In this Issue…

(SMA news to shout about!)

New SMA Care Booklets
Breathing Basics – covering critical aspects of respiratory care for SMA............................................................3
Caring Choices - for parents of infants newly diagnosed with SMA Type I.............................................................13

25th Anniversary Conference
See the full agenda for our annual family and professionals conference in Cincinnati, OH ........................................4

25 Voices of SMA
See the first profiles spotlighting families, clinicians and researchers from the SMA community .................................6

Legislative News
Updates on the SMA Treatment Acceleration Act and other topics from the new congress and administration ...........8

“Ask the Expert” Service
New system to submit important SMA care and health-related questions and search for answers online ................11

SMA Registry Online
New website launched ..................................................................29
Our annual conference this year will be the perfect time for us to celebrate 25 years of FSMA and all that has been accomplished. $50 Million raised and funded for SMA research over the last 25 years is an amazing achievement and has brought us incredibly far.

However, this will not just be a year of looking back. We are a community that is in no way comfortably satisfied with where we currently stand. Families of SMA is aggressively increasing our funding commitments for research and clinical programs to develop a treatment and cure. At the same time we are looking ahead to the future when we will begin to implement the results from our research efforts and begin to improve care for all those affected by SMA.

During the conference in Cincinnati there will be 30 workshops offered covering the latest information on critical topics in SMA care. Over 15 of the leading experts in the field of SMA medical care will lead these workshops. Also provided will be important sessions and demonstrations pertaining to daily living topics. As always, the International SMA Research Conference will run alongside our family meeting, giving many unique opportunities to interact with the most outstanding researchers and doctors in the field of SMA.

We are optimistic about the opportunities on the legislative front as the community will be reintroducing the SMA Treatment Acceleration Act to the new 111th congress. There are a number of articles in this issue highlighting the progress we expect on legislative activities for SMA this year.

In this Directions you can also read about the first booklets in a new series covering important topics on care for SMA patients. We have started the series off with Breathing Basics and Caring Choices. Expect to see more booklets on additional topics soon.

Sincerely,

Kenneth Hobby
Executive Director,
Families of SMA
Families of Spinal Muscular Atrophy has just released a new booklet in its ongoing series on SMA Care. This new booklet is focused on the critical aspects of respiratory care for children with SMA. The booklet was authored by Mary Schroth, M.D., a member of the Families of SMA Medical Advisory Council, and a leading expert on respiratory care for SMA patients.

Respiratory (breathing) problems are the leading cause of illness for children with SMA. They are the most common cause of death for children with SMA Type I and SMA Type II. Respiratory care interventions are essential to the survival and comfort of children with SMA.

This booklet reviews the following important topics:

- Why is respiratory care so important in SMA.
- What are common respiratory problems in children with SMA.
- Elements of respiratory care management in SMA:
  - Assessment, evaluation and monitoring.
  - Breathing exercises for lung development.
  - Coughing practice for airway clearance.
  - Removal of mucus from airways and lungs.
  - Measuring oxygen levels.
  - Breathing support options.
  - Care during a cold.
- Perioperative evaluation.
- What are special needs of children with SMA Type I.
- What are special needs of children with SMA Type II.
- What are special needs of children with SMA Type III.
- What respiratory equipment will you need at home.

This booklet was produced with the kind support of the Angel Baby Foundation.

Author:

Mary K. Schroth, M.D.
Associate Professor of Pediatric Pulmonology
Director, Pediatric Pulmonology Center Grant
American Family Children’s Hospital
University of Wisconsin School of Medicine and Public Health

Expert Review Panel:

- Richard S. Finkel, M.D.
- Albert M. Freedman, Ph.D.
- John T. Kissel, M.D.
- Richard M. Kravitz, M.D.
- Kristin J. Krosschell, M.A., P.T.
- Kathryn J. Swoboda, M.D.

If you would like a hard copy mailed to you please email us at info@fsma.org or call (800) 886-1762.
This is the largest conference in the world for those affected by SMA and for those involved in providing support and care for SMA patients. The weekend will be filled with networking opportunities with caregivers and other families, medical and care workshops, and an exciting children’s program.

The conference provides many opportunities to interact and receive first hand updates from the leading SMA researchers and clinicians from around the world who are working to develop a treatment and cure for SMA. At our 2008 conference, we hosted over 250 researchers and clinicians, representing 50 academic institutions and 23 companies.

Be part of an anniversary celebration as Families of SMA marks 25 years of research progress and service to all those affected by SMA.

- Sharing Your Type III Experience – Facilitated
- Palliative Care
  Tom Crawford, M.D., Johns Hopkins
- Grandparents Talk it Out - Facilitated
- Adults with SMA – Roundtable- Facilitated

12:00pm - 1:30pm Lunch on Your Own

1:30pm – 3:15pm Breakout Session #2
- Occupational Therapy Fun for Daily Living
  (Aquatic Therapy & Computer Adaptations)
  Carrie Barlag, PT & Beth McCarty, OTR/L, ATP, Aaron W. Perlman Center
- Life After Tracheotomy
  Richard Shell, M.D., National Children’s Hospital
- Healing Process #1
  Fred Troutman, Ph.D.
- Orthopedic Management
  John Grayhack, M.D., Children’s Memorial Hospital, Chicago

3:00pm – 5:00pm Toy Adaptation –
  Shannon & Justin Kuester – Parents of an SMA Child

5:30pm - 6:00pm Cocktails – Cash Bar
6:00pm – 9:00pm Conference Banquet

Awards Dinner and Silent/Live Auctions

Baby Room - Open During Opening Session and Workshops Only (ages 3 and under – quiet room with no wheelchairs)

Kids Room - Open During Opening Session and Workshops Only (ages 4 and older)
Saturday June 20, 2009

8:00am – 9:00am Continental Breakfast
Registration
Exhibitor and Vendor Tables
Fundraising Events Table
9:00am – 10:00am Kids Talk It Out (SMA Kids only – Ages 6 - 9 years old)
Al Freedman, Ph.D., Father of an SMA Child, Child & Family Psychologist
Angela Wrigglesworth, Elementary Education Teacher, Adult with SMA
Rocco Arizzi, Electrical Engineer, Adult with SMA
9:00am – 11:00am Siblings Talk it Out
Katlyn O’Brien, Sister of SMA Young Adult
9:00am – 10:30am General Session
It’s a Wonderful Life - Panel
11:00am - 2:00pm Type I Quiet Room Open
10:45am – 12:00pm Breakout Session #3
-Estate Planning for Families with Special Needs Children
Richard Dusterburg
-Keeping Your Lungs Healthy – Type I
Mary Schroth, M.D., University of Wisconsin School of Medicine
-Healing Process #2
Fred Troutman, Ph.D.,
-Yoga Therapy for SMA
Anne Buckley-Reed, OTR, RYT, Forkids OT
-Nutritional Care for SMA
Brenda Wong, M.D., Cincinnati Children’s Hospital
12:00pm – 1:30pm Lunch Outside (Location to be Announced) - Box Lunches Provided
Researchers and Children Relay Race
1:30pm – 3:00pm Breakout Session #4
-Physical Therapy. Occupational Therapy - What’s Best for Your SMA Child
Karen Patterson, PT, University of Wisconsin
Krista Kienitz, OT, University of Wisconsin
-Making the Most of Your Fundraiser
Jason McNeal, GGTs
-Transition to Adult Care for SMA
John Kissel, M.D., The Ohio State University
-Keeping your Lungs Healthy – Type II
Mary Schroth, M.D., University of Wisconsin School of Medicine
-Coping with SMA: How Families Meet the Challenge
Al Freedman, Ph.D., Father of an SMA Child, Child & Family Psychologist

2:00pm – 3:00pm -Yoga Demonstration – Follow up from Workshop
Anne Buckley-Reed, OTR, RYT, Forkids OT
3:15pm – 4:30pm Networking Session (Adults only)
Meet the Medical Professionals
Presenters will be available for your follow-up questions from their workshop sessions.
5:00pm – 7:00pm Adaptive Sports Demo
5:00pm – 7:00pm Dinner on Your Own
7:00pm - 9:00pm Kids Carnival and Bucket Auction

Baby Room - Open During Opening Session and Workshops Only (ages 3 and under – quiet room with no wheelchairs)
Kids Room - Open During Opening Session and Workshops Only (ages 4 and older)

Sunday June 21, 2009

8:00am – 9:00am Full Buffet Breakfast
Conference Wrap-up
9:00am – 11:30am Concluding General Session (Adults only)
Q&A: Research and Clinical Panel
To Be Announced - Leading experts in SMA research in the areas/fields of basic research, drug discovery, and clinical trials will participate.

Baby Room - Open During General Session
Childcare Room - Open During General Session

Conference Meals Include:
Thursday: Dessert Social
Friday: Continental Breakfast, Type I Families Luncheon (Invitation Only) and Dinner Banquet
Saturday: Continental Breakfast and Boxed Lunch
Sunday: Breakfast Buffet
Who am I?

I’m a doctor who lives and breathes SMA. I love to hang out with the kids, take care of them, talk to the families, and do research.

Dr. Crawford is Associate Professor of Neurology and Pediatrics at Johns Hopkins in Baltimore. There, he specializes in neuromuscular, neuro-motor, and ataxic disorders. As an active participant in FSMA’s growth since the early 90s, he has contributed greatly to both SMA clinical care and research.

Why did I become active in SMA?

Working with SMAers and their families is just the perfect match for me and it is very fulfilling. I think one the first babies I ever held – as a medical student in Pediatrics in 1981 – had Werdnig-Hoffmann Disease (SMA Type I). I was so moved by the experience of talking with the mother and noted that many others seemed uncomfortable around her grief. I arranged to do a specific rotation in pediatric neurology and was present when another child with Werdnig-Hoffmann was diagnosed. The thought crossed my mind, “this is a good place to dedicate a life.” I was comfortable with these kids, even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—even though I hadn’t had previous experience with children. I liked making a difference personally—

What is my contribution?

I think that my greatest contribution is to the clinical side of SMA, and I’m proud that I’ve brought so many good people into motor-neuron research. I’ve been very active with the FSMA Medical and Scientific Boards, and a principal investigator for Project Cure, the SMA clinical trials network, since it began. Audrey Lewis, the founder of FSMA, invited me to work with the organization in the early 90s and I’ve been with them ever since.

I’m devoted to helping children with SMA find better clinical protocols and effective therapies. It’s very important, however, to keep things in perspective, with an eye on what really matters in a child’s life, and to the family. I want them to have the therapies that will make them stronger, but it has to fit into normal life.

What do I like about FSMA?

FSMA has helped me greatly and I owe them a lot. They have enabled me pull all of my interests together. I get as much from the kids as they get from me. The conferences and other resources are so important. I’m privileged to be on the Scientific and Medical Advisory Boards, helping to shape the direction of science for SMA.

25 voices of SMA

As part of our ongoing celebration of the 25th Anniversary of Families of SMA we will be spotlighting “25 Voices of SMA” throughout the year. These will be profiles of families, clinicians and researchers from the SMA community.

The first voices are of The Trainor Family, Thomas Crawford, M.D., The Rappoport Family and Hans Keirstead, Ph.D.

25 Voices of SMA

The Trainor Family

We’re the Trainor family from Baltimore…Gene, Barbara, Caitlin (16), Grace (12), and Erin, who passed away from SMA Type I in 1994 at 5 months.

Gene’s the COO of a venture capital firm, the outgoing Chairman of the FSMA Board, and now remains on the Board. Barbara’s a former marketing professional. She’s a member of the FSMA Board, the volunteer Coordinator of the FSMA chapters, the volunteer Conference Coordinator for the SMA Annual Family and Professional Conference, and President of the Chesapeake SMA Chapter, serving Maryland and Northern Virginia.

Why did we become active with FSMA and the chapters?

When Erin passed away, we knew we wanted and needed to make difference in SMA. Despite our tragedy, we felt strongly about reaching out to families facing a similar diagnosis and giving them hope. We attended our first SMA Annual Conference in 1994 and started the Chesapeake Chapter…we’ve been working with FSMA for 15 years. This is such an important part of our lives. We are especially proud to have our daughters working and sharing this experience with us.

What is our contribution?

The Chesapeake SMA Chapter is now one of the largest SMA chapters with about 125 families. In 15 years, we’ve raised a total of $1.7 million dollars for SMA research through multiple fundraisers. More than 400 people now attend our Annual Crab Feast in the summer. Our annual Golf Classic, held each fall, has really grown. Last year we hosted 210 golfers on 2 courses. We’re proud that we’ve kept these events going and growing.

For FSMA as an organization, we like to think we’ve played a role in building and supporting the chapters—helping them to increase from 4 to 25; in improving the program of the Annual Conference; and in expanding the vision and leadership potential of the Board.

What do we like about FSMA?

FSMA is a wonderful organization—one that truly cares about families affected by SMA. Their balance between supporting families and creating hope for a treatment and cure is exceptional.

We know there will always be tragedies, but we have learned that it is possible to move on from them in a meaningful way. Each family’s story and the memory of each child sustains us—enabling us to continue giving our energy, our time, and our hearts to SMA.
25 Voices of SMA
The Rappoport Family

Who are we?
We are the Rappoport Family from Lake Worth, FL: Shaina, Adi, Jordan (9), Max (5), Sam (3) and Jacob, who died with Spinal Muscular Atrophy on October 1st, 2002 at 9 months of age. Jacob has always been our teacher and inspiration. Although we miss him everyday, our experience with SMA has given us gifts and miracles that we would not otherwise have experienced.

Adi is a Shareholder at a Florida law firm, specializing in Estate Planning, Business Planning and Taxation. Adi has served on the FSMA Board since 2003. Shaina is a graphic designer, and President of The Jacob Isaac Rappoport Foundation.

Why did we become active with FSMA?
In 2001, six weeks after Jacob was diagnosed, we attended our first FSMA conference, with a five month old Jacob in tow. We asked ourselves how it was possible to be part of this. But, when we were greeted with open arms and hearts, we knew we were in the right place. During the conference, someone announced a $100,000 donation to SMA research. At that moment, we knew we wanted to be involved in the fundraising efforts for such a meaningful cause and amazing organization.

What is our contribution?
After Jacob's death, we founded The Jacob Isaac Rappoport Foundation, which funds SMA research and administers programs that support affected families. In six years, we have raised more than $600,000 and contributed over $550,000 to Families of SMA. We have provided dinners, cleaning services and packages of toys to more than 100 SMA families. The Foundation has given many scholarships to the SMA Annual Family and Professionals Conferences, and hosted luncheons for affected families. We have given countless hours of emotional support to parents of newly diagnosed babies.

What do we like about FSMA?
We will never get used to living with SMA or without our son, but Families of SMA has always felt like home to us.

The people we have met through FSMA have truly been our lifeline. We simply cannot imagine walking this path without them. We are very proud that Jacob's legacy has been a part in the effort to find a cure.

25 Voices of SMA
Hans Keirstead, Ph.D.

Who am I?
I like to think of myself as an inventor and explorer—for injuries and diseases of the central nervous system. My goal is to invent treatments and translate them into therapies.

Dr. Keirstead, a world famous stem cell scientist, is the Co-Director of the Sue and Bill Gross Stem Cell Research Center and Associate Professor of Anatomy and Neurobiology at the University of California Irvine. He has been working with FSMA for many years, in SMA research and at the SMA Annual Family and Professionals Conference.

Why did I become active in SMA?
I am developing a potential treatment for spinal cord injury, which includes a method for making motor neurons from stem cells. When I looked for diseases that needed motor neurons, I found that SMA was the best fit. Type I SMA, in particular, fits all the criteria for motor neuron transplantation work. It is the most likely to respond to this treatment, the easiest to work with clinically, the least risky and the least costly. There are many scientific and practical reasons to work in SMA.

What is my contribution?
My team has injected spinal cord cells derived from stem cells into paralyzed rats and significantly increased their mobility. This work was recently approved by the FDA for testing in humans, and is expected to be the first clinical trial in the U.S. using human embryonic stem cells.

We are hopeful that our most recent discovery involving motor neurons derived from stem cells may lead to the first clinical trial involving motor neuron transplantation in children with SMA. The first step is a Phase I Clinical Safety Trial. We will test to see if the treatment is safe and does not cause any harm, including tumors and pain, or shorten life span. If the Phase I Test goes well, we will be able to do a Phase II Clinical Trial for efficacy. This will tell us if the stem cells extend life and/or help minutes to function better.

What do I like about FSMA?
I feel their pain and I see their dreams. FSMA has drawn me closer to the families. My team of 45 researchers, hears their stories and sees people with SMA in our lab. This empowers passion in everyone. What we are doing is very, very complex and needs so much support. FSMA has been there for every stage to help us along, providing knowledge, collaborators, and financing. We wouldn’t be where we are today without this wonderful organization.
A Letter to the SMA Community on the SMA Treatment Acceleration Act

Dear Families,

With the start of a new year, a new Congress, and a new Administration, we want to take this opportunity to provide you with an overview of the successes of the past year in building support for the SMA Treatment Acceleration Act and an update of next steps going forward.

At the close of the 110th Congress on January 3, 2009, the SMA Treatment Acceleration Act had 85 cosponsors in the House of Representatives and 21 cosponsors in the Senate, or approximately 20 percent of each legislative body. This represents remarkable progress for a bill that is so-called “disease-specific”, concerns an “orphan” disease, and was introduced just over one year ago. Comparable bills frequently take years and several Congresses before garnering this level of congressional support. It is a testament to your indefatigable grassroots efforts that we experienced such rapid success.

While we all should be very proud of last year’s accomplishments, much work remains in the coming year to achieve the ultimate goal of passage and enactment of the SMA Treatment Acceleration Act. Maintaining a strong grassroots effort is paramount to this effort. There are also a handful of “mechanical” and logistical actions that must take place in Washington, D.C. before we can again gear up our grassroots efforts.

