Welcome to Cure SMA

We are excited to announce that Families of SMA is now Cure SMA. In addition to this name change, we’ll also be making a number of other improvements to the way we communicate, starting with a new look and new website.

In 1984, a small group of families joined together so they could fund research toward a treatment and cure for SMA, and find new ways to support each other.

In the last 30 years, that small group has grown to a community of over 110,000 members and supporters. In those 30 years, we’ve funded $57 million in SMA research. Each year, we reach thousands of families through our newly diagnosed care packages, care series booklets, equipment pool, and other family support services. And our Annual SMA Conference is the largest SMA conference in the world.

Perhaps most importantly, we’ve made tremendous progress in research. Today there are more clinical trials and more paths to a treatment than ever before.

This is a time of great progress and promise for our community—thanks to the passion and support of our volunteer chapters and member families. And in order to continue growing, we need to speak clearly to a wider audience. That’s our primary goal in making these changes—to attract even more people to be a part of our work so that we can accelerate momentum toward a treatment and cure.

We are also committed to making it easier for our community to find information and connect with each other, and for all of our fundraisers to plan and host successful events. To that end, we have made significant improvements to our website and database, based largely on your feedback and ideas.

Be assured that our primary mission remains unchanged. We are as dedicated to funding new research breakthroughs, and to providing the compassionate, knowledgeable support that our families have come to rely on, as we have always been.

“Cure SMA” is our vision, and it’s also our rallying cry—it’s why we keep funding research and raising awareness. We’re confident that we can and will achieve that promise, and we’re proud to have it as our name.

If you have any questions on Cure SMA, please reach out to Kenneth Hobby, president, at kenneth@curesma.org. Or, feel free to reach out to any staff or board members. We’d love to chat with you about what’s next for us.

With Gratitude,
Kenneth Hobby, president
Rich Rubenstein, chair of the board of directors

On the Cover: Brady Chan, SMA Type II and Lucas Chan.
Cure SMA is dedicated to the treatment and cure of spinal muscular atrophy (SMA)—a disease that takes away a person’s ability to walk, eat, or breathe. It is the number one genetic cause of death for infants.

Since 1984, we’ve directed and invested in comprehensive research that has shaped the scientific community’s understanding of SMA. We are currently on the verge of breakthroughs in treatment that will strengthen our children’s bodies, extend life, and lead to a cure.

We have deep expertise in every aspect of SMA—from the day-to-day realities to the nuances of care options—and until we have a cure, we’ll do everything we can to support children and families affected by the disease.

Learn more about how you can help us reach a treatment and cure at www.cureSMA.org.

VISION AND MISSION STATEMENT
Cure SMA leads the way to a world without spinal muscular atrophy, the number one genetic killer of infants. We fund and direct comprehensive research that drives breakthroughs in treatment and care, and we provide families the support they need for today.

OUR VALUES

Innovation
Our commitment to a treatment and cure is not just about seeking solutions—it’s also about creating them. We’re working with some of today’s sharpest minds to advance a diversity of approaches and champion the most promising discoveries and methods.

Balance
As relentlessly as we pursue a treatment and cure, we are also strategic. We know the fastest way to a future without SMA is to take a comprehensive, unbiased approach to research and maintain a balance of optimism and realism.

Collaboration
Our community is everything to us. We would not have made it this far in our fight without the invaluable contributions of our researchers, doctors, and families. Together, we are—and always will be—stronger than SMA.

Respect
There is no “right way” to live with a disease like spinal muscular atrophy. Every person’s experience is different, and it’s every family’s right to decide what SMA means for them.

Compassion
Thanks to the Cure SMA community, no person is ever alone in facing this disease. We offer unconditional support to people affected by SMA and communicate openly and honestly, giving them clear and accurate information.

Determination
Our work is not done until we have a treatment and cure, and we’ll remain strong in our fight no matter what challenges come our way.
The 2015 Annual SMA Conference will be held in Kansas City, MO at the Westin Kansas City at Crown Center from Thursday, June 18th through Sunday, June 21st, 2015.

The entire community will gather once again to lend each other support and strength and to learn about the latest advances in research and care.

Earlier this year, we announced that our 2015 Annual SMA Conference will be held in Kansas City. We’ll be opening registration for the conference in just a few weeks. In the meantime, read a little more about the city and the hotel where our conference will be held, and get ready for the barbeque capital of the United States.

Known as the City of Fountains and home to some of the world’s best jazz and barbeque, Kansas City serves up urban sophistication with Midwestern hospitality.

The Annual SMA Conference will be held at the Westin Kansas City at Crown Center. In addition to the hotel, the Crown Center is comprised of hundreds of stores, restaurants and theatres—all just a stone’s throw from the hotel’s front doors.

The Science City at Union Station is directly connected to the hotel via The Link, an above-ground pedestrian path.

The hotel is also a short distance from the Kansas City Zoo, Imax theatre, Sea Life Aquarium, Legoland Discovery Center, Kaleidoscope, Worlds of Fun, and the Toy and Miniature Museum, the Kansas City Historic Jazz District and several museums and monuments.

We can’t wait for the conference—we hope you’re planning to join us!
Cure SMA provides critical services to families recently diagnosed with SMA

Cure SMA has invested nearly $57 million in SMA research in our 30-year history. But funding research is not the whole of our mission. We also provide compassionate, respectful support for all individuals and families who are affected by SMA.

One of the primary ways we do this is through outreach to newly diagnosed families. We know that an SMA diagnosis can be overwhelming. You may be confused, scared, frustrated, or uncertain—and your emotions may change from day to day, sometimes even hour to hour. But you are not alone.

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

Every newly diagnosed family who contacts us receives a care package from Cure SMA, full of toys appropriate to the child’s type of the disease, impartial information for parents, and useful items suggested by other SMA parents based on their own experiences. Since 2009, the Type I care packages have been generously funded by the Jacob Isaac Rappoport Foundation, and Adi & Shaina Rappoport, in memory of their son Jacob.

In just the past year, Cure SMA was contacted by over 400 newly diagnosed SMA families. Over 3,000 support items were shipped during the year, including Newly Diagnosed Care Packages, Wagons for SMA families and information for Medical Providers.

We provided 300 pieces of vital medical equipment to SMA families across the United States. Some of the resources provided by Cure SMA include:

- Providing SMA information to all newly diagnosed families helping each to understand and manage the disease better;
- Sending care packages of toys that have been recommended for SMA children;
- Housing a shared medical equipment pool which is available to families at no cost;
- The Annual SMA Conference; the nation’s largest gathering of those affected by SMA and leading researchers from around the world.

At our Annual Conference, we offer special content and support for newly diagnosed families. We offer scholarships so that every newly diagnosed family can attend free of charge and connect directly to the Cure SMA community. Through this program, up to four family member’s registration fees are covered, in the hopes that all newly-diagnosed families have the opportunity to attend the largest SMA conference in the world. At the conference, they can gather the most up-to-date information and care on SMA, and also connect with other families from around the world. These scholarships are all made possible due to the support from many donations, fundraisers and sponsorships specifically given to help more families attend this amazing conference. The Erin Trainor Memorial Fund is currently building an endowment for the permanent funding of the Newly Diagnosed Conference Scholarships, allowing newly diagnosed SMA families/individuals the opportunity to attend The Annual SMA Conference.

We won’t stop working toward a world without SMA, but until we have a treatment and cure, we’ll do everything we can to improve quality of life for children and families affected by the disease today.
The 2014 Annual SMA Conference Was A Great Success

The 2014 Annual SMA Conference was a fantastic success with almost 1,400 families, researchers and professionals in attendance! This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it for SMA families. The interactions between the researchers and families at one conference are so special. The Annual SMA Conference also provided our children an opportunity to make new friends and have a great time. There were so many wonderful events that made this conference incredibly special and successful for everyone.

The family portion of the Annual SMA Conference began Thursday, June 12th with the Newly Diagnosed Program where families, who were diagnosed from 2013 to present, were able to attend this special session prior to all of the other families’ arrival. It was a great way to slowly introduce new families to the conference as well as other families and professionals on a much a smaller scale. This program included:

- An introduction to the Conference and SMA Community
- Understanding Genetics and the Disease
- Evidence, Hope and Hype: Finding the Balance
- Life After Diagnosis – Parents Share Their Journey
- A Meet and Mingle with families, doctors, chapter officers and board members
- Optimal Care for SMA Type I
- Optimal Care for SMA Type II and Type III
- A Grieving Parents Session

While the conference was underway for families, the 18th Annual SMA Researcher Meeting was being held at the same time and is the largest SMA researcher conference in the world. Cure SMA organizes the conference and financially underwrites the meeting by covering hotel, travel and registration for research presenters. There were over 250 researchers that attended from around the world. These researchers represent institutions, biotech and pharmaceutical companies from all over. The personal connections made between families and researchers means so much to everyone who attends.
Highlights from The 2014 Annual SMA Conference Meet and Greet

The 2014 Annual SMA Conference, held in National Harbor, Maryland at the gorgeous Gaylord National Resort, began for all attendees on Thursday evening with our signature kick-off event, the Meet and Greet & Family Fun Fest. We welcomed new families, returning families, researchers, medical professionals and friends from all over the world.

This Thursday evening is a tradition at the Annual SMA Conference where everyone comes together to begin a weekend full of making new friendships and connections, learning the most up to date information, and being a part of a wonderful and caring community.

The ever-so popular Researcher Relay Race is always a highlight at the event as both researchers and kids line-up to see who can cross the finish line first, with one rule: that the researchers have to race in a manual wheelchair! All of the attendees stand around the outside of the course cheering on the racers to see who will be announced as the winner, and it is always the kids who cross the finish line first, leaving the researchers behind in the dust!

Another major component that makes the Meet and Greet such an enjoyable event is the Family Fun Fest with carnival games that are lined-up around the edges of the room. Kids and families rotate around the endless amounts of games ranging from Bozo Buckets, to a Treasure Chest, to the LollyPop Tree and so many more. The games are adapted so that everyone who plays has a chance to win some great prizes. There was even a candy table station and tattoo station, filled with goodies and fun for all!

The Meet and Greet is a special time for all attendees to come together in a fun and relaxed setting, prior to the start of the workshops.

To see photos from the 2014 Annual SMA Conference, please visit the Cure SMA FaceBook page.
ATTENDEE VIDEO FROM THE 2014 ANNUAL SMA CONFERENCE ONLINE NOW

Watch the main video from The 2014 Annual SMA Conference here:

http://www.youtube.com/watch?v=F-EGuCEMgsE&feature=youtu.be
Family Friendly Research Poster Session at The 2014 Annual SMA Conference

At the 2014 Annual SMA Conference, the second Family Friendly Research Poster Session was held. During the event, 31 research groups presented their scientific findings, including 10 different SMA drug programs. The unique event allowed for one-on-one interactions among the 1400 families and researchers in attendance to share information.

Posters Included:

- Allan Kaspar PhD from AveXis presenting on “Treating Type 1 SMA With Gene Therapy: chariSMA (scAAV9-SMN)”.
- Arnab Chatterjee PhD from CALIBR presenting on “Finding Drugs That Keep Motor Neurons Alive”.
- Arthur Burghes PhD from The Ohio State University presenting on “Creation And Correction Of SMA In The Pig Using Gene Therapy And Further Development Of Morpholino ASO Therapy”.
- Barrington Burnett PhD from The Uniformed Services University of the Health Sciences presenting on “Slowing SMN Protein Breakdown To Treat SMA”.
- Cathleen Lutz PhD, Laurent Bogdanik PhD from The Jackson Laboratory presenting on “Mouse Models Of SMA—What Can We Learn From A Mouse?”
- Christian Lorson PhD from University of Missouri presenting “Anti-Sense Oligonucleotides In SMA: Design And Function”.
- Christine DiDonato PhD from Northwestern University presenting on “Animal Models, Cell Requirements And Therapeutics For Milder Forms Of SMA”.
- Diane V. Murrell LCSW from Texas Children’s Hospital presenting on “The Experience Of Families Of Children With Spinal Muscular Atrophy Type 1 Across Health Care Settings”.
- Elizabeth McNeil MD from National Institute of Neurological Disorders and Stroke presenting on “NINDS-Current SMA Related Research”.
- Eric Dessaud PhD, Rebecca Pruss PhD from Trophos presenting on “The Path To The Discovery And Development Of Olesoxime (TRO19622) For SMA”.
- Panayiota Trifilis PhD (PTC Therapeutics), Sergey Paushkin PhD (SMA Foundation), Anne Marquet PhD (Roche), Irene Gerlach PhD (Roche) from Roche presenting on “An Oral Small Molecule Splicing Modifier For SMA Entered The Clinic”.
- Jeff Jasper PhD, Daren Hwee from Cytokinetics presenting on “Tirasemtiv Increases Skeletal Muscle Performance In SMA Mice”.
- Jocelyn Cote from the Cote Laboratory at University of Ottawa presenting on “Investigating The Contribution Of SMN’s ‘Favorite Protein Flavour’ (Methylated Arginines) In SMA”.
- Katherine Klinger PhD, Alison McVie-Wylie PhD from Genzyme Corporation presenting on “Progress Toward Gene Therapy For SMA”.
- Kathryn Swoboda MD, Rebecca Hurst MS RD CSP CD, Elizabeth Miller from the University of Utah presenting on “Pilot Study Of Glucose Load Tolerance And Fasting In SMA Type II”.
- Kejun Han PhD from the University of Colorado presenting on “Regulation Of SMN Localization By Ubiquitination”.
- Ko Laboratory at University of Southern California presenting on “Motor Circuit Defects And Restoration In SMA Model Mice”.
- Kristina Lemonidis from Isis Pharmaceuticals presenting on “Developing An Antisense Drug For The Potential Treatment Of SMA”.
- Lyndsay Murray Phd from University of Edinburgh presenting “What Makes Motor Neurons Vulnerable In SMA?”
- Marc-Olivier Deguise from the Kothary Laboratory at the University of Ottawa presenting on “Skeletal Muscles: Are They Important Contributors To Initiation Of Symptoms In SMA?”
- Matthew Halanski MD from the University of Wisconsin presenting on “Spinal Muscular Atrophy: A Multicenter Multidisciplinary Assessment”.
- Michael Tones PhD from Pfizer presenting on “Many Shots On Goal: The SMArt Thing For SMA”.
- Oscar Mayer MD, Vanessa Battista RN MS CPNP CCRC from Children’s Hospital of Philadelphia presenting on “Assessing The Challenges In Parental Medical Decision Making In Children With SMA-1 And SMA-2”.
- Novartis presenting on “SMA Drug Development at Novartis”.
- Rubin Laboratory at Harvard University presenting on “Discovering New SMA Therapeutics Using Human SMA Motor Neurons”.
- Sara Custer PhD from the Androphy Laboratory at Indiana University presenting on “Novel Drug Development For The Treatment Of SMA: A Two-Pronged Approach Aimed At Increasing SMN Production & Stabilizing SMN Protein”.
- Stephen J Kolb MD from The Ohio State University presenting on “SMA Infant Biomarker Study - Super Baby Progress In 2014”.
- Sumner Laboratory at Johns Hopkins University presenting on “Impaired Motor Axon Development In Spinal Muscular Atrophy”.
- Teresa Patitucci from the Ebert Laboratory at the Medical College of Wisconsin presenting on “Understanding SMA’s Impact On Cells In The Neural Circuit”.
- Yimin Hua PhD from the Krainer Laboratory at Cold Spring Harbor Laboratory presenting on “Mechanism Of Action Of ISIS-SMNRx”.
- Yong-Chao Ma PhD from Northwestern University presenting on “Traffic Jam In Motor Neuron And SMA Pathogenesis”.

2014 CONFERENCE
Family & Researcher Dance Party
Pajama Party
On Saturday evening, was the more relaxing event, Movie and PJ Party for families. Each person was greeted with a table full of treats and bags of popcorn. Families were provided with sheets and pillows to make the movie experience more comfortable, and also encouraged to sport their comfiest PJs. The movie that captivated the audience was Monster’s University, which was a huge hit for the children while parents caught up with old friends and met new ones.

Cure SMA Adaptive Arts Project for The 2014 Annual SMA Conference a Huge Success!

The Jacob Isaac Rappoport Foundation awarded Cure SMA with funding for an Adaptive Arts Project for the Annual SMA Conference which was a huge hit with all the children who attended!

At last year’s 2013 Annual SMA Conference we introduced families and kids to a fun way to create art. It was such a huge hit that we wanted to have Zot Artz back, and thanks to the sponsorship by the Jacob Isaac Rappoport Foundation, the creativeness and fun continued at this year’s conference too! All of the children at The 2014 Annual SMA Conference had a great time participating in such a fun and inspiring event. Children of all ages were able to take home wonderful art pieces that they painted with their wheelchairs. Cure SMA was excited to be able to offer kids this fun opportunity to express their artistic personalities!

A new addition to this year’s event was a bubble maker and side walk chalk which made the kids that much more excited to try all these artistic items out!

Zot Artz designs and makes adaptive art tools so that children can create amazing art pieces with adaptive tools and assistive technology which transforms wheelchairs into giant paintbrushes. Children of all abilities and ages can stamp, draw and paint to make their mark.

Cure SMA would like to thank the Jacob Isaac Rappoport Foundation for their generous support and making this fun event happen at the Annual SMA Conference!

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

To learn more about Zot Artz, please visit http://www.zotartz.com/.

the jacob isaac rappoport foundation
fighting spinal muscular atrophy
in memory of our shooting star
2014 CONFERENCE

Photo Booth

[Images of people posing with costumes and props]
This year’s Children’s Program was such an interactive and exciting time, had by all! Thanks to so many of our wonderful volunteers who helped make this weekend incredibly special for everyone, especially all of the children. The kids stayed engaged by participating in an array of arts & crafts, countless toys, movies, Wii video games, special entertainment guests and so much more!
Thank You to All of the Conference Children’s Program Volunteers

Each and every one of our volunteers has a major impact on the atmosphere and success of our conference, and every year their impact radiates throughout the weekend! Their long days of hard work provide families with the reassurance that their children are well cared for and are having fun, while they attend workshops, learning the latest information on SMA. The enthusiasm they provide for the kids in the children’s program, during the carnival games, the Dance Party and at our other events, is contagious with each event. Our volunteers take great pride in helping to ensure that at each conference they attend, families leave with the feeling that this was the best conference yet. Without you all, our conference would be no where near what it encompasses today. Thank you for everything!

We would like to especially thank the following volunteers who return year after year, leaving their jobs and families at home to help SMA families:

Volunteer for 25 years

George Ghorbanian,
Chicago Police Officer

George has volunteered at the conference since the Children’s Program started back in 1990. The 2014 Annual SMA Conference will be George’s 25th consecutive conference!! He takes time away from his job as a Chicago Police Officer, as well as his family and busy life, to travel each year and support the Children’s Program. George is the definition of a truly dedicated volunteer. Everyone at Families of SMA would like to thank George for all of his hard work and dedication! George is not only one of Chicago’s finest but clearly one of SMA’s finest.

Volunteer for 23 years

Patti Slojkowski
Dental Hygienist
Patti has two teenage children.

Volunteer for 20 years

Steve Smith
Physical Therapist
Steve has 3 daughters, ages 1 - 10.
Volunteer for 16 years

Kelly Milito
Hair Stylist
Kelly has 4 children, ages 12 - 19.

