



DIRECTIONS

FAMILIES OF SPINAL MUSCULAR ATROPHY



Families of SMA

Research. Support. Hope.

In this Issue...

**New Program Announced
For Medical Providers4**

SMA Awareness Month11

Family Support12

**2012 Annual SMA
Conference32**

**Fundraising and
Chapter Updates60**



What Families of SMA Means to Me

By: Dawn Cusack

Our lives changed on April 15, 1999. Our little angel, Kellie, was born via emergency C-section. She was so small, only 4lbs. Even then she was strong and fighting to live to be a part of this world. Living and breathing is something most of us take for granted. When you have SMA you fight for every day, every breath, and take no moment for granted. It is a blessing and a miracle.



I learned this lesson early on when Kellie was first diagnosed at fourteen months. We got the news that she might not live past the age of two. It's funny because my husband and I are both very stubborn if you tell us we can't do something, we work extra hard to prove you wrong and it always works in our favor. Kellie must have inherited this trait because she is twelve! By the way, her name means warrior in Gaelic or Irish. She has fought many respiratory battles and won. There were many times we were close to death, but Kellie would not give up! Life can be kind of scary but you know what, when your child has been so close to dying many times, not many things scare you anymore!

We are so grateful for our friends at Families of SMA. Our lives changed after our first conference in Boston, thanks to a scholarship that helped to pay for the conference. We realized that there is life after being diagnosed. It is not a death sentence, there is hope! I was so amazed by the many parents who started chapters and were dedicated to helping other families. After the awards banquet and seeing what others have done for fundraising I became determined to help too. My daughter and I have held many bake sales and just recently in November, we held a bowl-a-thon at our local bowling alley. We called it Strike Out SMA. Thank you Families of SMA for helping so many families and helping us with our fight for a cure!



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

©2012 Families of SMA. All rights reserved. No portions of this publication can be reprinted without written consent from FSMA.

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: Brooklynn Santos

On the Cover of Winter '11: Andrew Murray and Ford Sniezek

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.

Families of Spinal Muscular Atrophy is Thrilled to Receive Additional Funding for the Type I SMA Care Packages

The Jacob Isaac Rappoport Foundation and Adi & Shaina Rappoport have awarded Families of SMA with another year of funding for the Type I Care Packages, which are sent to all newly diagnosed Type I families as soon as they contact Families of SMA.

Families of SMA currently sends over 200 Type I Care Packages each year to newly diagnosed families. Many ideas for the items in the Type I 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.

Some of the items in the Newly Diagnosed Type I Care Packages include:

- Fisher Price Projector— This projector projects images onto the ceiling for the infant to look up at.
- Fisher Price Swing – This swing can recline back so that a SMA infant can lie comfortably and safely while swinging.
- Sheepskin Blanket - The sheep skin blankets have been found to help with preventing tissue breakdown and to reduce the need for turning children that cannot turn themselves.
- Hand Print Mold
- Linkadoos Link Pack - These are great to link toys or other objects low enough to tug on, such as, various toys to swings, car seats, etc. Also, they can be used as teethingers, since regular teethingers may be too heavy.
- Comfort Silkie Blanket
- Motorized Bubble Blower
- Beanie Baby – Could be used for positioning the infant.

These items are light enough for a SMA infant to hold:

- Lightweight Balloon on a Stick
- A Feather
- Finger Puppets
- O-Ball
- Cure SMA Lightweight Flag
- Lightweight Baby Plastic Spoons
- Lightweight Cat Toys

Many of these items include a label with a few sentences explaining each item.

These care packages are funded by the Jacob Isaac Rappoport Foundation, in memory of their Shooting Star, Jacob, who had SMA Type I. Jacob was born December 27th, 2001 and was an alert and happy baby. When he was nearly four months old,



Jacob was diagnosed with Spinal Muscular Atrophy. Soon after Jacob's diagnosis, Adi and Shaina quickly connected with Families of SMA and many other SMA families.

Shaina tells Jacob's story on the foundations website, "As a four month old, by appearances, it was impossible to tell that Jacob had a terminal disease. He simply was a beautiful boy, alert and quick to smile. As the weeks went on, it became clear that we would need to make some decisions about how we were going to manage Jacob's care".

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 nine years, they have raised nearly \$875,000; sent dinners, cleaning services and packages 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.

CONTINUING MEDICAL EDUCATION CONFERENCE

Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role

Families of SMA is offering a Continuing Medical Education Conference titled “*Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role*”, for medical professionals on Wednesday, June 20th, 2012 prior to the start of the 2012 Annual SMA Conference and Researcher Conference in Bloomington, Minnesota.

This CME Conference for medical professionals is the first of its kind for Families of SMA. The conferences have previously covered two components of SMA, Research and Support. The Research aspect covered scientific, research & clinical updates, while the Support aspect included the family support workshops and family networking. The announcement of this conference adds an exciting new 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 Gillette Children’s Specialty Healthcare, who are accredited to provide continuing medical education credit for medical professionals.



Families of SMA is launching this new 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, members of Gillette Children’s Specialty Healthcare, as well as Guest Speakers from Institutions across the US, will provide didactic presentations and host panel discussions.

The target audience is intended for 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 for the CME Conference on June 20, 2012 at the DoubleTree by Hilton Hotel Bloomington – Minneapolis South, is available on-line only at www.curesma.org.

Registration

Online registration only at <http://www.fsma.org/CME>
Space is limited; please sign up early!

Registration Pricing:

Physicians - **\$125**

Physical Therapists, Nurses, Nurse Practitioners, Physician Assistants, Occupational Therapists, Nutritionists, Respiratory Therapists, Speech Therapists, Allied Health, Residents & Fellows - **\$100**



CONTINUING MEDICAL EDUCATION CONFERENCE

Planning Committee
and Presenters**Families of SMA Medical
Advisory Council:**

Mary K. Schroth, MD
Pediatric Pulmonology
*Families of SMA, Medical Advisory
Council Chair, American Family
Children's Hospital, University of
Wisconsin School of Medicine and
Public Health*

Garey Noritz, MD
Pediatrics, Internal Medicine
*Nationwide Children's Hospital, The
Ohio State University*

Rob Graham, MD
Critical Care Medicine
*Children's Hospital Boston,
Harvard Medical School*

Susan Apkon, MD
Pediatric Rehabilitation Medicine
*Seattle Children's, University of
Washington School of Medicine*

Brian Snyder, MD, Ph.D.
Pediatric Orthopedic Surgery
*Children's Hospital Boston, Harvard
Medical School*

Richard Shell, MD
Pediatric Pulmonology
*Nationwide Children's Hospital,
The Ohio State University*

Barbara Godshall, MMSc, RD,
CSP, LD, CNSD
Pediatric Clinical Nutrition
*Cincinnati Children's Hospital
Medical Center*

Vanessa Battista, RN, MS, CPNP, CCRC
Pediatric Nursing
Children's Hospital Boston

John Grayhack, MD
Pediatric Orthopedic Surgery
*Northwestern University Feinberg
School of Medicine*

Kristin J. Krosschell, PT, MA, PCS
Physical Therapy and Human
Movement Sciences
*Northwestern University Feinberg
School of Medicine*

Richard Kravitz, MD
Pediatric Pulmonology
*Duke University Medical Center,
Duke University School of Medicine*

Karen Patterson, MS, PT, PCS
Physical Therapy
*University of Wisconsin School of
Medicine and Public Health*

Tom Crawford, MD
Pediatric Neurology
Johns Hopkins Hospital

Richard Finkel, MD
Pediatric Neurology
The Children's Hospital of Philadelphia

John Kissel, MD
Neurology
*The Ohio State University,
Wexner Medical Center*

Kenneth Silver, MD
Pediatric Neurology
*University of Chicago Comer
Children's Hospital*

Albert Freedman, Ph.D.
Child, Adolescent, & Family
Psychologist
Child Family Psychologist

**Other Planning
Committee Members:**

Jessica Clark
Families of SMA

Mary Grimm
Gillette Children's Specialty Healthcare

Kenneth Hobby
Families of SMA

Colleen McCarthy O'Toole
Families of SMA

Shannon O'Brien
Families of SMA

Karen O'Brien
Families of SMA

Julie Pearson
Families of SMA

Amy Schall
Gillette Children's Specialty Healthcare

Paul Fiore
Gillette Children's Specialty Healthcare

Candace Vegter
Gillette Children's Specialty Healthcare

**Gillette Children's
Specialty Healthcare:**

Stephen A. Smith, MD
Medical Director Neuromuscular
Program Pediatric Neurologist

Peter Karachunski, MD
Pediatric Neurologist

Steven Koop, MD
Pediatric Orthopedic Surgeon

Mark E. Gormley, Jr., MD
Pediatric Physical Medicine and
Rehabilitation Physician

Paul Kubic, MD
Pediatric Pulmonologist

Jason Kelecic, DPT
Program Manager

Paula Vander Schaaf, OTR/L, ATP
Occupational Therapist

Guest Presenters:

Rebecca Hurst, MS, RD, CD
Clinical Dietitian
Pediatric Motor Disorders
*Research Program
University of Utah*

Mary Marcus, MS, RD, CSP, CD
Co-Director and Nutrition Faculty
*University of Wisconsin Pediatric
Pulmonary Center*

Charlotte Sumner, Ph.D.
Johns Hopkins School of Medicine

Erin Seffrood, MS, RD, CSP, CD,
Pediatric Clinical Dietitian
American Family Children's Hospital

**Families of SMA Receives \$25,000
Sponsorship from BAYADA Home Health
Care for The 2012 Annual SMA Conference**

Families of Spinal
Muscular Atrophy is
excited to announce a
significant sponsorship
for The 2012 Annual
SMA Conference.



A subsidiary of BAYADA Home Health Care

This year, FSMA will
host The 2012 Annual SMA Conference in Bloomington, MN
where over 900 SMA families and researchers will meet to
participate in numerous workshops, networking opportunities and
gain valuable information about SMA and the progress of research
projects. FSMA is grateful for the tremendous support of BAYADA
Home Health Care for becoming the Presenting Sponsor of the
largest gathering of SMA families and researchers from around the
world.

BAYADA Home Health Care has been supporting Families of SMA
for over eight years at the local level through sponsorship of the
Annual PA Chapter Walk-n-Roll in Philadelphia, PA as well as the
Annual Gray's Gang Walk in Charlotte, NC. Thanks to Dr. Al
Freedman, father to Jack, and colleague of Mark Baiada, President
and Founder of BAYADA Home Health Care, for facilitating this
partnership.

*I became involved with FSMA through my colleague and friend,
psychologist and author Dr. Al Freedman, whose son Jack was diag-
nosed with the disease at the age of six months. Jack is now 16, and
over the years I have come to understand the challenges his family
faces on a daily basis. Getting involved with the local walk for a cure
was one way to give back to help Jack. Now, by sponsoring this con-
ference, BAYADA has an exciting opportunity to make a difference
in the lives of more and more families across the country.*

*Participating with FSMA on a national level will provide an oppor-
tunity for our staff to expand their clinical expertise in SMA, offer
support groups and other community resources to parents and
family members, participate in local fund raising and educational
events, and provide exceptional care in the home with compassion,
excellence, and reliability for children with SMA.*

~ Mark Baiada, President and Founder,
BAYADA Home Health Care

Founded in 1975 by J. Mark Baiada, BAYADA Home Health
Care provides nursing, rehabilitative, therapeutic, hospice,
and assistive care services to children, adults, and seniors in
the comfort of their homes. Headquartered in suburban
Philadelphia, BAYADA employs more than 17,000 nurses,
home health aides, therapists, medical social workers, and
other home health care professionals who serve their
communities from more than 240 offices in 20 states and
India. For more information, visit www.bayada.com.

Families of Spinal Muscular Atrophy Announces Special Session Topic for the 16th Annual SMA Research Group Meeting

An important way Families of SMA advances Spinal Muscular Atrophy research is the Annual SMA Research Group Meeting. The International SMA Research Group Meeting is the biggest SMA research conference in the world. For 16 years, FSMA has organized the conference, and financially underwrites the meeting by covering hotel, travel and registration for all research presenters for a total cost of about \$200,000 each year.

The 2011 meeting included over 110 presentations, including 36 podium presentations and 77 poster presentations. A total of 225 researchers attended the conference, representing 70 total institutions, 14 biotech and pharmaceutical companies, and 11 countries worldwide. SMA researchers say, "This is THE big meeting that brings everyone together and I cannot stress enough the value of this meeting. The meeting is the starting point for many collaborations and a place for researchers of all stripes to learn and place their work into the context of the broader field. Seeing the children with SMA alone is a huge value from this meeting, to always ground us in our goals for doing the work we do."

Once again in 2012, the conference will start off with a Special Session entitled, "Lessons Learned in Pediatric and Neurological Drug Development".

The SMA research community is primed to begin human clinical trials on the first novel drugs specifically designed for SMA, with both Repligen Corporation and Isis Pharmaceuticals starting Phase I trials in 2011 and others to follow soon. Many companies developing SMA drugs attended both the research and family side of the 2011 SMA Conference. By focusing on successful drug programs for other diseases, the session goal at the 2012 meeting will be to highlight areas of importance for SMA drug development, including special considerations in pediatric drug development, the use of biomarkers in early drug development, clinical trial design issues, the use of complex outcome measures in drug trials and development of combination therapies. During the panel discussion, the major findings from each talk will be related back to SMA drug development.

Opening Address - John Kissel, MD, Professor of Neurology, The Ohio State University. Overview of SMA Therapy Development: Challenges and Opportunities. Dr. Kissel will introduce the session by giving an overview of the state of SMA drug development.

Thirty-Minute Talks on Drug Development in Other Diseases by Leading Experts in Neurological and Pediatric Diseases.

1. Small Molecule Drug Development for Cystic Fibrosis: Strategy for Developing Combination Drugs for an Orphan, Pediatric Indication, Eric Olson, PhD, Vice President and CF Program Leader, Vertex Pharmaceuticals
2. Stem Cell Therapy for CNS Indications, Eva Feldman, MD, PhD, Professor of Neurology, University of Michigan
3. ASO Drug Development for ALS, Timothy Miller, MD, PhD, Assistant Professor of Neurology, Washington University
4. Gene Therapy and ASO Drug Development for Duchenne Muscular Dystrophy, Jerry Mendell, MD, Professor of Pediatrics and Neurology, Nationwide Children's Hospital
5. Outcomes and Biomarkers in Neuromuscular Disease Drug Development, with Focus on Electrophysiology, Jeremy Shefner, MD, PhD, Professor and Chair of Neurology, Upstate Medical University
6. Small Molecule Development for ALS, Douglas Kerr, MD, PhD, Director of Experimental Biology, Biogen Idec

Session Synopsis and Panel Discussion, Moderator: Douglas Kerr, MD, PhD, Director, Experimental Neurology, Biogen Idec. The moderator will present a short wrap-up relating the issues presented above to SMA drug development and introducing specific topics for the panel discussion.

SHARING PHOTOS



Aaron Phelps



Tambryn and Braelyn Campbell



Journee Weiberg



Janos Williams Tercs

NIH Provides Congress an Overview of the Federal Government's Spinal Muscular Atrophy Research Activities

As part of President Obama's budget request for fiscal year (FY) 2013, the National Institutes of Health (NIH), the nation's biomedical research agency, has provided Congress with a summary overview of its SMA-related research activities. This information is included in the NIH's budget justification materials and responds to Report Language included last year in the FY 2012 Labor, Health & Human Services, and Education Appropriations bill.

Specifically, the National Institute of Neurological Disorders and Stroke (NINDS) outlines its intention to fund a SMA biomarkers study as part of its NeuroNEXT initiative. NeuroNEXT is a new government-supported enterprise that provides clinical trials infrastructure for research related to neurological disorders such as SMA. The initiative closely mirrors, and was partially prompted by, a proposal included in the SMA Treatment Acceleration Act. The SMA biomarkers study is the first NeuroNEXT initiative – development of a SMA biomarker will facilitate clinical trials for SMA therapies. NINDS also summarizes its intention to enhance its funding of SMA-related translational research initiatives; such research is a top priority for Families of SMA since it helps to grow the drug pipeline.

Additionally, the National Institute of Child Health and Human Development (NICHD) provides an update on its newborn screening activities. Implementing newborn screening for SMA is critically important to ensure that new therapies can be delivered presymptomatically. Families of SMA has worked tirelessly since 2005 to encourage the NIH to support SMA-related newborn screening research and for the federal Advisory Committee responsible for establishing the newborn screening panel to include SMA. NICHD is funding a study that is analyzing a new and improved SMA newborn blood test as well as working to develop necessary protocols for addressing the needs of infants testing positive for SMA. Both components will be piloted in two states. The data gathered from this pilot study will be critical in the effort to add SMA to the federal newborn screening panel – Families of SMA submitted an initial application to the Advisory Committee in 2008.

NIH Also Releases 2011 Funding Amounts for Spinal Muscular Atrophy Research.

The National Institutes of Health (NIH), the federal government's medical research agency, released its most recent estimate of funding for disease categories which indicates that its base funding levels for SMA-related research has increased from \$10 million in fiscal year 2008 to \$19 million in fiscal year 2011. 2012 and 13 funding levels are projected to be similar.

Part of the increase in federal resources devoted to SMA-related research is the result of the success of a two, long-term strategies at Families of SMA to provide seed funding for critical drug discovery programs in order to leverage federal (and private) resources, and to raise awareness of SMA and SMA-related research among federal policymakers through advocacy and grassroots efforts.

Several recipients of NIH funding in fiscal year 2011 received their initial seed funding from Families of SMA.

For instance, NIH supported two projects related to the Quinazoline Program to treat SMA, now being developed by Repligen Corporation and two projects related to the Tetracycline Program to correct SMN2 splicing, including \$1 million in funding in 2011 to Paratek Pharmaceuticals from a 5-year multi-million dollar award.

The initial seed funding provided by Families of SMA played a critical role in positioning these four projects to receive highly-competitive NIH research dollars for the first time. Leveraging our initial seed money into greater industry or government investment is a key goal at FSMA.

Additionally, the NIH funded two grants in fiscal year 2011 to support the Families of SMA Annual Research Conference .

Pumped Up

By: Christen Brownlee, *Hopkins Medicine Magazine*, Fall 2011; October 3, 2011

This article has been reproduced with permission of Johns Hopkins Medicine Magazine, copyright, 2011

Nothing can keep Payton Mueller down, not even the Spinal Muscular Atrophy (SMA) that continues to take its toll on his young body. Doctors here share his optimism. Thanks to rapid advances in research and clinical care, the future for patients with SMA has never looked brighter.

The mood was light at the start of 10-year-old Payton Mueller's recent exam. While his mother and grandmother chatted with Hopkins pediatric neurologist Tom Crawford about Payton's recent activities and the family's trip from their home in North Dakota, the brown-haired boy fidgeted as only a pre-teen in a motorized wheelchair can—knocking the chair's joystick back and forth, repeatedly swinging it in little side-to-side arcs. He alternately grinned and dramatically grimaced as Crawford led him through a series of tests that measured strength all over his body. "Pull, pull, pull! Great job!" the doctor encouraged.

But near the end of the hour-long visit, the atmosphere took a somber turn. Payton, whose hips and knees are locked into right angles from muscle shrinkages called contractures, lay face down on the exam table. He groaned in pain as Crawford showed his mother how to press on his back to stretch these joints.

After Payton was back in his wheelchair, blinking away tears, Crawford explained why these agonizing stretches, along with all the other painful efforts to take care of his body, were necessary: "I'm planning on you doing a lot—college, a job, having a family, all that normal stuff," said Crawford. "You don't get out of anything."

That future wasn't always the case for kids with Spinal Muscular Atrophy (SMA), the condition that Payton has. Not long ago, parents were often told to take their SMA kids home, and love and cuddle them, since it wasn't likely that these children would live long. But now, with the help of better clinical care and new research, the potential has never been brighter for people with this disease. SMA patients are living longer and longer, and treatments that could radically improve their lives and outlooks hover tantalizingly on the horizon.

"SMA has gone from being one of the most

hopeless of the neuromuscular diseases to one of the most hopeful," Crawford says.

Earlier that day, 60-year-old SMA patient Joan Palmer arrived at the Johns Hopkins Outpatient Center for her appointment with adult neurologist Charlotte Sumner. Unlike Payton Mueller, who has used a motorized wheelchair for most of his life, Palmer just started using her wheelchair five years ago—a fact evident as the bespectacled patient with neatly bobbed hair cautiously backed her chair into a tiny exam room.

"Kids these days, they just naturally know how to use a toggle stick," Palmer says. "There's a bit of a learning curve for me."

Palmer is the rare SMA patient, in that she has lived well into adulthood. Unlike the vast majority of people with this disease, she has had enough strength to be, at various points, a high school cheerleader, a mom of three, a Girl Scout leader, and the chairwoman for her church's antique sale. "I've done what everyone else has done," she says.

That's because Palmer has what's known as SMA Type III. Though they're still plagued by muscle weakness, type III patients generally fare much better than type II patients like Payton, who can sit but not stand, or type I patients, who never sit or stand. About 1 in 6,000 people has SMA, with the devastating type I making up more than half of cases.

Although survival past their second birthdays remains difficult for type I patients, several advances in the past few decades now keep type II patients alive for decades longer than they've survived in the past—exactly how long is yet to be known as current patients grow older. For example, like most type II patients, Payton Mueller has undergone spinal fusion surgery to straighten his back—in the past, scoliosis was a major cause of death, with weakened back muscles causing the spine to curve dramatically, eventually crushing the lungs. Payton and most other kids also use a "cough machine," which helps clean out lungs too weak to cough up mucus that can make patients sick.

Though care advances such as these have slowly evolved over the last few decades, scientists only zeroed in on the reason for these disparities between SMA types relatively recently, explains Sumner. In 1995, French



Payton Mueller

researchers discovered a pair of nearly identical genes, named survival motor neuron (SMN), that cause the disease through a unique mechanism in which each plays a role. About 1 in 40 people carries a single flawed copy of the first of these, termed SMN1, usually unknowingly. SMA is caused when a child inherits two copies of the flawed SMN1 gene, so a couple in which each member is a carrier has a 1 in 4 chance of having a child with the disease.

Though a second, similar gene, called SMN2, can make up for some of SMN1's function, it does so much less efficiently, making significantly less of the necessary protein. However, says Sumner, people carry a varying number of SMN2 copies in their genome, ranging from one to four. These copy numbers loosely correlate to SMA type. Those with the lowest number of copies—and, therefore, with the least SMN protein—have the most severe forms of SMA, and those with the highest copy numbers generally have the least severe forms. Though extraordinarily rare, sometimes people with two copies of the faulty SMN1 gene, but a high SMN2 copy number, don't realize they have the disease at all.

"I once had a professional wrestler from Canada, who was a sibling of one of my adult SMA patients, come into one of my studies as a control," Sumner says, referring to people who don't have the condition being studied and are used for comparisons. After analyzing the wrestler's genes, however, Sumner found that he had SMA, too.

SMA first caught Sumner's eye during a neuromuscular fellowship at Johns Hopkins in 2000, soon after excitement over SMN1's discovery started spreading throughout the field. Later, at a research fellowship at the

National Institutes of Health, she started working on her first research project on the disease by chance, taking over for another researcher who left the lab. When she was offered a faculty position at Johns Hopkins in 2006, she continued that work here.

Though she'd originally planned to research other genetic neuromuscular diseases, such as some forms of ALS, the intriguing nature of SMA—and the promise of someday effectively treating this disease, possibly through harnessing the power of the SMN2 gene—commanded her attention. She and Crawford now lead the SMA charge here at Hopkins, seeing around 50 patients with this disease every year.

"It's such an incredibly important disease, and it has such interesting biology and a real chance for therapeutics. There are also such close collaborations between families affected by SMA, clinicians, and researchers—it feels like we're all in this together."

"Why wouldn't you want to work on this problem?" says Sumner. "I am so grateful that I ended up in this field. I thank my lucky stars every day."

Though she sees adult SMA patients, including Palmer, in the clinic, Sumner mostly deals with mouse models of the disease in the lab. These mice—genetically modified so that their own mouse version of the SMN gene has been removed, replaced by a single copy of the human SMN2 gene—are meant to mimic the most severe form of the disease.

Showing a visitor these animals in her lab, Sumner places a couple of SMA mice side by side with a pair of healthy mice that are about the same age, roughly 10 days old. The SMA mice look frail and sickly compared to their healthy brethren, measuring about a head shorter and about half their weight. With the tip of her index finger, she rolls one of the healthy mice onto its back. It immediately flips onto its feet, righting itself as if spring-loaded. After she does the same with an SMA mouse, the animal flounders like a bug stuck upside down, helplessly pawing the air in slow motion.

"He just doesn't have the strength to move his trunk," Sumner explains.

Animals like these are incredibly important



The Kershner Family

for understanding exactly why SMA exacts the toll it does. Though pinning down the responsible gene was a huge breakthrough, many questions remain about why patients develop their specific deficits. For example, though the same genetic quirk that causes SMA exists in every cell in the body, researchers don't yet know why the nerve cells that control muscle movement—specifically those that affect muscles in the trunk and parts of arms and legs closest to the trunk—seem to be uniquely affected. Additionally, these so-called motor neurons don't exist in isolation. Could the muscles they connect to, or the spinal cord they spring from, be part of the problem?

Using SMA mice, Sumner and her colleagues are trying to uncover the earliest events that distinguish these sick mice from healthy ones. Several years ago, they discovered that at two weeks of age, near the end of the animals' lives and at their weakest point, these mice still had plenty of living motor neurons—a stark contrast to many other neuromuscular diseases including ALS, in which weakness arises from the loss of these cells. That led Sumner's team to wonder whether that meant that SMA-affected motor neurons could live significantly longer than researchers had thought, but weren't functioning normally.

"This could have huge implications for therapy," says Sumner. "It's a lot easier to rescue a neuron that's there but dysfunctional compared to one that's dead."

Sure enough, recent research at her lab showed that the connections between neurons and muscles are abnormal in SMA mice. Over time, these bad connections cause muscles to have less activity, then weaken and shrivel. Further research suggests that problems with

the connections between neurons, known as synapses, are widespread throughout the animals' bodies.

"It's showing that we can't just rely on fixing the motor neurons in isolation and hope this solves the problem. It's really more complicated than that," Sumner says.

Besides just looking at the source of the problem, her lab is testing potential solutions, including a number of promising compounds. However, Sumner points out, it's important to remember that mice aren't people. Though some treatments, such as antisense oligonucleotides and gene therapy, have shown incredible promise in mouse models—sometimes even appearing to cure them of the disease—the same therapies may not work in the different physiology of humans. That's one reason why she frequently works with human tissue samples that she and Crawford collect during autopsies.

Dawn Kershner, whose son Oliver died from SMA three years ago, agreed for him to be autopsied for the two scientists' research. The blond-haired, blue-eyed infant seemed completely healthy when he was born.

"We counted ten fingers and ten toes and thought he seemed just perfect," she recalls.

But as the weeks went on, Kershner, a cardiologist at Union Memorial Hospital in Baltimore, and her husband realized that he wasn't gaining the same strength that she remembered seeing in her older son. Oliver's neck stayed floppy, and he eventually lost the ability to bring his hands up to his face. His pediatrician referred Oliver to Crawford, who diagnosed him with SMA at two months of age.

Kershner and her husband decided on hospice care for Oliver. Giving him a ventilator, feeding tube, and other life-extending but potentially uncomfortable treatments "just felt very unnatural to us," she says. Oliver died at 4 1/2 months old, on his brother's third birthday.

When Crawford asked Kershner and her husband whether they'd be willing to have Oliver autopsied, it was an easy decision for them. As a physician, she says, she knew how useful autopsies can be for furthering researchers' understanding of diseases—

Pumped Up cont.

knowledge that can't be attained in any other way.

"SMA is a horrible disease. If something as small as using Oliver's body for an autopsy could change the future for other families, so they wouldn't have to suffer from this experience," Kershner says, "we wanted to help."

Crawford assists in the specific portions of autopsies that relate to SMA on his own patients, work that he admits often feels awkward, uncomfortable, or incredibly sad. "I can't be there when they do the initial portion of it," he says. "But when they get to the actual tissues, they're not my patients, they're a science question."

"Each tissue collected from these autopsies is actually being used to answer a variety of science questions," Sumner says. She and her colleagues are studying fresh or frozen sections from muscles or the spinal cord to measure gene expression and protein levels. Tissues preserved in fixatives are shedding light on differences in structures between SMA and healthy patients. "Taken together," Sumner says, "autopsy tissues from SMA patients represent an invaluable gift for research that might eventually help countless other families."

Hannah Fallon and her mother, Kathy, also hope to help other families with SMA. Hannah, a blonde 14-year-old who likes to act, sing, and play video games—and happens to be in a wheelchair—is one of six siblings. She and her older brother Lance, now 24, both have SMA Type II.

By the time Hannah was diagnosed, around the time of her first birthday, Kathy Fallon and her husband had already amassed loads of expertise in dealing with SMA, painstakingly gathered from more than a decade of finding the right physicians, equipment and programs for Lance. They freely share their knowledge nowadays with other families dealing with the same issues.

"With Hannah, there hasn't been nearly as much guesswork," Fallon says, comparing her daughter's childhood to the often confusing and frustrating time she and her husband spent trying to figure out the best options for Lance.

In the future, she adds, kids with SMA may have even more options. Four years ago, Hannah participated in a multicenter drug trial to test a combination of compounds that showed promise in animal studies: an anticonvulsant drug called valproic acid and

levo-carnitine, a compound derived from an amino acid.

Five times over the course of a year, she came to Johns Hopkins to evaluate the effects of medication she was taking at home—either the real compounds or a placebo—through physical exams and blood tests. The hope was that researchers would see a marked improvement for those who received the real drugs in the ability to perform basic tasks, such as lifting their heads or rolling over.

Unfortunately, they didn't, says Crawford, who led Hopkins' portion of the study. But the researchers did learn a lot—not about treating SMA, Crawford explains, but about how to design a useful study to more accurately test future SMA therapeutics.

"It sounds so simple, but how do you measure something that's useful to patients and researchers?" he asks. "Patients are concerned about practical things, like feeding themselves, but researchers need something they can measure consistently in all the study subjects. It would be easy if everyone had exactly the same problem, but each of these kids is different, with different deficits."

"One thing this study taught researchers," Crawford explains, "is that the same activity can be defined in a variety of different ways by different researchers, thereby potentially impacting their reports on the effectiveness of test drugs. For example, whether a patient rolls over, sits up, or lifts his head isn't cut and dry—researchers use their own subjective guidelines to evaluate the success or failure of each of these actions. Additionally, SMA kids can devise workarounds by performing an activity in a way that doesn't truly measure what researchers had hoped."

"For future studies," he says, "researchers need a way to measure whether an intervention is truly effective in a way that everyone can agree on." That universal measure, he believes, is strength.

Though he and other neurologists who work with SMA patients often check their strength at exams using dynamometers, instruments that measure force, those tests are only consistently accurate if delivered by the same physician, Crawford explains. How a doctor wields the dynamometer—resisting a patient's pushes and pulls—can make a difference in measurements, he says, in a way that could lead to inconsistencies between measurements at different clinical trial sites.

"I do it my way, you do it your way. I can't train everyone to do it my way," he says.

To solve that problem, Crawford is developing a new device that researchers might eventually use as a standard measuring tool in future clinical trials. Its design is deceptively simple, he says: a piece of plywood with a post through the center and a stirrup attached. Painted red, the device looks like a fun toy to kids, rather than an odious task. Kids naturally want to yank on the stirrup, which connects to an internal dynamometer connected to a computer that records results.

"All you have to say is 'ready,' and kids want to pull as hard as they can," he says.

Crawford is currently building 12 of these devices and applying for a grant to evaluate them in a multicenter trial. If they prove useful, Crawford's strength testers could eventually be put to use in measuring the effects of other promising medicines on the forefront of SMA research, such as quinazoline, a drug that the FDA recently approved to be tested in Phase I trials in healthy volunteers.

This particular drug has an unusual story, Crawford explains. Rather than being the brainchild of a big pharmaceutical company, quinazoline was plucked out of thousands of other compounds after an advocacy group called Families of SMA paid a company to test these compounds on cells. Searching for a compound that raised the amount of SMN protein in cells, the company's scientists soon discovered that quinazoline did the trick.

Having paid the company for its work, Families of SMA held the rights to this drug. They eventually sold it to a small drug development company called Repligen for a nominal amount, with the agreement that Repligen would usher this compound quickly and effectively into clinical trials. If the company changes its mind, it's required to return rights to the drug back to Families of SMA so they can try to sell it to another development company.

Sumner and her colleagues are currently further testing quinazoline in SMA mice in the lab to better understand how it works and how to best follow its effects during a clinical trial. Eventually, says Crawford, it might be tested in his patients in a clinical trial at Hopkins. This drug, or others, might just be the break that SMA patients have been waiting for, Crawford says.

"When I started out in this field, the story was that kids with SMA Type II died in their school years. Now they're getting older with me," he says. "Now we get a chance to see how this plays out. It's been an incredible ride."

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 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 11th. Join 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.





Families of SMA Receives 250 Baby Einstein Discovery Kits for the Newly Diagnosed SMA Care Package Program

Families of SMA is so incredibly grateful to have received a donation of 250 Baby Einstein Discovery Kits, which is one of the items now included in all Newly Diagnosed Care Packages.

After learning about Wyatt's diagnosis, his parents Paula Lavigne and Chris Arnold, vowed to make his short life as happy as possible. Wyatt sat on Santa's lap, viewed the world on horseback and rode on a four-wheel all-terrain vehicle. He gazed at Christmas

tree lights, stared a giraffe in the face and petted a penguin. Wyatt also loved his Baby Beethoven Discovery Kit. Paula and Chris thought by including this item in every care package sent to newly diagnosed SMA families would be the perfect way to honor their son, Wyatt Teagan Arnold.

A Baby Einstein Discovery Kit is put into every Newly Diagnosed Care Package and sent out as soon as the newly diagnosed family has contacted

Families of SMA. These Baby Einstein Discovery Kits include a DVD, Book, Music CD and Parents' Guide.

Everyone at Families of SMA would like to extend our sincerest thanks to Paula Lavigne and Chris Arnold who have made such a wonderful donation to the FSMA Care Package Program in loving memory of their son, Wyatt Teagan Arnold.



Families of SMA Receives Funding for Bath Pads for Care Packages from the Joseph Lillo Spinal Muscular Atrophy Foundation

Families of SMA is incredibly grateful to receive funding to purchase 150 of the Leacho Safer Bather Infant Bath Pads, which are one of the items in all Newly Diagnosed Type I Care Packages. Care Packages are sent out to all newly diagnosed SMA families free of charge.

These bath pads are incredibly beneficial to SMA Type I infants as it allows them to bathe comfortably and safely while receiving adequate support and positioning for their bodies. Many families absolutely love these bath pads since it makes bath time a little easier and also their children enjoy taking baths in them. Some families have even used these bath pads around their home for a comfortable place for their SMA infant to lay on.

A Leacho Safer Bather bath pad is put into every Newly Diagnosed Type I Care Package and sent out as soon as the newly diagnosed family has contacted Families of SMA.

An incredible thanks to the Lillo Family for their love and support to the FSMA Care Packages in loving memory of their son, Joseph Dominic Lillo.

A special package from the Fight for Ford Event was delivered to the FSMA National Office. All of the children that attended this benefit for Ford Snizek created handmade cards to include in the FSMA Care Packages for newly diagnosed children. We love including these beautiful cards in the care packages! Special thanks to Meghan Nhar for hosting this event and having the kids put their creative minds towards a great project!



Thank you to the Starlight Children's Foundation for donating pillow pets to the FSMA South Florida Chapter. Families of SMA National Office received this generous shipment of adorable lady bug and bumble bee pillow pets to be included in our Newly Diagnosed Care Package Program. These pillow pets will be sent to all newly diagnosed families when they first contact FSMA!



Families of SMA is so grateful for a special delivery of stuffed animals and blankets from the Joseph Lillo Spinal Muscular Atrophy Foundation for Children for our Newly Diagnosed Care Package Program! These fun stuffed animals were donated in memory of Joseph Dominic Lillo. Thank you to the Joseph Lillo Spinal Muscular Atrophy Foundation for their amazing and continuous support!

Everyone at Families of SMA would like to send a heartfelt Thank You to Lorraine Panozzo who handmade a variety of beautifully crocheted sweaters, booties, hats and blankets for the FSMA Care Package Program. Thank you Lorraine for sharing your knitting skills and generosity with our families!



Families of SMA is so thankful to have received a shipment of 48 Linking Crab Toys donated by Jason Beasley and Rita Wright for our Newly Diagnosed Care Package Program! These toy crabs will be put into the Type I Care Packages, in memory of their son Eli Beasley-Wright. Thank you to Jason and Rita for these wonderful additions to our care packages and for your incredible support!





Our special weekly delivery of quilts is something everyone at the Families of SMA National Office looks forward to! It is so fun to marvel over the beautiful designs and colors, which are handmade by Cindy Bobolz for our Newly Diagnosed Care Package Program. For almost three years, Cindy has so generously donated over 500 quilts in honor of her granddaughter Nora Gooden! These beautiful quilts are sent to all newly diagnosed families when they first contact FSMA. We cannot thank Cindy enough for her dedication to SMA families and the Newly Diagnosed Care Package Program.

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

Here is an example of a great helpful hint from an SMA family:

- **Rain capes to protect the wheelchair out in the rain** - we came up with this idea after the Disney Conference rain. They cost around \$11 and are 100% Nylon, waterproof, wash easily and fit most neck sizes. The rain capes were purchased from cosmetic solutions (cosmeticsolutions.com).

