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Get a jump start! Register for the 2012 conference. See page 2 for details.

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Families of Spinal Muscular Atrophy is Excited to Announce that the Registration for the 2012 Annual SMA Conference in Bloomington, MN is Now Available!

Registration for the 2012 Annual SMA Conference, held in Bloomington, MN June 21st - June 24th, at the DoubleTree Hotel Bloomington – Minneapolis South, is now available.

We are now offering a new head start early registration fee which will be valid through February 1st, 2012, at 11:59pm Central Standard Time, when registration prices will increase.

You must complete your conference registration with Families of SMA prior to reserving your room at the DoubleTree Hotel for the Annual SMA Conference special room rate of $109 per night. This special room rate is available to SMA conference attendees from Sunday June 17th through Wednesday June 27th!

PLEASE NOTE THAT YOU MUST COMPLETE REGISTRATION PRIOR TO BOOKING YOUR HOTEL ROOM.

The DoubleTree by Hilton is just ten minutes from Minneapolis/St Paul International Airport. There is so much to do and enjoy while in the Minneapolis area. Shop, eat and be entertained at the massive Mall of America, root for the Twins at Target field, or catch Broadway musicals at Chanhassen Dinner Theatre. We are offering registered attendees free accessible shuttles to and from the Mall of America as well as free parking at the DoubleTree Hotel.

Since its opening in 1992, Mall of America has become a world-renowned leader in retail, entertainment and attractions. Receiving over 40 million

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visitors annually, Mall of America is home to over 520 retail stores and department stores, an amazing Nickelodeon Universe Amusement Park, an American Girl Store and movie theatres. From Mall of America to Target Field, the things to see and experience nearby are endlessly diverse. Families of SMA has been hosting this conference for 23 years. The weekend will be filled with networking opportunities with other families, workshops, a memorable kids program, a family fun fest, carnival, an exciting new SMA Family Dance Party and opportunities to interact with families and get first hand updates from the researchers. We are looking forward to reuniting as a community at this conference and showing our support for others. As always, the Family and the Research conferences will be running 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 for those affected by SMA. The interactions between the researchers and families at one conference is extremely special. The annual conference also provides our children an opportunity to make new friends and have a great time. We are expecting another great attendance of over 900 attendees.

Families of SMA will again be offering an amazing support program for all newly diagnosed SMA families to attend the annual conference.

Families of Spinal Muscular Atrophy will waive registration fees for any families newly diagnosed since the previous conference in 2011. Note: this includes up to 4 immediate family members.

Our goal is to encourage as many newly diagnosed SMA families to attend and experience the benefits of this amazing conference.

**The meeting gives families:**
- The opportunity to gather critical care and daily living information early after diagnosis.
- To learn directly from experienced SMA physicians.
- To network with other families.

Newly Diagnosed families should plan to arrive before noon on Thursday, June 21st as we will start a special general workshop program for newly diagnosed families on Thursday afternoon.

Information on registering through the Newly Diagnosed Program is now available on-line.

Highlights from the 15th Annual Spinal Muscular Atrophy Research Group Meeting

The International SMA Research Group Meeting is the biggest Spinal Muscular Atrophy (SMA) research conference in the world. Families of Spinal Muscular Atrophy (FSMA) organizes the conference, and financially underwrites the meeting by covering hotel, travel and registration for all research presenters. The 15th Annual International SMA Research Group Meeting was held June 23–June 25 at the Swan and Dolphin Resort in Disney World. 225 Researchers attended the conference, representing 70 total institutions, 14 biotech and pharmaceutical companies, and 11 countries worldwide.

The conference, the only open venue for annual communication between SMA researchers, has tangible 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 that generates a collaborative spirit, resulting in many productive research partnerships.

- Allows cross-disciplinary dialog among basic researchers, clinicians, and industry representatives, which is vital in creating effective therapies.

- Allows young researchers to interact with leaders in the field, which helps build the future of the SMA research community.

- Motivates SMA researchers by providing interaction with SMA families and patients.

In our latest conference survey, 95% of the researchers attending in 2011 indicated having an open and comprehensive SMA meeting is extremely important to the SMA research community, and specific feedback supports this statistic. Researchers indicated that: “This is THE big meeting that brings everyone together and I cannot stress enough the value of this meeting. The meeting is the starting point for many collaborations and a place for researchers of all stripes to learn and place their work into the context of the broader field. Seeing the children with SMA alone is a huge value from this meeting, to always ground us in our goals for doing the work we do” and; “This is my first FSMA meeting, and it was by far the most valuable and relevant meeting I have been to over the last three years. I had been regularly going to Neuroscience, but would choose this meeting over that meeting because of the focus on SMA and the unique experience to meet the families. This meeting does a great job of focusing various groups of researchers towards common goals.” Also, one researcher said: “This meeting is a huge service to the community and is critical for the exchange of basic and clinical information on the disease and the basic biology of SMA. The meeting clearly is having a positive influence on therapeutic development for SMA. As a drug discovery researcher I have found the meeting to be the most focused and effective way of interacting with the research community in SMA and the most effective way to gain a perspective on the state of the field.”

The conference is organized into sessions focusing on major unanswered topics in the field. This year there were five distinct sessions with an expert moderator leading the discussion of each. Additionally, two poster sessions occurred, allowing for presentations of very current and still under investigation research.

Over 100 abstracts were presented, including 36 as talks and 77 as posters. The conference started off with a Special Session organized by FSMA with invited speakers entitled, “Comparative SMA Pathology in Mice and Man with Therapeutic Implications”. Moderated by Dr. Thomas Crawford. The goal of this session was to compare and contrast SMA pathology observed in animal models of SMA and in humans to inform effective drug development. It started with a keynote address by Dr. Dwight Koeberl, MD, PhD, who discussed the successful drug development of Myozyme for the neuromuscular disease Pompe Disease. He emphasized the overall development plan for Myozyme, how animal models were used to advance the drug, how well the animal models predicted efficacy in humans, and strategies for newer second generation drugs.

The keynote address was followed by nine invited talks on comparative SMA pathology in humans versus mouse models. These talks reviewed the facts regarding the known defects in SMA animal models and in humans in a variety of tissues. Discussion focused on how changes compare among the different species. This included discussion on the relevance of non-neuronal defects in humans versus mice and what these tell us about which tissues to target for drug activity. Finally, the best use of SMA animal models in therapy development was discussed, including whether there are currently adequate animal models or more need to be generated.

2. Human SMA Studies. Moderated by Dr. Kathryn Swoboda. This session included discussion of new outcome measures and biomarkers for use in SMA clinical trials. Nicole Holuba from Columbia University gave a talk on a retrospective chart review of type I, II, III SMA patients. She showed that none of the SMA patients (n=102) had clinical evidence of congenital heart disease or dilated cardiomyopathy. Of the 22 who underwent cardiac evaluations, two had...
Talks on Comparative SMA pathology in Mice and Man.

1. Review of Peripheral Motor Neuron and Muscle Phenotypes in SMA Mice – Thomas Gillingwater, BSc, MBA, PhD, MCMII, FRMS, Professor of Neuroanatomy, University of Edinburgh.

2. Review of Motor Neuronal Circuitry Phenotypes in the Spinal Cord of SMA Mice. – George Mentis, PhD, Assistant Professor, Columbia University.

3. Review of Neuronal and Muscle Phenotypes in Humans, Including Recent Autopsy Studies -- Charlotte Sumner, MD, Assistant Professor of Neurology, Johns Hopkins School of Medicine.

4. Overview of Systemic / Extra-Neuronal Defects in SMA Mice (heart, GI, necrosis, bone, etc.) – Christine DiDonato, PhD, Assistant Professor, Department of Pediatrics, Northwestern University

5. Overview of Evidence Indicating Possible Autonomic Nervous System Deficits in SMA Mice – Brian Kaspar, PhD, Associate Professor, Nationwide Children’s Hospital.

6. Review of Heart and Vascular Defects in Humans – Sabine Rudnik-Schöneborn, MD, PhD, Professor, Institute of Human Genetics, University of Aachen.

7. Review of Systemic / Extra-Neuronal Phenotypes in Humans, Including Recent Autopsy Findings (adrenal insufficiency, GI issues, bone, etc.) – Kathy Swoboda, MD, Associate Professor of Neurology, University of Utah.

Session Synopsis and Panel Discussion – Moderator, Thomas Crawford MD, Associate Professor of Neurology and Pediatrics, Johns Hopkins University School of Medicine.

Dr. Martinez-Hernandez from the laboratory of Dr. Tizzano in Spain presented on early neuropathology in human SMA. She showed the presence of NMJ defects in early development in muscles from fetuses with severe SMA.

Two presentations were given characterizing NMJ defects in SMA mice. Results from the Summer lab at Johns Hopkins University suggested that increasing SMN in just muscle enhances survival, weight, motor function, and NMJ functionality, although expression in neurons has a larger impact. The authors suggest that healthier muscle may release a factor that improves motor neuron function. Dr. Ling from the Ko lab at USC presented a thorough study showing that NMJ defects are muscle type specific, rather than general across all muscles. Understanding what causes vulnerability will have important implications to therapeutic strategies. Finally, Dr. McCabe from Columbia University described motor synapse defects in the central nervous system in a fly model of SMA. He showed that inter-neurons are affected in the central nervous system. Also true in mice, SMA motor neuron pathology is not isolated to the periphery or neuromuscular junctions, but also to central synapses. In flies, rescuing genes have been found, perhaps identifying new therapeutic avenues.

4. Regulation of SMN Expression and Function with Therapeutic Implications. Moderated by Dr. Adrian K rainer. This session addressed the regulation of SMN expression and function with particular focus on how the SMN2 gene can be modulated for therapeutic benefit. Dr. Akten from the Steen lab in Harvard, reported that the E3 ubiquitin ligase is involved in SMN protein degradation. Inhibiting the E3 ligase in motor neurons leads to increased SMN levels and thus could provide a new therapeutic strategy for SMA. Faraz Farooq from the McKenzie lab showed that the p38 and STAT5 pathways regulate SMN levels. For instance, activation of the STAT5 pathway by the molecule prolactin increases SMN levels substantially in SM A mice and prolongs survival by 60%. Finally, Dr. Martinez-Hernandez continued on next page
Highlights from the 15th Annual Spinal Muscular Atrophy Research Group Meeting

Tiziano from Rome showed that the PI3 Kinase / Akt pathway leads to SMN enhancement after salbutamol treatment, leading to SMN2 splicing correction in patient fibroblasts.

5. SMA Therapeutic Development. Moderated by Dr. Douglas Kerr. Platform presentations updating many of the ongoing drug programs in SMA were given, including talks on small molecules to enhance SMN levels, gene therapy to replace the SMN1 gene, cellular therapy to provide beneficial motor neuronal progenitors cells to the spinal cord, and antisense oligonucleotides (ASOs) to correct SMN2 splicing. Leading companies working on SMA drug development, including ISIS Pharmaceuticals, PTC Therapeutics, and California Stem Cell, Inc gave talks.

The session included the following talks on ongoing drug programs:
- Stefania Corti PhD, University of Milan, Motor neurons from human Spinal Muscular Atrophy induced pluripotent stem cells free of vector and transgenic sequences as a model and cell source for transplantation
- Hans Kierstead PhD, California Stem Cell Inc., University of CA, Irvine, Stem cell derived therapeutic for Spinal Muscular Atrophy type I
- Paul Porensky MD, OSU, Antisense morpholino against ISS-N1 corrects SMA mice
- Yimin Hua PhD, Cold Spring Harbor Lab, Robust rescue of severe SMA mice by systemic neonatal administration of antisense oligonucleotide that corrects transgenic SMN2 splicing
- Frank Rigo PhD, ISIS Pharmaceuticals, Pre-clinical studies in mice and non-human primates support the development of an antisense oligonucleotide for the treatment of Spinal Muscular Atrophy
- Nikolai Naryshkin PhD, PTC Therapeutics, Small molecule compounds that correct alternative splicing of the SMN2 gene and restore SMN protein expression and function
- Kevin Foust PhD, Kaspar Laboratory, Nationwide Children’s Hospital, Biodistribution of IV injected AAV9 in young Cynomolgus Macaques

Most of the programs discussed in the drug session were represented during the final session at the SMA Families and Professionals Conference. Here the leading experts in the SMA Research Community answered questions from SMA families.

Three expert panels were held on: Basic Research, Therapy Development, and Clinical Research.

A video of this session can be found at SMA Community Connections, www.SMACommunity.org

Isis and Collaborators Present New Data on Isis’ ISIS-SMNRx at the 2011 Annual Spinal Muscular Atrophy Conference Hosted by FSMA

Restoring SMN protein in the CNS should provide significant benefit in treating SMA. Isis is currently conducting IND-enabling studies on ISIS-SMNRx and plans to begin clinical testing late in 2011 or early 2012 in patients with SMA.

Spinal Muscular Atrophy (SMA) is a motor-neuron disease that is a leading genetic cause of infant mortality. Most infants born with SMA Type I, the most severe form of SMA, die within their first two years of life. One child out of every 6,000 to 10,000 births is born with SMA. There are approximately 50,000 patients with SMA in the United States, Europe and Japan.

SMA is caused by a loss of, or defect in, the survival motor neuron 1 (SMN1) gene. SMN1 produces most of the SMN protein necessary for normal motor function. The severity of SMA is determined by the amount of SMN protein in the cell. Humans also have one or more copies of a related gene, SMN2, which produces a small fraction of SMN protein. An SMA patient who has multiple copies of the SMN2 gene produces more SMN protein and, as a result, has a less severe form of SMA.

Isis’ drug, ISIS-SMNRx is designed to modulate the splicing of the SMN2 gene to significantly increase the production of functional SMN protein. In preclinical studies, researchers observed proof-of-concept data demonstrating that an antisense drug can dramatically improve clinical features of SMA in a severe mouse model of SMN2.

The 2011 Annual SMA Conference brings together Families of SMA and leading researchers in the field of SMA research. At the conference Kathie Bishop, Ph.D., director of clinical development at Isis delivered a keynote talk outlining the elements necessary for conducting clinical studies in patients with SMA and presenting Isis’ ISIS-SMNRx drug program to the families. Dr. Bishop also participated in the Question & Answer session of the conference in which families can interact with leading SMA researchers in a moderated panel format.

During the scientific session, Isis scientists and collaborators presented new preclinical data showing that systemic administration of ISIS-SMNRx produced therapeutic benefit from SMN restoration in peripheral tissue, suggesting that there may be therapeutic benefit for an increase in functional SMN protein in tissues outside of the central nervous system. In addition, researchers showed that ISIS-SMNRx produced sustained activity in mouse models of SMA and that target tissue concentrations were achieved in non-human primates following a single intrathecal injection. Isis and collaborators also outlined ongoing work to develop new animal models of disease that will help Isis and its collaborators further understand the role of SMN in the pathology of disease.

FSMA thanks Isis for its Gold Sponsorship of the 2011 SMA Conference.
Everyone at FSMA,

Thank you very much for all your hard work, dedication and kindness – all of which made this year’s conference so wonderful for so many families. Cara and I are on the plane home – both sad about having to leave so many good friends. The spirit and strength of our community was so evident all weekend long. As Jack’s dad, I feel very fortunate to have so much support from all of you – and I feel very honored to be able to support other families along with you.

Thank you for everything you do every day.

Sincerely,

Al Freedman
of West Chester,
Pennsylvania

Dear Families of Spinal Muscular Atrophy,

Bob and I are so proud to be involved with such a wonderful organization as FSMA. The conference in Orlando was the best yet; it keeps getting better and better! A big “THANK YOU” to all the staff who work so hard to make this a special event each year for all of the families. Thank you to Kenneth Hobby for his leadership in keeping us focused on research for the cure and families. My greatest joy when attending the conferences is watching all the families love and support each other (not to mention the SMA children interacting with each other). As far as the report on the progress in our research efforts, it was the most positive year ever! I am so proud that our organization is not putting all its eggs in one basket; 11 projects in our pipeline and more to be added this year! Investing in different approaches such as drugs, cells, and genes, and now our government and companies are getting involved! This is huge! I know a cure is not going to come immediately, but we are moving in the right direction. Thank you to everyone involved who are helping to make this all happen. God bless you all.

Elizabeth and Bob Lockwood
of Cincinnati, Ohio

Dear Families of Spinal Muscular Atrophy,

On behalf of the Rivera family, we would like to thank FSMA for putting together such a great annual meeting. There was so much helpful information and we met some great people along the way. Tianna also met some new friends, Briahanna Johnson was one of them, once they met, they were inseparable. We all had such a great time. Tianna’s face was priceless when she saw all those kids on their power chairs just like her, what a great experience for my baby.

Thank you,

The Rivera Family
of North Port, Florida
Our 2011 conference in Disney World was certainly our most magical and successful conference yet. The final attendance number was 1,550, which is an amazing record number of attendees!

The Spinal Muscular Atrophy Conference in Florida was a fantastic success with over 1,550 families and researchers in attendance! This was 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 in the world for SMA families. The interactions between the researchers and families at one conference were 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.

At our 2011 conference, before all of the families arrived, we offered a Newly Diagnosed Program specifically for families diagnosed since 2010. 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 in session for the families, the 15th 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. 225 researchers attended from around the world. These researchers represent 70 institutions in total, 14 biotech and pharmaceutical companies, and 11 countries worldwide. The personal connections made between families and researchers meant so much to everyone who attended.
There were so many highlights at this year’s conference. The Conference started off on Thursday with an amazing Meet and Greet. This year’s Meet and Greet was certainly the most exciting one to date. Attendees enjoyed a dinner, as well as many snacks such as a Nacho Bar, Popcorn, Cotton Candy, and Sno Cones. There was a brief welcome for attendees as we began the ever so popular Researcher Relay Race. The Researcher Relay Race started 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. Ice cream was offered to all attendees as the most exciting part of the evening began – The Family Fun Fest and Disney Character Event. The Family Fun Fest was absolutely a huge hit! As each child entered the fun fest, they received a Mickey Balloon, as well as Mickey Ears and a Mickey pen to use when they received autographs from the Disney Characters. 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!

Most children, and even many adults, received Disney Character Tattoos. There was a balloon Artist who was amazing and offered everyone incredible creations.

Mickey Mouse, Minnie Mouse, Donald Duck and Daisy Duck joined the fun for families to enjoy for the final two hours of the evening. Attendees were able to take photos with the characters and receive their autographs, which made many children extremely happy!
2011 conference
Friday evening was our dessert party at Epcot.

The dessert party location was moved indoors since the evening had an unexpected rain storm. Thousands of glow sticks were passed out to attendees along with some purple and orange pom-poms. An amazing dessert buffet was served with large “tie-dye” cupcakes, chocolate brownies, rice crispy marshmallow squares with butterscotch chips, banana pudding trifle, along with beverages for all children and adults. Rain ponchos were passed out to attendees and those who were adventurous enough to go outside in the rain to view the fireworks and laser show. Although most people got a bit wet from the rain, it was a great night to be together and celebrate.
The Families of SMA South Florida Chapter was able to recruit local Walmart Stores to donate many items for the conference. One of the wonderful items donated was many pallets of bottled water. Because of this donation, we were able to pass out thousands of bottles of water to families throughout the weekend. These certainly helped keep families hydrated when they were outdoors or at the Disney Parks.

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 fun candy table for them to choose items from. Families scattered pillows and blankets on the floor for an official pajama movie night! Popcorn popped all night for families to eat as they watched the movie, Toys Story 3, which was a huge hit for the children.

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 23 other countries including: Argentina, Australia, Brazil, Canada, China, Czech Republic, Denmark, France, Germany, Greece, Iceland, Israel, Italy, Mexico, Netherlands, Pakistan, Slovakia, South Africa, Spain, Switzerland, United Kingdom, United States and Taiwan.

Cindy Bobolz, grandmother to Nora Gooden, and Bernice Croston, great aunt to Nora, handmade the most beautiful quilt to be raffled off at the conference! It was displayed at the registration area for all attendees to see. Not only was it beautiful to look at all weekend, but it raised funds for Families of SMA as well! Beverly Venedam, a long time volunteer and Aunt of Erin Trainor, was the lucky winner of the quilt.

Some of the presentations and videos from the 2011 Annual SMA Conference are now available on-line at www.SMACommunity.org
Something that made this year’s conference extremely special was all of the beautiful signs that were displayed all over the hotel. Expo Convention Contractors, of Miami, FL, Jennifer Miller Smith and Aaron Smith donated all of these signs to Families of SMA, worth over $30,000! There were spectacular signs and displays for both the Family Conference and the Researcher Conference. They also donated wonderful Pipe and Drape to display all of our conference banners at Thursday’s Family Fun Fest and Carnival, in our Children’s Program, as well as in our meeting spaces. 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 Jacob Isaac Rappoport Foundation and the Wyatt Kyle Sutker Foundation hosted another luncheon this year inviting all type I families to attend. It was another 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 over 200 attendees at this special luncheon, who had a wonderful time interacting with and supporting one another.

We would like to take this opportunity to thank all of the amazing companies and organizations for their generous support of the 2011 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 2011 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.

The 18 vendors represented at this year’s conference was a record and an amazing way to provide another service to families attending the conference by providing access and information on companies whose products may be of interest. 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.
Sunday started off with an exciting Disney Character Breakfast. Buzz Lightyear, Woody, Jessie and Bullseye, four of the Toy Story characters, were able to join our conference! Children and families took photographs with the characters and many received autographs from them.

The latest research developments were given to families at Sunday’s Closing Session which included a Research Update and Researcher Q & A. Leading experts in the SMA Research Community answered questions from SMA families. A summary and video of Sunday’s Closing Research Session is available on-line at www.SMACommunity.org
Our 2011 Children’s Program was extraordinary and, without a doubt, the best that we have ever had! 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 and so much more.

There was fantastic entertainment for the children to enjoy such as a face painter, a highly interactive stilt walker, a magic and comedy show, balloon making, build your own bear projects, a Knight’s adventure which included face painting, balloon twisting and a magic show fit for a King!

The children also enjoyed dance lessons and performed a fun dance during the Children’s Program.
The 2011 Annual SMA Conference brought together 1,500 families and researchers from around the world. At the conclusion of the conference the families gathered for a special and unique opportunity to hear about the very latest research announcements along with a Question & Answer session with the leading researchers and clinicians in the SMA field.

Videos of the entire session are now available at SMA Community Connections, www.SMACommunity.org.

The panels and speakers for this session held on Sunday June 26th included:

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

- **Major Findings from the 15th SMA Research Meeting and Next Steps.** Charlotte Sumner M.D., Assistant Professor, Department of Neurology, Johns Hopkins University.

- **Keynote: Why is Drug Development Difficult.** Douglas Kerr M.D., Ph.D., Medical Director, Experimental Neurology, Biogen Idec.

**Therapeutic Development/Drug Discovery/Translational Research Q&A Panels:**

**Panel 1 – Therapeutic Approaches Targeted at the Back-up SMN2 Gene:**

- James Rusche, Ph.D., Senior Vice President, Research and Development, Repligen Corporation

- Kathie M. Bishop, Ph.D., Director, Clinical Development, Isis Pharmaceuticals

- Charlotte Sumner M.D., Assistant Professor, Department of Neurology, Johns Hopkins University

**Panel 2 – Other Therapeutic Approaches (Targeted at Protection or SMN Replacement):**

- Brian Kaspar, Ph.D., Assistant Professor, Department of Pediatrics, The Research Institute at Nationwide Children’s Hospital, Columbus Ohio

- Katherine Klinger, Ph.D. Sr. Vice President, Genetics and Genomics, Genzyme Corporation

- Rebecca M. Pruss, Ph.D., Chief Scientific Officer, Trophos

- Chris N Airriess, PhD, Chief Operating Officer, M.D., MBA, Clinical Coordinator, California Stem Cell, Inc

**Clinical Research and Trials:**


**Panel 3 - Clinical Research Question and Answers:**

- Mary Schroth M.D., Associate Professor of Pediatrics, Director of Pediatric Pulmonary Center Grant, University of Wisconsin Children’s Hospital

- Thomas Crawford M.D., Associate Professor of Neurology and Pediatrics, Johns Hopkins School of Medicine

- Richard Finkel M.D., Professor of Neurology and Pediatrics, Director of the Neuromuscular Program, The Children’s Hospital of Philadelphia

- D Elizabeth McNeil, M.D. M.Sc., NINDS Office of Clinical Research
Photos Taken at the 2011 Annual SMA Conference Now Posted on the SMA Community Site

Many photos taken of children and families at the 2011 Annual SMA Conference in Orlando, FL are now posted on www.SMACommunity.org.

We were so very honored to have Kim Ruoff, an incredible photographer from Florida, attend the conference so that she could spend time taking photographs for SMA families. We had about 25 families who signed up and were able to have an individual photography session with Kim. Kim took thousands of photographs, each one so very special. These photos will be given to each family at no charge on a CD, so that each family can download and print all of these gorgeous images.

If you have any photos from the 2011 Annual SMA Conference, please email them to newsletter@fsma.org, as well as post them on the SMA Community site at www.SMACommunity.org. The conference photos are located in the 2011 Conference Group.

Families of Spinal Muscular Atrophy Announces Two Winners of The 2011 Annual SMA Conference Apple iPad Survey Giveaway

At the 2011 Annual SMA Conference in Orlando, FL, Families of SMA offered a wonderful incentive to all attendees who completed a conference survey this year. Families of SMA received a very generous donation of two Apple iPad’s, so any conference attendee that submitted a conference evaluation form before the Researcher Q & A, had their name entered into a raffle to win a new iPad! Bonnie Shiesley of New York, mom to Kale who has SMA type II, was the lucky winner announced that day, so Bonnie and her family were able to bring home a great new Apple iPad.

For those attendees who wanted to submit their surveys after Sunday’s Researcher Q & A at the Annual SMA Conference, we continued to accept surveys, both online and by paper version. A second winner was drawn on Sunday, July 24th at 9:00pm Central Standard Time. Congratulations to Pennie Adams of Texas, mom of two beautiful daughters with SMA Type II. Pennie was the 2nd winner of the SMA Annual Conference Survey Raffle!

Everyone at Families of SMA would like to thank the two amazing donors who provided us with these Apple iPad’s. Their generosity has made two SMA families very happy!

Thank you to all conference attendees who completed a conference survey. You have provided us with great feedback to consider for improving next year’s conference.

Congratulations Bonnie and Pennie!
2011 Annual Spinal Muscular Atrophy Conference Presentations and Workshops on Video Now Available Online

The 2011 Annual SMA Conference workshop presentations, as well as several videos of sessions, are now available online at www.SMACommunity.org.

Families of Spinal Muscular Atrophy is proud to announce another support program for SMA families and patients. Many of the conference presentations given during the 35 workshops are now available online. The presentations can be found at www.SMACommunity.org. Presentations will continue to be added as we receive them from conference speakers.

We are also offering several of the sessions on video including:

**The General Session Opening Remarks**

Presentations by:

*Paula Barrett*
Chair of FSMA Board of Directors
Board Governance of FSMA

*Kenneth Hobby*
President of FSMA
Progress and Where your Dollars are Invested

*Kathy Bishop, Ph.D.*
Director, Clinical Development, Isis Pharmaceuticals
Designing Effective SMA Clinical Trials

*Katherine Klinger, Ph.D.*
Senior Vice President of R&D, Genzyme Corporation
Orphan Disease Therapy Development

*Spencer Perlman*
FSMA Legislative Coordinator
Your Voice in Washington and Local Grassroots Advocacy

*Mary Schroth, M.D.*
Chair, FSMA Medical Advisory Council
Associate Professor of Pediatrics, University of Wisconsin School of Medicine and Public Health
Basics of SMA Care

**Hands on Physical Therapy**

Explores the overall general Physical Therapy needs of children with SMA. They provide and share examples of treatment ideas for the home that you can also share with your local PT. They also demonstrate the use of some therapy equipment that may help with your child’s PT goals.

Presenters of this workshop include:

*Kristin Krosschell, MA, PT, PCS,*
Northwestern University

*Karen Patterson, MS, PT, PCS,*
University of Wisconsin

*Jennifer Tucker, PT, DPT, PCS,*
University of Central Florida

**Breathing Basics and Care Options for SMA Type I**

Respiratory complications are a great concern for all children with SMA type I and their families. This workshop will discuss respiratory complications and choices for management including non-invasive ventilation (NIV) and invasive ventilation care and how to move secretions out of the airway.

Presenters of this workshop include:

*Dr. Richard Shell*
pediatric Pulmonologist and Associate Professor in the Section of Pulmonary Medicine at the Nationwide Children's Hospital and The Ohio State University in Columbus, Ohio

*Dr. Richard Kravitz*
Associate Professor of Pediatrics in the Division of Pediatric Pulmonary and Sleep Medicine at the Duke University Medical Center in Durham, North Carolina

**Good Nutrition for Children Fed by G-Tube**

These two dietitians provide a presentation on advancing the nutrition care of children with SMA, as well as a discussion on good nutrition for children fed through g-tubes. This workshop includes general information on calories, protein, fat, vitamins and minerals as well as water intake needs for children with SMA. Specific challenges in feeding children with g-tubes is discussed.

Presenters of this workshop include:

*Barbara Godshall, MMSc, RD, CSP, CD, LD, CNSD*
Pediatric Dietitian at Cincinnati Children's Hospital Medical Center in Cincinnati, Ohio

*Rebecca Hurst, MS, RD, CD*
University of Utah Clinical dietitian at the University of Utah's Pediatric Motor Disorder Research Program in Salt Lake City, Utah

Conference presentations and videos are now available online at www.SMACommunity.org. All Videos and Presentations are located in the Conference tab.

If you have not done so already, you must first register at www.SMACommunity.org to view the conference materials. Registration is simple and free.
2011 Annual Spinal Muscular Atrophy Conference
Leading Researchers Give Talks on Drug Development and Basic Research at the SMA Conference During the Researcher Q&A Session

Excerpts from talks given by Dr. Jill Jarecki, Dr. Charlotte Sumner, and Dr. Douglas Kerr are found below. Please see http://www.fsma.org/Research/News/ to read the full summaries of the three talks and to assess the slides from each of the talks.

Videos of this session are available at SMA Community Connections at http://www.smacommunity.org.

Excerpt from “Update on Families of SMA Research Activities”; Jill Jarecki, Ph.D., FSMA 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 $50 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, and 3) Clinical Trials to provide the means to test new drugs effectively. 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.

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, both with our basic research funding (example ASOs and ISIS given earlier) and with our direct drug discovery funding. Two 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 about a $5 million award from NINDS to progress to an IND.

FSMA is dedicated to creating a treatment and cure for Spinal Muscular Atrophy (SMA) by funding and advancing a comprehensive research program, as Dr. Jarecki just described. To further this aim, the FSMA Board of Directors recently approved almost $3 Million in new research investments for 2011. The current awards will be divided into three distinct research areas: 1) $700,000 for Basic Research 2) $1.5 Million for 2 new Drug Discovery Programs, and 3) $500,000 for Clinical Research. Funding will also include $250,000 for the 16th Annual SMA Research Group Meeting to be held in June 2012, the largest scientific conference dedicated to SMA research in the world.

Excerpt from “Major Findings from the 15th Annual SMA Research Group Meeting and Next Steps”; Charlotte Sumner, M.D., Assistant Professor of Neurology, Johns Hopkins University, FSMA Translational Advisory Board.

Dr. Sumner started out her talk with the idea that 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. SMA has a unique opportunity for therapy development, due to a low-functioning copy of this gene called SMN2. 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 perhaps by stabilizing the SMN protein.

