



DIRECTIONS

FAMILIES OF SPINAL MUSCULAR ATROPHY



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Families of SMA
Research. Support. Hope.

Families of Spinal Muscular Atrophy Provides Record Number of Resources and Support to SMA Families Worldwide

Along with funding SMA research, Families of Spinal Muscular Atrophy provides thousands of families with vital family support and resources that help families navigate life with SMA. Families of SMA gives a stable, unbiased platform for families to live active, engaged and hopeful lives.

Families of SMA is a resource for unbiased support. As caring parents and professionals, we can offer SMA families guidance and understanding when it is most needed. We provide services to individuals and families such as medical information and equipment, resources on SMA, an annual conference, local community support through our vast volunteer network, regular publications and web-based support.

In just the past year, Families of SMA was contacted by over 400 newly diagnosed SMA families. Over 3,000 support items were shipped during the year, including Newly Diagnosed Care Packages, Wagons for SMA families and information for Medical Providers. We provided 300 pieces of vital medical equipment to SMA families across the United States.

Some of the resources provided by Families of SMA include:

- Providing SMA information to all newly diagnosed families helping each to understand and manage the disease better
- Sending care packages of toys that have been recommended for SMA children



- Housing a shared medical equipment pool which is available to families at no cost
- The Annual SMA Conference; the nation's largest gathering of those affected by SMA and leading researchers from around the world.

Families affected by SMA turn to Families of Spinal Muscular Atrophy for information, guidance, encouragement and most importantly HOPE.

To contact the Families of SMA Family Support Department, please email FamilySupport@fsma.org.



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

Submissions

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

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

Change of address

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

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

On the Cover: Emma and Ruby Cannady, SMA Type III and SMA Type II

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.

August is SMA Awareness Month



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

Join Families of SMA in getting involved with the SMA community this August.

Show Your Support! The Families of SMA Awareness Ribbon is one of the recognized symbols in the SMA community. SMA prevalence is now one in every 6,000 children. Show your support for people with SMA by wearing the FSMA Awareness Ribbon. You can wear it as a pin on your shirt, a magnet on your car, a badge on your blog, or you can even make it your Facebook profile picture. By wearing the ribbon you can help educate the population on the potential of people with SMA! To purchase the FSMA Awareness Ribbon for your shirt, car, or refrigerator, visit <http://www.fsma.org/Fundraising/Merchandise/>.

SMA Candle Lighting. The Annual SMA Candle Lighting will be Saturday, August 9th. 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.



2014 ANNUAL SMA CONFERENCE

June 12th–June 15th, 2014
Gaylord National Resort
Washington, DC

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

SMA Conference Goals

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



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

The Gaylord National Resort & Convention Center has been carefully selected to meet the many needs of Families of SMA as the 2014 destination for the Annual SMA Conference. The hotel anchors the 300 acre National Harbor waterfront entertainment district, located eight miles south of Washington, DC and is situated along the shores of the Potomac River, across the river from Alexandria, Virginia. The hotel contains 2,000 guest rooms, seven restau-

rants, and features a 19-story glass atrium with views of the Potomac River. For your convenience the rooms are equipped with a mini-fridge, coffee maker and a 32 inch flat screen

TV along with other modern amenities. Take care of business with in-room high-speed Internet access. This hotel also has a smoke free policy.

After a day of meetings, indulge in outstanding restaurants and lounges onsite (like the Old Hickory Steak House, Pienza Restaurant, National Pastime Sports Bar & Grill and more). Relax in the Junior Olympic-sized, 24-meter lap pool, perfect for families and fitness. Enjoy the spectacular nightly Fountain Shows which project a majestic display of lights, sound and dancing water that shoots 60-feet high. Or, take a stroll through National Harbor which is an expansive waterfront that offers restaurants, shopping, entertainment, marinas and piers.

To register for the 2014
Annual SMA Conference, please visit
www.CureSMA.org


Families of SMA
Support. Research. Hope.



Legislative Updates

Being in our nation's capital for this year's conference allows us to take advantage of the close proximity we have to congress. The conference will incorporate many legislative topics and activities with representatives and government agencies such as the NIH, NINDS and FDA. The goal of these efforts towards our policy-makers is to significantly raise awareness of SMA, increase the level of federal support dedicated towards SMA research, and facilitate the development of therapies through public-private partnerships between Families of SMA, the federal government and the bio-pharmaceutical industries. More information about these possible opportunities will be available soon at www.curesma.org.

Newly Diagnosed Conference Program

Thanks to the generous funding provided by the Erin Trainor Memorial Fund, Families of SMA covers the registration fees for the Annual SMA Conference for all families newly diagnosed since the last conference. The mission of the Erin Trainor Memorial Fund (ETMF) is to generate substantial funds to be able to provide conference scholarships, allowing newly diagnosed SMA families/individuals the opportunity to attend the Annual SMA Conference. Our goal is to allow as many newly diagnosed SMA families as possible to attend and experience the benefits of this amazing conference. The meeting gives families the opportunity to gather critical care and daily living infor-

mation early after diagnosis where they can learn directly from experienced SMA physicians and network with other families. This program is automatically offered to all newly diagnosed families that contact Families of SMA.

For more information, please email familysupport@fsma.org

Continuing Medical Education Conference

Families of SMA offers a third component of Care for medical professionals at the Annual SMA Conference.

This year's Continuing Medical Education Conference will be held in conjunction with the Annual SMA Conference and Researcher Conference. The CME Conference, titled "Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role", for medical professionals, will be held on Friday, June 13th at the Gaylord Hotel. The conferences have previously covered two components of SMA; Research and Support. The Research aspect covered scientific, research & clinical updates, while the Support aspect included the family support workshops and family networking. This third conference provides an exciting component of Care to the Families of SMA Conferences by educating medical professionals on SMA. Families of SMA partners with an accredited hospital who can provide continuing medical education credits for medical professionals, and offers a full-day of lectures and educational materials to all attendees.

Exhibitor Opportunities

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

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



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



Contact us

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

FSMA Announces the Schedule and Workshop Titles for the 2014 Annual SMA Conference

The schedule is announced for the 2014 Annual SMA Conference held June 12th – June 15th, 2014 in National Harbor, MD.

Families of SMA released the schedule for the 2014 Annual SMA Conference, which will be held at the Gaylord National Harbor, in National Harbor, MD. The schedule includes the timeline of workshops and events, including the Family Fun Fest/Carnival, the Family Friendly Poster Session & SMA Dance Party and the PJ Party & Movie Night. Registration for the conference is now open. This conference will fill up fast so register while space and hotel rooms are still available.

Please visit the Conference Link on our website to see the schedule at www.CureSMA.org



Families of SMA is thrilled to announce the workshops offered at the 2014 Annual SMA Conference!

These 40 workshops cover the following topics:

- Breathing Basics and Care Choices for Type I
- Breathing Basics and Care Choices for Type II & III
- Genetics and Reproductive Options for SMA Families
- Hands on Physical Therapy
- Medical Management For Adults with SMA
- Young, Hip and Disabled: College, Work and More
- Obtaining Government Benefits
- Good Nutrition Principles and Round Table for Oral Feeders
- Nutrition -for G-tube Feeders
- Orthopedic Management
- Aquatic Physical Therapy for Fun and Function
- Recognizing and Managing Pain in SMA
- Ethics in Research
- Driving and Community Mobility
- Care for the Caregiver
- Dad's Time: A Workshop for Father's Only
- Event Success & Fundraising Resources
- Adult with SMA Roundtable
- Grandparents Talk it Out - Type I
- Grandparents Talk it Out - Type II and III
- Grieving and Loss
- Healing The Grieving Heart - Part I
- Healing the Grieving Heart – Part II
- It's a Wonderful Life
- Kids Talk it Out (Ages 10 and Older)
- Kids Talk it Out (Ages 6 to 9)
- Life Care Planning
- Keepsake Creation: Grieving Through Art
- Sharing Your Experience Type I
- Sharing Your Experience Type II
- Sharing Your Experience Type III
- Sibling Workshop (ages 5 – 11)
- Sibling Workshop (ages 12 – 17)
- Writing Our Stories: A Parent's Workshop in Healing Through Creativity
- Toy Adaptation
- What it Means to be a Teen on Wheels
- Yoga for SMA
- Dating, Relationships and Sexuality
- Personal Care Assistants – How to find, hire & manage your PCA's

ANNUAL SMA CONFERENCE 2014

Gaylord National Resort | Washington, DC June 12-15

Agenda

Wednesday, June 11th

3:00pm – 9:00pm Registration/Folder Pickup Open for all Conference Attendees

Thursday, June 12th

7:30am – 5:30pm Registration Open for all Conference Attendees

12:00pm – 4:00pm Newly Diagnosed Program
(For Newly Diagnosed Families Only)

6:00pm – 8:30pm Meet and Greet for all conference attendees/Family Fun Fest

Friday, June 13th

7:30am – 4:00pm Registration Open

7:30am – 5:00pm Exhibitors

7:30am – 8:45am Continental Breakfast

9:00am – 10:15am General Session

10:30am – 12:30pm Workshop Session #1

12:30pm – 2:00pm Lunch Break – on your own

2:00pm – 3:30pm Workshop Session #2

6:00pm – 7:30pm Family Friendly Research Poster Session

7:30pm – 9:30pm Dance Party

Saturday, June 14th

7:30am – 5:00pm Registration Open

7:30am – 5:00pm Exhibitors

7:30am – 8:45am Continental Breakfast

9:00am – 10:30am General Session – It's a Wonderful Life Panel

10:45am – 12:15pm Workshop Session # 3

12:15pm – 1:45pm Lunch Break – on your own

1:45pm – 3:15pm Workshop Session # 4

3:30pm – 4:45pm Workshop Session # 5

6:45pm – 9:30pm PJ Party/Movie Night

7:30pm – 10:00pm Adults with SMA Social

Sunday, June 15th

7:30am – 9:00am Continental Breakfast

9:15am – 11:30am Closing General Session/Researcher Q & A

Food Items Included for all registered attendees:

- 3 continental breakfasts held on Friday, Saturday and Sunday mornings
- Candy and possible light snack at the Meet and Greet
- Popcorn served at PJ Party & Movie Night



THE GAYLORD NATIONAL HARBOR HOTEL, WASHINGTON, DC

Friday, June 13, 2014

Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role



Jointly Sponsored by:

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



FSMA Continuing Medical Education Conference at The 2014 Annual SMA Conference in National Harbor, MD

The third CME Conference for medical professionals will be on Friday, June 13th in National Harbor, MD - Washington, DC at the Gaylord National Harbor Hotel.

This year's CME conference will be held in conjunction with the Annual SMA Conference and the Researcher Conference at the Gaylord Hotel. The CME Conference is titled "Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role".

This CME Conference for medical professionals is a great step that Families of SMA is taking toward broadening our support programs by educating medical providers. The Annual Conferences previously covered two components of SMA; Research and Support. The Research aspect covered scientific, research & clinical updates, while the Support aspect included the family support workshops and family networking. This third conference provides an exciting component of Care to the Families of SMA Conferences by educating medical providers on SMA. Families of SMA is thrilled to partner with the University of Wisconsin School of Medicine and Public Health, who are accredited to provide CME credit for medical professionals.

Upon completion of this course medical professionals will learn how to:

- 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 is a one day event for medical professionals only. The course will focus on the diagnosis of



infants and children with neuromuscular weakness, clinical application of care standards to individuals with Spinal Muscular Atrophy, and discussion of pathophysiology and therapeutic strategies for intervention. Members of the Families of SMA Medical Advisory Council, as well as guest speakers from Institutions across the U.S., 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.

Please visit the CureSMA.org website for more information on the CME Conference.



FSMA Offers Valuable Scholarship Opportunity for the Continuing Medical Education Conference on SMA

The Families of SMA Chapters will again have the opportunity to send one Medical Provider Delegate from each area to participate in the 2014 Continuing Medical Education Conference through a scholarship program.

Families of SMA is offering a Continuing Medical Education (CME) Conference called "Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role", for medical professionals on Friday, June 13, 2014.

This scholarship program will enable each chapter to have one Medical Provider from their area participate in a conference led by some of the leading SMA experts in the country. This truly is an incredible opportunity to ensure that another 31 Medical Providers from across the US have been educated by the FSMA

Medical Advisory Council as well as other top SMA experts. It is our hope that this will heighten SMA Awareness and SMA Education throughout the United States.

Under this program, Medical Providers will have the following covered:

- Registration
- Flight
- Hotel

We look forward to hosting these professionals and hope that once they return home they are able to spread awareness about SMA throughout their medical communities.

Thank to generous funding from Sweet Baby Zane, The Miller McNeil Woodruff Foundation and The Pennington Foundation Scholarship Who is Providing Opportunities for Medical Professionals to Attend the Families of Spinal Muscular Atrophy 2014 Continuing Medical Education Conference

Sweet Baby Zane has made an amazing opportunity available to 30 medical providers from across the country to attend. The Miller McNeil Woodruff Foundation has generously donated scholarships for seven medical providers from the state of Arkansas. The Pennington Foundation has provided an exciting opportunity for five medical providers from the state of Louisiana to attend the Families of Spinal Muscular Atrophy (FSMA) 2014 Continuing Medical Education Conference (CME) in National Harbor, MD at the Gaylord National Resort on Friday, June 13th.

These scholarships will cover registration fees, travel costs and lodging for two night's hotel so medical professionals can attend this one day event. All Guidelines and reimbursement information will be sent to scholarship recipients upon receiving notification of their scholarships.



Everyone at Families of SMA would like to extend our sincerest thanks to Sweet Baby Zane, The Miller McNeil Woodruff Foundation and the Pennington Foundation for donating scholarships for medical providers to attend the CME Conference in loving memory of Zane Schmid and Miller Woodruff. We would also like to thank Keith and Hillary Schmid, Patrick and Meredith Woodruff and the Pennington Foundation for their support of Families of SMA and its important programs.

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

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

Family Guide to Research



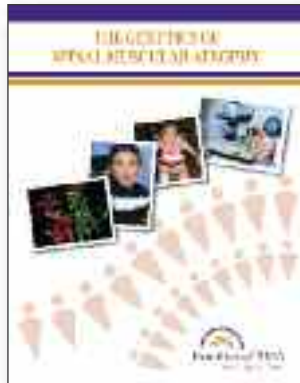
Families of SMA Family Support and Patient Services



Breathing Basics



The Genetics of Spinal Muscular Atrophy



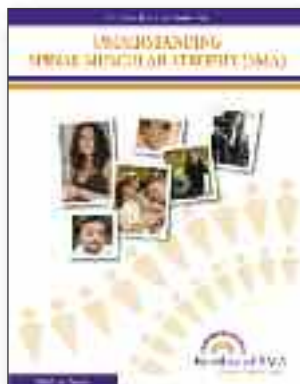
Nutrition Basics



Caring Choices



Understanding Spinal Muscular Atrophy (SMA)



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

Families of SMA Announces 2014 Legislative Agenda and Exciting Advocacy Opportunities; The Families of SMA Day on Capitol Hill



Our legislative advocacy efforts will culminate with “The Families of SMA Day on Capitol Hill” on June 11, 2014, the day before the official start of The Annual SMA Conference.

Families of SMA is pleased to present our Legislative Agenda for 2014 and to encourage the community’s active participation in several exciting legislative advocacy opportunities leading up to The 2014 Annual SMA Conference taking place in the nation’s capital, Washington, D.C. The initiatives described below will support and foster our research agenda and facilitate therapy development and care improvement programs sponsored by Families of SMA.

The Families of SMA Legislative Agenda for 2014 is designed to support and buttress the Research Agenda and to facilitate the development of innovative therapies and care protocols for SMA. Over the past several years, Families of SMA has built a strong relationship with our partners in the federal government and federal funding for SMA-related research has nearly doubled over the past decade due in part to the SMA community’s advocacy efforts. This federal support has served as a critical bridge supporting necessary translational research that builds upon the seed funding provided by Families of SMA for proof-of-concept studies and the large-scale clinical development supported by our colleagues at biotechnology companies and pharmaceutical corporations.

The 2014 Legislative Agenda has three components:

1. **Newborn Screening:** Facilitating the addition of SMA to the federal “Recommended Uniform Screening Panel” (RUSP), the federal government’s recommended list of heritable disorders that should be included in each state’s newborn screening program
2. **Food and Drug Administration (FDA) Engagement:** Fostering formal interactions with the FDA to provide families with the opportunity to share the SMA patient perspective with regards to the FDA’s review of SMA therapies submitted for federal approval
3. **Awareness:** Creating opportunities for the SMA community to educate lawmakers and policymakers about SMA and the importance of devoting increased federal resources towards SMA research and clinical development opportunities

Newborn Screening:

Newborn Screening is a public health program that began 50 years ago to test every newborn in the U.S. shortly after birth for a series of serious, but treatable heritable disorders. Babies have blood collected via a heel-prick within 24-48 hours after birth and the blood is tested in state public health laboratories. If a newborn tests positive for one or more of the disorders on the state’s screening panel, the family is notified, follow up confirmatory testing is completed, and, if the diagnosis is confirmed, the newborn is connected with the appropriate medical care so that treatment can begin.

Each state operates its own newborn screening program, but over the past decade the federal government has

greatly increased its participation and involvement. Since 2005, the federal government has maintained a Recommended Uniform Screening Panel (RUSP), which is a list of disorders that the federal government urges each state newborn screening program to include on its state panel. While state compliance with the RUSP is voluntary, the states are eligible for federal funding to support their newborn screening program only if they agree to screen for the disorders on the RUSP.

FDA Engagement:

The Food and Drug Administration (FDA) is the federal agency responsible for approving all medications and therapies marketed in the U.S. In order to receive FDA approval, a drug must be found both safe and effective. The bar for FDA approval is quite high and the process is complex, expensive, and time consuming. Typically, the approval process can take several years, cost tens of millions of dollars, and requires the successful conclusion of three phases of clinical trials to test the safety and efficacy of the therapy, as well as the scalability and replicability of preliminary results.

SMA is classified as an “orphan” disease (i.e., disorders that affect less than 200,000 persons in the U.S.) and Families of SMA’s industry partners are planning to utilize the various regulatory tools available to facilitate the approval of SMA therapies. Families of SMA is working closely with industry and the FDA on several initiatives aimed at fomenting greater understanding of SMA at the FDA and reaching consensus on biomarkers (biological tissues or processes that can be used to measure the effectiveness of a therapy at mitigating the impact of SMA) and outcome measures to demonstrate the effectiveness of SMA therapies.

Additionally, Families of SMA is working closely with its partners in government, academia, and the patient advocacy community to create one or more formal listening sessions for the SMA community to share the patient perspective. The goal of these sessions is to educate the FDA about SMA patients’ threshold for risk and expectations for outcomes. All therapies have side effects and varying degrees of effectiveness, so determining the level of risk that SMA patients find acceptable in a therapy and measuring the community’s expectations for an effective outcome can have a crucial impact on FDA’s determination of whether a therapy under its consideration should be determined safe and effective.

Families of SMA is seeking community involvement in making outreach to the FDA to encourage the scheduling of one or more formal listening sessions. In addition, an important component of any interaction with the FDA is to provide empirical data to support anecdotal evidence of the community’s viewpoint on risk and expected outcomes. Patient involvement in Families of SMA sponsored data collection surveys is critically important in ensuring the effectiveness of this initiative.

Lawmaker & Policymaker Awareness:

The policies that Congress debates and the laws it passes affect nearly all aspects of the everyday lives of families touched by SMA. Some of Congress’s responsibilities include providing funding for vital biomedical research and setting policies related to newborn screening and drug approval. It is important that the SMA community stay informed and involved to make their voice heard on these and other legislative issues of importance.

With The Annual SMA Conference taking place in Washington, D.C. in 2014, the SMA community has a unique opportunity to further increase the awareness of SMA among lawmakers. Introducing Members of Congress to our community is the single most effective way that we can impact federal policies that will support efforts to develop therapies and clinical management protocols.

The Families of SMA Day on Capitol Hill will take place on Wednesday, June 11th, 2014, the day before the official start of The Annual SMA Conference. Participating families will begin the day with a breakfast on Capitol Hill, which will include a training session on how to be an effective advocate. Families then will move on to their scheduled meetings with their elected officials; Families of SMA will set up the meetings and provide any and all necessary talking points and “leave-behind” materials. We anticipate that this will be a highly enjoyable and educational experience for all who participate.

If you have any questions about the Families of SMA 2014 Legislative Agenda or The Families of SMA Day on Capitol Hill, please contact Legislative Coordinator Spencer Perlman at spencer@fsma.org.

New Federal Regulations for Home and Community-Based Services (HCBS) Program: Offers Greater Autonomy, Choice, and Independence

The Centers for Medicare and Medicaid Services (CMS) has published a Final Rule implementing several changes to the regulations governing Medicaid's home and community-based services (HCBS) benefits. These critical benefits permit many persons with disabilities to receive various long-term services and supports in their home or a community-based setting so as to avoid institutionalization in a hospital, nursing home, or other facility.

The Final Rule ensures that state Medicaid HCBS programs are consistent with several important changes enacted as part of the Affordable Care Act (ACA). Most significantly, the new regulations define "home and community-based settings" and "person-centered plans of care" in a manner that greatly enhances an individual's autonomy, choice, and independence, and better meets an individual's personal needs. The regulations go into effect on March 17, 2014.

What are Home and Community-Based Services (HCBS)?

Home and community-based services (HCBS) are case management, homemaker, home health aide, personal care, adult day health, habilitation,¹ and/or respite care services that are provided to eligible Medicaid beneficiaries in their home or community. The HCBS program serves a variety of targeted groups, including individuals with physical disabilities, intellectual or developmental disabilities, and/or mental illness that would otherwise need to receive care in an institution such as a hospital or a nursing facility. In order to be eligible for HCBS,

individuals must meet state-established needs-based criteria that consider various factors, and may take into account the ability of the individual to perform two or more activities of daily living (ADL).²

HCBS is provided through the Medicaid program, which is a means-tested program jointly run and funded by the federal government and state governments that provides health care and long-term care to eligible low-income children, pregnant women, persons with disabilities, seniors, caretakers, or other adults. Federal law establishes the parameters of Medicaid, including mandatory and optional eligibility standards and services, but each state is responsible for implementing its own Medicaid program within these rather broad guidelines. Consequently, there is a great deal of variation between state Medicaid programs. In general, though, most children with disabilities and many adults with disabilities are eligible to receive Medicaid benefits and qualify for HCBS in all states.

How is Medicaid Eligibility Determined?

In order to be eligible for Medicaid, a person must meet certain income and/or categorical requirements set in federal statute and federally-approved state Medicaid plans. By law, if a child or adult with a disability earns less than the relevant state Medicaid income threshold and/or is eligible to receive cash assistance through the federal Supplemental Security Income (SSI) program, that individual is automatically eligible to receive Medicaid benefits; in most instances, a disabled child's eligibility for SSI and Medicaid is determined by ignoring or waiving parental income and

assets. Many states have higher income eligibility limits for adults and children with disabilities, and persons requiring institutional-level care (and HCBS), than for non-disabled persons. Some states permit certain disabled persons to receive Medicaid benefits despite earning well above the income threshold if that person is classified as "medically needy"; these individuals are able to "spend down" to Medicaid income levels, meaning that the person may subtract their incurred medical expenses from their countable income until the net result is below the medically needy income threshold set by the state.

How long has HCBS been available through Medicaid?

HCBS first became available in 1981 when Congress added a provision to the Medicaid statute authorizing States to seek a waiver from certain federal laws in order to provide HCBS for specified populations, including the disabled, if those persons would otherwise require an institutional level of care in a hospital or nursing facility. The intent of the provision is to address a perceived "institutional bias" in Medicaid that had kept disabled persons from receiving long-term care in their home or a community setting. Since 1965, Medicaid law has required states to cover long-term services and supports provided in a nursing facility, but prior to 1981, the program did not cover many of the non-skilled personal care and supportive services required by many disabled persons to remain in the community. States receiving the HCBS waiver are able to offer eligible beneficiaries HCBS and avoid unwanted institutionalization.

¹Habilitation services are services designed to assist individuals in acquiring, retaining, and improving the self-help, socialization, and adaptive skills necessary to reside successfully in home and community-based settings; it may also include some prevocational, educational, and supported employment services.

²ADL refers to tasks such as eating, toileting, grooming, dressing, bathing, and transferring.

How have HCBS benefits changed over the past 30 years?

The HCBS waiver took on added importance after the landmark 1999 Supreme Court case *Olmstead v. L.C.* The Court determined that the institutionalization of people who could be cared for in community settings is a violation of the Americans with Disabilities Act (ADA), which had become law in 1990. Soon after the *Olmstead* decision, every state added HCBS benefits to their Medicaid program via a waiver. A state must renew their waiver with CMS every three-to-five years.³

In 2006, Congress expanded the HCBS Medicaid benefit by permitting states the option to provide such services as part of their regular Medicaid plan (i.e., no waiver required) and to permit states to provide HCBS to individuals who require less than an institutional level of care and/or who are not otherwise eligible for Medicaid. This means that individuals who do not require long-term care in a hospital or nursing facility and/or are not otherwise eligible for Medicaid in the state would be eligible to receive Medicaid HCBS in most circumstances if they meet the other HCBS program eligibility requirements and earn less than 150 percent of the federal poverty level (FPL).⁴

In 2010, the Affordable Care Act (ACA) further expanded HCBS benefits in a

number of ways. First, it permits states that operate HCBS through their regular Medicaid plan to expand eligibility to beneficiaries with income up to 300 percent of the maximum SSI benefit (about 225 percent of FPL), so long as the persons meet the level of care required in an institution. Second, it permits states to target HCBS to certain populations and to alter the type, amount, duration, and scope of such services depending on the needs of the specific population.

The ACA also authorized states to provide home and community-based attendant services and supports through a new Community First Choice (CFC) program. The law increases the federal funding contribution for the provision of CFC services as an incentive to states to implement the program. Eligible individuals under the CFC includes persons who are enrolled in Medicaid and require an institutional level of care, or who require less-than institutional care but earn less than 150 percent of FPL, and meet the needs-based criteria.

Persons participating in the CFC program may receive home and community-based attendant services and supports to assist in accomplishing ADL, instrumental activities of daily living (IADL),⁵ and health-related tasks⁶ through hands-on assistance, supervision, or cueing. The services provided through the CFC are more

robust than under Medicaid's traditional personal services benefit. In addition to attendant services, CFC programs may also provide support for transition costs (e.g., first month's rent for transitioning into a community-based setting) and services that improve independence or substitute for human assistance (e.g., non-medical transportation services).⁷

What is the purpose of the new regulations recently promulgated by CMS?

CMS never finalized its proposed regulations to implement the 2006 statutory changes to the HCBS program because the passage of the ACA in 2010 made many of the policy changes prescribed in the proposed rules moot or irrelevant. Since 2011, CMS has been working to complete new regulations that would implement the changes made to the HCBS program by the ACA and to streamline and make more consistent the various regulations governing the HCBS, which had not been updated significantly by CMS since 1994.

What are the specific provisions in the Final Rule?