Volunteer for 15 years

Jeff Harris
City Planner in West Chicago

Volunteer for 14 years

Kelly Basso
Surgical Consultant
Kelly has 3 teenage and adult children.

Volunteer for 13 years

Peg Bailey
Nurse

Mary Blume
Hospital Administrative Assistant
Mary has 2 adult children.

Katlyn O’Brien
Elementary School Teacher

Volunteer for 6 years

Dan Rushton
Realtor

Hugo Trevino
Recent College Graduate
majoring in
Spanish & Translation Studies

Caitlin Trainor
Recent college Graduate with a degree in
Psychology

Grace Trainor
College Student studying Exercise and Movement Science

Mary Kate Venedam
Recent College Graduate with a degree in Film and Media Studies

Eileen Venedam
College Student studying Business

Annie Venedam

Jackie Staples
Event Planner
Jackie has a 8 year old son.
Volunteer for 4 years

Joy Martin
Physical Therapist
Joy has 3 children, ages 5, 7 and 9.

Donna Budil
Preschool Director
Donna has 2 kids, ages 11 and 14.

Brian Blume
Accountant
Brian has 2 adult children.

Kelli Blume
Graduate Student in Social Work

Kevin Blume
College student in Accounting

Megan Milito
College student in Special Education

Alec Basso
College student in Nursing

Mike Graney
Vice President of United Services

Jackie Graney
Director of Studio 22 Dance Studio

Collene Cahoy
College student in Elementary and Special Education

Caroline LaPelusa
College student in Nursing

Special Thanks
Volunteer for 3 years
Joy Martin
Ph  y s i c a l T h e r a p i s t
J o y  h a s 3 c h i l d r e n , a g e s 5 , 7 a n d 9 .

D onna Budil
Preschool Director
D o n n a  h a s 2 k i d s , a g e s 1 1 a n d 1 4 .

B r i a n  B l u m e
Accountant
B r i a n  h a s 2 a d u l t  c h i l d r e n .

K e l l i  B l u m e
Graduate Student in Social Work

K e v i n  B l u m e
College student in Accounting

M e g a n  M i l i t o
College student in Special Education

A l e c  B a s s o
College student in Nursing

M i k e  G r a n e y
V i c e  P r e s i d e n t  o f U n i t e d  S e r v i c e s

J a c k i e  G r a n e y
D i r e c t o r  o f S t u d i o 2 2  D a n c e S t u d i o

C o l l e n e  C a h o y
College student in Elementary and Special Education

C a r o l i n e  L a P e l u s a
College student in Nursing

S p e c i a l  T h a n k s
V o l u n t e e r  f o r 3  y e a r s
V o l u n t e e r  f o r 2  y e a r s
Special Thanks

Volunteer for 3 years

- Lexi Basso
  College student in Business Law

- Tori Zeman
  College student in Occupational Therapy

- Emily Yagihashi
  College student in Cellular & Molecular Biology

- Kailee Breslin
  College student in Speech & Language Pathology

- Danielle Austriaco
  College student in International Studies

- Alexandra Ferro
  College student in Management

- Maeve Gallagher
  College student in Communications and Public Relations

Volunteer for 2 years

- Georgia Slojkowski

- Meghan Martin

- Danielle Kirincich
  College student in Psychology

- Amanda Peck

- Meghan Villano

- Haley Besler
  College student in Communications
Annual SMA Conference - One Family’s Experience

Contributed by Stacey Zimmerman

My daughter Peyton turned 1 this past July. It felt like such a momentous occasion. When she was born, I expected to spend her 1st birthday celebrating all the milestones she had achieved over the course of the year. Instead, I was celebrating the fact that she was still alive and thriving despite the fact that her muscles do not work and she can do nothing for herself. Children like this have strength of spirit that many of us can only strive for over the course of a lifetime. I still hate her diagnosis, but if this is the path we are on then we have to make the best of it.

For our family that means taking Peyton out as much as possible. If the reward outweighs the risk, then count us in! That’s how we ended up going to the Annual Spinal Muscular Atrophy Conference in Washington D.C. this past June. My husband recently started a job with the Army as a military psychologist and had to be at training so Peyton, my mom, and I headed north to D.C. Peyton has so much medical equipment, and I am terrified of it getting lost or broken on an airplane so we drove and made it a two day drive. The conference was held at the Gaylord Hotel and Convention Center, a very, very nice place to stay. It was well worth the cost though just for the convenience of having to hop on the elevator to attend seminars and events instead of packing Peyton and her equipment in to the van every day.

The conference was a wonderful experience. Cure SMA holds both a family conference and researcher’s conference at the same time in the same place so that patients, caregivers, researchers, pharmaceutical reps, and doctors can all meet, mingle, and interact with each other. I think this is what makes this conference so forward thinking and special. Cure SMA focuses on making this one large community of everyone involved with or affected by SMA. There is no us versus them. The social events are designed so that everyone co-mingles and gets to know each other and learn from each other.

The seminars covered a gamut of areas from respiratory to nutrition to toy adaptation to much more. We attended the respiratory seminar run by Dr. Schroth. Fortunately, I did not learn a lot which means our local pulmonologist Dr. Brooks is doing a great job. However, it was good to hear the same things from a leading doctor to confirm that we are where we need to be with Peyton’s respiratory care - bi-pap, cough assist, suctioning, respiratory treatments, etc. Main point - Keep the lungs clear of secretions and we will keep our babies healthy and out of the hospital. Is the hospital avoidable? Not with our kids, but we can minimize the number of times we go if we stay on top of respiratory care.

The second seminar that stuck with me was nutrition. Nutrition has never really been an interest to me, so I find the idea of making all of Peyton’s food into a formula and making sure she gets all the nutrients she needs is extremely daunting. I did not leave the seminar feeling any less intimidated especially after all the different types of options families have, but I did leave with the idea that Peyton needs a dietician. Her GI doctor is good for the basic GI stuff, but I think a dietician will be able to give a little bit more individualized care than just prescribing a readymade formula that does not specifically take in to consideration the needs of a child with Spinal Muscular Atrophy.

We went to several other seminars as well. I was not successful at the Toy adaptation seminar. I broke the toy. Fortunately my brother in law was able to fix it, obviously not my area of expertise. The seminar for Type I’s was really great. It was good to meet other families and see how they manage things. Everyone was able to ask questions, give advice, and generally see that our kids are happy. It was so nice to be in an environment where I did not get stared at because I was suctioning my child or asked what was wrong with my child. I do not mind it actually, but it was still nice to not feel so isolated and different from the families around me. It was also inspiring to see all the families with older Type I’s. We have been told so many times that we will be lucky if Peyton makes it to 3 years old. I get it; we will be, but it was so encouraging to see Type I’s who were 5, 8, 13. It can happen.

All in all the seminars were great, meeting other families was wonderful, but one of my favorite parts of the trip was participating on a research panel for patients and caregivers. The day before the official conference started, eight of us had the opportunity to let doctors, researchers, and pharmaceutical companies know what is important to us in a clinical trial outcome. There were about 100 or so people at this workshop that are directly involved with clinical trials. It was a privilege to be there. We all acknowledged that the ultimate goal
is a cure, but until that happens, what do we want from these trials? As panelists, we ranged from weak Type I’s to Adults with SMA Type III. We represented all different types and degrees of strength. Despite the differences in the specific outcome for each type, we all agreed that even a small improvement in function, especially respiratory function, is a huge change in the quality of life. If I could suction a few minutes less a day or have Peyton off the bi-pap for a longer period of time or do one less respiratory treatment a day, which would be huge. Of course, researchers want more quantitative outcomes but they understood and were receptive to what we were telling them. The challenge for them is to come up with assessments that can provide the type of measurements they need.

I can say without a doubt that I do not regret my decision to take Peyton with me. She thrived. She loved to be up and about, staring and observing. She got to see so much and meet so many new people. Things she would never have experienced if I had been too scared to take her out of her safe sterile bubble. She is happy at home no doubt, but she loves to go out. Her entire demeanor changes when we go on a new adventure. Her little eyes dart all over the place taking in the scene and the people. Her face shines and her little smile lets me know this is what’s best for her. This trip was the best way for me to find out exactly how important it is for her mental and emotional state to be out and about as much as she can manage. We watch and listen and head home the moment she looks tired, but at least she is out and having as normal a life as her little SMA body can handle. This diagnosis may limit her but it will not define her and it will not keep her from going on adventures.

In Loving Memory of Peyton who passed away on July 25, 2014.

A very special thank you to the wonderful support we have received these past 5 years from the Expo Convention Contractors, and Jennifer Miller Smith, Aaron Smith and family of Miami, Florida. You truly have an important impact on making the conference such a great success year after year with all of your help throughout the conference and for all of the wonderful signs and banners you donate to our conference. Thank you for all you do!
Cure SMA Thanks the Sponsors for The 2014 Annual SMA Conference

Cure SMA is pleased to announce that we have 25 Sponsors registered for the upcoming 2015 Annual SMA Conference.

Cure SMA thanks all 25 of the Sponsors for their generous support of The 2014 Annual SMA Conference. These sponsors are partners in our community who are critical to success in the battle against SMA.

These partners contributed to scholarships and family assistance programs for the 2014 Conference and for travel and lodging expenses for the International SMA Research Meeting and the CME Conference. Without their support many patients, families, medical professionals and researchers would never be able to attend and join together in this fantastic meeting.

THANKS TO THE FOLLOWING 2014 ANNUAL SMA CONFERENCE SPONSORS:

Presenting Sponsor:

Platinum Sponsors:
Cure SMA was thrilled to have 25 Exhibitors attend the 2014 Annual SMA Conference!

Cure SMA thanks all of the 25 exhibitors for their generous support of the 2014 Annual SMA Conference. Exhibitors who attended the Annual SMA Conference were able to promote their products to SMA families, Researchers and Medical Care Providers from all over the United States. These exhibitors are partners in our community who are critical to success in the battle against SMA.

Cure SMA would like to thank the following Platinum Exhibitors:

- Quest Diagnostics
- BAYADA Pediatrics
- Sunrise Medical
- Permobil
- Ki Mobility

Cure SMA would like to thank the following Gold Exhibitors:

- LC Technologies, Inc.
- Triumph Mobility Inc.
- 3E Love
- Tobii ATI
- Philips Respironics
- Maxim Healthcare Services

Cure SMA would like to thank the following Silver Exhibitors:

- Thomashilfen
- BlueSky Designs
- Counsyl, Inc.
- PromptCare
- RespirTech
- Sundance Enterprises, Inc.
- Percussionaire
- SureHands Lift & Care Systems

Cure SMA would like to thank the following Bronze Exhibitors:

- Electromed, Inc.
- Hill-Rom
- Easy Walking Inc.
- WaterWayBabies
- AbleGamers Foundation
- International SMA Patient Registry

Save the date for The 2015 Annual SMA Conference, which will be held in Kansas City, MO, June 18 – 21, 2015! More information will be available soon on the Cure SMA website.

If you are interested in being added to our mailing list to receive exhibitor opportunities for the 2015 Annual Conference, please email exhibitor@curesma.org.
Thank you to all of the following companies who so generously donated items for our 2014 Annual SMA Conference. We are grateful that you cared enough to help support this great cause by donating items for our attendee registration bags and other events.

This conference does not happen without a tremendous amount of work and support as well as kind and generous donors like you.
Cure SMA Honors Scott Geller with 1st Annual Community Partnership Award

We are proud to announce Scott Geller, who chaired the Chesapeake Chapter Golf Outing for fourteen years, as the first ever recipient of the Annual Community Partnership Award. The award was presented at The Annual SMA Conference this June in Washington D.C.

Scott first attended the Chesapeake Chapter Golf Outing as a guest. Scott was immediately attracted to the grassroots efforts of the organization, its commitment to help families, and the real difference that fundraising can have to combat this disease. He quickly took on the role of tournament chair.

Scott took the event to extraordinary heights over this fourteen year journey. Without him, the golf event couldn’t have reached the many organizations it did in the financial community—a wonderful testament to the many companies and individuals who believe in Scott and CRM, and also support the work of Cure SMA.

During Scott’s tenure as Chair of this event, more than $1.8 million was raised for Cure SMA.

Scott saw a need to make a difference in the lives of so many children that he never met. We are ever grateful for his contribution and proud to present him with this award on behalf of the entire SMA community.

Cure SMA Awarded Grant from the National Institute of Health for the 2014 Spinal Muscular Atrophy Research Group Meeting

Cure SMA has been awarded a grant from The National Institute of Neurological Disorders and Disease (NINDS) and the Office of Rare Disease Research at The National Center of Advancing Translational Sciences (NCATS) of the National Institutes for Health (NIH) to support the 2014 SMA Research Group Meeting. We thank them for the fantastic support that helps bring together the SMA research community along with our families.

The SMA Research Group Meeting is the largest research conference in the world for SMA. It was held June 12, 13, 14 at the Gaylord National in National Harbor, MD. We had over 250 researchers that gave 110 updates on the latest breakthroughs in SMA research. Researchers were registered from 15 different countries, 70 different academic and government organizations, and 18 biotech and pharmaceutical companies.

Cure SMA hosts and organizes the SMA Research Group Meeting with the following goals:

- To enable open communication of early, unpublished scientific data, accelerating the pace of research
- To provide a forum to discuss timely topics in SMA openly with the entire research community
- To create a sense of community among SMA researchers, resulting in productive research partnerships
- To promote cross-disciplinary dialog among basic researchers, clinicians, and industry representatives
- To provide a venue to efficiently integrate new researchers and drug companies into the community
- To promote interaction between trainees and leaders in the field to build the future of the SMA research community
- To motivate SMA researchers by providing direct interaction with patients living with SMA

The research conference is held together with The Annual SMA Conference, which is the largest conference in the world for families affected by SMA and for medical professionals involved in providing support and care for SMA patients. Running the two conferences simultaneously gives the unique opportunity for SMA families, researchers, and clinicians to interact and meet each other.
SMA Researchers Talk About the Importance of the Spinal Muscular Atrophy Research Group Meeting

The SMA research group meeting, held each year as part of our Annual SMA Conference, is an anticipated event in the SMA research community. This meeting has a tangible impact on achieving our mission of a world without SMA.

Over the last decade, we’ve seen many advances in SMA research, from new techniques in gene therapy to drugs that show promise in slowing or stopping the progress of the disease. With such great promise in the research landscape, we know that no single group can develop a treatment for SMA alone—it requires collaboration between academics, industry, government, and families. From the start, Cure SMA has been working to bring all of those groups together, and the research group meeting is one of the primary ways we accomplish this goal.

At the research group meeting, the largest of its kind in the world, researchers share unpublished scientific data, accelerating the pace of research. The meeting also creates a sense of community among SMA researchers, encouraging collaboration and long-standing research partnerships. Moreover, it allows young researchers to meet and learn from leaders in the field, helping to build the next generation of the SMA research community.

“At the SMA Research Group Meeting, there is the opportunity for one-on-one interactions with those at all levels of scientific training from junior to senior investigators. Here researchers are free to ask multiple questions, get advice and help with proposals, and obtain reagents or suggestions on how to perform a technique.”

Arthur Burghes, PhD, Cure SMA Scientific Advisory Board member and Professor at OSU.

“I first attended the meeting in 2010, shortly after joining Isis Pharmaceutical and starting to work on their ISIS SMN-Rx drug development program. That meeting was invaluable for myself and the other Isis attendees, as it was where we held our first advisory meeting for the program and made initial connections with advisors, collaborators, foundation members, and potential clinical investigators, which has set the stage for many of our activities since then.”

Kathie Bishop, PhD, Vice President of Clinical Development at Isis Pharmaceuticals

Finally, and most importantly, the conference motivates SMA researchers by allowing direct interactions with families and patients living with SMA, through events like our Meet and Greet, Family Friendly Poster Research Session, and various panel discussions. These are unique and special experiences for all involved.

“This meeting is the best of its kind. The value of meeting with researchers, physicians and parents all in one place is immeasurable. Parents see how much we care, physicians see how much work is required for drug development, and researchers see how much their hard work is appreciated.”

The meeting is also the main forum in the SMA scientific community for integrating new researchers and drug companies as efficiently as possible.
The 2014 SMA Conference brought together over 1,400 families and 250 researchers from around the world. At the conclusion of the conference, families gathered for a special opportunity to hear about the latest SMA research from the leading experts.

The session addressed new advances, strategies, and challenges in SMA drug development, and leading experts on SMA drug development answered questions about their drug programs. Below slides of the keynotes presentations and the drug company status summaries can be found.

Speakers

Update on Cure SMA Research Activities, Jill Jarecki, PhD, Cure SMA Research Director

Considerations and Importance of Clinical Trial Participation, Thomas Crawford, MD, Professor Of Neurology & Pediatrics, Johns Hopkins University.

Government Panel on SMA Research and Drug Development

- John D. Porter, PhD, Program Director, Neurogenetics Cluster, National Institute of Neurological Disorders and Stroke, NIH
- Tiina K. Urv, PhD, Program Director, Intellectual and Developmental Disabilities Branch, Eunice Kennedy Shriver National Institute of Child Health and Human Development, NIH
- Ali Mohamadi, MD, Medical Officer, Professional Affairs & Stakeholder Engagement, Center for Drug Evaluation and Research, FDA
- Lei Xu, MD, PhD, Medical Officer, Office of Cellular, Tissue, & Gene Therapies, Center for Biologics Evaluation and Research, FDA

Panel on SMA Drugs in Development

- C. Frank Bennett PhD, Senior Vice President, Research, Isis Pharmaceuticals
- Brian Kaspar, PhD, Grant Morrow III, M.D., Endowed Chair, The Research Institute at Nationwide Children’s Hospital
- Douglas Kerr, MD, PhD, Director of Experimental Biology, Biogen Idec

- Pannie Trifillis, PhD, Director, Alliance Management – PTC Therapeutics Inc.
- Katherine Klinger PhD, Sr. Vice President, Genetics and Genomics and Presidential Fellow, Genzyme Corporation
- Michael Tones PhD, Director and Research Project Leader, Rare Disease Research Unit, Pfizer Worldwide R&D
- Rebecca Pruss, PhD, Chief Scientific Officer, Trophos
- Brian Tseng, MD, PhD, Director, Musculoskeletal Diseases, Novartis Institute of Biomedical Research NIBR

Written Drug Program Summaries

Isis Pharmaceuticals

ISIS-SMNRx is an antisense drug Isis is developing to treat SMA. SMA is caused by a loss of, or defect in, the SMN1 gene leading to a decrease in the amount of SMN protein. SMN protein is critical for the health and survival of nerve cells in the spinal cord that are responsible for neuromuscular growth and function. The severity of SMA correlates with the amount of SMN protein. Isis has designed ISIS-SMNRx to potentially treat all types of SMA by altering the splicing of a closely related gene, SMN2, which leads to the increased production of SMN protein. A Phase I clinical study evaluating the safety of single doses of ISIS-SMNRx in children with SMA has been completed.

