going to college, living independently, having a social life. Remember they are adults a lot longer than they are children. By using Job's ability, as an example, imagine great, world changing...and then see every detail through to the end.

3. Put first things first

The third habit reminds leaders that "to live a more balanced existence, you have to recognize that not doing everything that comes along is okay." It's about not only time management, but life management and setting priorities about the "day to day" and the long term. Sheryl Sandberg may have helped bring bottom-line focus to Facebook's "hacker way," but she's also unafraid to say that she leaves the office each day at 5:30 p.m.

Life management... it is okay to not do everything that comes along. Set priorities for the "day to day" and long term, so you can live a more balanced life.

4. Think win-win

The phrase "win-win" may be ubiquitous today, but it's likely popularity came from Covey's fourth habit, which means "agreements or solutions are mutually beneficial and satisfying." Most skilled negotiators and successful diplomats are good practitioners of the "win-win" habit, the most recent of whom is Secretary of State Hillary Rodham Clinton. As Susan Glasser, the editor-in-chief of Foreign Policy (who wrote the definitive recent profile of Clinton), told NPR, the message she sends is that "not only is America back in the world, but we're willing to be partners. This is not unilateral cowboy diplomacy anymore."

Think "Win-Win" every time you are negotiating. Which can be almost daily when advocating on your child's behalf. Keep the "win-win" habit in mind when working towards solutions or agreements that will be both mutually beneficial and satisfying.

5. Seek first to understand, then to be understood

Covey's fifth habit encourages good listening, which requires the intent to understand, not just respond. Former Procter & Gamble chief executive A.G. Lafley turned the consumer products giant into a listening machine, observing customers in their homes, championing the design process and bringing in external ideas to help make more consumer-focused products.

Seek first to understand, then to be understood. This habit can be used in all areas of our children's lives. We need to become good listeners to our children, which may require more intent observation of their behaviors, schedules and activities in order to understand and respond appropriately, not just respond! This habit encourages helping our children to be understood better in their daily lives.

6. Synergize

Covey may have called the sixth habit "synergize," but he could have just said effective people are good at bringing together groups of people and celebrating their diverse ideas. Whatever one may think of Duke University basketball coach Mike Krzyzewski, he's been able to build winning teams of all-stars at both the college and Olympic level, no matter what egos are involved.

"Synergize" assemble and build a winning and diverse team of all-stars. Choose, not only, the right doctors, therapists, teachers, aides, tutors, and coaches, but also, family and friends that we be effective in helping your child reach their potential.

7. Sharpen the saw

The final thing highly effective people do, Covey says, is to preserve and enhance "the greatest asset you have — you" and find ways "for self-renewal in the four areas of your life: physical, social/emotional, mental, and spiritual." In other words, they're well-rounded, balanced leaders. The manager of the world's biggest bond fund, PIMCO's Bill Gross, regularly finds ways to disconnect from communication and practices yoga on a daily basis. ("Some of my best ideas literally come from standing on my head," he told Fortune in 2006.)

Sharpen the saw... you are your child's best advocate. You must find ways to preserve, enhance and renew yourself on a daily basis. Without you where will your child be?

Please visit www.ParentingSpecialNeeds.org to view this on-line magazine.

Congratulations

- Congratulations to Michelle and James Fox, and big sister Malorie, on the birth of their new baby, Jacob, who was born July 30th, 2012!



- Congratulations to Brook and Jake Pritchett, on the birth of their twins!

- Congratulations to the Gellner family of West Fargo, North Dakota, on the birth of their new baby girl Olive Louise, born May 12, 2012!

- Congratulations Paula Lavigne and Chris Arnold from Nebraska on the birth of their twins Paxton and Penelope!

- Congratulations to Myles, Sarah and big sister, Stella Bartlett on their new baby boy, Oliver!

- Congratulations to Chad Wilges and Brandy Grant on the birth of their new baby!

- Congratulations Jim and Aleisha Weiberg, and big sister Journee, from Illinois, on the birth of their new baby boy, Jace!



- Congratulations to Cassie Stech and Britten Gilbertson, and big brothers Gavin and Caden, on the birth of their new addition, Mason Jo!

- Congratulations to Alana and Justin Coates on their new baby boy, Spencer, who was born on August 4th!

- Congratulations to Peter Rider and Glen Hill on their newest addition!



- Congratulations to the Grimes Family of Bowling Green, Kentucky on the birth of their beautiful twin girls, Lainey and Emily!



Congratulations to Emma Lockwood on being named Little Miss Wheelchair of Ohio!



The New England FSMA Chapter is delighted to announce that the winner of the 2012 Ms. Wheelchair Massachusetts contest is their very own Patti Panzarino! Patti gave a very eloquent and thoughtful speech about Creative Perseverance. She spoke about the influence of creativity in her life and the perseverance required to attain her goals related to creativity. Patti is an accomplished singer/songwriter and expressed her desire to use her platform of "Creative Perseverance" to reach other women with disabilities to achieve their dreams. Thank you Patti for your inspiration!

Congratulations to Max Brown of Livingston, NJ, who has SMA Type II, for receiving an award for service from the Livingston Advisory Committee for Disabilities (LACD) and for also being on the winning team of the 2012 Microsoft Firenze BXT competition. Max is attending Carnegie Mellow University School and Design and is studying graphic design.

Congratulations to Katie Norton of Ponte Verde Beach, FL, who has SMA Type II and who was one of 588 students worldwide to receive a perfect 36 on her ACT. Katie was also elected homecoming queen in her last year as a senior at Ponte Verda High School. She plans on attending college at the University of North Florida this fall.



Congratulations to Josh Garrison of Kingwood, TX, who has SMA Type II, on being a 2012 graduate of Kingwood Park High School! Josh plans on attending the University of Houston and majoring in accounting and finance.



Congratulations the Desroches family on the birth of their new baby girl, Ella Grace, who was born on August 16, 2012!

August is SMA Awareness Month

Families of Spinal Muscular Atrophy has been coordinating a National Awareness Month for SMA since 1996. Raising awareness of SMA in the general public can help lead to increased resources for SMA research and better care for SMA patients. The majority of people, including doctors, nurses and community members, do not know about SMA until it directly affects them.

One of the highlights of SMA Awareness month is the SMA Candle Lighting. The Annual SMA Candle Lighting was held on Saturday, August 11th. Many families and SMA organizations around the country participated by lighting a candle at sunset to remember those SMA Angels who have lost their battle with SMA and to honor those SMA Warriors who are still here fighting everyday! Here are the great pictures that were posted to the Families of SMA Facebook page!







- We love you and miss you Ashley Marie Christianson 4-21-90 -9-16-93
- A candle for our first born, the other angels, and the fighters. Sayre Matthew 12.16.05-12.27.05 Type 0



- Angel & Joe remembering Ashley!
- CANDLES for SMA honors all of the SMA Angels and those who live with SMA
- Candles for the babies who lost their battle with SMA. Mommy loves and misses you so much Brayden Lukas!



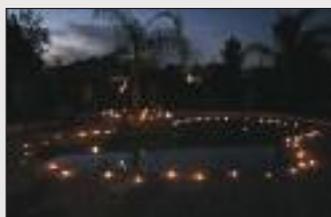
- For Bella and all the SMA Angels and Warriors
- For Buttercup.
- For Ella, her fellow warriors, and the angels who have gone before her.
- For every star that crossed my life and that live in my heart.



- For Logan and all others with SMARD or SMA
- For Lydia and for all the SMA Angels and Warriors
- For our little warrior, Kadence Lee and all angels before her and all warriors with and after her!
- For our niece, who earned her wings too early in life
- Honoring My angel Sir Amir Blanks August 19, 2011 - September 29, 2011
- I lit my candle for my beautiful niece Aubrey who got her angel wings April 24th 2011.



- In honor of my niece Sierra Pearl
- In honor of our sister, Scarlett Sepe, and all SMA families. Love, Isabella & Gavin Sepe
- In honor of our SMA Angels Cody and Bethany Irvine.
- In memory of "Our Lovely Angels" Jessica & Jaylin Gayle
- In Memory of Jocelyn Lee (4-22-2007- 8-14-2011)



- In memory of THEO JUNIOR BERRY 25.9.11 - 30.1.12 SMA TYPE 1
- These are our candle photos from Charleston, SC for baby Telaai Wiberg. Thank you for organizing this tribute!

- In memory of those who have passed away, and in honor of Kendra Scalia. We LOVE you! xo



- Keeping the flame lit for everyone...

- Louisa Mae - Dec 24, 2011-May 19, 2012 We will always love you!

- Lucy, with her candle for SMA

- Madison Reed SMA Type I during our 2012 candle lighting honoring all SMA Warriors and So Many Angels.

- My sweet angel Alex. Every minute spent with him was truly a gift! Always missing you Boog! 10-30-92--3-5-08



- Olivia and I lit our candle and said a prayer for all the SMA Warriors and for all the SMA Angels in Heaven!

- Our baby is new with SMA but still fighting the fight.



- Our candle for our beautiful grandson, Brayden Lukas Caddell, October 10, 2011 - January 7, 2012

- Our Candle Shines Bright!

- We hope for a cure. Sadie 3.5 years old diagnosed in Feb with SMA Type III.



- We lit our candle in honor of our son Michael. 10-9-08- 9-13-10



- Scarlette is lighting her candle for all the brave children and families fighting against SMA.

- Sending Lantern's over the Bay to pay tribute to all the SMA warriors and angles!

- SMA candle lightening in memory of my nephew baby stephen, love you forever

- TEAM ELIZA! is still fighting and waiting on a cure...candles for her and for the angels that went on ahead!!!



- The Butler family with our candle for Andy at the Greater Florida chapter's lantern lighting event.'



- The candles are lit in front of our house again this year for all the Angels

- The is a candle lit for the SMA angels and SMA warriors Mariah is SMA Type III she is on the left hand side

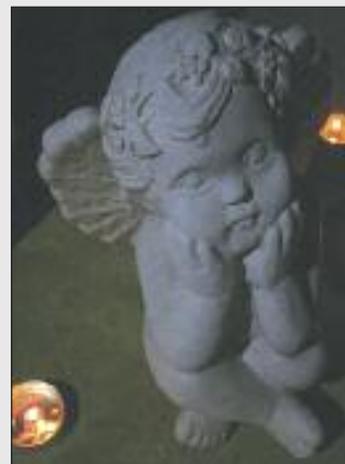
- The Stewart Family lighting candle in honor of Brandon Stewart.

- These are pics of Alli Williams and siblings lighting candles for their brother RJ and all the other angels and warriors.



SMA AWARENESS - SMA CANDLE LIGHTING





Decision Making About PGD Is Complex, Study Finds

Reprinted with permission of the Muscular Dystrophy Association, by Margaret Wahl on May 11, 2012 - 3:59pm

A study of 22 couples considering preimplantation genetic diagnosis (PGD) has revealed a four-phase decision-making process

Article Highlights:

Preimplantation genetic diagnosis (PGD) is a procedure used by prospective parents who are trying to prevent a disease-causing genetic mutation from being passed on to their offspring. The procedure can be physically, psychologically and financially stressful.

A study of couples considering PGD found there were four phases in their decision-making process: Identify, Contemplate, Resolve and Engage.

A University of Illinois at Chicago website provides a video by researcher Patricia Hershberger.

Decision making about *preimplantation genetic diagnosis* (PGD) is a complex, multiphase process for couples, a new study has found. Understanding it, the investigators say, may be helpful to prospective parents who know they're at risk for transmitting a genetic disorder, and to the professionals who advise them.

PGD is a procedure used by prospective parents who are trying to prevent a disease-causing genetic mutation from being passed on to their offspring. Couples first go through in vitro fertilization (IVF), in which their sperm and egg cells are brought together in a laboratory dish. PGD then requires removing a cell from an IVF-produced embryo and testing it for the specific genetic mutation. An embryo that doesn't show the mutation can be implanted in a woman's uterus.

IVF-PGD procedures can be physically, psychologically and financially stressful.

Beginning in 2009, principal investigator Patricia Hershberger at the University of Illinois at Chicago and her colleagues conducted in-depth interviews of 22 couples (44 individuals) considering PGD, interviewing each person separately.

Participants knew they were at risk for transmitting a genetic disorder to their offspring, including: Duchenne muscular dystrophy (DMD), Becker muscular dystrophy (BMD), facioscapulohumeral muscular dystrophy (FSHD), myotonic muscular dystrophy (MMD), unspecified muscular dystrophy, Charcot-Marie-Tooth disease (CMT), spinal muscular atrophy (SMA), and others.

Four phases in decision making

Hershberger and colleagues, who published their findings in the May 2012 issue of *Social Science & Medicine*, found that couples go through four phases in their decision-making process. The investigators called these phases Identify, Contemplate, Resolve and Engage.

The Identify phase is characterized by learning more about the genetic disorder for which the couple is at risk and more details about their at-risk status.

During the Contemplate phase, people explore a range of reproductive options, including using a sperm or egg donor, adopting a baby and using PGD. Some stayed in this phase for as long as three years.

In the Resolve phase, people either accept PGD, decline it or "oscillate" (go back and forth) about the procedure. Nine of the 22 couples in this study ultimately accepted PGD, four declined it, and nine remained "oscillators" at the end of the study period.

The final phase — Engage — is a time when people carry out their PGD decisions, such as beginning the hormonal treatments required to start IVF, or moving down a different path, such as going ahead with natural conception or adoption.

"What is striking and universal about the couples in this study is the genuine care and concern they expressed for their future child(ren) and their understanding of the profound significance of their decision — regardless of their decision type," the investigators say in their May 2012 paper.

More studies planned

The authors conclude their published study by saying, "There is a burgeoning number of couples who will face decisions about whether to use PGD; research in this area is critical to aiding modern couples, clinicians and policymakers as we navigate the continuing technological advances of the 21st century."

Future studies are being planned. More information can be found at PGD: Couples' Decision Making at the Genetic and Reproductive Interface, a University of Illinois at Chicago College of Nursing website about Patricia Hershberger's PGD research, featuring a video by Hershberger.

Learn more

For more information about preimplantation genetic diagnosis, here are some helpful resources:

- The Decision-Making Process of Genetically At-Risk Couples Considering Preimplantation Genetic Diagnosis: Initial Findings from a Grounded Theory Study, a summary of a paper published in the May 2012 issue of *Social Science & Medicine* (complete paper available with subscription)
- Love Letters and Preimplantation Genetic Diagnosis, a couple affected by facioscapulohumeral MD tries PGD, *Quest* magazine, April 2011
- Genetics: You, Me and PGD, a description of the study featured in this article, *Quest News Online*, July 23, 2009
- The Pain and Promise of Prenatal and Newborn Genetic Diagnosis, an exploration of the issues raised by new diagnostic technologies, *Quest* magazine, July 2007

Loving Memories

This section is designed so it can be removed from the center of the newsletter.



Photo of: Tonya Willingham and her daughter, Hanna

IN MEMORIAM

Malik Attila Cosby

May 25, 2000 – July 27, 2011



Malik's Story

On May 25, 2000 we became the proud parents of a beautiful baby boy named Malik Attila Cosby. Malik was about six months old when we began to notice that he was not reaching milestones and had stopped trying to lift himself up while laying on his stomach. We brought this up to his pediatrician and she referred us to Howard University's Child Development Center. We went there and were asked what felt like a million questions about our family medical history and during this time it came out that my mother had a daughter Shawn in April of 1974 with Werdnig-Hoffman Disease (SMA Type I). Shawn only lived to be five months old and they did not know a lot about SMA then and told my mother that she and her husband had bad genes and were not compatible. When I was born five years later in 1979, my mother was very concerned and had numerous tests run on me to find out if I was healthy.

This information alarmed the doctor's at Howard University and we were told to visit a neurologist at Children's National Medical Center and a genetist at Howard University. We met with Dr. Diana Escolar and explained everything to her. Malik underwent an electrostomogram and DNA test. Dr. Escolar explained to us that it was very evident that I was a carrier of the SMA gene and I received it

from my mother, but the only way Malik could have SMA is if his father is carrier as well. On May 15, 2001, we received the phone call that the test came back positive that Malik had Spinal Muscular Atrophy Type II. So his father and I are both carriers of the SMA gene or trait.

Malik lived a wonderful life and was able to accomplish all of his dreams and wishes. Malik spent his first Christmas in Hawaii and he traveled on a cruise to Jamaica. At three years old Malik wanted to ride a bike, so we had a bike specially adapted so that he could ride one. At five years old Malik wanted to meet Shaquille O'Neal and dunk on him, so through the Make-A-Wish Foundation Malik was able to meet Shaq and spend the day with him. At eleven years old Malik wanted to visit Disney World in Florida so my mother and I packed him up and all of his equipment and took Malik to Disney World for the Families of SMA conference in Orlando, Florida.

At the Families of SMA conference, I felt a lot better about SMA, I felt a sense of hope that my child would beat this SMA disease. Even though I have been attending conferences since 2001, this one just felt different for me. Malik was doing so well and he was able to meet and talk to adults with SMA Type II. Malik had a ball with his friends especially his best friend, Jerika Bolen from Appleton,

Wisconsin. We had not seen Jerika in a couple of years but they played and talked all day for the entire trip.

Malik suffered from scoliosis and had a rod placed along his spine when he was five years old. This surgery helped Malik tremendously with his lung development. Before he had this surgery he was prone to pneumonias regularly. After he received this surgery he rarely had pneumonia. Malik often spoke of not having any more surgeries, he was growing tired of these surgeries and we were beginning to talk with doctors about fusing his spine.

On July 25, 2011, Malik had his rod extension surgery as scheduled and did an excellent job. He came off of the ventilator like a champ and was breathing on his own. We stayed at the hospital for about four hours and were discharged home later that evening. Malik was happy to be home in his bed with his family and dog, Krypto.

On July 26, 2011, Malik had a normal day with his nurse Mr. Patrick and did not ask for any pain medicine, that whole day. I will not discuss the moments that led up to his death because I am not comfortable retelling them but he suffered a respiratory distress and was rushed to the hospital.



IN MEMORIAM

Jay Randall Marlow

January 25 - August 25, 2008



On July 27, 2011 at 1:35 a.m. Malik was gone to heaven.

While Malik's death hurt me tremendously, I knew my baby was free. I have always been afraid of death, going to funerals and being around dead bodies, but seeing my child that I gave birth to lay their put a sense of calmness to me. I touched him and he was still my baby, I kissed him and he was still warm. I was there to see him take his first breathe and I was there to hold his hand when he took his last breathe. Malik was finally free from all the hate in the world and most importantly free from Spinal Muscular Atrophy.

After his death I had my moments when I cried, but I did very little crying. Cry for what? My child is an Angel in heaven. When we went to see his body at the funeral home everyone was crying, but I did not shed a single tear. He looked so peaceful and free, my Superhero. He just looked different to me in a good way. He had a wonderful life and he brought so much joy and life to those he met. He taught me about SMA and many others. Now it is my mission to keep his legacy alive and raise awareness about SMA.

Malik will always be my baby and my SuperHero. I know he is watching over me everyday and telling me to keep my head up and be strong. He is walking around in heaven with all of his family and SMA friends.

Families of SMA opened up so many doors for me and my family. We have met so many families whom we are still close to. Families of SMA taught me so much about SMA, how to adapt toys for Malik, get the right type of equipment and the right questions to ask his doctors.

Lisa Couser of Bowie, Maryland.

August 25, 2012 marks the fourth anniversary that our first son, Jay Randall Marlow, lost his battle with SMA. Though Jay will forever be in our hearts, it's dates like these that bring tears, smiles, anger, and sometimes, relief. Relief knowing that Jay is whole and free of SMA; angry with the fact that such innocence had to struggle with a cruel and terminal disease; tears and smiles for the treasured memories made with the sweetest of souls.

We planted three butterfly bushes around our deck to draw butterflies - the same type of butterfly that helped carry our son's soul to heaven. After Jay passed away, we took him outside to enjoy the sun, the breeze and the closeness that we didn't get to feel during his last month with us because of how fragile he was. Just as we sat down with Jay, a beautiful monarch butterfly fluttered above his head, and then flew away. My heart knows that it was Jay coming to say one final goodbye and to let us know he was ok.

As the butterflies come to visit the blooms each summer, we are reminded of Jay, his final day with his family, his strength, his courage and the unending happiness that he brought to our lives. Though seven months will never be long enough, we gave a lifetime of love to each other that will never be forgotten.

I will miss being able to watch the Olympics with you again this summer, Jay. I know you had your fill of swimming, track and field and beach volleyball, but thank you for putting up with your sports fanatic mommy.

We love and miss you, sweet boy. We hope that when you see a monarch butterfly, that you will think of Jay as we do.

**With much love,
Lee, Marla, Emi and William Marlow
Highlands Ranch, Colorado**





IN MEMORIAM
Danielle Vaz

In memory of my granddaughter, Danielle Vaz, who had SMA Type I. She was with us for five and half years and now has her angel wings. God made the decision to give Danielle to us. God made the stars, trees and the moon and you, Danielle, that's why you were so special. He made you in a very special way; your eyes so they could twinkle, your mouth so you could smile and laugh. God made you like no one else. Since you were so special, he put you in just the right house where you would be warm when it was cold, safe when you were afraid, and where you could have fun and learn about heaven. So after looking for just the right family God sent you to us. God sent us Danielle; very wonderful for us to take care of. As the days, weeks and months have gone by somethings will stay the same. I will always love you. I will always hug you. I just want you to know that just in case you ever wondered. God loves you, protects you and you are now one of his angels. Heaven is a wonderful place, no tears there. Your angel wings will let you dance, sing, run and be happy. Now in God's arms you will never be hungry, get cold, be sick or afraid. You will be missed here on Earth always but in heaven you are close to God he will always be there to hug you.

Love forever,

Nana June Vaz of Wesley Chapel, Florida



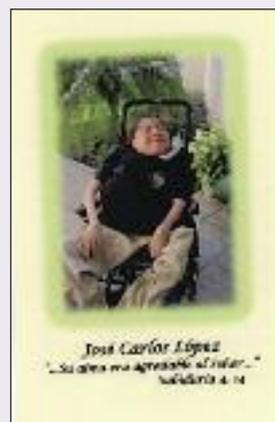
IN MEMORIAM
DANIKA MARIE DEITERING
NOVEMBER 16, 2011 –
JULY 14, 2012

I'll Be There

Daddy, please don't look so sad,
mama please don't cry
cause I am in the arms of Jesus
and He sings me lullabies.
Please, try not to question God,
don't think He is unkind
don't think He sent me to you,
and then He changed his mind.
You see, I am a special child,
and I'm needed up above
I'm the special gift you gave Him,
the product of your love.
I'll always be there with you
and watch the sky at night.
Find the brightest star that's gleaming,
that's my halo's brilliant light.
You'll see me in the morning frost,
that mists your window pane.
That's me in the summer showers,
I'll be dancing in the rain.
When you feel a little breeze,
from a gentle wind that blows
that's me I'll be there,
planting a kiss on your nose.
When you see a child playing,
and your heart feels a little tug,
that's me I'll be there giving your
heart a hug.
So Daddy, please don't look so sad,
Mama don't you cry.
I'm in the arms of Jesus and He sings
me lullabies.

From your Little Angel

IN MEMORIAM
José Carlos López



Jose Carlos was really born on Friday June 3rd of 2011 at 21 years of age. His burial was celebrated with the savior in a glorious manifestation of glory and hope. His life, while marked with a severe condition, was a life of joy, lived with intense passion. He made everyone around him happy and he fought with tenacity to obtain his academic goals. His finest realization in his personal life was to believe in his personal life loving our father Jesus and adoring the holy Eucharist. He now inhabits the light of eternal happiness.

We appreciate infinitely our father Jesus in allowing the great gift of his life entering ours and ask that he watch over him for all who love him on earth.

In memory of our beloved Jose Carlos.

With affection,
Ivonne and Anthony of Puerto Rico



IN MEMORIAM
HAYDEN BEESON
FEBRUARY 21, 2012 – JUNE 12, 2012



IN MEMORIAM

Elizabeth (Libby) Chapin

AUGUST 5, 1955 – JANUARY 8, 2012

A fighter and passionate soul living with SMA

By: Lisa Iannamico (caregiver for 18 years) & Meg Etherington (sister)

Leaving for college is a daunting prospect for most freshmen. As a normal teen, this would be a huge transition in your life from parental guidance and control to self disciplined independent, responsible young adult. If you were leaving home for the first time, knowing that nearly every aspect of your life - from bathing to meals, even going to the bathroom - was dependent upon another person, would you go? Libby Chapin did, at age 18. Libby moved from central New Jersey to Connecticut to attend Central Connecticut State University. There, she lived as independently as she could, as a member of a disabled community in a hospital. When she eventually transferred to Hunter College, in New York City, Libby lived in an apartment; there, she relied on family for most of her personal care.