The Final Rule implements changes to the HCBS waiver program to require greater transparency and consistency from states in amending and/or renewing their waivers, implements stronger requirements for states wishing

³In addition to offering case management, homemaker, home health aide, personal care, adult day health, habilitation, and respite services, a state with a HCBS waiver may offer the following optional services: (1) live-in caregiver support, (2) home accessibility adaptations, (3) vehicle modification, (4) non-medical transportation, (5) specialized medical equipment and supplies, (6) assistive technology, (7) personal emergency response system, (8) community transition services, (9) skilled nursing, (10) private duty nursing, (11) adult foster care, (12) assisted living services, (13) chore services, (14) adult companion services, (15) training and counseling services for unpaid caregivers, (16) consultative clinical and therapeutic services, (17) individual directed goods and services, and/or (18) bereavement counseling.

⁴At present, 12 states offer this expanded HCBS benefit to persons requiring a less-than institutional level of care (CA, CO, CT, FL, IA, ID, LA, MT, NC, NV, OR, and WI) and four other states are in the planning process (DE, IN, MD, and MN).

⁵IADL includes (but is not limited to) meal planning and preparation; managing finances; shopping for food, clothing, and other essential items; performing essential household chores; communicating by phone or other media; and traveling around and participating in the community.

⁶"Health related tasks" are defined as specific tasks related to the needs of an individual which can be delegated or assigned by licensed health care professionals to be performed by an attendant.

⁷Two states presently offer the CFC option (CA and OR). Eight states are in the planning process (AR, CO, LA, MD, MN, MT, NY, and TX).

New Federal Regulations for Home and Community-Based Services (HCBS) Program: Offers Greater Autonomy, Choice, and Independence (*continued*)

to establish the expanded HCBS benefit authorized by Congress in 2006, and implements certain requirements for the CFC program. Most importantly, the regulation aligns definitions and requirements within the three HCBS programs (waiver, expanded, and CFC), defines for the first time a “Home and Community-Based Setting”, and details the requirements of a “person-centered service plan” that forms the basis of all HCBS programs. These new definitions ensure that disabled persons utilizing HCBS will have greater autonomy, choice, and independence:

• **Home and Community-Based Setting:** The new regulations require “home and community-based settings” to have the following characteristics:

- The setting must be integrated in and support full access of individuals to the greater community, including (a) opportunities to seek employment or work in integrated settings, (b) engage in community life, (c) control personal resources, and (d) receive services in the community in the same manner as other persons in the community.
- A setting must be selected by the individual from among options that include (a) non-disability specific settings and (b) an option for a private unit in a residential setting. Setting options must be based on the individual’s needs, preferences, and resources available for room and board.
- The setting must ensure (a) an individual’s rights of privacy, dignity, and respect, and freedom from coercion and restraint; (b) optimize (but not regiment) individual initiative, autonomy, and independence in making life choices, including daily activities, physical environment, and with whom to interact; and (c) facilitate individual choice regarding services and supports, and who provides them.

Provider-owned or controlled residential

settings also must meet additional requirements.

- The unit or dwelling must be a specific physical place that can be owned, rented, or occupied under a legally enforceable agreement by the individual receiving services, and the individual must have, at a minimum, the same responsibilities and protections from eviction that tenants have under the landlord/tenant laws of the relevant jurisdiction.
- Each individual must have privacy in their sleeping or living unit, including entrance doors that are lockable by the individual with only appropriate staff having keys to doors, a choice of roommates (if sharing units), and the freedom to furnish or decorate their sleeping or living units.
- Individuals must have the freedom and support to control their own schedules and activities, have access to food at any time, and able to have visitors of their choosing at any time.
- The setting also must be physically accessible to the individual.
- The regulations also make clear that the following settings cannot be classified as a home and community-based setting:
 - Nursing facility
 - Institution for mental diseases
 - Intermediate Care Facility for Individuals w/ Intellectual Disability (ICF/IID)
 - Hospital
 - Any setting that is located in a building that is also a publicly or privately operated facility that provides inpatient institutional treatment, on the grounds of, or immediately adjacent to, a public institution, or that has the effect of isolating individuals receiving Medicaid HCBS from the broader community.

• **Person-Centered Service Plan:** The new regulations detail the requirements of the person-centered planning process and the components of the person-centered services plan.

- The **person-centered planning process** must be lead and driven by the individual or the individual’s representative (i.e., legal guardian or other comparable individual) when appropriate. The process must:
 - ✓ Include people chosen by the individual.
 - ✓ Provide necessary information and support to ensure that the individual directs the process to the maximum extent possible and is enabled to make informed choices and decisions.
 - ✓ Be timely and occur at times and locations of convenience to the individual.
 - ✓ Reflect cultural considerations of the individual and be conducted by providing information in plain language and in a manner that is accessible to individuals with disabilities and persons who are limited English proficient.
 - ✓ Include strategies for solving conflict or disagreement within the process, including clear conflict of interest guidelines for all planning participants.
 - ✓ Offer choices to the individual regarding the services and supports the individual receives and from whom.
 - ✓ Include a method for the individual to request updates to the plan, as needed.
 - ✓ Record the alternative home and community-based settings that were considered by the individual.
- The **person-centered service plan** must reflect the services and supports

that are important for the individual to meet the needs identified through an assessment of functional need, as well as what is important to the individual with regard to preferences for the delivery of such services and supports. The plan must:

- ✓ Reflect that the setting in which the individual resides is chosen by the individual and adhere the requirements of a home and community-based setting as defined in the regulation.
- ✓ Reflect the individual's strengths and preferences.
- ✓ Reflect clinical and support needs as identified through an assessment of functional need.
- ✓ Include individually identified goals and desired outcomes.
- ✓ Reflect the services and supports (paid and unpaid) that will assist the individual to achieve identified goals, and the providers of those services and supports, including natural supports (i.e., unpaid supports that are provided voluntarily to the individual).
- ✓ Reflect risk factors and measures in place to minimize them, including individualized backup plans and strategies when needed.
- ✓ Be understandable to the individual receiving services and supports, and the individuals important in supporting him or her.
- ✓ Identify the individual and/or entity responsible for monitoring the plan.
- ✓ Be finalized and agreed to, with the informed consent of the individual in writing, and signed by all individuals and providers responsible for its implementation.
- ✓ Be distributed to the individual and other people involved in the plan.
- ✓ Include those services that the individual elects to self-direct.
- ✓ Prevent the provision of unnecessary or inappropriate services and supports.
- ✓ Be reviewed and revised upon reassessment of functional needs at least every 12 months, when the individual's circumstances or needs change significantly, or at the request of the individual.



FSMA Awards \$600,000 for Basic Research to Identify New Approaches for SMA Therapies

Families of SMA is proud to announce our new basic research funding for 2014. We are leading the way to a world without SMA by advancing a comprehensive research program, of which basic research is a key component.

Basic research helps us understand what causes SMA, by revealing new and better ways of making drugs. There are currently five SMA drugs being tested in clinical trials, advances that would not be possible without the important discoveries made in basic research.

The latest issue of "Compass," Families of SMA's research newsletter, announces these seven basic research grant awards. Visit www.cureSMA.org to download a copy and read a description of the newly funded projects.

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

What function does SMN protein perform in motor neurons?

Dr. Han from the University of Colorado will investigate what controls the correct distribution of SMN protein into motor neurons, providing a greater understanding of SMN function.

What tissues are affected by reduced SMN protein?

The grants to Dr. Ko at the University of Southern California, to Dr. Ebert at the Medical College of Wisconsin, and to Dr. Burnett will help determine the exact cells that influence SMA disease pathology.

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

The funding to Dr. Murray of the University of Edinburgh will explore the molecular pathways controlling degeneration in SMA motor neurons to identify possible new drug targets in these pathways. Dr. Kothary from the Ottawa Hospital Research Institute will investigate non-SMN mediated mechanisms that provide therapeutic benefit in mouse models of SMA.

What are the best measures in animal models of SMA to predict human drug responsiveness?

Drs. Lutz and Bogdanik from Jackson Laboratory will lead a multi-center team investigating electrophysiological endpoints, which are currently used in patients, in drug testing in mice.

Opportunities for Involvement in Clinical Studies for SMA Patients and Families

Several clinical studies are currently enrolling SMA patients and families. Please see below for more details about each study:

1. Novartis will be recruiting for an SMA adult Biospecimen project in Massachusetts.

If you are at least 18 years of age, and have genetic documentation of Spinal Muscular Atrophy (SMA) diagnosis, you may be asked to join a biomarker non-investigational drug research study to help Novartis learn more about your disease and support SMA research. Participants will receive physicians visits at no cost, exams and payment for time and effort including travel. For more information, please contact Bay State Clinical Trials, 521 Mount Auburn St., Ste. 209, Watertown, MA, 02472 if you would like to learn more about this research study.

2. NeuroNEXT Infant Biomarker Study for SMA.

This study is enrolling infants less than 6 months of age who has been diagnosed with SMA. This study is looking for 5 more infants with SMA to enroll in the Infant Biomarker Study. There is a critical need for information about SMA biomarker in infants with SMA, and we believe this data will be essential for the design of future clinical trials in infants. There are 15 sites participating in this clinical study across the US.

3. Families of SMA has funded the following research for the Care Program.

Project specific updates:

- a) Oscar Mayer, MD at The Children's Hospital of

Philadelphia for "Decisions Related to Goals and Limitations of Care and the Challenges in Making Them for Parents of Children with SMA". This site is preparing to recruit parents for the project. The goal is to have 50 parents recruited. Families of SMA will assist with the site recruitment by promoting the project on the FSMA website.

- b) Timothy Lotze, MD at Texas Children's Hospital for "Quality Improvement Project to Reduce Gaps in Care in the Hospital Setting for Children with SMA Type I". This site has started interviewing families for their project.
- c) Matthew Halanski, MD at University of Wisconsin for "Spinal Muscular Atrophy Database: a Multicenter Multidisciplinary Assessment". The University of Wisconsin team has been working to have site ready by the end of December. The other sites are working on their IRB data use agreements.
- d) Kathryn Swoboda, MD at University of Utah for "Glucose Load Tolerance and Fasting in SMA Type II". This project is currently recruiting patients.

Please note that these are SMA Clinical Studies which are different than SMA Clinical Interventional Trials. For more information on SMA Clinical Interventional Trials please see www.clinicaltrials.gov

The NeuroNEXT Infant Biomarker Study for Spinal Muscular Atrophy Reaches Recruitment Milestone

As of October 2013, the spots for the infants without SMA have been fulfilled by 27 "Super Families." The study has also enrolled 12 infants with SMA. They are still looking for 15 more infants with SMA to enroll.

The NeuroNEXT Infant Biomarker Study, the first research study to be conducted through the NeuroNEXT Network, has reached a recruitment milestone. In 11 months time, the spots for the infants without Spinal Muscular Atrophy (SMA), or infant controls, have been fulfilled by 27 "Super Families." This is 13 months sooner than we had expected. "We sincerely appreciate the support of the advocacy networks and the friends and families affected by this disease. We know it was because of them we are able to reach this milestone ahead of our predicted schedule," stated Dr. Stephen Kolb the Principal Investigator of the study.

The study has also enrolled 12 infants with SMA. According to a census provided by the nonprofit group Families of SMA

(FSMA), the number enrolled is approximately half of the infants who have been diagnosed with SMA during our recruitment time period who likely fulfill our inclusion criteria. We are very pleased to be on target with our proposed recruitment rate for this population.

We are still looking for 15 more infants with SMA to enroll in the Infant Biomarker Study. There is a critical need for information about SMA biomarker in infants with SMA, and we believe this data will be essential for the design of future clinical trials in infants.

If you have an infant less than 6 months of age who has been diagnosed with SMA and you would like to learn more about this study, please contact the SMA Biomarker team toll free at 1-855-SMA-BIOM or go to the You Tube video by typing in your search engine, SMA Biomarker Video.

Isis Pharmaceuticals to Investigate a Higher Dose of ISIS-SMNRx in Children with Spinal Muscular Atrophy

Isis Pharmaceuticals, Inc. announced on November 22, 2013 that it plans to add a 12mg cohort to the ongoing Phase 1b/2a study evaluating ISIS-SMNRx in children with spinal muscular atrophy (SMA). In addition, a 12mg dose will be included in an open-label extension study for those children who have completed dosing in previous studies. The addition of the 12mg cohort will allow for the investigation of this dose in support of the Phase 2/3 registration-directed program planned to begin next year.

"We are pleased to be able to increase the dose of ISIS-SMNRx based on the safety and tolerability data we have seen to date in both infants and children with SMA. We have already begun the infant 12 mg dose cohort and look forward to sharing data from this study early next year. Considering the encouraging safety profile we have observed in children with SMA in the lower dose cohorts, the evaluation of the 12mg dose will provide additional information as we move into our Phase 3 program. We are also pleased that after reviewing the data, the FDA agrees that we can proceed in our investigation of a higher dose," said B. Lynne Parshall, chief operating officer at Isis. *"This addition of patients to the ongoing Phase 1b/2a study does not change our plan to report data on the first three cohorts by early next year."*

The Phase 1b/2a study of ISIS-SMNRx is an open-label, multiple-dose, dose-escalation study designed to assess the safety, tolerability and pharmacokinetic profile of the drug in children with SMA between the ages of 2-15 who are medically stable. In the ongoing Phase 1b/2a study in children with SMA, all patients have completed dosing in the initial three dose cohorts (3mg, 6mg and 9mg) and ISIS-SMNRx has been well tolerated over multiple doses. Patients who have participated in the Phase 1b/2a study are eligible to enter an open-label

extension study, which is designed to provide a single additional dose of 12mg to the more than 50 children with SMA who are eligible to roll over into this study. The investigation of the 12mg dose in both studies is anticipated to begin in December or January.



ABOUT ISIS-SMNRx

ISIS-SMNRx is designed to alter 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-SMN(Rx) for the treatment of patients with SMA. Isis is currently in collaboration with Biogen Idec to develop and potentially commercialize the investigational compound, ISIS-SMN(Rx), to treat all types of SMA. Under the terms of the January 2012 agreement, Isis is responsible for global development and Biogen Idec has the option to license the compound until completion of the first successful Phase 2/3 study.

Isis acknowledges support from the following organizations for ISIS-SMNRx: Muscular Dystrophy Association, SMA Foundation, Families of SMA and intellectual property licensed from Cold Spring Harbor Laboratory and the University of Massachusetts Medical School.



Do you have a child with Spinal Muscular Atrophy? Has your child participated in a clinical trial (a trial testing a potential treatment) within the past 10 years?

We invite you to take part in a survey conducted by Families of Spinal Muscular Atrophy (FSMA) and Parent Project Muscular Dystrophy (PPMD). FSMA is collaborating with PPMD to understand how the neuromuscular community thinks and feels about potential therapeutics and clinical trials.

To participate in the survey you must be a parent or legal guardian of an individual with spinal muscular atrophy (SMA), and your child is participating, or has participated, in a clinical trial in the United States or Canada within the past 10 years. (When we say "clinical trial" we mean a study that tests a potential treatment in humans to see if it is safe and whether it works).

The survey is anonymous and takes about 30 minutes to complete.

Please go to the following link to participate in this survey: <http://tinyurl.com/SMAParent>

PTC Therapeutics and Roche Announce Spinal Muscular Atrophy Drug Enters First Stage of Clinical Development

PTC announces that its SMA drug program, partnered with Roche, begins Phase I Safety Trials in healthy, adult volunteers. This brings the total number of SMA programs in clinical development to 5.

PTC Therapeutics, the SMA Foundation, and Roche announced that their joint research program in SMA has entered the first stage of clinical development aiming to assess safety and tolerability of an oral compound in healthy volunteers. SMA is a genetic neuromuscular disorder that leads to muscle weakness and mobility impairment and is considered the leading genetic cause of death in infants and toddlers.

Luca Santarelli, Head of Neuroscience, Ophthalmology and Rare Diseases at Roche commented: *"The compelling science behind this project and the highly synergistic alliance among Roche, PTC Therapeutics and the SMA Foundation are the groundwork for this research program. Although still early in development, we consider every step towards a therapeutic option in SMA of high importance for patients and families affected by this devastating and currently untreatable condition."*

The orally available small molecule compounds in the program are designed to target the underlying cause of the disease by increasing SMN protein levels in the nervous system, muscles, and other tissues. It is estimated that approximately one in 10,000 children are born with this rare genetic disorder.

2014 SMA Drug Pipeline Update

With this important achievement, the total number of SMA programs in clinical development is now 5. Clear evidence of progress is being seen in SMA with:

- 5 programs in clinical trials
- 15 programs total in drug development
- 12 companies involved in these drug programs



FSMA has funded almost two thirds of all the ongoing drug programs for SMA.

- 1) **Programs are advancing into the clinical trial stage of testing.** There are now 5 novel programs being testing in clinical trials for SMA with several more following behind.
- 2) **There is broad diversity of approaches in development.** Diversity is important in case a particular approach turns out not to have benefit in patients. Currently there are six drug strategies being advanced for SMA: a) Small molecule SMN enhancers, b) Neuroprotectants, c) ASOs to correct SMN2 splicing, d) Stem cell therapy, e) Gene therapy, and f) Regulators of muscle function.
- 3) **The total number of programs is growing.** There are now 15 novel SMA therapeutic programs in various stages of preclinical and clinical development, up from just 1 a decade ago. This volume is needed to balance the low probability of programs successfully advancing to final FDA approval.
- 4) **Funding and resources from government and industry are increasing.** There are now 12 companies actively investing in the SMA drug pipeline. Industry expertise and resources are essential to advance programs through the expensive and complicated clinical stages of development.

For the latest news visit the FSMA website:



www.curesma.org

Spinal Muscular Atrophy Study Opportunity From The Children's Hospital of Philadelphia

From The Children's Hospital of Philadelphia:

The Children's Hospital of Philadelphia (CHOP), with the generous support of FSMA, is conducting a study of how parents of children with Spinal Muscular Atrophy (SMA) make decisions about medical care for their children. If you are the parent or legal guardian of a child with SMA, we would like to invite you to participate in this study.

We are looking for parents or legal guardians of children with SMA. You must be 18 years of age or older. Your child must be less than 18 years old, or if older, your child does not make her/his own medical care decisions, and you make all medical care decisions on your child's behalf. You must also be able to read and write in English.

If you participate in this study, you will complete several study questionnaires. You will complete the questionnaires once, and then again 3 months later. The questionnaires take about 20 minutes to complete each time. Your responses to the questionnaires will be confidential. The questionnaires are available through a secure online hospital website, or they can

be mailed to you along with a postage-paid return envelope. You will be compensated for your time and effort. We aim to include in this study parents or legal guardians of children with SMA Types I, II, or III. We will continue to enroll participants in this study until all three of these types are well represented.

We hope that this study will help us understand better how parents think about the medical decisions they make for their children. Information obtained from this study may be useful in the future to enhance the quality of support that we give to our SMA patients and families.

If you would like to learn more about this research, and potentially to enroll in the study, please contact Karen Carroll, Senior Research Coordinator, at carrollk@email.chop.edu, or by calling 267-426-0373.

The principal investigator for the study is Oscar Hank Mayer, MD. His office is located at 3501 Civic Center Boulevard, 11th Floor, Philadelphia, PA 19104.

Project to Optimize Testing Measures for Spinal Muscular Atrophy Clinical Trials

In order to ensure accurate measurement of patients' responses to experimental drugs, a research project is being conducted to enhance and improve the rating scales currently being used in SMA clinical trials.

There are many SMA rating scales that have been used in clinical and research settings. These scales are designed to capture disease progression due to muscle weakness and/or loss of function in the upper and lower extremities. Having an accurate and highly sensitive method for measuring key changes in patient function and strength is critical to therapy development for SMA, in order to prevent potentially good drugs from being discarded unnecessarily.

The initial phase of this project utilized a statistical methodology called "Rasch analysis" to evaluate nine motor-performance rating scales currently in use around the world for SMA. (Rasch measurement methods are supported by regulatory bodies such as the U.S. Food and Drug Administration.) During the initial phase of the project, Rasch analysis was used to evaluate the effectiveness of each of the nine scales. The results of this effort were recently published in the peer-reviewed scientific journal *Muscle & Nerve*.

In phase II of this study, a new alternate scoring structure could be developed to maximize the properties of the original scales, resulting in valuable new tools for future SMA clinical trials. The strategy for doing so was discussed in a meeting with clinical investigators expert in SMA that took place in May 2013 in New York City.

"The Rasch bridging methodology enables researchers and clinicians to take the best of existing scales and move forward with an improved and more robust solution to measuring patient response, which is paramount to the acceptance of any outcome measure by the regulatory authorities. The use of this sophisticated approach for accurate appraisal is unique for neuromuscular disorders such as SMA," said Anna Mayhew, Ph.D., research physiotherapist at the MRC Centre for Neuromuscular Diseases at Newcastle University in Newcastle Upon Tyne in the United Kingdom, and one of the lead authors on the recent publication in *Muscle & Nerve*.

"We are excited about this project which will standardize important clinical outcome measures for studies in all types of SMA, and improve our ability to develop and test therapies effectively," adds John Kissel, M.D., of Ohio State University in Columbus, who served as chair of the task force.

The Patient Advocacy Group (PAG) of the International Coordinating Committee for SMA Clinical Trials, including Families of SMA, Fight SMA, Muscular Dystrophy Association, SMA Foundation and SMA Europe, is supporting the Rasch project.

Families of SMA Awards Clinical Care Research Grants



In this issue of “Compass” Families of SMA (FSMA) reviews four new Spinal Muscular Atrophy (SMA) patient care research grants. These projects were selected from applications received in response to our Clinical Care Research Project Request for Proposals (RFP).

Overview of the Clinical Care Research Program:

Since 1984 FSMA has been funding critical research to develop a treatment and cure for the disease, along with providing important resources and support for families affected by SMA. In 2013 we launched our Clinical Care Research Program to fund care research and drive improvements in patient care of SMA. The goal of this new program is to improve patient care and living with SMA.

Funding for this program will build upon the Consensus Statement for the Standard Care of SMA with data driven results on specific areas of SMA care. The results of these funded projects will help build an evidence base and demonstrate measurable, positive effects on the clinical management and lives of SMA patients. Key project information will then be used to provide the following:

- Educational programs for professional medical providers, such as the FSMA Continuing Medical Education Conference;
- New family-focused care publications, such as the FSMA Care Series Booklets; and
- Peer reviewed journal publications to influence insurance coverage.

This initial round of funding was designed to fund pilot studies which will demonstrate the feasibility of data collection, and which will then support submission of a larger study for FSMA or government funding.

Our RFP Solicited Projects on Key Areas in Clinical Care Research:

In order to fund the most critical research, our 2013 RFP solicited pilot studies that would help build an evidence base and demonstrate quantifiable, positive effects on the clinical management and lives of patients with SMA. These studies were in the following key areas:

- Respiratory interventions and breathing issues.
- Nutritional interventions and the impact of diet.
- Spinal deformity and surgical interventions.
- Psychological interventions to address mental health issues facing SMA patients and care givers, quality of life and the impact of the disease on the family.
- Studies to evaluate the impact of standing.

- Studies to evaluate the impact of medical devices.
- Studies that address the gaps in the delivery of care.

FSMA Clinical Care Research Grants are Selected by Our Medical Advisory Council:

Our Clinical Care Research Program is overseen by the FSMA Medical Advisory Council (MAC), to ensure that we fund important care research on SMA. The MAC is comprised of 16 highly recommended, leading SMA medical and clinical experts. The MAC sets the agenda for proactive, creative, collaborative leadership on issues to improve the quality of care for SMA. The MAC carefully reviews all of the Clinical Care Grant applications to make certain that they have a strong scientific merit with SMA relevance. All projects are considered on the scientific quality and relevance to the FSMA research mission of improving care for all SMA patients.

Importance of Our Clinical Care Research Grants:

The first project by **Dr. Mayer and Ms. Battista, RN, MS, CPNP, CCRC at the Children’s Hospital of Philadelphia** will fund a pilot study to assess the decision making process by parents of children with SMA on clinical care options. This work could lead to the most valuable data set on perceptions of parents with SMA Type I and SMA Type II children, and could provide the basis for a larger study. The second project by **Dr. Swoboda at the University of Utah** will fund a study to determine whether children/preadolescents with SMA Type II demonstrate impaired glucose tolerance after glucose loading and to determine whether these same children demonstrate intolerance of fasting. If the study confirms glucose metabolism abnormalities in children with SMA further study will be needed to develop appropriate management protocols and care guidelines. This pilot is needed to further understand glucose, insulin, and other responses to fasting and glucose loads in people with SMA. The third project by **Dr. Halanski at the University of Wisconsin** will fund a study to create a database of patients with SMA from multiple clinical centers. It will be used to compare outcomes across sites and to generally improve patient care. This work could help facilitate clinical management across clinical centers and establish evidence-based best practices for clinical management. The fourth project by **Dr. Lotze at Texas Children’s Hospital at the Baylor College of Medicine** will fund a project to enable health care providers to take better care of patients with SMA Type I in emergency centers and hospital settings. This work could allow quality improvement care for the hospital care of SMA Type I children driven by collaborative efforts of the healthcare providers and families to educate primary care providers.

Thank you for supporting these important Clinical Care Research projects.

Journal of Child Neurology Publishes on Nutritional Practices at a Glance: Spinal Muscular Atrophy Type I Nutrition Survey Findings

This study conducted by Dr. Kathryn Swoboda at the University of Utah consisted of a nutritional and medical history survey of children with SMA Type I collected in 2009-2011. Survey responses and comments indicate need for evidence-based nutritional guidelines for SMA.

Proactive nutritional management for children with Spinal Muscular Atrophy Type I can provide insight into improved Spinal Muscular Atrophy care. This observational study consisted of a nutritional and medical history survey of children with Spinal Muscular Atrophy Type I collected in 2009-2011. Forty-four caregiver survey responses were evaluated using descriptive statistics. Average age of Spinal Muscular Atrophy Type I subjects was 5 years (5 mo-16y). The subject cohort was composed of 22 males, 21 females, and 1 unreported. Nutrition support via feeding tube was utilized by 43 of 44 subjects. A majority of respondents reported using elemental or semi-elemental formula for subjects' essential caloric intake (34 of 44). Formula intolerance issues were reported by many caregivers (27 of 44). Half of caregivers implemented dietary changes on their own or with guidance from other families. Fifteen caregivers

consulted a registered dietitian. Survey responses and comments indicate need for evidence-based nutritional guidelines for SMA.

Unfortunately, there is a lack of any SMA-specific evidence-based research, aside from natural history data, to guide nutritional management of Spinal Muscular Atrophy. Thus, families are often left to deal with nutrition on their own. Further research and guidance is necessary for children with Spinal Muscular Atrophy Type I because improved nutrition and published nutritional management guidelines can improve clinical care and enhance quality of life for children with Spinal Muscular Atrophy Type I. As such, the aims of this survey were to bring attention to the role of nutrition in the management of children with Spinal Muscular Atrophy Type I, provide a current snapshot of the nutritional practices of these children based on their caregiver responses, and provide direction for future research into nutritional management of Spinal Muscular Atrophy Type I.

