



DIRECTIONS

FAMILIES OF SPINAL MUSCULAR ATROPHY



Families of SMA
Support. Research. Hope.

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The Jacob Isaac Rappoport Foundation has provided funding to send over 611 Type I Care Packages to Newly Diagnosed Families.

Families of SMA currently sends about 170 Type I Care Packages each year to newly diagnosed families. Many ideas for the items in the Type Care Packages came from SMA parents themselves, such as Shaina and Adi Rappoport. Shaina states, "I used to spend hours searching for toys that were light enough for Jacob to hold. After he died, I spent even more hours searching for similar toys for other SMA affected babies." With the help of this funding from the Jacob Isaac Rappoport Foundation, Families of SMA has been able to add many helpful items for newly diagnosed Type I families. Each Type I Care Package is valued at well over \$200.



These care packages are funded by the Jacob Isaac Rappoport Foundation, in memory of their Shooting Star, Jacob, who has SMA Type I. Jacob was born December 27, 2001 and was an alert and happy baby. When he was nearly four months old, Jacob was diagnosed with Spinal Muscular Atrophy. Soon after Jacob's diagnosis, Adi and Shaina quickly connected with Families of SMA and many other SMA families.

Shaina and Adi created the Jacob Isaac Rappoport Foundation after they lost Jacob. The foundation funds SMA research and programs that support affected families. In 10 years, they have raised over \$1 million; sent dinners, cleaning services and package of toys to hundreds of SMA families; provided scholarships for families to attend SMA conferences; hosted luncheons for affected families; and have spent countless hours lending emotional support to parents of newly diagnosed children.

"Through Jacob, we have learned that the human spirit is truly amazing. We know that nothing can bring him back to us, but we do know that by reaching out to other SMA families and by raising funds and awareness we can make a difference. We continue to be proud of our son, as he touches more lives every day with his courageous story. Thank you for touching our lives by being a part of his legacy."

To learn more about the Jacob Isaac Rappoport Foundation, please visit www.ourshootingstar.com.

Families of SMA would like to extend a heartfelt thank you to Shaina and Adi Rappoport and the Jacob Isaac Rappoport Foundation for funding these wonderful care packages for newly diagnosed Type I families.

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

Submissions

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

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

Change of address

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

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

On the Cover: Danielle Sheypuk

Mission Statement



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

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

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

Pfizer Licenses Families of Spinal Muscular Atrophy Quinazoline Drug Program from Repligen

Repligen Corporation announced on January 3, 2012 that it has entered into an exclusive worldwide licensing agreement with Pfizer Inc. to advance Repligen's Spinal Muscular Atrophy (SMA) program, originally licensed from Families of SMA (FSMA).

Families of SMA funded and directed the preclinical development of RG3039 with an investment of more than \$13 million. This was the first drug discovery program ever conducted specifically for SMA. Repligen licensed RG3039 in 2009 from Families of SMA.

"We believe this collaboration with Pfizer, a leading pharmaceutical company with specialized efforts in orphan and genetic diseases, has the potential to accelerate the development of therapies for SMA," said Walter C. Herlihy, Ph.D., President and Chief Executive Officer of Repligen.

"There is a critical need to expedite potential treatment solutions for rare diseases such as Spinal Muscular Atrophy, where patients have such limited options," said Jose Carlos Gutierrez-Ramos, Senior Vice President, Pfizer BioTherapeutics R&D. *"This partnership will combine our expert capabilities in advancing molecules for genetic diseases with Repligen's leading SMA program."*

"This licensing deal demonstrates the innovative collaborations that Families of SMA has successfully implemented between non-profit, biotech and big pharma," stated Jill Jarecki, Ph.D., Research Director for Families of SMA. *"These partnerships are critical for the development of new treatments for an orphan disease such as SMA. We are extremely pleased to see Pfizer taking the lead on the development and commercialization of the SMA program, following Repligen's development work and FSMA's original investment."*

RepliGen



FSMA began the Quinazoline/RG3039 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. The direction from FSMA provided the positive results necessary to license the program to Repligen and now Pfizer to leverage resources and expertise for clinical development.

Through FSMA's leadership and research funding of over \$13 million, a drug candidate has been created that treats the underlying cause of SMA. In preclinical studies, the drug has been shown to efficiently cross the blood brain barrier, a critical feature for a neurological drug, and prolong survival significantly in two different mouse models of SMA.

The FSMA research model is to fund early stage drug discovery programs for SMA and then partner with companies for later stages and accelerated clinical development. At the earliest stages of drug development, programs have less than a 1% chance of success. This inherent risk along with small patient populations has traditionally hindered industry from working on orphan diseases.

This new license agreement with Pfizer marks a significant advance for the SMA community by securing the commitment of one of the worlds largest pharmaceutical companies to develop potential treatments for the disease.

The SMA program includes RG3039, a small molecule drug candidate in clinical development for SMA, as well as backup compounds and enabling technologies. Under the terms of the agreement, Repligen is responsible for completing the first two cohorts of an active Phase I trial evaluating RG3039 in healthy volunteers, which it anticipates will occur during the first quarter of 2013. Repligen will also provide certain technology transfer services to Pfizer who will then assume full responsibility for the SMA program moving forward, including the conduct of any registration trials necessary for product approval. Repligen has previously received U.S. Orphan Drug and Fast Track designations for RG3039 for the treatment of SMA, as well as Orphan Medicinal Product designation in the EU.

Timeline of Events:

- **1990's:** The back-up gene in SMA, called SMN2, was discovered as a therapeutic target for SMA with funding from FSMA.
- **2000:** Families of SMA began the Quinazoline project with Aurora Bioscience/Vertex Pharmaceuticals by performing drug screens for SMA.
- **2003:** Families of SMA starts works with deCODE to optimize drug candidates and conduct pre-clinical development of a new drug for the treatment of SMA starting from the lead compounds discovered at Aurora/Vertex.

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Pfizer Licenses Families of Spinal Muscular Atrophy Quinazoline Drug Program from Repligen

- **August 2009:** Families of SMA received FDA Orphan Drug Designation for the clinical drug candidate in this program, for the treatment of SMA. The first time a new therapy specifically designed for SMA has ever reached the important stage of being awarded orphan drug status by the FDA.
- **October 2009:** Families of SMA licensed this series of compounds to Repligen Corporation for industrial scale investment and expertise for clinical development.
- **May 2011:** Repligen Corporation received approval from the FDA to begin human safety trials on the Quinazoline drug, now called RG3039. The first ever clinical trial approved for SMA with a novel drug specifically designed to treat the disease.
- **September 2012:** Repligen Corporation initiated a Phase 1b clinical study of RG3039 to further evaluate the safety and plasma pharmacokinetics of multiple doses of RG3039 in healthy volunteers.
- **January 2013:** Pfizer licenses the Families of SMA RG3039/Quinazoline program from Repligen.



Pfizer Inc.

Pfizer's Orphan and Genetic Disease Research Unit is adopting an innovative and collaborative approach to the development of new medicines whereby it looks to develop strategic partnerships with academic and commercial enterprises to create novel therapeutics across the spectrum of rare diseases.

www.pfizer.com

RepliGen

Repligen Corporation

Repligen Corporation is a life sciences company focused on the development, production and commercialization of high-value consumable products used in the process of manufacturing biological drugs.

www.repligen.com

For the latest news visit the FSMA website:



www.curesma.org

Families of SMA Committed \$2.5 Million to Funding SMA Research in 2012

Families of SMA is dedicated to creating a treatment and cure for Spinal Muscular Atrophy by funding and advancing a comprehensive research program, which includes:

- Basic Research to reveal the best ways of making SMA drugs
- Drug Discovery to make practical new drugs and
- Clinical Trial resources to help test new drugs

Families of SMA committed new funding to the following SMA research projects during 2012:

\$710,000 for Basic Research, announced in December 2012:

- \$140,000 to Christine DiDonato, Ph.D. at Northwestern University
- \$140,000 to Charlotte Sumner, M.D. at Johns Hopkins University School of Medicine
- \$75,000 to Yong-Chao Ma, Ph.D. at Northwestern University
- \$75,000 to Chien-Ping Ko, Ph.D. at University of Southern California
- \$140,000 to Jocelyn Côté, Ph.D. at University of Ottawa
- \$140,000 to Sara Custer, Ph.D. at Indiana University

\$1,450,000 for Drug Discovery, announced in May 2012:

- \$750,000 to Brian Kaspar, Ph.D. at Nationwide Children's Hospital to advance a CNS delivered gene therapy for SMA
- \$700,000 to Peter Schultz, Ph.D. at CALIBR for a novel SMA drug program to optimize small molecule SMN enhancers

\$110,000 for Clinical Trial Resources, announced throughout 2012:

- \$45,000 to The SMA Patient Registry to aid in clinical trial recruitment
- \$38,000 to Stephen J. Kolb, M.D. at Ohio State University for patient recruitment during the NINDS NeuroNext Biomarker Trial
- \$10,000 to Parent Project MD for a collaborative study exploring expectations in clinical trials
- \$17,000 to a Collaborative SMA Community Project to optimize clinical trial outcome scales

\$200,000 for the 16th SMA Research Group Meeting, held in June 2012:

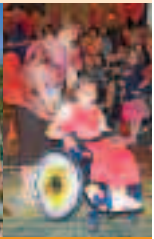
- \$200,000 for the largest meeting worldwide bringing over 225 scientists together to share new SMA research results and initiate new collaborations

Thank you to the amazing FSMA chapters and families who supported this work and progress towards a treatment and cure! We now have 3 clinical trials testing new SMA therapies, and an additional 10 programs in earlier stages of the drug development pipeline. Our research approach funds programs at early stages, and then partners with companies to take them through clinical trials. Supporting multiple programs gives different approaches for a SMA therapy, which increases the chances of success and accelerates the timeline to a treatment and cure.

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

2013 ANNUAL SMA CONFERENCE

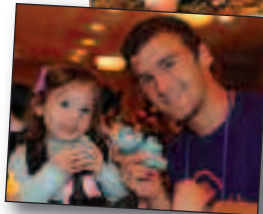
Every year, Families of SMA sponsors a conference to bring together the leading SMA researchers, clinicians, and families living with SMA. Families of SMA has been hosting the Annual SMA Conference since 1989. The weekend is filled with a wide variety of workshops, a memorable children's program, a family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



Let the Adventure, Fantasy and Fun Begin

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

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



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

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

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

SMA Conference Goals

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

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

**Families of SMA**
Support. Research. Hope.

2013 ANNUAL SMA CONFERENCE ANAHEIM, CALIFORNIA



Special Conference Theme Park Tickets

Come for the conference, and stay for the magic! Make the most of your free time with special Disney Meeting and Convention Theme Park tickets. Annual SMA Conference attendees are eligible for advance purchase of specially priced discounted Disney Meeting/Convention Theme Park tickets.

Newly Diagnosed Conference Program

Thanks to the generous funding provided by the Erin Trainor Memorial Fund, Families of SMA covers the registration fees for the Annual SMA Conference for all families newly diagnosed since the last conference. The mission of the Erin Trainor Memorial Fund (ETMF) is to generate substantial funds to be able to provide conference scholarships, allowing newly diagnosed SMA families/individuals the opportunity to attend the Annual SMA Conference. Our goal is to allow as many newly diagnosed SMA families as possible 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, learn directly from experienced SMA physicians, and network with other families. This program is automatically offered to all newly diagnosed families that contact Families of SMA.

For more information, please email familysupport@fsma.org



Continuing Medical Education Conference

Families of SMA offers a third component of Care for medical professionals at the Annual SMA Conference. A Continuing Medical Education Conference is held prior to the start of the Annual SMA Conference and Researcher Conference. The CME Conference, titled "Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role", for medical professionals, will be held on Wednesday, June 12th at the Disneyland Hotel. The conferences have previously covered two components of SMA; Research and Support. The Research aspect covered scientific, research & clinical updates, while the Support aspect included the family support workshops and family networking. This third conference provides an exciting component of Care to the Families of SMA Conferences by educating medical professionals on SMA. Families of SMA partners with an accredited hospital who can provide continuing medical education credits for medical professionals, and offers a full-day of lectures and educational materials to all attendees.

Exhibitor Opportunities

The Annual SMA Conference is a wonderful opportunity to promote your company or products to SMA families, medical professionals and researchers from all over the world. By hosting an exhibitor table or booth, you will be able to personally meet with many families and medical professionals, as well as be prominently displayed throughout the conference and online.

For more information, please email exhibitor@fsma.org.



Please visit www.fsma.org for more conference information.



Contact us

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To register for the Annual SMA Conference or the Continuing Medical Education Conference, please visit www.fsma.org.

Families of Spinal Muscular Atrophy Announces the Schedule for The 2013 Annual SMA Conference

Preliminary details and schedule have now been announced for the incredibly magical 2013 Annual SMA Conference held June 13th – June 16th in Anaheim, CA at the Disneyland Resort.

The schedule includes the timeline of workshops and events, which entail:

- An incredible 38 workshops.
- 6 possible events for families, such as an amazing Family Fun Fest / Carnival, a PJ Party & Movie Night.
- A new Researcher Poster Session for Families.
- Events combined with everyone's favorite Disney Characters!

At this year's conference we are launching a new Researcher Poster Session specifically for SMA families where they can speak directly with the researchers to learn the most up to date SMA research information. We are adding this new event to our Annual Conference, due to requests from both SMA families and SMA researchers for more one-on-one interaction with one another.

We are thrilled to also offer Annual SMA Conference attendees a special Disney event on Friday evening, which will include a unique group viewing of a Disneyland fireworks show!

The Continuing Medical Education Conference, led by the Families of SMA Medical Advisory Council, will start the conference off by training up to 200 medical professionals on care for SMA patients. This truly is an incredible opportunity for medical providers from across the US to be educated by the FSMA Medical Advisory Council as well as other top SMA experts. We hope that this will heighten SMA Awareness and SMA Education throughout the United States.

The weekend will also be filled with networking opportunities with other families, a memorable kids program, and opportunities to interact with families and get first hand updates from some of the 225 SMA researchers in attendance. We are looking forward to reuniting as a community at this conference and showing our support for others. The Family and the Research conferences will again be held simultaneously with one another.

Registration for the conference will be open through May 9th, when it will close, and there will be no onsite registration available. All registered attendees must wear a name tag and wristband, which is provided at the conference, throughout the weekend for access to any conference events, workshops, children's program and meals. You must complete your conference registration with Families of SMA prior to reserving your room at the Disneyland Hotel for the Annual SMA Conference special room rate of \$144 per night. This special room rate is available to SMA conference attendees from Friday, June 7th through Thursday, June 20th!

\$5 Rebate in Disney Dollars

Families of SMA is thrilled to offer 2013 Annual SMA Conference Attendees a \$5 rebate of Disney Dollars for each room night at the Disneyland Hotel and the Paradise Pier Hotel in the Annual SMA Conference attendee room block! This \$5 per night rebate of Disney Dollars can be used at the Disneyland Hotel as well as the Disney Theme Parks and the Downtown Disney restaurants and merchants. For example, if a conference attendee stays at the Disneyland Hotel or Paradise Pier Hotel, in the Annual SMA Conference attendee room block for 4 nights, they would receive \$20 in Disney Dollars! The Disney Dollars will be provided to 2013 Annual SMA Conference attendees at the Families of SMA registration area when attendees pickup their conference name tags and folders.

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

Agenda

Thursday, June 13th

- 7:30am – 5:30pm Registration Open for all Conference Attendees
 12:00pm – 4:00pm Newly Diagnosed Program
 (For Newly Diagnosed Families Only)
 5:30pm – 8:30pm Meet and Greet for all conference attendees/Family Fun Fest



Friday, June 14th

- 8:00am – 3:30pm Registration Open
 7:30am – 5:00pm Exhibitors
 7:30am – 8:45am Continental Breakfast
 8:45am – 10:15am General Session
 10:30am – 12:30pm Workshop Session #1
 12:30pm – 2:00pm Lunch Break – on your own
 2:00pm – 3:30pm Workshop Session # 2
 7:00pm – 8:30pm Researcher Poster Session for Families and Reception
 8:45pm Escort to Disneyland Park to enjoy a special group viewing of the fireworks show

Saturday, June 15th

- 7:00am – 4:45pm Registration Open
 7:00am – 5:15pm Exhibitors
 7:30am – 9:00am Continental Breakfast
 9:00am – 10:30am General Session – It's a Wonderful Life Panel
 10:45am – 12:15pm Workshop Session # 3
 12:15pm – 1:45pm Lunch Break – on your own
 1:45pm – 3:15pm Workshop Session # 4
 3:30pm – 4:45pm Workshop Session # 5
 6:15pm – 9:00pm PJ Party and Family Movie Night Event
 6:15pm – 9:00pm Adults with SMA Social



Sunday, June 16th

- 7:30am – 9:00am Breakfast Buffet
 9:15am – 11:30am Closing General Session/Researcher Q & A

Food Items Included for all registered attendees:

- 3 breakfasts: Friday, Saturday and Sunday mornings
 Dinner Buffet on Thursday evening
 Appetizers at Reception Friday evening
 Popcorn and Candy served at Movie Night, Saturday evening

Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role



Wednesday, June 12, 2013
The Disneyland Hotel
Anaheim, California



Jointly sponsored by:

University of Wisconsin School of Medicine
and Public Health
Office of Continuing Professional
Development in Medicine and Public Health
Department of Pediatrics
and Families of SMA



FSMA Offers a Medical Professional Conference at the 2013 Annual SMA Conference in Anaheim CA

Families of SMA will hold a Continuing Medical Education Conference for Medical Professionals for the second year in a row on Wednesday, June 12th, 2013, prior to the start of the 2013 Annual SMA Conference and the 2013 International SMA Research Group Meeting in Anaheim, California. The Continuing Medical Education Conference is titled “*Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role*”.

This CME Conference is a great step Families of SMA is taking toward broadening our support programs by educating medical providers and professionals. The Annual Conferences previously covered two components of SMA, Research and Support. The Research aspect covered scientific, research and clinical updates, while the Support aspect included the family support workshops and family networking.

The new addition provides a third component of Care to the Families of SMA Conferences by educating medical providers on SMA. Families of SMA is thrilled to be partnering up with University of Wisconsin School of Medicine and Public Health, who are accredited to provide continuing medical education credit for medical professionals. FSMA is also teaming up with surrounding local hospitals in California to further promote this event.

Families of SMA is providing this program in the hopes that it will help medical professionals:

- Identify when to refer children with delayed motor milestones for further evaluation
- How to apply best practices to the coordinated care of individuals with Spinal Muscular Atrophy
- Understand and promote care coordination between the community and specialty care for children with Spinal Muscular Atrophy



The CME Conference will be a one day event for medical professionals only. The course will focus on the diagnosis of infants and children with neuromuscular weakness, clinical application of care standards to individuals with Spinal Muscular Atrophy, and discussion of pathophysiology and therapeutic strategies for intervention. Members of the Families of SMA Medical Advisory Council, as well as Guest Speakers from Institutions across the US, will provide didactic presentations and host panel discussions.

The target audience is medical professionals such as: pediatric neurologists, pediatric rehabilitation medicine specialists, pediatric orthopedic surgeons, pediatricians, physical and occupational therapists, speech and language pathologists, nurse practitioners, nurses, physician assistants, nutritionists, social workers, respiratory therapists, residents, fellows and other medical providers.

Registration

Registration for the CME Conference, held on Wednesday, June 12, 2013 at the Disneyland Hotel in Anaheim, CA, is available on-line only.

Please visit <http://www.fsma.org/FSMACommunity/Conference/ContinuingMedicalEducationConference/> for more information about the CME Conference.

Families of Spinal Muscular Atrophy Introduces New Family and Researcher Event to The 2013 Annual SMA Conference



Families of SMA is offering a special session on Friday evening for families and researchers to interact, called The Family and Researcher Poster Session and Reception held at The Annual SMA Conference in Anaheim, CA. This Family and Researcher Poster Session and Reception is an exciting new addition to this year's conference as it will provide families and researchers a wonderful opportunity to connect and learn from each other.

Families of SMA has invited some of the researchers, who are also attending the Researcher Conference which runs simultaneously with the Family Conference, to create a family friendly poster to display for this Friday evening event. During this time, families are encouraged to rotate around to each of the different posters, asking any questions and learning directly from the researchers involved in that specific research project. The evening will also entail a light reception and some activities for the children to participate in.

Some of the 22 SMA researchers and institutions presenting posters include:

Androphy Lab - Indiana University
 Rossoll Lab – Emory University
 Kothary Lab – University of Ottawa
 Rubin Lab – Harvard University
 Schultz Group - CALIBR
 Ko Lab – University of Southern California
 Ma Lab – Northwestern University
 Cote Lab – University of Ottawa
 Isis
 Pfizer
 Kaspar Lab – Nationwide Children's Hospital
 DiDonato Lab – Northwestern University
 PTC / Roche
 CA Stem Cell
 Kolb Lab- Ohio State University
 Trophos
 Genzyme
 Cytokinetics
 Monani Lab- Columbia University
 Lorson Lab - University of Missouri
 We will provide more details about the special Friday Evening Event closer to conference.

For more information about the Annual SMA Conference, please go to the following link:
<http://www.fsma.org/FSMACommunity/Conference/>

The Spinal Muscular Atrophy Community Grieves the Loss of Families of SMA Former Board Member and Community Leader Amy Marquez



Everyone at Families of SMA sends our condolences to the family of Amy Marquez. Amy, who was diagnosed at the age of three months with SMA, was an inspiration to countless families worldwide.

She accomplished so much in her 41 years of life. Amy was diagnosed with SMA Type I at three months of age, along with five other siblings who also had SMA. Despite being homebound until the 6th grade, Amy went on to pursue her education and earned a B.A. Degree in Psychology and Criminal Justice. After completing a year of graduate studies in Gerontology, she opted to focus her energies on volunteer efforts and improving various aspects of life for those living with disabilities.

Employed as a co-advisor for disabled students at Daley College until 1997, Amy was also an AOL internet tutor until 2006 and a public advocate for Service Dog Teams after being paired with her first companion in 1995. Over time, Amy worked in a variety of professions, including accounting and consumer credit services. She married Steve Marquez in 1998, and gave birth to their children, Danielle, in 1999, and Harley, in 2004. Although being a wife and mother is a never-ending job, Amy had been actively involved with Families of SMA since 2001, as a member of the Families of SMA Board, a speaker at Annual SMA Conferences, a former officer for the Illinois FSMA Chapter, and founder of the FSMA Mentoring Program for Teens and Adults with SMA.

Our deepest condolences go out to Amy's husband Steve and their two daughters Danielle and Harley.

Families of SMA is gathering helpful hints and tips when it comes to managing activities of daily living with SMA. We hope to publish these in each issue of the Directions Newsletter and also in a possible Care Series Booklet.

■ **Tempur-Pedic Pillow** – This was great for Jack when he was relaxing at home. It provides a soft surface for him to lie on, and has rounded edges that helped keep him in a certain position. It also helped with ease of moving him without disturbing him.

■ **Junior Neck Pillow** – Since most children with SMA cannot hold their bodies in a midline position, they often need support to feel secure. We used a junior neck pillow (looks like an adult airplane pillow) to keep Jack's joints in line. While Jack was sleeping on his side, we placed the pillow between his legs and had each end supporting his stomach and back. A swaddling blanket works for positioning comfort as well.



■ **Activity Gym with Arm Slings**- Jack enjoyed sitting in his Boppy or lying on his tummy and batting at toys with aide of an arm sling. Arm slings give children mobility by supporting their muscles so that they can access their toys.

Sarah Bonelli, Arizona

Families of Spinal Muscular Atrophy Receives Generous Funding from The Charles Rosenbaum Foundation to purchase EasyS Medical Strollers for the FSMA Equipment Pool

Families of Spinal Muscular Atrophy is thrilled to receive generous funding from The Charles Rosenbaum Foundation to purchase medically necessary EasyS Strollers for SMA children. These 24 strollers, which retail for \$3,300 each, were purchased specifically for the FSMA Equipment Pool and will provide many families with a vital piece of equipment for their child.

Families of SMA would like to extend a tremendous thank you to The Charles Rosenbaum Foundation for creating the \$50,000 challenge for the Northern California Walk-n-Roll and for allowing FSMA to provide support to so many families across the United States. Families of SMA would also like to thank the Northern California Walk-n-Roll for meeting the \$50,000 challenge, created by The Charles Rosenbaum Foundation, which was used to purchase the EasyS Medical Strollers. This incredible donation will make such an impact on the lives of so many SMA families.

EasyS medical strollers are incredibly beneficial for children with SMA, as it allows them to be transported comfortably and safely while receiving adequate support and positioning for their bodies. Many SMA families have recommended this specific medical stroller as it is the only stroller on the market that lies completely flat. This feature can be very important for children with SMA Type I who may not be able to be on an incline. It also has larger vent tray options which allows families to transport some of the large pieces of medical equipment that they may require while out.

Medical strollers are often covered by insurance, but it usually takes families time to get them pre-approved through their insurance company. Unfortunately, most SMA families cannot wait the months that it could take before insurance approval.



Because of this, Families of SMA receives many requests for this specific stroller. With this generous funding from the Charles Rosenbaum Foundation, Families of SMA will be able to provide many SMA families with a safe stroller option while they wait

to get pre-approval for one of their own.

Families of SMA would also like to thank Thomashilfen, the EasyS stroller manufacturer for giving us a generous discount which has allowed us to purchase almost double the amount of strollers.

The Families of SMA equipment pool consists of many used items that have been donated by other SMA families. The equipment pool also consists of new items that have been purchased with the financial support of others. All items are made available to Families of SMA members free of charge.

For information on the Families of SMA Equipment Pool, please email Equipment@fsma.org.



Funding Received from Sweet Baby Zane and Hillary & Keith Schmid Has Reached Amazing Levels Enabling More SMA Families to Receive Necessary Equipment

Families of SMA is thrilled to receive additional generous funding from Keith & Hillary Schmid and Sweet Baby Zane to purchase additional E-Z-On Vests and Car Beds for SMA families through the FSMA Equipment Pool. These E-Z-On Vests and car beds are vital to the well being of SMA Type I infants and children, who may experience possible apnea and oxygen desaturation if they are placed in an infant or toddler car seat. These vests and car beds enable an infant with SMA to be positioned in the prone or laying down position, safely and comfortably in the car. A smaller and younger infant with SMA can use the Cosco Car Bed, which fits babies up to 26 inches long. When an infant is between 26 and 31 inches long, they can use the Hope Car Bed, which retails at \$1,000 each. Once a child outgrows the larger Hope Car Bed, one option is to transport them with the E-Z-On Vest. Thanks to Sweet Baby Zane's generous funding, the Families of SMA Equipment Pool has these car beds and vests readily available. The incredible grand total raised by Sweet Baby Zane's Fund for Families of SMA has been nearly \$80,000!

With this support provided from Sweet Baby Zane, Families of SMA is able to offer every newly diagnosed SMA Type I family one of these Car Beds. Now, with the addition of the E-Z-On Vests, we are able to offer families another option once their child outgrows the Hope Car Beds. We are thrilled to say that over 100 families have now received a Hope Car Bed directly from the funding of Sweet Baby Zane!

With this funding, so far Families of SMA has been able to purchase:

- 61 Hope Car Beds
- 40 Cosco Dream Ride Car Beds
- 45 E-Z-On Vests



The Hope Car Beds have now been sent to 101 newly diagnosed SMA families.

The Cosco Car Beds have now been sent to 34 newly diagnosed SMA families.

The E-Z-On Modified Laying Down Vests have now been sent to 14 SMA families.

In addition to these important pieces of equipment for the FSMA Equipment Pool, Families of SMA has also received funding from Sweet Baby Zane to send computer CD's filled with information on SMA to newly diagnosed SMA



families who live outside of the US. There have now been 181 CD's of information sent to newly diagnosed families internationally with funding from Sweet Baby Zane.

Funds raised by Sweet Baby Zane and the Schmid Family that are currently available will also now enable Families of SMA to purchase an additional 14 Hope Car Beds, 10 Cosco Dream Rider Car Beds and 30 E-Z-On Vests!

Since the passing of their daughter Zane, Keith and Hillary have been dedicated to bringing awareness to SMA. Along with their supportive community, Keith and Hillary formed Sweet Baby Zane and organized fundraisers in Zane's memory. Sweet Baby Zane raises its funds for Family Support programs at Families of SMA, such as purchasing important equipment for the FSMA Equipment pool. Thanks to the funding from Sweet Baby Zane, Families of SMA is able to offer every newly diagnosed Type I family that contacts Families of SMA a Car Bed.

Thank you Hillary, Keith, Avery, Zane, Brennen and Braxton for allowing Families of SMA to provide support to so many newly diagnosed SMA families and for making such an impact on the lives of so many SMA families worldwide!



Families of SMA is so grateful to have received handmade blankets from Caitlin Trainor for our Newly Diagnosed Care Package Program! On November 29, 2012, Caitlin and her classmate held an event on campus at Providence University, to make blankets for newly diagnosed children and to raise awareness for SMA. They made 16 blankets and raised money for SMA. This event was done as a part of her public and community service class. They had to organize an event to gather students in a common cause. She did this in conjunction with Res Life for an activity for students. She showed a SMA video at the event, attracted the students with a raffle and gift card drawing and attracted over 50 students. She even went on the radio station on campus to advertise the event. It created a great awareness on campus and she hopes to do another event next year and build on the awareness created this year! These hand made blankets will be sent to newly diagnosed families when they first contact FSMA, in memory of Caitlin's sister, Erin. Thank you Caitlin and everyone that helped make these blankets for our care packages and your incredible support!



Families of SMA would like to thank our wonderful volunteers from the University of Illinois at Chicago's National Residence Hall Honorary - Eternal Flames Chapter who assembled over 400 Newly Diagnosed Family Info Packs at a service event! Thanks to their hard work, this supply will last the National Office six months! We are so grateful for our volunteers whose support is truly making a difference in the SMA Community!



A special delivery from Carol Bixler was sent to the FSMA National Office full of beautiful handmade blankets for the FSMA Care Package Program in honor of her great-grandson Luke Bertsch. Luke loves snuggling with his blanket so Carol decided to make these in his honor and hopes that others might enjoy them as well! Thank you Carol for sharing your beautiful work with others!

The FSMA National Office had a wonderful visitor recently stop by! Brittany Street delivered some special hand picked items to include in a Type I care package for a newly diagnosed family. We are so grateful for this thoughtful and generous donation, which was made in memory of her sister, Megan Street. Thank you Brittany for making such an impact on the life of another SMA family!



Three new Dolphin Bath Chairs were donated from the Joseph Lillo Spinal Muscular Atrophy Foundation for Children for the FSMA Equipment Pool! These bath chairs will be sent to SMA families in memory of Joseph Dominic Lillo. Thank you to the Joseph Lillo Spinal Muscular Atrophy Foundation for these wonderful additions to the Equipment Pool and for their incredible support!



Families of SMA is so grateful to have just received a special delivery of Solar-Powered Dancing Santas, Snowmen & Penguins donated from the Helfrich Family! These Dancing Santas, Snowmen & Penguins were sent to SMA families in honor of their children Zander and Alexis. Thank you to the Helfrich Family for this wonderful donation!



Steve, Jennifer and Madison Losacco delivered special lightweight heart toys which were handmade by Jennifer's mom and aunt and many loving hands, to Families of SMA. These special items will be put into the Type I Care Packages, in memory of their daughter, McKenzie Losacco! Thank you Steve, Jennifer and Madison for dropping off these beautiful new toys!



Families of SMA is so thankful to have received a basket full of bubbles for our Newly Diagnosed Care Package Program! These fun bubbles will be sent to all newly diagnosed families when they first contact FSMA in memory of Tala Albakri. A special thank you to Tala's aunt and uncle Manel and Mutaz Salah and her parents Ghassan and Nisreen Albakri for donating these bubbles to our care packages!

Dear FSMA,
In celebration of the 175th anniversary of the organization of The Relief Society, the women's organization of the Church of Jesus Christ of Latter-day Saints (The Mormons) the women of the Santa Barbara area (called a "stake") gathered to participate in service to the community. These knitted caps were one of the projects in which some of the women participated. Our church is divided into geographic congregation, and each congregation has it's own Relief Society Group. Barbi Milne, who has a granddaughter, Blakeley Lewis, who has SMA, leads one of these groups. Barbi told us about your organization and your need for items such as these caps and we are thrilled to be able to contribute these knitted caps to your group to be used as needed. We probably will have more in the future as there are ladies who continue to make these caps as a hobby. Thank you for your service to those in need, and we appreciate the opportunity to contribute to such a worthy organization.



With Regards,
LeeAnn Olson
President, Santa Barbara Stake Relief Society



Dear FSMA,

Kalen loved his care package! The pictures tell the true story :) He was dancing with excitement! We can't thank you enough!

Love,
Kalen Kulas & Family of New York

Hi FSMA,

I just want to let you know that I received the information packet and care package yesterday. I am overwhelmed by the generosity and thoughtfulness of SMA members and volunteers that were involved in this program.