After earning her bachelor's degree in Egyptology, Libby set her sights on doctorate programs and eventually decided on the University of Pennsylvania. She settled into an apartment with a full time caregiver in West Philadelphia and began her studies. Unfortunately, after breaking her leg and hip (and thus being unable to attend classes) Libby decided to discontinue her goal of becoming an Egyptologist. Relearning hieroglyphs was a huge concern, as was the eventual prospect of participating in excavations. The logistics of attempting such a trip to an inhospitable climate was a daunting prospect in and of itself for someone with respiratory issues.

Her academic career on hold for the moment, Libby embarked on an illustrious new chapter of her life, more akin to that of a Hollywood starlet than a young woman of 'limited' ability. She met a few guys who introduced her to Philadelphia's

club scene and threw parties that would shake the foundations of her sleepy Rittenhouse Square building and it's other, more sedate occupants. Libby soon tired of the party scene, though, and decided to return to academia. This time, she elected to study Interior Design at Drexel University, a course of study in which she was able to bring a unique and creative perspective to Universal Design, producing designs that were both beautiful and accessible.

When her father passed away and her elderly mother moved to Philadelphia, Libby's career in design stalled. She created a beautiful design for her mother's apartment, many more for her own, and often gave advice to friends and family on the design of their own homes, Ultimately, though overseeing her mother's care - as well as her own - became Libby's full time job.

Her 'full time job' didn't deter Libby from having fun, though. Not the club scene - she was past that - but fun nonetheless. A supporter of the local arts, Libby held season tickets to various theaters over the years. She enjoyed all Philadelphia's vibrant theater scene has to offer, from touring performances of Broadway hits to unique, independent productions. She adored the ballet and as a child had day-dreamed of being a dancer. She attended performances by the Philadelphia Ballet Company and tried to foster a love for dance in the many young people in her life. She loved going to museums, in particular the Philadelphia Museum of Art and the University of Pennsylvania Museum of Archaeology and Anthropology, to which she had memberships.

Libby also enjoyed traveling, from day trips to NYC for Broadway shows or to tour the

Statue of Liberty, to traveling to sunny islands for a warm getaway. She visited Bermuda, Puerto Rico, St. Thomas & St. John. She even went skiing in the Poconos! What began as an idea to go snow tubing ended up with Libby swooshing down the slopes. On her recent trip to St. Thomas, Libby was able to experience something she hadn't felt in over 20 years: the warm ocean water and the relaxing effect of the waves. It was thrilling for her, so much so that she wanted to return the following spring to do it all again.

Not all of Libby's fun times were huge endeavors. She loved going to matinee to see the latest Hollywood thriller or cheesy romantic comedy and eat a bagful of popcorn. Some of her favorite days were spent with the children in her life just playing and laughing or taking the kids to water parks & amusement parks.

Libby lived much longer than her doctors could ever imagine. She was diagnosed at age 2 and her parents were told she would only live into her teens. Fortunately, Libby's parents were proactive with her therapy. She traveled often from central NJ to NYC and to Baltimore to receive care from the top doctors in the field in the late '50s and early '60s.

As her sister and full-time caregiver, we honestly believe the daily physical and hydro therapy, combined with Libby's positive outlook and her pure joy for life, are what enabled her to far surpass that early prognosis. Instead of accepting her 'fate' and succumbing in her teens, Libby led a rich, full life. She passed away last month at the age of 56, leaving a void in many lives that will never be filled. We are lucky enough to have many great memories and we will always have her in our hearts.

IN MEMORIAM

Jessica Lovely Angel Gayle

February 2, 2000-February 25, 2001



IN MEMORIAM

Jaylin Chante Gayle

January 27, 2001-September 11, 2002

Our Little Angel

She became our little Angel
 The day that she was born,
 She's in a better place now
 So we shouldn't be so torn.
 We're all going to miss her
 Because we love her like crazy,
 Sweet, soft and gentle
 As pretty as a daisy.
 She's like a beautiful spring flower
 Grown in the month of May,
 Our little Guardian Angel
 That watches us all day,
 Whenever we need help
 And when were in trouble
 She'll come down in her wings and halo
 Looking oh so lovable.
 Right now she's having fun
 Walking, talking and laughing out loud,
 One day we will be with her
 Till then she'll wait up in the clouds.
 In our hearts forever
 She will always remain so true,
 Jessica Lovely Angel Gayle
 We will always love you!

By: Cousin Francine Lopez

We Will Always Love You!

It was hard to look into those angelic eyes,
 and see how you suffered so,
 But God took his hand and closed your eyes
 and whispered, "Its time to go".
 No more hurt and no more pain,
 nothing but days filled with laughter.
 Now you'll play with sister in heaven above,
 and live happily ever after.
 I enjoyed when we played patty cake,
 I'll remember your playful smile,
 I can't wait till the day we all meet again,
 even if it may take a while.
 I now have two guardian angels,
 to keep me safe at times.
 Two beautiful little girls
 who are special and are always on my mind.
 Although we will miss you very much
 it's still hard to let you go,
 But just remember those times we've shared together
 and always remember, we love you so!

By: Cousin Francine Lopez

My name is Jessica and this is my sister, Jaylin, on the right. I was in the process of being tested for SMA around my first birthday. It was hard for me to sit up, I couldn't crawl or walk, but I loved being held by my mom. I liked when my mom and grandma would sing to me. My sister, Jaylin, had the same symptoms as me when she was born and was diagnosed with SMA Type I. We were able to get toys from SMA to help exercise her muscles. SMA also mailed information about this genetic disease to my mom and helped her meet other mothers that were going through the same thing.

Thank you SMA for the support you have given to our mommy, Nina, our sister Janae, and my two brothers; Sante, and Angel. The SMA organization means so much to my aunties, Bud and Grandma! You have educated our whole family about this genetic disease, and now my mom, aunties, Bud and Grandma raise money every year to help other families!

IN MEMORIAM

Dennis K. Morris

May 5, 1953 – March 15, 2012



Our HERO

Grandpa Dennis you are a true HERO... We will forever miss you and can't wait to see you again in Heaven.

Dennis K Morris, Ford's Grandpa, was a kind and gentle soul. He was very humble and I know that he is looking down on us and wishing that we were not giving him the attention he deserves. Dennis put his faith above all else and instilled this value in his family. His family meant the world to him and his love was unconditional. He sacrificed so much every day to help those in need whether it be family, friends, neighbors or even people he didn't know. He did this without ever expecting something in return; he just enjoyed helping those in need.

Ford's Grandpa knew how to do everything and he taught Ford's daddy everything he knows. Ford's dad is the man he is today because of his father. Ford's Grandpa enjoyed life and he had many interests and hobbies. He loved to fish, hunt, weld, garden, play guitar and saxophone. He loved logging, making breads and jerky, taking photographs, working on cars, flying air planes and even enjoyed bee keeping. What he loved most however was preaching and missions work. He shared the gospel of Jesus Christ all over the world, from the Dominican Republic to Ecuador, and on a daily basis. His life shone with the love of our Lord and Savior.

In the eyes of Ford's family, Grandpa Dennis was a Hero; someone who unselfishly, without even thinking would give up everything, even his own life for something or someone. Ford's grandpa lived up to that honor on March 15,, 2012 when he gave his own life to save the life of his grandson Ford. My husband was home alone when a fire broke out at our

home. He was able to get Ford out of the house but Ford cannot be left alone due to having a genetic degenerative disease called Spinal Muscular Atrophy Type I. Ford uses a cough assist to help him cough because he is unable to do it on his own. Ford needs suction constantly because he is unable to control his own secretions.

Ford's grandpa heroically ran into our house to save all of Ford's medical equipment. Grandpa Dennis did not stop until he retrieved every piece of life saving equipment Ford uses on a daily basis. Ford's Grandpa died saving Ford's life and we could never thank him enough. I know he wouldn't even want us to thank him and I know that he would say that he would do the same again. I hope that he knows how truly thankful we are for his sacrifice and unconditional love towards his family and grandson.

Ford's Grandpa loved him so much. He couldn't wait to get cleaned up from whatever he was doing to come to our house to see Ford. He loved singing songs with Ford and rubbing his legs. He held Ford's hands above his ear so Ford could feel it. Ford loves to feel his ear because it makes him laugh all the time. Grandpa loved to watch Ford move in his slings and swim in his hot tub. I know that Grandpa Dennis wanted to teach Ford so many things and I know he will get the chance when we get to Heaven.

Ford's Grandpa taught our family so much. He taught us what it meant to love unconditionally and to show the love of Christ to those around us. Grandpa Dennis prayed for us and prayed with us. He was a man who built your faith up when you felt like all hope had left you. His faith was displayed in his actions and he asked God daily to heal our son of SMA. Dennis's faith was unwavering and

he demonstrated this faith as he was praying before he took his last breath. I know he was asking God to continue to watch over and protect his family and I bet he was still asking God to heal Ford's body.

I hope that it only seems like a moment in time before we are with him again in Heaven. Our family does find comfort in knowing that Grandpa is where he wants to be. I can picture him sitting at the throne of God worshipping him for all of his days. "Better is one day in your courts, better is one day in your house, better is one day in your courts then a thousand elsewhere". We can't wait to see you again and thanks for being our HERO.



Dear Grandpa,

I wanted to tell you thank you for loving me the way you did while you were here with me. Thank you for being such a wonderful Grandpa. Thank you for looking at me like I was perfect even though my body is so fragile. Thank you for praying for me and asking God to heal me. Thank you for playing with me and singing songs with me on my little boom box. Thank you for teaching me what it means to love. I know there were many other things that you wanted to teach me as a young boy. Wait for me in Heaven so when I get there you can teach me all the things we didn't get to do. I am glad I got to be your grandson and I will miss you forever. Thank you for saving my life, you are my Hero.

Love,
Ford

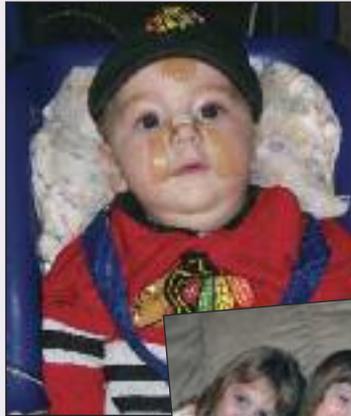
IN MEMORIAM

JOSPEH GERARD SAVINO

September 17, 2010 – May 19, 2011

Joseph was born on September 17, 2010. We were so happy to welcome another child into our lives. We were so surprised to hear from the doctor that we had a baby boy! Our family was now complete with three other sisters; Anna, Kara and Marisa. The doctor had told us he was a healthy 7 lbs. 3 ounce, 21 ½ in. long boy. When we followed up with the pediatrician after bringing him home from the hospital, everything was good and even at his two month check up everything checked out fine. I nursed and formula fed him, but found out that he was still hungry. So at nine weeks old, I started him with some rice cereal off the spoon. He would sit in his bouncy eating his food. Then in January, at his four month check up, it was then we were told that something wasn't right. The doctor put Joseph on his stomach and he wouldn't lift his head. The doctor said that maybe he was a little delayed and if I wanted we could wait and see if he makes any changes in the next month. We opted not to wait. Later that day my husband and I along with Joseph went to see a pediatric neurologist. He explained that Joseph does show some delay in his movements. Although he did have movements in his arms, legs and head, it wasn't where he should have been at his four month milestone. His movements were much stronger when we first brought him home from the hospital. The neurologist said his muscle tone was extremely low (hypotonia). We had blood tests done on him, which all came out normal. Then we were told to go see a genetic counselor. She too had told us that Joseph had low muscle tone and explained to us a little about Spinal Muscular Atrophy. Extra blood tests were done, and while waiting for these results my husband and I did some research. I kept telling my husband this can't be, there is no family history of SMA. Then weeks later the phone call came in, giving us the horrible news that our son did have SMA Type I.

In February, 2010 before Valentine's Day, Joseph was admitted to the hospital with a collapsed lung. During that time they put a NG tube in for his feedings, but we decided a G-tube would be better for him. His lung started functioning again at 80% so after two weeks we were able to bring our little boy back home to his sisters. We all treasured the time that we had with him. He would watch his sisters dance to Taylor Swift, and if he wasn't sitting with them at meal times, he sure would let us know. We had him home with us for three weeks along with a home healthcare nurse checking in on him every other day. One of those days the nurse did not like the way he was breathing, I bundled him up and rushed him to the doctor. As I waited to see the doctor his temperature was normal. Within 30 minutes his temperature spiked to 101 degrees. The doctor finally admitted him, again with a collapsed left lung. We were in the hospital for a week when the doctor suggested to go home. I



refused to bring our little Joseph home, because his lung was not functioning how it should have been. A week went by but his lung wasn't getting any better, until one day Joseph just wasn't himself. A fever came on quick, and the nursing team could not find a vein to draw blood. After being rushed to ICU where they stabilized him with a bipap mask; my husband and I made a quick decision with the help from another SMA mother to be transferred down to Children's Memorial Hospital in Chicago. He was brought to the Intensive Care Unit, where he spent another eight weeks of his life with a bipap mask. He had many treatments done by using the curass machine, IVP, and Cough

Assist to keep his lungs open and working. He did well after the first four weeks. He finally got to the point where he was only using the bipap at naps and bedtime.

During his hospital stay he had a few episodes where mucus plugs would get in his way of breathing. Then it was bipap mask back on 24/7. His sisters would come and visit him weekly at the hospital which brought a smile to his face! After a few weeks he had gotten better. We were finally ready to be discharged and again looking forward to going home to be with his sisters! Just two days before leaving the hospital, Joseph had another episode. This time it was much worse. His lung had collapsed once again. His heart rate was very high, and he was working harder to breath. He was struggling more and had to have more oxygen than usual. Our little boy had other plans; his plan was to be an angel. On May 19, 2011 at 12:01am, as my husband laid on one side of him and I on the other, telling him it was okay to go, it was then that he earned his "angel wings."

Joseph was only eight months old, but in those months he had touched so many lives. Although he was limited in his movements, he sure made up for it with his big brown eyes, his beautiful smile from cheek to cheek, and his long eyelashes that would melt everyone's hearts.

We have no idea why Joseph was taken from us so early, but we enjoyed what little time God gave us with him. There is no doubt that Joseph is our guardian angel who watches over us from Heaven. The pain still hurts, but we know that he is in a better place and free of this horrible disease. He is now crawling, walking and discovering that he can eat on his own. He will be in our "Hearts Forever!"

We love you!

**Audrey, John, Anna, Kara, and Marisa Savino
of Chicago, Illinois**

IN MEMORIAM

Alana Marie Bodenhamer

August 17, 2011 – April 25, 2012



This is my daughter, Alana Marie Bodenhamer. She was born August 17, 2011 (SMA awareness month!) weighing a healthy 8lbs. 8oz. She started developing problems when she was about six weeks old. It took the neurologists until April 13, 2012 to be able to give us a diagnoses; Spinal Muscular Atrophy. She had been tested when she was three months old, but all the tests came back negative, so the neurology team thought she might have had something else wrong with her. When treatment for the "other" diseases didn't seem to work, the neurology staff decided to have a test ran to look at every gene in her body. The test took two months; the result - Alana had a rare form of SMA, which only happens in about 3% of SMA cases. Alana had not one, but two mutations/deletions of her SMN1 genes. She passed away on April 25, 2012.

Since the passing of our beautiful angel, our family has been committed to raising awareness about this disorder and doing what we can to raise money for SMA.

Sincerely,
Michelle Senne



IN MEMORIAM
GAVIN HARVEY

SEPTEMBER 26, 2007 – MARCH 27, 2008



IN MEMORIAM
GABRIEL RODRIGUEZ

APRIL 21, 2011 – JUNE 18, 2011



IN MEMORIAM
HARRISON ELIAS WHITE

JUNE 17, 2010 – JULY 10, 2011



IN MEMORIAM
MYKA SCOTT

June 2, 2010 – February 3, 2011

IN MEMORIAM

Maggie Sue Kazlauskas

July 3, 2011 – June 11, 2012



I have dreamed about having a daughter like Maggie all of my life. If you ask anyone who knows me they will tell you that I have always wanted to be a mommy. Being a mommy to Maggie these past 11 months was the best experience of my life. Maggie brought me so much joy in everything that she did. When Maggie was first born I would just sit and stare at her, studying everything about her beautiful face. She was always full of smiles and from the moment we met we had a special bond. Maggie was always in my lap or right by my side, we were inseparable. I never felt alone as long as I had Maggie with me. When Maggie was diagnosed with SMA Type I at five months old I didn't know how I was going to continue living with knowing what I did about this horrible disease. I didn't understand how such a beautiful baby could possibly have anything wrong with her. The only way I made it through was having Maggie there with me and knowing that God was going to be with us no matter what happened. It was impossible to be sad with such a sweet and innocent baby depending on me, and eventually I tried to forget what the doctors had warned me was to come. We had such great times playing together. One of Maggie's favorite things was having daddy sneak up and tickle her, as he rubbed his scruffy face on her cheeks she would scream with delight. She would sit on my lap and stare over at her dad with so much love in her eyes. I remember tickling her with my hair and Maggie running her fingers through it. Maggie loved it when I would play with her legs. She also loved her bath time and swimming together in the hot tub. When Maggie turned seven months old we started having birthday parties for her. I knew that Maggie wasn't going to get enough birthday parties in her life and I wanted to celebrate each month with her. Maggie loved her balloons and she felt so strong tugging on the strings moving her huge Minnie mouse balloons in amazement. It was a very important day for us when Maggie was dedicated to Jesus; Maggie's aunt Missie sewed her dedication dress from my wedding dress. I knew that Maggie would not get the chance to wear it in the



future and it meant a lot to me when she was able to wear it when she was dedicated. Maggie also loved to look at herself in the reflection of her mirror. I think that she knew how beautiful she was. Maggie had the biggest blue eyes I have ever seen; when she looked at me it melted my heart because I felt so much love for her. On memorial weekend we went camping and Maggie sat right next to her dad in her high chair. We had such a great time the first two nights but on the third night she started getting sick so we had to leave early and come home. Maggie got really sick soon

after that but seemed to be getting better. I couldn't let myself believe that I could possibly lose her, she meant the world to me. So when Maggie passed away Monday morning, June 11, 2012 I was shocked and devastated. The last thing Maggie saw was her mommy and daddy comforting her; she was so brave when she took her last breath. I love Maggie with all of my heart and it is going to be torture living here without her for the rest of my life. The only thing that comforts me now is knowing that someday Maggie will open her eyes again and the first thing she is going to see is her mommy and daddy, and the only reason we can hope for that is because God sent his son Jesus to die on the cross for our sins so that someday we won't know pain and suffering. So goodbye my baby girl I will miss you and I will continue to pray with you every night our prayer, "Now I lay me down to sleep I pray the Lord my soul to keep." Goodbye my Maggie Sue, my Magpie, my pie. I will love you forever and you will always be in my heart and my memories!

A heartfelt thank you for the use of the car bed and special tomato seat. Our Maggie Sue was a true blessing and we had hopes for her to live long enough for a cure. We will continue to support Families of SMA so that another family may benefit from research.

Gods Blessing,
Kazlauskas Family of Dowagiac, Michigan

Maggie Sue Kazlauskas



They say you had SMA, Maggie
 And you would die, so very young
 But a cure might be on the way, Maggie
 To that hope we so desperately clung.

Now the time has come and you've gone away, Maggie
 Your sweet smile a memory sung
 In my heart you will always be there, Maggie
 Forever beautiful, and forever young.

Your eyes were as blue as the sky, Maggie
 Their beauty, was second to none
 Your smile was as warm as the sun, Maggie
 A beautiful gift, for everyone.

Now the time has come and you've gone away, Maggie
 Your sweet smile a memory sung
 In my heart you will always be there, Maggie
 Forever beautiful, and forever young.

We prayed for a miracle, from God, Maggie
 That on earth, his will would be done
 The miracle that he worked, changed our hearts, Maggie
 To prepare us for the return of His Son.

Now the time has come and you've gone away, Maggie
 Your sweet smile a memory sung
 In my heart you will always be there, Maggie
 Forever beautiful, and forever young.

On the great resurrection day, Maggie
 When God takes his children home, beyond the sun
 I'll be there is my prayer every day, Maggie
 Where you will laugh, you will play, and you will run.

IN MEMORIAM
ALISON MARIE WICK

FEBRUARY 14, 2012 – JULY 16, 2012



IN MEMORIAM
BLAKE NORWOOD
MARCH 1, 2012 – MARCH 30, 2012





IN MEMORIAM
KENNADY QUINNELL
JUNE 15, 2010 - MAY 28, 2011



IN MEMORIAM
TONY PRUITT JR
NOVEMBER 7, 2007 - JUNE 15, 2008



IN MEMORIAM
MADISON WHITE
September 30, 2011 – May 6, 2012



IN MEMORIAM
MOLLY LOUISE KENNEDY



IN MEMORIAM
JONATHAN CHARLES FINLAY
January 14, 2010 - March 19, 2010



IN MEMORIAM
JOSEPHINE OLIVIA VILLARREAL



IN MEMORIAM
Alexandra Meigs
 October 24, 2007 –
 January 1, 2008

Dear Alexandra (aka Mommy's Muffin-Stuffin):

You are such a big girl today! Happy 5th birthday in heaven! Know that we are here on earth celebrating your special day with balloons, cake, ice cream, gifts, music, dancing and singing. You were/are our Angel.

We hope you are breaking open that piñata full of candy and getting ready for Halloween. I can only guess your costume this year.

We miss you, Alexandra. Your little sisters are almost 2 years old and, while they are still learning how to talk, they do know you and often point to your pictures around the house, saying "Sissy!! Sissy!! Hmm? Sissy!" They love you and know you. And, your big sister, Avery (7), still draws pictures of you in her beautiful artwork. So, you are all around us.

Happy birthday, big girl. Mommy and daddy send baskets of hugs, kisses, tickles, squeezes and all the love that we can possibly send in our birthday/care package to heaven.

We will see you soon – maybe in our dreams, maybe thru a butterfly or dragonfly, maybe thru some other image but, certainly, one day in heaven.

Love, **Mommy & Daddy & Your sisters (Avery, Isabella and Calla) & Maribel**



Dear **Alexandra,**

We loved everything about you. Your eyes appeared "all-knowing" to me. It was as if

God knew too well the impact your presence would have on us. Your eyes looked as though you knew as well.

God designed your every moment here with us and we must conclude – HE...IS GOD. Only He can sift us through feelings of awe, glee, confusion, wrenching pain and sorrow to finally resolve a state of awesome love... His love.

We thank God for experiencing your arrival, your time on earth and the pain of His retrieving you. Your smile, your love – special gifts., We shall never forget you, Sweetpea.

Always,
Your Grandma and Grandpa Nelson

To our little Angel Alexandra:

Happy Birthday! Today you will be 5. We know you will have the best birthday with Jesus up in Heaven. So blow out your candles and may all your wishes come true. We know our only wish is to spend every birthday with you.

We love you, PaPa and Nana

Dear Alexandra,

My Angel flying high.
 I'm so happy it's your 5th birthday.

Celebrating a birthday in
 Heaven must be awesome.

I hope the Angels sing out loud - "Happy birthday to you, Happy birthday to you..."

As you blow out the candles on your cake.
 Make a wish to visit us tonight.

Happy Birthday!

With lots of Love, Hugs and Kisses,
Tia and Uncle Alvin

Dearest Alexandra,

"The best and most beautiful things in the world cannot be seen or even touched. They must be felt with the heart."

—**Helen Keller**

Happy 5th Birthday sweet girl! You have left an imprint in our hearts and lives, and we feel your presence around us.

We love and miss you, XOXO,
The Tsao Family

Twinkle, twinkle, little star,
 How I wonder what you are.
 Up above the world so high,
 Like a diamond in the sky.
 Twinkle, twinkle, little star,
 How I wonder what you are!

Dearest Alexandra - Happy Birthday - thank you for being the bright light that continues to guide our hearts and our thoughts from above. We love you - **Miki**

Dear Alexandra,

I hope you know how many people's lives that you have touched. You are loved and missed by so many - most of whom never had the pleasure of meeting you in person. You have an amazing family who honor your memory in so many ways. You will live on in all of our hearts - because they had the strength, dedication, and courage to share their love for you. Remembering you on your 5th birthday, sweet girl.

With love,
Sari (Jacob's mommy)

Dearest Alexandra,

It's so hard to believe that it has been five years since God gifted us with His beautiful cherub. We love you so much and not a day goes by that we don't feel your presence, sense the warmth of your smile, or see your beautiful face. Happy 5th Birthday, Sweetheart! We look forward to your birthday celebration on October 24th!