It is important to recognize that understanding the basic biology of SMA is what leads to effective therapy development. There are many questions we needed answered to make good drugs. These include understanding: 1) where SMN protein is used 2) what the protein does, and 3) how the reduction of SMN makes motor neurons sick.

More detailed understanding of these issues have led to our current list of drug strategies, which includes gene therapy, SMN2 activators, SMN2 splicing correctors, and cellular replacements. Basic research is, thus, very intertwined with drug development as it defines therapeutic.

Many different approaches to treating SMA have arisen from data the scientific community has learned from basic research studies, as Dr. Sumner just discussed. These include drug approaches that replace the SMN1 gene, like gene therapy. Those that activate the back-up gene SMN2, such as HDAC inhibitors, hydroxyurea, or quinazolines. There are also current drug candidates that correct SMN2 splicing, like the PTC compounds, the ISIS ASOs, and the Paratek compounds. There are neuroprotection strategies, such as stem cell therapy or the Trophos compounds that directly impact motor neuron survival and functionality. There are strategies based on preventing muscle atrophy and increasing muscle strength directly. As we discover more about the basic pathology of the disease more and more strategies arise as feasible points of therapeutic intervention.
In conclusion, understanding the basic biology of SMA is critical in finding new therapeutic interventions. Basic biology research into SMA has led to the multiple drug candidates currently on the horizon, but we still have much to learn in order to treat every type of SMA at every point of disease progression, which is the ultimate goal of all SMA researchers and of FSMA.

Excerpt from “Overview of the Drug Development Process”; Douglas Kerr M.D, Ph.D., Medical Director, Neurology, Biogen Idec, Member FSMA Scientific Advisory Board.

Dr. Kerr discussed the general drug development process from an industry perspective. He started out 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. This is his first conference as an industry representative.

He began by discussing why SMA has become attractive to Drug Companies. This includes factors like: 1) known cause: SMN deficiency, 2) back-up gene, 3) high unmet need, 4) belief the problem can be treated, 5) orphan disease status, and 6) profit potential.

Dr. Kerr next summarized the overall drug discovery 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, 3) then to FDA approval of the IND application allowing the start of human trials, 4) to safety studies often in healthy adult volunteers, 5) to Phase II proof of concept studies in patients, and 6) finally to larger Phase III pivotal FDA registration trials. The process even extends to post-marketing, where some safety issues only arise when very big populations are using a drug. In orphan diseases the overall process can sometimes be streamlined, but the total process typically takes 8 to 15 years.

Many different groups are critical to making a drug during the different stages of the process. In the early stages when the drug strategies are first identified, advocacy groups and government typically fund this work, which mostly takes place in academic labs. Families of SMA has played a big role here. Translational research (or also called pre-clinical drug development) is also funded by these groups and by small biotechnology companies. FSMA has played a big role here too by involving companies like deCODE, Paratek, and Repligen to engage in this type of research. FSMA has been instrumental in developing early innovation in regard to industry/academic partnerships in the translational research phase of drug development.

Typically it is critical that industry becomes involved in the drug process at some point, because it is long, expensive, and requires very specific expertise and resources. Often in orphan drug development, smaller-sized biotechnology companies (biotechs) are the first to engage during early drug development, which begins at IND approval. Small biotechs can run early stage human studies, but often engage a larger pharmaceutical partner for commercial and late-stage development activities. These activities are so complicated in terms of resources and money that they may be hard for smaller companies to execute on their own.

So what does it take for the FDA to allow drugs to be tested in humans? It takes many things, but 2 major ones. The drug must be shown to have benefit in animal and in-vitro studies. There must be extensive preclinical safety data supporting human use.

The earliest stages of a clinical drug program are designed to determine one major thing. Does the compound do ANYTHING (useful or deleterious) at all when given to a human? It is critical to determine this as quickly as possible. Specifically Phase I studies are used to determine the pharmacologic actions of a drug with special emphasis on safety and tolerability. The emphasis is not on efficacy, although occasionally it is possible to find early evidence of effectiveness.

Phase II trials are small studies in patients with well-defined inclusion and exclusion criteria. Phase II studies add to the information about the common unwanted pharmacologic properties of the drug (i.e. short term side effects). Most importantly, these studies include “proof of concept” measures / endpoints to determine if a drug works (called efficacy). Phase III studies are usually larger and include a broader patient population. These trials are designed for robust determination of safety and efficacy to figure out the overall risk-benefit relationship of the drug. These are the studies used to support FDA approval. Sometimes Phase II and III studies can be merged in orphan diseases.

What does the FDA consider when approving a new drug? Many things factor into this consideration. These include: 1) Is the drug safe and effective for its proposed uses? 2) Do the drug’s benefits outweigh its risks for this indication? 3) Is the drug’s labeling appropriate, i.e. is it being used in the right patients for the right things? and 4) Are the methods used in manufacturing adequate to assure its identity, strength quality and purity.
The 2011 Annual Spinal Muscular Atrophy Conference Received Support from the Florida Delegation

Families of Spinal Muscular Atrophy is proud to have received the support of four members of the Florida Congressional Delegation for The 2011 Annual SMA Conference.

Four members of the Florida Congressional Delegation sent letters of welcome and support to Families of SMA in honor of the 2011 Annual SMA Conference that took place at Walt Disney World in Orlando, Florida from June 23rd-26th. Both of the State’s U.S. Senators, Bill Nelson (D) and Marco Rubio (R), as well as Representative Daniel Webster (R-Orlando) and Representative Cliff Stearns (R-Gainesville), expressed their hope for a successful Conference. Families of SMA is appreciative of the support shown by the Florida Delegation.
In the Fall 2011 issue of “Compass” Families of Spinal Muscular Atrophy announced seven new basic research grant awards. These new awards encompass $745,000 for the basic research portion of the $3 Million in new research funding that FSM A announced earlier this year.

What is Our Research Goal at FSM A?
Our main goal is to accelerate the discovery of an effective therapy and cure for SMA by funding and advancing a comprehensive research program, using a three-pronged approach:

1) Basic Research to reveal the best ways of making SMA drugs
2) Drug Discovery to make new drugs
3) Developing Clinical Trial infrastructure to help test new drugs

Why is Basic Research Important to Our Overall Strategy?
Basic research is a critical component in finding a treatment for SMA. It provides fundamental information about what is going wrong in SMA by telling us when and where SMN protein is needed, and what SMN protein does in different cell types. This knowledge provides seed ideas for new ways of making drugs.

The FSMA basic research program is governed by our Scientific Advisory Board (SAB). The SAB carefully reviews all grant applications to ensure that we fund only the best quality research relevant to our mission.

What’s Exciting About Our New Research Grant Awards?
Three of the seven new grants for 2011 will help us to understand why motor neurons are selectively vulnerable to lowered SMN levels compared to other tissue types. These grants include projects led by Dr. Henderson at Columbia University, Dr. Rossoll at Emory University, and Dr. Kothary at the University of Ottawa.

The grant to Dr. Ko at the University of Southern California will help us understand exactly where defects in SMA occur and the reasons for observed selectivity at different muscles. The grant to Dr. Monani at Columbia University will help identify new genes that are protective against lowered SMN levels and will lead to new drug targets. Finally the funded projects led by Dr. Kolb at The Ohio State University and Dr. Simard at the University of Manitoba will validate molecular biomarkers that could make future clinical trials more efficient.

Since 2004, the FSMA Scientific Advisory Board has awarded 66 basic research grants for a total of $8,195,096 in funding. This is in addition to our drug discovery and clinical research funding. The grants have been awarded to 47 different principal investigators at 33 different institutions.
SHARING

SMA Candle Lighting for SMA Awareness

The following are the SMA Awareness posts that were made by families on our Facebook page.

Please join our Facebook Page at www.facebook.com/familiesofsma

- Angel Young has her candle lit for all the children and adults who have lost their battle and who continue to fight SMA and SMARD every day.
- Brooklynn Santos saying a prayer for all the SMA warriors and angels!
- For my son Michael Joshua Lum, he would have been 3 next week, August 21, 2008 – October 14, 2008, SMA Type I.
- For Philip Struble.
- This candle is lit for Chloe Ochoa (SMA Type III), all her SMA friends and the SMA angels who have sadly lost their battle with SMA. Hoping for a cure soon!
- In memory of our angel, Kennady Quinnell, and for all the SMA angels and warriors. God bless you all and your families!
- We’ve lit our outside torch for our grandson, Logan Terborg, and all the others that have SMArd or SMA.
- I lit these candles for Sawyer Ament and all the other SMA children and their families. For all the angels and warriors that have lost their battle with SMA and to those who continue to fight every day.
- Courtney Rosas, SMA Type I, 8 year old miracle, with her mommmy Candace. Courtney shining her light for SMA awareness.
- This is Andy Butler’s candle - it sits next to his picture in our bedroom, reminding us that he is with us always. Tonight, we lit it in memory of those we have lost to SMA and in honor of those still fighting the disease.
- Cole Pulkrabek - 8 years old.
- Veronica St. Onge, SMA Type I, almost 7 years old, lights her candle to remember and to honor.

• We lit our candle in memory of our angel Andy Butler and all of his friends. –The Butler family

• In memory of all of the SMA Angels who have lost their battle and in honor of those fighting everyday!! Bless you all! Xo

• In memory of Our Annie Girl! (October 13, 2009 – August 6, 2010) Annie Grimes would have been 22 months old today!

• Zion Hoskins, SMA Type II. Praying for all the warriors and angels of SMA. We love you all!

• Our candles from my son’s birthday cake.

• We lit our candles tonight in honor of our niece, Carlee Beam, who was diagnosed with SMA last year, and to remember all those affected by SMA and those sweet angels.

• Along with lighting two candles outside the sign my daughter and her dad made lights up every night. She does this for her best friend Kale Shiesley that has SMA.

• I’m lighting a candle for my sister April, who lost her battle with SMA Type II on 1/4/07 at the age of 25. I miss her dearly, but feel her with me everyday. My prayers are with those who are still with us.

• Worldwide SMA Candle Lighting - August 13, 2011-- Ally Krajewski, 5½ years old with SMA Type I.

• Ally’s praying for her friends who also have SMA and especially those who are Angels with God!

• We light our candle for all the angels who were taken away because of SMA and all of the children still fighting every day. Mommy and Daddy love you Ryan. We are so proud of how tough a little boy you are.

• Aiden Bundy Type I, 4½ yrs old.

• For all of the SMA Angels who have lost their battle with SMA and to honor those who are still fighting everyday!!

• Roman Anderson Type I, 4 years old.

• For my little Warrior Princess, Greer Ramsay and for all SMA Warriors & Angels. For those still fighting every day and for those whose battle is lost. Cure SMA.

• Brooke and Brielle Kennedy, SMA Type II.

• For Eva Grace Kelly, The Kelly Family~ ~For all battling SMA. May God Watch Over You.

• Claire & Lauren Gibbs.

• Sohum Shah and his friends lighting the SMA Awareness candle.

Continued over......
‘Twas The Month of SMA Awareness

By Barb Zahn

‘Twas the month of SMA awareness, and all over the world
Against all new mothers, sweet babies are curled.
Their newborn clothes hung in the closet with care,
In hopes that they soon will be big enough to wear.
These babies are nestled all snug in their beds,
While visions of birthday parties dance in their parents’ heads.
First bike rides, first dances, a high school graduation -
Perhaps even sending out their wedding invitations.
When at the doctor’s office, there will arise such a clatter,
Specialists will be called, to see what’s the matter.
Away for tests, these babies are whisked like a flash,
For it’s something far worse than a cough or a rash.
Blood draws, MRIs, and so much more to go,
You shake your head, cry, scream out the word “NO!”
Something that’s worse than any of your fears -
Your child’s life may be over in less than two years.
“SMA” the doctors say, and you stumble on the words,
This isn’t a name that you’ve ever heard.
Why weren’t you tested, before your baby came?
Spinal Muscular Atrophy, you shudder at the name.
Now pulse ox, now cough assist, now bipap and g-tube.
They can’t cough, they can’t breathe, they’ll choke on their own food.
To the therapist’s office! Break down insurance’s wall!
Respiratory treatments, surgery, surgery for all!
As you learn your child, your worries will fly,
When you meet with an obstacle, you will fight - sometimes cry.
So up against the world, to the naysayers, say “Shoo!”

You and your child have lots of living to do.
And then, in a twinkling, your child will grow,
When all those doctors told you to prepare for them to go.
You realize that your child is the one making the rules,
And it’s leaving these doctors stumbling like fools.
Though your child may never put weight on a foot,
They are stronger than most - you can tell with a look.
They are always moving forward, no time to look back,
So inspiring, they leave no time for you to slack.
Their eyes - how they twinkle! Their faces - so sweet!
Their skin is so soft, minds as sharp as any you’ll meet.
Mouths that may never utter a word,
These children don’t need voices to be heard.
They have parents - our children are blessings bestowed.
To enrich our lives, to help us be bold.
To teach us that what we feel in our heart,
Is more than enough to give us a start.
To find them a cure, to show them we care,
To tell them no matter what, we’ll always be there.
Fighting with doctors, with insurance, with school.
To give them the chance, to provide them the tools.
And to spread on the message, to get out the word -
Because we’re their voices, we need to be heard.
Not just this month, though it’s a good place to start.
Determination must be in EVERY heart.
So we’ll fundraise, we’ll write, we’ll blog and we’ll sell,
We’ll bake, we’ll sew, and always we’ll tell
All about this disease. Until SMA is out of sight.
And we can say - without a bipap, without a feeding pump, without a pulse ox - "My child, sleep tight."
Families of Spinal Muscular Atrophy Announces Repligen Corporation Receives FDA Approval to Begin Phase I Clinical Trial in SMA

First ever clinical trial approved for SMA with a novel drug specifically designed to treat the disease.

Families of Spinal Muscular Atrophy announced May 19th, 2011 that Repligen Corporation has received approval from the US Food and Drug Administration to commence a Phase I safety study in healthy volunteers for RG3039, formerly called Quinazoline495, which is being developed for Spinal Muscular Atrophy.

FSMA began the Quinazoline program in 2000 at the very initial stages of drug development, when risk is the highest. It was the very first industrial drug program for SMA ever conducted. FSMA fully funded the program with investments of $13 Million. The direction from FSMA provided the positive results necessary to license the program to Repligen Corporation and leverage larger funding for clinical development.

This clinical trial will be a double-blind, single ascending dose, Phase I study in healthy, adult volunteers to evaluate the pharmacokinetic and safety profile of RG3039 in up to 40 subjects. The study will be the first step in the clinical development of RG3039 as a potential treatment for SMA.

“It is very exciting after 10 years of hard work by FSMA leading this program, including a significant financial investment, for the drug candidate to be licensed to Repligen in 2009 and to now be able to announce the approval to advance into human clinical trials. This is a tremendous milestone for our community to have reached as we make progress toward a treatment for SMA,” stated Jill Jarecki, Ph.D. Research Director at FSMA. “We are very pleased to have received approval to initiate human clinical trials with RG3039,” stated Walter C. Herlihy, President and Chief Executive Officer of Repligen Corporation.

SMA, the leading genetic killer of children under the age of two, is typically marked by the degeneration of muscle movement including the muscles that control crawling, walking, swallowing and breathing. There are no approved therapies for the treatment of SMA to date, which affects one in every 6,000 babies. One in every 40 people carry the gene that causes SMA, indicating approximately 7.5 million carriers in the United States.

One of the goals at FSMA is to fund early stage drug discovery programs for SMA to the point where other groups will invest. At the earliest stages of drug development, programs have less than a 1% chance of success. This inherent risk along with low potential for profit due to a small patient population has traditionally hindered industry from working on orphan diseases.

FSMA has actively reduced the barriers to SMA drug discovery programs by providing: 1) early seed funding, 2) access to tools and reagents, 3) expert SMA advisors, and 4) established clinical trial protocols and networks.

FSMA recently released a Request for Proposals for new preclinical drug discovery programs to build a robust drug pipeline for SMA. Partnerships such as this one with Repligen, between non-profits, the government and companies are a very effective way to share the risks of developing rare disease treatments. This FSMA approach also enables the correct expertise for a particular stage of development to be brought into a program.

Repligen Receives U.S. Fast Track Designation and European Orphan Medicinal Product Recommendation for Spinal Muscular Atrophy

Repligen Corporation announced June 28th, 2011 that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for RG3039, a potential treatment for Spinal Muscular Atrophy (SMA).

Fast Track is a process designed to facilitate the development and expedite the review of drugs that treat serious diseases and fill an unmet medical need. Once a drug receives Fast Track designation, frequent communication between the FDA and the sponsor is encouraged throughout the development and review process. In addition, RG3039 has received a positive opinion for orphan medicinal product designation from the European Medicines Agency. European orphan medicinal product designation aims to encourage the development of drugs involved in the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition that affects no more than five in 10,000 persons in the European Union.

“Receipt of Fast Track designation and a positive opinion for European orphan medicinal product designation for RG3039 demonstrates the FDA and EMA commitment to the study and development of treatments for rare and serious diseases,” stated Walter C. Herlihy, President and Chief Executive Officer of Repligen Corporation. “This regulatory support adds momentum to our efforts to develop a novel treatment for patients with Spinal Muscular Atrophy.”
Hills West Valedictorian Stands Tall Despite Disability

Dianna Hu is at the top of her class and Harvard-bound even as she battles degenerative muscular disease.

By Rick Karas
Half Hollows Hill Patch, June 9, 2011

Half Hollow Hills West Class of 2011 valedictorian Dianna Hu of Dix Hills, New York is heading to Harvard.


That last line is not a typo. Neither is anything that came before it. It’s the resume of Dianna Hu, valedictorian of the Hills West Class of 2011.

“I think it’s such an honor because everyone at High School West is really dedicated to their work,” Dianna said upon learning she was head of the class. “Even more than that we’re dedicated to helping each other, I think that’s what comes first and foremost.”

No surprise, she’s heading to Harvard in the fall, with math and science as her primary interests. What may surprise you is that Dianna is largely confined to a wheelchair because of a disability she’s had since birth. Spinal Muscular Atrophy (SMA) is a neuromuscular disease that affects muscles associated with the spinal cord, and 1 in 40 people carry the disease.

SMA can certainly make navigating the halls of high school a challenge. Dianna has an aide to assist her, and she undergoes physical and occupational therapy at school and at home. Suffice to say, it hasn’t affected her in the classroom one bit.

“Although it affects my physical body,” she said, “it doesn’t really affect my mind at all and I think that’s what’s really so powerful, that I can still use my intellectual power to compensate for the lack of physical power.”

She’s compensated all right, with a near-perfect grade average, and SMA has spurred Dianna to do research at Stony Brook with Dr. Carlos Simmerling, a renowned chemistry professor. She’s even done work with a protein associated with her disability.

Her accomplishments in the face of physical limitations have not gone unnoticed by the staff at Hills West. Specifically, her upbeat attitude through her time there.

“The fact that she’s valedictorian for me is secondary,” assistant principal Frank Pugliese said. “[Secondary] to the fact that she does carry on with this incredible attitude and just incredible love of life.”

Dianna has spoken in front of large crowds, at disability awareness panels, for example. That experience should serve her well in her next challenge: addressing the graduating class as valedictorian.

“I imagine maybe my graduation speech might have a little flavor of that, it might just reflect on all my years being here and just being so loved by everyone,” Dianna said.

Dianna says she loves solving riddles, but one that’s easy to decipher is her fondness for Hills West, and those that have made her journey through its halls a stimulating one the last four years.

“It’s a sense of poignant nostalgia, I would love to be here forever if I could,” Dianna said. “When you’re surrounded at school every day by people that you love, it’s all great in the end.”
My husband and I conceived our second child just four months after we lost our first child to Spinal Muscular Atrophy. The decision to try to have a baby so soon after our son Andy’s passing was a difficult one. However, we hated being “childless parents” and felt ready to introduce another life to our family. After weighing all of our options, we conceived the baby naturally, and we prayed that the odds would be in our favor this time. It was assumed that, since there was no question about Andy’s SMA diagnosis, both my husband and I were carriers, which gave us a one in four chance of having another SMA-affected child.

We had to wait until I was 12 weeks pregnant with our second child before the pre-natal testing for SMA could be done and three additional weeks for the test results. It was an excruciatingly stressful and long wait. As a condition of the pre-natal testing, my husband and I also had to have blood tests to determine our carrier status.

So we were surprised to receive the result that, while I was a carrier of the gene that causes SMA, my husband appeared to not be a carrier at all or have any type of mutated gene that would cause the disease. We also learned that the new baby, a girl, was an SMA carrier, but would be unaffected by the disease. While we celebrated the health of our daughter Lucy, we were left with many questions. The most pressing being that, if my husband wasn’t a carrier, how did Andy have SMA?

Our geneticist gave us two potential answers. Either my husband WAS NOT a carrier, and the gene he passed to Andy mutated upon conception (called a “de novo” mutation), or he WAS an SMA carrier with a genotype that the blood test could not detect. She said that de novo mutations occurred in approximately 3 percent of the population of people diagnosed with SMA. Also, she explained that there is a subset of approximately 6 percent of carriers that can not be identified by the current blood test.

In order to find out more, our family recently participated in what is called a “Linkage Study.” Blood samples were taken from each of our parents, my husband’s siblings, each of us, and Lucy to determine who passed what gene to whom. Luckily, we had Andy’s cord blood stored as well, so we could get a complete picture of how the SMA-causing gene had traveled through our family to him. Scientists at the University of Pennsylvania conducted the study.

We received the results yesterday, learning that my husband is indeed a carrier of SMA.

So why am I sharing this story with you? Because if we had had carrier testing before we had children, the test results would have indicated that my husband wasn’t a carrier. I would bet that the doctor explaining those results to us would have said that we had nothing to worry about. AND WE STILL WOULD HAVE UNKNOWINGLY HAD A CHILD WITH SMA.

It is critical to understand that the SMA carrier test, while very important, is not 100 percent accurate. If you received a “normal” result from an SMA carrier test, but it is confirmed that your partner is a carrier, there still is a possibility that you might have a child with SMA. That said, we are recommending that everyone in our family who plans to have children test for their carrier status. And, if any of them are carriers – regardless of their partners’ test results - that they also elect to have pre-natal testing done with any pregnancy.

This piece of knowledge is Andy’s gift to us all.
On April 7th, the Families of Spinal Muscular Atrophy SAB met to assess 34 basic research grant applications from around the world for funding. The FSMA TAC met the next day to evaluate 5 new drug discovery programs for Spinal Muscular Atrophy. Families of SMA will now use their funding recommendations for our budget planning and fund raising.

FSMA has been funding basic research since our inception in 1984, and has invested over $25 million in this area alone. Our basic research grant program has funded 140 projects to 70 institutions around the world.

Families of SMA believes continued investment in basic research, leading to greater understanding of the exact nature, causes, and consequences of Spinal Muscular Atrophy, is key to ensuring the most effective SMA treatments can be identified and developed as quickly as possible. The ideas that arise from basic research form the basis of our drug discovery programs.

For instance, Dr. Arthur Burghes, our longest standing SAB member and Professor at Ohio State University, has stated: “Basic research has made major strides in SMA, leading to promising potential therapies that work when given early in SMA mice. However, these treatments need to work in humans and we do not yet know that this is the case, or when they have to be given to be effective in humans. Basic research still has a major role to play and will impact SMA therapies, not just the ones we know today but new approaches that could for example influence later stages of the disease.

Whether the therapy is a small molecule drug, gene therapy, an antisense oligonucleotide, a cellular therapy, or something yet unknown. An attack from multiple points is most likely to work and lead us to a treatment for SMA.”

FSMA is aiming to fund two new preclinical drug development programs in the next year, with more to follow. These multi-million dollar collaborations will focus on innovative methods of developing novel therapies for SMA, including both biologic and small molecule approaches. These programs will be multi-year in scope with a typical duration of three years.

Our TAC is made up of experts from multiple facets of drug development. The use of the TAC to select drug programs for funding fits perfectly with the overall FSMA research-funding model, which is based on the need for expert and independent prioritization and oversight of research programs. This approach ensures that FSMA funds only the most promising research, and that funded programs are run in a professional and efficient manner under the guidance of world-class experts.

After the projects are selected for funding, the TAC helps FSMA manage and oversee them through the formation of a joint steering committee for particular projects.

This program follows a long legacy for Families of SMA in enabling drug development programs for SMA. FSMA has been investing in and advancing pre-clinical drug research since 2000, with a total investment of $17 million in this area.

FSMA Drug Discovery Goals: As demonstrated by our past funding of preclinical drug programs, one of the long term goals at FSMA is to fund and de-risk early stage drug discovery programs for SMA. At the very earliest stages of drug development programs have less than a 1% chance of FDA approval. This inherent risk along with low potential for profit because of the small patient population has traditionally hindered industry from working on orphan diseases.

The follow-on investments made by the government, industry, and other groups in FSMA programs help demonstrate the value and success of the FSMA model and its scientific expertise. Partnerships between non-profits, the government and companies are a very effective way to share the risks of developing rare disease treatments. This FSMA approach also enables the correct expertise for a particular stage of development to be brought into a program.

Our overall goal in this area to build a deep and diverse therapeutic pipeline to maximize our chances for success in finding a treatment for SMA. FSMA has actively reduced the barriers to early stage SMA drug discovery programs by providing: 1) early seed funding, 2) access to tools and reagents, 3) expert SMA advisors, and 4) established clinical trial protocols and networks.
Families of SMA thanks the following members of our advisory groups for their dedication and efforts in our mission to develop a treatment and cure for SMA.

- Brian Pollok, Ph.D., Chief Scientific Officer, LIFE Technologies, Assay Development.

- Lee Rubin, Ph.D., Director of Translational Medicine, Harvard Stem Cell Institute, Neuronal Assay Development and High-Throughput Screening.

- Christine Brideau Ph.D., Executive Director, In Vitro Pharmacology, Merck & Co., Preclinical Drug Development.

- Jim Inglese, Ph.D., Deputy Director, NIH Chemical Genomics Center, Assay Development and High-Throughput Screening.

- Peter Hodder, Ph.D., Senior Director, Lead Identification, The Scripps Research Institute, Scripps Florida, Assay Development and High-Throughput Screening.

- Michael Vellard, Ph.D., Principal Scientist, BioMarin, Preclinical Drug Development.

- Joseph W. Lewcock, Ph.D., Group Leader, Department of Neurobiology, Genentech, Inc., Preclinical Drug Development.

- Arthur Burghes, Ph.D., Professor, The Ohio State University, SMA Biology.

- Charlotte Sumner, M.D., Assistant Professor, Johns Hopkins University, SMA Biology/ Neurology.

- Peter Grootenhuis, Ph.D., Senior Director, Vertex Pharmaceuticals, Medicinal Chemistry.

- Timothy Reilly, Ph.D., DABT, Director, Bristol Myers Squibb, Toxicology.

- Ex-officio member: John Porter, Ph.D., Program Director, NINDS, NIH, Neuromuscular Disease.

- Jasbir Singh, Ph.D., President, Jasin Discovery Solutions, Medicinal Chemist.

- Tom Crawford, M.D., Associate Professor of Neurology and Pediatrics at the Johns Hopkins School of Medicine.

- Douglas Kerr, M.D., Ph.D., Associate Director, Experimental Neurology at Biogen Idec.

- Adrian Krainer, Ph.D., Professor at the Cold Spring Harbor Laboratory.

- Rashmi Kothary, Ph.D., Associate Director and Senior Scientist at the Ottawa Hospital Research Institute and a Professor at the University of Ottawa.

- Samuel Pfaff, Ph.D., Investigator of the Howard Hughes Medical Institute and Professor at the Gene Expression Laboratory at the Salk Institute.

- Mark Rich, M.D., Ph.D., Associate Professor in the Department of Neuroscience, Cell Biology, and Physiology at Wright State University.

- Kathryn Swoboda, M.D., Associate Professor of Neurology and Pediatrics at the University of Utah School of Medicine.

- Katherine Klinger, Ph.D., Genzyme Corporation, Sr. Vice President, Genetics and Genomics.

Families of Spinal Muscular Atrophy Receives Grant from the National Institute of Neurological Disorders and Stroke to Support the 15th Annual SMA Research Group Meeting

Families of SMA has received grant support from the National Institute of Neurological Disorders and Disease (NINDS) at the National Institutes for Health (NIH). We thank them for their fantastic support that has helped 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 23rd – 25th at the Swan and Dolphin Resort in Disney World. We had about 225 researchers who gave more than 100 presentations on the latest breakthroughs in SMA research.

The research conference is held in conjunction with The Annual SMA Family Conference, which is the largest conference in the world for those affected by SMA and for those involved in providing support and care for SMA patients.

Running these two conferences simultaneously gives the opportunity for families affected by SMA and the researchers and clinicians who are looking for a treatment and cure, to interact.
Meet Martyn Sibley of Disability Horizons

Martyn Sibley has SMA type II, he lives in London - UK, has been a trustee for the UK charity ‘Jennifer Trust for Spinal Muscular Atrophy’ (JTSM) and is aiming to change the world for disabled people. We caught up with Martyn via email and asked him to share his personal story, current activities and future plans.

Hello to the Families of SMA in the USA! I am Martyn, a 27 year old guy across the pond and have been lucky enough to write for your newsletter. Thanks so much!

Starting way back, my first memory relating to SMA was getting my first electric wheelchair. I was three years old, could not drive it safely and caused carnage in my parents’ home. I attended the small, local village school where I was the only disabled person and really enjoyed a very integrated childhood.

At age eleven, I had to travel further because the local secondary school wasn’t accessible, but I kept my old friends, made new friends and after an unhappy first year, I began to settle in and excel. I then went onto university, gaining a degree in economics and masters degree in marketing.

Attending university was a big step for me. It was the first time my parents didn’t do my personal care. This meant four strangers on day one helping me undress, hoist and shower. Slightly strange, however, it was worth it because beyond the drinking and the degrees I learned about life. I met great people, learned to drive a car with hand controls, flew to Australia one summer and broadened my horizons.

Post university was very difficult. Finding a job, an accessible house, employing and running a care team and wanting to do this in London (not as near to my family) was all extremely hard. After a couple of years fighting through the challenges, life calmed down. I was then able to turn my attention to my career, my social life and more travelling (I have since been to California, Mexico and the Canary islands to swim with dolphins and SCUBA dive, as well as flying a plane in the UK). Essentially I had built the foundations and was ready to really grab life.

My career took me to a disability charity called Scope http://www.scope.org.uk/ where I was a fundraiser. Having learned more about disability as a social problem my creative juices began to flow. The next thing I knew I was harnessing my personal and professional life through my blog http://www.martynsibley.com/. With my twitter handle, @martynsibley I have grown my online community to over 10,000 people worldwide. Alongside this my good friend Srinivas Madipalli and I launched our own online magazine http://disabilityhorizons.com/ which we have big plans for too.

Right now I am self employed, running my own new media and social business for disabled people. I plan to change the world for disabled people. This is achieved by showing disabled people everything is possible, the sky is the limit and show them how. Meanwhile, with an online tribe of disabled people aspiring and achieving, society will have to adapt. Buildings will need to be even more accessible, people’s attitudes will change positively and employers will see the value of disabled people in the workplace and so on.

I also plan to travel the world while I run my social enterprise. I get such a buzz from meeting new people in new places. I think in the end everything will tie together. In the UK winters, my health is bad, so warmer climates suit me well. Then while I am traveling, I can imagine working with International Development projects to support disabled people in developing countries too.