In this study, ISIS-SMNRx was well tolerated at all dose levels tested with no safety or tolerability concerns. The compound is delivered by an injection into the lower back (an ‘intrathecal injection’) into the space containing cerebral spinal fluid below the spinal cord in order to best distribute the drug to spinal cord motor neurons. The intrathecal injection procedure was also well tolerated in the children. ISIS-SMNRx is now being studied in Phase II clinical studies that are designed to examine the safety and tolerability of multiple doses of the drug given over a longer time period. These studies are being conducted in children with Spinal Muscular Atrophy aged 2 to 15 and in infants with SMA who are copy missing from Word Doc.
AveXis / Nationwide Children’s Hospital

Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type I: This study is a phase I, single-site, dose escalation study to evaluate the safety and efficacy of gene transfer for Spinal Muscular Atrophy Type I (SMA Type I). Enrollment has begun at Nationwide Children’s Hospital in Columbus, Ohio. Infants between 0 and 9 months of age with SMA Type I may be eligible to take part in this first human trial. A total of nine patients will be enrolled to receive a one-time gene transfer infusion. Patients will continue to be monitored at Nationwide Children’s Hospital including physical exams and blood tests for two years after gene transfer.

Eligibility
• Age: Nine months of age and younger
• Must be diagnosed with SMA Type I as defined by the following features:

Inclusion Criteria
• Mutations of the SMN1 gene with two copies of SMN2 (no more and no fewer)
• Onset of disease at birth to 6 months of age
• Weakness of muscles and joints demonstrated at time of enrollment

Exclusion Criteria
• Active viral infection (includes HIV or serology positive for hepatitis B or C)
• Use of invasive ventilatory support (tracheotomy with positive pressure)* or pulse oximetry
• Current illness that in the opinion of the researcher creates unnecessary risks for gene transfer
• Current use of any of the following drugs: drugs for treatment of myopathy or neuropathy, agents used to treat diabetes mellitus, or ongoing immunosuppressive therapy or immunosuppressive therapy within 3 months of starting the trial (e.g. corticosteroids, cyclosporine, tacrolimus, methotrexate, cyclophosphamide, intravenous immunoglobulin, rituximab)
• Patients with Anti-AAV9 antibody titers >1:50 as determined by ELISA binding immunoassay.
• Abnormal laboratory values considered clinically significant (GGT > 3XULN, Bilirubin ≥ 3.0 mg/dL, Creatinine ≥ 1.8 mg/dL, Hgb < 8 or > 18 g/Dl; WBC > 15,000 per cmm)
• Participation in recent SMA treatment clinical trial that in the opinion of the researcher creates unnecessary risks for gene transfer.
• Family does not want to disclose patient’s study participation with primary care physician and other medical providers
• Patient with signs of aspiration based on a swallowing test and unwilling to use an alternative method to oral feeding

Genzyme (a Sanofi Company)

Genzyme (a Sanofi company) has an active program in SMA gene therapy, focused on AAV9-SMN gene delivery into the cerebrospinal fluid (CSF). In studies in mouse models of SMA, we have shown that delivery of AAV9-SMN1 into the CSF results in transfer of the gene to the spinal motor neurons, and expression of SMN protein. The treated mice show significant improvement in strength, motor function, and survival.

We have determined the lowest percentage of motor neurons that must be modified by AAV9-SMN-1 in order to make significant improvement in SMA mice. We have also shown that this level of gene transfer can be achieved using CSF delivered gene therapy in large animals, such as juvenile pigs and non-human primates. These findings provide the foundation for continued development of this therapeutic concept for SMA.

Pfizer

RG3039 (PF-06687859) is an inhibitor of the enzyme DcpS and improves survival and motor function in SMA mice. In the last year, Pfizer has completed transfer of this program from Repligen and has been actively working on the design of clinical trials, biomarker development and understanding the expected efficacious dose. Achieving a better understanding of effective dose prediction is the focus of current efforts for 3 reasons: 1) Data from a Phase 1 study already performed indicated that at doses up to and including 3 mg/kg there was significant inhibition of DcpS in the blood but no change
in SMN, the protein which is deficient in SMA patients. We expect that efficacy will require an increase in SMN. A subject in one of the Phase I studies already performed had an unexpectedly high concentration of drug in their blood: the reason for this is currently unknown.

No further clinical trials have been initiated in the last year. Ongoing work will drive the decision whether and how to progress this compound in the clinic. In the past year we have developed interesting backup DcpS inhibitor compounds, which are being studied in SMA mice and are also looking for other compounds that elevate SMN via other mechanisms.

Trophos

Trophos has released data showing that that patients treated with olesoxime were able to maintain motor function over the two-year period of the study and that typical health complications associated with SMA occurred less frequently than in patients treated with a placebo, leading to better well being. The data presented is from the recently completed international, double-blind, placebo-controlled study involving 165 Type II and non-ambulatory Type III SMA patients, ranging in age from 3 to 25 years old. The results show that olesoxime treatment preserved motor function for two years using the Motor Function Measure scale (MFM) D1+D2 as the primary endpoint. The MFM is a standardized neuromuscular disease-specific functional scale.

In contrast, patients in the placebo arm of the study experienced a loss of motor function starting from a mean score of 39 per cent at baseline to 37.1 per cent after two years. The mean loss of 1.9 points in motor function over the two-year study period confirms that the natural disease progression results in approximately 1 per cent per year loss of motor function in SMA patients. The difference in favor of olesoxime at 24 months is statistically significant (p equals 0.038) while the overall treatment effect on motor function measured at four visits during the study was highly significant (p equals 0.0045). Interestingly, the effect of olesoxime could be detected even within six months following initiation of treatment.

The results observed in olesoxime treated patients are consistent with the working hypothesis set up when designing the study. Data analyses considering age, gender, SMA type or country as covariates show these variables have no influence on the results. The safety of olesoxime was confirmed in the study. Moreover, even though the trial was not designed to address the point, data related to typical SMA complications showed a clear improvement and less frequent disease-associated events such as lower respiratory tract infection or spine surgery to treat scoliosis in patients treated with olesoxime.

Save the date!
Thursday, June 18th - Sunday, June 21st, 2015

2015 Annual SMA Conference  Kansas City, MO
Thank You to the Cure SMA Medical Advisory Council and Scientific Advisory Board for a Great Spinal Muscular Atrophy Conference

At our Annual SMA Conference in June, we asked families to write short notes of thanks to our Medical Advisory Council and Scientific Advisory Board for all the hard work they've put into planning the conference. We were overwhelmed by all of you who took the time out to write a note. Here are just the first 100 we received—a top 100 of sorts.

Thank you to the MAC and to the SAB for all their work, and thank you to all of our attendees for their passion and excitement. You make the conference easily one of our favorite times of the year!

Top 100 MAC and SAB Thank You Messages

1. You bring hope for us, Thank You!
2. Thank you for your hard work and dedication. Elizabeth Lockwood
3. Having a great time.
4. All the best for Cure SMA. 18 years of meetings!
5. Having a great time.
6. Awesome Conference! Thank you so much for your service, time, hard work and caring! I learned so much.
7. The feeling in Meet & Greet was amazing! Thank You!
8. We greatly enjoyed our weekend, God Bless You! Thank You! Stephanie Stauffer, Julice Brucecker
9. Thank you very much for all you do… Miriam H.
10. Big Thanks from “Big D” Texas Katie Sewell
11. Wonderful event, thank you! Kara Christensen #imwithmiller
12. Thanks so much for all that you do! You have opened a world of positivity and optimism for our family and our Gabby. thanks so much, Gabriella, Maggie and Jared Crawford
13. Buscar una cura, Buscar un camino, Es el destino de todos unidos… Por un proyecto comun Families AME Argentina (Find a cure Find a way, is the fate of all states … for a common project AME Families Argentina)
14. Thanks for the hard work! The Lee Family
15. Thank You! From the Barefoot Family
16. Thanks for this great opportunity 2 meet families & doctors & I love all the jets flying around here!! Hoping for a cure! Lewis Martin SMA I 9yrs. Old
17. Thank you for the inspiration. AB Reen
18. Thank you for all you do to help our children and support our families! XO.
19. U guys rock!
20. Thanks so much! The Nataraj Family
21. THANK YOU from Argentina’s parents!!
22. Thank you for your selfless hard work & time! We appreciate you! God Bless! The Briley Faith Turner Family (Josh & Crystal) type O angel baby.
23. Thank you so much! From: Davis Family
24. Thank you for all your hard work. The conference is awesome – as always
25. Thank Ya’ll so much!! God Bless
26. Thanks for all you do.
27. We can’t thank you enough! Ryan Wolff Family
28. Cure SMA is an awesome group
29. Thanks for all the hard work you guys do. It was very informing and fun specially since it was my first conference. Brandon Shaklee
30. Thank you. The Stewarts
31. Overwhelmed & sooooo much information. Thank You Deepi Jagola India
32. Many thanks, The Accardi Family
Thank You Messages

33. Thanks so much! The Heidi Hall Family
34. Thank you. From the Coffey Family
35. Your ongoing service & tireless efforts sustain our world! Much love, Jonathan, Kristen & Max Lasko
36. Because of your dedication, there is hope, thank you, The McLeons
37. Thank you for making this all possible! The Davis Family
38. “Blagodarya” Thank you, all! Lili from Bulgaria & her parents
39. Thank you so much for the Sweet Baby Zane Scholarship! This has been wonderful! Karen Terp
40. There is no better HOPE than coming to a conference & seeing all the smiles! Thank You. The Bertsch Family
41. Thank you for creating an event where everyone with similar questions and concerns can connect and now stay in touch.
42. Thank You! Gary, Ashley, Bella & Hanna Warfield
43. Find A Cure #Team Connor
44. This conference means the world to us. We can’t imagine life without it. Thank you so much for all you do. The Merulla-Bonn Family (Oscar)
45. You guys are the best!! Thanks for all you do! Terri Robinson
46. Thank you for making our lives feel normal
47. Our very first SMA Conference, what an awesome blessing to network with other families. Thank you!!!
48. 1st time conference attendee!! Great contacts info & support!! Ideas to come home with. Thanks for making this happen! You all rock. Barbara Runkle. Atlanta
49. Thank you so much for your hard work! Minnesota
50. Everything was great! Keep up the good work. You’re fighting a good fight. Love, The Silva Family
51. Thank you Cure SMA! Love, Sienna McCollum
52. Cure SMA staff, Thank you! Amazing conference as always! The one place where we all feel “normal”.
53. Thank you all for putting this together, it truly does help us cope. Accardi Family
54. INSPIRATION
55. Thanks, Cure SMA! We love you!
56. Thank you for all the love & caring. Because of you, there can be a difference.
57. Most Sincere Thanks! “Officer Morgan Stewart” & Family
58. This has been our first SMA Conference and we have so enjoyed it! From the bottoms of our hearts, thank you! Love The Bectons
59. Thank you for making this conference possible. It meant the world to my family and daughter to be here. Marroquin Family
60. Thanks for all your excellent work! Dick & Pat Wolff
61. Thank You beyond Words!!! The conference meant so much on so many levels. This is our life – and these are times we have forever! We Love You Love the Verdiles
62. Thanks You from The Aguero Family
63. Thanks for all you do! Your Friends, Madison & Annette
64. No way to say strong enough words to express how much this conference means to SMA community kids, adults & parents.
Thank You Messages

65. Thank you for the best time of our lives!

66. Thank you for receiving us and let the hope sunshine.
   Elaine Paquette

67. Thank You! So much fun!

68. Thanks you for your newly diagnosed scholarship, we wouldn’t have come without it and we are so glad we came. We hope / plan to see everyone next year. The Peda Family

69. Thank you for all you do! Keep up the great work! Mirabile Family

70. Thank you for your time and effort, as a Respiratory Therapist to several of the kids here, I thank you for this conference.

71. Keep doing what you’re doing.

72. The people..... The people.... An immediate sense of inclusion & understanding. I will not soon forget those angel faces guiding their wheelchairs. So much work…. you are wonderful…. and I say Thank You. Rosalie Smith

73. Thank you for caring.

74. Thank you! This conference is awesome!

75. Thank you for making this conference happen and available to SMA families who couldn’t afford to come. Thank you for caring! Find a Cure Robin Harris Texas

76. Thanks so much for all your hard work. The Humbles

77. Thank you! The McGrits

78. Thank You!! The Myers!

79. Thank you Peg Bailey.

80. Amazing gift to all

81. Thank You, Burton

82. Thank you for all your hard work. Love, The Oaks

83. Cure SMA & Researchers, You Rock!

84. Thank you for all your endless hours of hard work. Gratefully, The Sonnenbergs

85. Thank You All!! The Martins

86. Thank you. Sydney

87. Keep up your great work. Andrew & Fran Sinish

88. Thank you for such an awesome conference once again!

89. Thank you so much! You rock! The Murphy’s

90. Thank you very much, we appreciate everything you do. Riipinen-Raisanen

91. Thank you for all you do Cure SMA. Family of Philip Struble

92. Thank you so very much for this opportunity to be with other SMA families. This is our 2nd conference and we are so excited to see old friends & make new ones. Our granddaughter has type II & is so very special to us. Thank You, Freddie & Cathy Crawford

93. Thank you so much for all you do! Chessa, Cheryl & Robert

94. Thank you for all you do! Especially for the SMA Community. The Sonnenbergs

95. Most sincerely thanks! “officer Morgan Steward” & Family Cure SMA

96. Thank You! Mary, Hannah Adriana & Jonathan (type II) Sweeney

97. Thanks for your help. Love, Mateo

98. Thank you for all you do! Candance & Erin

99. Thank you so much. Heather, Mike Hailey & Maura

100. Thank you, I had a great ay at the Cure SMA conference. Playing, goodies and the little friends I met; all this makes me in a good mood. Ofelie
Being in our Nation’s Capitol gave this year’s conference an extra opportunity to raise awareness for SMA. Many families attended an educational breakfast on how to speak with their local congressmen about Spinal Muscular Atrophy and then set-off on their meetings determined to inform those in congress about SMA. The goal of these efforts towards our policymakers is to significantly raise awareness of SMA, increase the level of federal support dedicated towards SMA research, and facilitate the development of therapies through public-private partnerships between Cure SMA, the federal government and the biopharmaceutical industries.
SMA Day on the Hill

Melissa Milinovich, SMA Adult

“Tomorrow, I am going to Capitol Hill to work with legislatures and advocate for those with SMA. Tomorrow, I will be nervous but articulate. Tomorrow, I will try to change a small piece of the world for those of us with SMA. Tomorrow, I will be SMA strong.” - This is what I wrote on my Facebook wall the night before my trip to Capitol Hill and I had no idea the amazing experience that I was about to have.

On June 11, 2014, I arrived at the Capitol building with several other families to attend a breakfast briefing to enlighten us about what would occur throughout the day. The Capitol is a very busy place with people hurrying from place to place; I was a bit overwhelmed, to be honest. Our Cure SMA legislative genius, Spencer Perlman, told us what to expect and what we should focus on during our short time with each of our assigned legislatures. The three talking points given to us were: 1) assistance in engaging with the FDA to discuss “clinical endpoints” for SMA clinical trials; 2) support for Chairman Fred Upton’s “21st Century Cures Initiative”; and 3) increased funding for the National Institutes of Health. Besides these three topics, though, Spencer told us the most important thing to do was share our personal stories – share what SMA was to us – share what SMA meant to us – share how SMA affected our lives. The personal story task should have been the easy part but it proved to be the most challenging!

I was scheduled to meet with a representative from both Ohio Senator offices as well as a representative from my Ohio district House of Representatives office. Before my Ohio day began, though, I was given the opportunity to meet with Paul Ryan in the Wisconsin meeting. Although I was not from his state, he understood the overall reason we were all there to speak and seemed to really listen to what we had to say. This meeting was exactly what I needed to spur my advocacy fire and prepare me for my own Ohio meetings. My Ohio meetings were quick but productive. Each person listened to what I had to say, asked appropriate questions, and had even taken the time to read up on SMA before our meeting; it was truly impressive. Although I made sure to hit the talking points we were given in each meeting, I tailored what I shared of my own personal story based on my audience to make sure I connected on a personal level. To my surprise, I even got a bit emotional in one meeting because I was speaking about how I wanted a treatment so that I could make sure that I lived to see my daughter grow up. So much of my life is spent being positive and trying to ignore the black cloud looming in the back of my mind – having to speak about the reality of what could occur if a treatment is not found was not easy to speak about but necessary to get the point across.

All in all, I feel strongly that each of us that took the time to speak and advocate for SMA made an impact. It was an experience that I will never forget and I will be sure to keep engaging my state representatives whenever I can on issues that directly affect our community.
U.S. Senate Committee Encourages NIH to Continue SMA-Related Research Initiatives

The Senate Appropriations Committee has released its draft fiscal year (FY) 2015 Labor, Health & Human Services, Education, and Related Agencies Appropriations bill, which includes report language urging the National Institutes of Health (NIH), the nation’s biomedical research agency, to continue its ongoing work related to SMA-related therapy development and newborn screening projects.

Cure SMA worked closely with its friends in the U.S. Senate, including Senator Tom Harkin (D-IA) and Senator Mark Kirk (R-IL), to compose this language and to urge the Appropriations Committee to include it in the funding bill.

The two institutes within the NIH that are leaders in SMA research are the National Institutes of Neurological Disorders and Stroke (NINDS) and the National Institute of Child Health and Human Development (NICHD). Cure SMA collaborates regularly with the NINDS and the NICHD on a variety of initiatives. The report language included in the draft Senate spending measure will ensure that this public-private partnership will continue to grow and thrive.

NINDS Language
Spinal Muscular Atrophy [SMA] Translational Research — The Committee commends NINDS for its continued support of SMA therapy development projects across a broad range of therapeutic approaches, including its innovative work on validating SMA bio-markers and clinical trial endpoints through the NeuroNEXT program. The Committee encourages NINDS to continue to utilize this network for SMA initiatives, including for testing potential therapies. The Committee also commends NINDS for working collaboratively with its partners to leverage resources and spur discoveries.

NICHD Language
Spinal Muscular Atrophy [SMA] Newborn Screening — The Committee applauds the NICHD for its support of a pilot newborn screening project for the identification and follow-up of infants with SMA. Newborn screening holds tremendous promise to augment the treatment of SMA by creating the opportunity for early administration of therapies currently undergoing late-stage clinical testing. Preclinical evidence demonstrates that administering SMA therapies pre-symptomatically may improve outcomes. The Committee encourages NICHD to continue to collaborate with stakeholders in government and in the private sector to maintain SMA newborn screening pilot testing.

RESEARCH FUNDING CONNECTION BETWEEN ALS AND SMA FROM CURE SMA

With all of the great awareness being raised for ALS in the news and on social media thanks to the #ALSiceBucketChallenge, it is important to note a current connection between ALS and SMA in current research funding.

Cure SMA is actively funding a drug program with a chance of cross-over between ALS and SMA later downstream in the muscles.

The grant award from Cure SMA to Cytokinetcs is to support preclinical research on muscle function in a mouse model of spinal muscular atrophy (SMA) to be conducted with the company’s fast skeletal muscle troponin activator, tirasemtiv.

Cytokinetics is evaluating tirasemtiv as a potential treatment for amyotrophic lateral sclerosis (ALS).

Tirasemtiv, the lead drug candidate from Cytokinetics’ skeletal muscle contractility program, selectively activates the fast skeletal muscle troponin complex by increasing its sensitivity to calcium, thereby increasing skeletal muscle force in response to neuronal input and delaying the onset and reducing the degree of muscle fatigue.