Again, I would like to extend a heartfelt thank you to the SMA community.

Take care.
Maricel Sin of California

Hello Families of SMA!

Good day! I am Maria Florencia Lizarraga. I wanted to thank you wholeheartedly for the CD with all of the useful information on SMA I got at home. I never imagined that would come from as far as it did and is important to us.

Thank you very much for everything and for your time!

Goodbye!
Florencia of Argentina



Friends,

Our packet came today and I am amazingly overwhelmed by all of the awesome gifts! Thank you! Her therapists were telling me to find an old-school telephone toy and a bubble machine; guess what! Yep, in the box! I couldn't wait to call them and tell them all about it. They were also thrilled! "Thank you" just doesn't seem sufficient. You have blessed our family in such an amazing way! Thank you!

Blessings,
Kathy, Ariel and the rest of the Barker Bunch of South Carolina

The FSMA Family,

Thanks for sending us the new SMA Care Series Booklet. Morgan say's "hello" to all of you.

Knowing that FSMA is there for us and all the other families makes me so thankful.

Love,
The Steward Family of Georgia

Hi Families of SMA!

We received the package and car bed- you guys are amazing! We couldn't believe how much you sent! Thank you so much! We are so thankful to have so much support from you all and the wonderful people we've met through the SMA community.

Katie Holter of Georgia

Dear Families of SMA,

I just wanted to take a moment to say thank you from the bottom of my heart. My grandson, Jacob, was recently diagnosed with SMA and you have been in contact with my son and daughter-in-law, regarding information on SMA. The kids and Jacob received the care package from you and all of us cried tears of amazement at everything that was sent to them. Words cannot express the gratitude I feel in knowing we have such gracious, kind, caring, and supportive family with you all. The information that you have provided has really helped me to gain more knowledge and a better understanding of SMA. Jacob means the world to us and as with every other parent, we want nothing but the best for him and you all are a part of that best!



Again, thank you so very much.

Jacob's MiMi
Ivy Baxter of Florida



Hi Families of SMA,

We received the information packet in the mail today, thank you so much! Wow, it's a lot of information! It will be incredibly helpful to have all this info at our fingertips as things progress. We are so appreciative of this support system. We received the car bed as well as the care package. We are so thankful to have made contact with FSMA, your responsiveness and compassion is truly heartwarming. In the short time since we've received Ezra's diagnosis FSMA has been there with information, support, and a community to learn from and share with. Thank you for all that you do and being there.

Ethan & Emily Bessey of Maine



Hi, my name is Lizzy Morgan. As the UPS truck pulled up into my yard, I was not expecting anything from UPS. I went out and the man handed me a big yellow envelope and my cousin a big huge brown box. I went in the house and sat down with my little girl, Oriaha, who is a SMA Type II child. She has been in and out of the hospital so many times here in Hattiesburg. I always have been told that God gives you special children for a reason and I believe that from the bottom of my heart. Anyway as Oriaha and I opened the box, I was like "Oh my God!"; very shocked and my heart dropped. Oriaha was excited when she saw the lady bug pillow and all the other stuff. The quilt blanket was one of her favorites, she loves animals.



Oriaha was around eight months old when I knew something was wrong. I brought her to her pediatrician here in Hattiesburg, then he sent us to other doctors when she was around 15 months old, that's when we got our answer that she has SMA. Oriaha is a very strong willed child. We try to give her the best of life that we can. Oriaha attends preschool at Sumrall Primary Trek. Last year she had to be taken out and home schooled part of the year due to sickness. When we learned she had SMA Type I was scared especially when they told us that Oriaha wouldn't live to see two years old. Oriaha's now 5½; that's how God works. I am her mom and my heart hurts to see her not be able to walk, crawl, or roll herself over or set her head up when she drops it. When other kids play outside and she can't, it hurts. Oriaha and I strive hard to make it.

Thanks,
Lizzy Morgan, Terry Mackles and Oriaha
of Mississippi

Thank you so much FSMA for the lovely care package. There were so many great ideas and gifts. She loved the bubble blower, light projector, and the different kinds of blankets. I think her favorite thing is those cat toys, she won't let go of them! I want to thank everyone who contributed to making this happen. This package couldn't have come at a better time. I'd also like to say thanks for the organized binder. There was a lot of good information in there. It came at a time when we were starting to get buried in paperwork. I can't say it enough FSMA, thank you!

Timothy and Ruby Gill of Virginia



Hi Families of SMA,

We received the care package for our daughter, Tamara, who has been diagnosed with SMA Type II. She loves the toys. Thank you everyone at Families of SMA for opening your arms and welcoming us to the community.

Best regards,
Tuan Nguyen of Massachusetts

Families of SMA Awards \$710,000 for Basic Research to Identify New and More Effective Approaches for SMA Therapies

Families of SMA is dedicated to creating a treatment and cure for Spinal Muscular Atrophy by funding and advancing a comprehensive research program. Continued investment in basic research leads to greater understanding of the exact nature, causes, and consequences of SMA. This knowledge is key to ensuring that the most effective SMA treatments can be identified and developed as quickly as possible.

Basic research is vital to finding a treatment and cure for SMA. It provides fundamental information about what is going wrong in SMA by showing when and where SMN protein is needed. It also indicates how the SMN protein is working in different cell types. Having this kind of information provides seed ideas for new and better ways of making SMA drugs.

Many important questions in SMA basic research remain unanswered today. The current round of new research awards from Families of SMA will help answer some of these, including:

What function does SMN protein perform in motor neurons?

The projects of Drs. Custer and Androphy at the Indiana University and Dr. Cote at the University of Ottawa will provide a better understanding of what the SMN protein does in motor neurons.

What tissues are affected by reduced SMN protein?

The grants to Dr. Ko at the University of Southern California and Dr. Sumner at Johns Hopkins University will assess the impact of lowered SMN in two types of glia, or support, cells that interact with motor neurons in severe SMA mouse models. Also, the award to Dr. DiDonato will explore the tissues requiring SMN protein in a less severe SMA mouse model.

Are there SMA drug targets, in addition to SMN itself?

The funding to Dr. Ma of Northwestern University will explore the molecular pathways controlling degeneration in SMA motor neurons and look for ways to identify and validate new drug targets in these pathways.



When can SMN protein be provided back and still result in benefit in SMA?

The project of Dr. DiDonato also of Northwestern University is for a new less severe mouse model of SMA, which has the potential to more accurately reflect the human disease. This model will be used to assess when SMN is needed in less severe forms of the disease and how late in the disease course it can be added back and still provide benefit.

Please see our most recent *Compass* for more details on each grant.

Families of SMA Receives 36 Basic Research Grant Applications for New Funding

Families of SMA received 36 research applications for funding consideration by our Scientific Advisory Board (SAB). This was in response to a Request for Proposals released in July, 2012. The best projects were selected for funding by our SAB during a meeting this past fall. Project selection is based on both scientific quality and relevance to the FSMA mission of accelerating the development of an effective and safe treatment and cure for SMA.

The high volume of applications in this area demonstrates the great need for further investment and funding of basic research projects in SMA.

Why is Basic Research Important.

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. 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 that the most effective SMA treatments can be identified and developed as quickly as possible.

FSMA Basic Research Accomplishments.

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

- Mapping and cloning of the SMA gene, SMN1
- Identification of roles of SMN protein in the cell
- Discovery of the back-up SMA gene, SMN2
- Development of animal models for SMA
- Discovery that HDAC inhibitors boost SMN2 levels
- Identification of the nucleic acid sequence used in the ISIS ASO drug

FSMA has invested over \$26 million in basic research grants alone by funding over 155 projects at more than 70 institutions.

Meet the Newly Funded Families of SMA Basic Researchers



Dr. Chien-Ping Ko at the University of Southern California for \$75,000 to the role of glia cells in SMA.

Who are you?

My name is Chien-Ping Ko. I was born in Taiwan and came to the US in 1971. I received my PhD in physiology from Washington University in St. Louis, and did my postdoctoral training in neurobiology at the University of Colorado and then the National Institutes of Health. Since 1981, I have been a faculty in the Section of Neurobiology, Department of Biological Sciences, University of Southern California, Los Angeles, CA.

How did you first become involved with SMA research?

I have long been interested in how neuromuscular junctions (NMJs) work, form and maintain. In the middle of 2000s, my research group became interested in whether and how NMJs might be involved in motor neuron diseases. We first studied amyotrophic lateral sclerosis (ALS), a late-onset motor neuron disease that affects adults. By reading literatures on various motor neuron diseases, we were intrigued by the possibility of NMJ involvement in Spinal Muscular Atrophy (SMA). Given that the genetic basis of SMA has been much better characterized and various animal models are available, I thought that my expertise in the NMJ might contribute to better understanding of whether and how defects in NMJs and other nerve connections (called synapses) may play a role in SMA.

What is your current role in SMA research?

We have recently found that NMJs in several muscles (such as neck muscles) are severely defective and that synapses from proprioceptive sensory neurons onto motor neurons in the spinal cord are drastically reduced in a mouse model mimicking the severe type of SMA. We are now using NMJs in these vulnerable muscles and central synapse loss as benchmarks to test in vivo the effectiveness of

certain candidate compounds in this severe mouse model. We are also characterizing NMJs and central synapses in several new mouse models that are less severe. In addition, we are testing whether and how glial cells may play a role in the pathogenesis of SMA. Our ultimate goal is to better understand the fundamental biology of synaptic defects and hope to find treatments to restore these defects in SMA.



Dr. Jocelyn Cote at the University of Ottawa for \$140,000 for arginine methylation as a regulator of SMN activities in motor neurons.

Who are you?

I am a biochemist and molecular biologist by training with specific expertise in the field of post-transcriptional regulation of gene expression. Transcription involves the synthesis of so-called messenger RNA from a DNA template resulting in the transfer of genetic information from the DNA molecule to the messenger RNA. Post-transcriptional mechanisms include the mechanism that allows the messenger RNA to be differentially "edited or spliced"- depending on cell type or environmental conditions- through a process called alternative splicing. Post-transcriptional mechanisms also include the regulation of the transport, localization, and effective life span of the RNA messenger, as well as the specific modifications to the proteins that participates in these processes.

How did you first become involved in SMA Research?

I initially started working on SMA because I identified SMN, or more specifically a part of the SMN protein called the Tudor domain, as a domain capable of 'sensing' a special protein modification termed 'arginine methylation'. Following this discovery, we reasoned that these 'methylated' proteins might represent a major subset of proteins that would stop functioning normally in the absence of SMN in SMA patients, and that studying these

proteins might help us gain a better understanding of what SMN does in spinal cord motor neurons and how loss of its activity leads to SMA. After I first attended the Annual SMA Research Conference back in 2002 and met with SMA kids and their families, it became clear to me that I was going to do my best to contribute my expertise towards increasing our fundamental understanding of this disease in the hope that it would help one day in the elaboration of novel therapeutic strategies.

What is your current role in SMA Research?

My lab uses biochemical and cellular biology approaches in order to gain a better understanding of the precise function that SMN plays in spinal cord motor neuron, and how loss of that function leads to the disease. For example, we are trying to identify what are the other proteins and RNA molecules that SMN interacts with and controls in motor neurons, with the logic that this should give us some insights into what SMN is actually doing in this cell type in particular. Then, we assess if these SMN interacting partners could represent valid targets that might be easier to manipulate than SMN itself to improve the SMA phenotype.



Dr. Custer in the Androphy Lab at Indiana University for \$140,000 for the role of vehicle coat protein alpha-COP in new models of SMA.

Who are you?

I have a PhD from the University of Washington in Neurobiology and Behavior and I study hereditary neurodegenerative diseases in cell culture and mouse models.

How did you first become involved with SMA research?

I became involved with SMA after moving to Indianapolis and joining the research lab of Dr. Elliot Androphy. I had previously worked with some SMA Type II and III patients at an amazing therapeutic equestrian facility in

Woodinville, WA. Equestrian therapy is great for your core and for your spirit!

What is your current role in SMA research?

I am interested in learning more about the basic biology of SMA and how other proteins can influence the health and maintenance of motor neurons. We use a combination of cell models and animal models to address these questions. The more we know about the cellular environment in SMA, the more targets we have to aim at for therapeutic intervention.



Dr. Christine DiDonato at Northwestern University for \$140,000 for the when and where requirements of SMN in less severe SMA.

Who are you?

I am a molecular biologist with training in human and mouse genetics with specific expertise in the development and use of mouse models of human disease. I obtained my PhD in molecular genetics from The Ohio State University. I then pursued my training as a Postdoctoral Fellow at Hospital St. Justine Research Center in Montreal, Quebec, Canada and then at the Ottawa Health Research Institute in Ottawa, Ontario, Canada. I started my independent research group in 2003 at Ann & Robert H. Lurie Children's Hospital Research Center (formerly Children's Memorial Hospital Research Center). I am an Assistant Professor in the Department of Pediatrics at Northwestern University Feinberg School of Medicine.

How did you first become involved with SMA research?

I started working on SMA while a PhD student at Ohio State University under the mentorship of Arthur Burghes. At that time we were working to identify the gene responsible for SMA. It was during that time that I was "bitten" with the SMA research bug from our interactions with families. Since then my scientific career has focused on various aspects of SMA research, -- from working to identify markers useful for prenatal diagnosis and gene identification while a graduate student before the gene was cloned, to developing mouse model techniques while a post-doctoral trainee and then

with my own laboratory generating and characterizing SMA mice of varying severities and testing potential therapeutic treatment strategies.

What is your current role in SMA research?

My lab primarily focuses on development of longer-lived SMA mouse models and we use them to gain a better understanding of the tissues and cell types that are most affected by low levels of SMN and how this contributes to disease. We are also using these mice to ask specific questions about re-introducing or increasing SMN levels after symptom onset has occurred in milder mice. Finally we are using these milder mice to identify and test other therapies that are SMN-independent to improve the SMA phenotype.



Dr. Charlotte Sumner at Johns Hopkins University for \$140,000 to motor axon development in SMA.

Who are you?

I am a neurologist who cares for adults with Spinal Muscular Atrophy and other neuromuscular conditions, as well as a neuroscientist who investigates how mutant genes such as SMN cause motor neuron degeneration. We study animal models of SMA as well as human tissues and are particularly interested in developing treatment for SMA and related conditions.

How did you first become involved in SMA Research?

I became involved in SMA research after a colleague encouraged me to attend the Families of SMA meeting in 2001. After meeting families and being exposed to the challenges and promise of therapeutics development for SMA, I was hooked! I feel very fortunate to be part of this community and am very hopeful for the future.

What is your current role in SMA Research?

We are very interested in understanding why SMN protein deficiency results in muscle weakness and how this can be reversed or stabilized. We have investigated the nature of motor neuron dysfunction in SMA mice and more recently, we have aimed to define which cell types contribute to this process. In addition, we have examined the therapeutic

effects of several potential treatments in SMA mice including HDAC inhibitors and more recently RG3039.



Dr. Yong-Chao Ma at Northwestern University for \$75,000 to regulation of HDAC5 phosphorylation by Cdk5 in SMA.

Who are you?

Dr. Ma received his Ph.D. from the Weill Medical College of Cornell University and his postdoctoral training at Harvard Medical School/Children's Hospital Boston studying spinal motor neuron development.

How did you first become involved with SMA research?

Dr. Ma's lab focuses on research on the molecular mechanisms regulating spinal motor neuron development. This work caused him to ask how dysregulation of spinal motor functions may contribute to the pathogenesis of SMA.

What is your current role in SMA research?

Dr. Ma's group at Northwestern University/Children's Hospital of Chicago Research Center studies cell signaling and gene expression mechanisms regulating healthy spinal motor neuron functions. Understanding how normal processes are dysregulation could lead to a better understanding of spinal motor neuron degeneration in SMA. To address these questions, his group uses SMA mouse and zebra fish models, human SMA induced pluripotent stem cells, and cultured spinal motor neurons as model systems. They employ a combination of molecular biological, biochemical, and genetic approaches to address this problem. They also collaborate closely with several other laboratories with complimentary expertise to explore novel pathogenic mechanisms and therapeutic strategies of SMA.

Ms. Wheelchair New York: On the topic of dating, sex, and relationships

Dating, relationships and sexuality are fundamental to our health and happiness as human beings. As evidenced by a multitude of television shows and movies, Pop Culture tells us that “finding the one” is a universal struggle of significant magnitude. But grappling with relationships and feeling fulfilled sexually is more than just the plot of an episode of *Sex and the City*; it is essential to our emotional and physical wellbeing. And, while these issues are complicated for the general population, they are much more complex for someone dealing with a physical disability.



While dating and engaging in romantic relationships rank high in life’s priorities, they are frequently not recognized as such in the disabled community. In fact, our society, media included, seems to ignore the idea that people with physical disabilities have the same emotional needs and desires as the rest of the population regarding relationships, sex, and dating. This is exceedingly unfortunate because we do have the very same needs and desires but, in general, are highly likely to have been restricted in finding fulfillment in these areas. While there have been many campaigns designed to improve the lives of disabled people, very little attention had been paid to improving our romantic lives. As Ms. Wheelchair New York, and as a New York urban professional, I have chosen to pay attention, and I am putting it out there in a larger campaign designed to improve the dating and sexual lives and romantic relationships of people with physical disabilities.

I was diagnosed with Spinal Muscular Atrophy Type II at age two, and I got my

first motorized wheelchair in kindergarten. In most ways, my life has been very fulfilling. I have always been surrounded by loving family and many friends. I have traveled and been an advocate for people with neuromuscular disorders since I was a child, and I have achieved academically. I moved to Manhattan in 2000 to go to graduate school, where I obtained my Ph.D. in Clinical Psychology. I now live independently and manage home attendants while working full-time as a psychologist in an outpatient setting treating adults with serious and persistent mental illness. I eat at trendy restaurants, party in some of Manhattan’s chicest lounges, and shop at designer boutiques. But in this life, which is otherwise fabulous, I have struggled throughout with dating and finding romance. This is where my passion for the topic originated.

I was crowned Ms. Wheelchair New York in April 2012. I took my crown and my passion to change the dating lives of people with disabilities and I dashed straight for the media spotlights and press cameras to deliver my message. My campaign consists of two main parts: I want to expose society, on a large scale, to



the fact that people with physical disabilities are sexy, datable, and make great romantic partners. Research shows that society’s inaccurate assumptions and misconceptions about the sexuality of individuals with physical disabilities create the largest obstacles for the disabled in the domains of dating, relationships, and sex. Many negative stereotypes and attitudes exist, including viewing individuals with physical disabilities as asexual, physically unattractive, not interested in sexual activity, at a great disadvantage in the search for a romantic partner, or as unmasculine/unfeminine, weak/dependent, and/or infertile. Some explain the difficulties disabled people encounter by citing the lack of ease that able-bodied people feel with and probably convey to disabled people around this topic. Society is taught through popular media that no one could or should find a person with a disability sexually attractive. Through my own media appearances, which have included interviews on WNBC-TV, in *The New York Daily News*, SiriusXm Satellite’s Doctor Radio, *The Daily Mail* (UK), and Yahoo! News.com, I have challenged these stereotypes and stigmas head on. I am now working with my publicist on several television projects, including a reality television show, where through my own example I will expose the viewing public to the fact that dating someone with a physical disability can be both hip and sexy.

The second part to my campaign is engineered specifically to address the emotional needs of people with physical disabilities who struggle in the areas of sex, dating, and relationships. Struggles in these areas can lead to feelings of loneliness, isolation, depression, hopelessness, anxiety (i.e., not meeting the societal standards in regards to getting married and having children), and impact physical health. Inextricably linked to negative societal stereotypes, are



the consequences on the disabled person's self-concept and self-esteem as the ways that people evaluate themselves and their actions are frequently heavily influenced by other's expectations. People with physical disabilities frequently lack confidence and initiative when it comes to dating, romantic

relationships, and sexual relations. Negative stereotypes can become a self-fulfilling prophecy, leading some people to retreat from intimacy and sexuality all together. In this context, I have launched a private therapy practice, where I conduct therapy sessions via the internet through Skype. I have developed this practice to make it easier for people with physical disabilities to participate in therapy and address these issues. By doing sessions online, a person doesn't have to leave their home and worry about ways to travel and get to the therapist's office.

As Ms. Wheelchair New York, I have embarked on a large undertaking, and I think I have given new meaning to the phrase "The City that Never Sleeps." The journey has been amazing to me, as the media, publicity, and photo shoots have bumped me into a surprising celebrity-like status. Getting my hair styled and make-up done by artists who have styled top models, working with photographers who have shot Ms. USA and Ms. Universe, and being recognized by people on the street, I feel like I am living a dream in Manhattan. But it is the message, and the momentum behind the crown, that continues to drive me to improve the dating, sex lives, and romantic relationships for people living with physical disabilities. While it has been an honor to represent the City that gave me my first dates, my confidence in myself and my sexuality, and my desire to make romance possible for others with disabilities, I seek to raise this issue to a national and even a global level. In a society based on dating, relationships, and sexuality, it is time to integrate those with physical disabilities.

Danielle Sheypuk, Ph.D.
Licensed Psychologist
Ms. Wheelchair New York
daniellesheypuk.com

Dear FSMA,

My name is Craig (C.V.) Abbott II, I'm 23 years old and have Spinal Muscular Atrophy Type I. As you know, this is the most severe type of SMA. I was diagnosed when I was 6 months old, and given less than 2 years to live. My mother has always been a fighter. I guess that's where I get it from. My family doesn't believe in the word "no" and neither do I. I've never given up on my health, or on anything because of my disability. As a matter of fact, I believe it pushes me to do more.



Music has played a huge role in my life. My family started me in voice lessons when I was 5. I took them until I was 17. I sang at many churches, school concerts, in multiple choirs, I even opened up for our local Minor League Baseball team, twice. When I was 15 I developed an interest in playing the guitar. I was told by many people that I would never be able to play. So I bought a book, developed my own way of playing, and taught myself. Two years later, I began teaching lessons at a local music studio. I absolutely loved it. I was helping others learn, and I loved teaching them.

In my life, I've had many severe illnesses, usually pneumonia. I've been told that I wasn't going to make it through the night or through that bout of pneumonia. I won't lie, it's scary. But my family never gave up on me, and most importantly, I never gave up on myself. After I found out how rare I was, I began my autobiography. I want to share my story with anyone that is going through any hardships in their life, hoping I can inspire them to never give up.

If I can make it through so can you. Sometimes we just need a little motivation. Hopefully, I can be just that little bit you need. And if I can be, if I've helped just one person, then I feel all this has been worth it. If anyone is interested in contacting me, please do it through my website, which can be seen at www.craigvabbott.com. Thank you all for your time and God bless!

Sincerely,
Craig V. Abbot II of New York

A Half-Century of Living with SMA

by Ben Mattlin

I was six months old when my parents noticed the onset of my Spinal Muscular Atrophy (Type II). I couldn't sit up. We didn't know the cause then. This was in the early 1960s; the diagnosis wasn't well known yet, if it existed at all.

In fact, about the only thing the doctors and my parents could agree on was that I was a floppy baby.

At age six, the progression slowed so much that the neurologist said it had "plateaued." I thought that meant I would never get weaker again, but actually it just took longer.

I was pretty content as a kid in a wheelchair. Having never walked or stood, I had no sense of loss. My parents fought for me to attend regular schools at a time when the law was not on their side. The schools weren't accessible as we understand the world today, but I made do. I learned. I made friends. Life was good.

It was only when I moved away from home to attend Harvard—in 1980, the very year the university was first required to accommodate students with disabilities—that I felt a crushing sense of alienation. There were many reasons. The environment was unfriendly—cobblestones and brick paths are hard on wheelchairs. The administration wouldn't allow me to have roommates, which, I suppose, wasn't considered an aspect of campus life that had to be made accessible. And for the first time, I had to rely on paid attendants 24/7 instead of a mix of paid help and family as I'd had previously.

Even after graduation, I was stunned to discover no one would hire me. It was particularly odd because many of the employers liked my work. They would assign me articles to write for their magazines but only on a freelance basis. They didn't want me around the offices, or staff.

What could I do? It was still legal to discriminate against people like me.

I got by with the indefatigable support of my family and friends. And the stupid, stubborn sense that I was entitled to a regular life. I was also helped by technology. When voice-recognition computers became an affordable reality, I was one of the first to sign on. At this point my hands had grown so weak I could no longer use a keyboard or hold a pencil.

The disability-rights movement also came to my rescue. Not that the Americans with Disabilities Act got me a job, but it clarified some of my experiences and gave me a framework for coping. Plus I met other folks like me who may have been



vulnerable but weren't pitiful. In fact, they were righteous and powerful.

I began using my writing abilities to publish essays about living with a disability. It was only after my 40th birthday that I felt I had enough perspective to take up the daunting task of writing a book about where I'd come from and all the changes I'd witnessed.



My book was published late last year, just before I turned 50.

Maxwell Brown on the 2012 Microsoft Firenze BXT winning team

Maxwell Brown, who has SMA Type II and is from Livingston, NJ, was on the winning team of the 2012 Microsoft Firenze BXT competition. Maxwell is shown holding the trophy given to all of the finalists, one of four prizes he received for being on the winning team. Now a sophomore in the Carnegie Mellon University School of Design, he is studying graphic design.

Firenze BXT brings together multi-disciplinary teams to tackle real world problems facing Microsoft product groups. BXT stands for Business, user eXperience and Technology, with students from business, design and engineering schools forming teams. Maxwell's team competed against six other teams from Carnegie Mellon and their entry was judged the best by Microsoft employees and product managers. Maxwell's team from Carnegie Mellon was the first undergraduate team ever to make it to the finals of the competition, with a team comprised of Maxwell and another freshman, one sophomore and one first year MBA candidate.

The final phase of this student innovation competition was held on March 3-4, 2012 at Microsoft's headquarters in Redmond, Washington, where Maxwell and his three team members from Carnegie Mellon traveled to compete with students from Cornell University and Ohio State University. Only three schools had teams that were invited to compete in the final round of the competition, with this being Carnegie Mellon's fifth straight year in the finals.

The school teams were separated for the final round and Maxwell's team for the final round included two Cornell students and one Ohio State student, with Maxwell as the sole design student. The final round teams were given a new problem and less than 24 hours to come up with a solution, including preparing a presentation of their solution. The teams worked under the observation of general managers and other Microsoft employees. Photographs and information about the Firenze BXT competition can be found at www.facebook.com/firenzeBXT.

Maxwell uses a manual wheelchair and electric scooter to get around campus and around Pittsburgh, PA. He has his own room and bathroom at school and has aides assist him in the mornings and evenings. Maxwell is a member of the Sigma Chi fraternity and has started a public speaking team at Carnegie Mellon.



Dear FSMA,

Hi, my name is Jaci Moreno, and I have a daughter affected with SMA. Her name is Presley. This is not the first time we have been confronted with this disease. We lost Ethan Lucas Call on December 23, 2002 at about six weeks old. He was a sweet little boy and loved by all. He left behind his dad, Lucas Call, my little brother, wife, Erin Call and Averie, his big sister. Since then they have had two healthy boys, Gavin and Grady.

Presley was diagnosed shortly before her second birthday. We were devastated to say the least. We had only known the SMA that would take her life any day, and being two I was sure we would soon lose her as well. Eight years later we found ourselves sitting with the same doctor that treated Ethan. I was numb; no words can ever fully describe the fear and sadness that overwhelms you with grief, listening and trying to hear words coming out of the doctor's mouth as I watched Presley playing on the ground. I see the strength in many adults fighting life threatening diseases, but when it's a child, your child, it feels as if you are going to be put in the situation of watching every hope and dream go missing, along with the muscles they need to walk, eat, breath and even smile. It's out of your hands and your helpless, you feel as if you are in a cage with your hands tied just being forced to watch as your baby struggles.

Presley has more good days than bad, but I see time creeping in every once in a while whether its falling more or not being able to walk as far as she once did. Now three years later I have to say there are days when I see her fighting to be like her peers, pushing herself at her many physical and occupational therapies and countless doctor's appointments, just to maintain her health. Presley rarely complains but I see it, I see when she realizes she can't be part of an activity or walking around the whole playground just to find a spot low enough for her to



get up. Then collapsing, those are the worst. It embarrasses her so badly. We know now not to make a big deal so it's less noticeable.

Presley is unique in the way that SMA does not stop her (fully); she pushes and pushes me. Can I go to my friends? Can I spend the night at my BFFs? Just the other day she walked in and asked me, "Mom its time for me to learn how to skate!" As we all know this is what is going through our heads: What are they going to feed her? Is there junk food? What if she falls? Or has to be carried? And what happens if no one helps her up and down the stairs? And skate? Skate? What in her head thinks she can do this? And well that's just Presley; she refuses to allow her disability to stop her. If there is a chance she'll try it!

There is a very black and bold line with our kids and nutrition, as well as injury and fatigue. But someone forgot to tell Presley; she is truly amazing. On the days I'm tired of being strong, tired of fighting schools, doctors, and insurance companies (to list some) I just have to sit back and watch her. It's a blessing and a



curse. Only her body knows what is to become of her, and the hard questions she asks, "When will I learn to run? Can you teach me a cartwheel?" The fact that although she looks up to her big sister Phoenix, who is 15, she says "I can't wait to be big like her so I will be strong!" All I can think? It hurts my heart. Although her future is uncertain, Presley's view is not! She wants to be a veterinarian, a doctor, play soccer, learn karate and be a mom.

She has a father that would move mountains for her. She often tells me "I'm a daddy's girl." A big sister who is an amazing athlete and gives a 110% at whatever she does. Her sister wrote a

Marching to an iPad Drummer

Reprinted with permission of the Muscular Dystrophy Association (quest.mda.org), by Kathy Wechsler on October 1, 2012.



Here's yet another way technology lets the music come through

Article Highlights:

- Ethan Och, who has spinal muscular atrophy and uses a power wheelchair, has been a drummer with his school band for four years. He recently switched to playing drums on an iPad and iPhone when arm weakness made drumming with sticks too exhausting.
- Technology has allowed Och to continue playing with his school band, participate in competitions, compose and arrange music, and even march with a marching band.

"I hear cymbals, but I don't see them," Stephenie Och recalls a fellow parade watcher announcing as the Long Prairie-Grey Eagle Area High School Marching Band marched by this summer.

Upon closer examination of the percussion section, one could see 14-year-old Ethan Och, who has spinal muscular atrophy, "marching" from his power wheelchair, tapping on an iPhone strapped to his leg to play the cymbals on an application called Shaker. A student marched alongside pushing a guitar amplifier powered by a car battery.

Ethan, who lives with his parents, Stephenie and James Och, in Swanville, Minn., is a ninth-grader at Swanville High School and plays the drums in the Swanville Senior High Concert Band.

continued on next page



paper on Presley and said how much she looked up to her; she is working so much harder to do things we take for granted. Sage, her eight year old brother, races dirt bikes and is a good athlete. He walks straight from his class to Presley's, at school,

and waits for her knowing he needs to be there to hold her hand and let other kids know they can't knock her over. They also don't cut her slack; they push her to try new things, and they always include her somehow! She never misses a game or race; she is there and loves watching them! Presley has a mobility dog named Guinness, and I have to say he has been such a blessing to our family and to her. Now when she falls into a full collapse, she calls him over and gives the command to sit. Guinness won't leave her side till she is up and safe, and then she tells him its okay. Presley is participating in a research study and did great; she is getting ready to do another procedure in February. Although somethings were not her favorite she was so strong.

I'm writing this to thank my husband, Dave, and kids, Phoenix and Sage. I know Presley has more will and determination than we have ever seen in a small little girl. You guys make me so proud; you see when she needs a lift, but you have never treated her any differently than if she wasn't sick. Also, Presley has so many aunts, uncles, nieces and nephews that love and support her! You have all been amazing to her. I see the extra attention she is given and just the words of encouragement when she is down are much appreciated. I know people don't see a lot that goes on behind closed doors with a special needs child and how much it takes out of you.

To all of my SMA moms and dads, I know the nights you lay awake just praying, "I wish it was me that was sick," that we could handle this, right? Or "Why us, why our kids?" But we were chosen, we are up to this task! Let's make everyday the best day we can give them. Take time for us too. Without a strong foundation it will all crumble. Cry when you need to; ask for help when you need it! The people around us have no idea what we go through on a daily basis, but I know if we ask they would love to help! Presley has taught me so much but the biggest is she in no terms feels sorry for herself. So when my heart is breaking for her and I think I can't bare another day watching her fall and get back up and fall and get back up, watch other kids doing things she can't with a big smile (cause I know what she's thinking). I think how I can feel anything but grateful!

Everyone be safe I know we are ALL fighting different battles but we all have one thing we are fighting for... A cure!

Jaci Moreno of Utah

Marching to an iPad Drummer (*continued*)

Drumming 'old school'

When Ethan joined the Swanville Elementary band in fifth grade, he chose the drums because he wanted to try something new and challenging. He started playing the snare drum, timpani and bells, and his band director, Gina Christopherson, found a pair of lightweight drum sticks to make drumming less exhausting for him.