Who knows what the future holds, but I know I am personally and professionally very content at the moment. Having struggled, worked hard, had personal doubts and ignored most people’s sentiments, I am proud of myself at this precise moment. I hope in 40 years or so I will look back and see the part I played in disabled peoples’ lives, worldwide, being improved for the better.

If you have any questions, feedback or thoughts for Martyn you can contact him here http://martynsibley.com/give-me-a-shout.
Meet Srin Madipalli of Disability Horizons

Hello! My name is Srin Madipalli and I am 25 years old and I have SMA type II. I live in London and work as a lawyer at a commercial law firm in the city of London called Herbert Smith LLP. I graduated from University in 2006 with a first class degree from Kings College, University of London. Although I require 24/7 care, since starting university in 2003, I have lived independently with the support of PA’s who I directly employ and manage. When I went to university, I was lucky enough to live in halls of residence with a specially adapted room in the heart of central London and was able to make the most of student life. I now live in my own flat on London’s South Bank, which is conveniently near my office.

Even though I work very hard in my job, I also make sure I enjoy life to the fullest! Living in London means that there is never a shortage of great bars, clubs or restaurants to go to. I also really enjoy a range of other activities from going to museums, watching live music or seeing Arsenal play at the fantastic Emirates Stadium. One of the things that I hadn’t really done much of until recently was travel. It was something that I feared. I feared things breaking down. I feared being manually lifted. I feared the potential lack of accessibility. As a result, it had been many years since I had been anywhere and this was something I wanted to change. A unique opportunity to take some time off work for a few months then came up! Earlier in the year, my job at Herbert Smith was made permanent. Previously I had been a trainee on a fixed two year contract that was due to finish in September. While I really enjoy my job and all the opportunities it has provided me, one of the downsides is that it can be incredibly tough on hours. The deadlines on the cases and transactions we work on are usually tight and absolute, and that often means working late into the night or on weekends for prolonged periods, in order to meet deadlines. This can physically be an exhausting experience. So when I and my fellow trainee colleagues were offered permanent roles, we were also offered the chance to have some time off before starting our new roles.

As I had never taken any gap years, had always worked during summer holidays and hadn’t really taken a holiday in years, I snapped up the entire four months of leave to go travelling. I have had the most incredible series of trips that have taken me around the world. During my travels I went to Scotland, France, Belgium, Italy, Spain, Poland, the US, South Africa, Singapore and Bali. The main highlights of my travels were road tripping through California with my great friend and fellow SMA’er Martyn Sibley, going on safari in South Africa and seeing a pack of lions in front of our van and scuba diving in Bali. The scuba dive was the most extreme thing I have ever done, but it was also the most exciting and exhilarating. I have never felt such an adrenaline rush! A YouTube video of my dive can be found on my blog entry on martynsibley.com or on the following link http://www.youtube.com/watch?v=gFDEt1cgpw&feature=player_embedde.

I have learned so much about travelling with a disability and have pretty much overcome all my travel fears in the most direct of ways! On all my trips, I have only travelled with a single PA, and this was made possible by taking three fantastic pieces of equipment with me. The first is the Molift Smart hoist, which is relatively easy to fold and transport. The second is the Chameleon Shower Chair made by Borriniga, which is to date, the only shower chair I have ever found that can be collapsed and folded into a small carry bag. Thirdly and finally, on my travels, I used the Ottobock A200 wheelchair. This little wheelchair is incredibly versatile, maneuverable and easy to dismantle, but at the same time is sufficiently robust and tough to withstand the challenges of air travel. Not-withstanding the importance of a good PA and appropriate equipment, equally vital was finding accessible transport and accommodations. While this is relatively easy in Europe, the US and Singapore, in South Africa and Bali I had to use local tour operators that specialized in disabled travel since wheelchair accessible vans and accessible hotel rooms with a roll in shower chair were not common place. I have tried to collate this treasure trove of accessible travel information into a website recently created by Martyn and I called Disability Horizons (www.disabilityhorizons.com). We made this website to share articles written by a number of contributors on a variety of issues that are important to us such as travel, leisure, going out and simply having fun, which we hope people find both informative and entertaining. In this site, we also have noted every accessible hotel and transport provider we have used, as well as other bits of useful information. Even though I have SMA type II, I have seldom let it get in the way of what I ever wanted to achieve in life. An individual’s focus in life often determines their reality, and as I have always focused positively on what can be done, subsequently, I like to think that the reality of my life reflects that approach.
What was SMA Camp? It was many things: fun, adventure, hope. It was a forum for families to come together and share, and laugh, and cry and then laugh some more. It was a place where our children, living each day with SMA, became the majority; where siblings met, connected, and formed lifelong friendships that stretch across the miles. It was a wealth of information for the taking, networking, brainstorming and sharing what works and what doesn’t. For parents it was more than one could ever learn from any doctor or specialist. It was a place where families and children living with SMA showed the world that just because life is different for us doesn’t mean it’s awful or unenjoyable, it’s just different.

Camp was a whirlwind of activities. For the kids, there were scavenger hunts, crafts, obstacle courses, relay races, amateur skit performances, accessible playground equipment, swimming at the lake, kayaking, hiking and even zip lining! All of these activities were completely accessible, not to mention irresistible. For the adults, there was the pure joy of watching your child, or as in my case children take off, either driving or running, without a glance back knowing that the world they were in at that very moment held no barriers for them. That the people surrounding them would not stare, or gawk, or point, and that should a dilemma arise surrounding them was a wealth of knowledge and helping hands to make anything possible.

SMA Camp was held in beautiful British Columbia at Cultus Lake. The Camp was held over four days with an additional two days at the end when an amazing group called CRIS came out and did all kinds of adaptive outdoor activities with families. There was hiking, and we are not talking about just walking along a trail here, the feeling I felt when I watched Holli, a fearless teen, being manoeuvred down a goat trail beside a rushing waterfall was one I will never forget, I don’t think Holli will either! Max was trekked through the shallow end of a lake, and dunked a little in the process during his big hike, all to squeals of laughter. CRIS set up a zip line, which was beyond fantastic. I watched Luke, another teen attending camp with his family, with bated breath as he was hoisted higher, and higher, and higher and then whoa, down the zip line he comes with a grin so wide you could see nothing else! I watched Maliyah kayaking with her entire family, and well contributed to the splashes with the oars, on purpose! I watched kids be kids, chair or no chair, laughing and playing and helping one another. Camp this year was phenomenal, as always. My kids are already talking about next year.

SMA Camp was an experience our family will never forget, as we honked our horn and drove away our hearts remained, there in that open field, that place where the echoes of the laughter and tears and good times that we shared are frozen in time until we return again.

To register for next year’s camp or if you have questions, please contact Susi Vander Wyk at fsmacan@telus.net

Cultus Lake is ½ an hour from the Sumas, Washington/B.C. Canada border crossing. Next year’s camp will be July 24th – 29th, 2012.
SMA Camp in Canada
The second set of results from the Families of Spinal Muscular Atrophy funded CARNI-VAL clinical trial in ambulatory children published in PLoS ONE. The trial was completed by the Project Cure SMA Clinical Trial Network. The trial was registered at clinicaltrials.gov with identifier: NCT00227266.

Multiple lines of evidence, including treatment of patient-derived cell lines, animal models and open-label pilot human trials, have suggested that valproic acid (VPA) might have a therapeutic benefit in patients with Spinal Muscular Atrophy. The SMA CARNI-VAL TRIAL was a trial designed to evaluate the effectiveness of combined oral VPA and L-carnitine in two different groups of children with SMA. Group 1 of this trial targeted non-ambulatory SMA children 2-8 years of age, randomized to receive placebo or treatment for the first six months, then active treatment for an additional six months. Group two of this study was a twelve month open-label trial of VPA and L-carnitine in ambulatory children with SMA. Group two of the study involved an ambulatory group of 33 genetically proven SMA “standers and walkers” (type III), between the ages 3-17 years.

Participants underwent two initial baseline assessments of functional over a 4-6 week period and then were placed on VPA and L-carnitine for 12 months. Functional assessments were performed at baseline, 3, 6 and 12 months. Several primary functional assessments (outcomes / trial endpoints) were used, including safety, adverse event data, and efficacy. Efficacy (potential benefit) was measured by change in motor function at 6 and 12 months using the Modified Hammersmith Functional Motor Scale Extend (MHFMS-Extend), timed tests of gross motor function, and a fine motor module. Secondary outcome measures were tested at 6 and 12 months. These included maximum ulnar compound muscle action potential amplitudes, muscle strength by handheld dynamometry, pulmonary function measures, and patient and care-giver Pediatric Quality of Life Inventory scores.

Twenty-nine patients completed the 12 month open label study; VPA and L-carnitine were generally well tolerated, with only 1 patient developing a serious adverse event (dehydration) during the study. At least one adverse event occurred in 85% of all subjects but these were mild and similar to those seen in the group 1 study. The most common adverse events were pneumonia, gastrointestinal symptoms, fever and fractures; all except gastrointestinal symptoms were considered unlikely due to study medication. Excessive weight gain and abdominal pain were the most frequent drug-related adverse events. There was no significant change in any of the primary outcome measures at six or 12 months. There were some changes in secondary measurements. Some pulmonary function measures showed improvement at one year as expected with normal growth. CMAP amplitude did significantly improve at six and 12 months, suggesting a possible modest biologic effect directly on motor neurons, but that change was not clinically meaningful in improving participant function.

The results of this analysis, coupled with the data from the group 1, indicate that VPA at the doses used in these studies is not effective in improving strength or function in children with SMA. The results also demonstrate that the outcome measures implemented in this study are feasible, well-tolerated and reliable, and can be employed in future clinical trials in ambulatory children with SMA.

“While we are obviously disappointed that VPA did not have a positive effect on function, this study did provide additional valuable experience in doing clinical trials in children with SMA”, says Dr. John T. Kissel, MD, Professor of Neurology and Pediatrics at The Ohio State University and Project Cure Investigator. “We are also intrigued by the apparent effect of VPA on CMAP, which suggests that the VPA may be exerting some modest positive biological effects. The recently completed studies of VPA in adults and in type 1 infants should generate additional information on this important issue.”

In addition to testing the safety and efficacy of repurposed drugs, FSM A funding of Project Cure SMA provides the means for validation and development of new outcome measures that will make future clinical trials more efficient and sensitive to effects of the drugs being studied. Conducting these trials allowed the investigators to simultaneously test repurpose drugs and test new outcome measures for sensitivity and reliability.

**About Project Cure SMA**

Project Cure SMA is a collaborative initiative between Families of SMA and clinical investigators designed to help facilitate the rapid translation of promising new therapies to individuals with SMA. The primary goal of Project Cure SMA is to develop safe and well-tolerated clinical protocols to help identify effective therapies for SMA. Families of SMA has invested $6.5 Million into the Project Cure SMA network.

**Website:**

http://www.fsma.org/Research/Clinical/
National Institute of Child Health and Human Development at the NIH Funds a Pilot Study of Newborn Screening for Spinal Muscular Atrophy

We are pleased to announce that the National Institute of Child Health and Human Development (NICHD) at the National Institutes of Health (NIH) have agreed to fund a pilot study of newborn screening (NBS) for Spinal Muscular Atrophy (SMA). This pilot will take place in two states, Utah and Colorado, and will provide vital information confirming the efficacy and accuracy of the SMA screening technology, as well as the ability to provide crucial follow-up care to identified babies and support for impacted families.

NBS is an issue of critical importance to the SMA community because it holds tremendous promise for assisting with early interventions, and the development of improved and more standardized care protocols for all patients living with SMA. It also may assist in the development of potential drug therapies. Natural history, preclinical, and preliminary clinical data all suggest that potential therapies will demonstrate the greatest effectiveness when delivered presymptomatically. NBS is a key tool in providing early intervention for SMA infants and could facilitate the enrollment of presymptomatic children into clinical trials for therapies that would treat all SMA patients.

The SMA community has been engaged for several years in attempting to add SMA to the NBS panels in the 50 states by seeking approval of the U.S. Health & Human Services Secretary’s Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC). The SMA community submitted a formal nomination form to the ACHDNC in June 2008, making the case for why NBS in SMA is compelling:

(1) The relatively high rate of occurrence of the disease in the general population
(2) The severity of the disease
(3) The existence of a highly sensitive diagnostic test that has proven its utility on a large-scale newborn population basis
(4) The opportunity that newborn screening will provide for early administration of proactive medical treatments that, if provided pre-symptomatically, will increase the odds of improved outcomes
(5) The impact newborn screening will have immediately upon longevity and burden

Identifying SMA-affected individuals at birth also eliminates the pain and cost of unnecessary testing that otherwise would take place in attempting to diagnose the affected patient. This could eliminate the “diagnostic odyssey” many families embark upon and will provide parents with earlier genetic counseling prior to having a second affected child, which frequently occurs when diagnosis is delayed.

The ACHDNC reviewed the SMA community’s nomination form, but formally voted in November 2008 not to proceed with a formal workgroup review. Two reasons were provided by the reviewers: (1) at the present time, no treatment exists for SMA that could significantly improve the lives of those newborns identified to have SMA, and (2) the screening technology and related follow-up care protocols have not been pilot tested in a state newborn screening laboratory.

The recently approved pilot project addresses the latter point and will move the SMA community a step closer to achieving the milestones established by the policymakers at the ACHDNC. Ultimately, this will accelerate development of therapies to treat individuals affected by SMA.
Paper Published with Funding from Families of Spinal Muscular Atrophy on SMN Gene Therapy in Large Species for Ability to Target Spinal Cord, Brain, and Peripheral Tissues

Researchers at Nationwide Children’s Hospital and OSU publish a paper on gene therapy for SMA in the journal Molecular Therapy. The study assesses the ability of systemically injected AAV9 gene therapy vector to target the CNS.

“I would like to thank Families of SMA for the research support to help answer an important question with gene delivery for SMA. We specifically asked if there was a window of opportunity to target motor neurons in a species more closely related to humans in our FSMA funded grant,” said Dr. Brian Kaspar, Professor at Nationwide Children’s Hospital.

Adeno-associated virus type 9 (AAV9) is a powerful tool for delivering genes throughout the central nervous system (CNS) following intravenous injection. Preclinical results in pediatric models of Spinal Muscular Atrophy (SMA) and lysosomal storage disorders provide a compelling case for advancing AAV9 to the clinic. An important translational step is to demonstrate efficient CNS targeting in large animals at various ages.

In the present study, the authors tested systemically injected AAV9 in cynomolgus macaques, administered at birth through 3 years of age for targeting CNS and peripheral tissues. They show that AAV9 was efficient at crossing the blood–brain barrier (BBB) at all time points investigated. Transgene expression was detected primarily in glial cells throughout the brain, dorsal root ganglia neurons and motor neurons within the spinal cord. Systemic injection also efficiently targeted skeletal muscle and peripheral organs.

To specifically target the CNS, the authors explored AAV9 delivery to cerebrospinal fluid (CSF). CSF injection efficiently targeted motor neurons, and restricted gene expression to the CNS, providing an alternate delivery route and potentially lower manufacturing requirements for older, larger patients.

Their findings support moving the use of AAV9 for gene transfer to the CNS for disorders in pediatric populations towards clinical trials. The published preclinical efficacy data in a model of SMA and the effectiveness of motor neuron targeting within large animals in the current report highlight the need for safety studies utilizing SMN as the next step in advancing SMA gene delivery to clinical trial.

According to Dr. Kaspar: “This new work demonstrates that motor neurons can be targeted for a much longer period of time than was predicted in mice, which is extremely promising for treating SMA children where a drug or therapy has to reach motor neurons residing in the spinal cord and brainstem. We have also expanded our work into other routes of delivery including intrathecal gene delivery, which also showed promise for targeting motor neurons.”

Trophans Completes Patient Enrollment in Pivotal Efficacy Study of Olesoxime in Spinal Muscular Atrophy

Trophos SA, a clinical stage pharmaceutical company developing innovative therapeutics from discovery to clinical validation for indications with under-served needs in neurology and cardiology, announced September 8th, 2011 the completion of patient enrollment in the pivotal efficacy study of olesoxime in the rare neurodegenerative condition Spinal Muscular Atrophy (SMA). Over 160 patients have been recruited into the trial since its initiation in October 2010. The study is substantially funded by Trophans’ partnership with the Association Française contre les Myopathies (AFM). The trial protocol has benefited from the EMA protocol advice procedure. Efficacy results are expected in the second half of 2013.

“The completion of recruitment in this pivotal clinical study in only ten months given the rarity of SMA is a great achievement and a major step in the development of olesoxime as a potential treatment for SMA,” said Jean-Louis Abitbol, chief medical officer at Trophans. “SMA is a debilitating and disabling neuromuscular disease and there is a critical need for a treatment that can slow down or prevent the loss of muscle function in SMA patients, for whom no specific treatment exists today. Over 160 patients have been included in the study in only ten months, which reflects both the great commitment of patients and clinicians to find a treatment for SMA and the motivation and hard work of all involved. We anticipate the results of the trial in the second half of 2013 and hope this will be a historic moment for the medical community as well as those affected now and in the future.”
New Insights into a therapeutic approach to treating Spinal Muscular Atrophy

A recent issue of the Journal of Clinical Investigation includes two publications addressing SMN enhancing approaches for Spinal Muscular Atrophy.

Spinal Muscular Atrophy (SMA) is the most frequently inherited cause of infant mortality. Two independent research groups—one led by Alex MacKenzie, at Children's Hospital of Eastern Ontario Research Institute, Ottawa; and one led by Umrao R. Monani, at Columbia University Medical Center, New York, and Cathleen M. Lutz, at The Jackson Laboratory, Bar Harbor—have now generated new data in mouse models of severe SMA that provide hope that a therapeutic providing meaningful benefit to individuals with SMA can be developed.

SMA is caused by mutations in the SMN1 gene that reduce levels of SMN protein, leading to loss of nerve cells in the brain stem and spinal cord that control muscles. This, in turn, leads to skeletal muscle weakness, wasting, and premature death. Increasing levels of SMN protein in individuals with SMA is considered a viable therapeutic option.

In the first study, MacKenzie and colleagues find that prolactin treatment increases SMN levels, improves muscle movement, and enhances survival in a mouse model of severe SMA. As prolactin has been used in the clinic to augment lactation in mothers of preterm infants, this drug may have more immediate therapeutic potential than other drugs without a history of safety in humans. These results also suggest that new drug discovery efforts centered around the STAT5 signaling pathway may be of interest in SMA. Families of SMA provided partial funding for this study.

In the second study, Monani, Lutz, and colleagues find that increasing levels of SMN protein after disease onset in a mouse model of severe SMA has therapeutic benefit. These data raise the possibility that treatments designed to increase SMN levels could be effective, even when initiated at symptomatic stages of the disease.

Kathryn Swoboda, at the University of Utah, Salt Lake City, had an editorial article in the same issue of the journal about these two reports and their therapeutic implications in an accompanying commentary.

Dr. Swoboda concluded her commentary with the following: Predicting value for a given therapy is profoundly more difficult in patients than in animal models and analogous in some respects to peering into a crystal ball. As we look to the future, we should avoid the temptation to squander the opportunity to achieve modest yet meaningful benefit in already symptomatic patients. The substantial progress of the past decade, particularly the dramatic benefits achieved with a variety of therapeutic strategies targeting SMN in animal models, provides us with an increasing likelihood that at least one of the current therapies under development will prove successful in the next few years. The wealth of data continues to support a much greater benefit for mice treated as early as possible in the course of the disease.

Article in Bloomberg Markets Describes Several Spinal Muscular Atrophy Drug Programs

The article highlights the SMA Foundation. It mentions several programs in the SMA drug pipeline, including programs at Novartis, PTC Therapeutics, Isis, and Repligen Corporation.

From the Article:

“Benefactors such as Singh are taking a direct role in early drug research. They want to make it easier for companies to produce a medicine or venture firms to fund it. "We have focused on having lots of shovels ready and having the maps ready and having all the supplies ready, so companies are willing to prospect for SMA drugs," Singh says.

A quirk in the genetics of SMA increased their hope. In many inherited diseases, a crucial gene is missing or defective and the protein it makes is absent or doesn't work. In SMA, the body has a backup gene that produces small amounts of the SMN protein. That's why children with the disease live at all.

The SMA Foundation and Novartis scientists get together every three months to review progress. At a June 1 meeting, researchers from the foundation, Novartis, Columbia and Harvard crowded into a conference room to hear the latest results. "It is a full-court press," Fishman says. "We are pushing as hard as we can." Still, Novartis human trials are two years off at best, Fishman says.

Meantime, Repligen Corp. (RGEN), a Waltham, Massachusetts-based biotechnology company, in July began an initial safety test of its SMA drug on people. It licensed this drug from the charity Families of SMA, based in Elk Grove Village, Illinois.

Singh's foundation is closing in with two more efforts. PTC Therapeutics last year found compounds that boost the life span of mice with the disease. The company could begin human trials in late 2012, Chief Executive Officer Stuart Peltz says. Singh and Eng say they’re particularly excited by Isis Pharmaceuticals' progress, based on work by Krainer at Cold Spring Harbor. Isis published data in March showing that its drug could boost motor neuron levels—and survival—in mice with SMA. The medicine, which is injected into spaces around the spine, corrects the defect that causes the backup gene to produce too little protein.”
Families of SMA Receives Additional Funding from Sweet Baby Zane and the Schmid Family to Purchase Critically Necessary Car Beds for SMA Type I Families

The Families of SMA Equipment Pool received an additional 27 Car Beds to be sent to newly diagnosed SMA Type I families.

Families of SMA is thrilled to have received generous funding from Hillary & Keith Schmid and their group Sweet Baby Zane to purchase much needed car beds for the FSMA Equipment Pool. These 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 car beds enable an infant with special needs to be positioned in the prone or laying down position, safely and comfortably in the car.

Several months ago, Hillary and Keith offered to fund these car beds, in memory of their daughter Zane. Along with her twin sister Avery, Zane was born in January of 2009. Within a month, Hillary and Keith noticed that Zane was not moving her limbs as much as her twin sister. She was diagnosed with SMA Type I in February and on June 18th, 2009, Zane passed away, leaving a mark on everyone forever. She was only 5 months, 16 days, 1 hour, and 3 minutes old.

After Zane passed away, Hillary and Keith were determined to bring awareness to SMA. Along with their supportive community, Hillary and Keith 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 these car beds for the FSMA Equipment Pool. They originally provided enough funding for Families of SMA to purchase 30 Hope Car Beds, which have now been sent to over 50 SMA families.

Families of SMA has now been able to purchase an additional 27 car beds for the FSMA Equipment Pool, bringing the grand total of car beds purchased from Sweet Baby Zane and the Schmid Family to 57!

Sweet Baby Zane and Hillary & Keith Schmid also fund an important Family Support Program that FSMA now offers. They have provided Families of SMA with the funding to offer any international newly diagnosed SMA family a computer disk filled with information on SMA. We have been able to ship over 70 CD’s to SMA families worldwide so far.

Thank you Hillary, Keith, Avery and Zane for allowing Families of SMA to provide support to more SMA families and for making such an impact on the lives of so many!
The Family Education Day for SMA was created by Dr. Mary Schroth, the Chair of the Families of SMA Medical Advisory Council, and was held Saturday August, 13th in Madison, WI. Almost 80 attendees participated in this incredible day, which was the 3rd SMA Day that was hosted at The University of Wisconsin. The presentations cover a variety of topics to help families better understand various aspects of Spinal Muscular Atrophy (SMA). They were presented to an in-person audience and live over the internet, allowing parents to interact with and ask questions of caregivers and medical experts.

The schedule of presentations for The Family Education Day for SMA included:

- Deb Silvis, Outreach Specialist, UW PPC Family Involvement Faculty
- Kate Vogedes, Planning Committee, WI Chapter FSMA Executive Committee
- Katie Poole, Planning Committee
- Mary Schroth, MD, Director, UW PPC Training Grant
- Mary Marcus, MS, RD, Co-Director, UW PPC Training Grant

**National Perspective:**
- Kenneth Hobby, President, FSMA

**Research Update:**
- Christine DiDonato, PhD, Human Molecular Genetics Program, Children's Memorial Research Center, Northwestern University

**Communication Aids:**
- Bobbi Johnson, MS, OTR
- Mary Locast, OTR
- Blair Panhorst, MS, CCC-SLP

**Tips and Tricks for Community Activities and Travel Parent Panel**

**What Happens in the ER?:**
- Dr. Joshua Ross, MD, FAAP, Medical Director, Pediatric Emergency Medicine, UWHC

As the Chair of the FSMA Medical Advisory Council Dr. Mary Schroth has a crucial role in the planning of the Annual SMA Conferences, as well as Patient Care and Family Support Services at Families of SMA.

The Families of Spinal Muscular Atrophy Medical Advisory Council (MAC) is one of the most highly respected bodies of SMA medical and clinical experts in the U.S., setting the agenda for proactive, creative, and collaborative leadership on issues that improve the quality of medical care for those affected by SMA. The Council focuses on educating families, health care providers, and the public about SMA; expanding SMA standards of care; and translating positive research results into clinical practice. Dr. Mary Schroth is the Chair for the MAC. Mary is Associate Professor of Pediatrics and Director of the Pediatric Pulmonary Center Grant at the University of Wisconsin Children's Hospital.
Beautiful Lullaby Blankies were donated to Families of SMA to be put into the Type I Care Packages. Thank you Stephanie Humes for your wonderful contribution! Stephanie of Woodland Hills, California donated these adorable blankets to help raise awareness for SMA. Check out the Lullaby Blankies website to “help fight SMA one blankie at a time”!

www.lullabyblankies.com

Families of SMA,

Dear Staff,

I would like to thank FSMA for the very informative newsletters on SMA. It brings relief to both my husband and I to know that we are not alone in this very difficult journey. I also want to thank FSMA for the wonderful care package sent to our daughter Xamara. All those colorful toys and gifts brought a smile to our whole family. We are grateful to ALL the thoughtful individuals who put the care packages together. We thank you from the bottom of our hearts. May God bless you all.

The Treviño Family of San Antonio, Texas

Hello Everyone at FSMA!

We just wanted to say thank you so much for the information package as well as our daughter’s care package. We feel so blessed to have such an amazing organization on our side that is supporting us and many others. I know we are not alone. We all appreciate you guys tremendously.

Thank you,

The Burgess Family

Sarah and Chris Bonelli of Phoenix, Arizona

Dear Friends of SMA,

Today we received the information packets and care package for Ella! Thank you SO much!

We had NO IDEA that the care package would include everything it did! I can’t even express how grateful we are and how thoughtful it was of you to put it together and send to her! She is so excited!

Even though we just found out the diagnosis last Friday, today we’re feeling a little bit better because we’re realizing how much we’re not alone.

Thank you again, so much!

Sincerely,

Lindsay & Michael Casten of Naperville, Illinois

Hi FSMA,

We arrived home from vacation to find Paul’s care package. He was very excited! Thank you so much for all the information and support we’ve gotten. It’s only been 2 weeks since his diagnosis, but it’s a tremendous help to feel like we are not alone in this so thank you again for everything.

Amy and David Osborn of Cherry Valley, Massachusetts

Dear Staff,

I am sending you this email sent on behalf of myself, my daughter (Sasha) and my grandson (Zy’lek). We would like to say thank you for the care package and the information that you sent to us. It was very much appreciated and needed. It was a blessing to us. Please continue to pray for us as we are trying to understand this disease. The information that you sent to us is very helpful.

Thanks again!

Dayna Dance of Elizabeth City, North Carolina

FSMA,

We received the care package and car seat. Thank you so much for that! The care package was wonderful! One of the quilts has owls all over it and Jack has an owl that he LOVES to stare at so that blanket is perfect for him!

Our family really appreciates all that you have done for us. We are very lucky to have Families of SMA.

Sarah and Chris Bonelli of Phoenix, Arizona
Families of SMA received the second half shipment of the 100 Pathfinder wagons that were so generously donated from Radio Flyer. All of the wagons are a part of the Newly Diagnosed Care Package Program and are sent out to any newly diagnosed family who requests one. We cannot thank Radio Flyer enough for their incredible donation of these wonderful wagons!

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. Many SMA children will nap or watch their favorite television program in their 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. Thank you Radio Flyer Inc. for making such an impact on so many SMA families!

Thank you Marianne Utzat of Easton, Pennsylvania for making these wonderful lightweight rattles and blankets! These special items are put into the Type I Care Packages, in memory of Marianne's granddaughter, Samantha Utzat. They are each beautifully crocheted and we cannot thank Marianne enough for taking the time to make such a special gift for each newly diagnosed family.
An enormous thank you goes out to Allison and Bill Wehrkamp for donating these adorable beanie animals in memory of their daughter, Violet Madison Wehrkamp, for the Type I Care Packages. This beautiful message is on the back of each animal’s card: “We never know what we can bear until we face the deed; It’s then our inner strength prevails, with power to succeed.” The Wehrkamps have also donated sweet little security blankets through Allison’s on-line Etsy shop called Little Flower Violet. Thank you for your continued support, Allison and Bill!

The Families of SMA National Office had two wonderful visitors stop by in August! Donna Oker and Brittany Street of Elk Grove Village, Illinois, delivered a Type I Care Package for Families of SMA to send to a newly diagnosed type I family. They came up with all the ideas and items all on their own! We are so grateful for this incredibly generous donation, which was made in memory of their granddaughter and sister, Megan Lynn Street. Thank you Donna and Brittany for making such an impact on the life of another SMA family!

Hi FSMAs,
We had a blast at Camp Harkness in Connecticut! Some of us were there for 10 days and some came for a shorter period of time. Weather was great, so all-in-all a super time! I’ve just finished making reservations for three cabins for Columbus Day Weekend so we can do it all over again!
Take care,
Mary Ellen Barrelle of Norwalk, Connecticut

Families of SMA is so grateful for these extraordinary bracelets that Michelle Meredith of Pitcairn, Pennsylvania hand makes! These bracelets are put into each Type I Care Package, in memory of her granddaughter, Tesa Marie Driscoll. Thank you Meredith for these wonderful additions and for your support!
Families of SMA,
I wanted to thank you for the wonderful care package. Everything is wonderful. When I went through the box I could feel the love that went into it. My daughter Emily is currently in the PICU with pneumonia. I brought her some items from the box - she LOVES the portable projector and sound machine, as well as the cat toys, finger puppets and butterfly. I also brought one of the beautiful blankets and the book for her to enjoy also. Again thank you so much for your generous gift, it put a smile on my face during this difficult nightmare.

Thanks again,

**Jenn Lozina** of Cos Cob, Connecticut

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FSMA,
Thank You! After a long day of doctors, it was so wonderful to come home to our “box of love.” Our hearts are overflowing with gratitude! We are very grateful for all the care that you and the FSMA staff have put into this box. I thoroughly enjoy the information, the toys and all the goodies. Also thank you for the wagon, this will make traveling a world easier.

Thanks again!

**Danielle and Shawn Brown** of Athens, Georgia

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Dear FSMA,
Words cannot express how thankful we are for receiving the wonderful care package. Just shortly after Trevor’s diagnosis you were right there to fill us in on as much information as needed and also find a way to lighten our hearts in such a dark time. Trevor loves everything you have sent. Thank you for all of your support and kind actions.

Love the Rickerson’s,

**Trey, Brittany, and Trevor** of Hammond, Louisiana

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Families of SMA,
Thank you so much for the wonderful care package. What a wonderful box of helpful items. Sage loves all of it; her big brother, Dane, sure thought it was cool, too. The wagon will make it so much easier when going to the doctors’ offices, especially when she gets a little bigger.

FSMA has been such a God send to us. We feel like we are no longer in the eye of the tornado.