Grammy and Gramps

"I Carry Your Heart"

-*EE Cummings*

I carry your heart with me (I carry it in my heart) I am never without it (anywhere I go you go, my dear; and whatever is done by only me is your doing, my darling)

I fear no fate (for you are my fate, my sweet) I want no world (for beautiful you are my world, my true) and it's you are whatever a moon has always meant and whatever a sun will always sing is you here is the deepest secret nobody knows (here is the root of the root and the bud of the bud and the sky of the sky of a tree called life; which grows higher than soul can hope or mind can hide)

and this is the wonder that's keeping the stars apart

I carry your heart (I carry it in my heart)

Alexandra, you are with us. When ever we struggle and feel a little beaten down the thought of you comes like an angel and reminds us of the preciousness of life. You remind us to enjoy and find peace in life. Thank you angel, for bringing the true message of God to our hearts.

Love, Team Johnson

Katie



I had a sister named Katie,
She was my friend.
I miss her everyday,
We were always together.

Now I am lonely,
Now she is in heaven.

She didn't like being at school,
She always made her alarm beep.
She liked the color pink,
And thought she was a princess!

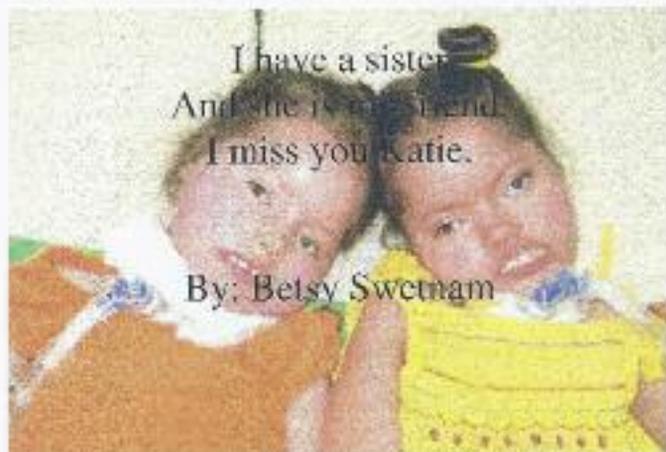


I wish she were here for field trips and plays,
Christmas was hard
And she missed all the games!

I wish she were here to go to high school with me,
I wish she were here to see all the boys!

Now she's in heaven dancing around,
Singing loud in her princess gown.

Now I can speak,
My eye gaze gives me freedom.
My future is full of brand new adventures!

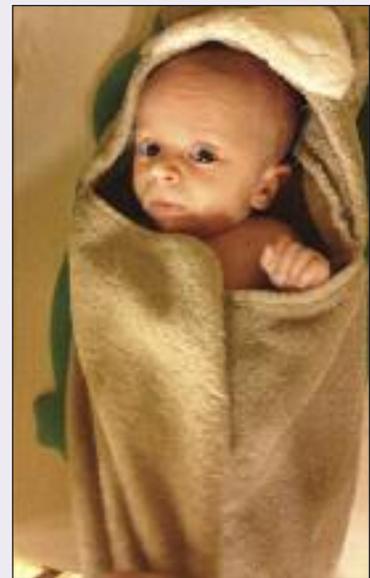


I have a sister
And she is my friend
I miss you Katie.

By: Betsy Swetnam



IN MEMORIAM
AARON TYSKA GEE
FEBRUARY 11, 2012 - APRIL 4, 2012



IN MEMORIAM
ALEXANDER ANTONIO VELAZQUEZ
MARCH 21, 2012 - JULY 13, 2012



IN MEMORIAM
AIDEN JAMES NAVITSKIS
DECEMBER 2, 2011 - APRIL 26, 2012

IN MEMORIAM
GRACE MACKENZIE O'NEILL
 DECEMBER 17, 2010 – JUNE 7, 2012

Grace, or “Gracie” as she’s known to her “Groupies”, lived life through expressions of determination, perseverance, and love. In the last days of her life, Grace was in the hospital with an upper respiratory infection. After a month and a unsuccessful extubation attempt, Gracie was determined to get home. In true form to her spirit she defied what doctors had predicted—an unsuccessful and possibly fatal attempt at extubation. Grace against odds, pulled through, and returned to her Bi-Pap. No one was to tell her when, where or how her life would end. Grace came home four days after her extubation. She made it back to her home, her favorite place surrounded by her family. Back to the life she knew and loved. Singing songs, playing games on her i-pad, and of course watching Mickey Mouse Clubhouse. Grace was enjoying family dinner around the table that night. Just as Grandma gave her praise how wonderful she was doing, “You go Gracie, 98% oxygen. You sure showed them how strong you are” and in that next moment Grace’s lungs collapsed and her heart slowly stopped. In our racing efforts to save her in our home emergency room, Gracie’s spirit, the one we all grew to love and adored knew it was time. She showed everyone how strong she was and she could persevere, after all, SHE MADE IT HOME! In the comfort of her home, family and Mickey Mouse, she passed away on her and God terms, not ours.

Gracie’s day to day life consisted of talking, playing with her toys, singing and dancing with her little sister Eileen and she even attended school. We celebrated monthly birthday parties, each with its own theme. She went places such as



Disney World (twice), our family’s cottage, and Bronners (an all year Christmas store full of decorations her eyes couldn’t believe).

It wasn’t until Grace had passed we realized what a true angel on earth she is. Her soft touch, warm presence and deep soulful eyes truly radiated “angel”. To be near her was simply “graceful”. As parents we believe we’re the ones who teach our children life lessons and help mold them into adulthood. For us, our child came here to teach us, along with many others, about life, love, and courage. She shaped us into the people we are today. At her funeral her Uncle Matt said it best, “Grace has taught us many things,

1. There is no such thing as too much Mickey.
2. You can have an entire conversation with your eyebrows
3. You don’t have to be big to be strong
4. If you don’t want to talk to someone, just close your eyes
5. Even when a prisoner of your own body, you can still be in control of your life
6. You can say, “I love you.” Without saying a word
7. Cherish every moment you have.

When we asked our family and friends to tell us what Grace meant to them, the most common answer was how she inspired everyone, with her courage, and strength. She found a place in everyone’s heart and will always be remembered. Her story challenged people’s inhibitions. She was the catalyst for others (even some in the medical field) to learn about SMA and spread awareness. She inspired others to live each day to its fullest.

Most importantly, she taught us all love. She didn’t love us because we chased her around, fed her yummy treats, and gave her all the best toys available. She loved us simply because we were there next to her. To hold her, lie next to her or watch Mickey Mouse with her, her eyes lit with love and peace. Grace granted us the peace to know that even though we couldn’t cure SMA, we meant the world to her. She was our angel; she asked for nothing and gave everything in return. For as many tears that have fallen for Grace, as many smiles will accompany them as we all remember Grace’s beautiful smile.

Grace may have only been here on earth for 17 short months but she did live a full life, to us. She actually lived more in those 17 months than those who live “a full lifetime”.

You will never find two prouder parents. Our daughter was a warrior and now is an angel. For that we stand strong and full of love. Grace Mackenzie is our daughter.

Fly high, run, play, and DANCE!
 DANCE! DANCE! We love you Gracie!

“Grace must find expression in life, otherwise it is not grace”

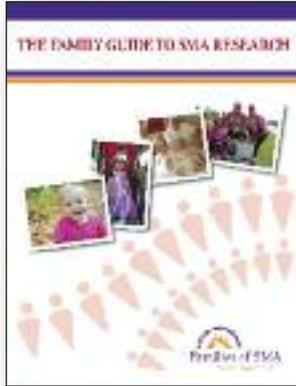
–Karl Barth

Families of SMA offers free informational booklets called *SMA Care Series* to all families, friends and professionals.

These booklets are available on a variety of specific topics such as Genetics and Diagnosis, Respiratory Care, Nutrition and many more. SMA Care Series Booklets are now also now available in Spanish, as well as on our website for download.

Families of SMA is proud to offer all of the SMA Care Series booklets translated in Spanish.

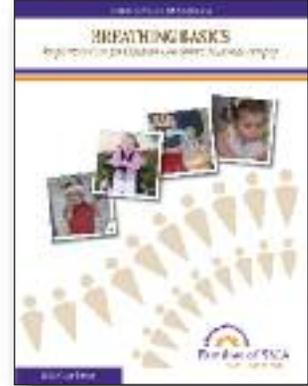
Family Guide to Research



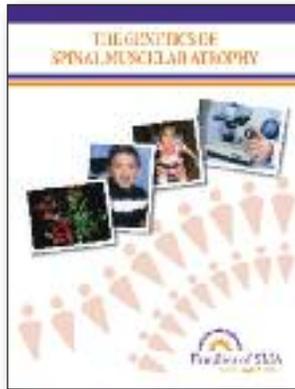
Families of SMA Family Support and Patient Services



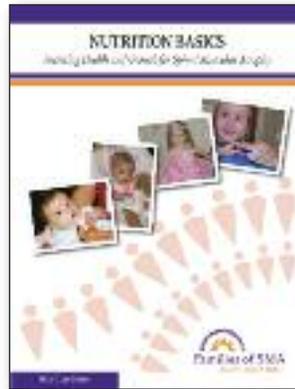
Breathing Basics



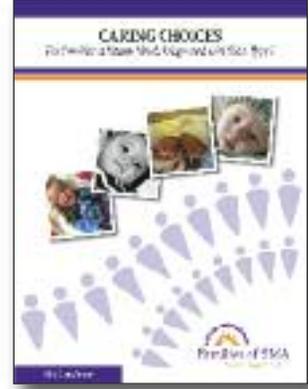
The Genetics of Spinal Muscular Atrophy



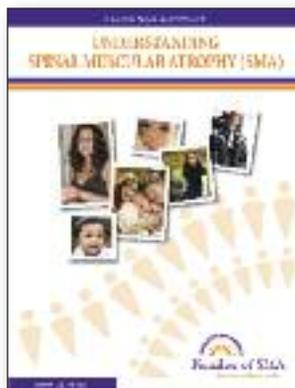
Nutrition Basics



Caring Choices



Understanding Spinal Muscular Atrophy (SMA)



If you would like a hard copy mailed to you please email us at info@fsma.org or call (800) 886-1762

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**Capitol Region, satellite of the
 Greater New York Chapter**
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 Amy & David Cunniff, President
 gnycapitolregion@fsma.org

Western New York Chapter
 (since 2003)
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 (716) 622-0804
 Bonnie Shiesley, President wny@fsma.org

OKI (Ohio, Kentucky & Indiana) Chapter
 (since 2004)
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 Cincinnati, OH 45254
 (513) 753-8222
 Beth Lockwood, President oki@fsma.org

Michelle Palmer, IN Representative
 oki@fsma.org

Are you interested in starting a new chapter for Families of SMA?

Please send an email to chapters@fsma.org.

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 Karen McRory-Negrin, President
pennsylvania@fsma.org

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 Sarah Boggess, President
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Texas Chapter

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 Kelly Coggin, President
texas@fsma.org

Utah Chapter

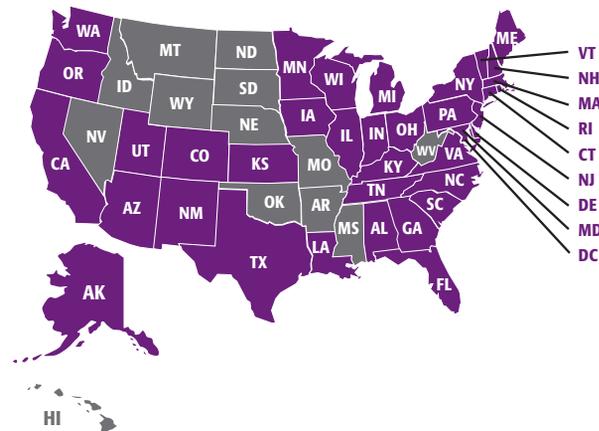
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 Shane Barber, President
utah@fsma.org

Pacific Northwest

(since 2006)
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 (306) 871-1084
 Russ Hargrave, President
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Wisconsin Chapter

(since 1999)
 PO Box 320516
 Franklin, WI 53132
 (414) 324-0499
 Contact: Kate Vogedes, Laurie King, Jenny Imhoff
wisconsin@fsma.org

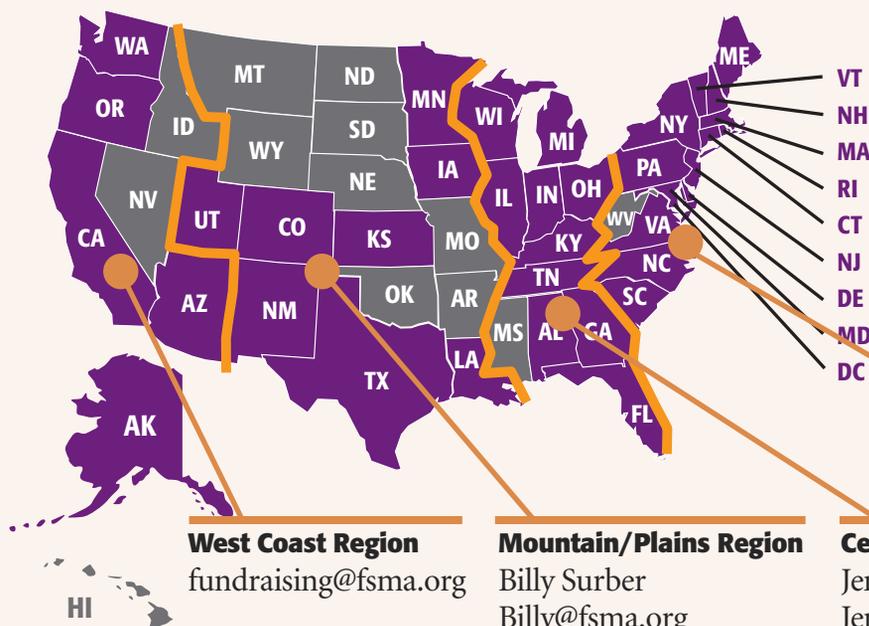


FSMA Chapters

Our Chapters make it their mission to support families and fundraise for SMA, giving hope to families in their community. Chapter fundraisers include Walk-n-Rolls, golf tournaments, and gala events. Chapter support includes providing resources to families affected by SMA, linking families together for mutual support, and providing public awareness in their communities for a wider awareness of SMA.

Have you ever thought about starting a chapter?

We want to hear from you.



West Coast Region
fundraising@fsma.org

Mountain/Plains Region
 Billy Surber
Billy@fsma.org

Central Region
 Jennifer Chaput
Jennifer@fsma.org

Eastern Region
 Sarah Rodriguez
Sarah@fsma.org

CHAPTERS ARE REACHING OUT TO COMMUNITIES ALL ACROSS THE COUNTRY.

Families of SMA currently has over 31 chapters in the United States, but we are looking to expand!

Support your community.
 Fundraise for research.
 Hope for families.

Begin to make a difference today.

Send an email to chapters@fsma.org to receive more information on how to start a chapter in your state.

ALABAMA

Mississippi

1st Annual Mississippi State Walk-n-Roll in Memory of Zachary Thomas Luccasen

On March 24th, 2012 the 1st Annual Mississippi State Walk-n-Roll was held at the Mississippi State University Drill Field in memory of Zachary Thomas Luccasen, the son of a Mississippi State University teacher. Thank you to Raven Young, the sister of Imanie Hartman, who also has SMA, for holding the event. Raven's hard work in planning the Walk-n-Roll raised \$1,747 for Families of SMA!

ALASKA

The Alaska Chapter has been busy getting organized and spreading SMA Awareness. We have gained several new members and have contacted the medical community in hopes of generating more awareness. If you are interested in joining the chapter or have any fundraising ideas, please e-mail us at Alaska@fsma.org.

ARIZONA

Virginia G. Piper Sports and Fitness Center

The Arizona Chapter toured the new Virginia G. Piper Sports and Fitness Center in Phoenix, AZ on Saturday, June 16th, 2012. The fitness center is a universally accessible fitness center designed specifically for individuals with disabilities. The center features a variety of activities including wheelchair rugby, basketball and soccer. There is a climbing wall, suspended track as well as a therapy pool, lap pool and whirlpool with elevators, chair lifts and ramps. The basketball court has hoops that can be raised or lowered for those in wheelchairs. Overlooking the basketball court is a walking track with a low-impact surface. We were able to participate in wheelchair soccer, basketball, rock climbing and activities in the gym.



Cochise Contractors sponsored dinner for all the families at the event as they have each and every year! We were thrilled with the many other sponsors, donations and raffle prizes donated for the auction and raffle events. Every hole had a sponsor and almost everyone won something at the dinner event! We look forward to next year as we celebrate the 17th Annual KDK SMA Smash Golf Classic. Please visit the KDK Smash website at www.kdksmash.com for pictures and more information about this event! We are so appreciative and thankful for all the wonderful support we have from Arizona families. We know how truly blessed we are to have so many generous family and friends help support us as we work to find a cure for Spinal Muscular Atrophy. Together we will find a cure for SMA!

KDK SMASH Golf Tournament

On Friday May 4th, 2012 the Arizona Chapter held the 16th Annual KDK SMASH SMA Golf Classic. It was a wonderful day and we had a huge turn out of golfers for the tournament with many more families and friends joining in for the dinner after the tournament. The AZ Chapter has been holding this tournament on the first Friday in May since 1996 with many of the same families and friends participating in the event since the very first year! With the support of the Arizona families and friends, the Arizona Chapter raised over **\$12,000** this year! All together, the KDK SMASH SMA Golf Classics have raised close to \$600,000 to help find a treatment and cure for SMA! We are so thankful for all the generous support received for this special event! A special thank you to the Kaler Family for all of your hard work and dedication in planning this event.



Arizona Bowling Fundraiser

We had a great time at our first family bowling event on May 19th, 2012. There were 18 individuals who came to bowl and we raised \$350 for our chapter. Everyone had such a great time we look forward to bowling again soon!

Angel Wolff
Phoenix, AZ



CAROLINAS

North Carolina

4th Annual Cure SMA Walk-n-Roll for Gray's Gang



On April 28th, 2012 we held the 4th Annual Cure SMA Walk-n-Roll for Gray's Gang in honor of Gray Dougherty in Charlotte, NC. The event was our best yet! We raised over **\$29,500** to be used for critical research so we can soon enjoy a treatment and cure for SMA. A fun walk along the AG Middle School and Selwyn Elementary campuses started the day. The day also included lots of games including corn hole, a punt, pass and kick competition, go fishing, cake walk, dance competitions, jumpy house, face and hair painting, balloon and magician, spin art and raffle opportunities to win fabulously donated prizes such as an iPad, Kindle Fire and lots more! One of the most rewarding parts of the day included meeting two new SMA families. We truly appreciate the loyal support of friends and family who work so very hard and participate each year to make the walk such a success!

*Abbie and Mike Dougherty
Charlotte, NC*



5th Annual LittleMan Memorial Golf Tournament



The 5th Annual LittleMan Memorial Golf Tournament and "Shoot the Links for a Cure" weekend in memory of Joseph Reese, SMA Type I, was held on Friday, June 15th and Saturday, June 16th, 2012 in Macon, NC. With a record number of sponsors, donors and golfers, we were hopeful that 2012 would be a record breaking year.

There were over 300 people at the dinner and auction, which included celebrity autographs, handmade items, and sports memorabilia. The live music of Steve Owens and Summertime wrapped up the night with some fantastic beach music. Over 225 people joined us on Saturday for a great day of golfing and fun!

We set our goal high this year, \$20,000, but knew that even if we only raised enough money to bring a smile to one child's face or help one family dealing with SMA it would be a mission accomplished. This year we surpassed our goal and were able to raise an amazing \$30,000, with **\$15,000** going to Families of SMA. Even though our son Joseph's stay here was short lived his presence

has been felt through so many with their love and support of this event.

*Blaine and Joanne Reese
Macon, NC*

Thank you to Blaine and Joanne Reese and the LittleMan Foundation for supporting Families of SMA. Your amazing \$15,000 gift will help fund research to find a treatment and a cure for SMA. Best of luck with the "Joseph's Change Gang" piggy bank project. We hope that all 250 piggy banks are adopted this year and that the project is a great success!



Stroller Warriors Running Club

Stroller Warriors™ Running Club of Camp Lejeune, NC, participated in the Quintilles Wrightsville Beach Half Marathon on March 18th, 2012, to raise money for Families of SMA. Military Spouse Magazine's 2012 Marine Corps Spouse of the Year, Stephanie Geraghty, founded this free club in 2010 as a means to help her fellow military wives. Her 3-year-old son Cole was diagnosed with SMA Type 2/3 last year. Three Stroller Warriors including Elizabeth Harlow, Amanda Zimmerman, and Rebecca Smith took the initiative to organize a fundraiser in Cole's honor. Upon completing the half marathon, the entire team had raised **\$7,360!** Stroller Warriors plans to participate in a similar event every spring to assist Families of SMA in their research for a cure and outreach to families. Thank you Families of SMA. We support you!



CAROLINAS (cont.)



1st Annual Loving Logan SMA Walk-n-Roll

A rainy day for the inaugural Loving Logan Walk-n-Roll for Spinal Muscular Atrophy in honor of Logan Moore held in Smithfield, NC fared well for fundraiser organizers and participants. The event raised just over \$4,250, eight times the fundraising goal first set by organizers. Walkers carried umbrellas, dawned raincoats and ponchos as they walked the one-mile course around Smithfield Community Park, shining through the clouds to unite in a cause.

During their walk they were reminded of why they were there, as course signs detailed facts about Spinal Muscular Atrophy. Signs throughout the course read, "SMA is the number one genetic killer of children under the age of two"; "One in every 6,000 babies is born with SMA"; "SMA can strike anyone of any age, race or gender." All proceeds from the event were donated to FSMA in support of the latest research and development of a treatment and cure for SMA.

Overwhelmed and overjoyed by the support of the local community, the Loving Logan organizers are already planning to host next year's event in honor of SMA victim Logan Moore who passed away nearly a month before his first birthday. This walk will take place each year as a reminder of his life and to raise awareness of the terrible genetic disease that claimed his life.

Next year's event is scheduled to be held in March. For more information regarding The Loving Logan Walk-n-Roll email lovinglogansma@gmail.com or visit www.fsma.org/lovinglogan.

Loving Logan Organizers would like to thank all race sponsors, fundraisers, and participants for taking part in this inaugural event and helping to raise SMA awareness on behalf of Logan Moore.

*Our Sincere Gratitude,
The Loving Logan Organization, Layne Moore and Family, Smithfield, NC, Dana Grimstead, Kill Devils Head, NC*

4th Annual Jocelyn Paige Lee Golf Tournament

The 4th Annual Jocelyn Paige Lee Golf Tournament on April 21st, 2012 in Louisburg, NC was a huge success again this year. It's funny it works sometimes, we had our lowest turnout of golfers but we raised more money than ever before! The tournament raised a little over \$6,000 dollars all going to FSMA and the groundbreaking research being done in the SMA community. This year's tournament was a success because of the dedication of family, friends and neighbors in our small community. They all are helping to keep Jocelyn's never give up attitude alive with their hard work and heartfelt contributions. It was a bittersweet day as this year was the first year that Jocelyn wasn't physically with us, but without a doubt you could feel her presence in the hearts and minds of everyone who attended. We were blessed to have a special guest performance by Caleb Oliver, former lead singer of the Christian Rock band Decyfer Down. Caleb graciously donated his time and talent in helping our daughter's foundation. We would like to thank all our sponsors and donors, as well as The River Golf and Country Club for being great hosts for the past four years. We look forward to bigger and better events in the years to come! Thank you all and God Bless!!!!

*Jennifer and Shane Lee,
Louisburg, NC*

Celebrity Bartender Tips

On May 17th, 2012 Jeff Mitchell participated as a celebrity bartender in Elizabeth City, NC and donated his tips to Families of SMA in memory of Haley Mitchell. Thanks to Jeff and the celebrity bartenders, \$120 was raised for Families of SMA! Thank you Jeff for your dedicated support!

CHESAPEAKE

Maryland

Parr Ridge Read-A-Thon

The students at Parr Ridge Elementary in Mount Airy, MD participated in a "Read-a-Thon" to raise money and promote awareness of Spinal Muscular Atrophy and Cystic Fibrosis on February 2nd, 2012. We have two students at our school who are affected by these disorders and we wanted to have the students understand the difficulties that these two students face on a daily basis and to be able to show support for their fellow classmates by raising money for your organization. Our Fine Arts and Fitness staff presented a wonderful informative program

and then the students had reading time together – all in their pajamas!

The students, families and staff raised over \$2,685 for Families of SMA! Please know that is it with concern and caring that these funds have been raised and sent to you to further research on these disorders and provide basic assistance to those who are affected.

Sincerely,

*Ann M. Blonkowski, Principal
Parr Ridge Elementary*

Virginia

Matthew and Emily's Wedding

Emily and Matt Chesnes set up a wedding gift registry on the Families of SMA website and asked their family and friends to donate in lieu of gifts. Their dedication, and the generosity of their family and friends, raised \$2,800 in honor of their nephew, Charlie Chesnes!