Note: Could be a risk of getting caught in wheelchair.

— Michelle Erwin, Massapequa, NY



Hi Families of SMA,

Just wanted to thank you guys for the awesome care package you sent for Dominik. He was so excited! Thanks again for all the great work you do; we would be lost without your organization. May God bless you richly this New Year, and may he bring us a cure!

The Stoop Family of Culver City, CA

FSMA,

Today we received our gift pack and it brought tears of joy to my wife and I. Thank you so much for supporting our little Blakely and our family in this time of need. In time we hope to help in similar ways to those who put their time and effort into these extremely thoughtful gifts. We will be in touch and again thank you from the bottom of our hearts.

Elliot, Janell and Blakely Lewis of Ogden, UT



Thank you for the amazing package. Graham doesn't know where to start. :)

We have appreciated the resources. We are digesting the information as best as we can.

Thank you again.

Anna Hopkins of Rogers, AR

Dear Families of Spinal Muscular Atrophy,

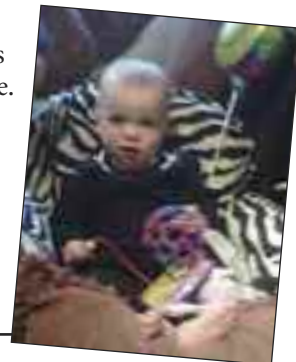
We received our care package today. It was heartwarming and a special day for our baby to be welcomed by her new "family." Many tears were shed, both in joy over being supported, but also fear and sadness as we begin to face the reality of our new life with her.

The Sepe Family of Barrington, IL

Families of SMA!

Thank you for this wonderful package. Words can not express our gratitude for your support!

Kacey Farrell of Cincinnati, OH



Thank you FSMA,

We love our wagon Love,

Mia and Bella Israel of Fort Lauderdale, FL



FSMA,

What an amazing gift. Thank you so much for all you've already done. We are very humbled and honored.

Thank you and I really appreciate being a part of this organization.

Thanks again,

Stephanie Geraghty of Jacksonville, NC

NeuroNEXT: Network for Excellence in Neuroscience Clinical Trials Sites Announced. First trial will be a multi-site Spinal Muscular Atrophy Biomarker Study

NeuroNEXT will provide a robust, standardized, and accessible infrastructure to facilitate rapid development and implementation of protocols in neurological disorders affecting adult and/or pediatric populations. The network includes multiple Clinical Sites, one Clinical Coordinating Center (CCC) and one Data Coordinating Center (DCC).

The following locations will be NeuroNEXT sites:

Data Coordinating Center:

- University of Iowa

Clinical Coordinating Center:

- Massachusetts General Hospital

Clinical Sites:

- Albert Einstein College of Medicine Yeshiva University
- Children's Hospital – Boston
- Children's National Medical Center
- Columbia University – Weill Cornell
- Emory University
- Massachusetts General Hospital
- Northwestern University
- Ohio State University
- Oregon Health and Science University
- SUNY (Buffalo, Downstate, Upstate, and Stony Brook)
- Swedish Health Services – Seattle
- University of Alabama at Birmingham
- University of California – Davis
- University of California – Los Angeles
- University of Cincinnati
- University of Colorado – Denver
- University of Kansas Medical Center
- University of Miami School of Medicine
- University of Pittsburgh
- University of Rochester
- University of Texas Southwestern Medical Center
- University of Utah
- University of Virginia – Charlottesville
- Vanderbilt University
- Washington University in St. Louis School of Medicine

Below Elizabeth McNeil, MD MSc, from the NINDS Office of Clinical Research, describes NeuroNEXT for FSMA in a recent addition of the Families of SMA newsletter Compass.

The National Institute of Neurological Disorders and Stroke (NINDS) has initiated NeuroNEXT to support clinical trials for neurological diseases. NeuroNEXT will have multiple clinical sites throughout the US. The goal of this new network is to facilitate the implementation of Phase II clinical trials, or “exploratory” trials. Before researchers and patients embark on a

“confirmatory” or Phase III trial which may take years and involve several hundred patients, they often want to answer some preliminary questions, for example: What is the best dose? Is there at least some signal that the new treatment works? Because a limited number of Phase III trials can be done at any given time, those should be focused on what appears to be the most promising path based on “exploratory” or Phase II trial findings.

While many areas may be explored in Phase II trials, two areas of particular relevance to SMA are biomarkers and clinical outcomes. While researchers have discussed and begun investigations of biomarkers for SMA, there are no qualified ‘biomarkers’ of SMA disease progression, disease stabilization or disease ameliorization.

NeuroNEXT aims to support a “Biomarker” study for SMA. Some patients have already participated in a biomarker study and may ask why another one is needed. The reason is that we need a biomarker that not only looks promising, but one that we know works, and one that can potentially be useful to the FDA as they evaluate a new treatment. By collaborating with FDA, NeuroNEXT will hopefully identify a biomarker which meets regulatory approval. Biomarker studies typically have no treatment aspect to them. Participating in a biomarker study is therefore a true act of altruism because there is no potential to directly benefit. However, the potential future benefit to all patients with SMA is great, if a biomarker is found that can accelerate the evaluation of new treatments.

Deciding which outcome measure to use is another challenge in clinical trials. A clinical outcome measure is a means to test how a patient functions or feels. Several outcome measures have been used in SMA research, but there is not yet one generally agreed upon outcome measure for clinical trials. SMA is a chronically progressive condition so changes may take a while to be evident. It is still not fully known what specific changes should best be monitored nor how much of a change should be considered ‘meaningful.’ Phase II studies can give insight into clinical outcome measures best suited for use in Phase III trials. Additionally, biomarker studies often measure a clinical outcome along with the biomarker to evaluate the possible association.

The road from ‘bench to bedside’ is long and fraught with pitfalls but careful planning, partnerships, and close attention to choosing the most promising paths increase the chances of reaching the desired destination.

NeuroNEXT aims to encourage private-public partnerships so that the most promising treatments can be tested, regardless of whether they come from academic, foundation or industry investigators.

American Medical Association Publishes Article on NINDS Clinical Trial Network NeuroNEXT with First Study on Spinal Muscular Atrophy

What are Biomarkers and Clinical Endpoints?

Clinical Endpoints: Sometimes referred to as clinical trial outcome measures, are the overall outcomes of an intervention that the clinical trial is designed to evaluate. Common endpoints are severe toxicity or disease progression.

Primary Endpoints or Outcome Measures: The key measurements or observations used to measure the effect of experimental variables in a study. Having reliable primary endpoints is critical for FDA registration (pivotal) trials.

Secondary Endpoints or Outcome Measures: Additional measures utilized to help evaluate therapeutic interventions, often used as go/no-go decision points early in the clinical development process, rather than as the key endpoints in FDA registration trials.

Biomarker: A measure that can be used as an indicator of a particular disease state or some other physiological state. It is a characteristic that when objectively measured is an indicator of a biologic processes, pathogenic processes, or pharmacologic responses to a therapeutic intervention. Biomarkers are often used in early drug development studies to help assess whether later larger more expensive trials are warranted.

Clinical trial network removes barriers common to studies of neurological diseases. First network study to be on SMA biomarkers.

With more than 600 neurological diseases—many of them rare—under its purview, the National Institute of Neurological Disorders and Stroke (NINDS) faces unique challenges in translating the findings from basic research into clinical practice. Completing clinical trials at individual academic centers is not possible, as patient populations are often scattered across the country. Yet creating a temporary consortium of clinical sites for each new trial, the traditional approach to multicenter trials, can be slow and inefficient. Additionally, industry has been somewhat reluctant to fund clinical trials in this area, according to Petra Kaufmann, MD, PhD, a neurologist and director of the office of clinical research at NINDS.

In response, NINDS has launched a nationwide clinical trials network designed to be flexible enough to study any of the hundreds of neurological diseases.

The Network for Excellence in Neuroscience Clinical Trials (NeuroNEXT) will include 25 clinical sites across the country and focus on Phase II studies. For efficiency, the network will make use of a central institutional review board, a data coordinating center housed at the University of Iowa, and a clinical coordinating center run by Massachusetts General Hospital. More information about the network is available at <http://www.ninds.nih.gov/NeuroNEXT>.

“NINDS has learned that one of the best ways to decrease the time and cost of clinical research is through well coordinated networks,” said Story Landis, PhD, NINDS Director, in a statement. “NeuroNEXT allows us to efficiently test new treatments, to move promising therapies along the pipeline, and to provide the expertise and resources that are needed for rapid implementation of large clinical trials.”

The institute already oversees the Neurological Emergencies Treatment Trials Network (NETT), which studies acute management of emergent neurologic conditions such as stroke, traumatic brain injury, seizures, and spinal cord injury. NINDS also supports a more traditional disease-centered network—the Parkinson Disease Biomarkers Identification Network, which is working to identify biological markers for risk, onset, and progression and to facilitate develop-

ment of treatments. Such clinical networks have established infrastructure that enables them to complete high-quality trials more quickly and economically than is possible for investigators who must create a new infrastructure for a specific trial, Kaufmann said.

But creating disease-specific networks for all neurological diseases wouldn't be feasible, Kaufmann explained. So she and her colleagues created a flexible network that could quickly launch trials on any number of conditions.

NeuroNEXT will focus on conducting trials to identify reliable biomarkers that are associated with neurological diseases and may help assess the effect of treatment. The focus on biomarkers will provide data that will allow the institute to prioritize Phase II studies, Kaufmann said.

The network is also designed to foster public-private partnership. Kaufmann explained that in addition to conducting trials funded by public agencies, the network will be able to conduct trials of experimental agents through cooperative agreements with the companies developing them. Kaufmann said she hopes that having an infrastructure in place that can rapidly test prospective therapies will encourage more companies to develop drugs for neurologic diseases.

NeuroNEXT will launch its first clinical trial this spring. The trial will probe biomarkers associated with spinal muscular atrophy (SMA), one of the most common neuromuscular disorders of childhood. According to Families of SMA, an advocacy organization, SMA affects 1 in 6000 infants and leads to difficulties with activities involving voluntary muscle movements such as walking, crawling, and swallowing. The genetic basis of this autosomal recessive disorder is well understood, Kaufmann noted, and animal studies have already pointed to potentially useful therapies. Now, the network hopes to tackle one of the main barriers to conducting clinical studies of these experimental therapies: the lack of reliable biomarkers. NINDS is also accepting applications for future trials, Kaufmann said. A new clinical trial network will conduct trials on many neurological diseases, including neurological disorders that are rare.

FDA Proposes to Revamp, Improve its Program for Approving Drugs for Rare Diseases

The U.S. Food and Drug Administration (FDA), the federal agency responsible for evaluating, approving, and regulating new drugs and therapies, recently released a draft five-year strategic plan that includes proposed policies and procedures for advancing the development of drugs for rare diseases such as SMA. Beginning in late 2012, the FDA's Center for Drug Evaluation and Research (CDER) expects to revamp and improve its Rare Disease Program by increasing outreach efforts to the rare disease patient community and providing specialized training in rare disease drug development to pharmaceutical companies, biotechnology firms, and FDA staff responsible for reviewing and approving applications for new drugs.

Specifically, FDA intends to add five staff positions to the Rare Disease Program; to develop and disseminate guidance and policy that will advance and facilitate the development of drugs for rare disorders; and to develop improved tools to evaluate the success of the Rare Disease Program. FDA also proposes to hold a public meeting by early 2014 to discuss complex issues in clinical trials for studying drugs for rare diseases.

As several potential therapies for SMA work their way through the drug development process, the FDA will become increasingly more important in the effort to identify a treatment. Historically, diseases such as SMA that are classified as "orphan" or "rare" face several hurdles in the design, execution, and interpretation of clinical trials that make it difficult to receive FDA approval. Some of these challenges include natural histories that are not well-described, small population sizes available to participate in clinical trials, and outcome measures that are not well-defined. The FDA's proposal intends to address these challenges and facilitate approval of drugs for rare diseases.

Families of SMA Funded Researchers Participated at the 2011 Society of Neuroscience Meeting Special SMA Symposium

The Society of Neuroscience (SfN) Meeting is the premiere neurobiology meeting annually with over 30,000 scientists attending. This year multiple presentations on Spinal Muscular Atrophy were given by scientists funded by Families of SMA. Events at SfN also included the annual SMA Satellite Symposium co-funded by FSMA.

The three speakers, all current or past Families of SMA grant award recipients, included:



Chien-Ping Ko, PhD, University of Southern California, who spoke on "Synaptic defects in the spinal and neuromuscular circuitry in SMA".



George Mentis, PhD, Columbia University, who spoke on "Functional and structural impairments in spinal cord circuitry in a mouse model of SMA".



Charlotte Sumner, MD, Johns Hopkins University, who spoke on "Disruptions of motor neurons, their axons, and target muscles in human SMA".

This year the symposium was held on November 14, 2011 at the Washington DC Convention Center, and it was entitled, "Pretzels and endplates: Motor neuron pathology and the role of SMN in motor neuron development". A growing number of SMA clinical trials are on the horizon, yet our understanding of neuronal development and degeneration in SMA is only now coming into focus. Therefore, the speakers presented cutting edge research that details the functional deficits of SMA neurons and how the emerging pathology in rodent models and human tissues provides an essential baseline for upcoming clinical trials as well as insight into SMN-associated function.

In addition to the SMA Satellite Symposium, there was another two-dozen presentations on SMA research given at the meeting. These included topics such as, the quinazoline compound called RG3039 that was licensed from FSMA to Repligen Corporation, stem cell therapy for SMA, gene therapy for SMA, potential new drug targets for SMA, mouse model studies of SMA and research into motor neuron pathology in SMA. These presentations included work from the labs of past and current FSMA funded researchers, such as Drs. Giancomo Comi, Hans Keirstead, Wilfried Rossoll, Chien-Ping Ko, Umrao Monani, Charlotte Sumner, Mike Kiledjian, Sibylle Jablonka, Laxman Gangwani and Natalia Singh, as well as other prominent SMA researchers.

Roche Licenses Spinal Muscular Atrophy Compounds from PTC Therapeutics for Development

Roche is entering into a partnership with PTC Therapeutics for an exclusive, worldwide license to the latter's Spinal Muscular Atrophy (SMA) program. It includes three preclinical-stage candidates and potential back-up programs. PTC has been developing the program in partnership with the SMA Foundation, which will retain an active role in the collaboration. The deal with Roche could see PTC receive up to \$460 million in development and commercialization milestones plus potentially double-digit royalties on commercial sales.



Roche and PTC have been collaborating since 2009 on the development of orally available small molecules based on the latter's GEMS™ (gene expression modulation by small molecules) platform. The SMA program developed by PTC is based on alternative splicing. "We found the science behind this program very compelling," notes Luca Santarelli, global head of Roche Neuroscience.

PTC is focused on the discovery and development of oral small molecule drugs that target post-transcriptional control processes. The firm's internal pipeline includes candidates against diseases in multiple therapeutic areas including rare genetic disorders, oncology, and infectious diseases.

Single Administration of Antisense Oligomer Delivered to the CNS Rescues a Severe Mouse Model of Spinal Muscular Atrophy

The Burghes laboratory at Ohio State University published a paper in *Human Molecular Genetics* showing a single dose of an antisense oligomer (ASO) can greatly benefit survival, weight gain, and motor function in severe mouse model of SMA.

In this study, the authors delivered a bolus ICV injection of anti-sense oligonucleotide (ASO) of morpholino chemistry to alter SMN2 splicing and increase SMN levels. Treated SMA mice had improvement in weight gain, motor activity, and increased survival from 15 days to over 100 days. Delayed CNS delivery (P4) had an intermediate advantage, evidence that earlier CNS treatment yields more robust effects, while delayed peripheral delivery after blood-brain-barrier maturation had only modest increased survival. This suggests that CNS delivery of SMN is key to therapeutic benefit in this SMA mouse model.

The authors suggest that CNS increases of SMN alone will likely have a major impact on SMA, and the early introduction by intrathecal delivery of morpholino oligomers is a potential treatment for SMA patients.

Repligen Provides Update on the Phase I Trial for Families of Spinal Muscular Atrophy Drug RG3039

Repligen Corporation provided a short update in their quarterly report released on the Phase I Trial in adult volunteers on the Spinal Muscular Atrophy Drug RG3039. This is the first novel drug specifically designed for SMA to reach the clinic. The drug, Quinazoline495, was licensed to Repligen from Families of SMA for clinical development in 2009. Families of SMA fully managed and funded the program to the stage of clinical development.

The Repligen report indicated:

We are developing RG3039 for Spinal Muscular Atrophy (SMA) which is a serious and debilitating neurodegenerative disease typically diagnosed in childhood. SMA is characterized by a defect in a single gene which results in diminished production of a key protein.

During the quarter we enrolled 24 subjects in a Phase I clinical study of RG3039 in healthy volunteers. To date there have been no serious adverse events in any subject.

Families of SMA fully funded and directed the preclinical development work with an investment of more than \$13 million prior to licensing RG3039 to Repligen. Families of SMA previously secured U.S. Orphan Drug Designation for RG3039, providing important regulatory and marketing incentives for the program. The work led by Families of SMA was the very first drug development program ever done for SMA.

Families of Spinal Muscular Atrophy Releases SMA Care Series Booklet on Nutrition Basics for SMA

“Nutrition Basics: Fostering Health and Growth for Spinal Muscular Atrophy” is Families of SMA’s newest release of the SMA Care Series Booklets, which are provided free of charge to all SMA families, as well as SMA Care Providers worldwide.

This 32 page booklet is focused on fostering health and growth for Spinal Muscular Atrophy. Nutrition is a complex issue for anyone, but especially for children and adults with SMA. As you go through this booklet you’ll see that SMA may present some nutritional challenges, but by educating yourself, talking to other parents and getting help from a registered dietitian, children with SMA can receive a variety of benefits from good nutrition.

A variety of important topics are covered in the Nutrition Basics booklet including:

- Nutrition 101 - Mastering the Basics
- Building Blocks - Understanding Nutrition for SMA
- Methods and Measurements - Assessing SMA Nutrition
- What, When and How Much? - Managing Nutrition in SMA
- Obstacles to Nutrition - Facing Special Feeding Challenges
- Finding the Balance - Preventing Overnutrition or Undernutrition

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.

Nutrition Basics was authored by an array of SMA nutrition experts from all over the US. Families of SMA would like to thank these authors who helped make this incredible booklet possible. They include:

- Mary Schroth, MD, University of Wisconsin Children’s Hospital
- Barbara Godshall, MMSc, RD, CSP, LD, CNSC, Cincinnati Children’s Hospital
- Rebecca Hurst, MS, RD, CD, University of Utah - Pediatric Motor Disorders Research Program
- Brenda Wong, MD, Cincinnati Children’s Hospital
- Kathy Swoboda, MD, University of Utah Medical Center
- Erin Seffrood, MS, RD, CSP, CD, American Family Children’s Hospital
- Mary Marcus, MS, RD, CSP, CD, University of Wisconsin
- Jamie Shish, RD, LDN
- Anne Meguiar
- Kathryn Rioch, RD, LDN
- Tonya Mason
- Jodi Wolff, RD, University Hospital Cleveland
- Connie Rizzo, MD, RD, LD

The SMA Care Series Booklets are produced with the kind support and generosity of the Angel Baby Foundation.



Families of SMA Releases Updated Care Series Booklet for Newly Diagnosed SMA Type I Families

This booklet is focused on the caring choices for parents of infants newly diagnosed with Spinal Muscular Atrophy Type I.

If you have just learned that your baby has Spinal Muscular Atrophy, we know this can be a difficult, confusing and worrisome time. Families of Spinal Muscular Atrophy is an organization dedicated to helping parents, like you, by offering support, funding research and providing education resources about SMA. At FSMA, we are always here to support you in any way possible.

The main topics of this new SMA Care Series Booklet review the decisions and basics of the main care options for newly diagnosed SMA Type I. Some of these include:

- Non-Invasive Respiratory Care
- Invasive Respiratory Care
- Palliative Care
- GI / Nutrition

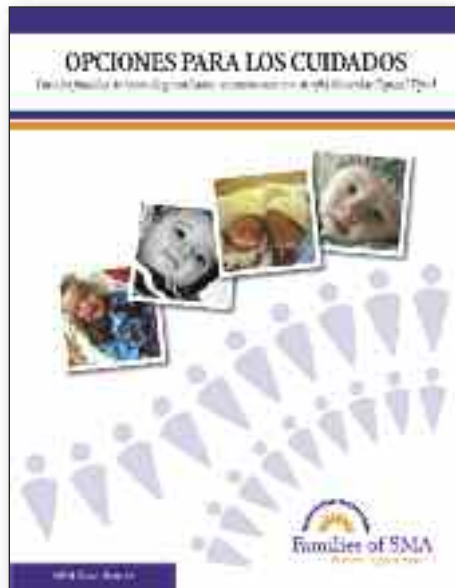
This booklet was updated by Vanessa Battista and other members of the Families of SMA Medical Advisory Council. It was produced with the kind support of the Angel Baby Foundation. We cannot thank them all enough for their efforts and great generosity.

Care Series Booklets now available in in Spanish

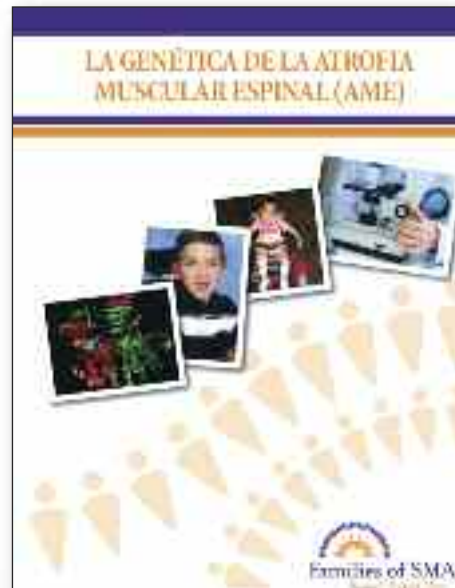
Families of Spinal Muscular Atrophy Releases SMA Care Series Booklet Translated into Spanish for Families

Families of SMA has begun a new program of translating all of the SMA Care Series Booklets into Spanish editions. These booklets will be on hand at the FSMA National Office as well as available for download on the Families of SMA website at www.CureSMA.org.

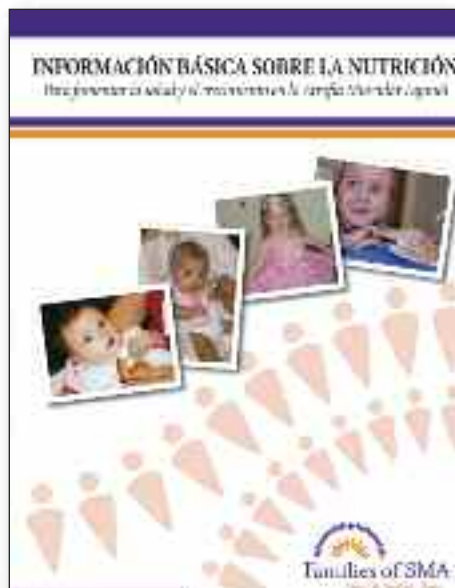
The following booklets that are available in Spanish are:



Caring Choices



The Genetics of Spinal Muscular Atrophy



Nutrition Basics



Breathing Basics

More booklets are scheduled to be translated, please visit the FSMA website for any new booklets.

Isis Pharmaceuticals Initiates Phase I Clinical Study in Patients with Spinal Muscular Atrophy

Isis Pharmaceuticals, Inc. announced on December 19, 2011, that it has initiated a Phase I study of ISIS-SMNRx in patients with Spinal Muscular Atrophy (SMA). Isis is developing ISIS-SMNRx as a potential treatment for all types of SMA.

The Phase I study of ISIS-SMNRx is a single-dose, dose-escalation study designed to assess the safety, tolerability and pharmacokinetic profile of the drug in children with SMA between the ages of 2-14 who are medically stable. In this study, ISIS-SMNRx will be administered intrathecally as a single injection directly into the spinal fluid. Intrathecal administration of an antisense drug, ISIS-SOD1Rx, has been shown to be safe and well tolerated in an ongoing Phase I study in patients with amyotrophic lateral sclerosis.

“SMA represents a serious unmet medical need with currently no available treatments. ISIS-SMNRx is our first drug to intervene in the splicing of RNA to increase the production of a normal protein, SMN. Together with Dr. Krainer’s lab, we have validated the antisense approach to treating this disease and are now advancing this program into clinical studies,” said C. Frank Bennett, Ph.D., Senior Vice President of Research at Isis. “We are committed to quickly developing this drug and are finalizing what we believe will be a rapid development path for this drug in all types of SMA. Once we evaluate ISIS-SMNRx as a single-dose in children with SMA, we will move to multiple-doses in our Phase I studies and eventually evaluate the drug in Phase II studies in children with SMA, including infants with SMA Type I.”

“Our strategy to treat SMA relies on a simple, powerful antisense method that boosts SMN protein levels by fixing a genetic RNA splicing glitch. Working with Isis, we have successfully redirected splicing to increase functional SMN production. We have thoroughly validated this approach in multiple animal models, observing marked improvement in modifying the disease course in both mild and severe models of SMA,” said Adrian Krainer, Ph.D., Professor of Molecular Genetics at Cold Spring Harbor Laboratory in Long Island, NY. “We look forward to translating this important discovery into an effective treatment for this serious disease.” Dr. Krainer is a member of the FSMA Scientific Advisory Board.

“We are very pleased to see the great milestone of a disease-modifying drug treatment advancing into clinical trials in SMA

patients,” said Kenneth Hobby, President of Families of SMA. “Our community has worked for a long time to reach the goal of moving specific therapies for SMA from the bench and into the clinic. This has been made possible by close interactions between basic researchers, families, clinicians, and industry. Families of SMA applauds Isis for investing in and leading drug developments efforts for this devastating, orphan disease.”

Isis Pharmaceuticals exclusively licensed certain 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.

“SMA is a terrible disease and the leading genetic cause of infant mortality. The ability of our drugs to specifically target RNA and drive the production of SMN may be able to compensate for the underlying genetic defect and offer some therapeutic benefit to patients with SMA. We are grateful for the support provided by Families of SMA in funding early research on SMA, which has significantly expanded the basic science of SMA and provided a roadmap to developing the first targeted therapy to treat SMA,” said Frank C. Bennett, Ph.D., Senior Vice President of Research at Isis Pharmaceuticals.

The United States Food and Drug Administration granted Orphan Drug Designation with Fast Track Status to ISIS-SMNRx for the treatment of patients with SMA.

For more information on the Phase I study of ISIS-SMNRx please visit: www.clinicaltrials.gov.

About Splicing

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. These diseases are referred to as splicing diseases and include SMA, cystic fibrosis and Duchenne’s muscular dystrophy.

Biogen Idec and Isis Pharmaceuticals Announce Global Collaboration for Antisense Program Targeting SMA

Biogen Idec and Isis Pharmaceuticals, Inc. announced on January 4, 2012, that they have entered into an exclusive, worldwide option and collaboration agreement under which the companies will develop and commercialize Isis' antisense investigational drug, ISIS-SMNRx, for the treatment of Spinal Muscular Atrophy (SMA). Isis' ISIS-SMNRx is designed to compensate for the underlying genetic defect that causes SMA.

Under the terms of the agreement, Isis will receive an upfront payment of \$29 million and is eligible to receive up to \$45 million in milestone payments associated with the clinical development of ISIS-SMNRx prior to licensing. Biogen Idec has the option to license ISIS-SMNRx until completion of the first successful Phase II/III trial. Isis could receive up to another \$225 million in a license fee and regulatory milestone payments. In addition, Isis will receive double-digit royalties on sales of ISIS-SMNRx. Isis will be responsible for global development of ISIS-

SMNRx through the completion of Phase II/III registrational clinical trials, with Biogen Idec providing advice on the clinical trial design and regulatory strategy. If Biogen Idec exercises its option, it will assume global development, regulatory and commercialization responsibilities.

"SMA is a heartbreaking disease – it can kill children before their second birthday and there are currently no therapies to treat the disease," said George A. Scangos, Ph.D., CEO of Biogen Idec. "It is exactly the kind of disease and program that we are focused on at Biogen Idec. The unmet need could not be any greater, the program fits with our mission to bring innovative therapies to patients with serious neurologic diseases, and Isis' antisense compound has the potential to be a highly effective, first-to-market therapy for this deadly disease. We have the utmost respect for Isis' scientific leadership and expertise in antisense technology, and we have crafted a collaboration that brings together our two

companies' strengths toward a common goal."

"Biogen Idec's expertise in the global development and commercialization of innovative new therapies for neurologic diseases is a great strategic fit to advance ISIS-SMNRx," said Stanley T. Crooke, M.D., Ph.D., Chairman of the Board and Chief Executive Officer. "This alliance is consistent with our business strategy to develop antisense drugs to proof-of-concept with a knowledgeable partner that is committed to supporting the rapid development of the drug. Given the severity of the unmet need in SMA, our proof-of-concept studies should also serve as the registrational trials for ISIS-SMNRx. We believe that, together with Biogen Idec, we will be able to expeditiously develop this investigational drug in hopes of bringing to market an effective and desperately needed treatment to improve the lives of children with SMA."

About ISIS-SMNRx

ISIS-SMNRx is designed to treat all types of childhood SMA by altering the splicing of a closely related gene (SMN2) to increase production of fully functional SMN protein. The United States Food and Drug Administration granted orphan drug status and fast track designation to ISIS-SMNRx for the treatment of patients with SMA.

In December 2011, Isis initiated the first Phase I clinical study evaluating ISIS-SMNRx in children with SMA. The Phase I study is a single-dose, dose-escalation study designed to assess the safety, tolerability and the pharmacokinetic profile of the drug in children between the ages of 2 and 14 who are medically stable. In this study, ISIS-SMNRx will be administered intrathecally as a single injection directly into the spinal fluid. Isis plans to follow this study with a Phase I multiple-ascending dose study.

Isis Pharmaceuticals exclusively licensed certain 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.

"SMA is a terrible disease and the leading genetic cause of infant mortality. The ability of our drugs to specifically target RNA and drive the production of SMN may be able to compensate for the underlying genetic defect and offer some therapeutic benefit to patients with SMA. We are grateful for the support provided by Families of SMA in funding early research on SMA, which has significantly expanded the basic science of SMA and provided a roadmap to developing the first targeted therapy to treat SMA," said Frank C. Bennett, Ph.D., Senior Vice President of Research at Isis Pharmaceuticals.

About Biogen Idec

Through cutting-edge science and medicine, Biogen Idec discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hemophilia and autoimmune disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company. Patients worldwide benefit from its leading multiple sclerosis therapies and the company generates nearly \$5 billion in annual revenues.

For additional information about the company, please visit www.biogenidec.com.

About Isis Pharmaceuticals

Isis is exploiting its leadership position in antisense technology to discover and develop novel drugs for its product pipeline and for its partners. Isis' broad pipeline consists of 28 drugs to treat a wide variety of diseases with an emphasis on cardiovascular, metabolic and severe and rare/neurodegenerative diseases, and cancer.

Additional information about Isis is available at www.isispharm.com

Newly Funded Spinal Muscular Atrophy Researcher Profiles at Families of SMA



Dr. Chien Ping Ko

Dr. Ko is a Professor at the University of Southern California and has been awarded a new basic research grant for \$70,000 from Families of SMA. Dr. Ko is also presenting at the Society for Neuroscience SMA Symposia this November, which is co-sponsored by Families of 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. I am now Professor and Head of the Section of Neurobiology at USC.

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 SMA. Given 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 SMA research?

We have recently found that NMJs in several muscles (such as neck muscles) are severely defective in a mouse model mimicking the severe type of SMA. We are now using NMJs in these vulnerable muscles as a benchmark to test whether certain candidate compounds could mitigate the neuromuscular defects in this severe mouse model. We are also characterizing NMJs in several new mouse models that are less severe. Our ultimate goal is to understand the fundamental biology of synaptic defects and to find a treatment to restore these defects in SMA.

Dr. Ko's lab recently published a study in Human Molecular Genetics using some of the FSMA funding. Most of the FSMA funding will be used for the next phase of this project.

Description of FSMA-funded project.

New Neuromuscular Preparations for In Vivo Evaluations of Drug Efficacy, \$70,000 for one year.

Objective: The project will use a mouse model of SMA to test the hypothesis of whether defects in neuromuscular synapses play a key role in the pathogenesis of SMA, and also to establish novel neuromuscular preparations for in vivo evaluation of drug efficacy in SMA.

Research Strategy: The project characterizes how certain muscles are highly vulnerable to loss of synaptic connections between motor nerves and muscle fibers. Also, these vulnerable muscles will be used to test for drug efficacy. The proposed study is expected to establish a group of vulnerable muscles that can be used for future in vivo drug testing in SMA animal models.

Significance of Project: The proposed studies will provide new understanding of the basic biology of synapse maintenance and disruption, which would in turn lead to novel insights into the pathogenesis of SMA and its apparent selectivity. Furthermore, this research can help lead to new therapies that promote synapse maintenance and prevent synapse disruption in SMA.



Drs. Rashmi Kothary and Lyndsay Murray

Drs. Kothary and Murray are researchers at Ottawa Hospital Research Institute and have been awarded a basic research grant for \$120,000 from Families of SMA.

Who are you?

Dr. Lyndsay Murray is a postdoctoral researcher in her second year post PhD, working at the Ottawa Hospital Research Institute. She works in the laboratory of Dr. Rashmi Kothary.

Dr. Kothary is Associate Scientific Director and Senior Scientist at the Ottawa Hospital Research Institute and Professor at the University of Ottawa in the Department of Medicine and at the Department of Cellular and Molecular Medicine. He also serves as the Associate Director of the Ottawa Health Research Institute and is a member of the Medical Research Council of Canada Scholarship. Dr. Kothary has a keen interest in modeling disease pathology in mice and has developed an intermediate mouse model of SMA.

How did you first become involved with SMA research?

A talented postdoctoral fellow, Dr. Christine DiDonato - now a Professor at Northwestern, introduced Dr. Kothary to the SMA research community in 1999, when she joined his group to learn about mouse modeling. Dr. Kothary has been in the SMA field ever since.

Dr. Murray first became involved in SMA research during graduate school. Her PhD work was focused on neuromuscular junction vulnerability in SMA, and she has continued her interest in SMA during her postdoctoral

studies in Dr. Kothary's lab. Dr. Murray obtained her Ph.D. from the laboratory of Dr. Thomas Gillingwater at University of Edinburgh in Scotland, and published several key SMA papers there.

What is your current role in SMA research?

Dr. Lyndsay laboratory-based research is focused on the mechanisms underlying motor neuron vulnerability in SMA. Although many recent advances within the field have generated potential therapeutic strategies, more work is needed to better define the mechanism of disease underlying motor neuron vulnerability, which should unveil new and specific therapeutic targets.

Description of FSMA-funded project

Identification and Characterization of Factors Critical in Regulating the Selective Vulnerability of Distinct Motor Neuron Pools in SMA Model Mice, \$120,000 for two years.

Objective: In SMA, motor neurons connect the spinal cord to skeletal muscle degenerate. However not all motor neurons are affected equally, with those targeting muscles for control of posture and respiration being the most vulnerable. The project objective is to determine the underlying differences in gene expression between motor neurons that are vulnerable in SMA and those that are less vulnerable.

Research Strategy: This team proposes to use mouse models of SMA to dissect out individual motor neurons from the spinal cord and use novel technology to compare the expression profile of genes that are turned on and off between motor neurons which are vulnerable and non-vulnerable to degeneration in SMA. This work will help identify genes that make motor neurons more vulnerable to degeneration in SMA.

Significance of the Project: This work will help uncover the fundamental mechanisms that make motor neurons vulnerable in SMA and will generate new avenues to develop therapeutic strategies by identifying new drug targets.

FSMA Canada has funded \$1.9 Million in Research since 2004, supporting 12 Basic Research Grants, the Montreal Site of Project Cure SMA that participated in two SMA clinical trials, and Laboratory Support for all of Project Cure SMA.

In this study they determine a critical threshold of SMN protein that dictates onset of SMA in an intermediate mouse model of SMA. With about 15% normal level of SMN protein, these mice display reduced body

weight, motor neuron loss and motor defects, but significantly less than serve mouse models of SMA. Also these mice are phenotype-free until P10 with a median life expectancy of 28 days, longer than severe mouse models. The work suggests that increasing SMN protein levels minimally could be of significant benefit.



Wilfried Rossoll and Dr. Claudia Fallini

Drs. Rossoll and Fallini are at Emory University and have been awarded a new basic research grant for \$140,000 from Families of SMA.

Who are you?

Claudia Fallini, PhD is a graduate from the University of Milan in Italy. She did most of her graduate research on the role of RNA regulation in motor neuron biology in the laboratory of Dr. Silani in Milan.

Wilfried Rossoll has received his PhD in Genetics from the University of Vienna/Austria. After working as a postdoctoral at the Institute of Clinical Neurobiology in Würzburg, Germany, he joined Gary Bassell's lab in Atlanta, GA. Currently he is Assistant Professor at the Department of Cell Biology at Emory University.

How did you first become involved with SMA research?

The interest in motor neuron biology that Claudia developed during her PhD found a perfect match in Dr. Bassell's lab, where research on basic cellular processes is complemented by the study of how these processes are deranged in neurodegenerative diseases such as SMA.

Wilfried Rossoll became first involved in SMA research as a postdoctoral fellow in Michael Sendtner's lab in Germany. At FSMA research meetings, we had for the first time the opportunity to directly meet the patients and their families, who lead the fight against SMA. This experience has always been a great inspiration and motivation for our research.

What is your current role in SMA research?