First and foremost, the SMA Treatment Acceleration Act must be reintroduced in the 111th Congress. When the 110th Congress came to a close, all outstanding legislation expired with it. Thus, the House version (H.R. 3334) and Senate version (S. 2042) of the SMA Treatment Acceleration Act from the 110th Congress can no longer be acted upon by the new (111th) Congress; in essence, H.R. 3334 and S. 2042 cease to exist.

We are working with our primary sponsors of the legislation to reintroduce the SMA Treatment Acceleration Act in the 111th Congress, which commenced on January 6, 2009. However, reintroduction may not take place until the early spring. The government relations professionals for the SMA community are currently engaged in high-level discussions with staff for the congressional committees of jurisdiction to make slight adjustments to the bill’s legislative language in order to facilitate its consideration and movement through committee and the Congress. This is a necessary and important step in moving any bill through Congress. As these discussions take place, we will be providing regular and timely updates as the committees are likely to recommend modifications to the legislation. Once these discussions have been finalized, the enhanced bill will be reintroduced in both the House and Senate.

After this reintroduction, we restart our grassroots efforts to build the ranks of cosponsors once again. Fortunately, it is infinitely easier to request that a Member re-cosponsor a bill that he or she has supported previously. However, it will be important to add new cosponsors to the ranks, too, in order to demonstrate that support continues to grow. Your role will be as important as ever in this effort.

Looking ahead, we are cautiously optimistic of our potential for success in the 111th Congress. While many significant hurdles remain (e.g., an increasingly crowded legislative calendar and a growing list of high-priority matters that will monopolize the attention of Congress and the incoming Administration) and passage is by no means guaranteed, several recent political events bode well for progress to be made:

- Barack Obama, a cosponsor the legislation in the 110th Congress, is soon to be inaugurated as the President of the United States. The significance of having the President on record as supporting the bill cannot be overstated.

- Rep. Eric Cantor, the lead Republican sponsor in the House, has been elected by his House Republican colleagues as Minority Whip, the number two position in the House Republican hierarchy. This dramatically increases his political clout with his colleagues.

Additionally, our lead sponsors remain incredibly devoted and supportive and we have a year’s worth of advocacy and educational efforts under our belt. Thus, senior staff for the congressional committees of jurisdiction is aware of and familiar with the SMA Treatment Acceleration Act and prepared to negotiate on specific legislative provisions. It is quite remarkable to be engaged in advanced discussions with committee staff over specific legislative language so soon after introduction for this type of bill.

First and foremost, the SMA Treatment Acceleration Act must be reintroduced in the 111th Congress. When the 110th Congress came to a close, all outstanding legislation expired with it. Thus, the House version (H.R. 3334) and Senate version (S. 2042) of the SMA Treatment Acceleration Act from the 110th Congress can no longer be acted upon by the new (111th) Congress; in essence, H.R. 3334 and S. 2042 cease to exist.

We are working with our primary sponsors of the legislation to reintroduce the SMA Treatment Acceleration Act in the 111th Congress, which commenced on January 6, 2009. However, reintroduction may not take place until the early spring. The government relations professionals for the SMA community are currently engaged in high-level discussions with staff for the congressional committees of jurisdiction to make slight adjustments to the bill’s legislative language in order to facilitate its consideration and movement through committee and the Congress. This is a necessary and important step in moving any bill through Congress. As these discussions take place, we will be providing regular and timely updates as the committees are likely to recommend modifications to the legislation. Once these discussions have been finalized, the enhanced bill will be reintroduced in both the House and Senate.

After this reintroduction, we restart our grassroots efforts to build the ranks of cosponsors once again. Fortunately, it is infinitely easier to request that a Member re-cosponsor a bill that he or she has supported previously. However, it will be important to add new cosponsors to the ranks, too, in order to demonstrate that support continues to grow. Your role will be as important as ever in this effort.

Looking ahead, we are cautiously optimistic of our potential for success in the 111th Congress. While many significant hurdles remain (e.g., an increasingly crowded legislative calendar and a growing list of high-priority matters that will monopolize the attention of Congress and the incoming Administration) and passage is by no means guaranteed, several recent political events bode well for progress to be made:

- Barack Obama, a cosponsor the legislation in the 110th Congress, is soon to be inaugurated as the President of the United States. The significance of having the President on record as supporting the bill cannot be overstated.

- Rep. Eric Cantor, the lead Republican sponsor in the House, has been elected by his House Republican colleagues as Minority Whip, the number two position in the House Republican hierarchy. This dramatically increases his political clout with his colleagues.

Additionally, our lead sponsors remain incredibly devoted and supportive and we have a year’s worth of advocacy and educational efforts under our belt. Thus, senior staff for the congressional committees of jurisdiction is aware of and familiar with the SMA Treatment Acceleration Act and prepared to negotiate on specific legislative provisions. It is quite remarkable to be engaged in advanced discussions with committee staff over specific legislative language so soon after introduction for this type of bill.
We look forward to continuing to work with all of our members and supporters in the coming year to move this important bill forward. We cannot stress enough our gratitude for your ongoing support and efforts as we navigate the legislative process. Please know that we will continuously update you on events in Washington, D.C., with regard to reintroduction and other crucial actions in the year ahead.

Sincerely,

Kenneth Hobby, Families of SMA
Martha Slay, Fight SMA
Cynthia Joyce, SMA Foundation
Annie Kennedy, MDA

If you have any questions concerning this note or the SMA Treatment Acceleration Act in general, please contact our GR professionals:

Spencer Perlm an, Families of SMA, spencer@fisma.org
Laura Lay, SMA Foundation, llay@wswdc.com
Caroline Gibson, Fight SMA, carolinegibson@fightsma.com
Annie Kennedy, MDA, akennedy@mdausa.org

Rep. Patrick J. Kennedy (D-RI); Rep. Eric I. Cantor (R-VA); Sen. Debbie A. Stabenow (D-MI); and Sen. Johnny Isakson (R-GA)

House Energy & Commerce Committee and Senate Health, Education, Labor and Pensions (HELP) Committee

You can find more information on the SMA Treatment Acceleration Act online at the Families of SMA website at www.curesma.org.

Economic Stimulus Bill

On February 17, 2009, President Obama signed into law the American Recovery and Reinvestment Act (P.L. 111-5), i.e., the economic stimulus bill. This legislation provides $787 billion for a wide range of government programs.

Included in ARRA is $8.2 billion for the National Institutes of Health (NIH) to support scientific research. This funding dramatically increases the funding level for the NIH and provides it the resources to support previously unfunded, high-quality scientific research projects and to support new science.

Within the funding provided to NIH, $800 million is reserved for high-impact projects that can be completed within two years. Priority is placed on short-term grants that focus on specific scientific challenges, new research that expands the scope of ongoing projects, and research on public and international health priorities.

Families of SMA supports several research projects that are likely to fit within these requirements and parameters and may benefit from this influx of federal support. We are working with our research partners to identify specific funding opportunities created due to the ARRA funding; it is our hope that potential acceleration of these projects will further our ongoing efforts to identify a treatment or cure for SMA.
Families of SMA Sponsored Clinical Trials

In 2001, Families of Spinal Muscular Atrophy established and single-handedly funded a clinical trials network called Project Cure SMA. This network has conducted natural history studies that increase our understanding of Spinal Muscular Atrophy disease progression, built models for designing SMA clinical trials, and now runs clinical trials with existing drugs. Families of SMA’s investment of $5.5 Million in five multi-center clinical trials is helping to test existing drugs that may lead to a treatment for Spinal Muscular Atrophy.

Summary of Progress in 2008:

SMA CARNI-VAL Trial:
This randomized placebo controlled phase II trial of Valproic Acid (VPA) and Carnitine in children with SMA Type II was recently completed. Preliminary results were presented at the 12th Families of SMA International Research Meeting in Boston this summer.

SMA VALIANT Trial:
This is a clinical trial enrolling ambulatory adults with SMA Type III ages 18-60 using VPA under the leadership of Dr. John T. Kissel at Ohio State University. There are 22 patients currently enrolled, and we are actively looking to enroll 14 additional patients. Study enrollment is projected to be completed by May 2009.

SMA CARNI-VAL Type I.
A clinical trial designed to evaluate the combination of Valproic Acid (VPA) and L-Carnitine for the treatment of SMA in infants with Type I SMA, called Carni-Val Type I, is being conducted by the Project Cure SMA Group. Specifically this trial, which is fully funded by Families of SMA, will assess the safety of VPA and L-Carnitine in infants and develop improved methods to assess the strength and motor abilities of severely affected infants. Thirty-six infants with SMA Type I, ages 2 weeks to 9 months at the time of enrollment, who have clinical features of SMA confirmed by genetic testing, will be enrolled in the trial. Because travel for infants with severe SMA is often difficult, study site investigators will be looking to enroll subjects who live close to the individual centers. The following six North American sites are currently enrolling infants:
Salt Lake City, Utah; Detroit, Michigan; Baltimore, Maryland; Montreal, Canada; Columbus, Ohio; Madison, Wisconsin.

As of February 2009, sixteen infants have been enrolled in the study. All sites are continuing to enroll. If you are interested in participating in this study, please contact the clinical coordinator at a particular site closest to you. More details regarding eligibility criteria and potential enrollment can be found at www.clinicaltrials.gov and www.projectcuresma.org. In addition, we recommend that families who are interested in being contacted for this trial or future clinical trials should register with the International SMA Patient Registry at Indiana University.

A list of all ongoing SMA Clinical Trials can be found at ClinicalTrials.gov

Project Cure SMA:
The clinical trials network currently includes 6 clinical testing sites in North America, 2 international sites, and the entire infrastructure needed to complete a clinical trial. Over thirty professionals are involved, including a full-time clinical trials manager, a central pharmacy, and a center for collection and statistical analysis of data.

We anticipate that clinical testing of novel therapeutics (non-FDA approved drugs), specifically designed for SMA, will commence in the next 2 years. Having a fully operational clinical network with a sufficient number of sites to conduct a pivotal SMA drug trial will help attract and encourage biotech and pharmaceutical companies to invest in SMA drug development.

Funding for all support staff and or all sites in the United States is being provided by Families of SMA. Funding for the site in Montréal, Canada is being provided by Families of SMA Canada. Families of SMA is also helping coordinate trials with the following International Project Cure SMA Sites:

Cordoba, Argentina:
The Argentina branch of Families of SMA has sponsored the first South American site of Project Cure SMA, under the direction of Alberto Rosa, M.D., Ph.D. In June 2008, Dr. Rosa launched the Randomized Placebo Controlled Phase II Trial of Valproic Acid and Carnitine in Children with SMA Type II. A total of 14 SMA Type II patients have been enrolled.

Cologne, Germany:
Dr. Juergen Christoph van Kleist-Retzow joined the Project Cure SMA Clinical Trial Network as the seventh study site for SMA CARNI-VAL Type I clinical trial. Funding for the site in Cologne, Germany is being provided by the “Initiative Forschung und Therapie für SMA”.

www.curesma.org

www.curesma.org
Families of SMA Launches
New “Ask the Expert” Service

Get answers to your important care and health-related questions with the help of Families of Spinal Muscular Atrophy Medical Advisory Board experts. Our free Ask the Expert feature connects you with leading SMA physicians and clinicians, who can answer questions on a wide variety of health-related topics.

Visit the FSMA Web Site to Submit a Question or Search Past Answers

We have also made every question that we have ever answered available to you and searchable by keyword. This is a compilation of one of the most comprehensive sources of information and knowledge on SMA care available anywhere.

DISCLAIMER: This feature is provided for informational purposes only.
The answers you receive from FSMA experts are not intended to be a substitute for individual medical advice in diagnosing or treating a health problem. The FSMA Ask the Expert feature is a resource for general, nonurgent questions. It is not a substitute for a doctor’s visit. You should contact a professional health care provider if you have any specific questions about your or your child’s medical condition.

Questions or situations requiring immediate attention should be directed to your or your child’s doctor. If he or she can’t be reached, call the nearest urgent care or emergency department. Call your doctor, 9-1-1 or visit your local emergency department if you have an urgent or emergency question.

Please keep in mind that Families of SMA does not, as an organization, support or endorse any particular treatment or therapy. All medical information presented should be discussed with a qualified physician.

Families of SMA Equipment Pool and Family Resources Available

Families of SMA has helped thousands of families over the last 25 years by loaning out critical equipment for medical needs and to improve quality of life. We have loaned more than 3,800 pieces of equipment to families in need, including: standing wheelchairs, therapy wedges, bathing and toileting chairs, cough assist machines, Bi-Pap’s, manual wheelchairs and specialized strollers.

Families of SMA is continuously receiving new pieces of equipment for the equipment pool. If you are looking for a specific item, please e-mail equipment@fsma.org.

The C.A.M. Fund of Families of SMA has sent car beds to more than 247 families for their Type I infants so that they can safely transport their children, and 250 sheepskin blankets for newly diagnosed Type I infants. The Tumbleweed Wagon Fund of FSMA has sent 160 radio flyer wagons to newly diagnosed Type I families.

With the help of Donna and Alex Abraldes, Mary Jane Utzat, The Wyatt Kyle Sutker Foundation, The Jacob Isaac Rappoport Foundation, The Angel Baby Foundation and Ryan’s Hope Foundation over 600 Type I families have received care packages with toys, videos, swings and other items to help stimulate SMA infants.
President Barack Obama Signs Stem Cell Executive Order

President Barack Obama recently lifted some restrictions on federal funding of human embryonic stem cell research. This executive order will permit federal funding for research on utilizing embryonic stem cells to identify cures for various disorders.

Now that the force of the federal government can support such research we hope this will expand the available resources and help advance this exciting research.

Quotes from the Press Release follow:

“Today, with the Executive Order I am about to sign, we will bring the change that so many scientists and researchers; doctors and innovators; patients and loved ones have hoped for, and fought for, these past eight years: we will lift the ban on federal funding for promising embryonic stem cell research. We will vigorously support scientists who pursue this research. And we will aim for America to lead the world in the discoveries it one day may yield.

At this moment, the full promise of stem cell research remains unknown, and it should not be overstated. But scientists believe these tiny cells may have the potential to help us understand, and possibly cure, some of our most devastating diseases and conditions. To regenerate a severed spinal cord and lift someone from a wheelchair. To spur insulin production and spare a child from a lifetime of needles. To treat Parkinson’s, cancer, heart disease and others that affect millions of Americans and the people who love them.

Ultimately, I cannot guarantee that we will find the treatments and cures we seek. No President can promise that. But I can promise that we will seek them — actively, responsibly, and with the urgency required to make up for lost ground. Not just by opening up this new frontier of research today, but by supporting promising research of all kinds, including groundbreaking work to convert ordinary human cells into ones that resemble embryonic stem cells.”

Families of Spinal Muscular Atrophy has invested significant resources in a Stem Cell Motor Neuron Replacement Program. We have invested $1.5 Million to develop a motor neuron replacement therapy for SMA, and we have made significant progress with our investment.

Our initial investment in stem cell research in 2000 funded efficacy studies using motor neurons from mouse stem cells. Results show that this therapy can provide benefit to rodents with motor neuron disease: a highly significant finding. In 2005, additional FSMA funding lead to the first, highly-pure therapeutic population of human motor neurons for cellular replacement therapy for SMA. This program is now progressing on the path to IND in collaboration with the biotech firm California Stem Cell, Inc. (CSC), and leading research centers at University of California-Irvine, and Johns Hopkins University. These motor neurons recently completed a series of critical animal safety studies prior to advancing into human trials for SMA.

Project Cure SMA Clinician Dr. Richard Shell Receives a Special Award

Dr. Richard Shell, a pulmonologist currently working on the Project Cure SMA Type 1 clinical trial in the Nationwide Children’s Hospital SMA Clinic, just received a special recognition award of excellence for Family Centered Care at Nationwide Children’s Hospital in Columbus, OH.
Dear friend,

Thank you for signing our petition, Petition-ToCureSMA.com, in favor of the bipartisan supported SMA Treatment Acceleration Act. With your help almost 50,000 voices are now speaking on behalf of the many children suffering from Spinal Muscular Atrophy (SMA), the #1 genetic killer of infants. We are extremely proud of this accomplishment and we could not have done it without your help. Your support to further SMA research has given us, as well as other families affected by this horrible disease, the hope that the life of our child will impact the future of so many children to come.

Many people have asked, “What will happen when the 50,000 signature goal is met?” The 50,000 mark has always been an arbitrary number; our main ambition was to reach as many people as possible. The more people who become aware of the devastation SMA causes and the hope that is on the horizon, the faster we will see an end to this cruel disease. Presenting an actual, growing list of constituents from the petition in support of the SMA Treatment Acceleration Act has been a powerful tool as they meet with the different legislators.

We started this petition in July 2008 as a way to do something after being told there was nothing we could do to save our daughter from SMA. This ‘something’ may not save Gwendolyn, but it has given us hope and eased the feeling of powerlessness. We can do something. We can take action. We can make a difference. By signing this petition, each one of you has made an impact. You have helped get the attention of Congress and you have helped build incredible momentum for this bill. The petition has been a success! Tens of thousands of people are now aware that this under funded disease needs attention.

And because of this petition’s success and in honor of the thousands of babies who have lost their fight with SMA and for those continuing to fight for their lives, we are proud to announce an increased goal of 100,000 signatures. 50,000 signatures are excellent, but 100,000 will be exceptional.

Thank you for all of your support,
Bill, Victoria, and Gwendolyn Strong.