THANK YOU!
Sincerely,

**Jane and Dermot Vardiman**

(Sage and Dane, too) of Euless, Texas

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FSMA,
My Name is Steven Carlino, my wife, Cathy and I have a daughter, Brianna with SMA Type II. She is twelve years old and she underwent spinal fusion in July of 2010 and is doing well. We hope everyday for some kind of treatment or cure.

Thank you for all you do,

**Steven and Cathy Carlino** of Malverne, New York

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Thank you so very much for the package. Karoline has so much fun watching the movie; she loves to sing. She likes all of the toys and I am very thankful for all of the information and very useful binder.

Thank you for all of the gifts and support in this hard time.

**Małgorzat and Andrzej Chowaniec** of River Grove, Illinois
CONGRATULATIONS

- Congratulations to John and Jamie Shish on the birth of their new baby girl, Lanie!
- Congratulations to Jessica and Joseph Stoop and big brother and sister, Josef and Alicia, on the birth of their new addition Dominik!
- Congratulations to Cricket, Trent and Reagan Goforth on the birth of their new boy, Trent Jr.!
- Congratulations to Gary, Paula, Jake and Rachel Saxton on the birth of their new baby girl, Katherine Grace, born on September 28th!
- Congratulations to Dr. Dmitriy Vaysman on being awarded Doctor of the Quarter at Good Samaritan Medical. He was nominated by the Erwin Family of Massapequa, NY for his excellence of service in the battle against SMA.
  
  To download a copy of this nomination letter, visit http://www.fsma.org/FSMACommunity/DailyLife/parenting/ParentingArticlesAndLinks/index.cfm?ID=6518&TYPE=1489
- Congratulations to Tanya Bowser and Tom Krajewski on their marriage in October of 2011!

CONGRATULATIONS to Cory Saldana and Ian Jacobson on their marriage

This joyous occasion happened on April 16th, 2011 in a private ceremony in Colorado Springs. Ian is serving in the army and stationed at Fort Carson. They were joined by parents, Scott and Virginia Jacobson from Tuscon, AZ as well as Cheryl and Dan Saldana, sister Katie, and Grandmother Eileen Saldana from Coon Rapids, MN. Cory, age 21, has had many accomplishments and has been excelling in school through her college years.

Congratulations to Debbie Cuevas for being named 2011 Woman of Distinction

Debbie Cuevas’ name is synonymous with compassion and perseverance. After her son Dylan was diagnosed with Spinal Muscular Atrophy (SMA), Ms. Cuevas was determined to overcome every obstacle and devote her time and energy to her son and the community at large.

Ms. Cuevas is a mother, friend, nurse, caregiver, physical therapist, SMA activist, and much more rolled into one. A true pioneer, Ms. Cuevas founded the Greater New York Chapter of Families of SMA in 2006, as a way to give back to her community. As President of The Greater New York Chapter of Families of SMA, Ms. Cuevas is instrumental in serving over 500 families who have been affected by Spinal Muscular Atrophy.

For the full article please visit the following web address: http://www.nysenate.gov/story/senator-skelos-honors-debbie-cuevas-2011-woman-distinction

Aurora Knowles (Type II) from Orlando, FL, was featured in a public service announcement for “Help Kids in Your Community,” which aired during “Extreme Home Makeover” to a national television audience! Allison Kerns (Type II) was in a commercial for St. Petersburg’s All Children’s Hospital/Johns Hopkins Medical! We’re proud of these amazing girls and the achievements of all of our SMA warriors.
A Dozen Abstracts Presented on Spinal Muscular Atrophy at the 2011 American Academy of Neurology (AAN) Meeting

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

-Utilizing a National Continuing Medical Education (CME) Series to Highlight Emerging Therapeutic Opportunities in Spinal Muscular Atrophy (SMA); Megan Montgomery, Cynthia Joyce, Susan Iannaccone, Nancy Kunzt, Robert Leshner, Julie Parsons, Darryl De Vivo.

-SMA CARNI-VAL Trial Part II: A Prospective, Single-Armed Trial of L-Carnitine and Valproic Acid in Ambulatory Children with Spinal Muscular Atrophy; John Kissel, Charles Scott, Thomas Crawford, Louise Simard, Kristin Krosschell, Gyula Acscadi, Bakri Elsheikh, Mary Schrot, Guy D’Anjou, Mark Bromberg, Bernard LaSalle, Kathryn Swoboda, Susan Sorenson, Gary Chan, Jo Anne Maczulski, Project Cure SMA Investigators Network.

-Nutritional Analysis in Infants and Children with Spinal Muscular Atrophy (SMA) Type II; Katherine Poruk, Abby Smart, Sandra Reyna, Kathryn Swoboda.

-DEXA Analysis of Fat Mass and Fat Free Mass in Infants and Children with Spinal Muscular Atrophy (SMA) Type I; Katherine Poruk, Gary Chan, Abby Smart, Rebecca Hurst, Sandra Reyna-Merida, Kathryn Swoboda.

-Alpha-Synuclein Expression Is Significantly Low in Spinal Muscular Atrophy; Gyula Acscadi, Kathryn Swoboda, Xingli Li, Kelley Murphy, Graham Parker.

-Safety Assessment of Intravascular Administration of scaAV9-SMN in Mouse and Non-Human Primate; Emily Nurre, Kevin Foust, Adam Bevan, Jerry Mendell, Arthur Burghes, Brian Kaspar.

-Design of a Phase II Clinical Trial To Demonstrate Efficacy, Safety and Evaluate Biomarkers in Patients with Spinal Muscular Atrophy; Jean-Louis Abitbol, Enrico Bertini, Carole Andre, Patrick Berna, Isabel Clemancon de Bellefois, Valerie Cuvier, Rebecca Pruss.

-Electrical Impedance Myography as a Biomarker in Spinal Muscular Atrophy; Seward Rutkove, Matthew Gregas, Philip Mongiovi, Basil Darras.

-Generation of Motoneurons from Spinal Muscular Atrophy-Induced Pluripotent Stem Cells Free of Vector and Transgenic Sequences: In Vitro and In-Vivo Analysis; Stefania Corti, Monica Nizzardo, Martina Nardini, Chiara Simone, Marianna Falcone, Giulietta Riboldi, Chiara Donadoni, Sabrina Salani, Giorgia Menozzi, Clara Bonaglia, Nereo Bresolin, Giacomo Comi.

-Synaptic Defects in the Peripheral and Central Nervous Systems in a Mouse Model of Spinal Muscular Atrophy; Karen Ling, Mingyi Lin, Zhihua Feng, Rebecca Gibbs, Chien-Ping Ko.

-Pre-Diagnostic Parent Experiences in Duchenne and Becker Muscular Dystrophies, Congenital Muscular Dystrophies, and Spinal Muscular Atrophies: A Survey of the National Task Force for the Early Identification of Childhood Neuromuscular Disorders; Katherine Mathews, Holly Peay.

So now that we’re well on our way to September and far past Lucy’s milestone second birthday, I feel I can blog about it. Too much pressure and expectation near the actual day. I do my best writing randomly.

As I’m sure you all know, ninety percent of children diagnosed with SMA don’t make it to their second birthday. Lucy has. Lucy has made it there, past there, and will continue to make it.

Negativity is not something that will help Lucy, or help us as her parents and caregivers. Some people choose to call their negativity “realism”. That being said, this disease is different for everyone. What is honest-to-goodness realism in regards to one child may be viewed as unnecessary negativity when applied to Lucy’s situation. And sometimes people are just negative - for what purpose, I don’t know. Pity? Attention? Depression? Any way you slice it, negativity is not going to help you or your child thrive.

Raising SMA awareness tends to be a double-edged sword. I want people to understand the severity of the disease, but I don’t want it limiting their thinking of Lucy. Lucy just isn’t a child we make fit into this world; we make her world fit her. She’s a toddler, and she likes toddler things - mainly copping an attitude when things aren’t going her way. And we do those toddler things with her: coloring, reading stories, watching Sesame Street, playing on the iPad. And there are some things that we do for her that you don’t do for your kids; keeping her healthy takes a little more than a Flintstone chewable and making sure she finishes her vegetables. But it’s our life. We are happy. We don’t get vacation. We don’t get date nights. Ever. We work around the clock. And yet we are happy. Why? Well, one very big reason is because Lucy is doing so well. But it’s also because we CHOOSE to be. Whining about what should be or could be or won’t be isn’t going to change what is.

Now, that’s not to undermine the pain of SMA. It hurts. It’s hard. But if you let those thoughts constantly overtake your conscious, you are doing yourself and your child a great disservice. I wouldn’t even say I have bad days; I have bad moments. Because if you let it go any longer than that, you’re just poisoning time that you could spend being happy. And there’s no one to blame for that but yourself.

I’ve addressed this because when you hear the above statistic, it may lead you to think that since Lucy is two, she is living on borrowed time. To me, that’s negative. Lucy is penning her own story, with God as her ghostwriter. Because the phrase “statistics say” doesn’t mean jack around here.

Lucy had a great second birthday. It was with family and friends (including the lovely Brianna McDanel!), cake and ice cream, and presents. Last year, after her first birthday (a huge bash in a ballroom), I thought we would be doing something even bigger for her second, since in the SMA world it’s a big deal. But when it got closer, I didn’t feel it was the way we wanted to celebrate this year. Yes, I’m thrilled that she’s beating the odds, and we celebrate every milestone - but to me, she’s a regular kid. Maybe some years we’ll have big blowouts, but I’d be lying if I said I wasn’t expecting to celebrate more birthdays with her. You can call that thinking whatever you want - for us, it’s our reality.
Families of SMA invites you to join SMA Community Connections

Families of SMA is excited to now have over 50 groups on the SMA Community! These groups have been started by members who are looking to connect with others who share something in common or who have specific interests they would like to discuss. Some of these groups include an SMA Teen group and an SMA Adult group where people with SMA can come and chat with each other. There are also groups for every FSMA Chapter allowing chapter members to easily connect with local families. There is also a group for Grandparents who are interested in connecting with other grandparents. If you are interested in connecting with others, please log-on and check out all of the groups or feel free to start your own!

This community site allows families from all over the world to connect with each other to chat, organize, and share stories, photos, news and information. Just log in and start sharing. Knowing there are others out there fighting alongside you is incredibly empowering, and the advice and personal anecdotes can make a real difference in the lives of you and your family. Since launching this website in June of 2010 we have over 2,200 members including SMA families, doctors and researchers.

If you have not already joined SMA Community Connections, sign up today at www.smacommunity.org to check out our newest features!

Some of the new features include:

• Over 50 Presentations which were given at previous conferences are now available for download and viewing.

• New Recorded Care Workshops from the 2011 Annual SMA Conference can be viewed online at SMA Community. These workshops include Hands on Physical Therapy, Breathing Basics and Care Options for SMA Type I, Good Nutrition for Children Fed by G-Tube, as well as the Q & A Session with Researchers. Also available online is the General Session Opening Remarks which includes presentations by Paula Barrett, Kenneth Hobby, Kathy Bishop, Katherine Klinger, Spencer Perlman & Mary Schroth.

• 2012 Conference Registration and Information.

There is also a Comprehensive SMA Resources Section with information, links & articles on the following important topics: genetics, respiratory care, nutrition, physical and occupational therapy, orthopedics, palliative care, education for SMA affected individuals, parenting issues, adapting toys for SMA children, Standard of Care documents, equipment, home renovations & accessible designs, insurance, travel and books on medical information and life with SMA. The community also has Commonly Asked SMA Questions, with searchable answers from our Medical Advisory Council.
Loving Memories

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

Calling All Angels

The heaven appear so much brighter tonight
As another sweet angel has just taken flight.

Leaving behind many hearts that won’t heal
And a mother who’s pain and sorrow we feel.

Calling all angels, come hear this plea
Please guide this child into eternity.

When a hero falls, it affects us all
In ways we can’t explain.
The hopes, the dreams, what the future brings
Is challenged once again.

Calling all angels, please calm my fear
Help me understand why he’s no longer here.

To live this life is no easy task
Our beliefs are queried, why we ask,
Does God need so many angels to be at His side?
Leaving family and friends on an emotional ride.

Calling all angels, please help to heal
A heart that is broken, a faith I can’t feel.

How do I tell the child I love
That another friend is in heaven above?
I choke down the sobs, my eyes fill with tears
As I do my best to abolish his fears.

Calling all angels, please help him to see
That children are dancing and running free.

We get through the day the best that we can.
Knowing that this will happen again.
We hope, we pray, we just wait to learn
A cure has been found, for which we all yearn.

Calling all angels, allow us to dream
Of ending the pain this disease can redeem.

By Debby St. Onge
IN MEMORIAM
Sydney Grace Potjer
October 18, 2004 – May 8, 2011

Sydney came into this world as a precious gift. A gift of love to her parents, a gift of friendship to all those lucky enough to have known her, a gift of courage and hope to all those that will continue to be moved by her memory. When we think of Sydney, we remember her infectious smile, her bubbly personality, her charm and wit, and most of all, her determination and will to live every day to the fullest. She inspires us to live fearlessly, to embrace every wonderful moment and really experience it. She inspires us to love each other despite our limitations and to help one another. She was always defined not by her disability but by all that she had to offer the world – she always wanted to know “how can I help?” She had a wonderful capacity for giving. If only we could all try and see ourselves and others the way Sydney did. She will continue to inspire us to live and love and to never give up. Sydney came into our lives bright and brilliant – forever changing us all….We love you and miss you, Sydney, with all of our hearts. Thank you for the gift of your presence in our lives….it was truly a privilege to know you.

Kathleen Salus of McHenry, Illinois

IN MEMORIAM
Tesa Driscoll
August 8, 2010 – June 9, 2011

Although Tesa’s sweet life was short, it was not without purpose. This poem by Helen Steiner Rice reminds me of what Tesa meant to her loved ones:

“A Wee Bit of Heaven”
A wee bit of heaven drifted down from above -
A handful of happiness, a heart full of love.

The mystery of life, so sacred and sweet -
The give of joy so deep and complete.

Precious and priceless, so lovable too -
The world’s sweetest miracle, baby, is you.

Author: Helen Steiner Rice

Tesa taught some precious lessons to her family and loved ones just by being who she was. Although with us for a brief time, Tesa allowed us to experience joy and pleasure through the simple blessings her life brought. We loved her wide, curious and bright eyes that watched us when we arrived, as well as the precious smiles, adorable expressions and tiny sweet voice that mimicked us and grabbed our hearts from the moment we met her.

We must try to enjoy what is good and true in life. Through Tesa, we have learned about innocence, and how pure and joyous life can be. Tesa was a remarkable little lady who had an adorable sense of humor from such a young age. She was able to make a connection and communicate with everyone she met. We will miss you dearly, baby Tesa, but your spirit will live on in each of us.

IN MEMORIAM
Katherine Swetnam
November 9, 1997 – January 24, 2011

Families of SMA,

Thank you for the angel you sent to us as a memorial for our daughter and sister Katie. Your support during this difficult time is most appreciated.

Doug, Annette and Betsy Swetnam of Fishers, Indiana
IN MEMORIAM
ADRIANNA ROBINSON
FEBRUARY 7, 2010 - JUNE 16, 2011

IN MEMORIAM
ANNIE GRIMES
OCTOBER 13, 2009 - AUGUST 6, 2010

IN MEMORIAM
ETHAN CARTER
MARCH 29, 2009 - OCTOBER 14, 2009

IN MEMORIAM
BLAKE E. HILL
MAY 31, 2002 – AUGUST 2, 2009

IN MEMORIAM
RON DUANE WILLIAMS JR.
FEBRUARY 17, 2003 - JUNE 9, 2003
Benjamin Michael Newfield was born on September 30, 2010. We were so full of joy to welcome another precious baby into our family. What a surprise to have a baby boy to join big sisters Grace, Elesia, and Brynna. A few days after Ben came home from the hospital, nursing became extremely painful and despite techniques from a lactation consultant Ben was not able to open his mouth wide enough and I developed an infection. We switched to bottle feeding which was a surprise to me after having had 3 nursing babies. Ben had busy days as a little brother. He joined along in rides to school, was an audience member at dance and school programs, and watched all the antics of his sisters. We all doted on him and loved his red hair!

A little before 2 months as it seems so many of these stories done better with this in the first few weeks of life). We lifting his head when lying on his tummy (in fact he had consulted my friend who is a physical therapist for ideas and really “therapized” him throughout the day. I am an occupational therapist in early childhood special education, birth-3, doing home visits with children with special needs and their families. At 3 months, I realized that Ben’s tone issues were not improving and seemed more concerning. We made a neurology appointment to rule out any “scary stuff”. We wondered if he might have extremely low tone (hypotonia) or cerebral palsy. From 2 1/2 months Ben had noisy breathing. He had developed a cold which didn’t seem to completely go away. He increasingly leaked from the sides of his mouth when drinking his bottle. He also seemed to breathe a lot with his tummy, which we attributed to the low tone. Our worry built as he approached 4 months.

A few days after he turned four months old, on February 2nd, Ben all of a sudden refused to take his bottle and was breathing with more effort than usual. Within a few hours, he developed a fever of 102 and Mike took him to the emergency room at Children’s St. Paul while I stayed home with the girls. His breathing got more out of control and they had to put him on a ventilator. I joined Mike at the hospital soon after and the intensivist told us she worried about SMA. The neurologist confirmed this belief the next morning. The blood test came back the next week but we pretty much already knew by then as he had all the signs. Ben was fighting pneumonia brought on by the parainfluenza virus (croup virus).

Despite our grief, we grasped onto the gift we hoped we were about to be given, more good time with our Baby Ben. Ben came off the ventilator a week later. After several more weeks of getting better from the pneumonia and having his G-tube/Nissen procedure, Ben was HOME. We treasured the time that followed so much and made many memories. Ben smiled all the time and would do a silent laugh/chuckle when tickled, during Peekaboo, or when his sister Ellie threw his balloons up in the air and caught them. He only cried when he was tired. He loved music therapy, especially the guitar. He loved to mouth light toys we placed in his hands (we propped his elbow up closer to his mouth with a stuffed animal so he could keep the toy in his mouth longer). He loved to knock down block towers and bat at Weebles toys. He also loved to bat at balloons or fiddle with the ribbons at the base on the balloon weight. Ben also loved books and would pay attention to them for so long. He would bat at the pages and could sometimes turn them with his little hand. He loved to play with my hair. He loved to watch the trees blow outside the living room window as he lay on the versa pillow or wedge playing. He also loved when we sang songs and moved his legs and arms. Ben had soft, gentle coos. We loved his “talking”.

Soon after Ben came home from the hospital, he would turn blue when upright in the infant swing or Tumbleforms chair so then he remained in side lying mostly or on his back with his head turned to the side. He could lie on a Tumbleforms wedge pillow (we just had to put something under his legs eventually as he began to have swelling). It also became difficult for him when we held him due to the gagging on secretions despite suctioning so we pretty much had to stop that but we gave him tons of snuggles lying next to him and also got a comfy versa pillow for him to lie on which we could put on our lap with him on top of it. We carried Ben around the house and up and down the stairs in a Moses basket which was the idea of one of our wonderful home nurses, and then when he hit the 15 pound weight limit we put the basket in a large duffel bag to reinforce it. When he outgrew our infant tub that we used on the bathroom counter, our palliative care nurse thought of an under the bed storage box to set on the counter and put him in. We used the soft head/shoulder insert sent in the FSMA care package to cushion his upper body. We could dump the soapy water over him. Ben slept through the night most of
the time right next to our bed with the bipap he used at night. He was on a continuous feeding of Elecare, with two to three hour breaks during the day.

Ben had two illnesses that he made it through with just increasing his respiratory treatments (nebs, vibration, cough assist) to three times per day instead of two, and also an antibiotic. On June 6th his heart rate increased to 200 for four hours and he was working so hard to breathe and we thought it might be his time. We had to start giving morphine and lorazepam. He did fight that illness and had another week and a half of time during which every few days his heart rate/work of breathing would increase again and he would need a dose of the meds. On June 22nd his heart rate became very high again and his work of breathing was so much and from that day on he needed constant medication and increases in the doses. That night he was struggling and needed oxygen (which he previously only had on hand just in case and had needed it briefly if he had a blue spell). He slept a lot and began to look upward, above our faces a lot. We feel he was seeing angels and beginning to cross over to the next world. On June 28th at 8:45 pm with the sunset, our beautiful Ben went to heaven. I was once again able to hold him in my arms as we told him we loved him and it was okay and to go to heaven. We are so happy for him not to have to work so hard to breathe and to now be able to move freely, though we miss him so very much. He is with us as we continue our journeys here. He taught us so much. We now look forward to heaven someday, which seems so much more real to us. The book “Heaven Is For Real” has really helped us. He taught us to cherish each moment in that moment, that quality not quantity is what matters. We also are forever changed by all the people who have reached out to us in so many ways, with prayers, cards, food, gestures to brighten our day. We now can be that help for others.

We know our Angel Ben is now playing with all the other precious kiddos who have gone before him. They will all watch over us and give us strength.

Nicole, Mike, Grace, Elesia, and Brynna Newfield, Baby Ben in Heaven of Mahtomedi, Minnesota
A friend of ours said she heard this song on the radio the other day and thought of us. I have heard this song a million times, but never really listened to the words. It is definitely appropriate for our lives right now. On March 28, 2011 I gave birth to our second son, Miller McNeil Woodruff. He was an 8 pound 12 ounce bundle of joy and a picture of perfection. In the 87 days that followed, this little boy would change our lives forever and impact the lives of everyone that came in contact with him.

After having Miller at home with us for about two weeks, my mom began to make comments about how he did not seem to move as much as most newborn babies do. My husband, Patrick, and I discounted her concern and continued to be thankful for our “laid back” baby. Nonetheless, we scheduled an appointment for Miller to see our pediatrician – it was just for our own peace of mind and we really felt kind of silly taking him in. We assumed the doctor would just laugh at our ‘parent paranoia’ and send us on our way; but that was not the case. As soon as Dr. Schaefer walked in the room, I knew something was wrong. He didn’t say much at all, which is abnormal for him. He usually comes bouncing in the room with a big smile on his face; however, today, that smile was replaced with a look of concern. It was on this day, April 20, 2011 that our world was turned upside down and we began our life with SMA (Spinal Muscular Atrophy).

We were sent straight to the hospital where testing began immediately and lots of blood was drawn, and the next day we went with Miller to Arkansas Children’s Hospital in Little Rock. As soon as we arrived at ACH, a team of doctors, nurses, residents, interns, social workers, and chaplains quickly greeted us. As the doctors began examining him, it looked as though they were having class and the subject was our baby. Once I calmed down and we got moved to a private room, my nerves were much better! Miller’s MRI, EKG, and chromosome testing came back normal. But, his EMG which measures the electrical activity of muscle was another story. Patrick and I watched as they gently inserted needles into Miller’s muscles while observing and recording an electrical pattern.

Much to our surprise, we didn’t have to wait days for the results. The doctor immediately wheeled her chair over after completing the test and informed us that our tiny baby had a severe case of SMA Type 1. Questions began to run through our minds. You are giving us a diagnosis? What about consulting the rest of the team? Surely this simple little test can’t give us a definite diagnosis! That is when Patrick asked the dreaded question; “So what is his prognosis?” We had already accepted the fact that we would be raising a disabled child who would probably never walk, but we were not prepared for what the doctor told us next. “I am so sorry to tell you, but Miller will most likely not live to see his 1st Birthday.” Our baby was dying, and there was nothing in the world we could do to save him! Although our hearts were breaking, we remained faithful as we knew we had to stay strong for Miller, and for his 3-year-old brother, Cole. We knew that Miller had a purpose and we were determined to give him the best opportunity to fulfill that purpose! I couldn’t wait to get our baby boy home and have our family of four all under one roof again! What I wasn’t prepared for was all of the equipment that had to come with us. I was picturing us coming home to our normal life, but what I quickly learned is that our “normal” wasn’t quite so “normal” anymore. Miller was still on oxygen, so our house was full of compressors, oxygen tanks, a pulse ox machine, as well as a suction machine. I was claustrophobic in our own house and felt like we were back in a hospital room.

Even though Home Health was coming to visit Miller at home, we still went to see Dr. Schaefer for an office visit. During our visit, he gave us great news! He thought Miller could tolerate being off of the oxygen during the day, as long as we hooked him up to his pulse ox machine periodically to check his saturations! This was the relief I needed! I needed some time to breathe. We were “wireless” for about 5 days; however, it was decided that Miller needed to be put back on oxygen. He was just having to work too hard to breathe without it. He was breathing between 60-90 times per minute, and normal is 30-60. This was hard to watch. I hated seeing him work so hard just to breathe. The only thing that gave me any peace, was reminding myself that he didn’t know any different.
Despite being in distress, his little face always looked as content as could be. I am so thankful that Patrick and I have been like-minded on all of our decisions throughout this whole process. We both agreed that in Miller's situation, palliative care was our best option. The word “palliative” means soothing, calming, and pain relieving. What this meant to Patrick and me, was that we were choosing “quality” of life over “quantity” of life. He would use oxygen, we would suction his secretions as needed, and we would insert a feeding tube when he lost the ability to swallow, but we would not trach him. For us as his parents, we felt that would have forced Miller to hang on to life that simply wasn’t there. If there was hope for a cure within Miller’s lifetime, we would have done anything and everything we could have to keep him alive. Unfortunately, that wasn’t the case.

At the beginning of June, we decided that Miller was ready for a feeding tube; therefore, we made the tough decision to switch from Home Health to The Circle of Life Hospice. This was a hard reality to face. Our baby was on hospice! That just didn’t sound right! Babies aren’t supposed to die! Even though it was difficult to swallow, it was such a blessing. We knew that if we got the opportunity to “know” when Miller’s final days would be, we wanted to spend them at The Circle of Life. I didn’t think I could handle having those memories at home. The thought of someone from a funeral home coming to our house to pick up our baby was just unbearable.

I had so much anxiety every day. I worried about how/when it was going to happen. Was he going to suffer? Would Patrick be there? I worried that if Patrick was at work when it happened, he might blame himself, thinking he could have saved him, or blame me, thinking I could have done something more. Knowing your baby is going to die is the weirdest feeling in the world. I never dreamed I would have to plan my own baby’s funeral.

From the time we switched to hospice, Miller had lots of ups and downs. When he was good, he was really good, but when he would get bad, he would get really bad, really fast! When he would get fussy, his oxygen saturation would immediately drop, his heart rate would sky rocket, and he would sweat like crazy! When this would happen, he would panic, which would make him even fussier. It was so scary to watch! He would struggle to breathe when this happened and stare at me with panic in his eyes! It broke my heart! The only way to calm him down when this was happening was to put him up on your shoulder. Nobody knew how to do this better than his daddy! That is one thing he made sure we knew. He was a DADDY’S BOY!!!

Wednesday, June 22, Miller had taken a turn for the worse and we decided it was time to go to the hospice house. Even though we had said from the beginning that we wanted to be at the hospice house when Miller passed, we hesitated when the time actually came. Part of us thought maybe it would be best to just stay at home, but the more we thought about it, we made the decision to go. Before leaving our house, I told Patrick that I wanted to sit in his nursery with him and rock just one last time.

Once we got to Circle of Life and settled in our room, we spent the rest of the day holding Miller and visiting with friends and family that stopped by to see us. Monsignor LeSieur came and gave him Sacrament of Confirmation and Sacrament of the Sick which was a special time and memory for us.

Everyone left the Hospice House at around 10pm and it was just the three of us. Patrick and I decided we would take turns holding him throughout the night. During my 1am-3am shift, I gave Miller permission to go ahead and go to Heaven. I assured him that we would be okay and that he would feel so much better if he would just LET GO! He was working so hard and I knew he was fighting for us! Patrick took his shift from 3am-5am while I slept. At 5:30 in the morning, I rolled over and found them both sleeping. I was so tired and wanted to just roll back over and go to sleep because I knew the day would be full of visitors. I resolved that I would sleep now and just hold him all day the next day. However, something urged me to go ahead and wake Patrick up to switch off so that he could lie down for a little while. Before lying down, he told me he loved me and then whispered to our son, “I love you, Miller.” Those were the last words little Miller heard his daddy say.

With Miller asleep on my chest, the nurse came in to give him some medicine and I expressed concern about how shallow he was breathing. The nurse said that it was because he was so relaxed and told me that he didn’t feel that Miller would pass that night, so I calmed down and went back to sleep. Looking back, I am so thankful that the nurse told us that, because if he had told us he thought it would be soon, we would have been panicked all night. When I awoke at 7:36am, I had my hand on his back and I could tell that he wasn’t breathing. I yelled at Patrick to wake up and told him to come over to us because I didn’t think Miller was breathing. He came over and put his hand on Miller’s back and we both sat there in silence. We had been warned that he may have episodes of not breathing and then start breathing again, so we didn’t want to rush out and get the nurse. After we knew he was not going to start
breathing again, we called the nurses in. One of the nurses turned to us with tears in her eyes and simply shook their head. I will never forget that moment.

We were given as much time as we wanted with Miller before the funeral home was notified. We held our baby boy and sobbed for about an hour before we told the staff we were ready. When the funeral home came to pick him up, we were told that we could choose to take him to the funeral home ourselves. As strange as it might sound, that was what we wanted to do. This way, he didn’t get taken from us, we got to drop him off. For whatever reason, that felt better to us. We got to the car and Patrick offered to drive and let me hold him in the back, but I wanted to drive. This had been our routine over the past month when we would travel with Miller because he could not tolerate his car bed anymore. This way, it seemed somewhat normal, just like we were going to the doctor or something. After taking a very slow ride to the funeral home, we carried Miller in, signed some papers, and handed him over. That was the hardest thing I have ever had to do. My only peace came in knowing that he was not there; but in Heaven looking down on us.

On our way home from the funeral home, we went to pick up Miller’s big brother. I went over and picked Cole up and asked him if he knew where Miller was. He said “in the car?” I said “no.” He said “at home!” I again said “no”. He said “Minner MACNeil in Heabin?” I said, “Yes, he’s in Heaven” (he had been telling us for a few weeks that Miller was going to Heaven). He hasn’t asked any more questions. When he asks, we will answer honestly, but in the meantime, we don’t see any reason to overwhelm him with info he isn’t ready for.

Walking into the house was not quite as hard as I imagined. I know having Cole with us helped! However, as I started unpacking Miller’s bag and putting up diapers and hanging up clothes, the reality of the situation hit me. I broke down crying. My baby boy would never be coming home again. I would never put another diaper on him or dress him in his clothes. The thought was too much to bear! We went back to the funeral home to make arrangements and take him a change of clothes, his favorite blankets, his blue dog, and the glow worm that his friend, Hudson, gave to him before he first left Mercy to head to ACH. We were allowed to see him again and he looked so peaceful. They wrapped him in his blanket and let us hold him one last time. Our precious baby boy was free of any cords, wires, or tubes. We “shhh’d” him and patted his bottom just like he liked for us to do before handing him back over. Handing him over yet again was hard; but, that was the closure we needed.

We had his memorial service on what would have been his 3 month birthday, June 28, 2011. It was absolutely beautiful! Everything about it was as picture perfect as he was! If Miller knew one thing; he knew he was loved. God blessed us with a precious child and allowed us the honor, privilege, and joy of having him in our presence for 87 days. In that short time through the sparkle of those big blue eyes and shy little smile, he lifted the spirits of many and changed the lives of others forever. He made us understand the true meaning of quality over quantity and gave us a new appreciation for every sunrise we see. Like the song says, Patrick and I will stand strong together and fulfill our promise to each other and to Miller that we will make the best of this unfortunate situation and NEVER let Miller be forgotten. As part of our commitment, we have started The Miller McNeil Woodruff Foundation to raise awareness, fund research and offer support to other families who are faced with this challenge. We will do this by donating the money we raise to Arkansas Children’s Hospital, Circle of Life Hospice, and Families of SMA.

