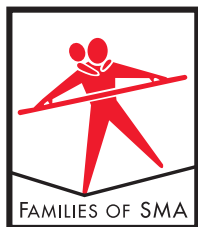




DIRECTIONS

Advancing research. Supporting families.

FAMILIES OF SPINAL MUSCULAR ATROPHY



SMA Treatment Acceleration Act Introduced in U.S. House and Senate

By Spencer Perlman, FSMA Legislative Affairs



Martha Slay, Fight SMA; Senator Debbie Stabenow (D-MI); Kenneth Hobby, FSMA; Cynthia Joyce, SMA Foundation.

For the first time ever, legislation has been introduced in the United States Congress to steer greater federal resources towards finding a treatment for Spinal Muscular Atrophy. The “SMA Treatment Acceleration Act” has been introduced in the House of Representatives by Rep. Patrick Kennedy (D-RI) and Rep. Eric Cantor (R-VA) as H.R. 3334 and companion legislation has been introduced in the Senate by Sen. Debbie Stabenow (D-MI) and Sen. Johnny Isakson (R-GA) as S. 2042. Introduction of these

ills is the result of a collaborative effort by the SMA community; the legislation is supported by Families of SMA, the SMA Foundation, and Fight SMA.

The SMA Treatment Acceleration Act is designed to aid the efforts of the investigators, clinicians, and families who have been striving to find a treatment or cure for SMA by providing federal support to the national non-profit organizations like Families of SMA that are investing substantial private funding into SMA research, clinical trials, and drug development. Passage of this landmark legislation will enable investigators to mount national clinical trials to demonstrate that identified therapeutics are safe and effective for SMA patients.



Zeke Lerner with Hugh Laurie (Dr. Gregory House)

HOUSE.

Cast & Producers Host FSMA Fundraiser

Families of SMA is extremely grateful for the support of HOUSE Executive Producer Garrett Lerner and the entire cast and production crew of the show. In October, Garrett arranged for the inclusion of SMA in an episode of the dark comedy, plus the cast, network and studios held a very special Hollywood fundraiser for FSMA.

This amazing event was sponsored by tv.com and raised over \$70,000 for research into SMA. We thank all of those involved with HOUSE who contributed so generously to our cause.

The event was covered in newspapers from New York to Los Angeles, and was broadcast on numerous TV stations and web sites including Entertainment Tonight, Fox News, and tv.com.

Garrett Lerner, his wife Kim, their son Zeke (SMA II) and daughter Lilly joined with the celebrities to help educate millions about SMA and raise funds for Families of SMA! Thank you.

This has been a fantastic year of fundraising events and research accomplishments. Our thanks go out to all of our chapters and individual families who put so much effort into raising funds for SMA research. 2007 had many highlights for our organization and community. The biggest was certainly the selection of a clinical candidate from the FSMA Quinazoline drug discovery program in June. We are working as fast as possible now to prepare an application to the FDA to begin human clinical trials in 2008.

Our work inside Project Cure SMA has progressed very well. We are now able to run several trials at once inside this leading multi-center clinical network for SMA. Our main goal for the coming year is to expand the network with new locations to allow for easier travel and recruitment into trials.

Legislative activity picked up as the SMA Acceleration Act was introduced into both the House and the Senate this fall. Our joint community-wide efforts with other advocacy groups have been successful in creating interest and momentum with this bill. We now are entering the hard work of building support with as many members as possible for this legislation. Your involvement with local representatives is so important for this effort to succeed.

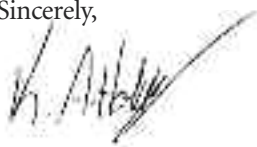
The SMA Drug Summit in September was a truly groundbreaking moment when 13 drug companies came together to hear updates from clinicians and the FDA and NIH on drug development in SMA. This demonstrated the success of our past and current efforts to attract these companies into the SMA field. Company involvement in clinical development will be critical to our future progress.

There are several review articles in this edition of Directions from the 2007 family and researchers conference. However, we are already well into planning for next year. In 2008 we will be holding our annual conference in Boston, MA. We are so excited to have the support of the amazing New England FSMA Chapter. Additionally, we selected Boston as it is home to many of the biotech and pharmaceutical companies now showing interest in SMA. It should be a great location for us all to come together next year.

Watch out for our annual holiday campaign starting soon. This year we are focusing on our efforts and funding of clinical trials.

So, it has definitely been a year of great progress, but every single day continues to matter in our mission to find a treatment and cure for SMA.

Sincerely,



Kenneth Hobby
Executive Director, FSMA



Mission Statement

Families of SMA is the largest international organization dedicated solely to:

- Eradicating spinal muscular atrophy (SMA) by promoting and supporting research
- Helping families cope with SMA through informational programs and support
- Educating the public and professional community about SMA

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. We are a volunteer-driven organization.

Submissions

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

Deadline for the next newsletter is:
Nov. 20th 2007.

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, PO Box 196, Libertyville, IL 60048-0196

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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.

Help FSMA Raise \$300,000 for Stem Cell Research.



Hans Keirstead, anatomy & neurobiology associate professor and co-director of the Sue & Bill Gross Stem Cell Research Center, is joined by representatives from the Dhont Family Foundation. The Foundation gave a \$100,000 grant for the purpose of stem cell research. In addition to the grant, which was facilitated by Families of Spinal Muscular Atrophy, the Dhont Family Foundation has offered a challenge: If FSMA can raise an additional \$100,000 for safety research, the foundation will match the funds.

Excerpted from the Congressional Record, September 12, 2007, p.S11510

Ms. Stabenow: Mr. President, today I am pleased to introduce the SMA Treatment Acceleration Act. I also thank my colleagues, Senators ISAKSON, WARNER, and WHITEHOUSE, for joining me in sponsoring this important legislation.

In April, I met with Malorie Fox, a beautiful 4-year-old from Ada, Michigan, and several other Michigan families about Spinal Muscular Atrophy, SMA, the number one genetic killer of children under 2 years of age. SMA is a degenerative disease that weakens the body's muscles until they can no longer function, that includes the ability to breathe.

Sadly, Malorie was diagnosed with SMA shortly before her first birthday. Her parents were told by her doctors that most children diagnosed with SMA never reach this milestone. Thankfully, Malorie survived, and with her parents Michelle and James, she continues to fight this disease. On her homepage, Malorie wrote: "My mommy & daddy focus on the things I CAN do, not those that I cannot."

The Dhont Family Foundation has already donated \$100,000 to FSMA for stem cell research.

Now Dhont has challenged FSMA members to raise an additional \$100,000 by the end of the year for this work and they will match that amount with an additional \$100,000 making a total of \$300,000!

To participate, donations must be earmarked by indicating "Stem Cell Research" or "Dhont Challenge" in your donation to FSMA.

Thank you to those who have already contributed \$40,000 to our target of \$100,000, and a special thank you to the Dhont Family Foundation for their generosity to FSMA and the SMA community.

Call or email the National Office if you have any questions about this challenge.

SMA Drug Summit

FSMA Joins with Other Advocacy Groups in Sponsoring Summit

On September 28th and 29th a historic meeting of SMA Advocacy Groups, representatives of biotech and pharmaceutical industries, the U.S. Federal Government and SMA clinicians joined together for the The SMA Summit on Drug Development. These stakeholders in the drug discovery and clinical research process discussed infrastructure, regulatory, and community support needs for evaluating treatments for SMA. The goal was to assist in the acceleration of development and approval of these drugs.

Families of SMA was proud to join other members of the International Coordinating Committee for SMA; including: MDA, Fight SMA and the SMA Foundation in sponsoring and organizing this event.

SMA Treatment Acceleration Act cont.

Specifically, the bill authorizes federal funding in order to:

- Upgrade and unify existing SMA clinical trials networks, like Project Cure, and establish a national clinical trials network for SMA;
- Establish a Data Coordinating Center to provide expert assistance and advice to SMA clinical trials sites;
- Expand and intensify federally supported research programs with respect to pre-clinical translational research related to SMA;
- Enhance and provide ongoing support to the existing SMA patient registry at Indiana University, which presently is funded by Families of SMA, in order to provide for expanded research on the epidemiology of SMA;
- Establish an SMA Coordinating Committee, consisting of representatives from relevant government agencies, investigators, clinicians, and affected families and individuals, to coordinate government activities relating to SMA, serve as the principal advisor to agency heads, and conduct a study to identify barriers to the development of drugs for treating SMA and report its findings and legislative recommendations to Congress;
- Establish a cooperative research initiative at the National Institutes of Health to ensure collaboration across multiple Institutes regarding research related to SMA;
- Require the Secretary of Health and Human Services to collaborate with the Food and Drug Administration to make recommendations for improving and expanding existing industry incentives to promote SMA drug development; and

- Establish and implement a program for providing information and education on SMA to health professionals and the general public related to advances in the diagnosis and treatment of SMA and the provision of care to SMA patients.

Families of SMA is encouraging all of its member families to continue contacting their Representatives and Senators to urge them to cosponsor the SMA Treatment Acceleration Act. An active grassroots campaign is crucial to forging a critical mass of congressional support for the bill and moving it through the legislative process towards passage and enactment into law. Legislation rarely moves through Congress without broad demonstrated support from several Members of Congress, and Members of Congress are significantly more willing to cosponsor legislation if their constituents are actively lobbying on the bill's behalf.

You are already making a difference: as a direct result of your calls, emails, and faxes to your Members of Congress in response to the Families of SMA "Call to Action" alert in September, there are now nearly 20 cosponsors of H.R. 3334! However, we hope to increase that number significantly and to spur similar results in the Senate for S. 2042. This can only happen with your help – your Members need to hear from you. The Families of SMA legislative action website,



SMA community members thank Senator Johnny Isakson (R-GA) for his sponsorship of the SMA Treatment Acceleration Act.

www.curesma.org/la_main.shtml, contains instructions for how to identify your Members of Congress, tips for contacting them, and a sample letter and talking points.

We strongly urge you to take the time to participate in this important grassroots effort and to ask your family and friends to join you. Please do not hesitate to contact me at spencer@fsma.org if you have any questions about the bill or contacting your Members. Additionally, please send me a copy of your correspondence so that we can track which Members have been contacted by families. With your assistance, we can build further support for the SMA Treatment Acceleration Act and begin moving the bills through the legislative process.

Join the legislative action committee, email: lac@fsma.org

Date _____

Sample Letter

The Honorable _____
 Member of Congress/United States Senate
 _____ House/Senate Office Building
 Washington, DC 20515 (House) /20510 (Senate)

Dear Senator/Congress(wo)man _____:

As your constituent, I am writing to you today to ask you for your support of H.R. 3334 (or S.2042 for Senators) “The SMA Treatment Acceleration Act”, which has been introduced by Congressman Patrick Kennedy (D-1st-RI) and Congressman Eric Cantor (R-7th-VA) in the U.S. House of Representatives and Senator Debbie Stabenow (D-MI) and Senator Johnny Isakson (R-GA) in the Senate. This Act will go a long way in our efforts to find a cure for Spinal Muscular Atrophy (SMA), the leading genetic killer of children under the age of two.

[If you wish, this is the ideal place to insert a few lines about your personal experience with SMA.]

As you may know, SMA is a hereditary disorder that destroys the nerves controlling voluntary muscle movement. It is the number one genetic killer of children under the age of two. SMA is a relatively common “rare disorder.” It is estimated that SMA occurs in about 1 in every 6,000 births. Approximately 1 in 40 individuals (7.5 million Americans) carry the gene that causes SMA, making it the second most common autosomal recessive genetic disorder. This incidence rate shows neither racial nor gender bias.

Presently, there is no known treatment for SMA, though there have been several exciting research breakthroughs over the past decade. Among more than 600 neurological disorders, SMA has been singled out by the National Institutes of Health as the disease closest to treatment based on scientists’ advanced genetic understanding of the disease.

In order to support the investigators, clinicians, and families who are working to find a treatment or cure for SMA, the SMA community, including Families of SMA, the SMA Foundation, and Fight SMA, has united behind the “The SMA Treatment Acceleration Act.” Specifically, “The SMA Treatment Acceleration Act” would provide for the following:

Federal support for a national clinical trials network for SMA;

Federal support to enhance the existing SMA patient registry and for expanded research on the epidemiology of SMA;

Establishes an SMA Coordinating Committee to include federal agencies, SMA researchers, and SMA families;

Establishes a trans-Institute research collaboration at NIH under the Director of NIH to ensure all relevant Institutes at NIH are contributing and collaborating on SMA research;

Requires the Secretary of the U.S. Department of Health and Human Services (HHS) to study and report to Congress on ways to improve existing incentives, as well as to recommend additional incentives, necessary to promote SMA drug development among private industry;

Lastly, the bill provides for the Secretary of HHS to establish a program to provide information and education on SMA to health professionals and the general public

We are excited about this legislation and to that end; we encourage you to sign on as a Cosponsor to the “The SMA Treatment Acceleration Act.” If you are interested in Cosponsoring or if you have any questions feel free to contact Rachael Bornstein in Congressman Kennedy’s office at (202) 225-4911 or Lindsay Shore in Congressman Cantor’s office at (202) 225-2815/ Oliver Kim in Senator Stabenow’s office at (202) 224-4822 or Tyler Thompson in Senator Isakson’s office at (202) 224-3643.

With your help, there is real hope for finding a cure for SMA. Thank you for your help!

Sincerely,

Name _____

Home Address _____

City, State, Zip _____

Email address (for response) _____

Standard of Care Document Published

The International Standard of Care Committee for Spinal Muscular Atrophy was formed in 2005, with a goal of establishing practice guidelines for clinical care of patients with SMA. The 12 core committee members worked with more than 60 spinal muscular atrophy experts in the field.

This document has been published in the August 2007 supplement of the Journal of Child Neurology.

While it is designed for medical professionals, this document, representing up-to-date insights of an international body of clinical experts, lays out a comprehensive discussion of the many care considerations in SMA, and highlights the critical importance of multidisciplinary approaches for patients' and families' benefit.

The article is available as a free download on the Journal of Child Neurology website.

We offer our thanks to all those who contributed to this project, which was lead by Dr. Ching Wang of the International Coordinating Committee for SMA Clinical Trials (ICC). Special thanks to the Patient Advisory Group of the ICC for supporting this initiative and to the Journal of Child Neurology for making the article available to the public. You can access this article in the "Research" section of the Families of SMA web site.

New Clinical Trials

1. **Controlled Trial of Valproic Acid in Ambulant Adults with Spinal Muscular Atrophy (VALIANT SMA STUDY)**. Location: Ohio State University. The primary objective of this Phase II trial is to assess the efficacy of oral valproic acid (VPA) in adults with SMA.

2. **STOP SMA, Phase I/II Study to Evaluate Effects of Sodium Phenylbutyrate in Pre-symptomatic Infants with Spinal Muscular Atrophy** at the University of Utah. The purpose of this study is to evaluate early treatment intervention in moderating SMA symptoms in pre-symptomatic infants.