The main emphasis of our work is on the role of SMN in spinal motor neurons, the cell type that is most severely affected in SMA. More specifically, we are investigating the function of SMN in the transport and translation of messenger RNA molecules along the long processes (axons) that transmit signals from the spinal cord to muscle cells.

Description of FSMA-funded project.

Effects of SMN on mRNA transport and local protein synthesis in motor axons, \$140,000 for two years.

Objective: SMN carries out an essential function in all cell types, though it still remains unclear why only the highly specialized and polarized motor neurons degenerate in the disease. This research group hypothesize that defects in the transport of messenger RNAs (mRNAs) along the axon and the synthesis of new proteins at the neuromuscular junction itself may be responsible for this selectivity.

Research Strategy: In this study, the researchers propose to investigate defects in mRNA transport and protein synthesis in motor neurons derived from the SMA mouse model. The team plans to identify specific defects in the regulation of mRNA metabolism that may contribute to the selective susceptibility of motor neurons to low levels of SMN.

Significance of Project: The proposed project will help gain insight into the mechanisms leading to specific motor neuron degeneration in SMA, paving the way to the development of new therapeutic strategies.

A portion of this program is generously funded by Stop SMA, a family foundation located in central Mississippi.

When Jeff and Dee Horton discovered that their daughter Evie was diagnosed with SMA in 2008, they focused their energies on increasing awareness of the disease while raising money to fund SMA research. In August 2011, Stop SMA pledged \$70,000 over two years to fund this Basic Research project at Emory University. Families of SMA is appreciative of the partnership between the Horton family and Stop SMA and gratefully acknowledges their contribution to the advancement of SMA therapeutic development.



Dr. Stephen Kolb

Dr. Kolb is an Assistant Professor of Neurology at The Ohio State University and has been awarded a basic research grant for \$70,000 from Families of SMA.

Who are you?

I am a physician-scientist at The Ohio State University. I am a neurologist with an interest in motor neuron disease including SMA, and I have a laboratory that is devoted to an understanding of the molecular basis of motor neuron diseases.

How did you first become involved with SMA research?

I became involved with SMA research by joining the Gideon Dreyfuss laboratory for my postdoctoral fellowship. As a neurology resident at the University of Pennsylvania, I met patients with SMA for the first time.

What is your current role in SMA research?

My laboratory is involved in a number of pre-clinical experiments in collaboration with Drs. Arthur Burghes and Brain Kaspar at OSU/NCH, as we move closer to SMA clinical trials.

Description of FSMA-funded project:

Validation of Spinal Muscular Atrophy Biomarkers in VALIANT Subjects, \$70,000 for one year.

Objective: In 2007, the Project Cure SMA Clinical Trial Network, funded by FSMA, initiated a Phase II Placebo Controlled Trial of Valproic Acid in Ambulant Adults with Spinal Muscular Atrophy (VALIANT; ClinicalTrials.gov identifier NCT00481013) led by Dr. John Kissel. The trial is now completed and the analysis of clinical outcomes is currently in progress. During the course of the trial, a large number of blood samples were obtained so that a systematic analysis of molecular SMA biomarkers could be achieved.

Research Strategy: The team plans to measure SMN mRNA in the Simard lab and SMN protein in the Kolb lab using well-characterized, validated assays in SMA patient blood samples. They will also assess a more novel assay of SMN functionality in human samples for SMN functional activity. In addition, they will assess HDAC activity in these samples, as this is the proposed

mechanism for Valproic acid. It is anticipated that they will detect any biochemical changes in SMA patient blood samples that are the result of VPA administration.

Significance of Project: The design of therapeutic clinical trials for SMA hinges upon the expectation that survival or objective improvement in phenotype will be achieved. However, this is greatly aided at early stages by molecular biomarkers. At the completion of this project, it is expected that this project will be able to provide clearer recommendations for the design of biological measures in SMA trials.



Dr. Umrao Monani

Dr. Monani is an Assistant Professor at Columbia University and has been awarded a basic research grant for \$160,000 from Families of SMA.

Who are you?

I am an assistant professor of Pathology and Neurology and a member of the Center for Motor Neuron Biology and Disease at Columbia University. I received my B.Sc. from St. Xavier's College in Bombay, India and a PhD from the Ohio State University.

How did you first become involved with SMA research?

I first became involved with SMA research in 1993 as a graduate student in the laboratory of Dr. Arthur Burghes, a pioneer in the field. In 1997 I attended my first FSMA conference and met Audrey Lewis (FSMA founder), realizing how truly fortunate I was to be researching Spinal Muscular Atrophy. I have been researching the disease ever since, first in private industry and currently in academia in my own laboratory at Columbia.

What is your current role in SMA research?

My lab uses mouse models to understand the biology underlying SMA with a view to eventually finding a safe and effective treatment for the human disease. We were amongst the first to demonstrate how important SMN, the SMA protein, is to the development and health of the neuromuscular synapses, structures where the motor neurons communicate with muscle cells.

Description of FSMA-funded project:

Exploring novel genetic determinants of disease severity in spinal muscular atrophy model mice, \$160,000 for two years.

Objective: SMA is caused by mutations in the SMN1 gene that lead to reduced SMN protein. The project stems from observations that members of the same family sometimes exhibit different SMA severities, even though they have same genotype (the same SMN1 mutation and same number of SMN2 gene copies). The researchers aim to better understand the genetic and biochemical pathways that lead from reduced SMN protein to the neuromuscular phenotype characteristic of SMA.

Research Strategy: Mouse models of the disease will be used to probe the existence of genetic factors that alter the course and severity of SMA. This project expects to successfully identify one or more factors that modify disease symptoms in SMA mice.

Significance of Project: The identification of factors that alter the disease in SMA mice will shed light on novel pathways that lead from SMN protein deficiency to neurodegeneration. Additionally, they could lead to the design of novel and more effective therapies for SMA by identifying new drug targets.



Dr. Louise Simard

Dr. Simard is Professor and Head of Biochemistry and Medical Genetics at the University of Manitoba and has been awarded a basic research grant for \$25,000 from Families of SMA.

Who are you?

I am a research scientist and geneticist who has sought to find ways to translate our basic understanding of SMA to practical outcomes for SMA families. Currently, I am Professor and Head of Biochemistry and Medical Genetics at the University of Manitoba, Faculty of Medicine.

How did you first become involved with SMA research?

My interest in SMA was spurred by neurologist colleagues at a time when the gene responsible for SMA had not yet been identified. The neurologists baited the hook, Families of SMA reeled me in!

What is your current role in SMA research?

As a geneticist, I have contributed by meeting families and talking genes and genetics, and by aiding in the transfer of DNA tests from a research lab to a diagnostic lab setting that makes these tests available to SMA families. I am also the lead author that created the

Genetics of SMA Booklet for Families of SMA. As a scientist, I have tried to understand how the SMN gene works to make SMN mRNA and SMN protein. I have also developed new protocols on how to measure these consistently and accurately in patients during SMA clinical trials. I have performed this task for multiple Project Cure SMA clinical trials, such as VALIANT. I hope that this information will one day translate into new therapeutic approaches for patients and families.

Description of FSMA-funded project:

Validation of Spinal Muscular Atrophy Biomarkers in VALIANT Subjects, \$25,000 for one year.

Objective: In 2007, the Project Cure SMA Clinical Trial Network, funded by FSMA, initiated a Phase II Placebo Controlled Trial of Valproic Acid in Ambulant Adults with Spinal Muscular Atrophy (VALIANT; ClinicalTrials.gov identifier NCT00481013) led by Dr. John Kissel. The trial is now completed and the analysis of clinical outcomes is currently in progress. During the course of the trial, a large number of blood samples were obtained so that a systematic analysis of molecular SMA biomarkers could be achieved.

Research Strategy: The team plans to measure SMN mRNA in the Simard lab and SMN protein in the Kolb lab using well-characterized, validated assays in SMA patient blood samples. They will also assess a more novel assay of SMN functionality in human samples for SMN functional activity. In addition, they will assess HDAC activity in these samples, as this is the proposed mechanism for Valproic acid. It is anticipated that they will detect any biochemical changes in SMA patient blood samples that are the result of VPA administration.

Significance of Project: The design of therapeutic clinical trials for SMA hinges upon the expectation that survival or objective improvement in phenotype will be achieved. However, this is greatly aided at early stages by molecular biomarkers. At the completion of this project, it is expected that this project will be able to provide clearer recommendations for the design of biological measures in SMA trials.

Funded by Families of SMA Canada.; FSMA Canada has funded \$1.9 Million in Research since 2004, supporting 12 Basic Research Grants, the Montreal Site of Project Cure SMA that participated in two SMA clinical trials, and Laboratory Support for all of Project Cure SMA under the leadership of Dr. Simard.



Dr. Christopher Henderson

Dr. Henderson is a Professor at Columbia University and has been awarded a basic research grant for \$160,000 from Families of SMA.

Who are you?

I am a neuroscientist interested in motor neurons, what allows them to develop normally and what triggers their degeneration in diseases like SMA and ALS.

How did you first become involved with SMA research?

When I was still a postdoctoral fellow in Paris, France, I collaborated with Dr. Michel Fardeau, one of the real experts on the disease. We showed in 1987 that muscle biopsies from SMA patients – which were then taken routinely for diagnosis – contain factors that prevent nerve regrowth. This was my first introduction to working with clinicians to understand disease mechanisms and I have tried to continue ever since.

What is your current role in SMA research?

My lab focuses on defining potential therapeutic targets in SMA. This requires studying the disease process in mouse models and attempting to define molecular events that, when prevented, will confer benefit. My recent partnership with Dr. Hynek Wichterle in co-directing the Project A.L.S Laboratory for Stem Cell Research, and our collaboration with Dr. Kevin Eggan at Harvard, has given us access to human motor neurons with the same genetic makeup as the patients. I am also one of the co-directors of the Motor Neuron Center at Columbia University, which brings together 40 groups working on motor neuron biology and disease, and which houses 15 groups working directly on SMA.

Description of FSMA-funded project:

Stem Cell Models of SMA: Molecular and Cellular Mechanisms, \$160,000 for two years.

Objective: One of the main challenges in understanding SMA is determining how the lack of a precise protein, SMN, found in all the cells of the body primarily affects motor neurons. The objective of this project is to establish and characterize human cellular models of the disease “in a dish” and to use these models to determine the molecular pathways affected.

Research Strategy: This team will develop unique new tools, using human stem cells carrying the genetic mutation responsible for SMA, which can be differentiated in vitro into any kind of specific cell type of the human organism. In this case, they will be turned into motor neurons. They will be used to generate a series of SMA motor neurons with differing levels of SMN protein and then characterized both at the morphological and molecular level, in order to better understand the pathological of the disease. Thus the project will generate a highly valuable set of tools for studies of SMA pathology in a dish.

Significance of Project: Even though the biological cause of SMA in humans has been identified and animal models of the disease have been developed, remarkably little is known about the cellular and molecular mechanisms that lead to the specific loss of motor neurons in the human patients. The use of this unique set of tools will help us to answer these questions and to lead to new targets for therapeutic strategies for SMA.



Sydney Span



Matthew Freitas



Maxwell Peppers



**Tambryn
Campbell**



Peter and Lucy Henkel



Ryan Cottor



Sara Greene



Noah Hyche



**Megan, Alyssa and
Griffen Kingkiner**



**Micah and Olivia
Hargrave**



Oskar Karlsson



Madi Ramirez



**Murphy and Anders
Potter**



The Abraldes Girls



Tambryn and Braelyn Campbell



Addison, Haley and Shannon Kuester



Tambryn and Braelyn Campbell



Aaron Phelps



Addison and Aspen Tarrence



Adam and Alex Blair



Nathan Cooper



Leila Grace Cox



Alejandro Ako



Al and Jack Freedman with Mark Baiada



Claire and Lauren Gibbs



Alex and Caroline Luccasen



Aiden Marie Neely



Alli Williams with her brother and sisters



Danny McHale



Caitlin and Grace Trainor

Practice Makes Perfect

CHOP's simulation training prepares clinicians for the unexpected

Article featured in the *Fast Forward: The 2010-11 CHOP Annual Report—The Children's Hospital of Philadelphia*—Visit www.chop.edu
Reprinted with permission 2012

The tension is palpable. “He’s seizing,” shouts a nurse. “Call a code blue.” Room 916 South fills with doctors, nurses, respiratory therapists. “I’ll take the lead,” says a second-year critical care fellow. “Start compressions.” A nurse steps up. Press, press, press. “Feel for a pulse.” “Is he seizing again?” It doesn’t matter that the patient is plastic. It blinks, it bleeds, it breathes, its heart beats. The code team responding is treating it like a real baby in distress. These clinicians are going through a dry run on a soon-to-be-opened patient floor at The Children’s Hospital of Philadelphia, making sure the newly assembled staff and the equipment are ready when real patients arrive. Children’s Hospital is always ready. Because of its vast expertise, CHOP draws patients with the rarest conditions; it provides cutting-edge treatments; it uses state-of-the-art equipment. All levels of care providers — from the newest-minted clinicians to the world-renowned specialist — want to be at their most confident when they care for their patients. Whether it’s rehearsing the safest way to change a long-term intravenous line, choreographing the delivery of a baby with an extremely rare birth defect or getting a head start on training at the Critical Care Fellow Boot Camp each summer, CHOP simulates scenarios clinicians will face so they can rehearse safely — first to competence, then to excellence. In CHOP’s pioneering hub-and-spoke training model, some simulations take place in a traditional simulation center, a mock patient room with an adjacent classroom; others take place in the units or operating rooms where patient care happens. Some simulations use high-tech medical manikins; others use trained actors as patients or parents. Still others, like for interventional radiology and robotic surgery, use special virtual reality training equipment, similar to the way pilots use a flight simulator. “These simulators are all just tools,” says Vinay M. Nadkarni, M.D., medical director of CHOP’s Center for Simulation, Advanced Education and Innovation. “It’s really about the education and practicing in realistic situations. It’s about being the very best we can be.

They’ve Come a Long Way, Baby

These are not a child’s baby-dolls. With a total price tag of more than \$1 million, the 18 medical manikins, additional body parts and

related high-tech equipment used for simulation training at Children’s Hospital are well worth the investment for medical personnel and, ultimately, our patients. Manikins are as tiny as a 1 pound, 1 ounce infant and as big as an older teen. Their realistic bodies hide sophisticated electronics that allow them to act and react as a real child or adolescent would. The accompanying computer gives practitioners the manikin’s vital signs in real time, showing if resuscitation efforts are improving the heart rate and oxygen levels. Manikins can be preprogrammed to act as if they’re having a seizure, a cardiac arrest, a blocked airway, an allergic reaction or a multitude of other problems. Switch out an arm and a nurse can administer an IV (a pouch inside accumulates the fluid). Attach a different leg and a physician can stabilize and cast a broken bone or suture a deep cut. To simulate rashes or other skin conditions, a manikin may wear makeup. The Center for Simulation, Advanced Education and Innovation uses manikins in more than 800 simulations each year — all preparing CHOP clinicians to provide the safest, most efficient, state-of-the-art care to children.

Ready for Change

Even the best clinicians need practice, want practice. As procedures grow more complex and equipment becomes more sophisticated, physicians and other practitioners at Children’s Hospital constantly strive to improve their skills so they stay on top. That takes practice, evaluation and feedback, and then more practice. “Whenever you bring in new techniques, new technology or new equipment, you don’t want to practice on patients, but in simulation,” says Nadkarni. “You test the environment to find potential or real errors that could happen. Then you fix any



problems before they ever happen with real patients.” For surgeons, CHOP created a new program called STACK — Surgical Training Advancement Competency and Knowledge — to practice minimally invasive surgery, in which surgeons manipulate instruments remotely, using tiny lights and cameras in the patient’s body to guide them. “We will have the same equipment in STACK as in the OR: same screens, same cameras, same everything,” says Thane Blinman, M.D., director of Minimally Invasive Surgery. “You deliberately practice until it becomes second nature. Our mission is for surgeons to know what every single piece of equipment does and how to use it — like a marine who can fieldstrip his rifle in his sleep.” Christina Mohammed and Omar Hardy could not take their baby, Omar Jr., home from Children’s Hospital without the skills to care for him and the confidence that they could use them. “After he had a plug in his airway and we saw all the nurses and doctors rush in, we knew how serious this is,”

The Senate Appropriations Committee Approved Version of the Fiscal Year 2012 Labor- HHS-Education Appropriations Bill Includes Two Pieces on Spinal Muscular Atrophy

says Omar. “Everything they were doing to him, we needed to be able to do at home. It scared me.” Omar Jr., 10 months, was diagnosed with Spinal Muscular Atrophy Type I at six months and spent three months at CHOP last year after he had a crisis near his home in the Poconos. The diagnosis means his muscles are weak, so his breathing is aided by a bi-level positive airway pressure ventilator, known as BiPAP, and his caregivers need to periodically suction his airway to clear mucus. “We were at his bedside all the time, so we already knew how to do the routine things: suctioning, putting him on BiPAP, giving him coughilator treatments,” Christina says. “But handling an emergency is different.” Simulation was the confidence builder Christina and Omar needed to feel empowered to apply the individual skills they had learned in the heat of the moment of an unexpected event. Omar Jr.’s doctor, Richard Lin, M.D., used a SimBaby medical manikin preprogrammed to go through a medical emergency. The parents needed to determine the problem and address it. “At first it was surreal — sort of like playing house,” Omar says. “But then it got intense. We were sweating.” Lin didn’t coach or interfere during the simulation, but reviewed what he observed they did right and wrong afterward. Christina says, “He studied our movements and communication and suggested ways to do things better.” Parents of Spinal Muscular Atrophy patients at CHOP go through simulation training for emergencies, such as a drop in the child’s oxygen level or an equipment failure, as many times as they need to feel comfortable they’ll be able to help their child through a crisis once they’re home. “It’s one thing to ask a parent, ‘If this happens, what will you do?’” says Lin. “It’s another thing to have a crisis and the parent has to figure out what’s going on and decide what to do.” The Hardy family is ready. “We may face serious situations and we don’t want to panic,” Omar says. “The simulations definitely raised our confidence.”

SMA Report Language: NeuroNEXT SMA Biomarker Validation Study

*Fiscal Year 2012 Appropriations for the
Departments of Labor, Health & Human
Services, and Education, and Related
Agencies (S. 1599)*

Senate Report 112-84

Department of Health & Human Services

National Institutes of Health

National Institute on Neurological Disorders and Stroke

Network of Excellence in Neuroscience Clinical Trials. – The Committee commends NINDS for its leadership in creating the Network of Excellence in Neuroscience Clinical Trials [NEXT] program, which will create a robust, standardized and accessible infrastructure to facilitate rapid development and implementation of protocols in neurological disorders affecting adult and/or pediatric populations. NINDS has indicated that the first project to utilize the new NEXT infrastructure will be a biomarker validation study of Spinal Muscular Atrophy [SMA]. Identifying one or more biomarkers for SMA would represent a significant step towards accelerating efforts to create effective treatments for this disease. The Committee also urges NINDS to continue to demonstrate strong support for translational research on SMA that will accelerate the development of therapies for testing in the clinic and facilitate the submittal of investigational new drug applications to the Food and Drug Administration. The Committee requests an update in the fiscal year 2013 congressional budget justification on the specific goals for the NEXT SMA initiative and its efforts relative to translational research on SMA.

SMA Report Language: SMA Newborn Screening

*Fiscal Year 2012 Appropriations for the
Departments of Labor, Health & Human
Services, and Education, and Related
Agencies (S. 1599)*

Senate Report 112-84

Department of Health & Human Services

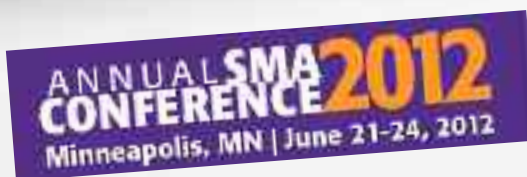
National Institutes of Health

*National Institute on Child Health &
Human Development*

SMA Newborn Screening. – The Committee applauds NICHD for funding a pilot study of newborn screening for Spinal Muscular Atrophy [SMA] that will confirm the efficacy and accuracy of the SMA screening technology. The Committee understands that newborn screening holds promise for assisting with early interventions of SMA and developing improved and more standardized care protocols for patients living with SMA, and it may also assist in the development of potential drug therapies. Natural history, preclinical and preliminary clinical data all suggest that potential therapies will demonstrate the greatest effectiveness when delivered presymptomatically. The Committee encourages NICHD to support the development of crucial follow-up care protocols for children identified with SMA through the pilot study, and to report on progress made in this area in the fiscal year 2013 budget justification.

Families of Spinal Muscular Atrophy Announces Schedule for The 2012 Annual SMA Conference

Families of SMA releases the agenda for the 2012 Annual SMA Conference, which will be held at the DoubleTree Hotel Bloomington – Minneapolis South. The schedule includes the timeline of workshops and events, including the Family Fun Fest / Carnival, the PJ Party and the new SMA Family Dance Party.



2012 Annual SMA Conference

Wednesday, June 20th

3:00pm – 9:00pm Registration Open for all Attendees

Thursday, June 21st

7:30am – 5:30pm Registration Open for all Conference Attendees
 12:00pm – 4:00pm Newly Diagnosed Program – Program Only for Newly Diagnosed Families
 5:30pm – 8:30pm Meet & Greet, includes Family Fun Fest / Carnival

Friday, June 22nd

8:00am – 5:00pm Registration/ Merchandise Sales Open for all Conference Attendees
 8:00am – 5:00pm Vendor Tables Open
 9:00am – 10:30am General Session Opening Remarks
 10:45am – 12:15pm Workshop Session #1
 12:15pm – 1:30pm Lunch (on your own)
 1:30pm – 3:00pm Workshop Session #2
 3:15pm – 4:45pm Workshop Session #3
 6:00pm – 9:00pm Families of SMA Family Dance Party

Saturday, June 23rd

8:00am – 4:45pm Registration / Merchandise Sales Open for all Conference Attendees
 8:00am – 5:15pm Vendor Tables Open
 9:00am – 10:25am General Session
 10:40am – 12:15pm Workshop Session #4
 12:15pm – 1:30pm Lunch (on your own)
 1:30pm – 3:00pm Workshop Session #5
 3:15pm – 4:45pm Workshop Session #6
 6:15pm – 8:15pm PJ Party

Sunday, June 24th

8:00am – 9:00am Full Breakfast
 9:15am – 11:30am Closing General Session/Researcher Q & A

To see a more detailed workshop agenda, please visit www.curesma.org.



Everyone at Families of SMA is looking forward to the 2012 Annual SMA Conference in Minnesota!

June 21st - June 24th, at the
DoubleTree Hotel Bloomington – Minneapolis South

For more information please visit,
<http://www.fsma.org/FSMACommunity/Conference/>

Congratulations to Families of SMA staff members Jessica (Banas) Clark and Sarah (Hunsicker) Rodriguez on their recent weddings!



Sarah and Stephen Rodriguez Jr.



Jessica and Brian Clark

Congratulations



■ Congratulations to Allison and Bill Wehrkamp on the birth of their new baby girl, Piper Violet Wehrkamp, born on November 29th, 2011!

■ Congratulations to Hillary, Keith and big sister, Avery Schind on the birth of their twin boys, Braxton and Brennen!

■ Congratulations to Carly Hewitt, who was crowned homecoming queen at her high school's homecoming football game on October 14, 2011! Carly is a 17 year old senior with SMA Type I.



Shaun Lesniak, son of Lynne Barrelle and Jeff Lesniak of Weston, CT, is one of 13 Weston High School seniors to be commended by the National Merit Scholarship Program based on the PSAT/National Merit Scholarship Qualifying Test taken in his junior year. More than 1.5 million students from across the nation entered the competition by taking the test and the

Weston HS winners were selected based on top (3%) performance in Connecticut. Shaun, who has SMA Type II, took the SATs this year and scored even higher. Congratulations Shaun on such a wonderful accomplishment!



Madison Wolff, SMA Type II, of Phoenix, AZ was featured on a billboard for wheelchair vans!



Braelyn Campbell



Carolyn Barrett



Ella Jayne Ecker



Connor Murphy



Easton King



Carlee Beam



Ford Snizek



Grace Nelson



Dylan and Heather Cuevas



Eleanor Bolton



Andrew and Patrick Murray



Ella Ecker



Haley Kuester



Hannah Hayes



Garrett Schulz



Jack Bolton



Jadon Burks



Emma Rubenstein



Eva Kelly



Gwendolyn Strong



Gray and Riley Dougherty



Jamie and Troy Lino



Jenna Boguhn



Jordan, Max and Sam Rappoport



Ethan and Ray Fantel



Lexus Stipanovich



Lucas and Lauren Regan



Josh and Daniel Tramontano



Lizzy Hallam



Katey Stare



Lucy Butler



Journee Marie Weiberg



Josh and Jacob Garrison



Journee and Josiah Weiberg



Melanie Lee



Robby and Danny McHale



Janos Williams Tercs

Update on the Spinal Muscular Atrophy Drug Development Pipeline from Families of SMA

Developing new therapies to treat and ultimately cure SMA is the driving force behind Families of SMA. To achieve this goal, Families of SMA aggressively invests in drug development research, funding the most successful and innovative scientists and organizations.

Several key details demonstrate the great progress being made:

- 1) Programs are advancing into the clinical trial stage of testing. There are now three novel programs actively being testing in clinical trials for SMA.
- 2) There is a broad diversity of approaches in development. This breadth is important in case one particular approach to treatment turns out not to have benefit in patients.
- 3) The total number of programs is growing. There are now 13 novel SMA therapeutic programs in various stages of preclinical and clinical research, up from just two, ten years ago. This volume is necessary to balance the low odds of only 1 in 10 programs successfully advancing through clinical trials to final FDA approval.
- 4) Funding and resources from industry are increasing. There are now nine companies actively investing in the SMA drug pipeline which is essential to advance programs through the expensive and complicated later clinical stages.

Clinical Programs

Our community has been successful over recent years in advancing basic research discoveries from the bench to the clinic, with three novel programs now testing drugs in human clinical trials. These include:

- Trophos with Olesxime currently in Phase II clinical trials.

- Repligen Corporation with Quinazoline currently in Phase I clinical trials.

- Isis Pharmaceuticals and Biogen Idec with Antisense currently in Phase I clinical trials.

New Increased Funding

Families of SMA invests in broad research initiatives. A major goal at FSMA has been to help build the SMA drug pipeline. Even with the community's current progress, FSMA believes it is critical to continue to grow the SMA drug pipeline. Statistics show that only 10% of all drugs initiating human clinical trials ultimately receive FDA approval. FSMA has been investing in and advancing novel pre-clinical drug research since 2000. The goal for our drug discovery funding is to build a diverse therapeutic pipeline to maximize the chances for success in finding a treatment for SMA.

FSMA will announce new funding for drug discovery programs in the coming months. This new funding is significant as FSMA will now begin to fund several programs concurrently which have different approaches to developing a therapy for SMA. This will both enhance our overall chances of success and also accelerate the timeline to approve SMA drugs. With this new funding, FSMA will have been involved in funding half of all the ongoing novel drug programs for SMA.

Partnering with Companies and Government

Drug development in SMA has seen advances because of collaborative efforts between academic, government, pharmaceutical and non-profit organizations. These collaborations are essential for effective drug development in orphan diseases.



FSMA is proud to have led the way in collaborations for many of the current programs in the SMA drug pipeline, including:

- Licensing the Quinazoline program to Repligen Corporation. Which was the first new drug designed for SMA to receive approval to start clinical trials.
- Licensing the Antisense program to Isis Pharmaceuticals. This program received FDA approval to begin clinical trials for SMA in December 2011.
- Obtaining NINDS grant award support for Paratek Pharmaceuticals with potential funding of \$5 Million.

Families of SMA is attacking the disease from every angle possible, and there are now more drug programs in development than at any other time. FSMA is the leading active funder of SMA drug development research in the world.

Additional Drug Pipeline Notes:

- The companies now involved in SMA drug programs include: Trophos, Repligen Corporation, Isis Pharmaceuticals, Biogen Idec, PTC Therapeutics, Roche, Novartis, California Stem Cell, and Paratek Pharmaceuticals.
- Four SMA drug development programs are no longer included in the current pipeline due to the programs being cancelled for either scientific or financial reasons.

Two Families of Spinal Muscular Atrophy Research Award Recipients Lead New Neuromuscular Clinic for SMA

Christine DiDonato, PhD and Kristin Krosschell, PT/MA along with Nancy Kuntz, MD from Children's Memorial Hospital in Chicago, IL seek to improve the quality of life for children with SMA.

This comprehensive neuromuscular clinic located inside Children's Memorial Hospital in Chicago has gone from one clinic per month to two due to the need to serve the Midwest SMA community. Drs. DiDonato and Kuntz and also Kristin Krosschell, PT/MA will focus not only on treating the symptoms of the disease but also educating the public about SMA with an emphasis on reaching out to a child's daily contacts including school personnel and primary care physicians.



The full article of *"In Touch With Research At Children's Memorial Research Center,"* can be found in the Winter 2011 newsletter.

Families of SMA has funded various research projects involving Dr. Christine DiDonato and Kristin Krosschell and has a long-standing history with Northwestern University's Feinberg School of Medicine. Since 2004, Families of SMA has awarded \$750,000 for Clinical and Drug Discovery programs including a section of the Quinazoline project.

View the Spring 2011 edition of *"Compass"* which discusses Ms. Krosschell's FSMA funded project titled, *"Reliability of the Modified Hammersmith Functional Motor Scale in Young Children with SMA."* This project was completed by the Project Cure SMA clinical trial network.

Families of SMA has awarded funding to Dr. DiDonato to develop new mouse models of SMA to be used in drug testing, including mild and intermediate models. She has used her new models to test a number of SMA drug candidates, including the Quinazoline Compound RG3039.

Read more about new FSMA basic research projects in the Fall 2011 edition of *"Compass"*.

SHARING PHOTOS



Lucas and Lauren Regan



Tambyn Campbell



Lizzy Hallam



Cole Geraghty

Nora's Special Birthday

By: TJ and Jamie Gooden

For a SMA Type I child, turning three is nothing short of amazing! To mark this huge milestone, we wanted Nora to have an extra special party. I have always been fascinated by the American Girl Phenomenon. We often get the catalogues in the mail and Nora and I page through them admiring all the beautiful dolls and accessories. Despite the fact that Nora is a little young, we thought it would be a great experience to visit the American Girl store and have a birthday celebration with a hand selected doll of her own. So, after a few phone calls, I was put in contact with the store Manager in Atlanta Georgia. She was so kind and accommodating and decided to open the Store and Bistro early for us to limit our exposure to large crowds and germs.

When we arrived at the American Girl store, we were greeted by our very own personal shopper "Andrew" who was so kind and patient. He described each doll to us and humoured us along the way. We went straight for the baby American Girl dolls but Nora insisted she wanted a big girl doll, so the search was on. After looking at almost every variety of dolls they offer (which is a HUGE selection), Nora picked out a doll that looked just like her; red hair and freckles. That of course started the outfit shopping. We thought the doll selection was huge—hah. We wanted to get Nora something else too. Heck she only turns three once! Convincing her was really easy, so she got to pick out a Bitty Baby doll as well.

After the shopping was over, we were escorted to a private back room to start the party and I had to do the credit card thing. Now, if anyone knows about these dolls, they know that most of them are upwards of \$100 each. Somehow our purchase totalled up to not nearly what I expected. As I stood

there puzzled, Andrew explained that they had picked up the cost of Nora's big doll. I was nearly moved to tears at their generosity. It wasn't about the money. We had expected to pay whatever it cost, it was more that I realized they understood our situation and were doing their part to make things better...at least for that day.

Still shaking my head in disbelief, I joined the party. The party room was wonderful and large with beautiful carpet, furniture and pink everywhere. There was also a bouquet of flowers and a goodie bag for Nora. We had a delicious brunch while Nora entertained us with a beautiful rendition of "This old Man". Once the cake arrived, we sang happy birthday with the entire American Girl staff and Nora blew out her own candles one-at-a-time.

When we arrived at home later that afternoon, we noticed there were more doll outfit boxes than we actually purchased. So even after all they did for us, the American Girl staff still surprised Nora with more gifts.

Overall Nora's birthday was exactly what we wished it would be—memorable! It was so unique and fun with so many moments we can look back on and smile. This birthday was extremely emotional for us, especially in the days leading up to it. We never dreamed we'd have the chance to see our little girl turn three. We are incredibly thankful to the American Girl staff and everyone who helped make this birthday extra special. It was truly a priceless gift.

TJ and Jamie Gooden of Knoxville, TN

Special thanks to Shutter Sweet Photography





The Cottor Family



The Villarreal Family



The Landre Family



The McHale Family



The Gooden Family



The Murray Family



The Norton Family



The Moyer Family



The Dindzans Family



The Meigs Family



The Saxton Family



The Strong Family



The Bolton Family



The Tarrence Family



The Williams Family



The Manfre Family



The Cooper Family



The Merulla-Bonn Family



The Andrade Family



The Armbrustmacher Family



The Anton-Jensen Family



The Stare Family



The Murphy Family



The Leiter Family



The Burks Family



The Hoffmann Family



The Tomko Family



The Campbell Family

Our Cross Country Road Trip

By: The Pillarella Family of New York

My name is Lisa Pillarella, and this past summer, my husband, John, and three daughters, Eloise (16), Samantha (12) and Jamilla (11), drove across country, from New York City to San Diego and back. This took about 30 days and was the trip of a lifetime. Our oldest daughter, Eloise, has SMA Type II. She is a junior in the International Baccalaureate (I.B.) program at Curtis High School on Staten Island, and has been studying art for most of her life, including summer and weekend programs at the Art Lab Summer Scholarship Program on Staten Island, and the Art Director's Club in Manhattan. As part of her public service for her I.B. diploma, she tutors 1st graders at a local elementary school twice a week, and is presently enrolled at a local college in Psychology, for which she will receive college credit. Eloise has used only a power wheelchair for mobility since she was 3, and has used a bipap at night and inextufflator as needed since she was about 5. She had a few week long hospitalizations when she was very young, and had collapsed lungs, was intubated for two weeks and was hospitalized for a month when she was six. She has not had (knock on wood) any hospitalizations since.

Because we had already driven across country once before (in 2003, before we adopted Eloise's younger sisters from New York City foster care), we knew where we hadn't yet been as a family and really wanted to go there. In 2003 we headed from NYC to Chicago (where I grew up), and then headed directly to Ft. Collins and the Rocky Mountain National Park, on to Moab, Arches, the Grand Canyon, Phoenix, L.A., up the California coast to Seattle, across Wyoming and Montana to Mt. Rushmore, on up to Minneapolis, back down to Chicago and home. This time, we decided to take the southern route.

We drove from NYC to the Great Smoky Mountains in North Carolina. There is a lovely trail up to the wackiest, futuristic curved ramp that goes up above the tree tops. It was made for power wheel chairs! The misty mountains truly appear "smoky" too. We then headed south toward the panhandle of Florida, but stopped first in Atlanta to see Dr. Martin Luther King's grave. It lies in a serene reflecting pool with a museum attached, and the Ebenezer Baptist Church is



close by, where you can go in and actually sit in the pews where his congregation once did.

Once in the Panhandle, we stayed in Panama City Beach, Florida. The state run beach has a protected inlet where Eloise could swim in the ocean with a donut ring, it was so gentle, and we even saw sea lions! From the hotel, we took a boat tour out to an uninhabited island, which also had beautiful, gentle surf. We took Eloise on the boat in her "Convaid Cruiser", which we take on every trip for "inaccessible moments". While we were taking the boat back to our hotel, dolphins began poking their heads out of the water, right next to the boat, so we lifted Eloise out of her chair and onto my lap at the edge of the boat so she could be closer to them. It was truly awesome.

From Panama City Beach, we traveled on to New Orleans, Louisiana, a town which we love, above all else, for its food. We made a resolution on this trip that we would only stop at a chain fast food joint under duress, and tried to make sure that the major meal of the day was either featured on the show "Diners, Drive-ins and Dives (DDD)", on the website "Road Food" or had great reviews on Yelp. The stop didn't have to be fancy, just well reviewed, and some holes in the wall are quite well reviewed in our experience. The kids loved the ambiance of the French Quarter, of course, although the cobblestones and historical nature of the place makes for enough inaccessible moments, that one day we just gave up and toured the Quarter in the Cruiser. One of the coolest things to do is a Cemetery Tour, and we chose a daytime one in the St. Louis Cemetery with a local cemetery preservation organization. The heat was crazy, but if you ducked into the shade of a mausoleum while the guide talked, it was bearable, and that guide was thorough. The adjoining museum is also interesting. LaFayette Cemetery is harder to find open, but the streetcar trip out to that neighborhood is wonderful (again, we needed the Cruiser). We couldn't leave New Orleans

Cont. on next page.

Our Cross Country Road Trip Cont.



without taking an alligator swamp tour, so we found one about 20 miles outside of town. During the tour, aside from seeing gazillions of alligators, each kid got to hold a baby alligator. The company owns the bayou on which the tour happens, so it's protected and beautiful. On the way back, we stopped at a DDD place that has unbelievably delicious turtle soup, among other things, and leaving town the next day, we had to stop at Surrey's, a DDD breakfast place that will always go down in Pillarella trip history: it won the "Overall Best Food Item as Decided by Everyone in the Car" Award, which was the Bananas Foster French Toast. I don't know if I've ever eaten anything more delicious in my life.