New Care Booklet from Families of SMA

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

“If you have just learned that your baby has Spinal Muscular Atrophy, we know this can be a difficult, confusing, and painful time. Families of Spinal Muscular Atrophy is an organization dedicated to helping parents, like you, by offering support and advancing research. We’re here to help you.”

The topics review the basics of the main care options for newly diagnosed SMA Type I:

• What is Non-Invasive Respiratory Care
• What is Invasive Respiratory Care
• What is Palliative Care

And, where you can go for support and guidance. This booklet was produced with the kind support of the Angel Baby Foundation.
Tropon SA extends its partnership with AFM and will perform efficacy trial of olesoxime in Spinal Muscular Atrophy. Orphan Drug Designation granted in US and SMA Phase 1b data to be presented at American Academy of Neurology

Tropon SA, a clinical stage pharmaceutical company developing innovative therapeutics for indications with under-served needs in neurology and cardiology, announced that it will receive EUR 6.7 million over the next three years from the Association Francaise contre les Myopathies (AFM), which will substantially cover the cost of the further clinical development of its lead drug candidate olesoxime (TRO19622) as a treatment for Spinal Muscular Atrophy (SMA). This funding will support an important clinical trial seeking to demonstrate the efficacy of olesoxime in SMA patients.

Tropon is currently undertaking a Protocol Assistance procedure with the EMEA in order to finalize the design of the clinical efficacy study and to determine the regulatory requirements for approval of olesoxime for the treatment of SMA. It is currently anticipated the trial will commence around the end of this year, subject to the outcome of the discussions with the EMEA.

“Tropon is very pleased to be renewing its long standing strategic partnership with the AFM for the continued development of olesoxime for SMA, which has been instrumental in advancing our SMA program” said Damian Marron, CEO of Tropon, “Olesoxime is uniquely suited for development for this devastating condition, for which no treatments exist today. This program is a key part of our ongoing commitment to rare and under served motor neuron diseases and this agreement underlines and strengthens our joint efforts to further the clinical development of olesoxime for SMA.”

Tropon further disclosed today that olesoxime has recently been granted Orphan Drug Designation (ODD) for the treatment of SMA by the US FDA, following the earlier, similar Orphan Medicinal Product designation by the EMEA. This is recognition of the potential of olesoxime for the treatment of SMA and brings a number of development, regulatory and marketing exclusivity benefits. Tropon has previously received orphan drug status for olesoxime for the treatment of Amyotrophic Lateral Sclerosis in the US and EU.

Olesoxime is the lead drug candidate from Tropon’s novel and proprietary cholesterol-oxime based pipeline of drug candidates that enhance the function and survival of stressed cells via modulation of dysfunctional mitochondria, through interactions at the permeability transition pore (mPTP). Preclinical studies have demonstrated that these compounds promote the function and survival of neurons and other cell types under disease relevant stress conditions (Bordet et al., JPET 322:709-720, 2007). Olesoxime has successfully completed a phase 1b study in SMA patients (see above), having previously completed phase I/Ib studies in healthy volunteers and ALS patients. These clinical trials demonstrated that the product is well tolerated, has an excellent safety profile and that once-a-day oral dosing achieves the predicted exposure level required for efficacy, based on preclinical models.

The American College of Medical Genetics Issues Guidelines on Carrier Screening for Spinal Muscular Atrophy

Dr. Thomas Prior from Ohio State University has written a set of guidelines regarding carrier testing for Spinal Muscular Atrophy that have been issued by the Professional Practice and Guidelines Committee of the American College of Medical Genetics. The guidelines were published in the journal Genetics in Medicine.

These guidelines recommend that SMA carrier testing should be offered to all couples having a baby regardless of race or ethnicity, as SMA is a common genetic disorder in all populations. Typically, tests to identify carriers of the gene responsible for SMA have been offered only to people with a family history of the disease.
Geron Receives FDA Clearance to Begin World’s First Human Clinical Trial of Embryonic Stem Cell-Based Therapy

Geron Corporation announced on January 23rd that the U.S. Food and Drug Administration (FDA) has granted clearance of the company’s Investigational New Drug (IND) application for the clinical trial of GrNOPC1 in patients with acute spinal cord injury.

This will help pave the regulatory path for Spinal Muscular Atrophy.

The clearance enables Geron to move forward with the world’s first study of a human embryonic stem cell (hESC)-based therapy in man. Geron plans to initiate a Phase I multi-center trial that is designed to establish the safety of GrNOPC1 in patients with “complete” American Spinal Injury Association (ASIA) grade A subacute thoracic spinal cord injuries.

See the status update online and in this issue of the FSMA Stem Cell Motor Neuron Program.

Families of SMA to Launch New Medical Advisory Council (MAC)

Families of SMA will expand its role in SMA medical affairs at the national level through a new Medical Advisory Council (MAC). The MAC will set the agenda for proactive, creative, and collaborative leadership on issues aimed at improving the quality of medical care for all those affected by SMA.

The new Council will focus on educating families, health care providers, and the public about SMA; expanding SMA standards of care; and translating positive research results into clinical practice.

Dr. Mary Schroth, a leading pediatric pulmonologist will serve as Chair for the new Council. Mary is Associate Professor of Pediatrics and Director of the Pediatric Pulmonary Center Grant at the University of Wisconsin Children’s Hospital.

Clinical experts in the following fields will be considered for membership on the MAC:

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

The new members of the MAC will be announced at the SMA Family and Professionals Conference in June.
Iowa Chapter

The Iowa Chapter’s 11th Annual BeaverDash Walk-n-Roll for SMA raised $25,000 and drew a crowd of over 1,000 participants plus had over 100 volunteers!

Long Island Chapter

The Demon Knights Motorcycle Club held a fundraiser in honor of Max Rubenstein and the NYC/Long Island Chapter of FSMA and raised a total of $3,100. They then drove their motorcycles in freezing temperatures to hand deliver the check to our chapter and to meet and spend time with the kids. All of the kids really enjoyed and appreciated meeting with the Demon Knights and hope to work with them again in the future. They are a very special group of individuals.

The NYC/Long Island Chapter is starting to work on their annual fundraising events. Our Annual Walk N Roll will be held in Long Beach on Saturday, June 6, 2009 and will honor Owen Schuler. Online registration is available on the FSMA website.

In January we held our first ever Holiday Celebration. The children and families who attended were greeted by Santa, the local Firemen and also the Demon Knights. The FDNY donated T-Shirts and the New York Yankees donated bucket hats and stickers. The kids were also surprised with a free Yoga Class which everyone enjoyed. Many thanks to the Tansey family for opening their home to us for this event.

Also in January we held a chapter meeting and were given a presentation from the Make A Wish Foundation. All of the families enjoyed Ilene Mandel taking time to meet with us.

Our Fundraising Coordinator, Michele Erwin, is working on a fundraiser collecting Soda Can Tops. We can redeem these Soda Can Tops for money. This fundraiser was brought to our attention by one of the SMA Grandfathers. Please try to save up your soda can tops. We intend on collecting them at our Chapter meetings so please hold onto them and try to bring them to the meetings. Our next chapter meeting will be sometime in March. Check our chapter website for more details.

For any information regarding the NYC/Long Island Chapter please email Debbie Cuevas at longisland@fsma.org.
The Kansas City Chapter of FSMA held its 8th Annual RoeFest Cure SMA Race-n-Roll 5K/1 mile Run/Walk on the first Saturday this past October. We had beautiful weather and it was a great event! Many chapter families from over 5 states were joining in on the fun. We would like to thank the chapter families for their participation and hard work and especially thank all of the sponsors, volunteers, donors and participants for making the event a huge success! We raised over $40,000 for FSMA!

In November, Thomas Winkler with the University of Missouri AIPEL group, organized the First Annual “Battle of the Bands to Help Cure SMA”. The event was held at the Blue Fugue in Columbia, Missouri and 6 bands battled it out for #1 band status! This event was in honor of Thomas Winkler’s friend Annie Hopkins of Chicago who has SMA. This was a great first time event and $650 was raised during the evening!
The Michigan Chapter of FSMA held its 5th Annual Flippin’ for a Cure Hamburger dinner on Friday, November 3, 2008 at the VFW Post 4090 in Portland, Michigan. Once again many families and friends gathered for great food, raffles and entertainment. Thanks to the VFW’s use of their facility and their wonderful cooks, the fundraiser was a huge success raising $4,000.

Mallory Armbrustmacher (SMA type II), the 13-year-old daughter of Ken & Cindy Armbrustmacher, addressed a group of insurance professionals at her Dad’s office. The Mid-Michigan Chapter of the CPCU Society for the third year in a row chose Families of SMA as their December Good Works Fundraising Project. They asked Mallory to return after speaking to the group last year. Mallory once again inspired those in attendance by sharing her experience of living with this disease, and gave an update on the challenges she overcame this past year.

Kenneth Hobby and Jill Jarecki also addressed the group via conference call explaining the ways in which FSMA uses contributions for research and family support. Thanks to the support of the Mid-Michigan Chapter and the generous support of its members, the Chapter raised over $3,000 for FSMA.

The Mary Spitzley family celebrated Christmas together in Westphalia, Michigan. For entertainment this year they held a silent auction with donated crafts and services. Half of the proceeds from the auction were donated to Families of SMA in honor of great-grandchild, Mallory Armbrustmacher. $400 was raised for Families of SMA.
Dear Friends,

As you may know Jim Gaudreau, president of the NE chapter and Paula Barrett, secretary of the NE chapter announced on November 1st, 2008 that they are stepping down from their positions on the NE Chapter level.

On behalf of all the families in New England and beyond I’d like to take this opportunity to thank them both for their dedication and service and wish them well in the future.

Paula is a parent of a teenager with SMA. In addition to the tremendous commitment that this entails she has also devoted much of her time to the SMA community, at the local and national levels. During her time as both a member of the executive board of FSMA National and secretary of the NE Chapter she has contributed greatly to our success. For example, this past August marks the 11th anniversary of the Andover Country Club Golf Tournament, which she and her husband Mike started. This event brings in approximately $40,000 each year!

Jim joined the NE chapter shortly after his daughter was diagnosed. His dedication to our cause became immediately apparent and in a short time he became President of the NE Chapter. During the 5 years of his presidency he introduced Comedy Night and 5k/Autumn Walk & Roll fundraisers which helped the New England chapter raise $1.2 Million. Jim also sits on the executive board of FSMA National. Every one of us probably has a story to tell about how Jim has helped us in one way or another and for him to do so much in such a short amount of time is amazing. Jim’s tireless efforts have helped raise the profile of our chapter to new heights.

How many hours have you both spent trying to make sure someone gets the equipment they need, or answering emails, or arranging the details of a fundraising event? Thank you for going those extra miles! When your eyelids are so heavy from exhaustion and the days roll into nights, you put yourself and personal life on hold. I know that this letter just barely skims the surface of all that you both have accomplished for the SMA community. Thank you Jim and Paula for your sacrifices, you have propelled us closer to cure than anyone can imagine. For this, from the top to the bottom of our hearts, we are grateful.

Happy Holidays and warm wishes to all.

Michelle Rollinson
I am happy to announce that the South FL FSMA Chapter is active. We are so excited to be a part of this. We are looking forward to a successful Chapter to have a network of family support, raise huge amounts of funds to find a cure for SMA and raise awareness about SMA in S. Florida!

We are also in the beginning stages of planning our Walk & Roll to cure SMA sometime in Spring. We are planning on having it coincide with the grand opening of a new all accessible play ground in Davie.

We are very excited to be able to start contributing in a large way towards research for a cure, getting families connected and offering support. We welcome any and all help in planning events.

Most of our events and meetings will be in Broward County, but I can put together meetings in other cities if requested. We can also help facilitate smaller fundraisers in your city if requested.

Together we WILL find a cure!

Our chapter is also working on a few other events yet to be announced so please check the chapter web page periodically.

Again, we are always welcome to new families becoming involved. The more help- the quicker we may be to the treatment and cure for SMA.

We look forward to reuniting with area families at the conference and hope to meet some new ones as well.

---

Welcome Wisconsin Families,
Happy New Year! We hope that 2009 brings you health and happiness.

As the beginning of a new year brings about resolutions and a new start we would like to invite you all to be a part of the fresh start for the WI chapter of FSMA and we want your input!

What would you like to see the chapter doing for you?
Would you be willing to volunteer for fundraisers and events?
What type of events would you be interested in participating in?
Would you be able to get together for a meet and greet with other families and Dr. Schroth in Madison? Possibly in June before the FSMA conference?
Would you be willing to be a part of a mentoring program for newly diagnosed WI families?
Please let us know any thoughts or ideas you have.

Sincerely,
Jenny Imhoff, Kate Vogedes, Laurie King & Scott Sheppard…
Your WI Chapter Executive Committee
E-mail: wisconsin@fsma.org

---

The Burbank Walk-N-Roll
Thank you all for coming on Sunday, November 16th. For those who could not make it because of the fires, you were in our thoughts and prayers. We appreciate all your love and support. And although there is no treatment now for SMA, there will be soon - because with every step and every roll, we are closer to a cure.

With over 500 registered, and $35,000 raised, we have a lot to be grateful for. Thank you to: all the families living with SMA who set up online fundraising pages, and formed teams of walkers - you are amazing! (Below is a list of top fundraising teams). To the Voosen family who was there with the event planning from start to finish; St. Francis Confirmation Students who helped with set-up, games and clean up; Foothill Civitan Club of Burbank for help with registration (and always being there), to the Providence High Flag Team for your wonderful entertainment; and to the Smentek and Henderson Families. This event would not be possible without your help.

Chris and Jen Elsner along with their family and friends brought in the New Year for a great cause, Spinal Muscular Atrophy, with a New Year’s Eve party. Fifty guests attended the fundraiser that included lots of food, drinks, a raffle and tons of fun for a $50.00 entry fee. Raffle prizes included ½ ctw. diamond earrings, a $100.00 gift certificate to Weissberger’s, and many other assorted prize packs. $500.00 has been donated to FSMA in honor of their daughter Peyton Lola who was recently diagnosed with type 1. Thank you Chris and Jen!
The results are tallied, and following are the top teams:

- Tyler’s Tigers $9,425 (thank you Zappia Family for online contributions of $2,976.00!)
- Team Meigs - Walking in memory of Alexandra $7,320.00
- Luke’s Zoo Crew $4,696 (Thank you Michael Weinberg for online contributions of $2,976.00!)
- Faith For Phillip $1,600.00
- Madison’s Magic $1,120.00
- Rockin Robyn’s Team $1,120
- Bulldogs (for Dominick) $1,045.00
- Jordan Balla $930.00
- Kim Beam $735.00
- Vic’s Posse $705.00
- St. Francis Xavier Confirmation Team $660.00
- Jordan’s Cool Crew $500.00
- William Miller in memory of Derek $500.00

The Top online fundraising team for the 2008 Cure SMA Walk-n-Roll in Riverside went to Steven Combre from Corona, CA. Steven also earned community service hours from his school by participating in the walk. Thank you Steven Combre!

Gradience Effect (find them on Myspace) and October 67 were our musical entertainment for the day. We were privileged to have these bands donate their time for us and play. Next year we will place the registration table away from the speakers.

2008 Cure SMA Sponsor’s:

- Alta Dena
- Altura Credit Union
- Better Life Mobility
- Eagles ‘506’ Bingo Committee & REAC
- Jadtec Security **
- K&N Customs Full RV Service
- Maxim Health care
- Pulmocare Respiratory Services
- Superior Concrete & Masonry
- Woodcrest Chiropractic Offices

We also received a few contributions from other generous places in the Inland Empire:

- Starbucks donated coffee and cups
- Agape Home Care, INC- cookies, pastries, etc...
- Malcolm Smith Motorsports- water bottles
- Aunt BeeBee the clown returned from last year and brought with her a sponsor from the Eagles “506” Bingo & REAC committee.

Thank you all so much!

Pictures of the Cure SMA Walk-n-Roll of 2008 in Riverside, CA are available online…http://picasaweb.google.com/curesmal/CureSMAWalk2008#

The Greene’s- Kenny, Christy, Laura, Sara Rose- SMA 1.
Our Goal for the Quarter Century “Wall of Strength” is to Raise a Quarter of a Million Dollars!

Please visit the FSMA website www.curesma.org to see the complete list of pictures, donors and their messages.
25th Anniversary
WALL of strength
Please visit the FSMA website www.curesma.org to see the complete list of pictures, donors and their messages.
Home Free Home -
"Tearing down barriers and building freedom"

Home Free Home is a national nonprofit organization dedicated to providing pro bono architectural design services to people who need to remodel their homes to accommodate a disability.

Anyone with any type of permanent physical disability can apply by e-mailing us at info@HomeFreeHome.org.

Founded in 2006, HFH is a group of volunteer architects, contractors and university students. Our goal is to create barrier-free designs for small construction projects, such as ramps, accessible bathrooms and kitchen remodeling.

Home Free Home:
• is an ALL volunteer organization
• relies on local volunteer architects and university students
• is expanding to a national level
• architects have already volunteered in New York, Texas, California, Georgia and Florida
• has a wait list of families on Long Island in immediate need

Their website is www.homefreehome.org.

The Center for Courageous Kids

The Center for Courageous Kids is hosting a SMA weekend April 17-19, 2009. The CCK is a “world class medical camping facility in the rolling hills of Scottsville, Kentucky providing a cost-free, safe, and fun camping experience for seriously ill and disabled children and their families.” For more information go to their website at: www.courageouskids.org.

Families of SMA Would Like to Honor the Fantastic Contributions of Karen Burton and Richard Grossman to the SMA Community

Karen Burton held the position of Recording Secretary for the Board of Directors of Families of SMA since her son was diagnosed with SMA. Karen has two sons - Jonathan, who has SMA Type II, and Jeffery. Karen helped Families of SMA manage our growth from just a few chapters and several hundred supporters to where we stand today – a global community with over 50,000 members and supporters and over 25 chapters. Karen was also the creator of the “Kids Carnival” that is now held each year and is a special highlight at the national conference.