If you are interested in being involved in a clinical study or trial, please contact the Indiana Patient Registry.

<http://www.iupui.edu/~medgen/hereditary/sma.html>



Genetics Pamphlet—Now Available

Families of SMA is excited to introduce the first in a series of educational pamphlets about Spinal Muscular Atrophy, The Genetics of SMA. This pamphlet was reviewed and edited by the Families of SMA Medical Advisory Board. It is available for download online or a hard copy can be ordered at no charge from the office.

Families of SMA would like to thank the Angel Baby Foundation. This brochure was produced thanks to their generous donation.



SMA Awareness Month Proclaimed in Michigan, Oklahoma, South Carolina and Cincinnati

Left: South Carolina Proclamation with a picture of Jackson Hedgepeth.

Center: At the Crosstown Roll-out the Cincinnati Mayor's Proclamation was announced.

Top Right: Ian Zurowski with the Michigan Proclamation.

Bottom Right: Brody Henry with the Oklahoma Proclamation.





A Canine Companion for Shea

Shea, an 11-year-old from Centreville, VA, uses a wheelchair due to spinal muscular atrophy. But her friends and passersby notice her for a different reason: her constant companion, a silky black retriever named Mercer. "I'm not known as the kid in a wheelchair," Shea says. "I'm the kid with the really cool dog. That changes a lot for me."

Her disease has left her with limited arm strength and compromised motor skills. So Shea's mother Megan started looking for an assistance dog for her daughter.

Megan says she learned about Canine Companions for Independence (CCI) on the Internet and was attracted to the organization because it was the only one she could find that did not charge up to \$25,000 for a dog.

In fact, CCI provides the dog totally free of charge, including training and ongoing follow-up services to each team for the working life of the dog.

Founded in Santa Rosa, Calif., in 1975, CCI uses golden retrievers, Labrador retrievers or a cross of the two breeds from specially selected breeder dogs. Volunteer breeder caretakers care for CCI breeder dogs and nurture newborn puppies for eight weeks until they are brought to one of CCI's five regional training centers.

From ages eight weeks to 18 months, volunteer puppy raisers care for, provide basic obedience training and socialize the puppies. Professional CCI instructors then teach the dog to master over 40 commands for six to nine months. The canine is then ready to attend Team Training and be matched with an adult or child. Team Training culminates in a joyful graduation ceremony for graduates beginning a new journey and for puppy raisers witnessing their puppies' achievements.

There are 1250 active CCI graduate teams in the USA, consisting of a dog and human partner. Most CCI dogs assist people with various disabilities, working with people in wheelchairs and the hard of hearing. Other CCI dogs work with rehabilitation professionals, helping to improve the physical or emotional health of their patients, or in special education settings.

In addition to helping Shea with physical tasks, Mercer also offers companionship. "He makes her life better," Megan says. "This has absolutely changed her world." She explains that when Mercer accompanies Shea to her doctor's appointments, he lowers her anxiety level considerably.

"When she has to go to (Johns) Hopkins, he lifts the burden just by being with her."

Shea beams when she talks about Mercer. At the mall, he carries her purse and will put his paws on the counter to hand a clerk a credit card. During the school year, he rides the bus with Shea. When they go into their classroom, he lies down under Shea's desk.

"When I drop a pencil or something, I say, 'Get it.' And he retrieves it. If I need something, he's right there. And he knows it instinctively. He gets up and waits for the command."

Shea says she might like to move to Manhattan when she is older because it is an "accessible city" and relatively easy to navigate. She says she might want to become a dentist or a lawyer or have a military job.

But for now, Shea plans to enjoy elementary school with Mercer by her side.

"We're two peas in a pod," she says.

For more information on applying and volunteer opportunities, please visit www.cci.org or contact your local CCI regional office by calling 1-800-572-BARK (1-800-572-2275).



In the spring of 2004, Shea Megale (SMA II) received her "Canine Companions for Independence." Shea's dog, Mercer, helps her with her day-to-day activities—like turning on the lights, picking items off the floor, getting her food card at lunch and giving her more independence!

Mercer also inspired Shea to become an author who has been featured on the Today show, USA Today, and more.

"MARVELOUS MERCER!" is the first book available in the series and there are three others in the works. In Mercer's first adventure, he does all his daily chores for Shea, carries her backpack, picks things up for her, tucks her in at night, and then lastly, turns her light off and settles into his kennel. Mercer has a secret, he and his friends spend their nights thinking about all kinds of crazy ways to help their owners experience many of the things you can, but author 12-year-old Shea Megale cannot.

Congratulations to Shea she is truly an inspiration!

To purchase the book or learn more about Shea visit her website www.walkforshea.com

Most importantly though, proceeds from the book are going to two cool causes, Spinal Muscular Atrophy research, and Canine Companions for Independence.

Mentoring Programs for Adults & Teens

Families of SMA, since its inception, has been committed to supporting individuals and families connected to Spinal Muscular Atrophy in a variety of ways, and has recently decided to expand its support services by utilizing the assistance of several adults successfully living with SMA, despite the limitations and obstacles that typically come along with the disease.

The panel of adults, known as the Adults with SMA Committee, will, as its first task, dedicate their energies to the teenagers and adults who need guidance with “life issues” that tend to emerge in adulthood, providing individuals a place to turn throughout such transitional periods via an official Mentoring Program. Individuals approaching these developmental stages often fade into the background, however FSMA recognizes that it is crucially important for them to have someone to identify with, talk and vent to, relate experiences with, and share a common ground.

Furthermore, the Mentoring Program will allow opportunities for others to network with peers in regards to accessibility issues, employment issues, personal assistance issues and/or concerns, relationship issues, etc.

The Adults with SMA Committee consists of:

Rocco Arizzi
Troy Justesen
Amy Marquez
Melissa Milinovich
Collin Pollock
Rachel Stewart
John (JJ) Wett
Angela Wrigglesworth

Individuals representing various age groups, genders, career specialties, SMA types, and lifestyle choices. They are a wonderful group of people who are approachable at any time of the day and more than eager to lend their assistance. For more information about the Program, to reach members of the Committee, or to request being paired with a mentor, please send all correspondences to:

By U.S. Mail: Families of SMA -
Attn: Mentoring Program
P.O. Box 196
Libertyville, Illinois 60048

By E-Mail: Mentoring@fsma.org

By Phone: (847) 224-9233

Holiday Fundraising

Families of SMA is proof that grassroots efforts can add up to major contributions and the holidays are a great time to implement a smaller giving campaign in your hometown or among friends and family.

1. Letter Writing Campaign. (see page 22). Several years ago, families around the country wrote letters to families and friends which they included in their holiday cards. Those letters (provided by FSMA with return envelopes), helped FSMA raise over \$250,000.

2. Angel trees – The FSMA office has paper angels designed with the letters SMA. Ask a local store to hang the angels from a tree (or on the wall) and customers can take a tag and turn it in at the checkout counter with a donation.

3. Donation cans – The FSMA office will send you a donation can that you display at local businesses. You can personalize the cans with pictures or brochures to encourage participation.

4. Campaigns for specific items – Schools and other community organizations like to “give-back” during the holidays. Tying a campaign to a specific item like a conference scholarship or an adaptive toy helps to both set a fundraising goal and give donors a sense of accomplishment.

5. Gift Card Purchases – Do you purchase gift cards for the holidays? Could you use a gift card to purchase an item for a family member or friend? When you purchase gift cards through FSMA in our “scrip” program, we receive a percentage of your purchase price as a donation to the organization. The dollars really add up.

To share other great fundraising ideas go to the “Fundraising” discussion on the FSMA forums and add your ideas. These do not have to be large events, the smaller contributions we receive collectively become a significant portion of our fundraising success.



SMA Family Camping Trip in Beautiful BC

The 7th annual SMA family camping trip is over for another year, with a change of location this year. It was held on the Vander Wyk dairy farm. The kids had a ball, they rode horses, collected eggs, made bonfires, swam, made rockets, made ice-cream, played board games and cards and scavenger hunts. They also made their own marshmallow shooter guns and had their own war! We also had our regular marshmallow war, something nobody ever wants to miss out on.

Next year the trip will be planned for the second half of July. Both Canadian and U.S. families are welcome. Please contact Susi VanderWyk if you are interested in joining us. Email susi@curesma.ca.

Pen Pals

Did you ever want to connect with another parent who is going through a similar situation? Does your child wish to get to know other kids with SMA? If your answer is yes, FSMA may have the solution—FSMA Pen Pals.

We will highlight various members looking for pen pals in each issue and then you can mail/email us and ask to be their pen pal. We will help connect you together.

For more information email info@fsma.org.

Megan is 14 years old and she has Type II SMA. She lives in England. She enjoys playing on her Nintendo DS and likes motocross bikes, art, animals and everything pink.

Hello, I'm Katharina and I have SMA II to III. I'm fifteen years old and I live in Germany. Five years ago I began to learn English in school and now I want to practice with writing e-mails or letters. I'm searching for a girl or a boy between thirteen and seventeen. I love playing chess, swimming, listen to the radio and playing on the computer. Is anybody interested to write to me?

Hi, my name is Dan Brace. I'm interested in becoming an fsma pen pal. My younger brother, Sam Jantzen, is 13 and has type 2. I also had an older brother that died from type 1 at a few months old. I am unaffected. To tell you a little about myself, I'm 22, and have lived in Cocoa Beach, FL for the past year after moving from Michigan. If their is anyone that would be a good match please give them my name and e-mail address.

*I am 16 years old
I enjoy school (when everything is going ok) I enjoy english, science, multimedia and sometimes maths.
I like to read, watch movies and I enjoy photography.
I think the only thing that is helping me get through each day is having my belief in God.
I would really appreciate speaking to someone who is going through what I am. I think and hope that it will help me accept things a bit better.
thank you
Jacqueline*

My name is Andrea Lavallee, my son Maxime has SMA type II and I would really love to be in touch with another family or parent that is experiencing what I am. Maxime was just diagnosed a month ago today and it would be great to have someone to talk to.

Schaumburg, Illinois

2007 FSMA Family & Professional Conference



For those of you who were able to join us for this year's conference, it was great seeing you. For everyone else, we wanted to take this opportunity to share some highlights of this year's event. One of the most exciting announcements is that at this year's conference over \$1 Million was raised to fund SMA Research.

Plans are now underway for next year's conference in BOSTON, MASSACHUSETTS! So watch out for details.

Amazing Fundraising

How did we reach \$1 Million in just a few short days? It started with a matching donation by the Wyatt Kyle Sutker Foundation, challenging attendees to the conference banquet to raise **\$12,500** in the silent and live auction. The Suters were so moved by the generosity of the community, and the dedication of the researchers, that they increased their donation to **\$20,000**.

The Jacob Isaac Rappoport Foundation announced a **\$100,000** gift with half being dedicated to a new Type I clinical trial planned by Project Cure SMA and half to drug discovery.

Then an anonymous donor contributed **\$250,000** toward stem cell research in honor of Audrey Lewis' years of hard work and service to FSMA.

This was followed by an announcement of a grant and a challenge. The Dhont Family Foundation has given FSMA **\$100,000** to be directed to speed up stem cell research at the University of California, Irvine. The Foundation has promised FSMA an additional **\$100,000** if our community can raise **\$100,000** for stem cell research. Already, the Jacob Isaac Rappoport Foundation has pledged an additional **\$10,000** to this challenge.

If you are interested in contributing to this challenge, please mark your donation with either "Dhont Challenge" or "For Stem Cell Research."

Finally, the weekend was capped off with a **\$500,000** pledge by real estate developer Jon Morrow and his family to fund drug discovery. Jon, an adult with SMA, encouraged the community to continue their efforts and push SMA research ahead.

This is proof once again of the combined strength of Families of SMA. Both large and small donations come together for an ultimate result that is truly amazing!



Some Amazing Kids

The fundraising and awareness work wasn't limited to the adults at this year's conference. A group of enterprising kids decided to make their time in childcare productive and began creating and selling bracelets and paper ties to raise funds for SMA. Their efforts continued all weekend, some kids even giving up their play time at the carnival to man a booth to continue selling. They raised almost **\$200!**

Another group of kids published the first edition of their very own newspaper. They interviewed researchers and other attendees.





**NEWS ANNOUNCED:
CLINICAL CANDIDATE SELECTED
Families of Spinal Muscular
Atrophy Selects First Novel
Clinical Candidate for SMA**

At the conference, FSMA announced the selection of a clinical candidate from the drug discovery program FSMA is conducting at deCODE Chemistry. FSMA will now be funding the studies necessary to apply to the FDA to begin clinical trials.

We're asking about the FSMA Family Conference!
Help us to evaluate and improve the FSMA conference by taking a quick survey. Just visit the FSMA web site and click on the survey link. This will help us in our planning of future conferences.



Thank You to the volunteers from Mario Tricoci who helped the kids at our conference feel like celebrities.

2007 conference summary

www.curesma.org



Special thanks to Audrey, Joe Lewis and the Lewis family for their sponsorship of the pony rides. They were a favorite activity.



Thank you to Wynne Lacey, Jackie Graney and The Chicago Rush Arena Football Adrenaline Rush Dancers for leading a cheerleading clinic at the conference.

Plans are now underway for next years' conference in BOSTON, MASSACHUSETTS! See back page for details



FAMILIES OF SPINAL MUSCULAR ATROPHY

COMPASS

A Publication Dedicated To Research Updates

This issue was produced based on transcripts from the 2007 FSMA Family and Professional Conference in Chicago.

Dear Families and Friends

Hopefully, everyone now should have read about the exciting announcement that we have a clinical candidate for our SMN enhancing drug discovery program. With a lot of work and effort, and making many related molecules to our original hit that came out of the Aurora screen, we have been able to optimize a drug candidate so that it is available in the brain, it stays in the human body for a long enough time, and it has efficacious activity. We are now beginning to embark on the studies that are needed to go into an IND application to the FDA which will allow us to do first in human testing.

So, now that we have heard the punch line to this entire talk, I'll just start at the beginning and give you a more comprehensive overview of the Families of SMA research programs for the last year.