Washington D.C.

On March 10, 2012 Erin Tassey ran the DC Half Marathon in honor of Charlie Zerzan. Thanks to Erin's dedication, hard work in training and her many generous supporters, over \$2,600 was raised for Families of SMA!

CONNECTICUT

1st Annual Cubby's Run

The goal of my event was to help raise awareness and money for Spinal Muscular Atrophy. I did my run on May 5th, 2012 for a young boy named Cubby Wax who lives in my town of Ridgefield, CT. Cubby was diagnosed with SMA in 1999 when he was about four months old. Despite having this disease, he is a thriving student and a very dedicated Mets fan. Even though Cubby is a Mets fan, we are still very good friends.

My project was to raise \$5,000, but thanks to everyone who helped, I raised over **\$11,000**. The run I did for Cubby was a 1-mile run, walk, and roll for anyone, but mainly kids. The run, walk, and roll was hosted by my local recreation center. This was my first time doing

a project like this so when we raised this much money and had over fifty people sign up, Cubby and I were very excited. Since this was the 1st Annual Cubby's Run, I will do it again next year. I hope we have even better results!

Sincerely,

*Ethan Hynes
Ridgefield, CT*

West Rocks Middle School Fundraisers

The West Rocks Middle School in Norwalk, CT held fundraisers throughout the month of April in honor of Cubby Wax. Thanks to the hard work and dedication of students, staff and families, \$1,225 was raised for Families of SMA!



Emma Hope Award

Emma Hope Goldsberry (SMA Type II) is shown here with the most recent Emma Hope Award winners at NES pageants. Kendal Ward won at the Miss Northeast America pageant, where \$500 was raised for FSMA. Madison Burton won at NES Midwest, and that pageant raised \$140. These are the latest pageants from NES director Tammy Rezendes that donates entry fees from the Emma Hope Award to FSMA. Those fees, along with fees from other pageants, photo contests, and other donations from pageant participants, have totaled over \$15,000 in donations for FSMA. The Emma Hope Award is given to the participant displaying the most kindness towards others.



GEORGIA

The Georgia Chapter is pleased to report that the 2nd Annual SMA 5K Eat 'n Run to benefit Families of SMA took place March 24th, 2012 in Columbus, GA and was another great success raising over **\$33,000!** We held another fundraiser at the Sips N Strokes in Sandy Springs on June 24th, 2012. Our last Sips N Strokes fundraiser, held in August 2011 for SMA Awareness Month, was a huge success.

President Rio and Vice President Ashley have been working with Quest Diagnostics to raise awareness and to plan the fall event. Already there have been over five dinner meetings scheduled with doctors where Ashley and a representative from Quest

Diagnostics will be meeting with the medical professionals in attendance to talk about SMA, educate them on the importance of screening and testing as well as the standard of care and other general SMA concerns and issues.

Thank you everyone who has supported the GA Chapter as we work to build up our chapter and really establish a community here in the Peach State. Look out for a review of the SMA Day with the Atlanta Braves and the 2nd Annual Workout for a Cause in the next issue of Directions!

Immaculate Heart of Mary Second Grade Lenten Service Project

Dear FSMA,

Please accept the \$200 donation made by my second grade religion classes from Immaculate Heart of Mary School in Tucker, GA. This was our Lenten Service Project on behalf of all the children affected by SMA. We are praying for a cure.

Blessings,

*Ruth Patch, Second Grade Teacher
Tucker, GA*



GREATER FLORIDA

Hello from the Greater Florida Chapter! Stretching from south of Sarasota to the North Florida panhandle, our chapter covers a large and diverse geographic region and includes many amazing families. In order to better serve their needs, our local board focused on events and activities during the first half of 2012 to enhance the supportive services we provide. From educational meetings to family gatherings, our goal was to foster relationships among chapter members. In the second part of the year, our main focus is on SMA awareness and fundraising. From May 2012 through July 2012, our efforts included the following:

Presentations with Quest Diagnostics

As part of FSMA's national partnership with Quest Diagnostics, members of the Greater Florida Chapter have been making presentations on the importance of carrier testing. In May and June, Katie Kerns, chapter president and mom to Allison (Type II), Audra Butler, chapter vice president and mom to Andy (Type I Angel), and Diana Baross, chapter member with Type II, attended dinner meetings in Tampa, Lakeland, Orlando, and Winter Garden with groups of doctors, nurse practitioners, and medical staff. After a presentation by Attorney Phil Duffy about risk management techniques that providers should follow, we spoke about our experiences with SMA, our involvement with FSMA, and the need for practitioners to educate their patients about this disease, which includes offering testing for it. Since the presentations, Quest staff has reported that several of the clinics represented have expressed interest in offering SMA carrier and pre-natal tests to their patients. The Greater Florida Chapter feels confident that our partnership with Quest Diagnostics will lead to much more awareness about the disease in the state of Florida and increased testing for it in the local medical community.



SMA Family Fun Day at LEGOLAND Florida



LEGOLAND Florida hosted Greater Florida Chapter members for this year's Family Fun Day. LEGOLAND Florida generously waived admission cost for our families, allowing them to spend a wonderful day at the amusement park without hurting their wallets! Participation

exceeded our expectations, with almost 100 chapter members registering for the free tickets! Todd Andrus, Sales Manager, and Marcy Harrison, Executive Assistant, worked very hard to accommodate the needs of our families, including providing us with an air-conditioned mansion and free cold bottled drinks when we needed to escape from the heat! Thank you, LEGOLAND Florida, for our best Family Fun Day yet! LEGOLAND is a great supporter of our August SMA Awareness Month efforts – keep reading for more exciting news!

Angel Dinner

Audra and Alan Butler hosted the first in a series of pot-luck dinners for families who have lost a loved one to SMA on Saturday, June 30. The goal of these dinners is to build stronger relationships between local bereaved families and to provide a supportive network of friends who understand. Five families attended this first dinner, enjoying great food and fellowship.

70th Birthday Fundraiser

In honor of her 70th birthday, Rosemary Perry, grandmother to Angel Andy Butler, asked for donations to FSMA in lieu of presents. Her birthday wish is to find a cure for SMA, and she is proud to have exceeded her fundraising goal to make that wish come true, raising more than \$1,300!

August SMA Awareness Month Events

Once again, the Greater Florida chapter reached out to city, county, and state elected officials, asking them to proclaim the month of August as SMA Awareness Month. We are also hosting the following events during SMA Awareness Month: Thirty-One Catalog Fundraiser, Painting with a Twist Fundraiser, Candlelight Memorial Ceremony, "Buddy Benefit Days" Fundraiser at LEGOLAND Florida, Macy's Shop for a Cause, and SMA Day with the Tampa Bay Rays. Look out for updates on these events in the next issue of Directions! For more information about these and other upcoming events, please visit. www.fsma.org/greaterflorida!



Cabane 1219 Fundraisers

The La Societe De Femme, Cabane 1219 held several fundraisers for Families of SMA in fall 2012 in honor of Jacey Kloiber. Jacey's grandmother is a member of the organization. Thank you to La Societe De Femme and Marie Pink for raising \$200 for Families of SMA!

While most of our fundraising and awareness events currently are held in the Tampa Bay or Orlando areas, we are hoping to expand the chapter's reach as more families in the northern region of the state join the chapter. If you live in Central or North Florida and would like to host an event, please contact Katie Kerns, chapter president, at 727-388-1888 (office), 727-512-4192 (cell), or greaterfl@fsma.org (e-mail). We also encourage you to check out our website at www.fsma.org/greaterflorida and to join our "Families of SMA – Greater Florida Chapter" group on Facebook (www.facebook.com/groups/79658598961/). Thank you!

GREATER NEW YORK

Chapter Update

Members of the Greater NY Chapter were pleased to participate in the filming of an episode of MTV's *Made* where a teen with SMA named Abby wanted to become a rapper. It was an honor and pleasure for all of us to spend time with Abby, her wonderful family, and the MTV crew helping raise SMA Awareness and making Abby's dreams come true.

We recently have had the pleasure of the addition of adding a satellite chapter to the Greater NY Chapter. The Capital Region Chapter will cover the areas around Albany, NY. Many thanks to Amy and David Cunniff for taking on this task and bringing support and awareness to the Albany, NY area. For more information regarding the Capital Region Chapter please email, gnycapitalregion@fsma.org.

NYC Half Marathon

The NYC Half Marathon Team raised an incredible **\$53,000** to support FSMA programs and research. There were many family members

who ran for loved ones in this race and helped raise an amazing amount of SMA Awareness. Many thanks to Michele and Douglas Erwin for all of the efforts they put into this successful event.

FSMA Greater New York Chapter Golf Outing



The 2nd Annual Greater NY Chapter Golf Outing was held in Hudson Hills Golf Course in Ossining, NY on Monday, July 9th, 2012. We have raised over **\$30,000** at this event. Westchester County Executive, Rob Astorino made an appearance at dinner and spoke about the importance of finding a treatment and cure for SMA. Many thanks to the Van De Loo and Rubenstein Families for organizing and running this successful event!

NY Islanders SMA Awareness Day



The Greater New York Chapter organized the NY Islanders SMA Awareness Day in memory of Deirdre Abraldes, held on March 4th, 2012. Thanks to the overwhelming support of families, friends and the local community, the chapter was able to raise **\$19,310!** Thank you to Debbie Cuevas and the Greater New York Chapter for organizing this event, and a special thanks to the Abraldes Family and Chris Foy for being a part of this memorable day. Your dedication and support raised lots of needed awareness and funds for SMA!

Long Island Marathon

Michael Delaney ran the Long Island Marathon for Families of SMA in honor of Maeve Coffey on May 5th, 2012 on Long Island in New York, NY. Thanks to Michael and his generous supporters, over \$7,260 was raised!

CA Montessori Children's Center Dance Class

On April 11th, 2012, the CA Montessori Children's Center in Oslandia, NY hosted a dance class to their families and donated the proceeds to Families of SMA in honor of Greyson Erwin. The event raised \$2,855! Thank you to Maureen Ligouri for once again organizing this event.



GREATER NEW YORK (cont.)

Clink for a Cure Wine Tasting

On April 28th, 2012, Jill Rebeor hosted the Clink for a Cure Wine Tasting fundraiser in honor of Ciara Rose Van De Loo. Thanks to Jill's hard work and dedication, \$100 was raised for Families of SMA!

the very first beautiful spring day in New York, we raised nearly **\$1,900!** We already have plans in the works to make the crop bigger and better for next year!

Capital Region

Crop for a Cure

On March 17th, 2012 the Greater NY Capital Region Chapter had its very first fundraiser – a scrapbooking Crop for a Cure held in Schenctady, NY. Ladies signed up to spend the day scrapbooking and enjoy Make-n-Take sessions with Creative Memories and Stampin Up representatives. We also had generous donations for raffles — all related to scrapbooking and stamping! Although we had a lower turnout out due to the fact it was



ILLINOIS

2nd Annual Tee Off with the Drive to Cure SMA in Honor of Ryan

On Saturday, May 19th the 2nd Annual Tee Off with the Drive to Cure SMA in honor of Ryan Manfre took place at Gleneagles Country Club in Lemont, IL. Ryan Manfre is a five year old boy who was diagnosed with SMA Type III at the age of two. Ken and Jeanne Emerson, Ryan's grandparents, organize the event, which has been a huge success. This year nearly **\$48,000** was raised for FSMA! There were 168 golfers and approximately 200 people who attended the dinner, which also included raffles, split-the-pot, and a silent auction. Brian and Becky Manfre, Ryan's parents, and Suzanne and Steve Fedea, Ryan's aunt and uncle, along with 19 volunteers of family and friends helped make the day run smoothly. Together we are striving to raise awareness and money to help find a treatment and cure for SMA! Save the date for our 3rd Annual Tournament on Saturday, May 18th, 2013! Visit www.fsma.org/golfforryan2013 to register.



ILLINOIS (cont.)

Illinois Chapter Walk-n-Roll



We had so many wonderful individuals and business that donated their time and money for this year's walk! Also a BIG thank you to everyone that helped volunteer before, during and after the walk, you are all amazing people!

Here is another wonderful wish... "I wish that every person affected by SMA has another Birthday."

See you next June... be good... stay healthy... spread awareness!

*Janet Schoenborn
Grayslake, IL*



The 2012 Walk-n-Roll for the Illinois Chapter was a dream come true! We had a record year for our chapter with over 500 people in attendance and raised over **\$45,000**. The walk had many meaningful moments for all individuals attending. It was a great day for teams to get together and show support for one another. The walk route was 2.5 miles and along the trail were several stations. One of the stations had a "Wish Box." many people wished for a cure, happiness and we even had a few people wishing for a water bottle! It was a hot day! We also had a birthday girl, Happy Birthday Ella! We had hula hoops, hopscotch, bubbles and a drawing station. The picture will be offered next year at the raffle!

The chapter purchased a tree in honor of Illinois Chapter that will be planted in September at the Independence Grove Forest Preserve by the North Bay Pavilion. The tree represents our families' strength and unity. It will be a wonderful place to call our own.



Cure for Chloe Christmas Ornament Sales

Dear FSMA,

Our annual fundraiser of the Cure for Chloe ornaments (beautifully designed by Chloe, SMA Type II) raised \$473 this year for SMA research! A big thanks to our family and friends for their continued support of Chloe in her fight against SMA and for giving so many HOPE for a cure! Thank you FSMA for all you do and giving us families so much support!

Love,

*The Ochoa Family
Kathleen, Jake, Chloe, and Tysen
Homewood, IL*

Riverman Travel Goals Fundraiser

Thank you to the staff of Alexander Travel, Ltd. in Peoria, IL for making Families of SMA your charity of choice. For every goal that was hit by a staff team member, \$25 was donated to Families of SMA. Eighteen goals were hit and \$450 was donated to Families of SMA!

Kendall Elementary PTA Memorial Fundraiser

On February 2nd, 2012 the Kendall Elementary PTA in Naperville, IL held a memorial for Drew Plotke who was a 1st grade student at the school. The PTA was able to raise \$340 in his memory. Thank you for helping us raise funds to find a treatment and cure for SMA!

Chapter Meet-n-Greet

The Illinois Chapter hosted a Meet 'n Greet event at the national office in Elk Grove Village, IL. Many families came out to hear from Kenneth Hobby, FSMA President, and Janet Schoenborn, IL Chapter President. Some of the wonderful local staff at Children's Memorial Hospital, as well as a representative from the Make-a-Wish Foundation of Illinois were in attendance as well! The meeting resulted in increased support and revenue for the Chapter Walk-n-Roll in June as well as some great networking and bonding for all of the local IL SMA families!



IOWA

Spin 4 Quinn

We had a spectacular day for the 1st Annual Spin 4 Quinn Trail Ride May 5th, 2012 in Hiawatha and Cedar Rapids, IA. Our goal was to raise \$3,000, but through the generosity, work, time, and help of friends, family, and well wishers, we were able to bring in over **\$13,000** for FSMA! About 70 bikers took off at 9:30 a.m. on the Cedar River Trail from Hiawatha through downtown Cedar Rapids to the southwest side of Cedar Rapids and back. After the 2008 flooding, the downtown is still recovering, so this gave people the opportunity to see recovery and new building projects as well as enjoy the scenic views. Many folks biked the full 20 mile round trip to Tait Cummins Park and back to Guthridge Park in Hiawatha with stops at McLeod Run and Sokol Park in Czech Village. Some riders stopped off at local businesses for a bite to eat! A group of third and fourth graders made it the full 20 miles with energy left at the end--they said they are ready for next year!

At 10:30 a.m. the other 70 adults and kids walked or biked around the mile trail at Guthridge Park near

our home. Quinn used his hand-pedal Amtrike for the last time when he decided he needs a bigger hand-pedal bike or tag-along bike for more speed to go on the BIG ride next year! Quinn,

our seven year old son who has SMA Type II, became a big promoter after the event at church when talking about how blessed and thankful we felt; he announced into the microphone during the service, "YOU CAN STILL DONATE TO HELP KIDS LIKE ME WALK!" His "patented" spinning move in his power chair that frightens and amazes us was the inspiration for the name of the event!

Our Tike's Trail activities included face painting by Lizzy's Creations donated by Jennifer and Bruce Borchering; create a Spinner pinwheel, CURESMA beaded bracelet-making donated by Lisa and Mark Anton, coloring books and crayons donated by Lisa and Steve Anton, and bean bag toss donated by Shelley and Jason Lamneck. Mollie Schlue and Andrea Lewerenz-Norris donated the trail mix snacks for the bikers, too! Our Jefferson High School girls' soccer team helped with activities and modeled the fantastic face-painting. Registration went smoothly with the help of Carol and Sabryna Scheidt, Amy and Jackson Williams, Cherryna Lock, and Jo Weidemann, and many others.

As a wrap up to the ride we gave Thirty-One Bags donated by Nancy AntonJensen, treats, and kites to the Best Team Name--appropriately on Cinco de Mayo, Team Paseo de Quinn; Best Decorated Bike, Quinn Anton-Jensen in dog bike; and Best Costuming by the Hiawatha Elementary Staff Members with matching Team Quinn Cougar Paw print shirts!

Lisa Anton and Judy Anton made this day possible with work and encouragement along the way, and lots of great ideas! Angela Dygdon donated the large Spin 4 Quinn

banner, had our volunteer t-shirts printed, in addition to her wonderful, creative skill in graphic design for the logo and signage--she received an "A" from her professor too! Our volunteers for communications and safety on the route and at Trail Water Stops were friends and family, Jan Woods, Jen and James Hoelscher, Larry Jensen, and Dave, Theresa, and Larry Anton and the Cedar Valley Amateur Radio Club who helped bikers and watched over the event with radio communications and care! Next year we plan to have the event on May 4th--Star Wars Day--" May the FOURTH be with you!"

Thanks and blessings to all.

Love,

Nancy, Brice, Grant, Drew, and Quinn Anton Jensen, Hiawatha, IA

Alan Shepard Elementary School Walk for SMA

Dear Families of SMA,

On May 17th, 2012, we held a Walk for SMA at Alan Shepard Elementary School in loving memory of Jack Lindaman and in honor of Ella Christopher, Type II-III. Over 350 students walked for about 45 minutes to an hour; we raised over \$1,600 to support your cause. We are proud of our efforts and we appreciate the work that you do. Thank you for this opportunity to support Families of SMA.

Most sincerely,

Sherri Marceau Eldridge, IA



KANSAS CITY

Missouri

Gracie's 1st Annual Steak Dinner for SMA

A steak dinner was held on April 21st, 2012 in Eugene, MO to raise money for SMA research. In addition to the dinner, a raffle and silent auction were held and clowns painted faces and made balloon animals for the children. Thanks to many volunteers and the donations of numerous local businesses, the fundraiser raised over \$6,000. The benefit was hosted by Sherry DeGraffenreid in honor of her granddaughter Grace DeGraffenreid who has SMA.



Phi Tau Omega Sorority Trivia Night (Delta Phi Chapter)

The Delta Phi Chapter of Phi Tau Omega Sorority in St. Louis, MO hosted their annual trivia event on March 12th, 2012. Thanks to Kathy Goodyear and the other sisters, \$1,094 was raised for Families of SMA in honor of Brittany Carpenter. Thanks to Brittany to coming to the trivia night and selling raffle tickets.



Pickmeister Superbowl Party

The Pickmeister Superbowl Party was held on February 8th, 2012. Thank you to Natalie Gibbs for organizing this event. \$100 was raised for Families of SMA!



LOUISIANA

Arkansas

Family Fun Day for SMA in Honor of Brooklyn Marie Gould

The night before our event the forecast predicted a 75% chance of rain so we were quite worried, but we were blessed with a beautiful 75 degree, sunny day. The Lutheran ladies ran the bake sale and we had many volunteers that baked some wonderful goodies. The men cooked up some wonderful hamburgers and hot dogs on the grill. Businesses and friends in the community donated items and gift certificates for the raffle and we even had handmade items from a local wood worker. The children had so much fun with the games, face painting and bounce house. The live band "Overcoming Affliction" entertained us with awesome music and even said the most heartfelt prayer for Brooklyn and for victims of Spinal Muscular Atrophy. They thanked us for building awareness and said they were going to spread the word and include a donation

bucket at all their future events. A man who was diagnosed in his 20's with SMA stopped by just to meet us and drop off a check! Another girl, who lost her nephew at five years old to SMA, stayed the whole day with us. She said her family is quite active in Michigan with fundraisers for FSMA. My sister Terry (Brooklyn's Great Aunt) came all the way from Florida to help with our event and brought a whole envelope of cards for Brooklyn. We have people and churches all over the world praying for a cure for SMA. We were on two television shows, two radio shows and the newspaper did a two-page article on Brooklyn and living with SMA. I can honestly say we had fun while building awareness and raising funds in Mountain Home, AR. I am so thankful to my family, friends and the community for coming together to make this all possible. I felt blessed to be surrounded by such wonderful, loving and caring people at our Family Fun Day for SMA event. We were able to raise over \$2,300!

*Paige Fenske
Mountain Home, AR*



LOUISIANA (cont.)

Flarble Toy Sale

Gordon and Jennifer Bopp of Rock Creek Molding in Glenwood, AR donate a portion of all Flarble sales from www.flarble.com to Families of SMA. Their friend's daughter, Tori Partee, was born with SMA. Thank you to Gordon and Jennifer for their continued support and donation of \$234!

Louisiana**1st Annual Rock-n-Bowl in Honor of Jackson Audibert**

The 1st Annual Rock-n-Bowl fundraiser was held on May 20th, 2012 in New Orleans, LA and was a family event created for the purpose of raising money for research and development of a treatment for SMA. The event was in honor of four year old Jackson Audibert and committee members consisted of family and friends of Jackson who wanted to be involved with Families of SMA and work together toward a common goal.

The committee's goal was to raise \$10,000. After deciding to hold a modest bowling event with food, music, and children's activities, the platform quickly grew to include live music by "The Wise Guys", a silent auction, a food menu that consisted of lunch, snacks, and deserts, and extended children's activities such as face painting, games, and dress-up picture taking. Sponsorships grew at a fast pace and thanks to the committee's marketing efforts, contributions and participants soon followed. Ticket sales were budgeted at 300. A total of 400 pre-event tickets were sold and an additional 85 tickets sold on event day. The committee's goal of \$10,000 was easily surpassed and we raised over **\$29,000**.

The chairman in conjunction with committee members established as a primary objective to conduct this fundraising event as a means of teaching young people the importance of donating and giving of themselves for the benefit of those less fortunate. As a tribute to that vision and effort, the 2012 Rock-n-Bowl FSMA fundraising event became a special opportunity that included four generations of family working together toward a common goal. That goal was to play a small part in the continuing effort to find a cure for SMA and to assist those families affected by it.

Special thanks are deserving to the many sponsors, donors, and individuals participating in the 2012 Rock-n-Bowl FSMA fundraising event and to a very special young man by the name of Jackson Audibert, in whose honor this event was held.

Thank you to Shawn Audibert, Becky Apken, Mike and Melanie Otilio, Brandy and Anthony Cangemi, and many family and friends for helping to organize hugely successful event!

Shake It for Ella Zumbathon

We held our 1st Annual "Shake It For Ella" Zumbathon on Saturday, June 2nd, 2012 in West Monroe, LA. Our daughter, Ella Jayne, was diagnosed in November 2011 with SMA Type II. After she was diagnosed, my husband and I knew we had to do something to raise awareness about SMA and raise funds for research. Zumba is very popular in our area and something I am very actively involved in. What better way to raise awareness, have a great time, and get in a great workout! So, "Shake It For Ella" was born. This was our first year and we did great! We had eight local business sponsors, 89 people participated in two-hours of Zumba, and over 120 people attended the event. We gave away 10 door prizes and also had the Chick-fil-A Cow on hand to Zumba along and sign autographs for the kids. This year we raised over \$5,000 for Families of SMA, and next year we hope to raise even more! Most importantly, over 150

people who didn't know about SMA, now do! Raising SMA awareness is what it's all about. We can't wait until next year's event!

*Jennifer and TJ Ecker
West Monroe, LA*

US Geological Survey Fundraiser

The United States Geological Survey Office in Baton Rouge, LA has named Families of SMA as their annual charity sponsor! Throughout 2012, all employees will have the opportunity to donate through payroll deduction and by participating in weekly lunch fundraisers! Each Thursday, lunches will be sold for \$7 and proceeds will be donated to fund the critical research and family support programs that will help find a treatment and a cure for Spinal Muscular Atrophy. The first "kick-off" lunch was a success and we would like to thank all of the US Geological Service Employees for their generosity and support of Families of SMA!