The next stop was Austin, TX, where our favorite attraction is the natural spring in the middle of town. They have a beautiful, rustic swimming area developed around it, including a super springy diving board into the deepest part of the spring. You slide yourself out of the water onto smooth rocks, and since Austin was having one of its hottest summers on record in 2011, the year round cool spring water was delightful. That's a good thing, because our car broke down there and we ended up at that spring for two days. There is also wonderful food in Austin, including Maria's, an extremely charming taco spot, and BBQ to your heart's delight. However, you need to get out of Austin, about 45 miles, to the town of Lockhart where there is a bizarre town-wide BBQ industry/rivalry going on. It just looks like an adorable western town, but there are three incredible BBQ places, Black's, Kreuz's and Smitty's, each with their own twist on the southwestern BBQ. You feel like you're on a ranch and you can walk right next to the flaming BBQ pits as you wait for your food.



Because of the longer layover in Austin, we felt that the Grand Canyon would just be too rushed, so we looked around for another natural wonder that would be more on the way to Phoenix, Arizona. We discovered that the Carlsbad Caverns would only take us about 4 hours (roundtrip) off of our beaten path, and were we glad that this accidentally came up. The Caverns are an extensive, **WHEELCHAIR ACCESSIBLE** set of caves about 800 feet underground in the southern tip of New Mexico. After taking the elevator down into the Caverns, you enter a wonderland of stalagmites and stalactites as you wind around them on a paved path for about a mile. The caverns are lit just enough for you to see the outrageous rock/crystal formations, so it's very spooky, and although they say the bats are long gone, there is still evidence of their old guano.

We continued on to Phoenix, which is also full of great food, especially Mexican, where we stayed with one of my best friends and her family. It's so nice after hotels to just stay in a house, and they have a beautiful guest house behind their own, so it was especially luxurious. In their yard they grow every type of fruit and vegetable, and they took us on a nighttime Phoenix botanical garden flashlight tour, where we saw every type of cactus, so we had quite the exposure to the flora of the southwest.

We continued on to San Diego, and made sure that we saw the S.D. Zoo and SeaWorld, both of them big hits with my youngest children, and of course totally accessible. Our hotel was right on the coast and we took a hike up a path used by hang gliders which was accessible to a point, so that Eloise could sit on the edge of a cliff overlooking the beautiful Pacific surf. Diners, Drive-ins & Dives spends

a lot of time in S.D., so we ate some fabulous seafood and Mexican here as well. We moved on to Las Vegas, which was not our favorite stop, although it was over 100 degrees Fahrenheit, and we don't gamble, so I guess that's not really fair to Las Vegas. We were excited to get back out on the road because we wanted to see three parks on the way to Ft. Collins, CO.: Zion, Bryce Canyon and Arches.

Zion National Park was wonderful because it is a canyon through which a power wheelchair accessible bus shuttles. It then drops you off at an unbelievably beautiful and yet also power wheelchair accessible trail, on which you can hike for about a mile. I just could not believe that the surroundings appeared so rugged, yet we were able to negotiate the trail in her chair. We stayed at a beautiful hotel right at the edge of the park that felt like a lodge, and then continued on the next day to Bryce Canyon. Bryce is famous for its "hoodoos" which are rock formations that look like weird, gigantic, contoured columns from the action of wind and water that has worn them away over the centuries. The rock is a stunning rosy orange, and the canyon is enormous. Again, a big wide crushed rock trail led straight down into it, however the steepness and lack of any sort of railing, fence, etc. on the most dangerous side of the trail held us back a bit here. Eloise went down into the canyon for a bit and hung out, but we were not successful in finding a fully accessible trail that got us very far. Still, once you're down into it even a few hundred feet, you really feel surrounded by something awesome.

By the time we left Bryce for Arches, we did not really have the time to search for accessibility here, although "Balanced Rock" (which looks like it sounds) is amazing and Eloise could get right up to it in her chair. By far the most famous sight here is the "Delicate Arch", which it seems has only one inaccessible trail to it. Several years ago, we carried Eloise in her cruiser to it, but I'm not sure that would go so easily this time, and so she looked at that one from afar.

From these parks, we continued on to Ft. Collins, CO to visit friends. There happened to be a great outdoor music festival. This is such a good stop if you are doing the cross country trip because it's a cute college town that's conveniently located about an hour from Rocky Mountain National Park, a must

see. After the orange rocky terrain that we had just left, the classic pines and mountain lakes of the Rockies were stunning. About an hour outside Ft. Collins, you can drive through Estes Park straight to the top of the Rockies. It's absolutely incredible how high you can get so quickly, and the beautiful, accessible hikes you can take on the way. I believe they are marked on the map that the rangers give you. Unfortunately, I can't remember all of the exact trail names. At the first stop up to the top, you could walk about $\frac{3}{4}$ of a mile on an occasionally bumpy, but accessible trail around a pristine, mountain lake. As we drove a little further, (and higher in elevation, causing some headaches that were well worth it) we could see other places to pull over, but there was another accessible hike along the drive that actually led to an accessible "back country" campsite. There was a picnic table elevated for easy access by wheelchairs, and the entire site was flat and traversable by wheelchair.

We continued on to the very top of the drive, past stunning glaciers, to the visitors' center. Just before this was a trail onto the tundra at the top of the mountain that was not exactly power chair friendly, but once we found a way to get her chair from the parking lot to the



asphalt trail, she was able to do the entire mile and a half round trip hike in her power chair. I don't believe this hike was marked as accessible, and it certainly looked completely inaccessible from the parking lot, so always jump out and check it out yourself. We're so glad we did, because it was one of the best hikes of the entire trip. Incidentally, we were prepared to use her "Convaaid Cruiser" which we always bring along in case there is an inaccessible location that we simply must see. (In Costa Rica, we actually carried Eloise in this same cruiser over a hanging bridge in the rainforest...yikes!) About a half a mile out onto the tundra (via this asphalt path), we started spotting marmots, and we were surrounded by glaciers and windswept terrain the whole way. You have to be careful though, up there. The wind picked up so quickly, that by the time we turned around and ran back to the car, we couldn't feel our hands and Eloise was an icicle. I actually had to operate her joystick to get her back down the trail. It was a little scary, but then again, most adventures are. On the way back to Ft. Collins we naturally had to stop at one of the numerous "rock shops" and buy some geodes to remember our trip.

From Ft. Collins, we high-tailed it back to Chicago as quickly as we could, although we were very tempted to spend a day at the Iowa State Fair. It rained, which helped make up our minds to move on. It was exciting to cross back over the "Mighty Mississippi" again, weeks after the first crossing. We stayed with my family in Chicago as long as we could, but

had to return for Eloise's MDA Camp and the wedding of other MDA friends, Holly Verdile and Linda Tirado.

What a trip, to say the least. We were so heartened by what seemed to be the increased accessibility of our country (or at least our national parks) since our last road trip 8 years earlier, and we were once again floored by the immensity and beauty of our country as well. Our children impressed us too. They were as into the adventure of it all as much as we were, and with the constant change of scenery, boredom wasn't really an issue. I should note that we do not have a TV in our car, and did not bring along anything to watch DVDs while travelling. It was all about listening to music or books on tape and enjoying each other's company, which we did, for the most part. Even the seemingly big problems we encountered didn't cause any meltdowns, mainly because the kids really helped out. For example, smack dab in the middle of the desert, the back door of our van broke, and every time we had to load Eloise out or in, one of her sisters had to stay inside and cam strap it shut, or get the straps undone. Adventures can be a pain in the neck, too. We encourage you to take one anyway, and if you have any questions, we'd be glad to tell you what we know. Have fun!

Medical Advisory Council Meeting Held at the FSMA National Office

The Families of SMA Medical Advisory Council hosted another day of successful and productive meetings at the FSMA National Office near Chicago, February 17th and 18th. The Medical Advisory 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. This latest meeting covered important SMA medical care topics including:

- The 2012 Annual SMA Conference workshops, based on feedback and surveys from the previous year
- The agenda for the Newly Diagnosed Program and the 2012 Annual SMA Conference
- The new program for medical providers, Continuing Medical Education prior to the start of the Annual SMA Conference
- Legislative activities and funding that could impact care for SMA Patients
- Topics for new SMA Care Series Booklets

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 in issues that improve the quality of medical care for those affected by SMA.

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; Diet and Nutrition; Respiratory Therapy; Nursing.



Would you like your primary care doctor or other medical professional to receive information on SMA? If so, please e-mail memberships@fsma.org with their contact information.

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

Nathan Edward Russell

August 3, 2003 – April 28, 2010



On Sunday November 6, 2011, I crossed the finish line of the 2011 ING New York City Marathon culminating two years of effort. When I signed up for the marathon entry lottery in November 2009, I had just watched Meb Keflezighi win the New York City Marathon; the first American to do so since 1982. Inspired, I registered the next day for the 2010 race and didn't even mention it to my wife, Jennifer. The chances of getting selected were pretty slim...10%...so why bother? I probably wasn't going to get picked anyway.

Fast forward to early April 2010...I was at work when I received an e-mail from the New York Road Runners Association reminding me that the lottery was going to be held in a couple of days. Sure enough, a few days later I received a congratulatory e-mail informing me that I had been selected for the 2010 marathon. I was stunned; Jennifer was even more surprised when I called her and said "Guess what, honey, I got picked to run the NYC Marathon this November". Little did we know how much our lives were about to change.

Nathan Edward Russell was born on August 3, 2003, and diagnosed on December 31, 2003, with SMA Type I. We decided to take a very proactive approach with his care and because he was a tough little boy, he proved the diagnosing neurologist wrong by celebrating his second birthday, then his third, fourth, fifth and sixth birthdays. On Easter Weekend 2010, we could tell that Nate didn't feel good but attributed it to allergies since he'd been outside driving his power chair. Over the next several weeks, he was up and down. He seemed to improve the third week of April but on Sunday, April 25, he started having problems again. Something was different this time; no one could seem to figure out what was going on. The next three days were a blur of worry, stress, sleeplessness, de-satting, coughing, etc. After another horrible night, we transported Nate to the PICU on Wednesday morning. At 9:45PM that evening, April 28, 2010, Nate passed away. He had fought SMA and its' effects on his respiratory system until the very end. He died with Jennifer and me at his side reminding him that he was greatly loved and an inspiration to many.

Our world was shattered. For the last six and a half years, our lives had revolved around Nate and now he was gone. To cope with the grief, I ratcheted up my running; nothing crazy just more consistent running. Near the end of June, I injured my right knee, a partial tear in the meniscus was the official diagnosis. My plan to run the 2010 NYC Marathon was over. Luckily, I was able to defer my entry to the 2011 marathon so I had arthroscopic surgery in December 2010. Although I was able to resume running in

January 2011, I really wasn't sure if I was going to be able to run the marathon. Was my knee going to hold up to the required mileage that goes with marathon training? After a couple of months of soreness and mediocre running, I made a key decision. I would "run my knee into submission". My training plan would be to run every other day so my knee would get accustomed to the pounding. This probably isn't the best training plan but it worked for me (and is still working today).

Now that my training was back on schedule, my focus was directed to honoring Nate and all of the other SMA kids. These kids battle SMA with everything that they have for every minute of every day. The "punishment" of running 26.2 miles pales in comparison to what they go through. No matter how bad things got or how difficult a day Nate was having, he always had a smile. When I became a dad, I planned to teach Nate so many things. In reality, he was the one that taught me the really important "life lessons".

The marathon was run on a beautiful day, clear, blue skies and cool temperatures. Wearing a bright yellow CoolMax shirt in honor of Nate (he loved SpongeBob Squarepants), I ran through the five boroughs of New York City. Nate was with me the entire way. During the rough patches, all I had to do was think about him and his battle against SMA then my aches and pains seemed pretty meaningless. Along Central Park South about 800 yards from the finish line, I saw Jennifer and my cousin, Monica. Needless to say, it was a very emotional moment as we hugged and kissed. After a few minutes with them, I moved on and finished up the rest of the race.

As I look back on this whole endeavor, it still amazes me on how it all played out. I could not have done this without support, patience and love from Jennifer and Nate's little brother, Owen. I am also extremely grateful to our family and friends for their support and generosity. Their support truly proves that Nate was a very special soul who had a profound effect on people. But most of all, a big thank you goes to Nate. You were a true angel on loan and I am honored to be your dad.

Thanks,
Trey Russell, dad to SMA Angel, Nathan Edward Russell





IN MEMORIAM
AUBREY GRACE LYDEN
JULY 4, 2010 - APRIL 24, 2011



IN MEMORIAM
ETHAN JAMES CARTER
March 29, 2009 - October 14, 2009



IN MEMORIAM
CHLOE BUSH
SEPTEMBER 3, 2006 - DECEMBER 18, 2011



IN MEMORIAM
KYLAH SCHULZ
APRIL 14, 2011 - AUGUST 5, 2011



IN MEMORIAM
SAMANTHA UTZAT
September 16, 2006 - September 14, 2007



IN MEMORIAM
DREW PLOTKE
JUNE 9, 2005 - DECEMBER 27, 2011



IN MEMORIAM
MATTHEW JAMES TROLAND, JR.
January 5, 2007 - June 27, 2007



IN MEMORIAM
**PORHILDUR THORHILDUR
NOTT MYRDAL**

IN MEMORIAM

Milania Faith Aguilar

June 7, 2011 – December 16, 2011

This is Milania.

We call her Millie Bug and she was born June 7, 2011. Our bug was a healthy 7lbs 15oz! When Millie was two months old she started having breathing problems; the doctors told us Millie had a “weak” wind pipe, but we knew it was something much deeper than that. We also realized Milania was not able to hold her head up or move around like other babies her age. On October 24th, we decided to take Milania to Texas Children’s Hospital because her breathing and eating were continuously becoming worse. Once we arrived we were told we had to stay and have tests run on our Millie Bug. Our stay went from being overnight to a week and a half. On November 4th our Millie Bug was diagnosed with SMA Type I. Spinal Muscular Atrophy is a motor neuron disease; the motor neurons affect the voluntary muscles that are used for activities such as crawling, walking, head and neck control, and swallowing. It is a relatively “rare disorder” where approximately 1 in 6000 babies born are affected, and about 1 in 40 people are genetic carriers. The doctors gave us our options: surgery or to take her home, comfort her and spend all of our time with her, as this disease progresses extremely fast. One night, in early December I realized Milania’s oxygen level was low and her heart rate was high so we took her to Texas Children’s ER, which led to another hospital stay. On December 9th Milania stopped breathing, but the doctors were able to help her immediately! On December 12th, our lives completely changed when Milania stopped breathing yet again and the doctors had to intubate our Millie. On December 16, 2011 Milania lost her fight with SMA. Milania has deeply touched thousands; she has changed lives and her memory lives on forever. She will truly and deeply be missed! We love you and miss you our sweet Millie Bug, we will see you again one sweet day, to the moon and back. Mommy & Daddy

Team Millie Bug of Baytown, TX



IN MEMORIAM

Skylar Marie Jones
November 19, 2009 – August 16, 2011
Life Without Skylar Marie Jones.

We held our sweet girl as she took her last breaths on August 16, 2011 when she was three days shy of 21 months old. It was a day I dreaded, like every parent knowing their child is terminally ill and not going to outlive them. I can't even describe the pain and the ache my heart felt as she left this world, and how my heart still hurts as she is missing from my life. I am comforted that my husband and I were both present with her when she passed away, that she was as pain-free as she possibly could have been and that she knew she was loved every second of her life through the end.

We all have similar stories; our children are beautiful, bright and full of life. They change the world in ways we never dreamed. Unfortunately, without a cure, we will all have similar endings. I pray this is not the case. I know we don't live forever on this earth, but my desire is that we could have full and happy lives with those we love. There are truths that I have had to come to terms with... Our world is broken - full of death, disease and suffering. Life is not fair. I am not in control. My daughter is dead.

I want to talk about life without Skylar because I don't think anyone knows how to handle the death of a child. I don't claim to, but I want to encourage other parents through the process. I'm not going to tell you it will be easy or pain-free because it won't. In fact, it's the worst thing that I've ever been through. I do want to tell you that I am still standing. My husband and I still love each other - more today than ever before - and we have hope for the future.

With Skylar's passing, death is very real. We've spent a lot of time thinking and talking about death, life, purpose, religion, etc. With the diagnosis of SMA, my husband and I realized how precious time was, but now even more so. We're much more intentional about how we spend our time and who we spend it with. We've had to come to terms with our grief and know

that just because we are grieving in different directions, doesn't mean our marriage has to go in different directions. We've grown close and are glad to have each other through this process because not many other people can grasp where we are in life. We've tried to make new friends, but when we hang out with other couples, conversation seems trivial and the relationship is shallow. When we hang out with other families, we feel like we connect more, but are out of place without children of our own. Relationships with families can be awkward and no one knows what to say. My husband and I don't have any other children and plan to adopt in the future when our hearts are ready, but for now, we're out of place as parents without their child.

I tell you all of this because grief is so personal and individual. None of us were meant to carry this heavy burden. It's not natural to lose your child. For all of the parents reading this, my hope is that you will be honest with yourself and the ones you love. I hope that you will take care of yourself and give yourself grace through this process. I hope that you learn how to set boundaries in your life to protect you from people who bring you down and grow the relationships that are life-giving. My husband and I would not be where we are today if it had not been for the amazing community of support we have in our lives through our church, our neighborhood and our friends.

Life without Skylar is hard. I spent one and a half years as a full time care-giver and mom and now am back in the working world starting my own companies. Yes, I am starting two different companies because one isn't hard enough :) I've somehow managed to keep my sense of humor through all of this which has been life-saving at times. Being back



in the "real world" is a culture shock. Most people are oblivious. I'm still grieving, but I know that life goes on and everyone who meets me doesn't have a clue what I've been through. I try to have patience and focus on the positive without being fake. If I'm having a downer of a day, then I do my best to avoid people so I don't have to deal with stupid people and stupid comments. You all know what I mean.

I am so thankful for the pictures, video and memories I have of my sweet girl. We had so much fun together and I loved being a mom! She was amazing and taught me so much in the short time that she was here. It is hard to watch videos of her body breathing, making little sounds, smiling and looking at me because I miss her so much and I want her back. It's also hard to watch videos of her choking and needing to be suctioned or coughed because a part of me is glad she's no longer suffering through a broken body. It's hard to think back of her suffering and pain - not even knowing the extent of what she felt. A part of me no longer wants to be in this world because I know how miserable it can be and I want to be with my sweet Skylar. I know I am still here for a reason and wouldn't dream of leaving Kyle behind. We now face fears of losing each other and strive to leave each day as if it were our last just in case it is. When Skylar died, a piece of my heart left with her. I like to think it's in Heaven with her and my hope is that we will be reunited when my body fails too - whenever that may be.

May peace and comfort find all of you where you are.

Ashley Jones of Atlanta, GA

IN MEMORIAM
Alex Devincenzi
 October 22, 2008 – April 12, 2009



Dear Families of Spinal Muscular Atrophy,

I wanted to share with you a photo of Alex's tree that was given to us by the Rocky Mountain Chapter two years ago at the Colorado Cure SMA Walk-n-Roll & Run. With permission from the City forester in our hometown, we planted it right away on his birthday in the park just a block from our house, and it's been growing ever since! We can actually see the tree from our house. When we walk by it we always say to the tree, "Send your roots down and your branches to heaven."

This year for Alex's birthday we finally had a stone made for its dedication. I particularly wanted to share this because we acknowledged FSMA as a way to raise awareness and as thanks from our family for the support.

Melisa Devincenzi and family of Louisville, CO



IN MEMORIAM
Benjamin Zimmerman
 April 27, 2011 – August 28, 2011

Dear Everyone at Families of SMA:

We wish to gratefully thank you for the wonderful care package received from Families of SMA.

Our girls (ages two and five) enjoyed giving Benjamin (SMA Type I) rides in the wagon, and they still often play with it. The music toy is still a comfort to us at bedtime, and it's a favorite thing for the girls to show to visitors. The sheepskin, handmade quilt and burp cloth were useful to us, and now are special keepsakes.

Benjamin really liked to be outdoors, so we were glad he was a summer baby. He was often in his Graco stroller with a reclining back, sometimes taking naps under a shade tree.

He also liked helium balloons, making them bob by lifting his forearm while lying on his back. He liked when we helped him patty-cake, and bath time was a special part of every afternoon while he was doing well, growing and cold-free.

We would certainly not have chosen to have a baby like Benjamin, but blessings in disguise that came with him are something we would not have wanted to miss.

We hope to meet him again someday in heaven where the roses never fade.

**Martin, Lucy, Anita and Klara
 Zimmerman of Colby, WI**



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



IN MEMORIAM
ZACHARY LUCCASEN
 JUNE 21, 2005-
 OCTOBER 12, 2005

IN MEMORIAM

Jack Ryan Bonelli

June 8, 2011 - December 23, 2011



Dear FSMA,

We cannot thank you enough for the support you gave us during this difficult time in our lives. We are so fortunate to have an organization that could provide us with the knowledge and equipment we needed to give Jack the best quality of life. We will forever be grateful for all that you have done.

Love,

Sarah, Chris and angel Jack Bonelli of Phoenix, AZ

Jack Ryan Bonelli



June 8, 2011 - December 23, 2011

To my sweet baby boy

From the moment you entered this world, with your eyes open wide,
There was no greater joy than the one I had inside.
Your tiny tiny fingers wrapped around mine,
And that was everything I could be fine.
As I carried you down the hallway in my arms,
I was then that I knew I would protect you from any harm.
I felt in love with you right from the start,
You and I, we would never be apart.
Bringing you home felt so complete,
I had already known the greatest joy.
I could feel that you knew that I was your mommy.
You looked my face in the first moment you saw me,
I couldn't wait to share this beautiful world with you,
memories to make memories so true.
As the days went on, you grew and grew,
and your eyes they had got even more blue.
It was not with a love something just when I right,
I would cry up and cry all through the night.
A mother's instinct to protect you,
although in this moment, I wasn't sure I could.
The confusion is what a child does not see,
I can love that everything was not as it.
That was the start of this horrible roller coaster ride,
But as a family, we stuck by each other's side.
Your tiny blue eyes were full of love,
I remember the weeks when you were looking up at me
If I saw a smile that was starting back down at you,
I wanted there to know you were angel too.
Only your smile, that was all we had,
Before starting everything, looking at and
One month to think of future plans,
one month to hold these precious little hands.
We still left these same two hands,
But now we had different future plans.
Our life is just normal wonder down,
So world seemed to turn a dark shade of brown.
Hiding outside the window with each pain,
So close we were, and I started to cry.
I knew the night angels were crying too.
I did not want to think that this was all true.
It seems like only yesterday, we were a family of three,
how could you be taken away from me?
You gave us love and life at the start of every day,
you talked with your eyes, and always wanted to play.
We will miss that everything that we will watch you grow,
what would you be like? All these things will be like to know.
You gave us a gift, that we can use this every day,
we will think of you and love you more every single day.
You're still here in our heart, and growing up wherever,
If you could see me now, you would know I love you.



IN MEMORIAM

Andrew Glenn Butler

January 8, 2009 – June 4, 2009

June 4, 2012, marks the third anniversary of the day that our son, Andy Butler, earned his angel's wings. While he is no longer here in our arms, we carry him instead in our hearts, and we see him in the beauty of the world around us...especially in dragonflies.

As dragonflies dance in the skies around us, we are reminded to recognize the joy in the smallest of moments and to more fully appreciate all of life's wonder. They are a sign of hope for our family, and a symbol of Andy's abiding courage, strength, and happiness. When a dragonfly visits, we know that our angel is near, and we find a little more peace.

This poem, which is based on the well-known story of the transformation of a water bug to a dragonfly, was written in Andy's memory by his Nana, Rosaleen Butler.

The Little Dragonfly

A bug beneath a pond one day
 Saw his friends go far away
 Up along a stem they climbed
 Leaving everyone behind
 Then one day up that stem he climbed
 Above the water he did find
 A world of colour and of light
 That sparkled in the bright sunlight
 He fell asleep, then woke to find
 His body changed, but not his mind
 Wings had grown and he could fly
 Back and forth beneath the sky
 I must go back and tell my friends
 This wondrous world waits here for them
 But wings and water don't combine
 So he would have to bide his time
 He flew around this heavenly place
 Then in the pond he saw his face
 Changed from who he used to be
 His friends won't know that it is he
 So he will wait until the day
 Up the stem they make their way
 And they can play and fly so free
 Together for eternity

On his third angel day and every day, we send all of our love to our angel Andy, and we will keep looking for the dragonflies. We hope the next time you see a dragonfly, you will think of him too.

We miss you so much, sweet boy.

Love,

Alan, Audra and Lucy Butler of Land O' Lakes, FL



IN MEMORIAM

Greta Lindboom

March 21, 2002 - November 14, 2011



Greta passed away very unexpectedly in November. She had always surprised us with her strength and resiliency after illness, so neither we nor her doctors expected her to leave us so soon. She led a very active and rich life despite her physical limitations. Greta was an enthusiastic teammate in power wheelchair soccer. She swam in a pool twice a week, participated in the church choir, was an active Girl Scout and had many friends and supporters at school and in the community. Sometimes it seemed that everyone knew Greta.

Her sparkling personality and sharp sense of humor was only partially hidden by a shyness and reserve that made her appear wise beyond her age. Few people knew, or would have expected, her daily medical routine of tubes and suction, Bipap and therapy.

Many did not even recognize that she could barely hold a book, could no longer straighten her legs and would topple over if unsupported. She made it all seem as carefree and happy as any 9-year-old's life should be. Greta will be deeply missed by her family and her many friends.

Dawn and Jon Lindboom of Rochester, NY



IN MEMORIAM

Aiden Scotty Bundy

February 6, 2007- November 9, 2011

From the outside looking in, Aiden's life was a tragedy. He was born with an incurable disease; was left paralyzed, unable to talk or walk and died when he was four and half. But those people do not know how amazing he truly was; that in despite of all the hardships, he had a smile that could light up the room. They never saw how he had a big personality trapped in a little body, they never saw how he could convey his emotions with a flash of the eye and they never got to see how the smallest things could bring him the greatest joy.

Aiden lived a big life despite his short years. He touched everyone that had the pleasure of meeting him. He accomplished more in four and half years than many achieve in a lifetime. Every day was an adventure with him and quite frankly life is boring without him. Aiden was an amazing kid and I miss every facet of our life together. I miss his smile, his laugh and even his attitude. I miss him rolling his eyes at me when I asked him to do something.

Not a day goes by that I don't miss him and I will miss him forever. I choose to celebrate his life rather than mourn all the things that could have been. Aiden was and will always be my amazing one and only biggie.

Scott and Dawn Bundy of Russellville, AL

IN MEMORIAM

Miranda Martin

June 26, 2011 – January 28, 2012

A butterfly lights beside us,
like a sunbeam...
And for a brief moment
it's glory and beauty
belong to the world...
Then it flies on again
and though we wish
it could have stayed...
we feel so blessed to have
seen it!



This is my sister Miranda; just like a butterfly. We feel so blessed to have had her and so heartbroken that she has “flown-on”. She was born June 26th, 2011 and diagnosed at four days old with SMA Type I. Her cord blood was tested, as SMA is no stranger to our family. A sister and brother greet her in Heaven, and my six and a half year old brother Lewis, is fighting a brave fight against SMA Type I! He is our miracle boy and a real fighter!

Miranda was perfect and healthy at birth, but by six weeks, she started to show weakness; what a heartrending time. Her doctor had great hope and put her on amino acid trial formula, to try and stop the rapid cell death. By ten weeks, though, we knew it was not helping, as she was rapidly growing weaker, so it was discontinued and another broken dream, our last hope was gone.

So we tried to cherish everyday with our angel-girly, and give her a good life, full of love as we tried to smile through the tears, again. She loved stroller rides in the warm summertime from big sister, Elaine, to rides on Lewis’ lap in the power-chair as he crooned to her, “We are SMA buddies”, to rough and loving play from big brother, Delmar. Also, she and I loved bath time; in the big tub of water, she was in her glory, smiling and cooing! And of course overflowing love from Mom and Daddy too!

At two and a half months we received the care package from FSMA! So kind to know we have friends who understand and care, and Mandie loved all the toys and the wagon! Her favorite was the light butterfly chime that we hung at her “nest” on the kitchen counter. She played, jangled and talked to that chime everyday, up till the day she “flew – on”; such a light toy and she loved it!

She also loved helium balloons; those got Mandie talking like no other toy would. Her eyes shined as she was able to control this toy ever so gently and carefully.

She had a happy, healthy six months full of love and happiness! The first week of January, she had a touch of pneumonia, but “handled” that with a nebulizer, chest PT, cough assist and antibiotics. In a week she was back to her happy self, however, there was still a rattle in her throat that she could not get rid of.

Just two weeks later Miranda and her big brother Lewis got a serious bronchial cold and were both so sick. They were doing 24-7 treatments, Bi-pap, cough assist, chest PT, antibiotics, nebulizer, draining treatments and more treatments; like all of the SMA patients and families know too well. Eventually, both of them ended up on oxygen. In a few days, our fighter, Lewis, pulled through and started to improve, but our delicate little butterfly, Mandie, was not. It had settled in her bronchial tubes and didn’t let go. By the end of the week, she was getting more oxygen and began regressing and was so miserable.

Then hospice care came, which we really appreciated! She was put on oxycodon and she finally relaxed then, only taking a few drops of Gatorade and choking on her own secretions. SMA was winning and we were heartbroken and so sad; we prayed that she would go peacefully to Jesus. “Fly away like a little butterfly,” her Daddy whispered, as she fought so bravely. With her Mom, Daddy, brothers and sister all circled ‘round her, along with her Grandma and Grandpa Aukey and Aunt Dawn too, she was encircled in love! We felt so helpless and we were all hurting for her. After a long afternoon of rapid breathing and switching breathing patterns, she began slowing down. Our butterfly flew away to Heavenly rest. We miss you so much our Mandie girl, yet we know you are healthy and perfect in Heaven, and we hope to meet you again, some sweet day!

Sisters hold each others hands for such a little while, but their hearts are joined forever!

Love you forever, my Mandie girl!

Love,
Your sister, Marcella of Bainbridge, OH

Butterflies in meadows
Beautiful, fragile, ephemeral
To see them tossed about by breezes
And dancing from flower to flower
I would think they go nowhere.

I am told
Butterflies endure the storms of summer
And winters cold
And that, finally, they go for the south
To Mexico, I am told.

Butterflies in meadows remind me of children
Tossed and dancing
I am told
That children too endure the storms of summer
and winter and that finally, they go far...
Beautiful, enduring, travelers!

By: Dr. Holmes Morton, MD



IN MEMORIAM
JACOB BERRIER
 SEPTEMBER 23, 2007 - FEBRUARY 3, 2010



IN MEMORIAM
VIOLET WEHRKAMP
 FEBRUARY 18, 2010 - AUGUST 27, 2010



IN MEMORIAM
BRYAN SAWYER SMITH
 DECEMBER 31, 2001 - SEPTEMBER 19, 2002



IN MEMORIAM
MICHAEL UNDERHILL
 DECEMBER 14, 2007 - DECEMBER 17, 2008



IN MEMORIAM
RILEY LONDON CLARK
 MAY 9, 2004 - SEPTEMBER 20, 2004



IN MEMORIAM
ANNA ELIZABETH GIVENS



The Butler Family



**Megan, Alyssa, Griffen and
Toby Kinginer**



Casey and Colin O'Neill



Abby, Matt and William Johnson



**Murphy and Anders
Potter**



Tambryn Campbell



Sydney Utzat



Rocco Arizzi



**Garrett, Baby Girl A
and Baby Girl B Grimes**



Rachel, Jake and Kate Saxton



Alli Williams



Braelyn Campbell



Christopher Weber



Lily, Charlie and Emma Sykora



Bri Johnson and family



Mikey, Hope and Noelle Hazel



Adam and Alex Blair



Wyatt and Jack Tranby

thank you!

Everyone here at the Families of SMA National Office would like to extend our sincerest thank you to Rosemary Francis, who has volunteered countless hours of her time every week, over the past year. We cannot thank you enough for all the help you have provided the National Office and we all so greatly appreciate it! Rosemary has been volunteering for the past year in honor of her niece Elizabeth Caballero, SMA Type I.

Lindsay's Hunt

My daughter was born with Spinal Muscular Atrophy Type II. At six months old we noticed that her legs would not bear weight and she was losing the ability to lift her head up when on her stomach. When we were told she had SMA the doctor's said take her home, love her, she will probably not live past the age of two. Lindsay is 14 years old, a Freshman Honors student and is planning for college. Living with SMA takes so many firsts away but she has accomplished so many other firsts. She works so hard for everything she accomplishes. Everyone dealing with SMA knows the limitations, but Lindsay has always found ways to get around those barriers. Her future plans for us are to get our van converted over so she can get her temporary drivers license and drive the van. I am afraid of this!

Other accomplishments that are in Lindsay's daily life are; she plays power soccer for the Madison Marauders, she plays piano and clarinet, which really helps for finger contractures and for strengthening her lungs.

SMA is not easy; Lindsay has been hospitalized four times, two times for RSV and two times for pneumonia. She had spinal surgery when she was ten for scoliosis; two rods were placed in, along both sides of her spine from top to bottom. Lindsay has the strength of a six month old in her hands, and has very limited strength in her neck. But things still work. She is a very hard worker in school and a great friend to all. I am very proud to call her my daughter.

Tracy Muench, of Campbellsport, WI

Disable

By: Sally Bittner Bonn,
Mom to Oscar, SMA Type II



disable v. 1. to make harmless or ineffective, unman, disarm *Private Sanders disabled the enemy's radio communication device.* 2. to incapacitate, indispose, cripple, ruin *The education system in the United States will be disabled by a bureaucratic bent toward standardization.* 3. tie the hands of, clip the wings of, pull the rug out from under *My husband and I were temporarily disabled by the news that our son will never walk.* 4. to make unable or unfit, weaken or destroy the capability of *Oscar's SMA disables otherwise healthy muscle tissue, making it impossible to move throughout life in a typical and expected fashion.* **disabled** adj. handicapped,

incapacitated *A disabled person must learn to use adaptive and assistive equipment such as a wheelchair, standing frame, gait trainer in order to perform the functions of a typical person without assistive equipment.* **dis** v. slang to show disrespect for *Some folks tend to dis those who are different from them.* **Dis** ruler of the underworld in ancient Roman belief *One must cross the River Styx before entering the Underworld and meeting Dis, in order to be judged.* **dis-** 1. prefix with the meanings "apart, asunder" (disperse, dissociate, dissolve) *Upstanding citizens have worked hard to disperse criticism, dissociate themselves from harmful rhetoric, with the end goal of dissolving prejudice.* 2. prefix having a privative, negative, or reversing force relative to the base noun, verb, or adjective: disability, disarm, disconnect, dishearten, dishonest, dislike, disobey *One might assume that a disability effectively disarms the person affected by such a condition, disconnecting him or her from society, disheartening his or her spirits, but that is a dishonest claim, greatly disliked and widely disobeyed by those affected by disability.* **able** adj. 1. having the necessary power, skill, resources, or qualifications to do something *Each person on this planet is able to shine in his or her own way.* 2. having or showing unusual talent, intelligence, skill or knowledge *Our son, who spoke his first word at around 8 months, began naming objects (door, book, dog, ballon, water) at eleven months, could accurately distinguish by sound between the Beatles and Mingus and show preference for one or the other at fifteen months, topped 100 words by sixteen months, knew most of his colors around eighteen months and could communicate concepts, using his words to do what his body can't, will continue to surprise us throughout his life with just how able he is.*

Dictionary definitions quoted from: Random House. (1991). Random House Webster's College Dictionary: McGraw Hill Edition. New York: Random House.