But even with the lightweight drumsticks, drumming was taxing for Ethan. His arm weakness and balance issues left him extremely fatigued, especially as the rhythm picked up speed. He became frustrated that exhaustion wouldn't allow him to continue drumming through an entire song.

"I was kind of bummed about it, but I decided I would try doing band until I absolutely could not do it anymore," Ethan says.

Although Ethan tried to keep a positive attitude, drumming was becoming extremely difficult by the time he reached eighth grade at Swanville Junior High School, and he feared he might not be able to continue.

Technology to the rescue

An alternative "pretty much fell in our lap," says Stephenie.

Although he didn't realize it at the time, Ethan started the ball rolling when he discovered a piano app (downloadable program) on his smartphone and brought it to school to show Christopherson. The band director was inspired to research drum apps, but unfortunately the one she found wasn't compatible with Ethan's smartphone. So Christopherson let Ethan borrow her iPhone during band class until a better solution could be discovered.

With the iPhone hooked up to a sound system, Ethan was able to play along with the rest of the band using the iPhone

apps Cowbell Plus and Shaker (now called Pocket Shaker). He played by tapping his finger on one of the images of percussion instruments on the screen, controlling the tempo and volume by how quickly and where he tapped the image.

Meanwhile, Christopherson and Ethan's mother, Stephenie, worked to get Ethan an iPad2 through his Individualized Education Plan (IEP). The iPad2, which he received a few months later, gave him access to more music apps, and, because it was his, allowed him to practice at home. He also could use it for classes other than band because of the many different apps available.

These days, Ethan uses an app called GarageBand for band class. This app follows the basic idea of the iPhone apps but, because the iPad2's screen is larger than the iPhone's, it can display a full drum set, allowing Ethan to play multiple percussion instruments using multiple fingers. He also uses Cowbell Plus, Shaker, Claves, WoodBlock, SnareDrum and Virtuoso, which allows him to play the xylophone. Some percussion apps are free, and others cost up to \$20.

Ethan's iPad2 was working well for band practice, but he wanted to compete with the rest of his band. Since no student had ever competed using an iPad2, Christopherson worked with the Minnesota State High School League to get approval for Ethan to participate in concerts and band competitions using his iPad2.

"I have been very impressed with Gina's enthusiasm and dedication to making sure that Ethan is able to continue with band," says Stephenie of the creative band director.

Drumming on the go

When Ethan was finishing up the eighth grade earlier this year, his band class was given the opportunity to join the Long

Prairie-Grey Eagle Area High School Marching Band for five parades during the summer. The band, in their blue-and-white shirts and black pants, was comprised of students from several area high schools.

Ethan was interested in playing in a marching band, but uncertain how to make it work. Christopherson, who served as the assistant marching band director, took on the project of making it possible. The iPad2 was too cumbersome, so she strapped her iPhone to his leg. He used Cowbell Plus and Shaker as the iPhone apps for marching band.

So that Ethan's cymbals could be heard, Christopherson's husband's came up with the idea of having another student push a stroller holding a guitar amplifier powered by a car battery attached to a power inverter.

Gettin' 'techy' with it

As technology keeps evolving, Ethan and other students with disabilities find themselves opening more doors than they may have ever thought possible.

"I think that every kid should get to participate [in band] whether they have a challenge or not," says Christopherson. "It's just a matter of figuring which instrument works and how we can get it done."

Ethan will probably stay in band until graduation in four years. He's thinking of participating in instrumental competitions for small groups, trios and duets.

"Ethan can do anything he wants to with music," Christopherson says. "He has a phenomenal ability to compose, so we're looking at doing some extra stuff this year with arranging and composing, because that's something that really excites him."

Repligen Initiates Phase 1b Trial of RG3039 for SMA Licensed from Families of SMA

Repligen Corporation has initiated a Phase 1b clinical study of RG3039, a novel small molecule drug candidate for the potential treatment of Spinal Muscular Atrophy (SMA). The primary objectives of the study are to further evaluate the safety and plasma pharmacokinetics (PK) of multiple doses of RG3039 in healthy volunteers.

Repligen licensed RG3039 in 2009 from Families of Spinal Muscular Atrophy (FSMA). FSMA funded and directed the preclinical development of RG3039 with an investment of more than \$13 million. This was the first drug discovery program ever conducted specifically for SMA.

Phase 1b Study Design:

This Phase 1b trial is a double-blind, placebo-controlled, ascending multiple dose study, being conducted at the Jasper Clinic in Kalamazoo, Michigan. According to the study protocol, each of 32 adult healthy volunteers will be randomized to receive ascending oral doses of either RG3039 or placebo. Within each of the four dose cohorts, six subjects will be exposed to oral doses of RG3039 and two subjects to placebo (3-to-1 ratio) for 28 days.

Secondary objectives are to correlate RG3039 PK with a panel of molecular biomarkers, and to determine the inhibitory plasma concentrations of RG3039 for the target enzyme DcpS. It is the company's expectation that cumulative Phase 1 study outcomes will inform the approach and design of a future efficacy study of RG3039 in SMA patients.

Phase 1a Results:

The initiation of this Phase 1b study follows on the completion by Repligen of a Phase 1a trial earlier this year. The Phase 1a study was a blinded, ascending single dose study of RG3039 administered to 32 healthy volunteers. Positive results from this single dose study were presented at the Annual Meeting of the American Academy of Neurology (AAN) in April 2012. Those results demonstrated that RG3039 was well tolerated at plasma levels exceeding those in which cellular DcpS was fully inhibited. The data showed evidence of a dose related drug exposure and target binding that resulted in greater than 90% inhibition of the target enzyme DcpS in peripheral blood cells for 48 hours.

RG3039 is an orally bioavailable small molecule inhibitor of an RNA processing enzyme called DcpS. RG3039 has been shown to increase production of the SMN protein in cells derived from patients. In addition, RG3039 has been shown to improve motor neuron pathology, mobility and lifespan in animal models of SMA.

"Completion of this stage of the RG3039 clinical development program in healthy volunteers, if successful, will provide the necessary foundation for more advanced trials involving SMA patients who are in critical need of a treatment for this devastating disease," said Walter C. Herlihy, Ph.D., President and Chief Executive Officer of Repligen. *"We appreciate the support of our collaborators in conducting this Phase 1b trial and look forward to sharing the results."*

FSMA began the Quinazoline/RG3039 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. The direction from FSMA provided the positive results necessary to license the program to Repligen Corporation and leverage larger funding for clinical development.

RepliGen

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

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

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

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

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



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

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

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

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

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



Participating clinical study sites are:

East

Boston Children's Hospital, Boston, MA

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

Children's National Medical Center, Washington, DC

Principal Investigator: Carolina Tesi-Rocha, MD
 Coordinator: Meenal Pathak, MB, BS
 Email: MPathak@childrensnational.org

Columbia University Medical Center, New York, NY

Principal Investigator: Claudia Chiriboga, MD
 Coordinator: Nicole Holuba, MSN
 Email: nh2282@mail.cumc.columbia.edu

State University of New York Upstate Medical Center, Syracuse, NY

Principal Investigator: Klaus Werner, MD, PhD
 Coordinator: Tanya Perry, BS
 Email: PerryT@upstate.edu

Midwest

Northwestern University, Chicago, IL

Children's Memorial Hospital, Chicago, IL
 Principal Investigator: Nancy Kuntz, MD
 Coordinator: Lauren Webb, BA
 Email: lwebb@childrensmemorial.org

Ohio State University, Columbus, OH (site of the protocol Principal Investigator)

Nationwide Children's Hospital, Columbus, OH
 Principal Investigator: Richard Shell, MD
 Coordinator: Karen Carter, CCRC
 Email: Karen.Carter@nationwidechildrens.org

Washington University in St. Louis School of Medicine, Saint Louis, MO

Principal Investigator: Anne Connolly, MD
 Coordinator: Charlie Wulf, BA
 Email: wulfc@neuro.wustl.edu

South

Emory University, Atlanta, GA

Children's Hospital of Atlanta, Atlanta, GA
 Principal Investigator: Maurice Sholas, MD PhD
 Coordinator: Rian Thornton, BS, CCRC
 Email: Rian.Thornton@choa.org

University of Texas Southwestern Medical Center, Dallas, TX

Children's Medical Center of Dallas, Dallas, TX
 Principal Investigator: Susan T. Iannaccone, MD
 Coordinator: Mariam Andersen, MA, CCRP
 Email: mariam.andersen@utsouthwestern.edu

Vanderbilt University, Nashville, TN

Principal Investigator: W. Bryan Burnette, MD, MS
 Coordinator: Tori Stromp, BS
 Email: tori.stromp@vanderbilt.edu

West

University of California – Davis, Davis, CA

Principal Investigator: Craig McDonald, MD
 Coordinator: Randev Sandhu, BS, CCRP
 Email: randev.sandhu@ucdmc.ucdavis.edu

University of California – Los Angeles, Los Angeles, CA

Principal Investigator: Perry Shieh, MD, PhD
 Coordinator: Angel Hu, BA
 Email: angelhu@gmail.com

University of Colorado – Denver, Aurora, CO

Children's Hospital Colorado, Aurora, CO
 Principal Investigator: Julie Parsons, MD
 Coordinator: Melissa Gibbons, MS, GCG
 Email: melissa.gibbons@childrenscolorado.org

University of Utah, Salt Lake City, UT

University of Utah Health Sciences Center, Salt Lake City, UT
 Principal Investigator: Kathryn J. Swoboda, MD
 Coordinator: Sandra P. Reyna, MD
 Email: sreyna@genetics.utah.edu
 Coordinator: Renee Gerardi, BS
 Email: renee.gerardi@hsc.utah.edu

Oregon Health and Science University, Portland, OR

Dorenbecher Children's Hospital, Portland, OR
 Principal Investigator: Erika Finanger, MD
 Coordinator: Rhonda Muhly, CCRC
 Email: muhly@ohsu.edu

Recruitment Video Released for the NINDS NeuroNEXT SMA Biomarker Trial

The trial named “Spinal Muscular Atrophy (SMA) Biomarkers in the Immediate Postnatal Period of Development” is currently enrolling infants with SMA, ages 0 to 6 months, at 15 different locations in the US. It is the first trial being conducted by the NINDS NeuroNEXT clinical trial network. The newly released recruitment video provides information about the trial and the participating sites.

See <http://tinyurl.com/d6oka42> to view the recruitment video for the trial.

The Network for Excellence in Neuroscience Clinical Trials, abbreviated NeuroNext, is a twenty-five site national clinical trial network created by NINDS of the NIH to test promising new therapies for both pediatric and adult patients with neurological diseases.

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

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



Families of SMA is funding patient travel for the study and will ensure the SMA families will have the ability to travel to and from the 15 study sites across the country. “We are very excited to begin enrollment in this SMA Biomarker study that will focus in on infants with SMA”, says Stephen Kolb, Protocol PI for the study. “This work is designed to pave the way towards efficient and successful SMA clinical trials and will inform the design of future clinical trials. We are thrilled to have the support of FSMA who are a critical partner in our efforts to inform the SMA community about this study and have provided essential funding support that will have a positive impact on the quality of data that will be generated by this study.”

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

Families of SMA has invested \$6 million for the development of clinical trial infrastructure, including testing protocols and clinical trial site readiness. FSMA funding has included three clinical trials at Ohio State University and the establishment of a care clinic geared towards infants with SMA Type I. This work paved the wave for the current major investment in SMA clinical trials by NIH.

Painting and Chalking: For Everyone and Everything

By David Block

Imagine while navigating your wheelchair down the street, the chair itself starts painting the road without your hands contributing! Imagine your wheelchair chalking up the road as you move across it. A few decades ago, these ideas would have seemed ludicrous. However, they are no longer ideas, but actualities thanks to the creative efforts of the full able bodied Dwayne Szot. The 50-year-old Szot of Bayfield, Wisconsin, developed these adaptive tools and many others. His compassion for people with disabilities ignited its first spark while he was growing up in a Midwest foster home. His foster brother and foster sister both had Cerebral Palsy.

Szot remembers that his foster sister's bus stop was about a mile away from their home and getting there required walking on a gravel road.

"I had to pull her in the wagon to her bus stop because she couldn't walk that fast," said Szot. "Her crutches also slowed her down."

His ability to build and fix things developed during his childhood. "I had no choice," Szot remembered, "because there was no hardware store near us. If you broke something, you had to fix it yourself."

Szot credited these childhood experiences as being his first steps toward developing Zot Artz.

The Launching of Zot Artz

After Szot earned his MFA in sculpture from Cranbrook Academy of Art in Bloomfield Hills, Michigan, the National Endowment for the Arts and the Rockefeller Foundation awarded him grant money to create Zot Artz. Established in 1990, Zot Artz developed a



number of mechanical tools to allow people with disabilities to experience the joy of creating art.

One invention was the chalk walk and roll. A chalk drawer attached to a walker or wheelchair, allowed the less able individual to see chalk cover up the road or sidewalk as he or she navigated through those areas.

Another invention was the art roller. This invention caused the wheelchair to paint while the wheelchair user rolled down the street. The art roller is a small compact roller printer that attaches to the front of the wheelchair with a bridge that has Velcro on it. "It is a printing press," said Szot. "It puts the paint onto the print."

Szot loves seeing the smiles on kids' faces when they can use these mechanical tools, because they now can draw and paint like their able bodied peers.

Szot remembered that when he attached the chalk walk and roll to a boy's wheelchair, the little boy was so excited that he immediately took off and rolled his wheelchair into a busy street.

"I had to chase after him because there was too much traffic," Szot said. "He was having a good time chalking up the street



and dodging cars. I was more afraid than him."

Szot's website explains that instead of finding ways for people with disabilities to participate in activities for able bodied individuals, Szot Artz created activities and projects for less able individuals, where the fully able bodied person can take part.

Participants benefit by enhancing their motor and social skills, concentration, and self esteem.

Zot Artz has created hundreds of special art events around the world, and helped schools, museums, residential and day centers, independent living centers, park and recreation programs and others put the art tools to work in their programs.

"I only have a few people working with me and we have a low marketing budget," said Szot. Word of mouth, appearing at disability expos, helps him get the word out about Zot Artz.

For more information, log onto www.zotartz.com



Lilee Ford with Kayla



Mariah and Nevaeh Rohelia



Maxwell Peppers



Jack Bolton



Logan Ruth



Josh Tramontano



Leila Grace Avramenko



Mia Fuentes



Maia, Viv and Kiera Hoang Shockley



Samantha Utzat



Emma Rubenstein



Ross Rosenfeld



Murphy and Anders Potter



Liadan and Keenan Conner-Park



Mabel Mckenzie



Sofia Scott



Mary Kate, Eileen and Annie Venedam



Kale and Kyler Shiesley



Presley Moreno



Lucia Pilkova



Ren Stein



Spencer Coates



The Sykora's



Megan and Madison Budreau



Suzanna Rose Slonka



Ray Fantel



Kyle and Carter Nunemaker



Savannah Williams



Nora Gooden



Jonathan Morrow



Lizzy Hallam



Caleb Elmer



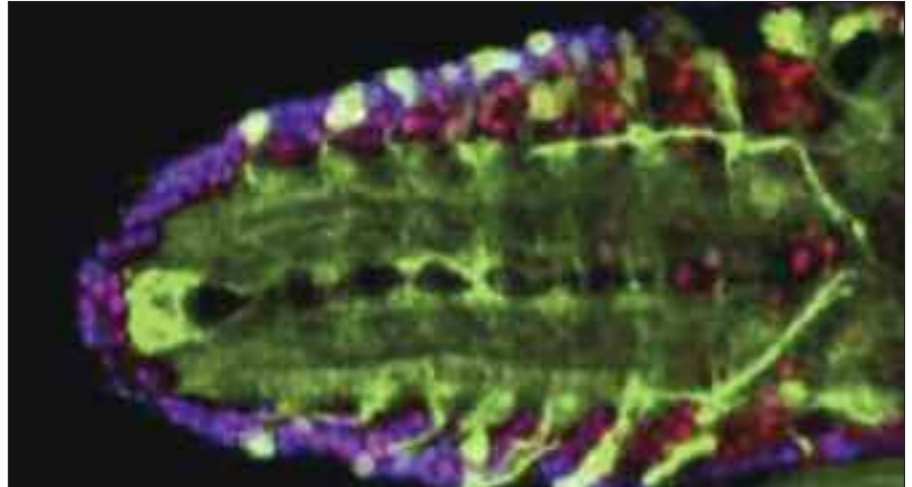
Sydney Horak

SMA Studies Conducted at Columbia University Published in Prestigious Journal *Cell*

Two studies by scientists in the McCabe, Pellizzoni, and Mentis laboratories at the Motor Neuron Center at Columbia University Medical Center (CUMC) suggest that dysfunction in motor circuits is critical in Spinal Muscular Atrophy (SMA), a genetic neuromuscular disease in infants and children. Families of SMA provided funding to the Mentis laboratory for this work in the first Audrey Lewis Young Investigator's Award. Both studies were published today in the online edition of the *Journal Cell*.

To study the cause of SMA, researchers led by the McCabe lab first worked with fruit flies that had been genetically altered so that every cell had a defective copy of the SMN1 gene. The flies' cells contained low levels of SMN protein, resulting in reduced muscle size and motor function, much as in humans with SMA. When fully functional copies of SMN1 were introduced into the flies' motor neurons or muscle cells, the flies unexpectedly showed no improvement. Only when SMN1 was returned to other motor circuit neurons—in particular, proprioceptive neurons and interneurons—were muscle size and motor function restored. While there may ultimately be differences between SMA mechanisms in flies and mammals, this work in flies should provide important clues to pathology in Spinal Muscular Atrophy in mice and humans.

In further experiments, the researchers demonstrated that in fruit flies with defective SMN1, proprioceptive neurons and interneurons do not produce enough neurotransmitters. When the flies' potassium channels were genetically blocked—thereby increasing neurotransmitter output—muscle size and motor function improved. The same effect was seen when the flies were given drugs that block potassium channels, suggesting that this class of drugs might help patients with SMA.



Motor circuits malfunction in lethal childhood disease (image of fly spinal cord equivalent). Brian McCabe, PhD/Columbia University Medical Center.

Supported by these findings, in July, the SMA Clinical Research Center at CUMC launched a clinical trial of a potassium channel blocker called dalfampridine (Ampyra) for the treatment of patients with ambulatory SMA. The study will assess whether the drug improves walking ability and endurance in adults with SMA Type III, compared with placebo. Claudia A. Chiriboga, MD, MPH, associate professor of Clinical Neurology at CUMC, is the lead clinical investigator.

Ampyra was approved by the FDA for the treatment of patients with multiple sclerosis in 2010. *“This drug is unlikely to be a cure for SMA, but we hope it will benefit patient symptoms,”* said Dr. McCabe. *“Other compounds at various stages of development hold promise to fix the underlying molecular problem.”*

The second study led by the Pellizzoni lab sought to determine how the loss of SMN protein—which is expressed in all cells—leads to the selective disruption of motor circuits. Working with models of SMA in mammalian cells, fruit flies, zebrafish, and mice, the researchers demonstrated that SMN1 deficiency disrupts a fundamental cellular process known as

RNA splicing with detrimental effects on the expression of a subset of genes that contain a rare type of intron. (In the process of RNA splicing, parts of RNA called introns are removed so a gene can be translated into protein.)

By studying the function of this group of genes affected by the loss of SMN1, the researchers discovered a novel gene—which they named stasimon—that is critically required for motor circuit activity in vivo. They further showed that restoring expression of stasimon was sufficient to correct some key aspects of motor dysfunction in both invertebrate and vertebrate models of SMA. Survival and correction of synaptic defects in mouse models of SMA have not yet been assessed.

“The potential added value of our study is that we’ve identified a novel gene that is targeted by the disease protein. When disrupted, this gene—stasimon—appears to contribute to the development of SMA in model organisms. The implication is that this gene and the pathway in which it functions might be new candidate therapeutic targets,” Dr. Pellizzoni added.

FSMA Translational Advisory Committee and Scientific Advisory Board Met in November 2012 to Evaluate New SMA Research Funding

Families of Spinal Muscular Atrophy is dedicated to creating a treatment and cure for Spinal Muscular Atrophy (SMA) by funding and advancing a comprehensive research program. In the fall, the FSMA SAB and TAC met at the national office to evaluate new research funding. The SAB assessed 37 basic research grant applications from around the world for funding. The TAC evaluated 7 drug discovery projects for SMA.



The organization is planning to award \$1.4 Million in new research funding over the next few months. This new round of research funding will be allocated into three areas: 1) Basic Research to understand the disease and provide ideas for drug making, 2) Drug Discovery to develop new SMA therapies, and 3) Clinical Research to help test new drugs effectively and to improve care for patients. This follows \$745,000 awarded to 7 basic research grants in 2011, and \$1.5 Million awarded in Spring of 2012 for two new drug discovery projects.

Funding for New Basic Research Grants.

This basic research provides fundamental information about what is going wrong in SMA, which then provides seed ideas for new ways of making drugs. Our current round of funding will

focus on proposals determining the functional role of SMN protein, finding the tissues where SMN protein is required, identifying new drug targets for SMA, and building new tools to facilitate SMA research.

Funding for New Drug Discovery Programs.

FSMA has been investing in and advancing drug research since 2000. The goal of drug discovery funding at FSMA is to build a large and diverse therapeutic pipeline to maximize the chances for success in finding a treatment for SMA. FSMA has been involved in funding half of all the ongoing SMA drug programs to date. New awards in the current round will focus on providing critical funding for early-exploratory projects assessing novel therapeutic approaches for SMA, or for specific aspects of current promising drug programs.

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

SHARING PHOTOS



Caleb Elmer



Maxwell Peppers



Lexi Richardson

Families of SMA Awards \$150,000 to Dr. Lee Rubin at Harvard to Advance a New SMA Drug Discovery Program

Families of SMA is dedicated to creating a treatment and cure for Spinal Muscular Atrophy by funding and advancing a comprehensive research program, including drug discovery programs to make practical new therapies. Dr. Rubin's project at Harvard University is the first of three drug discovery awards that will be given by Families of SMA in 2013.

Description of FSMA Funded Project:

A patient derived motor neuron screen to identify novel SMA Therapeutics.

Objective:

To identify novel SMA therapeutics by using patient-derived motor neurons for drug discovery and preclinical testing.

Research Strategy:

The researchers have made induced pluripotent stem cells from SMA Type I and Type II patient skin biopsies, and produced large numbers of motor neurons that can be used to model SMA. They will use these motor neurons in a screen for drugs that increase SMN levels. The researchers will test the candidate drugs in a 35 cell line panel that includes motor neurons from different patients and different SMA types.

Significance:

By using human motor neurons to screen for new SMA drugs, the researchers hope to identify compounds that can increase functional SMN levels in the cell type most affected by the disease. They will prioritize compounds that are already approved for use so they can move through the development pipeline more quickly. The researchers will test the candidate compounds across a panel of SMA patient-derived cells so that they can identify drugs that are effective across many genetic backgrounds and disease severities. The researchers hope that this innovative approach will lead to the

discovery of drugs that are mechanistically unique so that they may be used in addition to the therapies that are currently being developed. These cell lines can also help assess existing SMA drug candidates.

Earlier this year, FSMA's Translational Advisory Committee (TAC) met to evaluate research funding for new drug discovery projects for SMA. Families of SMA is planning to award three new drug discovery projects over the next few months.

This current award to the Rubin lab in The Department of Stem Cell and Regenerative Biology at Harvard University is for one year for \$150,000 with the goal of conducting cell-based screening in human motor neurons for new SMN inducing drug candidates. Subsequent funding to advance any newly identified molecules towards an Investigational New Drug application with the FDA will be evaluated by our TAC through our next planned funding cycle in Preclinical SMA Drug Development in 2014.

The SMA Drug Pipeline:

We now have 13 SMA drug programs in development, including 3 in clinical trials. This pipeline has expanded from just 4 programs 5 years ago. Families of SMA has funded half of all the ongoing drug programs for SMA. Our research approach funds programs at early stages, and then partners with companies to take them through clinical trials. Supporting multiple programs gives different approaches for a SMA therapy, which increases the chances of success and accelerates the timeline to a treatment and cure.

Families of Spinal Muscular Atrophy committed \$2.5 Million to funding SMA Research in 2012 with more planned in 2013. Our research progress provides us all with hope that one day we will live in a world without SMA.



Dr. Rubin's Profile:

Who are you?

I received my PhD in Neuroscience from The Rockefeller University and completed postdoctoral fellowships in Pharmacology from Harvard Medical School and in Neurobiology from Stanford University School of Medicine. I have worked both in academia and in industry, first as a Project Leader at Athena Neurosciences (now Elan Pharmaceuticals) and later as Chief Scientific Officer of Curis, a Cambridge biotechnology company. At Curis, I directed a project that identified the first small molecule hedgehog antagonists. One of these, Erivedge, was recently approved by the FDA to treat advanced basal cell carcinoma.

What is your current role in SMA Research?

I am currently Professor of Stem Cell and Regenerative Biology at Harvard University and Director of Translational Medicine at the Harvard Stem Cell Institute. Much of my effort is devoted to identifying therapeutics for orphan neural disorders such as Spinal Muscular Atrophy and Amyotrophic Lateral Sclerosis, using new kinds of stem cell-based screens. My lab also explores different chemical biology approaches for manipulating cell fate. Some of this work has been published recently in *Cell*, *Cell Stem Cell*, *Nature Chemical Biology*, *Developmental Biology*, and *Science*.

Also, working on the project is Dr. Maureen Lynes in the Rubin Laboratory. Dr. Lynes received her PhD in Cellular and Molecular Physiology at Tufts Medical School, and joined the Rubin laboratory as a post-doctoral fellow in 2011. Since joining the lab, she has been interested in using SMA patient iPS cells as a tool for identifying SMA therapeutics as well as for studying SMA pathogenesis.

Funding for New Drug Discovery Programs

FSMA has been investing in and advancing drug research since 2000. The goal of drug discovery funding at FSMA is to build a large and diverse therapeutic pipeline to maximize the chances for success in finding a treatment for SMA. FSMA has been involved in funding half of all the ongoing SMA drug programs to date. New awards in the current round will focus on providing critical funding for early-exploratory projects assessing novel therapeutic approaches for SMA. The FSMA research funding philosophy is based on expert and independent oversight of research projects. This approach ensures that FSMA funds only the most promising research, and that funded projects are run in a professional and efficient manner under the guidance of world-class experts.

Families of SMA Nominates Spinal Muscular Atrophy as a Disease for FDA's Patient-Focused Drug Development Initiative.

Families of SMA has submitted formal comments to the FDA urging the agency to include SMA on the list of diseases that will receive a public meeting as part of the FDA's Patient-Focused Drug Development. The comments make clear to the FDA that the severity of SMA and the tremendous progress made towards developing a robust therapy pipeline positions SMA as a perfect candidate to benefit from the new Patient-Focused Drug Development program.

The U.S. Food and Drug Administration (FDA) is implementing a new Patient-Focused Drug Development initiative that aims to obtain the patient perspective for a set of disease areas that currently do not have any FDA-approved therapies. As part of this initiative, the FDA will conduct public meetings on 20 different disease areas over the next five years to discuss the impact of the disease on patients, the spectrum of severity for those who have the disease, the measures of benefit that matter most to patients, and the adequacy of the existing treatment options for patients. The meetings will include participation of FDA staff, the relevant patient advocacy community, and other interested stakeholders.



Families of SMA and Nationwide Children's Announce Multi-Million Dollar Award from NINDS to Advance CNS Gene Therapy for Spinal Muscular Atrophy

Nationwide Children's Hospital (Columbus, OH) and Families of Spinal Muscular Atrophy (Elk Grove Village, IL) announce the award of a multi-million dollar cooperative agreement from the National Institute of Neurological Disorders and Stroke (NINDS) to advance a gene therapy development program for Spinal Muscular Atrophy (SMA).

This three-year multi-million dollar cooperative agreement to Brian Kaspar, PhD, principal investigator in the Center for Gene Therapy at The Research Institute at Nationwide Children's Hospital in the amount of \$3,752,462, funds pre-clinical drug development up to the filing of an Investigational New Drug Application (IND) to the Food and Drug Administration (FDA). This agreement represents an innovative collaboration between Government, Advocacy and Academic groups to advance a promising new therapy for SMA.

In May 2012, Families of SMA (FSMA) announced the award of up to \$750,000 to Dr. Kaspar. This ongoing award supports the preclinical development of a Central Nervous System (CNS)-delivered gene therapy for SMA. Direct CNS delivery likely allows for less virus to be used, which significantly increases the likelihood that older and larger SMA patients can be treated with gene therapy. With the funding from FSMA, Dr. Kaspar's team initiated studies to jumpstart the research prior to obtaining government and later commercial involvement. This cooperative award from the NINDS will now support advancing the program to the point of human clinical trials. The program will be evaluated using quantitative go/no-go milestones, determined by Nationwide Children's and NINDS.

SMA is an often-fatal genetic disorder resulting from the loss of both copies of the Survival Motor Neuron (SMN1) gene. This causes a chronic deficiency in the production of the SMN protein, which is essential to the proper functioning of the motor neurons in the spinal cord to the control of muscles in the limbs, neck and chest. SMA is typically marked by the deterioration of the muscles that control crawling, walking, swallowing or breathing. There are no

approved therapies for the treatment of SMA. Approximately 1 in 6,000 babies born is affected. One in 40 people, or approximately 8 million in the United States, are genetic carriers of the disease.

Gene therapy is an approach to treating diseases by replacing faulty genes. In the case of SMA, the most direct approach for a gene therapy is to replace the mutated SMN1 gene. In the past, the challenge with gene therapy for SMA has been to find a way to deliver the genetic material efficiently to motor neurons. In recent years, Dr. Kaspar's group was the first to demonstrate Adeno-Associated Virus 9 (AAV9) targeted motor neurons effectively. Administration of AAV9-SMN into one day-old SMA mice resulted in increased SMN protein levels in motor neurons, correction of synaptic function, and a significant extension of life span.



"At Families of SMA we are extremely pleased that our initial investment at an early stage of this program has provided the preliminary data to leverage larger funding from the NIH. We feel this grant award is positive validation of the Families of SMA research funding and partnering strategy, as well as for this approach for gene therapy in SMA," said Jill Jarecki PhD, Research Director at Families of SMA. *"The Families of SMA funding strategy for preclinical drug development is to invest seed funds to begin early-stage programs for SMA. As programs advance, we look for funding to transition from non-profit to government and commercial sources."*



"My research team at Nationwide Children's Hospital is excited to advance this promising cerebrosinal fluid delivery approach of AAV9-SMN to the clinic for SMA patients and we are extremely grateful to FSMA and NINDS for the support of this important work," said Dr. Kaspar, also a faculty member at The Ohio State University College of Medicine. *"We stand committed to bring SMA experimental therapeutics to the clinic in the most rapid and safe manner."*



"Development of therapies requires collaboration of academics, advocacy, industry, and government—no single party has the resources to do this alone. The collaboration between Dr. Brian Kaspar, Families of SMA, and the NIH is an exciting model in leveraging resources and expertise in the hope of accelerating therapy development for SMA," said Dr. John Porter, PhD, Program Director at the National Institute of Neurological Disorders and Stroke.

About Nationwide Children's Hospital:

Ranked 7th of only 12 children's hospitals in U.S. News & World Report's 2012-13 "America's Best Children's Hospitals" and among the Top 10 on Parents magazine's 2013 "Best Children's Hospitals" lists, Nationwide Children's Hospital is one of the nation's largest not-for-profit freestanding pediatric healthcare networks providing care for infants, children, adolescents and adult patients with congenital disease. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's Hospital faculty train the next generation of pediatricians, scientists and pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational and health services research at Nationwide Children's Hospital. In June 2012, the hospital celebrated completion of the largest pediatric expansion project in United States history including the addition of a third research building. All three research buildings include approximately 525,000 square feet dedicated to research. More information is available at NationwideChildrens.org/Research



Spinal Muscular Atrophy Researchers Participate at The 2012 Society of Neuroscience Meeting

The Society of Neuroscience (SfN) Meeting is the premiere neurobiology meeting annually with over 30,000 scientists attending. In 2012, multiple presentations on Spinal Muscular Atrophy were given by scientists funded by Families of SMA. There were two-dozen presentations on SMA research given at the meeting. These presentations include work from the labs of past and current FSMA funded researchers, such as:

Dr. Giancomio Comi of the University of Milan, Dr. Christian Lorson of the University of Missouri, Dr. Wilfried Rossoll of Emory University, Dr. Chien-Ping Ko of University of Southern California, Dr. Umrao Monani of Columbia University, Dr. Charlotte Sumner of Johns Hopkins University, Dr. Laxman Gangwani of Texas Tech University, Dr. Utz Fischer of University of Wurzburg, and Dr. Christopher Henderson of Columbia University.