While ALS and SMA are different diseases, with different causes and ages of onset, the same tissues in the body are ultimately impacted. The advantage for developing a therapy in SMA comes from knowing the exact genetic cause, having a back-up gene target in the body, and having the same cause for all those.
U.S. House of Representatives Approves Newborn Screening Legislation: Includes Provisions Advocated Cure SMA

The U.S. House of Representatives has approved by voice vote the Newborn Screening Saves Lives Reauthorization Act of 2014 (H.R. 1281). This legislation, which reauthorizes the nation’s newborn screening programs for another five years, includes several legislative provisions advocated by Cure SMA and our partners that aim to improve and streamline the process of adding new disorders (such as SMA) to the federal recommended newborn screening panel. A table of these provisions and the issues they address can be found here.

**What is Newborn Screening and Why is it Important to the SMA Community?**

Newborn screening is a state-run public health program that tests each infant born in the United States for serious but treatable heritable disorders. The federal government offers funding and technical assistance to the states through the Secretary’s Advisory Committee on Heritable Disorders in Newborns and Children (SAC), which is comprised of a panel of national experts. The SAC is responsible for recommending to the Secretary of Health and Human Services (HHS) which disorders should be added to the federal recommended newborn screening panel that states are expected to follow. The Secretary must formally approve a SAC recommendation to add a condition to the federal newborn screening panel.

At present, 31 disorders are on the federal panel. SMA is not currently on the panel, largely because it does not meet the SAC’s requirements for being “treatable.” Cure SMA and our partners have been working closely with legislators in the House and Senate for over a year to rework the federal newborn screening program to better facilitate the addition of new disorders to the screening panel; Cure SMA intends to nominate SMA for addition to the panel once approval of an SMA therapy by the U.S. Food and Drug Administration (FDA) appears imminent.

Newborn screening for SMA will allow children affected by the disease to be identified shortly after birth and to initiate therapy pre-symptomatically. This is critically important to ensuring that future infants born with SMA are able to receive treatment immediately, which evidence indicates will improve outcomes.

**Next Steps for The Newborn Screening Saves Lives Reauthorization Act of 2014**

The next step for H.R. 1281 is to secure Senate passage, which is very likely to occur within the next month. After Senate approval, the legislation must be signed into law by the President. The Obama Administration has signaled that they will sign the bill once it is approved by Congress.

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**Compassion**

Thanks to the Cure SMA community, no person is ever alone in facing this disease. We offer unconditional support to people affected by SMA and communicate openly and honestly, giving them clear and accurate information.
The House Energy & Commerce Committee, which has jurisdiction over the National Institutes of Health (NIH) and the Food and Drug Administration (FDA), held the first of several Roundtable discussions on May 6th as part of its new “21st Century Cures Initiative”, an effort to comprehensively review the nation’s therapy development apparatus from soup-to-nuts. This first Roundtable was led by Committee Chairman Fred Upton (R-MI) and included the involvement of NIH Director Francis Collins, several senior leaders at the FDA, and experts in biomedical research and drug development from academia, non-profit advocacy organizations, and the biotechnology industry.

The wide-ranging 2-hour discussion covered a variety of topics, but some of the major items discussed included the following:

• There was unanimous agreement that the NIH is in danger of “breaking” if it is not provided with more funding. The nation’s biomedical research agency has lost over 25% of its purchasing power over the last decade as a result of congressional spending cuts, which has slowed research.

• Nongovernmental representatives and Dr. Collins alike informed Members of Congress that the scientific enterprise would be vastly improved if certain regulatory burdens could be eased, especially those related to public-private partnerships.

• FDA staff indicated that the clinical trials system and the drug manufacturing system are archaic and need to be dramatically reformed. One potential solution recommended by FDA is to create more, and more robust, clinical trials networks. FDA staff also indicated that Congress should work to support and grow translational research, including more work on identifying biomarkers.

• Several nongovernmental representatives discussed the need for greater and better facilitated data sharing between all stakeholders, such as building data into electronic health records and expanding the use and utilization of patient registries.

• It was agreed by all participants that the 21st Century Cures Initiative should include the involvement of public and private insurers since the development of new therapies is useless if patients have no way to access them.

• The industry representatives recommended that the Initiative address tax policy as it relates to research and development since biopharma companies are the “third leg” of the three-legged stool that creates therapies in this country, along with government and patient advocacy groups.

At the end of the Roundtable, Chairman Upton stated that several additional conversations/Roundtables will take place over the coming months. The Cures Initiative is expected to culminate in one or more pieces of major legislation that will attempt to alter the manner in which NIH, FDA, and private stakeholders identify and develop new therapies.

Respect

There is no “right way” to live with a disease like spinal muscular atrophy. Every person’s experience is different, and it’s every family’s right to decide what SMA means for them.
Call Congress and Urge Passage of The ABLE Act

Congress appears to be on the verge of passing The Achieving a Better Life Experience (ABLE) Act of 2013, a bipartisan bill that would establish tax-exempt accounts to assist individuals with disabilities and their families in saving private funds for the purpose of paying for a variety of qualified disability-related expenses. The ABLE accounts are modeled after the tax-exempt 529 higher-education tuition accounts that are popular with many families across the country.

Like the 529 tuition accounts, an ABLE program would be established and maintained by a state government and ABLE accounts would have the same maximum annual contribution limits. The ABLE account funds could be used for qualified expenses which are made for the benefit of an individual with a disability who is a designated beneficiary of the account. Qualified expenses include:

- **Education**: Tuition for preschool thru higher education and expenses for books, supplies, educational materials, tutors, and special education services.
- **Housing**: Rent, purchase of a primary residence, mortgage payments, property taxes, and utility charges.
- **Transportation**: Mass transit, the purchase or modification of vehicles, and moving expenses.
- **Employment Support**: Job-related training, assistive technology, and personal assistance supports.
- **Health, Prevention, and Wellness**: Premiums for health insurance; mental health, medical, vision, and dental expenses; habilitation and rehabilitation services; durable medical equipment; therapy; respite care; long-term services and supports; nutritional management; communication services and devices; adaptive equipment; assistive technology; and personal assistance.
- **Miscellaneous Expenses**: Financial management and administrative services; legal fees; expenses for oversight; monitoring; home improvements, modifications, maintenance, and repairs at primary residence; and funeral or burial expenses.
- **Assistive Technology and Personal Support Services**.

**Medicaid Payback Provision:**

In the event the qualified beneficiary dies (or ceases to be an individual with a disability) with remaining assets in an ABLE account:

- The assets in the ABLE Account are first distributed to any State Medicaid plan that provided medical assistance to the designated beneficiary.
- The amount of any such Medicaid payback is calculated based on amounts paid by Medicaid after the creation of the ABLE Account.

The legislation has very strong bipartisan support in both the House and the Senate and could be taken up at any time. Please contact your Members of Congress to urge them to support this incredibly important bill.
Cure SMA Announces Basic Research Funding for Dr. Lyndsay Murray

Recently, we announced that Dr. Lyndsay Murray at The University of Edinburgh will receive a a Cure SMA basic research grant of $80,000 over two years, to explore the reasons causing motor neuron cell death in SMA.

Motor neurons are nerve cells in the brain stem and spinal cord that control muscle movement. We know that, in SMA, these cells cannot properly function and eventually die. By learning more about what happens to these cells and how they die, we may be able to develop more effective ways of making drugs to treat SMA.

MEET DR. MURRAY

Who are you?
I am a newly appointed researcher and lecturer at the University of Edinburgh.

How did you first become involved with SMA research?
I started working on SMA research during my PhD in the laboratory of Dr. Gillingwater and have maintained this interest during my postdoctoral studies in the laboratory of Dr. Kothary. SMA research will now be a key focus for me as I start my own lab at the University of Edinburgh.

What is your current role in SMA research?
I currently work on the basic science aspects of SMA research. My main focus is looking at what makes motor neurons vulnerable in SMA and developing methods to protect them from death and dysfunction.

Tell us about your project, “Investigating The P53 Signaling Pathway in Pathogenesis of Mouse Models of SMA.”

Objective: In this study we aim to ask two main questions. Firstly, how early does motor neuron cell death occur? Secondly, we want to investigate whether delaying cell death while administering other therapies, can increase the beneficial effects.

Research Strategy: We will use mouse models of SMA to investigate when motor neurons actually start to die in SMA. We will also use transgenic mice to restore SMN protein at symptomatic phases while simultaneously inhibiting cell death.

Significance: This work has important implications both for understanding the basic process of disease in SMA and for developing a new therapeutic approach, which could help patients who are treated after their symptoms begin. It will also begin to assess combination therapeutic approaches for SMA.

OUR FUNDING STRATEGY
Cure SMA is leading the way to a world without SMA by advancing a comprehensive research program. One of the key components of this strategy is basic research. We’ve awarded 79 basic grants for a total of over $9.5 million in the past 10 years.

Learn more about our basic research program, and about the other components of our research strategy: drug discovery, clinical trials, and clinical care.
Spinal Muscular Atrophy Mice Model Results Published in the Journal Science

The journal Science published results of a preclinical study demonstrating that treatment with orally available RNA splicing modifiers of the SMN2 gene starting early after birth prevents deficits in a mouse model of Spinal Muscular Atrophy (SMA).

Scientists from Roche Pharma Research and Early Development (pRED), PTC Therapeutics, Inc., the SMA Foundation, the University of Southern California and Harvard University collaborated to demonstrate that continuous treatment of SMA mice with these compounds increased life span, normalized body weight and prevented both disease-related motor dysfunction and neuromuscular deficits.

“Although still preclinical, these results demonstrate how SMN2 splicing modifiers could correct the molecular deficit that causes SMA,” said Luca Santarelli, Head of Neuroscience, Ophthalmology and Rare Diseases at Roche. “This study represents an important step towards developing a potential therapeutic option for this devastating and currently untreatable condition. Early clinical trials are currently underway to determine the safety and tolerability of this approach.”

“The investigational compounds used in this study represent the first orally available SMN2 splicing modifiers for SMA,” commented Stuart W. Peltz, CEO of PTC Therapeutics, Inc. “Using the experience and expertise in RNA biology we have gained at PTC over the last 16 years, we used our alternative splicing technology to identify and subsequently optimize investigational compounds that target the SMN2 splicing to produce the SMN protein. Our unique partnership with Roche and the SMA Foundation has allowed this project to rapidly move into clinical development.”

The study used chemical screening and optimization to identify orally available small molecules that selectively alter the splicing of the SMN2 pre-mRNA to produce stable full-length SMN protein. The SMN2 splicing modifiers described in the Science article penetrated into all mouse tissues tested, including brain, spinal cord and muscle, and thus improved SMN2 RNA splicing to increase SMN protein production in these disease-relevant tissues. As a result of the SMN protein increase, the compounds prevented the progression of SMA in a severe mouse model. These compounds also corrected SMN2 RNA splicing and increased SMN protein levels in cell cultures obtained from SMA patients, including stem cell-derived motor neurons. A Phase I clinical program to assess safety and tolerability with investigational compounds was initiated in early 2014.

“The findings of this preclinical study contribute significantly to our understanding of SMA and provide further evidence suggesting that our strategy to upregulate SMN with small molecules could be effective,” said Loren Eng, President of the SMA Foundation. “We are proud to have seeded this important work – we believe it could have a meaningful impact on the lives of patients who suffer from SMA.”

The drug is currently being tested in Phase I clinical trials, and PTC also provided an update on their trial results today.

In January 2014, a Phase 1a single ascending dose, placebo-controlled clinical trial in healthy volunteers was initiated. The primary objectives of this trial were to explore safety and pharmacokinetics of the drug candidate, RG7800. This trial has now completed and a multiple dose clinical trial in SMA patients is currently in preparation. Preliminary findings in the Phase 1a study indicate that RG7800 was well-tolerated at all dose levels studied. There were no deaths, serious adverse events (SAEs) or withdrawals due to adverse events (AEs) and no dose-related trends were identified. Additionally, RG7800 demonstrated a dose-dependent effect on SMN2 splicing, as shown by a change in the ratio of full-length SMN2 mRNA to SMN2 mRNA without exon 7 (SMND7), which may be interpreted as proof of mechanism in terms of the expected pharmacodynamic effect.
Tirasemtiv Demonstrates Improvements in Muscle Force, Grip Strength and Resistance to Fatigue in Preclinical Mouse Models of Spinal Muscular Atrophy

Cytokinetics announced that data from preclinical research relating to tirasemtiv in mouse models of spinal muscular atrophy (SMA) were presented at the 2014 Annual Spinal Muscular Atrophy Conference in National Harbor, Maryland. In these models, tirasemtiv increased muscle force and improved grip strength, grid hang time, and resistance to fatigue. These studies were supported in part by a grant from Cure SMA.

In both an oral presentation entitled “Small Molecule Tirasemtiv Improves Muscle Function in Two Mouse Models of SMA” and a poster entitled “Tirasemtiv Increases Skeletal Muscle Performance in SMA Mice” Cytokinetics’ scientists shared data from research performed in collaboration with Christine DiDonato, Ph.D., Associate Professor at the Feinberg School of Medicine at Northwestern University and colleagues at the Manne Children’s Research Institute, affiliated with Ann & Robert H. Lurie Children’s Hospital of Chicago and W. David Arnold, MD, Assistant Professor at The Ohio State University.

The objective of the research was to examine the effect of tirasemtiv on measures of muscle function in two mouse models of SMA that were generated in the DiDonato laboratory and exhibit mild to moderate neuromuscular dysfunction. In these models, isometric ankle plantarflexor force was measured following sciatic nerve stimulation and muscle fatigability was assessed by repeated sciatic nerve stimulation. Grip strength was assessed with a pull bar assembly connected to a force measurement gauge and grid hang time was measured by placing the mice on a grid and inverting it. The first model evaluated mild-severity “adult-onset” SMA mice and the second model evaluated “intermediate-severity” SMA mice.

In the “adult-onset” SMA mice, the authors noted that, in response to subtetanic nerve stimulation, mice treated with tirasemtiv produced increases in submaximal isometric force compared to vehicle-treated mice. In addition, treatment with tirasemtiv significantly improved resistance to muscle fatigue. In particular, grid hang time (mean ± sem) increased (138 ± 18 vs. 192 ± 34 seconds for vehicle and tirasemtiv treated mice respectively, p = 0.048) to levels similar to that of wild-type mice (197 ± 23 seconds). In the “intermediate-severity” SMA mice in which the authors noted that muscle weakness was more pronounced than in the “adult-onset” SMA mice, treatment with tirasemtiv also produced more force than vehicle-treated controls in response to subtetanic nerve stimulation. In addition, treatment with tirasemtiv improved forelimb grip strength in these mice (43 ± 4 vs. 52 ± 4 grams for vehicle and tirasemtiv treated mice respectively, p = 0.01) although it was not feasible to normalize grip strength to that of wild-type mice (95 ± 4 grams) in this more severely affected model. The authors concluded that in these animal models of SMA, tirasemtiv increased submaximal muscle force in situ, improved fatigue resistance in situ, improved grip strength in vivo in the “intermediate-severity” mouse model and improved grid hang time in vivo in the “adult-onset” mouse model.

“We believe these data lend support to the translation of tirasemtiv as a potential treatment for neuromuscular diseases such as SMA and we continue to evaluate opportunities to expand the development for this novel mechanism drug candidate. We would like to thank Cure SMA for their support of our research.”

Fady I. Malik, M.D., Ph.D., Cytokinetics’ Senior Vice President, Research and Early Development

“We are pleased to share results from our research performed with tirasemtiv in preclinical models of Spinal Muscular Atrophy,” stated Fady I. Malik, M.D., Ph.D., Cytokinetics’ Senior Vice President, Research and Early Development. “We believe these data lend support to the translation of tirasemtiv as a potential treatment for neuromuscular diseases such as SMA and we continue to evaluate opportunities to expand the development for this novel mechanism drug candidate. We would like to thank Cure SMA for their support of our research.”
About Tirasemtiv:

Tirasemtiv, a novel skeletal muscle activator, is the lead drug candidate from Cytokinetics’ skeletal muscle contractility program. Tirasemtiv selectively activates the fast skeletal muscle troponin complex by increasing its sensitivity to calcium and, in preclinical studies, demonstrated increases in skeletal muscle force in response to neuronal input and delays in the onset and reductions in the degree of muscle fatigue. Tirasemtiv was the subject of BENEFIT-ALS (Blinded Evaluation of Neuromuscular Effects and Functional Improvement with Tirasemtiv in ALS), a recently completed Phase IIb clinical trial. BENEFIT-ALS was a multi-national, double-blind, randomized, placebo-controlled, clinical trial designed to evaluate the safety, tolerability and efficacy of tira semtiv in patients with amyotrophic lateral sclerosis (ALS). BENEFIT-ALS did not achieve its primary efficacy endpoint, the mean change from baseline in the ALS Functional Rating Scale in its revised form (ALSFRS-R). Treatment with tirasemtiv resulted in a statistically significant and potentially clinically meaningful reduction in the decline of Slow Vital Capacity (SVC), a pre-specified secondary efficacy endpoint and a measure of the strength of the skeletal muscles responsible for breathing that has been shown to be an important predictor of disease progression and survival in prior trials of patients with ALS. The analyses of other pre-specified secondary efficacy endpoints produced mixed results. Results from BENEFIT-ALS were presented at the 66th Annual Meeting of the American Academy of Neurology on April 29, 2014 and at the Joint Congress of European Neurology on June 1, 2014. Cytokinetics expects to continue to analyze the data from BENEFIT-ALS to inform the potential further development of tirasemtiv in patients living with ALS.

Cure SMA Announces New Research Funding

Cure SMA is proud to announce that we will be committing $1.8 million to new research funding over the next 12 months.

Fifteen years ago, we had just one potential drug in the beginning stages of preclinical discovery. Today we have over a dozen, including five now in clinical trials. Though there’s great promise in the research landscape, there’s also a pressing need for continued and growing investment.

In response to this need, we’re increasing our funding for all areas of research—basic research to understand SMA, drug discovery to make practical therapies, and clinical care research to improve the quality of life for those with SMA. With the size of our community and the strength of our connections, we’re able to direct research at unparalleled scale and efficiency.

Check out our news page and our latest advances page to stay up to date on the research that we are funding. We anticipate announcing our next grants in late 2014.

All our research is funded by the generosity and dedication of our supporters. Find out how you can give in support of SMA research.
RFP Closes for Spinal Muscular Atrophy Basic Research Funding

On September 5, Cure SMA announced the close of our request for proposals (RFP) for basic research.

An RFP is an invitation for scientists to submit their best ideas for SMA research. They explain what they want to study, how they plan to study it, and why they think it will be effective for SMA.

The closing of the RFP means we can move on to the next step of the process, and make sure your donations reach scientists who can make a difference for our community.

The next step is a detailed review by our Scientific Advisory Board. They’ll review each of the 29 proposals we received, looking to see which projects are the most intriguing, which have a well constructed study plan, and which match up with the most pressing unanswered questions about SMA.

Most importantly, they are looking for the projects that show the most promise in getting us closer to our goal of a treatment and cure for SMA. Once this evaluation is complete, we determine which projects will receive a Cure SMA research grant.

At the end of this process, in early 2015, we’ll announce up to $700,000 in new basic research funding.

Why We Fund Basic Research

Basic research is the critical first step in the research process, and has led to many discoveries. For example, basic research is how we identified the SMN1 mutation that causes SMA. Basic research is how we identified SMN2, the SMA “back-up gene.”