In 2013, FSMA launched a new program to fund Care Research to drive improvements in patient care in Spinal Muscular Atrophy. This program is

focused on improving care and the quality of life for SMA patients. Dr. Swoboda received funding for one of our first Clinical Care focused awards to determine whether children/preadolescents with SMA Type II demonstrate impaired glucose tolerance after glucose loading and to determine whether these same children demonstrate intolerance of fasting. If the study confirms glucose metabolism abnormalities in children with SMA further work will be needed to develop appropriate management protocols and care guidelines.

The results of funded care projects will build an evidence base and demonstrate measurable, positive effects on the clinical management and lives of patients with SMA. These results will then be used to provide:

- Educational programs for professional medical providers, such as the FSMA CME day
- New family-focused care publications, such as the FSMA Care Series Booklets
- Peer reviewed journal publications to influence insurance coverage.

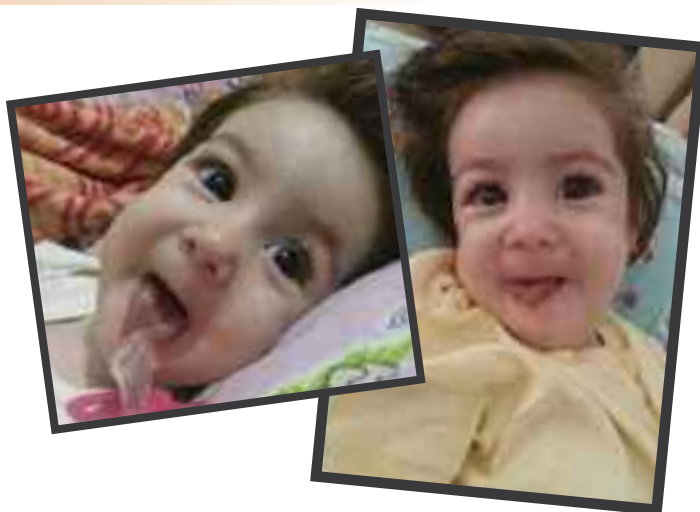
For the latest news visit the FSMA website:



www.curesma.org



Aiden Marie Bala



Andrew Murray



Andrew Sonnenberg



Asher Camp



Abbey Partridge



Addie, Molly and Will Piper



Alanis Suarez



Abby, Matt and William Johnson



Christopher Weber



Abrianna Reed



Alexander Davis



**Gabbie Bark, Maddie Bark,
Leah Vogedes, Jerika Bolen and
Mateo Medina**



**Ave, Delaney and
Jackson Long**



A'laysia Ford

THANK YOU FSM!!!

Jayne & Family



Jayne Gomez



Tucker Rutledge



Jake, Kate and Rachel Saxton



Evie Keister



Victoria and Ella Koblentz



Samuel Bell



Jonah Edwards



Jack Bolton



Jadon Burks



Ellie Stevenson



Lucy and Will Butler



Matthew and Christopher Weber



Joshua and Anna Rose Scurria



Luke Chylik



Juliana Elizabeth Aleman

Done with the word “Can’t”

By Scott Drotar

My entire life people have been telling me what I can’t do. When I was diagnosed with Spinal Muscular Atrophy Type II a doctor told my parents, “Scott can’t live past 3 years old. His lungs are too weak.” I am 27 years old. A few years later, when I was supposed to start kindergarten another doctor said, “Oh, Scott can’t go to public school. There are too many germs. Keep him at home.” I went to public school for 13 years, and I graduated my class valedictorian. After graduation, while I was preparing to go off to college and live on my own, everyone in my life said, “Scott, you can’t live on your own away from home. It will be too hard to arrange and too expensive.” I have been living on my own for almost a decade now, and it has not cost me any more than anyone else. These examples are just a few of the reasons that I decided a long time ago, that I am done with the word, “can’t.”

My name is Scott Drotar, and I was born with SMA Type II. I have never walked, and I have used a power wheelchair since I was 5 years old. I grew up in a small town in Northern Indiana surrounded by my loving family and friends. Even though my disability affects every part of my world, and makes even simple tasks such as brushing my teeth and combing my hair difficult, I have not let it prevent me from having a happy, successful life. I have a bachelor’s degree in mathematics from the University of Notre Dame and a master’s degree in psychology from the University of Kansas. I live on my own outside Kansas City, have my own car, and have an active social life. This success did not come easily however, and I have had to fight to overcome numerous obstacles throughout my life. As difficult as this disease has made my life though, and despite everything I have had to overcome because of it, as a result of these trials and tribulations I have learned a multitude of lessons about life, people, and happiness.

All of this wisdom I have learned from having to overcome the obstacles that life has thrown at me is what motivated me to develop my speaking program, Roll Models. Through this program, I use stories from my life about growing up disabled, as well as my training as a psychologist, to help people live happy, successful, and fulfilling lives. As I like to say, I am in the business of helping people. I also have a blog where I try to pass on some of the life lessons I have learned, as well as raise awareness for people with disabilities. You can find it, as well as more information about myself and Roll Models, at www.scottdrotar.com.



I am on a mission to improve the life of every single person I interact with. Today, I am going to continue on my mission by talking to you about two, little words, and how much they can limit you in life. These two dangerous, destructive words are “I can’t.”

We have all said these words before. Everyone does, you step on the scale and think “I can’t lose this weight.” Or you burn dinner and think “I can’t cook.” And the big one, you have a bad day and think “I can’t do anything right.” You all can recall occasions like this. If you did this only every now and then, it wouldn’t be a big deal, but the problem is that these two words pop up over and over again throughout your life. Every time you say you can’t do a certain activity, your subconscious mind makes a note of it. Once you say it enough times about something, your brain picks up on this pattern. Eventually, your subconscious mind will decide that since you can’t do this activity so often, that you shouldn’t waste time trying, and it will change your feelings about approaching the task.



When you consider doing it, you will no longer think “Let’s give it a try.” You will now think “I will never be able to do this. Why try?” Just like that, your own brain has taken away your chance to ever do this activity.

Think about that for a minute. By saying these two little words just a handful of times, you can lose your opportunity to ever do some activity. I don’t know about you, but having SMA has taken enough things away from me, so the last thing I want is for my own mind to limit me even more. I don’t want any activity taken away from me. If someone came up to you and said, “You burned the pot roast. You can’t ever cook again.” How would you react? You would probably feel angry that someone was trying to tell you what to do, and you most definitely wouldn’t listen to them. This is no different from what your own brain does to you after you say these two words. It’s even worse in your own mind though, because your brain is sneaky and does it to you without you ever knowing. That is why I decided, a really long time ago, that I was done with the words “I can’t.”

A perfect example from my own life as to how much these words can limit you, occurred when I was 15 years old. I was a freshman at John Glenn High School and in the marching band. I woke up one Saturday to go to a marching band competition and felt under the weather, but like all teenagers I thought I was invincible, so I ignored how I was feeling and went to the competition anyway. It turns out that I was not invincible, because the next morning I woke up barely able to breathe from the fluid in my lungs with a fever of 103 degrees. So, I went to the doctor. He looked me over, listened to the crackling in my chest, and took a deep breath. Just by the look on his face I knew it wasn’t good news. He said, “Scott, you are really sick, and I am not



sure how you are so lucid now with the fever you have. We need to get you to the ER to run some tests immediately.” I was scared, but I went to have the tests done. Once the results came back a couple hours later, the doctor delivered the bad news. He said, “You have 3 different types of pneumonia. You are really sick, and we need to take you by ambulance to a bigger hospital to get you the care you need.” Now I was really scared, but off I went.

At this point, my next actual memory is almost a week later. The next several days were so traumatic and stressful that my mind has completely blocked them out. According to what I have been told though, here is the gist of what happened. First, they tried to send a tube down my throat to remove the fluid in my lungs, this is called an intubation. When they tried however, first one lung collapsed, then the other. I went into full respiratory failure and stopped breathing. To get me breathing again, they had to make an incision and put a plastic tube in my throat. This is called a tracheostomy. Once they got me breathing again, and I was on my way back to my hospital room in the ICU, my right lung collapsed again. They rushed me back up to the operating room and saved my life again. After that I spent about 3 days in a drug induced sleep, while everyone waited to see if my body would fight off the infections or not. And after all of that horribleness, I finally woke up.

Imagine for a minute how you would feel if you woke up in the following situation. You wake up expecting it to be Monday in one hospital room, and you find out that it’s Thursday, and you are in another hospital room. On top of that, you have a strange, plastic tube in your throat that just throws with pain, and your lungs feel like somebody put a weed whacker in your chest. And to put the cherry on top of this wonderfully confusing scenario, when you try to speak, nothing comes out but a faint whisper. How would you feel? If you are anything like me, you would freak out a bit. And that is exactly what I did. I had no idea what had happened to me, I hurt all over, and I couldn’t even talk enough to pose the questions I needed to ask to find out. I lost it for a few minutes as I tried hopelessly to piece together what had happened.

Once I settled down some, and my family filled me in on the hellacious few days I had just survived, I went from freaking out to a silent panic. The following thoughts kept going through my head, over and over. The trache tube in my throat was not going anywhere anytime soon, which meant my voice was gone. They had me try some mechanical voice simulator devices, which are little vibrating boxes that you hold to your throat when you want to speak. They were difficult to use if they worked at all though, and they make you sound like a robot. So basically, I was stuck without the ability to communicate. How could someone that requires help to do everything, like I do, have any sort of quality of life without the ability to speak? I thought that life as I knew it was over. As that dawned on me, I slowly transitioned from silent panic to depression.

A day or two later, as I lay in my hospital bed healing, stuck in silence, and extremely depressed, one of my doctors came in to see how I was doing. He walks

in and says, “Scott, you look a lot better than you did a few days ago. How are you feeling?” I smiled, and then I mouthed the words “I can’t talk.” He replied, “Oh I am sorry. You need your cap.” I had absolutely no idea what he was talking about, but I watched him get into a cabinet, pull out a small, purple plastic thimble, and put it on my trache. He then said, “Speak, my boy!” And from my lips, clear as a bell, breaking the silence came two words, “I can’t.” At hearing my own voice, along with the obvious irony in my choice of words, an enormous grin spread across my face. I don’t think there was a dry eye on the floor of that hospital. I was crying, my mom, my dad, brother, sister, and even the nurses on the floor were in tears at hearing my voice, a voice that just moments before we thought was lost forever. These tears of joy were a welcome surprise after the ordeal I had just been through, and they started me on my journey back to life. This was also when I realized how limiting the words “I can’t” can be, because in a split-second I went from “I can’t” to “I can,” and from someone who would never speak again to a professional speaker.

This experience made me realize something about the words “I can’t.” It helped me realize that whether you can or can’t do something is mostly a matter of perspective. For example, I didn’t know that you can cap a trache tube allowing you to speak, and I knew that “I can’t talk.” Once the doctor changed my perspective by applying that tiny piece of plastic to my trache though, I knew “I can talk.” Had anything changed other than my perception of my reality? Not a single thing. The cap and I had both existed in precisely the same way prior to me talking. My world was exactly the same, but the way I saw it was drastically different. The only thing that was needed to go from “I can’t” to “I can” was to

continued on next page

change the way I viewed the situation. I firmly believe that having the words “I can’t” in your vocabulary is more toxic than any disease. It robs you of your freedom to do what you want. Because, “I can’t” so quickly turns into “I’ll never be able to,” and then you have been robbed of ever doing that activity. Take a moment to think about that and how powerful it is. Your own mind can completely change your reality. This phenomenon is not unique to this story either, and it can be applied in nearly any scenario. For instance, if I see a set of stairs leading to the entrance of a building, from that perspective, I “can’t” get inside. If I then go to the other side of the building, and I find a ramped entrance, then from that perspective, I “can” get into the building. Has anything changed other than my own perspective of the building? No. All I needed was a little perspective.

In order to get rid of this cancerous growth on the English language, you need to start approaching obstacles in your life differently. Do not ask yourself, “Can I do this?” because one of the two possible answer is, “No. I can’t.” Why ask a question that can immediately shut you down? Instead, try thinking, “What is stopping me from being able to do this?” This is a better question, because it gives you more information (your obstacles) and cannot be answered with the word, “can’t,” which means you can keep moving forward. From there you continue asking yourself more information seeking questions with open-ended answers until you accomplish your goal.

You will be amazed at how much more full your life will become simply by eliminating these two, little words from your daily vocabulary. You may even find yourself starting to question some of those things you already think you “can’t” do, and think of ways that you “can” do them. Just remember, the only difference between “I can’t” and “I can” is gaining a little perspective. If you take the time and effort to change your thinking in this way, I am confident that you will get more out of life. Without your own mind limiting you, who knows what you can achieve. And if you get to a point where you feel the words “I can’t” creeping into your mind, just remember this little rhyme I use in my Roll Models talks, “When you think you can’t achieve your objective, what you need is a better perspective.”

Helpful Hints

Families of SMA is gathering helpful hints and tips when it comes to managing activities of daily living with SMA.



I recently went on a trip to Mexico and met an amazing person there. His name is Guillermo Camarena. He started a company called EZ Snorkel. He created a way for people in wheelchairs to go snorkeling! The

way that the contraption works is the person in a wheelchair sits in a chair attached to a frame and there is a viewfinder attached. This is there so the person doesn’t need to have their head under water to see the sea turtles. It is also driven by a motor that can be driven by the person just using their pinky finger. If the person is not able to drive on their own, someone will go out with the person and help them drive around the water. He already helped the city of Akumal become accessible to wheelchairs by working with a hotel owner to make accessible rooms and bathrooms. He is going to be meeting with the mayor of Cancun to make Cancun more accessible. This is an amazing company and they are looking forward to doing some great things in the future. I really appreciated this because the woman I went with was in a chair, so I got to see it in action. People can now experience snorkeling and having fun in Mexico without the worry of accessibility.

Here is more information about EZ Snorkel and the best ways to connect:

<http://ezsnorkel.com/>
www.facebook.com/ezsnorkel
guillermo@ezsnorkel.com

– Bethany Behling, Maplewood, MN

Getting a Service Dog



A quick and simple trip to the store, is never quick nor simple for the Murphy's. Lise Murpy or someone has to stay close by to her 12 year old son, Connor. "Sometimes our fear is we cannot find Connor and if his head goes back he cannot yell for me. If we get separated he cannot tell me where he is," says Lise.

Connor has SMA. He uses an electric wheelchair to get around, hauling extra equipment that keeps him alive, like a suctioning machine. Spinal Muscular Atrophy affects all the muscles, the ability to walk, talk, even swallow. "So since he doesn't swallow, the spit builds up and he can choke and its very scary. So if a dog was there and pushed it forward, it would be big."

Lise is referring to a service dog. Dudley Arnold with Canine Assistants out of Alferetta Georgia says, "They learn about 90-95 different tasks. Retrieving, tugging doors open, elevator door buttons." And that's what Connor is getting, a canine companion thanks to a partnership from Sam's clubs and Milk-Bone Brand. They held a presentation at the Sam's Club in West Knoxville to make the announcement. Ken Iuso of Milk-Bone says, "It's about understanding community importance and Sam's has a big priority to give back to the



community, Milk-Bone is a treat, but also benefits a great way to bring awareness to this program."

Connor won't get his service dog for several more months, because he is in service dog training in Georgia. Then Connor and the dog will train for two weeks together before he comes home to the Murphy's. "If there is something specific the dog hasn't learned in training that Connor needs, he will be able to pick it up," says Dudley. Ken says, "Not only for a child, but for the parents, it's a major life change when they get the dogs." Michele Silva asks Connor "What is the first thing you want your dog to do?" Connor replies, "Open the doors!"

And they won't be just doors to the house, but to a whole new world.

Families of SMA is so grateful to have received so many wonderful toys for our Newly Diagnosed Care Package Program! These gifts were given in honor of the Bolton family- Holly, John, Eleanor, Jack and Baby Boy Bolton, from their friends and family in Davidson, North Carolina. These toys will be included in care packages which are sent to newly diagnosed families when they first contact FSMA. Thank you to the Bolton family and everyone that helped purchase these toys for our care packages!





A special delivery of PEZ dispensers arrived at the Families of SMA National Office for our Newly Diagnosed Care Package Program! These lightweight PEZ dispensers will be put into the Type I Care Packages, in honor of Graeme Mooney who has SMA Type I. Thank you to Graeme and his parents for these cute and clever items that we can share with other families!



The FSMA National Office had some wonderful visitors stop by! John, Audrey, Anna, Kara and Marisa Savino delivered head and neck cradlers to include in our care packages! These special items will be put into the Type I Care Packages, in memory of their son, Joseph Savino. Thank you John, Audrey, Anna, Kara and Marisa for dropping off these wonderful additions to our care packages!



Families of SMA is excited to have received another shipment of lightweight Disney Princess Dolls donated by Melissa Wayland for our Newly

Diagnosed Care Package Program! These dolls will be put into the Type I Care Packages, in loving memory of her daughter Olivia Hemmeain. Thank you to Melissa for your incredible support!

Families of SMA is so grateful to have received a special delivery of Crystal Light Drink Packets to include in the upcoming 2014 conference registration bags for all attendees! Thank you to Kraft for these wonderful additions to our conference and for your incredible support!





Riley Austin



Zane Wheeler



Peyton Zimmermann



Rachel, Jake and Kate Saxton



Michael Kelley



Nathan Cooper



**McKenna, Meghan and
Tyler Ellixson**



**Charlie, Lily and
Emma Sykora**

Hi FSMA,

Thank you for all the great information & care package which arrived today! Olivia loved it all. She and her sister fought over the balloon and flag. The quilt is gorgeous!

The reading material on the website and printed brochures have been a great resource for us and immediate family.

Thank you so much for your quick responses and support. It has truly helped during this difficult time.

Sincerely,

Maggie & Jesse McLean from Texas



Hi Families of SMA,

I just want to say thank you so much for the Convaid stroller. When I told Gabby she was finally getting one, she couldn't wait to get home from school. She was SO excited and happy – especially because it was pink! I spent 3 months going back and forth with the insurance company for the exact same stroller (even same color) only to continue to be denied. One call to you and I was fortunate enough you had one in stock and shipped in one day! I'm so glad I called and so grateful to FSMA for the donation. Thank you for your assistance. I couldn't be happier!

Jennifer Ramirez from Illinois



Dear Everyone at FSMA,

We are so touched and grateful for the care package that was sent to our daughter, Ellie. She will enjoy all of these things so much! We look forward to contributing to this wonderful program in the future.

Much love,
The Stevenson Family from Utah



Families of Spinal Muscular Atrophy,

THANK YOU!

We received our SMA Care package on 9/24, just 1 day after contacting FSMA regarding our son's diagnosis. We were so surprised to come home and find our package on our porch. It was truly uplifting and our son just LOVED his "presents" (as he called them!) :) FSMA is such an amazing organization who can provide joy in a time where not much joy is being felt! Thank you from the bottom of our hearts.

Doug, Amy and Emmett Keeton from Ohio



Hi Families of Spinal Muscular Atrophy!

We could not believe the generous gifts you sent. Thank you so much! We absolutely love everything!

Our sweet baby Max was diagnosed on his first birthday (type II) - not such a happy birthday this year. Although you wouldn't know it if you asked him... he was all smiles and laughs in every appointment :) He charmed them all.

He loves playing with all the toys and the sheepskin is such a treat! He sits on it every day or rolls around on it reaching for the toy animals you sent. He even started saying "Moo" because he loves the cow so much.

Thank you again for your gifts and support! The binder of information has been very helpful (once I worked up the nerve to read it). We appreciate the work you are doing to find a cure and we are hopeful. We plan to support that as much as we can.

Thanks again,
The Peda Family from Minnesota



The Sonnenberg Family from Pennsylvania

Dear Families of SMA,

Thank you so much for all of Jordan Eifert's gifts. We were amazed to see everything you sent her. She really likes to watch the windmill.

Thank you very much,
Briean and Jerry Eifert, parents of
Jordan Eifert from Ohio



FSMA,

Thank you so much, Rilee really loves everything you sent especially the book Smash-a-roo. Rilee has a wheelchair just like the one in the book. We really appreciate all the information you sent as well.

Amy Brown from Oklahoma

FSMA!

Thank you so much! We just received a large box package from FSMA in the mail today full of wonderful toys! Our son LOVES it! I can't believe what an amazing organization you have.

Thanks so much for brightening our day!
Pearl Wuebker from Iowa

Everyone at Families of SMA,

Thank you so much for the information packet. It's been so helpful to my family as we are learning all we can about our son's recent diagnosis of SMA II. The box of toys was such a great surprise and Evan loved it. He especially liked waving the little "Cure SMA" flag around.

Thanks for all you do for Families of SMA. We went to our first fundraiser a couple weeks ago and met some great people, who offered their support.

With sincere appreciation,
Lynne Vaudry from Rhode Island



Officer Morgan Day

Morgan Steward is confined to a wheelchair, but that wasn't nearly enough to keep the 5-year-old from his dream: He's always wanted to be a police officer, and he was made an honorary one in Covington, GA. He always said, "Mom, I want to be a police officer, I want to stop the bad guys." The local police chief found out about Morgan, who has Spinal Muscular Atrophy, and made plans for the ceremony. The city of Covington helped make a dream come true.

The event came about in part because of a picture posted on Facebook by Morgan's aunt, Alicia Steward. The image showed Morgan with a written note that read, "My name is Morgan Steward. I am SMA Type 2. My wish is to be a Police Officer." A few weeks later, a member of the force spotted that Facebook post in which Morgan shared his wish of becoming a policeman. At the time, members of the department were planning the annual Covington Christmas Parade and they asked Morgan to lead the parade as an honorary police officer. Chief Cotton then came up with the idea to make it official, and within 24 hours he got permission from the Covington City Council to have a swearing-in ceremony for Morgan.

The mayor was even involved, administering the oath: Morgan promised to obey his mom and be a good student, and he was then given an official uniform, complete with stitched-on police patches, a hat, an official police badge, and a key to the city. Morgan was official sworn in as an honorary police officer for the Covington Police Department, in this special ceremony. His first official duty: leading the Christmas parade through town in a police car. He's also been practicing reading his relatives their Miranda rights.





Wheelchair Accessible Obstacle Course

By: Chessa Birrell
Girl Scout Gold Award Project
Troop 22288
9-29-13



Please visit the cureSMA.org website to download or request a copy of the booklet on Chessa's wheelchair obstacle course and get ideas on how to make your own!



Hi,

My name is Chessa Birrell and I am 17 years old and have Spinal Muscular Atrophy Type II. I am an Ambassador Girl Scout and I have developed plans for a wheelchair accessible obstacle course for my Girl Scout Gold Award project. The

Gold Award is the highest award you can earn in Girl Scouts. Earning the Gold Award requires a suggested 80 hours of planning and implementing a challenging, large-scale project that is innovative, engages others, and has a lasting impact on its targeted community with an emphasis on sustainability.

I created a plan in PowerPoint to construct a fun temporary obstacle course for disabled children and adults that use wheelchairs or walkers. The plan includes many elements that can be used to set up a course. This course is not a permanent one, but one that people can construct or put together easily for a fundraiser, get together, picnic or just a day of fun at home.

For each element, I provide a list of materials, instructions on how to set it up, and discuss any safety concerns. I have tested each element before including it in the final plan.

I have run four different courses for Families of Spinal Muscular Atrophy – Georgia Chapter, Families of Children Under Stress (FOCUS), Titans Wheelchair Sports group, and with FOCUS and Camp Twin Lakes a course for 'tech-dependent' children.

For the last part of my project I am distributing the plans to you, disability organizations, and schools so you can use it to create obstacle courses at your events and fundraisers and share it with your families for fun activities they can do at home birthday parties or just everyday fun.

**Thank you,
Chessa Birrell
Ambassador Girl Scout Troop 22288**



- Congratulations to the Quinnell family on the birth of their new daughter, Charlotte Kennady!



- Grace and Camden Coggin welcomed new little sister Lillian Amara on December 23, 2013!



- Congratulations to Kevin and Mimi Chan and big brother, Brady, on the birth of their new baby boy, Lucas!

- Congratulations to the Miller family on the birth of their newest addition, Elliette, born on November 16, 2013!

- Congratulations to the Canahuati family on the birth of their new son, Carter Bryant, born on November 23, 2013!

- Congratulations to the Nowotny family from Florida on the birth of their new baby, Brig, born on January 2, 2014!

- Congratulations to Sierra and Jared Kulas and big brothers, Kalen and Kyan, on the birth of their new little girl, Evalée, born on January 14, 2014!



- Congratulations to Holly and John Bolton and big sister & brother, Eleanor and Jack, on the birth of their new addition, Robert Frederick, born on February 2, 2014!



- Congratulations to Bill and Victoria Strong and big sister, Gwendolyn, on the birth of their baby girl, Eleanora Tanzey, born on January 19, 2014!

- Congratulations to our own Allison Kerns (SMA Type II) who had the opportunity in November to be an extra in the feature film "Dolphin Tale 2," which will be released in theaters in 2014. She is a star in the making!

- Congratulations to The Losacco Family on the birth of their new baby girl, Zoey Skye!

Congratulations to Susi Vander Wyk, President of Families of SMA Canada, Recently Announced as Walmart Canada's 2013 Mom of the Year

With nearly 25,000 nominations Susi Vander Wyk, mom to Holli, SMA Type II and President of Families of Spinal Muscular Atrophy Canada was recently announced the winner of Walmart Canada's 2013 Mom of the Year!



Walmart Canada launched the second annual Mom of the Year Award to recognize the enormous contribution mothers make to their families and to their communities. In celebration of the incredible dedication Susi has shown, Walmart will donate \$100,000 to Families of Spinal Muscular Atrophy Canada which is Susi's charity of choice!

Susi became actively involved in Families of SMA Canada after her daughter, Holli, was diagnosed at the age of two by organizing charity dinners and dances. Susi also coordinates and runs a summer camp for the past thirteen years for children with SMA and their families to attend.

Congratulations to Susi Vander Wyk on winning such a wonderful award that is well deserved!

Visit the following link to read the whole article:
http://www.momoftheyear.ca/mom/?mom_id=41883



Matthew Freitas



Lizzy Hallam



Jonah Edwards



Patrick and Andrew Murray



Josh and Daniel Tramontano



Molly and Luke Chylik



Mark and Kaley Leiter



Mary Kate, Eileen and Annie Venedam



Murphy and Anders Potter



Ray and Ethan Fantel



Suzanna Hall



Luke Robert Harris



Families of Spinal Muscular Atrophy Scientific Advisory Board Meets To Evaluate Basic SMA Research Projects for New Funding

Families of SMA received a new batch of research applications for funding consideration this past fall. Our Scientific Advisory Board met in Chicago to select the best projects for funding from this batch. Project selection was based on both scientific quality and on relevance to the FSMA mission of accelerating the identification of a safe and effective treatment for SMA. Total funding of up to \$700,000 was committed to research projects for this new round.

Why is Basic Research Important:

Basic Research into SMA biology tells us what causes the disease. Understanding what causes SMA reveals new and better ways of making new drugs. Families of SMA believes continued investment in basic research, leads to greater understanding of the exact nature, causes, and consequences of SMA, which is key to ensuring that the most effective SMA treatments can be identified and developed as quickly as possible.

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

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

Through 2013, FSMA has funded over 150 projects at more than 75 institutions. With this new round of funding, we will have invested almost \$10 Million in Basic Research since 2004, with significant additional funding to drug discovery, clinical research, and care research.