Presentations included:

Astrocyte-specific restoration of SMN significantly decreases disease severity in a severe model of Spinal Muscular Atrophy. *H. Rindt, Z. Feng, J. J. Glascock, C. Mazzasette2, C.-P. Ko, C. L. Lorson.*

In vitro investigation of the glial contribution to Spinal Muscular Atrophy. *Z. Feng, C. Mazzasette, C. L. Lorson, C.-P. Ko.*

Selective restoration of SMN in motoneurons in two severe SMA mouse models. *X. Paez, B. L. Seaberg, M. J. Rimer.*

Quantification of full length and exon 7 skipped SMN mRNAs in human tissues using transcriptome sequencing database. *J. Zhou, X. Yang, X. Gao, R. Qin, H. Shen.*

Overexpression of hnRNP-R rescues axon pathology in Smn-deficient motoneurons. *R. Sivadasan, C. Drepper, T. Herrmann, C. Simon, C. Valori, M. Azzouz, M. Sendtner.*

SMN in motor neurons determine synaptic integrity in Spinal Muscular

Atrophy. *T. L. Martinez, L. Kong.*

Genetic analysis of SMN loss of function in invertebrate models. *A. C. Hart, M. Walsh, M. Dimitriadi.*

Rescue of the Spinal Muscular Atrophy phenotype in a mouse model by unmodified and octa-guanidine-conjugate morpholino oligomers. *M. Nizzardo, C. Simone, F. Rizzo, S. Salani, I. Faravelli, C. Zanetta, M. Falcone, M. Ruggieri, N. Bresolin, G. P. Comi, S. Corti.*

The JNK signaling mediate neuron degeneration in Spinal Muscular Atrophy. *L. D. Gangwani, S. Ahmad, C. A. Gabaldon, G. Zhu, R. J. Davis.*

Muscle cytochrome c oxidase defect and mitochondrial DNA depletion in Spinal Muscular Atrophy. *D. Ronchi, D. Vallejo, M. Ripolone, V. Melzi, G. Fagiolari, V. Lucchini, R. Violano, A. Bordoni, C. Lamperti, L. Villa, S. Corti, U. Balottin, N. Bresolin1, M. Sciacco, A. Berardinelli, M. Moggio, G. P. Comi1.*

Possible mechanism underlying impairments in axonal mRNA localization in Spinal Muscular Atrophy motor neurons. *C. Fallini, P. G. Donlin-Asp, G. J. Bassell, W. Rossoll.*

Targeted gene correction of Spinal Muscular Atrophy-induced pluripotent stem cells and motor neurons as a model and for cell therapy. *S. Corti, M. Nizzardo, C. Simone, M. Falcone, M. Nardini, D. Ronchi, C. Donadoni, S. Salani, G. Riboldi, F. Magri, F. Rizzo, G. Menozzi, C. Bonaglia, N. Bresolin1, G.*

Altered astrocyte function in both in vitro and in vivo models of Spinal Muscular Atrophy. *T. N. Patitucci, J. V. MCGivern, J. A. Nord, M. E. Barabas, C. L. Stucky, A. D. Ebert.*

Astrocyte mediated toxicity leads to motor neuron death in Spinal Muscular Atrophy. *S. Phani,2, A. Jacquier, D. Papadimitriou, D. Re, S. Kariya, U. Monani, S. Przedborski.*

Autophagy is involved in motoneuron degeneration in a mouse model of SMA.

A. Piras, L. Schiaffino, M. Boido, V. Valsecchi, M. Guglielmo, E. Tamagno, A. Vercelli.

Development of SMA mouse model plasma protein biomarker assays. *K. Mccarthy, D. T. Kobayashi, P. G. Zaworski, R. A. Poorman, D. E. Decker, J. Page, B. Nawa, S. Paushkin, K. S. Chen.*

Using a novel mouse model of intermediate SMA to predict patient drug response: How a co-clinical trial in mouse models can help optimize therapeutics. *M. A. Osborne, L. Bogdanik, C. Davis, K. Wardwell, W. Andrews, C. Lutz.*

Motor pool selectivity of neuromuscular degeneration in mouse and human Spinal Muscular Atrophy (SMA). *J. C. Lee, D. Papandrea, K. C. Kanning, W. K. Chung, D. J. Pisapia, A. P. Tenney, C. E. Henderson.*

Differential expression and altered splicing of neuexin, a presynaptic protein, in Spinal Muscular Atrophy. *P. Yadav, K. Z. See, R. Blum, M. Giegerich, U. Fischer, S. Mathavan, M. Sendtner, C. Winkler.*

Modulation of ERK and AKT signaling pathways alters SMN expression in Spinal-Muscular-Atrophy-like mice. *J. Branchu, O. Biondi, T. Collin, F. Leroy, C. Pariset, P. Lopes, C. Massaad, C. Chanoine, F. Charbonnier.*

Generation of an expandable and stable population of human motor neuron precursors derived from induced pluripotent stem cells for high content screening/imaging. *D. Sareen, L. Ornelas, A. Sahabian, M. E. Simpkinson, S. Grant, S. Bell, R. H. Baloh, C. N. Svendsen.*

Different types of exercise induce different effects in SMA-like mice. *F. Chali, B. Estournet, F. Charbonnier, O. Biondi.*

Building cellular circuits in vitro using iPSCs for neurodegenerative disease modeling. *J. V. MCGivern, T. Hosoyama, B. M. Heins, M. Suzuki, A. D. Ebert.*

NIH's Recombinant Advisory Committee (RAC) Approves Infant Trial for Systemic AAV9-SMN Gene Therapy for SMA. Team Plans to Submit IND to the FDA in 2013

In December 2012, the Kaspar Team at Nationwide Children's Hospital presented their AAV9-SMN gene therapy program for Spinal Muscular Atrophy to the NIH RAC today. In the coming months, the team will also submit an IND to the FDA for their approval to begin human clinical trials. FSMA funding is helping move this program into older and bigger patients with SMA.

The proposed trial would likely include infants up to six month old, who are non-ventilator dependent with a confirmed diagnosis of SMA Type I. A total of 6-10 patients will be enrolled over a period of time.

In 2010, FSMA awarded Dr. Brian Kaspar \$100,000 for "Optimizing Titer and Window of Opportunity for Targeting Motor Neurons via an AAV9 Vector in Newborn Non-human Primates". This work helped generate data included in the TAC review

Families of SMA has recently funded a new arm of Dr. Kaspar's project to help move AAV-SMN gene therapy into older and bigger children with SMA, in addition to infants. In May 2012, Families of SMA announced the award of up to \$750,000 for an important new grant to Dr. Brian Kaspar at Nationwide Children's Hospital. This award will support preclinical development of a CNS-delivered Gene Therapy for Spinal Muscular Atrophy. With funding from

FSMA, Dr. Kaspar's team will initiate the studies needed for an Investigational New Drug (IND) application for this therapy to the Food and Drug Administration (FDA).



"The direct CNS approach opens the possibility for treating older and larger patients. We are quite excited by our data, as well as the data of other multiple groups, on the power of a CNS directed gene delivery approach to increase SMN levels. This is extremely important funding from FSMA to allow us to collect additional pre-clinical data for a CNS delivered AAV gene delivery into the cerebrospinal fluid, which will be important information to present to the FDA. It also jump starts research prior to obtaining government and commercial involvement which we are actively pursuing. We are quite hopeful for a positive funding decision on a recent NIH proposal for co-funding of this project with FSMA." Brian Kaspar, PhD, Associate Professor, Principal Investigator The Research Institute at Nationwide Children's Hospital, The Ohio State University.

In addition, Families of SMA funded the start-up of the SMA clinic at Nationwide Children's Hospital where this trial will occur.

The RAC committee is just one of several regulatory steps that need to be completed to begin a human gene therapy trial. The second is submission of an Investigational New Drug applications or IND to the FDA. An IND is a request to begin human clinical trials to the US government agency called the FDA, which by law oversees human drug research. Like the RAC, the FDA will require to see all data that has been collected on the program. Data is required on:

- 1) Efficacy (evidence the drug works in animal or cellular models of the disease)
- 2) Bio-distribution (where the drug goes in the body)
- 3) Animal safety studies (typically 6 to 9 months of drug exposure in duration)
- 4) Manufacturing process

An IND can literally be thousands of pages long. Once the IND is submitted, the FDA has 30 days to respond.

Nature Medicine Publishes “Targeted Therapies for SMA: Call in the Backup”

The most common genetic killer of infants, a disease known as Spinal Muscular Atrophy, is caused by mutations in a single gene. The human genome contains its own backup system—near-identical copies of the defective gene—yet these secondary sequences rarely get used correctly. As Elie Dolgin finds out in this month’s issue of *Nature Medicine*, drug companies hope to change that, with the first wave of targeted therapies that reboot the body’s backup system now entering clinical trials.

Biological Strategies for SMA Drugs

SMN2 differs from the normal, functioning version of SMN1 by just a single nucleotide change: in SMN2, the sixth letter of the gene’s seventh coding region, known as exon 7, is a T rather than a C. That small difference has big consequences, though. The altered DNA letter usually causes the RNA processing machinery to splice out exon 7, leading to the creation of a shortened, less stable version of the SMN protein that rapidly degrades. Only around 5–10% of the time does the exon get left in place to yield a full-length, functional protein.

The more copies of SMN2 people with SMA carry, the more full-length protein they produce and the less severe their disease. One or two copies usually results in SMA Type I, the most common and deadly form of the disease. Around 60% of all children with SMA are in this category. They never develop the ability to sit, stand or walk, and most will die under the age of 2. People with SMA Type II or Type III typically have at least three copies of

SMN2, and, despite physical and respiratory disabilities, these individuals can live into early adulthood and beyond. Then there are rare individuals who harbor four or more copies of SMN2. Despite lacking a functional version of SMN1, these people have much milder forms of the disease and, in some cases, show practically no symptoms at all.

The dream for scientists, physicians and pharmaceutical companies alike is to bring more people with SMA effectively into this last category by helping affected children make the most of the SMN2 genes they already have. A number of different strategies are being pursued, including small-molecule drugs and anti-sense therapies, all with the same goal in mind: to therapeutically boost the levels of full-length SMN protein. “*Nature gave us this second copy [of SMN] that can make a little bit of full-length protein,*” says Basil Darras, director of clinical neurology at Boston Children’s Hospital and a site coordinator in the Isis trial. “*And now everybody is trying to develop*

drugs that will induce more full-length SMN protein production from the SMN2 gene.”

The predominant therapeutic strategy for treating SMA has focused on using what’s already there in the genome. Take the SMN2 gene and make it work better, the reasoning goes. But another tactic involves putting back what’s missing. The SMN2 gene doesn’t work all that well anyway. Another approach is to re-introduce fully functioning copies of SMN1 instead, using modified viruses for therapeutic gene deliver.

Overview of Current SMA Drug Programs

The article then goes on to discuss the companies and groups working on SMA drug therapies, including Isis and their ongoing clinical trials for ISIS-SMNRx, Novartis, Roche, Paratek, Repligen, the NINDS SMA Project, and finally the gene therapy projects of Dr. Brain Kaspar, Dr. Martine Bar kats, and Genzyme.

Linig up against a genetic killer: The SMA drug pipeline.

Mechanism	Developer	Drug type (name)	Phase
SMN splicing	Isis Pharmaceuticals & Biogen Idec	Antisense oligonucleotide (ISIS-SMNRx)	1
	Roche & PTC Therapeutics	Small molecule	Preclinical
	Novartis	Small molecule	Preclinical
	Paratek Pharmaceuticals	Small molecule	Preclinical
SMN translation	Repligen	Small molecule (RG3039)	1
SMN stabilization	NINDS	Small molecule (ALB-111)	Preclinical
SMN gene therapy	Genzyme	Viral vector	Preclinical
	Genethon & INSERM	Viral vector	Preclinical
	Nationwide Children’s Hospital	Viral vector	Preclinical
Neuroprotection*	Trophos	Small molecule (desoxime)	2

INSERM, Institut National de la Santé et de la Recherche Médicale; NINDS, National Institute of Neurological Disorders and Stroke.

*Desoxime, a mitochondrial pore modulator, is designed to protect neurons but does not affect SMN levels.

Isis Initiates Safety, Tolerability and Dose-Range Finding Study of Multiple Doses in Patients with Spinal Muscular Atrophy

This is an open-Label, dose escalation study to assess the safety, tolerability, and dose-range finding of multiple doses of the drug delivered intrathecally to 24 patients with SMA. It follows up on a single dose safety study completed earlier in 2012. The clinicaltrials.gov identifier is NCT01703988.

This study will test the safety, tolerability, and pharmacokinetics of escalating doses of ISIS-SMNRx administered into the spinal fluid either two or three times over the duration of the trial, in 24 patients with Spinal Muscular Atrophy. Three dose levels will be evaluated sequentially. Each dose level will be studied in a cohort of 8 patients, where all patients will receive active drug. The primary outcome measures will be the number of participants with adverse events, where the participants will be followed for the duration of the study, expected to be 36 weeks. The secondary outcome will be measurements of drug levels in the plasma and the CSF.

Isis Pharmaceuticals exclusively licensed intellectual property from the University of Massachusetts to develop this new drug for Spinal Muscular Atrophy. Families of SMA provided over \$500,000 in funding support for the University of Massachusetts' research program responsible for creating this intellectual property.

The Isis antisense therapeutic approach for SMA involves the use of a short, chemical structure, called an antisense drug, to increase the production of the protein, SMN. SMN protein is reduced in SMA and is associated with normal motor function. SMN2 is a closely-related gene that normally produces a truncated and low-functioning form of SMN protein. Isis designed an antisense drug that binds to the SMN2 RNA and drives the production of SMN protein. Using this approach, Isis hopes to provide therapeutic benefit to patients with SMA.

Splicing is a normal mechanism that the cell uses in order to produce many different, but closely related proteins from a single gene by varying the processing of the RNA. It is estimated that of the approximately 25,000 genes in the human genome, approximately 90% have alternative splice forms. In some cases, alternative splicing of RNA results in the production of proteins that are involved in disease.

NINDS Releases Update on the SMA Project

Update Synopsis:

In pursuit of its mission to reduce the burden of neurological disease, the National Institute of Neurological Disorders and Stroke (NINDS) established the Spinal Muscular Atrophy (SMA) Project, a networked contracts program to accelerate the development of therapeutic candidates for SMA. SMA is a neurodegenerative disease with variable severity ranging from limited motor neuron loss and normal life expectancy (Type III) to progressive infantile paralysis and death (Type I).

Early on, the SMA Project decided to focus on small molecule drugs, rather than gene therapy, stem cells or biologics. Indoprofen was chosen as the starting point for a medicinal chemistry program after a systematic review of FDA approved drugs that had demonstrated activity in SMA-relevant assays and models.

The Project adopted a multi-tiered system for compound synthesis and testing, which is referred to as a drug development "testing funnel" because the number of compounds decreases as the testing for desired drug properties advances. The goals of the medicinal chemistry optimization effort for indoprofen included increasing the potency and brain penetrance of the drug and eliminating its inhibitory activity on cyclooxygenase, which had previously been found to be a liability for clinical use.

Over a 5-year-period, the SMA Project synthesized and screened more than 1,400 analogs of indoprofen. Of the compounds screened in tier 1 assays, over 150 were selected for further characterization and advanced to the next tier of the testing funnel. This included several assays to measure increases in SMN protein levels in patient fibroblasts, and assessments of compound bioavailability, genotoxicity and safety profiles.

Starting in July 2012, the NINDS initiated discussions with several pharmaceutical and biotechnology companies to determine whether ALB-111 meets their technical requirements as a late-stage lead compound, and if so, to identify a partner to conduct the next steps of preclinical drug development.

See http://www.ninds.nih.gov/funding/areas/translational_research/sma_project/index.htm to for the entire update from NINDS on the SMA Project.

Congratulations



Congratulations to Chloe Ochoa, of Illinois who has SMA Type III, for winning three 1st place ribbons, at the Therapeutic Riding Show, in walk, trail and costume class!



Congratulations to the Butler Family who will be expecting their newest addition, Baby Will, in May 2013!

■ Congratulations to Brian, Stephanie, Caden and Cole Geraghty on the birth of their new baby girl, Hailey Grace born on October 31, 2012!

■ Congratulations to the Woodruff family on their beautiful baby girl, Mattie McNeil, born on September 24, 2012!



■ Congratulations to Liz and Michael TerHorst on the birth of their new daughter, Evelyn Kathleen, born on September 24, 2012!



■ Congratulations to Debbie, Ron, Dylan and Heather Cuevas on the birth of their new addition, Nicholas John, born on October 24, 2012!



■ Congratulations to Tony, Kristin and big brother, Jadon Burks, on their beautiful baby girl, Gracyn Jaye, born on December 14, 2012!

■ Congratulations to Dannete and Sean Lyden on the birth of their new baby girl, Zoey Julianna, born on January 4, 2013!

■ Congratulations to Tanya and Tim Krajewski on the birth of their new daughter, Olivia Blake, born on January 5, 2013!

■ Congratulations to Peter Rider and Glen Hill on their new baby boy, Calvin!

■ Congratulations to Marge Shively on receiving the Los Altos Hills Joint Community Volunteer Service Award for her advocacy in the fight against Spinal Muscular Atrophy!



Bryce Amiel



Ariana Dinzans



Austin and Jeff Bixler



Anna Landre



Brayden and Logan Janetzke



Aubriana Navarro



Aviv Taskar



Randy Jr. Eunice



Alexis Helfrich



Brett Wilson



Ariel Barker



Andrew and Patrick Murray



Alyssa Krider and Pat Stevenson



Ali Williams



Zander and Alexis Helfrich



Ella and Rileigh Ecker



Emma Banach



David Dalton Hendrix



Brandon Russell



Ella Savarese



Emma and Mabel Cannady



Caitlin and Grace Trainor



Zion Hoskins



Ellie Kerrigan



Chloe Ochoa



Charlie Sykora



Anna Rose Scurria



Eloise, Sam and Jamilla Pillarella



Eloise Pillarella with Claire

The Families of Spinal Muscular Atrophy Medical Advisory Council Meets to Advance SMA Patient Care and Family Support

Members of the Families of SMA Medical Advisory Council (MAC) attended two days of meetings on December 7th and 8th at the FSMA National Office. This 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, a leading pediatric pulmonologist serves as 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.

This productive meeting covered important Patient Service and Family Support topics, including:

- The agenda, speakers and logistics for the upcoming 2013 Continuing Medical Education Conference for medical providers.
- The entire process of support for newly diagnosed SMA families when they are connected with Families of SMA including the FSMA Information Binders.
- An evaluation of 2012 Annual SMA Conference workshops based on feedback and surveys.
- The agenda and speakers for the Newly Diagnosed Program at the 2013 Annual SMA Conference, as well as finalizing all conference workshops.
- An analysis of the options for FSMA funding of new patient care research.
- An evaluation of current SMA Care Series Booklets, as well as potential future booklets.
- Review of items currently available in the FSMA Equipment Pool, as well as potential future items to include.



The Families of Spinal Muscular Atrophy Medical Advisory Council 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 Medical Advisory Council, who all volunteer their time to be a part of the MAC, was formed in June 2009.

The MAC is composed of experts in the following fields:

Neurology (pediatric and adult); Pediatrics; Pulmonology; Pediatric Orthopedic Surgery; Pediatric Critical Care (NICU specialist); Physiatry (rehab medicine); Psychology; Genetic Counseling and/or Medical Ethics; Physical Therapy; Occupational Therapy; Respiratory Therapy; Nursing.

In the last year, Families of SMA has provided direct services to over 3,600 SMA patients and families. These services include medical equipment, informational packets, newly diagnosed care packages and medical provider informational packets.

Loving Memories

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



Photo of: Tonya Willingham and her daughter, Hanna

IN MEMORIAM

Charles "Logan" Patrick

October 11, 2006 – December 22, 2012



When our son Logan was diagnosed with Spinal Muscular Atrophy Type I at 5 ½ months old on March 27, 2007, our world came to a screeching halt. We had never heard of SMA like most parents. All we heard the doctors say at first was that our baby probably wouldn't live to see his 2nd birthday. We decided to do our own research and reach out to other SMA families and Families of SMA. We learned so much from other parents. We learned how to take care of Logan mostly from other SMA families. What a blessing! Families of SMA has been a great supporter of our family as well.

Logan truly beat the odds every day. Each day was truly a blessing with him. When he was diagnosed, we knew his journey and mission on Earth would be short. We asked God to use his testimony to win lives for Him. Logan loved life and taught so many people about courage, hope and strength. He moved mountains with his strength without moving a muscle. Despite his physical limitations, his mind was not affected, and he was very bright like most SMA kids. He was reading on a second grade level in kindergarten. Logan started kindergarten in August, 2012. He was mostly homebound, but we took him to school for a few hours a week so he could experience "school". He loved his classmates and loved showing off. He had all the girls wanting to do things for him. He had a whole conversation with one of his "girlfriends" by moving his eyebrows up and down!

Even though Logan lived only 6 years and 2 months, he experienced so much in his lifetime. He enjoyed going to the beach, Disney World, camping, concerts, participating in a 5K race, going to the zoo and the aquarium, swinging and jumping on the trampoline in the backyard, tailgating at and attending University of Alabama football games, etc... He also enjoyed riding in a golf cart, driving a pontoon boat, and playing baseball with Miracle League of Tuscaloosa.

Logan inspired us to keep fighting the fight against SMA every day. He is now walking hand-in-hand with Jesus in Heaven and flying high with all the other angels. Logan is doing all the things he's never been able to do here on Earth. He is completely healed now. We look forward to the day when we'll see him again! Logan still inspires us to keep fighting the fight for all those precious babies still fighting SMA. Fly high, Angel LoLo! We love you and miss you so much!

Logan's Little Life

Logan was a little man, well beyond his years.

He laughed and played, and shed very few tears.

He lived life to the fullest, a little daring was his way.

When rolling downhill, "Let me go!" he would say.

God saw he was getting tired and the cure was not to be.

So he put his arms around him and whispered, "Come with Me."

He touched each and every one of us somewhere deep inside.

But now he's up in Heaven, running by Jesus' side.

Think of him when you see a twinkling star, He will always be with us, not very far.

Logan is now at rest. God only takes the best.

Love,
Mommy, Daddy, and Sister Landry

"But those who hope in the Lord will renew their strength. They will soar on Wings like eagles; they will run and not grow weary, they will walk and not be faint." Isaiah 40:31



IN MEMORIAM
JULIUS TRIMBAK TELANG
JUNE 7, 2012 – JANUARY 2, 2013



IN MEMORIAM
ISAIAH DENNIS
APRIL 27, 2011 – DECEMBER 12, 2011



IN MEMORIAM
JESSICA IRENE FERNANDES
JANUARY 5, 2002 – DECEMBER 27, 2005



IN MEMORIAM
KAL WARRINGTON SILVERT
JANUARY 1, 1990 – SEPTEMBER 3, 2012



IN MEMORIAM
ANGELINA ROSE COX
JULY 16, 2012 – JANUARY 9, 2013



IN MEMORIAM
COREY COX
AUGUST 24, 2012 – NOVEMBER 13, 2012



IN MEMORIAM
Brian Sawyer Smith
*DECEMBER 31, 2001 –
SEPTEMBER 19, 2002*



IN MEMORIAM
ERIN EILEEN HEALEY



IN MEMORIAM
AVERY MANFULL
AUGUST 31, 2012 – OCTOBER 7, 2012



IN MEMORIAM
GAVIN RIVERS
JULY 30, 2012 – DECEMBER 31, 2012

IN MEMORIAM

Lorelle Z. Burkholder

DECEMBER 22, 2010 – MARCH 31, 2011



*Oh, Beautiful Baby...
Sweet hands will not hold
Sweet lips left untold.
Oh, Baby, Our Child...
Lying peacefully still
A spot in our hearts
you always will fill.
Rest, Little Angel...
On wings soar away
For we shall see you
In heaven one day.*

Our baby daughter was diagnosed with SMA at eight weeks old. She was our 10th child and our sixth daughter. This was our first child to have this genetic disorder. Our prayer was to have a healthy child, one that can play with her siblings. But God saw it best this way. So we want to say, "Thy Will Be Done". We know God is good in all his ways although we do not understand. She was such a beautiful and precious baby to her family. We treasure her big smile she often gave us. And loved to watch her eyes when she laid under her crib mobile. Her eyes told us how much she would have loved to move. God has her in his keeping. We have her in our hearts.

Her Family,

Ralph & Arlene Burkholder and family of New York
Karen 22, Roseann 20, Delton 18, Carolyn 15, Loren
13, Darlene 10, Nathan 8, Jaden 5, Janette 3



IN MEMORIAM

PETE NOLIN

JULY 11, 2010 – JANUARY 2, 2013

Dear Families of SMA,

Thank you for your sympathy and support, and thank you for the card, book and Willow Tree angel's embrace you sent upon our son, Pete's passing. We will be eternally grateful for the care package you sent upon his diagnosis in March 2011 as well and particularly the sheep skin that was enclosed in that care package. Pete slept on it every night and it really did a lot to keep him more comfortable as he got weaker.

Sincerely,

Daniel, Janna & Gus Nolin



IN MEMORIAM
ELISE CLAIRE ENGLE
JANUARY 2012 – JULY 2012



IN MEMORIAM
BROOKE BINNING
JUNE 11, 1998 – DECEMBER 18, 2002

IN MEMORIAM
Dylan Richard Speece
OCTOBER 2010 – OCTOBER 2012

To the entire staff at FSMA:

I am sending this check for the annual campaign in memory of my son, Dylan Richard Speece (Type I), who passed away on October 31 at age 2. The donor is a private k-8 school where a few of my friends work. You likely will have received quite a few donations by now in memory of Dylan, since we encouraged our friends and family to donate in lieu of sending flowers.

I wanted to take this opportunity presented by my rare use of snail mail to thank the entire staff of FSMA for the wonderful support programs from which my family has benefited. The newly diagnosed care package, the informational booklets (the respiratory one was a God send to me and my nurses), the beautiful book and angel you sent after Dylan's passing, and all the information about ongoing research all made us feel informed, a little more in control of an uncontrollable situation, and as though we were indeed part of a family – not one that we had

ever anticipated joining, but one that we know we could turn to if we had questions. I still keep in touch with Kelly Coggin, our chapter president and think she did a wonderful job of representing this great organization, too.

While we mourn over Dylan in the near term, all I can offer is my gratitude and hopefulness that a cure is not far down the road for all our families. Down the road when I am stronger, I hope that perhaps I too can help other families.

My family thanks you from our hearts.

Regards,

Teri Speece of Texas
Mom to Colin 7, (unaffected by SMA) and Dylan our SMA
Angel forever 2



IN MEMORIAM

Sierra Pearl Gayhart

December 11, 2006 – January 13, 2007

Sierra Pearl Gayhart was born four weeks early by emergency C-section at Elkhart General Hospital because her heart rate was really low. To give a little background, we had gotten a clean bill of health at the start of my third trimester after a long first and second. At her first appointment (at 12 weeks), they found a cyst on the back of her neck and diagnosed her with Cystic hygroma, so back and forth to IU Med Center we went and after an amniocentesis and many ultrasounds and tests, the cyst miraculously disappeared! We were on the moon about it until the next ultrasound showed her umbilical cord on her neck so then it was stress tests twice a week. She never really moved which was worrisome to me as my sister and I were pregnant at the same time and my experience was so different than hers; now I know why. During my last stress test my doctor didn't like how they had been going so he sent me to the hospital to get a biophysical ultrasound and the results must not have been good

because he came in and said she looks ok but to be safe, we are just going to take her! I was sick, as far as we knew she was healthy but something told me otherwise, I was just so worried about her and if she'd be ok! Well, when she was born, she was blue and floppy and the whirlwind of craziness started. Luckily, they did their best and got her intubated after the team from Memorial South Bend arrived to take her to their hospital. I got to see her briefly and then three days later, finally got to hold my Pearly girl! She was at Memorial S. Bend for 2.5 weeks and then sent down to Riley Children's Hospital for further testing as they just couldn't find what was wrong with her. She not only didn't move but had breathing and feeding tubes, and retained fluid like crazy! It also didn't help that it was Christmas season and most of the labs were closed for the holiday. So, on January 10, 2007, when I actually got to hold her again as if you moved her she would have all kinds of issues, we got the test results back; positive for SMA. I had

already been asking about giving her to God as her quality of life wasn't good and I didn't want her to be in any kind of pain, but they wanted me to try to wait and get answers. She had gotten some type of infection and her lungs kept collapsing, veins blowing out, so she had a central line IV. I gave her to God on January 13, 2007 and as I was sitting in the room with her, having our last few minutes of alone time, I looked over at her O2 monitor to make sure she was doing ok and it was blinking 100! It had been in the 60's and 70's for the past week or so. It was a sign to me that what I was making was the right choice. She will be forever in my heart and soul, my little Princess on a Pea.

I'm doing my best to keep my daughter's memory alive! God Bless you all for spreading awareness and helping us keep our angels memories alive!

God Bless,
Erica Weidner of Indiana

IN MEMORIAM

Andrew Glenn Butler

January 8, 2009 – June 4, 2009



A Letter to Andy on his Fourth Birthday – January 8, 2013

Dear Andy,

Happy fourth birthday, my angel! I can't believe it has been four years since the amazing day that you were born. The day that changed our lives forever. Your daddy and I smile at the memories of your birth – of the awestruck pride and wonder we felt that day. But, now your birthday is very bittersweet for us – we miss you so much. Of course, your little sister Lucy demanded that we celebrate your day in style so that is what we plan to do – she loves a party as much as you once did. Today, we will focus on the joy you brought to our family.

We'll begin by visiting your garden with bubbles and a small present that Lucy picked out just for you. As always, we will release 20 balloons – one for each week you were in our arms – and hope that they will line the streets of heaven. I think you'll especially like the wonderful fourth birthday balloon that Gigi found for you. After lunch, we'll go to the bounce house place, because Lucy insisted that's what you would want to do. She says that she wishes she could jump with you. Oh, Andy, how I wish that too.

Sometimes I dream of another life, one without disease and heartbreak. One without Spinal Muscular Atrophy. I dream of your little boy smell and the tousled curl of your hair. Of the lilt of your voice and the warm squeeze of your hand in mine. Of hearing you say, "I love you too, Mommy." Of how this should have been the year you started pre-school. Of play dates with mud pies and other crazy messes that only a boy and his friends can make. Of all the milestones, so often taken for granted by others, that simply were not meant to be for us. But, I know that these dreams are selfish and that they are mine alone. You are in a place of pure beauty beyond these mortal fantasies.

And, I know that, even though we can no longer hold you in our arms, you are still here with us. Your presence surrounds us just like the breeze that will carry your birthday balloons and bubbles to the sky. You are the sound of Lucy's laughter. You are the light behind your Daddy's eyes. You are the strength in your little brother-to-be's kicks as he grows in my belly. You are my soul. You are all that is good in this world. To you, it doesn't matter that you never had the chance to celebrate your actual

birthday here on earth. You've been with us all along.

So, we will end your birthday celebration today with candles and cupcakes. We'll reminisce about your monthly birthday parties, and I'll remember how you hated the frosting you tried when you were just four months old. Lucy will wear a party hat and make a mess in your honor. Your daddy and I will laugh through the tears, as you taught us to do. We will celebrate your life as fully and completely as we can.

Because, my beautiful boy, your life counts. You only lived for 140 days, but each was miraculous. You were taken from us way too soon, but your spirit endures. You will never be replaced. Your handprint is forever on our hearts. We may not be together in this physical world, but we are never far from you. We may grow as a family and move forward in this life, but you are never left behind.

You will always be our firstborn son. You are the child who made me a mother. You are the one who taught me what love truly is. The love that sustained me when all was lost and allowed me to climb from the darkness of grief. The love that gave me the strength to once again embrace what the future holds for our family. The love that brought your younger sister and brother into being. The love that has allowed my heart, little by little, to heal. Andy, your love is the greatest gift that I ever received.

I am passing your gift of love on to your little sister and little brother-to-be. I want them to know you as I do – an incredible boy who loved with his entire being and who watches over us from heaven. A boy who faced so many obstacles, but never stopped laughing. I want them to carry your spirit of hope in their hearts and to share it with the world as you did. I want them to look up to their big brother like any other younger sibling would. But, most importantly, I want you to know how proud I am of you – now and forever – and how lucky I am to be your mom.

I love you, Andy. Happy birthday, my darling boy.