In the last year, Families of SMA funded \$4,000,000 in research. We funded 28 different basic research grants to academic laboratories and universities. We funded two different drug discovery programs: The one that I mentioned above, which are Quinazoline compounds to enhance SMN expression, and the other at Paratek Pharmaceuticals, which is a much earlier stage program to look at Tetracyclines to correct SMN2 splicing defects. We have also been funding the Project Cure SMA Clinical Trials Network. There are about 30 people now in

Project Cure SMA at 7 sites; 5 in the US, 1 in Argentina, and 1 in Montreal. The Project Cure SMA Network has been working on a Phase II trial for the last several years to look at the combined regimen of Valproic Acid and Carnitine in children with Type II and Type III SMA. We heard at a Project Cure SMA meeting this week that 91% of study visits have been completed and the trial will end in November of 2007. We are expecting data from the trial by the time of this conference next year. Also, the Ohio State site, under the direction of Dr. John Kissel, started an adult SMA ambulatory trial. We have also been funding the Indiana SMA Registry to help patient recruitment into clinical trials. This is a registry that all of you can join and then be contacted to enter clinical trials. This registry, which we started 20 years ago, is open for use by all researchers around the world. Now I would like to go into a bit more detail about the three areas of research that Families of SMA funds. For the last couple of years, we have been funding basic research, drug discovery, and clinical testing about at equal levels. Therefore, our research funding is basically divided into thirds, with a third of our money going to each of those areas. The reason we allocate our funds like this is that we feel these three areas are very interconnected and essential to developing SMA therapies. The basic research allows us to understand

how SMA is working in motor neurons and in cells. Understanding this is critical to designing effective drug discovery strategies. The drug discovery strategies then lead to compounds to be tested in humans in clinical trials. So, all of these activities must go on simultaneously to be effective and efficient. In addition, we also fund alternative therapies like stem cells and some gene therapy work.

I now want to talk a little bit on why basic research remains so important to SMA. Even though we have now gotten to the point that we are testing drugs in the clinic, there is still a lot we do not understand about the basic mechanisms of SMA and how the protein SMN works. Understanding these mechanisms is going to be critical to designing new drugs and making them better and more effective. So what we do know is that SMN protein is required for motor neuron function. We know that motor neurons are very specialized cell types. They have cell bodies which are in the spinal cord and they expand very long axons out to the muscles in your body. This can be a very long distance from your spinal cord to your foot say. SMN is found in two places in this cell. First, in the main cell body, and second way down in the axon. We still do not know whether or not the critical function of SMN in SMA is up in this cell body or in the distinct function down in the axon, or it may be both. But basic research is

continued on next page



continued

the path to answering this question. Knowing the function of SMN is critical to help us really focus in and develop drugs in a better way. There are also a number of other questions that basic research can answer, such as how far into SMA disease progression can SMN levels be increased and still have a therapeutic benefit? We do not know very much about how SMN expression or levels are controlled and regulated? Knowing these things, and having a greater understanding about the way that SMN functions, will also allow us to intervene at different points in this pathway. If SMN requires other proteins A, B, and C to do its job, you may be able to do drug discovery projects directly on proteins A, B, and C – and not just focus on SMN. This could really help us move forward.

There are also other questions that we can ask in our basic research such as can we replace SMN protein with gene therapy? Can we directly replace motor neurons with stem cell therapy? All of these questions are really important and they are mostly looked at in the academic labs in the universities. They are funded through our basic research program.

Now I want to return to the subject of drug discovery and drug development and give you an update on where the two drug discovery programs that we fund are in this process. Before I do that, I am going to give you an overview of how drug development works so you have some kind of context to put all this information into.

Drug development is divided into two main steps. One is preclinical drug discovery and the other is clinical development. They are very distinct steps. Preclinical is the stage before you test in humans, and then clinical development is when you begin testing drugs in humans. Getting to this milestone of testing in humans is a huge step in the process. You probably have all heard statistics about this process taking about 10 to 15 years and costing about 1 billion dollars. As SMA is an orphan disease the FDA has different guidelines to help expedite orphan disease drug develop-

ment, so luckily these statistics do not directly apply to our situation.

When we started our first drug discovery program at Aurora BioSciences, which was seven years ago now, we began at the start of this whole process where we knew that there was a target: the SMN2 gene whose expression could potentially be increased to have therapeutic benefit. At Aurora, they developed an assay to see whether SMN2 expression could be increased. They used this assay to test thousands of compounds to look for some that could increase SMN protein levels. At the point where you find compounds that do this, they are called hits. A hit is then shown in cellular models of SMA to increase SMN levels. When you have a compound that does something desirable in a Petri dish it does not mean it can act as a human drug however. A huge amount of effort has to go into turning that hit into a real drug. That process is called lead optimization.

In order to turn a hit into a drug, many relatives of this original hit compound are made - usually several thousand - until you have one that looks good enough to be considered a clinical candidate. When your clinical candidate is selected you then run through a pre-clinical package of safety studies, which takes about 9 to 12 months to complete. When we do pre-clinical safety studies we are looking to see if this compound is safe enough to be tested in human beings. Once you have that data, you can file an application to the FDA, and they will review it and let you know if you can begin testing in humans in phase I clinical trials.

For the Families of SMA Quinazoline collaboration with deCODE chemistry, we have just selected a clinical candidate for the program and are now beginning pre-clinical safety tests. In our second drug discovery program, with Paratek Pharmaceuticals, we are currently working on compounds to correct SMN2 splicing. This is a much earlier stage project where we have a hit that we are beginning to optimize into a drug.

This process of lead optimization, mak-

ing thousands of versions of that compound, is essential because a drug needs to do many things at once. It does not only have to increase SMN levels, but it needs to be present in the blood stream, and in our case, cross the blood-brain barrier. This is something that has to be engineered into our compound. In addition, the compound has to be safe and selective. It can't be toxic. Many compounds will have a few of these characteristics but not all, and that means they are not going to be a human drug. This is what makes drug discovery a long, complex, expensive and high-risk process.

Just to give you a quick update on the status of the Paratek program: Paratek is focusing on a tetracycline compound that can directly correct SMN2 splicing. It causes exon 7 to be included in the SMN protein, so making it fully functional. As I said, this project is in very early stages of lead optimization. We are just starting to understand what parts of the compound are required for activity. Over the next year, we are going to make hundreds of analogs of our hit and look for one that can cross the blood-brain barrier.

Note: More details on our clinical programs will be included in the next edition of Compass which should be available in a few months. Some quick notes from Dr. Sandra Reyna on the current clinical trials we are funding are opposite. Also, to learn more about the status of the Quinazoline project, I encourage you to read the remarks given by Dr. Mark Gurney from deCODE Genetics.

Sincerely,

Jill Jarecki, Ph.D.

Research Director, Families of SMA.

In the spring 2007 issue of Compass was a transcript of a clinical trials chat with Dr. John Kissel. During the chat Dr. Kissel referred to a study of hydroxyurea. The study which he referred to was conducted in Taiwan and was not the study currently underway at Stanford University. The Stanford Study is still blinded and the results entirely unknown.

Quinazoline Project Update

Given by Dr. Mark Gurney at the 2007 FSMA Conference

I am pleased to report that the compound that we have created for you is showing benefit in a genetic model of the disease, the SMA mouse model. Also, the data that is emerging is showing that the compound is quite tolerable in baby mice and also in adult rats. These are the two main things that are needed to now advance towards the clinic: the first is that our compound works and then second, that the compound looks as if it has the potential to be safe. The next step is to show, in fact, that it is safe enough to advance into human clinical trials. That is a fairly straightforward process. The FDA works with drug companies and has clearly articulated what are the safety studies that need to be done.

Those safety studies basically fall into three types. First, we have to show that the compound does not have an effect on the functioning of any organ systems: so that it does not adversely affect the brain, the lungs, or the heart. Second, we need to show that on repeat dosing, the compound has no toxicity, or at least if we dose up, we can show that the window between toxicity and the efficacious dose is wide enough to enable the compound to go in to the clinic. Third, for a pediatric disease such as SMA, we need to show that the compound does not affect either prenatal development, or postnatal development.

So, this is a big hurdle. It was a big hurdle to get this far to show that we could make a compound that was drug like; that had efficacy in the animal model; and that had pharmacokinetics that would support treatment for a disease that affects primarily the brain.

The time required at deCODE to conduct pre-IND studies is generally on the order of 9 months. This data generated from these studies allows us to file the Investigational New Drug application, the IND. At that point in time, we begin conducting preliminary studies

of the compound in healthy adults, and we ask: when we give a single dose of the drug, is it absorbed, does it appear into the blood, and then is there any indication that it is acting via the mechanism of action that we designed? If we find that in the single dose that the compound is behaving properly, then we would do a second study in healthy adult subjects which is called multiple dose tolerability. From that study, we obtain information as to whether the drug accumulates in the body on repeat dosing, and again check that it is exerting the mechanism that we desired.

At that point in time we would have preliminary data on tolerability that allows us to move into actual patient clinical trials. From our experience, this has usually taken about eight months with the first dose in a human, to a completion of Phase I.

So I think from where we are standing now, if everything goes smoothly, and this is not an easy industry to be working in, but if everything goes smoothly, we could see maybe 9 months to an IND and then another 8 months in Phase I that you would need to begin your initial studies in SMA patients.

I think we have come a long way. FSMA has been one of the very first disease foundations to try and undertake drug discovery. You have shown that you can conduct a high throughput screening campaign, that you can identify hits, that you can work with a chemistry company to turn those hits into leads, and now show that those lead compounds have benefited animal models of SMA. So that has been quite a long road, a very difficult one, and now we will see as we progress, do we have a compound that is safe enough to take into human clinical trials, and we should know that on the order of months rather than years!

Current Clinical Trials in Project Cure SMA

By Dr. Sandra Reyna

CARNI-VAL. Project Cure SMA's first randomized, placebo-controlled trial tested valproic acid (VPA) and carnitine in children with SMA type II. When the remaining dozen study visits are completed by Thanksgiving CARNI-VAL will meet its ambitious goal of taking a full 90 participants through the entire trial. So what's the upshot? First, achieving that participation goal is scientifically crucial. It allows us determine the safety and efficacy of VPA in treating the symptoms of SMA. Second, having produced a rigorous study by requiring random assignments of drug and placebo arms in the larger cohort of type II children, we meet the FDA requirements for a well defined study of efficacy. In other words, if analysis of the data shows that VPA does improve the motor function of SMA children, we could move closer to possible FDA approval of this promising treatment.

In mid-November 2007, Project Cure SMA researchers begin the final step in the process -- data collection and entry will be completed and sophisticated biostatistical analysis will take place. Results will be ready by mid-2008. Currently our investigators remain blinded to the drug and placebo assignments, as required. Project Cure SMA owes much to Families of SMA, who funded this trial in its entirety and to Abbott Laboratories, who donated the VPA in the form of study drug and placebo, and to Sigma-Tau

Pharmaceuticals, who donated the carnitine in the form of study drug elixir and placebo.

VALIANT. The preliminary data of the Utah pilot trial on VPA also led to the decision to embark on an adult trial of VPAs. Thus VALIANT was conceived. In late November, 2006, FDA approval was sought for this trial of adults with Type III SMA. To focus the research and give VPA its optimum chance at showing efficacy in adults, only those adults who are still able to walk unaided were included between the ages of 18-60 years.

The Project Cure SMA adult trial is conducted under the direction of John Kissel, M.D., of The Ohio State University. Families of SMA has provided full funding, while Abbott has donated study drug and placebo. Following the design of CARNI-VAL, our adult trial is randomized and placebo-controlled. However, unlike CARNI-VAL, half of all 36 project participants will begin with placebo, half with study drug, and then switch after 6 months. Dr. Kissel received FDA approval for the trial at the end of 2006 and all institutional regulatory requirements have been met, allowing enrollment to begin in mid-summer 2007. To date, 4 participants have been screened and included. Each participant to complete the study will engage in study visits over a 13-month period.

FUNDRAISING stars

www.curesma.org



“Team Skylar’s Fight” had a fun-filled summer, swimming and swinging to fundraise over **\$55,000** and still counting. In late



June, 20 team members, ranging in ages from 15 to 50, braved 58 degree water and swam from Alcatraz Island to the shore as part of the **3rd biannual Skylar’s Swim**. We are pleased that everyone completed successfully and we are already recruiting and training for 2008. In August, 28 aspiring Ryder Cup golfers then participated in The Wannabe Cup (www.wannabecupgolf.com) in Solvang, CA. During the closing ceremonies, Steve Astephen graciously volunteered to donate the profits from a week’s worth of sales from the golf apparel company, Pahr72.com. Whew....what a great summer. We can’t wait to do it again!

From,
Jim and Liz Bahrenburg of Sonoma, CA
In honor of Skylar Bahrenburg



Dear Families of SMA:

Please accept these donations of behalf of my niece Tayler Dyrda. I raised the funds by completing the Goofy Challenge at Walt Disney World in January of this year. I ran half a marathon on Saturday and a full marathon on Sunday. That is 39.3 miles in two days. A challenge yes, but nothing like the challenges Tayler faces every day. Tayler is my running inspiration. She is a spirited 11 year old, who enjoys school and dressing-up. She is a blessing in our lives, as are you. I appreciate all you do to find a cure for SMA. Thank you and God Bless.

Sincerely,
April Novak, from Muscle Shoals, AL

Dear Friends of FSMA,

On Sunday July 15, 2007, the Kropsch and Magnago Family, in honor of their daughter Catarina – SMA Type I, hosted her 1st birthday party and



decided to help the fight against SMA by asking for donations instead of presents. **Catarina’s 1st Birthday Lunch** was a success! She turned 1 year old surrounded by family and friends with a lot of good vibes. As she shares with her Daddy a great affection for all that is under the sea, including the love for the family aquarium and especially for the clown fish, there was no other perfect decoration but “Finding Nemo”!

Lots of colored balloons, Finding Nemo figures and a very special under the sea cake made the party a big hit!! Mommy did a panel with lots of Catarina’s pics and SMA posters for awareness-raising. We were amazed by the extraordinary people who stepped up to be a part of the cure, helping dreams come true! Family and friends enjoyed a lovely lunch and afternoon. Catarina’s 1st Birthday was a great triumph raising over \$2,600 for FSMA!

Kind Regards,
Izabel Kropsch Magnago
From Rio de Janeiro, Brazil



Stevens Walk 2007 was held in May at Newton Lake Park NJ, by Mike, Terri, Steven and Matthew Potter. There were over 200 walkers at the event. There was great live entertainment (Steven's grandfather's band), prizes, wonderful weather, a great playground for everyone to enjoy, and special guests including Hammer from the Riversharks and Vince Papale. The event raised an incredible **\$25,741** for SMA research. They are already looking forward to Steven's Walk 2008!

Mary McHale, Nancy Dindzans, along with family, friends and committee members, held the **7th Annual Concert for a Cure**. This year was a record breaking year! Concert for a Cure raised **\$105,000** for Families of SMA. The attendees enjoyed a wonderful evening at Blackhawk Country Club, featuring music, dinner, and a live/silent auction. To date, Concert for a Cure has raised **\$430,000** for FSMA, in honor of Danny McHale, Ariana Dindzans and Isabella Andrade!