Richard Grossman has been on the Board of Directors since its inception. Mr. Grossman was FSMA’s General Counsel and assisted the organization with many complicated agreements and contracts. Richard was instrumental as Families of SMA established relationships with universities and pharmaceutical companies around the world.

Both Richard and Karen gave all their time and efforts as volunteers. We thank them both for helping our community with their amazing talents that they both contributed to help move Families of SMA forward and closer to a treatment and cure.
### Alabama Chapter (since 2005)
PO Box 71918  
Tuscaloosa, AL 35407  
(205) 979-6493  
Rhyann Granger, President—alabama@fsma.org

### Arizona Chapter (since 1997)
85 W. Combs, Ste #101, PMB 410  
Queen Creek, AZ 85240  
(480) 752-8093  
Karey Kaler, President—arizona@fsma.org

### Chesapeake Chapter (since 1995)
PO Box 354  
Cockeysville, MD 21030-0354  
(800) 762-0113 or (410) 561-9355  
Barb Trainor, President—chesapeake@fsma.org

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

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

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

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

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

### Long Island New York City Chapter (since 2006)
PO Box 322  
Rockville Centre, NY 11571  
(516) 214-0348  
Debbie Cuevas, President—longisland@fsma.org

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

### Michigan Chapter (since 2004)
PO Box 500, Ada, MI 49301  
(517) 669-1665  
Ken Armbrustmacher, President—michigan@fsma.org

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

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

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

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

### Pacific Northwest (since 2006)
PO Box 173  
Galvin, WA 98544-0173  
(206) 284-1888  
Rick Jones, President—pacwest@fsma.org

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

### Rocky Mountain Chapter (since 1998)
PO Box 1913  
Arvada, CO 80001  
(970) 349-0481 or (877) 591-4023 (toll free)  
Loree Weisman, President—rockymt@fsma.org

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

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

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

### Tennessee Chapter (since 2001)
PO Box 7025  
Knoxville, TN 37921  
(865) 945-7636  
Lise Murphy, President—tennessee@fsma.org

### Texas Chapter (since 2005)
PO Box 627  
Hutto, TX 78634  
(512) 846-2239  
Eric Ostermayer, President—texas@fsma.org
Utah Chapter (since 2009)
Park City, UT
(435) 901-4461
Shane Barber, President—utah@fsma.org

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

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

NEW

CHAPTERS ARE REACHING OUT TO COMMUNITIES ALL ACROSS THE COUNTRY.
Families of SMA currently has over 26 chapters in the United States.

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

CHAPTEARS ARE REACHING OUT TO COMMUNITIES ALL ACROSS THE COUNTRY.
Families of SMA currently has over 26 chapters in the United States.

Do you live in one of the following states?

- Montana
- Idaho
- Nevada
- New Mexico
- Wyoming
- North Dakota
- South Dakota
- Nebraska
- Oklahoma
- Arkansas
- Missouri
- Mississippi
- Georgia
- South Carolina
- North Carolina
- West Virginia

SUPPORT your community.
Fundraising for RESEARCH.
HOPE for families.
Begin to make a difference today.

Contact Barbara Trainor at chapters@fsma.org to receive more information on how to start a chapter in your state.
Mike Morris Supports Families of SMA

Design Home 2008 to Support Spinal Muscular Atrophy Research.

Families of SMA is proud to have been the featured charity and benefactor for the “Design Home 2008” project. A special “thank you” goes out to Tom Hennessy of 5 Eyewitness News (KSTP-TV), who aired 192 promotional spots from July through September.

Go to Families of SMA YouTube page to see the video.

We would also like to thank: Al Maas and his staff of A. Maas Construction, the “Design Home 2008” home builder, and former Minnesota Viking and current KFAN radio host Mike Morris for his continued dedication in helping to find a treatment and cure for Spinal Muscular Atrophy.


Thank you for your support and efforts in creating more awareness about SMA and the work Families of SMA continues to do!

Family Guide to the Consensus Statement for Standard of Care in Spinal Muscular Atrophy

This guide in concert with the full set of guidelines published in the “Journal of Child Neurology” allows SMA families to gain better access to a greater number of clinical care options. Doing so will be essential to the ongoing efforts at Families of SMA to enable clinical trials for SMA, where standardized clinical care is essential to success.

The new publication is designed to guide patients and families in their discussions with doctors and health-care specialists. The committee emphasizes that these guidelines are only suggestions and should not be considered absolute requirements for care. The Family Guide covers recommendations on these topics:

1) Confirming the diagnosis of SMA
2) Managing breathing
3) Managing eating and nutrition
4) Managing movement and daily activities
5) Preparing for illness

Please see our web site to download a copy of the full guide, or email info@fsma.org or call (800) 886-1762 and we will mail a copy to you.

Join the FSMA E-List!

With each research breakthrough or other important event on the road to a cure, Families of SMA sends an e-mail to everyone on its e-mail list. If you would like to join our e-list please e-mail info@fsma.org (Use “subscribe FSMA” in the subject line.) with the following information: Name, E-mail address, City and state or country of residence.
Indianapolis, IN, October 15, 2008.

After many months of anticipation we are pleased to announce the unveiling of the new International Spinal Muscular Atrophy Patient Registry Website! The Website now allows individuals to participate in the Registry completely through on-line interaction including registering, completing questionnaires, learning about and signing up for new research study opportunities and completing annual information updates.

We are excited about the enormous improvement in the speed with which we will be able to communicate with both the research and family community and hope that you will take a moment to visit our new website. Please email any comments on the SMA Registry to: smareg@iupui.edu

The SMA Registry is currently helping to recruit for several studies, including:

• Pilot Study of Biomarkers for Spinal Muscular Atrophy (SMA) (BforSMA): The purpose of BforSMA is to identify potential biomarkers (measures) that can be used to evaluate SMA disease severity. A “biomarker” is something that can be measured to assess disease and treatments (cholesterol level is a biomarker for heart health).

• The Role of Motor Unit Number Estimation (MUNE) in Adults with Spinal Muscular Atrophy: MUNE is a research study that follows the course of motor neuron loss in adults with SMA using the electrophysiological technique of motor unit number estimation (MUNE).

• Prospective Controlled Trial of Valproic Acid in Ambulant Adults with Spinal Muscular Atrophy (VALIANT SMA) Study: VALIANT SMA is a research study to assess the efficacy of oral Valproic Acid (VPA) in adult patients with SMA.

• Outcome and Effect of Pregnancy in Spinal Muscular Atrophy: This is a research study of the effects of pregnancy and delivery on adult women with SMA.

• Phase I/II Trial of Valproic Acid and Carnitine in Infants with Spinal Muscular Atrophy Type I (CARNI-VAL Type I): Carni-Val Type I is a research study to evaluate the combination of Valproic Acid (VPA) and L-Carnitine for the treatment of SMA in infants with SMA type I. These clinical trials are being conducted at several universities across the United States and one location in Canada.

To find out more about all of the open studies the SMA Registry is helping recruit for, please visit https://smaregistry.iu.edu/Participant/researchStudies.asp. If you are interested in any of the studies listed, you can join the Registry and request to have your contact information forwarded to the researchers conducting that study.

About the Registry:
The International Spinal Muscular Atrophy Patient Registry (the Registry) was founded in 1986 at Indiana University. The Registry connects patients and families interested in participating in research and researchers interested in studying SMA. The Registry contains information from over 1,600 families and over 1,700 individuals with SMA from all over the world and continues to grow. The Registry has helped recruit participants for clinical trials and has provided data for important SMA research studies. The Registry helps centralize information on this rare genetic disease, provides families a way to learn about research studies and provides researchers a way to find research participants.

Individuals and families affected by SMA are invited to join the Registry. Participants are asked to complete questionnaires about the symptoms, treatment, medications, and other experiences with SMA.

Participant information is stored in a secure database. Researchers who are interested in studying SMA can request two types of data from the Registry, de-identified information and identifiable information. De-identified information does not contain any names or personal identifiers, and can be given to researchers without having to contact Registry families. Identifiable information includes information that can identify you and will never be released without getting your written permission to do so. Identifiable information includes data such as names and dates of birth. Some researchers may also request contact with families to obtain specific information or to request participation in a research study. In these instances, the Registry will contact each potential participant to ask if they are willing to share their identifiable information for a research project.

In 2008 the Registry joined the group, Translational Research in Europe for the Assessment and Treatment of Neuromuscular Diseases (TREAT-NMD) in a global collaboration to further the research goals of the neuromuscular disease community.

The SMA Registry is supported by the Patient Advisory Group of the International Coordinating Committee for SMA Clinical Trials which includes Families of SMA, Fight SMA, Muscular Dystrophy Association, SMA Foundation, and other SMA advocacy groups.
uniting families with researchers to find a treatment and cure for SMA

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

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

This project is supported by the Patient Advisory Group of the International Coordinating Committee for SMA Clinical Trials, which includes Families of SMA, FightSMA, the Muscular Dystrophy Association, the Spinal Muscular Atrophy Foundation, and other SMA advocacy groups.

For more information call 1-866-482-0248
e-mail smareg@iupui.edu https://smaregistry.iu.edu
MEMBERSHIP form

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

Suggested Annual Donation for Membership
Family $30
Professional $35
International $40

* We ask for a donation 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 these publications provide valuable information and useful support.

Payment Method
☐ Check
☐ Money Order
☐ VISA
☐ Mastercard
☐ Discover

$ Amount enclosed or to be charged

Credit Card #
Expiration Date
Name on card
Signature

Memberships can be made online at www.curesma.org
Dear Families of SMA,

We are sending you a picture of our little girl who was afflicted with SMA Type I. We thought you could picture her in the Loving Memories section of your booklet. Her name is Lanae Sue Martin 11/20/07-8/10/08.

We so much appreciate your efforts to find a cure for this horrible disease. It was so hard for us to watch our baby grow weaker and weaker. She passed away peacefully in her daddy’s arms on a Sunday morning. Her main weakness was in her back and she also choked often her last month here.

On her memory card at her funeral we had the following verse- “To my family and friends- Just remember God is Love and he lives in heaven above. Always pray to Him and read His Holy word. It matters not what life may throw; whether storms or roses grow, just hold His hand and think these words...My God is love.”

It’s so easy to get bitter when hardships come; but God does not want us to succumb to that. He wants us to look to Him in faith believing their souls are now resting in Heaven.

Our Prayers to All SMA Families-

Michael and Melanie Martin and Weston-12, Kendon-9 and Marita-6 Martin

P.S. Lanae was our 3rd child affected, out of 6 children total.

Joseph Dominic Lillo

June 4, 2008 – August 29, 2008

Little I knew that morning, God was going to call your name, in life we loved you dearly, and in death we do the same. It broke our hearts to lose you, you did not go alone, for part of us went with you, the day God called you home. You left us beautiful memories, your love is still our guide, and though we cannot see you, you are always by our side. Our family chain is broken, and nothing seems the same, but as God calls us one by one, the chain will link again.

Much love – your friends.

Always,

Dominic, Rose & Francesca Lillo
**Loving memories**

**Francisco Rodriguez Jr.**

January 2, 2008 – August 2, 2008

I want to tell you thank you for the beautiful angel that you sent to our home and the precious card. It melted my heart when I opened the box. The angel sits in my angel’s corner next to his pictures and every time I look at it I actually feel a little comfort because it makes me remember that my son is being taken very good care of in Heaven. He is in God’s hands now and I know one day I will be there to hold him in my arms. Thank you and everyone at FSMA you all are a blessing from God as well as our precious angels here on Earth and in Heaven.

Angela Lopez

---

**Brendon Jordan Dupree**

July 26, 1990 – February 2, 2009

Brendon Jordan Dupree, 18, of Louisville, KY, passed away Monday. Brendon suffered from Spinal Muscular Atrophy Type I.

Brendon is survived by parents, COL Ron and Shana of Louisville, KY; siblings, Rachael and spouse, Capt. Brett Taylor, of Williamsburg, VA, and Jake and Evan of Louisville, KY; grandparents, Gerald and Sarah Dupree of Huntsville and a host of uncles, aunts, cousins and friends. Due to lifelong military assignments, Brendon has a host of friends and supporters throughout the world.

Brendon was preceded in death by his great-grandparents, Claude and Zuba Dupree and Carl and Janie Yeager, great-aunt, Betty Turner and great-uncle, Donald Dupree.

He was laid to rest in Huntsville, Alabama in his father’s home town.

---

**Kelly Grace Durham**

December 5, 2003 – July 7, 2004

Kelly Grace Durham, “Baby Girl”, was born December 5, 2003. She had the most beautiful blue eyes and the longest eyelashes you have ever seen. On April 5, 2004 at 4 months old, she was rushed to the hospital. About 2 weeks and many tests later, she was diagnosed with SMA Type I. It was very devastating to all of us. We had no idea what Spinal Muscular Atrophy was much less could we believe “Baby Girl” was going to die from it. She was hospitalized and never came back home. If you could look past the breathing tube, you never would have known she was sick because she always had a smile for everyone. She fought the devastating illness for 3 months, each day batting those beautiful eyes and giving us those big smiles. On July 7, 2004, at 7 months old, she lost her battle with SMA. The whole time she was sick, we kept hoping for a miracle. She was only here for 7 months but she brought us more joy and showed us more love than most people experience in a lifetime. We think of “Baby Girl” each day and love and miss her deeply.

Kelly’s Mommy & Daddy & Nana & PawPaw Durham
Good Morning,
Weighing 7 lbs. 3 ozs. 20 inches long, Eden Kathleen Foley arrived June 15, 2007 to very proud first time parents, Shana and Nathan; first grandchild of the Shannon/Foley union.
During Eden’s eight week wellness check-up, her doctor became concerned with her lack of reflexes and her floppiness. Eden was scheduled for an appointment at Cincinnati Children’s Hospital with the neuro-muscular team. She was evaluated and officially diagnosed August 27, 2007 with Spinal Muscular Atrophy Type I.
After receiving this diagnosis, it was amazing that no one knew what it was. My daughter researched the disease, thus beginning everyone’s education as to Spinal Muscular Atrophy. When told Eden would never crawl, walk, sit up, or even hold her head up, have swallowing and breathing problems and would probably not live to be 2 years old, the families and their friends were devastated. People began prayer chains thru out the United States; my daughter’s co-workers established Eden’s Garden, a fund to help with expenses, my son-in-law co-workers gave gift cards, gas cards, etc. to help with the expenses. All the while the SMA education continued.
As my daughter contacted the FSMA organization and learned of other families with SMA children, the education, resources and other information filtered in to our situation. The FSMA and the families there became a remarkable source of information and life time friends were made.
The SMA journey became very real. The nasal and gastric feeding tubes, suction machines, bi-pap breathing machines and the masks that go along with them, oxygen tanks, o.t., p.t., nutritional changes, and frequent hospitalizations became a part of Eden’s life. The only time Eden really cried about anything was when she had a wet diaper and when the cough assist machine was used. She cried, we cried.
Early September, Hospice of the Bluegrass became a part of Eden’s life, also. There were other children in Lexington with SMA, so as hospice came on board, education and resources were again in play. As Eden was admitted several times to CCH, we became close with other SMA children from Ohio and Northern Kentucky. At one time, 2 children from Lexington and 1 from Northern Kentucky were on the same floor next door to each other. The families really bonded together as we each learned of the other’s progression into the SMA world. This sort of communication has continued to this day. During her fifth CCH, Eden sadly became Angel Eden, January 18, 2008. When I returned to work in late January, a fellow co-worker told me a “pennies from heaven” story. I began collecting pennies at first, then realized a nickel was five pennies, a dime ten pennies, etc. The first part of April, when news became available of the Lexington Walk/Roll benefit for FSMA, my fellow court services and juvenile division co-workers set up bins with Angel Eden’s picture and the SMA story. People began bringing their change containers from home for this project which has been titled “Angel Eden’s Pennies from Heaven” to help her past, present, and future friends with SMA research. Please accept the enclosed check for $664.96. We hope to have more pennies to send next year.
I believe education is the key to more research and legislation. The more we discuss SMA with people who have never heard of the disease, the more people become aware. We tell someone, and then they tell someone. This was evident to me when people from all walks of life contributed to the Angel Eden Fund.
Missing Angel Eden’s big blue eyes and her infectious smile, GREAT. Being blessed with her in my life, even though for only seven short months, PRICELESS.
Sincerely,
Angel Eden’s granny “P”
Penny Shannon

The picture enclosed is of me and Angel Eden the night before she passed away.
Daniela Borisova Tilee
February 26, 2008 - September 5, 2008

Our daughter Daniela was born on the 26th of February 2008. I wanted to have a baby so much. Everything was going well until I noticed how difficult the bathing was- she became so flaccid. And we also realized she didn’t move her legs at all. Deep in my heart I felt something was going on. She was two months old when the genetic tests confirmed she had SMA Type I. We knew nothing about that disease. We got the info from the internet because we are from Bulgaria and the Bulgarian textbooks didn’t give us enough information. She passed away in the hospital on the 5th of September 2008 at 8:55am after a long fight. I wanted to believe she wasn’t in pain. She had pneumonia. That was her first time in the hospital after she was diagnosed and I hoped she would get better. I thought she would manage. To hear that your baby will die for sure is the most horrible thing that could happen to any parent. I have no words to express my real feelings bit I am sure that an SMA parent can understand me. You ask yourself everyday why? Why my lovely baby? This is the most terrible disease I can think of. There is no chance, no hope. For four months I knew I will lose her one day. You cannot be prepared for the death of your baby. She was a real fighter. She fought for her life for four long hours. This was a real agony. She stopped breathing so many times but her heart kept beating and somehow she took a breath once again and once again. I wanted this to end as fast as possible. This was a real torture for her. I wanted so much to help her somehow and I stayed there so helpless. I miss her every second. I will never forget her. I hope she is an angel now and we will meet again one day. I read so many stories about SMA children. I want to say courage to all parents of SMA children. It is hard to believe that in the 21st century there is no cure for your child. I can only hope she is better now and that she is at the most peaceful and beautiful place. I don’t know this for sure but I hope so. We love you Danny!
Danella “Danie” Cruz  
_Saturday, September 15, 1997 – December 13, 2008_

Danella “Danie” Cruz was called home to the Lord on Saturday, December 13, 2008. She was born on September 15, 1997 in Charlotte, North Carolina, she was in the 6th grade. Mrs. Alice Jones was constantly by her side. Prior to graduation from the 5th grade at Sandy Grove Elementary School in Raeford, North Carolina she was awarded the President’s Award for Educational Excellence on June 5, 2008: this from the President of the United States. Danie was on the honor roll in both Sandy Grove Elementary School and East Hoke Middle School. Her favorite subjects in school were math, reading, computer science and she was in the chorus when she attended both schools. While in the hospital, Danie was elected Vice-President of the Sandy Grove Elementary School Student Council, she won by one vote.