Always and forever,
 Mommy
 Audra Butler of Florida

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

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

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

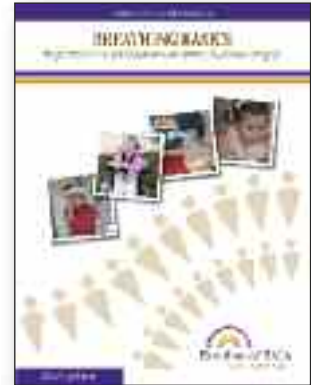
Family Guide to Research



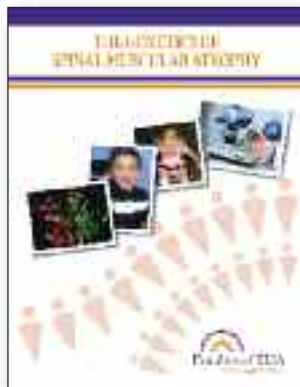
Families of SMA Family Support and Patient Services



Breathing Basics



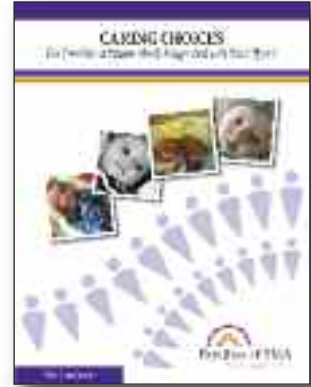
The Genetics of Spinal Muscular Atrophy



Nutrition Basics



Caring Choices



Understanding Spinal Muscular Atrophy (SMA)



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

Families of SMA Publishes a New and Comprehensive SMA Care Series Booklet Called *Understanding Spinal Muscular Atrophy*

This new SMA Care Series Booklet gives an in depth overview of Spinal Muscular Atrophy written for SMA families and Medical Care Professionals.

The Understanding SMA booklet was written by Vanessa Battista, MS, RN, CPNP, CCRC and other members of the Families of SMA Medical Advisory Council. If you would like a hard copy of this booklet mailed to you please email us at info@fsma.org or call (800) 886-1762.

Some of the topics in this important booklet cover the following important areas:

- Comprehensive explanation of SMA and what causes it
- Diagnosing SMA
- The genetics of SMA
- What to expect after receiving a SMA diagnosis
- What you should know, with some details on each type of SMA
- How Families of SMA can help

Additional free SMA Care Series Booklets, available in both English and Spanish, from Families of SMA include:

- Caring Choices: For Parents of Infants Newly Diagnosed with SMA Type I
- Breathing Basics: Respiratory Care for Children with Spinal Muscular Atrophy
- The Genetics of SMA
- The Family Guide to SMA Research
- Nutrition Basics
- Families of SMA Family Support and Patient Services



Families of SMA Releases *Family Support and Patient Services Guide* as the Latest SMA Care Series Booklet

FSMA has just released a new SMA Care Series Booklet called *Families of SMA Family Support and Patient Services*. The SMA Care Series Booklets are available for no charge to all SMA Families and professionals.

We are here to help all SMA patients and families by providing: Understandable information and emotional support; Resources to live a healthy and comfortable life with SMA; and Connections with other families.

This new booklet provides details on the many programs that FSMA provides to SMA families and patients, including:

- Newly Diagnosed Care Package Program for Type I, Type II and Type III Families
- Radio Flyer Wagon Program for Newly Diagnosed SMA Families
- The FSMA Equipment Pool
- The Annual SMA Conference
- Car Beds for Newly Diagnosed Type I Infants
- Newly Diagnosed Binder Program
- The Families of SMA Website
- Ask The Expert Service
- Information for Medical Providers
- SMA Social Networking
- Local Support Through the FSMA Chapters
- The Continuing Medical Education Conference
- The Newly Diagnosed Conference Program
- The Conference Scholarship Program



Julissa Gonzalez



Jenna Boguhn



Alexis and Zander Helfrich



Aimee Syer



Dylan, Heather and Nicholas Cuevas



Emma Bancach



Ella Ecker



Eris Castro



Kaly Diane Hudson



Ethan and Ray Fantel



Eleanor and Jack Bolton



Lexi Richardson



Zander Helfrich



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 michigan@fsma.org

Minnesota Chapter (since 1992)
 PO Box 32813
 Fridley, MN 55432
 (651) 470-8832
 Wanda Wosika, President
 minnesota@fsma.org

South Jersey/Delaware Chapter
 (since 2002)
 PO Box 538
 Medford, NJ 08055
 (866) 774-9533
 Jessica Moyer, President
 southjersey@fsma.org

New Mexico Chapter (since 2009)
 PO Box 90845
 Albuquerque, NM 87199
 (505) 353-1128
 Natasha Abruzzo, President
 newmexico@fsma.org

Greater New York Chapter
 (since 2006)
 PO Box 322
 Rockville Centre, NY 11571
 (516) 214-0348
 Debbie Cuevas, President
 greaterny@fsma.org

**Capital Region, satellite of the
 Greater New York Chapter**
 (since 2012)
 Amy & David Cunniff, President
 gnycapitalregion@fsma.org

Western New York Chapter
 (since 2003)
 PO Box 444
 North Tonawanda, NY 14120
 (716) 622-0804
 Bonnie Shiesley, President wny@fsma.org

OKI (Ohio, Kentucky & Indiana) Chapter
 (since 2004)
 PO Box 541012
 Cincinnati, OH 45254
 (513) 753-8222
 Beth Lockwood, President oki@fsma.org
 Michelle Palmer, IN Representative
 oki@fsma.org

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

Please send an email to chapters@fsma.org.

Pennsylvania Chapter
(since 2003)
PO Box 4307
Philadelphia, PA 19118
(866) 647-8113
Karen McRory-Negrin, President
pennsylvania@fsma.org

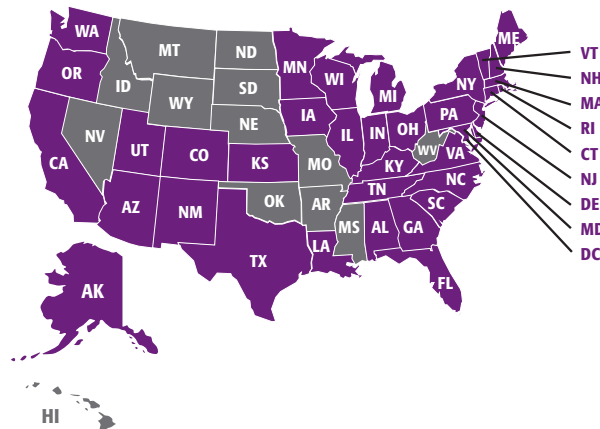
Tennessee Chapter (since 2001)
PO Box 7025
Knoxville, TN 37921
(865) 945-7636
Sarah Boggess, President
tennessee@fsma.org

Texas Chapter (since 2005)
PO Box 1115
Hutto, TX 78634
(516) 759-2509
Kelly Coggin, President
texas@fsma.org

Utah Chapter (since 2009)
PO Box 4137
Park City, UT 84060
(435) 901-4461
Shane Barber, President
utah@fsma.org

Pacific Northwest (since 2006)
PO Box 173
Galvin, WA 98544
(306) 871-1084
Russ Hargrave, President
pacwest@fsma.org

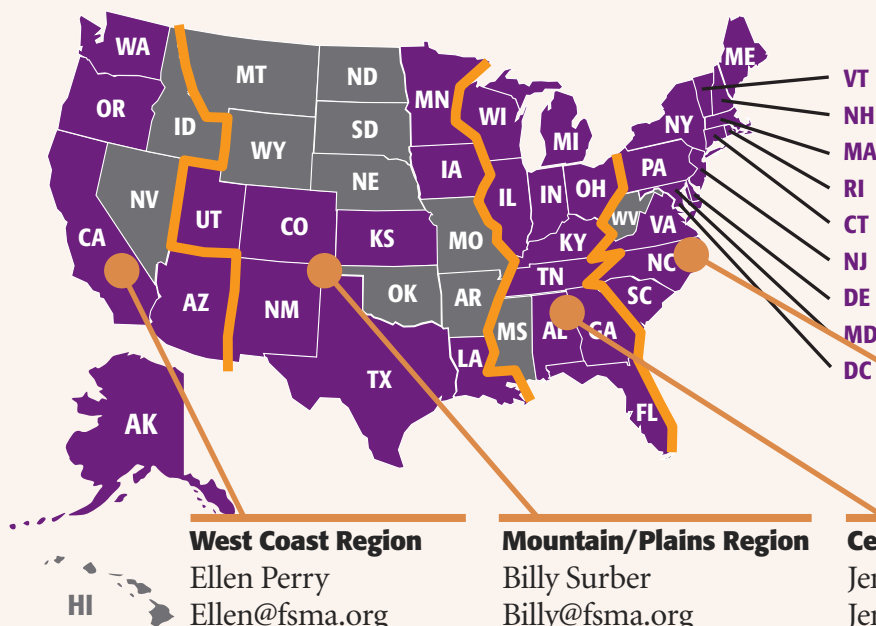
Wisconsin Chapter (since 1999)
PO Box 320516
Franklin, WI 53132
(414) 324-0499
Kate Vogedes, Laurie King,
Jenny Imhoff
wisconsin@fsma.org



FSMA Chapters

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

Have you ever thought about starting a chapter?
We want to hear from you.



West Coast Region
Ellen Perry
Ellen@fsma.org

Mountain/Plains Region
Billy Surber
Billy@fsma.org

Central Region
Jennifer Chaput
Jennifer@fsma.org

Eastern Region
Sarah Rodriguez
Sarah@fsma.org

CHAPTERS ARE REACHING OUT TO COMMUNITIES ALL ACROSS THE COUNTRY.

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

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

Begin to make a difference today.

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

ALABAMA

Alabama

10th Annual Alabama Walk-n-Roll

The Alabama Chapter of Families of SMA held their 10th Annual Walk-n-Roll to cure SMA on October 6th, 2012 at Snow Hinton Park in Tuscaloosa, Alabama. With an amazing turnout of over 250 people traveling in from all over the states of Alabama, Mississippi, Arkansas and Texas, the event raised a record **\$38,000** – almost \$10,000 more than in 2011! About \$9,200 of that total came from the Silent Auction held at the event on Saturday morning. Kenneth Hobby, President of Families of SMA, joined the Alabama Chapter for the event to celebrate their 10th annual year and update the crowd on the direct impact of their fundraising dollars into the research, support and care initiatives that Families of SMA will fund in 2013.

Jennifer Patrick, Alabama Chapter Vice President and mother of Logan Patrick, SMA Type I, said, “We will always remember and honor those who have gone before us and keep hoping a cure will be found soon. We want to thank all the sponsors, volunteers and participants that made this a very special day! It wouldn’t have been possible without everyone. Also, special thanks to Kenneth Hobby, the FSMA National President, for attending our walk! We appreciate all the support from Nationals.” After six years, Logan Patrick became an SMA angel on December 22, 2012. Together we will find a cure!

Families of SMA would like to especially thank the Alabama Chapter leadership; Rhynn Granger, Jennifer Patrick and Carla Burkes, for their dedication and hard work serving Families of SMA year-round, and for making their Chapter Walk-n-Roll a huge success year after year! Additionally, we would like to thank and recognize the Smith family for founding the Alabama Walk-n-Roll in 2002, and the Peterman and Willingham families for founding the Alabama Chapter in 2005. Without the continued support of these families, the Alabama Chapter wouldn’t be where they are today.



3rd Annual Cookies for Ava Bake Sale

The 3rd Annual Cookies for Ava Bake Sale in honor of Ava Johnson, SMA Type I was held on August 26th, 2012. The event, held at the Knollwood Church in Mobile, AL, raised \$585 for Families of SMA! Thank you Ebony Arnold for hosting this event.

Mississippi

Real Story Publishing Fundraiser in Honor of Natalie

On October 4th, 2012 an article was published in The Columbus Packet newspaper in Columbus, Mississippi about Natalie Gregg who was recently diagnosed with SMA Type I. The article spread SMA awareness and resulted in donations totaling \$335 for Families of SMA! Thank you to Real Story Publishing and Kevin and Ashley Gregg for making this possible.



ALABAMA (cont.)

Mississippi

Stop SMA Partners with Families of SMA Through Fundraising Events Held Throughout the Year



Stop SMA pledged \$70,000 to The Bassell Lab in August 2011 through a Families of SMA research grant. In February 2012, Jeff, Dee, Evie and Jake Horton traveled to Emory University to visit The Bassell Lab. They met the research team and watched a presentation concerning the research being done on SMA. While there, they visited with Gary Bassell, Wilfried Rossell, Claudia Fallini and the rest of

the team members that are working on a cure for SMA. Stop SMA held the following events this year as part of their pledge to Families of SMA in honor of Evie Horton, SMA Type II.

Laugh Away SMA



The Laugh Away SMA Comedy Showcase presented by Stop SMA was held August 4th, 2012 at Hinds Community College in Jackson, Mississippi. This year the event starred nationally renowned comedians Henry Cho and Jon Reep. The large crowd laughed away the night while raising much needed funds for SMA research.

3rd Annual Stop SMA Golf Classic

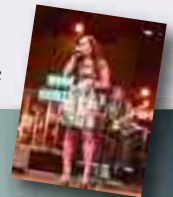
The 3rd Annual Stop SMA Golf Classic was held October 22nd, 2012. An exciting afternoon of golfing and competition was had by all at Lake Caroline in Jackson, MS.



Everyone was honored to have special guests State Treasurer Lynn Fitch and Congressman Gregg Harper join us for the festivities.

Country Christmas with Skylar Laine

Skylar Laine, American Idol Finalist, performed two benefit concerts on behalf of Stop SMA on December 1st, 2012. The concerts were held at the beautiful Jackson Academy Performing Arts Center in Jackson, MS. Skylar's unforgettable performance raised funds and SMA Awareness!



Thank You, Stop SMA!

Thank you to Jeff and Dee Horton and Stop SMA for your generous support throughout the year! Your dedication to supporting the Families of SMA research model is outstanding and inspiring.

ALASKA

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

ARIZONA

7th Annual Arizona Walk-n-Roll

The 7th Annual Arizona Walk-n-Roll held November 18th, 2012, was a great success! Families and friends came together to raise funds to help find a treatment and cure for SMA at Eldorado Park in Scottsdale, Arizona. The 8th annual event raised **\$16,800!** Thank you to Angel Wolff, Holly Cotter and the Arizona Chapter for planning this successful event!

Arizona Chapter Night at the Ballpark

The Arizona Chapter hosted a Night at the Ballpark on September 16th, 2012. Families and friends cheered on the Arizona Diamondbacks as they played the San Francisco Giants at Chase Field in Phoenix, AZ! Thanks to everyone that participated and purchased tickets, \$125 was raised for Families of SMA.



CAROLINAS

North Carolina

Love, Hope & Strength Fundraiser

The First Annual Love, Hope & Strength Fundraiser held in Raleigh, North Carolina in honor of Brady Chan, SMA Type II, was greeted with overwhelmingly positive show of support from the community. We more than doubled our original goal before the event actually took place November 2nd, 2012! We had a great turnout at an amazing venue and raised almost **\$12,500** by the end of the evening. With a sincere heart, we want to say thank you to all of our donors, sponsors and volunteers!

*Mimi Chan
Cary, NC*

South Carolina

Mistletoe Jam

On December 13th, 2012, our dear family friend Don McInerney hosted the first annual Mistletoe Jam. The event was held at the Zenzera Coffee & Wine Bar in Landrum, South Carolina. Many local musicians played Christmas Carols and fan favorites. We shared Families of SMA pamphlets, handed out bracelets and pens and raised almost \$2,325. It was a festive evening with great holiday music, happiness and holiday cheer. We hope to continue it every year!

*Alexandra Genovese, SMA Type II
Dix Hills, NY*



CHESAPEAKE

Maryland

Chesapeake Chapter Annual Crab Feast and Silent Auction

The Chesapeake Chapter hosted the Annual Crab Feast & Silent Auction on August 5th, 2012 at the American Legion Hall in Towson, Maryland. The event, attended by over 300 people, was a tremendous success raising over **\$48,000** for the Erin Trainor Memorial Fund for Families of SMA. Thank you to Beverly Venedam and Gene and Barbara Trainor for planning the event again this year!

Our Little Jewels 4th Annual Benefit Golf Outing

On September 28th, 2012, Our Little Jewels held their 4th Annual Benefit Golf Outing at The Links at Challedon in Mount Airy, MD in honor of Julian Lewis, SMA Type III. There were 124 golfers, 30 hole sponsors and 5 special sponsors that generously supported the event. Over \$10,000 was raised at this year's outing, \$5,000 of which was donated to Families of SMA!



CHESAPEAKE (cont.)



this disease.

The MAC Foundation, Inc. sent a proclamation request to the Governor of Maryland, Martin O'Malley in July 2012, requesting August become SMA Awareness Month. The request was granted. The MAC Foundation received a Proclamation making August SMA Awareness Month in the State of Maryland.

SMA Awareness and Blanket Fundraiser

I had an event on January 29th, 2012 at my school, Providence University, to make blankets for the newly diagnosed care packages and to raise awareness for SMA in memory of my sister Erin Trainor, SMA Type I. It was sponsored by Campus Ministry and Residence Life. We had coffee, desserts and door prizes donated by local businesses. It was a great turn out with over 80 students in attendance! We made 16 blankets and raised \$100! My friend Christina D'Agostino helped me a tremendous amount with planning and organizing the event and the volunteers.

Caitlin Trainor
Providence, RI



Throughout 2012, Our Little Jewels raised over \$40,000. We were able to give donations to Families of SMA for their research programs. OLJ also supported several programs that offer activities for children with physical and mental disabilities.

James and Colleen Lewis
Ellicott City, MD

This year, one of the major highlights of The MAC Foundation was the 1st Annual Gala, An Evening of Hope, which was held October 5th, 2012 at the Camelot in Upper Marlboro, MD. That evening, The MAC Foundation was able to raise over \$25,000 and spread awareness about SMA.

This year the MAC Foundation donated \$970 to cover a scholarship for the 2013 SMA Conference. The MAC Foundation's goal is to continue granting SMA kids wishes and ways to win!

Lisa M. Couser
Bowie, MD

MAC Foundation Gala

The MAC Foundation had an immense year in educating and promoting awareness, raising funds for research and working towards finding a cure for Spinal Muscular Atrophy. The foundation celebrates the life of Malik Attila Cosby and those individuals and families that have been affected by



CONNECTICUT

Emma Hope Award

Congratulations to some of the most recent Emma Hope award winners from NES Pageants!

Peggy Sue won at the NES Southeast Pageant, where she raised \$150 for Families of SMA. Aubrey won at both the NES East Coast States Pageant and at the NES Nationals Pageants, and raised \$1,000. Madison Burton won at the NES Midwest Pageant, where she raised \$240. Madison Wright won at the NES Maine Pageant, where she helped raise \$100. Jordan Roberts, the NES Southern New England Pageant winner, raised \$120. Ashlee Raposa won at the NES Southern New England Pageant, where she raised \$100. These are the latest pageants from NES director Tammy Rezendes that donate entry fees from the Emma Hope Award to Families of SMA, and these seven pageants raised a total of \$2,070!

Emma Goldsberry, SMA Type II, raised \$155 more by selling "Emma Hope" bracelet charms at the pageants. These amounts, along with fees from other pageants, photo contests and other donations from pageant participants, have totaled nearly \$20,000 for Families of SMA!

Jonathan Goldsberry
Bristol, CT



GEORGIA

SMA Day with the Atlanta Braves

SMA Families and supporters cheered on the Atlanta Braves when they played against the Los Angeles Dodgers during a beautiful Saturday evening game at Turner Field in Atlanta, Georgia on August 18th, 2012. Almost \$5,000 was raised for Families of SMA in memory of Louisa Lehmann, SMA Type I. Thank you Anami and Tim Lehmann for organizing this event and spreading SMA awareness! Tickets have been purchased for 2013 on Saturday, August 18th. Visit www.fsma.org/braves to buy your tickets today!

2nd Annual SMA Workout for a Cause

The 2nd Annual SMA Workout for a Cause was held on August 4th, 2012 at World Camp CrossFit in Albany, GA. The event was a huge success raising \$3,600 for Families of SMA in honor of Mateo Landa, SMA Type II! CrossFit gyms across the world participated by raising money and doing the “Mateo” workout. Thank you Rio Landa, GA Chapter President, for organizing this event.



GREATER FLORIDA

Chapter Update

Greetings from the Greater Florida Chapter! Stretching from south of Sarasota to the North Florida panhandle, our chapter covers a large and diverse geographic region and includes many amazing families.

The Greater Florida Chapter believes that family support is critical to our mission. In addition to hosting fun and innovative SMA awareness and fundraising events, we also work to provide families with resources, assistance and camaraderie.

In September 2012, we were devastated to lose two of our SMA warriors, Amy Marquez, SMA Type I, and Colin Lee Ohmstead-Hubbard, SMA Type II. We hold their families in our hearts and will continue to fight for a cure.

Throughout the year, members of the Families of SMA Greater Florida Chapter have worked with Quest Diagnostics offices throughout our territory to help inform and encourage SMA carrier testing through scheduled speaking engagements within the medical community.

We have a very active chapter and look forward to expanding our events into your area in 2013.

If you live in Central or North Florida and would like to host an event, please contact Katie Kerns, Chapter President, at 727-388-1888 (office), 727-512-4192 (cell) or greaterfl@fsma.org (e-mail). We also encourage you to check out our website at www.fsma.org/greaterflorida and join our “Families of SMA – Greater Florida Chapter” group on Facebook. Thank you!

From August 2012 through December 2012, our chapter’s efforts included the following:

August SMA Awareness Month Events

Once again, the Greater Florida Chapter reached out to city, county, and state elected officials, asking them to proclaim the month of August as SMA Awareness Month.



Thirty-One Catalog Fundraiser

From August 1st through 14th, 2012 our chapter participated in a Thirty-One Catalog Fundraiser, reaching \$250 in sales. A portion

of the proceeds were then donated to Families of SMA.

Painting With a Twist

On Saturday, August 4th, 2012, 42 of our chapter members and friends spent an afternoon together in the hopes of painting away SMA! They created original paintings, enjoyed good food and wine and received a complimentary SMA Awareness Month gift! Painting With a Twist, located in St. Petersburg, FL, generously donated the proceeds from the event, totaling \$563, to Families of SMA.

Candlelight Memorial Ceremony

Traditionally, our chapter comes together on the second Saturday in August to celebrate our SMA Warriors and Angels with a



GREATER FLORIDA

Candlelight tribute. This year instead of candles, we released Fire Lanterns from the historic Pier in St. Petersburg, FL on August 11th, 2012, which floated over the water in a beautiful display of color and light. After our tribute, we continued the celebration at Jonny Reno's where the view was gorgeous, and the live music was great fun!



LEGOLAND Florida Fundraiser

From August 18th through August 31st, 2012, LEGOLAND Florida invited the Greater Florida Chapter to participate in its "Buddy Benefit Days." LEGOLAND discounted tickets for our chapter to sell, and then even more generously donated \$15 dollars of every ticket sold to Families of SMA for a total contribution of \$1,995! We are proud to be "buddies" of such a great and charitable organization and look forward to continuing our partnership in 2013. We especially would like to thank LEGOLAND employees Adrian Jones, Todd Andrus and Marcy Harrison for all of their support and their amazing spirit of giving!



Macy's Shop For A Cause

Chapter members sold \$5 Savings Passes to be used at Macy's stores nationwide on August 25th, 2012 to help raise awareness for SMA and to raise funds for research.

SMA Awareness Day with the Tampa Bay Rays

Marking the second year of SMA Awareness Day with local MLB team, the Tampa Bay Rays, our chapter was out in full force as the Rays hosted the Texas Rangers on September 9th, 2012. We enjoyed our seats on the TBT* Party Deck again, where we had an amazing view of the PSA on SMA that aired on the JumboTron. Families of SMA also was the featured charity for the night, with a booth in the Rays' Community Corner. We were able to raise \$735 and the Greater Florida Chapter was the featured charity in the Rays Community Corner again on October 3rd, 2012!

20th Annual Maluko Golf Classic

Celebrating its 20th anniversary, the Tampa Maluko Golf Classic was held October 29th, 2012 at Emerald Greens in Tampa, FL. The format for the golf tournament is a four player scramble with a shotgun start. The Maluko Golf Classic began as a simple way to show passion for the game of golf and the camaraderie of good friends, while supporting causes near and dear to the hearts of the families who organize the event. One of those causes is Families of SMA, in honor of Tyler Hernandez, Type II. Thanks to the continued efforts of the Hernandez, Romaele, Leto, and Menendez Families, the Maluko Golf Classic is now one of our chapter's largest and most-anticipated fundraisers! The 20th anniversary event raised an astounding \$40,000 for Families of SMA.



4th Annual Greater Florida Walk-n-Roll

More than 200 people turned out on November 10th, 2012 at Ft. Desoto Park in Tierra Verde, FL, to participate in the 4th Annual Greater Florida Walk-n-Roll, which raised \$21,000 for Families of SMA!

The team spirit was high at the event, with family and friends wearing brightly colored shirts in support of their teams, which included Allison Wonderland (in honor of Allison Kerns, SMA Type II), Andrea's Army (in memory of Andres Trakas, SMA Type I), Andy's Army (in memory of Andy Butler, SMA Type I), Brooklynn's Brigade (in honor of Brooklynn Santos, SMA Type II), Cooper's Crew (in memory of Cooper Nowotny, SMA Type I), Francisco's Forces (in memory of Francisco Rodriguez, Jr., SMA Type I), Maia's Mission (in honor of Maia Shockley, SMA Type II), Team Amy Marquez (in memory of Amy Marquez, SMA Type I), Team Princess Tianna (in honor of Tianna Rivera, SMA Type II), Team Taylor (in memory of Taylor Bowser, SMA Type I) and Waiting on Matt!



GREATER FLORIDA (cont.)

Before the walk, the Tampa Bay Crossfire power soccer team treated fans to a demonstration of their skills, encouraging everyone to get out and play! With a warm-up stretch with Christie Bruner of Baby Boot Camp, the walk began and we were on our way! After the walk, friends and families had the chance to meet the Ray Team girls and mascot Raymond from the Tampa Bay Rays baseball team, enjoy custom cupcakes from Dough Mom, sample free ice cream bars from Unilever, and buy great items from amazing vendors like Thirty-One Gifts and The Pampered Chef. Chick-fil-A donated a delicious lunch for all walk participants too!

Andy's Army won the prize for the largest team with more than 60 walkers, and Team Taylor was the highest fundraiser with a team total of more than \$2,000. Andy's Army and Maia's Mission were named the second and third highest fundraisers, respectively.

All in all, the 4th Annual Greater Florida Walk-n-Roll was a great celebration of hope, and we can't wait to do it again in 2013! Thank you to all of our amazing event organizers, generous sponsors, donors and dedicated fundraising teams!

12th Annual Garage Sale and Costume Crusade at The Village Early Learning Center

On November 3rd, 2012 The Village Early Learning Center in Brandon, FL hosted its 12th Annual Garage Sale and Community

Fair to raise money and awareness for Families of SMA. A crowd of people showed up to find great deals and help show their support from the community. Almost 3,000 raffle tickets were sold for the event! This year also included entertainment provided by some of the school's "Adventure Club" as they showed their stuff square dancing and getting the crowd involved.

The week of events originally started with the school's annual "Costume Crusade." Every year the children of The Village ELC dress up and parade around in their costumes hoping to raise money and awareness for SMA. "Mr. Joe," one of the village owners, who has SMA Type III, competes in a wheelchair race with some worthy opponents. Four year old Brooklynn Santos, SMA Type II, was this



year's winner! Thank you to Thomas Tucker, SMA Type II, age 14, for coming out each year to compete and support the fundraising efforts as well! Next year they hope to raise more money and be able to officially classify their event as the "Largest Garage Sale in Brandon." This year their efforts raised a total of \$650!

Thank you to Joe and Susan Miller for organizing this event and for your continued support!

Luncheon in Honor of Andy Butler

Each month at Bob Sikes Elementary School in Crestview, FL, a different grade level sponsors a staff lunch and gives the proceeds to a charitable organization. This December, Kim Kiefer, Andy's aunt, held a lunch to benefit Families of SMA. The teachers raised \$200 in memory of Andy Butler, SMA Angel Type I.

Christmas Angel Dinner

The Trakas family hosted the second gathering of our chapter's families of angels on December 9th, 2012 for a luncheon and a cookie exchange! This meeting was held in memory of Andrae Trakas, SMA Type I.

GREATER NEW YORK

ING New York City Marathon 2012

The ING New York City Marathon 2012 was to be held on November 4, 2012, but due to Storm Sandy was cancelled. Even though they did not get the chance to run, Douglas Erwin, David Scott, Laurette Zion and Julie Fazio did a tremendous job training and fundraising for the race! Thanks to the amazing efforts of our runners, together they raised \$27,715 in support of Families of SMA. Special thanks to Michele Erwin for organizing the event.

8th Annual Greater New York Walk -n-Roll to Cure SMA

The 8th Annual Greater New York Walk-n-Roll to Cure SMA was held on September 15th, 2012 at the Long Beach Boardwalk in Long Island, New York. The fun filled day included face painting for the kids and a 50/50 raffle. Thank you Debbie Cuevas and the Greater New York Chapter for organizing the Walk-n-Roll. Because of your hard work and many generous donors and participants, over \$22,600 was raised for Families of SMA!

NY Mets SMA Awareness Day

The Greater New York Chapter celebrated SMA Awareness day with the NY Mets on August 26th, 2012 at Citi Field in New York, NY. Family and friends cheered on the home team during the NY Mets vs. Houston Astros game! The fundraiser, held in memory of Owen Shuler, SMA Type I, raised almost \$8,000 for Families of SMA! Many thanks to the NY Mets and the Greater New York Chapter!



GREATER NEW YORK (cont.)



2012 NYC Triathlon

In January 2010, soon after meeting my 6-month old niece for the first time (I live in Australia), I received news from my family that she, Amanda Lam was diagnosed with SMA Type I. The realization that my niece will never reach the milestones that other children reach and would remain mostly immobile for the rest of her life made me appreciate the ability within my own body. I signed up for my first short distance triathlon immediately after her diagnosis. Every day that I trained, even to this current day, I think about Amanda and other SMA children.

After completing that first triathlon (a massive feat considering how I panicked in the open water and swam in the wrong direction!), I ramped up my training to include half-marathons and more triathlons.

By 2011, as Amanda continued to grow, I started eyeing the NYC Triathlon as a goal. When it was announced that the 2012 triathlon would be held a week after her 3rd birthday, I signed up thinking the timing was perfect. I could return to NYC to celebrate her birthday with the family – something I had desperately wanted to do since she was born – but to also start a fundraiser in honor of my niece who had inspired me to do triathlons. Even though she never will be able to – I could, for her.

On July 1st, we celebrated Amanda's 3rd birthday together as a family. By July 8th, on the morning of the NYC Triathlon, we raised over \$5,300 for Families of SMA – over twice the amount that we originally expected to raise. It was one of the proudest moments of my life. Participating in the NYC Triathlon was one of the best moments in my life – not only for the physical challenge, surrounded by my family, but because of the funds and awareness we raised for SMA. It is something to feel truly proud of.

*Sandy Lam
Australia*

Hiking the Colorado Rockies

The Hiking the Colorado Rockies fundraiser took place on July 27th through August 1st, 2012. The event was organized by Kirk Reynolds of the Greater New York chapter and his company Discover Outdoors with Kristen McCain as our participant. The group travelled to Francie's Cabin in Breckenridge, CO which they called home for the week. The days went by too quickly and on day five, the hikers had to say their goodbyes to an amazing adventure. Thank you to everyone for helping us raise \$4,725 for Families of SMA in honor of Ellie Stitzer, SMA Type III!

*Kirk Reynolds
New York, NY*

3rd Annual Charity Golf Outing in Memory of Stephen Ruocco

On October 7th, 2012 the LaGrange Council 13378 of the Knights of Columbus in Fishkill, NY held our 3rd Annual Charity Golf Outing in memory of Stephen Ruocco, SMA Type I. We had planned the original event in 2010 to help the family of baby Stephen John Ruocco provide for his care. On May 13th, 2012 this brave little baby lost his battle with this devastating disease so we decided to turn our support to Families of SMA and raised \$1,500. Thank you to all of this year's participants and to Christian Jones for helping organize the event!

*William Link
Grand Knight
Fishkill, NY*

Macy's Shopping for a Cause

The Greater New York Chapter sold \$5 passes for the Macy's Shop for a Cause event. On August 25th, 2012, anyone who purchased a pass received up to 25% off their purchases. In total, \$720 was raised for Families of SMA! Thank you to Michele Erwin for organizing, and to everyone who participated and bought a pass.

Penny Harvest Fundraiser

On July 10th, 2012, the students of PS 100 Glen Morris School in Queens, NY held a Penny Harvest Fundraiser in memory of Deirdre Abraldes, SMA Type I. Thanks to the hard work of the students and their coach Debbie Fiallo, \$500 was raised for Families of SMA!

Block Island Lemonade Stand

The Block Island Lemonade Stand was held on October 25th, 2012 in Northport, NY. Family and friends of Laurette Zion came together to raise money toward her New York Marathon fundraising goal for Families of SMA! They were able to raise \$100 in honor of Emily Lozina, SMA Type I.

Awareness Month Fundraiser

Last year I initiated a monthly donation program at my job and have picked a different organization each month. Whoever is interested donates \$1 for that particular cause. This month was for SMA Awareness. We also dress in the color of the organization. In this case, we all wore orange or purple on August 3rd, 2012 and raised \$24!