Right now, scientists are working on gene transfer techniques that may treat the SMN1 mutation. They’re working on small molecules and other methods that may help the SMN2 gene function better. Many of these projects are also receiving Cure SMA funding, and all of them started with basic research discoveries.

Without basic research, the SMA drug pipeline would not continue to grow and diversify. We need both a breadth and a depth of options in our quest for an effective SMA therapy. Basic research is our investment in future drug development for SMA.
NATIONAL INSTITUTES OF HEALTH RELEASES THREE GRANT OPPORTUNITIES RELATED TO NEWBORN SCREENING FOR DISEASE LIKE SMA

The National Institutes of Health (NIH), which is the federal government’s biomedical research agency, has released three related grant opportunities to facilitate the development of beneficial treatments for infants that have been identified through newborn screening as having conditions such as SMA. Demonstrating the benefits of a treatment is a primary criterion for inclusion of a condition on the federal newborn screening panel; these funding opportunities will support efforts by scientific investigators to utilize newborn screening to facilitate the development of SMA therapies.

Cure SMA has been working with federal policymakers for several years to facilitate the inclusion of SMA on the federal newborn screening panel. Recently, the House of Representatives passed the Newborn Screening Saves Lives Reauthorization Act of 2014, which includes several provisions advocated by Cure SMA. This legislation also reauthorizes the NIH to support newborn screening related research such as the funding opportunities released this week.

Three new NIH funding opportunities will support research on newborn screening. Each funding opportunity is open for three years and the NIH will accept applications on a rolling basis with reviews occurring three times annually.

The funding of these grant opportunities responds to several years of report language, included in the annual congressional appropriations bills that fund the NIH, urging greater attention to SMA newborn screening activities. Cure SMA worked closely with supportive Members of Congress to secure this important language that has led to the recent funding opportunities announced by the NIH.

Cure SMA Featured in Assay and Drug Development Technologies

Cure SMA was featured in the July/August issue of Assay and Drug Development Technologies, a scientific journal that focuses on the development of new drugs. In honor of SMA Awareness Month, SMA was also featured on the front cover.

Assay reaches scientists in 170 countries worldwide, making this a unique opportunity for us to influence SMA research. Especially during August, we work to spread awareness of SMA among our friends, family, and co-workers. In the same way, we’re also working to spread awareness of SMA throughout the scientific community.

The issue featured a how-to article on preclinical drug development for SMA, including how to discover, validate, and optimize drugs that enhance survival motor neuron (SMN) protein levels. Individuals with SMA do not produce this protein at high enough levels.

The lead author for this article is Jill Jarecki, PhD, Cure SMA’s research director. The article’s other authors are an interdisciplinary group from academia, industry—including Pfizer and PTC Therapeutics—and SMA advocacy groups.

The issue also featured an interview with Jill on Cure SMA’s research funding strategy, and the progress that we’ve made in finding a treatment and cure for SMA.

Our thanks to the rest of the paper’s authors—Drs. Jonathan Cherry, Dione Kobayashi, Maureen Lynes, Nikolai Naryshkin, F.D. Tiziano, Phillip Zaworski, and Lee Rubin—and to the editor-in-chief of Assay, Dr. James Inglese of the National Center for Advancing Translational Sciences (NCATS) at NIH. Dr. Inglese is also a member of the Cure SMA Translational Advisory Council.
Isis Begins Phase III Clinical Trial

On August 1, Isis Pharmaceuticals, Inc. announced the initiation of a pivotal Phase III study evaluating ISIS-SMNRx in infants with spinal muscular atrophy (SMA). The Phase III study, ENDEAR, is the first of several planned studies in a broad and comprehensive late-stage clinical development program for ISIS-SMNRx. Isis plans to initiate a second pivotal study in children with SMA later this year.

“The successful advancement of ISIS-SMNRx from a preclinical drug candidate to late-stage studies within just a few years reflects the effectiveness of our strategic alliance with Biogen Idec and the benefit of working closely together with combined expertise, and the support from the SMA community. As we continue development of ISIS-SMNRx and initiate the two pivotal studies in infants and children with SMA, we are in the planning stages for clinical studies in additional patient populations,” said B. Lynne Parshall, chief operating officer at Isis. “The clinical and preclinical data we have generated to date, including data in multiple open-label clinical studies, across multiple measures with ISIS-SMNRx, support the initiation of these studies, which fully assess the safety and efficacy benefits of this experimental treatment.”

“Cure SMA is pleased that Isis is advancing to the next phase of clinical trials for ISIS-SMNRx. Controlled trials are the gold standard in proving the safety and efficacy of any drug,” said Kenneth Hobby, president of Cure SMA. “SMA is a devastating disease with no current therapeutic options. Cure SMA applauds Isis for progressing its development program in an expedient manner, and looks forward to additional trials in patients with SMA beginning later in 2014.”

This is the first ever Phase III trial for a new drug developed to treat the underlying cause of SMA in the US, with original funding from Cure SMA. ENDEAR, a Phase III study of ISIS-SMNRx, is a randomized, double-blind, sham-procedure controlled thirteen month study in approximately 110 infants diagnosed with SMA. The study will evaluate the efficacy and safety of a 12 mg dose of ISIS-SMNRx with a primary endpoint of survival or permanent ventilation. Additional efficacy endpoints are also included in the study.

For further study information:
- Please visit www.clinicaltrials.gov.
- See the full announcement from Isis.
- Read frequently asked questions and answers about this trial.
- Visit our research publications page, and download the Fall 2013 issue of Compass.
Isis Presents Update on Spinal Muscular Atrophy Drug Trials at the American Academy of Neurology Meeting

Isis Pharmaceuticals, Inc. provided an update on both of its ongoing open-label Phase II clinical studies of ISIS-SMNRx in infants and children with spinal muscular atrophy (SMA) at the 66th American Academy of Neurology (AAN) meeting in Philadelphia, PA. Isis reports they are on track to initiate Phase III study in infants with SMA mid-year and on track to initiate Phase III study in children with SMA in the second half of the year.

Results from Phase II study in infants with SMA

In the study in infants with SMA, a total of 15 infants have been dosed as of April 7, four infants in the 6 mg cohort and 11 infants in the 12 mg cohort. The 12 mg cohort is continuing to enroll patients.

In the 12 mg cohort:

- Seven infants have received all three induction doses and been evaluated after their last induction dose. These patients constitute the per protocol efficacy population (PPEP).
- Of these seven, five are alive without the need for permanent ventilation. The two infants who have had an event (one death and one permanent ventilation) each experienced the event in connection with pneumonia.
- The median age of the infants in the PPEP in the 12 mg cohort is 9.6 months (calculated using age at event or on April 7 for patients who have not experienced an event).
- Three of the infants not included in the PPEP remain on study and had not yet reached their third induction dose on April 7. One infant died prior to receiving a third induction dose.

In the 6 mg cohort:

- The PPEP in this cohort is comprised of all four infants dosed.
- Two infants are alive without the need for permanent ventilation, one is currently on long-term ventilation and one infant, unfortunately, died due to an accident.
- The median age of the infants in the 6 mg cohort is 14 months (calculated using age at event or on April 7 for patients who did not experience an event).

Results from Phase II study in children with SMA

In the study in children with SMA, time and dose-dependent increases in muscle function scores, as measured by the Hammersmith Functional Motor Scale-Expanded (HFMSE), were observed in children treated with multiple-doses of ISIS-SMNRx. Children in the 3 mg, 6 mg and 9 mg cohorts achieved mean increases of 1.5, 2.3 and 3.7 points, respectively nine months following the first dose. Encouraging preliminary results were also observed in two additional functional tests: the six-minute walk test (6MWT), and the upper limb module (ULM) test. In the 6MWT, performed with nine ambulatory children, a mean increase of 22.7 meters was observed at 9 months. In the ULM test, which utilizes a 9 item scale (max score of 18), performed with 10 non-ambulatory children, a mean increase of 2.3 was observed at 9 months. In all children treated with ISIS-SMNRx to date, the drug has been well tolerated at doses as high as 12 mg.

In addition, analysis of cerebral spinal fluid (CSF) samples from children in this study demonstrated dose-dependent increases in SMN protein levels over time in patients treated with ISIS-SMNRx with the maximum effect observed in the 9 mg cohort in which the mean SMN protein level more than doubled by Day 86 from baseline (n=9). Children in the 12 mg cohort have not yet been evaluated. These results are consistent with the increases in SMN protein levels.
AveXis Announces Dosing of First Patient for Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type I

AveXis, Inc., a synthetic biology platform company, today announced that the first patient in the Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type I has now been enrolled and dosed by Nationwide Children's Hospital. This trial utilizes chariSMA, the gene therapy product developed by Dr. Brian Kaspar, of Nationwide Children's Hospital, and licensed to AveXis for development and commercialization. chariSMA utilizes AAV9 to deliver the functional SMN gene to patients. In preclinical studies, AAV9 has been shown to cross the blood-brain-barrier, delivering SMN to motor neurons and significantly improve survival and motor function in an animal model of SMA.

"Over the past few months so many individuals, organizations and foundations have worked tirelessly to bring chariSMA from the bench to the bedside. We are thrilled to be at this inflection point and hopeful that the nonclinical results we have seen will be manifested in the clinic," said John Carbona, CEO of AveXis. The clinical trial is led by Dr. Jerry Mendell, at The Research Institute at Nationwide Children's Hospital, having received IND approval and Fast Track designation in September 2013.

For further details on the trial, enrollment criteria, eligibility and contact information please see the complete posting, which can be found at http://clinicaltrials.gov/ct2/show/NCT02122952.

What has Cure SMA's role been in the project?

- In 2010, Cure SMA awarded $100,000 to Dr. Brian Kaspar at Nationwide Children's Hospital for systemic gene therapy development
- In 2012, Cure SMA awarded Dr. Kaspar $750,000 to advance a CNS-delivered gene therapy to treat older and bigger SMA patients
- In 2013, NINDS awarded $3.8 Million to Dr. Kaspar in collaboration with Cure SMA to advance the CNS-delivered gene therapy to IND

Cure SMA would like to thank all those who have contributed funding to Cure SMA for this particular program, including special gifts from The Michael and Chandra Rudd Foundation, The Miller McNeil Woodruff Foundation, and The Jacob Isaac Rappoport Foundation.

Determination

Our work is not done until we have a treatment and cure, and we'll remain strong in our fight no matter what challenges come our way.
Trophos Presents Pivotal Phase II/III Study Results on Olesoxime in Spinal Muscular Atrophy at The American Academy of Neurology Meeting

The 66th American Academy of Neurology (AAN) Meeting was held in Philadelphia. Over 10,000 neurologists were in attendance. Trophos presented late breaking data on their pivotal phase II/III trial on olesoxime in Spinal Muscular Atrophy there.

Trophos is a clinical stage pharmaceutical company developing innovative therapeutics for indications with under-served needs in neurology and cardiology. The company has a novel and proprietary cholesterol-oxime based chemistry platform generating a pipeline of drug candidates. The lead product, olesoxime (TRO19622), is being developed for SMA and multiple sclerosis.

The company indicated that the data shows that patients treated with olesoxime were able to maintain motor function over the two-year period of the study and that typical health complications associated with SMA occurred less frequently than in patients treated with a placebo, leading to better well being.

The new data presented at AAN is from the recently completed international, double-blind, placebo-controlled study involving 165 Type II and non-ambulatory Type III SMA patients, ranging in age from 3 to 25 years old.

The results show that olesoxime treatment preserved motor function for two years using the Motor Function Measure scale (MFM) D1+D2 as the primary endpoint. The MFM is a standardized neuromuscular disease-specific functional scale. In contrast, patients in the placebo arm of the study experienced a loss of motor function starting from a mean score of 39 percent at baseline to 37.1 per cent after two years. The mean loss of 1.9 points in motor function over the two-year study period confirms that the natural disease progression results in approximately 1 per cent per year loss of motor function in SMA patients.

“Analysis of the data from the pivotal trial of olesoxime provides further evidence of its neuroprotective effect, with a statistically significant impact on maintenance of motor function for the two years of the trial compared with placebo,” said Dr. Enrico Bertini, the principle investigator of the study. “Results from secondary endpoints were also promising. The olesoxime-treated group experienced fewer adverse events caused by the disease itself. SMA is a devastating condition which, even in its less severe forms, leads to progressive muscle wasting and the loss of mobility and motor function. Olesoxime has the potential to be the first ever treatment approved especially for SMA patients.”
PTC Therapeutics Announces New Phase 1a/2b Clinical Trial

PTC Therapeutics, Inc. announced today that its joint development program in Spinal Muscular Atrophy (SMA) with Roche and the SMA Foundation (SMAF) has started a Phase 1b/2a study in adult and pediatric patients. The placebo-controlled, randomized, multiple-dose study will enroll approximately 48 patients with SMA.

Because of a mutation in the survival motor neuron gene 1 (SMN1), individuals with SMA don’t produce survival motor neuron protein (SMN protein) at high enough levels. This program is one of several underway that target SMN2, the SMA “back-up gene.” This drug is the first orally available SMN2 splicing modifier for SMA to be tested in patients.

Drug Development Milestones

2014 has been a landmark year for SMA drug development. We now have 17 ongoing drug programs, and six have reached clinical trials—both the highest numbers ever. We also have 12 companies working on SMA drugs—another record number—including many of the largest pharmaceutical companies in the world. PTC and Roche are two of these major partners.

We’re excited to see our SMA community continue to expand. The more partnerships we can build, the more quickly we can move toward a treatment and cure for SMA.

Isis Announces Second Phase III Clinical Trial

Isis recently announced the start of CHERISH, a multi-center Phase III clinical study evaluating the efficacy and safety of the investigational compound, ISIS-SMNRx, in non-ambulatory children with Spinal Muscular Atrophy (SMA). The study is a double-blind, randomized, sham-procedure controlled study in approximately 117 children with SMA at study centers in the US, Canada, Europe and Asia Pacific. This study was initiated in November.

The study will enroll children who are 2-12 years of age and who have been diagnosed with Spinal Muscular Atrophy, and who pass additional screening evaluations conducted at the study site to confirm eligibility. The main inclusion/exclusion criteria for this trial are onset of signs and symptoms of SMA >6 months of age; 2-12 years of age; can sit independently but has never had the ability to walk independently; without severe scoliosis.

This is the second Phase III study for ISIS-SMNRx. In September, Isis announced the launch of ENDEAR, a Phase III clinical trial to study ISIS-SMNRx in infants with SMA type I.

More information on both ENDEAR and CHERISH can be found at www.clinicaltrials.gov or www.smastudy.com.

In addition, you can download a list of frequently asked questions, or see our recent news articles about ISIS-SMNRx and other clinical trials.
Cure SMA is pleased to announce that we are accepting applications for 2014 funding of clinical care research projects, under a competitive review by our Medical Advisory Council (MAC). The goal of this program is to improve care and living with SMA.

**Program Overview:** The deadline for submission was August 29, 2014. Applications were accepted for pilot studies with funding at a maximum level of $50,000, including 8% indirect costs. We anticipate granting awards for four SMA pilot projects in this funding round. This program awards funds for research into the clinical, psychological, or social management of SMA.

**Scientific Priorities:** Cure SMA would like to encourage applications on novel research, the results of which will build an evidence base and demonstrate measurable, positive effects on the clinical management and lives of patients with SMA. Cure SMA is soliciting and funding pilot studies and studies with pilot data that may lead to full-scale multi-center studies. Competitive projects will include a research design and methods, strong scientific merit with high SMA relevance.

**Cure SMA Funding Strategy:** Cure SMA invests in four areas of research: Basic Research, Drug Discovery, Clinical Research, and Care Research. Cure SMA has invested over $57 Million in SMA research since our inception in 1984, with $35 Million in funding in the past decade alone.

The aim of the Clinical Care Research Program is to improve the care and the quality of life for SMA patients. The funding for this program will build on the consensus statement for the standard of care for SMA with data driven results on specific areas of SMA care. The results of funded projects will build an evidence base and demonstrate measurable, positive effects on the clinical management and lives of patients with SMA. These results will be used to provide:

- Educational programs for professional medical providers, such as Cure SMA CME Day
- New family-family focused care publications, such as Cure SMA Care Series Booklets
- Peer reviewed journal publications to influence insurance coverage

Cure SMA is proud to announce that we will be committing $1.8 million to new research funding over the coming year.

Fifteen years ago, we had just one potential drug in the beginning stages of preclinical discovery. Today we have over a dozen, including five now in clinical trials. Though there’s great promise in the research landscape, there’s also a pressing need for continued and growing investment.

In response to this need, we’re increasing our funding for all areas of research—basic research to understand SMA, drug discovery to make practical therapies, and clinical care research to improve the quality of life for those with SMA. With the size of our community and the strength of our connections, we’re able to direct research at unparalleled scale and efficiency.

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**Save the date!**

Thursday, June 18th - Sunday, June 21st, 2015

2015 Annual SMA Conference  Kansas City, MO
Congratulations

Congratulations to Danyelle, Terence Sun and big sister, Ruby, on the birth of their new baby boy, Landon!

Congratulations to the Lehmann Family of Georgia on the birth of their new baby girl, Eleanor Sarah, born on July 18th!

Congratulations to Amanda and Eric Jurgovan and big sister, McKenna, on the birth of their new addition, Emery Fae, born on April 17th!

Congratulations to the Chu Family on their new addition, Jaxon William, born on July 3rd, 2014!

Congratulations to Eric Arnold, 28 years old, who just recently passed the bar exam! We wish you all the best in your future and the opportunities this achievement may bring to you!

Cure SMA,

Here are some photos of an accessible playhouse that was custom built by volunteers from BCBSNC as an auction item. The playhouse is 4’x5’ with a height of approximately 6’. It features an entryway and exit on both sides, with optional ramp for accessibility, hinged doors and windows. The winner of the playhouse went to an SMA family!

Mimi Chan of North Carolina
Dear Cure SMA,

We have included a couple of pictures from our trip to New York City to be on Fox and Friends morning show. Our church was doing a video series on “overcoming obstacles”, and they asked if they could do a video of our children who daily needed each other to climb the mountain of “SMA”. During the video, our son unexpectedly poured out his heart for his sister with SMA. The video went viral, and much to our surprise, we received a phone call from Fox News in NYC asking us to come out and be interviewed about the video. While the video is not solely about SMA, we are hoping that the unexpected attention has raised awareness about the disease and the need for a cure. Thanks to Cure SMA for being a wonderful source where we can send people for more information.

Walt and Tracy Cochran of Kansas

To check out the video of Lindsay Cochran, SMA Type II and her brother Trenton, visit http://www.huffingtonpost.com/2014/01/21/the-cochran-family-brothers-love-for-sister_n_4638713.html?utm_hp_ref=tw

Check out this great video of Cara Freedman whose brother Jack has SMA! Cara wrote this essay for her English class at school about her brother Jack. Thank you Cara for sharing your story and for spreading SMA Awareness! https://www.youtube.com/watch?v=wAl9louKJSY&feature=youtu.be

What is SMA? Debuts on Access TV and YouTube

We are proud to announce the production of “What is SMA?” To view this access TV show and share it with your friends visit www.youtube.com/KMVT, search for “AAUW Connections” and scroll down the list of shows.