Alabama Chapter (since 2005)
PO Box 71918
Tuscaloosa, AL 35407
(205) 979-6493
Rhyann Granger, President
alabama@fsma.org

Alaska Chapter (Since 2010)
PO Box 200632
Anchorage, AK 99520
(907) 929-3412
Meghan Lowber, President
alaska@fsma.org

Arizona Chapter (since 1997)
PO Box 43861
Phoenix, AZ 85080
(602)314-4902
Angel Wolff, President
arizona@fsma.org

Northern California Chapter
(since 1998)
PO Box 9014
Santa Rosa, CA 95405
(707) 571-8990
David Sereni, President
ncalif@fsma.org

Southern California Chapter
(since 1999)
1070 E. Orange Grove
Burbank, CA 91501
(818) 846-6589
Rick Montoya, President
scalif@fsma.org

Carolinas Chapter
PO Box 30157
Winston-Salem, NC 27130
(252) 303-0014
Jennifer Lee, President
carolinas@fsma.org

Rocky Mountain Chapter
PO Box 1913
Arvada, CO 80001
(970) 349-0481 or
(877) 591-4023 (toll free)
Marynell Larson, President
rockymt@fsma.org

Connecticut Chapter (since 2003)
PO Box 185744
Hamden, CT 06518
(203) 288-1488
Jonathan Goldsberry, President
conn@fsma.org

Greater Florida Chapter (since 2009)
PO Box 13335
Tampa, FL 33681-1335
(727) 388-1888
Katie Kerns, President
greaterfl@fsma.org

South Florida Chapter (since 2009)
PO Box 268122
Weston, FL 33326
(954) 499-9399
Jennifer Smith, President
southfl@fsma.org

Georgia Chapter (Since 2010)
PO Box 70744
Albany, GA 31708
(229)436-6926
Rio Landa, President
georgia@fsma.org

Illinois Chapter (since 2006)
PO Box 684
Grayslake, IL 60030
(847) 373-3762
Janet Schoenborn, President
illinois@fsma.org

Iowa Chapter (since 2000)
PO Box 326
Johnston, IA 50131
(515) 986-2181
Staci Bailey, President
iowa@fsma.org

Kansas City Area Chapter (since 2004)
PO Box 1214
Mission, KS 66222
(913) 262-6468
Natalie Gibbs, President
kansas@fsma.org

Louisiana Chapter (since 2005)
PO Box 1189
Prairieville, LA 70769
(225) 744-3812
Krista Scurria, President
louisiana@fsma.org

New England Chapter (since 1987)
PO Box 2902
Woburn, MA 01888
(508) 476-2174
BJ Mirabile, President
newengland@fsma.org

Chesapeake Chapter (since 1995)
PO Box 354
Cockeysville, MD 21030
(301) 703-8630
Tina & Derek Lewis, President
chesapeake@fsma.org

Michigan Chapter (since 2004)
PO Box 500
Ada, MI 49301
(517) 669-1665
Ken Armbrustmacher, President
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

**Capitol Region, satellite of the
Greater New York Chapter**
Amy & David Cuniff, President
gnycapitolregion@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

NEW

NEW

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

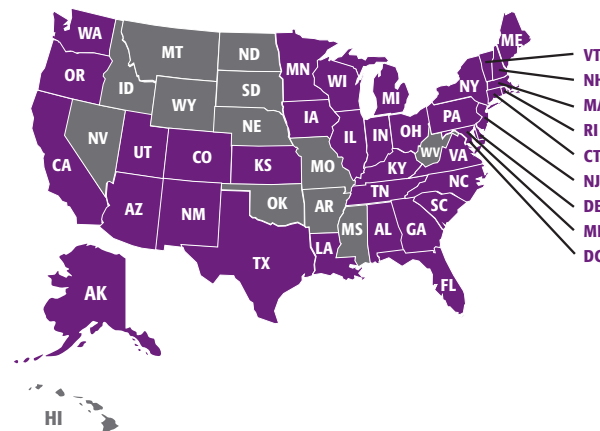
(since 2009)
PO Box 4137
Park City, UT 84060
(435) 901-4461
Shane Barber, President
utah@fsma.org

Pacific Northwest

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

Wisconsin Chapter

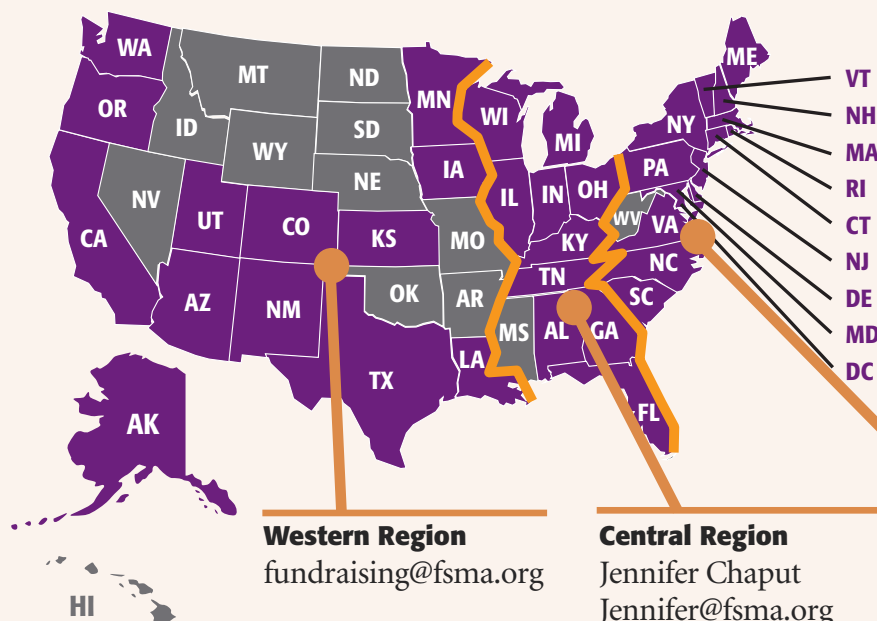
(since 1999)
PO Box 320516
Franklin, WI 53132
(414) 324-0499
Contact: Kate Vogedes, Laurie King, Jenny Imhoff
wisconsin@fsma.org



FSMA Chapters

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

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



Western Region

fundraising@fsma.org

Central Region

Jennifer Chaput
Jennifer@fsma.org

Eastern Region

Sarah (Hunsicker) 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 Chapter Walk-n-Roll

The Alabama Chapter's Walk-n-Roll to Cure SMA on October 29th, 2011 in Tuscaloosa, Alabama was a huge success! Many thanks to everyone who donated and/or participated in the walk. We raised over **\$26,000** and there were over 200 people in attendance! The University of Alabama's Big Al, the Chick-fil-A cow and Betty the Clown all made guest appearances. There was entertainment for the kids such as a bouncer, face painting, making of yarn dolls, carnival games and more. We held a silent auction that raised over \$7,000! In conjunction with the Walk, we added a chili cook-off this year! All the entries were delicious... especially on a cool, fall day!

Look for more information to come for the Alabama Chapter Pancake Breakfast in June or July of this year! We also have tentatively set the date for the 2012 Walk-n-Roll for Saturday, October 6th. More information will be forthcoming for the Walk as well!



2nd Annual Cookies for Ava Bake Sale

On August 28th, 2011, Ebony Arnold hosted the 2nd Annual Cookies for Ava Bake Sale in honor of Ava Johnson at Knollwood Church in Mobile, Alabama. Church members donated their time and talents by baking lots of goodies for the sale! In order to create awareness in her community, Ebony was "arrested" on August 13th and her generous family and friends posted her "bail"! Ebony was also featured on a local news segment which helped to educate her community about SMA and promote the bake sale. Through Ebony's hard work and the generosity of her family and friends, the event raised \$1,300 for Families of SMA!

ALASKA

The Alaska Chapter has been busy getting organized and spreading awareness about SMA. 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

The Arizona Chapter of FSMA held its 6th Annual Walk-n-Roll on Sunday, November 20th, 2011. Two hundred thirty six participants attended the walk at El Dorado Park in Scottsdale, Arizona and were able to enjoy the beautiful weather. We would like to thank all the families for their participation and hard work! We also send out a special thank you to all of the sponsors, volunteers and donors for making this event possible and helping our chapter raise **\$18,500** this year!

A story was featured on the local television station (Channel 12-AZ Central) about Madison Wolff, SMA Type II, during the week of the Walk-n-Roll. The featured story called "Beating the Odds," talked about SMA and how individuals could get involved in the Walk-n-Roll.

CAROLINAS

North Carolina

LittleMan Memorial Golf

On February 1st, 2006, we welcomed a bouncing 8lb 4 oz. baby boy named Joseph Blaine into our family. We were on top of the world. Little did we know that just eight short weeks later our happy world would come crashing down around us. When Joseph was just about two months old we noticed that something did not seem quite right. We were referred to one of the leading children's hospitals in the nation, Duke University, to have a neurological consult. Before we could even make that appointment we found ourselves sitting in the emergency department because Joseph had developed pneumonia. In less than 24 hours we had the devastating news that Joseph had SMA Type I or Werdnig-Hoffman's Disease. What a whirlwind our lives turned into in the days to come. We went from having a perfect baby to a terminally ill one. Even though we found ourselves with a lot of questions, we were fortunate to have a wonderful resource in the staff at Duke that helped us to learn about SMA, and we were welcomed into the Families of SMA with open arms. The care packages and information that came in the days after Joseph's diagnosis helped us to better understand SMA as a disease and how to cope with having a sick child. For the next three months we were able to have few hospital visits and were able to keep Joseph at home as much as we could. Our little boy got his wings on July 1, 2006 at the age of 5 months.

After Joseph passed away we thought long and hard about a way to keep his memory alive and to help the two organizations that were such a great resource to us in our time of need. The first annual LittleMan Memorial "Shoot the Links for a Cure" Golf Tournament was held in June of 2008. The first tournament consisted of 18 four-man teams and a silent auction. Over the last three years we have raised a total of \$38,000 with each year growing bigger and bigger. In our 3rd year, we even had one of Joseph's doctors, Dr. Jeremy Baker, fly in from Texas for the tournament. The proceeds from the tournament were divided among Duke Children's Hospital and Families of SMA.

The 4th Annual LittleMan Memorial Golf Tournament was held on June 17th and 18th, 2011. Activities for Friday night included a dinner for sponsors and players, followed by a live auction. The live auction and silent auction were a big hit! We were again at capacity for teams for the first and second flight of golf on Saturday. Over \$16,000 was raised and divided between Duke and FSMA.

The 5th Annual LittleMan Memorial "Shooting the Links for a Cure" Golf Tournament will be held the weekend of June 15th and 16th, 2012. The weekend will include the same activities as years past, but we are moving our location for our Friday night festivities due to an increase in sponsorship and participation. It is overwhelming the love and support that we receive from friends,

family, and complete strangers to help us raise money for two worthy causes.

One special item that is included each year at the tournament is a Radio Flyer wagon. Joseph was given a Radio Flyer wagon shortly after his diagnosis, through the Tumbleweed's Wagon Fund for FSMA honoring the memory of Braden (Tumbleweed) Ray Campbell. Joseph loved rides in his wagon. Each year the date of the tournament is painted on the wagon. It is one of the most sought after items at our event.

It is an honor to help raise awareness of SMA and to honor the memory of Joseph by hosting this event each year. We are looking forward to a bigger, better tournament in 2012!

*Blaine and Joanne Reese
Macon, NC*

2nd Annual Rusty Rudder Golf Tournament

Eric Paul hosted the 2nd Annual Rusty Rudder Golf Tournament in Lake Norman, North Carolina on September 13th, 2011. In total, the event raised \$1,000 for Families of SMA! Eric is close friends with Christen Tinsley and wanted to raise money to help support her nieces, Brooke and Brielle Kennedy, who both are affected by SMA!



Blaine, Joanne and Joseph Reese

CAROLINAS (cont.)

13th Annual Haley Mitchell Ski-a-thon

The 13th Annual Haley Mitchell Ski-a-thon was held on October 8th, 2011 at Beaver Lake in Gates County, North Carolina. It was a beautiful fall day with a big crowd in attendance. The total amount raised by the water skiing participants and the silent auction topped **\$25,000!**

This was the last of a terrific series that raised over \$425,000 in its 13 year tenure. Haley passed away on October 26th, 2010 shortly after her 13th birthday. She had SMA Type I.

This Ski-a-thon was a very unique fundraiser that garnered a tremendous amount of exposure and awareness of SMA over the years, in addition to the dollars raised. Skiers came from all over to participate in the heartwarming event after raising donations in their neighborhoods and workplaces. The event was extra special to the children that came as friends of Haley that learned so much from her.

The Mitchell family along with many others involved will continue to support the fight against SMA in other ways.



South Carolina

Fight Against SMA Team Coby

Throughout 2011, Diana Snyder from Charleston, South Carolina coordinated the Fight Against SMA Team Coby fundraising effort. Thanks to her hard work, Diana raised \$874 for Families of SMA in honor of Coby Kulis!

CHESAPEAKE

Message from Barbara & Gene Trainor:

After 18 years, Gene and I are confidently passing on the daily work of the Chesapeake Chapter to Derek and Tina Lewis of Mt. Airy, Maryland. As many of you know, Gene and I have relocated to San Francisco and it only makes sense to transition the daily work of the chapter. We feel very fortunate to have met the Lewis' and share their passion to make a difference. With the help of volunteers, Gene and I will continue to run our annual Golf Tournament and Crab Feast. In addition, we will focus much of our time on the Erin Trainor Memorial Fund. Please help me welcome Tina and Derek to the Chesapeake Chapter Family. They are eager to meet many families and coordinate events. Thank you Tina and Derek for continuing the mission of FSMA by advancing research and supporting families!

Message from Derek & Tina Lewis:

Derek and I are thrilled and honored to represent the FSMA Chesapeake Chapter. We feel so fortunate to follow in the footsteps of Gene and Barbara, who have created an incredibly strong network of friends and family, to help advance research and to support all of the families in our area. It is our mission to continue the efforts toward finding a cure for SMA. We are also working diligently to build local support groups for those affected by SMA and their families.

Our Chesapeake Chapter will be represented by some really amazing people who are affected by SMA and have the same mission that we have. Our son Julian has SMA. He has taught us so much about life. He is our motivation to do everything in our power to find a cure for SMA. Joan Palmer, our Vice President, has SMA. Our secretary, Beverly Venedam, had a niece, Erin Trainor, who passed away from SMA in 1994. Joe Calvert, our treasurer, has a sweet daughter, Olivia, who is affected by SMA, Type II. This is a strong group of people who are sure to make a difference in the lives of those affected by SMA. Thank you for your time and your support toward our mission!



Kenneth Hobby with Derek and Tina Lewis

Please feel free to call the Chesapeake Chapter at (301) 703-8630 with any questions and/or concerns. We look forward to meeting you all!

Upcoming Events:

Mark your calendars for the 19th Annual Crab Feast and Silent Auction on August 5th, 2012 and the 18th Annual Charity Golf Classic on September 21st, 2012! Watch out for details on both events on our Chapter page at www.fsma.org/chesapeake.

Maryland**Our Little Jewels 3rd Annual Benefit Golf Outing**

Our Little Jewels held its 3rd Annual Benefit Golf Outing on Friday, September 30th, 2011. The outing netted over \$9,000, of which \$3,500 was given to FSMA, \$2,500 to MDA and \$3,000 to two local programs the offer activities for disabled children.

We will also be holding our 6th Annual Spring Fundraiser beginning in mid-February and concluding on Thursday, April 5th, 2012 at Kelsey's Irish Restaurant in Ellicott City, MD. Between 200-300 people are expected to attend throughout the night. This is the sixth year that Kelsey's has hosted our event. There will be raffle items donated by the Baltimore Ravens, Baltimore Orioles, Philadelphia Phillies, local radio station 98 Rock FM, Jason's Wine and Spirits and many others from local businesses. Kelsey's donates 10% of the day's gross income to Our Little Jewels.

Sincerely,
James and Colleen Lewis, Our Little Jewels Inc.
Ellicott City, MD

Colleen Lewis, Bob Preston, Gina Clark and Kenneth Hobby**Baltimore Running Festival**

On October 15th, 2011 Tom, Maureen Kennedy (Tom's Aunt) and I helped to support Families of SMA by running/walking at the Baltimore Running Festival in memory of Ciara Rose Van De Loo. Even with a late start, we were all able to cross the finish line in one piece. We also met our goals to raise awareness of the disease and raise funds to help families affected by SMA. We set up a gift registry on the FSMA website and asked family, friends, and co-workers to donate to our cause. It's a great fundraising approach for those who are new to fundraising and we're excited to say that we received \$705 in donations!

Many thanks to the generous people who donated to Families of SMA and supported us throughout our training. In addition, a special thank you to the Van De Loo family for sharing their lives with us.

Kathy Krispin, Catonsville, MD

Dear Families of SMA,

Please find the enclosed checks totaling \$155 dollars as donations to SMA research on behalf of Grant, four, and Pierce LeHew, seven months. These monies were collected during the month of August as part of our campaigning to create awareness for SMA. I know it isn't much, but I know it will be put to good use as there is a continuing hope that there will be a cure in the near future.

*Thank you from the bottom of our hearts,
The LeHew Family, Crofton, MD*



CONNECTICUT

Upcoming Events: Please join the Connecticut Chapter for the 1st Annual Cubby's run for FSMA that will be held on Saturday, May 5th, 2012, at the Ridgefield Rec Center in Ridgefield, CT. For more details and to register for the event please visit www.curesma.org/cubbysrun.

SMA Summer Camp

We had a blast at Camp Harkness and the weather was great! Some of us were there for 10 days (Shaun & us, Armand & Co, MJ and Brenda from NY). Some came for a shorter period (Joey & parents from MA), some came for only a couple days and went home for the nights (Marie Eleni & Family). Connor and family came for a day from MA and others came just for the picnic on the 21st (Brandon, Emma, Yvette, Isabella and their families & friends). Weather was great so all-in-all we had a super time and are looking forward to next year!

*Take care,
Mary Ellen Barrelle, Norwalk, CT*



Emma Hope Award

Emma Goldsberry (SMA Type II) is shown here with the most recent Emma Hope Award winners at NES pageants. Ava Botelho won at the Miss Elegance pageant in August, where \$250 was raised for FSMA. Mylie Love won at NES Michigan, and that pageant raised \$580. The winner at NES Nationals in November



was Karrah Arterberry, and that pageant raised \$500. These are the latest pageants from NES director Tammy Rezendes that donate entry fees from the Emma Hope Award to FSMA. Those fees, along with fees from other pageants, photo contests, and other donations from pageant participants, have totaled over \$10,000 in donations to Families of SMA. The Emma Hope Award is given to the participant displaying the most kindness towards others.

Jonathan Goldsberry, Bristol, CT

Dear Families of SMA,

We are pleased to be able to make the enclosed donation in the amount of \$1,000 to Families of SMA in memory of our beloved son, Michael.

Mikey was diagnosed with Spinal Muscular Atrophy at the age of eight months and passed away on May 19th, 1998. He was two years and nine months old. To celebrate Mikey's life, our family raised the funds necessary to construct "Mikey's Place", a wheelchair accessible playground in Wethersfield, Connecticut. Countless numbers of children of all abilities and their families continue to enjoy our special park! Many people have told us that they have never witnessed such an outpouring of support for a project such as ours. Truly, there is so much goodness in this world.

We recently hosted our Annual Mikey's Place Golf Classic with the proceeds going towards the Preservation Fund for our park. We want to be assured that Mikey's Place will remain in pristine condition for many generations to come. Please accept a portion of the proceeds from this event as a donation in Mikey's name with the hop that we can make a difference in

the lives of children like our precious son and that someday a cure may be found. Please continue your good work of helping so many children and their families.

*Sincerely, Dan, Mary and Danielle Daversa
Wethersfield, CT*

Dear Families of SMA,

Each payday, our staff has a Dress Down Day where staff members make a donation in exchange for dressing comfortably for the day. During November, money was collected for Spinal Muscular Atrophy. On behalf of the staff at Tolland Middle School, we would like to donate the enclosed checks totaling \$168. We know you can always use the extra money and hope these checks will help!

*Sincerely, Roberta Gavin
Tolland Middle School — Tolland, CT*

Kid's Cut-a-thon

On August 28th, 2011, Erin Simons hosted the Annual Kid's Cut-a-Thon in honor of Cubby Wax's 12th Birthday at the Shine Salon in Ridgefield, Connecticut! The salon provided back-to-school hair cuts, sold FSMA bracelets and had a cupcake decorating station! In total, \$810 was raised for Families of SMA! Thank you to Erin and the staff at the Shine Salon for organizing this wonderful event!

Past Time Club 3rd Annual Golf Tournament

On September 9th, 2011, Mark Chandler hosted the 3rd Annual Past Time Club Golf Tournament at Fairchild Wheeler Golf Course in Fairchild, Connecticut. Thanks to family, friends and a great turnout of golfers, the event raised \$2,000 for Families of SMA in honor of the Dobson Family!

GEORGIA

The Georgia Chapter continues to work on meeting regularly as a chapter and establishing fundraisers. We were scheduled to have a Georgia gathering for SMA families last fall planned by VP Ashley Jones, but her daughter, Skylar Marie Jones, passed away on August 16th, 2011; therefore, the family gathering has been postponed until the spring. We are looking forward to meeting everyone and holding our first major fundraiser as a chapter this year - details in the next issue.

We would also like to share our excitement about the 2nd Annual SMA 5K Eat n' Run on March 24th, 2012! For details on the Eat n' Run visit www.fsma.org/healcaleb. Thank you also to our Chapter President, Rio, who had \$1,025 donated to FSMA from family and friends in honor of her son, Mateo, for his birthday!

Georgia has suffered many losses of SMA children in 2011 and we want to offer our support and condolences to all families and friends affected. There are many SMA families still fighting and we hope we are able to make a difference in educating medical professionals to help better care for our kids, offering support to families, answering questions, raising awareness and financial support to help meet the needs of SMA families as well as researchers searching for a cure! We are hopeful that 2012 is going to be a great year! Thank you for your support as our chapter gets established and starts growing some roots this year!

GREATER FLORIDA

Hello from the Greater Florida Chapter!

Covering central and northern Florida, our chapter is made up of many wonderful, inspirational families, and our local board works hard to provide them the support that they need. Whether it's guidance for a newly diagnosed family, assistance finding doctors or help with IEP meetings, our goal is to be a meaningful resource to families – while, at the same time, we work to raise SMA awareness throughout our region.

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

Our awareness and fundraising efforts from September 2011 through February 2012 included the following:

19th Annual Maluko Golf Classic on October 11th, 2011

This year, the Hernandez, Romaele, Leto and Menendez families came together once again to host the Maluko (defined as "good friend," made up by event organizers) Golf Tournament to benefit FSMA (in honor of Tyler Hernandez – SMA Type II) and Kayla's Hope for Kids. When the tournament started 21 years ago, the Boys and Girls Club, the place where most of the Malukos spent time as kids, was the beneficiary. That changed in 1996 to Families of SMA, when Tyler Hernandez was diagnosed with SMA Type II. This year, more than 130 golfers and many sponsors participated in this incredible event held at the Emerald Greens Golf and Country Club in Tampa, Florida. With strong community

support behind it, this year's tournament raised a whopping \$38,500 for FSMA. In addition, Best Buy was able to supply ten employee volunteers for the event and donated \$1,000 to our cause. The Greater Florida Chapter is thankful to be part of such an amazing event and look forward to next year's tournament!

11th Annual Costume Crusade and Carnival on October 28-29th, 2011, and Parking Lot Sale on November 5th, 2011

Joe (SMA Type III) and Susan Miller of The Village Early Learning Center of Brandon, Florida, also continued to host their annual week of fundraising and SMA awareness events. At the Village Early Learning Center, infants through Pre-K students paraded in costume and then participated in carnival games on Friday, October 28th. Parents and community members were asked to purchase raffle tickets for beautiful baskets of items donated by parents, small businesses, and community agencies. On Saturday, October 29th, the Village Early Learning Center hosted a three-hour "Halloween in the Halls," a haunted house for older children and preschool carnival games for the younger ones. The Community Fair and Parking Lot Sale was the final event for the fundraiser, taking place on Saturday, November 5th. Small businesses were invited to display their services or products. Household donations from the Village Early Learning Center's 177

families were sold, and the much-anticipated basket winners were drawn! These fun and community-spirited events raised almost \$1,600 for FSMA.

3rd Annual Chapter Walk 'N Roll, November 19th, 2011

More than 200 people turned out on a beautiful Saturday at Ft. Desoto Park in Tierra Verde, Florida, to participate in our chapter's 3rd Annual Walk 'N Roll fundraising event. The team spirit was high, with family and friends wearing brightly colored shirts and holding signs supporting their teams, which included Allison Wonderland (in honor of Allison Kerns – Type II), Andrea's Army (in memory of Andrea Trakas – Type I), Andy's Army (in memory of Andy Butler – Type I), Brooklynn's Brigade (in honor of Brooklynn Santos – Type II), Cooper's Crew (in memory of Cooper Novotny – Type I), Francisco's Forces (in memory of Francisco Rodriguez, Jr. – Type I), Maia's Mission (in honor of Maia Shockley – Type II), Sun City Center (in honor of Mike Goynes – SBMA), Team Ava (in honor of Ava Davidson – Type II/III), Team Kathy (in honor of Katherine Bray), Team Taylor (in memory of Taylor Bowser – Type I), and Team Toro (in honor of Isabelle Toro – Type II).

Before the walk, Bay News 9's Virginia Johnson served as the local celebrity guest speaker, reading the names of our chapter's Angels and Warriors. "It was a deeply humbling and joyful occasion," remarked Johnson in a Bay News 9 report on the event.

Friends and families also had the chance to meet the Ray Team girls and Raymond from the Tampa Bay Rays baseball team, watch a live Power Soccer demonstration by the Tampa Bay Crossfire, stretch with Christie Bruner of Baby Boot Camp, enjoy custom cupcakes from Dough Mom, and learn about the Kidz Club. Chick-fil-A donated a delicious lunch for all walk participants, and the Chick-fil-A cow also made an appearance to the delight of the crowd. Kids of all ages and



Team Allison Wonderland

GREATER FLORIDA (cont.)



abilities enjoyed the bounce house, interactive arts and crafts, face painting and fun games! And, of course, Mystical Moments Entertainment was there to DJ, and Leslie Montenegro took professional photos of all teams!

With amazing prizes for the raffle and silent auction that included Tampa Bay Lightning #4 Vincent Lecavalier's autographed game-used hockey stick, Tampa Bay Rays signed merchandise, a hot air balloon ride, a Walt Disney World Swan & Dolphin hotel stay, a Tradewinds Resort hotel suite, Seaworld/Aquatica tickets, Busch Gardens tickets, Pirates Dinner Adventure tickets and more, the Walk 'N Roll put the "FUN" in fundraising! This year's event raised almost \$23,000 and set the bar high for next year!

SMA Fundraiser at University of Tampa, December 2th, 2011

Brooklynn Santos (Type II), her mother Shawn, and Audra Butler (mother to Andy Butler, Type I Angel) were invited by the University of Tampa's Florida Eta Chapter of Alpha Epsilon Delta (AED) to speak to a group of college students who plan to enter the medical field, about their families' journeys with SMA. Earlier in the semester, Dr. Rebecca Bellone had provided her students, many of whom are part of AED, with an overview of the genetics of SMA as part of the Molecular Biology course she teaches. Dr. Bellone reviewed the current literature on SMA with the class and then encouraged her students to do more. This student-organized fundraiser and awareness event was a direct result of Dr. Bellone's call-to-action. Thank you to Ms. Castelbolognesi and Adriana Batazzi who helped to organize the fundraiser. In total, \$167 was raised through cash donations and a raffle for FSMA during the three-hour event, with a matching sum going to the Santos family to help pay for Brooklynn's medical needs. One of the students who attended the event said, "My mother has always told me to pursue a career that I would love and enjoy. I decided to go into science for the purpose of helping others knowing that I would be rewarded in ways far more valuable than money. I'd like to thank Brooklynn and Audra Butler for reminding me of this. Through their struggles and sadness, I found inspiration and hope."



Chapter Meeting, February 4th, 2012

For our first official chapter meeting of 2012, we joined forces with the local Muscular Dystrophy Association (MDA) to present the "ABC's of IEPs." The meeting, which was held at All Children's Hospital/Johns Hopkins Education and Conference Center, addressed the laws mandating Individualized Education Programs or IEPs, explained how to get the services SMA-affected children need in their schools, and offered a forum for parents to hear first-hand from others who have been through this process. Featuring Special Education Attorney and former Special Education teacher Mark Kamleiter, as well as Paula Keyser, family resource specialist with All Children's Hospital, this informative meeting provided families with the necessary tools to get the services their children need.

PSCU Charity Event — Candle Lighting

Katie Kerns and the Greater Florida Chapter hosted a candle lighting event during SMA Awareness Month on August 13th, 2011 at Sunset Beach in Tarpon Springs, Florida. The event had a great turnout and the beach ended up making a donation of \$175 to Families of SMA! Thank you to the PSCU for your generosity and to all of the families who attended this wonderful event!

Coming Soon

In the coming months, the Greater Florida Chapter will be launching a series of dinners exclusively for families of angels to better connect and support those who have lost a loved one to SMA. The first dinner will be hosted in March by the Butler Family. More information will be posted on our web page and in our Facebook group. And, for more family networking for the entire chapter, we'll also be holding our 4th Annual SMA Family Fun Day this spring!

For the second year in a row, we will be teaming with the Tampa Bay Rays for SMA Day at Tropicana Field, which will take place this summer. At the game, we'll spread SMA awareness to our entire community (and hopefully a national television audience too)! In addition, a "Fondue-raiser" at the Melting Pot and appearances at several local events are also being planned. We're excited about 2012! Watch for our next update!

On July 2nd, 2011, our nephew Tristan Kiefer married his lovely wife, Janel, in Minot, North Dakota. On that beautiful summer's day, they donated \$250 to FSMA, in lieu of wedding favors and in memory of Tristan's baby cousin, Andy Butler, who had SMA Type I.

As their first anniversary approaches, we wanted to share how proud we are of Tristan and Janel for their love, thoughtfulness, and generosity. We know that Andy was with them on their wedding day and continues to watch over their family from heaven.

Audra and Alan Butler, Land O Lakes, FL

Dear Families of Spinal Muscular Atrophy,

The students at Holy Family Catholic School held a Charity NUT Day to support Families of SMA. A Charity NUT Day is when students dress out of uniform and donate a dollar or more for a specific charity. We have a family at our school that is affected by SMA, so we decided to hold this Charity NUT Day not only to raise funds for your organization, but also to educate our students about SMA.

Enclosed is a check for \$230 for FSMA. We hope this donation helps your organization provide for those affected by SMA.

God bless, Brittany Gorr — Community Service Chairperson and Sister Florence Ann Marino, IHM-Administrator, St. Petersburg, Florida

GREATER NEW YORK

NY Yankees SMA Awareness Day

The Greater NY Chapter celebrated SMA Awareness Day with the NY Yankees in September 2011. We were thankful to receive a donation check of \$10,000 from Quest Diagnostics in an on field presentation. New York Yankees star Center Fielder, Curtis Granderson, joined the families on the field for this award presentation. The New York Yankees made the day in honor of Ciara Van De Loo. Many thanks to the New York Yankees and especially Carol Laurenzano for helping us raise tons of SMA Awareness.



about their research on SMA and further inspiring hope in our families that one day there will be a treatment and a cure for Spinal Muscular Atrophy.



Please save the date for our 8th Annual Walk-n-Roll which will be held on Saturday, September 15th, 2012 at the Long Beach Boardwalk in Long Beach, NY.

Congratulations to all of the SMA children who participated in the annual Henry Viscardi Wheelchair Basketball Tournament. Also congratulations to those SMA Children who participate in the NY Miracle League Basketball Team.

Thank you to Friends of Philly in Howard Beach, New York who donated \$2,500 from their annual golf outing. We would also like to thank the Long Beach Varsity Football Team for donating over \$800 to our chapter with special thanks going to Scott, Andrea, & Cadence Martin from Long Beach, New York for all of their efforts. Many thanks to the Cleere Dance Project for donating over \$1,000 for our two Zumba events with special thanks to Brittany Cleere of Huntington Station, New York for organizing and promoting these two wonderful events.



Please save the date for our annual SMA Awareness Day with the NY Mets on Sunday, August 26th, 2012.

7th Annual Greater New York Chapter Walk-n-Roll

The Greater New York Chapter held its 7th Annual Walk-n-Roll to Cure SMA on September 17th, 2011 in Long Beach, New York. This year's Walk-n-Roll was in memory of Keira Sweeney and her parents, Jacqueline and Mike, made a beautiful speech in her honor. With the support of over 20 Greater NY Chapter SMA families, we raised nearly \$75,000 which set a chapter record. Special thanks to our top fundraising teams Sweet Baby Jack, Team Emily, and Miles for Max. Our deepest condolences go to the family of Baby Jack who lost his battle with SMA in December. Thank you to all of the families and friends who came out to support this amazing day.

The Greater New York Chapter would also like to thank Dr. Adrian Krainer for speaking to all of the families at the walk. Dr. Krainer is an SMA researcher and a member of the Families of SMA Scientific Advisory Board (SAB) who is currently involved in a study on Antisense Therapy as a potential drug for SMA. Thank you also to Dr. Chris Henderson and colleague Dr. Mathieu Desclaux for speaking

GREATER NEW YORK (cont.)

We are very proud to welcome members of New York's Capital Region into our chapter as a Satellite Chapter and look forward to working with them in the future.

Capital Region

NY State PBA Fundraiser

We were blessed when friends and fellow New York State Trooper Roy & Suni Swann approached our family about being the recipients of a NYS Trooper Signal 30 Benefit Fund Fundraiser. What an awesome and humbling experience to have so many people come forward and work together to help us cover our son Caleb's (SMA Type II) medical expenses. We agreed to the fundraiser on the condition we would be able to donate a portion of the proceeds to Families of SMA. As much as the expenses for therapy and handicap accessible housing and transportation were mounting, we want a cure!



The fundraiser was held on September 1st, 2011 and raised \$1,200, after having to be moved due to Hurricane Irene! In addition to the fun of the actual event, which included bouncy houses, a

bar-b-q dinner, silent auctions, raffles, crafts for the kids, face painting and more, the event allowed our family the opportunity to raise awareness in our community about SMA. The local paper did a positive article about Caleb's battle with SMA, and the challenges we face,

and two local TV stations ran stories – even mentioning our intentions to hold a fundraiser for FSMA this coming summer. The community support was absolutely amazing and as a result we were able to communicate our passion about how we need to help find a treatment and cure for SMA. We are so grateful for the support of Dave's fellow troopers, Roy & Suni's tireless efforts, and all the people who joined with us to help find a cure for SMA!

*Trooper David & Amy Cunniff
Caleb & Zachary
Schenectady, NY*

Jumpin' Jeepers Fundraiser



The Jumpin' Jeepers Fundraiser in memory of Max Rubenstein was held on Monday, October 10th, 2011 in Mahopac, New York. Instead of our initial plan of donating admissions from the four hour time frame, we ended up donating our admissions for the whole day! We also put out a donation bucket to collect loose change from our visitors!

Suzanne, one of the co-owners, accompanied myself and Michele Weisblat throughout the event. We handed out balloons to the children as they left and handed out Families of SMA pens and bracelets to the parents. Some of our regular customers that came in weren't aware of the fundraiser at first, but were happy to donate some change! The children had a lot of fun here, as they always do! We also held a free raffle during the fundraiser in which the prizes were a party package, an annual membership and a ten visit pass.

We were more than happy to organize this event in memory of Max and appreciate all of the help that Families of SMA provided to us. In total, we were able to raise \$200 for Families of SMA! I think we really helped increased awareness about your organization and perhaps we can do this again in the future!

*Ingrid Torres
Mahopac, NY*

2011 ING New York City Marathon

On Sunday, November 6th, 2011, Trey Russell finished the New York City Marathon in memory of his son Nathan. Through the generosity of his family and friends, Trey was able to surpass his gift registries' \$10,000 goal and raised **\$12,312** for Families of SMA! Thank you Trey for your hard work and dedication in both training and fundraising for this race!

To read Trey's full story about his experience in running the New York City Marathon, please turn to page 47.

Birthday Fundraiser in Honor of Sawyer Ament

On Saturday, October 23rd, 2011 we had our first fundraiser to honor Sawyer on his 2nd birthday and to spread awareness of SMA in our community. Friends, family and supporters from the community came to meet Sawyer. There were raffles, a 50/50 drawing, SpongeBob, clowns, a magician, a DJ and a band.



Restaurants in the neighborhood donated all the food and the Lief Ericson Bar & Grill in Bayridge, Brooklyn provided the room. In total, we raised \$1,500 for FSMA, \$1,000 for Columbia Presbyterian SMA clinic in NYC and \$1,000 for the Sawyer SMA Fund to help cover cost for medical supplies and equipment.

It was a wonderful event and I would like to thank FSMA for helping us on this special day.

*The Ament/ Walters Family
Brooklyn, NY*

GREATER NEW YORK (cont.)

Sean's 4th Birthday

On June 11th, 2011, Eileen Prymaczek from Whitestone, New York raised \$250 in honor of Sean's 4th Birthday! Thank you Eileen!

Hair Studio II Extensions Fundraiser

From August 17th to September 17th, 2011, Katie Macchia and Hair Studio II in West Islip, New York hosted a fundraiser for SMA Awareness Month! From selling the purple and orange hair extensions, \$360 was raised for Families of SMA! Thanks to Katie and the Hair Studio II for hosting this fundraiser for Families of SMA!

Macy's Shop for a Cause

The Greater New York Chapter sold \$5 passes for the Macy's Shop for a Cause Event! On August 27th, 2011, anyone who purchased a pass received up to 25% off their purchases! In total, \$596 was raised for Families of SMA! Thank you to Michele Erwin for organizing and to everyone who participated in the event!

PVH December Sample Sale Fundraiser

PVH Corp. in New York, New York donated **\$10,000** to Families of SMA from this year's sample sale. FSMA is thankful for the company's continued support. Thank you to Tiffany Vargas for helping to coordinate the fundraiser!

2nd Annual Charity Golf Outing in Memory of Stephen Ruocco

The 2nd Annual Charity Golf Outing in Memory of Stephen Ruocco was held on October 10th, 2011 in Hopewell Junction, New York! Thanks to Christian Jones and the Knights of Columbus for all of their hard work and dedication in planning this event! In total \$3,500 was raised for Families of SMA in memory of Stephen Ruocco.

KeyBank Giving Thanks Fundraiser

The KeyBank Giving Thanks Fundraiser was held on November 10th, 2011 in New York, New York! Thanks to Robin Oliveras for organizing the event and to all of the employees for their participation and generosity! In total, \$500 was raised for Families of SMA!

Dear Families of SMA,

We are very pleased to be able to donate more funds in honor of our daughter Emily Lozina, SMA Type I, who just celebrated her 1st birthday last week. Since her diagnosis last May, we have been spreading awareness of SMA, and collecting funds through donations and also selling COUSINS FOR A CURE-SMA bracelets. Please accept this new donation of \$518 to be added to Emily's gift registry. We are proud that we have been able to raise over \$6,000 through her gift registry and almost \$23,000 for Team Emily for the Walk-n-Roll event held last September in New York. We strongly support FSMA mainly because of its support for research, but also for the support, equipment and supplies offered to families battling against SMA.

We look forward to collecting more funds and spreading awareness of SMA. Thanks for the wonderful work you do.