Meredith and Patrick Woodruff of Bentonville, Arkansas
IN MEMORIAM

Tommy Pence
March 23, 2009 – February 9, 2011

Tommy was our first child and we were the happiest people in the world when he was born, healthy and beautiful, on March 23, 2009. Like all new parents, we were excited to watch him grow up and we dreamed of all the fun things we would do with our little boy. When he was diagnosed with Spinal Muscular Atrophy Type I at eight months of age, our perfect world came crashing down around us. We were told he would never walk, crawl or even sit up. Worst of all, the doctor told us the disease was degenerative, and he would eventually lose the ability to swallow and even breathe. His life expectancy was one to two years. We were devastated. The days and weeks that followed Tommy’s diagnosis were absolutely terrible. We now knew that all of our hopes and dreams for Tommy were impossible. It took months to accept Tommy’s fate, but eventually we were able to cope. We tried not to think of our future and just made sure that Tommy was happy. We took him out with us and treated him like a normal kid. He went to family barbecues and ate at restaurants with us. We took him to the beach and the zoo. He loved bubbles and his pets. He would feed his dogs goldfish over the side of his chair and laugh when we told him not to. He loved to watch Curious George and Sid the Science Kid; he would smile and do his own little “dance” by waving his arms around when the theme song came on. He loves to play with balls and would roll his ball on his wheelchair tray towards us so we could roll it back over and over again.

Most kids with severe SMA are unable to talk, but Tommy was fortunate enough to have the gift of language. His first word was “cat”. “Mama”, “Dada” and “ball” quickly followed. We read to him every night before bed. He loved Dr. Seuss books, and his favorite was the ABC book. He liked to turn the pages in his books by himself. He amazed us with his intelligence, and was proud to share his knowledge with everyone. When he was only 18 months, he started pointing out all the letters in his ABC book and naming the letters as he turned the pages. We would write letters on a piece of paper and he would yell out their names, beaming with pleasure when everyone would say “Wow, Tommy!” He also enjoyed counting, coloring and playing with cars. He learned many words, but his favorite was “no” and he would grin and shake his head when he said it in response to all sorts of questions.

We are very grateful that Tommy was healthy and happy until the end of this life. His first hospitalization was his only and last. Tommy passed away on February 9, 2011 at Rady Children’s Hospital in San Diego, in the loving arms of his mom and daddy. We loved Tommy so much, and will miss him terribly. Although Tommy was with us for less than two years, the impact he had on our lives will always be with us. He was our special little guy, and he will never be forgotten.

Sincerely,

Carrie and Evan Pence of Temecula, California

IN MEMORIAM

Kennady Quinnell
June 15, 2010 – May 28, 2011

Families of SMA,

Thank you for your support during this difficult time. We would have been completely lost without everyone’s support and information on the website. The book you sent brought tears to our eyes and we read it to Kennady when we went to visit her after receiving it in the mail. You all change lives, thank you for all you do. We will continue to spread awareness and fight for a cure for SMA.

Love,

Corey, Erin and Angel Baby Kennady Quinnell of West Allis, Wisconsin
IN MEMORIAM
Wyatt Arnold
July 9, 2010 - December 12, 2010

IN MEMORIAM
Gabriel Rodriguez
April 21, 2011 - June 18, 2011

IN MEMORIAM
Kash Golobic
February 15, 2008 - October 9, 2008

IN MEMORIAM
Maliel Cosby
May 25, 2000 - July 26, 2011

IN MEMORIAM
Tala Albakri
March 16, 2010 - September 8, 2011

IN MEMORIAM
Cruciano Fanaro
April 9, 2008 - September 7, 2008

IN MEMORIAM
Nathan Russell
August 3, 2003 - April 28, 2010
Knocking SMA Out of the Park – One Mother’s Perspective

By Shawn Santos

This year, the Greater Florida chapter of FSMA held our first-ever SMA Awareness Night with the Tampa Bay Rays, a Major League Baseball team that plays in St. Petersburg, Florida. We were very excited to learn that the game, the last in a three-game series against the Boston Red Sox, would be broadcast to a national audience on ESPN.

Knowing that this was an incredible opportunity to reach 20,000 people in the stadium (and millions on TV), we asked David Baggs, the Rays staffer who helped us coordinate the event, if we could share our message in a public service announcement to be played on the Jumbotron. Thankfully, he said, “YES” and scheduled a 90 second opening for us in the pre-game show. Now, we just needed to produce that PSA!

Our Chapter Vice-President Audra Butler quickly wrote the script, and Chapter Secretary Laurie Sore found a friend, Bill McArdle, with a video production company who was willing to produce our PSA for free. So, on a rainy day in early July, most of our Board members and our kids, including, Chapter President Katie Kerns and her daughter Allison, Volunteer Coordinator Lisa Hoang and her daughter Maia, Audra, Laurie, and my daughter Brooklyn and me, met at a park in Tarpon Springs to film the PSA. After we had taped our parts, we started talking to Bill about how the PSA should end. That is when I suggested that Brooklyn could chant “CURE SMA,” which she had started doing one day at the national conference! She was adorable, and it was the perfect way to wrap up the PSA.

After promoting the game for months, David from the Rays informed us that we had sold enough tickets to allow one of our chapter members to deliver the rosin bag to the pitcher’s mound. Laurie, who took the lead on organizing the event, put the names of the kids in our chapter in a hat, and her son picked the winning name. To my surprise, Brooklyn was selected to be the bat girl! When I told her the news, she was so excited that she could not stop talking about it everywhere we went! She told any and everyone we met that she was going to be a “bat girl” on TV!

Finally, game day—July 17, 2011—arrived. My husband Jayson and I had to bring Brooklyn to Tropicana field in St. Petersburg extra early, since she was part of the pre-game activities. Once we got to the stadium, we met with Rays staffer David and Steven, our professional photographer from Thee Photo Ninja, who would capture this wonderful experience for us in pictures. We got our press passes and went down to the field. Honestly, I don’t know who was more excited—Brooklyn or her daddy!

As we waited on the sideline, Brooklyn was introduced to “Ray,” the team’s official mascot. Then, our PSA played on the big screen, and the crowd began to cheer as Brooklyn chanted “CURE SMA.” Just when we thought we couldn’t get more excited, the game’s announcer came over to us and told Brooklyn that there was a change in plans – she was going to deliver the ball for the first pitch to the mound, instead of the rosin bag. Our “ball girl” was thrilled to hold the actual ball that the Rays were going to be using during their game!

Finally, it was time for Brooklyn’s turn on the field. The announcer instructed her to go to the pitcher’s mound, drop the ball, then turn around and wave to the crowd. She took off across the field, with Jayson accompanying her, and with an SMA banner that said “The Rays Helping Knock SMA Out of the Park” on the back of her wheelchair. She was featured on the Jumbotron as she made her way to the mound, which was her dream come true (although she was a little upset with her daddy because he was blocking her view of the screen). Once she dropped the ball off, they both turned around, and I couldn’t really tell if Brooklyn or Jayson had the bigger smile! We were all so happy and proud to spread so much SMA awareness!

Once off the field, we went up to the party deck where all our supporters were sitting to watch the game. On the way to our seats, tons of people commended Brooklyn on what a great job she did delivering the ball. She thought she was famous (and rightly so)!

After the pictures, Brooklyn and Allison went exploring around the stadium, getting attention from everyone they met. Thanks to Katie, the girls also got a sneak peek at the official “cow rays,” who were swimming in a big tank. We left the game early to get our star to bed, which is a good thing since the game went on to be the longest one in Rays’ history with 16 innings! This game will always be remembered, and we are so proud of Brooklyn and our chapter’s role in it. This definitely was an amazing experience, which we hope to repeat next year and in the years to come.
Families of SMA is supported by the tireless efforts of dedicated families, circles of influence and volunteers, who execute hundreds of fundraising events across the country each year. Their dedication to fundraising in support of the Families of SMA mission allows us to continue aggressively funding the research that will create the best odds for finding an effective treatment and eventually, a cure for SMA.

We have expanded the Chapter section of our Directions Newsletter to include all events, updates and announcements for all activities across the country. You can find your write-up or a listing of your event within your local Chapter’s section under the state in which the event took place. Thanks again for all that you do!

Do you want to see your Chapter updates or Fundraising Event in the next issue of Directions?

Please send submissions to our Development Team at fundraising@fisma.org
Team Families of SMA Running to New Heights

Families of SMA is excited to announce that we have been accepted as an Official Charity Partner for the New York City Half Marathon in March 2012 and the 2011 Chicago Half Marathon & 5K that will take place in September. Together, with a total of 36 committed runners, Families of SMA supporters are on track to raise over $48,000 towards important new research and family support programs!

The advantages of being an official charity partner in an organized run are varied, but frequently include discounted race entry fees and a charity tent for Team FSM A runners to gather at before and after the race. Plus, running as an official team allows for the opportunity to meet and socialize with other local runners who believe in the mission of FSM A. Most races have multiple charity partners as well, so there is also always an opportunity to meet other charity runners and spread awareness about our cause, and teach other runners about Spinal Muscular Atrophy.

With your help, we want to keep our new running program going strong, and encompass many more races across the country. Do you know of a race in your area that you think you could recruit runners to participate and fundraise in? If so, we want to hear your ideas! Send an e-mail to fundraising@fsma.org or call our National Office at (800) 886-1762. Let us know which race you could recruit runners for, and we’ll take care of the rest- it’s that easy!

In the meantime, Team Families of SMA will once again be a charity partner in the 2012 New York City Half Marathon on March 18th, 2012. To join our team in this exciting 13.1 mile race through NYC, please visit www.fsma.org/nychalfmarathon today as availability is limited. Families of SMA is also a charity partner in Cincinnati’s Flying Pig Marathon on May 5th, 2012, which is one of our new races for 2012! Sign up to become a member of Team Families of SMA today in Cincinnati, Ohio by visiting www.fsma.org/flyingpig.

Keep checking the Families of SMA Events Calendar to learn more about participating in many more upcoming races around the country!

Fundraise Online with a Personalized Gift Registry Page

Families of SMA wants to help you raise funds for Spinal Muscular Atrophy as easily and effectively as possible. Because of this, on the Families of SMA website, in addition to our events calendar, we offer the capability to start your own personal Gift Registry Webpage. Gift Registries are a great way to set up an individual fundraising page that is not connected to a larger fundraising event. For example, if you were to create a Birthday Gift Registry, instead of family and friends buying you birthday presents, they can make a donation to support the mission of Families of SMA. This is the best way to get the people close to you involved in helping to develop a treatment and cure for SMA!

Gift Registries help to facilitate your own individual fundraising efforts, while allowing you to add a personal touch with words and photos. Setting up a registry takes only a few minutes. To find out how, please visit www.curesma.org/Fundraising/GiftRegistries.
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<td>43861</td>
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<td>9014</td>
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<td>Arvada, CO 80001</td>
<td>(970) 349-0481 or (877) 591-4023 (toll free)</td>
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<td>185744</td>
<td>Hamden, CT 06518</td>
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Have you ever thought about starting a chapter?  

We want to hear from you.

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

and may differ from current fundraising totals by the time you receive this newsletter.
Alabama Chapter

The Alabama Chapter has been hard at work this past spring and summer! On April 2nd, Logan Patrick and his mom Jennifer attended "Women's Night Out" at Flatwoods Baptist Church. Jennifer was able to spread awareness of SMA and share how it has affected their life. The church donated $300 to Families of SMA!

On April 7th, the Kiwanis Club of Tuscaloosa, AL invited Logan and Jennifer Patrick to come speak at their meeting about SMA as well. They donated $100 to FSMA.

Schlotzsky’s Deli in Tuscaloosa held a fundraiser for the Alabama Chapter on April 17th. The chapter invited friends and family to eat at Schlotzsky’s, and the restaurant donated 20% of the profit from that day to FSMA. It totaled almost $300. Unfortunately, a tornado destroyed Schlotzsky’s Deli just 10 days later, on April 27th, but they graciously donated the money to FSMA anyway.

Sparkman High School in Harvest, Alabama held an Awareness Day on March 17th, in honor of Chloe Bush, SMA Type I. The school was able to raise $1,069 for Families of SMA!

Mississippi

Laugh Away SMA

The 2nd Annual Laugh Away SMA Comedy Showcase presented by Stop SMA took place on Saturday, August 6th, 2011 at the Muse Center on the Rankin Campus of Hinds Community College. This family-friendly event is a unique way to spread the word about SMA and raise money to fund research.

This year, the event starred nationally renowned comedians Henry Cho and Brian Regan, two incredible entertainers and compassionate individuals. This was Henry’s second year participating and he was instrumental in recruiting Brian to perform.

In front of a crowd of 1,450, the night began with a check presentation to Gary Bassell, Ph. D., of Bassell Labs at Emory University in Atlanta. Stop SMA has partnered with Families of SMA, each funding half of the $140,000 needed over the next two years to investigate the effects of SMN on mRNA transport and local protein synthesis in motor axons. This important project will help gain insight into the mechanisms leading to specific motor neuron degeneration in SMA, paving the way for the development of new therapeutic strategies for a cure.

Applebee’s in Northport, AL held a Pancake Breakfast for the Alabama Chapter on July 16th. We had a great turnout and raised $1,000. Thanks to Rhyann Granger and Jeff Taylor for donating a 3-night stay at a condominium on the beach to raffle off in conjunction with the Pancake Breakfast! Congratulations to Bobbie Able for winning the trip!

Alaska Chapter

The Alaska Chapter has been busy getting organized and spreading awareness about SMA. So far 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 feel free to e-mail us at Alaska@fsma.org!

Dr. Bassell and Families of SMA President Kenneth Hobby were on stage for the presentation and spoke to the audience about the importance of supporting Spinal Muscular Atrophy. Both gentlemen were very inspiring in their words about this disease, the progress that is being made and the “enormous hope” in finding a treatment. Dr. Bassell conveyed how hard scientists are working in his lab and the “new spirit” they received from knowing about the Laugh Away SMA event.

The room was full of passion, encouragement, love and faith that we will have the answers soon. It is our hope that Laugh Away SMA will help us to realize that goal a little faster. “A cheerful heart is good medicine…” Proverbs 17:22.

Thank you to Jeff and Dee Horton and Stop SMA Foundation for your generous support!
Arizona Chapter

15th Annual KDK SMASH SMA Golf Classic 2011

On Friday, May 6th, 2011, the Arizona Chapter held the 15th Annual KDK SMASH SMA Golf Classic. It was a wonderful day and we had a huge turnout of golfers for the tournament. The AZ Chapter has hosted this tournament on the first Friday in May since 1996. Many of the same families and friends have participated in the event since year one! With the support of Arizona families and friends, the KDK SMASH raised over $14,000 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!

Cochise Contractors sponsored the dinner for all the families as they have each and every year! We were also thrilled with the many other sponsors and 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 Friday, May 4th, 2012 as we celebrate the 16th Annual KDK SMASH SMA Golf Classic. Please visit the KDK SMASH website at www.kdksmash.com for pictures and information about this special event!

We are so appreciative and thankful for all the wonderful support we get for the KDK SMASH Golf Classic. We know how truly blessed we are to have so many generous families and friends help support us as we work to find a cure for Spinal Muscular Atrophy.

Families of SMA is especially thankful to the Kaler Family for continuing to put on this amazing event each year.

Carolinatas Chapter

North Carolina

3rd Annual Gray’s Gang Walk-n-Roll

The 3rd Annual Gray’s Gang Walk-n-Roll was held on April 9th, 2011 in Charlotte, North Carolina in honor of Gray Dougherty. The event had a wonderful turnout and a great time was had by all! Attendees enjoyed a DJ, crafts, a cake walk and a field goal contest! Because of the outpouring of support from family and friends of the Dougherty Family, the event raised $28,708 for Families of SMA! Thank you to the event sponsors, everyone who came to walk and Abbie and Mike Dougherty for hosting the event!

3rd Annual Jocelyn Paige Lee Golf Tournament

JPL Foundation was very busy this Spring, because just one month after their well-attended Benefit, supporters hit the links! On May 14th, 2011, The River Golf and Country Club in Jocelyn’s hometown, Louisburg, North Carolina was the destination. The event included a Shotgun Start and Captain’s Choice. Participants could test their skills by participating in the Longest Drive and Closest to the Pin Contests. It was a great day and raised a total of $4,326!
South Carolina

**Sangaree Middle School Fundraiser**

Sangaree Middle School of Ladson, South Carolina, raised $529 for FSMA. The school authorized students to wear hats for the day if they donated $1, and teachers to wear jeans for the day if they donated $5. Charlie Tisdale, a special education teacher at the school and his wife, Lori, ran the 2011 NYC Half Marathon on March 21st, 2011 in memory of their first daughter, Rachael Marie Tisdale (May 9th, 2005 – January 6th, 2006). The fundraiser helped Charlie surpass the goal of his charity run for Team Families of SMA. Together, Charlie and Lori raised over $3,000 in memory of beautiful Rachael.

Thank you to Charlie, Lori, and everyone at Sangaree Middle School for your support of Families of SMA!

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**Car Show Fundraiser**

On May 22nd, 2011, Amy Bright organized a Car Show Fundraiser in Easley, South Carolina. In total, the event raised $250 for Families of SMA! Thanks for all of your hard work, Amy!

Thanks to the efforts of Christie Tolson, the Governor of South Carolina proclaimed August SMA Awareness Month! Pictured is Holly Tolson and the official proclamation!

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**Chesapeake Crab Feast**

Summer and Fall are the busiest seasons for the Chesapeake Chapter. This summer, the chapter held its 18th Annual Crab Feast and Auction on August 7th, 2011 in Towson, MD. 2011 proved to be another great year and hosted record attendance close to 400 people! With a record year in profits, the auction made over $9,000. Even with the economy in a downturn, so many family members, friends, and businesses came through with wonderful donations. In total, the event raised $31,000! Everyone involved with the Crab Feast has great hopes for an even better year next year! At the event, the Chapter welcomed Derek and Tina Lewis of Mount Airy, Maryland who will join the Chesapeake Chapter team of officers. The Lewis’ will be assisting the Chesapeake Chapter in supporting and connecting SMA families in the Maryland, Virginia, and DC areas.

A big “thank you” goes out to the Trainor and Venedam families for all of the time and energy that they give to making the Crab Feast a success year after year!

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**17th Annual Charity Golf Classic**

The 17th Annual Charity Golf Classic presented by Cramer Rosenthal McGlynn, LLC was held on September 23rd, 2011 in Windsor Mill, MD. The rain didn’t dampen the spirits of the golfers who traveled from as far away as California, Illinois and New York to participate in this fun-filled event. It was revealed that proceeds from this golf outing will benefit the Erin Trainor Memorial Fund established in loving memory of Erin Trainor, daughter of Gene and Barbara Trainor of California. This fund will provide scholarships for newly diagnosed families to attend the Annual SMA Conference.

Congratulations and thank you to event organizers Scott Geller, the Trainor and Venedam families for making the 17th Annual Charity Golf Classic the most successful to date! You raised over $164,000 and counting!
Connecticut Chapter

Emma Hope Award from NES Pageants
Emma Goldsberry (SMA Type II) is pictured with Nadia White and Morgan Scarbro, the latest recipients of the Emma Hope Award from the NES Pageants. Nadia won at the Miss Northeast America pageant, and Morgan won at NES Nationals. These pageants are the latest from NES Director Tammy Rezendes that donate entry fees from the Emma Hope Award to FSMA. The fees from these two pageants totaled $500, and those fees along with fees from other pageants, photo contests, and other donations, have totaled over $10,000 in donations for FSMA from NES. The Emma Hope Award is given to the participant displaying the most kindness towards others.

The Connecticut Chapter had a wonderful time at SMA Camp again this year. For the full details, please see page 38.

Be on the lookout for more information about this year’s Walk-n-Roll at the beginning of 2012!

Georgia Chapter

SMA Workout for a Cause
The Georgia Chapter held their first SMA Workout for a Cause at World Camp Crossfit on June 18th, 2011 in Albany, GA. The event was a huge success raising about $5,000. Over 10 CrossFit gyms from Florida, Georgia and Hawaii participated by raising money and doing the workout “Mateo”, named for Georgia Chapter President Rio Landa’s son. It was a challenging workout, but it was great to see such wonderful support for FSMA.

The Georgia Chapter is getting excited about the 2nd Annual 5K Eat ‘n Run whose planning is in the works for 2012! Read on to hear more about this year’s Eat ‘n Run!

SMA 5K Eat ‘n Run
Our 1st Annual SMA 5K - Eat ‘n Run was held on March 26th, 2011 in Columbus, Georgia. We were blessed with an overwhelming response from our community and raised a total of $54,606, surpassing our $50,000 goal! In addition to dozens of other supporters, we had over 600 5K participants.

We started planning the fundraiser shortly after Caleb Merriken’s diagnosis of Spinal Muscular Atrophy Type II. Our committee wanted to raise money as a tangible way of supporting a cure for SMA. Caleb, 3, is the son of Kanaan and Kari Merriken, who are part of a nine person planning committee. The fundraiser is an important aspect in each of our lives because we have a personal connection to Caleb.

“The opportunity to raise money for a cure has allowed us to take part in this battle that so many face every day. We are excited to begin planning our 2nd Annual event, which will be held in March of 2012, and we are grateful to our community for the support they have shown.” - Kari Merriken

Many thanks to Kanaan and Kari Merriken for your tireless efforts in supporting Families of SMA.
Hello from the Greater Florida Chapter! Covering central and north Florida, our chapter is made up of wonderful, inspirational families, and our board works hard to provide them the support that they need. Whether it’s guidance for a newly diagnosed family, assistance finding doctors, or help with IEP meetings, our goal is to be a meaningful resource to families – while also raising SMA Awareness in all of the communities that we serve.

Most of our fundraising and awareness events currently take place in the Tampa Bay or Orlando areas, but with the addition of new families in the northern part of the state, we are hoping to expand the chapter’s reach. In addition to these efforts, we are pleased to announce our newly reorganized Board. Members include Katie Kerns, President; Audra Butler, Vice President; Tanya Krajewski, Treasurer; Laurie Sore, Secretary; Lisa Hoang, Volunteer Coordinator; and Shawn Santos, Sponsorship and Procurement. We are very proud of our Board and appreciate the tremendous amount of work each of these women do for our chapter.

Our awareness and fundraising efforts for the first half of 2011 included the following:

**Gift of Giving Event at Shore Acres Elementary in St. Petersburg, FL, on March 9th**

Katie Kerns represented FSMA at this wonderful awareness event. With more than 20 charities present, this was a great opportunity to share information about SMA with parents, students and other non-profits.

**Spring Chapter Meeting in Brandon, FL, on March 12th**

We introduced a new meeting format that included a brief update by Katie Kerns, followed by information breakout sessions for chapter members. Our Type I families shared ideas about how our chapter can help them further, and our friend Penny Perez from Putnam Healthcare Advocates gave us TONS of wonderful information on insurance, Social Security and how to deal with bills! We are planning another meeting in the same format for fall 2011.

**Quaker Steak & Lube Bike Night in Clearwater, FL, on March 16th**

The Greater Florida Chapter booth at this popular event featured rare Harley Davidson Ireland t-shirts and FSMA merchandise. With about 1,000 bikers who regularly attend, it was a great way to reach out to the community about the impact of SMA. The Kerns, Butler and Sore families represented FSMA at this fun event, with Xander Sore, son of Laurie (Type III), enthusiastically collecting change and pointing attendants to our booth!

**St. Patrick’s Day Event in Orlando, FL, on March 17th**

Sponsored by O’Shucks Pub and Billiards, this marked our chapter’s first Orlando event! Greater Florida Chapter member Diana Baross (Type II) was instrumental in organizing a great way to celebrate St. Paddy’s Day, which featured a $100 cash prize and free tickets to Orlando’s hottest, newest night-time show, Treasure Tavern. Of course, a special appearance was made by St. Patrick himself, along with a few of his leprechauns! This fun evening raised more than $400 for FSMA!

**Applebee’s “Dining to Donate” Night in St. Petersburg, FL, on April 28th**

At our chapter’s first “Dining to Donate” night, almost 20 different families, couples, friends and individuals enjoyed dinner with SMA kids Maia Shockley (Type II) and Allison Kerns (Type II) and their families.
Ten percent of all the money spent by those presenting our FSMA “Dining to Donate” flyer was donated to FSMA. The “Dining to Donate” program is a great way for our friends and family members to get together, catch up and raise money without even trying—the easiest way! With approximately $574 in sales, Applebee’s donated $57 to FSMA.

**PSCUFS Charity Fair in St. Petersburg, FL on Tuesday, July 19th**

Katie Kerns attended this event to promote our chapter’s events and to recruit more community volunteers! PSCUFS handles MasterCard and VISA accounts for Credit Unions across the country. The company currently has over 750+ employees (where Michael Kerns, father to Allison Kerns, Type II, is employed.) We hope to get lots of volunteers from this event!

**SMA Day with the Tampa Bay Rays in St. Petersburg, FL on Sunday, July 17th**

We sold a total of 189 tickets to this Major League Baseball game, raising $567 for FSMA and even more awareness. In the pre-game show before the Rays took on the Boston Red Sox live on ESPN, a very special public service announcement played on the Jumbotron in front of approximately 20,000 fans. The 90 second video featured four Greater Florida chapter officers, Katie Kerns, Audra Butler, Lisa Hoang and Shawn Santos, who explained the impact of the disease and showed photos of the SMA-affected children in our region, as well as important SMA facts. You can watch the PSA at this link on YouTube: www.youtube.com/watch?v=jT2_KT7DFHg. Right before game time, ball girl Brooklyn Santos (Type II) brought the game ball to the pitcher’s mound, with the slogan of “Kicking SMA Out of the Park” on her wheelchair. Brooklyn’s experience as the ball girl at the game – which was the longest game in Ray’s history – was even covered by a newspaper in the Catskills, NY! Along with the awareness, we also raised $591 at the game!

**SMA Family Fun Day in Tampa, FL on Saturday, May 21st**

Held at Freedom Playground at MacFarlane Park in the South Tampa area, the Family Fun Day was a great opportunity for chapter members to get to know other SMA families in our region! The event featured games and activities for kids, yummy food for all, and even special prizes for families in attendance, including gift certificates, cookie baskets and more. In fact, the Trakas family won the grand prize of a vacation stay at a gorgeous beachfront condo in Ft. Myers Beach. As part of this celebration of families, a professional photographer was there to take pictures of each family. Of course, the most special part of the day was the balloon release ceremony, where we remembered our SMA angels and cheered on our warriors.
The chapter also organized a variety of events during SMA Awareness Month in August:

- Chapter members, family and friends came together for food, drinks and a good cause at "Dining to Donate" Nights at Applebee's on Monday, August 8th, in North Tampa and Monday, August 15th in Valrico/Brandon and at the "Give Back" Night at Chili's Bar and Grill on Wednesday, August 17th, in Pinellas Park/St. Petersburg, FL.

- On Saturday, August 13th, we paid tribute to our chapter’s angels and honored our warriors at the Candlelight Ceremony in Tarpon Springs, FL at Sunset Beach. Chapter member Heather Toro made lovely awareness ribbons for all participants, and Audra Butler shared a poem in memory of her son, Andy, at this emotional event. The park also surprised us with a $175 donation to FSM A.

- The Greater Florida Chapter headed back to Tropicana Field in St. Petersburg for Community Corner with the Tampa Bay Rays on Tuesday, August 23rd. From the time the gates opened until the conclusion of the second inning, chapter members passed out information to fans to promote SMA awareness, shared their stories and answered questions. We were thrilled to see FSM A featured on the big screen again at this game against the Detroit Tigers!

- For the entire month of August, Thirty-One Representative, Dawn Trayer donated half of her commission to the Greater Florida Chapter. Thirty-One offers stylish, affordable products, from signature purses and totes to storage solutions.

- The cities of Tampa, Tallahassee, Clearwater and St. Petersburg all made official proclamations for SMA Awareness Month. Katie Kerns personally accepted the proclamation from Mayor Frank Hibbard at the Clearwater City Council's August 4th meeting, and Audra Butler joined her to accept a proclamation from Mayor Bill Foster at the St. Petersburg City Council’s meeting on August 18th.

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**A Facebook Connection Leads to Fundraising Success**

My son, Andy, was born in January 2009. When he was just 9 weeks old he was diagnosed with Spinal Muscular Atrophy (SMA), which is the number one genetic killer of children under the age of two. I opened a Facebook account in April 2009, so I could easily keep friends and family updated about his daily health and the progression of the disease.

Deb Shoman Young, a friend from high school, was one of my first Facebook friends. She followed my posts on Andy’s triumphs, setbacks, hospitalizations, and ultimately, his death in June 2009. During the two years since then, she has followed my journey through grief to acceptance.

At the start of 2011, Deb decided that she wanted to lose the 50 pounds that she had gained with the births of her two beautiful and healthy baby girls, but she knew that she needed inspiration to be successful. So, she turned to Facebook and to my son’s story. Inspired by Andy’s fight and in honor of his memory, she now is working to raise $1,000 for Families of Spinal Muscular Atrophy and awareness for the disease as an incentive for her weight loss efforts.

She promotes her fundraising page at www.fisma.org/GiftrReg/Debyoung regularly on Facebook. She posts about SMA. She posts about her challenges in her quest to fit in her skinny jeans and her accomplishments when working out. She posts about Andy. Her posts are commented on and reposted by friends and family – people who now know about SMA because of her efforts. She has received a great deal of encouragement and support. As a result, Deb is now very close to meeting both of her goals - fundraising and weight loss.

Her success also inspired me to take control of my health. In April 2011, I launched “Getting Fit to Fight SMA” – a campaign to raise money for FSM A while losing weight and getting in shape. As of July 21, I have lost 43.6 pounds – and I’m still going strong. I’d like to think that Andy is proud of his mama and her friend Deb, as he watches from heaven – I know I’m proud of him and the difference he has made in the fight against SMA. My donation page is www.fisma.org/GiftrReg/AndysArmy.

All because of Facebook. All because of friendship. All because of Andy.

Thank you,
Audra Butler

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This year, the Greater Florida chapter was also honored to be part of FSM A’s national conference at Disney World in Orlando. Our national team worked so hard to make sure we all had every topic, question and detail taken care of. It was a “magical” conference, and all of our chapter members in attendance had a wonderful time.
Greater New York Chapter

SMA Awareness Day with the New York Mets
The Greater New York Chapter was honored at SMA Awareness Day at Citi Field on August 21st. The SMA children in attendance received an on field spirit award with Mets player David Wright. David Wright spent time with the kids and signed autographs. During the game the Mets played a PSA about SMA. At the end of the game, the kids were given VIP treatment during the Mr. Met Dash and were able to run and ride the bases. This day was to honor the memory of Stephen Ruocco.

SMA Awareness Day with the New York Yankees
We also celebrated SMA Awareness Day with the New York Yankees on September 3rd. Before the game started, there was a special presentation about SMA given to the entire stadium. A few of our SMA families and sponsors were able to be on the field while the presentation played and our FSMA logo was prominently displayed on the Jumbotron where it was shown during the entire game. Thank you to our sponsor, Quest Diagnostics, who supported our SMA awareness with the NY Yankees, with a gift of $12,600. We also thank Greater NY Chapter President Debbie Cuevas for organizing as well as all of the wonderful SMA families and friends that came out for the day!