Linda Shively, mother of Jessica Fernandes, SMA Type I, (2002-2005), hosts and welcomes Pat and Dick Wolff, grandparents of Madi Wolff, SMA Type II, age 11 and Shriya Shah and her son Sohum, SMA Type I-II, age 11 explain what it is like to live with Spinal Muscular Atrophy from a personal point of view. They discuss what is being done for awareness and fundraising locally and nationally. Dick aptly explains the genetics of SMA and the research that Cure SMA is funding.
Cure SMA Receives 200 Bath Kneeler Pads for the Newly Diagnosed SMA Care Package Program in Memory of Joseph Lillo.

Cure SMA is so incredibly grateful to receive a donation of 200 Bath Kneeler Pads from the Joseph Lillo SpinalMuscular Atrophy Foundation! These bath kneeler pads will be included in all Newly Diagnosed Care Packages in memory of Joseph Lillo.

These bath kneeler pads are beneficial to SMA parents by providing cushioned support for their knees and legs which allows them to bathe their child comfortably. Many SMA families love these bath kneeler pads since it provides a welcomed relief for tired knees which makes bath time a little easier.

Thanks to the Joseph Lillo Spinal Muscular Atrophy Foundation, a bath kneeler pad will be put into every Newly Diagnosed Care Package and sent out as soon as a newly diagnosed family has contacted Cure SMA.

These care packages are so generously funded by the Jacob Isaac Rappoport Foundation, and Adi & Shaina Rappoport, in memory of their son Jacob. The Jacob Isaac Rappoport Foundation has been fully funding the Type I Care Package Program since 2009. Cure SMA has sent hundreds of these care packages so that each child diagnosed with SMA in the United States can receive one.

Everyone at Cure SMA would like to extend our sincerest thanks to the Joseph Lillo Spinal Muscular Atrophy Foundation who have made such a wonderful donation to the Cure SMA Care Package Program in loving memory of Joseph Lillo. We would also like to thank the Jacob Isaac Rappoport Foundation for their continued support towards Cure SMA and its important programs.

To read more about the Type I, Type II or Type III Care Packages, please visit: www.curesma.org/support-care/

Cure SMA is incredibly thankful to have received dry erase calendars that have been donated by Michael and Stacey Zimmermann to our Newly Diagnosed Care Package program! These dry erase calendars will be included in the type I care packages that will be sent to newly diagnosed families when they first contact Cure SMA in memory of their daughter Peyton. Thank you to Michael and Stacey for this amazing donation to our care packages and for your incredible support!

A special thank you to Barbara Hetzer of Illinois who donated these hand knitted baby blankets and hats for our newly diagnosed care packages program.

Cure SMA is so grateful to have received a special delivery of teething key rings donated in honor of Barrett Silversmith for the SMA Type I Care Packages! These will be included in the care packages that will be sent to newly diagnosed families when they first contact Cure SMA.
Cure SMA would like to thank Angie Lee, SMA Type II and some of her high school friends for coming in on their day off of school to volunteer at our National Office! We are so grateful for our volunteers whose support is truly making a difference in the SMA Community!

A special delivery of stacking cups, arrived at the National Office, donated from Pamela Wright in honor of her granddaughter Audrey Quynh Wright for the SMA Type I Care Packages! Thank you for your incredible support, Pamela!

Thanks to some wonderful high school volunteers from Maine South High School in Park Ridge, IL, the truck was packed full with over 1,000 boxes on 16 pallets and ready to be shipped to the Gaylord National Resort for the Annual SMA Conference in June 2014!

Cure SMA received an exciting delivery of beanie babies that have been donated to our Newly Diagnosed Care Package program! Thank you so much to Betty Ortegus for this wonderful donation in memory of her nephew Christian Ward.
Hi Cure SMA,

Thank you so much! The care package was amazing! Everything in it is so helpful!

Thank you again! Getting this diagnosis is very difficult, but your organization makes it easier!

Nicole Robinson of Arizona

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Cure SMA,

Thank you SO much for letting Nella borrow so much equipment! The easy S stroller has given us a new sense of independence, and we used the car bed to travel to Wisconsin this past week to see Dr. Schroth! You have been a blessing to our family during a very scary part of our lives. Thank you for all you do for families affected by this disease.

Love, The Grutter Family of Missouri

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Hello Cure SMA,

My wife and I recently received our care packet and we would like to thank you all for everything, our son Mekhi loves everything, especially his sheepskin. The information in the package has been extremely helpful as well. Thank you again from the bottom of our hearts.

Deione Watkins of Georgia

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Dear Everyone at Cure SMA,

Our family wanted to let you know how receiving the care package meant so much... not just the things for Hannah to use, but the thoughtfulness and love, the time and energy you invest to get this to us.

The binder was really needed as well... it is wonderful to have such an organized resource, all ready to go!

Thank you all for helping us begin this new path life has brought us, I hope we can give back as much as we have been given!

Sincerely,
The Shepherdson’s of Washington
Hi Cure SMA,

My name is Jill Schwarck and my daughter Lily was diagnosed at the beginning of April with SMA Type I. I wanted to first of all, thank you and your organization for the binder of information we received as well as the wonderful care package. Lily loves her new toys and soft blankets. We are so thankful for the support we have received from the organization and information available to us. Thank you again!

It is very much appreciated.

Sincerely,
Jill Schwarck and Levi Schwarck of Iowa

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Hi Cure SMA,

We would like to send our most sincere THANK YOU for the wonderful care package you sent to our son Laszlo as well as the information package. During this very rough time, you have been a light in the darkness. Laszlo was so enthralled with the package that I couldn’t even get him to look up for the photo.

Thank you so so much.
Valerie (mother), Steven (father), Townes (brother), and Laszlo of Pennsylvania

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Good Morning Cure SMA,

I just wanted to take some time to let you know that the Braune family received their packages on Thursday evening. Brittany was overwhelmed by the kindness and thoughtfulness of your organization! Being such a new diagnosis, she obviously had a difficult time reading the stories and notes on everything, but she said she couldn’t be more grateful! And I received my informational packet on Friday afternoon.

Again thank you so much from the bottom of our hearts,

Gisella, Claudio and Tania

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Hi Cure SMA,

Thank you, thank you, thank you so much to you, the organization and all the parents that sent us some very well thought items. Tania has been enjoying them a LOT!

I still need to do the pictures and send them to you. I’ll try to get some done this week so you can see all the fun she is having with everything that was sent.

This gesture really touched my husband’s and my heart a lot, we cried when opening the box, going through the items and thinking about the fact of how some people that are “strangers” to us, can have such a warm gesture with our daughter and our family. I am very happy organizations like yours exist.

My words are left short to really explain how you made us feel, I wish I could hug everyone that contributed to us receiving the welcome package.

Also the informational booklets are great to learn more about SMA, expectations, proper cares, etc.

- Anton, Stephanie, and Elke Kliwer of Colorado

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Thank you so much for giving us a chance to attend the conference as a newly diagnosed family, we had a great time and learned tons.

XOXO - The Gritter Family, Don, Holly & Lylah of Michigan

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Jennifer of Texas
Congratulations to Garey Noritz on being named Physician of the Year! Dr. Noritz has been a part of our Medical Advisory Council for over 5 years and has been an important contributor to the Annual SMA Conference, our SMA Care Series Booklets and so much more.

**PHYSICIAN OF THE YEAR**

Garey Noritz, MD
Hospital Pediatrics

The Physician of the Year award was established to honor and recognize one physician at Nationwide Children’s for providing the highest quality family-centered clinical care, supporting and demonstrating Zero Hero principles and behaviors, demonstrating excellence in teaching, contributing to new knowledge through research and practicing One Team Values.

Garey Noritz, MD is the Director of the Complex Health Care Program and the Comprehensive Cerebral Palsy Program. He also plays an integral role in the SMA Clinic, Traumatic Brain Injury Clinic, Bone Clinic, Palliative and Hospice Care Team and the Partners for Kids Program. In all aspects of his practice, Dr. Noritz is focused on providing the highest quality family-centered care for each of his patients. Dr. Noritz firmly believes that every parent knows their child best and views them as an important part of the medical team. He recognizes that emotional, social and developmental support are integral components of health care. As a result, parents feel valued, respected and more confident in caring for their child with complex care needs. Dr. Noritz values each and every member of the many multidisciplinary teams that he works with. He has also collaborated with others to develop a strong research program that collects ongoing data of children with Cerebral Palsy to further the knowledge base for the medical community and to directly impact the care of the children that he treats.

**Innovation**

Our commitment to a treatment and cure is not just about seeking solutions—it’s also about creating them. We’re working with some of today’s sharpest minds to advance a diversity of approaches and champion the most promising discoveries and methods.
Loving Memories

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

Photo of: Tonya Willingham and her daughter, Hanna.
In Memoriam
Kimberly Faenza

My wife Kimberly Faenza passed away 6 months ago from a car accident, she had SMA Type II, was 42 years old and the best mother and wife anyone can ask for. My son (3 years old) and I will miss her forever.

In Memoriam
Isabel Poluchowicz


We wish to express our sincere thanks for all your support during and after our daughter Isabel’s life. From the newly diagnosed care package, equipment pool, advice, informational mailings and the annual conference where we met Dr. Schroth… your help has been very valuable to us and Isabel. Thank you for keeping us in your thoughts and prayers.

Anne & Andrei Poluchowicz

In Memoriam
Ta’Bria Neosha Collier

Words cannot express how much we appreciate your words of kindness, visits, phone calls, your prayers for strength, the gifts of flowers, food and support. Thank you so much for allowing our heavenly father to use YOU to help anchor our family through this very difficult time.

The Family of Ta’Bria Neosha Collier

In Memoriam
Jordan Clapp

September 14, 2013 – February 16, 2014

Dear Cure SMA,

Thank you on behalf of my entire family for all you do to work towards a cure for SMA. My son Jordan Clapp passed away from Type I on February 16, 2014. Thank you for your tireless work to save future children’s lives!

Sincerely, Parents of Jordan Clapp, Jenna & Paul Clapp
In Memoriam
Alexander Antonio Velazquez

In Memoriam
Jackson Roth

In Memoriam
Bren Scott Truesdale Jr.
August 7, 2013 - February 26, 2014

In Memoriam
Sophia Marie LaVoi
August 5, 2013 - June 5, 2014
In Memoriam
Kayleann Renee Early
October 9, 2012 - April 25, 2014

In Memoriam
Anneliese Reynolds
June 1, 1938 – August 28, 2014

Anneliese Reynolds, age 76 of Marceline, died Thursday, August 28, 2014 at Boone Hospital in Columbia. She was born on June 1, 1938 in Legnitz, Germany to Alfred and Anna Emma (Kambach) Sebenhaar. She married Donald Reynolds on December 25, 1968.

Anneliese had worked as a Registered Nurse. She was a bible school teacher, worked with Meals on wheels program, American Field Service-Foreign Exchange program, was a member of the Bethany Baptist Church in Marceline and had lived in Salisbury for 12 years and Marceline the past 23 years.

She is survived by her husband Don Reynolds of the home in Marceline; son Kirk Reynolds and wife Kennedy, Ridgefield, Connecticut; daughter Dietlinde Stitzer and husband Joe, Columbia, MO; brother Manfred Sebenhaar, Johannesburg, South Africa; sister Gudrun Buse, Heidelberg, Germany; and two grandchildren, Ellie and Grayden Stitzer. She was preceded in death by her parents and one brother Dietmar.
In September we announced the close of our for request for proposals (RFP) for clinical care research.

A clinical care RFP is an invitation for scientists to submit their best ideas for projects that address the clinical, psychological or social aspects of SMA. They explain what they want to study, how they plan to study it, and why they think it will help those affected by SMA.

The closing of the RFP means we can move on to the next step of the process, and make sure your donations reach scientists who can make a difference for our community.

This next step is a review by our Medical Advisory Council. They will carefully review all proposals to see which projects are the most intriguing, which have a well constructed study plan, and which match up with the most pressing unanswered questions about SMA.

Once this evaluation is complete, we determine which projects will receive a Cure SMA research grant. In December, we will announce those grants, which will include up to $50,000 for each project, for a total of $200,000 in new clinical care research funding.

**WHY WE FUND CLINICAL CARE RESEARCH**

Clinical care research is the fourth prong in our research strategy. The first three—basic research, drug discovery, and clinical trials—focus primarily (though not exclusively) on helping us find a cure for SMA.

While we work toward a future without SMA, we’re also working to give individuals and families the information and resources they need to live active, engaged, and hopeful lives today. That’s why we’re funding clinical care research. Clinical care research seeks to understand the issues that affect daily life for people with SMA, from breathing to nutrition.

Clinical care research can also be used to help educate the medical community about SMA. The more health care providers understand SMA, the more families will be able to access a doctor who understands and can explain the care options available to them.

**CURE SMA CONTINUING MEDICAL EDUCATION CONFERENCE PRESENTATIONS NOW AVAILABLE**

Cure SMA held its third Continuing Medical Education (CME), Interdisciplinary Perspectives on Spinal Muscular Atrophy: Defining Your Role in National Harbor, MD. This unique one day event was successfully attended by over 100 medical professionals caring for those with SMA from around the United States. Cure SMA partnered with the University of Wisconsin School of Medicine and Public Health who are accredited to provide continuing medical education credit for medical professionals. This conference focused solely on clinical care, and included didactic presentations and panel discussions from both regional and national expert faculty.

For more information about the CME, please visit: http://www.curesma.org/support-care/for-healthcare-providers/cme-conference/

The CME Conference continues to add a critical component of care to the Cure SMA Conferences by educating medical professionals on SMA. We look forward to seeing you in 2015!
Update from Dr. Lorson at University of Missouri on Spinal Muscular Atrophy Program being Funded by Cure SMA

Christian Lorson PhD, investigator in the Bond Life Sciences Center and Professor of Veterinary Pathobiology at the University of Missouri, has been working on the development of a novel compound found to be efficacious in animal models of disease. The compound is an antisense oligonucleotide (ASO). It increases life span in severe mouse models of SMA by four times and in intermediate mouse models of SMA by seven times. In April, a patent was filed for Lorson’s compound for use in SMA.

Cure SMA awarded $150,000 to Dr. Lorson and Dr. Burghes to investigate these new antisense therapies for Spinal Muscular Atrophy. The goals of the current Cure SMA funding for the program are two-fold:

1) to optimize the nucleic acid sequence of the drug, and
2) to establish the optimal dose of the ASO in severe mouse models of SMA.

“We are very grateful to Cure SMA for funding our ASO project as we hope we can continue to develop therapeutics that target an additional genetic element within SMN2. SMA is a very complex disease and it is possible that more therapeutic options will be required to effectively combat this disease,” stated Chris Lorson, Ph.D., Professor at University of Missouri.

Dr. Lorson’s therapeutic antisense oligonucleotide repairs expression from the back-up gene for the disease, called SMN2. The research was published May in in the Oxford University Press, Human Molecular Genetics. The drug developed by Lorson’s lab is conceptually similar to ISIS-SMNRx already in clinical trial developed by Isis Pharmaceuticals and a team of investigators at Cold Spring Harbor Laboratory headed by Dr. Adrian Krainer. Isis recently announced they have started Phase III clinical trials infants with SMA with their drug.

The Lorson drug is still in preclinical stages of drug development and several years from early stage clinical trials for safety. As mentioned, Cure SMA funding was awarded to optimize the candidate drug. Once the final drug candidate is identified, a full battery of biodistribution and safety studies will need to be completed for the FDA in order to initiate clinical trials on this new drug.

Cure SMA also recently awarded additional funding of $150,000 to Dr. Krainer at Cold Spring Harbor for related work.

“We are very grateful to Cure SMA for funding our ASO project as we hope we can continue to develop therapeutics that target an additional genetic element within SMN2. SMA is a very complex disease and it is possible that more therapeutic options will be required to effectively combat this disease,”

Chris Lorson, Ph.D., Professor at University of Missouri.
SHARING PHOTOS

Jovanka Daniel

Isaac Postma and Malorie Fox

Leah Vogedes and Landon Sun

Jack Freedman

Isabelle Perge

Lizzy Hallam

Laila Mei Yi McLauglin

Jaaziel Bass

Maura Nakanant

Ian Zurawski
SHARING PHOTOS

Olivia McLean

Maxwell Peppers

Maggie Alexander

Malorie Fox

Ollie Perry-Hoffman

Maxwell Peppers

Max Lasko
SHARING PHOTOS

Evan Vaudry

Emily Lozina

Madison Smith

Lylah Gritter

Ian Zurawski

Raelyn Toice

Eva Kelly

Hanna Warfield

Make today a breakthrough.

DIRECTIONS Fall 2014
This is our Alissa Aleah AGuero

Born on October 18, 2004 she sat, crawled, and walked until December 24, 2007. It was Christmas Eve when she ran towards the Christmas tree to grab a gift and fell fracturing her tibia. Her fracture healed and in February 2011 she stopped walking and began walking on her knees. After several fractures she was then referred to a geneticist. After labs and other tests, she was diagnosed with Osteo Genesis ImperFecta (OI).

Her fractures became more frequent and her muscles were getting weaker and weaker. Alissa was referred to a neurologist by her primary care provider. The day of the appointment came, we explained Alissa’s history and her diagnosis of Osteo Genesis ImperFecta (OI). The neurologist replied “Hm”, while holding his chin, “We will have to do some labs and a muscle biopsy and as soon as we get results the nurse will call you with an appointment to discuss the results.”

The results were in, so we were called; we were informed that Alissa had SMA Type III. So now not only did she have brittle bones but also weak muscles. The neurologist said it was a rare combination. My husband and I looked at each other as we were so confused; we had no idea what SMA was. We had so many questions but we were speechless at the same time; we went home that day feeling lost. The doctor explained the different types but as I was driving home all I could remember him saying was SMA when just a few months back the geneticist said OI. I was confused and scared.

As days went by I kept on getting calls from the Children’s Hospitals from the referral department as now Alissa would have to be monitored by an endocrinologist, GI, pulmonologist, orthopedist and she would also need therapies. I thought to myself what am I going to do. As I took Alissa to her appointment she was referred to MDA and we also met a family that gave us information about Cure SMA.

We attended our first Annual SMA Conference in Florida, June of 2011. Our life has definitely changed but we thank God everyday for our Alissa as she is a wonderful girl who now drives a powered wheel chair that I cannot keep up with. As she continues her battles with SMA and OI, she tells me not to worry. Alissa is my hero, she gives me strength to face life every day. Every day she wakes up with a smile and goes to bed with a smile.
Meet Caitlin Messick
Posted by Olivia de Fouchier on Aug 28, 2014

Olivia de Fouchier/Old Gold & Black

Growing up with spinal muscular atrophy, Caitlin Messick, a junior at the university, has been accustomed to doing things a little differently.

Messick, a Camden, ME native, was diagnosed with a motor-nerve disease, also known as SMA Type III, when she was two years-old, after her parents noticed that she walked with a constant limp. Until she was in 6th grade, Messick pursued normal activities just as her classmates and two younger siblings did.

“It would take me a little longer to do things, but I would always find a way,” she said. “I played soccer, tennis and even swam.”

This changed in the winter of 2004, when Messick was told that she needed to receive a full spinal fusion, surgery that would place titanium rods in her spine.

The doctors told Messick and her family that the operation would prevent her from walking again.