*Fondly,
The Lozina Family
Cos Cob, CT*



ILLINOIS

Charlie's Birthday Party

Our daughter, Maria Rose Spina, was born on December 10th, 2005. She was diagnosed with SMA in July 2006, and thanks to FSMA, that devastating news was somehow eased by having somewhere to turn. We enjoyed our Maria, every second of every day, and were blessed that her true fight was not until September, and her battle, although lost on October 12th, 2006, was gentle on her. Like all those who experience loss, especially the loss of children, we'll never forget! But it is truly touching when those we love, remember. Maria and Charlie were 40 days apart in age, although Charlie doesn't have specific memories of her, we know Maria's always with him (and with each and everyone of us in our large wonderful family, especially her siblings, Peter, Anthony and Genevieve).

This year, our sister, Felicia (DiGiovanni), and our nephew Charlie had the wonderful idea to have guests of Charlie's 6th birthday party make donations to FSMA rather than receive traditional birthday gifts. And, how wonderful of Charlie to have agreed (not because he's our nephew, but we don't know many kids who would be willing to forego gifts). We are so grateful to Charlie's classmates and the friends of the DiGiovanni family who generously donated! In total \$215 was raised for Families of SMA! Hopefully, in Charlie's lifetime, if not sooner, we will see a cure!

Thank you Felicia, Tommy, Charlie & Anita for always remembering- We love you!

*Marian & John Spina
Elmwood Park, IL*

SIU Football Game Fundraisers

At Southern Illinois University-Carbondale, the American Marketing Association has begun to raise awareness of Spinal Muscular Atrophy campus-wide. In the Fall of 2011, I became Vice President of AMA and my primary focus was to educate people on SMA in an exciting environment, and to raise funding for research. I pushed tailgating as our best opportunity to market "AMA for SMA." Our first home football game was the only one we lost money on. After that, social



ILLINOIS (cont.)

media outlets and word of mouth led to individuals wanting to participate in something greater than themselves and more than just tailgating. AMA's tent was decked out in FSMA's purple and orange colors with banners, signs, bracelets, newsletters and pamphlets informing people about SMA. Also, President Natalie Miceli and I educated members on SMA so that we could all spread the word. I created a sign and slogan for our tent that read "Toss, Flip & Pong for a Cure." This helped students recognize that our tent had games such as bags, flippy cup and beer pong and they could play all day if they donated something. Between games and donations in exchange for beer and jello shots, we were able to generate \$438 for FSMA, while having a blast!



The SIU's American Marketing Association also raised \$200 from our annual Turkey Trot event, a 5K that students pay \$15 to participate in. Each participant received a purple and orange t-shirt along with an FSMA bracelet. In the past, Turkey Trot money was donated to Special Olympics, but this year we were able to split the funds. It's the best feeling to walk around campus or go to AMA events and see countless people wearing FSMA wristbands. I wanted to raise awareness and money for Spinal Muscular Atrophy because I volunteer at the SMA Annual Conference and each year I meet new affected persons who continue to be some of the most intellectual, inspirational and



beautiful individuals I have ever met. Also, my big sister Shannon O'Brien (now 25), was diagnosed at age two and could walk until 4th grade. Everyday I wish more than anything that she could have all of the same abilities I do. Her courage motivates me to continue this fight. I am hopeful that one day we will find a cure.

*Traci O'Brien
Grayslake, IL*

5th Annual Kyra's Idea... Angie's Hope Fundraiser for a Cure



My name is Kyra Scadden and I'm 12 years old. Five years ago I started a fundraiser called Kyra's Idea, Angie's Hope because I wanted to raise money to find a cure for my best friend Angie Lee, who has SMA Type II. For the first four years we held a garage sale, but this year my family and the Lee's changed it a little. Instead of a neighborhood sale at my house, we had a party. My family is very familiar with that term and it showed. The party took place on Saturday, September 24th, 2011 at our local Fox and Hound Pub and Grille. It was advertised at all of our schools as a "Family Pasta Party". The Lee's and my family, the Scadden's, hosted, and 200 people attended. Because of all the people, the raffle, the silent auction and the prize wheel, we raised **\$20,400**. I think that everyone that came had an amazing time and I know that I got a lot out of it.

I remember meeting Maddie (SMA Type II) at the sale last year and this year meeting Ella Casten (SMA Type II) and her family, which are memories that I will cherish forever. Being a part of Kyra's Idea, Angie's Hope is just the best feeling. I am proud to say that I helped raise more than \$75,000 dollars over the last five years and that money will be used to help the families affected by SMA and help find a cure. We've already started planning next year's event and we're all very excited. Please go to www.angieshope.org to find out more.

*Kyra Leanne Scadden
Naperville, IL*

Mama Bracelet Sales

By the time our daughter, Ella, was diagnosed with SMA Type II, Mama Bracelets had existed for almost one year. In that time we had raised money through bracelets and necklaces for several organizations including The Rett Research Trust, The American SIDS Institute, Little Friends, Inc. (Autism Awareness), Autism Speaks, and LUNGevery.



When Ella's diagnosis was confirmed, we immediately went to work creating and designing a bracelet collection for women, men, and children that would follow our product's reputation of simplicity and style. Our "Cure SMA" Awareness Bracelet Collection was conceived.

Through all sales of these bracelets we lovingly donate 30% of the proceeds directly to FSMA in support of the research for treatments and a cure. Through the generosity of many people in our community and across the country we have raised over \$2,130 so far for FSMA!

To read more about Mama Bracelets, or to order your bracelet today, please visit www.fsma.org/mamabracelets or www.mamabracelets.com.

*Lindsey and Michael Casten
Naperville, IL*



Dear FSMA,

Just a quick note to everyone letting you know that our **CANDLES for SMA** website is all new: new look, new function, a few new scents to choose from and new candle choices. Please check it out and consider placing an order. Proceeds are sent to Families of SMA to help find a cure, and it keeps me, Lisa (SMA Type III), moving. To date, we have donated \$1,723! A big thank you to all of you who have placed orders! Please visit www.candlesforsma.com to place an order.

Lisa Woods
Dixon, IL



2011 Chicago Half Marathon & 5K

On September 11th, 2011, Team Families of SMA ran in the official Chicago Half Marathon & 5K. This was the first year that



Families of SMA participated in the race as an official charity partner! FSMA made a great showing at the race, with a total of 16 registered runners that came out from all over the country. Our community came out from New York, Massachusetts, Missouri, and across the state of Illinois! Our 16 member team ran to honor or remember the lives of multiple individuals affected by SMA, including: Greyson Erwin, Cruciano Fanaro, Maura Nakanant, Shannon O'Brien, Grant Russell, Macarthur Sohl, Ciara Van De Loo and Josiah & Journee Marie Weiberg. Together, our runners were able to raise a total of **\$14,500** for Families of SMA!

To participate with Team FSMA next year, watch the Families of SMA website for more details. Next year's race will be on September 9th, 2012 – mark your calendar!

2011 Chicago Marathon

Marta Meyers of Madison, Wisconsin and Jessica Johnston of Chicago, Illinois ran the Chicago Marathon on October 9th, 2011 for Team Families of SMA! Marta and her niece Jessica both ran the marathon in honor of Jonathan Meyers Davis, SMA Type II, and in memory of Rosie Meyers Davies, both children of Marta. These two amazing women finished the marathon and raised \$3,528 for Families of SMA. Thank you for all of your hard work in fundraising and training for the marathon!

Children's Bracelet Donation

On December 24th, 2011, Kay Wittman collected donations from her students in Jerseyville, Illinois for FSMA bracelets! Thanks to her generous class, Kay raised \$75 for Families of SMA in honor of her daughter Amy, SMA Type III.

First Annual SMA Walk-n-Roll to Cure Tala

The First Annual SMA Walk-n-Roll to Cure Tala was a huge success! The event raised twice as much as was originally anticipated, bringing in **\$22,894** for Families of SMA, all in Tala Albakri's name! The walk took place at Centennial Park, in Orland Park, Illinois on the evening of Saturday, July 20th, 2011. The walk brought in hundreds of participants and featured dinner, a raffle, DJ, bounce house, and photo booth.



ILLINOIS (cont.)

Manel Salah, Tala's aunt, organized the walk. Here is what she had to say before the event: "I want everyone to know why I'm planning this Walk-n-Roll for my niece Tala. She was diagnosed at six months with Type I Spinal Muscular Atrophy. We thought she was "delayed" when she couldn't pick up her head or roll over. We were patient and hoped for the best. As time went by doctors kept saying she was "delayed" until she hit six months old. After seeing a couple of specialists and taking a blood test she was diagnosed with Type I SMA. We were all devastated. We had never even heard of SMA. She has been hospitalized four times with pneumonia and the most frustrating part for her parents were that some doctors and nurses at the hospital weren't even familiar with SMA! Tala was the one teaching them! We understand that God brought Tala into our lives for us to love her and cherish every moment with her. She is teaching us and it is our job to teach others about SMA. This walk is to honor my precious niece Tala. She is our angel. This walk is not only to raise money for FSMA but to raise awareness about this horrible disease. It would mean the world to my family if you all would come out and show your support and walk with us in honor of Tala."

Unfortunately, shortly after the event, Tala passed away on September 8th, 2011 at the age of 18 months. She was surrounded by family and loved ones at the hospital. Her life and her beautiful smile will forever live on in their hearts, and in the memory of everyone that met her.



IOWA

R Studio Yoga Fundraiser

Each Month, R Studio in Urbandale, Iowa has a Yoga Donation class. For SMA Awareness Month in August, Families of SMA was the charity of choice. Michelle Soyer, who organized the donation class, said, "It was an easy, fun way to inform others about Spinal Muscular Atrophy and allow them an opportunity to support FSMA." In total \$524 was raised at the class! Thank you to Michelle and R Studio for your support of Families of SMA.

Race 4 Grace



The 5th Annual Race 4 Grace was held in conjunction with RAGBRAI (The Register's Annual Great Bike Ride Across Iowa) from July 24th-30th, 2011. The Race 4 Grace Team for RAGBRAI has been riding together since 2007. Team members include family, friends, relatives and community members of Grace Nelson who have learned about Spinal Muscular Atrophy and are dedicated to helping find a treatment and a cure for the disease. This year, hundreds of family and friends supported the team through generous donations. Anyone who donated received a Race 4 Grace t-shirt and in turn, helped spread awareness, as all of the team shirts had the message "Help Fight SMA" on the back. In total, we were able to raise \$3,428 for Families of SMA! Thank you to everyone who participated in the race and donated to the event! We could not have been as successful without your support! We are already looking forward to the 6th Annual Race 4 Grace in 2012! We will be working hard throughout the year to recruit even more riders for next year!

*Nic and Michelle Nelson
Sioux City, IA*

Cheer Competition Fundraiser

On February 26th, 2011, Megan Ramirez of Le Claire, Iowa set up a table at the local Cheer Competition and sold baked goods, clothes, bows, tutus and more while talking about SMA! In total, Megan raised \$650 for Families of SMA in honor of her daughter, Madison (SMA Type II). Thank you to Megan for not only raising funds, but also creating SMA Awareness in your community!

Silpada Jewelry

Throughout June of 2011, Nancy Anton-Jensen of Hiawatha, Iowa hosted a Silpada Jewelry Fundraiser. Thanks to the generosity of family and friends, Nancy was able to raise \$425 for Families of SMA in honor of her son, Quinn (SMA Type II). Thank you Nancy for all of your hard work in organizing the fundraiser!

Fundraiser in Honor of Cael Rudkin

In June of 2011, Joey Rudkin of Rhodes, Iowa hosted a fundraiser in honor of his son Cael (SMA Type II). Thanks to the support of family and friends, Joey raised \$1,220 for Families of SMA! Thank you Joey for all of your hard work in planning this event!

15th Annual Beaverdale Beaverdash

The 15th Annual Beaverdale Beaverdash was held on Saturday, September 17th, 2011 in Beaverdale, Iowa. The event had a wonderful turnout this year and thanks to the support of family, friends and the community, **\$28,760** was raised for Families of SMA! Thank you to Julia Anderson and Julie Greenwood for their hard work in planning and organizing the event as well as to all the participants and supporters who helped to make the event a success!



LOUISIANA

2nd Annual Jazz 'n Jam

On January 28th, 2012 the Louisiana chapter of FSMA hosted their 2nd Annual Jazz 'n Jam for SMA Research at Louisiana State University. Over 30 Jazzercise instructors from around the state and over 300 participants from all over the south attended!

The instructors hosted the first two classes and celebrity choreographer Tim Roberts wrapped up the morning with an amazing hip hop class! We also had demonstrations by the Han Mu Do Demonstration team, Heart and Soul Jump Rope Team as well as Junior Jazzercise. The LSU Softball team arrived to greet everyone and have a little fun too! The stunt team from GI Joe and Spiderman 4 were also in attendance. Jambalaya was donated by the award winning Ascension Parish Jambalaya Festival Association. The kids had a blast with Chris the magician and Susan the face painting party artist. In all, **\$16,530** was raised in three hours of fun! The 3rd Annual Jazz 'n Jam will be held in January 2013. We are also planning our 1st Annual SMA Family Crawfish Boil to be held in May 2012! Watch out for details on both events on our Chapter page at www.fsma.org/louisiana.



KANSAS CITY

Kansas

11th Annual Cure SMA
Race-n-Roll

The Kansas City Chapter of FSMA hosted the 11th Annual Cure SMA Race-n-Roll in Roeland Park, Kansas on Saturday, October 1st, 2011. Once again, we had a perfect day for a 5K Run/Walk and we had over 700 participants and volunteers! We had a record nine teams represented: Brett's Brigade, Charlie's Crew, Groovy Gibbs Girls, Hunter and Cody's Crusaders, Kennedy's Crusaders, Lindsay's Trailblazers, Moving4Jadon, Peter's Posse and Samantha's Spirit. The event started off with Peter Voskovitch, who has SMA Type III, playing the national anthem on his electric guitar. A special guest and race participant, Charlee Bisch, Miss Missouri Outstanding Teen, helped pass out trophies to all our superstars living with SMA as well as awards to our participants. Charlee's platform for Miss Missouri Outstanding Teen is SMA Awareness.

Due to amazing sponsors, donors and participants, the Cure SMA Race-n-Roll raised just over **\$40,000!**



Missouri

Dear Families of SMA,

On Wednesday, October 5th, 2011, the Volleyball teams from Lutheran High School of St. Charles, Missouri hosted a "Wipe Out SMA" night to benefit Families of Spinal Muscular Atrophy. The volleyball games were played in memory of Seth David Leppien, who passed away from SMA Type I on November 4th, 1992. His sisters, Meagan and Hannah, are on the Varsity Volleyball team. Meagan and Hannah served the first balls of the game. Purple and orange volleyballs were used for the special event.

The Freshman, JV and Varsity teams sold orange and purple t-shirts, headbands and SMA bracelets to raise money for the cause. Anyone who bought a wristband and wore it to school that Friday was able to wear jeans to school that day. The teams decorated the school with Hawaiian signs to advertise the event and handed out leis to everyone that attended the benefit game. Tropical music was played throughout the night. The night began with a beautiful rendition of the National Anthem sung by Abbie Versemann, a student at Lutheran High, and the evening ended with the players and fans joining in a circle to sing the Doxology.

The event raised a total of \$644 and succeeded in raising awareness about Spinal Muscular Atrophy.

*Sincerely,
Jana Leppien
St. Charles, MO*

Nebraska



Elmwood Post Office Fundraiser

This is a photo of my husband, Chris Arnold, and me at the Elmwood Post Office in Omaha, Nebraska with postal employees Todd Farrell and Lisa Swaffer Mason. Todd was our son Wyatt's (SMA Type I) letter carrier! Through their efforts, the employees at the post office chose FSMA as their recipient for last year's Combined Federal Campaign, the long-running program for federal agencies to support charitable efforts. They invited us to the post office to talk to the employees about FSMA and Wyatt, and several of them took home FSMA educational fliers.

*Paula Lavigne and Chris Arnold
Omaha, NE*

MICHIGAN

Chemical Bank 5K & Fun Run

On July 30th, 2011, the Chemical Bank 5K and Fun Run was held in honor of Sydney Potjer. From my perspective the race was huge success. We had a record number of participants: 1,172. Our local news station FOX 17 covered the fundraising efforts with a nice story on the Saturday evening after the race. In total, Sydney's parents, Kami and Tim Potjer, raised \$5,200 for their "Sydney Potjer Never, Ever, Ever Give Up" fund and donated \$1,025 to Families of SMA!

*Jodi Sevigny
Byron Center, MI*



Annual Hot Dog Fundraiser

The Michigan Chapter hosted the 7th Annual Hot Dog Fundraiser for Families of SMA on May 9th, 2011. In total, \$545 was raised!

Blink Triathlon

On June 15th, the Michigan Chapter also coordinated a team for the Blink Triathlon which was held in Grand Rapids, Michigan! In total, \$150 was raised for Families of SMA! Thanks to Thomas Crawford and David Rossi for supporting this event!

8th Annual Flippin' for a Cure

The Armbrustmacher Family and the Michigan Chapter hosted their 8th Annual Flippin' for a Cure Fundraiser at the VFW Hall in Portland, Michigan on November 4th, 2011. Thanks to the support of their family, friends and community, the event raised \$3,406 for Families of SMA! Thank you to the Armbrustmacher Family for your continued support!

Spitzley Family Auction

On December 26th, 2011, the Spitzley Family, friends of the Armbrustmacher's, hosted a Family Auction! In total, the event raised \$448 for Families of SMA in honor of Mallory Armbrustmacher (SMA Type II). Thank you, Spitzley Family!

MINNESOTA

11th Annual Minnesota Walk-n-Roll



The Minnesota Chapter hosted their 11th Annual Walk-n-Roll event on Sunday, September 11th, 2011 at Lake Phalen Pavilion in St. Paul, Minnesota. The morning brought bright sunshine and

mild temperatures - perfect weather for a walk! We were pleased with the great turnout and happy that everyone showed up with a smile on their face! Not only did we have the perfect day for the walk, but families also enjoyed a cake walk, candy guessing game and a raffle. Thank you to

everyone who came out and participated at the walk and for all of our fundraising teams: Team Anco Brothers, Team Ben, Team Bradley, Team Madisyn and Team Sophie. Together we were able to raise over **\$15,000** for Families of SMA!

A big thanks also is deserved by all of our event volunteers who helped the event run smoothly. Your time and effort are truly appreciated and the walk would not have been as successful without your help.

We always love to hear your feedback in order to constantly improve our Walk-n-Roll. If you have any suggestions or comments, please e-mail them to Minnesota@fsma.org. Don't forget to save the date for the 12th Annual Minnesota Walk-n-Roll on Saturday, September 15th, 2012!



18 For Emerson



The 1st Annual 18 for Emerson Charity Golf Tournament was held on August 29th, 2011 in Prior Lake, Minnesota to raise money for Families of SMA in their efforts to find a treatment and a cure for Spinal Muscular Atrophy. We had 74 golfers sign up and participate, along with more than 60 people who just came out for the dinner and auction. In total, we raised over **\$32,000** for Families of SMA. The guest of honor, Emerson, was in attendance doing her best to avoid the crowds, but still had fun riding around the course on the golf cart. We were thrilled with the event's total as well as how much fun everyone had in the inaugural event. Thank you to everyone that came out to support our event.

*Matt and Deb Chambers
Chanhassen, MN*

MINNESOTA (cont.)



Sam's Club Fundraiser

On August 27th and 28th, 2011, the Minnesota Chapter held a cookout fundraiser at the Sam's Club in Fridley, Minnesota. The Chapter set up a tent, table and a grill outside of the store and sold hot dogs, brats and hamburgers with all proceeds going to Families of SMA. Dedicated Sam's Club employees volunteered their time alongside chapter members to help make the event a success! Thanks to the great weather and the support of family, friends and Sam's Club employees, the event had a great turn out. In total, the Minnesota Chapter raised over \$500 to help find a treatment and a cure for Spinal Muscular Atrophy!

2nd Annual William Anco Memorial 5K

There were two main reasons why I started the W.A.M. 5K. Ever since I started running I have wanted to organize a 5K, but I didn't know where I would hold it, where to start, or what the purpose of the race would be. Part of the answer came in 2008 when the high school I work at built a new stadium with artificial turf. Now that I had the where, I was looking for a purpose and I found it through one of my good friends who had two sons that were diagnosed with Spinal Muscular Atrophy. My friend pointed me to Families of SMA as an organization that would use donations that the 5K collected to fund their mission of finding a treatment and a cure. I now had my place and purpose, and then it happened.

On February 11th, 2010, my friend's youngest son who was diagnosed with SMA, passed

away at the age of 21 months. As devastating as this was and still is, I had a feeling this race had to take place. With the permission of my friend and his wife, we moved forward to hold the race and name it after their son, William Anco.

Our 2nd Annual W.A.M. 5K happened on April 30th, 2011 and thanks to the generosity of family, friends and the community, over the past two years, we have raised over \$2,900 for Families of SMA and the Anco Family to help their other son, Ben, and his quest to find a cure! We are now busy planning the 3rd Annual W.A.M. 5K that will be held on May 5th, 2012 at Chisago Lakes High School in Lindstrom, Minnesota.

Scott Buchkoski
Lindstrom, MN



Spaghetti Dinner

On November 29th, 2011, Eileen Saldana hosted the Spaghetti Dinner at the Coon Rapids VFW in Coon Rapids, Minnesota. It raised over \$2,000 to benefit Families of SMA. Thank you Eileen!



South Dakota

Racing to Cure SMA

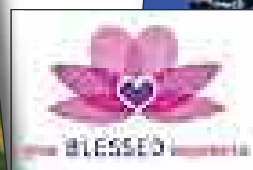
Natosha Voss stepped out to help raise awareness and funds to Cure SMA. She set her sights on a full marathon, and began training in April 2011. While training, she always wore a t-shirt that said "CURE SMA" so everyone could see her purpose. Before she left the house to go on a run, she always told her daughter Denalli that she was going to "run for her." Denalli is four years old, and was diagnosed with SMA Type II at 13 months of age. She is currently unable to walk independently. Denalli loved the thought of her mom running for her and would wait anxiously by the door, anticipating her mom's return.

Training went well, and Natosha was able to raise \$200 before her first half marathon. Shortly after the first half marathon, a jogging stroller was donated. Being as Denalli loved the thought of her mom running for her, she was now going to be able to run with her mommy! Natosha and Denalli trained for two weeks together then ran their first half marathon in unison on September 11, 2011. Denalli absolutely loved the entire 2 hours and 23 minutes it took to run the 13.1 miles. By the end of the second marathon, Natosha was able to raise another \$1,000 - bringing their fundraising total to \$1,350!

Currently Natosha and Denalli are scoping out stroller friendly marathons to run in the upcoming season to raise more awareness and funds to cure SMA. Cute little Denalli is all smiles when the wheels hit the road, and Natosha is more than determined to do everything within her power to Cure SMA within Denalli's lifetime.

Natosha also has an etsy store online (www.oneblessedmommie.etsy.com). The profits of selected items will go to Families of SMA, and will help fund the cure for SMA. Please support the cure, and help spread the word in

everything you do. Because with you, there will be hope!



NEW ENGLAND

Massachusetts

Fundraisers in Honor of Owen Norton

The Flaherty School Coin Drive, held on April 12th, 2011, was organized by Lena Daly of Braintree, MA and raised \$800! A week later, on April 17th, Lena also hosted Norton's Super Sunday Fundraiser.

Through her hard work and the support her husband, JD, and a group of equally remarkable moms from Braintree, Lena raised **\$10,000** in honor of Owen Norton! Thank you to Lena for all of your hard work in organizing these wonderful events!

Dear Families of SMA,

I am pleased to enclose a check for **\$23,000** representing a grant from SmithFest Foundation, Inc to FSMA to allow two families fully paid scholarships to The 2012 SMA Conference in honor of Coby Kulis who lost his battle to SMA Type I on February 27th, 2011, and the difference to be allocated to the Gene Therapy Program. This grant is made possible by proceeds from SmithFest, held on October 1st, 2011.

SmithFest Foundation Inc. is a nonprofit organization with proceeds of Foundation events benefitting local 501(c)3 organizations. Our mission at SmithFest Foundation is to deliver fun and extraordinary charitable events through creativity, passion and a high level of participant service.

Best wishes and continued success in your fundraising efforts.

Sincerely,

*Frederick Smith, Executive Director
Lowell, MA*

Halloween Board Break Fundraiser

At Alpha Tae Kwon Do Academy we hold a Halloween Party every year in honor of Rachel Rollinson. It is a great way to get our community involved in a fun activity. This year we decided, after the costume contest, that we'd try to raise some money for FSMA by having Andrew break some boards. Each board cost \$5 and he would stack them, then break them. The crowd threw their money in the can and the boards stacked up. Andrew broke many with the first strike and the rest with his second. Thankfully no broken bones or bruises! We raised about \$175 in 10

minutes. If we could only raise that much every ten minutes, my 8-year-old thinks SMA would be cured!

Our family will always be grateful to everyone at FSMA. They helped us through a very difficult time and are still there whenever we need them. If we did not have to worry about paying the bills you can bet that we'd be fundraising for FSMA 24/7. But for now we need to settle for the little things we can do to help.

Sending our love and hugs to you all,

*Michelle, Andrew,
Kevin, Rachel (in spirit) and Ryan
Rollinson
Chelmsford, MA*



5th Annual Fall Classic Charity Golf Tournament

The 5th Annual Fall Classic FSMA Charity Golf Tournament was held on October 14th, 2011. Even though the weather did not cooperate, there was a great turnout of golfers for the tournament! The day was extra special because William toughed it out and spent the whole day by the club thanks to his nurses Wendy & Mrs. Williams (his nurse from middle school who volunteered her time to lend a hand!). William even took team photos with each foursome!



Our silent auction and t-shirt sales added to the success of the event, raising over **\$47,500**. Our "Life Is Good" t-shirts this year had the saying "tee it high and let it fly" as well as the FSMA logo, and were available to all participants for a \$20 donation to FSMA.

Help us add to our total! We still have a small supply of this year's Life Is Good Tournament



t-shirts available for a \$20 donation to FSMA. It is a cool green color with Jake golfing and the saying "tee it high and let it fly." The back has the curesma.org logo and 2011 tournament information. They make a great gift- a comfy shirt that helps raise money and spread awareness every time you pull it from the drawer! Sizes S-XXL are still available. To get in on the fashion and good will, contact Heidi Johnson at heidikjohnson@comcast.net.

Thank you to everyone who helped make the day so great and for supporting us over the past five years.

*Heidi & Trip Johnson
Medfield, MA*

Thank you to Heidi and Trip for all of their hard work in organizing this tournament over the past five years. Your dedication to Families of SMA is truly appreciated!

Crafts for a Cure

Heidi and Trip Johnson hosted their Annual Crafts for a Cure Fundraiser in honor of their son, William, on December 30th, 2011. In total, they raised \$164 for Families of SMA! Thank you to the Johnson Family for your continued support!

Thanksgiving Family Reunion Fundraiser

My 12 year old daughter, Julia Mahon, requested donations for FSMA during our Thanksgiving Family Reunion. She raised \$35 with the help of her younger cousins by providing massages and nail polish treatments. My family is close to a young boy, William Johnson from Medfield, MA, who has SMA. I was fortunate enough to work with William as his Physical Therapist in Early Intervention and we have all been touched by William and his fabulous family. Sadly, Julia's younger cousin is also connected with another child with SMA. So, it seemed like a great opportunity to raise some money for a great organization. It is always an honor to support Families of SMA and we are happy to help.

*Karen Mahon
Franklin, MA*

NEW ENGLAND (cont.)

Benefit in Memory of Coby Kulis

On April 29th, 2011, Robin La Tour hosted the Benefit in Memory of Coby Kulis at the Lowell Firefighters Club in Lowell, Massachusetts. Thanks to the support of family and friends, the event raised \$3,655! This event would not have been as successful without the hard work and dedication of Robin! Thank you for your support of Families of SMA!

Joukowsky Fundraiser

On June 11th, 2011, Natasha Joukowsky of Framingham, Massachusetts hosted a family fundraiser. Thanks to her hard work in planning the event, Natasha raised \$400 for Families of SMA! Thank you, Natasha!

12th Annual Connor James McKeon Memorial Golf Tournament

The Connor James McKeon Memorial Golf Tournament held our 12th annual tournament on July 30th, 2011 at Pine Ridge Country Club in Oxford, Massachusetts. The Connor James Memorial Golf Tournament



was started in 2000 in memory of our angel, Connor, who was born on December 18th, 1999 and became an angel on February 21st, 2000. This was another successful CJM Memorial Tournament. We had 98 golfers and an additional 20 people joined us for the dinner all helping us in raising \$2,500 towards finding a cure for SMA. This brings our 12 year total donation for Families of SMA to \$32,500. For the past 12 years, we have been blessed with many family and friends who participate in the golf event to celebrate the life of our son and nephew. The 13th Annual Tournament planning is underway and scheduled for Saturday July 21st, 2012.

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
Putnam, CT*

*Pat and Marissa McKeon, Connor's uncle and
aunt, Worcester, MA*

St. Agatha School's Walk for SMA

On October 28th, 2011, the students of St. Agatha School in Milton, Massachusetts held a walk to benefit Families of Spinal Muscular Atrophy. The students obtained pledges, and then walked laps around the school for FSMA. School staff member Michelle Tierney organized and coordinated the event that was held in honor of 8th grader, Aileen Farrell, SMA Type III. Aileen and her sisters Anna, age 11, and Margaret, age 9, attend St. Agatha School as well. Mrs. Tierney organized and coordinated all pledges, donations, student participation and volunteers for the walk. The children got a surprise when they were visited by Boston Bruins mascot, Blades, and former Bruins player, Bob Sweeney! Blades and Mr. Sweeney took pictures with each classroom, signed autographs and even participated in the walk itself.

The St. Agatha School Walk for SMA raised over **\$10,000!** The Farrell family would like to express their deep gratitude to the caring administrators, teachers, faculty, parents and students of the school community who showed their support in a big way by helping to find a cure in the fight against SMA. We wish to acknowledge the staff at the Boston Bruins Foundation who helped make the day extra special for all the participants. Lastly, we would like to send a very special thanks to Mrs. Tierney for all of her hard work and effort to make this event so successful and FUN!

Sincerely,
The Farrell Family, Quincy, MA

**5th Annual Ride for Alex**

The 5th Annual Ride for Alex was held on Sunday, September 18th, 2011 at Old Rochester Regional High School in Dartmouth, Massachusetts. About 100 people and 35 riders attended the event and enjoyed music, raffles and local vendors including the Minuteman Harley Davidson and Colonial South Chevy from Dartmouth, MA. The weather was great other than the wind which forced us to be creative and use a lot of tape to hold down all the raffle buckets and signs.

Alex and his family had a great time at the event. Alex was able to show the crowd a little of what he can do with his eye gaze computer and was even "vocal" on the microphone! He is an amazing little boy and it was great to show the crowd how much of an inspiration he has been to all that have met him.

In total, the event raised over \$5,000 and \$725 was donated to Families of SMA!

Sincerely,
*Tammy and Scott Foisy
Dartmouth, MA*

NEW ENGLAND (cont.)

New Hampshire

Swing Away for SMA

Swing Away for SMA softball game and balloon release was held during SMA Awareness month on August 27th, 2011 at Greeley Park in Nashua, New Hampshire. There was a great turnout and outpouring of support from family, friends and the community at the event. Attendees enjoyed raffles, face painting and a bounce house! In total, \$623 was raised for Families of SMA in memory of Kamdyn Matthew Lessard. Thank you to Matthew Lessard and Sharon Silva for organizing this event!



Maine

Bella's Benefit for SMA

I am fundraising for my angel, Isabella. I am her voice and educator. My fundraising goals are to teach my community what SMA is, who is affected, and how they are affected. In this tough economy, to provide assistance in any way possible is spectacular. I know when I was travelling roundtrip 70 miles every day to see Isabella in the hospital and living on only one income, it was very tough. I want to help other families in my areas, which are in the situation that I was in, pay an electric bill, or fill their vehicle with gas.

On September 30th, 2011, we held our first Comedy fundraiser coupled with a quilt raffle, and it was very successful. In total, we raised \$1,500. During our comedy event, we also had a table set up with raffle items donated generously by local businesses, including Hair Excitement in Portland, Dynamic Hapkido Defense in Gray, Maine Lobster Boys/Tucker C. Jordan, and Howard Johnson Hotel/Seasons Bar and Grille to name a few. Informational flyers on SMA were left on all comedy tables, and a few people did come up to us to talk and learn more about our story.

Future events to be planned include another comedy fundraiser on July 27th, 2012. Stay tuned to the New England Chapter website for details. If you are interested in volunteering at any event you may contact me directly at (207) 432-1087.

Thanks,

*Sarah Sliter
West Newfield, ME*



Rhode Island

2nd Annual Working on Walking Golf Tournament and Dinner

On August 8th, 2011, the Smith and Silva families joined together and hosted the 2nd Annual Alyssa Silva's Working on Walking Golf Tournament which was held at Louisiquett Golf Club in North Providence, Rhode Island. Friends and family gathered to support fundraising efforts for Families of Spinal Muscular Atrophy. Sixty eight golfers enjoyed a beautiful day with their choice of playing a nine hole or 18 hole format. In addition to our golfers, we were also fortunate to have thirty-three hole sponsors! The tournament was enjoyed by all.

The celebration continued at Twin River Casino and Event Center with 165 people gathering to support this worthy cause. Music, raffles and a silent auction created an evening of festivities. The Smith and Silva families have been blessed with the support of so many people embracing this cause. The generosity of our friends and family is truly amazing. Over the past two years, we have been able to raise over \$10,000 to support the efforts of Families of SMA.



NEW MEXICO



On-going Events:

Bike Logan

Holly Agajanian and her partner Virginia are selling their house and going on a year long bike tour! Their plan is to ride in ten mountain bike races and raise funds in honor of Logan Ragland. To make an online donation and to check out their race progress, please visit www.fsma.org/bikelogan.

NORTHERN CALIFORNIA

1st Annual Sacramento Walk-n-Roll

When Nina Daya and her Cisco co-workers set their sights on hosting the first Walk-n-Roll for Families of SMA in Sacramento, they had no doubt it would be a rich and rewarding experience.

In three short months, Cisco employees Emily Klover, Maddy Dunham, Demiah Andreotti Bik, the larger Cisco community, Nina's family, and those touched by SMA and surpassed their \$10,000 goal, raising **\$10,672** for Families of SMA. The total revenue will make significant difference in the fight to end SMA and points to a "pent up demand" for more events like this one in the Sacramento region. Cisco employees quadrupled the amount they raised last year and provided an opportunity for those touched by SMA to gather together to raise funds and awareness of this disease.

For Nina and her family, some of the highlights of the planning process came in the form of very moving emails and personal messages from strangers sharing loss, hope and enthusiasm for the event. When the first email from a young mother who lost her first child to SMA reached her inbox, Nina stated, "I would have hosted the walk for one family member."

The Cisco team is also grateful for the support of the Monterey Trail High School Key Club for their participation. We had many compliments on the event's organization thanks to our wonderful volunteers' "can do" attitude.

Thank you to our many local sponsors (pictured below) who supported the walk and contributed to our success. Also, a big thanks to Erin Quinonez from the Department of Parks and Recreation for helping us with the permit process to secure our walk venue and for answering all our questions along the way!

We are eager to start planning our 2nd Annual Walk-n-Roll at Land Park on October 14th, 2012 and welcome other SMA families, friends, healthcare workers, and the larger community to consider volunteering and/or fundraising early next year.

Upcoming Events

Please join the Northern California Chapter for our upcoming events:

- Pathways of Promise will be held on Saturday, March 31st, 2012 in Sacramento, California. For more details on the event, please visit www.fsma.org/pathwaysofpromise.
- Concert for a Cure will be held on Saturday, May 12th, 2012 in Danville, California. For more details and to reserve your tickets for the event, please visit www.concertforacure.org.



OKI (OHIO, KENTUCKY AND INDIANA)

Indiana

Hamilton Heights High School Honor Society Service Project

On September 6th, 2011, the Honor Society at Hamilton Heights High School in Arcadia, Indiana put on a fundraising and awareness event for Families of SMA. Alexander Davis, SMA Type II, visited the event for a picture session with the Honor Society officers. Everyone in the school was impressed by the luminary display that was created by honor society members. Most importantly, everyone was asking questions about Spinal Muscular Atrophy! In total, the event was able to raise \$170 for Families of SMA- outstanding for a small school community! It was one of the Honor Society's most successful events to date and they are planning on making it an annual event! I think one of the most touching moments for me was when one of my sophomore boys came to class holding up his SMA bracelet with a huge grin on his face. I think he gave up his lunch money to be able to donate. I compare this moment to two years ago when my husband's doctors at the Indiana University Medical Center all said "SMA—what's that?"

Julie Davis, Grandmother to Alexander & English Teacher at Hamilton Heights High School, Arcadia, IN



Upcoming Indiana Walk-n-Roll on August 18th, 2012. For more info visit www.fsma.org/inwalknroll.

OKI (cont.)

4th Annual Cody Munz Memorial Golf Scramble

The 4th Annual Cody Munz Memorial Golf Scramble was held on September 10th, 2011 at Wood Wind Golf Course in Westfield, Indiana. There were prizes for the 1st and 2nd Place teams, a 50/50 raffle, as well as longest drive and closest to the pin contests! Thanks to the generosity of family, friends and event participants, \$4,000 was raised for Families of SMA. Thank you to Rhonda and Matt Munz for organizing the event as well as for your continued support of Families of SMA!