After she was born, she attended Lewis Chapel Missionary Baptist Church in Fayetteville, North Carolina for seven years. Reverend Dr. John D. Fuller, Sr. was the Pastor. She then attended Rockfish Church in Raeford, North Carolina from the age of seven, 2004, until she was called home. Pastor Dan Stanley is the Pastor at Rockfish Church. Danie accepted Jesus Christ as her personal Savior in 2007 and her father, Reverend Benny Cruz had the privilege of baptizing her. She loved the Lord and she hated missing church. At Rockfish Church, she was a member of Pastor Nathan’s Sunday Morning Class.

Danie was an exceptional young lady and she was blessed by the Lord with a contagious personality and a magnetic attitude. Everybody who came in contact with Danie left her side with a piece of her happiness and some of her positive attitude. On more than one occasion her parents had been told by various people, “You know, I was having a bad day and bad times. After meeting your daughter and seeing her positive outlook on life I said to myself “Why am I complaining? I look at her and she gives me strength to go on.”

At home, she was a bundle of happiness. She had a sense of humor that was second to none. She was constantly joking and “messing” with her father. She would hit her father, when her mother was not looking, and when he retaliated she would call out “Momma” daddy is messing with me.” She enjoyed singing in the shower and also in her bed. Her favorite show was “Hannah Montana and the Cheetah Girls.”

Danie is survived by: her father, Reverend Benny Cruz; her mother, Donella F. Cruz; her older sister, Maritza Scott of Tampa, Florida; her brother, Ben Cruz III of Houston, Texas; her grandmother, Cora Lee Carroll of Dillon, South Carolina; her grandmother, Teodosia Cruz of Chicago, Illinois; her godparents, Joseph and Martha Levister of Fayetteville, North Carolina; her godparents, Curtis and Darlene Lawrence of Fayetteville, North Carolina; aunts, uncles, nephews and many friends; specifically, Brianna, Caitlin and Richard.

Jake Thomas Faso  
_May 27, 2008 – December 14, 2008_

To Whom It May Concern,

Please accept this donation on behalf of “The Samuel Group of Companies” in memory of my nephew and Godson, Jake Thomas Faso. Our Jake “Buddy” passed away peacefully from SMA on December 14, 2008 at the age of 6 ½ months. Although Jake was only with us for a short time, he taught our family more about faith, courage and love than most people will during their lifetime.

We hope that this donation will help continue the research necessary in finding a cure while at the same time help in assisting other families touched by this most devastating disease.

Sincerely,

Paula Orlowksi
Jay Randall Marlow

Forever 7 Months
A Poem for Angel Jay
From Your Mommy – With Love

My sweetest angel, baby Jay
So innocent and pure
Born with a disease
For which there is no cure.

It’s SMA Type I
The doctors said that day
What is in our future
It’s hard for them to say.

They tell us you’re very weak
Giving you six months to a year
Just take him home and love him
They tell us through our tears.

Your beautiful little body
You can just barely move
Your mind and spirit strong
How often you will prove.

Your eyes take everything in
As we read, sing and play
Your eyebrows, so expressive
Smiles brighter than any day.

Your decline is much too rapid
We still don’t want to believe it
You’re growing body’s failing you
But how amazing your fighting spirit.

Your love for life is strong
But you said you’re ready, though
We hold you, love you, say goodbye to you
We had to let you go.

It’s only been two Mondays
Since your spirit passed away
We will be ever thankful
For the beautiful gift we made.

My sweetest angel, baby Jay
Still innocent, but now free
The angels finally came for you
In our hearts you’ll forever be.
LOVING memories

IN MEMORIAM
COURTNEY BUSS
JUNE 7, 2008 – DECEMBER 1, 2008

IN MEMORIAM
THOMAS MICHAEL WILDES
JANUARY 18, 2000 – MARCH 30, 2001

IN MEMORIAM
LOREN PICINICH
JUNE 24, 2008 – DECEMBER 15, 2008

IN MEMORIAM
HANNAH JEAN CAMPBELL
JANUARY 18, 2008 – OCTOBER 18, 2008

IN MEMORIAM
ISABELLA ROSE TROUP
NOVEMBER 9, 2006 - MARCH 8, 2007
IN MEMORIAM
MICHAEL UNDERHILL
DECEMBER 14, 2007 – DECEMBER 17, 2008

IN MEMORIAM
ISIAH SANTANA

IN MEMORIAM
ALEXANDRA NINA MEIGS
OCTOBER 24, 2007 – JANUARY 1, 2008

IN MEMORIAM
SADIE MAE SOUTHCO T T
SEPTEMBER 13, 2006 – OCTOBER 15, 2006
FSMA merchandise

NEW Items!

Baseball Cap | $10
New FSMA Logo.

Canvas Tote | $15
Choose either Flower or Seascapes design.
Artwork by Katie Gardner 11/89–4/03.

Cotton Tote bag with Zipper | $17
Together design.

FSMA Car Magnet | $5
1 design.

Travel Mug | $10
New FSMA Logo.

Sport Bottle | $5
New FSMA Logo.

Cure SMA Bracelet | $2
Available in small or large.
Created in honor of Steven Potter.

Families of SMA T-Shirt | $12
1 design on short sleeve white shirt.
Kid sizes: 2/4 6/8 10/12 14/16
Adult sizes: S M L XL

25 Year Grocery Bag | $5
Large bag with logo on either side.

Mail your Orders to FSMA National Office or fax to 847-367-7623
Order form is on Page 42

FSMA Car Magnet | $5
New FSMA Logo.

Travel Mug | $10
New FSMA Logo.

Sport Bottle | $5
New FSMA Logo.

Bag | $10
New FSMA logo.

Families of SMA T-Shirt | $12
1 design on short sleeve white shirt.
Kid sizes: 2/4 6/8 10/12 14/16
Adult sizes: S M L XL

TOTES

FSMA Car Magnet | $5
New FSMA Logo.

Travel Mug | $10
New FSMA Logo.

Sport Bottle | $5
New FSMA Logo.

Bag | $10
New FSMA logo.

Families of SMA T-Shirt | $12
1 design on short sleeve white shirt.
Kid sizes: 2/4 6/8 10/12 14/16
Adult sizes: S M L XL

25 Year Grocery Bag | $5
Large bag with logo on either side.

Cure SMA Bracelet | $2
Available in small or large.
Created in honor of Steven Potter.

Canvas Tote | $15
Choose either Flower or Seascapes design.
Artwork by Katie Gardner 11/89–4/03.
Directions  |  Spring 2009

FSMA merchandise

**American Cards | $6**
Alyssa Silva’s “American” note cards. 6 different cards.

**Thank You Cards | $6**
Alyssa Silva’s “Thank You” note cards. 6 different cards.

**Donation Gift Card**
$10 ea. 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.

**“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 M L XL
Long Sleeve,
Kids sizes only: 2/4 6/8 10/12 14/16

**FSMA “Moving Forward” T-Shirt | $12**
Sizes: S M L XL

**License Plate Frame | $7**

**Angel Wing Pins | $10**

**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

**Cookbook for a Cure | $14**
The FSMA cookbook contains almost 400 recipes from SMA families all over the world!

**12 Fruit Notecards | $6**
4 each of 3 designs (12 cards). Artwork by Holly Campbell, in memory of nephew Charlie, SMA Type I.

Merchandise can be ordered online at www.curesma.org
Donation Form

I want to make a donation in the amount of $ [ ]

In honor of [ ]

Donor Name [ ]

Donor Address [ ]

Notice of donation—Name & Address [ ]

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

Gift Certificate Order Form

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

Retailer [ ]

Ship order to [ ]

Shipping Up to $499 - $8 • $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

Item [ ]

Ship order to [ ]

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
Create your own FSMA Fundraising Team!

We’ve made it simple to set up your own Fundraising Team. The key to a successful team depends on asking people you know for support. You set your team goal and each member of the team sets his/her own personal goal that contributes to the team. As the team leader you are notified via email when a donation is made. Use the chart below to help identify people you know and organize them into categories. Start with family and friends. Next, ask coworkers and acquaintances. Before you know it, you will have a team of 25 people or more!

Who do you know?

- High School/College Alumni
- Community/Civic/Social Clubs
- Competitors & Colleagues
- Church/Synagogue
- Fraternity/Sorority
- Friends
- Your Children’s Sports Teams
- Your Family
- Vendors/Suppliers
- Child’s School
- Neighbors
- Your Company

Host a 25th Anniversary Walk-n-Roll!

Families of SMA Turns 25!

Celebrate a Quarter Century of Determination and Dedication.

It may sound like a lot of work, but hundreds of families will attest hosting a Walk-n-Roll is an amazing opportunity to spread awareness in your community about Spinal Muscular Atrophy and raise critical funds for SMA research.

A walk can be as simple as asking family and friends to gather at a local park and walk in honor of your loved one. Your event can act as a model and inspiration for other SMA families.

We can help!
- Online Registration and Fundraising
- Send an eblast or postcards to members in your locality
- Signs, Posters, and Banners
- Set up your event on the FSMA Website
- Order Walk-n-Roll T-Shirts
- Send Event Merchandise

Once you’ve selected a date and location please call or email the National Office at 800-886-1762 or fundraising@fsma.org.

Help us reach our goal to hit the $50 Million mark for Spinal Muscular Atrophy Research.
The starting line for the 2008 Jack Rabbit Run looked a little different this year. Moondog, the Cleveland Cavaliers mascot, was front and center. Moondog was not the fastest but we were honored to be the first 5K he completed dressed in his gear. It started off sunny and then ended with some light showers. The Inlet Dance Theatre was still able to perform in the rain and no one got injured. The rain did not seem to dampen anyone’s spirits. Many families were involved and helped raise over $50,000 for FSMA. In addition to the run/walk there was a large raffle, St. Paschal Baylon Clown Ministry, Eat-n-Parks Cookie Cruiser and Smiley, a booth by Starbucks Coffee, awards, kids fun run, music, and lots of food…. Each year we are deeply touched that this one morning demonstrates how exceptional people are in helping others to keep hope alive in finding a cure for SMA.

Mary Beth Kotheimer
Chesterland, OH

The Haley Mitchell Ski-a-thon was held on October 4th, 2008 on a perfect fall day. The morning chill burned off to a comfortable 70’s and abundant sunshine for the rest of the day. The atmosphere was as warm, with everybody attending psyched for the great skiing conditions and a successful fundraiser for SMA. Many good performances were logged for skiers looking to inch up the rankings list before retiring the 2008 season. Between rounds, everybody enjoyed the food, live music and friendly competition under the silent auction tents. When Pam Keaton tallied up all the donations and pledges at the end of the day, we had raised an astounding $35,500!

Our family and ski club would like to thank each and every person involved. Everybody was so willing to give their time, talents and money to make this a success. We are truly blessed to have so many good friends.

This tournament is a tribute to many good things. It shows how a little girl can bring folks together to accomplish something amazing. It touches each heart and reminds us to appreciate what we often take for granted and it demonstrates how we can utilize the sport we love to help a great cause.

Jeff Mitchell
Elizabeth City, NC
Chuck and Megan Hernandez of Lutz, FL held the 17th Annual Maluko Golf Tournament in honor of their son, Tyler. The tournament was held at Plantation Palms Golf Club in Land O'Lakes, FL. They had another great turnout this year and collected $39,315 to benefit Families of SMA.

Northern California’s Walk-n-Roll 2008, referred to as Walk SMArt (SMA Research and Treatment), was held under sunny skies on September 27 in Emerald Glen Park, Dublin, CA. The bright and beautiful day was matched by the warmth and beauty of the families and individuals who came together to walk. The names of friends and family members affected by SMA were written on “bricks” that were placed at the start of our course creating a tangible reminder of the reason for our walk. Families and friends who meet annually to participate in this walk welcomed those new to SMA. There were many memorable moments including music by The Don Veca Trio and a large turnout of teenagers whose energy was contagious. Over 150 people participated in the walk and hundreds more contributed. Our walk raised nearly $35,000 for FSMA. We preceded our walk with a Newsletter last Spring that featured our Northern California SMA kids. Nearly $5,000 was contributed to FSMA in response to this newsletter. We are looking forward to this year’s walk that will be held at the same location on September 26, 2009.

Tim and Mindy Rush of Saginaw, TX held the 6th Annual Odds on a Cure Casino Night fundraiser in honor of their daughter, Savannah on October 25, 2008. Once again, the event took place at the Will Rogers Roundup Inn in Fort Worth, TX. The evening featured dinner by El Sombrero, Live Music by White Hot Soul, Slot Machines, Texas Hold Em Tournament, Black Jack, Live Auction, Silent Auction, Raffles, and much much more! The event raised almost $30,000 for Families of SMA.

Dear FSMA,
We held the 3rd Rally for Reagan Golf FORE a Cure on August 1, 2008. It was an outstanding day for golf and getting together with family, old friends and new friends. We are really excited because this year we were so fortunate to have four other SMA families join us…Scott & Lisa Sheppard and angel son Grant (SMA1), Kate Vogedes and daughter Leah (SMA2), Eric & Angie Nelson and son Ben (SMA2), and Angie & James Damon and daughter McKenzie (SMA2). I made them all guests of honor. This was our most successful event yet raising over $25,000 for SMA.

Joseph and Jenny Imhoff
New Berlin, WI

Kevin and Linda Arnold of South Charleston, WV held their annual Arnold Family Golf Tournament in honor of their son, Eric. In October, friends and family of the Arnold’s came together, with Eric as greeter, MC, and award presenter they were able to raise $18,000 in support of Families of SMA.

Dear FSMA,
On Sept. 6, my family produced a fundraiser, “Rosie’s Concert to Benefit SMA Research,” which despite tropical storm “Hanna” which hit Montclair just before the concert started, was a huge success and exceeded our goal to
FUNDRAISING stars

Dear FSMA,
I am following up with you regarding the “Design Home 2008” project, which helped raise funds for Families of SMA. On behalf of the home builder A. Mass Construction along with the support of their vendors, TL Concepts our promotional partner with the project, and 5 Eyewitness News we are pleased to donate $12,285 to your charity.

Best Regards,
Tom Hennessy
In honor of Luke Hennessy from Lakeville, MN

To Our Friends at Families of SMA,
On Friday, September 12, 2008, the Fourth Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Clinton, Wisconsin. Grant (type I) was our third child and he taught us a great deal in his short time with us. One of his lessons was that life with SMA is difficult and help is always needed for equipment, medical costs and ongoing research. We are pleased to be sending you the proceeds of the golf outing held in his memory. Enclosed are two checks. The first one is for $11,500 which is the proceeds raised from the scramble. The other check is for $500 which is a donation made by our local Wal-Mart in Darboy, Wisconsin. The money may be used as you see fit.

Thank you for all that you do for families dealing with Spinal Muscular Atrophy. Please know that the Sheppard family is available to return help that has been graciously sent in our direction.

Sincerely,
Scott and Lisa Sheppard
Sherwood, WI

Al, Nancy and Rosie Mercuro
Upper Montclair, NJ

Kyra Scadden of Naperville, IL, along with fellow May Watts fourth grader and friend Angie Lee (SMA Type II) held the second annual Naperville Garage Sale to benefit FSMA. The community-wide sale took place on September 20, 2008 and raised an amazing $12,500! This year, several families, friends, and community members came out in droves, both to donate items to sell and take advantage of the wonderful bargains.

Raise funds and awareness to find a cure for spinal muscular atrophy (SMA). The event raised $16,005, with additional donations still coming in months later. For those who came out and supported our event on such a horrible night, we thank you but there are many more I need to thank for making this night such a success. Many who could not attend gave to a special online donation site we set up and are still giving. Thank you. To the kind people at the First Congregational Church of Montclair, the tireless volunteers at the Outpost in the Burbs, Frungillo Caterers, Township of Montclair and The Montclair Times, thank you.

Thank you to our family, friends and neighbors and the Montclair High School field hockey team for putting up posters in town and their continued support. A special thank you to the Gottlieb Foundation for their generous donations and too many other corporate sponsors such as ICOM. Most of all, I need to thank the Board of Trustees at the Outpost in the Burbs for their support, and Outpost President Gina Aurierma: without her help this concert would have never happened. Gina’s grace and persistence in getting the WFUV’s Pete Formatale over the years to ask Rock ‘n’ Roll Hall of Famer Richie Furay to donate his time for our event was instrumental to our success. Of course, to Pete, Ritchie and Alan Marzelli for a wonderful night of music as well as Bill Lauf who opened the show and won the audience over with a special song he wrote for Rosie. Thank you all. It is truly a “Good reason to know somebody loves you.”