During the month of August, the Chapter participated in the Macy’s Shop for a Cause event raising over $600 and Purple Hair Extensions at Hair Studio II to honor SMA Awareness Month. Some of our chapter families also participated in a BBQ at Gracie Mansion with Mayor Bloomberg to honor the anniversary of the Americans with Disabilities Act.
Hudson Hills Golf Outing
The Greater NY Chapter held its very first Golf Outing on July 18th at Hudson Hills Golf Course in Ossining, New York. The day’s events included a Putting Contest, Hole In One Contests, Raffles and Silent Auction. Westchester County Executive, Rob Astorino, attended the dinner and presented Debbie Cuevas, the Greater NY Chapter President, with a Proclamation declaring the entire month of July 2011 as SMA Awareness Month in honor of the Golf Outing. There were twelve SMA Families from the Greater NY Chapter represented at this Golf Outing. The kids who attended were invited into the clubhouse for Wii, crafts and other fun games. Many thanks go to Golf Committee Members Ron Cuevas, Rich Rubenstein and Douglas Erwin. Thanks also to all of our Golf Outing Volunteers and to the staff at Hudson Hills Golf Course for all of their help on the day of the event. The Greater NY Chapter would also like to thank Jeff Rhode for donating his services and photographing our golf event. We were blessed to raise almost $40,000 for this event. What an amazing effort by all of our families, volunteers and golfers.

Dear Families of SMA,
Enclosed is the $215 check that was the profit from my Pampered Chef show on March 12th, 2011. My mom, Mary, was the consultant. She has enclosed her commission check of $86. Her company offers a matching program. So, all in all we will end up with a donation of $387 all together. Yeah! This is all in memory of our angel, Eleanor Rose Cisco.

Take Care,
Kari Ann Cisco
Holbrook, New York

Owen’s 3rd Angel Anniversary Memorial Bowling Event
On August 16th, 2008, our adorable 5 month old son, Owen Vincent Shuler, passed away from SMA Type I. The weeks and months have quickly slipped into years since we last held our perfect little boy in our arms. It seems impossible that so much time as passed. Thankfully Owen’s little sister, Natalie (who turned one on Aug 9th) has brought happiness back into our lives, but the hurt of losing Owen has not, nor will it ever fade.

SMA robbed Owen of a long healthy life and we could not control it, but we are determined to make sure he is never forgotten. This summer on August 13th, 2011, to commemorate Owen’s 3rd Angel-Anniversary, we held a memorial bowling event. Over 100 of our family and close friends came out to remember our brave baby boy. We bowled, we laughed and I think it’s safe to say that we all had a good cry. We honored Owen by being together, talking about him, watching videos and we fought back against SMA by spreading awareness.

We can’t thank everyone enough who came out to the event or sent us well wishes. It’s often difficult to talk about Owen without crying but having your love and support makes it easier. A very special thank you to my sister Maureen Gill, and friend Jean Beltran who took charge of the Registration table and my dad Ben Gill, and friend Lauren Lundy O’Connor who ran the SMA Awareness table; where we sold “Fight for Owen, CURE SMA” bracelets and handed out pamphlets with Owen’s story and information on SMA. The event would not have been possible without their help!

What made the day even more special was having six incredible SMA families come from near and far to pay tribute to Owen. Thank you to “our SMA family”; the Cuevas, Kloiber, Nelson, Purk, Ruocco and Van De Loo families. Six different families, in different phases of their own SMA journeys, coming together to remember our sweet little boy. We are so thankful to have each of you in our lives and wish more than anything that we were not bound together by this cruel disease. We look forward to the day we can all celebrate the END of SMA in honor of all warriors and angels.

Dorothy, Jon and Natalie Shuler
Stony Point, NY

www.FightForOwen.com

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Dorothy, Jon and Natalie Shuler
Stony Point, NY

www.FightForOwen.com
Illinois Chapter

Illinois Chapter Walk-n-Roll
The Illinois Chapter held its Annual Walk-n-Roll on June 12th, 2011. It was a beautiful Sunday morning with a record number of people in attendance. The 2.5 mile walk was held at Independence Grove in Libertyville, Illinois. A local Pom Squad kicked off the event with a special cheer and also helped to motivate participants during the walk. As participants walked along the paved path, there were 25 activity stations that were in honor or memory of affected chapter members. All attendees also enjoyed a face painter, balloon artist, raffle, tattoo table and a T-Shirt Spinner Station. Our T-Shirt Spinner was a huge success and everyone at the walk could personalize their own t-shirts! At the end of the walk, medals were given to all walkers as they crossed under the balloon arch finish line! A great time was had by all and we are looking forward to seeing everyone again next year!

Janet Schoenborn, you never cease to amaze us with your energy and devotion to Families of SMA — thank you!

3rd Annual Bowl-a-Thon in Memory of Zachary Brian Deutschle
The 3rd Annual Bowl-a-Thon in Memory of Zachary Brian Deutschle was held on May 14th, 2011 in Aurora, Illinois. This year, Brian and Sherri Deutschle were able to raise $1,980 in memory of their son, Zachary.

First Annual Tee Off with the Drive to Cure SMA in Honor of Ryan
Tee Off with the Drive to Cure SMA in Honor of Ryan was a huge success in its first year! This now-annual golf tournament was held by Ken and Jeanne Emerson, grandparents of Ryan Manfre, SMA Type III, at Glen Eagles Country Club in Lemont, Illinois. Ken and Jeanne were joined by their daughters and sons-in-law, Becky and Brian Manfre (parents of Ryan), and Suzanne and Stephan Fedea, making the tournament a huge hit. Ken was hard at work for months before the event, which was held on May 21st, 2011, rallying support and golfers from all over the Chicagoland area. The Lemont area really came together and overwhelmed the family by their generous hearts and willingness to support such a worthy cause. All together, the Emerson, Manfre and Fedea families raised $48,743, with an outstanding 43 foursomes (172 golfers!) that all showed up for the big day, with even more joining in for the banquet and silent auction that followed! Mark your calendars for next year’s tournament, scheduled for May 19th, 2012, and visit www.fsma.org/GolfForSMAandRyan for more information as the date approaches!

First Annual Kevin James Lintz Memorial Golf Tournament
Dear FSMA,

The 1st Annual Kevin James Lintz Memorial Golf Tournament was held on July 30th, 2011 at Rolling Hills Golf Course in Godfrey, Illinois. This first year tournament raised $6,125 thanks to the support of family and friends, and will now be held every year on the last Saturday in July. This tournament was held as a memory, a birthday present, and a show of the power of love when generous and loving people come together in search for a cure for a devastating disease. Thank you for your relentless support and dedication to this cause. We truly hope this money will help the families that have gone through this terrible ordeal and assist in finding a cure.

Guardian Angel from heaven so bright,
Watching beside me to lead me aright
Fold your wings around me,
and guard me with your love,
And softly sing songs to me
from Heaven Above.

Thanks,
Shawn, Jenny and Kevin Lintz

Dear Families of SMA,

On behalf of Mercer’s Chicago office employees, we are pleased to present you with $610 for Families of SMA. Our colleague, Michelle Reuter, friend of Ryan Manfre (SMA Type III), brought your organization to our attention. The money was raised during a successful jeans day on Friday, May 13th where several employees participated to raise awareness of your organization and to enjoy a casual day at work. Thank you for all you do to help so many people.

Sincerely,
Lynne Schauwecker, Fundraising Coordinator
Chicago, IL

Dear Families of SMA,

At CCC Technologies, Inc. we hold a charity poker tournament every month with the proceeds going to an employee-suggested charity. In July, we played for Families of SMA thanks to the suggestion of one of our Senior Account Executives, Kevin O’Brien, whose family has been involved with FSMA for quite some time.

Enclosed, please find a check for the proceeds of our charity game held on July 7th, 2011, along with cash and check donations made by employees in the total of $500.

Thank you very much for all you do to support families affected by SMA and all you do to help work for a cure.

Sincerely,
Kim L. Kiefer, Director of Marketing
Elk Grove Village, IL

and may differ from current fundraising totals by the time you receive this newsletter.
Iowa Chapter

Dear Families of SMA,

Enclosed please find a check for $250, donated on behalf of Madison Ramirez, by our Student Physical Therapy Organization. Madi and her mother participated in a class session on SMA for Doctor of Physical Therapy Students at our university. They were both very inspirational in sharing their experiences with SMA.

Most sincerely,
John O. Barr, PT, PhD
St. Ambrose University
Davenport, IA

Kansas City Chapter

Kansas

The Kansas City Chapter hosted a Family Day at Deanna Rose Children’s Farmstead in Overland Park, KS. Thank you to all of the families who attended!

Fundraiser in Honor of Ayden Trammel

A fundraiser was held on Sunday, June 5th, 2011 in honor of Ayden Trammel, SMA Type I. Ayden is four years old and lives in Lincoln, Nebraska. The event took place in Phillipsburg, Kansas where his grandparents and other family members live. The event was held in the local Alco store parking lot. Alco, Frito Lay and Pepsi furnished hotdogs, buns, chips, drinks and all the fixings. All we had to do was show up and raise money! We cooked from 11:00 until 2:00 and took in $379 in donations from our generous community. Not bad for three hours work!

Thank you to FSMA for all you do to help families cope with this dreadful disease—hopefully a cure is on the horizon.

Thank you,
Donna Studley, Grandparent to Ayden Phillipsburg, KS

Missouri

Bommarito Z Club Car Show

The Annual Bommarito Z Club Car Show was held on August 7th, 2011 by the Gateway Z Car Club. The event had an excellent turn out and raised over $14,400—our best year yet. Families who attended enjoyed food, a DJ, door prizes, a raffle and a silent auction! There were also plenty of awesome cars for all to check out! Thank you to Jerry at the Bommarito dealerships and to Janet Hutchinson and Steve Colesworthy, the driving forces behind the event. We are grateful to the Z Club, President Tami Dunn and all members for their support. Thank you also to Kathy Goodyear, Rita Schmidt and the Carpenter Family for their help throughout the day. Without all of your support, the event would not have been as big of a success!

Benefit Yard Sale

$777 was raised by the 8th Grade Peer Helper class of Warrenburg Middle School in Warrensburg, Missouri by organizing a Benefit Yard Sale. Peter Voskovitch, SMA Type III, has been a member of this group for three years. We were very proud and touched that FSMA was their charity of choice for the last two years. We would like to express our thanks to their teacher Ms. Ashlee Cochran for her dedicated work with this talented group of kids.

Vicki Voskovitch
Warrensburg, MO
Michigan Chapter

7th Annual Families of SMA Michigan Chapter Run, Walk-n-Roll

The 7th Annual Families of SMA - Michigan Chapter Run, Walk-n-Roll is in the books as one of our MOST successful! Over 300 participants joined us to run, walk and roll towards a cure. Together we raised over $41,000 - almost twice as much as we raised last year! Event organizer Holly Schafer described the day by saying, “Every year somehow I am still overtaken with the energy that I feel during this event. It is so wonderful to watch all of those affected by this horrible disease coming together for such a fun time”.

Thank you to everyone involved. The event could not happen without the support of each volunteer, fundraiser, and participant. Thank you also to all of our sponsors. We can’t wait to see everyone next year for the 8th Annual Run, Walk-n-Roll on Saturday, May 5, 2012!

Malorie Fox Golf Tournament-Year #8

We are thrilled to report that the Malorie Fox Golf Tournament for FSM - Year #8 was another success! We had return golfers, as well as a few new faces — so we must be getting the word out there about SMA. The weather, however, did not cooperate! With storm after storm, the golfers only got in approximately 2-5 holes each. We had 26 golfers, 21 family/corporate sponsors, numerous generous donations of silent auction items, and 19 eager volunteers. The total raised that will go towards Spinal Muscular Atrophy research was approximately $11,100 - fantastic! Over the past eight years, we’ve raised just over $73,000 – AWESOME! Let’s keep the momentum going! Thank you Michelle Fox and family!

Abbeys for Abbey

On May 19th, 2011, the House Family of Ann Arbor, Michigan hosted a fundraiser in honor of their adorable daughter Abbey. They called the event Abbeys for Abbey. A number of their amazing friends came together to make it a memorable night. Two friends, Derek and Derek, made a homebrew “Abbey” beer. Swing Shift, a Western Swing band that a few friends are a part of, donated their time. All of the guests danced up a storm and had an amazing time! Abbey’s Mom Melissa said, “Abbey had the best time dancing and twirling around in her wheel chair”. The event raised $1,345 for Families of SMA – Michigan Chapter in honor of Abbey House.

Michael Church ran in the 2011 Kalamazoo Marathon for FSMA on May 8th, 2011. Through his hard work and fundraising efforts, he was able to raise a remarkable $3,283 in honor of his daughter, Maria Francesca.

The Ruggers Up and Under of Kalamazoo, Michigan, hosted a golf tournament for FSM in honor of Brielle and Brooke Kennedy, on June 25th, 2011. The tournament raised a total of $299!
Brooke and Brielle Kennedy

In honor of SMA Awareness month, Brooke and Brielle Kennedy received a proclamation from the Governor of the state of Michigan making August 22nd, 2011 Spinal Muscular Atrophy Awareness Day. Our family wanted to do something special for the Awareness Day, so we held two fundraisers. The first one was “Dining to Donate” through our local Applebee’s restaurants that raised a total of $207. The second fundraiser was an Art Auction of paintings created by Brooke and Brielle. They painted and named their own works of art to sell to raise money for SMA research.

The Brielle and Brooke Art Auction for SMA was so much fun. It was an idea of a friend of mine and I really didn’t think it would take off…but it did! …and in a big way! It was so much fun to watch the daily bidding and seeing our friends get so competitive for such a great cause! Our biggest seller was the “NO!” painting by Brooke that was sold to Steve Churchill for $750! It feels great to have so much support from our friends and get this kind of a great response. We can’t wait to do it again next year!

Both fundraisers for SMA Awareness Month brought us new Facebook Fans, raised a great deal of awareness about SMA and they were both so much fun! We hope that the art creates a conversation piece in the new owner’s house to raise awareness for SMA. Total money raised by Brooke and Brielle for the month of August for Families of SMA was $1,957!!

Sincerely,
The Kennedy Family
Eric, Sarah, Brielle and Brooke
Mattawan, WI

Brielle and Brooke Art Auction Paintings
Several teachers once again organized a 5K Run/Walk fundraiser in Lindstrom, Minnesota on April 30th, 2011. Several of the board members attended, never leaving the inside of the high school gymnasium. The downpour of rain was steady, hard, and long-lasting. Lots of hard work went into organizing the event, and they raised $1,000!

Since our spaghetti dinner last November was so successful, we decided to try a Pancake Breakfast on April 10th, 2011 with the Lion’s Club. We raised $175.

Our 2011 Spaghetti Dinner took place November 27th. Watch out for more info in our next update.

Patty Schwerr, Diane Pouliot and Lori Halvorson attended the conference in Florida. Their conference experience left them very impressed. We were excited to hear that the 2012 Conference would be in Bloomington, Minnesota! Details are being planned and we need lots of support from FSMA members, families and friends!

Ride Away SMA
This year we decided to expand our usual Pancake Breakfast held in memory of Lindsey Ronningen by adding on a Motorcycle/Classic Car Fun Run.

We called it “Ride Away SMA”- Laurie Potter deserves full credit for the name! We still did the pancake breakfast in the morning along with the bike and car registration. For our first year of doing it this way, we had about 85 bikes! We had a five stop run and then met back at an establishment where we had our silent auction, food and bean bag tournament. We ended up raising over $10,000 and hope to continue going this route as it was a huge success!

Amy Allen
Rochester, MN

Grandma’s Marathon
For a long time I had really grinded on what I could do to somehow make a meaningful contribution to my high school friend Aaron High’s family, whose son has SMA. I knew about Families of SMA through the different fundraisers and events that the High’s post on their website HopeForMax.com. I just needed to figure out how I could make more than just my typical yearly monetary contribution.

Over the past few years my wife Lori and I have been running Grandma’s Half Marathon and in 2009 we ran the full. Now I am not going to win the marathon, so it’s really just a sense of accomplishment like it is for most people. So I thought rather than just wasting four hours and hundreds of hours of training for what boils down to a personal goal, why not add some actual meaning to this pain! What a fool I have been for the last couple years with this obvious fundraising solution staring me straight in the face!

So in any case what Lori and I decided to do was run the race this year in honor of Max. We had never met Max, but we felt like we knew him. My son is four years old and the faces and likes and dislikes are interchangeable. He was automatically close to our hearts whether the High’s and Max were aware of this or not. We felt like he was an extension of our son and although we do not experience the direct stress and unknowns that Max’s parents did, it was routinely on our minds, reminding us of how lucky we are to have the luxury to complain about normal life garbage.

Aaron and Tracy High are absolutely awesome parents and their level of love and devotion to their son Max is very obvious. They carry around a huge weight every minute of every day and maybe we could lift a couple ounces of pressure off their shoulders with some fundraising!

I work in real estate and keep a very large data base. I put my assistant to work on a mass email that included around 1,400 people in total. I was more than impressed with the people that you wouldn’t expect to donate who donated. Many clients, family members, and friends took it seriously and donated substantial amounts. In total we raised $2,251. The race went really well and both my wife and I finished faster than the year before, right around 4.5 hours. This year we hope to do it again and we will order some running shirts. If I have any advice for someone that will be doing a similar fundraising event it would be don’t be afraid to send to your entire email list. At least 20% of the donations were complete surprises to me. You just never know who has this condition close to home.

Adam Swor,
Duluth, Minnesota

Dear Families of SMA,

For a class service project, a group of 13 freshmen hosted a Spaghetti Dinner to raise money for SMA. One of our friends suffers from SMA and for our class project we raised about $4,700 in total. We are sending these checks to you in hopes that we can all come closer to a cure for SMA.

From all of us in
Hopkins West Junior High 9A
ALM, Minnetonka, MN

and may differ from current fundraising totals by the time you receive this newsletter.
**Minnesota Chapter Cont.**

**North Dakota**

**Jack Attack on SMA Round 7**

The Jack Attack on SMA Round 7, hosted by Kristi and Rod Gellner in honor of their son Jack, SMA Type II, was held on Saturday, June 11th, 2011 in Fargo, North Dakota. Thanks to the support of family and friends, the event was a huge success! After the walk, all attendees enjoyed a picnic lunch and the children got balloons and the chance to get their faces painted. Thanks again to everyone who came and walked or donated to help us find a treatment and a cure for SMA. In total, Jack Attack Round 7 raised $16,333 for Families of SMA! Together we will KNOCKOUT SMA!

**New England Chapter**

**Massachusetts**

**11th Annual New England Chapter Cure SMA Walk-n-Roll**

The New England Chapter had a record breaking year at the 11th Annual Cure SMA Walk-n-Roll which they hosted on Saturday, May 14th, 2011 at Wompatuck State Park in Hingham, MA. Many thanks to “Team Murphy” in honor of Cianan and Cecilia Murphy and “Team Aileen 2011” for hosting the event! This year’s top fundraising teams included the Johnson Family’s “Walking for William” team, the Norton Family’s “Team Norton,” and the Sohl Family’s “Mac’s Pack.” Together, we raised a record breaking $125,000! Thank you to all who helped by organizing the event, fundraising, donating raffle items, volunteering, attending the walk and bringing your friends and family. The walk would not have been a success without the support of our sponsors or families! Thank you to Silvia Murphy for all of your hard work in organizing the walk and to Stacey Farrell for organizing the raffle!

**Joey Romanoski’s 9th Birthday**

Joey Romanowski turned nine years old on April 3rd, 2011, but instead of asking for gifts, he asked his friends and family to make donations to Families of SMA – New England Chapter. This is the third year in a row that he has requested donations to FSM A in honor of his friend William Johnson who has SMA. Joey’s mom said, ”Joey is committed to donating his birthday money until there is a cure for this terrible disease”. Thank you to Joey for raising $285 to support of Families of SMA. We are blown away by your generosity!

**New England Chapter Ocean Show and Ice Cream Social**

On April 10th, 2011, about 50 people from the SMA community in New England gathered for a social event. Families came from Massachusetts and Rhode Island to enjoy an afternoon of fun, which included an entertaining Ocean Show along with delicious ice cream sundaes. Paul Erickson, who had worked at the New England Aquarium in Boston for 27 years shared his knowledge and love of ocean creatures through videos and slides as well as live music. Everyone was left with a sense of what it’s like to dive in the ocean. There was plenty of time to chat and share information as ice cream was devoured by all. The entertainment and food was coordinated and donated by the Sean W. Venezia Foundation.
Dear Families of SMA,

In June 2009, our close-knit group of high school girl friends played in a charity Kickball tournament. On top of all of the fun we had, we saw such an amazing opportunity for us to do our part and make a difference that we decided to plan and host our own. The six of us got together throughout that summer to plan and hold our first annual Saved by the Balls Kickball Tournament in September 2009. Right away we knew part of our mission would be to donate all proceeds to a different worthy cause every year. After a successful first tournament we were ready and excited to plan our second annual with hopes of donating even more to a new cause.

Planning for the next tournament was well under way and it was time to decide where the proceeds would go. One of our committee members, Krista Heraty, mentioned Families of SMA, New England Chapter. Her boyfriend, Jim Norton, has a cousin named Owen, of Braintree, MA, who was diagnosed in 2004 with SMA Type II. He was only two years old. Owen has never walked or crawled, but he manages to keep his family on their toes. He excels at school, Wii and Nintendo DS, and water therapy, and is always imaginative and goofy. Owen is one of four children, and his family - particularly his parents, Don and Kathy - are among the most giving, caring and selfless individuals we know. None are more deserving than Owen and his family, which is why we felt strongly that this year’s tournament should go towards funding research for SMA and support for SMA families like the Norton’s.

After an extremely successful second annual tournament, with over 150 participants and 20 volunteers, we are proud to say we were able to donate $3,180 to Families of Spinal Muscular Atrophy, New England Chapter in honor of Owen Norton. It means so much to us to have been able to make this donation to a very deserving cause in honor of a very special young boy.

Best Regards,

Krista Heraty, Rachel Karll, Melissa Manolakis, Valerie Marks, Tayla Moore & Cara Murphy

• On March 7th Peace Tree Apparel, Inc. raised $100 in honor of Macarthur Sohl of Massachusetts. Thank you for your thoughtful gift!

• Philip Meneghini, of Ipswich, MA ran a race in memory of his sister Victoria's birthday in March of 2011 and raised $175. Way to go Philip!

• On Friday, July 5th, 2011 Courtney Kraus coordinated all branches of Westfield Bank in a Dress Down Day in memory of Violet Marie Clendenin. Thanks to the support of employees, Courtney was able to raise $101 for Families of SMA!

• On February 13th, 2011 Kari Sagan of Southwick, MA hosted the 1st Annual Violet Marie Clendenin Benefit and Fundraiser. Thanks to Kari’s hard work and the support of her family and community, she was able to raise $425 in memory of Violet Marie Clendenin.

• Big thanks to Stephen Buonconti of West Springfield, MA for running the Boston Marathon on April 18th, 2011 for Families of SMA. Steven raised $8,800 in honor of his daughter, Joanna Buonconti, SMA Type II! Thanks for your hard work and dedication!

• The Flaherty School Coin Drive, held on April 12th, 2011, was organized by Kari Norton of Dorchester, MA and raised $800! A week later, on April 17th, Kate also hosted Norton's Super Sunday Fundraiser. Through her hard work and the support of family and friends, Kate Norton raised $10,000 in honor of Owen Norton! Thanks Kate!

• On April 20th, 2011, Juliet Bockhurst of Westwood, MA celebrated her birthday in honor of William Johnson. Family, friends and party guests helped Juliet raise $250 in honor of William! Great job, Juliet!

• The New England Chapter held a fundraiser at their local T.G.I. Friday's to raise money for Families of SMA. Thanks to Sarah Tronbly for setting up the fundraiser which raised $129. Thank you to all who contributed to the event!
Dear Families of SMA,

It is with great pleasure that Taylor and I are able to send a check for $25,000 to Families of SMA on behalf of the Hailey Mae Foundation. On March 12th, 2011, Taylor and I held our 7th Ski Away SMA event at Magic Mountain in Londonderry, Vermont. This year the weather was great and skiers young and old spent the day enjoying the terrain at Magic, testing their speed on the race course and seeing if there was any bounce left in their knees at the extreme competition. With Nancy and Jen’s help, the night time festivities couldn’t have been better!

After seven years of holding our Ski Away SMA fundraiser in memory of our daughter Hailey, we never cease to be in awe of the generosity of our friends, family and total strangers. We find great comfort in knowing that Hailey continues to touch so many lives.

The donation we send you this year not only comes from efforts from Ski Away SMA 2011, but also from a fundraising event our dear friend Maureen Aneser held in Belle Mead, New Jersey. Not only has Maureen single-handedly organized our raffles and silent auction items at every Ski Away SMA event, but she has also taken it upon herself to bring the efforts of the Hailey Mae Foundation to her hometown. We are forever grateful to Maureen and her husband, Rich.

We wish you all the best and can’t wait for next year!

Fondly,
Marie and Taylor Smelser

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**New Hampshire**

**Constantine’s Carry for a Cure**

The First Annual Constantine’s Carry for a Cure Flag Football Tournament was held on August 13th, 2011 in Keene, New Hampshire in honor of three year old Constantine Salce. Constantine’s parents, Dominic and Luciane Salce, organized the event and raised over $8,200 for Families of SMA! They not only surpassed their $5,000 goal, but spread SMA awareness throughout their community.

Over 300 people attended the event thanks to the support of family and friends and the tireless efforts of the Salce Family who promoted the event on their local radio stations. There were numerous teams that signed up to play in both the competitive and recreational flag football divisions. The competitive division winners were the 80-Percent Unbeatables and the Keene Blackbirds were named the recreational division champions.

The event also had a raffle with some fabulous prizes thanks to the generosity of family and friends. Some of the prizes included a Super Bowl XXXVII football helmet signed by the New England Patriots and a “Red Sox Experience” package that included game tickets and a guided tour of Fenway Park, on-field access and the raffle winner’s name on the scoreboard! Other prizes included movie tickets and other autographed sports memorabilia.

The event could not have been as big of a success without the support of their sponsors. Thank you to Markem Imaje, Greenwald Realty Associates, Fenton Family Dealerships, Brewtopia, Noyes Volkswagen of Keene, Plotkin’s Mattresses & Furniture, and Fairfields.

Again, BIG thanks go to the Salce Family for all of their hard work and dedication in planning this successful first year event!
New Mexico Chapter

“Bugaboo” Workout of the Day
On August 13th, 2011 the New Mexico Chapter, along with CrossFit Albuquerque hosted the third annual “Bugaboo” Workout of the Day (WOD) to raise money and awareness. “Bugaboo” is named for Benjamin “Bugaboo” Abruzzo who was born with Type I SMA and lived just 200 days. The event is a workout that is 200 Thrusters- one for each day of his life. The New Mexico Chapter and CrossFit Albuquerque hold a local event and reached out to the worldwide CrossFit community to support this event. Ten CrossFit Affiliates did the workout and raised $200 a piece. Here in Albuquerque, we had over 70 participants and the total raised was in excess of $8,000. The sun was shining and people of all types came out and struggled through a tough workout in honor of Benjamin and in support of the fight against SMA. We had a great day, met some amazing people and most importantly, remembered Benjamin and continued the fight against this disease.

Big thanks to the Abruzzo family!

Northern California Chapter

9th Annual Northern California Walk-n-Roll
On August 20th, 2011, the Northern California Chapter hosted their 9th Annual Walk-n-Roll in Golden Gate Park in San Francisco, CA. A great group turned out for the walk from Peacock Meadow down to John F. Kennedy drive to a beautiful waterfall located within the park. Thank you to everyone who came out to the event and to Dick and Pat Wolff for all of your hard work! We raised over $33,000 for Families of SMA to help find a cure for Spinal Muscular Atrophy.

Once again, Dick and Pat Wolff pulled out all of the stops to produce a spectacular event! Thank you so much!

Pathways of Promise
We held our 3rd Annual Rockin’ a Cure Dinner, Dance, Silent and Live Auction on Saturday, March 5th, 2011 in Sacramento, California. A small group came together to contribute to our shared vision of raising awareness for and ultimately finding a cure for Spinal Muscular Atrophy. We lost our two angels, Luke and Meagan McWilliams in 2007, and vowed that we would do everything we could to ensure that no other parent or child had to go through what we endured. A good time was had by all and some of the highlights of the evening included Ryan Manriquez emceeing the raffle (he was born to be on stage), the incredible and moving speech given by Meri Stratton Phelps (son, Aaron), and the amazing outpouring of support. Thanks to the generosity of all in attendance, we were able to raise a total of $29,852 this year! In total, Pathways of Promise has raised over $74,000 over the past three years! Thank you to all involved!

Colleen & Glenn McWilliams
Carmichael, CA

Wannabe Charity Golf Tournament
The annual reunion of the Wannabe Charity Golf Tournament was held in Lake Tahoe, Nevada this year, from August, 4th-6th, 2011. The event always begins with an "Opening Ceremony" and ends on the last day with “Closing” which hosts a variety of prizes, awards, announcements of winners, and pictures of the many “teams”. The tournament is fashioned somewhat like the Ryder Cup with two teams. They are “Blue” and “White”. This year we had 32 players. There is always a good amount of bantering back and forth... before, during, and after the event!

The tournament became a charity in the year 2004, after one of our families had a son who was diagnosed with SMA. It just felt like the right thing to do for everyone involved. “Angel” Skylar Bahrenburg is never far from their thoughts as they reunite each year, with a purpose greater than themselves.

Many thanks to Marilyn Belcher for organizing this amazing event.

and may differ from current fundraising totals by the time you receive this newsletter.
Concert for a Cure

2011 marked the 11th Annual Concert for a Cure. Family and friends gathered at Blackhawk Country Club in Danville, California on May 21st, 2011 to support our vision of a world where Spinal Muscular Atrophy is treatable and curable. This year’s theme was “Reaching for a Cure in Miracle-Ville” and 11 years of miracles were celebrated that night.

In 2010, the Concert for a Cure Volunteer team created the Shining Star Award. This award is to be given annually to an individual, business or organization that has demonstrated exceptional dedication and support for making Concert for a Cure a success! This year, the Volunteer Team is proud to honor Piatti Ristorante & Bar for 10 years of commitment. For the past 10 years, Piatti and John Perkins have continued to stand by our side in this fight for a cure with financial sponsorship, personal donations, auction items, volunteering and strong participation at each event. Even during difficult times, John has always been our shining light of hope. Thank you for your continued support!

Concert for a Cure would not have been as successful without the enthusiasm, hard work and dedication of our committee members and volunteers. Thank you to Anna Calderon, Nancy Dindzans, Andris Dindzans, Stephanie Jacobs, Mary McHale, Raelene Sprague, Domini Tarman, Wendy Thompson, Rich Calosso, Julie Doris, Marcela Kamp, Valerie Phelps, Donna Starkweather, Kim Tarman, Courtney West, Cindy Fox, Kathy Krull, Jean Sarver, Sofia Striffler, Dave Thompson and Mary Wheeler.

We would also like to thank the wonderful sponsors who supported Concert for a Cure: True Wireless, Charles Schwab Bank, The Aveson Family, The Trainor Family, Ipirian, TeamLogic IT, Fidelity National Home Warranty, Piatti Ristorante & Bar and All Automotive of San Ramon. Your sponsorship was crucial to the success of the event.