Survey Published of 24 SMA National Registries, Mapping the Differences in Care for 5,000 SMA Patients

Researchers analyzed the TREAT-NMD national patient SMA registries in order to provide a snapshot of data by SMA subtype and compared the results.

“This study included 5,068 SMA patients in 25 countries. A total of 615 patients were ventilated, either invasively (178) or non-invasively (437), 439 received tube feeding and 455 had had scoliosis surgery. Some of these interventions were not available to patients in all countries, but differences were also noted among high-income countries with comparable wealth and health care systems. This study provides the basis for further research, such as quality of life in ventilated SMA patients, and will inform clinical trial planning.”

Data featured in this study was included from the International SMA Patient Registry. The International SMA Patient Registry was founded in 1986 at Indiana University and is supported by Families of SMA.
<http://www.fsma.org/Research/Clinical/PatientRegistry/>



Seth and Caroline Gilley



Presley Moreno



Ruby Sun



Zander and Alexis Helfrich



Sienna and Andrew Sonnenberg



SMA Association of Romania



Sheyenne Whitley

AveXis-BioLife Licenses SMA Patent Portfolio from Nationwide Children's Hospital and The Ohio State University.

In October 2013, the Research Institute at Nationwide Children's Hospital received Fast Track designation from the U.S. Food and Drug Administration for its scAAV9.CB.SMN gene therapy product for the treatment of Spinal Muscular Atrophy (SMA). This new gene therapy product created by scientists at The Research Institute was granted Fast Track status after demonstrating preliminary effectiveness in mouse models of SMA, potentially addressing this unmet medical need. SMA is the most common genetic cause of infant death. SMA is an autosomal recessive disease caused by a genetic defect in the SMN1 gene that encodes for the SMN protein. SMA manifests in various degrees of severity which all have in common general muscle wasting and mobility impairment.

BioLife, a synthetic biology platform company soon to be renamed AveXis, has been granted exclusive rights to the spinal muscular atrophy gene therapy program developed at The Research Institute at Nationwide Children's Hospital and The Ohio State University in Columbus, Ohio.

The scAAV9.CB.SMN gene therapy product currently being developed received an IND approval in September 2013 to initiate Phase I clinical testing in SMA Type 1 patients. Jerry R. Mendell, M.D., director of the Center for Gene Therapy and Principal Investigator in The Research Institute at Nationwide Children's Hospital will lead the clinical trial.

The Research Institute at Nationwide Children's Hospital Receives Fast Track Status for Spinal Muscular Atrophy Treatment

More about the Fast Track Designation:

"Fast track," an important hastened phase in the nation's drug review and approval process, signifies that the FDA can expedite the review and development of the scAAV9.CB.SMN gene therapy product which, in preclinical work, has shown to slow the progression of SMA symptoms.

Three SMA Drug programs have now been given Fast Track designation by the FDA, including Nationwide Children's, Isis Pharmaceuticals, and Pfizer.

Isis Provides FSMA an Update on Clinical Development Plans for Spinal Muscular Atrophy in October 2013.

Isis-SMNRx Program Update on Ongoing and Future Clinical Trials

Infant Program Update:

Isis Opens Cohort 2 of the Clinical Study in Infants with Spinal Muscular Atrophy

This study is evaluating the investigational compound, ISIS-SMNRx, in infants with Spinal Muscular Atrophy (SMA) Type I. This Phase II study is an open-label, multiple-dose, dose-escalation pilot study, designed to examine the safety and tolerability of ISIS-SMNRx in infants with SMA and to provide data to define the optimal dose for future studies in infants. To meet enrollment criteria, infants must be between the ages of three weeks and seven months, live in close proximity to a study site and pass additional screening evaluations conducted at the study site. The study is being conducted at 4 centers in the United States and Canada who are looking for eligible subjects -

- Stanford University Medical Center in Stanford, California
- Nemours Children's Hospital in Orlando, Florida
- Columbia University Medical Center in New York, New York
- The Hospital for Sick Children (SickKids) in Toronto, Ontario, Canada

For further study information and contact information for the study sites, please visit www.clinicaltrials.gov and search for ISIS-SMNRx.

Next Steps:

A controlled Phase III study in infants is planned to begin early in 2014. This study will be larger than the current ongoing study and will be conducted in the US and internationally.

Child (Type II and Type III) Program Update:

Isis Completed Dosing and Enrollment in the Multi-Dose Phase II Dose Escalation Study in Children with Spinal Muscular Atrophy.

This Phase II study is a multiple-dose, dose-escalation study designed to examine the safety and tolerability of multiple doses of the drug given over a longer period of time. This study is being conducted in children age 2 – 15 with SMA Type II and Type III. The study is fully enrolled, has completed dosing, with data expected once the follow up is completed early next year.

Next Steps:

A larger controlled Phase III study in children is planned to begin in 2014. This study will be larger than our current ongoing study and will provide more opportunity for enrollment at an increased number of participating sites, including Canada and other international sites.

If you would like further information, please contact your study site or you can contact Isis directly at: Kristina Lemonidis (klemonidis@isisph.com).



Hello, my name is Aiden Silvers and I'm a poet. My poems express my feelings and affect people's lives in a positive way. The thing is, I don't try to write poems. It just comes to me like it's from God.

I've had many struggles and close calls in my life, like sickness, surgeries, procedures and accidents, I've stayed strong, kept positive and used my faith in God to get me through it.

I was born in South Florida on August 13th, 1994 with a disease called Spinal Muscular Atrophy. This disease started to take effect on me at the age of 2. My parents thought something was strange when I began to stop walking and resorted back to crawling and rolling soon after. The Doctors couldn't figure out what was wrong with me until they decided to do a muscle biopsy and found I had SMA. Soon after my diagnosis, my mother ran out on me and my dad, and he turned down a record deal to raise me. When I was 10 years old my father met an angel inside a woman's body who was recently divorced from an abusive relationship and was also raising a 2-year-old girl. They now have 4 kids together, 2 boys and 2 girls. We have been together 9 years now and will always stay that way. My dad can finally resume his music career.

"Bball"©

By Aiden Silvers

Dribble, dribble, dribble, spin, spin, spin.
Do everything I can to win, win, win.

Get up and down the court before anybody else.
You can try your best but you can't match this stealth.

Jump higher than the boards, I'm harder than this court.
You better take a foul cause I ain't fixin' to abort.

Average a Triple Double every night, comon put up a fight.
On the biggest stage under the brightest lights
that's when you'll kiss your chances goodnight.

The trophy's mine, just try to take it. I give it my all.
Man ya'll be in trouble if I could play Bball.

"I See"©

By Aiden Silvers

I see waves forming, high and low.
The oceans glow from jellyfish lingering below.

I see trees leaning, leaves blowing in a storm.
I see a shelter that's comforting, soft and warm.

I see the moon lit brighter than ever, stars flashing in the night.
I see someone strong and caring who holds her loved ones tight.

When I see I'm not a part of this place it kills me on the inside.
All of these things happen, when I see into your eyes.



The Hoffmann Family



The Schmid Family



The Chan Family



The O'Neil Family



The Freitas Family



The Richard Family



The Trainor Family



The Tomko Family



The Barrett Family



The Gooden Family



The Horton Family



The Armbrustmacher Family



The Sun Family



The Weisman Family



The Woodruff Family



The Lozina Family



The McHale Family



The Sykora Family



The Murray Family



The Tranby Family



The Bolton Family



The Butler Family



The Lino Family



The Meigs Family



The Burks Family



The Gibbs Family

Spinal Muscular Atrophy Researchers Participate at The 2013 Society of Neuroscience Meeting

The Society of Neuroscience (SfN) Meeting is the premiere neurobiology meeting annually with over 30,000 scientists attending. It was held November 9 to 13, 2013 in San Diego, CA. This year multiple presentations on Spinal Muscular Atrophy were given by scientists funded by Families of SMA.

There were almost two-dozen presentations on SMA research given at the meeting. These presentations include work from the labs of past and present FSMA-funded researchers, such as Drs. Giancomo Comi, Brian Kaspar, Arthur Burghes, Wilfried Rossoll, Chien-Ping Ko, George Mentis, Laxman Gangwani, Yong-Chao Ma, Lee Rubin, Christine Beattie, and Matthew Butchbach, as well as other prominent SMA researchers.

Below is a list of the SMA Presentations at SfN in 2013:

1. Long term physical exercise is beneficial for SMA-like mice. F. CHALI¹, B. ESTOURNET², C. DESSEILLE¹, P. LOPES³, C. PARISE⁴, *F. CHARBONNIER¹, O. BIONDI¹; ¹Biol., Univ. Paris Descartes, Paris, France; ²Hôpital Raymond Poincaré, AP-HP, Garches, France; ³Univ. d'Evry-val-d'Essonne, Evry, France; ⁴Hôpital Trousseau, Paris, France
2. Genome editing strategies for the development of a treatment for SMA. M. RUGGIERI, C. SIMONE, M. NIZZARDO, F. RIZZO, G. RIBOLDI, S. SALANI, C. ZANETTA, I. FARAVELLI, N. BRESOLIN, G. COMI, *S. CORTI; Univ. of Milan, Milan, Italy
3. Manipulating the agrin/neurotrypsin system improves muscle trophism in SMA. *M. M. BOIDO¹, E. DE AMICIS¹, S. HETTWER², R. FARIELLO², A. VERCELLI¹; ¹Neurosci. Inst. Cavalieri Ottolenghi, Univ. of Turin, Orbassano (TO), Italy; ²Neurotune AG, Schlieren, Switzerland
4. The In vivo efficacy of small molecule compounds modifying alternative splicing of the SMN2 gene in 7 mice. *Z. FENG¹, K. LING¹, H. RATNI², I. GERLACH², O. KHWAJA², F. METZGER², X. ZHAO³, M. WEETALL³, N. NARYSHKIN³, G. KARP³, P. TRIFILLIS³, E. WELCH³, J. COLACINO³, J. BABIAK³, N. ALMSTEAD³, K. MCCARTHY⁴, K. CHEN⁴, S. PAUSHKIN⁴, C.-P. KO¹; ¹USC, Los Angeles, CA; ²pRED, Pharma Res. & Early Development, Neurosci. DTA, F. Hoffmann-La Roche Ltd., Basel, Switzerland; ³PTC Therapeut., South Plainfield, NJ; ⁴Spinal Muscular Atrophy Fndn., New York, NY
5. Translating two gene delivery routes for spinal muscular atrophy. L. BRAUN¹, K. FOUST², K. MEYER³, L. SCHMELZER¹, L. FERRAIUOLO³, S. MCELROY¹, A. BURGHESE², J. MENDELL³, *B. K. KASPAR³; ¹The Res. Inst. at Nationwide Children's Hosp., Columbus, OH; ²The Ohio State Univ., Columbus, OH; ³Ohio State Univ., RI at Nationwide Children's Hosp., Columbus, OH
6. Systemic administration of SMN inducing compounds ameliorates SMA-like and non-SMA like phenotypes in a mouse model of intermediate SMA. *C. M. LUTZ, M. OSBORNE, L. BOGDANIK, C. DAVIS, W. ANDREWS; The Jackson Lab., Bar Harbor, ME
7. Identifying the genetic and molecular mechanisms of smn-1 synaptic defects. *M. DIMITRIADI¹, G. KALLOO¹, K. NGUYEN², D. HALL², A. HART¹; ¹Neurosci., Brown Univ., Providence, RI; ²Neurosci., Albert Einstein Col. of Med., Bronx, NY
8. Involvement of plc and erk pathways in spinal muscular atrophy astrocyte. *T. N. PATITUCCI, J. V. MCGIVERN, J. A. NORD, A. K. SMITH, M. E. BARABAS, C. L. STUCKY, A. D. EBERT; Cell Biology, Neurobiology, and Anat., Med. Col. of Wisconsin, Milwaukee, WI
9. The survival of motor neuron protein in axonal mRNA localization and transport in spinal motor neurons. *C. FALLINI^{1,2}, P. DONLIN-ASP², J. P. ROUANET², G. J. BASSELL², W. ROSSOLL²; ¹Neurol., UMASS Med. Sch., Worcester, MA; ²Cell Biol. and Ctr. for Neurodegenerative Dis., Emory Univ., Atlanta, GA
10. Developing disease-relevant neural cell models from induced pluripotent stem cells. *C. Y. TAY^{1,2}, S. KESAVAPANY², L. W. STANTON¹; ¹Genome Inst. of Singapore, Singapore, Singapore; ²Neural Pathways DPU, GlaxoSmithKline R&D China, Singapore Res. Ctr., Singapore, Singapore
11. A stem cell model of the motor circuit reveals distinct requirements of SMN for motor neuron survival and function. *C. M. SIMON, A. JANAS, F. LOTTI, L. PELLIZZONI, G. MENTIS; Col. of Physicians and Surgeons, Motor Neuron Ctr., New York, NY
12. SMA skeletal muscles in primary cell culture have normal morphology, survival, growth, and response to DNA damage. *S. FAYZULLINA, L. J. MARTIN; Johns Hopkins Sch. of Med., Baltimore, MD
13. Non-cell autonomous mechanisms induce hyperexcitability of motor neurons in a mouse model of spinal muscular atrophy. *E. FLETCHER, G. Z. MENTIS; The Ctr. for Motor Neuron Biol. and Dis., Columbia Univ., New York, NY

14. Plastin acts a genetic modifier of neurodegenerative diseases. *M. WALSH, E. WINGROVE, A. HART; Neurosci., Brown Univ., Providence, RI
15. Regulation of the cytoskeleton in spinal muscular atrophy (sma). N. HENSEL, B. FÖRTHMANN, H. BRINKMANN, *P. CLAUS; Neuroanatomy, Hannover Med. School, Neuroanatomy, Hannover, Germany
16. Modeling the early phenotype at the neuromuscular junction of spinal muscular atrophy using patient-derived iPSCs. *M. YOSHIDA, S. KITAOKA, N. EGAWA, M. YAMANE, K. TSUKITA, T. NAKAHATA, H. INOUE, M. SAITO; Ctr. For Ips Cell Res. and Application, Kyoto, Japan
17. Function of ZPR1 in neurodegeneration and pathogenesis of SMA. *L. D. GANGWANI¹, N. GENABAI¹, S. AHMAD²; ¹Biomed. Sci., Texas Tech. Univ. Hlth. Sci. Ctr., El Paso, TX; ²Georgia Regents Univ., Augusta, GA
18. Regulation of motor neuron degeneration in spinal muscular atrophy. *Y. MA¹, N. MILLER¹, B. YANG¹, J. MCGIVERN², K. QUINLAN³, C. COWAN⁴, A. EBERT²; ¹Northwestern University/Children's Hosp. Res. Ctr., Chicago, IL; ²Med. of Wisconsin, Milwaukee, WI; ³Northwestern Univ., Chicago, IL; ⁴Harvard Med.
19. Understanding the regulatory mechanisms of SMN stability. *R. CHAUHAN^{1,3,2}, B. AKTEN^{1,2}, H. LE⁴, H. NAWABI², N. LITTELMAN⁵, Z. HE², L. RUBIN⁵, R. KING⁶, C. BEATTIE⁴, J. STEEN^{1,2,3}; ¹F. M. Kirby Ctr. for Neurobio.; ²Boston Children's Hosp., Boston, MA; ³Proteomics Center, BCH, Boston, MA; ⁴The Ohio State University, Dept. of Neurosci., Columbus, OH; ⁵Stem Cell and Regenerative Biol., Harvard Univ., Cambridge, MA; ⁶Dept. of Cell Biology, Harvard Med. Sch., Boston, MA
20. Altered function of spinal motor neurons in severe SMA mice. *M. A. HARRINGTON¹, H. ZHANG², C. PHILLIPS¹, A. W. HARRIS³, M. E. R. BUTCHBACH³; ¹Biol. Sci., Delaware State Univ., DOVER, DE; ²Dept. of Pain Med., Univ. of Texas M.D. Anderson Cancer Ctr., Houston, TX; ³Nemours Biomed. Research, Alfred I. duPont Hosp. for Children., Wilmington, DE



The Ins and Outs of Antisense Drugs for Spinal Muscular Atrophy

In December, Families of SMA released a new Compass newsletter on The Ins and Outs of Antisense Drugs for Spinal Muscular Atrophy. This newsletter was produced in a collaboration between Families of SMA, Biogen Idec, and Isis Pharmaceuticals. It describes the scientific strategy behind Antisense Drugs. It also provides updates on the ongoing research and clinical trials for this class of SMA drugs.

In this edition of Compass, read about:

- The ABC's of Antisense Drugs
- ISIS-SMNRX Program Update on Ongoing and Future Clinical Trials
- FSMA Grant Award to Dr. Adrian Krainer at CSHL to Assess Influence of Backbone Chemistry on Antisense Drugs
- FSMA Grant Award to Drs. Arthur Burghes and Christian Lorson for Novel Antisense Therapies
- Report on New Data from the Completed Phase I Study Evaluating the Safety of Isis-SMNRX

Please go to www.curesma.org to download a copy.



Alexis and Zander Helfrich



Brady and Lucas Chan



Christopher Flores



Danny McHale



Caleb Berny Richard



Charlie Sykora



Carolyn Barrett



Claire and
Lauren Gibbs



Brent Dixon



Eloise, Samantha and Jamie Pillarella



Ella Koblentz



Grayson Peters



Madison Smith



Christopher and
Emily Lozina



Andrew and Sienna Sonnenberg



Anders and Murphy Potter



Avery Wheeler

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

Isabel Poluchowicz

JUNE 26, 2011-JUNE 28 2013



Poem that Isabel's Mom sent Isabel:

Do not stand at my grave and weep

I am not there. I do not sleep.

I am a thousand winds that blow.

I am the diamond glints on snow.

I am the sunlight on ripened grain.

I am the gentle autumn rain.

When you awaken in the morning's hush

I am the swift uplifting rush

Of quiet birds in circled flight.

I am the soft stars that shine at night.

Do not stand at my grave and cry;

I am not there. I did not die.

Mary Elizabeth Frye - 1932



IN MEMORIAM
AIDEN ODELL

JUNE 17, 2005 - OCTOBER 23, 2005



IN MEMORIAM
NATALIE RENEE GREGG

JULY 2012 - SEPTEMBER 9, 2013



IN MEMORIAM
NEVAEH RAE NELSON

JULY 17, 2013 - SEPTEMBER 6, 2013

IN MEMORIAM

KATHIE JEAN VINSON

MAY 17, 1954 – MARCH 22, 2013



IN MEMORIAM

LANDON BRADLEY CLUGSTON

OCTOBER 18, 2013 – JANUARY 30, 2014

God saw you getting tired and a cure was not to be,
so He put His arms around you and whispered,
“Come live with me”.

With tearful eyes we watched you suffer
and saw you fading away;
we loved you dearly, we could not make you stay.

A golden heart stopped beating;

your hardworking hands put to rest.

God broke our hearts to prove to us, He only takes the best.

God will love you and keep you until we meet someday.

IN MEMORIAM

Clayton Bryant

JUNE 29, 2013 – FEBRUARY 21, 2014

Dear Friends and Family,

Clayton was born June 29, 2013. I had no problems with pregnancy until my 8th month where Clayton slowed down moving. I went to the doctor he drew my blood and said that my baby was fine and that sometimes babies' just move slow. When I had Clayton he appeared to be a perfect, normal baby until about 3 days after he was born I noticed he was breathing weird. I told the doctor about it and he sent me to the ear, nose, and throat doctor and they told me he had Laryngomalacia and that he would grow out of it when he is 18months.

When Clayton was about 3 weeks, I noticed his hands turning inward, during that time Clayton broke out with a heat rash so I took him to the emergency room where I explained all my concerns of floppiness, his head shape, everything, but the doctor told me it was just a VIRUS. A virus, really? When I took him for his 6 week check up the doctor told me that this isn't normal so he made an appointment at the St. Francis Children's Hospital and sent us for X-ray's. That day the results came back showing he had ulnar deviation in his hands and arms so he made an appointment with Shriners.

While waiting I took him back to the doctor because things were progressing and I was concerned as to why he was not moving his legs and arms like my oldest son could do at his age, so I asked her to do a full body examination. She said his reflexes were very weak and she referred us to a genetic doctor and to do more X-rays and CT scans. They came back that his bones were fine. In disbelief I knew something wasn't right so when his genetic appointment. came he was about 3months old and she examined him, looked at me and said we need to admit him.

Upon admittance, they ran a battery of tests. When we did the muscle biopsy the neurologist came in with a depressed look on his face and said he thinks he has Spinal Muscular Atrophy I'm like what is that? He explained very little but I knew it had to be something serious because of the look on his face. I googled it and my heart dropped; I was thinking to myself not my child. How did this happen to me, so I asked the doctors how this could be. One doctor said to me, in front of my baby, maybe it was just a faulty bad egg. I was angry that she would tell me this; this is my baby not just some bad egg. After all the test were run



from sleep studies, MRI's, CT scans, throat studies, tons of lab work we were finished and getting ready to go home, but Clayton caught pneumonia. They treated it and sent us home 2 days later.

At 4 months I took him to his sleep Neurologist appointment for results and he said he had severe sleep apnea so he admitted him because he was concerned. They said he hadn't gained much weight and I found out he indeed tested positive for SMA Type I and he may soon need a feeding tube. They did X-rays and noticed he had pneumonia so they told me that I need to consider a tracheostomy and I was thinking I was not going to do that. When they did the check for clear airway they said he didn't need it right now so after 3

weeks we were released with a machine and oxygen for the night.

On December 19, at almost six months old, Clayton was acting sluggish; not wanting to eat and just laying there so I took him to the ER and they admitted him. I was concerned because they hadn't given him anything for fluids. I asked the nurse for an IV for him because he was eating very little and she said that he is fine as long as he's eating something. I remember asking several times for an IV and they said the same thing, they didn't want to because they had given him lacesix to dry up the pneumonia. I noticed my baby losing weight so I finally said give us an IV or I'm leaving and going to another hospital and they did. It felt like they were just waiting for him to die and I told myself I never wanted my baby to pass away in that place.

A few days later on Christmas Day they sent us home and Clayton started this new health pediatric daycare for babies with special needs and Clayton loved it, but Clayton had lost quite a bit of weight with his last hospital stay. He had lost much weight so we made an appointment so he could get a feeding tube. Clayton had good days and bad days with eating.

On January 24, at almost 7 months, Clayton woke up breathing kind of fast so I gave him his breathing treatment and watched him to see if it would slow down and it did some so I took him to daycare and went to work. They called and said his stats were low and that they put him on oxygen so when it wasn't coming up we decided to call 911 and have him transported to get oxygen. Once we got there the doctor told me he needed to be intubated he won't make it long. I was so afraid and scared I



didn't know what to do, so I went outside and just prayed and asked God. I came back in crying, saying just do it, so they intubated him.

The PICU rooms were all full so they transported us to another hospital and the PICU doctor there said we need to talk about long term goals for him to live longer. That was the first time I didn't hear it's only going to get worse or there is nothing we can do or we can't do anything to help him. The doctor seemed as if he cared and I knew he did, even the nurses did. Almost a week in, I let them trache him and Clayton got his g-tube as well. I was so scared I didn't want to ever consider the trache, but I did and the doctors were so surprised how well he was doing with it. He had gotten off the vent after a week and half and didn't need oxygen anymore so we were getting ready to go to New Orleans for vent and trache training. The doctor told us he had taken care of twins 20 years ago with this disease so he introduced my mom and me to her and she shared her story and we cried and prayed together while still waiting to go to training.

Our doctor was off for a few days and another doctor from the other hospital we came from took care of us. He decided that since he was doing so well he was going to cancel the trip to New Orleans and that he was ready to go home. I was thinking the other doctor wasn't going to be happy but I thought ok. As I was preparing to go home I was thinking I was in over my head as I'm a single mother, with help, but that depended on other

people's schedules. We went home and the first night was fine.

Two days later I put Clayton's talk valve on so I could hear him and I put him in front of the TV so I could get things done, since I hadn't been home in a month. He hated that talk valve; he whined until finally I thought he was asleep. I went to check his breathing and he was still breathing but I noticed it was slower than normal so I went in the bathroom and then something clicked in my head that said go back and check. By then he wasn't breathing at all so I grabbed him and called 911 and she walked me thru CPR. He wasn't doing anything, all I could hear was air coming thru his trache. The EMTs rushed in and grabbed him really fast and started doing CPR on him. It took them 30 minutes to find a heartbeat and once they did he was on life support, where he was declared brain dead.

Clayton lost his battle to SMA on February 21, 2014 at almost 8 months old. I thought to myself I could handle it; the thing I wanted most was to make Clayton happy and with everything he went through he smiled through it all. He touched and brought so many people together in this short time. He had the biggest and brightest personality. He never said anything but you knew what he was thinking with just his eyes; he told a story with his smile and eyes. I thank God that Clayton chose me to be his mother.

Alexis Bryant of Louisiana



SMA Community Mourns the Passing of David Cuniff

Families of SMA and the entire SMA community mourn the loss of 35 year old David Cuniff of Duanesburg, NY.

David is the father to Caleb, who has SMA Type II and Zach, and husband to Amy. David and Amy quickly became involved in the SMA community and lead the charge throughout the Albany, NY area to help find a treatment and a cure for Spinal Muscular Atrophy. Together, they started the Capital Region Satellite Chapter of Greater New York and hosted numerous fundraisers throughout the year.

David was always positive and a joy to work with, and will always be remembered. Our thoughts go out to his family during this very difficult time.



Meet Newly Funded Families of SMA Researcher: Dr. Yimin Hua

Dr. Hua is a Research Investigator at Cold Spring Harbor Laboratory in the laboratory of Dr. Adrian Krainer. He is a long-term collaborator of Isis Pharmaceuticals.

The grant award to Dr. Yimin is the fourth drug discovery project funded by Families of SMA in 2013 with a total investment of \$550,000. This program will systematically assess the effect of backbone chemistry on the therapeutic efficacy of Antisense Oligonucleotides (ASO) that target the ISS-N1 region of the SMN2 RNA. ASOs that bind to the ISS-N1 region will be compared. This is the binding region of the drug ISIS-SMNRx that is currently being tested by Isis Pharmaceuticals in SMA clinical trials. This funding is being awarded to Dr. Yimin Hua in the laboratory of Dr. Adrian Krainer at Cold Spring Harbor Laboratory. Both scientists are long-term collaborators with Isis Pharmaceuticals and actively participated in the pre-clinical development and characterization of the mechanism of action of ISIS-SMNRx.

Who are you?

I am a molecular biologist, and currently a Research Investigator at the Cold Spring Harbor Laboratory (CSHL). I obtained my Ph.D. degree in molecular endocrinology in 1998 from Sun Yat-sen University in China. During 2000-2004, I worked on SMA pathogenesis with Professor Elliot Androphy and Dr. Jianhua Zhou in the Tufts Medical Center and University of Massachusetts Medical School. In 2004, I joined Professor Adrian Krainer at the CSHL to develop antisense drugs to treat SMA. The area of my expertise is focused on studying gene expression regulation, particularly RNA splicing regulation, and using antisense strategies to design therapeutic approaches.

How did you first become involved with SMA research?