*Best Regards,
Eileen Lai
Westbay, NY*

Capital Region

1st Annual Capital Region 5K Run, Walk-n-Roll

The Greater NY – Capital Region Chapter held their 1st Annual Capital Region 5K Run, Walk-n-Roll on September 8th, 2012. Our goals were to raise awareness of SMA, bring together SMA families in our area, and raise funds for Families of SMA. We succeeded in all of these goals – and had a great time doing it!

We dedicated our race in memory of Josephine Lucy, who lost her battle with SMA Type I in June 2012. Her mother, Christine, shared Josie's story with us – and brought many of us to tears in the process! Josie's precious life, along with the other SMA angels represented, were honored, which also helped to create a sense of urgency to find a treatment and cure for SMA.

Over 275 people attended our event – many of whom were friends and family of 10 SMA affected individuals – 5 angels and 5 warriors. It was wonderful to connect with others who live daily with SMA. Due to our phenomenal fundraising teams, we exceeded our \$10,000 goal and raised over **\$33,000**. A special thanks to all our wonderful volunteers, Team Captains, and fundraisers who made this day so special. We also want to thank Hannah Balta of HB Photography who graciously offered her time and talents to our event!

*Dave and Amy Cunniff
Duaneburg, NY*

ILLINOIS

Hamman Family Hawaiian Luau

On July 7th, 2012, Lisa and David Hamman hosted the Hamman Family Hawaiian Luau in Naperville, Illinois. This biennial event is held in memory of Henry Hamman, their son who had SMA Type I. Friends, family and neighbors all came together to enjoy the hog roast, hula dancers, dunk tank and Hawaiian themed entertainment that took over the entire block on a beautiful Saturday afternoon. This year over **\$49,000** was raised from the event! Thank you to the entire Hamman Family for your extremely generous support of Families of SMA over the years!

Sophie's Walk & Roll for SMA

On June 16th, 2012, we held a Walk-n-Roll event to celebrate the life of our daughter, Sophie TerHorst, SMA Type I, and raise awareness and funds for Families of SMA. The event took place in Lincoln Park in Chicago, IL, where we took Sophie on daily walks. The start and finish line were located at a tree that was planted in memory of Sophie and participants had the option of walking either a one or two mile loop. Afterwards we had a catered BBQ which allowed everyone to mingle and remember Sophie. Since Sophie loved Elmo, participants wore Elmo t-shirts and carried a red carnation during the walk. Everyone later laid these by her tree at the finish line.



We had an incredible turn-out and can't thank everyone enough for the great support. We could never have expected this, but our generous family, friends and co-workers donated over **\$43,000** for Families of SMA! We know that this money will help countless families who are given the horrible SMA diagnosis, as well as provide money towards research for a cure!

*Mike and Liz TerHorst
Chicago, IL*

6th Annual Kyra's Idea... Angie's Hope Fundraiser Wipe Out SMA Family Pasta Party

The Scadden and Lee families hosted the 6th Annual Kyra's Idea... Angie's Hope Wipe Out SMA Family Pasta Party at The Foundry in Aurora, IL, September 22nd, 2012. Thanks to the raffle, silent auction and penny war money from Hill Middle School, almost **\$19,000** was raised in honor of Angie Lee, SMA Type II! Thank you to the Scadden and Lee families for your very generous and continued support of Families of SMA.

16th Annual Chicago Half Marathon & 5K

On September 9th, 2012, Team Families of SMA ran in the official Chicago Half Marathon & 5K. This is the second year that Families of SMA participated in the race as an official charity partner. Our community came out from all over the Midwest and raised over **\$12,200!** Thank you to our 17 runners for all of your hard work in training for the event and fundraising for Families of SMA.



Haymarket Pub & Brewery Fundraiser



On August 19th, 2012, friends and family of Isabel Poluchowicz, SMA Type I, held a fundraiser at Haymarket Pub & Brewery in Chicago, IL. Thank you to Isabel's parents, Anne and Andrei, for generously donating \$900 to Families of SMA from the event!

Candles for SMA

I have a story behind our donation this month. Back in April, I was informed of a candle wax contest from the supplier of my wax. NGI told us to make and send a product using their wax to be judged in August. The winner would win \$500. I decided to make one of my Personalized Pillars with Avery's picture on it in hopes of winning. Well, with the help of SMA Angel Avery Canahuati, SMA Type I, my candle won!



*Sincerely,
Lisa Woods, SMA Type III
Dixon, IL*

www.CANDLESforSMA.com

Lisa Woods generously donates a portion of proceeds from sales of her homemade candles to FSMA each month. So far this year, Lisa has donated \$823. Thank you Lisa for your continuous and extremely generous support of Families of SMA through Candles for SMA!

ILLINOIS (cont.)

SIU Football Game Fundraisers

In the fall of 2012, Traci O'Brien and members of the Southern Illinois University-Carbondale chapter of the American Marketing Association raised funds and spread SMA awareness! The group hosted tailgates before home football games and donated the proceeds, totaling \$222, to Families of SMA in honor of Shannon O'Brien, SMA Type III, Traci's sister. Thank you to Traci for organizing the tailgates.

Kendall Elementary School Fundraiser in Memory of Drew

On September 17th, 2012, Girl Scout Brownie Troop 1165 from Kendall Elementary School in Naperville, IL donated a portion of their cookie sale money to Families of SMA. Thank you to the troop and Kathy Shepherd for the generous donation of \$200 in memory of Drew Plotke, SMA Type I.

IOWA

16th Annual Beaverdale Beaverdash

On September 15th, 2012, more than 600 participants and volunteers gathered in Beaverdale, Iowa for the 16th Annual Beaverdale Beaverdash. This year's event was again a tremendous success, and thanks to our generous participants and donors, we raised over **\$23,000** to support Families of SMA's research and family support programs. Thank you to everyone who attended the event, especially our volunteers. Without your continued support, the Beaverdash would not be possible.

We would like to send a special thank you to the Mercy Rehab staff, Mike Franke and Stan Smith for all of their help! We also would like to thank Sue Replinger, Michelle Soyer and Staci Bailey who are always there to provide all the help we need throughout the entire process, and the Freundl family who graciously took the time to pen our sponsorship letter.

We are so thankful to those who have supported this event over the past 16 years and we can't wait to see everyone at the 17th Annual Beaverdale Beaverdash.

*Julia Middendorf-Anderson
and Julie Greenwood
Iowa*

Young & Wild Special Needs Rodeo Benefit

From May 18th through 20th, 2012, the Young & Wild Rodeo Company of Fairfield, IA held a special needs rodeo for children of all special needs along with a main event rodeo. The special needs rodeo included pony rides, face painting, tattoos and a petting zoo. The main event rodeo featured mutton busting, youth rodeo, junior bull riding, bull riding and a special appearance by American Freestyle Bull Riders and Miss Teen Rodeo Iowa. The Young & Wild Rodeo Company collected \$1,485 in donations for Families of SMA. Families of SMA is near and dear to the Smith family, owners of Young & Wild Rodeo. The Smith family has made a commitment to support Families of SMA and help find a cure for Addison and all of her friends. Addison is a very close friend of their daughter, Tarrence, SMA Type III.

*Kara Tarrence
Fairfield, IA*



KANSAS CITY

Kansas

Kansas City Chapter 12th Annual Cure SMA Race-n-Roll

Celebrating it's 12th year, the Kansas City Chapter Cure SMA Race-n-Roll brought together more than 500 SMA families, volunteers and community members on October 6th, 2012 at Bishop Miege High School in Roeland Park, Kansas.



A record 16 teams were represented this year:

Alana's Army, Brett's Brigade, Bri's Army, Charlie's Crew, Fusion with Zeal, Groovy Gibbs Girls, Hunter and Cody's Crusaders, Kennedy's Crusaders, Lindsay's Trailblazers, Moving4Jaden, Peter's Posse, Samantha's Spirit, Sam's SMA Warriors, Team Bubba, Team Lexie, and The Binky Brigade. Thanks to the support of the teams and the dedication of donors, sponsors and volunteers, we were able to raise more than **\$38,000** this year!

Thank you all for your support and we will see you again next year for the Kansas City Chapter 13th Annual Cure SMA Race-n-Roll. Keep an eye on your inbox for details!

Missouri

10th Annual Bommarito Z Club Car Show

Our 10th Annual Bommarito Z Club Car Show was held September 9th, 2012 in Saint Louis, Missouri to benefit Families of SMA. It was a great success raising a total of \$14,600! We are grateful to all the Z Club members and president, Tami Dunn, for their support.

Thank you to Steve Colesworthy, Bommarito, Kathy Goodyear, Rita Schmidt and the Carpenter Family (Candy, Scott, Brittany and Brandon) for their help throughout the day! Without them it would not have been as successful. The event is held annually in honor of Brittany Carpenter, SMA Type II, and in memory of Madeline Schmidt, Brian Goodyear and Michael Goodyear, all SMA Type I.

*Sincerely,
Janet Hutchinson
Kansas City, MO*

A Birthday Bash for Tilly

On July 1st, 2012, friends and family gathered to celebrate the 2nd birthday of Tilly McRoberts, SMA Type II, in St. Louis, MO! Her parents, Leslie Derrington and Glen McRoberts, collected money for Families of SMA in lieu of presents and raised \$2,250! Thank you to all of Tilly's guests for their generous donations.



MICHIGAN

FSMA Jeans Day at Meritor

On July 27th, 2012 the Meritor office in Troy, Michigan held a Jeans Day to benefit Families of SMA! Thanks to the hard work of event organizer Kendra Park and the generosity of Meritor employees, \$2,875 was raised in honor of Brooke and Brielle Kennedy, both SMA Type II!



Rugger's Up and Under Golf Tournament

The Rugger's Up and Under Golf Outing was held at The Prairies Golf Club in Kalamazoo, MI on June 9th, 2012. The event, held in honor of Brooke and Brielle Kennedy, both SMA Type II, raised over \$1,300 for Families of SMA!

Michigan Chapter Hot Dog Cook-Out

The Michigan Chapter hosted the Hot Dog Cook-Out for Families of SMA on May 5th, 2012. In total, over \$1,100 was raised for Families of SMA!

Chemical Bank 5K & Fun Run

On July 28th, 2012, the Chemical Bank 5k and Fun Run was held in Bryon Center, MI. Kami and Tim Potjer, parents of SMA Angel Sydney Potjer, SMA Type I, raised \$250 for Families of SMA. Thank you Kami, Tim and Jodi Sevigny at Chemical Bank for your continued support!

Spa-Toe-Pia Pennies for Polish Fundraiser

Mindy and Sue Kazlauskas, mother and grandmother of Maggie Sue Kazlauskas, SMA Type I, are clients of my spa, Spa-Toe-Pia in Dogawac, MI. They would bring Miss Maggie Sue in to get pedicures and I fell in love with this beautiful child. After she passed away I wanted to do something in memory of her, so I created "Pennies for Polish." We polished children's toes and/or fingers for coin donations during the month of July. All of the \$1,500 raised from the event was donated by our clients. We will continue to do this every July in Maggie Sue's memory. We hope to inspire other salons to follow us in our mission to raise one million pennies for Families of SMA!

*Jacqueline Hale
Dowagiac, MI*



MINNESOTA

12th Annual Minnesota Chapter Walk-n-Roll

The 12th Annual Minnesota Chapter Walk-n-Roll held September 15th, 2012 at Lake Phalen in St. Paul, Minnesota was a success! Our teams continue to get bigger each year. We enjoyed beautiful weather and were able to spread our activities outdoors with SMA volunteers helping with the cake walk and more! Thank you to everyone who helped plan the walk, all of our fundraising teams, donors and sponsors. Because of your dedication and generosity, we were able to raise over **\$18,800** to help find a treatment and cure for SMA!

2nd Annual Ride Away SMA

The 2nd Annual Ride Away SMA fundraiser was held on June 6th, 2012 in Rochester, MN. Before the ride, everyone enjoyed a pancake breakfast, bake sale and silent auction at the Peace United Church of Christ.

Despite all the rain we had that day, we still had about 75 bikes participate in the 125 mile Motorcycle & Classic Car Fun Run! The



MINNESOTA (cont.)

run ended at Shar's Country Palace where we had more food, door prizes and both a live and silent auction. It was a great turn out and I anticipate it to keep growing every year. We were able to raise over **\$18,000!** A special thank you to Eric Ronningen and Amy Allen, parents of SMA Type I angel Lindsey Ronningen, and the Lindsey Ronningen Benefit Fund for all of your support.

A donation of \$4,000 came in memory of a dear friend of ours, Steve Siegle. He was tragically killed in a motorcycle accident this past October and in lieu of flowers, donations were made in his memory to FSMA. Steve was a wonderful friend and supported Ride Away SMA for the past two years.

Thank you!
Amy Allen
Rochester, MN

SMA Home for the Cure



The SMA Home for the Cure has been sold thanks to Everlast Enterprises, a team of generous sponsors, and a dedicated group of volunteers. Jeff Martineau Andrade is a Delano-area realtor and father to Bella, SMA Type II. He reached out to his business

contacts to recruit donations of skills, time and building materials for the home. The SMA Home for the Cure could not have been completed and sold without the support of the home's sponsors. Thank you to Coldwell Banker Burnet, Burnet Title, Everlast Enterprises, WCCO, PHH Home Loans and Beacon Bank for their dedication. The SMA Home for the Cure raised funds that will be used for family support programs and research efforts to develop a treatment and cure for SMA.

Abigail Rose Homstad Memorial 5K Walk/Run



On August 11th, 2012, over 200 people gathered in Carlton, MN at the Willard Munger State Trail to participate in the 1st Annual Abigail Rose Homstad Memorial 5K Walk-n-Run in memory of my daughter who passed away from SMA Type I. More than \$7,500 was raised to benefit Families of SMA and support the research they are funding to find a treatment and cure for SMA. Thank you to all the donors, participants and volunteers who helped make the inaugural year of this event such a tremendous success. A special thanks to the amazing team at Kohl's who volunteered

and the Minnesota Chapter leaders for their support. See you all in the fall!

Stacey Homstad
Cloquet, MN

1st Annual FSMA Booya!

The 1st Annual Booya held on October 21st, 2012 raised over \$900! It happened to be a very cold day, which made it a perfect day for everyone to come in a have a big bowl of booya! Thank you to Wanda Wosika for organizing the event and for working with the United Special Sportsman Alliance and local businesses for donations!

Spaghetti Dinner

The 3rd Annual Spaghetti Dinner was held at the local VFW Post 9625 in Coon Rapids, MN on November 25th, 2012. The bake sale and silent auction again were big hits and the outcome was tremendous! We were able to raise over \$460! Many thanks to everyone who supported the Spaghetti Dinner, we couldn't do it without you! Special thanks to the Minnesota Chapter and Eileen Saldana for planning this event.

18 Hole Women's League

My 18 hole golf course league from Blue Oaks Gold Club in Hayfield, MN held their Guest Day, August 2nd, 2012. During this event, we hosted a raffle filled with wonderful items donated by members of the league club. This year, as chairperson, I chose Families of SMA to receive the \$380 proceeds we made at the event in honor of my granddaughter, Isabella Martineau Andrade, SMA Type II.

Joan Ottinger
Plymouth, MN

NEW ENGLAND

Chapter Update

Members of the New England Chapter gathered for a family social on Saturday, December 1st, 2012. Kids enjoyed crafts, cupcakes and bingo, while adults had the opportunity to connect and share experiences.

If you are not receiving the New England Chapter emails please contact Silvia Murphy at silvia@fsma.org.

Silvia Murphy
Chapter Vice President

NEW ENGLAND (cont.)

Massachusetts

6th Annual Fall Classic FSMA
Charity Golf Tournament

William, SMA Type II, and the Johnson family would like to extend a most heartfelt thanks to all the golfers, guests, donors and sponsors who made the 6th Annual Fall Classic Families of SMA Charity Golf Tournament such a huge success. The event was held on October 12th, 2012 in Bellingham, Massachusetts. With everyone's generosity, the event was able to raise over **\$60,000** for Families of SMA. These vital research dollars get us closer to finding a cure for William and all who suffer from this horrific disease. A special thanks goes out to Herb Grace from Memorial School for lending his talents behind the mic once again for the evening's live auction, and to Steve and Kathy Cook for their incredible efforts before, during and after the event. None of the golfers were able to win the hole-in-one prize of a new car provided by Clay Family Dealerships, but congrats go out to the tournament winners Medfield residents Shawn Farrell, Brian McKenzie and Rich Mynahan.

Special thanks to Will Aguilera for his outstanding fundraising efforts, raising over \$11,000 for this year's golf tournament and for entertaining us all with his banana costume on the course!

*Heidi, Tripp and William Johnson
Medfield, MA*

Bike Run & Benefit



On July 28th, 2012, the Jacques family hosted the Bike Run & Benefit in East Taunton, MA, to celebrate the life of Ashleigh Michelle Jacques. Bikers and runners completed a 50 mile journey from Raynham to Francis Farms in Rehoboth to help find a cure for SMA. A benefit lunch was held immediately following the bike run with participants and supporter's enjoying a variety of food and entertainment. Guests listened to live music, and played softball, volleyball, horseshoes, Bocce and enjoyed a Moonwalk.

Ashleigh's older sister, Brianna, age 4, set up a lemonade stand to help support Families of SMA. She also sold her paintings and drawings.

According to Brian Jacques, "The fundraiser went very well. It's important because SMA is one of the neuromuscular diseases that are most close to finding a cure." The Jacques family hope to increase awareness about the rare disease to help other families who have children with SMA, and encourage early detection.

Thank you to Kristen & Brian Jacques and the New England Chapter for your hard work in hosting the Bike Run & Benefit. We are deeply saddened by the loss of Ashleigh and keep them in our thoughts during this difficult time. Your efforts raised funds that will be used for critical SMA research and family support programs, but you are also inspiring hope that one day we will find a treatment and a cure for Spinal Muscular Atrophy.

To learn more about Ashleigh's inspiring story, please visit her page at <http://ashleighsfightsma.webs.com>

2012 Halloween Hustle

On October 28th, 2012, we held our first fundraiser in honor of our son Paul Osborn, SMA Type III, with the help of the Tri Sharc Triathlon Club. Since Paul was diagnosed last



NEW ENGLAND (cont.)



along with face painting, many raffles, a great DJ, photographers who made individual picture key chains for guests and the best cupcakes from Mama Cakes, who



summer Families of SMA has been a wonderful resource for us and we wanted to give back, not only for our own son, but for all those with SMA. The Halloween Hustle 5k and

Family Fun Run, held at the beautiful Dean Park in Shrewsbury, MA, got off to a great start despite drizzly gray weather as we all warmed up to the Monster Mash! We had over 60 participants for both the 5k and the Fun Run. Together we raised over \$1,000 for Type II and Type III care packages. Special Thanks to Matt Pearson, the Tri Sharcs, Stacey Farrell, The Boston Bruins, Panera Bread, Shaws Supermarkets and all those who came out to support us!

*The Osborn Family
Cherry Valley, MA*

Victory For Violet

This has been an eventful August for us! We've done several things that have brought about SMA Awareness in our area and even raised some money for Families of SMA. On August 13th, 2012 our town Selectmen declared August SMA Awareness Month in our town of Southwick, MA! This is a great step for us in raising awareness in the area and getting our story out. We also were on the front page of two local newspapers, which spread even more awareness!

This all led up to our first annual Victory for Violet Dance Away SMA Fundraiser, held in Southwick, MA on August 18th, 2012 in memory of our daughter, Violet Clendenin, SMA Type I. We were lucky to have two Melha Shriner Clowns entertain the kids,



even makes a cupcake called "The Violet" for our Violet! We had a little segment about our fundraiser on the 11 O'clock news! So far we have raised over \$2,000 for Families of SMA!

*Jason and Kelly Clendenin
Southwick, MA*

13th Annual Connor James McKeon Memorial Golf Tournament

The 13th Annual Connor James McKeon Memorial Golf Tournament was held on July 21st, 2012 at Pine Ridge Country Club in Oxford, MA. The Connor James Memorial Golf Tournament was started in 2000 in memory of our SMA Type I angel, Connor, who was born on December 18th, 1999 and became an angel on February 21st, 2000. This was another successful CJM Memorial Tournament under a beautiful sunny sky. We

had 92 golfers and an additional 18 people joined us for the dinner, helping us to raise \$1,650 towards finding a cure for SMA. This brings our 13 year total donation for Families of SMA to \$33,100. For the past 13 years, we have been blessed with many family and friends participating in the golf event to celebrate the life of our son and nephew. For more information on our event and to see more photos you can find us on Facebook under "Connor James McKeon Memorial Golf Tournament."

*Mike and Missy McKeon, Connor's Parents
Pat and Marissa McKeon,
Connor's Uncle and Aunt
Worcester, MA*

6th Annual Ride for Alex



The 6th Annual Ride for Alex was held September 16th, 2012 at Old Rochester Regional High School in Mattapoisett, MA. Attendees enjoyed a two hour motorcycle ride from Bridgewater and down towards the Cape, a barbecue and even a raffle! Thank you Tammy Foisy and James Pateakos for organizing this event. This event raised \$185 for Families of SMA in honor of Alex Pateakos.

NEW ENGLAND (cont.)

Rhode Island

3rd Annual Working on Walking Golf Tournament and Dinner



This year's event was one we'll always remember.

As our planning continued throughout the summer, the central theme of hope emerged. Each guest was greeted with a green ribbon symbolizing "hope". The hope that not another parent would hear the diagnosis of SMA. The hope that not another child would have to endure the suffering of this disease. The hope that this disease would soon be treatable. Our guests listened and learned about the advancements being made. They understood and realized that this event was much more than a great day of golf and dinner. It was truly an opportunity for them to be part of the cure.

Many obstacles needed to be overcome for us to succeed. The committee worked together and overcame the barriers placed before us. We hosted another successful event earning more than **\$15,000** for FSMA.

We can't be more grateful to Keith Niles, PGA professional at Louisquissett Golf Club in North Providence, Rhode Island for all the assistance he provided and to Twin River Casino for the wonderful dinner and providing the professional guidance we needed to ensure our 300 guests a wonderful evening. We are looking forward to our next year's event which will take place in August.

Mary Smith and Alyssa Silva, SMA Type I North Providence, RI

New Hampshire

Constantine's Carry for a Cure Flag Football Tournament



Constantine's Carry for a Cure Flag Football Tournament was held on August 4th, 2012 at Fuller School Field in Keen, New Hampshire. Participants had a fun filled day of flag football in honor of Constantine Salce, SMA Type II. The tournament, organized by Dominic Salce, raised over **\$6,000** for Families of SMA!

New Hampshire Half Marathon



Several months ago, I made a new friend. Just as we were getting to know one another, her son was diagnosed with SMA Type I. Sweet Ezra Bessey, with his bright eyes and magnetic grin, will not get the chance to move through life as every child should. On November 24th, 2012 I ran the New Hampshire Half Marathon to raise money for Families of SMA in honor of Ezra, and raised **\$2,365!**

*Shelby Hardy
Hallowell, ME*

Maine

One More Trip Fundraiser

The Band of Heathens wanted to do what they could to help raise money for FSMA when they learned that their long-time manager and good friend Ethan Bessey's son, Ezra, was diagnosed with SMA Type I. As the band was in the studio



NEW ENGLAND (cont.)

finishing up their next album, they decided to release one of the new songs "One More Trip", which takes a look back on a hard year to find solace in memories and in love, exclusively as a download after a donation of any amount to FSMA at www.fsma.org/onemoretrip. As word spread and fans of the band started to hear about Ezra and SMA, donations started coming in. To date, the band has raised almost \$5,000 and brought awareness of SMA as well as Families of SMA to thousands of people who had never heard of this terrible disease.

The Band of Heathens, based out of Austin, TX, perform 200+ dates a year throughout the US and Europe. You can learn more about the band at www.bandofheathens.com. The "One More Trip" download fundraiser can be found at www.fsma.org/onemoretrip.

*Ethan Bessey
Hallowell, ME*

Bella's Benefit for SMA Comedy Fundraiser

My event this year was a comedy fundraiser held July 27th, 2012 at Season's Event & Conference Center, located within the Howard Johnson's Hotel in Portland, Maine. The event was held in memory of my daughter, Isabella Sliter, SMA Type I. The three comedians that so generously donated their time were Tammy Pooler, Paul Hunt and headliner Mike Bunker. Although we did not have a big crowd for our first comedy fundraiser, we did have a ton of fun! Here's hoping next year will be bigger!

*Sarah Sliter
West Newfield, ME*



NEW MEXICO

"Bugaboo" Cure SMA Awareness WOD 2012

On Saturday, August 18th, 2012, the New Mexico Chapter of Families of SMA hosted the "Bugaboo" Cure SMA Awareness WOD 2012 at the Balloon Fiesta Park in Albuquerque, New Mexico. The event was once again a tremendous success, raising almost \$17,000 to fund research to find a treatment and cure for SMA. Thank you to all who have attended the event in the past and who continue to support it each year. A special thank you to Laura Kelly and Laura Hines for their hard work and dedication in making this year's event the best to date. See you all in the fall for the next installment of the Bugaboo WOD!

*Natasha Abruzzo
President New Mexico Chapter FSMA
Albuquerque, NM*

NORTHERN CALIFORNIA

2nd Annual Cure SMA Sacramento Walk-n-Roll

On October 14th, 2012, families, friends and local Cisco employees strapped on their sneakers to help raise money and awareness for the 2nd Annual Cure SMA Sacramento Walk-n-Roll in Sacramento, CA. Nina Daya and her committee put together this amazingly successful event in honor of her daughter, Malena Daya, SMA Type II, and all the local SMA families. Thanks to the generosity of many donors and volunteers, \$13,800 was raised this year!



NORTHERN CALIFORNIA (cont.)

Northern California Walk-n-Roll

Ten years! That's how long Northern California families have gathered for fundraising and socializing in honor and memory of our inspiring SMA members. The Northern California Walk-n-Roll, held August 25th, 2012, was presented with an amazing opportunity. The Charles Rosenbaum Foundation challenged us to raise \$50,000. If we could accomplish this huge task, they would match the funds dollar for dollar. We mobilized our families and friends and together with the grant raised over **\$114,000** for Families of SMA research and patient services! Thank you to the Rosenbaum family for this extremely generous support!

Our unprecedented fundraising was not the only exciting part of our Walk-n-Roll. We again held our walk in San Francisco's beautiful Golden Gate Park. After our traditional stop at the Conservatory of Flowers for our group photo we strolled down John F. Kennedy Drive to picturesque Rainbow Falls.

This year the San Francisco firefighters brought the "Crucible", a restored fire truck with a BBQ on back, and together with our volunteers grilled hamburgers and hot dogs for all of us. The Black Sheep Brass Band returned this year to entertain and lead us on our walk. After our walk we enjoyed lunch, games, a raffle, catching up with each other and greeting new families in Peacock Meadow, our event site.

Thank you to The Charles Rosenbaum Foundation, our fundraisers, donors and event planners, without you this event would not have been such a success!

*Pat and Dick Wolff
Sunnyvale, CA*



NORTHERN CALIFORNIA (cont.)

Climb4SMA



With over \$3,600 raised for SMA research, my climbing partner – Chris Ivey and I woke up around 12:30 a.m., the morning of July 21st, 2012 to head up Mount Shasta in Siskiyou County, CA. On the way down, Chris got a great photo of me with our fundraising banner which we both signed and gave to my nephew, Logan Ragland, who has SMA Type II and is the inspiration for this climb. I am

very honored and thankful for the many pledges donated to this effort, and am very encouraged by the many SMA clinical trials now underway.

*Dan Ragland
Sacramento, CA*

5th Annual BACFill

On September 8th, 2012, 50 people gathered inside Alameda High School in Alameda, CA to participate in the 5th Annual BACFill Crossword Tournament. Thanks to the generosity of the tournament participants, \$1,460 was raised for Families of SMA! We look forward to next year's event.

*Andrew Lawrence
Alameda, CA*

Connie Fidel's 50th Birthday Party

Donations were collected at Connie Fidel's Birthday Party in Dublin, CA on October 6th, 2012. Thanks to the generosity of family and

friends, \$975 was raised on her behalf in honor of Danny McHale, SMA Type II!

Barbecue for a Cure

Barbecue for a Cure, an annual adventure in barbecue and music in support of Families of SMA, that took place on August 28th, 2012 in Citrus Heights, CA. The event was a tremendous success and raised \$720. The entertainment also added to the excitement and importance of this event because 27-year-old Jeremy Waggoner, SMA Type I, and his musician friends wrote and performed a personal tribute to "Life with SMA." The recognition of Families of SMA, and the added awareness of Spinal Muscular Atrophy that our event generated in the local Sacramento news media has been the most rewarding of all for those involved, since informing and educating the public about our cause has been our goal from the very beginning.

*Wayne and Fidela Waggoner
Antelope, CA*

OKI

Ohio

Aubrey Grace Lyden Memorial Golf Scramble



The First Annual Aubrey Grace Lyden Memorial Golf Scramble for Families of SMA is officially in the books! The outing was held August 5th, 2012 at the Lakeside Golf Course in Lake Milton, Ohio in memory of our daughter, Aubrey Grace

Lyden, SMA Type I. We couldn't have been happier with our turnout, despite the foreboding forecast for the day and the rain we did in fact end up getting.

Not a single team dropped out due to the weather, we even added one the morning of the outing! We had a grand total of 30 teams of four golfers, and an estimated 150 people came for the dinner and auction afterward.

Our goal for the event was \$10,000. We raised almost \$24,000 and are shocked, humbled and honored by the generosity of the people who donated to our cause. It has been amazing to have complete strangers hand over checks of significant sizes in memory of Aubrey. She was only here on Earth for 9 months, but her life is leaving one heck of a legacy! We plan on holding this outing 10 years in a row during SMA Awareness Month!

*Sean and Dannete Lyden
Boardman, OH*

is the light of our lives along with her sister, Finley. About 84 hackers showed up to support Blake and Families of SMA. Our event raised \$13,630 and all participants had a great time on a perfect fall afternoon.

*Mark Farrell, Grandfather of Blake
Montgomery, OH*



First Annual Birdies for Blake Golf Outing

The First Annual Birdies for Blake Golf Outing was held on September 29th, 2012 at Green Crest Golf Course in Cincinnati, OH. Blake is a two year old with SMA Type II and



OKI (cont.)

US Marshals Annual Golf Outing

The US Marshals Annual Golf Outing was held on September 22nd, 2012 at Mallard Creek Golf Course in Columbia Station, OH. Thanks to the generosity of all the golfers, \$3,000 was raised for Families of SMA in honor of Zander and Alexis Helfrich who both have SMA. Thank you Ryan Helfrich and Donna Faff for your hard work in organizing this event and the US Marshalls for your support!

Ricky's 1K Fun Run

On November 21st, 2012, the town of Liberty Center, OH hosted a fun run at Liberty Center High School in memory of Ricky Roell, SMA Type I-II. Thank you to Kendra Roell for helping to organize this event and the residents of Liberty Center for their generosity. In total, \$800 was raised for Families of SMA!

First Lutheran Church Rummage Sale

From October 19th and 20th, 2012 the congregation at First Lutheran Church in Findlay, OH held a rummage sale to benefit Families of SMA. Thanks to their hard work, along with the support of Thrivent Financial Lutherans, \$634 was raised!

*Joanne Marquart
Arlington, OH*

Lee Ann's Dairy Delight – Cones 4 A Cure



We held the Cones 4 A Cure Fundraiser at Lee Ann's Dairy Delight on August 18th, 2012 in honor of my granddaughter, Leah Miller of Springfield, OH. We raised \$549 for Families of SMA! I hope one day for a cure to help all the SMA families who have such sweet blessings that suffer with this disease.

*Lee Ann Lopez
Springfield, OH*

Kentucky

SMA Awareness Month Fundraisers 4 Annie

During Awareness month, we remembered Annie Grimes, SMA Type I, by hosting a couple of fundraisers. Howard Family Dental and Dr. Christy Cole hosted a night at the ballpark with the Savannah Sand Gnats in Savannah, GA. The Buckhead Cafe here in our hometown of Bowling Green, Kentucky hosted a Dine 4 Annie Night August 22nd, 2012. Annie's Nana, Sheila Violette, also sold raffle tickets for various prizes. It was a great month with lots of support! Over \$1,695 was raised in August 2012 in memory of Annie! Thank you for all you do. We look forward to hosting more events in the near future.

*Sincerely,
The Grimes Family
Eddy, Nicole, Garrett, Lainey, Emi & ^Annie^
Bowling Green, KY*

gathered for a fun-filled morning of activities. Everyone enjoyed lots of games, the scenic walking trail and the butterfly release. Thank you Michelle Palmer for organizing the event in memory of your daughter, Taylor, SMA Type I, and all of the teams for their hard work in fundraising. Over \$17,000 was raised to help find a treatment and cure for SMA!