The 8th Annual Bommarito Automotive/Ford Motor Credit & Mazda American Credit Golf Tournament held May 6th 2007 with 232 Golfers enjoying the beautiful day at Whitmoor Country Club for a four person scramble, dinner and auction. The auction displayed jewelry items, a Florida Gulf Condo, baseball tickets and auto-

graphed sports items, which went on a silent and live auction. This SMA event would not take place without the help of many volunteers and SMA families. The event raised **\$90,000** for Families of SMA.



On March 3rd, Mike and Theresa Smith held the **2nd Annual Night to Remember Auction for SMA**, a silent auction fundraiser for FSMA's Ohio Chapter. It was held in memory of their son Logan Michael who died in February of 2005. They had over 150 people in attendance and were able to raise **\$26,000** for Families of SMA. City Barbeque Restaurant helped out with some excellent food and Dan Edwards from their local news WDTN-TV donated his time to emcee for the evening. They had over 100 auction items, including round trip airline tickets for two from AirTran Airways and Southwest Airlines. They also

received **\$5,000** from their local Milton Union Eagles #3621 Aerie and Auxiliary. They already have the next event scheduled for March 1, 2008.

This past June, Rod & Kristi Gellner hosted their annual **Jack Attack on SMA** in West Fargo, ND, in honor of their son Jack Gellner. This year's event, despite the rain, raised almost **\$19,000** to benefit FSMA. Even those wrapped in garbage bags and shielded with umbrellas had a blast!



Family and Friends gathered at the **Rally for Reagan Golf FORE a Cure** on August 19, 2007 at the Oaks Golf Course in Cottage Grove, WI. They had a beautiful day of golf and a fun filled dinner reception with a raffle and silent auction. The event raised an outstanding, **\$16,500** for FSMA, in honor of Reagan Imhoff.



FUNDRAISING stars

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The 5th Annual Nicole Nadeau Golf Tournament was a huge success! The event, held by Jack & Kim Nadeau of Springboro, OH, raised **\$13,150** in honor of their daughter, Nicole Nadeau. This is an amazing event that grows each year.



The 10th Annual Walk for Shea was held in Centreville, VA, on April 29, 2007 with almost 600 people in attendance. The Shea Megale Fund raised **\$10,000**, in honor of Shea, for Families of SMA to continue funding research for a treatment and cure for SMA.



Chris Neeb, of Medfield, MA, participated in the Ironman Triathlon. He had an amazing time of 11 hours, 51 minutes and 22 seconds. He did so with the inspiration of William Johnson, to benefit Families of SMA, and raised over **\$8,000!**



Chris Neeb and William Johnson

Amy Drysdale, of Rochester, MN, held another successful pancake breakfast fundraiser and raised over **\$7,200** for FSMA, in memory of her daughter Lindsey Ronnigen.

On March 31st, **Brandy Baugher and family**, as well as the Star Fund, held the **Crop for a Cure Fundraiser**. The event was held at Parkville Fire Company in Hanover, PA, where 88 women participated in a scrapbooking fun-filled day from 9am – 9pm. “Crop for a Cure” included great food, door prizes and raffles, free make and take classes, on-site vendors and a silent auction as well as plenty of room to scrapbook and have wonderful conversations with family and friends. They raised over **\$6,400** for Families of SMA, in memory of Emmy Rose Baugher.

Several of the Cleveland-areas top chefs gathered on June 18th at **Lockkeeper’s Restaurant** in Valley View, OH for the first **Amelia’s Feast**, in honor of Amelia Wong. Guests dined on an elegant seven course meal, each paired with wines. The event raised **\$5,260** for FSMA.

James Robbins and Janet Morgan, of Baltimore, MD, donated **\$5,050** to Families of SMA by hosting many benefit concerts between December and May, in honor of their son Callum Robbins.



Racheal Luccasen held a SMA Car Wash in Homewood, AL, in memory of her nephew Zachary Luccasen. The event raised over **\$4,800** for FSMA on what would have been Zachary’s 2nd birthday.

Kay and Vince Wittman, of Jerseyville, IL, held the **12th Annual Amy Wittman Golf Classic for Medical Research** on June 16th at the Wolves Crossing Golf Course. This event is the largest golf tournament in Jerseyville and is held in honor of their 21-year-old daughter Amy. The event charges **\$30** per golfer or **\$120** per team and they usually have 50 – 60 teams. They give out prizes to



Amy Wittman Golf Classic

the golfers and hold a raffle and silent auction. With their extremely large family and many friends they are always able to hold a successful and fun event! This year they raised almost **\$4,500** for Families of SMA!



Derek Smith Golf Outing

The 9th Annual Derek W. Smith Memorial Golf Tournament was held on May 18th in Winchester, VA, and was another great success! The golfers were a bit damp, but their spirits were high as they played in the rain. The determined 75 golfers and 20 volunteers brought the tournament to a successful conclusion. The tournament ended with a luncheon and award of trophies. The event, held annually by Delores and Bruce Fry, raised \$4,400 for Families of SMA.



Sotero & Marla Ramirez hosted the Peter's Walk-n-Roll for a Cure, in Monticello, AR, and raised over \$2,300 for FSMA. The Sugartown Riders won first place for the most money raised - \$350. With such a good time had by all, the Ramirez family is thinking about having another walk in the spring!



The Narragansett, RI Lions Club, along with Denny and Diane Denelle and the Denelle family held the Pier Plunge Fundraiser event on January 1, 2007. The event was held in honor of Daryl Denelle. The Pier Plunge raised \$4,000 for Families of SMA.

On June 9, 2007, **Tim Teehan of Alexandria, Virginia**, a good friend of **Rocco Arizzi**, participated in Granny Gear's "24 Hours of Big Bear" mountain bike race. Tim and Rocco solicited friends and family to sponsor the ride to benefit FSMA. The 24-hour-long race was held at Big Bear Lake Campland in Hazelton, West Virginia. Tim participated in the individual division and raised over \$2,700 from sponsors all over the country.

The first annual "Ayden's Swim for a Cure" was held in Phillipsburg, KS on July 8, 2007. Ayden Trammell was diagnosed with SMA Type 1 in December, 2006. Ayden's friends and family organized the event which consisted of a balloon launch and swim party for all children who collected donations for SMA research. The kids did a great job and collected \$2,673 in donations. Bicycles were awarded for 1st and 2nd prizes and Walmart donated a \$50 gift card for 3rd prize. Many businesses in town and Mattel also donated many other prizes. Everyone had a great time and Ayden was there and took his first swim.



My name is Ashley Hendrickson and I live in Carterville, IL.

On June 11, 1998 I was blessed with a beautiful little SMA angel named Brooke Leigh Binning.

In memory and recognition of Brooke and all the other angels I have started making necklaces and bracelets/anklets that are the FSMA colors (red and black).

If you are interested in ordering one for yourself or for friends and family you can do so by emailing me at hendrash@verizon.net.

The necklaces are \$5.00 each + shipping and handling and the bracelets/anklets are \$3.00 each + shipping and handling.

Ashley Hendrickson



Ayden's Swim for a Cure

The staff and students of **Charles A. Bernazzani Elementary School**, in Quincy, MA held a gingerbread decorating night at the school. The students got to decorate their gingerbread men with all sorts of frosting and candy all the while knowing that their donation was going to a very worthy cause. They held the event in honor of their fellow student, 3rd grader Aileen Farrell and raised \$2,356 for Families of SMA.



The students from the **Awareness of Rare Diseases Club**, at Convent of the Sacred Heart in Greenwich, CT chose to raise funds and awareness for FSMA. They held both a Penny War and talent show fundraiser. The combined events raised \$2,200 for FSMA.

Angie and James Damon hosted a golf outing and softball tournament in honor of their daughter, McKenzie. These events in Brooklyn, WI raised over \$2,160 in support of FSMA.

The Delta Phi Chapter of Phi Tau Omega Sorority, in St. Louis, MO, has a main goal to help organizations involving children. The women chose to again support Families of SMA by donating the proceeds from their Trivia Night, totaling \$1,500.

Robert L. Monetti, Custom Builders, of Brielle, NJ held an “**Egg Hunt for Charity**” and asked the attending families to donate to a children’s charitable cause. They raised \$790 for Families of SMA, in honor of Anna Landre.

Through raffle ticket sales and donations made to FSMA, **Sandra Cromer**, of Hanover, PA, raised almost \$550 in memory of her granddaughter, Emmy Rose Baugher.

Tom & Rita Shutes of Lafayette, IN, held their first garage sale to benefit FSMA. The Shutes family raised \$500 in memory of their daughter Audrey Nicole Shutes.

Donna Abraldes, of Holbrook, NY, visited PS 100 school in South Ozone Park, NY to work with the Penny Harvest Team and make some of the care packages that are sent to newly diagnosed type I infants. The students also brought in pennies from home. The roundtable “Penny Masters” students from Danielle DiMare’s 5th grade class chose SMA to share their pennies with. This is the third year that PS 100 has raised \$500 for Families of SMA in memory of Deirdre Abraldes.

The University of North Dakota Bach- elors Club, spearheaded by David Barta, held the first annual Charity Extravaganza. The event was a free concert and charity raffle to raise money on behalf of the Jack Attack on SMA, in honor of Jack Gellner. The event was held on December 3rd at the Christus Rex Lutheran Campus Center in Grand Forks, ND and raised \$466 for FSMA.



The Z-Club Annual Car Show was held on July 15th at Bommarito Nissan in St. Louis, MO. The Z-Club Board Members and Steve Coleworthy, General Sales Manager, helped with the car show. Kathy Goodyear, Janet Hutchinson, Candy Carpenter and Rita Schmidt sold refreshments, which were donated by the Z-Club, to raise funds for Families of SMA. They raised \$420.

Thank you to everyone who participated in **Kennedy’s Chick-Fil-A day!** The Caldwell family raised \$400 for Families of SMA, which was awesome! Chick-Fil-A donated 15% of sales, so they ate an awful lot of chicken and milkshakes!!! The support Kennedy received was just incredible. It really made her feel GREAT!

Wayne & Della Waggoner set up an information booth at Della’s hospital retirement party, in Auburn, CA, in honor of their son Jeremy’s 22nd birthday. They raised \$300 for FSMA and raised awareness about SMA in their community.

Peter Roper of Mystic, CT, sold Cure SMA bracelets, in honor of Marley Robinson, and raised \$145 for FSMA.

Gina Nee, a mortgage consultant in Montrose, MN, recently donated \$80 to FSMA, a portion of her closing compensation, on behalf of her client Ron Silbaugh.

Employees of **Grubb & Ellis Management** of Southfield, MI recently donated the proceeds from an Art Show/Fair. They donated \$50 for Families of SMA, in honor of Elizabeth & Olivia Werstein.

Weddings

David and Jen Antila, of Minneapolis, MN, donated to Families of SMA in honor of their wedding day. They made the donations in honor of Charlie Cowan.

Katie and Joseph Hartley, of Abington, PA made a donation to Families of SMA in honor of their many wedding guests.



Hosting a fundraiser is a wonderful way to raise awareness and funds for SMA.

Whether large or small, this is a great way to get friends and family more involved with fundraising for Families of SMA. We couldn't accomplish our goals to find a treatment and cure for SMA without your help. This year our chapters and individual families did an absolutely outstanding job fundraising! Their accomplishments include:

- Over 30 Walk-N-Rolls.
- Over 20 Golf Tournaments.
- 8 Dinner & Auction Galas.
- 5 Bike Races and Marathons.

Other great events held throughout the year included:

- Swimming Events.
- Community BBQ's.
- Ski-a-Thons.
- Garage Sales.

All these events raised over \$2M in just the last year! We need your help and support in 2008.

Please call us or email if you have ideas or questions and would like to get involved.

Call: FSMA at 800-886-1762

Email: fundraising at fsma.org



Holiday Letter Writing Campaign 2007

Did you know that a majority of our donations come from families and friends?

Did you know that a FSMA letter campaign held in 2003 raised over \$250,000?

This holiday season FSMA needs your help. It is as easy as A, B, C:

- A.** Fill out and mail, or fax the form below or email the information to let us know you want to participate.
- B.** Compile your mailing list and write a brief letter explaining to family, friends, doctors, therapists and co-workers, why FSMA matters to you.*
- C.** Take the packet that FSMA sends you (with the campaign letters and return envelopes) and MAIL!

FSMA will send thank you notes to anyone who donates and notify you of the donations recieved on your behalf.

We hope you will join us in this campaign. Together we will find a cure! Call us at 800-886-1762 or email info@fsma.org with any questions.

*If you need help with a letter, FSMA has examples available – check the web site under the fundraising section or call/email the office and we can send them to you.



Families of SMA Holiday Letter Writing Campaign

**Yes! I want to help,
please send me**

_____ (number of letters)



Full name(print): _____

Address: _____

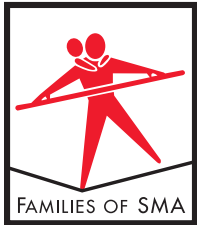
City/State/Zip: _____

Phone: _____ Email: _____

Return form by FAX: 847-367-7623 or Email your information to: info@fsma.org
Mail: FSMA, PO Box 196, Libertyville, IL 60048-0196

This section has been designed to enable you pull it out. It includes membership forms, donation forms, as well as a memorial section.

MEMBERSHIP form



Suggested Annual Donation for Membership

Family	\$30
Professional	\$35
International	\$40

* We ask for a donation for membership to help cover the costs associated with the printing and mailing of our newsletters. You should receive quarterly editions of both the Directions newsletter and Research Compass. We hope that these publications provide valuable information and useful support to all our members.

Payment Method

- Check
- Money Order
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Amount enclosed or to be charged

Credit Card #

Expiration Date

Name on card

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Family Name

Email

Address

City, State, Zip

Home Phone

Work Phone

Mobile Phone

Mother/Wife/Guardian

Father/Husband/Guardian

Relationship to affected person

Affected person name

M / F Date of Birth

SMA Type

Current Status

Date of diagnosis Date of death (if applicable)

Other persons NOT affected by SMA (siblings, children, parents)

Name M / F Date of birth

Name M / F Date of birth

Can we add your name/address to our family contact list? YES NO

Return form to **FSMA Membership**, PO Box 196, Libertyville, IL 60048-0196 or FAX to 847.367.7623

FA07

Donation Form

FA07

I want to make a donation in the amount of \$

In honor of _____

Donor Name _____

Donor Address _____

Notice of donation—Name & Address _____

Payment Method

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Gift Certificate Order Form

FA07

Gift Certificate vendor list available online or by calling 800-886-1762.