In May of 2000, I came to the USA as a postdoctoral fellow in Elliot Androphy's lab. I was shocked to learn that the lack of SMN causes SMA, the devastating infantile disease. I immediately felt very much interested in studying the pathogenesis of the disease and developing therapeutic drugs.

What is your current role in SMA research?

I have been working on SMA since I moved to the USA in 2000. In the past few years I focused on developing antisense drugs to treat SMA through correcting SMN2 splicing. The antisense drug candidate is now in the Clinical Phase II trials. I also recently uncovered that peripheral tissues play essential roles in rescuing SMA mouse models. My research effort is currently focused on optimizing antisense therapy for SMA and searching for SMN downstream gene targets that are critical in SMA pathology.



Meet Newly Funded Families of SMA Researcher: Dr. Adrian Krainer

Dr. Krainer is a Professor at Cold Spring Harbor Laboratory, a member of the FSMA Scientific Advisory Board, and a long-term collaborator of Isis Pharmaceuticals.

The grant award to Dr. Krainer's lab is the fourth drug discovery project funded by Families of SMA in 2013 with a total investment of \$550,000. This program will systematically assess the effect of backbone chemistry on the therapeutic efficacy of Antisense Oligonucleotides (ASO) that target the ISS-N1 region of the SMN2 RNA. ASOs that bind to the ISS-N1 region will be compared. This is the binding region of the drug ISIS-SMNRx that is currently being tested by Isis Pharmaceuticals in SMA clinical trials. This funding is being awarded to both Dr. Yimin Hua and Dr. Adrian Krainer at Cold Spring Harbor Laboratory. Both scientists are long-term collaborators with Isis Pharmaceuticals and actively participated in the pre-clinical development and characterization of the mechanism of action of ISIS-SMNRx.

Who are you?

I have been working on RNA splicing mechanisms for 30 years. My lab also studies the role of defective splicing in genetic diseases and cancer, with a focus on development of targeted therapies based on splicing mechanisms.

How did you first become involved with SMA research?

I attended an SMA workshop at the NIH around 1999. It was reported then that the SMN2 gene has a splicing defect due to a mutation in exon 7. We had been studying a mutation in exon 18 of the BRCA1 gene that caused skipping of that exon, and that got me interested in pursuing the underlying mechanisms, which later led us to developing methods for correcting defective splicing.

What is your current role in SMA research?

My lab collaborated with Isis Pharmaceuticals to develop and characterize the mechanism of action of a novel antisense drug, ISIS-SMNRx. We continue to study this type of compound, both to help in its development as a therapeutic, and to obtain insights into SMA disease mechanisms using mouse models.

FSMA Funds 25 Research Projects in 2013

FSMA invests in four research areas: Basic Research, Drug Discovery, Clinical Research, and Care Research. FSMA has invested over \$55 Million in research since 1984, with \$35 Million in the past decade alone. In 2013, we provided funding to 25 different research projects across these four areas.

We believe that investment in all of these research areas is essential in finding effective SMA treatments and eventually a cure for the disease. Basic Research into SMA biology tells us what causes the disease. Understanding what causes SMA reveals new and better ways of making new drugs. Then Drug Discovery programs convert Basic Research findings into SMA drug candidates. Clinical Research gives the infrastructure needed to test drug candidates. Care Research identifies the best care practices to improve the quality of life of SMA patients.

2013 Funding Summary:

Basic Research to Understand SMA:

In 2013 we provided funding to 11 different basic research projects on SMA. In early 2014 we anticipate funding \$700,000 in basic research towards another 6 or 7 projects. With these new grants, we will have invested almost \$10 Million to Basic Research since 2004. The Basic Research projects with FSMA funding in 2013 include:

1. Motor axon development in SMA. Charlotte Sumner, MD, Johns Hopkins University
2. Regulation of HDAC5 phosphorylation by Cdk5 in SMA. Yong-Chao Ma, PhD, Northwestern University
3. The role of glia cells in SMA. Chien-Ping Ko, PhD, University of Southern California
4. Arginine Methylation as a Regulator of SMN in Motoneurons. Jocelyn Côté, PhD, University of Ottawa
5. The role of vesicle coat protein alpha-COP in models of SMA. Sara Custer, PhD, Indiana University
6. The when and where requirements of SMN in mild SMA. Christine DiDonato, PhD, Northwestern University
7. Stem Cell Models of SMA. Christopher Henderson, Ph.D., Columbia University
8. Validation of Spinal Muscular Atrophy Biomarkers in VALIANT Subjects. Stephen Kolb, MD, PhD, OSU
9. Selective Vulnerability of Distinct Motor Neuron Pools in SMA. Rashmi Kothary, PhD, University of Ottawa.
10. Novel genetic determinants of severity in SMA model mice. Umrao Monani, PhD, Columbia University
11. SMN in mRNA transport and local protein synthesis in motor axons. Wilfried Rossoll, PhD, Emory University.

Drug Discovery to Identify New Therapies:

There are now 15 drugs in the SMA drug pipeline with 3 in clinical trials and several more anticipated to start in 2014. FSMA has been involved in two-thirds of all SMA drug programs and has invested over \$19 Million since 2000. FSMA believes it is critical to build the SMA drug pipeline using multiple approaches, so there are back-ups if the current lead programs fail, given just 10% of drugs in clinical trials obtain FDA approval. FSMA is currently funding 6 Drug Discovery programs on:

1. Gene Therapy to Dr. Brian Kaspar at Nationwide Children's Hospital
2. The Isis Antisense Drug to Dr. Adrian Krainer at Cold Spring Harbor Laboratory
3. New Antisense Drugs to Dr. Arthur Burghes at OSU and Dr. Chris Lorson at the University of Missouri
4. Drug Screens in Motor Neurons to Dr. Lee Rubin at Harvard
5. Muscle Enhancing Drugs to Dr. Jeff Jasper at Cytokinetics
6. Novel Small Molecules at to Dr. Peter G. Schultz at CALIBR

Care Research to Improve Quality of Life:

In 2013 FSMA initiated a new program in Clinical Care Research. The results of funded projects will build an evidence base for the clinical management of SMA, improving the quality of life for patients. We funded four Care Research projects in June of 2013 to:

1. Oscar Mayer, MD at The Children's Hospital of Philadelphia
2. Timothy Lotze, MD at Texas Children's Hospital
3. Matthew Halanski, MD at University of Wisconsin
4. Kathryn Swoboda, MD at University of Utah

Clinical Research to Effectively Test Drugs:

In 2001 Families of SMA started the Project Cure SMA Trial Network. The network developed clinical infrastructure and protocols to test candidate drugs for SMA. Overall they conducted 5 clinical trials. FSMA continues to fund collaborative projects to build clinical trial infrastructure and has invested \$7 million in this area. Our 2013 Clinical Research projects include:

1. The SMA Patient Registry to aid in clinical trial recruitment
2. Stephen J. Kolb MD at OSU for patient recruitment for the NIH NeuroNext Biomarker Trial
3. Parent Project MD for a collaborative study exploring expectations in clinical trials
4. Collaborative SMA Community Project to optimize clinical trial outcome scales

Families of Spinal Muscular Atrophy Contributes Research Funding to Sixteen SMA Journal Articles in 2013

Families of SMA is proud to have contributed funding to the research published in the following journal articles in 2013. FSMA has invested over \$55 Million in research to accelerate identification of a treatment and cure for SMA. This includes 25 research projects in 2013 alone.

Please see the FSMA Funding Facts Page for all funded projects.

Funding for these studies was awarded from FSMA to the following institutions: Columbia University, Indiana University, University of Utah, Ohio State University, Johns Hopkins University, University of Manitoba, Wayne State University, University of Wisconsin, Hospital Sainte-Justine, Harvard University, University of Ottawa, Northwestern University, Rutgers University, University of Southern California, Emory University, University of Cologne, and University of Milan.

Mapping The Differences In Care For 5,000 Spinal Muscular Atrophy Patients, A Survey Of 24 National Registries In North America, Australasia And Europe. Bladen CL, Thompson R, Jackson JM, Garland C, Wegel C, Ambrosini A, Pisano P, Walter MC, Schreiber O, Lusakowska A, et al. *Journal of Neurology*.

Antisense Oligonucleotide Mediated Therapy of Spinal Muscular Atrophy. Sivanesan S, Howell MD, Didonato CJ, Singh RN. *Translational Neuroscience*.

Nutritional Practices At A Glance: Spinal Muscular Atrophy Type I Nutrition Survey Findings. Davis RH, Godshall BJ, Seffrood E, Marcus M, Lasalle BA, Wong B, Schroth MK, Swoboda KJ. *Journal of Child Neurology*.

Spinal Muscular Atrophy: Development And Implementation Of Potential Treatments. Arnold WD, Burghes AH. *Annals of Neurology*.

Dynamics Of Survival Of Motor Neuron (SMN) Protein Interaction With The Mrna-Binding Protein IMP1 Facilitates Its Trafficking Into Motor Neuron Axons. Fallini C, Rouanet JP, Donlin-Asp PG, Guo P, Zhang H, Singer RH, Rossoll W, Bassell GJ. *Developmental Neurobiology*.

Rasch Analysis Of Clinical Outcome Measures In Spinal Muscular Atrophy. Cano SJ, Mayhew A, Glanzman AM, Krosschell KJ, Swoboda KJ, Main M, Steffensen BE, Bérard C, Girardot F, Payan CA, Mercuri E, Mazzone E, Elsheikh B, Florence J, Hynan LS, Iannaccone ST, Nelson LL, Pandya S, Rose M, Scott C, Sadjadi R, Yore MA, Joyce C, Kissel JT. *Muscle and Nerve*.

Genetic Circuitry Of Survival Motor Neuron, The Gene Underlying Spinal Muscular Atrophy. Sen A, Dimlich DN, Guruharsha KG, Kankel MW, Hori K, Yokokura T, Brachat S, Richardson D, Loureiro J, Sivasankaran R, Curtis D, Davidow LS, Rubin LL, Hart AC, Van Vactor D, Artavanis-Tsakonas S.

Proceedings of the National Academy of Science.

The Dcps Inhibitor RG3039 Improves Survival, Function And Motor Unit Pathologies In Two SMA Mouse Models. Gogliotti RG, Cardona H, Singh J, Bail S, Emery C, Kuntz N, Jorgensen M, Durens M, Xia B, Barlow C, Heier CR, Plasterer HL, Jacques V, Kiledjian M, Jarecki J, Rusche J, Didonato CJ. *Human Molecular Genetics*.

The Dcps Inhibitor RG3039 Improves Motor Function In SMA Mice. Van Meerbeke JP, Gibbs RM, Plasterer HL, Miao W, Feng Z, Lin MY, Rucki AA, Wee CD, Xia B, Sharma S, Jacques V, Li DK, Pellizzoni L, Rusche JR, Ko CP, Sumner CJ. *Human Molecular Genetics*.

SMA Valiant Trial: A Prospective, Double-Blind, Placebo-Controlled Trial Of Valproic Acid In Ambulatory Adults With Spinal Muscular Atrophy. Kissel JT, Elsheikh B, King WM, Freimer M, Scott CB, Kolb SJ, Reyna SP, Crawford TO, Simard LR, Krosschell KJ, Acsadi G, Schroth MK, D'Anjou G, Lasalle B, Prior TW, Sorenson S, Maczulski JA, Swoboda KJ; for the Project Cure Spinal Muscular Atrophy Investigators Network. *Muscle and Nerve*.

How Genetic Modifiers Influence The Phenotype Of Spinal Muscular Atrophy And Suggest Future Therapeutic Approaches. Wirth B, Garbes L, Riessland M. *Current Opinion in Genetics and Development*.

Reliability And Validity Of The TIMPSI For Infants With Spinal Muscular Atrophy Type I. Krosschell KJ, Maczulski JA, Scott C, King W, Hartman JT, Case LE, Viazzo-Trussell D, Wood J, Roman CA, Hecker E, Meffert M, Léveillé M, Kienitz K, Swoboda KJ; Project Cure SMA Investigators Network. *Pediatric Physical Therapy*.

Vitamin D Intake Is Inadequate In Spinal Muscular Atrophy Type I Cohort: Correlations With Bone Health. Aton J, Davis RH, Jordan KC, Scott CB, Swoboda KJ. *Journal of Child Neurology*.

Genetic Correction Of Human Induced Pluripotent Stem Cells From Patients With Spinal Muscular Atrophy. Corti S, Nizzardo M, Simone C, Falcone M, Nardini M, Ronchi D, Donadoni C, Salani S, Riboldi G, Magri F, Menozzi G, Bonaglia C, Rizzo F, Bresolin N, Comi GP. *Science Translational Medicine*.

Nuclear Fragile X Mental Retardation Protein Is Localized To Cajal Bodies. Dury AY, El Fatimy R, Tremblay S, Rose TM, Côté J, De Koninck P, Khandjian EW. *PLoS Genetics*.

SMN Is Essential for the Biogenesis of U7 Small Nuclear Ribonucleoprotein and 3'-End Formation of Histone mRNAs. Tisdale S, Lotti F, Saieva L, Van Meerbeke JP, Crawford TO, Sumner CJ, Mentis GZ, Pellizzoni L. *Cell Reports*.

You're Not What I Expected ...

I expected somersaults the day that you were born.
Acrobats and basketballs and old world records torn.



My dreams for you, admittedly, were pretty wild and gigantic.
You'd dance on stage, you'd win the games, you'd make your fans quite frenetic.

I expected many things as proud mamas so often do.
But I never expected to be so awed and overwhelmed by you.

Your legs are weak, your voice so soft, your fingers a bit clumsy.
But those things don't matter much to this totally love-struck mommy.

The world may see a little one whose skills are somewhat lacking.
They see your wheels, your special needs, instead of all that's working.

What I see is not what lacks but what overflows from you.
Your gentle spirit, soft kind eyes, God's spirit shining through.

You're not what I expected, because my baby you're so much more.
This disease may try to limit you, but our God will help you soar.

Your light, it shines for all to see, your personality is like a beacon.
It draws people from far and wide, they love you in an instant.

You're not quite what I expected, my darling this is true.
Yet there isn't even just one thing I'd want to change in you.

You're perfect you see, the very best part of me. You're brave and good and true.
And contrary to what the world may say, there is nothing that you can't do.

You're so much more than I expected!

Excerpt: Adele Keister, "You are God's perfect masterpiece: We love you! Mommy and Daddy."





Bryce Amiel



Daniel and Josh Tramontano



Dougherty Family



Leah Vogedes



Lauren Gibbs



Kayleann Early



Brooklynn Santos



Ethan and Ray Fantel



**Kyle, Hayden and
Jake Smelser**



Cole and Mattie Woodruff



Eleanor Bolton



Emily Lozina



Allegra Keys

Families of SMA Releases Version of Family Guide to Spinal Muscular Atrophy Research Translated in Spanish

Families of SMA has recently released the Spanish version of this booklet, which covers the basics of Spinal Muscular Atrophy research for SMA families.

Families of SMA has translated all of the FSMA Care Series booklets into Spanish editions. There are a total of seven SMA Care Series Booklets now translated and available from Families of SMA. The Spanish Version of the Family Guide to Research is the last current SMA Care Series Booklet to be translated. 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.

This guide helps to answer questions such as what are the key areas of SMA research and what are the costs for conducting SMA drug development and clinical trials. 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 important areas of SMA research:

- SMA Researchers
- Drug Research
- Gene Therapy
- Stem Cells
- Clinical Trials
- Government Research and The FDA

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



Free Care Series Booklets from Families of SMA:

Caring Choices: For Parents of Infants Newly Diagnosed with SMA Type I
 Breathing Basics: Respiratory Care for Children with Spinal Muscular Atrophy
 The Genetics of SMA
 Families of SMA Patient Services and Family Support
 Nutrition Basics: Fostering Health and Growth for Spinal Muscular Atrophy
 Understanding Spinal Muscular Atrophy

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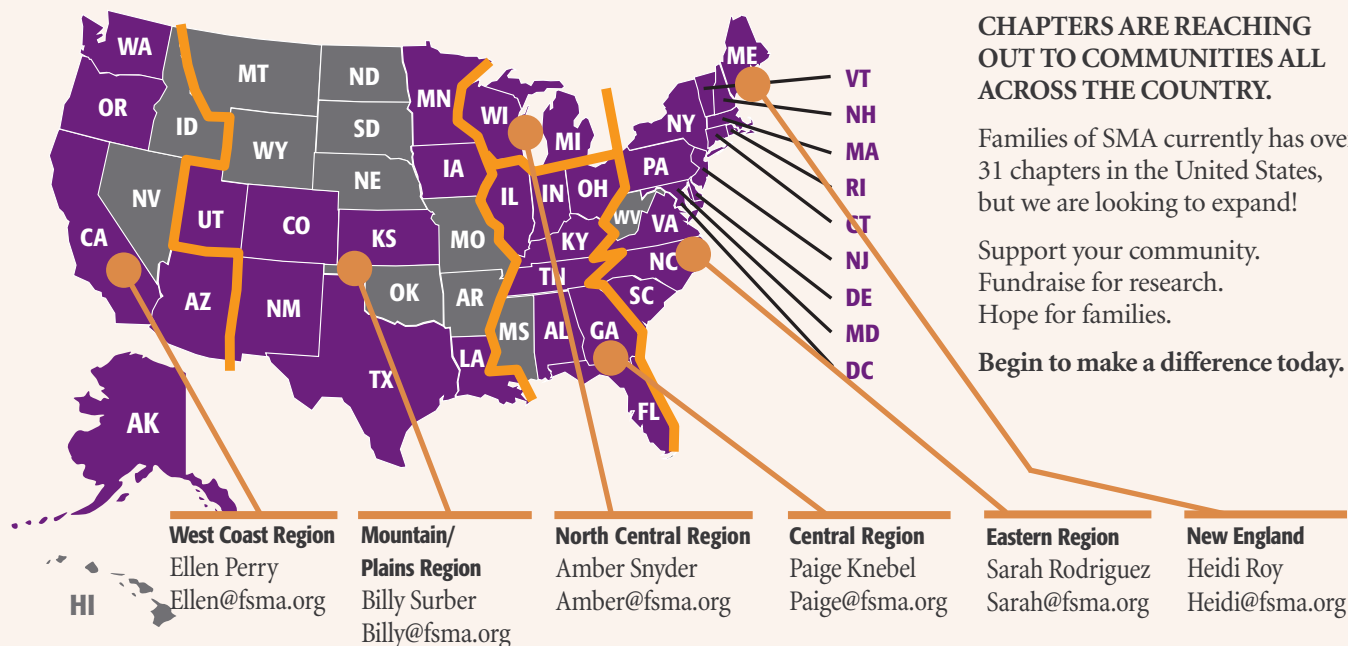
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FSMA Chapters

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

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



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

Promotional Materials from Families of SMA Make Planning a Fundraiser Easy and Fun

Fundraising Materials:



- Toolkits, Manuals and Sample Booklet (Walk-n-Roll, Golf Tournament, Dinner/Gala & Bowl-a-Thon)
- Promotional Tips
- Banners and Yard Signs
- Families of SMA "At a Glance" Flyer
- Purple & Orange FSMA Bracelets
- Plastic Event Bags
- Donation Cards
- Purple & Orange Golf Tees
- Temporary Tattoos
- Coin Canisters
- Families of SMA Pens
- Angel Tags
- Start Flags
- Pop-up Signs
- Tablecloths
- And more!



Merchandise



FSMA Bracelet | \$2



Purple Grocery Bag | \$4



Families of SMA T-Shirt | \$10

Youth Sizes: S M L
Adult Sizes: S M L XL

Golf Umbrella | \$10

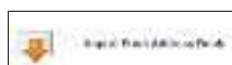


Cinch Bag | \$5



To view a complete
list of Families of
SMA Merchandise visit
[www.fsma.org/Fundraising/
Merchandise](http://www.fsma.org/Fundraising/Merchandise)

New Event Website Upgrades



Quick and Easy Way to Invite Donors

Families of SMA recently added a feature to our event websites which allows you to upload e-mail contacts directly from your address book (Gmail, Outlook, Yahoo!, AOL and a number of other programs are supported). With this new feature, it takes just a few minutes to share your story with all of your friends and family when fundraising!



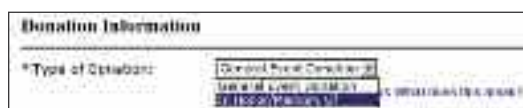
Easy Search Function to Find a Fundraiser

An easy drop-down list is now available as a search method to find teams or participants registered for an event.



Use Social Media to Share Your Page

On your personal fundraising page, you can click on the sharing buttons to post a link to your page on Facebook, Twitter and other social media sites. This is a quick and effective way of asking your friends and family to support your efforts to raise money to help find a treatment and cure for SMA!



Other Website Features

- Gifts made to an event or personal fundraising page can be made in honor or memory of a loved one.
- Donors can mark their contributions as "Anonymous" and remain anonymous on all event pages and personal fundraising pages.
- Participants can post their offline cash and check donations to appear online in their personal, team and event totals to showcase their fundraising success before event day!

ALASKA

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

ALABAMA

11th Annual Alabama Walk-n-Roll

The 11th Annual Alabama Walk-n-Roll was held in Tuscaloosa, AL on November 2nd, 2013. The Walk-n-Roll was a huge success and raised over **\$28,000** to help find a treatment and cure for SMA. We had approximately 225 people in attendance. There were 4 children with SMA and 6 newly diagnosed families in attendance. Everyone enjoyed the face painting, petting zoo, games, silent auction, chili cook-off, bounce house, fire truck, ambulance and police car. We are grateful to ALL the sponsors who generously donated to the cause. Thank you to Jennifer and Jason Patrick and their amazing team of volunteers for organizing this event!



ARIZONA

Chapter Updates

Changing of the Guard

Holly Cotter has stepped down at Vice President but will continue as a member-at-large on the AZ Chapter board. Cassandra Byrd will now serve as Vice President.

8th Annual Arizona Walk-n-Roll

The 8th Annual Walk-n-Roll was held on Sunday, November 17th, 2013, at the El Dorado Park in Scottsdale, AZ. It was a beautiful and sunny 70 degree day. The walk had 13 teams register and participate in the event. Howler from the Phoenix Coyotes and the Suns Gorilla joined us to help kick-off the walk. A special thank you to our good friends, Arizona BBQ Association, for donating all the food again this year for an incredible lunch. We had amazing sponsors, vendors, balloon launch, and games including a balloon artist and face painter. Ellen Perry, Events Manager – West Coast, from the national office attended the event and gave an update on research and funding. The 2013 Walk-n-Roll raised over **\$15,000**.



ARIZONA (cont.)

Addie's Army Crossfit Workout

On September 28th, 2013 Crossfit Maricopa located in Maricopa, AZ hosted an event to benefit Families of SMA in memory of Addison Bailey Faust who lost her battle with SMA Type I at only six months old. In celebration of Addie, the crossfit-style workout included seven rounds of: one deadlift (365/255), seven burpees, 23 KB swings (53/35) and 25 double unders (or 75 singles). The event generated SMA awareness and \$2,940 for research. Thank you CrossFit Maricopa!

**Wayne Industries**

Madison Wolff was selected to take a tour of the top secret Wayne Industries in Phoenix, AZ, along with several close friends. She was able to see the famous bat cave, slide down the bat pole and get a ride in the bat mobile! As part of this wonderful opportunity, Madison was able to select a charity of her choice to receive a \$1,500 donation and she chose Families of SMA. Thank you to Charles Keller for creating Wayne Industries and allowing us to tour this amazing place and for the donation to Families of SMA!

**Texas Roadhouse Dinner Fundraisers**

On August 1st, 15th and 29th, 2013 families gathered at three Texas Roadhouse locations (Peoria, Meza and Metro, AZ) for an evening of wonderful friends and great food. On each date, Texas Roadhouse generously donated 10% of sales to Families of SMA, which totaled over \$540!

**Inaugural SMA Bike Classic**

Scot Raab and the Arizona Chapter of SMA held the Inaugural Bike for a Cure in Flagstaff, AZ, on September 22nd, 2013 and raised \$150! Individuals were served a pasta dinner the evening before the race to get everyone carb'ed and ready for the next day. The participants road through beautiful Fort Tuthill on the custom designed bike trail. Thank you to Scot for organizing and coordinating this event and also to Deer Valley High School for coming up to Flagstaff to attend.

CAROLINAS

North Carolina**1st Annual Carolinas Chapter Walk-n-Roll**

The 1st Annual Carolinas Chapter Walk-n-Roll took place on November 9th, 2013 in Raleigh, NC. It was a great day with many SMA families and friends coming together to help find a treatment and cure for SMA. Thanks to the generous support of family and friends, the event raised over **\$11,000** for Families of SMA! Special thanks to BAYADA for attending and participating in the festivities. Thank you to Jennifer Lee, Carrie Ann Boles, Rebekka Mastin, Alana Coates and Johanna Elsner for organizing the inaugural Carolinas Chapter Walk-n-Roll.

Haley Mitchell's Memorial Gift Registry

Haley Mitchell, SMA Type I, passed away on October 26, 2010, shortly after her 13th birthday and also shortly after the 12th Annual Haley Mitchell Ski-a-thon. In October of 2011, the final Haley Mitchell Ski-a-thon to honor her memory was held and the event that has been so wonderful. This year Jeff and Valerie Mitchell of Elizabeth City, NC raised over \$7,600 in Haley's memory through a letter writing campaign. Thank you to Jeff and Valerie for their continued support of Families of SMA!



CAROLINAS (cont.)

6th Annual JPL Foundation Silent Auction

The 6th Annual Jocelyn Paige Lee Foundation Silent Auction was held on August 17th, 2013, in Louisburg, NC. The silent auction had live music by The Lebeaus and food provided by The Cook Shack! There were door prizes, a 50/50 raffle, and dinner at the fundraiser, which they wound up raising \$4,840 in the end! A special thanks to Shane and Jennifer Lee for organizing this event and continuing to support Families of SMA.



honor of Gray Dougherty, SMA Type II! The event raised about \$275 for Families of SMA! Thank you to Mrs. Smith for all of your hard work in organizing this fundraiser.

Nags Head Elementary Dress Down Week

My school held an event in memory of our colleague's grandchild, Logan Moore, SMA Type I, on September 23rd, 2013. We raised this money during a dress down week at our school, Nags Head Elementary in Nags Head, NC, and wanted to remember this little one in this way. We hope this \$95 contribution will make a difference in a child's life or those family members affected by this disease.

Judith Fearing
1st grade teacher, Nags Head Elementary

South Carolina

2nd Annual Mistletoe Jam

On December 5th, 2013, Donny McNerney hosted the 2nd Annual Mistletoe Jam at Zenzer Restaurant in Landrum, SC to raise money for Families of SMA. The event was held in honor of Alexandra Genovese, SMA Type II. It was a huge success and raised \$3,995! "I

want to find a cure, but in the meantime, the money we raise can help with functionality in kids' lives," McNerney said. "Until we find a cure, we can offer these kids hope." A special thanks to Donny McNerney and Chris Genovese for organizing this event to support Alexandra!

Ellie's Army



There are no words to express how thankful we are for everyone who donated their time, money, gifts, and/or positive thoughts and prayers to make Ellie's Army Poker Run a huge success! The event held on August 31st, 2013 in South Carolina in honor of Ellie Burns, SMA Type I, raised \$50 for Families of SMA. Thank you to Christina English for her help with this event!