MICHIGAN

8th Annual Michigan Chapter Run, Walk-n-Roll & Family Fun Day

The 8th Annual Michigan Chapter Run, Walk-n-Roll & Family Fun Day was held on April 28th, 2012. This event had a great turnout thanks to the support of friends, family and the local community, and the walk raised over **\$41,000!** Everyone had a wonderful time with great music, clowns for the kids and a beautiful park with a 75% handicap accessible playground. Thank you to Holly Schafer and all of the Michigan Chapter for raising awareness and funds throughout your area!

Brayden's 1st Annual Bowling FUNdraiser

On Saturday, March 17th, 2012, Mike and Christina Janetzke hosted Brayden's 1st Annual Bowling FUNdraiser for FSMA in honor of Brayden Janetzke at Northway Lanes in Muskegon, MI. Friends and family gathered to support fundraising efforts for Families of SMA. We

had 27 teams come out to bowl, enjoy pizza and pop, win door prizes, and participate in our 50/50 raffles. We had various local businesses, family and friends donate to our 20 item silent auction. We beat our goal raising more than \$5,000. Brayden was overjoyed with his first time bowling, while just turning two the week before, this made for a great event to celebrate life. We could not have made this possible without our lane sponsors and volunteers. We are ever so grateful for everyone that came out to bowl and raise funds for Families of SMA!

*Mike and Christina Janetzke
Norton Shores, MI*

Bracelet Sales in Memory of Maggie Sue Kazlauskas



Our sweet baby girl Maggie Sue was born July 3rd, 2011... so beautiful, so perfect... then in December she was diagnosed with SMA Type I, something we had never heard of. How fast you learn when you're the one affected by it.

Right away FSMA sent their overnight care package to Jim and Mindie,

Maggie's parents, that was so informative and helpful. We can't thank FSMA enough.

So in April as a family we wanted to help people, friends, and family become aware of SMA and help find a cure. We ordered our first 100 bracelets... then a week later another 100... then a few weeks later 200 more!

What a supportive community of friends and family we have here in Dowagiac, Michigan. Like us, they too had never heard of Spinal Muscular Atrophy! We are

very proud to have so far raised \$2,725 dollars for FSMA and are continuing to sell bracelets and raise funds for families and research.

Our Maggie Sue was only with a us short 11 months, but left us a lifetime of love and memories we will forever cherish. She is our angel.

Again thanks for all the work FSMA does.

Sincerely,

*Kazlauskas and Baerg family
Dowagiac, MI*



Thank you to Susan and the rest of the Kazlauskas family for all of your hard work in raising funds and awareness in memory of Maggie Sue!

1st Annual Charity Craft & Vendor Show in Honor of Alyssa Simmons

The 1st Annual Charity Craft & Vendor Show in Honor of Alyssa Simmons went really well, and we raised \$1,800! We are looking forward to our 2nd annual show next spring and will keep everyone posted. Thank you for all your assistance in making this a successful event, especially for Alyssa Simmons, who we honored at the event.

*Lisa Dzierzawski, Wendi McTaggart and Traci Marleau
Warren, MI*



MICHIGAN (cont.)

St. Mary’s National Jr. High Honor Society Fundraiser

On May 4th, 2012 the St. Mary’s National Jr. High Honor Society in Saint Clair, MI held a fundraiser in memory of Alyson Marie West. The event raised over \$1,300! Thank you to all of the event organizers as well as the event participants and supporters for making this fundraiser a success.

“Jean” Therapy in Honor of Connor Petzke

Connor Petzke was diagnosed with SMA Type II in January of this 2012. During the last full week of school, the staff members of Whitmore Lake Public Schools in Whitmore, MI paid to wear jeans. For each day that a staff member participated, they paid \$5 to donate to SMA research. If a staff member participated in the entire week, then the “cost” was \$20. In addition to wearing jeans, each participant wore a ribbon during the week to

recognize Connor and SMA. Jennifer Petzke (a staff member and mother of Connor) organized the fundraiser and collected the money, which benefitted Families of SMA. We raised \$1,285! A big thank you to Superintendent Kim Hart for supporting the idea and to all of the staff for their participation. Almost every teacher participated in the fundraiser!

*Jennifer Petzke
Brighton, MI*

TerraCycle Brigade Fundraising

Merrick Wolfe and Tracey Vincent have been hard at work collecting juice pouches and trading them in to TerraCycle to support Families of SMA. They raised \$510 in memory of Jessie Vincent, SMA Type I! Thank you to Merrick and Tracey for helping us raise funds to find a treatment and cure for SMA.

Premier Designs Jewelry Party in Honor of Brielle & Brooke

Thank you to Sherry Crossley for all of her hard work in hosting the Premier Designs Jewelry Party in honor of Brielle and Brooke Kennedy, who both have SMA Type II. Your hard work and dedication resulted in over \$500 for Families of SMA!

Families of SMA and the Michigan Chapter would like to extend our deepest thanks and gratitude to Ken and Cindy Armbrustmacher, parents of Mallory Armbrustmacher, SMA Type II. Ken and Cindy served as the Michigan Chapter President and Secretary and have recently stepped down to spend more time with their family. Thank you Ken and Cindy for helping to build up the Michigan Chapter to be one of our strongest and most active chapters in the country! We sincerely appreciate all of your hard work, time and dedication to our cause and for your years of service we’ll be forever grateful!

MINNESOTA

Chapter Update

We were so pumped up for the FSMA conference to be held in Minnesota for the first time ever and truly hope it can happen again. It was such a thrill to be part of an international happening, and we had fun networking with SMA Families from all over the world!

The Minnesota Chapter had a Meet-n-Greet that was far too short. We even had goodie bags for each family and lap blankets for the children. Lemonade and cookies were enjoyed by all.

Looking forward to the next Families of SMA conference in June 2013 in Anaheim, CA!



William Anco Memorial 5K (W.A.M. 5K)

On May 5th, 2012, Scott Buchkoski hosted the 3rd Annual W.A.M. 5K in memory of William Anco in Lindstrom, MN. Thanks to the generosity of family, friends, and the community the event raised over \$3,000! Thank you, Scott!

Polar Plunge for Team Madison

Dear FSMA,

On January 25th a polar plunge was held in Forest Lake, MN. Team Madisyn participated in this event with six plunges. We were able to raise \$70!

*Thanks,
Jamie, Jason and
Madisyn Mevissen
Andover, MN*



North Dakota

Jack Attack on SMA: Round 8

Kristi and Rod Gellner held the Jack Attack on SMA: Round 8 in honor of their son Jack, SMA Type II, on June 9th, 2012 in Fargo, ND. Thanks to the continued support of family and friends the event raised over **\$21,350** for Families of SMA. The balloons and temporary tattoos for the kids along with the picnic lunch for all made it a fantastic day! Thank you to everyone who walked or donated to help us KNOCKOUT SMA. A special thank you to our SMA fighting champs Jack Gellner (SMA Type II), Cole Pulkrabek (SMA Type III), Trish Kuemper (SMA Type III), Chloe Bartholome (SMA Type II), Everly Bartholome (SMA Type II), and Ashley Wohl (SMA Type II)!

NEW ENGLAND

Massachusetts

12th Annual New England Chapter Cure SMA Walk-n-Roll



The New England Chapter held its 12th Annual Cure SMA Walk-n-Roll at DCR Wompatuck State Park in Hingham, MA on Saturday, May 19th, 2012. Over 700 participants – from as far as Vermont and Connecticut — attended and together raised over **\$110,000** for Families of SMA. The weather was great at this family fun walk in the park. Entertainment included overflowing raffle tables, balloons and face-painting, carnival games and prizes, a fire truck, visits from Ms. Wheelchair Massachusetts (Chapter member Patti Panzarino) and Snoopy, as well as the crowd's favorite treat -- Hoodsie cups. The walk was once again organized by Silvia Murphy, Stacey Farrell, Kathy Norton and Kate Norton,

who were ably assisted by many generous volunteers. Local businesses supplied t-shirt sponsorships, refreshments, and raffle donations.

Many SMA families were represented by participants or donations, including those of: Amy Ashdon, Addison Barrett, Carolyn Barrett, Mark Butler Jr., Carrie Capone, Ethan Carter, Annika Cederlund, Mikael Chicoine, Chloe Chu, Maya Cutrone, Daryl Denelle, Shayla Dunn, Aileen Farrell, Diego Fleitas, Glen Graves, Brianna Gross, Jennifer Hanrahan, Angelica Hebb, Gregory Hewett, Joseph Hoadley, Robert Hodge, Ashleigh Jacques, William Johnson, Brian MacDonald, Morgan LaFrance, Melanie Lee, Kamdyn Lessard, Vivienne Liedtke, Cianan and Cecilia Murphy, Aiden Navitskis, Owen Norton, Casey and Colin O'Neill, Paul Osborn, Patti Panzarino, JoAnn Perfido, Dahrian Pimentel, Greer Ramsay, Connor Reilly, Lauren Rogowicz, Stephanie Rose, MacArthur Sohl, Ciara Rose Van de Loo, Rachel Varley, Cubby Wax, Brendan White, Thomas Witowski, Kristin Wormald, and Oskar Zoffer.

Thank you to Silvia Murphy and all of the New England Chapter members, families and friends for raising funds and SMA awareness!

Boston Marathon

To Families of SMA,

My daughter, Joanna Buoniconti, suffers from SMA. On Monday, April 18th, 2012 I ran the Boston Marathon in her honor and raised \$6,892. This is the 8th year I have participated in the race, and I am so proud to have done this as a tribute to Joanna. I am most grateful for the support of my friends and family, and I am most hopeful that their kindness and generosity will help find a much needed cure for SMA.

Very truly yours,

*Stephen J. Buoniconti
West Springfield, MA*

Shamrocks for SMA

On March 9th, 2012 Courtney and Paul Davidopoulos hosted the Shamrocks for SMA event in honor of their son, Matthew (SMA Type I) in Lawrence, MA. The St. Patrick's Day themed fundraiser featured music from The Gobshites, a popular Irish folk band, and Model Citizens, a local favorite. In total, the event raised \$4,527 for Families of SMA! Thank you Courtney and

Paul for all of your hard work in planning this event.

3rd Annual Cubist Silent Auction & Bake Sale

On February 1st, 2012 the Information and Business Solutions Department of Cubist Pharmaceuticals, Inc. in Lexington, MA held its 3rd Annual Silent Auction and Bake Sale in honor of MacArthur Sohl, SMA Type II. Thanks to the generous support of Cubist's employees, \$3,111 was raised for Families of SMA!

Toshiba Fundraisers in Honor of Coby Kulis

We at Toshiba Business Solutions held several fundraisers during the month of April to benefit Families of Spinal Muscular Atrophy. We had an employee whose son was diagnosed shortly after birth. We raised \$700 in memory of Coby Quinn Kulis. We sincerely hope that this will assist you in helping these children and their families.

Sincerely,

*Maureen Rapsomanikis,
Human Resources Director
Wilmington, MA*

Ben Sullivan's Birthday Fundraiser in Honor of Owen Norton

Our son, Ben Sullivan, raised \$328 at a birthday party he had on April 14th, 2012. He had an ice skating party and in lieu of gifts, he asked for donation to Families of SMA in honor of his friend Owen Norton of Braintree, MA.

Sincerely,

*Kristen and Ben Sullivan
Braintree, MA*

Morahan Birthday Fundraiser

On January 26th, 2012 we had a birthday party for my eight year old triplet children Thomas, Michael and Deirdre Morahan. We invited the entire class and asked that in lieu of gifts that donations be made to either Families of SMA and/or Cure Me I'm Irish. Both organizations are close to our hearts as we are friends with Owen Norton of Braintree, MA who has SMA. We raised \$815 for Families of SMA!

Sincerely,

*The Morahan Family
Braintree, MA*



NEW ENGLAND (cont.)

Spring Events in Honor of William Johnson**Yard Sale and House Party**

Spring is a busy time for the Johnson family and events for the New England Chapter of FSMA. In an effort to collect pledges for the area Walk-n-Roll, the Johnson's host an annual yard sale as well as an annual house party. With the community's help, the yard sale this year raised over \$2,100 on May 5th, 2012. The 7th Annual Extreme Soiree where the Johnson's house is emptied of furniture and filled with fun for an evening of generous giving was held on May 11th, 2012. Thanks to Laurie Young and her friends, each year a band fills the space where the dining room table usually goes and helps the crowd dance the night away all while making a difference for William and SMA. This year's party raised over \$4,000 toward Team Walking for William and when combined with the yard sale as well as other pledges, William's team raised nearly **\$12,000** for the 12th annual Cure SMA Walk-n-Roll in Hingham, MA. Joining William at the walk and contributing to that total was a group of 6th grade girls who call themselves "Friends Helping Friends" who were happy to join their buddy and make a difference for SMA.

Birthday Fundraisers

William also had two friends host two separate 10th birthday parties on the same day who both asked to receive donations to

FSMA instead of presents. These special 4th graders are Madison Colleran and Joey Romanowski, who selflessly gave up gifts to help their pal William, and others like him, have a brighter future. Joey has done this for three years in a row. Way to go Madison and Joey!

*Heidi Johnson
Medfield, MA*

8th Annual Wishes for William Super Scrapbooking Sunday

Our 8th Annual Wishes for William Super Scrapbooking Sunday in honor of William Johnson raised \$1,177.

This year more than any other year we were so happy to be able to continue to put this event on. Everyone that participated enjoyed the day and benefit knowing they were helping to raise awareness for Families of SMA. It is a small amount toward a huge goal, and as always, we hope next year will be even more successful.

Sincerely,

*Micaela and Jay Stanley
Medfield, MA*

Paul's Birthday Fundraiser

On March 27th, 2012 Paul Osborn turned four years old! His parents, David and Amy, set up a birthday gift registry in his name. Paul's family and friends donated over \$1,330

to Families of SMA in Paul's honor! Thank for helping us raise funds to find a treatment and cure for SMA!

*David and Amy Osborn
Cherry Valley, MA*

Rhode Island**ZogSports Fundraiser**

We are honored, humbled, and in awe (we didn't start out very well) to have won the ZogSports Fall 2011 Bowling Championship. Our team came together to have fun after work and to spend time with one another. Then when we were asked to choose a charity that we could donate to if we won – we all thought of our friend, Ian and our drive to win took on another meaning. Ian was diagnosed at the age of 14 with Spinal Muscular Atrophy. Although we know that life has been made harder for Ian – he doesn't let SMA get in his way. He has graduated from Georgetown University for both his undergraduate studies as well as Law School. He also continuously impresses us with his musical talent. However, it is Ian's perspective on life (he is a half glass full kind of person), which makes him an incredibly supportive friend and inspiration. Therefore, if we can help the Ian's of the world – we are honored and humbled.

Thanks,

Deb

Thank you to the Foot Ballers and Blame is on the Lane teams and Zog Sports for your contributions totaling \$600!



NEW ENGLAND (cont.)

Race in Honor of Victoria Meneghini

On February 11th, 2012 Philip Meneghini ran a race in Massachusetts in memory of his sister, Victoria. Many thanks to Philip for raising awareness and \$200 for Families of SMA!

Vermont**Ski Away SMA in Memory of Hailey Mae Smelser**

Dear Families of SMA,

Taylor and I are thrilled to be sending you a check for **\$18,000** that was raised at this year's Ski Away SMA event at Magic Mountain in Londonderry, VT. We were very lucky to have so many turn out for the event, because Mother Nature really did not cooperate all year in the way

of snow! We had a great time, and are so thankful for the many friends and family who have supported us year after year.

We are also so amazed at the advancements in research and the growing awareness of SMA. So much has been accomplished since we were blessed with Hailey's presence for four months and eleven days in August of 2002. We are so grateful the many beautiful faces of SMA have your voice and leadership fighting the battle for them.

All the best,

Marie and Taylor Smelser
Hailey Mae Foundation
Armonk, NY

NEW MEXICO

Flying Star Dinner Night

On Tuesday, January 31st, 2012, Flying Star Café in Albuquerque, NM hosted a fundraiser in support of Families of SMA. Flying Star donated 10% of the evening's sales to raise funds and promote awareness of SMA. Members of the local FSMA chapter were on site to share information and eat for the cause. The evening was a delicious success, raising over \$360 in just four hours! Thank you to Talia Sierra for all of your hard work in organizing this event!

NORTHERN CALIFORNIA

Pathways of Promise

The Pathways of Promise held its 4th Annual event, A Night in Miracle-Ville, on Saturday, March 31st, 2012 in Sacramento, CA. Almost **\$18,000** was raised at the event thanks to generous sponsors and donors. The fun filled night included gourmet food and wine, dancing, and both a live and silent auction. This spectacular event was in held for all those living with SMA and in honor of Luke and Meagan McWilliams. Pathways of Promise is a small, but dedicated, group who came together to contribute to their vision of raising awareness and

finding a cure for SMA. Thanks to the hard work and dedication to the Pathways of Promise committee, their annual event has raised over \$90,000 in their quest to find a cure for SMA! Thank you to Glenn and Colleen McWilliams, Meri Stratton-Phelps, Carrie Manriquez, and all of the other volunteers that make this event a success year after year!



NORTHERN CALIFORNIA

Concert for a Cure

The 12th Annual Concert for a Cure held May 12th, 2012 at the Blackhawk Country Club in Danville, CA, was a great success! Thanks to the support and generosity of friends, families, and the local community, the event raised nearly **\$124,000** this year. This year's theme was "Catch a Cure" and 12 years of miracles were celebrated at this milestone event.

Mary and Joe McHale founded the Concert for a Cure in 2001 shortly after the diagnosis of their son, Danny, with SMA Type II. Nancy and Andris Dindzans joined the efforts shortly thereafter when their daughter, Ariana, was diagnosed with SMA Type III. Both Danny and Ariana will turn 12 years old this year. The event has grown exponentially from its humble beginnings – it raised \$7,500 in its first year – **and during this, its 12th year, the event surpassed \$1 million raised for SMA research.**

Each year our volunteer team awards an individual, business or organization that has demonstrated exceptional dedication and support to make Concert for a Cure a success with the Shining Star Award. This year we honored **St. Joan of Arc Ministries** for their 12 years of unwavering commitment and support. Since 2001, the organization has supported our event and the mission of Families of SMA. They have acted as partners in the fight against SMA through financial sponsorship, personal donations, auction items, volunteering of their time and strong participation each year. Please join us in thanking the St. Joan of Arc Ministries for all of their support.

This amazing milestone would never have been reached without the enthusiasm, hard work and dedication of our volunteers. Thank you to Kate Aveson, Anna Calderon, Andris Dindzans, Nancy Dindzans, Julie Doris, Kristen Johnson, Mary McHale, Valerie Phelps, Jean Sarver, Raelene Sprague and Mary Wheeler.

We could not have reached our \$1 million goal without the generous sponsorships from families and local businesses. Thank you to the event sponsors who supported us this year: The Aveson Family, Fidelity National Home Warranty, St. Joan of Arc Ministries, McHale Engineering, All Automotive of San Ramon, Eden Jewelry and Loan, Empire Realty Associates, Piatti Ristorante & Bar, Scott Design, Team Logic IT, True Wireless, and Alpha Graphics.

The McHale and Dindzans families have worked tirelessly to raise funds that have advanced research efforts to develop a treatment and cure for the number one genetic killer of infants. The SMA community wishes to honor their remarkable efforts and the unbreakable spirit of their staunch supporters.



OKI (OHIO, KENTUCKY AND INDIANA)

Ohio

9th Annual OKI Chapter Walk-n-Roll



On May 5th, 2012 the OKI Chapter held the 9th Annual Walk-n-Roll at the Cintas Center in Norwood, OH. Even the odd rain sprinkle would not dampen the spirits as our OKI SMA families came together to raise almost **\$90,000**. A great time was had by the approximate 560 attendees with carnival games, face painting, PB & J sandwiches and amazing raffle prizes. Our SMA heroes took the stage to chat with Honorary Chairperson Jen Dalton of Local 12 and a special butterfly release brought a tear or two in honor of our angels. Time spent with our SMA family is as always...priceless. A special thanks to Elizabeth and Beth Lockwood and everyone who helped organize this event! Thank you to raffle chair persons Gloria Logeman and Marilyn Pater for hosting a garage sale to benefit the walk. Thanks to their efforts \$1,500 was raised!

14th Flying Pig Marathon



In the early morning hours of May 6th, 2012 several OKI runners laced up their shoes to take part in the Cincinnati Flying Pig Marathon. Some ran the whole 26.2 miles, while others completed the half marathon and four team relay. They were cheered on by some of our OKI members lovingly referred

to as squealers! We are proud of all our runners for FSMA who raised over \$7,000, but have to give a special shout out to Elizabeth Lockwood, grandmother of Emma and Nick Lockwood who ran the last leg of the relay staying strong across the finish line with her teammates that included sons Kevin and Rob and half marathon runner daughter-in-laws Beth and Ann.

Olivia and Caleb's Sunday Funday



On July 8th, 2012 Olivia and Caleb's Sunday Funday was held at Emerald Lake in Norton, OH was a huge success! It was a gorgeous 80 degree, sunny day. We had 230 guests and volunteers having fun in the sun! With all the love and support from all of our family and friends, we raised **\$10,000** in honor of Olivia and Caleb Burgess, who both have SMA Type III. As a parent, I want to help. I don't want my children, or any other children, to suffer any longer. The least I could do as a single mom of two with SMA was spread awareness and raise money to help find a treatment and a cure! Our event could not have been more perfect. Our water ski show was a hit, everyone generously bidded on our 95 auction items, and the cornhole tournament was a lot of fun!

Julie Burgess
Norton, OH



Families of SMA is honored to have received a total of \$715 in honor of Caitlin Brown's, SMA Type I, graduation from Northern Kentucky University. Congratulations on all of your success, Caitlin!

Kentucky

Northern Kentucky University Open Mic Night



My name is Holly Ritchie and I am a member of Phi Sigma Pi – Epsilon Rho at Northern Kentucky University with Caitlin Brown. As you may have heard from Caitlin, last month our Fraternity held an Open Mic event for the benefit of SMA. For this reason, we are donating all of the proceeds from the event to Families of SMA. In total, our event raised \$222. We hope that this little bit of money is able to help you.

Being in Phi Sigma Pi with Catie, as well as being her friend, has made me really appreciate what those with SMA have to overcome on a day to day basis. This being said, I am very happy that I was able to take part in the open mic and raise a little more money for the cause. I am not the only one who feels this way as many members of Phi Sigma Pi – Epsilon Rho feel the same, and because of this we have made Families of SMA our local philanthropy.

I, and everyone in Phi Sigma Pi – Epsilon Rho, look forward to continuing to help raise money to find a cure.

Sincerely,

Holly Ritchie, Service Committee Chair
Epsilon Rho Chapter of Phi Sigma Pi
Newport, KY

OKI (cont.)

3rd Annual Crop 4 Annie

Dear FSMA,

Our 3rd Annual Crop 4 Annie was held April 21st, 2012. We are honored to be donating \$2,334 in Annie Grimes' memory. We look forward to continuing this event in 2013.

Sincerely,

*The Grimes Family
Eddy, Nicole, Garrett, Lainey, Emi and Annie
Bowling Green, KY*

Remke Bigg's Markets Sixth Sense Collection Bins

It is our pleasure to award you with the earnings from the Sixth Sense collection bin in our store. As you know, this program is one that rewards customers who use recyclable bags for their grocery purchases by giving them 6¢, as well as a 6¢ token to place in your bin. Our hope is that it also encourages everyone to consider protecting our environment while enhancing contributions to our community, and specifically to your organization.

We are happy to make a contribution of \$99 to your organization, and we appreciate the good that you provide to our community! Thank you for allowing us to serve your needs. We appreciate your business!

Sincerely,

*Mark Sander, Store Director
Erlanger, KY*



Holy Trinity Lutheran School Chapel Offerings

The students and faculty of Holy Trinity Lutheran School collected \$68 during our weekly chapel offering on March 24th, 2012. It is our hope that it can be used to help someone in our community.

*Bill Hiskey, Director of School Ministries
Bowling Green, KY*

Indiana

Putnamville Correctional Facility Dinner Proceeds

On February 2nd, 2012, the Putnamville Correctional Facility in Greencastle, IN donated a portion of the proceeds from visitor dining. The event raised \$500 in honor of Zion Hoskins!

Home Laureates Homemaker's Club Holiday Candies and Nuts Sales

The Home Laureates Extension Homemakers Club each year sells holiday candies and nuts. All proceeds raised are given to non-profit groups. This year the club voted to donate \$100 to Families of SMA in honor of both Katie and Betsy Swetnam.

Best Regards,

*Jackie Johnston, Treasurer
Muncie, IN*

PACIFIC NORTHWEST

Chapter Update

On April 29th, 2012 our families gathered for a potluck dinner and meeting. The kids had a great time blowing bubbles and drawing while the adults chatted and shared information. It is always great to reconnect with our SMA family and welcome new friends at these fun gatherings.