Messick was given one year before she had to have the surgery, one year to walk freely, one year before needing a wheelchair.

She remembers well the date of that surgery, December 2, 2005.

It was her best friend’s birthday.

“I didn’t really think about it that much until it was actually there,” she said about the surgery that was critical for her health but would change her mobility forever.

“I cried the morning of, but before then I just lived my life to the fullest.” As an eleven year-old, she took on a heavy burden.

“I always felt the need to be strong for everyone else,” she said, “without really thinking about what it meant for me.”

Throughout it all, her friends and family were very supportive, Messick said. A fan of the brilliant wheelchair-bound physicist Stephen Hawking, she laughingly said she and her friends joke about wheelchair accessibility, asking if places are “Hawking accessible.”

Messick says it has always been easy for her to adapt to changes and new environments.

When the time came to apply to college, she did not think twice about her intention to attend.

“College was always the next step for me,” she said. “It was never a question of whether I was going to go; it was a question of where I had to go.”

Messick chose to apply and attend Wake Forest because the school just felt right, she said, and it happened to be the most wheelchair accessible campus she visited.

Although she now lives 500 miles from her parents, who live in Wilmington, DE, the distance is not an issue, either.

“I wanted to establish myself and my independence like everyone else did,” she said. “You love your parents but you love the distance, too.”

In order to manage without her family’s support, Messick had to make arrangements.

Although schools usually hire medical assistants from external agencies, Messick chose to offer the position to fellow students.

“I knew there were students who were getting ready for medical school and needed the extra money and experience,” she said. “I figured I could let them help me out while I helped them out, too.”

Messick has tried to live the college experience like everyone else.

She attends sporting events, tailgates and parties. Even though accessibility is not always easy, Messick stays positive.

“There are difficulties, but I don’t notice them,” she says.
“I’m still there, still participating and having fun.”

Messick says that Wake students do not treat her differently, although she does note with a smile that people are probably more willing to hold the door for her.

“I make people look at me and not at my chair,” she said. “I ignore it, so they ignore it.”

When asked about her dating life, Messick responded with a shrug.

“Guys are generally sweet, but they tend to pity me rather than see me as someone they could be with,” she said.

“That’s something I’ve kind of accepted in my life.”

Messick does not let that affect her outlook. She now lives with five suitemates whom she sees as sisters and she can always count on them. When talking about the future, Messick said that as with anyone else, she has “her hopes and dreams,” and does not want to let her SMA interfere with them.

“Who knows what the world has in store for me,” she said. “We’ll see when I get there.”
Expanding your family after a diagnosis of SMA

Contributed by Anami Lehmann

Having our daughter Louisa was one of the most exciting times in our life, and then we were hit with the diagnoses of SMA. Like many other parents out there we did not know what this was and did not have a family history. We were shell shocked and did everything we could to keep her healthy and safe. We knew we wanted more children, but we needed to figure out what our options were.

Let me prefix this with saying that the choice is between you and your partner. There is no right or wrong answer. Do what is best for your family. The goal here is to provide options and information if you choose to expand your family.

Natural Conception

With that being said many families still choose to try to have a child using Natural Conception. Some families are successful and have a child that is either a carrier or a non carrier. However, many families are not successful and have multiple children with SMA. Please keep in mind: “If both parents are carriers of the chromosome 5 gene flaw, the risk of each pregnancy producing a child with the disease is 25 percent. This risk doesn’t change no matter how many children a couple has. The “dice are rolled” with each new conception”.

In Vitro Fertilization (IVF) with Preimplantation genetic diagnosis (PGD)

This risk does not change with IVF with PGD. The plus side of IVF with PGD is you only transfer “Healthy” Embryos. “Healthy” embryos are defined as embryos without SMA. These embryos can still be a carrier and there is still a 3-5% chance that the embryo can be affected with SMA. This is an expensive procedure, but depending on your health insurance policy, some insurance will cover a portion of the cost. Because this is such an expensive procedure some couples may choose to do natural conception but then test the ongoing pregnancy with chorionic villus sampling (CVS).

Donor Sperm/Egg

A slightly more affordable option than IVF with PGD is IVF with either a donor sperm or egg. With this option the donor is prescreened for being a carrier of SMA.

If you and your family choose to go with IVF please choose a clinic you are comfortable with. Many of the clinics have free informational sessions and will answer many of your questions during or after each session or make an appointment to talk specifically to the Doctors/Nurses you will be working with and their staff. If possible talk to SMA families who have had experience with these clinics.

For more information on the genetics of SMA, IVF or PGD please visit the cureSMA.org website to view the Genetics SMA Care Series Booklet.

Adoption

This brings me to the last option that I would like to discuss, Adoption. This can be just as expensive as IVF/PGD, and there are defiantly other risks and benefits associated with this option. Your family can either work with a private adoption agency or with the Division of Family and Children Services. There are several different types of Adoptions:

• Private Domestic
• Domestic Agency
• Adoption Consultants
• International Adoptions
• Foster to Adopt

http://www.jlsfamilylaw.com/georgia-adoption-options/

After Louisa’s passing in May of 2012 my husband and I looked at all our options and we made the decision that was best for our family. We are glad to announce that we had Eleanor Sarah Lehmann on July 18, 2014.
Candle Lighting

SMA Awareness Month

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

One of the highlights of SMA Awareness month is the SMA Candle Lighting. The Annual SMA Candle Lighting was held on Saturday, August 9th. Many families and SMA organizations around the country participated by lighting a candle at sunset to remember those who have lost their battle with SMA and to honor those with SMA who are still here fighting everyday! Here are all the great pictures that were posted to the Cure SMA Facebook page!
SMA AWARENESS MONTH
SMA AWARENESS MONTH
Cure SMA Chapters

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

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Have you ever thought about starting a chapter?

WE WANT TO HEAR FROM YOU.

CHAPTERS ARE REACHING OUT TO COMMUNITIES ALL ACROSS THE COUNTRY.

Cure SMA currently has over 31 chapters in the United States, but we are looking to expand!
Support your community | Fundraise for research | Hope for families | Begin to make a difference today

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

The Arizona Chapter enjoyed their 2nd Annual SMA Day at Chase Field with the Arizona Diamondbacks on June 22nd, 2014. The Diamondbacks lost to the Giants, but it was a win for Cure SMA.

Inaugural Byrds FORE a Cure 2014 Golf Classic

One hundred golfers joined us in Prescott, AZ on April 26th, 2014 for a successful day of increasing awareness of SMA, fundraising and great golf! Some unusual snowy weather made for a chilly day, but our players powered through the elements and eighteen holes and then won some great prizes – 1st place team won Nike drivers, 2nd place team won Nike putters, and the third place team received PF Chang’s gift cards. We are grateful to our wonderful sponsors and the local restaurants, golf courses, retail stores, businesses and the Diamondbacks for their incredible support of our Arizona Chapter of Cure SMA. With their help, and the generosity of all our golfers and dinner attendees, Byrds FORE a Cure raised over **$20,000** to Cure SMA! Thank you to ALL!

Cassandra Byrd
Phoenix, AZ

AZ Cure SMA Day at Mellow Mushroom Pizza Bakers

On May 6th, 2014 the AZ Cure SMA Day at Mellow Mushroom Pizza Bakers took place in Phoenix, AZ. The fundraiser allowed you to bring in the flyer to any Arizona Mellow Mushroom and they donated 25% of your bill to Cure SMA! In total, over $400 was raised for SMA research. Thank you to Cassandra Byrd for organizing this fundraiser!
NORTH CAROLINA

3rd Annual Loving Logan SMA Walk-n-Roll
The 3rd Annual Loving Logan SMA Walk-n-Roll was held on March 22nd, 2014 at the Smithfield Community Center in Smithfield, NC. Loving Logan is in memory of Logan Moore, who passed away from SMA type I in February 2012. In this event’s third year, over $6,000 was raised for Cure SMA! Thank you to Dana Grimstead and Layne Moore for organizing yet another successful event and for raising awareness of SMA! Keep up the great work!

1st Annual Jacob’s Joy Fundraiser
The 1st Annual Jacob’s Joy Fundraiser was held on May 10th, 2014 in North Carolina in memory of Jacob Berrier, SMA type I. The fundraiser raised over $1,400 for Cure SMA. Thank you to Justin and Kristin Berrier for organizing this fundraiser!

6th Annual JPL Golf Tournament
The 6th Annual JPL Golf Tournament was held on April 26th, 2014 at The River Golf and Country Club in Louisburg, NC. This year’s tournament, held in memory of Jocelyn Paige Lee, SMA type I, was an amazing success in so many ways. In total, $1,000 was raised for Cure SMA. Thank you to Shane and Jennifer Lee for organizing another successful golf tournament.

SOUTH CAROLINA

2nd Annual Rex’s Ride Motorcycle Run
The 2nd Annual Rex’s Ride Motorcycle Run took place on June 7th, 2014 at Clover High School in Clover, SC. Family and friends gathered to raise $1,000 for Cure SMA in memory of Rex Clark, SMA type I. Thank you to John Clark for all of your hard work in planning this event!
MARYLAND

2nd Annual Chesapeake Chapter Walk-n-Roll

The 2nd Annual Chesapeake Chapter Walk-n-Roll was held on April 26th, 2014 in Mount Airy, MD. The Walk-n-Roll exceeded the success of the previous year in raising well over $23,000 for Cure SMA. Family, friends, sponsors and individuals from the local community all came out and volunteered to make the day a success. More than 275 people participated in the one mile family fun walk around Old National Pike Park in Fredrick County, MD.

At the completion of the walk, Derek Lewis thanked the 18 teams of walkers and sponsors who supported the event, including BAYADA, Quest Diagnostics, and Our Little Jewels. Melissa Wayland and Mark LeHew shared their personal experiences with SMA. For the rest of the day, family and friends enjoyed lunch provided by Good to Go Markets, face painting, and a balloon artist for the kids. Families stayed well into the afternoon enjoying meeting other local families from the region and sharing their stories. Families traveled from across the Mid-Atlantic to join in the fun. A special thanks to Tina and Derek Lewis and Beverly Venedam for organizing another successful event! Keep up the great work!

Quarter Auction in Loving Memory of Olivia to Cure SMA

The Quarter Auction was held at the Fraternal Order of Police Lodge-34 in Middle River, MD on January 11th, 2014. All profits benefited Cure SMA in memory of Olivia Hemmeain, SMA type I. A special thanks to Melissa Wayland for organizing this event, raising over $1,500 for Cure SMA!

Connecticut Chapter

3rd Annual Cubby’s Run for Cure SMA

On May 4th, 2014, the 3rd Annual Cubby’s Run for Cure SMA was held at Ridgefield Rec Center in Ridgefield, CT. The event was hosted by Ethan Hynes in honor of his best friend, Cubby Wax, SMA type I. Ethan and Cubby met playing on the same baseball team when they were six years old and have been friends ever since. This year, the event raised over $30,500 for Cure SMA! Thank you to Ethan for all of his hard work and dedication in planning this event!
Friends of Julianna Benefit Dinner

My daughter Julianna was diagnosed with SMA type II in October 2013. Learning about the disease and the struggles that we might have to face is not an easy thing.

We have a huge support system here in Norwich, CT and a lot of family and loved ones wanted to help. It was suggested that we have a benefit to help with the financial expenses we will face, as well as to help spread awareness as myself and everyone I know had never heard of SMA.

We organized “The Friends of Julianna Benefit Dinner” in a short amount of time, and the event was held on January 31st, 2014. The dinner was a huge success, with the help of friends and family members selling tickets and passing out flyers everywhere. We had around 200 people attend the dinner. We also had a countless number of donations from people and businesses across the country. In total, we raised $1,350 for Cure SMA.

It is very important to me, Julianna, and everyone affected by SMA that we do everything in our power to help find a treatment, and eventually a cure! It is our hope that someday we can stop this disease from taking away what is rightfully ours!

Sincerely,
Julianna and Heather Lariviere
Norwich, CT

West Rocks Middle School Fundraisers

West Rocks Middle School in Norwalk, CT keeps fundraising for a cure in honor of Cubby Wax, SMA type I, son of teacher Laura Wax.

By making and selling band bracelets and walking for a cure in April, WRMS students raised over $220!

We are very proud of our students! Together we will find a cure!

Robin Gredinger
Norwalk, CT

Greater Florida Chapter

CHAPTER UPDATE

Greetings from the Greater Florida Chapter!
Stretching from south of Sarasota to the north Florida panhandle, our very active chapter covers a large and diverse geographic region and includes many amazing families. We invite you to stay connected by regularly checking our “Cure SMA – Greater Florida Chapter” group on Facebook.

We also send a monthly e-mail newsletter to our chapter members. Not sure you are on the e-mail list? Send a message to greaterfl@curesma.org with your name and contact information today!

State of the Chapter:
The Greater Florida Chapter of Cure SMA raised almost $70,000 in calendar year 2013. That brings us to more than $260,000 in contributions to SMA research and family support programs since our chapter was established in January of 2009. Our chapter’s goal is to add at least another $60,000 to that total by December 2014, and we are on our way there!

Every dollar raised by our chapter is used to fund SMA research and family support programs. From lemonade stands to golf tournaments, every fundraiser makes a difference. THANK YOU for being a part of our success!

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

2014 Board Members
President: Audra Butler
Vice-President: Laurie Sore
Treasurer: Shawn Santos

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

If you have any questions for our executive board, please feel free to contact us at 727.388.1888 or greaterfl@curesma.org.

New Chapter Address:
The Greater Florida Chapter has a new mailing address. If you need to send us a fundraising check (or just want to drop us a line), you now can reach us at: 18865 State Road 54 #115, Lutz, FL 33558
**Chapter Initiative:**
Our board is working on creating a comprehensive list of health care providers in our chapter area. With this list, we can better support the medical needs of our members, especially newly diagnosed families, and better educate the healthcare professionals who serve us about SMA. But, we need your help to create this much-needed resource. Please go to our “Cure SMA – Greater Florida Chapter” group on Facebook and fill out the online form that is posted there – it will only take a minute of your time, but it will help so many!

**2014 Conference:**
We’d like to extend a huge thank you to Dr. Laura Drach with All Children’s Hospital in St. Petersburg. Dr. Drach took time out of her busy schedule to attend the Annual Conference as our chapter’s medical provider delegate, with the goal of learning more about SMA and how to better care for her patients living with the disease. We really appreciate her commitment to continuing education and the well-being of her patients. If any of your healthcare providers are interested in attending the 2015 conference, please let us know!

Another thank you goes to Lisa Shockley, chapter secretary, who was our chapter’s representative at the conference. Lisa loved meeting everyone and sharing her conference adventures on our chapter’s Facebook page!

**Alpha Tau Omega Tennis Tournament**

In line with our goal to continually benefit the community, our chapter of the Alpha Tau Omega fraternity hosted of annual philanthropic events to raise money for a local charity. This year, we have teamed up with the Cure SMA in an effort to raise awareness for SMA in honor of Elizabeth Caballero, SMA type I.

Our fraternity decided to host a premiere philanthropic tennis tournament to help further research about this rare disorder. On April 5th, 2014, ATΩ hosted our first annual philanthropy tennis tournament at Tom Brown Park. With over 20 organizations, including 16 Florida State University sororities, ATΩ raised $10,000 for Cure SMA.

As a brotherhood, we are extremely proud of the work we have done to help fight for this uplifting cause. My brothers and I want people suffering from SMA to know that they can count on the Episilon Sigma chapter of the Alpha Tau Omega fraternity at Florida State University for our continuous support towards Cure SMA.

David Butter
Tampa, FL

**Holy Family Church Fundraiser**

The Holy Family Church Fundraiser took place on June 1st, 2014. Audra Butler of the Greater Florida Chapter organized this successful event, raising over $1,000 for Cure SMA! Thank you, Audra, for organizing the Holy Family Church Fundraiser and raising awareness of SMA!
St. Patrick’s Day PotLUCK
On March 15th, 2014, eight families attended an afternoon potluck event at the Andrea Trakas Memorial Playground at Simmers-Young Park in Winter Haven, FL. The fundraiser brought more than 50 people and a TON of delicious food with them! The kids had a great time on the accessible playground, which is dedicated to preserving Andrea’s memory, SMA type I. The adults had fun catching up with old friends and even making some new ones!

Painting for Andy
On Saturday, June 7th, 2014, 17 friends and family members picked up their paintbrushes on a sunny Saturday in June to create a masterpiece in memory of Andy Butler, SMA type I, and to raise money for Cure SMA in his name. The painting, which was in hues of gold and brown, featured a sunset at sea and the words “Cherish Yesterday, Dream Tomorrow, Live Today.” Painting with a Twist in Trinity, FL hosted the event and donated a portion of their proceeds, totaling $260.

Thirty One Tote Bag Drive
On May 2nd to May 15th, 2014, the Greater Florida Chapter collected $20 donations to purchase Red Wave Lunch Totes to include in Cure SMA’s Newly Diagnosed Care Packages. A portion of the sales proceeds went back to Cure SMA!
NYC Half Marathon
The NYC Half Marathon took place on March 16th, 2014 in New York, NY. Cure SMA and the Erwin Family teamed up to run in the 2014 NYC Half Marathon. The day consisted of a great race and raising money to help find a treatment and a cure for SMA. The New York City Half-Marathon took runners through famous landmarks such as Central Park and Times Square and annually attracts more than 10,000 runners. In total, the NYC Half raised over $32,000 for Cure SMA! Thank you to Michele and Douglas Erwin for organizing this fundraiser and raising awareness of SMA!

Ski Away SMA
Our Ski Away SMA 2014 fundraiser was a great success. Friends, family and newcomers came together once again to join the fun on and off the slopes at Magic Mountain on March 15th. The day and night included: ski races, an extreme skiing competition, tubing, après ski fun, raffles, a silent auction, dinner, music and video footage of our angel Hailey, SMA type I, and snapshots of the day on the slopes.

We are so fortunate to have so many who support us in carrying on the memory of Hailey SMA type I, and committed to helping Cure SMA find a treatment and cure. Taylor and I wouldn’t be able to pull off the weekend without the support of the great people at Magic Mountain and our families and friends. As always, we are incredibly grateful for the support of Maureen and Rich Aneser. Each year they pour their hearts into insuring our event is a huge success. This year we were able to raise $25,000 for Cure SMA and Maureen and Rich were a huge part in making that happen!

Marie and Taylor Smelser
Armonk, NY
4th Annual SMA 5K – Eat ‘n Run
The 4th Annual SMA 5K – Eat ‘n Run was held on March 1st, 2014 at Hardaway High School in Columbus, GA. This event also had a balloon release to honor the memory of those lost, and to pay a tribute to those who are fighting against SMA. This event was a huge success, raising over $30,700 for Cure SMA! A huge thank you goes to Kari Merriken for organizing this successful event! Keep up the great work!

Peyton’s Pals
Our daughter Peyton Grace Zimmermann, SMA type I, was born on July 2nd, 2013. She was officially diagnosed with SMA type I on December 30th, 2013, although we had a tentative diagnosis for a while. Nonetheless, it was devastating. She continues to get brighter and brighter as her body slowly weakens, and it breaks our hearts. We were told to contact Cure SMA when we were diagnosed and were surprised and blessed by the immediate response to our family. We received a care package, wagon, and car bed within just a couple of weeks of contacting the organization. Any email we’ve sent has been responded to so quickly and with such obvious care. Cure SMA has truly touched our hearts, and we want to thank you for that.