Megan and Jonathan Burns
Mt Pleasant, SC

Providence Day School Fundraiser

Providence Day School in Charlotte, NC held a fundraiser on December 16th, 2013, in



CHESAPEAKE

20th Annual Crab Feast and Silent Auction

The Chesapeake Chapter reached a milestone this year! We celebrated and sponsored our 20th Annual Crab Feast and Silent Auction on August 4th, 2013 in Towson, MD. Another record year for us, we sold out the event with over 350 people, served over 36 bushels of crabs (that's more than 200 dozen), and with the support of MANY people, raised more than \$51,000, including \$9,000 from the silent auction. The success of this event would not have been possible without the generous support of area businesses, volunteers and ticket "captains" responsible for selling out the event. Special thanks to Beverly Venedam for coordinating the silent auction, John Nolan and Dan Venedam for organizing ticket sales, the Knights of Columbus for staffing the wheels, and Eileen Venedam for managing the service volunteers. All proceeds from the event support the Erin Trainor Memorial Fund.



CHESAPEAKE (cont.)

19th Annual Charity Golf Classic

The Chesapeake Chapter held the 19th Annual Golf Classic presented once again by Cramer Rosenthal McGlynn, LLC in Windsor Mill, MD on September 20th, 2013. This marked the 15th and final year where Scott Geller served as Golf Chairman of this event. We are so thankful to Scott and Cramer Rosenthal McGlynn for their passion, support and friendship over these many years. Not only have they been instrumental in raising millions of dollars for Families of SMA over the years, they have increased awareness of SMA throughout the community by reaching so many people unaffected directly by SMA. With more than 45 sponsors and 160 golfers, we raised almost **\$136,000!** Special thanks to Barbara Trainor and Beverly Venedam for coordinating all of the logistics, the many volunteers who ensured everything ran smoothly from mulligan sales to 50/50's to Hole-in-One contests, and Mary Kate Venedam for organizing the photography on the course this year. All proceeds from the event support the Erin Trainor Memorial Fund.



CHESAPEAKE (cont.)

Eastern Savings Bank Fundraiser

On August 28th, 2013, the Eastern Savings Bank Fundraiser raised over \$800 for Families of SMA! A special thanks to Deborah Scible for organizing this fundraiser at the Hunt Valley, MD branch and for your support of Families of SMA.

Our Little Jewels 3rd Annual Benefit Golf Outing



During 2013, Our Little Jewels held its 6th annual spring fundraiser and 5th Annual Fall Golf Outing. The spring fundraiser was held at Kelsey's Irish Pub in Ellicott City, MD. In April, the event raised **\$15,870**. The fundraiser was attended by over 200

people. The fall golf outing was held at The Links at Challedon in Mt. Airy, MD in September. This event raised \$17,296. The outing sold out for the 5th year in a row. There were 124 golfers and 16 volunteers.

During the past 6 years, Our Little Jewels has raised over \$200,000. This money is divided among Families of SMA, MDA, Kennedy Krieger's "Bennett Blazers Sports Program for Physically Challenged Children", The Kids Korral Horse Farm, Kaleidoscope Therapeutic Ring Center, The MD Council for Special Equestrians, St. Jude Children's Hospital and The Autism Project in Anne Arundel Co., MD.

We are very proud that we have been able to help many physically and mentally challenged children get involved in these programs, as well as helping to fund the research efforts of FSMA. Our Little Jewels looks forward to an exciting 2014.

West Virginia

17th Annual Arnold Family Golf Outing

On October 14th, 2013 at The Berry Hills Country Club in Charleston, WV, the 17th Annual Arnold Family Golf Outing took place. In the invitations to the golf outing, Linda composed a letter explaining some of the aspects of the disease, current research, status, and of course, about Eric. The response from family, friends, and the community at the tournament was overwhelming!

This golf tournament started in 1996. Kevin Arnold wanted to know what Linda wanted for her birthday. She decided she wanted to do something for Families of SMA. And the golf tournament continued for 17 years and counting! The event was hosted by Linda and Kevin Arnold in honor of their son, Eric Arnold, SMA Type II. Eric has been an integral part of making the tournament so special. Thank you, Linda and Kevin, for hosting yet another successful golf outing and for your endless support of funding promising research for SMA!



CONNECTICUT

SMA Kid's Cut-A-Thon in honor of Cubby Wax

Held on August 25th, 2013, the SMA Kid's Cut-A-Thon in honor of Cubby Wax, SMA Type I, took place in Ridgefield, CT. The event raised almost \$2,000 for Families of SMA. Thank you to Erin Simmons and her staff for organizing this fundraiser and for your continuous support

West Rocks Middle School Fundraiser

The community service club of West Rocks Middle School in Norwalk, CT implemented a fundraiser for Families of SMA during the spring of 2013 that was very successful and raised \$1,200. We recognize that there is a need for additional research for a treatment for SMA. Our hope is that this contribution can help in the continued research for a cure and treatment of SMA.

Alimina Ball
Coordinator of Community Service Club

GEORGIA

SMA Day with the Atlanta Braves

The 2nd Annual SMA Day with the Atlanta Braves took place on August 17th, 2013 in Atlanta, GA. The event was a great opportunity for SMA families to meet while enjoying a Saturday evening game at Turner Field! The event was a huge success, raising \$3,688 for SMA research! Thank you to Anami Lehmann for organizing such a successful event! Keep up the great work!

**Graeme's Fundraiser**

Thank you to Kyley Jones of Fayetteville, GA, for \$2,700 through a gift registry in honor of her nephew, Graeme Mooney, SMA Type I!

**Fundraiser for Miller — Go Miller Go**

Thank you to Lindsay and Eason David of Atlanta, GA for raising over \$2,500 for Families of SMA in honor of their son Miller David, SMA Type I!

S.M.A.C.K.

S.M.A.C.K. took place on October 30th, 2013 at Pike Middle School in Georgia. The Middle School Class raised money throughout the school year in 2012-2013 to support a student's brother who has SMA! The fundraiser raised over \$2,000 to support Families of SMA! Thank you to Pike Middle School to organizing this fundraiser and for your support for Families of SMA!

GREATER FLORIDA

Chapter Updates**Greater Florida: So What's Next?**

The Greater Florida Chapter's executive board is changing this year, but our mission of service remains the same. From Pensacola to Naples and everywhere in between, we are working together as a chapter to increase awareness of SMA, raise funds for its cure, and provide support for affected families.

2014 Board Members

President: Audra Butler
Vice-President: Laurie Sore
Secretary: Lisa Hoang

Katie Kerns, who restarted the chapter in 2009 and has been such a great champion for our members, will now serve as the main point of contact for newly diagnosed families. We'd like to thank Katie for her leadership during the past five years, the passion she has dedicated to our cause, and her continued commitment to our chapter.

As we move into 2014, we hope to build upon our chapter's successes and to create more positive momentum. We are now looking for a volunteer for the executive board position of treasurer. In the coming weeks, we will be creating committees to help support our fundraising and awareness efforts too. Please let us know if you are interested in being more involved in chapter leadership or in any of our activities. Also, please let us know what kind of events you'd like to see on our 2014 calendar!

Whether you serve on the board or on a committee, attend a meeting, volunteer your time, or participate in a chapter event, YOU are making a real difference in the fight against SMA. Together, we WILL find a cure!

All the best in 2014,
 Audra, Laurie, and Lisa

Greetings from the Greater Florida Chapter!

2013 has been a busy very for our chapter! Make sure to stay connected with us by regularly checking our website at www.fsma.org/greaterflorida and joining our "Families of SMA – Greater Florida Chapter" group on Facebook (<https://www.facebook.com/groups/79658598961/>). We also send a bi-monthly e-mail newsletter to our chapter members. Not sure you are on the e-mail list? Send a message to greaterfl@fsma.org with your name and contact information today!

If you are Interested in starting a fundraising event in your area, please send an e-mail to greaterfl@fsma.org! Thank you, Greater Florida, for another great year so far!



GREATER FLORIDA (cont.)

Lanterns Over the Bay – Annual SMA Candle Lighting and Picnic

More than 55 chapter members attended this year's evening picnic at a beautiful waterfront park in St. Petersburg, FL on August 10th, 2013 to light lanterns to honor our SMA warriors and to remember our angels. It was a beautiful night, filled with camaraderie, celebration of loved ones, and hope.

21st Annual Tampa Maluko Charity Golf Classic



The Maluko (defined as “good friend” made up by the event’s organizers) began as a simple way to show their passion for the game of golf and the camaraderie of good friends and good cheer. This year, they held their 21st golf tournament at the Carrollwood County Golf and Country Club in Tampa, FL on October 28th, 2013 to raise funds for charities close to their hearts. A generous donation to Families of SMA was made on behalf of Tyler Hernandez, SMA Type II and the Hernandez

family in the amount of \$32,940. Thank you to Chris Leto and Joy Leto for all of their work in planning this event!

5th Annual Greater Florida VIRTUAL Walk-n-Roll

In a first of its kind event for Families of SMA, the Greater Florida Chapter hosted an innovative online fundraiser for the entire month of November. The virtual walk featured weekly team competitions, interactive web posts, and great prizes to excite and involve its participants. Through the virtual walk, we raised just under \$13,000 to fund SMA research and family support programs.

The Tampa Bay Lightning Foundation



inspiring others in the Tampa Bay community. Chapter member Elio Navarro, SMA Type II/III was selected as one of these individuals! The Tampa Bay Lightning Foundation awarded him with \$50,000 to give to local charities and he has chosen to donate \$10,000 to our Greater Florida chapter! Congratulations and thank you, Elio!

13th Annual Costume Crusade

Every year the children of the Village Early Learning Center in Brandon, FL dress up and parade around in their costumes

hoping to raise money and awareness for SMA on October 25th, 2013. An annual tradition at the event is the wheelchair race – this year the competitors were Joe Miller, SMA Type III, and Allison Kerns, SMA Type II. Allison was crowned as the winner! This year the event raised \$2,384. Thank you for Joe and Sue Miller for all of your support of Families of SMA!

City of Winter Haven Lunch Fundraiser

Featuring the great cooking of their brother-in-law, the Nowotny family teamed up with the city of Winter Haven and other local businesses, including Renee Butler Realty of Winter Haven and Reunion Resorts of Orlando, to offer delicious lunches for \$7 a plate to all employees on October 21st, 2013. This fundraiser, in memory of Cooper Nowotny, SMA Type I Angel, raised a phenomenal \$1,581!



Donation from Image Forward LLC in Honor of Ava Davidson

Image Forward LLC made a generous donation of \$1,000 to the Greater Florida Chapter. The company held a contest for its employees this summer, with a donation going to the winner's charity of choice. Lewis Davidson, father to Ava Davidson, SMA Type II/III won! Thank you to the Davidson family for selecting Families of SMA to receive this substantial donation and to Image Forward for being such a charitable company!



LEGOLAND Buddy Benefit Days

In support of our SMA Awareness Month activities, LEGOLAND Florida made available deeply discounted park tickets to Greater Florida chapter members and friends for a limited time in August. LEGOLAND Florida generously donated \$15 of every ticket purchased back to Families of SMA, raising a total of \$660.

GREATER FLORIDA (cont.)

Fondue-raiser hosted by The Melting Pot

Friends and families came together to enjoy a four-course fondue meal, with a portion of the proceeds donated to Families of SMA on October 24th, 2013. This night of friendship, fun, and yummy food raised \$635!

**SMA Day with the Rays**

Once again, our chapter members came out to Tropicana Field in St. Petersburg, FL on August 17th to raise SMA awareness and money for a cure! The Tampa Bay Rays donated approximately \$625 in ticket sales to Families of SMA, and our chapter PSA played on the jumbo-tron for all to see!

**Painting with a Purpose Fundraiser**

Friends and families gathered to paint a keepsake picture on August 3rd, 2013 in St. Petersburg, FL, while enjoying each other's company and a little wine, too! This was our second time Painting with a Purpose, and it was another successful event, raising \$428 for the chapter!

William's 1st Birthday Party in Honor of Andrea Trakas

For our son William's first birthday in Tampa, FL on May 4th, 2013, we requested that guests make donations to a few charities that are close to our family's heart, Families of SMA is one of those charities. We have very close family friends who lost their daughter Andrea Trakas to this horrible disease and we hope that the \$310 raised will help bring us closer to a cure.



We are blessed that he is now a healthy little boy. We know all too well the helpless feeling of having a child that is ill, as William arrived six weeks early after an extremely complicated pregnancy and life threatening delivery. After a month in the NICU, we were finally able to take our little guy home and he has been thriving ever since. It is our hope that all children have the best quality of life so that they may celebrate many more birthdays. Thank you for making sure these donations get where they can do the most good and we plan to continue to support Families of SMA throughout the future.

Stephanie, Tom, Sophia and William Stuhlsatz
Tampa, FL

Night of Family Fun at the Village Early Learning Center

Complete with haunted houses, trick-or-treating, face painting, bounce house and lots of fun, "The Village" and the Village Family Foundation hosted another great community event on October 26th, 2013 in Brandon, FL. Last year, we raised more than \$3,000, and this year their goal was to exceed that!

Date Night at the Cork & Olive Wine Bar & Café

The Village Early Learning Center also hosted a night out on the town for a worthy cause at Cork & Olive Wine Bar & Café in Brandon, FL on November 2nd, 2013. With karaoke, tasty hors d'oeuvres, and lots of great raffle prizes, it certainly was a wonderful night out. Even better, 15% of the proceeds were donated to Families of SMA!

GREATER NEW YORK

9th Annual Greater New York Walk 'N Roll to Cure SMA

The 9th Annual Greater New York Walk 'N Roll was held on September 21st, 2013, in Long Beach, NY. The day included a 50/50 raffle and face painting for the kids to enjoy! Almost **\$30,000** was raised to support Families of SMA! Thank you, Debbie Cuevas and all our amazing teams, for another successful year and helping us raise this outstanding sum to help cure SMA!

Greater NY Chapter Golf Outing

The Great New York Chapter Golf Outing took place on October 7th, 2013, in Ossining, NY. The outing included a fun day of golf, lunch, and a dinner after the tournament! The outing raised over **\$19,000**. Thank you to Richard Rubenstein and Ron and Debbie Cuevas for organizing this event!

GREATER NEW YORK (cont.)

ING New York City Marathon 2013

The ING New York City Marathon was held on November 3rd, 2013, in New York, NY. The marathon raised **\$28,940** to help find a cure for SMA! Thank you to all of our runners for your hard work in training for the marathon and raising funds for SMA: Mandy Cassamasima, Shannon Dionis, Julie Fazio, Frank Loverro, Juliano Lozina, David Scott, Michelle Van De Loo and Laurette Zion. Thanks to Michelle Erwin for all of your work organizing this event!



NY Mets SMA Awareness Day

SMA Awareness Day was held at the New York Mets vs Detroit Tigers game on August 24th, 2013 in memory of Benjamin Hill Rider, SMA Type I. The game had plenty of food and beverages for everyone as they cheered on their favorite team! The event raised **\$16,837** this year for Families of SMA! Many thanks to Debbie Cuevas for organizing this fun-filled event, and special thanks to Glenn Hill and Peter Rider for your support!



NY Yankees SMA Awareness Day

The NY Yankees SMA Awareness Day was held on August 9th, 2013 at Yankee Stadium in Bronx, NY. The game was the NY Yankees vs. Detroit Tigers, and was a blast for all of the families! The event also wound up raising **\$12,550** for Families of SMA! Thank you to Debbie Cuevas for organizing this event!



Origami Owl Fundraiser

The Origami Owl Fundraiser held September 24th, 2013, raised over \$300 for Families of SMA! A special thanks to Holly Verdile for organizing this fundraiser and for your continuous support for Families of SMA.

Mabel's Labels

We held an online fundraiser this fall through "Mabel's Labels" where a portion of the sales were collected toward Families of SMA. We raised \$117 in honor of our daughter, Emily Lozina, SMA Type I. We appreciate everything that Families of SMA does in terms of research, support and hope for families like ours, living with SMA.

Jenn, Kresh, Chris and Emily Lozina
Cos Cob, CT



GREATER NEW YORK (cont.)

Capital Region

2nd Annual Capital Region 5K Run, Walk-n-Roll

The 2nd Annual Capital Region 5K Run, Walk-n-Roll was held in Loudonville, NY on September 7th, 2013. The event was a great success. Thanks to their amazing fundraising efforts, they donated over **\$17,000** to help find a cure for SMA! Thank you to Amy Cuniff and the late Dave Cuniff for organizing the event in honor of their son, Caleb Cuniff, SMA Type II, and all those affected by SMA!

David Cuniff was an amazing father and advocate for SMA. He will be greatly missed by all who knew him and our thoughts continue to go out to the Cuniff family and friends.



Kure SMA Day

A huge thank you from Kure for Kulas and Families of SMA to our sponsors, volunteers and contributors. Thank you for supporting an amazing cause, not only for Kalen and Kyan, who both have SMA Type II, but for so many SMA families across the world! Kreate Miracles! Because of everyone's generous support, we were able to raise \$1,769 for Families of SMA at the event held on September 14th, 2013 in Binghamton, New York.

Together We Will Find a Cure!

Sierra and Jared Kulas
Port Crane, NY



ILLINOIS

7th Annual Kyra's Idea... Angie's Hope Strike Out SMA Fundraiser

On September 21st, 2013, the 7th Annual Kyra's Idea... Angie's Hope Strike Out SMA Fundraiser took place in Aurora, IL. The event is held every year in honor of Angie Lee, SMA Type II, by the Lee and Scadden families. The day included a pasta party, silent auction and lots of fun games. Over **\$25,000** was raised for Families of SMA.

17th Annual Chicago Half Marathon & 5K

Members of Team Families of SMA ran the Chicago Half Marathon & 5K on September 8th, 2013 in Chicago, IL. This is the third year that Families of SMA participated in the run as an official charity partner. Together, the runners raised over **\$11,700** to help cure SMA! Thank you to all of our runners for their hard work in training to run this race!



ILLINOIS (cont.)

Taste of Chicago Cook Off

In July of 2013, Chef Abraham of Fat Rice in Chicago, IL entered a cook off at the Taste of Chicago, the nation's premier outdoor food festival, and won \$5,000 to support his favorite local charity, Families of SMA. The money he won was donated to Families of SMA in honor of his niece, Abigail Lehner, SMA Type III.

We would like to give a special thank you to Chef Abraham for choosing Families of SMA as your favorite charity at the Taste of Chicago. Thank you also to PepsiCo and Lipton Tea for sponsoring the donation and the cook-off!



resembled that of SMA. This began our month long journey of living an emotional rollercoaster.

While waiting for an appointment with the neurologist, I combed the internet for any information, which is where I learned about Families of SMA. I spent hours crying while reading about families affected by SMA. That morning, I had never even heard of this terrible disease, but by that afternoon, it had become a very possible life changing reality for our family. After waiting for the results of Cole's blood work for a little over a week, we received the news that Cole did not have SMA. I'll never forget the moment when I got the call. I sat sobbing in the parking lot of the grocery store, unable to control my emotions.

While we are so thankful for a healthy baby, we know that many others are experiencing overwhelming pain, sorrow and grief that we briefly experienced. SMA is a disease that is close to our hearts, and our goals are to help bring awareness to others and to eventually help find a cure. Cole turned one on June 21st, 2013 a day that we weren't sure he would see. We had a big party to celebrate his life. In lieu of gifts, we asked for donations to be made to Families of SMA. Our \$200 donation is in memory of those whose lives were lost too soon and to honor those who continue to fight.

The Bramley Family
Chicago, IL

Chicago Cubs Rooftop Game Fundraiser

Families of SMA teamed up with Beyond the Ivy for an afternoon rooftop game against the Atlanta Braves on September 22nd, 2013, in Chicago, IL. The game included food, drinks and rooftop views of the game! Thank you to Shannon O'Brien for organizing this event and raising over \$5,000 for Families of SMA!



Cure SMA Awareness Benefit Concert

The Cure SMA Benefit Concert held in Springfield, IL was a great success! The concert lasted until about 1:00 a.m. and it consisted of music, a raffle, and silent auction. The event raised \$110 for Families of SMA! Thank you to Justin Hoyt for organizing this wonderful benefit concert!



Lake Geneva Triathlon

Horace Seeley-Brown ran the Lake Geneva Triathlon held in Lake Geneva, IL, in September 2013. He did this in honor of his friend and fraternity brother, Steve Cannady, his wife Gina and three daughters. Emma and Ruby Cannady were both diagnosed with SMA. Thank you to Horace for raising over \$4,100 for Families of SMA!



Music for Mckenna

On December 7th, 2013, Music for Mckenna was held at Norge Ski Club Clubhouse in Fox River Grove, IL. The event was in honor of Mckenna Jurgovan, SMA Type I. The night featured music from the Early Baroque and Baroque by Castello, Frescobaldi, Kapsberger, Bach, Telemann, de Visee, Marais, and others for recorder and theorbo, performed by Mirja Lorenz and Joel Spears. The event was a huge success, as it raised \$420! Thank you to Mirja Lorenz for all your hard work in organizing this event.



Cole's 1st Birthday Party

When our son Cole was four months old, we received life altering news. At his four month check up, his pediatrician referred us to a neurologist due to his low tone and sporadic tongue movements. While she gave us no specifics as to why he was being referred, Google was able to tell us what no parent should ever hear: Cole's symptoms

IOWA

Small Heroes, Mighty Cause: Cascade 5K for SMA

On August 2nd, 2013, the Small Heroes, Mighty Cause: Cascade 5K for SMA was held at Cascade River View Park in Cascade, IA. The event, held in honor of Colin Schlemme, SMA Type II, was a huge success, raising almost **\$34,000** for Families of SMA! After the run, there was a live band in the River View Park Amphitheater! A special thank you to Amanda Schlemme for organizing this event! With your help in raising awareness and funds, we are that much closer to finding a cure for SMA!

**17th Annual Beaverdale Beaverdash**

The 17th Annual Beaverdale Beaverdash took place on September 14th, 2013 in Beaverdale, IA. This year's event was once again, a major success! Hundreds of people gathered to run the Beaverdash this year, and they ended up raising over **\$26,000** for Families of SMA! Thank you to all the volunteers and everyone who participated in the event! A special thanks to Julie Greenwood and Julia Anderson for organizing another successful Beaverdash event! Keep up the great work!

KANSAS CITY

Kansas**Kansas City Chapter 13th Annual Cure SMA Race-n-Roll**

The Kansas City Chapter held their 13th Annual Cure SMA Race-n-Roll on October 5th, 2013 in Roeland Park, KS. The Race-n-Roll was a huge success, raising over **\$37,000** for Families of SMA! Thanks to Kristal Wilson and Kim Sykora for organizing this event!

Matthew's 10th Birthday

My son Matthew celebrated his 10th birthday on July 4th, 2013 with friends. Instead of gifts, he asked for donations to support Families of SMA in honor of his good friend Charlie Sykora of Leawood, KS who has SMA Type I. We are so thrilled to donate \$175 from the party. Thank you so much for all the good work you do!

Dan Greer
Leawood, KS

**Missouri****11th Annual Bommarito Z Club Car Show**

The 11th Annual Bommarito Z Club Car Show took place on September 8th, 2013 in Bommarito Nissan, in St. Louis, MO to benefit Families of SMA. Everyone was able to enjoy food, drinks, and a DJ and able to take a look at amazing cars. Each year, this event is in honor of Brittany Carpenter, SMA Type II, and in memory of Madeline Schmidt, Brian Goodyear and Michael Goodyear, all SMA Type I. In total, over **\$17,000** was raised for Families of SMA!

Thank you to Janet Hutchinson and Steve Colesworthy for organizing such a fun-filled day!

LOUISIANA

Rock-n-Bowl

The Rock-n-Bowl Fundraiser in New Orleans, LA held on July 28th, 2013 was in honor of Jackson Audibert, SMA Type II. The event was a blast, and included bowling, face painting, food, beverages, silent auctions and music provided by The Yat Pack! The event raised **\$37,400** to help find a cure for SMA! A special thanks to Mike Otillo for organizing the rock-n-bowl and making this whole event possible.

Art Fair Fundraiser

The Art Fair Fundraiser was held in Yellville, AR on October 12th, 2013. A special thanks to Mary Bauss for organizing this art fair and raising \$700 for Families of SMA.



MICHIGAN

Spa-Toe-Pia Pennies for Polish Fundraiser

The Spa-Toe-Pia Pennies for Polish Fundraiser was held on September 1st, 2013, in Dowagiac, MI, in honor of Maggie Sue Kazlauskas, SMA Type I! The fundraiser was a huge success, raising \$1,500 to support Families of SMA! A special thanks to Jacqueline Hale for organizing this fundraiser!

Chemical Bank 5K & Fun Run

We had close to 1,000 participants in this year's 9th Chemical Bank 5K and 1/2 Mile



Kids Fun Run held on July 27th, 2013 in Byron Center, MI. While the number of people participating in our event is exciting, the best part is the support that we are able to provide to our many race beneficiaries.

On behalf of Chemical Bank, I am pleased to donate \$500 to Families of SMA. We enjoy the opportunity to give back to the community through donations. It has been a privilege to include the Families of SMA as a race beneficiary in

all 9 years of the race. Let's hope it can help find a cure very soon!

Thank you to Tim and Kami Potjer for their continued support and help with this event!

Scott Ellison
Chemical Bank 5K Race Founder
Byron Center, MI

Locketts for Lana (Origami Owl) Fundraiser

The Locketts for Lana (Origami Owl) Fundraiser took place on August 31st, 2013. The fundraiser raised \$200 for Families of SMA! Thank you to Jane Trastain for your hard work in organizing this fundraiser in honor of Lana Wilson, SMA Type I!

MINNESOTA

13th Annual Cure SMA Walk-n-Roll

On September 14th, 2013, the 13th Annual Cure SMA Walk-n-Roll took place at Silver View Park in Mounds View, MN. There were over 300 registered family and friends who participated in the 5K. At the walk-n-roll, there were a variety of games to play, a raffle, and an opportunity to meet other families! The fundraiser attendees helped to raise \$11,700 this year! A special thanks to Team Sophie who raised \$2,040, and Team Zenderman, The Ryan Inselman Team and Team Ben who each raised \$500! Thank you to all of the amazing volunteers who made this day possible and the Minnesota Chapter board for all of their hard work planning this event.

Ottinger 141st Birthday Celebration

My husband, Miles, and I decided to have a birthday celebration for ourselves to celebrate



our combined age of 141 years on August 1st, 2013 in Plymouth, MN. We sent an evite and advised no cards or gifts, but we would accept donations to Families of SMA in honor of our granddaughter, Isabelle (Bella) Martineau Andrade, SMA Type II. About 50 people attended including our grandchildren Bella, Nico, and Megan. It was a beautiful day filled with friends, a fun banjo band, great food and fun for all. Our party raised \$1,400 for Families of SMA!

Miles and Joan Ottinger
Plymouth, MN

Abigail Rose Awareness Event

Stacy Homstad organized the Abigail Rose Awareness Event throughout August, which happens to be SMA Awareness Month! The fundraiser raised over \$1,000 for Families of SMA! Thank you to Stacy Homstad for organizing this event in honor of Abigail, SMA Type I!