Retailer	Qty	\$ Amount	TOTAL
_____	_____	_____	_____
_____	_____	_____	_____
_____	_____	_____	_____

Payment Method

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Expiration Date _____

Name on card _____

Signature _____

Ship order to

Phone _____

Shipping Up to \$499 **-\$8** • Over \$500 **-\$10**

Subtotal \$ _____

Shipping \$ _____

Total \$ _____

Return form to **FSMA Gift Certificates**, PO Box 196, Libertyville, IL 60048-0196 or FAX to 847.367.7623

Merchandise Order Form

FA07

Item	Size	Qty	Description	\$ Amount	TOTAL
_____	_____	_____	_____	_____	_____
_____	_____	_____	_____	_____	_____
_____	_____	_____	_____	_____	_____

Payment Method

- Check Money Order
 VISA Mastercard Discover

Credit Card # _____

Expiration Date _____

Name on card _____

Signature _____

Ship order to

Phone _____

Shipping Up to \$25 **-\$4** • \$26 to \$50 **-\$7** • \$51 to \$75 **-\$10** • Over \$76 **-\$13**

Subtotal \$ _____

Shipping \$ _____

Total \$ _____

Return form to **FSMA Merchandise**, PO Box 196, Libertyville, IL 60048-0196 or FAX to 847.367.7623

Mia Garza a bright, beautiful and lovely daughter past away on 4/14/07. She brought us joy and happiness we would of never known without her in our life. She made us grow and learn to love without conditions.

We are so blessed to have such a great gift be placed in our life. Thank you FSMA for your continued research and support.

May the cure be found soon.



IN MEMORIAM
MAKENZIE MITCHELL



ETHAN CHITKARA
6/19/06 - 10/02/07

NATHAN P. LIGGINS
12/30/05 - 8/30/06



To the Families of SMA,

This letter is in loving memory of Nathan P. Liggins. Our son past away August 30th, 2006. He was only 8 months old and was diagnosed with Type I SMA.

It has been extremely difficult to accept, grieve and cope with. Our family and close friends have truly helped us get by. And at the same time, very thankful that our 4 year old daughter is healthy and SMA free.

We had raised money in November through the Walk-N-Roll in Burbank, CA and now I humbly send a donation on behalf of my coworkers and students. The story behind it is sweet. My coworker and close friend, Mr. Miranda and along with Mr. Jordon and others, are both teachers at the intermediate school where I teach. They asked for my permission to inform the students about Nathan and what happened to my family. They had a fundraiser and in only one month they raised \$300. – All in Nathan's memory! I cannot thank them enough for wanting to help other children stricken with SMA and help find a cure. In addition, to celebrate our daughter Katelyns 4th birthday, we held a party and decided to donate all of her monetary gifts to you in honor of Nathan.

We love and miss him so much!

Sincerely,

Silvia and Paul Liggins, West Covina, CA

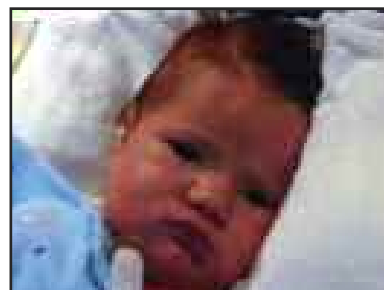


ASHLEY BROOKE PAITE
2/8/07 - 4/8/07



In Loving Memory
BROOKE LEIGH BINNING
6/11/98 - 12/18/02

JACKSON HEDGEPEETH
1/5/07 - 5/15/07



There will never be enough words to thank you for all of the support to my family. Families of SMA is the greatest! We knew that every question, concern, problem, whatever came up, would be answered with one phone call to you. Our J-Man is free now but we will continue to fight this horrible thing and look forward to finding the cure very soon!

Thank you for everything!

Nichole and Joseph Hedgepeth,
Proud parents of Jackson Cole

FSMA merchandise

www.curesma.org



FSMA Holiday Ornament | \$4



Snowman or Angels of Hope Cards | \$8

Set of 8 cards. Specially created for FSMA by Silvia Heller.

Holiday Note Cards | \$5

Alyssa Silva's "Working on Walking" note cards for the holidays. 5 different cards.



12 Fruit Notecards | \$6

4 each of 3 designs (12 cards). Artwork by Holly Campbell, in memory of nephew Charlie, SMA Type I.



License Plate Frame | \$7



Angel Wing Pins | \$10



SMA Awareness Pin | \$15

One design in sterling silver.



12 Window boxes Notecards | \$5

4 each of 3 designs (12 cards).



FSMA Lapel Pin | \$5

TOTES



Canvas Tote | \$15ea.

Choose either Flower or Seascape design. Artwork by Katie Gardner 11/89-4/03.



Cotton Tote bag with Zipper | \$17

Together design.



Canvas Tote | \$10

CureSMA design.



FSMA Car Magnet | \$5

1 design.

FSMA merchandise



12 Charlie Notecards | \$10

Artwork by Merrle Peterson, in memory of son Charlie, SMA Type I.

Cookbook for a Cure | \$14

The FSMA cookbook contains almost 400 recipes from SMA families all over the world!

Spring Note Cards | \$6

Alyssa Silva's "Working on Walking" note cards. 6 different cards.

Cure SMA Bracelet | \$2

Available in small or large. Created in honor of Steven Potter.

Donation Gift Card \$10ea. or 5 for \$40

Have you ever needed a gift for a teacher or a birthday gift for a "hard to buy for" person? These elegant cards are a perfect gift for when you want to thank someone for their kindness, honor someone who makes a difference in your life, or mark a celebration. Instead of buying teachers, doctors and anyone else another scarf or trinket, consider giving a FSMA gift card.



FSMA Ladies Vest | \$30

Blue sizes: XS M L XL
Black sizes: S M

FSMA "Moving Forward" T-Shirt | \$12

Sizes: S M L XL



Wipe Out SMA | \$12

1 design on short sleeve shirt.
Kid sizes: 2/4 6/8 10/12 14/16
Adult sizes: S M L XL



Cure SMA T-Shirt | \$12

1 design on short sleeve red or white shirt.
Kid sizes: 2/4 6/8 10/12 14/16
Adult sizes: S M L XL

"Together" T-Shirt | \$15 (short sleeve) \$18 (long sleeve)

1 design on navy shirt. Kid sizes: 2/4 6/8 10/12 14/16
Adult sizes: S L XL.
Long Sleeve, Kids sizes only: 2/4 6/8 10/12 14/16

Mail your Orders to
FSMA National Office
or fax to 847-367-7623
Order form is on Page 24

THE INTERNATIONAL
**SPINAL MUSCULAR ATROPHY
PATIENT REGISTRY**



...**uniting families**
with SMA...



...with the **researchers
& doctors** studying SMA...



...giving **tomorrow a
fighting chance.**

<http://smaregistry.iu.edu>

Participants

Visit the site for more information or to learn how you can join the registry.

Researchers

Visit the site to learn how the registry can help connect you to the people and data your research needs.

The International SMA Patient Registry is maintained through the Department of Medical and Molecular Genetics at Indiana University School of Medicine.

Alabama Chapter (since 2005)

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 Birmingham, AL 35243
 Phone: 205-979-6493
 Tonya Willingham, President—
 alabama@fsma.org

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 Karey Kaler, President—
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Chesapeake Chapter (since 1995)

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 Tel: (608) 223-9408
 Marta Meyers, President—
 wisconsin@fsma.org



The Krajewski Family



Harley, Amy and Danielle Marquez



The St. Onge & Shofner families at the Illinois Walk



Jungin Angie Lee



Chloe's Crew

Illinois Chapter

Families of SMA Illinois Chapter 1st Annual Walk-N-Roll

Sunday, June 24, 2007 in Schaumburg, Illinois at the Hyatt Regency Woodfield

The Families of SMA Illinois Chapter was honored to host its 1st Annual Walk-N-Roll on June 24, 2007 at the Hyatt Regency Woodfield in Schaumburg, a gathering that offered a wonderful conclusion to the FSMA Family and Professional Conference. The weather was most definitely in support of the event, and over 200 individuals came out to participate and join in on the festivities throughout the day. Many thanks go out to the generous sponsors and donors who helped make the Walk-N-Roll a tremendous success, including Tito Renghini from Good Tymz Entertainment, "Mr. Pick It" from Harris Kal Productions, Dave Stritter from Magic Dave Productions, Walk Disney World, Woodstock Harley Davidson, Starbucks Coffee, Einstein Bagels, Dunkin' Donuts, Tree Top Juice, Sam's Club, Carabassa Restaurant, Baby Einstein, Comcast, Omnicare Pharmacy, the Spina Family, Brian Schoenborn, Tina Krajewski, Sandi Potempa, and the Moms' Club of Chicago! As a direct result of the efforts and support put forth by all involved, the Illinois Chapter Walk-N-Roll has raised \$33,000 to date, with donations still rolling in. Thank you to all who have helped make this event an amazing experience and a huge success!



Samuel & Janet Schoenborn

"Silhouette Images Against SMA" Portrait Party—Update

Held on Saturday, March 10, 2007 in Lake Forest, Illinois

The portrait party & fundraiser "Silhouette Images Against SMA" was hosted by Carolyn Eichelman, Illinois Chapter Board Member, in her home, in honor of Samuel Schoenborn. The event took place on Saturday, March 10, 2007 between the hours of 10:00 AM & 5:00 PM, and raised \$1,500 to aid research and help put a stop to Spinal Muscular Atrophy. Many thanks go to Carolyn and all who assisted with and participated in this event, and congratulations on a job well done!

Shop for a Cure Update...Festival to Benefit SMA

Sunday, March 11, 2007 in Libertyville, Illinois

Sherri Kullander & family hosted the 9th Annual Fundraiser for Spinal Muscular Atrophy on March 11, 2007 in Libertyville, Illinois, which consisted of a shopping extravaganza and an afternoon packed with fun events. Guests were able to purchase favorite items from vendors such as Longaberger®, Pampered Chef®, Partylite®, Tupperware®, and Tastefully Simple®, to name a few, in addition to trying their luck at the raffle bursting with an array of items. Once again, this event proved to be a huge success, raising nearly \$5,300 for SMA research! Sherri and family, as always, thank you for all your effort and continual support of Families of SMA.



OKI Chapter

Walk-n-Roll Kentucky 2007

August 11th, over 125 people took part in the first annual Kentucky FSMA walk n roll at Keeneland Racetrack. The walk route louped through horse barns and out through Keeneland grounds. Entertainment included facepainters, a clown, live band and a raffle. The raffle was m'ceed by local celebrity Dave Baker. To date, over \$6500 has been raised for FSMA. Thanks go out to the KY FSMA committee - Connie Allen, Kim Bucci, Amy Craiglow, John Cropp, Michelle Schlafer, Sarah Snider and Sandee Woodworth and also to the Lockwoods and Linda Schwab for all their help in making the first KY FSMA walk a success.

The FSMA Annual Garage Sale took place June 15th and 16th at the home of Rick and Shelly Uhlenbrock in New-

town. This is the 4th year for this garage sale in honor of Emma and Nicholas Lockwood, the Uhlenbrock's niece and nephew who have SMA Type 1. They, like many others want to do what they can to help find a cure for this disease. Lots of families and their friends donated items for the sale helping to raise over \$5,000 for research. Just wanted to mention their grand total for the 4 years is over \$11,800...that is very impressive and a big help in finding a cure! A BIG THANKS to Shelly, Rick, and their family and friends for all their hard work!



Cross Town Rollout

Louisiana Chapter



Kansas City Chapter





Colorado Chapter Walk & Roll

Iowa Chapter

The Iowa walk-n-roll (at the school) was in honor of Elijah Brock.



Western NY Chapter

The weather was perfect for the Western NY Chapter walk. About 400 participants had an amazing day. We raised over \$50,000!

Many thanks to Tammy from Strip-tees for replacing 1/2 of our shirt order at the last minute because UPS damaged it. She certainly saved the day.



New England Chapter



Nairi's Walk for SMA in New Jersey!

Pennsylvania Chapter



It has been a year of change for our Chapter. It is with extreme gratitude that we thank our founding Treasurer, Connie Smith, for all that she has done to launch our Chapter. Due to expanding career responsibilities, Connie has decided to step down from her post.

We are proud to welcome Josephine Tripodi, grandmother to Jake Saxton, as our new Chapter Treasurer! She has hit the ground running, and with our 4th Annual Walk-n-Roll only weeks away...we could not be more appreciative!

We are also excited to announce the addition of two more Chapter Officers! With change, there is always great potential for growth. Allyson Henkel, mom to Peter, has worked hard to fortify our Corporate Sponsorship base. Allyson has agreed to lead as our Corporate Sponsor Chair.

In an effort to maximize our family outreach, Paula Saxton, mom to Jake, has stepped up as our Membership Chair. She is also working to promote awareness through the media in the Philadelphia region.



The Saxton Family

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

Please contact our Chapter Coordinator, Barb Trainor at fsma-chesapeake@comcast.net

Morgan Kelly is a Fighter

By Angela Floyd

My name is Angela Floyd and I am Morgan Kelly's Mother. My daughter Morgan is a Fighter! I have taught her well!

About 25 months ago we started the process for a power chair for Morgan. Never did I imagine that I would have to pull an army of people together to help me help Morgan get the chair she so desperately needed.

I found myself contacting anyone and everyone that would listen.

With the help of Craig Kraft - seating director, Suzanne Weiss - speech /language pathologist Arleen Sands - Physical Therapist, Mike Bobala - Seating specialist, Dr. Cara Novick - Orthopedics, The Shiriners Orthopedic Children's Hospital in Tampa FL, Douglas Towne and Robert Figueroa - of the Disability's Realties Group, Bruce Bays - President and CEO of Custom Mobility, Inc., Lyndie Gordon - Senior Attorney for the Advocacy Center for Disabled People, Governor Charlie Crist, Congressman Adam Putnam, State Senator Paula Dockery, State Representative Seth Mckee, Ollie Cantos - Associate Director for Domestic Policy at the White House for his Strength and support...Last but not least myself.

As you can see quite the army. And with everyone's help the day finally came!

This is a wonderful day indeed, not just because it represents the next chapter in Morgan's life, but because it symbolizes the kind of independence that is truly possible when children are provided with the tools and equipment that turn the dream of empowerment into a definable and concrete reality. This has been a long journey, but the value of the destination has most certainly been worth the effort. Thanks to the help and



Morgan in her New Power chair. She previously had a manual chair.

support given by everyone that I contacted whether in direct ways or in spirit, my beautiful little girl is better able to be just like any other vibrant and energetic nine year old. She moves about at will, just like other kids, and, as every other child who has gone before her, she now even has the ability to drive her parent's crazy by not staying in one place for very long.

There are, of course, other children out there who are just as deserving of assisted technology as Morgan. Like her, they too have their own hopes and dreams, goals and aspirations, like her and as every parent and guardian will rightfully assert, they deserve freedom too. And so I take this priceless opportunity today to call upon officials in government to take their rightful place of leadership by finding ways to increase their support for the independence of this states children with disability's. I also call upon non- profit

organizations to work in partnership with parents and other organized groups, to enhance service delivery for those who simply seek to have a chance to succeed in this world.

I also reach out in partnership to members of the business community to work in close collaboration with the disability community to do their part to make things better.