Fabulous auction items and the generosity of all Concert for a Cure attendees and supporters helped to raise a record amount of $156,000 at this year’s event! In total, Concert for a Cure has raised $900,000 over the past 11 years for Families of SMA! Please join us next year on May 12th, 2012 so we can reach our goal of raising $1 Million dollars!
Dear Families of SMA,

I am a teacher at Stonegate Elementary School and my son, Cody Munz, lost his battle with SMA in 2008. This year Stonegate held its fifth annual Once Upon a Cure Fundraiser event on June 3rd, 2011 and donated all of its proceeds to FSMA. It was a day filled with a lot of fun activities. Our theme this year was “Jungle”. Each classroom chose a children’s book with a Jungle theme for their booth activity. The children raised money by getting sponsorships for well-children or those battling for their health in some way. The names of those children were placed on storybook signs around a common area across from our school where the event was held. Students took turns walking to music from a DJ, working in the booth their class provided, and participating in the different activities at the booths.

I am very pleased to mail you these checks totaling $6,284. We couldn’t have done it without all the support of our staff, parents and students!

Sincerely,
Rhonda Munz
Zionsville, IN

Ohio

10th Annual Nicole Nadeau Golf Tournament

On behalf of the Nadeau Family and the OKI Chapter, please find enclosed a check for $52,922, which represents the net proceeds from the 10th Annual Nicole Nadeau Golf Tournament that were not sent directly to FSMA via the online registration or by check. In total, the 10th Annual Nicole Nadeau Golf Tournament raised $17,052 to help find a cure for SMA and a total of $143,000 since the inception of the tournament.

The event was well attended for our 10th year conducting the tournament. This year, 132 golfers and over 150 people in total participated.

Thank you Jack and Kim Nadeau of Springboro, OH for your passionate support of the FSMA mission in honor of your daughter, Nicole!

OKI Walk-n-Roll 2011

The 8th Annual OKI Walk-n-Roll took place on April 30th, 2011 at Xavier University’s Cintas Center. Kevin Lockwood kicked off the walk with an opening speech and introduced Honorary Chairperson, Brad Johansen. Brad moved around the stage introducing and talking with each SMA child and adult on stage. The sun was shining so participants could enjoy the Xavier campus as they walked. Along with the walk, many enjoyed the kid’s carnival, magician, balloon sculpting and raffle. Once again this year there were many amazing items for the raffle. A big thank you to all those who gave of their time and talent to make this event a success! Also thank you to the approximately 500 participants helping us to achieve our goals. The OKI chapter was able to raise $72,251 in total! The OKI 9th Annual Walk-n-Roll is scheduled for May 5th, 2012, so mark your calendars now!

Spaghetti, Music and Auction (SMA) Dinner

The Helfrich family held the Spaghetti, Music and Auction (SMA) dinner on May 14th, 2011 in Akron, OH. They were thrilled to have over 250 people in attendance, and served a delicious spaghetti dinner. The evening also included a wonderful perform-
Grimes Family Raises over $14,000 for Families of SMA, and Counting!

Over the past 12 months, the Grimes Family has been dedicated to hosting and organizing non-stop fundraising events for Families of SMA in memory of their forever nine month old girl, Annie, who passed away from SMA Type I in August, 2010.

On February 27th, 2011, Team Annie participated in the 2011 Disney Princess Half Marathon in Orlando, Florida. “Aunt” Christy Cole Harpring participated in the Half Marathon and finished the 13.1 mile run through Epcot in 2:32:06! Jenny Jones Neville participated in the Beauty & the Beast 5K run and finished the 3.1 mile run in less than 36 minutes! On the day of the event, Team Annie cheerleaders Erin Read, Marci Laborda and Kellie Houchins motivated Christy and Jenny and raised SMA awareness within the Disney Parks! In total, Run 4 Annie raised $3,500 in Annie girl’s memory for Families of SMA!

The 2nd Annual Crop 4 Annie was held on April 17th, 2011 at Greenwood Park Church of Christ in Bowling Green, Kentucky. Twenty eight scrapbookers attended the event and took part in a silent auction which included over sixty items! With the support of Joanna Ballard, Emily Comer, Ashley Hudnal, Kathy Pruett and Shelia Violette, the event was a big success and raised $2,375 for Families of SMA in Annie’s memory.

In Savannah, Georgia on June 9th, 2011, Howard Family Dental and Wilmington Family Dental sponsored A Night at the Ball Park… 4 Annie! For each employee and their family who attended the Savannah Sand Gnats game, a donation of $5 was made. Because of the generosity of Dr. Howard and his staff, $200 was raised at this event!

The most recent Grimes fundraiser, Cocoa’s 4 Annie, was held on June 11th, 2011 at Cocoa’s Dessert and Martini Bar in Savannah, Georgia! The community of Wilmington Island came together to raise money and awareness for SMA in honor of Annie Grimes. They held a silent auction and sold food, beverages, Annie girl cupcakes, coozies, t-shirts and bracelets. Cocoa’s also featured live music and specialty drinks such as “Punch Out SMA” and the “Angel Annie Martini”. In total, $5,271 was raised for Families of SMA in memory of Annie!

The Grimes Family also hosted two fundraising events during SMA Awareness Month. On August 19th, 2011, Ashley Furniture Homestore offered 20% off total purchases to customers who donate $20 to Families of SMA and matched all donations that were made! Thanks to the generosity of family and friends, $2,650 was raised for Families of SMA! SMA Awareness Night at the Ballpark was held on August 27th, 2011. Families were able to watch the Bowling Green Hot Rods take on the Great Lakes Loons and see a post-game fireworks show for only $10. In total, 96 tickets were sold for the game and $288 was raised for Families of SMA in memory of Annie! Throughout SMA Awareness Month, the Grimes Family also sold 4AnnieGirl t-shirts and collected personal donations which totaled $350!

Families of SMA would like to thank the Grimes Family for their dedication to fundraising to help inspire hope that one day there will be a treatment and cure for Spinal Muscular Atrophy. The Grimes Family is an inspiration to all and through their continuous hard work, Annie’s memory will live on forever.
Pacific Northwest Chapter

With the addition of several new families to our chapter, we are rapidly expanding our ability to fundraise and raise awareness. It has been great to meet new families who are committed to finding a cure for SMA. We look forward to bigger and better chapter events with all of our new families. We are in the process of planning quarterly meetings for our chapter and increasing support for our chapter members.

Washington

3rd Annual SMA Day with the Seattle Mariners

In April of 2009, my niece, Gabriella, lost her fight with Spinal Muscular Atrophy at just seven weeks old. It was at that time that I became associated with the Families of SMA Pacific Northwest Chapter. This summer, July 17th, 2011 marked the 3rd Annual SMA Day with the Seattle Mariners. This event serves to raise both funds and awareness while fostering the bond of families, friends and supporters of SMA. We sold a record 141 tickets this year and were able to raise $800 at the event! With our three year veterans, as well as with the families new to our organization, we enjoyed a great day out at the ballgame and the kids even got to run the bases!

Jennifer Stack
Seattle, WA

Madelaine Hargrave’s Birthday Party in Honor of Micah Hargrave

In February of this year, our eight year old daughter, Madelaine Hargrave, picked FSMA as her chosen charity in lieu of gifts for her birthday. Madelaine feels very connected to SMA because her cousin, Micah Hargrave, lives and breathes each day with SMA. Madelaine and Micah are very close and always enjoy and look forward to their visits together.

Madelaine held her party at a local pool here in Anchorage, AK. The children were invited to join us for a swim, cake and treats. If they chose to make a donation to FSMA instead of gift giving it was greatly appreciated, but not expected. Our family was overwhelmed by the generosity of the families who attended the party. Madelaine made a donation of $330 to her cousin’s Pacific Northwest SMA Chapter. Instead of goodie bags, we handed out SMA tattoos and wristbands - the kids LOVED them! It was indeed a very successful party.

As parents, we felt it was important to instill in our children a sense of giving to others. From the time our children are 7 years old, they no longer receive birthday gifts from their friends, but instead choose a charity that they have researched and feel a connection. We strongly urge other families to give this a try; you might be pleasantly surprised at the outcome!

Jen Hargrave
Anchorage, AK

Dear Families of SMA,

My name is Todd McKinlay and I am the Camp Director at Hidden Valley Camp in Granite Falls, Washington. We are a co-ed resident youth camp serving campers ages 7 to 15. Each session during the summer we have a “County Fair” which is a carnival day where we raise a little money. At the end of each fair we ask the campers to come up with nominations of charitable organizations for whom they would like to send the money. We then vote as a camp and this session FSMA was nominated and then won the vote. Enclosed is a check for $60.

Thank you Todd and campers for your support of Families of SMA!

and may differ from current fundraising totals by the time you receive this newsletter.
Although our Chapter has been so energized by the work of our devoted families, unfortunately we have also been reminded recently of the devastating effect of SMA. We write this submission with a heavy heart, as we remember our four Chapter children who lost their courageous battles this past Spring and Summer. We continue to extend our support to the Balakrishnan Family, the Shao Family, the Driscoll/Meredith Family, and the Lee Family.

TOGETHER WE MUST FIND A CURE!

The PA Chapter would also like to acknowledge and thank our Chapter Treasurer, Josephine Tripodi, who has diligently served us for several years. We are so grateful for all of the time you have dedicated, Josephine! We are pleased to announce that Tara Maida agreed to assume this important role as of November 1st, 2011. Thank you and welcome, Tara!

Here is a glance at what our families are doing to fight and cure Spinal Muscular Atrophy:

Chris Cooter, Road Captain of Calvary Riders, and friend of the MacDougall Family, has been mobilizing support to fight SMA! For six years, he has organized the “SMA Motorcycle Ride to Fight SMA.” This year, he added an additional fundraiser through the Steak and Hoagie Factory Stores. Thank you Chris for all you do!

Steak and Hoagie Factory

During March and April of 2011, Chris distributed coupons from the Steak and Hoagie Factory throughout the Philadelphia region. Thanks to Chris, the store donated a portion of proceeds to Families of SMA. This was a great opportunity for SMA awareness!

SMA Motorcycle Ride

On May 14th, 2011, Chris Cooter hosted the 6th Annual Motorcycle Ride to Fight SMA. Over 55 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 $900 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 upcoming information on next year’s event- same time, same place.

Moore College of Art & Design Saves Money for SMA

From April 25th-29th, 2011, students at Moore College of Art & Design donated their spare change for an SMA fundraiser. Cara Scudner, a Resident Assistant at Moore, set up the event in honor of her cousin, Vivianne Heather Jones, who has SMA Type I. The fundraiser was organized and staffed by members of Moore’s ResLife staff. Students dropped change into cups labeled with all the majors at the school and each day a tally was posted with the “winning” major.

The final total raised was $140. Great job! But the big question is…what was the “major of choice?” Thanks to Cara! Go Vivianne!

SMA Fashion Fundraiser

An SMA Fashion Fundraiser was hosted by Dominique Daniela on May 12th, 2011 at the Stephan House, New Hope, PA honoring Jake Saxton and Matthew Reilly.

All proceeds raised from the evening were generously donated to Families of SMA in support of the 8th Annual Walk-n-Roll in Philadelphia. A special thank you to Dominique Santiago of Dominique Daniela, New Hope/New York designer; Dana Faith, NYC jewelry designer; and Godze Yilmaz, Philadelphia t-shirt designer, who generously donated portions of their sales for the evening. Over $2,500 was raised in support of the Walk-n-Roll and a good time was had by all who attended. AMAZING!
Jocelyn Paige Lee Foundation

The Jocelyn Paige Lee Foundation was founded to benefit Jocelyn Paige Lee, who was born on April 22nd, 2007 to Jennifer and Shane Lee, as well as help fund SMA research. At about 6 months old Jocelyn was diagnosed with SMA Type I. Since then, the Foundation has continuously donated to FSMA in Jocelyn’s honor.

Tragically, Jocelyn Paige Lee earned her Angel Wings on August 14th, 2011, just a few months after her joyous 4th Birthday. She was a beautiful, spirited little girl, and will NEVER be forgotten. Our Chapter members were blessed to meet Jocelyn at the JPL Benefits and at the Orlando Conference. We thank her devoted uncle, Derek Schmidt, for all his selfless contributions on behalf of his beloved niece and our Chapter.

Please continue to keep the Family of Jocelyn Paige in your thoughts.

4th Annual Jocelyn Paige Lee Benefit

On Saturday, April 16th, 2011, the JPL Foundation held the 4th Annual Silent Auction Dinner Benefit. It was another huge success! The event was held at St. Elizabeth Anne Seton Roman Catholic Church in Bear, Delaware. The evening was filled with dinner, DJ dancing, and bidding on over 50 silent auction items. Benefit tickets and Raffle tickets (for a 3 day/2 night vacation, donated by Derek Schmidt, Jocelyn’s uncle), were sold before the Benefit by all the Foundation’s members.

It was a great night and Jocelyn seemed to be having a wonderful time. The DJ was fantastic, people were dancing and the Silent Auction was a hit. Big thanks go out to Derek and the Committee for a job well done!

For information on the Jocelyn Paige Lee Golf Tournament, please visit the North Carolina section.

Malvern Blooms Family Festival

“Since Zane’s passing, we are determined to bring awareness to this horrific disease. We, along with our supportive community, will organize fundraisers in Zane’s memory and inform the public about SMA. We don’t want any other families and children to experience our pain.”

— Hillary and Keith Schmid

Malvern, PA came out to fight SMA! On Sunday, May 1st, 2011, the town hosted its Annual Malvern Blooms Family Festival. This year, the Malvern Business Bureau wanted to pair this event with a non-profit charity and the Sweet Baby Zane Team and Families of SMA were selected!

Hillary and Keith Schmid hosted this huge event...a family day with children’s activities, over 100 street vendors and live music. It was a beautiful day! Hillary set up an informational tent to educate the residents about SMA, displaying literature and a Type I car bed. The grand total raised that day was $4,273!
Ava Mae’s Family

“Ava Mae was a blessing to all and continues to impact people’s lives. Her spirit and will to live continues through the two heart valves she donated to two infants upon her passing, and also through our family dedicating ourselves in her memory and honor to find a cure for this terrible disease!”

— Jeff and Shelley Kloiber

Pittsburgh Marathon

Amanda Kloiber, cousin to Ava Mae, participated in the Pittsburgh, PA Marathon on May 15th, 2011 as a fundraiser for FSMA. She chose to run the Race in memory of Ava Mae and asked that donations be made to FSMA on Ava’s behalf. Amanda designed a t-shirt with the “Team Ava Mae” and the FSMA logos displayed. Way to go, Amanda!

Macquarie Group Foundation

Ava’s dad, Jeff Kloiber, Associate Director, Corporate Real Estate, of Macquarie Group, pursued a charitable donation from his company to MATCH all the funds raised by the Kloiber’s for this year’s Walk-n-Roll. After all the hard work undertaken by the Kloiber family, Macquarie Group Foundation generously donated $14,423 . . . for a total of $28,896 raised by Team Ava Mae!

As the philanthropic arm of Macquarie Group, The Macquarie Group Foundation supports a range of community programs in Europe, the Americas, Africa, Asia and Australia. In all of these regions, Macquarie contributes meaningfully to the communities in which company staff live and work. It is so inspiring to see corporate citizenship alive in our communities. Thank you Macquarie Group!

8th Annual PA Chapter Walk-n-Roll

On May 15th, 2011, The Pennsylvania Chapter hosted the 8th Annual Walk-n-Roll to Cure SMA! Over 1,000 people joined our effort to cure this horrendous disease while enjoying games, a petting zoo, raffles, a butterfly release honoring our angels and a leisurely stroll along Boathouse Row in Philadelphia, PA.

Philadelphia Mayor Michael Nutter Proclaimed this day SMA Awareness Day in the City of Philadelphia. The Office of U.S. Senator Robert Casey was represented and issued a Letter of Support for the efforts led by FSMA on the hill. Channel 6 anchor Walter Perez also returned as the chapter’s honorary emcee, inspiring the crowd to keep hope alive as we seek a cure. Along the walk route, signs were placed, displaying photos of our children, as well as sponsor logos and facts about the disease.

Thanks to the generosity of so many people, $147,273 was raised for SMA Research from corporate sponsors and individual donors alike! It was a great day uniting SMA friends near and far. Thank you all!
Two PA Foundations Working Together to Fight SMA

The Lyla Mertz Foundation and Little Flower Violet decided to join forces and hosted two fundraisers together this year! Thank you Jennifer Mertz and Allison Wehrkamp for ALL you do in the name of your precious girls to help other families.

“SMA is a terrible disease and we want all to understand the disease and help us fight for a better future for other children afflicted.”

—Jennifer and Steve Mertz

PA Chapter Walk-n-Roll Retail Tent

Thanks to Jennifer Mertz and Allison Wehrkamp, the PA Chapter Walk-n-Roll offered a retail tent for the first time ever! Both foundations donated all proceeds to FSMA.

The Lyla Mertz Foundation reported sales of 38 “Bags for Lyla,” and 4 “Aprons for Lyla,” for a total of $1665. “Bags for Lyla” currently has 10 styles of bags to choose from, all named after members of Lyla’s care team during her stay at the Lehigh Valley Hospital. They are also happy to introduce “Aprons for Lyla ~ finding a cure for SMA one stitch at a time”. You can view bags or aprons on their website: www.teamlyla.org.

Little Flower Violet sold beautiful hair clips and blankets and also donated their proceeds... $400 to support our efforts! The Little Flower Violet Store is featured on Etsy.com and offers Little Flower Violet Bracelets, T-shirts, and Lullaby Blankies. Please visit them at: www.littleflowerviolet.com.

Summer Fair Fundraiser

The SMA Summer Fun Fair held on June 4th, 2011 was a joint event organized by Jen and Steve Mertz of the Lyla Mertz Foundation and Bill and Allison Wehrkamp of Little Flower Violet. Numerous Chapter families attended this wonderful Lehigh Valley summer fair.

The event featured games, crafts, a Chinese auction and a bake sale. They raised $1,650 in memory of Lyla Mertz and Violet Wehrkamp. A great time was had by all. They hope to make this an annual event that will continue to grow each year.

Mary Kay Fundraiser Hosted by Allison Wehrkamp

“August is National SMA Awareness Month. August also has an extra meaning to us. August 2010 was the first time we heard the words Spinal Muscular Atrophy. SMA didn’t just hit us, it blindsided us.”

—Allison and Bill Wehrkamp

In the spirit of SMA Awareness Month in August, and in memory of Violet Wehrkamp, a Mary Kay Fundraiser was held on August 13th, 2011. Violet is the beloved daughter of Allison and Bill Wehrkamp.

Mary Kay beauty consultant, Jess Corcoran, provided facials and makeovers. Allison and Jess featured fun “mock-tails” and tea sandwiches for all the guests, and many people stopped by throughout the afternoon to be pampered. 20% of the sales from the day were donated to Families of SMA. The total donation from event sales was $200, with an additional $56 in individual donations. Because online sales were still accepted until September 1st, 2011, a final total is still being calculated.

Allison hopes to host a second Mary Kay Party in February, honoring Violet’s 2nd birthday.

and may differ from current fundraising totals by the time you receive this newsletter.
**Pennsylvania Chapter Cont.**

**2nd Annual SMA Awareness Night with the Phillies!**

On June 14th, 2011, the PA Chapter hosted the 2nd Annual SMA Awareness Night at the Philadelphia Phillies Game! Our Chapter families were joined by SMA families from New Jersey, Delaware and New York.

Because over 600 tickets were sold by the PA Chapter, our organization was invited to educate the fans about SMA in a Public Service Announcement on the new Jumbotron! AND . . . 8 year old Jake Saxton, was bestowed the honor of throwing out the first pitch on the Phillies pitcher’s mound!

Jake is an avid Phillies and Flyers fan and made our Chapter proud! Facts about this terrible disease and photos of all of our wonderful children, either attending or being represented at the Game, were recognized in our PSA. Big thanks to Norman Einhorn of Unique Video and Amy Pruitt of Society Hill Studios for ALL THEIR HELP!

A percentage of our ticket sales were donated by the Phillies to FSMA totaling $1,168. What an amazing opportunity to spread SMA Awareness!

**Italian American Club of Canal Walk**

Because of the continued work by grandmother Barbara Perre, an event was held by the Italian American Club of Canal Walk (IACC). The total donation made on behalf of these efforts was $250.

Barbara Perre is devoted to continuing fundraising efforts to fight SMA in honor of her grandson Peter Henkel. Thank you Barbara for all that you do for us!

*Peter Henkel, siblings and cousins skiing*
Emmy’s Crop for SMA
Brandy Baugher and Sandra Cromer hosted the 5th Annual Emmy’s Crop for SMA on March 12th, 2011 at the Old Parkville Fire Company in Hanover, Pennsylvania. Almost 100 women scrapbooked from 9 am to 9 pm while enjoying time with family and friends. Scrapbookers enjoyed great food and great prizes throughout the day. The women participated in free make and take classes, best page contests, silent auctions and live auctions through the event which created awareness for SMA. Participants also enjoyed free mini Zumba classes and massages! With help from friends, family, and crop supporters, this year’s event was able to raise $8,000 to help find a treatment and cure for Spinal Muscular Atrophy. The funds raised are being donated to Families of SMA in memory of Emmy Rose Baugher. Emmy received her angel wings on December 27th, 2006 at six months of age. Over $35,000 has been donated to Families of SMA in memory of beautiful Emmy Rose. With everyone’s help and support, “together we will find a cure.”

Hanover Eagle Riders #1406 Benefit Ride
Hanover Eagle Riders Group held a motorcycle ride to benefit FSMA on May 21st, 2011. In total, the group was able to raise $500 in honor of Emmy Baugher! Thank you for your support of Families of SMA!

Love from “Lukie’s Family”
“A little angel baby graced our lives for a short time, but our hearts forever. His big brown eyes entranced you; his smile melted your heart.” — Joseph and Tara Maida

“Rebels All Stars” Win School Talent Show for SMA!
Kadyne Maida, sister of SMA Angel Lukie Maida, is also a competitive cheerleader on the “Rebels All Stars.” Members of her Junior Squad competed in the 2011 Bangor Middle School Talent Show and won for Best Group Performance! These amazing kids choreographed the routine, mixed their own music, and PRACTICED for weeks.

Their Prize? A $262 donation from Bangor Middle School to Families of SMA, in memory of Lukie Maida! The Team was comprised of: Kadyne Maida, Jeff and Joe Vanderpool, Sammi Ennico, Aaliyah Mitchell, Kaitlyn Sortore, Michaela Hawkins, Tyler Antonoli, Natasha Frey and Sierra McDermott.

These kids were truly AMAZING! We are so proud of them. They are so very dedicated to our Mission . . . finding a Cure for SMA!

Lukie’s Summer Yard Sale
On Saturday, August 13th, 2011, the Maida Family hosted Lukie’s Summer Yard Sale at Washington Pet Shop in Pen Argyl, PA.

It started as an idea by the Maida Family to sell their “treasures” and grew to quite a bit more. They were so excited that this simple idea raised $1,067! They sold many different types of items: clothes, house-wares, books, movies, decorations, etc.

The Maida Family set up a face-painting stand, while also offering hot dogs, snow cones and beverages. The Washington Pet Shop hosted the event by generously offering their prime location as a venue! They donated four raffle items and provided $5 discounted Dog Nail trims. In addition to the Maida Yard Sale proceeds, Washington Pet Shop raised an additional $331 for FSMA . . . for a total of $1,398!

As an SMA Awareness tool, a table was set up at which the Maida Family provided literature and information about SMA. Candles were also lit in honor of the National SMA Candle-lighting that evening. Despite the clouds, the rain held up and they had a wonderful day, and an extremely successful event!

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
Rocky Mountain Chapter

Grandmothers Club

On April 30th, 2011, the grandmothers from the Rocky Mountain Chapter met at Poppies in Denver, Colorado for brunch. This is our second social function and we look forward to many more get-togethers. We enjoy exchanging stories about our grandchildren with Spinal Muscular Atrophy and supporting each other. For more information on the “Grandmothers Group,” please contact Marynell Larson at (970) 667-9655.

8th Annual Rocky Mountain Chapter Charity Golf Tournament

On May 9th, 2011 golfers gathered at Pradera Golf Club in Parker, Colorado on a warm but windy day. The 8th Annual Rocky Mountain Chapter Charity Golf Tournament began bright and early with a putting contest and coffee bar. The tournament got underway with a 10:30am shotgun start and included a betting hole, closest to the pin, longest drive, and longest putt contests. Lewis Padilla, Larry Subia, Ron Chavez and Andy Vigil made up the winning foursome. The tournament raised $17,000 for Families of SMA research and support programs.

Thank you to Gillian and Martin Faith for organizing the tournament again this year! We look forward to seeing everyone out on the links again next year.

Barefoot Books Fundraiser

As former chairperson of one of the annual FSMA-Rocky Mountain Chapter events, I was looking for an opportunity to continue my fundraising efforts towards a cure for a disease that has given us too many angels. As an independent sales ambassador for Barefoot Books, I decided to sponsor an event in which 20% of all orders placed through me by December 15th, 2011 will be donated to Families of SMA.

To view the catalog, please visit www.barefootbooks.com. To guarantee that your purchase is reflected in my donation to FSMA, all orders must be placed through me. You may email me at spellmanfive@msn.com or call me at (303) 918-6586 to place your order. Feel free to email or call with any questions as well.

Thank you for your continued support of FSMA,
Joy Spellman

Tenth Annual Evening of Hope Gala

The Rocky Mountain Chapter held the Tenth Annual Evening of Hope Gala on March 12th, 2011. The evening was held at the Plaza at the Denver Merchandise Mart with over 300 guests in attendance. Dinner, dancing, silent and live auctions were the order of the evening and we were able to exceed our revenue from the previous year raising over $30,000. As important, we were sure to raise awareness for this disease that has given us too many angels.

Mark McIntosh, local sports media celebrity, emceed the evening. Mark took the time to interview our local SMA kids and made them feel like superstars. His ability to incorporate his message and spotlight our families created a special atmosphere that made the evening a success. Local Daddyblue Band rounded out the entertainment; couples danced, kids in wheelchairs rolled and everyone enjoyed the music that was in spirit with the evening!

Heartfelt thanks to everyone who helped make this, and all ten Evening of Hope Galas, enormously important to FSMA and the families it supports. As quoted by this year’s Gala chairperson, “The FSMA logo promotes: Research. Support. Hope. We’ve got the financial backing (finally!) for research. The National Office and the local chapters have done a great job of figuring out how to offer support. But it is the families who have defined hope. Thank you to the Rocky Mountain FSMA members who rarely say “no” to me whether I am asking them to stuff envelopes, seeking out donations or tie ribbons on Boxes of Hope. Most of you are dealing with the day to day struggles of life with an SMA kiddo or facing the grief that comes with having an SMA angel. You all do it with amazing grace and in a way that honors every single SMA warrior.”

Planning for the 2012 Evening of Hope is already under way...look for your invitation to join the most special evening in the Rocky Mountain region!

Thanks to event chair Joy Spellman and the 2011 Gala Committee, the Rocky Mountain Chapter raised over $30,000 for Families of SMA. Reaching the 10th anniversary of an event is a huge accomplishment. Congratulations to the Rocky Mountain Chapter, and thank you for your continued support over the years!
Jacob Isaac Rappoport Foundation

The eighth annual Jacob’s Run, Walk & Roll to Cure SMA, hosted by the Jacob Isaac Rappoport Foundation, was our best walk yet! Attended by more than 600 people, the event raised $102,373!

The event was a spectacular day. After a one mile fun run/walk and lunch, families enjoyed arts and crafts, pony rides, clowns, bounce houses, a rock wall, petting zoo, music and a live basketball show! The day could not have been the success that it was without the help of our generous sponsors, tireless volunteers, and persistent fundraisers! Thank You!

Our Shooting Star Sponsors included the Rooms To Go Children’s Fund and Best Buy.

This year’s event was even more special because many SMA families from our South Florida FSMA Chapter participated through fundraising and in the event itself. Special thanks to our top three fundraisers: Rachel & Joshua Altchek, Corinne & Matt Painter, In Memory of Chloe and Lisa & Lewis Birnbaum.

Jacob’s Run, Walk & Roll to Cure SMA is always a true celebration of life and love, in memory of our shooting star, Jacob. And while we know nothing can bring him back—we do know that we will continue to fight in his memory, and in memory of all the other shooting stars we have met along our journey—until a cure is found!

Families of SMA would like to extend our sincere thanks to Shaina and Adi Rappoport and their family, friends and supporters, for putting on yet another successful walk in 2011. Your hard work and determination shines through year after year, and we cannot thank you enough!
**South Florida Chapter**

**5th Grade Essay Contest**

Rylee Kilmann participated in a 5th grade class assignment to write a persuasive essay about a non-profit organization. Rylee decided to write about Madison Smith and how SMA impacts her daily life. Rylee was chosen as one of 18 finalists, and then won first place in the fifth grade essay competition! Rylee will be donating her $1,000 prize to Families of SMA!

**South Jersey/Delaware Chapter**

In May, the Potter family hosted their 6th Annual Steven’s Walk to Drum Out SMA and raised an extraordinary amount again—$25,000! On June 3rd, 2011, to honor our son Steven’s 10th birthday, we held Zumba Away SMA Zumbathon and raised over $6,000! Thank you to my friends, Taira and Sheri, and my mom, Joan Smith, and sister, Justine Nichols, who helped make this event successful!

On Saturday, July 30th, 2011, my father-in-law Steve Moyer hosted the SMA Golf Tournament in memory of Steven Moyer in Mountain Top, PA. We had a beautiful day and raised over $2,700. Thanks, Dad!

Jessica Moyer, South Jersey/Delaware Chapter President

**Upcoming Events:**

- **Friday, April 27th, 2012:** The Moyer Family will be hosting the 5th Annual Steven’s Swing for a Cure Golf Classic. To participate or sponsor the event, please visit [www.fsma.org/stevenswingforacure2011](http://www.fsma.org/stevenswingforacure2011).

- **Sunday, May 13th, 2012:** For the South Jersey/Delaware Chapter’s 10th Anniversary, we will be participating in the 2012 Delaware Marathon. To run with Team Families of SMA, please visit [www.fsma.org/delawaremarathon](http://www.fsma.org/delawaremarathon).

I am currently looking for a Co-President in Delaware or North Jersey to help me with the Chapter. For more information, please e-mail southjersey@fsma.org or call Jessica Moyer at (866) 774-9533.

**New Jersey**

Dear Families of SMA,

On behalf of Hill Wallack LLP’s Staff Relations Committee, I am enclosing a donation in the amount of $170. Every month members of our staff are permitted to wear jeans to work for charity “Denim Days.” An employee who makes a $5 contribution to the Charity of the Month may wear jeans on the 1st Friday of the month. On August 5th, 2011, the Staff Relations Committee raised $170 for Families of SMA. We are hoping that our donation will help your endeavor.

Very truly yours,

Hill Wallack LLP

On Friday, February 4th, 2011, the Lanes Mill Sunshine Club held a Denim Day Fundraiser. In total, the group was able to raise $50! Thank you for your support of Families of SMA!