Al, Nancy and Rosie Mercuro
Upper Montclair, NJ

www.curesma.org
Dear FSMA,

2.4 mile swim, 112 mile bike, 26.2 mile run. That’s what an iron-distance triathlon consists of. Yes, it’s physically demanding and requires discipline, perseverance, and a bit of luck, too. I have completed one IM-distance race in 2005; I along with my brother-in-law raised over $14K in sponsorship dollars for pancreatic cancer (my dad had recently passed away).

Just when life started to return to a new normal for our family…WHAM! My niece Andrea Trakas was diagnosed with SMA. We lost her nearly one year ago, January 29, 2008 at 7 months-old. I decided that another IM race was needed. My goals were two-fold…raise $7K (I was doing this race solo) and to finish the race faster than the last one. An iron-distance race is peanuts compared to what it takes to deal with SMA, what it does to your heart, body, and soul…especially if you’re a parent. I watched, in awe, my sister Donna and her husband Pete deal with each issue with such grace and love. If they can do that, I can do this race.

We sent emails to all our friends and co-workers…two in fact. I’d like to think folks think just like me, and that’s why they gave. But, when you see the face of an angel, when you know that she, her sister, and her parents, Donna and Pete (my sister and brother-in-law) did nothing to deserve this disease, asking people is pretty simple. People were more than generous in their gifts. Some of my friends and former students, who really have little money to give, gave.

People, who don’t know me, gave. It’s a testament to the loving example Donna and Pete gave to all of us, dealing with this disease, with strength, faith, and love for each other and for both their daughters.

When I lined up to start the swim we were singing the national anthem, I thought of my niece, my dad, and my sister. Thank goodness I had my goggles on…no one could see the tears in my eyes. I knew my real goals were accomplished: 1) letting people know about my sweet niece; 2) raising awareness of SMA to a lot of folks that didn’t know about it; 3) raising over $8K for Families of SMA.

As for the race, I was physically and mentally ready, but got a bit unlucky…my family spread the stomach bug about two weeks prior to the race; I got sick about ten days prior. I thought I was fully recovered come race day, but I wasn’t. I finished the swim, and struggled but finished the bike. By mile 9 of the run, I had stopped sweating. By mile 12, I realized I was badly dehydrated, and decided it would not be a good idea to continue. As a race official drove me to the finish line, I arrived to see my entire family waiting for me, including Andrea’s five cousins, and her big sister. Even though they didn’t cross the finish line with me, we sent all our pink butterfly balloons up to heaven and gave three cheers for “Sissy”.

I love you, Andrea.
Mike Palios
Tampa, FL

To Our Friends at Families of SMA,

It is with great joy we have enclosed checks totaling $7,615, the proceeds from our 5th annual Lukie’s Fall Festival. Once again God blessed us with a beautiful day, so many people came out to support us, even in light of the troubled economy. It is always so humbling to see the outpouring of support for our cause.

Thank you for all that you do!
Tara, Jospeh, Kadyne & Anna Maida Roseto, PA

Stacey and Steve Hassel of Cornelius, OR held their annual Abbey & Garrett’s Walk-n-Roll in honor of their children on September 13, 2008. There were about 50 people this year and the weather was beautiful. There was a silent auction and raffle. The community pitched in and donated fruit, donuts, and coffee. The walk raised $5,800 for FSMA. It was a wonderful day!

Shane and Jenifer Barber of Park City, UT held an Extreme Makeover Viewing Party in honor of their daughter, Sarah and in support of the Aker’s Family episode of ABC’s Extreme Makeover Home Edition on October 5, 2008. They raised an incredible $5,000 at their viewing party fundraiser for FSMA.
Hi FSM A,
It was in September of 2007 that I first became aware of the Big House/Big Heart Race. It seemed to provide such a great opportunity to raise funds for SMA research. The concept was unique since each charity could solicit their own volunteers who would recruit donors to sponsor them as a walker or runner in the race. All overhead costs were incurred by the race developers and thus all funds raised by SMA volunteers went directly to SMA research. My first goal was to reach as many SMA parents and family members as possible and so I contacted Ken and Cindy Armbrustmacher who direct the Michigan SMA chapter and Holly Schaefer who has developed a SMA Run/Walk/Roll in Lansing, Michigan. They helped to get out the word to SMA families and wonderful people like Melissa House and Liz Elsby surfaced and offered to volunteer. My brother Bill and his wife Tracey, who lost their little Jessica as a result of SMA complications one day shy of her first birthday, were very anxious to be involved. They contacted family, friends and work colleagues who all gave so generously in spite of very tough economic times in Michigan. We were able to raise over $5,000, all of which went directly to FSMA. On the day of the race, September 28th, Jessica’s parents, brothers, grandparents, aunts, uncles and cousins came out to honor her sweet memory and to remember all the other precious children whose parents are waiting anxiously and working toward a cure. Jessica’s father said that he felt her presence as we finished the 5K race running through the tunnel and into the University of Michigan “Big House” stadium. On that beautiful fall day I think we all felt Jessica had placed the wind at our backs and the sun on our faces.

Mary Vincent
Ann Arbor, MI

Dear FSM A,
We are sending you $1,800 from a premier jewelry party we had in August. My friend Kelsey Huber sells this Jewelry and wanted to do a fundraiser for Morgan. 50% of the profits went to FSMA and we sold over $3,000 worth of jewelry! We also received $1,500 free jewelry which we are selling to friends and co-workers who wanted to help raise even more money. We should have between $2,300 to $2,500 by the time all the money is in. Morgan is truly blessed to have so many caring people in her life! We also had help from Rita Schmidt, Kathy Goodyear & Janet Hutchinson who despite their losses with family members with SMA continue to help us fight SMA! Morgan is doing great and is working hard everyday with keeping her muscles as strong as she can. She is still on the depakote from the SMA study out in Utah and is gaining strength.
Thank you everyone at FSMA for caring and helping us get closer to the cure!
Love,
Sue and Morgan Fritz
Saint Peters, MO

Mark and Cari Kelley of Grand Isle, VT participated in a fundraiser to benefit Families of SMA in honor of their daughter, Jacqueline. The Kelley family raised an amazing $1,370 to help fund SMA research.

A special thanks to the Marshfield High School DECA students who once again this year thought of FSMA. Through a variety of events they raised $1,183.98 for FSMA in honor of Reagan Imhoff during their Homecoming week! They charged an admission to the Powder Puff game (the seniors won!), they had a Miracle Minute Collection at halftime of the boys’ football game, a bake sale, and the Booster Club donated the proceeds from all popcorn sales. They also sent us an awesome Powder Puff t-shirt that had “Rally for Reagan” on the back. Your continued support means so much to our family and all of those affected by SMA.

Joseph and Jenny Imhoff
New Berlin, WI

Mary Vincent
Ann Arbor, MI

Mark and Cari Kelley of Grand Isle, VT participated in a fundraiser to benefit Families of SMA in honor of their daughter, Jacqueline. The Kelley family raised an amazing $1,370 to help fund SMA research.

Dear FSM A,
We are sending you $1,800 from a premier jewelry party we had in August. My friend Kelsey Huber sells this Jewelry and wanted to do a fundraiser for Morgan. 50% of the profits went to FSMA and we sold over $3,000 worth of jewelry! We also received $1,500 free jewelry which we are selling to friends and co-workers who wanted to help raise even more money. We should have between $2,300 to $2,500 by the time all the money is in. Morgan is truly blessed to have so many caring people in her life! We also had help from Rita Schmidt, Kathy Goodyear & Janet Hutchinson who despite their losses with family members with SMA continue to help us fight SMA! Morgan is doing great and is working hard everyday with keeping her muscles as strong as she can. She is still on the depakote from the SMA study out in Utah and is gaining strength.

Thank you everyone at FSMA for caring and helping us get closer to the cure!

Love,
Sue and Morgan Fritz
Saint Peters, MO

Mark and Cari Kelley of Grand Isle, VT participated in a fundraiser to benefit Families of SMA in honor of their daughter, Jacqueline. The Kelley family raised an amazing $1,370 to help fund SMA research.

A special thanks to the Marshfield High School DECA students who once again this year thought of FSMA. Through a variety of events they raised $1,183.98 for FSMA in honor of Reagan Imhoff during their Homecoming week! They charged an admission to the Powder Puff game (the seniors won!), they had a Miracle Minute Collection at halftime of the boys’ football game, a bake sale, and the Booster Club donated the proceeds from all popcorn sales. They also sent us an awesome Powder Puff t-shirt that had “Rally for Reagan” on the back. Your continued support means so much to our family and all of those affected by SMA.

Joseph and Jenny Imhoff
New Berlin, WI

Mark and Cari Kelley of Grand Isle, VT participated in a fundraiser to benefit Families of SMA in honor of their daughter, Jacqueline. The Kelley family raised an amazing $1,370 to help fund SMA research.

A special thanks to the Marshfield High School DECA students who once again this year thought of FSMA. Through a variety of events they raised $1,183.98 for FSMA in honor of Reagan Imhoff during their Homecoming week! They charged an admission to the Powder Puff game (the seniors won!), they had a Miracle Minute Collection at halftime of the boys’ football game, a bake sale, and the Booster Club donated the proceeds from all popcorn sales. They also sent us an awesome Powder Puff t-shirt that had “Rally for Reagan” on the back. Your continued support means so much to our family and all of those affected by SMA.

Joseph and Jenny Imhoff
New Berlin, WI
Pamela Kuhn of Wisconsin Rapids, WI held the Ride to Fight SMA in memory of her daughter Shania Jo Hasselman. The event raised $1,090 for FSMA to support SMA research.

Families of SMA,
April 5, 2008 would have been our daughter Aleah’s 1st birthday. My husband and a wanted to do some type of fundraiser in her honor. I was pregnant with twins and on bed rest so our options were limited. We decided to do an Angels of Hope campaign using the paper angels that were mailed to us. Using the website I made a sign with Aleah’s picture and facts about SMA. Family and Friends posted the signs selling the angels and gave out literature about the disease. We are happy to announce that it was a success. Not only did the angels sell but we still have people contacting us to make donations. So far we raised $1,050.

At the end of April my husband’s uncle Giovanni past away very sudden and unexpected. We are an extremely close family and as you may already know in lieu of flowers his wife decided she wanted donations to SMA. It was very touching for my husband and me.

Mike and I are happy to announce on May 13th the twins arrived. Layla Giovanni 4lbs. 14oz. (named after her late sister & late uncle) and Maximus Mario Durazzo 3lbs. 1oz. (named after “Gladiator” and his grandfather). It was a mix of emotions for us because not only did I find out I was pregnant with twins right when everything happened with Aleah, we had to return to the hospital that she passed in. During the month of November I spent three weeks with her in the PICU. Now here I was for a week in the NICU. During delivery my doctor notified the hospital that the babies would need to be tested for SMA. It was amazing how some people really were not sure what SMA was. We had the entire staff crossing their fingers for us, especially once they heard of our story. Some of the nurses and doctors that we became friendly with at PICU came down to visit us. When we spoke to the pediatric doctor in regards to the SMA testing he took it upon himself to contact a neurologist to come and examine the twins. It was the same neurologist that treated Aleah. Dr. Sultan knew exactly who we were. In a strange sense it made me feel good because I know how busy and backed up he is and he took the time to come down to my room and speak with me. The test results took 4 weeks to come back. It was probably the longest 4 weeks of our lives. Maximus is a carrier but Layla is fine – such a blessing. I’ll never understand or get over losing Aleah. When I was in the hospital with her and found out I was pregnant many suggested that I have testing done to see if the twins would be okay. Many suggested that I have an abortion because I was dealing with too much. I put my trust in God. Although, I did not agree with what happened to Aleah I knew there was a reason why this was happening. It was a tough pregnancy and a very emotional one. My trust paid off. God blessed us and although we lost Aleah I feel like in some way she has saved generations of our family. We are now aware of SMA. We have brought awareness to anyone that comes into our lives. Once I brought those babies home from the hospital it was no longer such a dreadful place for me. I even walked into the PICU (because I was discharged before the twins and would go for feedings) to say hello to the nursing staff. I’ve encountered some truly wonderful people during this time. Even Jessica Moyer who just lost her father, phoned me to see if I had the babies and see if everything was alright. This organization has really meant a lot to us. Aleah’s spirit will always surround us and I can only hope that we make a difference in finding a treatment for this disease.

Enclosed is a copy of the sign I had made and below is a list of the business that sold the angels. I will be in contact with someone soon because we will be baptizing the twins in October. The theme will be SMA awareness therefore; I will need to speak with someone in regards to ordering some items. Hope all is well for everyone and their families on your end and look forward to sending in some more checks.

I have to apologize I wrote this letter awhile ago and it’s just been hectic trying to get to the post office to mail the package. I want to thank you for the angel pins they are beautiful and we wore them proudly at the twins baptism. I gave them out with favors and also gave out other merchandise that I had at the house (bracelets, magnets and cards).

I will be in touch soon to purchase some items for the holidays and we are doing another small fundraiser during the holidays.

Thanks for everything again,
Sincerely,
Vicky & Mike Durazzo
Toms River, NJ

Dear Families of SMA,
We’re pleased to donate $1,000 in memory of our son, Andrew Todd. The money was raised by selling knitted hats at a local craft fair. The hats are called “Drew’s Derbies”. Thank you so much for all you do to find a cure for SMA.

Sincerely,
Shelly, Tom, Kate & Sarah Madsen
Omaha, NE
Dear FSM A,

Enclosed you will find donations totaling $1,000 that were raised in honor of our son, Cole (SMA Type I). My husband, Rick, ran the ½ marathon in honor of our son and to raise money for SMA. In addition to this, we ordered 70 Wipe Out SMA T-shirts that our supporters wore for the marathon. Enclosed is a picture of my husband and our son showing off their Wipe Out SMA Shirts.

Sincerely,
Dori Fiedler
Rothsay, MN

Nic and Michelle Nelson of Sioux City, IA, held for the first time “Race 4 Grace” in honor of their daughter, Grace. The event raised an outstanding $910 for Families of SMA.

Hi FSM A,

We had another successful year planting, growing and selling pumpkins in memory of our precious baby girl, Lainie Grace Border. This year the event raised $795. Lainie lost her battle to SMA in February 2003 when she was just 4 months old. We have done our best to honor her and keep her memory alive by continuing to help fund research and support other families dealing with this devastating disease. This is the 4th year we have celebrated Lainie’s life with this fun fall fundraiser. We hope to continue this fundraiser for many, many years to come. And we are honored to be able to send you the proceeds to support all of the wonderful work you do at FSM A! Thank you for everything you do, and please don’t stop until we find a cure!

Sincerely,
Julie, Bret and Luke Border
Lewis Center, OH

Families of SMA,

We are happy to send in another donation to FSM A from the employees and loyal customers of Sammy’s Restaurant in Grand River, OH. Our four year old daughter Amelia is living with SMA. Her “Nana” Karen Buling is a waitress at Sammy’s. She has a special collection bucket at the counter to collect money to help find a cure for SMA. Her customers have helped raise over $600 for other Families of SMA fundraisers. When there is no specific event going on, they continue to give selflessly and from their hearts. We are so proud of Nana and her friends! Keep up the good work!

Melissa Wong

Families of SMA was the honored beneficiary of the Byron Bank 5K Race in Byron Center, MI in honor of Sydney Potjer. Byron Bank donated $500 to FSM A in honor of the race.

The Central Connecticut State University Car Club of New Britain, CT raised $500 for Families of SMA through various fundraising activities.

Kaitlin Cooper and Arica Ludwig organized another successful Rally for Reagan event at the Waukesha North’s GVB game tonight. They had a number of gift certificates and North paraphernalia for the raffle and also held a 50/50 raffle in which Jeannette Learned donated her winnings back to FSM A! Kaitlin got Reagan on the microphone to thank everyone for coming...I think it
Greetings,

Joseph and Jenny Imhoff
New Berlin, WI

The Hoffmann family of Milwaukee, WI held their annual 53rd Street Block Party and Raffle to benefit Families of SMA in honor of their daughter, Jackie Hoffmann. They sold raffle tickets for $2 each or 3 for $5 and were able to raise $404 from the evening.

On August 15, 2008 Megan Hernandez of Lutz, FL held the 7th Annual Southern Living at Home Party in honor of her son, Tyler and SMA Awareness month. The event raised $400 for Families of SMA. A special thank you to Missi McCombs, the Southern Living at Home representative for donating her proceeds from the day’s sales.

Jessica and Ken Adams of Washington, NC organized Portraits for a Cure in honor of their daughter, Jamie. The event took place on November 7, 2008 at several portrait studios located within the community. The portrait studios agreed to donate the day’s sitting fees to Families of SMA. The event raised $350 to benefit SMA.

Greetings,

Every year Motorcyclists for Jesus sponsors a Motorcycle show in Boyertown, Pennsylvania and the proceeds benefit Children’s Miracle Network. Motorcyclists for Jesus sponsors a food table and this year the proceeds were split between Children’s Hospital in Philadelphia and Families of SMA. We are pleased to present a check in the amount of $350.00 to help in finding a cure for Spinal Muscular Atrophy.

We are glad to be able to support Families of SMA through our annual Ride to Fight SMA in May and hopefully the Bike night will provide additional funds into the future.

God Bless you and your work for this cause,

Christopher Cooter

Dear FSMA,

Please accept this $310 in honor of Jaclyn Hoffmann from Mari Franzen (Jackie’s brother Mikey’s girlfriend Lauren’s younger sister). Mary had kids over for her Sweet 16th birthday party and asked for donations to FSMA.