NEW ENGLAND

Maine

Birthday Gift Registry in Memory of Sullivan Bushey

Thank you to Brittany Madore and Tim Bushey of Limerick, ME for raising over \$675 in memory of their son, Sullivan Bushey, SMA Type I, through a birthday gift registry.



NEW ENGLAND (cont.)

Massachusetts

7th Annual Fall Golf Classic



Well, the golfers weren't singing but they sure were "Swinging" in the Rain on Friday, October 4th, 2013, for our 7th Annual Fall Golf Classic in honor of William Johnson, SMA Type II, in Bellingham, MA. Despite the very unexpected change with the weather (a 30% chance of spot showers was really over three hours of relentless downpours in Bellingham), most everyone stuck it out and helped us raise over **\$47,000** for the New England Chapter of Families of SMA. So to all the waterlogged golfers, sponsors, auction donors, volunteers, dinner guests and supporters, we thank you from the bottom of our soggy hearts! Extra special thanks once again to Will Aguilera for sporting a monkey suit on the course (warmer than the banana costume last year) in his creative fundraising effort which added over \$10,000 to our final total.

Heidi and Trip Johnson
Medfield, MA

The Halloween Hustle



The 2nd Annual Halloween Hustle and Family Fun Run was held on a beautiful crisp autumn day. We added to the fun this year by adding trick or treating along the fun run route, and having a Halloween hunt to find treats and goodies in the field! We also had a fantastic raffle, and a huge turnout for our kids costume parade. This event is held in honor of my son Paul Osborn SMA Type III,



who is my inspiration to help other families living with SMA. I am humbled by the efforts of Matt Pearson and his Tri Sharks Triathlon Club who organize the event each year. We were honored this year to be joined by two other SMA families without whose support we never would have reached our fundraising goal, so a heartfelt thank you to the families of Nico Sapienza and Brendan White! Thank you to everyone for helping us raise \$7,400!

David and Amy Osborn
Cherry Valley, MA

Celebrate Ashleigh Bike Run & Benefit



Our 2nd Annual Celebrate Ashleigh Bike Run & Benefit was held on August 3rd, 2013, in Rehoboth, MA in memory of our daughter, Ashleigh Jacques, SMA Type I. This year we had 45 motorcycles for the 75 mile bike run, and raised almost \$4,700. The bike run was followed by a benefit which included a cookout, DJ, raffles, moonwalk, face painting, spirit mediums and a karate demonstration. We also had a pipe and drum band played by local firefighters. There was a lot of fun for the entire family! Our oldest daughter, Brianna, worked very hard and made many drawings which she sold and donated the proceeds to Families of

SMA. Ashleigh's grandmother made handmade rose petal angel beads to sell. A family friend made bracelets with feather charms in memory of Ashleigh. We were very thankful for the great turnout we had. This year has been very difficult after the loss of our daughter Ashleigh. We will continue to host fundraisers yearly as a way to celebrate our little angel, and to raise awareness and funds to find a cure for SMA.

Kristen and Brian Jacques
East Taunton, MA

Victory for Violet SMA Weekend

The Victory for Violet SMA Weekend event on August 3rd-4th, 2013 was held in Southwick, MA. It started with a Poker Run on August 3, and dinner after. There was also a local girl who has SMA who cut the ribbon to start the ride. Sunday's event included a hair event at a salon in town. A local pizza place sold slices of pizza and donated the money to Families of SMA! The weekend event was a success, raising about \$1,500! A special thank you to Kelly Clendenin for organizing this weekend event!



Johnson Garage Sale

The Johnson family hosted a garage sale on October 26th, 2013 in Medfield, MA. The yard sale raised about \$1,450 for Families of SMA! Special thanks to Heidi and Trip Johnson for hosting and to The Schilling Family for donating to this garage sale fundraiser! Keep up the good work!



NEW ENGLAND (cont.)

7th Annual Ride for Alex

On Sunday, September 15, 2013, the 7th Annual Ride for Alex was held at the VFW in Freetown, MA. This event was started by one of his nurses, Tammy, who noticed how much was not covered by medical insurance. Tammy and her husband had been involved in other motorcycle fundraisers, and knew they could organize one for Alex! With the help of their friends and family, they have been able to make this a yearly event—and one everyone looks forward to! It is a day to celebrate Alex and all that he has accomplished. He enjoys meeting everyone as much as they enjoy meeting him! Over the years, this event has raised money to provide Alex with a wheelchair swing, computer equipment to help him communicate with his friends and family, and most importantly a wheelchair accessible van! Because of the van, Alex is able to go places with his family, such as trips to Maine, sledding, and hockey games just to name a few! This year's event raised \$545 for Families of SMA!



Rhode Island

4th Annual Working on Walking Golf Tournament and Dinner

On August 12, 2013, the 4th Annual Working on Walking Golf Tournament and Dinner took place in North Providence, RI. The golf tournament included golf, prizes and a sit down dinner at the Twin River Banquet Room. There was also a raffle and silent auction held at the dinner which included prizes such as a 32G iPad2, Red Sox tickets, and Patriots tickets! The event ultimately raised **\$35,200** for Families of SMA. Thank you to Mary and Fred Smith and Alyssa Silva, for making this event possible and your generous support of Families of SMA!



CURE SMA Quilt by Melanie Vagnini

Talented seamstress, Melanie Vagnini, once again donated her beautiful CURE SMA quilt to be raffled off during the month of October. This year, the raffle was offered nationwide to raise funds and to raise awareness, once the winner starts putting the quilt to use. Gladys Miedel, of Mattawan, MI, was the fortunate winner! And special thanks to Melanie for her incredible support over the last 10 years!

Heidi and Tripp Johnson
Medford, MA



Brooklyn's 1st Birthday to Raise Funds for SMA

On September 11th, 2013, Brooklyn Bouchard, SMA Type I, turned one in heaven, but here we held a first birthday party for her! Margarita's Mexican Restaurant allowed us to share a portion of the night's profits to be donated to Families of SMA in Brooklyn's name. Friends gathered, ate and spent time looking at pictures of Brooklyn, along with enjoying our dear friend and volunteer, Buddy the clown. Buddy graciously came from Vermont to donate his time and made all kinds of balloons for kids and kids at heart. My dear friend, Jennifer Geddes, worked so hard to make dozens of cupcakes with white frosting and purple sprinkles to be handed out! Brooklyn always loved when we celebrated her monthly birthdays with her when she was here and loved frosting so it was a great tribute for the night. We had a small table for people to visit, to buy bracelets and magnets and even sold some "Bows for Brooklyn" that were hand made and donated by another friend, Jennifer Rabbitt.



We raised \$867! We are grateful for everyone who joined us and remembered and honored Brooklyn's 1st birthday with us. We look forward to celebrating her in the years to come!

Stephanie Bouchard
Loudon, NH



New Hampshire

Cure it with Comedy

The Cure it with Comedy fundraiser was located at the Radisson Hotel in Manchester, NH on August 2, 2013. While watching the live comedy acts, guests enjoyed food and entered to win one of the exciting raffle items. This fundraiser was a great way to laugh together and have fun listening to four nationally acclaimed comedians--Joe Materese, Jonathan Katz, Tony V, and Paul Nardizzi. Cure it with Comedy fundraiser raised \$4,353! A big thank you to Dominic and Luciane Salce for organizing this event in honor of Constantine Salce, SMA Type II.

NEW MEXICO

5th Annual "Bugaboo" WOD

On August 24th, 2013, the annual "Bugaboo" WOD took place at the Balloon Fiesta Park in Albuquerque, NM. This event was once again a

huge success, raising **\$17,000** for Families of SMA! A special thanks to Laura Hines, Natasha Abbruzzo, and their dedicated volunteers for all their hard work in organizing this event.

NORTHERN CALIFORNIA

11th Annual Northern California Walk-n-Roll

The 11th Annual Northern California Walk-n-Roll, held August 24th, 2013, was an amazing turnout of families and friends supporting FSMA. This year's walk took place in a new location in San Francisco's beautiful Golden Gate Park. The morning started with a slight drizzle and fog but when it was time to walk the sun was shining. The 225 participants gathered at the scenic Pioneer Log Cabin picnic area and walked around Stow Lake. We raised **\$63,000** for Families of SMA research and patient services.

Once the walk concluded everyone enjoyed catching up with each other in a picnic style lunch, balloon artist, face painters, and an incredible raffle that consisted of various themed baskets, gift certificates, sports memorabilia and an iPad.

Thank you to everyone that participated, supported a walker, and donated to make the Northern California Walk-n-Roll such a success! We had many new families at the walk this year and look forward to seeing everyone again in 2014 - if not sooner!

3rd Annual Cure SMA Sacramento Walk-n-Roll



On October 13th, 2013, families, friends, local Key Clubs, and Sacramento Kings Foundation joined together to bring awareness of SMA to the downtown Sacramento community and surrounding areas. This year's walk stepped off from the Capitol steps with over 100 families and friends supporting FSMA and enjoying the day on the Capitol with various food trucks, entertainment from the NBA team mascots, Kings Exhibition game followed by lots of interactive activities for all ages. FSMA was one of 14 charities that partook in this incredible opportunity provided by the Sacramento Kings Foundation.

Nina Daya and her committee put together this amazingly successful event in honor of all FSMA families and friends. Thank you to the generosity of many donors and volunteers over \$13,300 was raised this year!

6th Annual BACFill

On September 14th, 2013, 60 people gathered at California State University, East Bay, in Oakland, CA to participate in the 6th Annual BACFill Crossword Tournament. Thank you to the generosity of the tournament participants, \$1,370 was raised for Families of SMA! We look forward to next year's event.

Andrew Lawrence
Alameda, CA

2nd Annual Barbecue for a Cure

In its 2nd year, Barbecue for a Cure continues to be an annual adventure in music and food in support of Families of SMA that took place on August 10th, 2013 in Roseville, CA at the home of Wayne and Fidela Waggoner. The event was a great success in raising awareness and research funds of \$1,258. The entertainment also added to the excitement and importance of this event because 28 year old Jeremy Waggoner, SMA Type I, and his musician friends, The Yesterday's Fish, played a return concert.

Jeremy has passed away since the event in August. Families of SMA sends our deepest condolences to Jeremy's parents, Wayne and Fidela Waggoner, and family.



OKI

Ohio

2nd Annual Birdies for Blake Golf Outing

The 2nd Annual Birdies for Blake Golf Outing was held on September 14th, 2013, at Green Crest Golf Course in Liberty Township, OH. The fundraiser raised over **\$17,000** for SMA research! A big thank you to Mark Farrell for organizing this golf outing in honor of Blake Farrell, SMA Type II, as well as everyone that came out to enjoy a great time at the golf course!



2nd Annual Aubrey Grace Lyden Memorial Golf Outing

Thank you to the Lyden Family for an excellent golf outing! The 2nd Annual Aubrey Grace Lyden Memorial Golf Outing was held on August 3rd, 2013 in Lake Milton, OH. They had an outstanding turnout with wonderful people! We have a preliminary count for today with a total of **\$15,700**. A special thank you to everyone that donated money and items for the auction. Dannete and Sean Lyden hold their annual golf scramble in memory of their beautiful daughter Aubrey Grace Lyden, SMA Type I.



Pints for Half Pints Fundraiser for Maggie and Charlie

The Pints for Half Pints Fundraiser held in honor of Maggie and Charlie Monnin on October 19th, 2013, in Bellbrook, OH. There were samplings of local home brews, heavy appetizers, raffle and auction prizes, and plenty of college football! The fundraiser ended up raising **\$6,718** for Families of SMA! Thanks to Scott and Nora Monnin for organizing this fundraiser!



US Marshals First Annual 5K Race and 1 Mile Fun Walk

On June 15th, 2013, the US Marshals 5K Race and 1 Mile Fun Walk was held at Cleveland Federal Courthouse in Cleveland, OH! The event raised nearly **\$4,000** in support of Families of SMA! A big thanks to Ryan Helfrich and the US Marshal Service for doing such a tremendous job organizing this 5K race and walk event in honor of Zander and Alexis Helfrich.



US Marshals Annual Golf Outing

Ryan Helfrich organized the US Marshals Annual Golf Outing on September 7th, 2013 at Cherokee Hills Golf Course in Valley City, OH in honor of Zander and Alexis Helfrich. The fundraiser was very successful, raising **\$3,500** for Families of SMA! Thank you to Ryan and the US Marshals Service for organizing this fundraiser!

The Jet Express Put-in-Bay Boat Line Company Ticket Sales Fundraiser

On July 5th, 2013, the The Jet Express Put-in-Bay Boat Line Company Ticket Sales Fundraiser took place in Put-in-Bay, OH. The fundraiser raised over **\$3,000**! Thank you to Todd Blumensaadt for organizing the event again this year in honor of William Blumensaadt, SMA Type I!

Bob Evans Community Fundraiser

The Bob Evans Community Fundraiser took place on August 12th, 2013 in New Philadelphia and Dover, OH. The fundraiser raised over **\$300** for SMA research! Thank you, Dan Newland, for organizing this fundraiser and for your continuous support of Families of SMA!

OKI (cont.)

Bevan & Associates LPA, Inc. Jeans for Charity Program

The employees of Bevan & Associates LPA in Boston Heights, OH began a program at the beginning of 2007 called "Jeans for Charity." Each Friday an employee may wear jeans in exchange for \$1. The funds are collected for the month and then a name is drawn from those who participated in the program. The winner chooses the charity of his or her choice. Families of SMA was chosen by one of our employees on June 19th, 2013. We were able to collect \$176. We wish you continued success with the mission of your organization.

Bracelet Sales and Lemonade Stand in Memory of Mario, Jr.

The Bracelet Sales and Lemonade Stand in Memory of Mario, Jr., SMA type I, was held on September 21st, 2013 in North Olmsted, OH. The lemonade stand turned out to be a hit, bringing in \$90 to help support Families of SMA! A big thanks to Amy Thompson for organizing this bracelet sale and lemonade stand fundraiser.

Kentucky

First Annual Deacon Alexander Memorial Car Show



The Deacon Alexander Memorial Car Show held August 24th, 2013, was a success! Held in Walton, KY, the day included a bake sale, an inflatable for the kids, a mini walk-a-thon, and a makeover booth! The car show was popular and raised over \$3,000 for Families of SMA! Thanks to Amanda Perry for organizing this car show in memory of Deacon Alexander Perry, SMA Type I!



Indiana

2nd Annual OKI Chapter Indiana Walk-n-Roll



The 2nd Annual OKI Chapter Indiana Walk-n-Roll was another success! The event was held on August 24th, 2013 in Indianapolis, IN at Fort Harrison State Park. Friends and family gathered to walk for SMA and enjoy games and the beautiful scenery! Thank you, Michelle Palmer, for another great year in organizing this event! Over **\$21,000** raised to support Families of SMA this year! Thank you to everyone who donated and attended the walk!



6th Annual Cody Munz Memorial Golf Scramble

The Cody Munz Memorial Golf Scramble was held on August 24th, 2013 in Westfield, IN in memory of Cody Munz, SMA Type I. The golf scramble was a huge success, raising over \$2,000 for SMA research!

Thank you to Rhonda and Matt Munz for organizing the scramble again this year!

Muncie 70.3 Ironman

Thank you to Eric Sitzman of Westfield, IN for completing the Muncie 70.3 Ironman in July 2013 and raising almost \$1,500 for Families of SMA in honor of Morgan Sitzman.



Frontier Communications, Fort Wayne Access Design Charity Fund Drive

On July 1st, 2013, the Frontier Communications, Fort Wayne Access Design Charity Fund Drive took place in Fort Wayne, IN. The event was very successful, bringing in over \$500 for research to cure Spinal Muscular Atrophy! A special thanks to Roger Wilhelm for organizing this fundraiser in memory of Cody Munz, SMA Type I! Keep up the great work!

PACIFIC NORTHWEST

Chapter Updates

Stay connected with our chapter and network with other families on our chapter Facebook page, Pacific NW Chapter of FSMA (www.facebook.com/groups/121125384587392/) Contact the chapter at pacwest@fsma.org if you need more information about getting involved with our chapter or organizing an event.

6th Annual Pacific Northwest Walk-n-Roll

On September 29th, 2013 we held our 6th Annual Pacific Northwest Walk-n-Roll at Farm Game Park in Auburn, WA. Despite lots of wind and rain, we had a great day. This year we changed our location in hopes of growing our event and providing a fun family day. The Auburn Game Farm was a great venue with wheelchair accessible paths and a covered picnic shelter. We really needed the shelter from the rain that day! In addition to a new venue this is the first year we had a DJ, games, face painting, vendors and even a food truck at lunch. Thank you to all the sponsors, generous friends and family we were also able to have a raffle for the first time this year. Our fundraising goal was surpassed and we raised almost **\$21,000** for Families of SMA!!

event. I'm so glad for this opportunity to help. I know that every little effort is worth something.

Katie Deaton
Naches, WA



Oregon

15th Annual Wannabe Charity Golf Tournament

On August 15th, 2013, the annual Wannabe Charity Golf Tournament was held in Park City, UT. The event had two days of golf including a first match and then a final match with closing ceremonies at Bandannas Bar & Grill. The event was a success, having a total of \$9,201 donated to help cure SMA. Thanks

to Joe Belcher for organizing this golf tournament in memory of Skylar Bahrenburg, SMA Type I!



Swing Dance Fundraiser

My Swing Dance fundraiser held on November 1st, 2013 in Naches, WA was a huge success! A lot of families were excited to have an event in which the whole family could get involved. I was really surprised that most people had never heard of SMA. Once everyone knew about the disease, they were eager to help and donate to the cause in honor of Peyton Yates, SMA Type III. Overall, I was able to raise \$454 at the

PENNSYLVANIA

5th Annual Zane's Run

The 5th Annual Zane's Run was held on September 29th, 2013 in Malvern, PA and raised almost **\$25,000!** It was a beautiful day and a well attended event. There were several new additions this year that helped make the day successful: new venue, new course routes, and a raffle of over 30 donated items. Zane's Run is organized by Zane's parents, family & friends. The proceeds from the event go towards purchasing the New Hope Car Beds & Dream Ride car seats for newly diagnosed families, international CD's to help those understand SMA living abroad, and new this year, providing medical scholarships to the 2014 SMA Annual Conference.

Although Zane, SMA Type I, only graced us for 5 1/2 months, she is deeply missed and continues to make an impact on many peoples' lives.

Hillary and Keith Schmid
Malvern, PA

Philly Half for SMA

Members of the Philadelphia Families of SMA community organized a half marathon fundraiser in Philadelphia on September 15th, 2013. The Rock 'n Roll Half Marathon gave the Cannady Family a chance to join forces with friends from near and far and Families of SMA to raise funds for research, support, and awareness. In total, 10 participants ran on behalf of the charity team and raised over **\$18,000!** The team was made up of other SMA families and friends of the Cannady Family spanning time and distance all the way back to high school and up to recent life in New York and Philadelphia.

Participants pledged their time in preparing for the race, raising money, and in the friendship shared on a beautiful sunny fall day for the run. The team has already planned to reassemble in coming years for the race each year to continue the fight against SMA for both Emma and Ruby Cannady, SMA Type III, and all children that fight SMA each day.

Steve and Gina Cannady
Jenkintown, PA

**The Lyla Mertz Foundation Fundraisers**

The Lyla Mertz Foundation held five fundraisers to bring almost **\$11,000** to Families of SMA. The 4th Annual Dance Away SMA event took place on September 28th, 2013. The dance had over 140 people enjoying dinner and live

music by the Chas Band. The event also had a Chinese auction. In total, this fundraiser raised over \$5,500

for Families of SMA. The Swing for a Cure Golf Tournament held on August 11th, 2013 at the Mohoning Valley Country Club in Lehigh, PA had approximately 80 golfers that attended. This golf event raised \$4,017. The First Niagara Bank's dress down fundraiser allowed employees to dress down to raise money to help cure SMA. This fundraiser ended up raising a total of \$305. Also, Lyla's Aunt Debbie sewed ten Penn State aprons to sell to benefit FSMA. The Mertz's friend Tim Lupcho sold all ten at a home PSU game and raised \$250 in support of SMA. The Mertz family then sold Team Lyla shirts for an additional \$55 donation. Thank you to the Mertz family for your tireless efforts in organizing such fun and successful fundraisers and for your continuous support for Families of SMA!

Phillies Game & SMA Awareness Night

Fun was had by all on September 8th, 2013 when 200 SMA families and friends came out to support the Phillies and fight SMA! It was a great game! We were well represented with many of our families, as well as our friends from BAYADA Home Health Care. Our chapter video was projected on the larger-than-life phanavision for the entire stadium to meet our kids and learn about the effects of SMA.

Thank you to those that came out for helping us spread awareness to 40,000 people!



PENNSYLVANIA (cont.)

The 10th Annual Lukie's Fall Festival

Ten years ago, we invited some of our family and friends over for dinner. During dinner, we told everyone that we had an idea and needed their help. Our idea was to have a small festival to raise money for Families of SMA, in memory of Lukie. We wanted to raise money, but also wanted to give families in our community the opportunity to have a fun, yet inexpensive family day. We wanted them to come enjoy the festival, have a great time together, and create special memories, while helping us to raise awareness about SMA, and also raising money to help find a cure. We decided that we wanted to have the festival in the fall, because Lukie was with us for the entire fall season. We chose Columbus Day weekend because that is when we received Luke's diagnosis of SMA and it is also when our battle with this disease began. We all decided that Pen Argyl Park would be the perfect venue. Most importantly, we decided what to call the event: Lukie's Fall Festival. Lukie's formal name is Luke, but everyone called him "Lukie" as a nickname. We had come up with a plan, which later that year became the 1st Annual Lukie's Fall Festival!

On Saturday, October 12th, 2013, we celebrated the 10th Annual Lukie's Fall Festival. It was a beautiful, warm and sunny fall day. The festival began with our "10 Years of Hope" opening ceremony. We opened in prayer, shared memories of our Lukie and our family's story of our journey with SMA. We honored and prayed for all of the SMA angels, heroes, their families and a special prayer for Lukie's aunt who is battling breast cancer. We sang "Happy Birthday" and presented a cake for Lukie's mom, Tara who was also celebrating her birthday that day. We were honored to have Sarah Rodriguez, National Development Manager of FSMA. Sarah spoke about SMA and how family events, like ours, help to fund the progress being made towards a treatment and cure. Lukie's grandma surprised us with a special guest, Mayor Sabatine, the mayor of Pen Argyl. Mayor Sabatine presented our family with a proclamation declaring that Saturday October 12th 2013, would be "Lukie's Day" in Pen Argyl. At the end of the ceremony, we shot confetti cannons up into the sky. It looked so beautiful and filled us all with hope as the crowd was showered with white dove-shaped confetti.

The day was filled with lots of entertainment, live bands, a DJ, magician, bagpipers, clowns, mascots, gymnasts and cheerleaders. The moon bounces, photo booth, hayride, mini golf, and carousel were continually filled with kids having a wonderful time. The food stands and bake sale table were busy all day. The aromas of all the wonderful festival foods and snacks filled the air. The annual pie-eating contest resulted in messy faces and many laughs. This year, we had our first Kid's Costume Parade. Many children came dressed in their costumes; we handed out flags and maracas to them. Ronald McDonald led the children through the park; laughing, singing, waving their flags and showing off their costumes. Lukie's pumpkin patch was filled with pumpkins! Kids could come and pick out the perfect pumpkin and decorate it to bring home. The arts and crafts table was busy with little artists creating projects. There was so much to do!

We are so thankful that so many families came out to support our festival with many coming all 10 years. It was so wonderful to see them enjoying the event. Many of them travel quite a distance to come, so we are so honored to have their support.

This year, the 10th Annual Lukie's Fall Festival raised \$9,323. Over the past ten years, we have raised over **\$78,000** for FSMA through Lukie's Fall Festival. Each year, our family and friends work very hard to join together with our local community, to create a beautiful, successful event. Each year, the festival continues to grow beyond our imagination. Families come back year after year to enjoy the entertainment, food, games and to have family-fun while supporting our efforts to help make a difference in the community and to find a cure for SMA in memory of our Lukie.

Thank You,
Joe, Tara, Kadyne & Anna Maida



ROCKY MOUNTAIN



Colorado

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

On September 7th, 2013, more than 400 people gathered at the Clement Park in Littleton, CO for one purpose, to help bring an end to SMA. For 16 years, this event has brought together SMA families from across the region. Whether you participated in the event, gave of your time, donated a silent auction package or made a monetary donation, your support is so greatly appreciated. With almost **\$40,000** raised to help fund research and family support programs, the 2013 event was again a fantastic success! A special thanks to Julie Lino for organizing the event again this year.



Montana

Veronica's Closet

At the end of June, we once again held the "Veronica's Closet" garage sale in Kalispell, Montana in honor of Veronica St Onge, SMA Type I. A lot of people got some real bargains and we raised \$600 for Families of SMA. Thanks to the hard work of Veronica's Aunt Karen Scullion, Uncle Dave, Aunt Jen Crosswhite, and cousins, Tristan and Taylor, the event was a huge success!

Thanks for being there,
Debby St Onge
Malvern, PA

SOUTHERN CALIFORNIA

Reach 4 Sky



On Sunday, October 6, 2013, Team Reach4Sky successfully completed the San Diego to Coronado Sharkfest swim! All 30+ 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 60, 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 400 donors and raising awareness of SMA. So far, we’ve received 385 donations, and have raised **\$41,200** along the way.

On behalf of Team Reach4Sky, 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 Reach4Sky on successfully completing the Sharkfest swim!

1st Annual Long Beach Marathon

The 1st Annual Long Beach Marathon took place on October 13th, 2013 in Long Beach, CA. The marathon raised over \$8,000 for SMA research. Thank you to Rickk and Autumn Montoya for all your hard work in planning this marathon fundraiser! Special thanks to all of our dedicated Team FSMA runners: Chrissy Algarin, Brenda Barberena, Monica Espinosa, Anna Little, Kim Marlow, Michael McGowan, Rickk Montoya, Jonathan Ricotta, Esmeralda Romero, Michelle Slack, Angelina Vaca, Michelle Vaca, Patrick Vaca, Joshua Velchansky, and Samantha Velchansky.



Inaugural Zumbathon for SMA

On August 25th, 2013, the inaugural Zumbathon was held in Menifee, CA. We had an incredible turnout with over 250 dancers, spectators and instructors filling the Marion V. Ashley Community Center in Menifee, CA. Complimentary snacks to keep the energy up were supplied by the local Albertsons and Starbucks. An outstanding raffle of local goods and services assisted in propelling sales and donations over \$7,000... all for the benefit of Families of SMA! But most importantly, the message resonated SMA awareness to our neighbors and full community, as a local newspaper, the Press Enterprise, featured the Zumbathon and wrote extensively on the fight against SMA. We even received the support from the Mayor of Menifee, Mr. Scott Mann, who presented a proclamation which declared August to be SMA Awareness Month in the City of Menifee.

We could not have done this event without the fantastic support of family and friends in our Homeowners Association, “The Oasis.”



SOUTHERN CALIFORNIA (cont.)