In thinking about what I could say at the end of my story that would be inspirational, I do admit to having a short but temporary period of writers block. What could I possibly say to be of inspiration to you?

And then, I realized how in striving for inspiration, words were completely unnecessary, because the only thing that any of us needs to do for inspiration is to look to kids like Morgan and others who simply want to grow up to be happy, healthy, and productive citizens, just like everyone else .

We need not pity them. We instead just need to give them a hand up to enable to take on their own fundamental sense of personal responsibility to make their way in the world and to give to the world in the same way that others without disability give. In so doing, they inspire us. And, as we learn from their example and do our part to make a difference, we in like manner may inspire them.

Thank you for reading my thoughts and story on how we fought the government and WON! She is my little hero. Everyone just KEEP fighting .

Angela Floyd

My Story

By Hannah Soyer

When my mom told me I would be attending this year's Kids Talk It Out session (ages 10 and older), I'll admit I was a trifle nervous. In my mind, discussing your affliction with other kids dealing with the same thing seemed awkward. Adults would say that it would give us a chance to give each other advice and know you're not alone, but truthfully, or so I thought before the session, what is there to discuss? I mean, the other kids there know what you're going through, but it's just a fact of life. I had SMA and the sooner I learned to accept that the better. That was probably the one and only thing I had right.

On the morning of the session, I rolled into the room where we would be gathering, and was introduced to Dr. Al Freedman, a psychologist from West Chester, Pennsylvania. He would be leading the session alongside Ms. Angela Wrigglesworth, a third grade teacher from Houston, Texas, who was diagnosed with SMA Type II.

I was determined to talk as little as need be so as not to draw attention to me. However, as everyone formed a circle and the introductions began, I began to ease.

The first question we discussed was "What are some disadvantages of having SMA?" We went around the circle, many kids saying: "people staring", "watching friends do stuff, such as running or jumping, that we can't do", "sometimes places we go aren't accessible for our wheelchairs", and, sadly, "I sometimes feel like a burden to my family when they take me out because it's so much work for them to take me". I finally worked up enough courage to contribute by saying that I hated it when I would be with my friend and someone would come up to us and say "What's her name?" (meaning me), to my friend instead of me. We talked

about what we did when these situations occurred and how to deal with these things.

Dr. Al then asked us what were some advantages to having SMA? We all agreed that getting to cut in line, getting in free to certain places, driving fast (of course), and having the opportunity to meet a lot of great people were definitely some.

I think the question that made me think the most was "If you could have one day where your wheelchair wasn't needed, what would you do?" What would you do, Hannah? I caught myself thinking. Many ideas popped into my head such as travel to Europe, go sailing, or spend a normal day walking and see how people react. I pictured each of these experiences in my mind, savoring every one. And yet, and yet...something was definitely missing. What was it? My chair? The usual struggle I had learned to turn into an adventure? The stuff that made up me?

I finally decided I would go to my dance studio, where I take wheelchair dancing, and participate in all the other classes. Something would still be missing, but at least I would be doing something I couldn't do otherwise.

Other kids said: "I'd climb a tree", "I'd play hopscotch", "I'd sneak out of my bed and play when my mom and dad were sleeping", "I'd do lots of things with my friends and I'd feel normal", "I'd run around and be happy all day", "I would like to bungee jump off a 300 foot bridge over a river", and "I'd walk around all day at the mall and go shopping."

By now, our session was drawing to a close. "One more thing before you leave," Dr. Al said. "Let's all go around and say one wish that we have." We took a minute to ponder this. I could feel all the wishes suspended in mid-air, shim-

mering like a thousand angel wings:

"I wish I didn't have SMA."

"I wish we could find a cure for SMA."

"I wish everyone with SMA could walk."

"I wish we could all be accepted and not judged."

"I wish I could run and jump."

"I wish more people would know about SMA."

"There was no such thing as SMA."

I myself had to pass when it came to me. All the things mentioned were things I wished for also. Weren't they? Yes. Obviously. But still. As the session ended and we gathered around for a group picture, I wondered. When you got down to the truth, no, the very core of the truth, I would feel incomplete without my disability.

A cure for SMA would be miraculous. Many lives would be saved and tears would be dried. However, I can't help but feel...lucky to have experienced the opportunities I have in my twelve years of life.

As much as I felt nervous about attending the Kids Talk It Out session, I was so glad that I did. I had met new friends, acquired new ideas, and learned such an abundance of things it is hard to put into words.

"Bye, thanks for coming!" Ms. Angela and Dr. Al said as I followed everyone out the door. "I'm so glad you came!" I paused and smiled before venturing out into the chaotic entrance-way of the hotel. "Thanks," I said to each of them. I meant it.

Hannah Soyer is twelve years old and is affected with SMA Type II.

She lives near Des Moines, Iowa, with her mom, dad, older brother, and cat.



Julie & Alex Marshall

2nd grader John Rice raised \$5 for FSMA in honor of his friend and fellow Boy Scout Alex Marshall.



Lindsay May, of Quincy, IL, held a lemonade stand this summer, where she sold lemonade and made cookies. She raised \$30 in

memory of her brother Christian May.

Katelyn Liggins, of West Covina, CA, donated all of her 4th birthday money to Families of SMA in memory of her brother Nathan.

For Veronica St. Onge's 3rd birthday, Debbie and Robert St. Onge, of Bigfork, MT, asked their friends and family to give donations to Families of SMA in lieu of birthday gifts. Thanks to the generosity of Veronica's Dads company, they were able to raise \$730 for FSMA.



For Tommy Testa's 6th birthday party, The Testa family, of Boardman, OH, asked The Ohio 501st Garrison to attend. This organization's members are Star Wars enthusiasts who have authentic looking Star Wars costumes. They collected donations from friends and family. They raised \$220 for Families of SMA! It was a big hit for Tommy and his friends, while they helped to raise money for a cure.



New Washington Middle-High School leaders of America with Jenica Miller

Family and Consumer Science Teacher, Juanita Oakley's New Washington Middle/High School Chapter of Family, Career, and Community Leaders of America made raising awareness of Spinal Muscular Atrophy one of their service projects for this year. They made a bulletin board about SMA and conducted a fundraiser for SMA research in honor of Jenica Miller, SMA Type II. The fundraiser included hosting a school wide hat day and collecting donations at the school and from the community. The students raised \$623 for FSMA.

8-year-old Philip Meneghini, of Havenhill, MA, raised \$1,000 for Families of SMA, by running the Bradford Valentine Race. He wanted "to do something" for his little sister Victoria, who passed away in 2003.

The Greenfield Elementary School, in Greenfield Center, NY, held a student council Walk-a-Thon. They raised \$120 for Families of SMA.

Grant Stoner, Allie Yurkovich and Cameron Ruby, of Jefferson Hills, PA, held a lemonade stand to raise funds for Families of SMA. They raised \$50 at their sale!

At Lexus Stipanovich's 9th birthday party in May, she asked her friends and family to donate to Families of SMA instead of buying her birthday gifts. She raised \$585 for FSMA in memory of her brother Myles.

Delaine Fuller, of Saginaw, TX, raised \$275 by having a lemonade stand in honor of her best friend Savanna Rush. She sold



lemonade and baked goods. She worked at her lemonade stand from 10:30 am – 5:00 pm and only took one break to go to the bathroom – in 90 degree weather! Delaine also designed adorable t-shirts that were worn by all of Savanna's loved ones.

Mitchell Kotheimer and his classmates, John Mino, Natalie Kosir and Angela Brekalo, from Peaceful Children Montessori School in Kirtland, OH, raised funds for Families of SMA. They collected \$100, in honor of Mitchell's brother Jack, by walking the neighborhood streets gathering money and distributing information about SMA.



Congratulations to John Burton who has been selected as one of Kankakee Valley High School's Super Seniors. John hopes to attend the University of Illinois next year to study engineering. In addition to his academic excellence, John is an amazing volunteer and role model for Families of SMA. He is also involved in his church's youth ministry, 4-H and National Honor Society.



From Children's Talk It Out Sessions—FSMA Conference, Chicago, June 2007

Facilitated by Al Freedman, Angela Wrigglesworth, and Rocco Arizzi



Charlie Vincent, and his Third Grade Class at Scott Elementary School, in West Bloomfield, MI, sold SMA Bracelets to raise funds for FSMA. They raised an amazing \$838 for FSMA, in memory of Charlie's sister Jessica!

The students of PS/MS 43 in New York, NY, held a Penny Harvest in honor of Alexa Rodriguez. The kids raised \$250 dollars worth of pennies for FSMA.

The students at Hearn Elementary of Frankfort, KY, held a Penny War for FSMA. The students raised \$559 to help support research for SMA.

For the 7th year the West Rocks Middle School's Community Service Club in Norwalk, CT, held a Penny Drive fundraiser in honor of Cubby Wax. Cubby's mom, Laura, is a well respected sixth grade teacher at West Rocks Middle School and they wanted to support a worthy charity. The students raised \$1,112 to support research for FSMA.

Lydia Wallis, of the Stars Team in Longview, TX donated \$190 in honor of Matthew Wallis. Go Stars Team!

Dear Families of SMA,

For my 8th birthday, I asked my friends to donate or give me a check for FSMA instead of a gift. Some of my friends gave me gifts and donations. I would really like the doctors to find a cure for SMA. My baby cousin, Colin Lynch, died from the disease of SMA and I really wanted to do something in his memory. I really miss him.

One of my friends has a family friend who has SMA and is 18 years old. He is now in college in California and I am going to try to get in touch with him to see how he is doing.

I hope you can make a cure for SMA!

From your friend, Erik Fenstermaker, Philadelphia, PA

"If you could have one day out of your wheelchair, what would you do?"

I'd play basketball with my brother.

I would like to bungee jump off a 300 foot bridge over a river.

Go snorkeling and skydiving.

I would go right to the dance studio and dance all day long.

I'd go rock climbing.

I'd go out for a jog and then I'd drive a car.

I'd run around and jump everywhere and trick my sister.

I'd invite all my friends over and jump and skip up to all of them and say hi.

I'd climb a tree.

I'd walk around all day at the mall and go shopping.

I'd do lots of things with my friends and I'd feel normal.

I'd run around and be happy all day.

I'd go swimming.

I'd race cars.

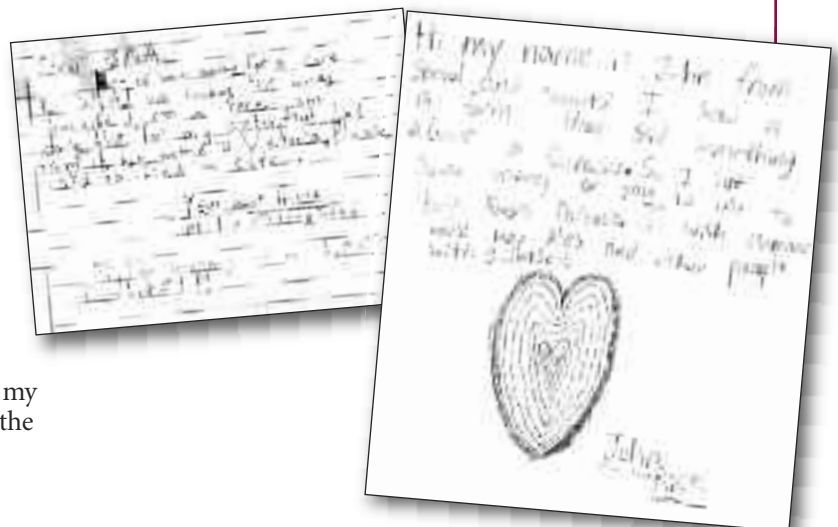
I'd jump rope and go on the swings all day.

I'd run after my brother.

I'd play hopscotch.

I'd sneak out of my bed and play when my mom and dad were sleeping.

I'd have a sleepover with my friends and sneak up on the top bunk.



Share the Power of a Wish[®]

Every child has a dream – and the Make-A-Wish Foundation is dedicated to making dreams come true for children with life-threatening medical conditions by granting his or her favorite wish. Elizabeth, who has Spinal Muscular Atrophy, realized a dream come true when the Make-A-Wish Foundation arranged for her to meet her favorite princesses.

Three-year-old Lizzy is a bubbly young girl from Illinois who loves dolls and watching princess movies. She often dresses up like a beautiful princess with glittery hair bows and sparkling jewelry make her feel the most elegant. According to doctors, Lizzy is a “miracle baby.” Born with Spinal Muscular Atrophy, she was given six months to live after birth. Because of her condition, she has to make frequent trips to the doctor and must use a wheelchair.

To Lizzy, her wish represents appreciation for life and a memory that will remain in her heart forever. Her parents said that Lizzy had the time of her life

and loved the personal attention she received from everyone during her Make-A-Wish trip.

There are many children just like Lizzy who could benefit from having a wish granted. Many Make-A-Wish families view their child’s wish experience as part of the treatment process because it provides a renewal of hope and allows each child the opportunity to concentrate on the magic of being a kid.

To be eligible for a wish, a child must be under the age of 18 with a life-threatening medical condition. There is no waiting list to have a wish granted and there is never a cost to the family.

Children can be referred by their parents, their medical professional or even him- or herself.

If you would like more information about how to refer a child for a wish, contact the Make-A-Wish Foundation.



Elizabeth Hallam enjoys her Make-a-Wish experience.



My daughter McKenzie Damon’s 3rd grade class in Brooklyn, WI made a donation in her honor when we had our SMA golf outing. The kids donated \$35 in honor of her. I have attached a picture of the class. I will be sending the check in with several other checks from the golf outing and our softball tournament we held in April and May.

You Have The Power To Grant A Wish!

The Make-A-Wish Foundation grants wishes to children living with life-threatening medical conditions to enrich their lives with hope, strength and joy. If you know a child who could benefit from having a wish granted or want to get involved in making wishes come true, contact us today!

800.722.WISH
www.wish.org





Amelia Wong



Grace Degraffenreid



Cassandra Evans



Sami & Cole Steven Abraham



Britta Halvorson



Ayden Trammell



Jake and Rachael Saxton



Christian Perez



Murphy Potter

Sykora Family at Disney



David and Sean Smith



Julia Bartczak



Ally Krajewski and Sofia Salus



Colby Russ



Melissa and Claudia Milinovich



Jerika Bolen and friend Amanda



Nicole, Joseph and Jackson Cole Hedgepeth



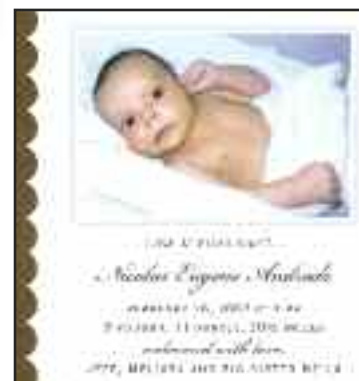
Tambryn and Braelyn Campbell



The Bahrenburg Family



Lily, Charlie Sykora and Brett Wilson



Evan and Nathan Deddeh



Courtney Brooke Rosas



Amy & Russ Colby and Judy Berry



Kristin and Payton Webb



My name is Anita Balistrei and I was born on July 24, 2006 in Palermo, Sicily. At the age of 6 months old, my mom, dad and family noticed that I wasn't developing with my moving skills (like crawling and doing a lot of things that babies do at that age).