Xander Powers Golf Benefit



The 2nd Annual Xander Powers Golf Outing held September 22nd, 2012 in Ossian, IN was a huge success! We raised enough money to donate over \$3,000 to your great cause in memory of Xander Powers, SMA Type I! Thank you for helping to make a difference in many lives. We hope that we see a cure for this horrible genetic disorder that took our precious Xander in our lifetime.

*The Powers/Kenline Family
Ossian, IN*

Indiana

OKI Chapter Indiana Walk-n-Roll



The 1st Annual OKI Indiana Walk-n-Roll was held on August 18th, 2012 at Fort Harrison State Park in Indianapolis, Indiana. Friends and family

Hamilton Heights High School Honor Society Service Project

The Hamilton Heights High School National Honor Society from Arcadia, IN would like to give a \$100 donation to Families of SMA in honor of Alexander Davis, SMA Type II. Alexander's Grandmother, Jill Davis, has been a wonderful faculty member here at Hamilton Heights and a big supporter of our National Honor Society for many years. It is a pleasure for our students to have the opportunity to give back to Mrs. Davis' family. We look forward to contributing to Families of SMA in the years to come!

*Sincerely,
The Hamilton Heights High School National Honor Society*

PACIFIC NORTHWEST

Washington

5th Annual Cure SMA Seattle Walk-n-Roll

The 5th Annual Cure SMA Seattle Walk-n-Roll was held October 14th, 2012 in Seattle, Washington. The Pacific Northwest Chapter, family and friends gathered at the Bathhouse Theatre in Greenlake Park to raise funds and spread SMA awareness. Thanks to the hard work and dedication of the chapter, Rick Jones and Russ and Kelly Hargrave, over \$12,000 was raised!

Trash the Cash for a Cure White Trash Party & Fundraiser

This past summer we had a small get-together with family and friends on August 18th, 2012 in Yakima, WA. The original plan was to have a White Trash Party in honor of our daughter Peyton, SMA Type III. To incorporate the SMA theme, we called it, "Trash the Cash for a Cure." Guests were asked to dress in their best "White Trash" outfits, bring a very white trash side dish or drink and drive their best white trash rig. While at the "shin-dig" I had a garbage can set up for donations for Families of SMA. I educated the crowd about SMA, genetics, current research and carrier testing. There were only around 25 guests there, but we were able to raise \$350. We are hopeful and optimistic that there will be a treatment or cure someday!

*With all our love,
The Yates Family
Yakima, WA*



PENNSYLVANIA

Chapter Update

Greetings from the Pennsylvania Chapter!

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

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

TOGETHER WE WILL FIND A CURE!

Sweet Baby Zane Fights SMA

On September 30th, 2012, the 4th Annual Zane's Run was held in Malvern, PA. Zane's Run is held in memory of our daughter, Zane Schmid. She was a beautiful baby girl who passed away when she was 5 1/2 months old from SMA Type I.

Each year, the event gets bigger and better, as our family and the co-organizers strive to grow. The weather was beautiful, there were roughly 600 attendees, and many local companies sponsored the event. We sincerely appreciate all the people who support our event by attending, volunteering and donating. Further, we received a great surprise this year. Through an anonymous donor, an additional \$25,000 was contributed to Zane's Run, an incredibly generous donation! Over \$45,300 was raised for Families of SMA! A special thank you goes out to Meridith Bebee for all of her hard work and dedication in organizing this event!

We love and miss you Zane . . .
To finding a CURE,

*The Schmid Family
(Keith, Hillary, Avery, Brennen & Braxton)
Exton, PA*



PENNSYLVANIA (cont.)

The Lukie's Crew Rocks the Roseto Centi'anni Parade



Check out all the new photos of The Lukie's Crew at the Roseto Centi'anni Parade! We had so much fun and "LT the Lukie Tiger" was so excited to be there. Congratulations Roseto! Lukie's Fall Festival Won 2nd Place!

9th Annual Lukie's Fall Festival

On Saturday October 6th, 2012, the 9th Annual Lukie's Fall Festival was held in Pen Argyl, PA in memory of Luke Maida, SMA Type I. It was a cloudy, chilly day with a few sprinkles of rain from the dark clouds. The weather did not matter though because wherever you were in the park, the sun shined through all the people who came out to support us.



We began our day with the Opening Ceremony where we remembered our Lukie, all the SMA Angels and honored all the SMA Heroes. Lukie's pumpkin patch, the historic carousel, hay ride, mini golf, bounce houses and fire safety house were always filled with happy faces. The games, face painting, sand art, and free book giveaway kept everyone busy.

The stage was filled with performances by the Deadliners Band, bagpipers and cheerleaders, and the annual Pie-Eating Contest! We were honored to host many SMA Families from near and far, and are always happy to have their support! Thanks to you, we were able to raise over **\$10,300!**

For the 9th Annual Lukie's Fall Festival, the family of SMA Hero Omar (Junie) Hardy, SMA Type I, surprised the Maida family with a donation to cover the cost of renting the park. Omar's wonderful family has volunteered at Lukie's Fest for the past two years, and are such a wonderful help. Thank you to the Mohammed and Hardy Families for all your continued love and support! "Together We Will Find a Cure!"

*The Maida Family
Roseto, PA*



The Gary in the Morning Radio Show

Thank You to The Gary in the Morning Radio Show on Pocono 96.7 FM and Elissa for hosting the Maida Family on your radio show in Port Jervis, NY in fall 2012. You helped us spread Awareness about SMA and let everyone know about Lukie's Festival in memory of Luke Maida, SMA Type I. We had a great time!



PENNSYLVANIA (cont.)

9th Annual Lily Kennedy Golf Outing

The 9th Annual Lily Kennedy Golf Outing held October 7th, 2012 was a great success! The event, held at the Iron Masters Country Club in Roaring Springs, PA, was sponsored by St. Francis University Social Work Club under the direction of Dr. Mark Lynch and Professor Suzanne Black. Thanks to the generosity of golfers and donors, \$2,900 was raised for Families of SMA in memory of Lily Kennedy, SMA Type I! Thank you to the St. Francis University Social Work Club for your hard work in planning this event.



Philadelphia Phillies Game! Our chapter families were joined by SMA families from all over Pennsylvania.

We were invited to educate the fans about SMA in a Public Service Announcement on the new Jumbotron in front of 43,651 fans! Facts about this terrible disease and photos of all our wonderful children were recognized in our PSA. Big thanks to Norman Einhorn of Unique Video and Amy Pruitt of Society Hill Studios for all of their help! A percentage of our ticket sales were donated by the Phillies to Families of SMA totaling \$454. What an amazing opportunity to spread SMA Awareness! Thank you to Karen Negrin for organizing this event.

Emmy's Crop for SMA



Instead of our annual Crop Party, this year we held a raffle in memory of our daughter Emmy Rose Baugher, SMA Type I, December 12th, 2012. It was something simple, but we still got the word out about SMA while helping a good cause! We were able to raise \$2,000!

*Brandy Baugher
Hanover, PA*

3rd Annual SMA Awareness Night with the Phillies

On August 24th, 2012, the PA Chapter hosted the 3rd Annual SMA Awareness Night at the

family, Carol Todd, and Jennifer McFadden, hosted the Mountain House Country Store Fall Festival in Saylorsburg, PA to benefit Families of SMA in memory of Luke Maida, SMA Type I. They had giveaways, a parking lot flea market, hot dogs, caramel apples drinks, and an SMA Awareness table setup by the Maida family. Thank you so much Carol and Jennifer for donating a portion of sales and raising \$275 for Families of SMA!



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

*In Friendship,
The PA Chapter of Families of SMA*

Thank You to "DDD Today"

DDD Today is a Publication of the Division of Developmental Disabilities from the NJ Department of Human Services. They reached out to the Phillies to ask about promoting one of the charities that sponsor the Phillies Corporate Night. The Phillies provided them a list of all participants and DDD Today picked us! Volume VI, Issue VIII was featured in August 2012 and promoted our event, but more importantly they informed their readers about the great work we do supporting our SMA families.

Bosco's Friends Helping Friends Fundraiser

On October 16th, 2012 Jeanette Foor promoted the Bosco's Friends Helping Friends Fundraiser being held at the local Bosco's department store in Altoona, PA. Jeanette sold \$5 passes for discounts to the store in honor of her granddaughter Eden Cheslock, SMA Type II, and raised \$340 for Families of SMA. Thank you, Jeanette!

Mountain House Country Store Fall Festival

On October 13th, 2012 friends of the Maida

Lyla Mertz Foundation



3rd Annual Dance Away SMA

On September 29th, 2012, Lyla's birthday, the Schnecksville Fire Company Pavilion in Schnecksville, PA was the place to be! The popular Chas Band had everyone up on their feet. A hot dinner, spirits, live entertainment and an overflowing Chinese Auction guaranteed that the 3rd Annual Dance Away SMA would be a huge success! One hundred and thirty-eight guests gathered together to

PENNSYLVANIA (cont.)

help us raise over \$5,730! Jen and Steve Mertz go to great lengths to honor as many children and adults challenged by SMA as possible. Adorning the walls of the pavilion are countless beautiful smiles, reminding us all of the reasons we give. All proceeds were donated to Families of SMA to continue their fight to cure this horrendous disease in memory of Lyla Mertz, SMA Type I.

Swing for a Cure 2012

On August 26th, 2012, Lyla's friends came together on the greens of the Mahoning Valley

Country Club in Lehighton, PA to help CURE SMA. It was a beautiful day, but the fun was not just reserved for the golf course. Contests including a hole in one, closest to pin, and longest drive inspired a healthy competition among the participants! Fun was had by all as Mertz family and friends finished up with a delicious barbeque. With the support of 60 golfers, over \$2,000 was raised for Families of SMA in memory of Lyla Mertz, SMA Type I!

Silpada Fundraiser

A Silpada jewelry open house event was held

November 3rd, 2012 at the home of Robin Hagy to support the Lyla Mertz Foundation and Families of SMA. Shelly Brown, Silpada sales consultant, graciously donated a portion of all sales from the party. The total donation was \$825. Thank you, Robin!

Tastefully Simple Fundraiser

During the month of September 2012, Marisa Mertz, Tastefully Simple sales consultant, donated a portion of her sales to Families of SMA. The total donation was \$220. Thank you, Marisa!

ROCKY MOUNTAIN

Colorado**Colorado's Cure SMA 5K Walk-n-Roll & Run**

Thank you to everyone who participated, donated and volunteered for our Cure SMA Walk-n-Roll & Run September 8th, 2012! This year's walk was held at Clement Park in Littleton, Colorado.

Because of your dedication to raising awareness and funds to help find a treatment and cure for SMA, we broke all of our records

and raised nearly **\$56,000** in support of Families of SMA. That's a \$10,000 increase over last year's event! Thank you!

Special thanks goes to the Mile High Tumblers and the local reps who participated from Quest Diagnostics and BAYADA Home Health Care. We would like to send an extra thank you to the following top five teams and individuals that devoted so much energy to raising funds for this great cause. Your dedication to the Walk-n-Roll & Run is inspiring and appreciated.

Top 5 Fundraising Teams: Team Jack, Team Lino, Angel Jay's Brigade, Team Anthony "L-O-V-E" and Team Adriana

Top five Fundraising Individuals: Ketan Jobanputra, Julie Lino, Carlos Gomez, Amy Shaklee and Marla Marlow.

*Julie Lino
Denver, CO*

Run for Sasha

My name is Emma Reynolds of Fountain Valley, CO and I ran a half marathon in honor of my little sister, Sasha Grace Reynolds, on July 1st, 2012. Sasha was diagnosed with SMA Type I when she was born. She died January 10th, 1999, when she was 8 days old. I have always wanted to do something to commemorate her life, so I ran and raised over \$628!

*Best regards,
Emma Reynolds
Fountain Valley, CO*



SOUTHERN CALIFORNIA

Chapter Update

The Southern California Chapter is Rolling Along! This past holiday season concluded one of the busiest years ever in the chapter and we are hoping to keep the momentum going this year. From Walk-n-Rolls, to bake sales, to garage sales and even a little skydiving, we had a one of our most successful years to date. The chapter would like to thank everyone in the area who participated, donated and attended the various activities organized this last year. Special thanks to all of our families who continue to give and support our efforts by donating and attending more than one event if not all of them. All of you provide the spark needed for us to take our chapter and more importantly our cause to the next level. We hope you all enjoyed the holiday season and are ready to do it all again this year even bigger!

SOUTHERN CALIFORNIA (cont.)

5th Annual Southern California Chapter Walk-n-Roll



The 5th Annual Southern California Cure SMA Walk-n-Roll held November 4th, 2012 in Burbank, CA was a huge success. Over 500 walkers and rollers turned out for a beautiful day of family fun at the Johnny Carson Park. Over **\$56,700** was raised at this year's event! Thank you to those who worked tirelessly to make this happen, to our teams of walkers and to our sponsors. Because of your help, we are truly one step and one roll closer to finding a treatment or cure for SMA! Special thanks to event organizer Rosie Roope and Chapter Presidents Rickk and Autumn Montoya.

Jump for SMA



Jump as in SKYDIVING. Yes, you heard it correctly. We were able to find some very brave and devoted heroes to take up the task of fundraising and skydiving to find a cure. On August 18th, 2012, a few hundred friends, families and supporters gathered at Skydive Perris in Perris, CA to cheer and root on 19 jumpers and enjoy a day in the sun. Our jumpers were comprised of parents of SMA children including angels, friends and relatives. Each jumper was tasked with raising a set amount of money and to either skydive or participate in an indoor wind tunnel experience in a show of support for SMA. Everyone in attendance braved the 100+ degree heat while enjoying the food, music,

pool and the exhibition of heroism provided by our brave jumpers. In all, over **\$13,000** was raised for the cure and many checked a big one off of their bucket list.

4th Annual Inland Empire Cure SMA Walk-n-Roll



On September 23rd, 2012 we held our 4th Annual Inland Empire Cure SMA Walk-n-Roll at the La Sierra University track in Riverside, CA. Approximately 200 people participated this year. Even though it was a hot day, it was nice to see all the people that came to support us and our fight against SMA! We are so happy to say that we reached our goal and raised over \$6,930!

I would like to thank everyone that helped us put this walk together, especially Angel G., Letty Portocarrero, Delker Sumier, Wagner Portocarrero, Christy Green, Christy Ekkman, Nicole Wagley, and Rickk and Autumn Montoya. We also can't forget about our band, The Northern Strangers! Thank you and as I always say, "This is not a one person fight, we must unite our strength and fight against SMA!"

*Evelyn Vasquez
Moreno City, CA*

Orange County Mustang Club Fundraiser

The Orange County Mustang Club in Orange County, CA donated over \$582 in honor of Kennedy Montoya, SMA Type II. This money comes from a 50/50 raffle held in Kennedy Montoya's name on October 9th, 2012. Kennedy is my grandson, and the club has adopted him as their "special friend." When he is available, he picks the winner at our car shows. I want to thank the Orange County Mustang Club for their continued support in finding a cure for this disease.

*Renee King
Garden Grove, CA*

Finding a Cure Through Clutter

Jaclyn Davis hosted Finding a Cure Through Clutter August 24th through 26th, 2012 in honor of Isabella Davis, SMA Type II. Family, friends and strangers donated items to be sold at the yard sale and recyclable items to cash in. Thanks to Jaclyn and the Davis Family, \$980 was raised for Families of SMA!

Angel Sky Mom Designs



Angel Sky Mom Designs donated over \$96 in memory of Skylar Bahrenburg, SMA Type I. This money is a portion of the proceeds from hand-crafted jewelry and keepsakes made by Liz Bahrenburg, Skylar's mom! Thank you to Jim and Liz Bahrenburg for making this donation possible.

Chapter Family Picnic

The chapter hosted a Family Picnic in the park to connect with old friends and welcome new families. Approximately 15-20 families from throughout the southern California area gathered on July 29th, 2012 at Craig Regional Park in Fullerton, CA for a day of fun, food and games. It was a "BYOL" (bring your own lunch) themed event with desserts and drinks provided by the chapter. The children had fun rolling free together in the grass and playing various games. There were also plenty of arts and crafts as well as a spin art machine where everyone was welcome to make a personalized colored frisbee. Parents enjoyed the opportunity to relax and talk amongst each other and many bonds were made.

SOUTH FLORIDA

2nd Annual Gala of Hope

The 2nd Annual Gala of Hope to Cure SMA was held on November 8th, 2012 at the Ferrari-Maserati of Ft. Lauderdale in Ft. Lauderdale, Florida. The event was held in honor of Mia Israel and Madison Smith, both SMA Type II. Thank you to the Gala of Hope event organizers, moms to Mia and Madison, Fiorenna Israel and Jennifer Miller Smith, South Florida Chapter President, for all of your hard work and dedication to planning this amazing event.

The event featured a cigar lounge with complimentary cigars, a huge silent auction and Fund-A-Need program, generous servings of food and desserts, and free tickets to Improv Comedy Club. Artist Gary Longordo exhibited a variety of artwork at this year's gala with a percentage of sales generously donated to Families of SMA. Also, once again this year, we were excited to have Kavita Channe, reporter for Fox Sports/Prep Zone, VIP TV & 1stDownnDirty join us at the exclusive red carpet entrance! Super Bowl champion Plaxico Burress from the New York Giants joined us for the evening, as well. Finally, the evening wrapped up once again with a drawing for a \$10,000 gift certificate to J.R. Dunn Jewelers, one of the event's top sponsors!

Because of Fiorenna and Jennifer's hard work soliciting sponsors, gathering auction items, recruiting donors and attendees, and planning logistics with many, many volunteers, over **\$123,000** was raised to help find a treatment and cure for SMA! The Gala of Hope has now raised over \$253,000 for Families of SMA in just two short years!



SOUTH FLORIDA (cont.)

The Great EsSkate Fundraiser

The Great EsSkate Fundraiser was held on November 7th, 2012 in Miami Beach, FL. The annual three-day in-line skating and vacation event donated a portion of their proceeds to Families of SMA totaling almost \$2,000! The donation was given in honor of event organizers Donna Wiedema, Francisco Lopez, Bernadette West, Rita Starr, Corrine Kirkland, Bruce McNamara, Paolo Tomasi, Damir Romic and Larry Kaplan. Thank you to The Great EsSkate and its organizers for their generous support!

SMA Family Luncheon

On Sunday, September 16th, 2012 the South Florida Chapter of Families of SMA teamed up with Quest Diagnostics to provide all chapter members with an SMA Family Luncheon at Dave & Buster's in Hollywood, FL! The evening proved to be a great time for all of the families that were able to attend and spend a night socializing with friends and meeting new local chapter members. Thanks to Quest Diagnostics and Jorge Hernandez for funding this awesome get-together for our local families!



SOUTH JERSEY/DELAWARE

New Jersey

Cocktails for a Cure

On October 27th, 2012 Jessica Moyer and Jocelyn Talbert hosted the Cocktails for a Cure fundraiser in memory of Jessica's son, Steven Moyer, SMA Type I. Guests enjoyed live music, food, silent auction and a 50/50 raffle! Through the generosity of many attendees, the event raised over \$7,800! Thank you to Jessica and Jocelyn for all of your hard work planning this event!

Delaware

SMA Golf Tournament in Memory of Steven Moyer

The Blue Ridge Trail Golf Club in Mountaintop, PA was the setting for the 10th

Annual SMA Golf Tournament in Memory of Steven Moyer on August 25th, 2012. Our annual tournament is dedicated to the memory and influence of our grandson, Steven L. Moyer, who passed in 2002 at nine months of SMA Type I complications. Since the first event \$30,000 dollars has been raised. This year we raised **\$10,100!**

The success of our 10 tournaments is a tribute to the area golf community, business community and our many friends who have made this event a yearly success.

*Pat and Steve Moyer
Magnolia, DE*

Red Clay Market Day Fundraiser

North Star Elementary raised \$1,370 in honor of Natalie Dell'oso, a 4th grader at our school who has SMA Type III. Each year the fourth grade students at North Star Elementary in

Hockessin, Delaware organize a Market Day where we utilize the skills we have learned in our Economics unit. The students become entrepreneurs for the day and utilize the knowledge that they have learned to run their own businesses. Each year as a class we decide what charity will benefit from our Market Day success, and this year we raised \$1,370 for Families of SMA. In spite of all that Natalie has been through, she is always smiling, kind and a friend to everyone. As soon as we started to discuss what charity we would donate our funds to, many students immediately thought of Natalie. Thank you for all of the support you provide to children with SMA.

*Sincerely,
Fourth Grade Team from North Star
Elementary
Hockessin, DE*

TENNESSEE

Carlee's BIG SMA Shoot Out in Support of the Families of SMA- Tennessee Chapter

Carlee's BIG SMA Shoot Out is the first of its kind as a Families of SMA fundraiser! The Shoot Out was a sporting clay and skeet shoot held on October 27th, 2012 at the Nashville Gun Club in Nashville, Tennessee in honor of Carlee Beam. This event brought in 110 shooters, more than 50 volunteers and another 100 attendees. In its first year the event raised more than \$54,600 for Families of SMA.

The event consisted of shooters competing for prizes, fun shoots, lunch and a silent auction that raised over \$6,000 on its own. Premier Orthopedics was the event's Title Sponsor, along with 30 other main contributors and many other monetary donors.

Cure for Carlee was created by Jason and Kelli Beam in order to educate the community about SMA and other muscle disorders.



TENNESSEE (cont.)



Our four year old daughter, Carlee, was diagnosed with SMA Type II-III at age two. For more information please visit www.cureforcarlee.com.

*Kelli and Jason Beam
Murfreesboro, TN*

Thank you to Kelli Beam for all of your hard work and dedication in planning this wonderful event. Not only did you raise funds that will be used for critical research and family support programs, but you also are inspiring hope that one day we will find a treatment and cure for Spinal Muscular Atrophy!

First Annual Tennessee Chapter Walk-n-Roll Away SMA

The Tennessee Chapter hosted Walk-n-Roll Away SMA August 19th, 2012 at the Ijams Nature Center in Knoxville, TN. Everyone enjoyed a day full of adventure while spreading SMA awareness. Attendees were able to participate in road rides, take guided mountain bike rides or walk and stroll through the Ijams Greenway. There were also lots of kid friendly activities including a treasure hunt, insect zoo and face painting. Thanks to the generosity of donors, sponsors and attendees, almost \$20,000 was raised for



Families of SMA! Thank you to Chapter President Sarah Boggess and Denita Guerry for your hard work in organizing this event.

TEXAS

Bella Benefit for SMA

On September 29th, 2012, more than 100 people gathered at the Legacy Park in beautiful Arlington, Texas in memory of three beautiful SMA Type I angels, Isabella Madison, Addison Bella and Natalia Isabella, by participating in the 1st Annual Bella Benefit. The entire community rallied behind our event and showed us all what it looks like when a group of people get together to make a difference in this world. After setting and raising our goal three times, the Bella Benefit raised over \$12,700 to support the amazing research funded by Families of SMA! Thank you to all of the families, friends and entire Arlington community for their tremendous support in creating this event. We can't wait to start planning for 2013!

*Jose & Valerie Trevino
Edgar & Jacqueline Valencia
Fort Worth, TX*

2nd Annual FSMA Texas Bowl-a-Thon

On September 22nd, 2012 more than 200 people gathered at the AMF Showplace Lanes in Austin, TX for the 2nd Annual FSMA Texas Bowl-a-Thon. The day was a tremendous success and raised close to



\$12,000, blowing the previous years total out of the water and setting the stage for an even more spectacular event in 2013.

Thank you to all the families, friends and SMA superstars who participated. See you in the fall!

*Kelly Coggin
Hutto, TX*

Wine and Cheese in Honor of Ezra

On Saturday, December 1st, 2012, more than 100 people gathered at the home of Adam and Kara Gross, in Houston, TX to participate in the 1st Evening of Wine and Cheese for Ezra. This intimate wine tasting event was created to honor Ezra David Bessey who was recently diagnosed with SMA Type I. Thanks to our amazing friends and family, more than \$5,500 was raised to benefit Families of SMA and support the research they are funding to find a treatment and cure for SMA. Thank you

to all the donors, participants and volunteers who helped make the inaugural year of this event such a tremendous success. A special thanks goes to the Tasting Room Wine Café for donating the wine for the evening's event.



UTAH

The Utah Chapter has been busy getting organized and spreading SMA Awareness. We have gained several new members and have been planning some upcoming events! If you are interested in joining the chapter or have any fundraising ideas, please e-mail us at Utah@fsma.org.

WESTERN NEW YORK

Chapter Update

The Western New York Chapter covers an area that reaches east to the Syracuse area and south to the Pennsylvania border. It is sometimes hard to reach out to these areas as most of the board members and our meetings are located near Buffalo. This being said, we are currently working on establishing a satellite of our chapter started by a newly diagnosed family in the Binghamton area. This family, which has two sons with SMA Type II, would like to establish the satellite Chapter near the Syracuse area so that more families can get involved, attend events and network with other SMA families closer to home. If you are interested in becoming a part of the satellite chapter, please contact Ashley Ross at Ashley@fsma.org or (800)886-1762 for more information! We look forward to working together to raise awareness.

9th Annual WNY SMARt Walk for a Cure

The 9th Annual SMARt Walk for a Cure was held on August 4th, 2012 at Beaver Island State Park in Grand Island, NY. It was a beautiful day as almost 400 friends and family members gathered together to raise awareness and funds to find a treatment and a cure for SMA. Long lines formed for the balloon making clown and the face painter. Over 200 beautiful baskets and gift certificates were featured in the raffle. A girl's and boy's bike, autographed sports memorabilia, watches, sunglasses, a fitness package and a ring were among the bigger ticket items that drew a lot of attention. Once again, State Senator George Maziarz and local DJ Nickolas Picholas were graciously co-chairmen for the event. Mayor Robert Ortt of North Tonawanda proclaimed August as Families of SMA Month in the city and August 16th Families of SMA Day in honor of Kale Shiesley's birthday,

Thank you to all of our teams that



contributed helping us raise an astonishing **\$74,380**: Joe's Squad, Charli's Team, Alex's Team, Kale's Krew, Team Wienchowski, Jenna's Team, Jake's Team, and Angel Nicholas. A special thank you to our friends from Kohl's for volunteering to help sell SMA merchandise at the event. In addition, Kohl's also donated \$500.

Karen Shiesley
Chapter Secretary

Man's Night Fundraiser



Man's Night started in 2008 when a small group of guys decided to get together for a "guy's night." A night where the buds could get together, eat, drink and enjoy each other's company. We made our own sausage and BBQ sauce, shared a glass of scotch and ate like kings. Before the night was over, guys were raving about the food and couldn't wait to do it again. So we did it again and again, each time adding new items to the menu and each time getting more and more guys wanting to attend. In 2010 Man's Night became so big we decided to turn it into an annual charity event.



WESTERN NEW YORK (cont.)

This year, Man's Night VI was held November 10th, 2012. It was our biggest and best event to date. Over 150 guys attended and we were able to raise \$1,230 for Families of SMA in honor of Kale Shiesley, SMA Type II. The Man's Night event has become a truly unique experience that is constantly growing and evolving. It has become a passion for those of us who organize it and put it on. After every event is over we find ourselves anticipating next year's.

Andrew Pentheros

*Man's Night Co-Founder & Event Coordinator
Buffalo, NY*

Independent Health Dress Down Day

A Dress down day was held to start the fundraising for our 10th walk. It was sponsored by the accounting department of Independent Health. Over \$1,200 was raised at this one day event.

WISCONSIN

2nd Annual Kennedy's Dream Walk-n-Roll

On Saturday, September 29th, 2012 we held the 2nd Annual Kennedy's Dream Walk-n-Roll for SMA at Fox Brook Park in Brookfield, Wisconsin. Kennedy's Dream Team participants dressed in "Kennedy Flair" and gathered to walk and raise money for SMA.

Children had fun decorating event t-shirts, getting temporary tattoos, playing with balloon animals and making sand art jewelry. Kennedy's Dream Team helped us raise over \$12,000 for research and to fund a special item for newly diagnosed families, the Frogg Togg Chilly Pad, in memory of our daughter Kennedy Elizabeth Quinnell, SMA Type I.

We want to thank everyone who volunteered, attended and donated to our walk.

Kennedy changed our lives forever; she taught us so much about loving, living and appreciating life. We are devoted to continue her journey to fight against SMA, so please accept our donation towards finding a cure for SMA. For more pictures of the walk visit www.kennadysdream.org.

*Erin and Corey Quinnell
Milwaukee, WI*

8th Annual Grant Sheppard Memorial Scramble

On August 19th, 2012 the 8th Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Chilton, WI. The total raised in SMA Type I angel Grant's memory this year was \$8,000. The weather that day was absolutely perfect! The golfers and volunteers had a fantastic day filled with lots of fun and laughter. Everyone had a great time golfing, at the silent auction and bucket raffles helping to raise money for Families of SMA. Together we will find a cure!

*Scott, Lisa, Peter and Lily Sheppard
Sherwood, WI*

2012 Round for a SMA Cure

On August 3rd, 2012, Linda Marcou and her committee hosted the 2012 Round for a SMA Cure Golf Tournament in Menomonee Falls, WI. Thanks to their hard work in organizing the tournament and the support of family and friends, the event raised \$6,000 in honor of Benjamin Nelson, SMA Type II.

Team Kherington's Ride 4 SMA

Team Kherington's Ride 4 SMA was held on August 4th, 2012 in Janesville, WI in memory of Kherington Shippee, SMA Type II. The day included a vehicle and motorcycle ride, drawings, a silent auction and a live performance by The Manberries. Thank you to Colleen Hoover and Melissa Shippee for organizing this event! Almost \$3,900 was raised for Families of SMA!

Twin Cities Marathon

Our friend Sheri Rick ran the Twin Cities Marathon in Minneapolis, MN in honor of our daughter, Jackie Hoffman, SMA Type II, on October 7th, 2012. Thanks to Sheri's hard work in training and running, and the generosity of many donors, \$1,900 was raised!

*Love,
Paula, Skip and Jackie Hoffman
Milwaukee, WI*

Fond du Lac County Month-Long Dress Down Fundraiser

The Fond du Lac County Department of Social Services in Fond du Lac, WI held a Month-Long Dress Down Fundraiser to benefit Families of SMA. The department was able to raise \$1,500 in honor of Mateo Medina, SMA Type I! Thank you to Lorie Gregor and Amy Medina for organizing this successful event.

Damon Family Softball Game

On September 1st, 2012, Angela and James Damon hosted the Damon Family Softball Game in Brooklyn, WI. They raised \$175 thanks to the generous support of family and friends in honor of McKenzie Damon, SMA Type II. Thank you to Angela and James for once again organizing this game!

Promotional Materials from Families of SMA Make Planning a Fundraiser Easy and Fun

Fundraising Materials:

- Toolkits, Manuals and Samples Booklet (Walk-n-Roll, Golf Tournament, Dinner/Gala & Bowl-a-Thon)
- Banners and Yard Signs
- Families of SMA "At a Glance" Flyer
- Purple & Orange FSMA Bracelets
- Plastic Event Bags
- Donation Cards
- Promotional Tips
- Purple & Orange Golf Tees
- Temporary Tattoos
- Coin Canisters
- Families of SMA Pens
- Angel Tags
- Handheld Flags
- Start Flag
- Pop-up Signs
- Tablecloth
- And more!



Merchandise



Cinch Bag | \$10



Purple Grocery Bag | \$4



"Supporting Hands" T-shirt | \$12
Youth sizes: S M L • Adult sizes: S M L XL



Greeting Cards | \$6
(for a pack of 5 cards and envelopes)

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



Seascape Art Canvas Tote | \$10

Run with Team Families of SMA

Families of SMA is excited to announce that so far, we have been accepted as an Official Charity Partner for four running events in 2013!

Each year, Team Families of SMA invites a dedicated group of individuals to participate in marathons, half-marathons and other running events across the United States who share one vision: *a world where Spinal Muscular Atrophy is treatable and curable*. As a member of Team Families of SMA, you have a direct impact on the SMA Community through raising awareness and building our network of support. Dollars raised are helping to fund the research, support and care initiatives that Families of SMA will fund in 2013.

Registration for current Team Families of SMA events is now available online.



NYC Half Marathon

Sunday, March 17th, 2013
New York, NY
www.fsma.org/NycHalf

The 15th Annual Flying Pig Marathon

Sunday, May 5th, 2013
Cincinnati, OH
www.fsma.org/FlyingPig

2013 Delaware Marathon

Sunday, May 12th, 2013
Wilmington, DE
www.fsma.org/DelawareMarathon

17th Annual Chicago Half Marathon & 5K

Sunday, September 8th, 2013
Chicago, IL
www.fsma.org/ChicagoHalfMarathon

Join Us for Our New Fundraising Workshops

From the seasoned “pro” to the novice event planner, Families of SMA hosts workshops to help everyone increase their effectiveness as fundraisers.

Visit www.fsma.org/fundraising to register today!



Building and Managing a Successful Fundraising Team

The most successful fundraising events are those that rely on fundraising teams to generate donations. Join us for this workshop and we will show you how to build and train your teams to help you reach your goals.