Ohio

Lyden Ribbon Sales

For SMA Awareness month my sister Emily and my cousin Amanda came up with the idea to make ribbons and put them at local businesses in our area to raise awareness and money for Families of SMA. We made ribbons and distributed them around our area with canisters asking for donations and giving a description of why we wanted to raise the money. After losing my daughter Aubrey in April 2011 to SMA Type I. I felt the need to do something to make a difference, even if it was just a small one. I always have the support of my family and they come up with some good ideas and suggestions for fundraising. We want to try to do anything we can to make some kind of difference so hopefully someday a cure can be found. I think every penny helps us to get closer to that goal.



We are from Youngstown, Ohio and not many people know what SMA is. We didn't know what it was either before Aubrey's diagnosis. I think doing this fundraiser helped to make people more aware of what SMA is and that it can happen to anyone, anywhere. That is a step in the right direction. I am proud to say that our fundraiser raised \$600 that was donated to FSMA. We are more than happy to help in any small way that we can to help make a difference and put an end to Spinal Muscular Atrophy in memory of

Aubrey and for all of those who are affected by this disease.

*Dannete Lyden
Boardman, Ohio*

Ricky's 1K Fun Run

On July 23rd, 2011, the town of Liberty Center, Ohio hosted this 1K Fun Run at Liberty Center High School in memory of Ricky Roell. Thanks you to Kendra Roell for helping to organize the event as well as to all of the runners who participated! In total, \$700 was raised for Families of SMA!

Emma and Nick's Lemonade Stand

Every Wednesday throughout SMA Awareness Month in August, the Lockwood Family hosted Emma and Nick's Lemonade Stand! In total, they were able to raise \$190 thanks to the support of family, friends and their community. Their efforts were even featured on their local WCPO News Station! Congratulations to Emma and Nick for inspiring others in your community and creating SMA Awareness. Thank you to the Lockwood Family of Cincinnati, Ohio for your continued support of Families of SMA!

Dear Families of SMA,

For the past six years, I have held a Pampered Chef Fundraiser in the month of December. I do this in honor of my twins, Owen and Grant, who were born December 28th, 2004. It is hard to believe they would have been seven this past year. Unfortunately, SMA Type I took their lives way too soon (Owen at 11 weeks; Grant at 15 weeks).

Jeanette Arnold, Pampered Chef Consultant, has once again donated her profit from the sales along with the Pampered Chef Company totaling \$350, a \$50 cash donation from my boys' Great Aunt, as well my donation totaling \$450 this year.

I know nothing will bring back my beautiful Owen and Grant, but I know the struggles and pain that SMA families face. One day we will no longer have to lose our loved ones. Thank you for all the work that you all do for the SMA cause!

Sincerely,

*Vanessa Burns
Marietta, OH*

Dear FSMA,

I hosted a Premier Designs Jewelry Party on August 13th, 2011 and my good friend Jan Hastings (a Premier Designs Representative) donated part of the proceeds to help our fight with SMA. Please accept this check for \$50 in honor of Zander and Lexie Helfrich. Thanks for everything you do for all affected families.

Sincerely,

*Ryan, Diana, Zander and Lexie Helfrich
Medina, OH*

Premier Designs Jewelry Party

Julie Burgess of Wadsworth, Ohio hosted a Premier Designs Jewelry Party for her family and friends on October 5th, 2011. In total, she was able to raise \$805 for Families of SMA in honor of her children, Caleb and Olivia, both with SMA Type II. Thanks for all your hard work, Julie! Julie is also planning another fundraiser, the Ski Show, Cornhole Tournament & Auction on July 8th, 2012. Check out www.fsma.org/OKI for more details!

Put-in-Bay Fishbowl Poker Run

On October 15th, 2011, Jennifer Blumensaadt hosted the Poker Run at the Fishbowl in Put-in-Bay, Ohio! In total, the event raised \$286 for Families of SMA. Thanks for all of your hard work in organizing this event in honor of your son, William!

SMA Fundraiser & Awareness Event

Friends of Keith and Hillary Schmid, Jennifer and Jeff Bachelor, worked very hard organizing an SMA Event in Ohio in memory of Zane! It was held at Shenanigans Pub & Grub in Newark, Ohio. Ten percent of each bill, in addition to raffle proceeds, totaled \$911 raised for Families of SMA.

They organized a table filled with giveaways, SMA information pamphlets, raffle items, and pictures of Zane. This 12-hour SMA Fundraiser & Awareness Event was a great success!

Thank you to Jennifer & Jeff and all those who turned out to make this event GREAT! We appreciate your hard work and what you've done for all those affected by SMA.

OKI (cont.)

Kentucky

Bomb Away SMA



In early August of 2011, Beth Pruitt (Dani's mom- SMA Type I) called me one night. She was bummed we were not doing a Walk N Roll for the year, but she had thought of a fundraiser and wanted to share her idea. Beth asked me if I had ever heard of a money bomb. Not knowing what a money bomb was, she explained to me that a politician had raised millions of dollars within 24 hours in donations for his campaign. So I was definitely interested after listening to Beth's idea and searching the term "money bomb" on the internet. I told her I would call the FSMA office the next day and get things rolling. We set up a Facebook event page, emailed our family, friends and those that participated in the 2010 Walk N Roll. We were on it. Our money bomb took place on

Saturday, August 14th, 2011, which would have been the day of our annual Walk N Roll. We contacted every person we knew to spread the word of the money bomb and get as many people as we could to donate any amount of money to FSMA. We collected checks for those that could not donate online. By the time the "money bomb" was over we had raised over \$4,000. This was amazing considering the little time it took to get this event going. The FSMA fundraising team made this possible by setting up our page on their site, adding pictures, and details to help those donating understand why it was (is) needed. We would like to thank FSMA for helping us pull this together within a matter of days. Who knows, maybe this will become an annual event!

Shana Foley
Lexington, KY

Grimes Family Fundraisers

Thank You to UAW Local 2164 for hosting Region 3 Women's Conference and giving us the opportunity to share our story and raise SMA awareness! These hard working women of The General Motors Plant in Bowling Green,



Kentucky and surrounding states donated to several charities including \$598 to Families of SMA in Annie's memory! We were able to share our story, educate others and meet new friends. 105 women were in attendance, on October 22nd, 2011.

In addition, Annie Girl Cookbooks were a great hit over the past few months! Thank you to Shelia "Nana" Violette for creating this project and raising SMA awareness. The cookbooks featured over 300 recipes, color pictures of Angel Annie Grimes and SMA facts. We were proud to sell 335 cookbooks raising \$2,310 for FSMA in Annie's memory! Cookbooks can still be ordered by visiting www.4AnnieGirl.com.

Nicole and Eddy Grimes
Bowling Green, KY



PACIFIC NORTHWEST

On Sunday October 16th, 2011 we held our **4th Annual Walk-n-Roll** at Greenlake in Seattle, Washington. 400 walkers set out on a chilly fall day for a walk around the lake. This was our best year yet with over **\$17,000** raised! After our walk several families and friends met for a picnic and began plans for next year.

On November 13th, 2011 Kelly Hargrave of Port Orchard, Washington and Gene Cysouw hosted a **Stacy McBride Collections jewelry party** to benefit FSMA. Stacy McBride consultant, Rebecca Sorter, donated all proceeds from the party to FSMA. She brought lots of beautiful jewelry and purses for the open house. Guests shared wonderful food and tried on jewelry. Rebecca donated \$200 to FSMA.

Wreaths of Hope Fundraiser

John and Sybil Kuhn of Centralia, Washington hosted the Wreaths of Hope Fundraiser! Thanks to their hard work, they raised \$880 for Families of SMA!



PENNSYLVANIA

Greetings from the Pennsylvania Chapter!

As we welcome 2012, we are excited to build upon the great success our Chapter has had over the last year . . . fundraising for a CURE, supporting each other, and raising awareness about this horrendous disease. In that united spirit, we would like to also welcome the Sniezek, Mohammed, and Caspi families to our local community! TOGETHER WE WILL FIND A CURE!

Please take a look at what our amazing families have achieved these past six months and what's in store!

Emmy's Family Fights SMA

The 6th Annual Emmy's Crop For A Cure

Every March for the last five years, Brandy Baugher, mom to Angel Emmy Rose, and Sandra Cromer have hosted the Annual "Emmy's Crop for a Cure," an all day scrapbooking event. In an effort to increase family involvement, they are considering a Walk-n-Roll, instead of the Crop for 2012.

The 5th Annual Emmy's Crop for SMA kicked off on March 12th, 2011 at the old Parkville Fire Company in Hanover, Pennsylvania. Almost 100 women scrapbooked from 9am to 9pm while enjoying time with family and friends. Scrapbookers enjoyed great food and great prizes throughout the day. The women participated in free make and take classes, best page contests, silent auctions and live auctions throughout the event . . . creating awareness for SMA.



Participants also enjoyed free mini Zumba classes and massages! With help from friends, family, and Crop supporters, this year's event raised \$8,000 to help find a treatment and cure for Spinal Muscular Atrophy. Great job ladies and best of luck with your Walk-n-Roll!

Hanover Eagle Benefit Ride

Emmy Rose's family was very busy last year planning events in Emmy Rose's name. In May, Joyce Kromer of Hanover, Pennsylvania organized the Hanover Eagle Benefit Ride and raised \$500 toward a CURE! Many thanks to those who came out and rode to support this wonderful cause.

Fundraising by Sweet Baby Zane

The 3rd Annual Zane's Run

On September 25th, 2011, Hillary and Keith Schmid hosted their 3rd Annual Run in Malvern, Pennsylvania in memory of their daughter, Zane. The first two years were very successful, growing in both funds raised and participants. This year, with the continued help from their amazing friends, Meridith Bebee and Carrie Kane, they exceeded all expectations . . . raising **\$20,781**! It was a fantastic turnout especially with the newly added 5K Race. Runners came to compete, and left schooled about SMA! And hats off to Zane's dad, Keith, who designed the shirts himself this year! Looking forward to racing with the Schmid's at the 4th Annual Zane's Run this coming Fall.

McElwee Dinner Party

From the moment of Zane's diagnosis, the Schmid's speak of the support received from family and friends. But they admit surprise at the outpouring of gestures from complete strangers . . . cards, meals, money, flowers, emails, and even a beautiful cherry blossom tree! It came down to strangers being affected by Zane's story, and the simple act of caring about others.

Last Fall, Tom and Katie McElwee of Conshohocken, Pennsylvania were these compassionate strangers. They learned about Zane and the effects of SMA through a family member and reached out to inform the Schmid's that they had ALREADY hosted a party in Zane's name.

At their event, they handed out Sweet Baby Zane bracelets, shared Zane's story, and spread awareness about SMA. While speaking, they passed a donation bucket around to the partygoers. They raised \$700 in donations and then Tom & Katie matched the proceeds . . . raising the final total to \$1,400!

Hillary remarked: "They sent me a kind note, encouraging me to keep up the fight. I sat there and thought (again) of how proud I am of Zane for being the person she was to affect people so much. Kind words, thoughts, and actions do make a difference whether you know someone or not. Pay it forward!"

Fundraiser for Julie Masters

In September 2011, Devon Health in Kings of Prussia, Pennsylvania hosted a Fundraiser honoring Julie Masters. Again . . . we see people coming together to support our mission to cure this horrendous disease. Friends and supporters of Julie rallied and raised \$355 in her name. Thank you Devon Health for stepping up, raising necessary funds, and most important, raising awareness as we seek to CURE SMA!



PENNSYLVANIA (cont.)

The Lyla Mertz Foundation

Dance Away SMA

On October 1st, 2011, the family of Lyla Mertz hosted the 2nd Annual Dance Away SMA Fundraiser. There were 162 people in attendance, and fun was definitely had by all! Including the Mertz Family, six families from the FSMA PA Chapter were present in the house! This event featured a delicious dinner and live music by the Chas Band. Aside from a crowded dance floor, participants also enjoyed a chinese auction displaying over 70 items.



So many photographs of children faced with the challenge of SMA were prominently displayed along the walls. Pertinent SMA facts accompanied the children's posters, along with a "Bags and Aprons for Lyla" kiosk open for business.

Dance Away SMA raised a total of \$6,137 that night! Way to go Jen and Steve Mertz!

Bags and Aprons for Lyla

"Bags and Aprons for Lyla" are purses and aprons that are handmade by Lyla's friends and family. They currently offer numerous styles of bags, all of which are named after Lyla's Care Team during her stay at The Lehigh Valley Hospital. Each bag has a tag with a ladybug button that says "Bags for Lyla" on one side and "Love you Little Angel" on the opposite side. The aprons are equally unique, sewn together with love (and beautiful fabrics).

Since the last report, the Lyla Mertz Foundation has sold bags and aprons for a total profit of \$1,376. The demand for these beautiful items still remains high, so the Mertz's

continue to sell them with the goal of raising funds and awareness for SMA.

Flea Market Proceeds Benefit SMA Research

Last November, Easton residents enjoyed rummaging through treasures at the Easton Area Senior Center Flea Market. It wasn't an ordinary weekend flea market, but one dedicated to a philanthropic cause. All proceeds from the table rentals and the food sales were donated to the Lyla Mertz Foundation. At day's end, a total of \$659 was raised and donated to Families of SMA!

Silpada Party Jewelry Party and Dress Down Day

November 2011 was a busy month for the Mertz Family. Mid month brought the 2nd Annual Silpada Jewelry Party... a great opportunity for women to enjoy some quality time with friends while shopping for beautiful jewelry. Jen Mertz promoted the actual Event, as well as, a continued online opportunity to order items for those unable to attend.

Also last fall, led by Lyla's grandmother, the employees of First Niagara Bank in Lehigh, Pennsylvania held a "Dress Down Day." Each employee paid \$5 to wear their "Team Lyla" tee shirts to help spread the word about the effects of SMA.

These events jointly raised over \$400 donated to FSMA in Lyla's memory.

Lukie's Family Fights to Cure SMA!

The Proctor Wedding Dollar Dance



Congratulations and thank you to Jessica and Thomas Proctor who were married on Saturday, October 8th, 2011. Jessica (Joe Maida's stepsister) and her husband Tom volunteer each year at Lukie's Fall Festival and are dedicated to helping us CURE SMA. They dedicated a special Dollar Dance at their wedding to benefit our cause. When the

Dollar Dance was ready to begin, they asked their guests to open their hearts (and wallets) and make a donation to Families of SMA in Memory of Lukie. They raised \$115 for Families of SMA!

Spirit Day at Bangor Middle School



On October 6th, 2011, the Bangor Middle School Cheerleaders of Bangor, Pennsylvania held a "Spirit Day" for students and faculty. The faculty donated money to be able to dress down and the students paid one dollar to wear hats during school. The middle school faculty and students raised \$307 for FSMA!

The Cheering Squad also made daily announcements the week leading up to the Spirit Day, and passed out brochures encouraging students to attend Lukie's Fall Festival on October 8th, 2011. While promoting Lukie's Festival, they simultaneously raised awareness about the disease and what SMA is all about! The cheerleaders did a wonderful job and were proud to support their fellow cheerleader Kadyne Maida, the Maida Family, and the cause.

SMA Families Unite to Fight SMA

At the 8th Annual Lukie's Fall Festival, Tara Maida and Christina Mohammed joined forces! Christina Mohammed, amazing Mom to SMA Warrior Omar Hardy, sold beautiful jewelry as her way to help find a cure for SMA. Christina set up a table to sell the jewelry and succeeded in raising \$133 for Families of SMA!



PENNSYLVANIA (cont.)

Lukie's 8th Annual Fall Festival

On October 8th, 2011 the 8th Annual Lukie's Fall Festival, in memory of Luke Anthony Joseph Maida "Lukie," raised \$8,646 for Families of SMA. The weather was a picture perfect autumn day in the Pocono Mountains of Pennsylvania. The Maida's had the largest festival attendance to date, and there were smiles everywhere! During the opening ceremony, Lukie's family remembered their precious Lukie, honored all the SMA Angels and Warriors, and thanked everyone for their continued love and support.

The day delivered lots of entertainment, live bands, clowns, irish dancers, magicians, mascots, DJ, blood mobile, bagpipe, drum corps and cheerleaders. The moon bounce houses, hay ride and carousel were continually filled with kids having a wonderful time. The annual pie-eating contest resulted in messy faces and many laughs! Lukie's Grandma even entered this year.



The children were eager to play all the games, see the firemen, have their faces painted, hair colored or braided, watch the entertainment and most of all, have fun with their families. Inside the community hall, there were



approximately 100 raffle prizes... something for everyone. Lukie's Pumpkin Patch was filled with pumpkins, gourds and mums that children decorated. There was even a booth offering FREE BOOKS for the kids.

Tara Maida mentions all the good food, baked goods and candies, and how honored they were to host Families of SMA President, Kenneth Hobby: "His presentations about SMA and the progress towards a cure were very informative, and gave festival attendees a better perspective of our cause. Everyone agreed that it was a fantastic day & can't wait till next year for the 9th Annual Lukie's Fall Festival, Saturday October 6th 2012."

May Naish and Joia McGivern's Birthday Celebration

Abigail Negrin, daughter of FSMA Pennsylvania Chapter President, Karen McRory Negrin, was May Naish's little friend. May would come visit and play with Abby who suffered from SMA Type I.

Since Abby lost her battle with SMA, May has consistently volunteered at the Annual Chapter Walk and has recruited people to walk with "Abby's Army."

This is where Joia comes in. After learning about Abby's story, Joia decided to join May and ask that in lieu of birthday presents, guests instead donate to FSMA in memory of Abby. All told, \$440 was donated to FSMA! Thank you girls for such generosity!



A "Mitzvah" from the Liberman Children

Jacob and Sydney Liberman of Radnor, Pennsylvania donated \$3,000, a generous portion of their gift money totaling from their B'nai Mitzvah to Families of SMA in honor of their good friend Peter Henkel. The Liberman family has been especially kind to Peter and supportive of Families of SMA. Jacob and Sydney's father has printed and donated beautiful FSMA banners to hang in Philadelphia for the PA Chapter Walk-n-Roll. Peter's Mom and PA Chapter Vice President, Allyson Henkel, is so grateful for such thoughtful friends!

"Fight for Ford" Events — Raise Funds & Awareness



Ford Sniezek was diagnosed with SMA Type I on February 3rd, 2011 at four months of age.

Since his diagnosis, his family has rallied around him in an effort to raise money to offset Ford's medical expenses, as well as to raise awareness and funds for SMA research. Many members of Ford's family and friends have joined together to organize fundraisers under the united name "Fight for Ford." These fundraisers are intended to pay for medical expenses not covered by insurance, as well as to fund Families of SMA that supports Dr. Kaspar's Gene Therapy Program.

Fight for Ford, Man vs. Machine

Ford's Aunt Jenn Henderson and Grandfather Fran held a fundraiser called 'Fight for Ford, Man vs. Machine' on September 24th, 2011 which through many blessings turned out to be a beautiful day after the forecast called for rain . . . and more rain. This fundraiser was held at Moraine State Park in Portersville, Pennsylvania and featured an auction, a live band, food, games and even an appearance from precious Ford. Ford's Grandpa was also seen racing a motorboat in his kayak! This event was very successful, raising \$400, and many asked for it to become an annual tradition.



PENNSYLVANIA (cont.)

Fight for Ford Spaghetti Dinner



On the very next day, September 25th, 2011, Ford's other Aunt, Meghan Nhar, hosted a Spaghetti Dinner at the Warwick Volunteer Fire Hall in Warwick, Rhode Island. A large crowd came to feast on spaghetti and have a great time. This event also featured an auction and raffles as well as a DJ, games for the kids, and clowns. The organizers set up a card-making station for those interested in creating handmade cards to be included in the FSMA Newly Diagnosed Care Packages. The Sniezek family is extremely grateful for the hard work dedicated to this event that raised \$300... and so much AWARENESS!

Corn Hole Tournament



Friends of Ford's family (the Beachem's, Plukard's and Volland Families) also wanted

to show their support! They decided to organize a "Corn Hole Tournament" on October 8th, 2011 at a local sportsman club near Butler, Pennsylvania, and donated \$500. Along with the Tournament, there was also food donated by a local market, bake sale, raffle drawing and auction. Ford's dad was able to attend this event to express the family's gratitude for all the community's love and support.

Fight for Ford Dinner & Auction

Finally on October 22nd, Ford's Grandparents Rev. Tom and Dee Henretty, along with other family and friends from Erie, PA also held a Fundraiser at the Lakeview Community Church. Many families and community members enjoyed food, drinks and desserts. This event featured an Auction and another card making station where cards of encouragement were created for newly diagnosed families. Because of Skype, Ford and his parents were able to share in this event.

The Sniezek's were so excited to have been able to express their thanks for all those who came out to the event and enjoyed sharing Ford's story with a local News station!

The PA Chapter sends their deepest condolences to the Sniezek Family for the loss of Ford's heroic Grandpa, Dennis Morris.

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

In Friendship,

The PA Chapter of FSMA

"The Sniezek's have been extremely blessed with so much love and support from their local community, as well as family and friends all over Western PA and other states. No one we knew had ever heard of SMA until our son was diagnosed. Through all of these events, thousands of people are now aware of Spinal Muscular Atrophy. Thanks to the efforts of family and friends, a total of \$15,000 was raised in honor of Ford, with \$1,500 going to Families of Spinal Muscular Atrophy. The Sniezek's and their families will continue to make it their goal to raise as much awareness and funds for SMA as possible so that a cure will be discovered soon! We also continue to trust God and thank him daily for providing for Ford's every need."

- Kayla Sniezek

Bosco's Friends Helping Friends Fundraiser

Bosco's Department Store hosted the popular FRIENDS HELPING FRIENDS to benefit area non-profit organizations on Tuesday, October 4th, 2011. Every 501(c)3 non-profit organization is eligible to participate and is supplied, free of charge, with \$5 tickets to sell. Everyone who purchases a \$5 ticket receives a 25% discount shopping pass for purchases made only on Friends Helping Friends day and are eligible to win fabulous door prizes including shopping sprees as well as, valuable merchandise items. The non-profit organization keeps the entire \$5 from every ticket sold.



This is the second year that Altoona, PA's "Friends and Family of Eden Cheslock" participated in the event at the Altoona Boscov's store. Fifty-six non-profit organizations participated. We sold 150 shopping passes and received \$55 in donations for a total of \$805 raised for FSMA in honor of two year old Eden Cheslock of Dover, Delaware.

Jeanette Foor, Eden's Grandmother
Altoona, PA

Little Flower Violet Fundraisers



In the spirit of SMA Awareness Month in August and in memory of my daughter, Violet Wehrkamp, a Mary Kay fundraiser was held on August 13th 2011. My Mary Kay beauty consultant, Jess Corcoran, provided

facials and makeovers. We featured fun "mock"-tails and tea sandwiches for our guests and people stopped by throughout the afternoon to be pampered. 20% of the sales from the day are being donated to Families of SMA. The total donation from sales for the

PENNSYLVANIA (cont.)

day of the event was \$200 and there was an additional \$56 in donations. Online sales are being taken until September 1st.

Also, for the Pennsylvania Chapter Walk-n-Roll, my Little Flower Violet hair clippies and blankies had a total donation to FSMA of \$400.

*Alison Wehrkamp
Effort, PA*

Texas Roadhouse Gift Card Fundraiser

On November 28th, 2011, Emily Kapp of Mechanicsburg, Pennsylvania hosted the Texas Roadhouse Gift Card Fundraiser in Mechanicsburg, Pennsylvania. Emily was able to raise \$134 for Families of SMA in honor of her boyfriend's nephew, James Sidelinger, and created awareness for SMA in her community!

CTPA Phillies Game Fundraiser

The Cheltenham Township Police Association in Cheltenham, Pennsylvania hosted their fundraiser on September 30th, 2011 and raised \$350 in honor of Gavin Crews! Thank you to all who participated and the Kevin O'Donnell for organizing the event!

Dear Families of SMA,

Each year my Social Work Club at Saint Francis University runs a golf outing for FSMA. We did the same this year. When I told my sister, Thomasene from Carrollton, Ohio about FSMA and your work and that I was disappointed about our tournament, she spoke with her husband Dallas and they decided to donate \$1,000 in honor of Lilian Kennedy.

My club and I will continue to try and do our best to support FSMA.

*Sincerely,
Mark Lynch
Seward, PA*

ROCKY MOUNTAIN

Colorado

11th Annual 5K Cure SMA Walk-n-Roll & Run Colorado

We could not have asked for a more beautiful fall day. Overlooking the lake at Clement Park in Littleton, Colorado, you saw a sea of red T-shirts inscribed with "Got Hope?" all to celebrate in the 11th annual Rocky Mountain Chapter "5K Cure SMA Walk-n-Roll & Run" held on Saturday, September 10th, 2011!

With over 300 attendees, this year's event was our most successful Colorado walk to date raising over **\$42,000!** A heartfelt thanks goes to the many donors, generous sponsors, dedicated participants and hard-working volunteers. You are what makes our event a success each year!

Our 2012 event is set for Saturday, September 8th, 2012. Challenge yourself - set your fundraising goal a bit higher and get started collecting donations early! Call or email Julie Lino if you would like more information about our Walk-n-Roll (303) 936-5684 or LINOBJ@Q.com. Register, donate, sponsor now! Utilize the FSMA site to create a team page at www.fsma.org/ColoradoWalk2012 today. See you in September! J

2011 Cure SMA Walk-n-Roll & Run Colorado



Thank you for making a difference!

ROCKY MOUNTAIN

Rocky Mountain Chapter MEMORY LANE:



**** Attention new and old RMC SMA families ** If you would you like an In Honor Of or In Memory Of lawn sign made and displayed at our RMC local events, please send a .jpg photo of your SMA loved one to LINOBJ@Q.com. We display them at our golf tournament and Walk-n-Roll.**

Dear Rocky Mountain Chapter,

I wanted to share with you a photo of Alex's tree that was given to us by the chapter two years ago at the Colorado Cure SMA Walk-n-Roll & Run. With permission from the City forester in our hometown, we planted it right away on his birthday in the park just a block from our



house, and it's been growing ever since! We can actually see the tree from our house. When we walk by it we always say to the tree, "Send your roots down and your branches to heaven."

This year for Alex's birthday we finally had a stone made for its dedication. Attached is a photo of the tree and then one with his brother, Jack. I particularly wanted to share it with you because we acknowledged FSMA as a way to raise awareness and as thanks from our family for the support.

Melisa Devincenzi and family
Alex SMA Angel Type I (10/22/08 -
4/12/09)
Louisville, CO



Montana

Veronica St. Onge's Birthday

Dear FSMA,

Enclosed is \$785 that was raised from Veronica's 7th birthday celebration. Money was donated in lieu of presents as well as from the now famous Annual How Creek Duck Race! Everyone had a wonderful time and we educated several more people on what SMA is all about. As always, thank you for your support and dedication to the SMA Community.

Sincerely,

Debby St. Onge, on behalf of Veronica Rose, age 7, SMA Type I
Bigfork, MT

SOUTHERN CALIFORNIA

Hello from sunny Southern California. There has been a lot happening within the Chapter and we are so excited to share with our SMA Family. First we would like to take this opportunity to introduce ourselves. My husband Rickk Montoya who recently assumed the role as Chapter President and I, Autumn have been involved with Families of SMA for well over five years. Together we are the proud parents of our eight year old son, Kennedy (Type II). We have been very committed to the FSMA community and are both so excited to take on this wonderful opportunity. Since taking on this responsibility we have met plenty of new families and have enjoyed hearing their stories. We have such a strong Southern California SMA Family and are so happy to be able to call them our "Family"

We've been very busy with lots more heading our way. In October we had our "Dave and Busters" fundraiser, "Meet and Greet" dinner. We were so happy with the turn out. It was so much fun watching the kids play games together and seeing the families sitting together sharing their lives. Dave and Busters so generously donated 10% of the entire restaurants earnings and gave us a check for nearly \$400.

Also in October a few Southern California Families gathered for a dinner hosted by a local Olive Garden restaurant to help with their disability awareness month for their employees. Dinner was yummy and more importantly, bonds were built. Thank you everyone!

We were so blessed as a Chapter to host the first annual SMA kids

Holiday party in December. We had over 15 Families come and enjoy an afternoon of hot chocolate and holiday sweets. Holiday arts and crafts, Holiday bingo (with some fun prizes), and no kids party is complete without the Wii. The kids had so much fun playing with each other and the adults enjoyed the company of "Family" being together for the Holiday season. We even had a surprise guest show up... .SANTA! He brought every child a gift and he even received a "Santa" letter from one of our little guest. We are so thankful for all that came and enjoyed this day with us. Also at the event raffle tickets were sold for a beautiful quilt donated to the Chapter from a local quilting club. The winning raffle ticket will be drawn on February 14th and so far it has risen over \$850.

As a chapter we have also been the recipient of many donations from local companies such as Singer Lewark Accountants & Consultants in connection with their "Casual Friday" fundraisers, as well as employee designated contributions from others such as IBM. In the last three months alone over \$1,000 has been raised from these sources. We are extremely grateful for the support we have received from these and other local organizations and businesses.

There is so much more in the near future for us. In February we hosted another Dave and Busters meet and great dinner in San Diego to meet some new Families and raise funds and awareness for SMA. On every Thursday in April, all 15 Southern California Hooter's locations will be hosting a SMA Day, in which every supporter with a flyer will have

SOUTHERN CALIFORNIA (cont.)

20% of their bill donated back to FSMA. Also, Evelyn Vasquez and her Inland Empire team will be hosting a "Walk and Roll" sometime late spring. We are also planning a family picnic and skydiving fundraiser in mid-August. Details for all these events will be shared as soon as they are available. We ask that all our families keep an eye out for our email announcements. If you haven't received any recently, we suggest you either call the chapter or email us with your current information at scalif@fsma.org

We're so proud and happy to see what this family has accomplished. Together we will all continue to work hand in hand in our goal of finding a cure.

*Rickk and Autumn Montoya
Garden Grove, CA*

Orange County Mustang Club Fundraiser

Renee King, Grandmother to eight year old Kennedy Montoya, is the secretary of the Orange County Mustang Club in the County of Orange in California. The club has three car shows a year and there is always a 50/50 Drawing for SMA. Kennedy is the one to pick the winning ticket and present the gift to the winner. The club raised almost \$800 in its first two shows this year! At the second show, the Surf City Garage Car Show in Huntington Beach, the gentleman who won donated the money back to Families of Spinal Muscular Atrophy.

Rickk and Autumn Montoya, Kennedy's parents, also set up a table with information and sold baked goods to raise money. Rickk said, "We feel we accomplished our mission of putting a face to the FSMA name. For so many years now, the Orange County Mustang Club has been donating proceeds from their car shows, yet other than seeing Kennedy in attendance, they knew very little about the disease. Well that has changed now. Many of the members strolled by our Awareness table en route to Autumn's treats and either asked a few questions or unexpectedly got an earful."

Thank you to Renee, Rickk, Autumn, and all of the members of the Orange County Mustang Club for your continued support of Families of SMA and your dedication to raising funds and awareness in honor of Kennedy.



IE All Star Jam



The IE All Star Jam was held on Sunday, October 16th, 2011 at the Citrus State Historic Park in Riverside, California. The venue was a perfect setting for family and friends to come together and enjoy a concert all while raising funds and awareness for Families of Spinal Muscular Atrophy. The crowd listened to performances by local bands, Too Many Kennys, Cold Comfort, Latin Blues Band, and TimePeace. Attendees also enjoyed food, a raffle and activities for the children! Thanks to the support of family, friends and the local community, the event was a success and raised \$250 in honor of Phillip Herrera! Thank you to Evelyn Vasquez for your dedication in planning the All Star Jam for Families of SMA!

Team Reach for the Sky

On Sunday, October 9th, 2011, Team Reach for the Sky successfully completed the San Diego to Coronado Sharkfest Swim! All team members made it safely to shore. And nary a shark was spotted in the process, although a few swimmers did report seeing a mean-looking halibut near the finish line!

Team members ranged in age from 11 to 58, and in swimming skills from former state record holders to "I just hope to finish the race" novices. Teammates Maggie Matthews, Mark Matthews, Brooks Bahrenburg, Chris Kellerman, Hannah Kang, and Francine Grimmer all earned medals by finishing in the top three of their age groups. But the swim wasn't about winning medals. Our purpose was to have fun, finish safely, and raise money to help find a cure for Spinal Muscular

Atrophy. And that's where you came in.

While planning the swim, we set a goal of 500 donors and raising awareness of SMA. So far, we've received 440 donations, and have raised **\$36,989** along the way.

On behalf of Team Reach for the Sky, thank you for your contribution to FSMA. One day, researchers WILL find a way to overcome this terrible disease, and your contribution brings us one step closer to the cure.

In his short life, Skylar had a profound impact on those who knew and loved him. We are grateful for your part in allowing us to honor his memory in such a meaningful way.

Wow – what a race, what a weekend, what a wonderful way to celebrate the life of Skylar Bahrenburg!

Families of SMA would like to congratulate and thank the Bahrenburg, Matthews and Amlicke families for their hard work and dedication in planning the event. The response that was received from your family and friends is truly incredible. The funds and awareness that you raised is inspiring hope that one day we will find a treatment and a cure for Spinal Muscular Atrophy. Congratulations to all of members of Team Reach for the Sky on successfully completing the Sharkfest Swim!

Dear Families of SMA,

For Diego's 1st Birthday, we wanted to do a fundraiser for FSMA. Your organization and all the wonderful families we have learned about through FSMA have been such a support for our family. We are glad to mail in \$150! We had a 50/50 raffle and all of Diego's

guests gladly participated! The days and months go by so quickly, Diego is now 18 months and continues doing well! Again thank you for all

that you do for the SMA community. I believe that one day because of all your hard work and dedication, a treatment and a cure for SMA will be found!

Best Regards,

*Mojarro Family
Alhambra, CA*



SOUTH FLORIDA

Gala of Hope to Cure SMA

Two Mothers Come Together to Bring Hope and Strength to the SMA Community

The First Annual Gala of Hope Cure SMA was held on Saturday, November 17th, 2011 at the Gryphon Club within the Seminole Hard Rock Hotel and Casino in Hollywood, Florida. I decided I wanted to put together an event that would not only raise money for research but would raise major awareness in South Florida like never before. My husband and I could not accept the fact that it took us one year of visiting over ten specialists to diagnose our daughter. Mia was diagnosed with SMA Type II on May 16th, 2011 - just days after her second birthday.

I joined forces with Jennifer Miller-Smith, mom to Madison, who also has SMA Type II. Jennifer is also the President of the FSMA South Florida Chapter. Together we were able to recruit an amazing team of woman who volunteered to help right away! Anything from helping to sell tickets to recruiting sponsors and auction items. Not only did my friends and family come together but also my fellow coworkers and business relations. As soon as I turned to them for sponsorships, they were all excited to be a part of the event in one way or another.

The night consisted of a step-and-repeat red carpet entrance with VIP treatment, interviews and videography from Kavita Channe of 1stDownNDirty, along with a display of Harley-Davidsons, Ferraris and Maseratis. Rock With U Entertainment provided amazing music and an MC, there was a Cirque du Soleil style performance and a night of live singing from several recording artists. We had fresh hors d'oeuvres generously donated locally by 6 top restaurants, along with tasty desserts, and let's not forget a deluxe open bar!

The silent auction consisted of over 50 items ranging from spa treatments, to fine jewelry, a big screen TV, and even a live puppy. One of the biggest successes of the night was a raffle prize generously donated by J.R. Dunn Jewelers for a \$10,000 jewelry shopping spree. Throughout the evening, we had volunteer girls selling \$100 raffle tickets in exchange for one entry and a glass of Champagne. We raised over \$13,500 just through this one raffle item! We also had the pleasure of some celebrity guests such as DJ Laz, Nina Alicia, Gabby Villanueva, Superbowl Champion Leonard Marshall, Miami Dolphin players Terry Kirby, OJ McDuffie, Jeff Cross and K-Jana Carter.

The night was full of fun, entertainment, and most importantly, Hope. With over 400 guests in attendance, we were able to raise just over **\$130,000** for Families of SMA, along with some serious SMA awareness all over South Florida! In 2012, we hope for an even bigger turnout! As a mother, I refuse to stand by and let SMA take over our lives. We won't stop until we find a cure!

Fiorenna Israel
Mom to Mia, Type II
Ft. Lauderdale, FL



MPS Credit Union

Throughout the month of August, three MPS Credit Union Banks in Florida participated in fundraising for Families of SMA! Each bank displayed coin cans and had informational materials about SMA for their customers. In total, \$1,200 was raised in honor of Sofia Maria Santana! Thank you to Ana De La Luz, friend of the Santana Family, for organizing this fundraiser and educating your community about Spinal Muscular Atrophy!

Toy Story 2 Charity Event — Do The Right Thing Insurance Group

The Toy Story 2 Charity Event was held in Ft. Lauderdale, Florida on Wednesday, December 14th, 2011. Thanks to the support of Stanley Gerome and the Do the Right Thing Insurance Group, \$200 was raised for Families of SMA in honor of Madison Woodard.

SOUTH JERSEY/DELAWARE

This year, Families of SMA South Jersey/Delaware Chapter will be celebrating our 10 year anniversary! The Chapter has several fun and exciting events organized for the year so far, and we would love to have you join us.

Upcoming Events

- **April 27th, 2012 – 5th Annual Steven's Swing for a Cure**
To participate, please visit www.fsma.org/stevensswingforacure2011
- **May 12th, 2012 – 5th Annual Cure SMA Walk-n-Roll in honor of Katherine Santiago**
To register for the walk, please visit www.fsma.org/katherinesantiago
- **May 13th, 2012 – 2012 Delaware Marathon and Half Marathon**
To learn more or make a donation, please visit www.fsma.org/delawaremarathon
- **May 19th, 2012 – 7th Annual Steven's Walk to Drum Out SMA**
Register today and start your fundraising at www.fsma.org/drumoutsma2012
- **June 2nd, 2012 – Children Helping Children Trike-a-thon**
To donate and learn more about the event, please visit www.fsma.org/childrenhelpingchildren.