My parents took me to the pediatrician and they tried therapy, but that didn't work. The doctors

then recommended for my parents to take me to Milano to see some specialists as to why I don't crawl or sit up by myself. My blood tests revealed that I have the SMA gene.

My Mom & Dad and entire family just love me and couldn't imagine life without me. I really light up the room. I am always smiling, I love to eat and yes I laugh often. You can see me in my pictures (there will be more to come) that I have a sparkle in my eye.

My Mom & Dad take me to the beach and I enjoy going in the water. Although I can't crawl or walk right now, I am hoping that I will someday and be able to receive all of the medical care available for children with SMA. They don't know my type yet, but think it might be 2. The doctors have said that the disease has affected me so far from the waist down. My Grandma & Grandpa say I'm like a Shirley Temple.

Dear SMA,

The Stars Team is very proud to help our cousin and friend, Matthew Wallis. We are overjoyed each time we get a letter from you all. We would like for you to know that we have added a new member to the team. Her name is Lauren Bally. We are praying that our small gifts of money will be able to help find a cure for Matthew and others.

With hope,

*Lydia Wallis, Faith Brandy, Ashley Gordy and
Lauren Bally of Texas*

Kaitlyn's Story

I would like to tell the story of a most beautiful girl. The girl is our daughter, Kaitlyn. Kaitlyn was born April 24, 2002. She was a load, squirmy and perfect baby. Three months later, Kaitlyn was diagnosed with SMA type 1. We'll never forget the words, "Take your daughter home and love her. She will not make her first birthday and for sure not her second" Well Kaitlyn made her first, second, third, fourth and now her fifth birthdays!! Living with SMA is not something we wanted but we would not change our lives with Kaitlyn. Like the Garth Brooks song says "I could have missed the pain but I'd had to miss the dance" That is exactly how we feel.



Kaitlyn lives a great life everyday by strength, love, stubbornness, and the help from many interventions. Kaitlyn used a bi-pap machine, cough assist machine, she is hooked to an oximeter, she is tube fed, suctioned, has chest and ROM therapies twice a day. Kaitlyn has the movement of her fingers and that is pretty much it. She communicates with her eyes and is vocal without words that are familiar to the "regular" world. Kaitlyn uses a computer, clock communicator and has many switch toys. Kaitlyn is very smart and is doing very well in a home based school program.

Kaitlyn has a big brother named Liam. Liam is very protective of his sister. Liam is almost 9 years old and very busy. He has a hard time playing with Kaitlyn because he has a small attention span. They watch t.v. together, play puppets and fetch together. None the less he loves her very much and is very proud of her.

Kaitlyn enjoys her therapy tub she received from Make-a-Wish. In the tub she can move her legs freely. It is something that she is proud of.

I just wanted to share with the SMA world our precious daughter. We love her so much and look forward to many more wonderful, challenging, loving and special years with her.

Thanks,

Hali Harapchuk

Dear FSMA,

I just wanted to take the time to let you know just how much you have helped my family. Tori was diagnosed with type II SMA the day before her first birthday. Such devastating news for what is supposed to be a time of celebration. Doctors had no hope or encouragement for our future. Tori is our only child and how could we give up on something that was so perfect and special to us? In my despair, I went to the internet to find some kind of explanation. I found Families of SMA!!! I found out about the FSMA Convention in Chicago, IL and decided that is where I would find out some answers to my questions.

Three weeks after Tori's diagnosis, I found myself at the 2007 SMA Convention. I just want to say thank you for the love, hope, and encouragement you have provided me and my family. When we arrived, we were at a place we didn't belong, or didn't want to belong. After spending three days with some of the most wonderful individuals we had ever met in our lives, we realized that we do belong to this special group of people. Doctors could give us no hope, no encouragement, and no real understanding of what was ahead of us. Families of SMA has given us peace, hope, and friendship. We now understand that it isn't over, but it is just the beginning...and what a wonderful beginning it will be.

Thank you for your help. I don't know where we would be without you.

Love,

Misty Partee (of Kirby, AR), (Tori's Mother)

Dear FSMA,

We just wanted to thank everyone for everything. We really had a GREAT time. It was so good to see all of our extended family. We just wish we had more time with everyone. All of the doctors, guest speakers, and vendors were wonderful as always. We learn so much with each conference we attend that really helps us. You have been so helpful and kind to us and we truly appreciate that. We are so thankful for all of your hard work and true dedication, with you all we know that a cure will be found! Thank you all very much for your passion to cure SMA! Someone asked a panel who their hero was and why? I thought about that question and came to the conclusion that FSMA was mine, because of your commitment to never give up until a cure is found. Thank you very much!!!

Sincerely,

Scott, Candy, Brittany and Brenden Carpenter

Dear FSMA,

Five years ago, Adi and I attended our first FSMA conference, here in Chicago, with a five month old Jacob in tow. He had been diagnosed only 6 weeks before the conference. We were greeted with open arms and hearts, and while we asked ourselves how it was possible that we had become a part of this family, we knew we had come to the right place.

During the conference, a \$100,000 donation was announced, and it was in that moment that I knew I wanted to be involved in the fundraising efforts of such an amazing organization.

In our fourth year of fundraising, the Jacob Isaac Rappoport Foundation is proud to present the enclosed contribution of \$110,000 to Families of Spinal Muscular Atrophy. This brings our total contribution to FSMA to \$350,000 over the past 4 years. As we agreed, \$50,000 will be allocated to the type one clinical trial; \$50,000 will be allocated to the DeCode campaign; and \$10,000 will be

allocated to stem cell research. I am in awe of our supporters and our volunteers who have made this possible. As always, I am in awe of our son, who continues to touch so many lives and make a difference in the fight against SMA.

We would like to honor the SMA families in South Florida who are instrumental in making our fundraisers a success. In addition to making our contribution in memory of our son Jacob Isaac Rappoport, we would like to also make it in honor of Taylor Bowser, Jack Freedman, Zeke Lerner, Natalie Quintana, and in memory of Shelly Cahlon, Payton Freeman, Katie Gardner, Caitlyn Munson, Ryan Nolan, Shreeya Patel, Drew Selogy, Alexander Tizzoli, Carl Vanderveen, Tyler Yunes, and Margaret Zayas. Their families have touched our lives with their incredible support and truly helped to make our event a success. Finally, we would also like to make our donation in honor of Audrey Lewis, who has been an inspiration and a constant support to us.

We cannot possibly thank you enough for all that you do.

Sincerely,

Shaina and Adi Rappoport & The Jacob Isaac Rappoport Foundation

Dear FSMA,

This year I attended my first FSMA Conference in Schaumburg. I was very impressed with what I experienced, all the way around. First, the Woodfield Hyatt Regency was wonderful in accommodating the whole event and the special needs of the families. The facility was very easy to get around, even with the large number of special-needs people who attended. All the staff assisting were more than friendly and courteous and willing to help in any way. I thank them for all they did in facilitating our Conference.

I was also educated by all the families and the kids who I had the good fortune to meet or see while attending the conference. The level of fun available to

continued over

the families was to me, measured by all the smiles and activity happening throughout the entire event. Some of the families went home tired, I'm sure, but the fun everyone was able to experience was worth a few moments of being tired. Thank you to the parents and siblings, and everyone who was there related to SMA, the organizers of this year's conference, and all who attended made it an enormous success. It was all about letting everyone get together and relax so they could enjoy themselves for a weekend with other families coming from the same position. The kids all seemed to have had a very good time. Believe me, they all will carry those great memories with them forever.

The Conference was a huge success from where I sat. I too, have SMA, type 2. I am 46 years old and this was the first time I was privileged to see others with SMA and their families to this extent. I loved the information the researchers had to tell us. I am very pleased and excited by their progress. I thought it was extremely touching to get the families with SMA and the researchers together in such a happy and fun way. I also, had a lot of respect and admiration for the researchers because they care enough to find a cure.

I feel fortunate to have attended the workshops with such experts there to educate us. I thank the speakers who held the workshops. All that we were able to learn or witness from those who hosted the workshops was of incredible value, from the adults with SMA, as well as from the younger people, and those too, who did not have SMA. I have a lot of hope for the future because of the informative workshops. I can appreciate, regardless of the limitations some of us may have, we are not limited just because we do live with SMA.

I felt ashamed to not have spoken up at the time, and let people know of the possibilities for their future in driving. So many of the people there have accomplished so much in their lives that I was in awe of them. For me to speak up would have been rude. My only accomplishment is my independence. I

Dear FSMA,

I attended my first FSMA conference last week. All of the sessions were outstanding. The speakers, doctors and volunteers were awesome! It really helped me to understand SMA and discuss with other families how everyone copes with this disease.

Your office did an excellent job putting together the conference!

Thanks,

Laurie Jachimiec, Houston, TX

live in my own home, with my four cats and my dog. I am newly divorced after almost 25 years of marriage. I had to struggle to maintain my independence through the divorce and since then, but I feel very blessed to now be able to successfully live independently. I have to attribute my success to wonderful, loving people around me helping me. I do have my driver's license and I did drive to the conference on my own in my own van. I was fortunate to be given the determination to try to do whatever I can. Not all of us have all the same abilities, but we can have the idea and the desire to try to do what we want to do. I do want everyone to know the possibilities are there in any area, to accomplish more than we think we can sometimes. I fought for myself to be given just the chance to try to get my driver's license. Almost ten years ago now, I did finally take the time to accomplish that goal. It was a huge challenge and I really had to push myself to keep trying until I was successful. The technology is there to accommodate some of our limitations, when and if we do not accept the idea that we have to do without. There are those of us who do not want to drive, or cannot because our limitations may be overwhelming. I did not get my driver's license when I was young because I was told at the time it would have been impossible. At that time it was impossible. But as I grew older, and older, I knew I just had to be given the chance to try, even just to show myself if I

could or could not. I had to challenge the agencies and the heads of the programs at the state level to get the chance to try, but I was willing to go through that because I knew if for some reason I were able to achieve what I wanted, then there was a chance for others like me for their future to drive as well.

Now, the abilities I have because I am capable of driving are countless. The independence I have is appreciated beyond just the mobility. It made it possible for me to live by myself. Even though I may not be able to drive forever, I am living with what makes my world now. Accomplishing that one dream has opened my world up huge. I want you to understand, as long as there is hope, there is a chance to achieve anything. There is a chance for some of us to obtain the ability to drive, so with that hope, it also means there is hope for a cure for SMA as well. Never give up hope.

Kelly Miller

CONGRATULATIONS

- Congratulations to Laurie, Mark and Murphy Potter on the birth of their son / brother Anders Matthew Charles Potter born on September 11th.
- Congratulations to Renee, Sam and Sami Abraham on the birth of their son/brother Cole Steven Abraham born on July 10th.
- Congratulations to Melissa and James Milinovich on the birth of their daughter Claudia Milinovich born on April 5th.
- Congratulations to Mary Ellen Barrelle, of Rowayton, CT. She was awarded 1 of only 27 grants from the Prudential CARES Volunteer Grant Program. The Prudential Foundation donated \$1,000 to Families of SMA in honor of Mary Ellen's hard work, on behalf of the FSMA CT Chapter.

Emergency Preparedness for Children With Special Health Care Needs

ABSTRACT. *Children with special health care needs are those who have, or are at risk for, chronic physical, developmental, behavioral, or emotional conditions and who also require health and related services of a type or amount not usually required by typically developing children. Formulation of an emergency care plan has been advocated by the Emergency Medical Services for Children (EMSC) program through its Children With Special Health Care Needs Task Force. Essential components of a program of providing care plans include use of a standardized form, a method of identifying at-risk children, completion of a data set by the child's physicians and other health care professionals, education of families, other caregivers, and health care professionals in use of the emergency plan, regular updates of the information, 24-hour access to the information by authorized emergency health care professionals, and maintenance of patient confidentiality. Pediatrics 1999;104(4). URL: <http://www.pediatrics.org/cgi/content/full/104/4/e53>; children, special health care needs, emergency preparedness.*

ABBREVIATIONS.

US DHHS-MCHB-NHTSA EMSC, US Department of Health and Human Services, Maternal and Child Health Bureau, National Highway Traffic Safety Administration, Emergency Medical Services for Children; EMS, emergency medical services; AAP, American Academy of Pediatrics.

Emergency care of children with special health care needs is frequently complicated by a lack of a concise summary of their medical condition, precautions needed, and special management plans. This policy statement introduces a standardized information form that can be used to prepare the caregivers and health care system for emergencies of children with special health care needs. Emergency data sets, summaries, or “passports” have been used in several of the US Department of Health and Human Services, Maternal and Child Health Bureau, National Highway Traffic Safety Administration, Emergency Medical Services for Children (US DHHS-MCHB-NHTSA EMSC) demonstration grant projects. Use of such emergency data has been advocated by the EMSC program through its Children With Special Health Care Needs Task Force.

This statement describes essential components of an emergency information program. Figures 1 and 2 show a blank form and a sample form. Implementation of this program by a pediatrician or other health care professional or as part of a comprehensive EMSC program in a state will improve the ability to care for children with special needs.

Children with special health care needs are those who have or are at risk for chronic physical, developmental, behavioral, or emotional conditions and who also require health and related services of a type or amount not usually required by children.¹ Children with special health care needs frequently require emergency care for acute life-threatening complications that are unique to their chronic conditions.