**4th Annual Cure SMA Walk-n-Roll in Honor of Katherine Santiago**

The 4th Annual Cure SMA Walk-n-Roll in Honor of Katherine Santiago was held on May 15th, 2011 in Millburn, New Jersey. Friends, family and other members of the community joined together to walk, raise money and remember a little girl who made such an impact on all of their lives. Katherine Santiago was a kind, nine-year old girl who loved crafts, and the color purple. Although she suffered from SMA, Katherine never thought of herself or was considered handicapped. Six years ago, Katherine passed away, but her spirit still lives on in all her friends and family who honored her at the walk. In total, the event raised $7,058 for Families of SMA! Thank you to the event sponsors, attendees and Allie and Randi Mazzella for organizing the event!
Southern California Chapter

Baby Joseph Remembered in Annual Lemonade for Life Event

Mikayla Jean’s brother Joseph lost his battle with Spinal Muscular Atrophy when he was only 8 months old in August 2003. Every year in August, Mikayla and her family have a Lemonade Stand to raise money for research and to help other families with this disease. All donations received are given to Families of Spinal Muscular Atrophy.

This year’s event celebrated seven years of remembrance since Baby Joseph went to heaven. Neighbors, business owners and friends came by to donate funds and share in the cause. The day provides healing for a mother who has lost her son and a sister who has lost her beloved baby brother. It gives hope to families facing similar circumstances and brings together a community. In total, the event raised over $1,500 for Families of SMA.

Kim Donnelly
Highland, CA

Dear Families of SMA,

Please accept this donation of $2,500 in memory of our son Thomas (Tommy) Hart Pence. This money was collected through the generosity of friends, family and my co-workers at Vista Murrieta High School. We are grateful for the help that your organization has given us and hope that someday in our lifetime we will see a cure for this heartbreaking disease.

Sincerely,
Carrie and Evan Pence
Oceanside, CA

Dear Families of SMA,

I am enclosing several checks totaling $140 as donations in Memory of Tommy Pence. His parents were Evan and Carrie Pence. We are all members of Court Appointed Special Advocates of Ventura County Council. His Great Aunt, Jane Pence, is one of our Council members.

Thank you,
Marcia M. Bhattacharya
Camarillo, CA

Dear Families of SMA,

On April 16th, 2011, we had our Annual M&M Memorial Softball SMASH Tournament in memory of our children, Makayla Josette Torres and Michael Joseph Torres. We raised $895. We also had a small Scentsy Fundraiser that helped us raise $55. Two donations were given to us the day of our tournament from Kenneth and Jennifer Waldrop and the Gavilan College Physician Assistant Program.

We are sending a total of $1,030 in memory of our children. It’s not much, but we hope it helps at least with something!

Sincerely,
Joe and Dora Torres
Dos Palos, CA

Tennessee Chapter

Tennessee Chapter Charity Golf Tournament

The Tennessee Chapter of FSM A held their 7th Annual SMA Charity Golf Classic this year on April 24th, 2011. The tournament was held in Knoxville, TN and raised $17,518 this year for Families of SMA! Thanks to all of our amazing participants and volunteers who make it all possible.

Jellico High School Duck Race

The Jellico High School Student Council held its first annual FSM A Duck Race. Jellico High is a small school located in northeast Tennessee. In honor of present junior member, Casey Douglas, the student council decided to adopt FSM A 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. With t-shirts and bracelets, the council generated a lot of interest and questions. The council members sold approximately 300 duck race chances and 100 t-shirts, raising $2,000 for FSM A. After being flooded out on April 16th, 2011, the race was rescheduled for the 23rd. Much was learned from this first race and we are looking forward to a much larger and more organized race next year, hopefully with a little less water.
Dear Families of SMA,

We had our 4th Annual Cure SMA: Run-Walk-N-Roll honoring Seth Gilley (SMA Type II) on May 7th, 2011 at the Stephenville City Park. It was a big success, raising $12,259 for Families of SMA. It was a beautiful day enjoyed by over 350 people. Following the two mile fun run/walk, we had live music performed by a local Christian band, 100-Watt Witness. The children enjoyed visiting with Sparky and our local fire department, face painting, popcorn, bounce houses and a water slide. New this year, our local Tarleton State University therapeutic horseback riding program (T.R.E.A.T.) provided a petting zoo and horseback rides. The silent auction was very successful and even more special this year, because Debby Ryan (AKA “Bailey” on The Suite Life on Deck) donated autographed pictures, DVDs and clothing worn on the show. Debby is a cousin to Seth, and during a special phone call from her in April this year, she said she wanted to help our cause by sending some items for the auction. She was so sweet, and we are so thankful for her support and kindness! The event ended with a hamburger lunch provided by donations from local businesses and cookies from Seth’s church family, Graham St. Church of Christ.

It was a wonderful 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, Texas

Candle Lighting for Rudy

Today is a special day for us. Today, August 16th, 2011, our son, Rudy Colunga, would have been one year old. We decided to hold this event in order to raise awareness of SMA, to honor all of the SMA families, and to honor our son. We are honored to have the opportunity to raise awareness of SMA and the effects it has on children and their families. FSMA helped us so much in our time of grief that we want to do whatever we can to help out. This past year has been very hard on us, but we are ready to start spreading awareness on a higher level. We plan on doing this every year and maybe hold other events in our community. We had a nice turnout and collected $120 overall. Our good friend, Amanda Davis with InFocus Portraits, was able to come by and take pictures of the event.

Rudy and Amanda Colunga
Tomball, TX

Dear FSMA,

We (the family of Matthew Wallis) pray daily that the money we raise will help find a cure for Matthew. Thank you for working hard to find a cure. As always, this donation of $128 was raised by the patients of Dr. Mark Wallis. Their donations as they check out of Dr. Wallis’ office are much appreciated and very generous. Please continue your efforts, we will too!

Sincerely, The Wallis Family, Longview, TX
Utah Chapter

When our little girl died, I thought my heart would shatter. It felt like a natural law had been broken—why was the parent here and the child gone? As I struggled to make sense of our tragedy, it seemed like I was only allowed a moment of grief. Everyone is sympathetic. Everyone is kind. Then life rolls on. Back to work, back to school, back to the crazy pace. One foot in front of another. But even as you move along with the pace of life, you know you’ll never really go back to the way things were.

It was the pace of life though, that inspired an idea. I wanted to do something to pay tribute to our daughter—to acknowledge her courage, her patience, her tenacity, and her inspiring example. What better way for me to do that, than to require that same tenacity from myself? I could take putting one foot in front of another, and turn it into something meaningful. I decided to run a marathon.

Now, I don’t run. I’m not an avid exerciser. I sit in a cockpit all day and eat in airport food courts. I really wasn’t sure if I could do this, but Saydria’s death taught me that I could survive lots of things I wasn’t sure I could. So, I set the goal and started training.

FSMA helped me to set up a gift registry page. I wanted to raise $2,000 to help support other families the way we had been supported. Our friends and family were amazing. They are not wealthy people and these are not good economic times, but dollar by dollar, with love and encouragement, they helped me surpass my goal and raise $2,006 in memory of Savaria Paulean Reeves.

My in-laws arrived the night before the race with a package. Inside was a t-shirt with the words, “I Run For My Angel” across the chest, and below a photo of my sweet little girl. The rest of the box contained shirts for the extended family with Saydria’s picture and a caption reading, “For Families of Angels”. Now, Saydria could run the race with me, right next to my heart.

In April 2011, one year after her diagnosis, I ran the Salt Lake City marathon—my first and last. I wanted this to be something I would only ever do for her—a celebration of her difficult, but poignant journey through life. And as I crossed the finish line, I felt her with me and I knew that she will always be with me, every step of the way.

Casey Reeves, Riverton, UT

Western New York Chapter

8th Annual Western New York WALK for a Cure

The Western New York Chapter held its 8th Annual WALK for a Cure on August 6th, 2011 at Beaver Island State Park. The weather forecast was threatening but, as always, the day was beautiful. The families and friends of FSMA came together and we raised over $65,000.

We had the biggest basket raffle with over 100 baskets and gift cards. Large raffle items included two bikes, a signed Patrick Kane hockey shirt, three professional basketball shirts, signed Jim Kelly football, iPod, a gym membership and fitness plan with Bonnie and a beautiful Corning glass vase. The raffle, along with the 50/50 brought in $5,000.

Special thanks goes to all the teams that contributed to help make this a special day: Jake’s Buddies, Blair Team, Oscar-Go, Joey’s Team, Kale’s Krew, JJ’s Journey, Team RJW, the Grieg’s, Sutkers/Shermans and the Weiser’s.

Nicholas Picholas, from KISS 98.5, once again did a great job moving the crowd and keeping everything on time. He introduced co-chairs Mayor Robert Ort and Senator George Maziarz. Mayor Ort proclaimed August as SMA Awareness Month and August 16th, 2011 as SMA Day. August 16th is Kale Shiesley’s 10th birthday and the day was set aside in his honor due to the efforts of his good friend Bailey Walter.

Bailey Walter, Kale’s friend since 1st grade, now in the 4th grade, has made it her mission to help find a cure. She collected in her neighborhood, placed donation jars in several business establishments, sent 200 letters to friends and relatives about the Walk-n-Roll and convinced the mayor of North Tonawanda to proclaim August SMA Awareness month. The mayor also had a dress-down day. In total, Bailey raised $58 for Families of SMA in honor of Kale! Thank you for all of your hard work!

Hideaway Restaurant Fundraiser

Also, during August, the Hideaway Restaurant donated part of the proceeds earned in one week to FSMA. The employees wore Walk shirts and donation jars were set up. Thank you to Lori May and all of your employees.

Wine Tasting Fundraiser a Golf Fore SMA Event

On March 5th, 2011 Jill Rebeor hosted a Wine Tasting Fundraiser in New York and raised $313 for Families of SMA! A few weeks later on May 21st, 2011, Jill also held the 2nd Annual Charity Golf Tournament: Golf Fore SMA at Battle Island Golf Course in Fulton, New York. Thanks to the support of family and friends, the event raised $4,181 to help find a treatment and a cure for Spinal Muscular Atrophy! Thanks for your hard work, Jill!

JL Automotive Fundraiser

On March 5th, 2011, JL Automotive raised money and SMA Awareness through holding a donation jar fundraiser! In total, JL Automotive was able to raise $75 for Families of SMA thanks to the generosity of their employees! Thank you for your support!
Chapter Updates

Wisconsin Chapter

Going the Distance for Those Who Can’t

On May 21st, 2011, 22 boys from Cub Scout Pack 579 in Greenfield, Wisconsin gathered in the Greenfield High School gymnasium to raise money for Families of SMA in honor of Peyton Lola Elsner. The event, Going the Distance for Those Who Can’t, was a 50-yard Dash-a-Thon. The boys took time out of their non-stop schedules to obtain pledges to support their efforts. They then gathered bright and early on a Saturday morning to make those supporters proud. Each boy ran 50-yard-dashes and together completed an unbelievable 2,360 dashes! We even had to reduce the amount of time running to prevent exhaustion as the boys would not give up and ran with amazing determination! They touched all of our hearts. Their determination shone through as they earned an amazing $2,000 for Families of SMA.

Kristi Biloff—who organized the Dash-a-Thon—said, “My only goal in organizing an event like this was for the boys to realize that they are lucky and privileged to be able to run, walk, crawl or jump. Many children take those abilities for granted, and I know there are many children in the world that can’t do these things. Our kids should know that they have an ability to be proud of and to use for greater causes! These boys are my inspiration to continue this event and continue to help families affected by SMA!”

Thank you to Kristi, all of the Cub Scouts from Pack 579 and everyone who supported these amazing boys in their fundraising efforts.

Par 4 Peyton

On May 13th, 2011, the 3rd Annual Par 4 Peyton Golf Tournament was held in Greenfield, WI. On behalf of Peyton Elsner and all other children affected with SMA, 175 kind-hearted friends came together to raise funds to find a cure. The day was a success and all the golfers enjoyed their time on the course. The fun continued back at The Muskego Lake Clubhouse with dinner and music. The Boots rock band sang a song that they wrote for Peyton and all others with SMA! There were over 125 raffle items, in addition to both silent and live auctions, so many people left with some great items. We are so thankful for everyone that supports SMA and our efforts to find a cure. In total, the event raised $1,000 for Families of SMA!

Alex Reddick, Peyton’s cousin, also held a can drive, raising over $130. He presented the check at this year’s golf outing and is looking forward to continuing his drive throughout the year.

Laura and Marlen Weisenberger of Rothschild, WI held a fundraiser through Scentsy and raised $425 in honor of their daughter, Tessa (Type I, age two.)

North Crawford Bake Sale

This past spring the second grade class at North Crawford School in Soldier’s Grove, Wisconsin taught by Mrs. Julie Kruizenga and Mrs. Lisa Green held an annual two day bake sale as part of their math curriculum. Students sold baked goods throughout the lunch hour to all K-12 students and staff. A cake raffle was also held for staff. The students chose two charities to split the earnings- Families of SMA and their local food pantry. In total, $284 was donated to FSMA in honor of Easton King (SMA Type II) whose mom is a 7-12 science teacher at North Crawford School and Kaleb Fortney (SMA Type III) whose uncle is a 6-12 business education teacher there as well.

Dear Families of SMA,

Each year our church family dedicates part of our outreach efforts towards causes/issues that directly affect congregational members. This year we nominated our grandson, Braden Patrick McCarthy, and Families of SMA as one of the recipients and we were awarded the enclosed check, which we happily present to you.

We were honored when one of the girls who has been a sitter for Braden did a lot of research on her own about SMA and as a result she convinced the two local high school girls teams to make SMA their cause this year. They raised more than $1,000!

I imagine that I am no different than any of the thousands of grandparents who are hoping and praying that a cure will be found for SMA before we leave this earth. We are grateful for all that has been done so far. We know that without the work that has gone before, our precious grandson would not be here. Instead, he is making great strides in his power wheel chair. We look forward to learning more in Florida at conference.

Sincerely, Richard H. Carlson
New Berlin, WI

Ben Nelson, my son, celebrated his 5th birthday on March 28th, 2011! In lieu of gifts, we asked that his birthday party guests consider a donation to FSMA. Enclosed please find two checks given in response to that request totaling $120. Thank you, and keep up the great work.

Very truly yours, Eric Nelson
Menomonee Falls, WI

Campbellsport Junior High Student Council Penny Wars

The Campbellsport Junior High School Student Council in Campbellsport, Wisconsin did something that they have never done before—they gave money to Families of SMA. In early April, the student council needed a charity/organization to donate money to from a Penny War they were going to hold. One of their fellow students, Lindsay Muench, has SMA Type II and was in their student council. She suggested donating the money to Families of SMA and they thought that was a great idea! The Penny War for the Families of SMA was put together right away. At first, the penny war for the 7th and 8th grade didn’t raise as much money as they thought it would. In the end, money poured in and the 7th and 8th grade raised over $400! Everyone on the student council was super excited and was very glad that the money went to help a person they knew and cared for.

Please Note: The amounts raised and shown on the following pages are totals as of August 26, 2011,
Promotional Materials from Families of SMA
Make Planning a Fundraiser Easy and Fun

New Fundraising Materials:
- Manuals and Sample Booklet (Walk-n-Roll, Golf Tournament, Dinner/Gala & Bowl-a-Thon)
- Banners and other colorful signage
- Families of SMA “At a Glance” flyer
- Families of SMA “Results & Progress” flyer
- New FSMA Bracelets
- Donation tags
- Plastic event bags
- Donation Cards
- Promotional Tips
  - Balloons
  - Purple & Orange Golf Tees
  - Temporary Tattoos
  - Fridge Magnets
  - Coin Canisters
  - FSMA Pens
  - Angel Tags
  - And more!

Event Website Updates

and may differ from current fundraising totals by the time you receive this newsletter.
New Event Website Upgrades

Quick and Easy Way to Invite Donors

Families of SMA has recently added a feature to our event websites which allows you to upload e-mail contacts directly from your address book (Gmail, Outlook, Yahoo!, and a number of other programs are supported). Thanks to this brand new feature, it takes just a few minutes to share your story with all of your friends and family when fundraising with a Gift Registry Page, or for an event on our events Calendar.

Use Social Media to Share Your Page

In the top right corner of your personal fundraising page, click on these buttons to post a link to your page on Facebook, Twitter or other social networking sites. This is another fast, easy way to share your story with friends and family members, and to ask them to support your efforts to raise money for a cure for SMA.

Also New This Year:

- Gifts made to an event or to a personal fundraising page can now be made in honor or memory of a loved one
- An easy drop-down list is available as a search method to find the teams registered for an event
- Event scrolling boxes now list both names associated with a donation, when applicable
- Donors can mark their contributions as “Anonymous” and remain anonymous on all event pages and personal fundraising pages throughout the website
- Participants are able to post their offline cash and check donations to appear online in their personal, team and event totals, to showcase their fundraising success

Merchandise

Car Magnet | $5

Orange and Purple Ribbon Lapel Pin | $5

FSMA Rubber Bracelet | $2

“Together we will find a cure” T-shirt | $12
Youth sizes: XS S M L • Adult sizes: M L XL XXL

To view a complete list of Families of SMA Merchandise visit www.fsma.org/Fundraising/Merchandise

Merchandise can be ordered online at www.curesma.org or by calling the FSMA National Office at 1-800-886-1762
Dear Families of SMA,

On Monday, February 8th, 2011, the Sayreville War Memorial High School Boys and Girls Swim Teams held a collaborative Swim-a-Thon to raise money for your foundation. Please accept the following $1,403 donation on behalf of Carly Hewitt, our number one fan, in memory of her father, Ed Hewitt.

Sincerely,
Sarah Rodis Magaw, Varsity Swim Coach, Parlin, NJ

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Dear Families of SMA,

Enclosed you will find a little donation from Addie and Molly’s summer “Lemonade Stand for SMA” in memory of their brother, Benjamin Piper. $25 – they were so excited! Thanks for all that you do!

Love,
Megan Piper & Family,
Pittsburgh, PA

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Riley Mercer elected to forego birthday presents for herself this year and asked for a charitable donation instead. Riley raised enough money to buy Tambryn Campell an iPad. Check out the letter she sent to her friends!!

Dear Friends:

As you know, each of the last few years I have asked for a charitable donation instead of gifts on my birthday. This year will be no different, and in conjunction with my church.

The reason this is so important to me is that my Aunt Courtney (my mom’s BFF) lost her two children, Wayland and Piper, to SMA when they were infants. SMA is a genetic disease that attacks nerve cells, called motor neurons, in your spinal cord. These neurons communicate with your voluntary muscles - the ones you can control, like in your arms and legs. As you lose the neurons, your muscles weaken. This can affect walking, crawling, breathing, swallowing and head and neck control.

There are many types of SMA. What you are able to do depends on the type you have and how it affects your breathing. There is no cure. Medicines and physical therapy help treat symptoms.

Since losing Wayland and Piper, Aunt Courtney, who used to be a teacher, has worked with other kids with SMA. She and others have found that the iPad makes it easier for kids who can’t control their muscles to participate in school and do things we take for granted, like turning the page of a book. An iPad will help Tambryn, who is as smart as you and me, read books and signal her teacher when she needs help.

I hope you will consider making a donation, instead of buying me a gift. The donations will be combined with a special collection at St. Luke’s with the hopes of paying for the iPad in full.

Thank you for your consideration, and thank you for being my friends.

Love, Riley Mercer of Plano, TX

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I am Who I am and Nothing will Change that

By: Lauren Gibbs of Roeland Park, Kansas, 14 Years Old, SMA Type III

When they got the news, they thought it was doom
As fate was not in that small doctor’s room.
And though they knew that it was going to be a long run,
Somehow they knew that it was all going to be fun.

For some reason this thing had a way to bring people as one
It may be hard, happy, or even heartbreaking, but that’s life and there’s no other way than to live through it.

If SMA was not in my life, I would be different.
Some people might think that I would be indifferent.
But for me, I would not be me.
Fact to be, SMA is in my family tree.

I know that I’m not normal,
But is normal what you would want to be?
Having SMA makes me, me.

SMA not only brings people together, but it forms a family.
A family that no matter what hardships we go through, we all have one thing in common.
One thing that could change a life forever.

I am who I am and nothing will change that.
My granddaughter, Audrey, was diagnosed with Type II Spinal Muscular Atrophy at the age of eleven months. In 1994, we didn’t know what her future would hold. Unable to walk, she has been in a power wheelchair for sixteen years, but when God takes something away, he always gives you something else. Audrey has been blessed with a good mind, a kind heart, and an angelic voice.

Today, at the age of eighteen years she weighs approximately fifty pounds and requires aid for daily physical care. Her mother, my daughter, has been her primary caregiver all along. Yet even in the face of enormous adversity, in a few short weeks, Audrey will graduate from high school, has been named Valedictorian of Wekiva High School’s Class of 2011, and has been accepted to the University of Miami where she intends to major in neurobiology and research for SMA and other muscular diseases. Audrey has worked diligently, excelling in academics and challenging herself with high level curriculum. She also works tirelessly on community service and school sponsored projects, some of which bring awareness to this little known disease affecting and challenging her daily life. Just like her classmates and other typical high school students, Audrey has interests outside of academics too. She joined her school bowling team and served as captain for two years. She participates in weekend fundraisers, car washes and is active in her church.

Through church, Audrey has discovered her love for music and song. Participating in church choir since second grade, Audrey has developed a passion for choral singing and earned a seat in the Florida All State Chorus five consecutive years. She plans to continue singing in a church nearby the University of Miami campus. Interestingly, we believe there is a direct correlation between her reduction of hospitalizations for pneumonia and her lungs having strengthened from singing.

Audrey has set her goals very high and continues to awe and amaze her family and friends. Like so many graduating high school students, she is ready to move onto the college campus and begin the next stage of her life. She has been named recipient of The George W. Jenkins Scholarship awarded to students who show strong academic credentials, financial need, and a history of overcoming adversity. Audrey is still in need of funding to pay for a caregiver, as up until now, her primary caregiver has been her mother. She will continue to pursue her goals with quiet determination, hope and her beautiful smile. I thank you once again for understanding the need for continued support of research to discover a cure for this genetic disease. As the very proud grandmother of an eighteen year old victim I pray daily for the help Families of Spinal Muscular Atrophy so desperately needs.

Vina Springer of Apopka, Florida

Kale Shiesley of North Tonawanda, New York was asked to participate in the first baseball game for the opening of Miracle League of Western New York, which is a handicap accessible baseball playing field. Past and present members of the Buffalo Sabres attended the opening day celebration, joining other special needs athletes in a one-on-one baseball game.

Kale played second baseman with his official Sabres baseball cap turned backwards and chewing gum like all baseball players do. He caught a line drive for an out and up at bat hit a home run, bringing the other bases in to score! Kale was just thrilled with excitement and so was his family and friends who were watching and cheering him on.

It was a magical day not only for Kale, but for all the other athletes who participated too. Many parents had to wipe away tears of joy, but everyone out there was beaming with pride!

It was an awesome day.
Sofie Santana

Tie Dye Guys—Austin and Jeff Olander

Seth and Caroline Gilley

The Johnson Family with the Governor of Oklahoma

Tambryn and Braelyn Campbell

Xamara Trevino

Sofie Santana

Steven Potter

Colin Ohmstead

Trevor Rickerson

Anthony Asselin
Spinal Muscular Atrophy, words I had never heard before, literally brought me to my knees. Our first child, Spencer, was diagnosed at eleven months old, after months of fear. Waiting, watching, willing him to bear weight on his legs, to roll over, to get to sitting. The milestones all parents watched for would not be celebrated. We were told Spencer would never walk, never stand, never play as other children do, and he would most likely succumb to respiratory illness very early in life. The grief, the heartache were unbearable.

I remember so vividly, grieving in the quiet dark of our house for days. Holding Spencer, praying that this diagnosis was wrong. Praying that our lives would go on as other families’ lives did. I prayed that God would give me a miracle.

Gradually, I understood that I had to face our reality, the nightmare so many parents fear. Leaving our house, standing on the front porch, I found the world was not as I had left it. God’s grace totally encapsulated me. I could feel the love, the prayers lifted for us. God was tangible. He surrounded me. This was the first time that I knew my little family was not alone. We were being held in the palm of God’s hand, and in the hearts of a very prayerful and loving community. And I knew we would be alright.

Twenty three years later, I know my life; my family’s lives have all been touched by this event. And what I had so vehemently prayed would go away, was God’s plan for us. Once we accepted that, we could go on. I remember my husband saying, “We will just have to make Spencer the best person we can, despite this disease.” With that focus in mind, I was able to move forward. I have been able to accept God’s plan for our lives, the easy and the difficult. What I have found is that God’s plan for me has been richer than I ever could have hoped to dream - more than I knew to pray for.

Trevor was born four and a half years after Spencer. He is tall and strong, strong enough to lift his brother time and time again. He is gracious enough to kneel at his brother’s feet to put on Spencer’s shoes. He studies, he cooks, he wins photography awards, he climbs mountains. Somehow he knows, at such a young age, that life is precious and every minute must be used, as my husband always says, for adding value.

Spencer has graduated from The University of Texas’ School of Architecture and is working for Miro Rivera Architects in Austin. Spencer and Trevor live together. They take care of each other. This is an arrangement, I could not have conceived of. If I had thought of it, I would have been praying for years. God’s plans have been greater than my own imagination.

Soon after Spencer’s birth, I learned the magazine I had been working for had gone bankrupt. Obviously a “bad” thing to happen at this time in our lives, but this event enabled me to move from advertising into education. Something I had never considered until this time. Consequently, I have been given the opportunity to work with teachers, children, and families who are dealing with disability. My own experiences have made me compassionate and empathetic, but also a strong advocate for children. This was not a path I knew to follow. I still take a new step each day in the direction God has chosen for me.

I know without a doubt, that my marriage is stronger; my relationships are deeper and more honest given the events in my life. I know Spencer is one of the strongest people that I know; he goes forward, touching the lives of those around him, expecting to encounter the world despite his disability. I know Trevor is a compassionate man - gentle, patient and driven to take every opportunity available to him, while adding value to the world. How much my husband and I have learned together, I can not begin to share the enormity of it.

By Kimberly Cook of Corpus Christi, Texas
Happy 4th Birthday Stella Turnbull!

Chloe Ochoa with Sam Schoenborn

Cooper Somers

Colin Lee Ohmstead

Journee Weiberg

Karoline Chowaniec

Logan Staples

John Rossmiller

Connor DeLuca

Garrett Schultz

Jadon Burks

Chase Langland

Hanna Eide and Lily McNair

Lucas Braga Vaz

Christian DuVair

Alexa Helfrich

Happy 4th Birthday Stella Turnbull!
I’M SO LUCKY

As she sleeps I stand in awe.
Her face so perfect, without a flaw.
Does she dream of dancing and jumping rope?
Does she know my heart is full of hope
That someday she will walk and play,
Skip and run and take ballet.
I smile because deep down I know
It matters not what fate did sow
I see a girl unlike no other.
And I’m so lucky to be her mother.
I hate to think of who I’d be
If God had not chosen me
To be the one to guide her way
And bear witness each passing day
To a soul so pure and full of love
Sent to me from heaven above.
I smile because I can see
The immense responsibility
Of caring for God’s special pearl
And I’m so lucky that she’s my girl.
The man who passes on the street
Averts his eyes so they don’t meet
A child different from the rest
He fails to see how very blessed
Our lives became when she was born
How we rejoice, and never mourn.
She smiles at him and then says “hi”
He doesn’t look as we pass by
What he and many fail to see
Is I am so lucky that she is with me.
Pity me not for you don’t see.
The endless love she brings to me.
While you may see her less than whole
I see a child pure of soul
So open your eyes or you will miss
Seeing firsthand that angels exist.
I smile as I watch her sleep
I feel myself start to weep
I don’t know when or where or how
I just know how lucky I am right now.

Written for Veronica Rose
by her lucky mom Debby Crosswhite St Onge
SHARING photos

Kale and Kyler Shiesley
Peter Henkel
Rebecca Gordon
Lucy Butler

Nataly
Nathan Cooper
Omar Hardy
Scarlett Landefeld
Stella Turnbull

Madison Smith with Darrell Gwynn
Maxwell Peppers
Stella Turnbull with mom, Sarah
Savannah Norton

Maxwell Peppers
Tambryn Campbell
Mia Fuentes
PJ Desroches
FSMA publications

Family Guide to Research
This guide helps to answer questions such as what the key areas of SMA research are and what the costs for conducting SMA drug development and clinical trials are. Clear definitions and graphics are included to help explain and illustrate how SMA drugs begin and the key steps involved in developing new therapies for Spinal Muscular Atrophy.

Nutrition Basics
This new booklet is focused on the basics of good nutrition for children with Spinal Muscular Atrophy. The booklet was authored by several SMA nutrition experts.

Breathing Basics
This new booklet is focused on the critical aspects of respiratory care for children with Spinal Muscular Atrophy. The booklet was authored by Mary Schroth, M.D., a member of the Families of SMA Medical Advisory Council, and a leading expert on respiratory care for SMA patients.

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

The Genetics of Spinal Muscular Atrophy
Confused about genes, proteins, DNA and how SMA is diagnosed? Read this helpful pamphlet. It includes definitions, explanations and diagrams from genetics expert Louise Simard, Ph.D. and the FSMA Medical Advisory Council.

Caring Choices
This booklet is focused on caring choices for parents of infants newly diagnosed with Spinal Muscular Atrophy Type I. And, where you can go for support and guidance.

New Care Series Publications Coming Soon!
The Families of SMA Medical Advisory Council (MAC) is busy working on a new publication for SMA families and professionals called Understanding SMA, Adult Onset SMA and Musculoskeletal Care for SMA. Check out the FSMA website for further updates.
uniting families with researchers to find a treatment and cure for SMA

Researchers
Visit the site to learn how the registry can help connect you to the people and data your research needs.

Participants
Visit the site for more information or to learn how you can join the registry.

This project is supported by the Patient Advisory Group of the International Coordinating Committee for SMA Clinical Trials, which includes Families of SMA, FightSMA, the Muscular Dystrophy Association, the Spinal Muscular Atrophy Foundation, and other SMA advocacy groups.

For more information call 1-866-482-0248
e-mail smareg@iupui.edu https://smaregistry.iu.edu
Today is a Great Day

By: Kathleen Weber Piazza

As I sit and write this entry, there are a few things I need to say immediately:

- Today...is Nicholas’ 1st day of college!
- Today...is proof that dreams do come true!
- Today...is a miracle in the works!
- Today...is the hardest, scariest, most anxiety ridden day that I have known in a long time!

My heart feels like it is going to beat right out of my body...doesn’t anyone around me see it? Can’t you hear it?

Sending your child to college is an emotional time in any parent’s life. Imagine sending your medically fragile, wheelchair bound, totally dependent child who “they” all said would be “dead before the age of 2” (“they” being the doctors who first diagnosed him, I believe it was a “world renown” Dr.) Imagine the stress I am going through. I really don’t think you can unless you are a parent of a child with SMA. We SMA parents are a unique breed. We never asked to join this club, yet we are dedicated to it 100%. We can create, build and adapt anything so that our special child can have the most normal life experience ever!

As I sit here in SUNY New Paltz, Wooster Science Building (we actually found a great little lounge area complete with a huge rock coffee table; geology is what Nick’s major is and he currently is in a geology lab class), I am overwhelmed by the fact that my son is exactly where he wants to be. He is living his dream! amazing! I can barely write as the tears flood my eyes and stream down my face. I could never have imagined when we were first diagnosed 17 years ago that this is where we would be today!

I’m trying to blend into the walls so that Nick feels his accomplishment without me hovering, but let me tell you, it is very difficult! I have always been the one caring for him, sensing what he needs, I think sometimes before he even does. Now he is a young man, “walking” through the halls of college. I thank his nurse for attending with him, I know he is in good hands, but still I worry, wait, I pray. As an SMA parent I think we worry more than any other parent on this planet! It sometimes becomes unbearable, all the worrying, a feeling like nothing is possible. But today, everything is possible! Today, is a great day!