Paula Hoffmann
Milwaukee, WI

Hello Families of SMA,

Please accept the following donation of $265 in memory of my niece, Audrey Marie Snyder (May 2, 2001 – October 15, 2001). In honor of my beautiful niece, and to celebrate the strength and courage of families who are affected by SMA, I decided to run the 2008 Chicago Marathon. After training for five months, and running with the spirit and thoughts of Audrey, I completed the marathon in 4 hours, 55 minutes and 35 seconds.

Thank you for continuing your fight for SMA.

Kelly Jones
Troy, MI

Dear Families of SMA,

The City of St. Charles held a “Giving Friday” on Friday, August 29, to support the wonderful work that Families of SMA does.

Enclosed are 14 checks – one from the City of St. Charles for $148, and 13 checks from individuals (totaling $138) for a total donation of $286.

We consider it a privilege to partner with Families of SMA.

Thank you for all that you do.

Sincerely,

Teri Grandt, St. Charles, IL

Gina Bish of Andover, KS donated an additional $210 from the sale of Larkie Lu Bows at www.larkielubows.com in honor of her daughter, Larkin.

Greetings,

Recently, OneShield, Inc. held an employee appreciation event for the successes we have had over the past year. At the event we held a 50/50 raffle ticket drawing, and would like to donate the proceeds to Families of SMA. The OneShield family, have been directly touched by the great efforts your organization has contributed, and we would like to extend our gratitude for the wonderful work you do. With an organization like Families of SMA we understand that progress is being made, and with contributions to your organization you can do even more.

OneShield, Inc. is a leading provider of Web-technology solutions to property and casualty insurance industry. Our vision/mission is to provide technology driven solutions that automate the sales, product management and service of insurance products for carriers and MGA’s.

Please accept the enclosed check for $175.

Thank you and Kind Regards,

Jennifer Toto
OneShield, Inc.
Westborough, MA

To Whom It May Concern,

The faculty and staff at the Sandusky County Board of MR/DD, Sandco and School of Hope, sponsor a dress down day once a month for an organization or worthy cause that we feel is beneficial to our community and others in need. This past week we sponsored Families of SMA. Enclosed you will find a money order in the amount of $30 and a check for $15 totaling $45. We are happy to be able to help your organization in this small way and know that this money will be put to good use – as you see the need.

Sincerely,

Cherie Palmer
Fremont, OH
To Whom It May Concern,

Enclosed are checks and money orders in the amount of $2,083 that is being donated to FSMA by Mikayla Jean and her family, in memory of her little brother Joseph. Joseph has been our little angel since August 30, 2003. Every year Mikayla and her cousins have a lemonade stand asking for donations to be made in memory of Joseph. This year was the biggest year ever!! This year the principal of Mikayla’s school was so kind as to let us pass out flyers to the entire school, which brought us a lot more people. Mikayla and her cousins do the majority of the work, regardless of the fact that it is usually over 100 degrees in Southern California. The children work very hard and are very grateful of everybody that gives their donations. We also want to thank everybody at FSMA and all the doctors and research companies for all their hard work while they try to find a cure.

Sincerely,
Kim Donnelly
Highland, CA

FSMA,

My son, Grayson Traylor, celebrated his 9th birthday on February 14, 2009. This year, instead of gifts, he asked his friends to bring a cash donation of any amount to donate to Families of SMA. I am thrilled to report that Grayson and his friends raised $2,000 in Matthew Wallis’ honor.

The local newspaper also did a spotlight on the party. It was quite a day, and I know it will impact my family and those who participated for years to come. I am very proud of Grayson and his friends. They all hope that their donations will get us closer to a cure for Matthew.

The kids enjoyed the SMA bracelets and balloons you sent. Thank you! Also, we had the party at Pump It Up! In Longview, TX, and they gave us a substantial discount on their facilities and food. I am very appreciative of their support as well.

Michelle Traylor
Longview, TX

Dear FSMA Staff:

Once again we celebrated Veronica’s (4th!) birthday and asked for donations in lieu of presents. This year we added duck races to the day which proved to be a lot of fun! There was almost a casualty, as an over zealous duck racer got a little too excited cheering his duck down the creek! It was a great day, everyone had a lot of fun, and we managed to raise $825 for SMA research and support. It is with great pride that we send this money to our friends at FSMA in honor of Veronica Rose.

Debby St. Onge
Bigfork, MT

Dear Families of SMA:

In honor of Alyssa Silva (Cumberland, RI) please accept this donation for $802.30. The confirmation class of St. Marks (North Attleboro, MA) held a Bowl-A-Thon to raise funds for SMA. The Bowl-a-thon was held November 23, 2008. The kids had a great time for a great cause. This was part of a community service project. My name is Kimberly Carbone, I am a nurse who works with Alyssa Silva. She is an amazing person who inspires many. The students of St. Marks were honored to participate in this event. Alyssa stopped by to meet the kids, it was a really nice day for all. We took some pictures of the event which are enclosed.

Thank you,
Kimberly Carbone, RN
Lily Sykora from Leawood, KS celebrated her 7th birthday with lots of friends at a skating party. Her little brother Charlie, who has SMA type I, also enjoyed the fun zooming around in his EasyS chair with Dad and Mom pushing him from behind. Lily made her parents proud by exhibiting a spirit of giving by asking for donations in her brother’s name for Families of SMA in lieu of gifts for herself. Lily was pleased to raise more than $550 for FSMA.

The fifth grade students at Westwood View Elementary School in Westwood, Kansas chose fundraising to help fight SMA as their heroes project for a fellow student, Claire Gibbs with Type II SMA and also her sister Lauren who is in the 6th grade with Type III SMA.

The 5th grade classes chose a Bake Sale and Hat Day for SMA! All of the fifth grade kids joined in and promoted the event by making signs for the school, writing letters to take home, and even making announcements that included a fight SMA rap song! Whoever wanted to wear a hat to school all day paid at least $1 and the fifth graders collected from the K-6th grade classes. Then, at the end of the school day, a huge all school bake sale was held in the gym! The event was fun and a huge success with the class raising over $500!!

In November 2008, Caroline Genster sponsored a bake sale at her school in Seattle, Washington to benefit Families of SMA. Caroline’s goal was to raise money for research and education for Families of SMA. Caroline’s sister, Nora, has SMA type II/III. Caroline and her friend Mollie McCarthy planned the event with great support from the school and her classmates. The final tally for the day was $480.

Dear FSMA,

For Addison’s 2nd Birthday we requested friends and family make donations to FSMA instead of buying gifts, so here is the $301 we collected. We hope that this small contribution helps us reach the $50 million mark! I don’t know where we would be without FSMA and we are forever thankful for everything you have done for us.

Love,
The Kuester’s
Justin, Shannon, Haley & Addison
Hartford, WI

Dear FSMA,

Amy Haake presented Beth Lockwood at the OKI Chapter $208 that she had raised for FSMA. Amy celebrated her Sweet Sixteen for SMA last September and asked guests for donations in lieu of gifts. Amy has been involved with FSMA for many years and has been a part of many Walk and Roll events, first in Colorado Springs and for the past several years in Cincinnati where she now co-chairs the registration table. Amy is always ready to help at any FSMA event; here she is pictured with MJ Purk at the OKI Chapter Halloween Roller Skating Party last October.

Dear FSMA,

This is from the fundraiser hosted by May Watts Elementary School “Do Something Club”. They did a penny drive starting September 2008 and handed it to me. The fundraising was in honor of Jungin Angie Lee and they raised $136.52.

Edmund Lee and Kwiyoum Kim
Naperville, IL
Dear Families of SMA,
On July 28, 2008 our daughter Mackenzie would have been 11 years old. To celebrate her birthday, her little brothers had a lemonade and brownie sale to raise money and awareness for SMA. Two little neighbor girls decided they wanted to help so their mother made a platter of cookies to sell also and they stayed all day to help with the sale. In the end they made $43 for SMA. Quite good considering we sold everything for .50 cents each!! Here is a picture of the kids that day! Thank you so much for everything you do! Happy Birthday Mackenzie Shay Burza, we love and miss you so much!

Love,
Mom, Dad, Kaden, Jace & Jevin Burza
Grand Haven, MI

Dear FSMA,
When I had my 11 year old birthday party I already had everything I wanted. The only thing I was missing was for everybody with SMA to be healthy, for example, my friend’s brother, William Johnson, who has SMA. Instead of gifts, I asked for donations for FSMA. Please use this money to help find a cure.

Sincerely,
Mithchell Romanowski
Medfield, MA

Dear FSMA,
I am very grateful that you sent me a magazine. Thank you so much. I hope you are pleased with this donation. Please direct the money towards making SMA victims more comfortable this Christmas.

Matthew is doing well. We work hard to make him stronger, and his special wheelchair allows him to stand in his chair every day.

I recently wrote a paper in English describing my experience with Matthew, and I got to share with my classmates about SMA. Thank you for all you do, it is much appreciated in our family.

Merry Christmas!

Sincerely,
Lydia Wallis
Longview, TX

P.S. Attached is a recent picture of my cousin Matthew who has SMA.

Dear FSMA,
For a number of years now, Tammy Rezendes and her company, NES Pageants, have been donating to FSMA. Tammy first learned of SMA when one of her contestants, Emma Hope Goldsberry (my daughter), was diagnosed with the disease over three years ago. During that time, the Rezendes family has become great friends with our family, and Tammy has made Emma a Lifetime Queen. That title allows Emma to enjoy all of the perks of being a beauty queen without having to compete. Tammy also created the Emma Hope Award, and at each pageant, the contestant that displays the most kindness toward others is the winner. Entry fees for the award are donated to FSMA, and along with fees collected from special photo contests and other donations, NES Pageants has donated over eight thousand dollars to FSMA. Twelve-year-old Shannon Bauer from Bethlehem, CT is the winner of the Emma Hope Award from the last pageant, and she is shown here accepting the award from Emma.

Thanks,
Jonathan Goldsberry, Bristol, CT

P.S. Here’s a picture from my party. I’m in front. Matt Marie, Matt Johnson, & Evan Lautz in back.

1984-2009
25 years
FAMILIES OF SMA
$50 million raised for SMA research
Bayada Nurses sponsored a Pediatric Professionals conference at the University of Delaware in November. Jack and Cara Freedman were both invited to speak at the conference. The title of their presentation was “All the Nurses and Therapists are Our Friends.” They did a great job!

An honorarium was offered to Jack and Cara for their time and efforts, which Jack and Cara asked to have donated to Families of SMA.

Thank you, Nurse Bridget

Submitted by Dr. Al Freedman, Consultant and Jack’s Dad

Last Thursday, the morning of the Delaware Pediatrics conference, Jack was not feeling well. He had come home from school the previous day with symptoms of an upper respiratory infection. We were not sure if it was safe for Jack to make the trip to the University of Delaware to be a presenter with his sister, Cara. Of course, Jack repeatedly told us, “I really want to go! I really want to go!” My wife, Anne, and I, were not sure what to do. Jack’s nurse, Bridget O’Donnell (PAO), arrived at our home at 7:00 am. She greeted Jack and our family, completed her usual morning routine with Jack, provided respiratory treatments, and examined Jack carefully. With ten minutes to spare before our departure time, Bridget made the assessment that Jack could safely make the trip and participate as a presenter. So off we went. Anne and Bridget together got Jack up into his wheelchair while Cara gathered her script and a stool to stand on at the podium. Bridget sat with Jack in the van, helped him get on and off the stage safely, and sat in the front row during the presentation so she could observe Jack carefully. Jack and Cara were able to complete their presentation successfully. The purpose of my children’s presentation was to highlight the importance of Jack’s nurses and therapists in his life. Bridget’s presence, expertise, and good judgment last Thursday morning helped us to feel comfortable going on as planned with our trip to Delaware. The irony was not lost on me that Bridget provided yet another example of exactly what my children’s presentation was really about—that each of you can make a significant difference in the lives of your clients, each and every day. Thank you, Bridget.

KIDS corner

Families of SMA,
Each Christmas my 5th grade class picks a project to contribute to instead of exchanging individual gifts. This year’s class chose to raise money for SMA. One of our class members has SMA, Brandon Cavendish. Brandon is a great inspiration to all who know him. He is such a bright, happy child. Brandon has faced and will face many challenges. We hope that this small gift will help Brandon and all children with SMA.

Sincerely,
Linda Sparks, 5th grade teacher
Summerville Elementary School, WV

Brandon Cavendish and his classmates

Al and Jack Freedman

Jack and Cara Freedman with nurse Bridget
FAMILY letters

Dear Families of SMA,

We wanted to tell you all Merry Christmas & thank you for all you do. Taj is doing great. He won Homecoming King at his high school, Christmas & thank you for all you do. He is loving school and My nephew Kyle Crews was born in October of 1984 and was diagnosed with SMA in November of 1985. He just celebrated his 24th birthday, but his life has certainly been challenging. When he was diagnosed there was so little known about his disease that there were only about two paragraphs written about “Werdnig-Hoffman” in a pediatric medical textbook. His pediatricians had to research and study it because they didn’t really know anything about the disease. And now look how far you have come. I just read an article from “Reuters” that US Scientists have taken cells from the skin of a child with SMA and have been able to recreate the disease. As they said in the article, “Now we can start from the beginning of development and replay the disease process in the lab dish.”

Tara Cossey, Taj, Jay & Brian Kelseyville, CA

Dear Families of SMA,

My nephew Kyle Crews was born in October of 1984 and was diagnosed with SMA in November of 1985. He just celebrated his 24th birthday, but his life has certainly been challenging. When he was diagnosed there was so little known about his disease that there were only about two paragraphs written about “Werdnig-Hoffman” in a pediatric medical textbook. His pediatricians had to research and study it because they didn’t really know anything about the disease. And now look how far you have come. I just read an article from “Reuters” that US Scientists have taken cells from the skin of a child with SMA and have been able to recreate the disease. As they said in the article, “Now we can start from the beginning of development and replay the disease process in the lab dish.”

Tara Cossey, Taj, Jay & Brian Kelseyville, CA

Dear FSMA,

Thank you so much for taking my question under consideration and answering it so thoroughly. I was at that point in the grieving process that demands that you seek answers and alternatives. Having a child with SMA is such an incredible joy that it is easy to get lulled into a sense of security prompted by a mindset of intense gratitude that your child is alive and doing well. It is easy to forget or just fail to realize that there are and will be many other opportunities for grieving.

It is wonderful to have somewhere to ask questions and get unbiased information on which to base a decision. That was all I needed. In information and knowledge there is power over the fear that comes with feeling victimized by the unknown. With kindest regards and many thanks,

Neva Cohen Mesquite, TX

Dear FSMA,

My wife and I were given the devastating diagnosis of type II SMA for our only child Maximilian (Max) on September 27, 2007. Since then, life has been filled with many ups and downs (much like everyone else in the same situation). My family has been so very fortunate to have the support of my squadron in addition to the long list of physicians and therapists that work with Max. Even though there hasn’t been a whole lot that people can do to help they never stop asking. While my squadron was deployed last fall to Southwest Asia I took a few minutes to explain one way that they could help – by their donations to the Combined Federal Campaign. I have sent a copy of the email that I sent explaining how FSMA and the MDA has personally made such a difference in my family’s life. After all of the pledges were counted my fellow (150-ish) squadron members committed to donating a total of $12,200 to both organizations. While this is only a drop in the bucket when considering how expensive research is, I

Steven Judge Spring Hill, FL
Dear FSMA,

In lieu of favors for our wedding on August 30, 2008, my husband and I would like to make a donation to Families of SMA. We would like to thank our good friends the Ritcher family for supporting us on our special day and for giving us the information on your charity.

Thank you so much.
The Buss Family
Redondo Beach, CA
Almost every little girl dreams of the day when they will have a little baby bump and feel that mysterious feeling of a little person growing inside of them. I was no different. Wanting to have a baby was always in the back of my mind but I never thought that it would happen to me, at least not in the traditional manner. After I got married, my husband and I had talked at length about having a surrogate mother carry our biological child or the possibility of adoption. Although I was all right with either of these options, I came to realize that it was not really how I wanted to add to our family.

After our second anniversary, I told my husband that I wanted us to seriously consider the possibility of a pregnancy. I explained to him that I wanted to feel our baby growing inside of me; I wanted him to put his hand on my stomach to feel the baby kick; and I wanted to have all of the pregnancy experience myself. After this conversation, we sat down and had several conversations about what a pregnancy would actually mean for us. We talked about the strain it would have on my body and we talked about all of the possible outcomes. As hard as this was, it was necessary that we went into this adventure with our eyes wide open.

Once we decided that we were ready for the possibility of a pregnancy, I decided that the next responsible step to take was to have myself checked out by all of my specialists. I met with my neurologist, cardiologist, and pulmonologist to discuss the different aspects of a pregnancy and how my body would handle it. I had many different tests conducted including blood work, an Echocardiogram, and Pulmonary Function tests to see how healthy I was at the time in order to see if my body could handle the added stress of a pregnancy. After my specialists agreed that I was in good enough physical shape to take on the strain of a pregnancy despite my SMA, I began the daunting task of looking for a high risk OB/GYN. My family doctor had been conducting my yearly woman’s health exams but she was honest with me when she stated that she was not skilled enough to handle a high risk pregnancy. Finding an acceptable high risk OB/GYN proved harder than I thought due to the negativity and stereo-types of the society we live in. The first two we met with were so negative that I left their offices scared to death. They both told us that I would automatically deliver at 26 weeks, our baby would probably be still-born or die shortly after birth, and have mental and physical disabilities if it survived. I am an intelligent woman and I realized that there were going to be risks. However, those doctors did not have crystal balls; they based their opinions on the fact that I was in a wheelchair and their own personal prejudices that they had. I was determined to find a high-risk OB/GYN that was willing to support MY decision to have a baby and one that would support me to make it the best experience possible.