Washington

4th Annual SMA Day with the Seattle Mariners



On June 16th, 2012 the Pacific Northwest Chapter hosted the 4th Annual SMA Day with the Seattle Mariners in loving memory of Gabrielle Stack. The Seattle Mariners vs. San Francisco Giants baseball game brought together families and friends and raised \$814 for Families of SMA!

PENNSYLVANIA

Greetings from the Pennsylvania Chapter!

We are so proud of the work of our Chapter families this year. In 2012, our members prioritized the following goals: advancing SMA awareness, raising necessary funds for a cure, but most important...lending a hand to a fellow SMA friend.

Please take a look at what our amazing families have achieved these past six months!

TOGETHER WE WILL FIND A CURE!

Libby Chapin's Legacy Extends to Many SMA Families

On January 8th, 2012, the SMA Community lost a special woman. Elizabeth "Libby" Chapin lost her battle to SMA at age 56 years old. She was a pillar of strength and a positive force...dearly missed by her family and friends. Libby's family reached out to the PA Chapter with a generosity unmatched in our nine years of existence.



In Libby's name, her sister and brother-in-law, Meg Etherington and Brian Moores, donated numerous items to SMA families including: a handicap accessible van, two wheel chairs, countless pieces of equipment and medical supplies. Although much of the equipment has been donated, we still have some items available for donation. Please contact Karen McRory-Negrin, Chapter President, with any questions at pennsylvania@fsma.org.

"New Hope's Got Talent" Spreads SMA Awareness



Dominique Daniela extends her generosity once again by sponsoring an event to support the New Hope Community's Families of SMA. Honoring Jake Saxton and Matthew Reilly, her business (www.dominiquedaniela.com) along with "Dance Junction" of Peddler's Village, scouted out the talent on March 4th, 2012!

Devyn Rush from American Idol and many rising stars showcased their talents by singing, dancing, and joke telling for a good cause! There was the fantastic sound of DJ Jim DiTulio, great food, and desired Raffle items for all! Thanks so much Dominique for all you do!

Sweet Baby Zane Fights SMA

Bill Brazer Benefit - "A Helping Hand for our Kids"

On Friday, March 9th, Sweet Baby Zane Foundation was one of two charitable organizations chosen as recipients for the event's donations. It was a fun and casual evening featuring the music of Rugby Road with special guests, Bob Lowery and GT Corrigan. Guests met at The Great American Pub in Conshohocken, PA and enjoyed a delicious dinner buffet, 50/50, and raffle.

SBZ donated a portion of the funds raised to the PA Chapter of Families of SMA...and for that, we are very grateful. Thanks Hillary and Keith! And a big welcome to your beautiful twin boys, Brennen and Braxton!

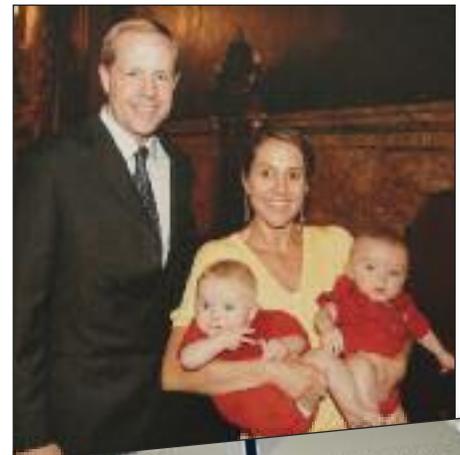
H.R. 768: August 2012 — SMA Awareness Month in PA

Hillary Schmid shares:

"On June 11th, 2012, my parents, Brennen and Braxton, and I traveled to Harrisburg, our state capitol. I had been working with our local state representative, Duane Milne, in getting a Resolution passed. We got to see it in action last week. Representative Milne spoke about SMA, Zane and our family. We are happy to say that H.R. 768 was passed declaring August 2012

Spinal Muscular Atrophy Awareness month in the state of Pennsylvania. Ironically, we received a copy of the official declaration and two pictures of Duane, the boys and I in the mail today, on Zane's Angel day."

Here's a short link of Representative Milne speaking: www.youtube.com/watch?v=SQ7awiaD47w&feature=youtube_gdata_player



Jocelyn Paige Lee Foundation

The JPL Foundation would like to thank all who came out for their 5th Annual Benefit Dinner on March 31st, 2012. This was a very difficult event as it was:

"The first since our borrowed angel Jocelyn has returned home to the Lord. Jocelyn has touched many people over the years and we have been able to raise both awareness as well

PENNSYLVANIA (cont.)

as funds to help find a cure. This year we raised over \$6,000!” said Derek Schmidt.

Derek and Nicole Schmidt, along with Jocelyn’s parents Jennifer and Shane Lee stood strong as hosts on this emotional night at the Saint Elizabeth Ann Seton Roman Catholic Church in Bear, DE. Approximately 200 people attended and enjoyed great music, great food, a silent auction, and raffle. Proceeds of the event were donated to the PA Chapter of Families of SMA.



Lukie’s Tigers host Lukie’s Spring Yard Sale

Thank you to everyone who came out to support Lukie’s Spring Yard Sale on April 28th, 2012. It was a big success! We raised \$515 for the FSMA PA Chapter Walk Team, Lukie’s Tigers! Thank you Washington Pet for hosting us and a special shout out to Pam from Washington Pet, who raised \$55 trimming dog’s nails. Thank you to the Lukie Crew who is always there to do anything they can to help find a cure for SMA!

Little Flower Violet

On Sunday, April 29th, 2012, Dining Out Philadelphia partnered with Little Flower Violet and hosted a Cover Party and Runway Fashion Show with a portion of proceeds benefitting the PA Chapter of FSMA in Philadelphia, PA!

Along with drink specials, complimentary hors d’oeuvres and music by Schaffer Sound Productions, the exquisite ambiance of Del Frisco’s Steak House was the perfect setting for a first class event! Dining Out Philadelphia offered the Chapter an opportunity to honor the memory of Violet Wehrkamp, while also educating the large crowd about the devastating effects of SMA. We raised \$25!

Chris Cooter and Calvary Riders Support Our Cause!

Chris Cooter, of Calvary Motorcycle Riders, and friend to the MacDougall Family, has been mobilizing support to fight SMA! For seven years now, he has organized the “SMA Motorcycle Ride to Fight SMA” in Horsham, PA.

On May 12th, 2012, Chris Cooter hosted the 7th Annual event. Over 50 bikers from all over the Delaware Valley kicked off the event in Horsham, PA. They enjoyed a beautiful motorcycle ride through Bucks County, PA while raising over \$2,200 to cure SMA! Bikers and non-bikers alike were invited to enjoy this great event. The day included: a ride patch, a pin, great food, and a Raffle. Look for information on next year’s event. Same time, same place.

Livia’s Lemonade Stand

In honor of her cousin, Abigail Negrin, Livia Leonetti decided to contribute to Abby’s Army Walk-n-Roll Team by raising her own

donations! She spent the day before the annual walk selling lemonade in Absecon, NJ and spreading the word about SMA! Great job Livia! And CONGRATULATIONS for raising \$31 to go toward research to cure SMA!

PA Chapter’s 9th Annual Walk-n-Roll to Cure SMA

The 9th Annual PA Chapter Walk-n-Roll held on Sunday, May 20th, 2012 in Philadelphia, PA was a big success! Hundreds of “walkers and rollers” turned out for a beautiful day of family fun, including a petting zoo, games, and a balloon center donated by PA member Laurie Peters.



For those looking to shop, PA member Christina Mohammed sold her beautiful hand crafted jewelry . . . not to mention, an overflowing raffle organized by Barbara Perre, grandmother to Peter Henkel. This year, we raised \$103,308! A huge thanks to all our corporate sponsors and volunteers!



PENNSYLVANIA (cont.)

We were grateful to have joining us:

Philadelphia's Mayor Michael Nutter, ABC Action News Reporter Walter Perez, Deputy Mayor and Health Commissioner Don Schwarz, FSMA President Kenneth Hobby, and national sponsors BAYADA Home Healthcare and Quest Diagnostics . . . all there to support our wonderful PA families!



Many thanks to our 27 amazing SMA Teams:

Abby's Army, Aviv's Fan Club, Captain Morgan Ahoy, Charlotte's Team, CHOP Team, Fighting the Fight, Hope for Haydon Jack Freedman's Team, Jake's Team, JPL Foundation, Julia Feinburg's Team, Little Flower Violet, Lukie's Tigers, Lydia Love Bug, Matthew Reilly's Crew, Omar's Soldiers, Sweet Baby Zane, Splendiferous Schuylkill Saunterers, Team BAYADA, Team Clayton, Peter's Philadelphia Eagles, Team Ellixson, Team Libby, Team Lyla, Team Matthew, Team Syrea, and Team Quest Diagnostics.



Bat Mitzvah Generosity

Anya Lesser donated a generous portion of her gift money from her Bat Mitzvah to Families of SMA in honor of the memory of Abigail Negrin.

What was especially remarkable about this mitzvah was that Anya never knew Abby, but donated solely because her friend May Naish, who shared her own special relationship with Abby Negrin. Abby's mom and PA Chapter President, Karen McRory-Negrin, is so grateful for such thoughtful people!



Jillian Hazel is Spreading the Word at School!

In honor of her cousin, Matthew Freitas, Jillian Hazel decided that she wanted to do something to fight SMA. She decided to engage the students at her school, St. Anastasia Church School in Newtown Square, PA, and started selling FSMA bracelets at lunch to her classmates for a week.

The school community then got further involved and hosted a Zumba party where students made donations to attend. In total, Jillian raised \$500!!

"Shoot for a Cure"

Andy Park and Erin Conner-Park, parents of SMA Angel Tiernan Conner-Park would like to thank everyone who has helped make this year's "Shoot For a Cure" a great success.

"This year we managed to raise \$2,400 to help fund a cure for SMA. We need to thank the following companies and groups for their help:

All Season Trap and Skeet — We cannot thank enough the Club Officers and members who attended the event and helped with the shooters. We also want to extend thanks to All Season for allowing us to use their great range.

Ruger Firearms — They graciously donated five Carhart Jackets for our shooters and side raffles.

Fat Boy Game Calls — Doug donated four beautiful handmade box turkey calls. He once again outdid himself with these wonderful calls.

L. B. Toneys Alamo Gun Shop — Mike has helped with the gun tickets for the past three years.

Ram Septic — Every year Ram supports our event with generosity.

Larry and Amy Way — With amazing skill and dedication, they managed to solicit over \$1000.00 in donations. Thank you. We would like to recognize The Independent Political & Social Club, The Swedish American Club, and The Sandy Hose Company No. 1 for these donations.

Special thanks to:

Josette Harbison, Stacy Park, Kelly Park, Tom Dinger, Sue Toney, Tom Schaffer, Greg Dixon, Steve Meholic, Mark Anthony, Jim Petley,

Adam Hollobaugh, Todd Brown, Phil Kuntz, Randy Tyler, Polly Cleaver, Lisa Schaffer, Dave Larko, Diane Chicola, Virginia Conner, Jim Conner, James Park, and Dave Harbison.

Finally, we would like to thank everyone who helped sell tickets, setup for the shoot, clean up after the shoot, and who assisted with the shooter registration and raffles.

Thank you ALL very much! We hope to see you next year."

Sincerely,

*Andrew Park and Erin Conner-Park
Dubois, PA*

Huge thanks to all our Families and Friends that work tirelessly in support of our mission to CURE SMA!

*In Friendship,
The PA Chapter of FSMA*

Memorial Dinner in Honor of Emily Coddington



Dear FSMA,

Our beautiful sweet daughter Emily Coddington (SMA Type I) would have celebrated her 3rd birthday on May 4th, 2012. \$200 was given in honor of her birthday by guests and the memorial dinner we had on her birthday. Thanks to the FSMA community for all you do!

*The Coddington Family –
Terry, Julie, Sarah and Ryan
Uniontown, PA*

Junie's Jewelry 2 Cure SMA

Thank you to Christina Mohammed for donating the proceeds for Junie's Jewelry to Cure SMA to Families of SMA. Your generosity raised \$233!

Sincerely,

*Stephanie Haegley
Philadelphia, PA*

ROCKY MOUNTAIN

Colorado

11th Annual Evening of Hope Gala



dancing. Jason Luber of KMGH-TV was the Master of Ceremonies. Jason is seen daily on 7News and ABC's Good Morning America reporting traffic conditions on the Denver metro area's busy roads. Jason was a great addition to the evening. He was light-hearted, funny, and most importantly, very interested in helping raise awareness for SMA.

The popular Chris Daniels & the Kings provided a mix of funk and soul to the evening with their seven piece band. Chris Daniels was amazing with the SMA kids, and he was so touched by their zest for life and love of music, that he provided several autographed copies to those who continued to occupy the dance floor — wheelchairs and all!

Mid-evening, Jason Luber interviewed three families - all of who were gracious enough to share their stories about how SMA has impacted their lives. Nicole and Brandon

Shaklee shared details about how Brandon's (SMA Type III) life has changed since being diagnosed. Brandon continues to thrive, though hitting a few roadblocks, and is now an active driver on the roads of Denver! Sherri and Katerina Casas spoke from a Type II perspective. Though wheelchair bound, Katerina has been a team manager and statistician for many of her high school's sports teams and will be attending college this fall at a Colorado University. Finally, Lee and Marla Marlow shared the story of their son, Jay, who lived seven months to the day with Type I.

Though Jay's life was brief, the impact of his struggles continues to drive Lee and Marla along with their family and friends to raise awareness in his memory.

The silent auction consisted of over 40 items ranging from restaurant gift cards, to a \$500 Aveda products basket and an \$800 golf package. Beside the silent auction, the Boxes of Hope raffle items were also a big part of our evening's fundraising efforts. We managed to sell 200 boxes for \$10 each. The

winners took home a 32" flat screen TV, a Kindle, a Keurig coffee maker, Chris Daniels & the Kings CDs and DVDs, movie passes, Comedy Works passes and various gift cards. A big thank you to our SMA kiddos for selling these to the audience through out the evening!

In this evening filled with fun, entertainment, family and friends, we were able to raise over **\$24,000** for Families of SMA. We are very proud of this accomplishment, and most importantly, knowing that we raised awareness of SMA. Thank you, FSMA, for your support of our efforts and for helping us each step of the way!

*Marla Marlow,
2012 Evening of Hope Chairperson
Mom to SMA Angel, Jay Randall Marlow
(1/25 - 8/25/2008)
Highlands Ranch, CO*

9th Annual Rocky Mountain Golf Tournament

The 9th Annual Rocky Mountain Golf Tournament took place on Monday, May 21st, 2012 at the Pradera Golf Club in Parker, CO. We had a wonderful turnout once again and the weather was terrific - the very first tournament that we have had exceptional weather all day!

For the first time ever, we had a Hole-in-One winner on one of the Par 3 Holes. The winner was John Copeland of Denver. John was also a member of the tournament's winning team along with Michael Borgelt, Matt Miler and Steve Atherton. Congratulations to our winners!

We really appreciate the loyal support of our golfers that return year after year to support this event and we are always delighted to welcome our new players! Because of this tremendous support, Martin and I are able to keep planning the event and send the proceeds to FSMA! This year we were able to raise almost \$20,000 for Families of SMA!

Thank you to Gillian and Martin Faith for all of your hard work and dedication in planning this event once again.



The 11th Annual Evening of Hope Gala for SMA was held on Saturday, March 10th, 2012 at the Denver Merchandise Mart in Denver, CO. The event was a collaboration of efforts put forth by our Rocky Mountain Chapter volunteers - SMA mothers, grandmothers, aunts and friends: Marla Marlow, Joy Spellman, Nicole Shaklee, Gillian Faith, Marynell Larson, Janet Mulay and Sherri Casas.

The evening consisted of hors d'oeuvres, dinner, a silent auction, family interviews, live music and

SOUTH FLORIDA

South Florida Chapter Coin Can Fundraiser

Thank you to Jennifer Miller Smith and the South Florida Chapter for putting coin cans in local businesses. Your efforts raised awareness in your local community and \$237 for Families of SMA!

Jacob's Run Walk & Roll to Cure SMA Hits \$1 Million Milestone for FSMA

Jacob's Run, Walk & Roll was held this year on April 1st, 2012 in Boca Raton, Florida. At this landmark event that has now become a staple to local south Florida residents, the Jacob Isaac Rappoport Foundation officially reached a total cumulative fundraising milestone of \$1 Million for Spinal Muscular Atrophy (SMA) research and support.

The Jacob Isaac Rappoport Foundation was created in memory of Jacob, Shaina and Adi Rappoport's son and shooting star, who had SMA Type I. Jacob was born December 27th, 2001 and was an alert and happy baby. When he was nearly four months old, Jacob was diagnosed with Spinal Muscular Atrophy. Soon after Jacob's diagnosis, Adi and Shaina quickly connected with Families of SMA and many other SMA families. Jacob passed away on October 1st, 2002 at the age of nine months. Through Jacob's Run, Walk & Roll, The Foundation's main fundraising event, his memory lives on in the hearts of hundreds of family members, friends, colleagues and local SMA community members.

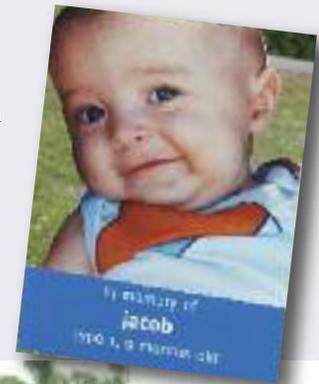
In nine years, The Jacob Isaac Rappoport Foundation has made a tremendous impact on the entire SMA community. With the \$1 Million that they have raised, The Foundation has supported many diverse programs, including:

- Care Packages for newly diagnosed SMA families.
- Multiple basic research awards which led to breakthrough discoveries on the genetic cause of SMA.
- New drug discovery programs, including the first ever new SMA drug program to be approved to begin clinical trials.

The Jacob Isaac Rappoport Foundation and Shaina & Adi Rappoport

Families of SMA is extremely grateful for the continued support from the Jacob Isaac Rappoport Foundation. For the past 11 years Shaina & Adi and their foundation have helped to provide a wonderful opportunity for Type I families, at each SMA Conference, to come together for a Type I Lunch. Shaina and Adi also sponsor all Newly Diagnosed Type I Care Packages that are sent out to any newly diagnosed Type I family who contacts Families of SMA. These care packages are filled with great, helpful ideas for newly diagnosed families. Their support extended to this year's conference in Minnesota, as they have helped 100 Newly Diagnosed individuals attend the conference through their \$13,000 sponsorship.

Families of SMA would like to extend a heartfelt thank you to Shaina and Adi Rappoport and The Jacob Isaac Rappoport Foundation for all of the money they have raised over the years; the dinners they have sent; the cleaning services and packages of toys they have sent to hundreds of SMA families; the scholarships they have provided for families to attend SMA conferences; the luncheons they have hosted; and the countless hours they have spent lending emotional support to parents of newly diagnosed children.



SOUTH JERSEY/DELAWARE

If you would like to plan an event, the chapter would love to hear from and support you. Please visit www.fsma.org/southjersey to see all of the local events. We welcome all area families so please contact Jessica Moyer at (866) 774-9533 or email jnjmoyer@comcast.net.

We wish you a healthy, happy Fall!

Thank you to Jessica Moyer and the entire South Jersey/Delaware Chapter for all of your hard work in planning so many successful events and for spreading much needed SMA awareness in your area!

Delaware

5th Annual Steven's Swing for a Cure Golf Classic

On April 27th, 2012, Jessica and Jason Moyer hosted the 5th Annual Steven's Swing for a Cure Golf Classic in memory of Steven Moyer at Jonathan's Landing in Magnolia, DE and raised over **\$26,000**. A big thank you to Joan Smith and Justine Nichols who created and donated many of the prizes. Also a sincere thanks to Matt Corozzi for once again helping us out! We had over 85 golfers and many generous sponsors!

Delaware Marathon

The Delaware Marathon in Wilmington, DE was held on May 13th, 2012. Team Families of SMA had five teams run the marathon relay, one half marathoner, Lauri Brown, who ran in honor of her son Max, and four full marathoners- Jason Moyer, Jessica Moyer, Nate Robinson and Phil Wandless who ran in memory of Steven Moyer. Thank you to the following teams: Jeanette Hammon and the Delaware Downstate Striders, John Cheslock and Team in honor of Eden Cheslock, Ron and Lisa Smith and Justine and Ben Nichols in memory of Steven Moyer, and the two teams in memory of PJ Desroches. With everyone's support we raised over **\$17,000!!** We plan on participating in this event next year with an even bigger turnout!

*Jessica Moyer
Magnolia, DE*

New Jersey

7th Annual Steven's Walk to Drum Out SMA

On May 19th, 2012 the Potter family hosted Steven's Walk to Drum out SMA in honor of Steven Potter, SMA Type III, in Haddon Township, NJ. They had an amazing turnout and raised over **\$27,000!** At the walk, there was live music, food, special guest appearances and Chinese auction baskets. Participants started off with a stretching exercise to live music to get pumped and then

Steven led the gang on the 2.2 mile walk. It was a really fun day for family, friends and even the pet dog! Thank you to Theresa and Michael Potter for planning this hugely successful event.

5th Annual Walk-n-Roll to Cure SMA in Memory of Katherine Santiago



The 5th Annual Walk-n-Roll to Cure SMA in memory Katherine Santiago was held on May 12th, 2012 in Milburn, New Jersey. Katherine Santiago was a bright, beautiful and courageous girl. She and my daughter, Allie Mazzella, met in the first grade. They shared a love of arts and crafts, playing with dolls and watching movies. Katherine was a huge Harry Potter fan and convinced Allie to start reading the Harry Potter series (which she too became passionate about). Katherine had an amazing smile and infectious laugh. Although Katherine was in a wheelchair, she didn't let that stop her from having fun.

After Katherine passed away, Allie really wanted to do something to remember her wonderful friend. It wasn't until after Katherine passed away, that we learned Katherine had SMA. We had never asked why she was unable to walk; I think we felt like we would be prying. Allie was only 13 years old when she along with the Santiago Family (including Katherine's little sister Julia) began

organizing the first Walk-n-Roll. Our hope at the time was to get a few people who had known and loved Katherine to join us and maybe raise a little money. Friends, neighbors, teachers, Julia's Girl Scout troop, Allie's family from out of town – all arrived on a sunny day in May to walk, roll and honor Katherine.

Also in attendance at that first walk (and every year since) was a girl named Darcy Trinco who has SMA. One of her teachers had read about the walk online. Since they lived only a few towns away, Darcy, her family, and several teachers decided to join us. Meeting Darcy was inspiring for Allie and Julia. It was then that they realized the walk would not only honor Katherine but it would also raise money and awareness to help other kids with SMA, including their new friend Darcy.

This year, the event has raised over **\$13,000**. We are truly awed that a small idea could turn into something so big.

*Allie, Julia, the Mazella Family and the Santiago Family
Milburn, NJ*

Appetite for a Cure in Memory of Aleah

Hello Family and Friends of SMA,

On November 26th, 2007 I lost my second daughter, Aleah, to SMA. In 2009 we held our first dinner benefit, Appetite for a Cure, at our family owned restaurant. With



SOUTH JERSEY/DELAWARE (cont.)

about 100 guests and only two weeks of planning, we proudly raised \$3,000.

This year I wanted to go bigger. My dear friend, Mike Blandina, helped me secure Hemingway's Café in Seaside Heights, NJ as our venue, we secured many sponsors and spread the word about our event on social media. An hour before the event even started we had made \$5,400. It was such a great feeling! We ended up raising nearly \$9,000.



I cannot thank our sponsors, participants, and donors enough. The evening was a huge success and this benefit has been so satisfying as well as rewarding to me. I am not able to buy my baby girl a birthday or Christmas present so this is what I like to do to celebrate her short-lived life. For it to be as successful as it has been, I feel truly blessed. Thank you everyone

for all your support and love. Without it this event never would have been possible. Thank you Families of SMA for always being such an inspiration.

Sincerely,

*Victoria and Michael Durazzo
Toms River, NJ*

Children Helping Children Trike-a-thon

The Children Helping Children Trike-A-Thon was held on June 2nd, 2012 at the Jack in the Box Learning Center in Alpine, NJ in honor of Laura Nellen. This event raised over \$5,000. Many thanks to Laura for being the inspiration for such a wonderful event and Maria Lignos for all of her planning!