Moreover, we were blessed to be assisted by the professional and efficient guidance of our West Coast, Events Manager, Ellen Perry.

It's all for the kids who fight SMA every day. SMA robs them of the simple joys of being young, running on the sand on a warm summer day or simply walking through a field to reach a quiet pond or stream, are the simple dreams we want all SMA afflicted children to realize. Their courage and strength feeds our resolve to hold these events, and raise the necessary funds to support the fantastic research, within in the various FSMA supported projects and programs across the nation.

5th Annual Inland Empire Cure SMA Walk-n-Roll

On November 3rd, 2013 we held our 5th Annual Inland Empire Cure SMA Walk-n-Roll at the beautiful Rancho Jurupa Park in Riverside, CA. This was an incredible new venue for the Walk this year which

brought out over 250 participants. Throughout the day, fun was had by all with an amazing raffle, a cookout-style lunch, live music, a deejay, and a huge area for all the kids activities. The morning started out a little cool and turned into a sunny warm afternoon enjoyed by all our friends and families supporting Families of SMA. Thank you to all the teams that participated and helped raise over \$6,800.

A huge thank you to our sponsors: Bayada Pediatrics, Quest Diagnostics, Fusion of Ideas, Reylenn Construction, LM Promotions, and Ashley Furniture.

See you again next year!

Evelyn Vasquez
Moreno City, CA

HALLOWEEN: 35 years of Terror!

The Halloween franchise celebrated 35 years of screams and terror this past fall. Pasadena, Ca, hosted the 35th Anniversary celebration where the actors, writers and crew were present to speak to guests along with hosting an autograph and photo sessions. Robert Zappia, writer, Halloween H2O, hosted an autograph and photo session throughout the weekend and collected donations in honor of Families of Spinal Muscular Atrophy. Over the course of the weekend, Robert was able to donate over \$325 to Families of SMA! Thank you for your support!

Dave & Buster's Night for SMA

The Dave & Buster's Night for SMA was held on April 16 2013 in Orange, CA. The evening consisted of food, fun, and games as families got together to socialize and help raise money for a cure to SMA. The event raised about \$43 for Families of SMA! Thank you to Autumn and Rick Montoya for your hard work in planning this event.

SOUTH FLORIDA

South Florida Chapter Family Photography Day

The Family Photography Day event was held on August 17th, 2013 in Davie, FL. The event featured a professional photo shoot with Kim Ruoff Photography, in honor of SMA Awareness Month! Kim Ruoff is the organizer of this fundraiser, as she helped raise \$850 for Families of SMA! She has brought this fundraiser to fruition as a way to raise SMA awareness and support all of our local SMA families during the month of August. Thank you to Kim for your continuous support of Families of SMA!



SOUTH FLORIDA (cont.)

3rd Annual Gala of Hope

The 3rd Annual Gala of Hope to Cure SMA was held on November 7th, 2013 at the Ferrari-Maserati of Ft. Lauderdale in Ft. Lauderdale, Florida. The event was held in honor of Mia Israel and Madison Smith, both SMA Type II. Thank you to Mia and Madison's mothers, Fiorenna Israel and Jennifer Miller Smith, for all of the hard work and dedication they have put into planning this phenomenal event every year!

The event featured a hookah lounge, a impressive silent auction and Fund-A-Need program, generous servings of food and desserts, a live band and an aerial champagne artist. After a night of fun, food and dancing the evening wrapped up with a drawing for a \$5,000 gift certificate to J.R. Dunn Jewelers and a Weekend in a Ferrari generously donated by Ferrari-Maserati of Ft. Lauderdale.

Because of Fiorenna and Jennifer's hard work soliciting sponsors, gathering auction items, recruiting donors and attendees, and planning logistics with many, many volunteers, over **\$128,000** was raised to help find a treatment and cure for SMA!



SOUTH JERSEY/DELAWARE (cont.)

Chapter Updates

Happy 2014! Our chapter has had another great year of fundraisers. We are looking forward to an upcoming SMA family get-together in the spring. The chapter is searching for members who are interested in becoming chapter officers or hosting fundraisers. If you are interested or would like more information, please contact Jessica Moyer at atnjmoyer@comcast.net.

Thanks for an amazing 2013!

6th Annual Steven's Swing for a Cure Golf Classic



On September 20th, 2013, we once again hosted Steven's Swing for a Cure at Jonathans Landing Golf Club in Magnolia, DE. We had over 80 golfers and raised over **\$116,000**. We were blessed to have **\$100,000** donated by an anonymous supporter of Families of SMA! Thank you to all that supported this event, including the many sponsors.

Jessica and Jason Moyer
Magnolia, DE

Cocktails for a Cause-Raising Your Awareness Now With Ryan's Buddies

Cocktails for a Cause was held in memory of Ryan James Reilly, SMA Type I, on June 27th, 2013 in Wood Ridge, NJ. Ryan was born on June 19th, 2011, and joined all other SMA angels on March 6th, 2012. Ryan's Buddies, a union of the Rosenbower family and friends, was formed to commemorate Ryan's life, as well as to raise funds and awareness for Families of SMA and other worthy causes.

More than 150 guests enjoyed an evening of cocktails, food, fantastic raffle prizes and dancing. The guests were treated to a wonderful memorial video produced by Ryan's mom, Wendy, and her friend, Jen. This tribute to Ryan's beautiful life included many pictures, as well important information about SMA. Another highlight of the evening was the speech and presentation given by the lovely Sarah Rodriguez, the National Development Manager for Families of SMA, who traveled from Illinois to support the event.



Thanks to the overwhelming and continued support and generosity of family, friends and colleagues, our event was an enormous success.

A total of **\$36,850** was raised from the event. Our family is inspired to continue to raise funds for research and patient care. Please LIKE our Facebook page and follow our efforts at: www.facebook.com/RyansbuddiesNJ.



"Cocktails For A Cause" was sponsored by Chilton Hospital of Pompton Plains, NJ; PromptCare Respiratory of Clark, NJ; Cooper Square Realty, New York City; Rutherford P.B.A Local #300, Rutherford, NJ; Rockland County P.B.A. in New York, and School of Rock in Wayne, NJ. Generous donations were also received from many individuals and businesses from the surrounding communities.

Special thanks to Glenn Rosenbower, Lori Zorat, Wendy Rosenbower, Joan Rosenbower and the many volunteers who made this great event possible!

SMA Golf Tournament in Memory of Steven Moyer

The SMA Golf Tournament was held on July 27th, 2013 in memory of Stephen Moyer, SMA Type I. The event held at Blue Ridge Trail Golf Club in Mountain Top, PA, included a fun day of golf, a buffet, and prizes as families got together to raise money to help cure SMA! The event was a huge success, as it raised \$3,700! A special thanks to Steve Moyer, Stephen's grandfather, for organizing this event!

SOUTH JERSEY/DELAWARE (cont.)

Holiday Fundraiser in Honor of Salvatore

As the holidays approached, our family was reflecting upon another year passing and how fortunate we have been. Our community was hit hard last year with Super Storm Sandy and the devastating fire to Seaside boardwalk this past fall. Many of our friends and family members were affected by these events, but the Morrongiello's were not directly impacted.

Last year, we did not have a fundraiser due to the above circumstances, but felt this year our family and friends were in a better financial position to make a contribution to Families of SMA, no matter how big or small. Our friends and family are always there for us with continued support. We are extremely grateful.

Our son, Salvatore Morrongiello, was diagnosed with SMA Type III while he was in high school. He is now 21 and living a relatively normal life. He will be graduating Montclair State University in May with hopes of a career in the broadcasting field. Like all SMA patients, he has many limitations and struggles daily, but we are truly blessed and thankful that at this point, he is still mobile on his own accord and can get around without any aid. For this, we are a fortunate family.

Over the years, Families of SMA has made such progress with fundraising, awareness and reaching out to so many people, that we felt we were able to provide a festive setting this year to help raise a few dollars to help towards a cure. Those few dollars turned into a little over \$2,100 at our holiday party held in December 2013 at our home in Toms River, NJ. We are so proud to have so many generous people in our lives who wanted to make a contribution to such a worthy cause. With hope in our hearts and progress being made, we believe every little bit goes a long way.

The Morrongiello Family
Toms River, NJ

Christmas Shopping for a Cause

On November 24th, 2013, Caroline Desroches hosted the "Christmas Shopping for a Cause" event in Wilmington, DE to raise money for SMA. The event was a success, raising \$1,423 to support Families of SMA in memory of her son, PJ Desroches, SMA Type I!

The event brought together several families and six different vendors to help support the "Christmas Shopping for a Cause" event. Thank you to the representatives who helped us make this year's event a great success: Lia Sophia, Thirty-One, Scentsy, Arbonne Cosmetics, Dove Chocolate, and Tupperware. Thank you to Caroline and everyone who supported the event through a purchase or donation.



Farleigh Dickenson University Field Hockey Benefit Game



I decided to organize the game held on September 21st, 2013 not only to raise money for SMA, but to raise awareness as well. My cousin Carly Hewitt, SMA Type I, has been my biggest fan for nine years, since I started playing field hockey in middle school. Everyone knew Carly, my teammates and their parents, but no one really knew what SMA was. We sold purple and gold t-shirts that said "Carly's Team" on the front, and HOPE on the back with the wheelchair heart as the "o" at our game in Teaneck, NJ. They sold like wildfire! Everyone wanted them! Even the referees wore them the day of the game. My teammates and I all sported temporary tattoos and wore yellow headbands. Between the t-shirt sale and admissions, we were able to raise \$1,000. The most rewarding parts of the event were Carly being named honorary captain for the day, and being able to share the field with her as my co-captain. We did the pregame coin toss together, which we won.

For the first time ever, Carly got to experience what being on a team is like and the point of view from the field instead of the stands. I will never forget this game, it will go down in history as the best field hockey game I have ever played. Even though we lost the game by one, we won off the field. We did something to make a change and fight for a cure.

Lanie Andrews
Sayreville, NJ

Pampered Chef Fundraiser

On November 13th, 2013, Tanya Drennen, sister-in-law to Jessica Moyer, held a Pampered Chef Party and donated almost \$200 to Families of SMA in memory of Steven Moyer, SMA Type I. Thank you, Tanya!



TENNESSEE

2nd Annual Tennessee Chapter Walk-n-Roll Away SMA

The 2nd Annual Tennessee Chapter Walk-n-Roll Away SMA event was held on August 17th, 2013 at the Ijams Nature Center in Knoxville, TN. Attendees participated in road rides, guided mountain bike rides and walks through the Ijams Greenway. The event also included a lot of activities for the kids such as face painting, a treasure hunt, and an insect zoo! Thanks to the generous donors of this event, we were able to raise **\$10,477** for Families of SMA! Thank you to Sarah Boggess and Denita Guerry for organizing this event!



Seven Months for SMA — In Honor of Alana C. Whited

On August 10th, 2013, the Seven Months for SMA fundraiser culminated with a Candlelight Walk Memorial in memory of Alana Whited, SMA Type I. The walk was in Lebanon, TN, celebrating SMA Awareness Day for all the families who have or are currently suffering the effects of SMA. There was a Candlelight Lap at sundown in memory of Alana and anyone else who has lost a loved one. It was a beautiful ending to the Seven Months for SMA event! The event was a huge success, raising \$5,575! A very special thanks to Brittany Whited, Vickie Whited, and Heather Landers for organizing these fundraisers!



TEXAS

3rd Annual FSMA Texas Bowl-a-Thon



The Texas Chapter hosted their 3rd Annual Bowl-a-Thon August 10th, 2013. Families came from all over Texas to enjoy an afternoon of bowling, balloon art, face painting, silent auction and FUN in Austin, TX! The event, which includes a silent auction, raised almost **\$11,000**. The event concluded with participation in the annual SMA candle lighting remembering those that have lost their battle with SMA and honoring those individuals and families that still live with SMA every day.

Thank you to Kelly Coggin for all of her hard work in organizing this event!

Bella Benefit for SMA

The Bella Benefit for SMA took place October 19th, 2013 in at Mike Lewis Park in Fort Worth, TX. This event is in memory of Addison Bella, SMA Type I. The event raised \$2,950 for SMA research! Thanks to Valerie Trevino for organizing this event.



TEXAS (cont.)

Texas Chapter Families Give Back on Thanksgiving

Thanksgiving Day 2013 was a little brighter for the families and staff at Texas Children's Hospital in Houston on the 3rd floor ICU and 7th floor PICU. Cameron Williams and the William's family helped 150 people at the hospital have a great thanks giving. Thank you to Applebee's, Johnny Carinos, Sheree Norton and the community for their help!



WESTERN NEW YORK

174th Attack Wing Golf

The 174th Attack Wing Golf held October 18th, 2013 raised \$980 in the name of Angelina Facciolo, SMA Type II. Each year, the members of the 174th Attack Wing, located in Mattydale, New York host a golf tournament, The Desert Open, and donate the proceeds to charity. The members of the 174th Attack Wing would like to thank you for the work you do in fighting this disease.

Kelly and Gary Smith
North Syracuse, NY



6th Annual Macy's Shop for a Cause for FSMA

Chapter members sold \$5 savings passes to be used at Macy's stores nationwide on August 24th, 2013 to help raise awareness for SMA and funds for research. Thank you to Diane Blair of Hamburg, NY for coordinating and raising \$385 for Families of SMA!

Casual for a Cause Day

Casual for a Cause Day took place in New York on October 15th, 2013. The fundraiser allowed people to dress casually in order to receive donations to support Families of SMA! They ended up raising \$276 for SMA research! Thank you to Janice Wojtanik for organizing this casual day fundraiser.

Kale Shiesley's Birthday Party

Kale Shiesley's Birthday Party was held on August 16th, 2013 in New York to honor Kale,

SMA Type II. A special thanks to Bonnie Shiesley for organizing this fundraiser and helping raise \$180 to go toward SMA research!

Armor Elementary School Dress Down Day

Armor Elementary School celebrated a Dress Down Day on September 27th, 2013, to support Families of SMA in honor of Alex Blair, SMA Type II! The students were able to dress down for the day by making a donation! The event raised over \$80 for Families of SMA! Thank you to Jennifer Grotke for organizing this fundraiser and supporting Families of SMA.



WESTERN NEW YORK (cont.)

10th Annual WNY SMArt Walk for a Cure

On Saturday, August 3rd, 2013, the 10th Annual SMArt Walk for a Cure was held at Beaver Island State Park in Grand Island, NY. Over 400 friends and family gathered for a 3 mile walk along the Niagara River and returned to a lunch of hot dogs, refreshments and desserts. KISS 98.5 DJ Nick Picholas provided music and commentary throughout the morning activities. Senator George Maziarz, co-chair, announced that Senator Skelos had proclaimed August as FSMA month. Mayor Robert Ortt of North Tonawanda was also on hand to proclaim August 16th as FSMA Day in the city. August 16th is Kale Shiesley's birthday.

While the walk and lunch was taking place, tickets were being sold for the basket raffle, 50/50 drawing, and also for some big ticket items. Over 150 beautiful baskets were donated, along with bikes, signed hockey jerseys, an iPod, athletic gear and much more. Over \$1,400 was donated for the 50/50 drawing. Many thanks go to Lori and Ron Faso and Paula Orłowski for all their hard work on the raffles. Every year it gets better and better.

The WNY Chapter raised over **\$65,000** for the walk! Thank you to all event participants. Through your dedication, you raised SMA awareness and funds that will be used for research and family support programs that will impact the lives of all SMA Families.



WISCONSIN

3rd Annual Kennady's Dream Walk-n-Roll



The 3rd Annual Kennady's Dream Walk-n-Roll was held on October 6th, 2013 in Brookfield, WI. The day included a walk, lunch, a raffle, and youth activities. The walk-n-roll was a success, raising over \$13,400 for Families of SMA in memory of Kennady Quinnell,

SMA Type I! Thank you to Erin and Corey Quinnell for all of the time and dedication they put into this event.

9th Annual Grant Sheppard Memorial Scramble for SMA

On August 23rd, 2013, the 9th Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Chilton, WI. The total raised in Grant's memory for this year came to \$8,100.



As a bonus, the City of Appleton Fire Department joined us as they held their own annual outing. When they learned of our cause, they jumped right in with their enthusiasm and dollars! The cooperation we witnessed at Grant's outing has, again, affirmed our belief in humanity and their giving hearts. So, thank a firefighter if you have the chance. Their efforts, combined with that of the Families of SMA, will lead the way to a cure.

Thank you for all that you do for the families dealing with SMA. Together we will find a cure!

Scott, Lisa, Peter and Lily Sheppard
Sherwood, WI

Drag Queen Bingo FUNdraiser

Barb Verbos, Laura Dolezar and Paula Hoffmann were a few of the 75 participants at the Bingo FUNdraiser November 20th, 2013. The group gathered at Hamburger Mary's, a local chain restaurant in Milwaukee, WI, for a fun night of bingo. The fundraiser was in honor of Jackie Hoffmann, SMA Type II!

Laura Dolezar, Jackie's aunt, organized the very fun evening and helped raise over \$1,400 for Families of SMA. The night was a big success! Many of the participants asked the family to host another night because of how much fun they had. The location at Hamburger Mary's was a success as well, as



they said they would love to have more fundraisers at this location.

Fond du Lac County Department of Social Services Dress Down Days

In August 2013, Fond du Lac County Department of Social Services, did a week long dress down for Families of SMA. Workers could pay \$10 for the week or \$3 for a day and "dress down" in jeans and t-shirts. This is the 2nd year DSS has done this dress down event.



Although August is SMA awareness month, employees of DSS are educated on SMA throughout the year, as Mateo's Grandmother Lorie Gregor, Uncle Alfredo Medina and myself are all employed there and talk, brag and share stories of Mateo and SMA on a daily basis. This year the Department raised \$346. Mateo was born in July 2011 and DSS has been very supportive of me needing time off during his surgeries, hospitalizations, illnesses, lack of nursing coverage, etc. DSS has done a variety of fundraisers for my family during his first year and now continue to support SMA though yearly dress downs for Families of SMA. These dress downs mean a lot to me as it shows my co-workers are willing to donate their money to finding a cure and supporting other families that are facing the tough realization of this disease.

Amy Medina
Fond du Lac, WI

Annual Hoffmann Family Block Party and Raffle

The Hoffmann Family held the Families of SMA Raffle again at this year's 53rd Street Annual Block Party in September. Each year in honor of our daughter Jackie Hoffmann, SMA Type II, we organize a small raffle of donations by selling individual tickets for \$1 or 6 for \$5. It doesn't seem like much but it is so much fun and we do raise money for Families of SMA at the same time. Our items are more crazy fun than valuable but we have about 30 items to raffle off and the kids and

adults look forward to this raffle before dinner every year. This past September, we raised \$330 and also raised awareness of Families of SMA and all their efforts to fund a cure and the support they provide our families by handing out bracelets and literature.

Easton King's 8th Birthday

Thank you to Laurie and Todd King of Boscobel, WI for collecting donations in honor of their son Easton's, SMA Type II, 8th birthday! Thanks to the generosity of family and friends, \$600 was raised for Families of SMA!



5th Annual Par 4 Peyton

In July 2013, Unite 4 Peyton held the 5th Annual Par 4 Peyton Charity Golf tournament to raise money for SMA families at The Broadland's Golf Course in North Prairie, WI. Over the past 5 years, we have been able to help over twenty families living with Spinal Muscular Atrophy. We have granted items from iPads, standers, seating systems, therapy and more. We are committed to helping children with SMA live life to the fullest. We continue to raise funds to help families provide their children with the necessary items they need, but are not covered by insurance. We are so grateful that we have such a great group of supporters year after year. We could never do this without the support from our volunteers, and those who attend our events year after year!! We also believe in the work that Families of SMA does. When Peyton was first diagnosed in August of 2008, we turned to the internet to research what SMA was. We had never heard it, and needed answers. It was a breath of air to find the Families of SMA website, and know that there was a place to turn for help. Peyton will turn 6 in April and we are so blessed for each and every day that we have with her.

Michael and Tina
Reddick, Unite 4 Peyton
Muskego, WI





Kale Shiesley's friends donated to FSMA instead of

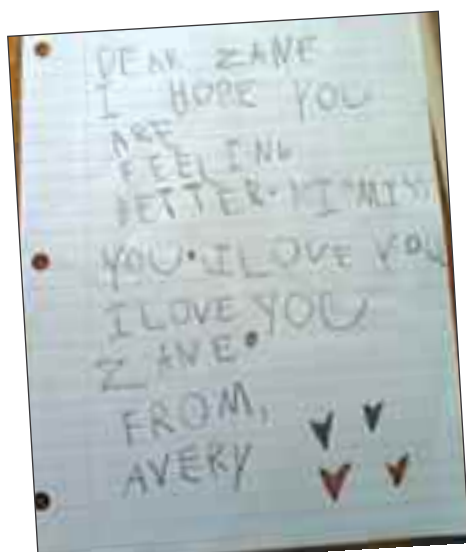
giving gifts. Special thanks to Kale's friends: Bailey, Jackson, Thomas, Samantha, Connor, Michael and Wyatt. They raised \$180.

Avery came to me the other day and said, "Mom, I want to write a letter to Zane. Can you help me spell?"

This is what she wrote. Totally her words, her thoughts.

I wanted this to be included in the newsletter because I feel it shows the sorrow the siblings feel as well. Even though Avery doesn't remember her sister (or maybe she does because of the twin thing), she certainly feels the sorrow.

Thank you,
Hillary of Pennsylvania



The Thompson girls organized a sale of hand made bracelets, SMA bracelets, lemonade and candy bars to make this donation in their brothers memory of Mario Jr.

Lemonade and Cookies Stand in Honor of Lewis Martin

On this very warm day in July, myself and my two sisters and two cousins had a "Lemon-aid and cookies" stand for money to send to you! Sister, Marcella and her new husband Stephen helped us.

My uncle Luke made a wooden stand to look like the old time lemonade stands. Aunt Dawn used real lemons to make the tasty drink, folks came back for refills! Her huge chocolate chip or oatmeal raisin cookies were in their hands to eat later...

We had made posters of information about SMA and people asked questions and learned of the disease. One lady asked

"What is SMA?" and I answered "I have SMA!" My sister Marcella said "He wore his SMA badge proudly"

I have two sisters and a brother in heaven that were also born with SMA. Uncle Luke and Aunt Dawn lost their first born daughter, my cousin Ann Marie, that died at 8 months old. Our family truly hopes deeply for a treatment or cure!! May God lead you on the path to a cure.

Keep on researching! I am waiting...

Lewis A. Martin
8 years old, SMA Type I from Ohio

The Put-in-Bay Elementary 1st Grade School Note Card Sales Fundraiser

We are the First Grade class at Put-in-Bay Elementary School in Put-in-Bay, Ohio. Last year our teacher took pictures of our art projects and made them into note cards. We sold them to raise money for SMA. One of our classmates' brothers died from SMA when he was a baby. We hope the money will find a cure. The sale raised \$523.88.

Hope, Lyla, Ella, Joe, Jordynn, Elora, Emil

Garage Sale in Honor of Joshua Gordon

Our daughters, Rebecca (15 years old) and Emily (12 years old) Gordon, held a garage sale in August of 2013 to sell their old toys, games, dolls, furniture, and other items to raise money and awareness for SMA. Their brother, Joshua, passed away as an infant from SMA Type I back in 1997. While this happened before they were both born, Joshua has always been a part of their lives and so has Families of SMA. Throughout the morning, they were able to let everyone know about SMA and, as a result of the items they sold, are donating \$110.

David and Carolyn Gordon
Rockville, MD

"As the father of 2 daughters with SMA Type 1, I find that they are the ones who show the model of strength! How ironic that their physical strength is impaired, yet they find a great inner strength. They find the happiness and joy in every day, even when they are the ones who have to sacrifice and suffer."

"A community of friends and family
who are always there to support you."

"Supporting my extended family. I recently found out about SMA and run for a beautiful little girl. I work hard everyday to spread the word, build others and my own knowledge and support the cause as much as possible."

ST

"Strength is Living for today! Finding the beauty in every moment! Even when it gets tough my twins continue to smile! Jason and Jacob are the light of our lives and many others! It is not what they can't do but what they can! It's so true that strength comes from places you never knew you had it! Staying positive and crying when needed are also very important! "Our fight matters" Lots of love and light to the entire SMA community!"



“Accepting what we can’t change but facing each day as though anything is possible.”

“Knowing when someone just needs you to listen and hold their hand instead of trying to solve their problem.”

“Not found in your muscles, but in your spirit. It’s pushing back despair to face an SMA diagnosis with courage and hope. It’s smiling in the face of grief. It’s the healing power of laughter. Even when SMA threatens to take everything, it’s believing in a cure and not giving up until that belief is a reality.”

“Strength is smiling when you want to cry.”

“Having the courage and commitment to participate in clinical research on top of all the other doctor’s appointments and therapy sessions these children and families have to endure. I am so honored and privileged to work with these patients and families in this capacity because without them and their dedication and patience, we couldn’t move closer to a cure everyday. Despite being told their research efforts may have no direct benefit on them, so many families still have the strength to help just knowing their efforts may benefit another child down the road. That takes a strong heart!

STRENGTH

reliving some of the hardest moments of my life.”



WHAT DOES **Strength** MEAN TO YOU?

Defining Your Inner Strength

By: Alyssa Silva, SMA Type II, 23 years old

Although not many of us enjoy hearing the blatant truth, sometimes life isn't easy. We often see people finding themselves in less than ideal situations and succumbing to their problems instead of finding the strength to fight. However, strength isn't something that can be found overnight. It also isn't something that just comes and goes. Strength is all about experiences. Cold, painful, raw experiences that you sometimes wish you could permanently erase from your mind. Yet, without those dreadful memories is the absence for growth, and the understanding of what it means to

have strength. Living with a terminal illness has really helped me to grasp what I feel is the true meaning behind this word, and this is what I've learned.

Strength is knowing what suffering feels like and learning to rise above it. It's about being kicked down over and over again and having absolutely no idea how you're going to get yourself back up, but somehow always do. It's about believing in yourself. Believing in yourself so passionately that you're not afraid of facing the situation at hand. Strength is telling yourself that you are good enough, brave enough, and strong enough to overcome that obstacle. Because you can. It's realizing that you are capable of whatever you set your mind to. Strength is persevering.

Strength is about being a fighter. It's about continuing to fight even though you know you're losing the battle. It's about losing, but then coming out stronger. It's the little voice inside of your head, telling you that you always have to be willing to fight, and fight hard. Strength is

having the power and goal-oriented mindset to

chase your dreams. Because, honestly, you'll never know unless you try. It's about not letting your adversities and setbacks hold you down, but rather understanding that you are in control of your own destiny and happiness.

Strength is having hope where hope was once lost and holding onto faith even through the darkest of times. It's about finding the strength to move forward and leave your past behind, but never forgetting how you got to where you are in this moment in time. It's about taking each experience, the good and the bad, and learning a valuable lesson from it. It's about being able to accept the circumstances we cannot humanly change. And, most importantly, strength is about the ability to find the beauty in every little perfect and imperfect aspect of your beautiful life.

How would you define strength?

To view more of Alyssa's work, please visit her blog, "Living with Spinal Muscular Atrophy", at alyssaksilva.com.