In July 2006, I went off my birth control pill and figured it would take awhile for me to get pregnant since I had been on it for so long. In August 2006, we finally found the perfect OB/GYN and were excited to finally have everything ready to go for a pregnancy. Little did I know, when we met with her for a pre-conception consultation, I was already pregnant! When I missed my period in September, I took a home pregnancy test and my heart skipped a beat when I saw the minus sign turn to a plus sign. It’s hard to describe the feeling I had at that moment but it was a feeling I will never forget. I immediately scheduled my first prenatal appointment and we decided who we were going to tell right away. I was pretty nervous about having a miscarriage so we decided that we’d only tell my personal assistants and my parents before I had my first ultrasound. We told my Mom on the phone and this was pretty nerve-wracking for me. I knew that my Mom would be happy to have a grand-child.
from me but I also knew she’d be scared to death of losing me. Much as I had expected, my Mom was very supportive and understood that we had gone into this adventure with our eyes wide open to make it as positive as possible.

My pregnancy as a whole was pretty normal. My body handled it better than I could have hoped for and I had very little complications. At my 31 week appointment, I started having contractions that morning and my breathing was becoming more labored. My OB/GYN was off-site so I saw one of her partners who felt it was best to admit me to the hospital in order to try to stop my contractions and to give me oxygen support. The oxygen helped me a great deal but my contractions continued to build over the next few days without any dilation (this had nothing to do with my SMA; all the women in my family go into pre-mature labor so this was somewhat expected). Although my body was handling things well, I was fearful of being so physically tired by the time of the C-section, my life became all about Claudia. I spent as much time as my husband and my nurses would allow me in the NICU which was delaying my own recovery. I didn’t care how I was feeling, I simply wanted to be with Claudia every second possible. I was released from the hospital one week after Claudia was born but I had to leave her there in the NICU. That was the hardest thing I had ever done. After I was released, my husband and I went to the hospital every day to spend as many hours with her as I could tolerate. She ended up spending two ½ weeks in the NICU in total to learn to eat and to gain some weight. They had prepared us that she would need to stay in the NICU until around when she actually should have been born. She astonished everyone by coming home when she did.

Today, Claudia is a happy, healthy toddler who has reached every one of her milestones on-time or early. I am fully recovered and I absolutely LOVE being a mother. Although I still work full-time, I work from home and my entire life revolves around our little sunshine. She brings more joy, laughter, and excitement to our lives than I ever could have imagined; I praise God every day for allowing us to have this precious miracle. Despite everything I went through, I would do it all over again! She is definitely worth every good and bad time during my pregnancy/delivery.

Over the next several days, I dealt with many things that I had not expected. Physically, I had more pain than I was prepared for. I’ve dealt with pain my entire life; however, this pain was different. Because of where a C-section incision is, everything you do hurts including sitting, coughing, laughing, and breathing. Mentally, I was exhausted. The moment I woke up from my C-section, my life became all about Claudia. I spent as much time as my husband and my nurses would allow me in the NICU which was delaying my own recovery. I didn’t care how I was feeling, I simply wanted to be with Claudia every second possible. I was released from the hospital one week after Claudia was born but I had to leave her there in the NICU. That was the hardest thing I had ever done. After I was released, my husband and I went to the hospital every day to spend as many hours with her as I could tolerate. She ended up spending two ½ weeks in the NICU in total to learn to eat and to gain some weight. They had prepared us that she would need to stay in the NICU until around when she actually should have been born. She astonished everyone by coming home when she did.

Today, Claudia is a happy, healthy toddler who has reached every one of her milestones on-time or early. I am fully recovered and I absolutely LOVE being a mother. Although I still work full-time, I work from home and my entire life revolves around our little sunshine. She brings more joy, laughter, and excitement to our lives than I ever could have imagined; I praise God every day for allowing us to have this precious miracle. Despite everything I went through, I would do it all over again! She is definitely worth every good and bad time during my pregnancy/delivery.

On April 5, 2007, they took me into surgery at 7:30 am and Claudia was born at 8:04 am and was taken straight to the NICU. She was 4 lbs, 4 oz and 16.5 inches long which was actually a good size for being 9 weeks early. Her final APGAR score was 8 which they said was great considering she was under the same anesthetic I was under. She needed a little help breathing so they had her on a C-PAP right away but other than that she was much better health-wise than most premature babies. After 24 hours, she was put on standard oxygen support and strived every moment after.
Update on Families of SMA Funded Stem Cell Motor Neuron Replacement Program

**FSMA has invested significant resources in alternative approaches that show promise to cure Spinal Muscular Atrophy rather than just treat the symptoms. In particular, we have invested $1.5 Million to develop a motor neuron replacement therapy for SMA, and we have made significant progress with our investment.**

Our initial investment in stem cell research in 2000 funded efficacy studies using motor neurons from mouse stem cells. Results show that this therapy can provide benefit to rodents with motor neuron disease: a highly significant finding. In 2005, additional FSMA funding lead to the first, highly-pure therapeutic population of human motor neurons for cellular replacement therapy for SMA. This program is now progressing on the path to IND in collaboration with the biotech firm California Stem Cell, Inc. (CSC), and leading research centers at University of California-Irvine, and Johns Hopkins University. These motor neurons recently completed a series of critical animal safety studies prior to advancing into human trials for SMA.

Motor neuron replacement is at the leading forefront of current scientific knowledge, and as such is very high risk. However, this approach allows for the possibility of replacing lost motor neurons and so holds great promise for the patients and families in our community.

*“FSMA provided the first financial support for my research program investigating the development of high purity human motor neuron populations from stem cells, and their application to animal models of SMA. This work has grown into a multi-tiered program that now involves several funding agencies, an industry collaborator, a clinical collaborator and FDA relations. I will always consider FSMA my partner in pioneering this technology, and moving it towards human use.”* Hans S. Keirstead, Ph.D., Associate Professor of Anatomy and Neurobiology, Co-Director of the Sue and Bill Gross Stem Cell Research Center, University of California at Irvine.

Pre-clinical efficacy studies have been completed, demonstrating correct localization of CSC motor neurons in the ventral spinal cord, cell growth from the spinal cord toward the limbs, synapse formation with target muscle and functional reinnervation leading to restoration of limb function in animal models of motor neuron loss.

**Results of the Safety Study for SMA Type I Clinical Application:** The pivotal safety study, required to support an application to begin FDA clinical trials, has recently been completed. Although final data analysis is still in progress, there were no negative outcomes of this study, leading to the conclusion that motor neuron replacement will be a safe strategy in the treatment of diseases such as SMA characterized by motor neuron loss.

The purposes of this study were to evaluate the capacity of a motor neuron progenitor cell population to form tumors following transplantation into the spinal cord, to determine whether motor neuron transplantation would induce...
allodynia (increased pain sensitivity), and to evaluate the biodistribution and potential toxicity of motor neurons transplanted into the spinal cord. Results showed:—No mortality attributed to treatment with motor neuron progenitors occurred.—No tumors occurred that were attributed with the transplanted motor neurons.—No statistically significant differences occurred between the cell buffer (control) and treatment groups in the clinical allodynia (pain) assessments.—No toxicological profile differences between control and treatment groups.—No gross or histologic findings were attributed to the transplantation of CSC motor neurons.

Preparations for Clinical Trial Approval: California Stem Cell has developed methods for the manufacture of clinical grade human motor neurons. It is the intent of CSC to gain approval to begin FDA-approved clinical trials for the use of these cells in development of a cell replacement therapy for SMA Type I. CSC is now preparing for a final FDA pre-IND meeting to take place in the first quarter of 2009, keeping us on track for a formal application in the second quarter of 2009 to begin a Phase I/IIA clinical trial in SMA Type 1.” Chris N Airriess, Ph.D., Chief Operating Officer, California Stem Cell, Inc.

Other preparations currently underway include: An external clinical trials coordinator has been engaged and medical community focus groups held to develop the clinical strategy for SMA trials; CSC manufacturing facilities and procedures have been audited for compliance with guidelines for clinical manufacturing.

Please see our website for more background information on this program.

Be a “Directions” Contributor

Photos, poems, articles based on your knowledge and experiences, summaries of great FSMA fundraisers…we want them all! This is your chance to share. Your contributions will help to make this publication even better. Please email text either in the body of an email or attached as a word document. Photos submission requirements are:

- dimensions 1600 x 1200 pixels
- jpg format
- 2 megapixel
- Approx. Print Size 4” x 6”

All materials can be sent to newsletter@fsma.org or via mail to the National Office, Newsletter, Families of SMA, PO Box 196, Libertyville, IL 60048-0196.
Hanna Macellero as a Disney Birthday Princess

Jackson and Savannah Hamman

Jackson and Savannah Hamman

Gwendolyn Strong

Halsey Rosbrogh

Halsey Rosbrogh and Jacob Slaymaker

Hannah Eide

Hope, Mikey and Noelle Hazel

Isabella Kurek

Gray and Riley Dougherty

Greyson Erwin

Allison, Mark and Kaley Leiter
Workshop on Clinical Outcome Measures in Spinal Muscular Atrophy

TREAT-NMD issued a press release announcing the results of a recent meeting with the European Medicines Agency on clinical outcome measures for SMA.

TREAT-NMD, an EU-funded ‘network of excellence’ that aims to accelerate cutting-edge treatments for rare inherited neuromuscular diseases, announces a successful meeting on spinal muscular atrophy (SMA) outcome measures with the European Medicines Agency (EMEA).

A TREAT-NMD-led workshop hosted at the offices of the EMEA in London helped set the collaborative agenda for future trials in SMA. Participants included 50 representatives from the neuromuscular field, including healthcare professionals, scientists, patients and pharmaceutical industry representatives. EMEA representatives included the chairs of the Human Medicinal Products, Pediatric Medicines and Orphan Drugs Committees. There was active participation from all parties. Input from the International Care Committee (ICC) for SMA ensured that there was global representation at the meeting, the outcomes of which will also be shared with the US Food and Drug Administration (FDA).

In a new development for the neuromuscular field, the workshop focused not on discussing product-specific issues but on establishing broader common ground between the regulatory authorities and those interested in running clinical trials in SMA. In order for trials to move through the approval process without delays, consensus between trial planners and regulators on endpoints and novel methodologies is essential.

The SMA community is working extensively together and the meeting demonstrated this close link as all present spoke with a united voice on the most appropriate outcome measures for particular clinical situations. The community was complimented on its proactive approach to regulatory topics, its organization and its international teamwork in addressing clinical trial questions for SMA.

Families of SMA helped fund this meeting.

Gwendolyn’s PVC Play Apparatus

Rotating mobile attaches to the apparatus as well. Gwendolyn’s upper arm/shoulder strength has declined over the last several months so normally we place small pillows or rolled blankets underneath her shoulders and upper arms to help her to play at mid-line, but we’ve also tried suspending/supporting her upper arms in slings made of socks that we’ve hung from the apparatus cross bars and that has worked relatively well. I’m sure we’ll be making modifications as time goes on, but for now Gwendolyn loves her new found play freedom. We hope your child will enjoy this apparatus, too!

Materials we used for Gwendolyn’s PVC play apparatus

- 3/4 inch PVC pipe (two 8 foot length sections)
- PVC T connectors (10); used for the four cross bars on top and one cross bar on the back
- PVC elbow connectors (8); used to connect the top/bottom rails with the sides
- PVC pipe cutter
Bri Johnson

Ariana Dindzans

Christopher Munoz

Chase Langland and his Mom

Ally Krajewski on her 3rd birthday

Connor Murphy

Daniel Tramontano

Evie Horton

Charlie Sykora on his birthday

Ally Krajewski

Ayden Trammell

Addie and Molly Piper

Bella Andrade
Farihah Mehmud

Isabella and Nicholas Andrade

Ariana Cloutier

Amanda Lapka with the American Idols

Dani Pruitt

Caitlyn and Grace Trainor

Charlie Sykora with Mom and Dad at Roefest

Robyn Plaster painting a dolphin

Courtney Rosas

Emma and Max Rubenstein

Eleanor and Jack Bolton
God has blessed us with another wonderful adventure for Courtney to add to her little book of life. On Tuesday January 13, 2009, I called a local bowling center, Alpha Lanes to inquire about all the usual things I have to ask about in order to take Courtney out any where. I spoke with the manager, a wonderful lady named Debbie who, after I told her about Courtney having SMA and all her special requirements she said she would be honored to have Courtney visit her bowling center. I was concerned though because she told me there would be some leagues playing there that evening. It is not good to take Courtney out where there is a large crowd of people indoors. I then called the Clear Lake Lanes Bowling Center and spoke with Shaun. He told me there were no leagues playing there on that evening so I decided this might be a better choice for Courtney’s safety against catching the flu or RSV. I called Debbie back to let her know about my decision and she totally understood, she wanted what is best for Courtney.

This was going to be our second Girls Night Out with Tania. This time she brought her younger sister Thalia to join us. As we arrived at the center and opened the door there was a sign saying “Welcome Courtney”. I said could this be for our Courtney? I couldn’t believe how nice these people were to greet Courtney like this. As we entered we met the manager Mike and also met Shaun. They had already saved an area off to the side with two lanes just for us. There were balloons placed all around our area for Courtney, I rolled her around and let her touch each balloon string and helped her hold it. All the girls went to get their shoes and returned to start bowling. Mike and Shaun walked up to us and wanted to present Courtney with something so she could always remember her bowling day. They gave Courtney a very special bowling ball, a Puss in Boots ball from the movie Shrek, they also gave both Courtney and Kendall AMF bowling t-shirts. I was almost ready to cry, not believing how kind these people were. I spoke a little with Mike getting to know him a little better. In that short time I came to realize that he was a sincere, caring person who truly was thrilled to have Courtney there. He and Shaun kept coming back to see if we needed anything. This evening turned out to be so wonderful and we found out that Courtney loves to bowl! She was smiling as Candace held her little hand and helped her to push the ball down the guard. We would clap and yell for her even if the ball went in the gutter, saying good job Courtney. She loved it! I think we will definitely have to take Courtney bowling again. I will never forget how generous Mike and Shaun were, nor the smiles on Courtney’s face.

After we left the bowling center we decided to all go out to eat. We hardly ever do this anymore. We went to the Olive Garden nearby, it was pretty busy and we were told it could take up to thirty minutes to get a table with a place for Courtney to be away from everyone. But once again God blessed us and within ten minutes we were escorted to a large table set off to the side all by itself, perfect for Courtney. We had a wonderful server named Brandon, the best server I have ever had before. He was so friendly and attended to all our needs, he was perfect. Since this was the 13th day of the month, Courtney’s birthday date each month, I asked Brandon if they could sing Happy Birthday to Courtney. They sang and it was wonderful.

Later that evening after we arrived home, Courtney started to gag and throw up, we had to take her to the ER just after midnight. As the ambulance was pulling up to the hospital like so many times before, fear took hold of me. I silently thanked God for our wonderful Girls Night Out, for Mike, Shaun and Brandon. I also prayed, please Lord don’t let this be the time that you take our angel back to heaven. Just a little more time…..

Courtney had IV fluids for several hours and then we were allowed to take her home, trying to keep her from being exposed to so many illnesses that she could get at the hospital. She had another day on Friday of being sick but we were able to handle it at home this time.

Courtney will be having her 6th Miracle Birthday celebration on April 13th.
Stella Turnbull

Sami and Cole Abraham

Scott, Nikki and Leah Miller

Nico and Bella Andrade

Roman Anderson

Samuel Schoenborn

Sophia Salus

Sydney and Scott Craven

Sydney Potjer and friends at the 2008 conference

Madison Smith

Sydney Potjer and Malorie Fox

The Sykora family with Santa

Sydney Horak - Miss Wheelchair Texas
The Eide family

The Hoffmann family

The O'Neill family

The Hoffman family

The Murray family

The Madsen family

The Kennedy Family

The McHale family

The Meigs Family

The Cole family

Abbey and Jordan Partridge

The Madsen family

Taylor and Sydney Schoenborn

Treyton, Stella and Sayer Turnbull

The Macellero Family at Disney

Veronica St. Ongé

Zeke and Lily Lerner

The Henry family

Families of SMA
Gwendolyn’s PVC Play Apparatus

By Bill Strong

Our daughter Gwendolyn is 13-months-old and has SMA1. As I’m sure many of you can relate, we have found it difficult adapting toys to suit Gwendolyn’s needs to keep her mind challenged and to allow her to play independently. Light toys, feathers, ribbons, rings, balloons, balls, music, DVDs, etc. are all favorites, but we’ve found that because she needs to be on her back most of the time, hanging her favorite things directly above her makes her happiest.

We’ve used various items to hang toys from in the past, but we’ve found that while most of them did some things well, none of them could handle her DVD player, a musical mobile, or other large items as well as toys for Gwendolyn to grasp at varying heights. So, we learned from what has worked and what hasn’t worked and created a new play apparatus for Gwendolyn made completely of PVC pipe. I know time is precious for all of us caregivers, and the good news is this play apparatus can be completed in well under 2 hours and costs less than $30 using basic items you can find at your local hardware store.

It’s been a huge hit with Gwendolyn!!! We hang her toys directly above her from the apparatus rails and cross bars using string loops, velcro straps, or plastic interlocking rings. We have found Gwendolyn especially likes bells, maracas, and wind chimes, which she bangs together so she can experience cause and effect. We can easily change her favorite toys and rotate in new toys to keep her stimulated. Her DVD player, which she absolutely loves, attaches to the apparatus using velcro straps or ribbon and now plays directly above her. She can now lay directly beneath her DVD player and giggle at her favorite Elmo DVD while playing with her hanging toys at the same time – she loves it. And her favorite musical,

continued on page 63