Best Meatball in Secaucus in Memory of Daniel Cevallos



On Sunday April 29th, 2012 in Secaucus, NJ the Secaucus Knights of Columbus Council 12769 hosted its 1st Annual Best Meatball in Secaucus Contest to benefit Families of SMA in memory of Daniel A. Cevallos. Nineteen contestants put their best meatballs forward to win one of our three great prizes including a Meatball Trophy and an American Express gift card. The competition was fierce, and the meatballs were of a great variety in taste and style. Our first judges were the people who attended. Everyone in the auditorium placed their ballots for their favorites and we paired it down to just four contestants. Our panel of four judges tasted each of the four

preliminary winner's meatballs and put down their vote based on taste, texture, appearance and originality. After all voting was completed we had our winner, Doreen Rutter! Big thanks to everyone that came out and participated in this wonderful event. It was your support that helped us raise \$2,500 for Families of Spinal Muscular Atrophy!

*Paulina Recalde
Secaucus, NJ*

Denim Day Fundraiser



Dear Families of SMA,

Lanes Mill Sunshine is happy to contribute to the Families of SMA. We raised \$250 in support of Salvatore Morrongiello, the son of a Lanes Mill Elementary School staff member. Our group recognizes all the wonderful things you do to treat and possibly cure SMA. We hope that this small donation will help to continue your dedication to fund cutting-edge research and service all families throughout your community.

Sincerely,

*Shannon Scott
Brick, NJ*

SOUTHERN CALIFORNIA

Dine for SMA at Hooters

On Fridays in April 2012 Southern California Hooters restaurants donated a select portion of their proceeds to Families of SMA. In total the proceeds added up to \$120! Thank you to all of the Hooters diners and Rickk and Autumn Montoya for your support!

TENNESSEE

8th Annual FSMA Charity Golf Classic

The 8th Annual FSMA Charity Golf Classic held in Knoxville, TN was a huge success. With the help of an awesome committee, a very organized TN Chapter president Sarah Boggess, and our wonderful sponsors, we raised over **\$23,000** for SMA research. Our event was held in April at Gettysvue Country Club. We had about 20 teams participate in a four person scramble. Extra funds were raised during the tournament from the Happy Gilmore putting contest, silent auction, gambler's raffle, and a TV board, where we sold squares and picked a winner for a big flat screen. A big thank you to Papa Murphy and Jack Stalker for donating all the awesome pizza for our golfers and volunteers!! Eagle Distributing of Knoxville donated all the beer, thanks Jeremy! A special thanks to Lise and Jack Murphy, Dorri Boggess, Carolyn Monkus, Sandy Hedberg, Appalachian Underwriters, Sports Animal talk radio, all our volunteers and the Gettysvue CC staff. We are looking forward to our 9th annual tournament at Gettysvue in May of 2013!

Jellico High School Rubber Duck Race

The Jellico High School Student Council held its 2nd Annual FSMA Duck Race. Jellico High is a small school located in Jellico, TN. In honor of a present member, Casey Douglas, the student council decided to adopt FSMA as their fundraising charity. The council realized that although everyone in town knew and loved Casey, no one really knew anything about the disease that she has had her whole life. The council member sold duck race chances and bracelets, raising \$1,800 for FSMA. Thank you to Casey Douglas, the Jellico High School Student Council, and Barbara Carter, student council advisor, for all of your hard work and dedication!

Tennessee Chapter "Living Room" Concert

On April 22nd, 2012 we held our first Living Room Concert, raising over \$1,200. Lise and Jack Murphy hosted the event at their home in Knoxville, TN. All Occasion Catering donated a delicious assortment of entrees.

Thank you to Dorri, Neal and Artie for taking time from your Sunday evening to prepare and serve such a fabulous meal. After dinner, Nashville recording artist Cindy Morgan, accompanied by Joe, performed a selection of her songs. Thank you to neighborhood "sound engineer" David for the use of his equipment and for joining Cindy in an impromptu duet and to Andrew for his solo performance.



TEXAS

5th Annual Cure SMA: Run, Walk-n-Roll in Honor of Seth Gilley

The 5th annual Cure SMA: Run-Walk-n-Roll honoring our son, Seth Gilley (SMA Type II), was held May 12th, 2012 in Stephenville, TX. It was a big success, raising **\$12,922** for FSMA! The fundraiser was kicked off with a two mile fun run/walk, followed by live music from "The Backroads Brothers", a local band.

Tarleton State University therapeutic horseback riding program, TREAT, provided horseback rides and a petting zoo. Our local fire department was there with "Sparky" giving out "high-fives" at the finish line. Seth's Nana arranged a special surprise for Seth and his friends by having Spiderman and Batman attend the fundraiser! Seth's great grandma had her annual quilt raffle. Seth's big sister, Caroline, organized a lemonade stand. Face painting, popcorn, bounce houses, silent



TEXAS (cont.)

auction, and hamburger lunch were among the many other fun activities provided. "Little Man's Biker Crew" from Cleburne also helped us raise money for FSMA. The LaFountain family organized the bike run from Cleburne to Stephenville in memory of their SMA angel, Layton Bradlee LaFountain.

It was an incredible day! Thank you to all that participated and especially to the SMA families that attended. A huge "thank you" goes out to our small community of Stephenville and to our wonderfully supportive family and friends, for loving us and for continually wanting to support efforts in finding a cure for SMA.

*Chad and Lacy Gilley
Stephenville, TX*



Blake's Journey/Running for Blake



Dear FSMA,

Blake's Aunt Emily raised \$1,655 for Families of SMA. Along with family and friends she would like to help the FSMA cause. We love the kind hearts that you all have. Please know that

Blake's journey will continue to keep FSMA in our prayers and thoughts as well as our goal to spread SMA Awareness.

Love to all,

*Karen Schmidt, Grandmother
Texarkana, TX*

Baskin Robins Benefit for Baby Sage

Thank you to Jane and Dermont Vardiman for holding the Baskin Robins Benefit in honor of their daughter, Sage (SMA Type I) in Euless, TX. Thanks to their hard work and generosity \$240 was raised for Families of SMA!

Hello from the Wallis Family!

Wallis Dermatology patients have raised another \$218! We are honored to help fund the effort to raise money for Matthew Wallis and other kids needing a cure for SMA.

*The Wallis Family
Longview, TX*



UTAH

The Utah Chapter has been busy getting organized and spreading SMA Awareness. We have gained several new members and have been planning some upcoming events! If you are interested in joining the chapter or have any fundraising ideas, please e-mail us at Utah@fsma.org.

WESTERN NEW YORK

Drake Elementary School Dress Down Day Fundraiser

On February 3rd, 2012 Drake Elementary School held a dress down day to benefit Families of SMA. Thank you to all of the students and teachers that participated. Your generosity raised \$110 to help find a treatment and cure for SMA!

Mini Walk for SMA

I had originally planned the Alfred Walk for SMA as my final project for the Drawn to Diversity class I was taking. The point of the class is to learn and teach about diversity in our surrounding community. I decided I would educate the community about my disease.



While doing research for the event, I turned to the Families of SMA website. I decided to contribute the proceeds of my event to Families of SMA because of the supportive community the organization has created for people with SMA. The event would now raise awareness and celebrate diversity on my campus as well as raise money to aid another community I was a part of.

While it was not a long walk or large event several of my friends, my parents and I made the walk across campus and each donated to the cause. This walk represents the sometimes difficult walk I make every day there going from the dorm to the most distant building I need to access and back again. Some of the donations came from family and friends that could not attend my event but wanted to support my cause. We want to thank you for all that the organization does to help and support Families of SMA and hope our \$100 donation will be helpful.

*Sean Hilliard
Alfred, NY*

WESTERN NEW YORK (cont.)

Kohl's Scholarship Award

Bailey Walter, a friend of Kale Shiesley (SMA Type II), was nominated for the Kohl's Scholarship Award for all the work she does for raising awareness and donations for FSMA. She designed the "Let Them Dance" bracelets that she sells because she wants to find a cure for SMA so Kale and she can dance together. For her birthday, she had a roller skating party where instead of presents she asked for donations to FSMA. She also has lemonade sales and has a permanent sign on her house that lights up showing her support for finding a cure for SMA. She also organized the FSMA recognition day last August for Kales birthday in North Tonawanda, NY. We just found out Bailey won the national Kohl's contest. She won a \$1,000 scholarship for college and a \$50 Kohl's gift card for all of her efforts! Congratulations to Bailey and thank you for all of your hard work and dedication!



WISCONSIN

Marathons for Addy

Sean Hampe is aiming to run more than 52 half marathons in a year as well as five marathons in 5 weeks with the goal of raising \$25,000 for Families of SMA! He is running for Addison Kuester who has SMA Type I. Thanks to Sean's dedication and hard work, he has raised \$1,500 so far! Thank you, Sean!

St. Joseph's Parish Bucks for Blue Jeans Fundraiser

On June 6th, 2012 the St. Joseph Parish and School in Menomonie, WI held a Bucks for Blue Jeans fundraiser. Thanks to the generosity of students and teachers, almost \$220 was raised for Families of SMA!



Dear Families of SMA,

A dear friend of mine is going to back to school for a second career and she is studying interior design. As one of their projects they had to refurbish a chair and then auction it off. They each had to pick a charity for \$75 from their chair to go to, so my friend Amy picked Families of SMA. Thank you Amy for your generosity for making Families of SMA your charity of choice.

Shannon Kuester
Hartford, WI

Are you interested in hosting a fundraiser? Families of SMA has manuals, sample booklets and dedicated staff to help you through the entire planning process. Please feel free to contact us anytime at fundraising@fsma.org to get started today!

Promotional Materials from Families of SMA Make Planning a Fundraiser Easy and Fun



Fundraising Materials:

- Manuals and Sample Booklet (Walk-n-Roll, Golf Tournament, Dinner/Gala & Bowl-a-Thon)
- Banners and Yard Signs
- Families of SMA "At a Glance" flyer
- New FSMA Bracelets
- Plastic Event Bags
- Donation Cards
- Promotional Tips
- Purple & Orange Golf Tees
- Temporary Tattoos
- Coin Canisters
- Families of SMA Pens
- Angel Tags
- Handheld Flags
- And more!



Merchandise



Cinch Bag | \$10



Purple Grocery Bag | \$4

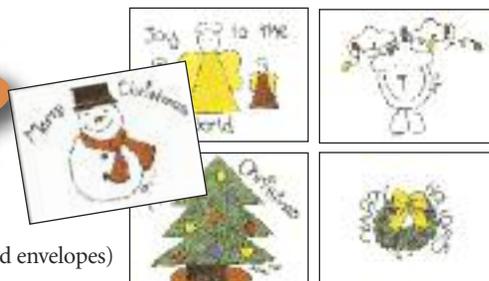


"Supporting Hands" T-shirt | \$12
Youth sizes: S M L • Adult sizes: S M L XL



Greeting Cards | \$6
(for a pack of 5 cards and envelopes)

To view a complete list of Families of SMA Merchandise visit www.fsma.org/Fundraising/Merchandise



Holiday Cards | \$5
(for a pack of 5 cards and envelopes)

Event Websites

Did you know that the Families of SMA National Office can build a customized website for your event?

It makes it easy for your friends and family to be able to register for your event and collect online donations toward their fundraising goal! Check out some of the great features our websites offer to enhance your event.



Customized Banner

We can create a banner customized with pictures featuring your event logo, friends, family and the child who inspired you to host your event

Widget and Video Integration

Widgets and videos can be uploaded to the website to make your website interactive for visitors.

Register and Donate Buttons

- Registering or donating to your event is just a click away!
- For ticketed events, a “Purchase” button option is available to make ticket sales a breeze!

Scrolling Lists

Your event website can showcase your event’s generous donors and top fundraisers automatically in two scrolling boxes.

Progress Bar and Amount Raised

The progress bar shows the total amount raised and how close you are to your event goal!

Use Social Media to Share Your Page

On your personal fundraising page, you can click on the sharing buttons to post a link to your page on Facebook, Twitter and other social media sites. This is a quick and effective way of asking your friends and family to support your efforts to raise money to help find a treatment and a cure for SMA!



Other Website Features

- Gifts made to an event or personal fundraising page can be made in honor or memory of a loved one.
- An easy drop-down list is available as a search method to find teams or participants registered for an event.
- Donors can mark their contributions as “Anonymous” and remain anonymous on all event pages and personal fundraising pages.
- Participants can post their offline cash and check donations to appear online in their personal, team and event totals to showcase their fundraising success!

Bake Sale in Honor of Gray's Gang

I have enclosed a check for \$188.50 from Elisabeth Drake, a twelve year old, who lives in my neighborhood. Elisabeth's language arts class was involved in a bake sale on Saturday and they decided to give Gray's Gang their proceeds from the sale. They made a whopping \$188.50! I told her that must be some delicious brownies and cupcakes!

We were so very excited and grateful she chose us for her charity of choice.

Thanks!

Abbie Dougherty of Charlotte, North Carolina

The 7th Grade CCD Class from St. Thomas More in Braintree, MA recently hosted a lemonade stand and raised \$300 selling lemonade and snacks.

The girls donated \$150 in Owen Norton's honor for Team Norton's SMA Walk-n-Roll. The girls also donated \$150 to the Julie Fund Walk – in memory of Julie McAvinn and Women's Cancers.

The girls involved in the lemonade fundraiser were: Nicole Daly, Danielle Lagos, Caroline McGowan, Jane McAvinn, Madison Calderara, Annie Martin and Annie Lee.

Congratulations to the girls for their hard work and raising money and awareness for two great causes!

Thank you,

Kathy Norton of Braintree, Massachusetts
Owen's Mom

Lemonade Stand and Bake Sale in Honor of Zane Schmid

Hi my name is Emma Keller, I am nine years old. My mom's best friend is Hillary Schmid. Hillary lost her baby Zane to SMA. I hate SMA. It makes babies die. I love Zane and her twin Avery. My last day of school was June 8th. I decided to have a lemonade stand/bake sale. I wanted to give all the money to help other babies who have SMA. Next year I am going to do the same thing on the last day of school. I hope this check helps.

Love,

Emma Keller of Pennsylvania

National Junior Disability Championship



Griffen Kingkiner, 13 of Montgomery, NJ participated in his second NJDC competition in Arizona this past August. NJDC (National Junior Disability Championship www.njdc2012.com) is an annual competition for disabled athletes of all kinds. Having SMA makes it very difficult to classify Griffen, he is one

of only a few athletes with a degenerative disease! After careful consideration, Griffen was nationally ranked (using Paralympic classifiers) as an F42 which is equivalent to an athlete with a single leg amputation above the knee. It is very tough to compete against athletes with normal upper body strength, but that doesn't stop Griffen!! He trains very hard all year knowing that he has to balance training with fatigue. He is careful to make every throw count following his coaches suggestions.

Griffen trained and participated in javelin, shot put, discus, archery, table tennis and swimming. He earned medals in every category. In 2011, Griffen set a new national record in javelin throwing 16.93 meters (that's over 55 feet). He beat that in 2012 for the silver medal throwing a personal best of 18.46 meters (that's over 60 feet)! Javelin is one of his favorite sports and he wants to continue throwing when he enters high school next year.

The highlight of the week for Griffen was table tennis. He got to play in several categories including doubles, singles in class and singles round robin! He practices a lot at home and it really paid off at nationals. He earned a gold medal in singles!

After 6 long days of competition in the heat of Arizona, he was happy to have some family time to visit Sedona and The Grand Canyon. It was an experience of a lifetime!



Olivia Burgess



Scarlette Ricotta



Stella Bartlett



Nadine Natcho



Nadia Rodriguez



Julissa Gonzalez



Journey Weiberg



Mia Ehorn



Melissa Milinovich



Macey Grace Hammerbeck



Tianna Rivera



Tambryn and Braelyn Campbell



Lucia



Mateo Medina, Addison Kuester, Leah Vogedes, Arianna Martin and Peyton Elsner



Lizzy Hallam



Lindokuhle Angel Sibiya



Matthew Williams



Charlie Sykora



Cole Geraghty



Emily Lozina



Ella Ecker



Eva Grace Kelly



Ioanna Tsakiri



Dani Pruitt



Dylan Cuevas



Dylan Cuevas



Hannah Smith



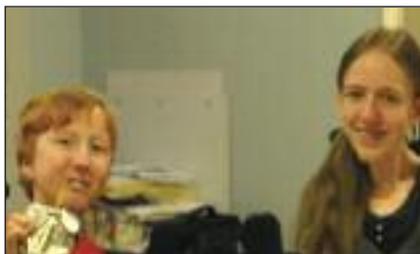
Evie Horton



Dr. John Kissel and Dr. Mary Schroth



Dominick Trevethan



Danny and Dana



Isabella Perge



Hunter and Thayne Devereaux

Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role

Wednesday, June 12, 2013
The Disneyland Hotel
Anaheim, California



Jointly sponsored by:

University of Wisconsin School of Medicine
and Public Health
Office of Continuing Professional
Development in Medicine and Public Health
Department of Pediatrics
and Families of SMA





The Wilson Family



The Shore Family



The Geraghty Family



The Kerns Family



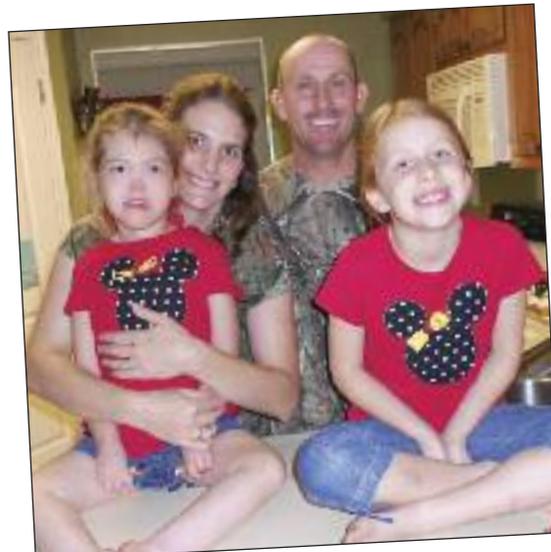
The Kelly Family



The Gonzalez Family



The Thompson Family



The Campbell Family



Vincenzo Schabach Fedrigo Family



The Deveraux Family

Repligen Reports Positive Results From Phase I Clinical Trial of RG3039 for Spinal Muscular Atrophy

Repligen Corporation (NASDAQ:RGEN) announced positive results from a Phase I study to evaluate the pharmacokinetic (PK) and safety profile of RG3039, a novel small molecule drug candidate for the potential treatment of Spinal Muscular Atrophy (SMA). SMA is a inherited neurodegenerative disease in which symptoms of progressive damage to motor neurons including loss of muscle function typically appear very early in life and often progress to severe physical disability and early loss of life. The Phase I trial was a blinded, ascending, single dose study of RG3039 administered to 32 healthy volunteers. The study results demonstrate that RG3039 was well tolerated at all doses administered, with no serious adverse events reported. The data also showed evidence of a dose-related drug response resulting in 90% inhibition of the target enzyme. These outcomes may help to establish appropriate RG3039 dosing regimens for future studies, including potential efficacy studies in SMA patients.

“The safety and PK outcomes from our Phase I study of RG3039 are encouraging, and we look forward to initiating the next steps for this drug candidate in alignment with guidance from the U.S. Food and Drug Administration,” said Walter C. Herlihy, President and Chief Executive Officer of Repligen. “The agency has previously granted Orphan Drug and Fast Track designations to RG3039, in recognition of the unmet medical need that exists for patients with SMA and the urgency to advance a treatment for this devastating disease.”

Repligen licensed RG3039 in 2009 from Families of Spinal Muscular Atrophy (FSMA), a patient advocacy organization that funded and directed the preclinical development of RG3039 with an

RepliGen

investment of more than \$13 million. This was the first drug discovery program ever conducted specifically for SMA. Repligen’s research efforts including this Phase I study have been partially supported by a grant from the Muscular Dystrophy Association (MDA).

RG3039 is the first clinical-stage drug candidate to target the core genetic deficit in SMA in order to treat the biochemical deficits caused by decreased levels of the survival motor neuron (SMN) protein. This key protein is necessary for normal neuromuscular function but is insufficiently produced in SMA patients. RG3039 is an orally bioavailable small molecule inhibitor of an RNA processing enzyme called DcpS. RG3039 has been shown to increase production of the SMN protein in cells derived from patients. In addition, RG3039 has been shown to improve motor neuron pathology, mobility and lifespan in animal models of SMA.

Top-line results from this Phase I study of RG3039 are scheduled to be presented as part of a special neuroscience program at the 64th Annual Meeting of the American Academy of Neurology (AAN). The AAN meeting was held April 21-28, 2012 at the New Orleans Ernest N. Morial Convention Center. James P. Van Meerbeke, Research Assistant from the lab of Charlotte J. Sumner, M.D., Associate Professor of Neurology and Neuroscience, Johns Hopkins University School of Medicine, will present the abstract titled “The Therapeutics Effects of RG3039 in Severe Spinal Muscular

Atrophy – Mice and Normal Human Volunteers,” during “The Future of Neuroscience Conference: Neurologists and Neuroscientists Defining the Next Generation of CNS Therapies,” took place on April 27.

In addition to the Phase I clinical trial outcomes, the AAN presentation highlights the results of earlier mouse model studies conducted with RG3039 at Johns Hopkins University and in the lab of Dr. Chien-Ping Ko at University of Southern California. In these preclinical studies, which provided proof of principle for conducting human clinical studies, administration of RG3039 resulted in a significant improvement in survival, increased maximum body weight and improved motor behavior in severe SMA mice. The effects were associated with increased SMN2 transcript levels and improved neuromuscular junction morphology and physiology. At therapeutic levels of RG3039 in mice, the target enzyme DcpS was inhibited greater than 90%. This appears to parallel outcomes from the Phase I human study, which achieved greater than 90% inhibition of DcpS in peripheral blood cells for 48 hours with a single dose of RG3039 and in absence of any toxicity.

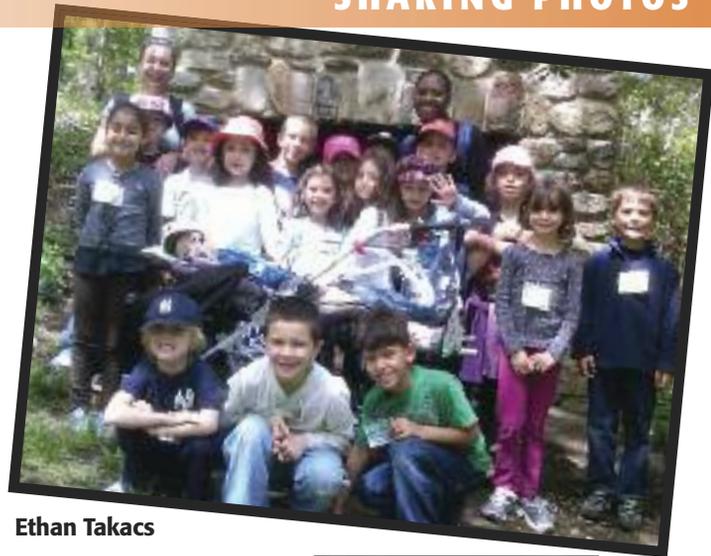
In addition to the presentation at AAN, Dr. David Jacoby, Medical Director at Repligen Corporation, presented the RG3039 Phase I study outcomes during the 2012 International SMA Research Group Meeting. This FSMA organized meeting was held in Minneapolis from June 21-23, 2012. Dr. Jacoby’s presentation titled, “A Phase I Study in Healthy Volunteers to Assess the Safety, Pharmacokinetics and Pharmacodynamics of the DcpS inhibitor RG3039,” was scheduled for Friday, June 22, 2012 at 3:40 p.m.



Journey Weiberg



Iman Casoojee



Ethan Takacs



Kelsi Jackson



Lindokuhle Angel Sibiya



Lucas Hoelscher



Kayley Shade



Lizzy Hallam



Macey Grace Hammerbeck



Josephine Anthony



Kale and Kyler Shiesley



Lucia



Madison Nielsen



Keira Harrison



Julissa Gonzalez



Julissa Gonzalez

Families of SMA Announces \$1.4 Million Planned for Next Round of Spinal Muscular Atrophy Research Funding

Families of Spinal Muscular Atrophy (FSMA) is dedicated to creating a treatment and cure for Spinal Muscular Atrophy (SMA) by funding and advancing a comprehensive research program.

The organization is planning to award \$1.4 Million in new research funding over the next 6 months. This new round of research funding will be allocated into three areas:

- 1) Basic Research to understand the disease and provide ideas for drug making
- 2) Drug Discovery to develop new SMA therapies
- 3) Clinical Research to help test new drugs effectively and to improve care for patients

Families of SMA released two distinct Requests for Proposals for new research funding to the SMA research community in July, 2012, with the new funds to be awarded in late fall of 2012. This follows \$745,000 awarded to 7 basic research grants late last year, and \$1.5 Million awarded recently for two new drug discovery projects.

Funding for New Basic Research Grants:

This basic research provides fundamental information about what is going wrong in SMA, which then provides seed ideas for new ways of making drugs. Our current round of funding will focus on proposals determining the functional role of SMN protein, finding the tissues where SMN protein is required, identifying new drug targets for SMA, and building new tools to facilitate SMA research.