Emergency hospital and prehospital care is believed to be negatively affected

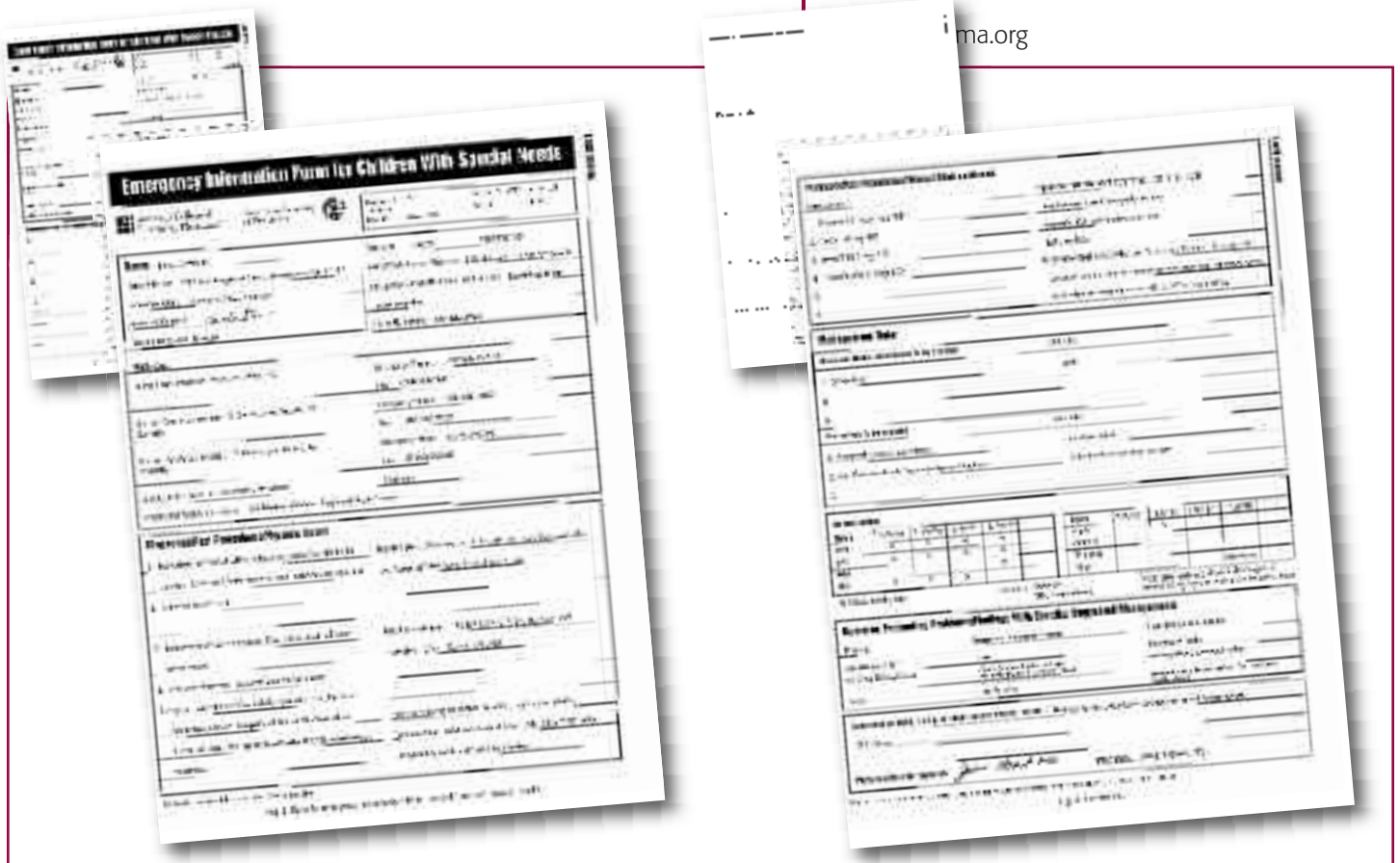
by a frequent lack of accurate timely information about the children's special needs and particular histories.

To address this identified need for the group of children with special needs, creation of a passport plan or emergency medical information set has been advocated by the US DHHS-MCHB-NHTSA EMSC program through its Children With Special Health Care Needs Task Force Report of January 1997. The report notes:

“If the child is at risk for future medical emergencies, the child and family should participate in developing a written emergency care plan. Copies of this plan should be kept in easily accessible places at the child's home and any other location where the child regularly spends time. The plan should include provisions for any special training that will be needed by emergency medical personnel, family members, or other persons who may be called on to provide emergency care for the child.”² To date, the efficacy of this method in improving care for children with special needs has not been studied. However, several US DHHS-MCHBNHTSA EMSC projects have used an emergency information set in populations with special needs.

Projects in New Mexico, Wisconsin, Ohio, and the Ohio–Kentucky–West Virginia region have used wallet cards or 1-page summaries that are given to parents.³ The wallet cards have separate pages for demographics, diagnoses, conditions, and medications and can be updated by exchanging single cards.

Currently in West Virginia, a single page (front and back) summary is being tested throughout the West Virginia MCHB Children With Special Health



Care Needs Division.⁴ Adjuncts to the program include window stickers identifying the homes of children with special needs and linkage to an emergency telephone number such as 911, which will alert emergency medical service (EMS) professionals to look in the refrigerator for a vial containing the summary.

Sherman and Capen⁵ recently described a program to streamline and standardize access to care for asthmatic children with a history of life-threatening events. Termed the Red Alert Program, the parents, health care professionals, EMS providers, schools, and emergency physicians were educated about the need for early access to aggressive acute treatment of the child's asthma, and the parents were given written documentation of the history of severe asthma.

The emergency information set or passport should result in improvement in the emergency care of children with special health care needs. The emergency information set should be consid-

ered a part of the overall plan of service advocated by the American Academy of Pediatrics' (AAP) Committee on Children With Disabilities.⁶ In addition, completion of the summary will fulfill the need for a medical history for any child wanting to participate in child care, school, day camp, or resident camp.⁷ Implementation of this program through a pediatrician or other health care professional, in a child care facility, through a school system,⁸ or as part of a comprehensive EMSC program will improve the ability to care for these children.

RECOMMENDATIONS

The AAP offers the following recommendations:

1. A brief, comprehensive summary of information important for hospital or prehospital emergency management of a child with special health care needs should be formulated by the child's caregivers, health care professionals, and all subspecialty providers.

2. The summary, or emergency medical data set, should be updated regularly and maintained in an accessible and usable format.

3. Parents, other caregivers, and health care professionals should be educated to optimize use of the summary. Parents and other caregivers should be encouraged to take the summary with them for all health care encounters.

4. Mechanisms to quickly identify children with special health care needs in an emergency should be established and should be available to local EMS and hospital personnel.

5. A universally accepted, standardized form should be used for summaries. Figures 1 and 2 show a suggested form entitled "Emergency Information Form for Children With Special Health Care Needs." Essential data elements include the patient's name, birth date, date of last summary update, weight, guardian's name, emergency contacts, pediatricians and other health care professionals, primary emergency department,

major chronic illnesses and disabilities, baseline physical and mental status, baseline vital signs and laboratory studies, immunization history, medications, medication allergies, food allergies, and advanced directives.* The AAP and its chapters should encourage local adoption of the American College of Emergency Physicians/American Academy of Pediatrics form.

6. Rapid 24-hour access to the summary should be ensured. Copies should be accessible at home, school, during transportation, and in the emergency department in addition to a copy in the records of treating physicians. Linkage to an emergency telephone number such as a 911 dispatch or some other method of assuring rapid access is desirable. Especially important is identification of the most appropriate EMS squad to be called in areas without a 911 dispatch. Schools and child care facilities should be encouraged to include the emergency summary as part of a child's individual health plan.
7. Confidentiality of the form should be carefully maintained. Parental permission to establish the emergency information form and distribute it to appropriate agencies should be obtained and kept on file with the originator of the form or at a central repository.

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* Most states have a standard advanced directive form, which is required for EMS to honor the advanced directive to withhold emergency lifesaving / measures; however, the emergency data set or summary can identify a need to look for the standard form.

Preparing for a Hospital Stay

By Julie Lino

Look and listen to your child NOW while they are healthy.

1. Listen to your child and learn his/her healthy breath sounds. You can purchase an inexpensive **stethoscope** at any drug store or uniform shop. Learn the different chambers/lobes of the lung. This not only will help you hear a difference when they are sick, but will help your child become familiar with the medical devices. Let them be part of their own respiratory treatment by letting them listen to you, brother, sister, pets, etc.
2. **Pulse Oximetry**—Health insurance and Medicaid often covers this piece of equipment. Periodically check their oxygen saturation (SAO₂) and heart rate. Check it while sleeping and awake. Learning your child's healthy SATs with help when they become ill. These SATs change due to a variety of normal activities, but also can be a red flag to an underlying infection—the heart pumps harder when trying to maintain a 'normal' O₂ level.
3. **Observation**—If you don't already do this now, pay attention to his/her breaths. What is your child like when 'healthy'? Discuss 'labored breathing' with your doctors. In younger children, if they develop a collapsed lobe, you can sometimes visually see a difference in their chest—perhaps a dip under his/her armpit or are his/her nipples parallel?

Every SMA child is different. You will be the best resource for your child's medical team.

Hospitalization Tips

The following tips are for a hospitalization. They are the compiled opinions of SMA parents based on their child's hospitalizations and experiences. The following are examples of what other SMA parents have done and might help you. No doubt about it, a hospitalization is stressful. Keep the door of communication open with your medical team.

1. Know and be okay with the fact that most people, even those in the medical and respiratory professions, know little or nothing about Spinal Muscular Atrophy. It may be your "job" to educate the medical team caring for your child. This may likely apply when referring to SMA medical protocols from Dr. Swoboda, Dr. Schroth, Dr. Bach, and techniques / procedures that are discussed in SMA literature or during the FSMA conference breakout sessions.

You know your child best. Especially his or her routine and what they are like when "healthy"

Go in with the possibility you might have to make a decision regarding ventilation: Oxygen, BiPap, IPPV, vest, intubation and tracheotomy. Explore these options now and investigate these procedures and possible outcomes so you are aware of them.

Try to keep calm and be friendly... there will be situations of conflict with your medical team. You can apologize later for tense moments, but know that everyone is trying to do the best for your child, including you!

2. Prepare now for a hospital stay. One can never be emotionally prepared for a stay, but being prepared with materials will assist you and your

medical team. Make a pocket folder or binder and fill it with reference information. Have this file readily accessible in case of an emergency. You may be able to review the information while waiting in the ER and/or once you get settled in a room.

BINDER CONTENTS: a) Protocol for your child and "Do Not Resuscitate" order (if you want or have one) b) Photocopied articles printed from the www.FSMA.org, www.doctorbach.com, web sites, articles from FSMA DIRECTIONS, materials from the FSMA conference, and pertinent emails. If there are articles and procedures you'd like your medical team to adhere to, provide copies for them. Also make sure copies of these get put into your child's chart. Every nurse and doctor has access to it. (You may also have to refer to them); c) A notebook and pen to journal, document your emotions, conversations with medical team, procedures, all medications and purpose administered, anger, everything! This is not only a great stress release, but also a valuable reference tool for exhausting days later and/or the next hospitalization. Do not be afraid to document in front of your medical team—it is okay to educate yourself and keep track of events; and d) Telephone numbers and email addresses of SMA families you'd like to connect with and doctors with whom you want to consult with.

This is an abbreviated version of this article. The complete article can be found online at www.curesma.org.

THE JENNIFER TRUST RECEIVED AN EMAIL FROM A YOUNG LADY IN POLAND WHO HAS SMA TYPE III. SHE'S AN ACADEMIC, VERY SPORTY, AND A SUCCESSFUL MODEL. BUT IF SHE IS ONE OTHER THING, SHE IS AN INSPIRATION! HERE'S HER STORY, IN HER OWN WORDS AS KINDLY TRANSLATED BY SANJA UDALL FROM BIDFORD ON AVON ENGLAND:



My name is Agata Jablonska. I am a 25-year-old model from Wroclaw Poland. I have a M.A. in philosophy, I am a member of swimming club for disabled people called "Start" and I am in a wheelchair.

My personal aim in life is to eliminate barriers and stereotypes in society.

I have Spinal Muscular Atrophy. It is an incurable condition, manifesting itself in a substantial deterioration of my whole body. I am a typical example of this, but I do not want to give up my dreams because of my physical limitations. I strive to show how to overcome difficulties and live a fulfilling life achieving one success after another.

I have completed studies in banking and finance as well as philosophy. The studies have awakened in me the wish, and motivation, to get to know people. In the near future I am planning to devote myself to moral values of disabled people. I am a person who endlessly tries to show others that disability can be an opportunity for success and a strong reason for big achievements.

I am also successful in sports. For several years I have belonged to the Wroclaw Swimming Club "Start". I have won medals many times.

My main swimming style is backstroke. My ambition is to represent my club at

swimming galas across my country. Swimming gives me the feeling of independence. Also I am treating it as a way of fighting this debilitating condition.

I have been modelling from the age of seventeen. My first professional engagement achieved great success – I got a title of "The Best Girl in 1998" for the Wroclaw newspapers. That was the crucial moment in my career as a photo model. My best characteristics are my facial features, my face is very expressive what secured my win in Polish Internet competition "Regular Features" in 2006.

At the present I am modelling for the best Polish model agency. I have had lot of photo sessions, modelled clothes, been on television programs and had interviews in magazines. I am a person that people easily recognize and I treat my job very professionally.

I am not sitting between four walls. I am striving to integrate and succeed in the world. At the moment I am preparing myself for an international competition for modelling in a wheelchair "Beauties In Motion International 2007" which will take place in Hanover Germany – the website address is <http://www.beautiesinmotion2007.com/> I wish to show that people in wheelchairs are as beautiful and sexy, that you can fulfil your dreams, constantly overcoming limitations. By my example I want to motivate other women.

I wish them to have courage and to achieve a success in what they are good at. Through my actions, as well as the belief in what I do, I have realized that the wheelchair does not have to be treated negatively – rather contrary - it can become an important tool in your life.

My greatest satisfaction is the realisation that the success I have achieved encourages other people. I am making society overcome stereotypes. In my opinion being disabled is all in people's minds, not in the limitations resulting from the malfunction of the body.

I work every day in a Capital Group Impel S.A. dealing with Human Resource problems. I am using my knowledge and my skills, which I continuously endeavour to perfect. I am aware that I work in a firm that does not discriminate against people for their disability, but appreciates their professionalism. Work gives me self-awareness as well as motivation to further development.

I always wish to show that it is worth making an effort to achieve your goals.

With best regards to you all

Agata Jablonska

You can see Agata's photo portfolio at <http://www.jablonska.org/>

Thank you Agata for your inspirational article and we wish you all the best with your various careers. Thank you as well to the Jennifer Trust in the U.K. for sharing this story with FSMA.



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- Kids' program
- Continental Breakfast before Opening Session
- Annual Full Banquet Dinner & Silent Auction
- Luncheon –adults only – Research Q&A
- Annual Kids Carnival, a favorite with the kids

Join us for the 2008 Families and Professional FSMA Conference in Boston, Massachusetts. One if by land, two if by sea, come discover Boston's history. The Boston Park Plaza Hotel is in the heart of the city and will host the 2008 conference. Boston is easy to get around, either on foot or by the user-friendly public transportation system, called the T.

The hotel is only 3 miles from Logan International Airport. Within walking distance of the hotel are restaurants and infamous Newbury Street with plenty of shopping. Take in a Red Sox game or explore the Freedom Trail. No visit to Boston would be complete without a stroll through the Boston Common and Public Garden and a ride on the famous swan boats. FSMA has been hosting this conference for over 20 years.

The weekend will be filled with networking opportunities with other families, and opportunities to interact with researchers and get first hand updates from the researchers.

For more information including hotel and reservation numbers go to the FSMA website.