If you would like help planning your own fundraising event please contact Jessica Moyer at (866) 774-9533 or southjersey@fsma.org.

Thank you all for your tremendous support and let's make our 10th Anniversary one to remember!

Warmest wishes,
Jessica Moyer
President



Fundraiser in Honor of Natalie Dell'Oso

Thank you to Kim Snyder for hosting the fundraiser in honor of Natalie Dell'Oso in Wilmington, Delaware! Thanks to her hard work, she was able to raise \$517 for Families of SMA!

Glen Meadow Middle School Kiwanis Club Bowl-a-Thon

On November 11th, 2011, Dawn Cusack and the Glen Meadows Middle School Kiwanis Club of Vernon, New Jersey hosted a Bowl-a-Thon event! In total, \$390 was raised in honor of Dawn's daughter Kelly, SMA Type II, who is a member of the Kiwanis Club! Many thanks to the Kiwanis Club members and Dawn for raising funds and awareness for Families of SMA!

Gift Wrap at Barnes & Noble

During the holiday season on December 17th and 23rd, 2011, Marcy Fantel organized a Gift Wrap Fundraiser at her local Barnes & Noble near Kendall Park, New Jersey! Thanks to everyone's generosity, Marcy raised \$1,050 for Families of SMA in honor of her son, Ray!

Cocktails for a Cure

The Cocktails for a Cure fundraiser was held on Wednesday, September 28th, 2011 at Marlton Tavern in Marlton, New Jersey. Guests enjoyed a silent auction, live music, a raffle and an appetizer buffet! In total, \$4,860 was raised for Families of SMA in memory of Steven Moyer. Thank to Jocelyn Talbert, sister of Jessica Moyer, for putting this event together!

3rd Annual Hoop, Walk and Roll



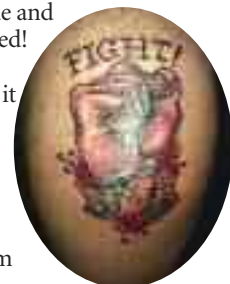
On October 15th, 2011, the 3rd Annual Hoop, Walk and Roll was held at Van Horne Park in Skillman, New Jersey. Over 200 people turned out to walk and hula hoop for SMA. There was live music, kid's games, refreshments, silent auction items and lots of raffle baskets. We were very pleased with the turnout taking the severe winds into account!! The event raised over **\$37,000!!** That's over \$70,000 in three years!!

Thanks to the efforts of several SMA families and their friends (Fantel, Kingkiner, Mason, Smith, Watson, and Weingart), the event was a big success!



Morrongiello Family Christmas Fundraiser

On December 17th, 2011 we were having a small Christmas gathering at our home. The idea started out when our home was finished being decorated. The lights, the tree and the decorations both inside and out, were finally finished! Looking upon everything, we decided that it looked so nice that we should have friends and family over to enjoy being together for the holidays. After reading an update from the FSMA chapter, we thought of asking our friends and family to not bring anything such as food or drinks, but to make a small donation to FSMA. Our guests were truly generous as we raised over \$1,300! Our first fundraising event was a success! Our family and friends want this to become an annual event and we are already making plans for next year. We are planning to have an "Open House" earlier in the season and hope to raise another generous contribution. We are truly appreciative to our family and friends for their continued support and generosity over the past years since our son was diagnosed with SMA Type III. We are truly blessed and keep hope in our hearts that there will be a cure in the near future.



Sal & Robin Morrongiello
Toms River, NJ

TENNESSEE

The new year brought some changes to the Tennessee Chapter. After 10 years, and much thought, Lise Murphy has decided to step down as the Tennessee Chapter President. Lise started the chapter in 2001 after her son was diagnosed with SMA. Over the years our fundraisers have grown from a yard sale to annual events including a golf tournament and a fall dinner and comedy show. Lise was the driving force in raising over \$300,000 to find a cure for SMA. But now that her children are getting older she feels the need to concentrate her time and energy on their activities. Lise will remain active in the chapter as Treasurer and would like to thank the countless volunteers who have given so much of themselves to make the Tennessee Chapter the success that it is. One of those volunteers, Sarah Boggess, will be taking on the role of president. Sarah (SMA Type II/III) has been an active member of the chapter since its inception. She is a recent graduate of the University of Tennessee and is now pursuing a Master's Degree in Plant Pathology. We look forward to learning from her unique perspective and hope to provide even greater support to our families.

This past November we held our 9th annual comedy event "Horsing Around for a Cure" at Hunter Valley Farm in Knoxville, Tennessee. The evening included a silent auction, music, dinner and some hilarious comedy. Thank you to everyone who made the evening a night to remember - our donors and volunteers, Manny's Catering, music by The Green Fleece (very talented UT students) and of course our comedians Leanne Morgan and Rocky LaPorte. The event raised over **\$26,000** and planning has already begun for the 10th annual comedy fall benefit.

Spring 2012 promises to be a busy time for the Tennessee Chapter. We will host a "Living Room" concert on April 22nd, 2012 featuring country Christian singer Cindy Morgan. This will be followed on the 23rd of April by the 8th Annual Golf Tournament. Both events will be held in Knoxville, Tennessee. On May 19th, 2012 we head to Murfreesboro for a Skeet Shoot. On August 19th, 2012, join us for the 14th Annual Tennessee Walk-n-Roll Away



SMA. To register or donate, please visit www.fsma.org/TNWNR. Details for these events will be forthcoming online on the FSMA events calendar and Tennessee Chapter Website.

Additionally, two fundraisers were held in Western Tennessee in honor of Carlee Beam. The first was at Sips 'N Strokes in Franklin, Tennessee and raised over \$600. Faithful Strokes in Murfreesboro hosted a similar event and raised over \$400. Guests enjoyed food, friendship, painting instruction, and learned a little about SMA. Carlee (Type II/III) was the star of both nights and through her, many learned about her determination to beat SMA.

Also, during the 2012 tax season Jason A. Jones's firm is donating a portion of the proceeds from tax preparation services in Rutherford County, Tennessee. One of the charities chosen is FSMA in honor of Carlee Beam. In addition to this generous donation, the firm is challenging all other CPA firms and tax preparation services in Rutherford County to make similar contributions. Thank you Jason!

Finally, we encourage our members and friends to join the Tennessee Families of SMA Chapter Facebook group. You will receive updates on events, be able to view photo albums of our kids and events, and share your stories. If you are not on the TN FSMA e-mail listserv, please e-mail the chapter at Tennessee@fsma.org and we will be happy to add you.

Gospel Concert Fundraiser

On September 24th, 2011 a gospel concert was held at Immanuel Baptist Church in Lebanon, Tennessee in memory of our little girl, Alana Christine Whited. We wanted to help spread the word about Spinal Muscular Atrophy to help others learn about this disease and the symptoms to look for in children living with SMA. Our goal was to raise money to help other families who are going through what we did with our little princess. It is not easy and every helping hand or voice makes a difference. We want to keep Alana's spirit alive and let her know how much we love her by helping Families of SMA.

Alana touched a lot of people with her warm smiles even through her sickness. Several groups belonging to MSGMA (Mid-South Gospel Music Association) gave their talent and time and we truly appreciate all the hard work that everyone put in. During the concert, a slideshow was shown in memory of our daughter to show how much joy she brought to us in only a short six months. Along with this concert, we have placed donation boxes with Alana's picture, information on SMA and the link to Families of SMA's website in local business's during the Christmas season. We also placed Alana's Gift Registry page on our Facebook pages around the holidays and her birthday so that we could raise money for this cause. Through everyone's kindness and generosity we raised \$2,925 for Families of SMA! We hope to do many more benefits in honor of Alana and continue to spread the word about SMA in hopes to one day find a cure for this disease.



Sincerely,

*The Whited Family
Lebanon, TN*

UTAH

The Utah Chapter kicked off 2012 with a successful Chapter Meeting and is now hard at work preparing for our upcoming events! Be sure to mark your calendars for the Utah Chapter Walk-n-Roll on Saturday, June 16th, 2012 in Salt Lake City, Utah. For more details and to register your team for the walk, check out www.fsma.org/utah. The Utah Chapter is also hosting a Barn Bash at the National Abilities Center in Park City, Utah on September 15th, 2012.

TEXAS

40 in 40 Fundraiser

Courtney Buck West, a mother of SMA angels, turned 40 in November and we planned a BIG surprise! We invited her closest friends and family to join us in raising \$40,000 in 40 days for Families of Spinal Muscular Atrophy. Courtney lost her two precious children, Wayland and Piper, to this devastating disease. We wanted to support Courtney's mission in finding a cure for SMA and make a birthday wish come true!

The "40 in 40" fundraiser kicked off in August 2011, SMA Awareness Month, at a Cory Morrow concert at the Whitewater Amphitheater in New Braunfels, Texas. His fans helped raise over \$2,000 for FSMA. On September 23rd, we officially began the 40 in 40 drive. Invitations were sent out to over 150 friends and family. They helped us spread the word through e-mails, blogs, and Facebook. Donations were made on a hidden webpage through the FSMA website, and our star started to shoot across the board. Courtney's former colleagues at seven elementary schools in San Antonio, Texas also participated in the drive. Comal Crossfit members in Bulverde, Texas, worked together to raise over \$3,000 for FSMA at the Kids 4 Kids Games. Over 100 children ages 2-17, learned more about Spinal Muscular Atrophy from a local Physical Therapist who works with SMA patients before competing in Crossfit style relays and activities.

On November 5th, at Courtney's surprise 40th birthday party, we presented her with a big envelope. When she opened it, she found an even bigger surprise, a check for \$40,000! With the help of friends, family, and other generous donors, we reached our goal and raised **\$42,055** in honor of our dear friend



Courtney West and in memory of little angels Wayland and Piper. We hope others will be inspired by the 40 in 40 fundraiser. Together we can make a difference and make more wishes come true!

Melissa Freund
Spring Branch, TX

After the surprise, Courtney wrote:

What a surprise of a lifetime! Saturday night I walked into the backyard of my parent's home, totally caught off guard by a "Surprise" 40th birthday party. Jake, my husband, proceeds to open a bottle of bubbly while everyone stands around in a circle. So in my head I am thinking, "Oh boy, here comes the roast!" My dear, Saint of a friend Melissa starts talking about this "idea" she had back in July for what she wanted to do for my 40th birthday. What begins to come out of her mouth was so unbelievable that for the first time in my life, I was completely speechless. Everyone in the circle then presented me with a huge check made out to FSMA for \$40,000 in honor of my birthday. I am very blessed and honored to have such dedicated people in my life.

To my beautiful angel babies, Wayland and Piper. I see you every time a frog hops my way or a ladybug lands on my hand. I see your faces in the sun and in the moon. Thank you for being such an inspiration to everyone around us. Because of you, our family and friends, raised \$40,000 in 40 days to help find a cure for SMA. I am so proud of you both. Thank you for always being with me.

To my devoted family and friends. Thank you everyone for this amazing birthday gift of \$40,000 for FSMA in 40 days. Since my birthday presentation of the check – I have now heard all the secret planning, seen the 40 in 40 webpage, looked at all the pictures from the different fundraisers, read all your emails, read all your hand written notes and finally wrapped my head around what has happened. I am absolutely in awe of the love and generosity that encapsulates my life. I cannot thank you enough for what you have done. This has truly been a miracle.

Love,
Courtney West
Plano, TX

Volleyball Tournament and Family Day

Erica Parks hosted the Volleyball Tournament and Family Day on June 18th, 2011 in Timpson, Texas! Thanks to all of Erica's hard work in planning the event, she raised \$539 in honor of her son, Landon!

Zumbathon

On June 30th, 2011, Myra Macareno hosted a Zumbathon in Kingsville, Texas! In total, \$400 was raised for Families of SMA! Thank you Myra for planning the event!

USA FIT Everyone's Marathon for Savannah Smiles

Meridith Struble, a close friend of Paul and Sheree Norton, organized the Savannah Smiles team for the second year in a row at the USA Fit Marathon that was held on January 29th, 2011 in Sugarland, Texas! While "turning miles into smiles," the team raised \$8,005 in honor of Savannah Sue Norton! Over the past two years, Team Savannah Smiles has raised \$20,456 for Families of SMA! Thank you Meridith for organizing the team and to all of the runners for your hard work and dedication in both fundraising and training for the race!



TEXAS (cont.)

Butterbraids Fundraiser

Mindy Rush hosted the Butterbraids Fundraiser on December 1st, 2011 in Saginaw, Texas. Thanks to the support of family and friends, Mindy was able to raise \$2,408 for Families of SMA in honor of her daughter, Savanna Rush. Thank you Mindy for your hard work and dedication in organizing this fundraiser!

Texas Chapter Bowl-a-Thon

The Texas Chapter hosted their Bowl-a-Thon on Saturday, September 24th, 2011 at the AMF Showplace Lanes in Austin, Texas! In conjunction with their online silent auction for the event, the Bowl-a-Thon raised \$8,335 for Families of SMA! Thank you to all of the teams and families that participated in the



event as well as to Kelly Coggin for organizing the event!

Dear Families of SMA,

Thank you so much for continuing your research in search of the cure for so many, including my cousin Matthew. I trust that all \$75 collected from the "Empty Your Pockets for SMA" container at my dad's dermatology practice is being put to very good use! I don't say that to sound uppity- I really am trusting y'all!! So thank you for everything that you do, and I pray that this money will help you find a cure for my cousin and so many others.

Love,

*Lydia Wallis
Longview, TX*

WESTERN NEW YORK

Hideaway Bar and Grill Fundraiser SMA Awareness Week Fundraiser

During SMA Awareness Month on August 13th, 2011, Bonnie Shiesley hosted the Hideaway Bar and Grill Fundraiser in North Tonawanda, New York. Thanks to the generosity of family and friends, the event raised \$531 for Families of SMA! Many thanks to Bonnie for putting together this wonderful awareness fundraiser!

Upcoming Events:

Mark your calendars for the 9th Annual WNY SMArt Walk for a Cure that will be held on Saturday, August 4th, 2012 at Beaver Island State Park in Grand Island, New York. For more details and to register your team for the walk, visit www.fsma.org/wnywalknroll2012 today!



WISCONSIN

7th Annual Grant Sheppard Memorial Scramble for SMA

On Friday, September 2nd, 2011, the 7th Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Chilton, Wisconsin. The golf outing was held on what would have been Grant's 7th birthday. We are proud to be sending you the proceeds that may be used to suit the needs of the organization. Enclosed you will find a check from the proceeds of the golf outing for \$7,000. In combination with the total raised on the website and other donations, the total raised in Grant's memory is \$9,000.

The day was our first rain out in seven years! A terrible storm with over 80 mile per hour winds came in very quickly and trees were falling on

the golf course. We had to bring in all golfers to the clubhouse for their safety, but everyone still had fun at the silent auction and bucket raffles helping to raise money for Families of SMA.

Thank you for all that you do for the families dealing with Spinal Muscular Atrophy. Together we will find a cure!

*Scott, Lisa, Peter and Lily Sheppard
Sherwood, WI*

Team Kherington Benefit Fundraisers

Our now two year old daughter Kherington was diagnosed with SMA Type II last January. This summer many friends got together and held

WISCONSIN (cont.)

several benefits in her honor. The people who have shown support were named "Team Kherrington." I believe there were five benefits held by different groups of friends and family from April through August. Since her diagnosis is so new we are still on waiting lists for financial support so these benefits have helped us purchase a used van, put a ramp on our house, pay for part of her wheelchair as well as many of the other small things we need daily. We were able to attend the conference at Disney this June, (and can't wait until you have it there again by the way), and we learned a lot of great things.

All together, we are sending you \$2,175 from the money raised by Team Kherrington. We appreciate everything Families of SMA does and know that you will put our money to good use in finding things to help all families with Spinal Muscular Atrophy and supporting all of us along the way. Thanks again!

*Brandon, Melissa and Kherrington Shippee
Janesville, WI*

Parkview School District Class Council Fundraisers



Our student council chose to have a different cause we would fundraise for each month due to the vast interests our students have. During the month of October, we chose SMA because one of our students has a god-sister named Kherrington Shippee who was diagnosed in January 2011. We held a hat day and a pretty successful bake sale in Kherrington's honor. We also sold more baked goods at our annual Haunted Hallway, and people were more than generous there as well. It really became a community endeavor and we raised \$200!

Thank you for coordinating such a worthwhile enterprise.

*Geri Acker
Parkview Senior High School, Orfordville, WI*

Mayo Clinic Jeans for a Cause

The Mayo Clinic Health System in Menomonie, Wisconsin, held their Jeans for a Cause fundraiser in honor of Families of SMA and in honor of Daniel Herrmann. All together, the Mayo Clinic Health System, Red Cedar was able to raise \$421 for Families of SMA!

Hoffmann Family Fundraisers

On September 11th, 2011, Paula Hoffman hosted the Annual Hoffman Family Block Party and Raffle in Milwaukee, Wisconsin! Thanks to her hard work in planning the event, \$516 was raised for Families of SMA! In October, Paula also coordinated the Craigslist Fundraiser which raised \$200 in honor of Jackie Hoffmann!

Thank you to the Hoffmann Family for your continued support of Families of SMA!

7th Annual Hot Lap for SMA

Many thanks to Pam Kuhn for hosting the 7th Annual Hot Lap for SMA on August 27th, 2011 in Wisconsin Rapids, Wisconsin! In total, the event raised \$940 for Families of SMA in memory of Shania Hasselman.

2011 Round for a SMA Cure

On August 12th, 2011, Linda Marcou and her committee hosted the 2011 Round for a SMA Cure Golf Tournament in Menomonee Falls, Wisconsin. Thanks to their hard work in organizing the tournament and the support of family and friends, the event raised \$7,000 for Families of SMA in honor of Benjamin Nelson!

Etsy Flip Flops Fundraiser

Tanya Fleege from Kenosha, Wisconsin hosted an online Etsy sale on August 20th, 2011. Through donating 25% of sales from the site, Tanya was able to raise \$180 for Families of SMA in honor of Violet Wehrkamp. Thanks for all of your hard work, Tanya!

Damon Family Softball

On September 13th, 2011, Angela and James Damon hosted the Damon Family Softball Game in Brooklyn, Wisconsin. Thanks to the generous support of family and friends, they raised \$550 for Families of SMA in honor of McKenzie Damon! Thank you for your hard work in organizing this game!

INTERNATIONAL

The International Section features fundraisers held by families affected by Spinal Muscular Atrophy from around the globe. Thank you for your support of Families of SMA.

Canada

Dear Families of SMA,

I am the house director of Baillie House, a house at Appleby College in Ontario, Canada. The house has 71 girls in it and we did our annual Retro Dance Fundraiser where the girls plan a dance for the rest of the school and any money raised goes to a charity of their choice. This year we raised \$308 and they chose Families of SMA as the charity as one girl has a family friend who is affected.

Thanks,
*Robin Leslie-Spencer
Oakville, Ontario*

Mexico

Tito Camiro's 11th Birthday Party

From Mexico, Tito Camiro and his 5th grade friends collected \$1,000 at his 11th birthday party. They donated the money to the Families of SMA 2012 Annual Campaign.

SMA HOME for the CURE



Join Families of SMA for the
2012 SMA Home for the Cure
in the Clover Springs
neighborhood in Delano, MN.

Everlast Enterprises is teaming up with
WCCO, Coldwell Banker Burnet,
Beacon Bank and others to build a
beautiful Two Story Home to be
completed June of 2012.

Proceeds from the sale
will benefit the organization
Families of Spinal
Muscular Atrophy (SMA),



Presented by:



BURNET



Families of SMA

Support. Research. Hope.

www.curesma.org

What is SMA?

- SMA is the number one genetic killer of children under the age of two which destroys the nerves controlling voluntary muscle movement.
- 1 in 40 people carry the gene that causes SMA
- 8 million Americans are carriers

For more information on the house, SMA or how to get involved, please contact Families of SMA at (800) 886-1762 or info@fsma.org

Promotional Materials from Families of SMA Make Planning a Fundraiser Easy and Fun



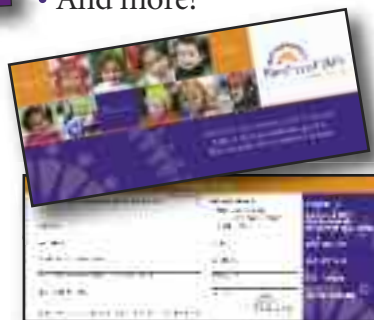
Are you interested in hosting a fundraiser? Families of SMA has manuals, sample booklets and dedicated staff to help you through the entire planning process. Please feel free to contact us anytime at fundraising@fsma.org to get started today!

New Fundraising Materials:

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



Event Website Updates

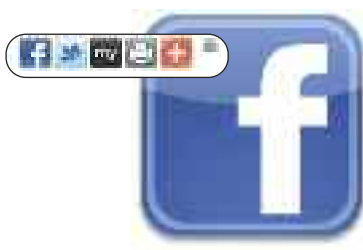


New Event Website Upgrades



Quick and Easy Way to Invite Donors

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



Use Social Media to Share Your Page

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

Also New This Year

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

Merchandise



Purple Grocery Bag | \$4



Car Magnet | \$5



Orange and Purple Ribbon Lapel Pin | \$5



"Together we will find a cure" T-shirt | \$12

Youth sizes: XS S M L • Adult sizes: M L XL XXL



FSMA Rubber Bracelet | \$2

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

Merchandise can be ordered online at www.curesma.org or by calling the FSMA National Office at **1-800-886-1762**

Summer Trip to the Other Side of the World

By: Morgan Duffy, SMA Type III

Stanford's campus generally buzzes during the winter months. People are anxiously applying for internships, fellowships and scholarships to answer the question: what are you doing this summer? After my freshman year, the answer was simple: I am going home to relax and spend time with my family and friends. The distance between California and Scranton, PA was a difficult adjustment for an only child. Little did I know, just one year later I would be taking a trip to the other side of the world. In this, I will reflect solely on my travels to Indonesia.

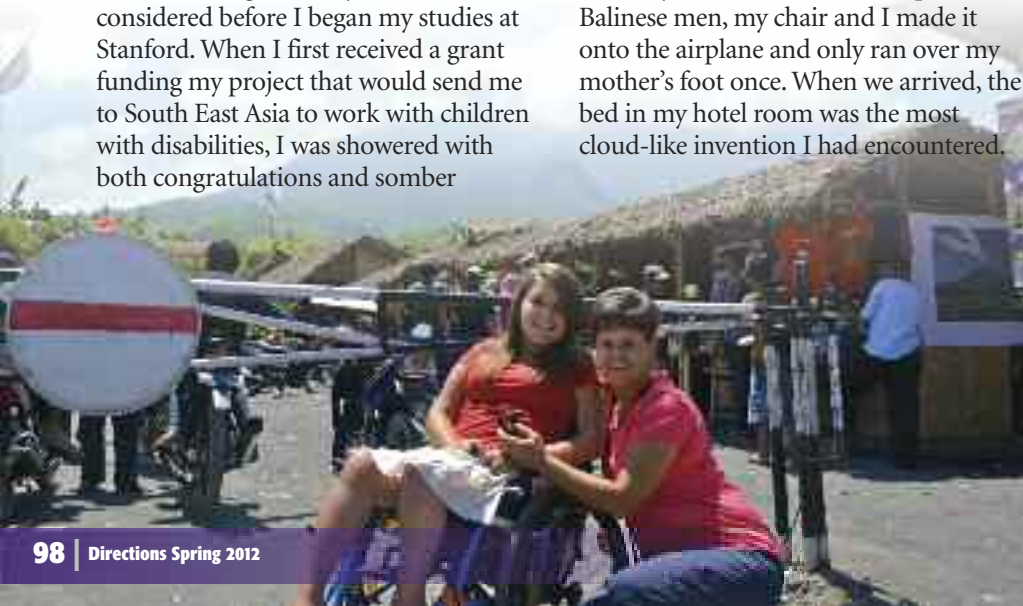
My sophomore year I began taking courses about health, disparities and culture. While sitting in my psychology class I realized that no professor addressed a matter most important to me, addressing health disparities for children with disabilities and chronic illness. After a few months of exploration and talking with organizations, I decided that I was going to travel to a city in Indonesia to work with children with disabilities and their families. As a young woman with SMA III, I have used a power wheelchair since I was 11 years old. Although my parents encouraged me to circumvent any limitation that I may have found during my childhood or adolescence, the idea of traveling to a developing country was something that they had never considered before I began my studies at Stanford. When I first received a grant funding my project that would send me to South East Asia to work with children with disabilities, I was showered with both congratulations and somber

questions about safety, accessibility and being a white woman in a Muslim world. I was terrified of leaving the United States for the very first time. On September 1, 2011 my mother and I boarded a flight to Taiwan, the first of three legs to Yogyakarta, Indonesia.

International travel is not for the faint of heart. It takes courage and spontaneity. The domestic terminal of the Balinese airport was as humid as it was congested. Like the roads without speed limits or street lights, chaos is imminent. We walked in and passed through security without blinking once. Our assistant brought us to the desk and helped us check our baggage. Seems like a normal travel day? Nope. The gate was on the second floor of a building without an elevator so they shuttled us to the medical gate that was filled with elderly people in wheelchairs, a hospital bed and a shower. This is when our attendant insisted on taking my passport. Then came time to pee: no toilet paper. My mom runs out of the bathroom yelling for toilet paper. We look out the window to the jet way which appears to be a lane-less highway filled with people, bikes, baggage trucks, transport busses, oh and airplanes!! I thought someone was definitely going to get run over by a Boeing 737! The plane had only stairs and with the help of 4 Balinese men, my chair and I made it onto the airplane and only ran over my mother's foot once. When we arrived, the bed in my hotel room was the most cloud-like invention I had encountered.



I wondered to myself, why am I here? What was I thinking as I laid sore in a country with roosters crowing outside our window and humidity reaching in through the window panes with every passing hour of the hot afternoon. I learned two days later. The children that I met embodied resilience. In a country where health care is only for the super rich, where disability is seen as a living death sentence and where social systems that are supposed to support those in need merely perpetuate inequality, smiles and laughter were worth more than a years worth of work and frustration to get to their country. As I reflect on my experiences, and plan for my second summer in Yogyakarta, I remember the endless gratitude and kindness that the people extended to my mother and I just for our brief presence in their lives and interest in their children. Without much knowledge of Indonesian culture, it is still easy to understand what links us together: the mutual experience of disability. The disability community, no matter how diverse in opinion, shares an experience that links us cross-culturally to understand that children with disabilities need encouragement to take on the role of self-advocates and create bridges into their community. Although we may wait on science for the answers to ease health issues and suffering, while waiting access and advocacy needs to be highlighted. It's this message that drives me to take on the challenges of traveling to a rural community in South East Asia for a second summer.



The Sport of Sled Hockey



Steven Potter is a 14 year old with SMA type III. He has played sled hockey for the Virtua Wings of Steel in Voorhees, NJ for about 3 years. Sled hockey is basically just like regular hockey, but the players sit down in bucket sleds. The buckets have ice hockey blades on the bottom of the sled. The players have regular hockey sticks that have been cut in half. At the one end is the blade to hit the puck and the other end are very sharp metal spikes that they use to push themselves. This sport is a very competitive sport. Virtua Wings of Steel had a young man on their team that won a gold medal in the USA Olympics.

Steven's team travels as well. We have been to Amelia Park in Mass, Baltimore Maryland for a tournament, and we are planning another long trip to Dallas Texas for the Disable Festival. Stevens's team will play teams from all over the country. Steven and his team-mates just got to play at Citizens Bank Park in Philadelphia, PA on the Winter Classic Ice. That was so exciting for Steven and his team. Just to see the smile on my son's face on that ice brought tears to my eyes. Almost like he couldn't believe he was out there, where the Philadelphia Flyers Alumni had played and where the Philadelphia Flyers played in the Winter Classic.

Steven is unable to play sports with his classmates. Playing sled hockey has been awesome for Steven. All of the people that play have some type of disability. No one plays with SMA. It is quite challenging for Steven on the ice because he has trouble keeping up with the rest of the team. That does not stop him! He has wonderful teammates that are great with him, along with great, caring coaches. Steven has had 2 goals and a couple of assists, so he is right in there with the rest of the kids playing.

I don't know what Steven would do if there comes a day when he can't play sled hockey any longer. This has become his freedom. He loves being on the ice and having a great time with his team and meeting other people with disabilities around the country that play this sport.

If you would like to check it out please visit www.virtuawingsofsteel.org or Google Sled Hockey. It is an intense sport.

Thank you Tom Brake and the rest of The Virtua Wings of Steel Sled Hockey Team for making my son so happy. We are blessed to have met this sport!

Theresa Potter of Haddon Township, NJ



Stress and Growth Over Time: Caregiving and Bereaved Parents of Children with Life-Limiting Illnesses

OVERVIEW

Caring for a child with a life-limiting illness is stressful, and the death of a child due to such an illness can be very traumatic. The negative aspects of caregiving are well documented in the literature. However, it has also been suggested that in the face of such adversity, parents can experience personal growth and positive change. Little is known about positive outcomes of caregiving, particularly for parents caring for seriously ill children.

This research is a continuation of the study "Caregiving Parents of Children with Life-Limiting Illnesses: Beyond Stress and Coping to Growth". An additional 3 years of funding has been awarded to explore the stress and personal growth of parents over time whose child has died. New knowledge obtained from this study will be of tremendous value to parents, practitioners and policy makers concerned with childhood life-limiting illness. Pathways leading to positive outcomes will be identified through this study and will then be used to optimize the emotional, social, and spiritual care

available to patients and families receiving pediatric palliative services, including bereavement care.

THE STUDY

The primary aim of this research addresses the question "What are the factors that allow parent caregivers to survive and even grow in the face of the stressful circumstances of caring for a child with a life-limiting illness?"

This research is comprised of two phases. Phase One will use 8 short scales to study demographics, personal resources, spirituality, stress and growth and will be sent to the participant twice, one year apart. A stamped return envelope will be provided along with an information sheet explaining the study in detail. Phase Two consists of in-depth interviews, conducted with a select number of Phase One participants. There is no obligation to participate in the interview phase and parents may opt out of the study at anytime without consequence. It is expected that parents will need approximately 1 to 1.5 hour(s) to fill out the questionnaires and approximately 1.5 to 2 hour(s) to complete the interview.

PARTICIPANTS

Mothers, fathers, and other caregivers (e.g. grandparents, guardians) who have lost a child to a life-limiting illness are invited to participate. All parents and other caregivers are welcome regardless of the length of time they have been caregiving and at any stage after the child's death, so long as the child was under the age of 18. More than one parent caregiver per family may participate in the study and both mothers and fathers are strongly encouraged to share their experiences.

If interested in participating or obtaining more information about the study please call 1-800-810-0721 and leave a message. A research assistant at Wilfrid Laurier University will return your call. Alternatively you may contact the research coordinator at the telephone number or email address below.

Kathy Wilson, Project Coordinator
Manulife Centre for Healthy Living
Wilfrid Laurier University
Waterloo, ON
519-884-1970 ext 5261
kwilson@wlu.ca

SHARING PHOTOS



Ford Snizek



Connor Murphy



Braelyn Campbell



Ford Snizek

Have you experienced the death of your child from a life-limiting illness?

Dr. Susan Cadell, Associate Professor, Wilfrid Laurier University, Faculty of Social Work and colleagues are currently conducting a study of bereaved parents who have cared for a child with a life-limiting illness. We are looking for parents/guardians who are willing to share the story of caring for their child, the story of their death and how that experience changes parents' lives.

We recognize that sharing the life and death of your child is both sacred and difficult. The information provided will help caregivers, social workers and medical staff better understand and support families during the care and death of a child. We appreciate the courage and time taken to assist us in this study.

For this study, you will be asked to complete a set of questionnaires at two separate times. The questionnaires will be sent to you by mail and will take 1 to 1.5 hours to complete. Participants will not be identified in the study.

Questionnaires are available in either English or French.

For more information, or if you are interested in participating in this study, please contact the Research Coordinator at 1-800-810-0721.

STRESS AND GROWTH OVER TIME:
CAREGIVING AND BEREAVED PARENTS OF CHILDREN
WITH LIFE-LIMITING ILLNESSES

Principal Investigator: Dr. Susan Cadell

Co-Investigators: Dr. Hal Siden (University of British Columbia), Dr. Lynn Straatman (Canuck Place Children's Hospice), Dr. Betty Davies (University of Victoria), Dr. Rose Steele (York University), Dr. Stephen Liben (Montreal Children's Hospital), Dr. David Hemsworth (Nipissing University)





Family Guide to Research

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

The topics in this new booklet cover the following areas of SMA research:

- SMA Researchers.
- Drug Research.
- Gene Therapy.
- Stem Cells.
- Clinical Trials.
- Government Research and the FDA.



Patient Services & Family Support

This booklet has details on the following Families of SMA programs:

- 1) Programs For Newly Diagnosed Families:
 - Including our special Type I programs such as: Care Packages; Sheep Skin Blankets; Radio Flyer Wagons; Dinners; and Home Support.
- 2) Families of SMA Equipment Pool.
- 3) Medical Care.
 - Including the “Ask the Expert” Service.
- 4) Daily Living.
- 5) Local Support.
- 6) How to Keep up to Date.
- 7) The Annual SMA Conference:
 - Including the FSMA Newly Diagnosed Conference Program.



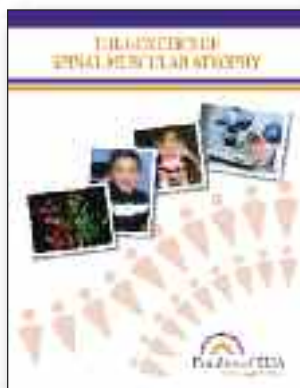
If you would like a hard copy mailed to you please email us at info@fsma.org or call (800) 886-1762

Breathing Basics

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

This booklet reviews the following topics:

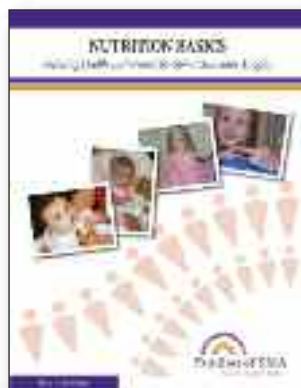
- Why is respiratory care so important in SMA?
- What are common respiratory problems in children with SMA?
- Elements of respiratory care management in SMA
- What are special needs of children with SMA Type I, Type II and Type III?
- What respiratory equipment will you need at home?



The Genetics of Spinal Muscular Atrophy

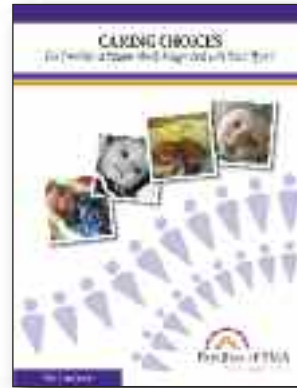
Confused about genes, proteins, DNA and how SMA is diagnosed?

Read this helpful pamphlet. It includes definitions, explanations and diagrams from genetics expert Louise Simard, Ph.D. and the FSMA Medical Advisory Council.



Nutrition Basics

This 32 page booklet is focused on fostering health and growth for SMA. Nutrition is a complex issue for anyone, but especially for children and adults with SMA. As you go through this booklet you'll see that SMA may present some nutritional challenges, but by educating yourself, talking to other parents and getting help from a registered dietitian, children with SMA can receive a wide variety of benefits from good nutrition.



Caring Choices

This booklet is focused on caring choices for parents of infants newly diagnosed with Spinal Muscular Atrophy Type I.

Topics review the basics of the main care options for newly diagnosed SMA Type I:

- What is Non-Invasive Respiratory Care?
- What is Invasive Respiratory Care?
- What is Palliative Care?

And, where you can go for support and guidance.

New Care Series Publication Coming Soon!

The Families of SMA Medical Advisory Council (MAC) is busy working on a new publication for SMA families and professionals. *Understanding SMA and Musculoskeletal Care for SMA*. Check out the FSMA website for further updates.

Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role

Wednesday, June 20, 2012
DoubleTree by Hilton Hotel
Bloomington — Minneapolis South.



Sponsored by:



www.curesma.org

My My SMA

By Lillian McNair, SMA Type I, Kindergarten, Finalist

Once upon a time there lived a girl named Lily. She was beautiful. She lived with her mommy and daddy. Lily had SMA. She needs help to walk and talk. Lily is a loving girl who likes to write. She is very smart.

Lily was taking a walk in her wheelchair. Lily liked to go on walks with her nurse Katie. She loves Lily. Lily likes to go to her Grandma Cheryl's house. Lily loved her Grandma. She likes reading to Lily. When she visits, Lily has fun reading. Lily was a good reader! Lily talks with a journal. She loved journaling!

Lily has a ventilator to help her breathe. Lily likes her ventilator. Lily has a machine that helps her swallow by suctioning her mouth. She has lots of Saliva. She needs lots of suctioning from her nurses. She likes her nurses. Her nurses are nice.

Lily has a machine that helps her eat through her tummy. Mommy wishes she had a tube in her tummy. Only special people have them. Lily also has oxygen to help her when she can't breathe. She likes oxygen. Lily can't breathe on her own. Lily has a machine that says what her oxygen levels are and heart rate. It is a good machine. When I am sick it makes a sound to let my mommy know I need help. My mommy always knows!

Last night I got sick and dropped my sats. I got scared. My mommy came in and rescued me. She gave me kisses. I felt better. Lily was a brave girl. She was so brave and strong. Lily wasn't made like everyone else. She was made like God wanted. I'm special because my mind is strong and diverse. Diversity means we are all special in our own way!