Funding for New Drug Discovery Programs:

FSMA has been investing in and advancing drug research since 2000. The goal of drug discovery funding at FSMA is to build a large and diverse therapeutic pipeline to maximize the chances for success in finding a treatment for SMA. FSMA has been involved in funding half of all the ongoing SMA drug programs to date. New awards in the current round will focus on

providing critical funding for early-exploratory projects assessing novel therapeutic approaches for SMA, or for specific aspects of current promising drug programs.

“Families of SMA is pleased to announce the plans for this additional \$1.4 Million in new research investments”, says Jill Jarecki, PhD, FSMA Research Director. “We believe that multi-dimensional and consistent research investments are key to creating the needed momentum in SMA research. This next round follows the recent funding of nine new awards in basic research and drug discovery by FSMA in just the last year. We have been successful in implementing our model of early diversified investments followed by licensing programs to industrial partners for clinical development or obtaining large-scale government funding.”

Recent FSMA awards include:

- Families of SMA Awards New Funding to Advance a CNS Delivered Gene Therapy for SMA.
- Families of SMA Announces \$700,000 Funding for Novel SMA Drug Program at Newly Created California Institute for Biomedical Research.
- Families of SMA Awards 7 New Basic Research Grants for \$745,000 to Develop New Approaches for SMA Therapies.

The FSMA research funding philosophy is based on expert and independent oversight of research projects. This approach ensures that FSMA funds only the most promising research, and that funded projects are run in a professional and efficient manner under the guidance of world-class experts.

In addition to funding critical research, FSMA is increasingly dedicating resources to areas that focus on improving patient care. The organization will also announce new programs in this area over the next year.

FSMA Releases Request for Proposals for Preclinical Drug Projects in Spinal Muscular Atrophy

Families of SMA invites proposals for pre-clinical drug discovery programs for Spinal Muscular Atrophy (SMA), under a competitive review by our Translational Advisory Committee (TAC).

RFP Overview:

Proposals should involve innovative methods of advancing novel therapeutic candidates for SMA, either biologics or small molecules. Novel methods of boosting SMN levels and non-SMN based approaches are both welcome. We anticipate granting awards for two SMA projects focused either on obtaining proof-of-principle data for early-stage exploratory projects or on specific aspects of ongoing preclinical drug programs. Awards will be for one year with maximum funding of \$200,000.

Program Stage:

We invite proposals encompassing any stage of pre-clinical drug development from early discovery to IND submission to the FDA.

Scientific Focus:

Projects should be focused in two areas. The first area of focus is on validating innovative new approaches or mechanisms of actions for potential SMA drugs. Projects will involve obtaining proof-of-concept data in disease relevant animal or cellular models, which would justify further investment in the approach. Non-SMA based approaches would be well suited for this focus area.

FSMA anticipates a 2013 drug discovery RFP to enable programs of larger scope with validated mechanisms of action.

The second focus is for specific parts of ongoing SMA drug programs, based on already validated scientific strategies. Funded studies could include a subset of the following:

1) assessment of pharmacology / biodistribution, 2) animal model testing, 3) in vitro ADME data (hCYP inhibition, microsomal stability, etc.), 4) in vitro screening of safety (Ames, hERG, etc.), 7) animal toxicology studies, or 8) IND preparation.

Overall Timeline:

RFP Release Date: June 28, 2012
Letter of Intent Deadline: July 20, 2012
Invitation for Full Proposal: July 31, 2012
Proposal Due Date: August 31, 2012 (midnight ET)
Award Notification: December 2012.

Families of SMA Releases a Request for Proposals for Basic Research Projects in Spinal Muscular Atrophy

Families of SMA is pleased to announce that we are accepting grant applications for 2012 funding of basic research projects, under a competitive review by our Scientific Advisory Board (SAB).

Program Overview:

The deadline for submission is August 31, 2012. Applications will be accepted for one or two years of funding.

Scientific Priorities:

Families of SMA would like to encourage applications on novel research that will enhance our understanding of SMA disease pathology at the molecular, cellular, and biochemical level; generate key reagents and tools to facilitate drug development and clinical trials; and identify new therapeutic strategies for SMA.

Please note Families of SMA released a Request for Proposals focused on Preclinical Drug Discovery on June 28. This separate RFP will provide funding for aspects of ongoing drug programs and funding for proof-concept of studies on new therapeutic strategies in models of SMA.

We envision that studies funded by our Basic Research RFP will fall into one the following key areas, which are listed in random order:

1. Studies leading to increased understanding of the molecular and biochemical mechanisms regulating SMN expression or mediating SMN function, with the overall goal of identifying upstream regulators of SMN expression / splicing / function and downstream effectors of SMN functional activity, which will lead to novel drug targets for SMA.
2. Studies resulting in greater understanding of the pathophysiology of SMA, using established animal models of the SMA. This includes focus on the tissue requirements for SMN protein, clarifying the cellular autonomy of the disease in motor neurons, peripheral versus central manifestations of the disease, and other areas.
3. Work focused on generating research tools, such as new animal models for SMA, phenotypic cellular assays for SMA, biomarkers or outcome measures for clinical trials, newborn screening protocols, and others.

Timeline:

RFP Release Date: July 5, 2012
Proposal Due Date: August 31, 2012 (midnight ET)
Award Notification: December 2012

U.S. Senate Appropriations Committee Approves Report Language Requested by FSMA

Language Urges NIH to Continue Efforts that Support Development of Spinal Muscular Atrophy Drug Therapies and SMA Standards of Care. The United States Senate Committee on Appropriations approved the fiscal year 2013 Labor, Health & Human Services, and Education Appropriations bill that includes report language requested by Families of SMA. This language urges the National Institutes of Health (NIH), the federal government's biomedical research agency, to continue its successful collaboration with Families of SMA and its biotechnology partners in the development of SMA drug therapies for testing in clinical trials and to fund care studies that will facilitate the development of standards of care for individuals affected by SMA.

A contingent representing Families of SMA travelled to Capitol Hill in Washington, D.C., on March 21st to request the language. The Families of SMA representatives provided an update to Members of the U.S. Senate and the U.S. House of Representatives on recent advances in SMA-related biomedical research and urged Members to continue their strong support of increased federal funding for the development of SMA drug therapies and standards of care. Congressional support for these initiatives has been instrumental in accelerating the progress of SMA drug development programs.

Why the Language is Important?

Over the past five years, a highly successful therapy development model has emerged, with Families of SMA providing seed funding for pilot studies of SMA therapies, the federal government supporting further advancement of the research through grants from the NIH, and industry partners subsequently initiating clinical trials to test the safety and efficacy of the drugs. This model has led to a very robust and diverse SMA drug pipeline that includes three therapies currently in clinical trials. The report language requested by Families of SMA and approved by the Senate Appropriations Committee facilitates continuation and expansion of this paradigm.

Families of SMA also requested that Members urge the NIH to support care studies that will facilitate the development of clinical practice guidelines. Members were made aware that in the absence of evidence-based care protocols, many SMA patients receive care that is inconsistent with best-practices. Development of practice guidelines will bring uniformity to the medical care received by SMA patients and significantly improve the management of the disease.

Annual Conference

In addition to discussing these important policy matters, the Families of SMA contingent also met with staff for several Members of the Minnesota Congressional Delegation to discuss the Annual Conference taking place next week.

Families of SMA understands the importance of communicating regularly with federal lawmakers and policymakers and will continue to engage Members of Congress on issues of importance to the SMA community. Please contact Legislative Coordinator Spencer Perlman at spencer@fsma.org if you have any questions about Families of SMA's legislative, regulatory, and public policy activities.

President Obama Signs Into Law a Bill to Expedite FDA Approval of Drugs for Rare Diseases



President Obama has signed into law legislation that will advance therapy development for rare diseases like Spinal Muscular Atrophy (SMA). The Food and Drug Administration Safety and Innovation Act provides new tools to the Secretary of Health and Human Services (HHS) and the Director of the Food and Drug Administration (FDA) to help expedite the approval process for drugs that treat rare and life-threatening conditions. Families of SMA is supporting two therapies that currently are in clinical trials and could benefit from this new authority.

Under the provisions of the FDASIA, if a new drug demonstrates the potential to address unmet medical needs for a serious or life-threatening disease or condition, the Secretary of HHS can deem it a "fast track product" and employ more broadly effective processes for the accelerated development and review of the innovative new medicines. Additionally, if a drug shows early clinical evidence that it could be significantly more effective than existing therapies, the Secretary can declare it a "breakthrough therapy" and expedite the review and development of those drugs.

The new law also establishes a process whereby the Secretary of HHS can consult with external experts on rare diseases, targeted therapies, and genetic targeting of treatments concerning the severity of and the unmet medical need associated with rare diseases. The Secretary also may consult with these external experts on the willingness and ability of individuals with a rare disease to participate in clinical trials, an assessment of the benefits and risks of therapies to treat rare diseases, the general design of clinical trials for rare disease populations and subpopulations, and the demographics and the clinical description of patient populations. This ability of experts in SMA to work formally in a collaborative fashion with HHS and FDA officials is tremendously beneficial and will facilitate the drug approval process.

Families of SMA thanks Congress and the President for enacting these new important tools that will expedite the approval process for drugs treating rare and life-threatening diseases like SMA.

Congress Close to Passing Bill to Expedite FDA Approval of Drugs for Rare Diseases

Congress is on the cusp of approving legislation that will advance therapy development for rare diseases like Spinal Muscular Atrophy (SMA). Both the House of Representatives and the Senate have overwhelmingly approved their respective versions of legislation to reauthorize the Prescription Drug User Fee Act (PDUFA); each chambers' PDUFA bill provides new tools to the Secretary of Health and Human Services (HHS) and the Director of the Food and Drug Administration (FDA) to help expedite the approval process for drugs that treat rare and life-threatening conditions.

Under the provisions of the PDUFA bills, if a new drug demonstrates the potential to address unmet medical needs for a serious or life-threatening disease or condition, the Secretary of HHS can deem it a "fast track product" and employ more broadly effective processes for the accelerated development and review of the innovative new medicines. Additionally, if a drug shows early clinical evidence that it could be significantly more effective than existing therapies, the Secretary can declare it a "breakthrough therapy" and expedite the review and development of those drugs.

The legislation also establishes a process whereby the Secretary of HHS can consult with external experts on rare diseases, targeted therapies, and genetic targeting of treatments concerning the severity of and the unmet medical need associated with rare diseases. The Secretary also may consult with these external experts on the willingness and ability of individuals with a rare disease to participate in clinical trials, an assessment of the benefits and risks of therapies to treat rare diseases, the general design of clinical trials for rare disease populations and subpopulations, and the demographics and the clinical description of patient populations. This ability of experts in SMA to work formally in a collaborative fashion with

HHS and FDA officials is tremendously beneficial and will facilitate the drug approval process.

While the House and the Senate both have passed these bipartisan bills, the chambers still have work to do before the bills can become law. The next step in the legislative process is to form a bicameral conference committee to reconcile differences between the respective versions. Although the legislative language on the subject of expedited review for life-saving drugs and therapies is the same in both chambers' versions, there are some significant differences on other, unrelated components of the legislation, such as medical device regulation and incentives for manufactures to create new antibiotics. It is not believed that these differences are deal breakers, though, and Congress aims to have a completed PDUFA bill on the President's desk by the end of the month.

It should be noted, though, that in the current political climate, there is no guarantee that the process of combining the legislation will be easy. Families of SMA encourages you to contact your Members of Congress to urge them to complete their work on PDUFA and send the legislation to the President with the language expediting the approval process for drugs treating rare and life-threatening diseases like SMA.

Below are links to the section of the House website and the Senate website that lists each Member's address and phone number, as well as a link to a web form that permits emails.

<http://house.gov/representatives/>

http://www.senate.gov/general/contact_information/senators_cfm.cfm

Families of SMA joined with 119 other rare disease organizations in an urgent letter to the key committees tasked with reconciling the legislation in the House and Senate.



Alex Blair's Make-A-Wish

Alex (SMA Type II – 9 years old) has been a big fan of trains since he first saw a Thomas the Train movie with his big brother, Adam. So when it was time to think about what Alex's wish would be, we knew trains would be involved. After a lot of brainstorming with family and friends, the idea of a train room proved to be the way to go. What turned out as a tabletop layout sort of grew into a remodeling project in half of the basement. Alex's very dear friend, Rob Bauer, became the mastermind behind the entire project.

As word spread of Alex's wish and contacts were made, the newly formed Upstate NY Chapter of the TCA (Train Collectors Association) volunteered to help. Over a period of several weekends, members showed up at the Blair household to lay track, troubleshoot, and wire the layout & accessories. As this was the first activity for the chapter, many unacquainted TCA members got to know each other and develop friendships.

People were coming and going helping to create the ultimate train layout



for Alex. Volunteers came in to help create the room and paint murals on the walls. Extra trains were donated along with some extra pieces to set up a wonderful layout. One family donated buildings that a relative made over 20-years ago to add to the layout. It was truly a project filled with love from everyone.

One of the volunteers took a piece of wood and hooked up easy to touch buttons on it so Alex could control some of the automated buildings. He can press a button and a fire truck with a siren comes out of the firehouse. Another button starts the music for a diner. Others start a car wash and gas station. Further work will be done so Alex can control each of the four trains and trolley on their tracks. He simply loves having control.

A "Grand Opening" Party was hosted by Make-A-Wish in June with cake and balloons to thank all of the family and friends that gave up many evenings and weekends to help make Alex's Wish come true.



Meet AJ Brockman

Digital Artist

An intro to yourself and who you are, etc, etc.

My name is AJ Brockman, and I'm a digital artist. I also have spinal muscular atrophy (SMA), a progressive neuromuscular disease, and have been wheelchair-bound since age 2. My disability definitely influences who I am, but it does not define me. I view myself as being "differently abled" rather than "disabled," and I live life to the fullest every day.

How you got into digital art?

As a kid, I still had fairly good use of my hands and was able to paint traditionally, but I developed an impressionistic style because "dabbing motions" were physically easier for me to do than actual brushstrokes. As I began to lose the use of my hands, I was forced to come up with a different way to be productive and express myself. I found the answer in high school, when I took a course called Commercial Art Technology and learned to use computer software for ad layout and graphic design. Once I mastered the tools and programs, the transition from brushes to computer was seamless. I create all of my artwork with one finger on my left hand by manipulating the computer mouse (touchpad). It's an extremely time-consuming process, but I am able to achieve maximum precision and detail in my work. I then use sophisticated printing methods to output my work on canvas and even metal.

What inspires your work?

I usually just paint what I'm in the mood to paint, but I draw lots of inspiration from the beachside lifestyle here in South Florida. Not only is this genre something I truly enjoy, but it resonates extremely well with my audience; some of my most popular pieces have that South Florida-beachy theme. I tried experimenting with some Autumn-type settings this year and was pleased with the results, so that's something I might continue to develop, and I'm constantly dabbling in other styles and subjects.

I have started to accept more commission work lately — everything from pet portraits to client-directed landscapes. With every new project I do a ton of research into the emotional connection with the client, historical significance, and/or reason for the chosen subject matter. Not only does this enhance my actual technique and skills, but it also leads to a more fulfilling experience overall. My clients and I go through what I like to call a "journey" together, and it becomes much more than just an art commission.

Challenges / barriers you have had to overcome?

One of the most common misconceptions of my work is the "digital photography" parallel. Although my work is often compared to digital photography, it's not the same thing by any stretch of the imagination. It would be like someone looking at an oil painting and saying "Wow, what a great watercolor!"

But perhaps the biggest challenge I face is simply getting people to recognize me as the artist; patrons are more inclined to ask my booth attendants or crew members if they are the artist. Unfortunately, some people still do not believe disabled people can be productive members of society. I feel a responsibility to educate others on the amazing things people with disabilities can accomplish through hard work and determination, and my artwork is a great way to do that.

Current projects / what you are up to?

I have a bunch of upcoming art shows and exhibits. This includes a rotating gallery at a very prestigious bank here in the states, Northern Trust. I will also be



participating in one of South Florida's biggest art and music festivals, Sunfest, along with many other commitments.

I am involved with Canine Companions

for Independence, one of the nation's premier service organizations. This year I received my second service dog, Dre, and have the honor of being the "Featured Graduate" for one of their largest fundraisers this year. To commemorate this incredible event, I have created a one-of-a-kind digital painting.

Community involvement is also very important to me. I regularly volunteer at our local schools, giving speeches, doing disability outreach and donating my artwork for fundraisers. I've also started to give local digital art tutorials/seminars to other individuals with physical disabilities.

Info about the documentary project and how people can get involved?

My most important new project is starting a nonprofit production company, No White Flags, with my manager. We plan to produce full-length documentaries on individuals with physical or mental disabilities who never give up and have overcome extreme obstacles in their lives. I encourage everyone to visit www.nowhiteflagsproduction.com to learn more.



My name is Caitlin Brown and I have SMA Type I. I have a trach and a g-tube and I am on a ventilator. I am 23 years old and I've recently graduated from Northern Kentucky University with a Bachelors Degree in Theatre Arts.

I wasn't supposed live past two years old much less graduate from college. I've always enjoyed proving people wrong! I had to overcome so many health problems while in college in order to graduate and I'm here to tell you if I could do it so can your child with SMA. I have been told my whole life that I could not act in a play because my wheelchair is too modern and distracting. So, for my Honors Capstone Project at NKU I decided to direct a play from 1895 and put the ambulatory actors in wheelchairs. It was a huge success and I received many positive comments on my work. I learned a lot from this experience and so did the actors and the audience. Members of the audience even told me that after a few minutes they no longer noticed that the actors were in wheelchairs.

Probably what I am most proud of though is that I am writing a play about what it is like to have SMA, to increase awareness. Just because somebody has SMA does not mean they can't achieve their dreams. My dream is to be a actress/director/playwright in New York City. Some people do not believe this will ever happen, but I can't wait to prove them wrong again!

Hunter's Hope



Hunter is a 12 month old baby with Spinal Muscular Atrophy (SMA), a terminal illness. Hunter is a very busy baby who has traveled and seen many things during his first year, despite living with SMA. According to Curtis and Khrystal Davis, Hunter's parents, their goal with Hunter is to have fun, and let him live to his fullest potential. He is a very happy and active baby. Their goal with regard to SMA is to generate awareness, education, and to increase funding for research for SMA treatment and ultimately a cure. Curtis and Khrystal further state, "We are making the days count, rather than counting the days. Hunter has SMA, but SMA does not have him."

Hunter visited the great wall of China, together with his parents, Curtis and Khrystal Davis on August 22, 2012. August is SMA awareness month, and Hunter's father, Curtis Davis had a business trip planned to China. Curtis and Khrystal thought it would be a wonderful opportunity for Hunter to both visit the Great Wall, and spread SMA awareness. The family visited the Great Wall, and held a banner that read Hunter's Hope for a SMA Cure. The Great Wall was the second wonder of the world Hunter has visited. In June, Hunter visited Chichen Itza with his family in Mexico.

SMA is the number one genetic killer of infants and toddlers. SMA occurs in 1 of 6000 births. 1 in 40, nearly 10 million Americans is unknowingly a carrier. When two carriers have a child, there is a 25% chance the child will have SMA. Despite SMA being the number one genetic killer of infants and children, SMA is widely unknown until a loved one is diagnosed. The National Institute of Health (NIH) identified SMA as the disease closest to treatment out of more than 600 neurological disorders.

Hunter was born July 31, 2011, and diagnosed on September 31, 2011 at Children's Hospital in St. Louis. The doctor's provided a prognosis of 3-12 months, with it likely being towards the lower end given the severity and early onset of his symptoms. Hunter is fighting against the odds. This provided inspiration to organize Hunter's Hope to provide SMA awareness, education, and funding for research to realize treatment and ultimately a cure.

Ability Jobs Plus



Richard Hill recently created an Internet job portal, similar to the job site Monster, and it is dedicated to helping disabled individuals find work. Richard is the CEO and Founder of Ability Jobs Plus,

www.AbilityJobsPlus.com, with a goal to help the 3 million unemployed disabled veterans, and the 54 million unemployed disabled individuals (non military) find employment.

Richard was diagnosed with Spinal Muscular Atrophy back in 1987. Richard is an experienced web designer and he got the idea for AbilityJobsPlus.com in 2007 when he sought assistance from a local employment agency. "I was told they wouldn't help me find work because I use a walker to get around," Hill said. "It's been in the back of my mind since then." With the help of a silent partner, Hill created the site and launched it January 3rd.

The goal is to get the information to employers because it is them that make the site go.

"There are over 54 million disabled individuals within the U.S. and about 2.9 million disabled veterans," said Hill, who wants to help them find work. "The disabled community is the largest minority in the U.S. and the world, with over 650 million disabled people on a global scale."

If you need any info or have any questions please feel free contact Richard Hill at:

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Families of SMA is gathering helpful hints and tips when it comes to managing activities of daily living with SMA. We hope to publish these in each issue of the Directions Newsletter and also in a possible Care Series Booklet.

Here is an example of a great helpful hint from an SMA family:

■ **Outlet Adapter for the Car** – Since much of Jack's equipment had rechargeable batteries, we were able to travel for a few hours with his equipment – unattached by power cords. Some machines, like the Cough Assist, do require an outlet. A car adapter can help with equipment that requires an electrical outlet as its only power source.

■ **Stroller Hooks** – When Jack traveled in a stroller, we used stroller hooks to help keep his equipment tangle-free. Sometimes his oxygen cord and baby bag would hang from them, sometimes it's the suction machine, and other times it's his feeding pump. We even used these in the car to keep things organized – it's amazing what you can hang!



-Sarah Bonelli, Phoenix, Arizona

■ We used an under the bed storage box and set it up on our counter, then put the soft bath pillow from the FSMA Type 1 Care Package inside the box. This way we could pour water over our son for bath-time and not have to stop or precariously handle his little self in the regular tub.

■ We loved the Versa pillow (air-filled pillow) as a soft place for Ben to lie on his side or on his back with his head turned to the side. We could hold the Versa pillow in our arms with Ben lying on that.

■ Weebles toys are great for babies with SMA as they only require a little bit of force to move back and forth. Lying baby on his side is a great position for batting at these toys. We sometimes put the Weebles on top of several big books to make them high enough if our son, Ben, was on his wedge or Versa pillow.

-Nicole Newfield, Mahtomedi, Minnesota



Allison Kerns



Alana Whited



Bama Shore with his siblings



Bri Johnson



Alli Williams



Alyssa and Jordan Simmons



Brett Wilson



Brett Wilson



Aaron Phelps Meets The Wiggles!



Eva Grace Kelly



Jenna Boguhn



Addison Tarrence





Aiden Bala



Luke Bertsch



Lindsay Cochran with Cody and Hunter Edwards



Audry Hill



Braelyn Campbell



Alexis Helfrich



Audrey Wendt



Charlie Sykora



Caleb Merriken



Alexander Davis



Jacob Fox with big sister Malorie



Amelia Wong



Amelia and Ben Wong

Dear FSMA

While on the topic of thanksgiving I thought of how much gratitude you guys deserved. You always help other people by developing medicine and running conferences every year. I am one person in specific that has greatly benefitted from your work. Some of the things I am most grateful for are, as I said, the conference and the medicine you try to develop.

Two years ago I went to my first FSMA conference and met some new people with SMA. It was a great experience to talk to other people with SMA and get to know in more detail what it is like. I went to some kids meetings but spent most of my time talking in the kid's room. My parents gained a lot of knowledge about SMA by attending a lot of meetings and came out ready to face new challenges. These conferences were only helpful to us and only carried benefits. I even had more courage when I left, I felt it was not bad at all, that I was not disabled, I was difabled (differently-abled).

The meetings were helpful too, in the kid's room I made a new friend and if we were having trouble with something we could just talk to each other. This is something I am thanking you for because you organized the entire meeting and made it happen, so without you we would never meet and I wouldn't have anybody to talk to if I am having trouble with something. Being in the kids room also helped me think about what I might want to be when I grow up. I wanted to be able to help other people but it seemed

some jobs were out of my reach, so I thought maybe I could be a physical therapist and you helped me think of that.

Thank you also for trying to help us in yet another way, developing medicine. What you do is truly helpful and extremely nice, especially because you do it in hopes of helping other people with SMA. I really do believe you are close to a cure/treatment, so keep trying and I am sure you will find one. Even if it takes a while, thank you for trying, it helps me even if I don't get a medicine, just knowing that some people are trying.

Thank you for what you have done for me and everybody else with SMA I appreciate it and I am sure my family and everybody else with SMA appreciates it too. You are so nice and believe me you really deserve a nice THANK YOU!

With gratitude,
Griffen Kingkiner, age 13, SMA Type III
Skillman, New Jersey