-Wednesday, July 10, 2013 at 6:30 p.m. (CST)

-Wednesday, November 6, 2013 at 10:30 a.m. (CST)

The Ask

In order to get people to donate to your cause, you first have to ask them! Join us for this workshop and we will show you how to create a clear and direct ask. Having an ask is the first step in creating a successful fundraiser!

-Wednesday, May 1, 2013 at 6:30 p.m. (CST)

-Wednesday, September 4, 2013 at 10:30 a.m. (CST)

Fundraisers in Honor of Owen Norton

Will Lacey, a Braintree, MA third grader, raised \$315 for Families of SMA in honor of Owen Norton. In lieu of birthday gifts, Will requested donations for SMA. Will suffers with his own disease, Neuroblastoma. He and his family are always supporting our fundraising efforts for SMA. Thanks Will!

Neighborhood friends of Owen Norton had summer lemonade stands for SMA. Caitlin Callahan and Emma Flannery raised \$10.25. Meghan Duffy and Delia McGuire raised \$6.50. The Norton Family is very proud and grateful of the continued support towards a cure for SMA from our Braintree, MA community.

Thank you, Kathy Norton, Owen's Mom
Braintree, MA

Hi Everyone,

Avery came to me the other day holding her piggy bank and said "I want to give this to help the kids like Zane, her twin sister." Yes, tears! On my part. So, here is her message word for word:

"Avery is giving the money to help the kids. I hope you feel better."

Thank you,
Hillary Schmid of Pennsylvania



"Mom and Me" by Morgan Sidor wins a National Art Contest 2012 All Kids Can Create!

In May 2012, Morgan Sidor was spending his 8th birthday in the intensive care unit in the Children's Hospital of Philadelphia with pneumonia. He was missing the PA Chapter Walk-n-Roll that he had signed up for and was looking forward to attending on his actual birthday. He was very disappointed and resting in his hospital room when a phone call from his school, HMS School in University City, came to announce that he had won a national art contest!

His HMS School art specialist, Eiko Fan had submitted a painting of his called "Mom and Me" in the 2012 All Kids Can CREATE contest "What Inspires Me." He won! For the month of August, his painting would be displayed alongside the works of 101 winning student artists on the walls of the Martin Luther King, Jr.

Memorial Library in Washington, D.C.

Ms. Fan established the art program 25 years ago to help children with severely limited mobility access art. For the contest entry, she placed Morgan's hand in the middle of the canvas, where he wiggled his finger to move the acrylic paint and plaster.

His painting was chosen from more than 3,000 entries by students from ages 5-15, with and without disabilities, in the VAS and CVS Caremark sponsored exhibition. We are so proud of you Morgan!



Hot Chocolate Fundraiser

My coworker's kids asked what our recent fundraiser was all about, and when she told them it was for SMA and Brady Chan, they wanted to hold their own fundraiser. They gathered their friends on a chilly and windy Sunday afternoon, and set up a hot chocolate, hot cider and freshly baked cookies stand in their neighborhood. After 3 hours of FUNdraising they earned \$59 for FSMA :-)

Mimi Chan of North Carolina



The Henkel Family is Reaching New Heights

This is Peter Henkel, who has SMA Type II, with his siblings and dog, Phin, in our accessible tree house. The tree house is designed especially for Pete by Uncle Dan and lots of sweat equity by Dad and brother, Ian.

Now we're working on a chair for the zip line!



Dear Moneta Eudana
Thank you for the beautiful blanket. I love it!
LOVE DEMYA RIVERS

Dear SMA
Thank you for the gifts.
Love DEMYA RIVERS

Demya Rivers, Tennessee



Cousins for a Cure in honor of Emily Lozina

This summer, Cousins for a Cure was busy raising awareness of SMA and collecting funds toward a cure with bi-coastal lemonade stands. Three lemonade stands were set up across the country in Sammamish, WA, Cape Cod, MA and Greenwich, CT. The 11 cousins (and 1 big brother) who sold lemonade handed out FSMA bracelets, pens, and info flyers. The age range was from four years old to 12 years old. Through their hard work and dedication, they raised \$250 to be donated to FSMA in honor of their little cousin Emily Lozina, who is 19 months old with SMA Type I. We are so proud of the hard work and support from all of the cousins, and Emily's big brother. These proceeds will support Laurette Zion's, Emily's aunt, NYC Marathon fundraising efforts.

The Lozina Family, Kresh, Jenn, Christopher and Emily
Cos Cobb, CT

Kitah Vah Hewbrew School Fundraiser

Ali Badain, friend of Ariana Dindzans, SMA Type III, and her 6th grade Hebrew School class, Kitah Vah, decided to learn more about SMA. After learning about SMA, the students collected money earned through chores and allowances to help find a cure and raise SMA awareness! Thank you to Ali and the Kitah Vah class for raising \$53 in support of Families of SMA!



Barzach Lemonade Stand

On July 20th, 2012 Michael Barzach of West Hartford, CT sold lemonade in memory of Jonathan Barzach. Thanks to Michael's hard work, he raised \$36 for Families of SMA! Michael also wrote a paper for school about SMA and Families of SMA. Thank you Michael for spreading SMA awareness in your community!

Hello,

My name is Kennedy Robinson and I am 9 years old. I was diagnosed with SMA Type I when I was 9 months old. I have never let my condition hold me back from doing the things that I love and want to try. I was baptized by total submersion, my choice, when I was 3 years old. I sing in the children's choirs at my church Good Shepherd Temple Of Praise. I love dressing up and playing on my custom built swing and my trampoline. I love to swim and sing karaoke with my dog Tink. I have a great support system that includes my mom, Tiffany, older sister, Courtney, my Granny, Papa, my Nannie, uncles and cousins. They help me with school and we play games together all the time.



Kennedy J. Robinson with help from my Nannie Michelle of Texas



Moon Watcher

He never gets rest
Always hoping for the best.
Wishing and praying it would go away
Always wanting to run around and play.
Looking at the man in the moon
Wishing his hero would come soon.
Waiting for the cure to appear
Hoping one day his family wouldn't shed another tear.
He gets stronger everyday
Wishing it would go away.
Yea, he's a fighter
Always watching the moon get brighter.

By Ally Spivey, 8th grade, Lake View, SC

For her little brother, Ethan, SMA Type I, 8 years old

My First Day at Harrison High

By: Kate Saldana, SMA Type II

November 19, 2012
2nd Period English I
Memoir

As I sit down to write this memoir, I am using the lessons that I learned about perseverance. It reminds me of the first day of my high school career on August 15, 2012.

It was bright and early in the morning, I barely slept the night before because I was so nervous and excited for the next day. My mother came into my room to wake me up, but I was already wide awake. My mother was surprised to see this because I am never awake in the mornings. She helped me get up and ready for the day. As I was eating my yummy eggs and bacon that my dad had made for me, there was a knock at the door.

It was Emily. Emily is my nurse. She comes to help me every day. I am an average 15 year old teenage girl, but I am also different from most.

You see, I was born with a genetic condition called Spinal Muscular Atrophy or SMA. SMA is a disease that causes my muscles to not function properly. Since my muscles aren't very strong, like normal teenagers, I am in a wheelchair. This condition also affects my lungs. At times it can be hard for me to breathe, so I have a tracheostomy, which is an artificial airway, and a ventilator to help when my lungs get tired. Because of my condition I need nursing care twenty four hours a day.

So now you have a better picture of me, let's continue.

While finishing getting ready I can hear the old, yellow school bus rumbling down the street and the brakes squeaking loudly as it stopped in front of my house.

My dad yells from his office, "Kate, Your Limo is here!"

I was confused, "A Limo?"

"No Silly I am talking about your school bus."

"OHHHH! OK. I am off to school"

As Emily and I leave for school, my Mom yells, "Have a great day, Make bad choices!" while smiling.

While waiting for the wheelchair lift on the school bus to come down, my dad runs out the door and said "Wait I need a picture!"

"Gosh Dad really!"

"Ok Emily get in closer to Kate, this is a BIG day!"

As dad snapped the picture I replied, "Ok Dad, I have to go to school now"

Then came the bumpy ride down the street to school.

I get more and more excited and less nervous the closer we get to school. As I was getting off the bus, I was amazed at the number of students standing outside the front entrance. I didn't recognize anyone from my old school.

As I entered the school, I was amazed at the size of the building. I wanted to be early to my first hour class so that I could meet my teacher. Since I am wheelchair bound I need special tables to sit at with Emily.

The first thing I noticed was that I had no place to sit. I had hoped the other classes would have tables for me.

My first day had started off on a bad foot. First hour went great, but then came the struggle to get through the small hallways packed with students.

As Emily and I try to get through the hallways, nobody is moving out of my way.

Emily became very annoyed with the complete lack of manners and rudeness of the kids in the hallways. Emily tells me,



"Just run them over, then they will move out of your way!"

I replied, "Ummm, I don't think that is such a good idea!"

After all the pushing and shoving to get through the crazy hallways we finally made it to second hour. Second hour was English I with Mrs. Duffy as my teacher. Her class didn't have a table for us either! Emily and I looked at each other and sighed. We both were getting even more irritated because we had talked to the school two times previously to make sure they would have everything done by the first day.

"This day just keeps getting better and better!" Emily said sarcastically!

The bell rang telling us it was time to move to the next class.

I looked at Emily and said, "Let's try this again!"

The hallways seemed a little less packed during this passing period, maybe it was because I only had to go around the corner to get to my next class.

We finally made it to History, we were exhausted! I looked around the room.

Hoping this class would have a table, but

again no such luck. Our irritation level was rising.

Emily said, “Ugh!! It makes me upset that none of your classes are prepared for you!!”

I was so happy the next hour was lunch. We still had to bulldoze our way through the hallways to get to the lunch room but it was sooo worth it because I was starving from all the work it was taking just to get from class to class.

My Mom had not packed me a lunch for the day; I planned on trying school lunch. I had heard it was good but wasn't sure what to expect.

As Emily was getting my tray she said, “Wow! “This looks so gross!! I don't think I would feed this to my dog!”

We went and found the end of a table to sit at so I had room to eat. I tried the food and told Emily, “You are right, this is not good at all. It tastes like CRAP!!”

It was getting close to the end of lunch which meant another trip through the overcrowded hallways.

It was getting a little easier to maneuver my way through the hallways but still a huge challenge.

Fourth hour was Biology, My favorite class. We again noticed there was no table for us. We were getting use to the unpreparedness of the school. It was only the first day of school and hopefully we could get it taken care of soon. Biology class went fast; it was already time to move through the halls again. Math class was up next!! Math class was really close to my Biology class. Still people were so inconsiderate and bumping into my chair and stopping and standing where it was impossible for me to make my way through.

As we walked into my Math class, Emily and I looked at each other and smiled.

We were surprised to see there was actually a table for us! It was the only class so far that had been prepared and I was excited.

The final hour of the day was Spanish. I was really looking forward to this class, something new and exciting.

As we walked around the hallways, we couldn't find the class at all. We looked and looked. Finally we asked an upperclassman. She said “Oh it's down the stairs on the left.”

“Ok, Thanks!”

Emily and I looked at each other with disappointment.

You see there are a lot of stairs at Harrison High School that have wheelchair lifts

so that I could go down, but it just so happened that this stairwell happened to be without one and I really needed to get down to my class.

“Well, this just sucks. Doesn't it?”

“Yes, yes it does.”

There was no way to get down to my class. And I was afraid I would have to change my schedule and not have Spanish.

So we decided we needed to take a trip to the counseling office.

As we were making our way around the school to the counseling office, we were stopped by the security guard. He asked, “Why aren't you in class?”

I replied, “Because I can't go down the stairs.”

He looked at me puzzled!

Emily explained that my class was down stairs and that there was no way for us to get down there so we were headed to get it taken care of.

At the counseling office, we waited our turn to get to see the counselor. There must have been a lot of problem with everyone's schedules because it was packed in there.

I finally got into her office and she said “yes how can I help you Kate?”

“Well my schedule says that I have Spanish but the classroom is down stairs.” I said.

“Wow that's a bit of a problem!” She said with a shocked face.

“Yea, but I still want to take Spanish and I'm not sure how it's possible.” I said.

She told me, “since you can't get down the stairs to get to that class you can just sit in here for the rest of this period and I will have that class moved up stairs by tomorrow so you can still take Spanish.”

“Ok thank you SO much for all of your help!” I said with a smile.

Well as you can see the first day of my high school career didn't start out as I planned it to but Emily and I made it out alive! (Thank goodness!)

Being in a wheelchair has many advantages and disadvantages, but I just keep going through life telling myself it is what it is and I have to keep pushing on never giving up no matter what happens. All of these lessons about perseverance have made me a better and stronger person and I hope they will help you throughout your life as much as they did mine.



The Wilson Family



The Trainor Family



The Ruth Family



The Gee Family



The Pruitt Family



The Smelser Family



The Woodruff Family



The Armbrustmacher Family



The Sykora Family



The Murray Family



The Fantel Family



The Eide Family



The Burton Family



The Rappoport Family



The Bolton Family



The Kearns Family



The Janetzke Family



The Long Family



The Tomko Family



The Saxton Family



The Nunemaker Family



The Scurria Family

Families of Spinal Muscular Atrophy Advocacy Update 2013

Families of SMA's Legislative Strategy is Leading the Way Towards the Development of New SMA Therapies.

Five years ago, Families of SMA established a strategic goal to develop, implement and execute a sophisticated federal legislative agenda supported by an effective grassroots campaign. The goal of the effort is to significantly raise the awareness of SMA among policymakers, increase the level of federal support dedicated towards SMA research, and facilitate the development of therapies through public-private partnerships between Families of SMA, the federal government, and the bio-pharmaceutical industries.

Over the past half-decade, Families of SMA has led the way in the conceptualization and introduction of the SMA Treatment Acceleration Act, securing language in Congress's annual appropriations bills urging greater federal support for SMA biomedical research at the National Institutes of Health (NIH), the federal government's medical research agency, and engaging policymakers on a wide array of matters relative to the therapy development and approval processes. Due to these efforts the federal government's investment in SMA research and the SMA drug pipeline have never been more robust than at present.

Public-Private Partnerships

The increased NIH support for SMA research has accelerated the SMA drug pipeline. Families of SMA has worked closely with lawmakers and the NIH over many years to develop what has become a highly effective and sophisticated collaborative research enterprise that supports several basic, translational and clinical therapy development research programs for SMA. Under this three-stage construct, Families of SMA and our partners in government and industry work cooperatively to leverage resources and spur discoveries.

- In stage one, Families of SMA provides the initial seed funding for promising drug discovery programs. This work is funded by the efforts of our families and supporters and is critical to jumpstarting the therapy development process.
- In stage two, the NIH subsequently awards grants to meritorious projects to support further development of the therapies and prepare them for introduction into the clinic. Research at this stage becomes increasingly expensive and requires the government's intervention.
- The most promising therapies advance to stage three, in which biotechnology and pharmaceutical partners support and conduct the highly complex and expensive Phase II and III clinical trials that are necessary to secure drug approval from the U.S. Food and Drug Administration (FDA). Only

industry possesses the expertise and resources to support research at this stage of the process.

There are multiple examples of the success of this paradigm:

Quinazoline Program: Families of SMA began funding a project to turn up the SMN promoter to produce more SMN protein in 2000 using the compound Quinazoline. The project was licensed to Repligen Corporation in 2009 and received FDA Orphan Drug status that same year. The FDA approved a Phase I clinical trial in May 2011. This program was recently licensed to Pfizer for development.

Tetracycline Program: Families of SMA began funding a project to correct the defective splicing in SMN2 using the compound Tetracycline in 2006 in collaboration with Paratek Pharmaceuticals. The NIH awarded Paratek a multi-million dollar grant through its Cooperative Program in Translational Research (CPTR) in 2009 to further the research. The goal of this program is to submit an investigational new drug (IND) application to the FDA.

Antisense Oligonucleotides Program: This therapeutic approach uses small pieces of genetic material (oligonucleotides) to improve the functioning of the SMN2 gene. Families of SMA funded the early proof-of-concept research for this therapeutic strategy. Isis Pharmaceuticals received FDA approval permitting it to initiate a Phase I clinical trial in 2011 and recently began to collaborate on the clinical development of the project with Biogen Idec.

Gene Therapy Program: Gene therapy aims to replace the defective SMN1 gene with a healthy version. Families of SMA awarded several grants to support ongoing research at The Ohio State University. The result of an application submitted to the NINDS requesting additional support for this effort is currently pending.

Since 2008, funding for SMA initiatives at the NIH has nearly doubled from \$10 million to \$19 million, annually, and several important NIH-sponsored SMA research initiatives are underway:

The NeuroNEXT Network: In 2011, the NIH created "NeuroNEXT: Network for Excellence in Neuroscience Clinical Trials," a network of 25 sites across the country providing clinical trials infrastructure for neurological disorders. NeuroNEXT is very similar to the clinical network envisioned in the SMA Treatment Acceleration Act. The first research project to utilize NeuroNEXT is a SMA biomarker study at the Ohio State University, which aims to identify an efficient and precise means of measuring the impact of drug treatments.

Imminent Spending Cuts Threaten to Curtail Expanding Federal Investment in SMA Research

Newborn Screening: In 2010, the NIH awarded funding to support a multiyear clinical investigation of newborn screening in SMA. The grant is funding a study to test the efficacy of screening every newborn in Utah for SMA and enrolling identified infants into a medical home to coordinate and provide proactive care interventions and protocols. This effort will assess the impact of providing pre-symptomatic care on patient outcomes.

The SMA Project: In 2003, NINDS selected SMA from among the 600 neurological disorders under its purview to initiate a government-directed drug development program. The SMA Project tested the ability of Indoprofen analogs to increase SMN protein. Pre-clinical work has been completed and licensing opportunities are being pursued at the present time.

Pending Applications: Several highly competitive SMA-related research projects are awaiting the results of their applications to the NIH, including various basic research studies and initiatives related to drug development and gene therapy.

Looking Ahead

Families of SMA is proud of the success the legislative plan put into effect in 2007, but more work remains to be done. We will continue to engage lawmakers and policymakers throughout the federal government to spur continued development and approval of one or more therapies. We look forward to continuing to work together in these efforts – words cannot express our gratitude for your ongoing support and efforts.

Several New NIH-Sponsored SMA Initiatives are in Jeopardy if Congress Fails to Prevent “Sequestration”.

The federal government’s investment in SMA research has never been more robust than at present. Since 2008, funding for SMA initiatives at the National Institutes of Health (NIH - the federal government’s medical research agency) has nearly doubled from \$10 million to \$19 million annually.

This significant increase in federal resources devoted to SMA research is the result, in part, of the success of Families of SMA’s legislative strategy that aims to raise awareness of SMA and SMA-related research among federal policymakers through advocacy and grassroots. Thanks to families contacting their Members of Congress, resources and tools are being put in place at the NIH to support drug discovery efforts that will lead to a treatment for SMA. Unfortunately, these efforts may be threatened if Congress permits deep spending cuts to take place in January.

The NIH Support for SMA

Families of SMA has worked closely with lawmakers and the NIH over many years to develop what has become a highly effective and sophisticated collaborative research enterprise that supports several basic, translational, and clinical therapy development research programs for SMA. Under this construct, Families of SMA and our partners in government and industry work cooperatively to leverage resources and spur discoveries.

Some examples of NIH-sponsored SMA research initiatives include:

- **The NeuroNEXT Network:** In 2011, the NIH created “NeuroNEXT: Network for Excellence in Neuroscience Clinical Trials,” a network of 25 sites across the country providing clinical trials infrastructure for neurological disorders. NeuroNEXT is very similar to the clinical network described in the SMA Treatment Acceleration Act. The first research project to utilize NeuroNEXT is a SMA biomarker study at the Ohio State University, which aims to identify an efficient and precise means of measuring the impact of drug treatments.
- **Newborn Screening:** The NIH is funding a study that is testing the efficacy of screening every newborn in Utah and Colorado for SMA and enrolling identified infants into a medical home to coordinate and provide proactive care interventions and protocols. This study will assess the impact of providing pre-symptomatic care on patient outcomes.
- **Tetracycline:** In 2010, the NIH awarded Families of SMA partner Paratek Pharmaceuticals with a multi-year, multi-million dollar award through its highly competitive Cooperative Program in Translational Research to develop a tetracycline (TC) drug therapy. Families of SMA provided the seed funding for this project. The goal of this project is to submit an

continued on next page

Imminent Spending Cuts Threaten to Curtail Expanding Federal Investment in SMA Research

Investigational New Drug (IND) application to the FDA.

- **The SMA Project:** Beginning in 2003, the NIH self-funded a drug development project that tested two therapies, Indoprofen and Benzamidazoles.
- **Pending Applications:** Several highly competitive SMA-related research projects are awaiting the results of their applications to the NIH, including various basic research studies and initiatives related to drug development and gene therapy.

Budget Cuts

Under current federal law, deep spending cuts are scheduled to go into effect automatically on January 2, 2013 unless Congress intervenes. These cuts, known in budget parlance as “sequestration,” will reduce the budget of several federal programs by as much as ten percent and threaten SMA research.

The NIH is expected to be cut by 8.2% in the sequestration process which would take NIH funding back to 2004 levels. Such a spending reduction will be dramatically disruptive to the NIH’s medical research initiatives and significantly inhibit scientific discovery for years to come. A critical bridge between Families of SMA’s seed efforts and the clinical trials supported by the biotechnology and pharmaceutical industry will be lost as several of the SMA-related research programs conducted or supported by the NIH are in jeopardy of being cut back, delayed, or terminated entirely under sequestration, thus reversing a decade of progress in building greater support for SMA research within the NIH.

Contact Congress

Congress has the power to change the law and ensure that any deficit reduction efforts are carried out in a manner that does not threaten critical research for diseases such as SMA. Contact your Senators and Representative to tell them to reverse sequestration and ensure that the NIH is not cut.

Talking Points:

- I am calling to urge the [Congressman / Congresswoman / Senator] to amend the Budget Control Act and ensure that sequestration does not go into effect.
- Sequestration will cause the budget of the National Institutes of Health to be reduced by 8.2%, which would devastate research into lifesaving therapies for diseases such as Spinal Muscular Atrophy (SMA).
- My [son / daughter / etc.] is affected by SMA, which is the leading genetic killer of children under the age of two. The NIH is supporting several initiatives aimed at developing a treatment for SMA, many of which are very promising. Sequestration will setback this research.
- Please tell the [Congressman / Congresswoman / Senator] that I strongly urge [him / her] to protect the NIH from sequestration and to find a more equitable and fair way of reducing the deficit.

If you have any questions about NIH funding for SMA and the impact of sequestration, please contact Legislative Coordinator Spencer Perlman at spencer@fsma.org.



Jordan Block



Bri and Sammi Johnson



Aiden Marie Bala



Mabel Mckenzie



The Abralde Sisters



Rado Eneva



Jake, Rachel and Kate Saxton



Jake Saxton



Ross Rosenfeld



Oriaha Mackey



Lucy Butler



Logan Ruth



Jackson Long



Mallory Armbrustmacher



Malorie and Jacob Fox



Madi Ramirez and Ella Christopher with their special needs cheer team the Iowa Elite



Madi Ramirez turns 7!



Garrett Schlutz



The Moyer Kids



Logan Ruth



Alexis Burgoon



Kennedy Robinson



Joshua M. Gordon



Aiden Marie Bala



Veronica St. Onge



Gray and Riley Dougherty



Kyan Kulas



Jacob Snowden



Gabriel, Greyson and Vivian Erwin



Journee and Jace Weiberg



Jadon Burks



Eva Pruba



Fletcher Koll



The Gooden Family



The Pillarella Family



The O'Neill Family



The Norton Family



The Dinzans Family



The Williams Family



The Pruba Family



The Grimes Family



The Potter Family



The Schmid Family



The Negrin Family



The Stare Family

SMA Family Adventure Camp 2012 (12th annual)

– Cultus Lake, BC, Canada

Wow! What a camp we had this year! We had families attend from coast to coast, and what an awesome way to come together, at a camp filled with fun and laughter. Some of us were returning, some were newly diagnosed and some just decided to give it a try for the first time, but ALL of us left as a tight knit group. Camp isn't just about the one week, the friendships, mutual support and communications last all year long.

This year was full of SMA camp traditional and new activities. We had a pajama party family dance, played Capture the Flag, T-Ball, scavenger hunts, musical bubbles (like musical chairs but accessible), obstacle course, marshmallow war (where we all throw marshmallows at each other, every man for himself), crafts, zip lining, horse back riding, kayaking, biking, hiking, camp fires, swimming, set off rockets, walks for ice cream and a lot of laughing.

What SMA kids get from camp – Letting go completely, knowing that they are surrounded by people that not only “get it” but are living it and embracing it! They have incredible memories of the amazing things they accomplished, giving them the pride that they earned and deserved. They make

friends that they know they will have for a lifetime, someone to communicate with even if its just to say hi and remember when. The value of all of this is immeasurable!

What the siblings get – The ability to just hang with kids that get what it's like to have to take the back seat sometimes, kids that know what it's like to unconsciously do the “avoidance” dance as their sister or brother is moving the wheelchair around. They have the chance to also make family memories where the whole family could participate in all of the activities. This camp isn't just about the kids with SMA, the siblings are celebrated and included in all of the activities!

What the parents get – Simply...they get tears, tears of finally being surrounded by others who understand the unconditional love, tears of celebration of the smallest of accomplishments, tears of inclusion for their family, tears of laughter, and in the end, tears of good bye, because it is so hard to say it at the end of camp.

But next year, we'll wrap our arms around each other again and we'll enelope the new families and do it all again!



SMA Family Camp 2013

Next year's camp has a pirate theme and will be held again at Cultus Lake, BC, Canada which is just half an hour from the Washington/BC border.

Camp dates will be August 7 – 13, 2013

If interested in registering, or if you have any questions or concerns, please contact Susi Vander Wyk at fsmacan@telus.net



Come join the fun!

We can easily make it work for families that are not “camping families”.

Accommodations – If renting an RV, the RV rental is \$500 for the week, otherwise bring your own tent or RV. Power hook up available.

Amenities - Showers, wheelchair accessible washroom with lift, tracking and change table, all meals supplied.

Cost - \$60 per person for the week of camp.



SMA Family Adventure Camp 2012





BAYADA client Isaiah M., (second from left) was born with multiple congenital anomalies, and is pictured with his father Chris, mother Joy, and brother Joseph.

Choosing a home health care provider for your child

Nothing is more important than the health and well-being of your child. Home health care services provide needed assistance to children faced with complex medical conditions, challenging behavioral issues, or other special needs, as well as offering support to parents and families. Asking home health care providers these questions will help you learn more about them and the individuals who will be coming into your home.

1. How long has your home health care company been serving the community?
2. Does your company specialize in pediatric care?
3. Is your company certified by any accrediting body, such as CHAP (Community Health Accreditation Program) or The Joint Commission, and state licensed?
4. How do you ensure that your nurses have the skills to care for my child? Are your nurses as qualified as hospital nurses?
5. What sort of background checks are performed before a nurse is hired? Are your health care professionals fully insured?
6. As a parent, what sort of participation can I expect in planning my child's care and routine? Do you consult with my child's physicians?
7. How will the nurses know what my child needs? How will the nurse interact with my child when not performing medical procedures?
8. How many nurses will be caring for my child? Can I meet the nurses before they start to work? What happens if a nurse isn't a good fit for our family?
9. How will I know which nurse is scheduled for the day? How are schedule changes communicated to me? How does the office cover my child's care if the nurse is unable to work a scheduled shift?
10. Who coordinates all of the services your home health care agency will provide to my child and family?
11. Will your company assist me in obtaining any medical supplies or equipment that my child needs?
12. Will your company assist me in getting my home health care services approved, maintained, and paid for?
13. Do you protect your clients with written standard procedures and policies?
14. How will our personal information be protected? Will the nurses be respectful of our home, belongings, and beliefs?
15. If I have a question or concern, is someone available 24 hours a day, every day?

Contact us

To arrange care or learn about pediatric home health care services, call **800-305-3000** or visit **www.bayada.com**.



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On Being an Ostrich

By: Debby St. Onge

Mom to Veronica Rose, 8 years old, SMA Type I

The injustice of SMA once again rears its ugly head. In the past couple weeks at least five more families have had to say goodbye to their child, all less than seven years old. Two of those were Veronica's good friends. Not just people we knew on line, but kids she has actually met and spent time with. How do you tell your child news like this? How do people live like this, watching and knowing that any day it could be you?

Sometimes I wish I was an ostrich. I could bury my head in the sand and not have to feel my heart being torn out and broken over and over again. I would not have to live with the fear that someday it will be me who is forced to say goodbye to my child. I would not have to find the words to explain to my child why this has happened to yet another friend of hers. I would not have to appear strong and resilient every day, while my emotions are crumbling into a hot mess all around me.

So maybe I am part ostrich. How else can a person protect their psyche when dealing with a child killer like SMA? It can either shatter you to the point of not being able to function, or make you a stronger, better person. For the sake of my child, who I love more than anything on this earth, I am glad I fit in the latter. Besides, an ostrich is not a pretty animal and I would get tired of having sand up my nose.

"You never know how strong you are, until being strong is the only choice you have." Unknown.

This is a quote that describes many of us perfectly. Strength is the one key factor in the battle with SMA. My SMA friends and family are the strongest people I have ever known in my life. When you look up "strength" in the dictionary there should be a picture of a parent or person with SMA in the definition. But how can you be strong all the time when someone's child dies pretty much every day of the year? You can't. That would make you non-human, incapable of feeling human emotions. That would make you an ostrich.

So we lift our heads out of the sand and we trudge on. We deal with our grief in whatever way is most comfortable. Some turn to prayer, some meditate, some medicate, some exercise, some cry and lash out, some bury their head back in the sand. But we deal. We look to our children for guidance, we revel in what they can do, we take immense joy in the simplest things, and we move forward. What else can you do?

You learn how easy it is to push certain thoughts and emotions to the back of your mind. The problem is, they are always still there, waiting to resurface every time you hear of another child



passing. The same fears are then brought forward, crippling you, and reminding you of this reality that we live day to day. But at the same time, those fears should never be stifled. They are our

reminders of the fragility of life; of the importance of not letting your guard down or thinking the battle has been won, because as long as SMA exists, it will remain a constant foe for all of us.

No one deserves nor invites SMA into their lives. When you are handed that tiny, crying, beautiful little bundle for the first time you make a lifelong commitment to love and protect. It's a game changer for everyone. So when you are told your child has SMA you either recoil or hit it head on, never realizing the level of fight and strength you have within you. It's your child. You would move heaven and earth for them, so to have this disease that robs them of so much can rock you to the core.

And a cure? While I must remain hopeful for someday that being a reality, I stopped putting all my eggs in that basket years ago. The best thing I believe I can do is remain strong and fight SMA for my child. I can do that by providing her with the best possible care, and giving her a happy and fulfilling life, full of opportunity.

The problem is that it is far too physically and emotionally draining to be strong all the time. So, while I will forever be strong and resilient on the outside, there are times when I am going to choose to be an ostrich, if only for a while. I would hate to think of all the joy I would have missed out on otherwise. But some days, I do believe it is okay to be an ostrich.

PS – I do realize that an ostrich really does not bury its head in the sand. Call it poetic license.

August is SMA Awareness Month

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

Join Families of SMA in getting involved with the SMA community this August.

Show Your Support! The Families of SMA Awareness Ribbon is one of the recognized symbols in the SMA community. SMA prevalence is now one in every 6,000 children. Show your support for people with SMA by wearing the FSMA Awareness Ribbon. You can wear it as a pin on your shirt, a magnet on your car, a badge on your blog, or you can even make it your Facebook profile picture. By wearing the ribbon you can help educate the population on the potential of people with SMA! To purchase the FSMA Awareness Ribbon for your shirt, car, or refrigerator, visit

<http://www.fsma.org/Fundraising/Merchandise/>

SMA Candle Lighting. The Annual SMA Candle Lighting will be Saturday, August 10th. Join with families and SMA organizations around the country by lighting a candle at sunset to remember those SMA Angels who have lost their battle with SMA and to honor those SMA Warriors who are still here fighting everyday! Please feel free to post a picture of your family participating in this event on our facebook page at the following link:

<https://www.facebook.com/familiesofsma>



Make a difference.

There are numerous activities that you can carry out in your local community to promote general awareness for Spinal Muscular Atrophy. Get involved today and engage your local area by writing an article for your newspaper or online publication, host a candlelight vigil or balloon release in honor of our SMA kids, contact your elected officials and request a proclamation stating August is SMA Awareness Month for your town or city. To help you with your planning, Families of SMA can provide you with several materials and templates. For more information on ways to make a difference, please visit

<http://www.fsma.org/Fundraising/AwarenessMonth/>

Connect with your FSMA Chapter. Many Families of SMA local chapters hold special events in their communities throughout the month of August so be sure to connect with them to find out about any planned events.