On February 19th, 2014, some friends of ours organized a t-shirt campaign called Peyton’s Pals through Bonfire Funds to raise money for Peyton’s care. We would like to donate half that money to Cure SMA to be used for other families whether it’s through research or much needed items for SMA children. In total, we raised $1,750 for Cure SMA. This donation is in honor of Peyton Grace Zimmerman, SMA type I.

Thank you,
Michael, Stacey, and Peyton Zimmermann
Atlanta, GA

The Lizard Thicket Fundraiser
On March 1st, 2014, The Lizard Thicket Fundraiser was held at Lizard Thicket Stores in GA. This fundraiser raised over $1,700 for Cure SMA! Thank you, Sheri Waynick, for organizing this event and raising awareness of SMA!

4th Annual Tee Off with the Drive to Cure SMA in Honor of Ryan
The 4th Annual Tee Off with the Drive to Cure SMA was in honor of Ryan Manfre, SMA type II. The event took place on May 17th, 2014 at Glen Eagles Country Club in Lemont, IL. Ken and Jeanne Emerson organized the event along with a committee of friends and family in honor of their grandson, Ryan. This year, the event raised over $45,600 for Cure SMA!

Thank you to Ken, Jeanne and all of the volunteers for your hard work and dedication that helped make this year’s event such a huge success!
8th Annual Illinois Chapter Walk-n-Roll
The 8th Annual Illinois Chapter Walk-n-Roll took place on June 22nd, 2014 at Independence Grove in Libertyville, IL. Hundreds of people gathered at the event for a day of family fun! Everyone enjoyed the DJ, face painter, balloons, and magician. Thanks to the hard work of Chapter President, Janet Schoenborn, and many volunteers, the event raised over $39,500 for Cure SMA!

St. Peter School Spirit Day
St. Peter School in Quincy, IL hosted a school spirit day to raise awareness of SMA! The spirit day was a success, raising over $800 for Cure SMA! Thank you to the St. Peter students for raising awareness of SMA and all of your hard work in fundraising for Cure SMA!

Iowa Chapter

Alan Shepard ES Pink and Sparkles and Superheroes Day
As a student council project for the month of February, Alan Shepard Elementary decided to raise funds and awareness for SMA. We chose this project in honor of Ella Christopher, SMA type II-III, and in memory of Jack Lindaman.

As a student council, we sponsored a “Pink and Sparkles and Superheroes for SMA!” day in which students could dress up in pink, sparkles, superhero gear, or all three!

We asked students to bring in donations during the week of February 10th, with our special dress being on February 13th, 2014. In total, we raised over $3,159 for this fundraiser.

Sincerely,
Alan Shepard Elementary
Eldridge, IA

Kansas City Chapter

KANSAS
Wine Tasting Night
Kali hosted a wine tasting night in honor of Nella Grutter, SMA type I, in May 2014. Thanks to Kali’s hard work and the generosity of her family and friends, over $3,800 was raised for Cure SMA!

MISSOURI
Gracie’s 3rd Annual Steak Dinner for SMA
Thank you to Sherry DeGraffenreid for hosting Gracie’s 3rd Annual Steak Dinner for SMA on April 26th, 2014! Family and friends from Eugene, MO gathered together to raise SMA awareness and funds for Cure SMA in memory of Gracie DeGraffenreid, SMA type I. Gracie passed away shortly before this year’s event. Thanks to the generosity of everyone that attended, more than $6,500 was raised in her memory.

Phi Tau Omega Sorority Trivia Night
Thank you to the ladies of the Delta Phi Chapter of Phi Tau Omega sorority of St. Louis, MO for raising $1,900 for Cure SMA! The chapter members hold this event each year in February in honor of Brittany Carpenter, SMA type II.
Pelican’s Half Time Event
Thank you to Krista Scurria for organizing a half time with the New Orleans Pelicans in New Orleans, LA to benefit Cure SMA in April 2014. Thanks to the overwhelming support of the spectators, more than $11,000 was raised!

Briley FAITH’S Walk & Run for a Cure
The 2nd Annual Briley FAITH Turner 3K Walk and Run for a Cure, held June 7th, 2014, was another success! We had over 100 participants and raised nearly $7,000! The walk was held in Alexander, AR, at Pinecrest Memorial Park, who is the founding sponsor. We were honored to have Alanny Oaks, SMA type II, and her family attend this year. Much to our surprise, there was a family who just moved from Iowa that came because their mom took care of a little boy with SMA. She heard about the walk from the doctor’s office that Briley’s aunt works at and wanted to support others with SMA.

A couple of “musts” within the walk, were Briley’s Bows—a bowtique set up by Briley’s Idgie, for little girls and mommas to dress up their hair, a bounce house for the kids to release energy, and Arkansas Therapy Outreach provided free face painting. Many local businesses donated items for the Briley FAITH Silent Auction, from Photography family sessions, boutique shopping certificates, bikes, grills, and the list goes on and on. Over 15 restaurant gift cards were raffled off to help raise additional funds for Cure SMA.

The 3k walk toured the park, passing the geese on the pond, the tall white cross in the middle gardens, and eventually coming to the Bradford pears lined in the Garden of Tranquility. As one passed Briley’s final earthly resting place, you were greeted by pink roses and a picture of what Briley looks like now, in Heaven; those big brown eyes smiling at you. Pink balloons were continually released at her grave as runners and walkers passed by.

New Orleans Rock-n-Roll Marathon
SMA families and supporters from Missouri down to Louisiana participated in the 2014 New Orleans Rock-n-Roll Marathon in New Orleans, LA on February 2nd, 2014 to benefit Cure SMA! Runners ran in honor of Matilda “Tilly” McRoberts, SMA type II, and Jackson Audibert, SMA type II. Thank you to the following people for running the race and fundraising for Cure SMA: Shawn Audibert, Alicia Beranek, Matt Davidson, Lindsey Derrington, Dana Derrington, Johanna Duren, Don Erickson, Brian Felger, Dan Levinson, and Kenny Malter. Over $4,600 was raised for Cure SMA!

Briley FAITH Turner lived an inspiring 61 days here on this Earth. Though she never made a sound, even a cry, her life impacted many for the better. We look forward to the day of holding our precious baby girl again, hearing her cry her first cry, and seeing her first smile! Our hope and prayer is that by keeping her memory alive, we can increase awareness of SMA, provide support to those in need, and help raise funds to find a cure for this terrible disease.

Crystal Turner
Benton, AR
10th Annual Michigan Chapter Run, Walk-n-Roll and Family Fun Day

The 10th Annual Michigan Chapter Run, Walk-n-Roll was held May 3rd, 2014 at Hawk Island Park in Lansing, MI. SMA families and friends from all over the state gathered for a fun-filled day to raise money for a treatment and cure!

Thank you to Ken and Cindy Armbrusmacher for all of their help planning and putting on the race each and every year. Along with their family and friends who volunteer, they have been an integral part of the event’s success for the past ten years! Deb Postma, who has made the kid’s fun run exciting for everyone and carried that excitement over into the raffle. Melissa House and Team Abbey for the amazing job they do fundraising each year. Wolfe Company and Linda and Harvey Wolfe for being such generous sponsors over the years. Thank you to Jammin’ DJ’s who have been at our event for all 10 years!

A special thank you to Holly Schafer for all of the hard work she has put into planning this event for the past 10 years! Holly’s hard work helped the event grow each year. The 10th Annual Michigan Chapter Run, Walk-n-Roll raised over $58,800 for Cure SMA!
We laid it out over the course of a week. During charity week, we sold SMA t-shirts, sunglasses, bracelets, magnets, and lanyards at every sporting event. In attempt to get the whole district involved, we sent representatives to every school to educate the younger kids as well. We also had some of the spring athletes speak in a video explaining what SMA is and showed it at an assembly. My aunt, Sydney’s mom, spoke at the assembly as well and it was very effective, showing a personal perspective. Some of the spring sports teams came together and bought t-shirts with their numbers on the backs to wear as a team to their game that week. At school and sporting events, we set out signs with the faces of SMA angels on them which were also very effective. It was a very busy week but so worth seeing our district come together in support of SMA.

Everyone in student council worked very hard to make this fundraiser successful and we ended up raising a total of $1,800 as well as a bunch of awareness. It makes me smile every time I see someone wearing their SMA t-shirt now because it reminds me that people care. My teacher, my peers, my school, my district, and my community, they care because they now know what SMA is.

Elyssa Oostdyk
Dorr, MI

**3rd Annual Bowl with Brayden 4 Cure SMA**
The 3rd Annual Bowl with Brayden 4 SMA event was held on May 10th, 2014 in Muskegon, MI. Everyone enjoyed an evening full of bowling, raffles and lots of fun! The event raised over $3,000 in honor of Brayden Janetzke, SMA type II. Thank you to Christina Janetzke for all of her hard work planning this event each year!

**Wayland High School Student Council Fundraiser**
When my Student Council class at Wayland Union High School was looking for a charity to raise money for, I was so excited to suggest that we choose Cure SMA because so many people are unaware. My student council classmates were quick to come up with awesome ideas of how we could help and even quicker to support me. We brainstormed and planned nonstop until our fundraiser came together.

The 3rd Annual Bowl with Brayden 4 Cure SMA event was held on May 10th, 2014 in Muskegon, MI. Everyone enjoyed an evening full of bowling, raffles and lots of fun! The event raised over $3,000 in honor of Brayden Janetzke, SMA type II. Thank you to Christina Janetzke for all of her hard work planning this event each year!

**10th Annual Michigan Chapter Hot Dog Fundraiser**
Thank you to Rob and Alisha VanderJagt for hosting the 10th Annual Michigan Chapter Hot Dog Fundraiser held on May 3rd in Lowell, MI. The event raised over $600 in honor of Malorie Fox, SMA type II.

**Minnesota Chapter**

**William Anco Memorial 5K**
The William Anco Memorial 5K was held on May 3rd, 2014 at Chisago Lakes High School in Lindstrom, MN. Chisago Lakes High School students rallied together to raise funds for Cure SMA in memory of William Anco. Thanks to the hard work of the students and their teacher, Scott Buchkoski, over $4,100 was raised!

**Save the date!**
Thursday, June 18th - Sunday, June 21st, 2015

Cure SMA 2015 Conference Kansas City, MO
NORTH DAKOTA

Jack Attack on SMA: Round 10

On June 7th, 2014, the Jack Attack on SMA: Round 10 Walk-n-Roll took place in Lindenwood Park - Rotary Shelter in Fargo, ND. In total, the event raised over $15,000 for Cure SMA! Thank you to Kristi and Rod Gellner for hosting the Jack Attack on SMA: Round 10 Walk-n-Roll in honor of their son, Jack, SMA type II! For the past ten years, the Gellner family has been raising funds to help us knock out SMA! Kristi has done an amazing job planning this event every year. For the past ten years, the Gellner family has been a tour de force in raising funds to support the work at Cure SMA. SMA families from across the great state of Nebraska have traveled to be a part of this incredible event and support Cure SMA and the Gellners.

A special thank you to our SMA fighting champs who participate in the event: Jack Gellner, SMA type II, Cole Pulkrabek, SMA type III, Trish Kuemper, SMA type III, Chloe Bartholome, SMA type II, Everly Batholome, SMA type II, and Ashley Wohl, SMA type II!
We owe much thanks to the volunteers and business partners who helped with this event. A team from Biogen Idec, who are researching treatments for SMA, participated for the first time and made a generous $3,000 sponsorship donation. Many of the volunteers have been coming for 5-10 years and they keep the whole event running smoothly. We are very grateful to the local businesses and friends who donated most of the supplies, refreshments, and raffle items. We hope to see everyone again next May!

Silvia Murphy
Norwell, MA

New England Chapter

MASSACHUSETTS
14th Annual New England Chapter Cure SMA Walk-n-Roll

The New England Chapter hosted their 14th Annual Cure SMA Walk-n-Roll on Saturday, May 17th, 2014 at DCR Wompatuck State Park in Hingham, MA. Silvia Murphy, Stacey Farrell, and Don Norton teamed up to organize this walk that brought together about 700 participants -- including 30 families affected by SMA -- and raised over $118,000. This brought the overall total of the New England walk to over $1 million!

Many families and friends came together from throughout New England to raise money for a cure for SMA. The top fundraising teams this year were: Team Murphy, Goin’ for Owen, Walking for William, Wizards of Oz, Team BraeKer and Team Aileen; followed by Mac’s Pack, Team Vivienne for a Cure! and Team Melanie Lee. Also contributing over $1,000 each were Chloe’s Club, Angelica, Mikaela’s Crew, Team Addison Barrett, Team Nico, ForEva, Team Ian, and Glen’s Gang. Also participating were Team Evan, Team Greer, Ella’s Entourage, Walk for Tamara, and Team Carolyn. Other SMA individuals that were represented include: Ethan Carter, Brian McDonald, Dahrian Pimentel, Mark Butler, Annika Cederlund, Maya Cutrone, Lauren Rogowicz, and Sean Venezia.

The forecast was grim and the volunteers had to set up in pouring rain, but by starting time they were rewarded with blue skies and eventually sunshine! The walk was 1.5 miles on a paved path through the woods, including a picturesque water view. Participants were entertained by a visit from Blades (the Boston Bruins’ mascot) and two ice dancers. They also enjoyed the local fire truck, face-painting, carnival games with prizes, a great raffle, and an assortment of refreshments. The favorite treats, as usual, were the ice cream cups donated by H.P. Hood and the potato chips donated by Utz.
2014 Rogo Cup SMA Golf Tournament
The 2nd Annual Rogo Cup Golf Tournament for SMA was held on June 2nd, 2014 at the Atlantic Country Club in Plymouth, MA. The Rogowicz, Butler, Spiegel and Scully families hold the tournament in memory of Mark Butler, SMA type I, in honor of Billy Spiegel, SMA type I, and Lauren Rogowicz, SMA type III. We are so very grateful, humbled and blessed to have such wonderful support from our sponsors, donors, golfers and volunteers again this year. With their support we raised over $45,000 to support Cure SMA. We traded umbrellas for sunscreen this year and a great time was had by all! We can’t thank our supporters enough. Thanks to them and so many other great Cure SMA supporters and events, we have hope and see real results toward a treatment and cure for SMA.

Joe Scully
Marshfield, MA

Jordan’s Journey Comedy Event
Jordan’s Journey Comedy Event took place on April 12th, 2014 at Meppo Shriner’s Auditorium in Wilmington, MA. The event was very successful, raising over $5,600 for Cure SMA! This fundraiser is in memory of Jordan Clapp, SMA type I. Thank you to Jenna and Paul Clapp for organizing this event!

Race in Honor of Victoria Meneghini
My name is Philip and my sister died from SMA in 2003. Her name is Victoria. Ever since she died, I have been running a road race close to her birthday every year. Ironically, it is also close to Valentine’s Day. Every year, I also try to raise money to help find a cure for SMA. On February 10th, 2014, I collected $250 for Cure SMA. I hope you use it to help other people with SMA.

Sincerely,
Philip Meneghini
Ipswich, MA

Melrose Cooperative Bank Denim Day
The Melrose Cooperative Bank Denim Day fundraiser raised $210 for Cure SMA! These funds were donated by the employees of Melrose Cooperative Bank for the privilege of wearing denim to work every Friday. Your charity was chosen to receive the funds we collected for the month of June. This fundraiser took place in Melrose, MA during the month of June.

Thank you.
Christine Wainer
Melrose Cooperative Bank
Melrose, MA

Raising Funds for SMA One Painting at a Time
Arianna Milani, a senior high school student in Petaluma, CA, has donated $500 to the New England Chapter Walk-n-Roll for Cure SMA. She has made her generous donation in honor of Mikaela Chicoine of Salem MA, Arianna’s cousin who is affected by SMA. Ari combined her love for her cousin with her love for art to raise funds and awareness for SMA.

As part of her curriculum at Casa Grande High School, Ari needed to come up with a senior project that included a minimum of 20 hours of non-profit work. The assignment is designed to have students give back to their community. Arianna, a talented artist, decided to base her project around her artwork because it is something she is passionate about.

Ari asked for donations for Cure SMA in lieu of payment by selling her artwork at local community events and by commission artwork. She also launched a successful website for her senior project raising awareness for SMA. Check it out at ariartwork.com. Ari has spent countless hours on her project and was thrilled to personally deliver her donation to Silvia Murphy, the main organizer of the New England Walk-n-Roll.

Arianna Milani
Petaluma, CA
NEW HAMPSHIRE

Cure It with Comedy

The 2nd Annual Cure it with Comedy took place on April 19th, 2014, at Milly’s Tavern in Manchester, NH in honor of Constantine Salce, SMA type II. A new venue and a new line-up added up to a successful event! Nick LaVallee, a native of Manchester, NH, and a regular at the Laugh Factory in Las Vegas and Hollywood, was the opening act. Rich Vos, from Last Comic Standing and Comedy Central was the headliner. Both Nick and Rich seemed to have as much fun on stage as the audience had watching and listening to them! The evening also involved several raffles which included New England sports memorabilia and a pair of tickets for the upcoming Boston Bruins/Detroit Red Wings Play-off game! In total, this event raised $6,000 for Cure SMA! A huge thank you to Dominic and Luciante Salce for their continued support and enthusiasm for planning fundraising events in New Hampshire!

Angels for Brooklyn

On March 9th, 2013, we lost our beautiful baby girl, Brooklyn Elizabeth, to SMA type I at just six months of age. Knowing that the angel anniversary date was approaching, and that it was going to be a very emotional one for us, we decided to do something in Brooklyn's name. During the entire month of March we held "Angels for Brooklyn" at Hill Top Pizza in Epsom, NH. Hill Top is owned and operated by Brooklyn’s daddy, Mathew Bouchard, and her uncle, Christopher Bouchard. This gave us plenty of opportunity to spread awareness in a new town and allowed people to learn about Brooklyn. Mathew and Christopher hung all of the purple angels around the restaurant to be displayed as they continued to be purchased. A total of $200 was raised in the month of March, in honor of our Brooklyn. We would like to thank Hill Top Pizza for being our host, along with everyone who came in and donated.

Brooklyn is missed very much, and not a day goes by that we’re not talking about her and thinking about her!

Stephanie Bouchard
Epsom, NH

New Jersey / Delaware Chapter

CHAPTER UPDATE

The chapter has been very busy the past few months with many events hosted by several families and businesses. We were fortunate to have three walks in New Jersey.

Thank you to all of the other families that have in some way done your part to raise awareness or much needed funds to find a treatment and cure for SMA! If any families would like to host a fundraiser and need help, please feel free to contact me at jnjmoyer@comcast.net, as I am more than willing to support your efforts.

Finally, I want to tell you about my experience at the National Conference in Washington, D.C. Five or so years had gone by since I attended a conference. As I sat listening to the latest advances in research, I was amazed! It was such a proud feeling that for the past 12 years I have been part of something so great and that my efforts are making a difference. As many of you know, my son Steven passed away from SMA over 12 years ago, but in his memory, I feel that my calling is to see a treatment and cure for SMA. My take away from the conference (besides visiting with many tremendous, inspirational families), was that every penny counts and that this organization will be the reason SMA will not exist some day!

Jessica Moyer
President
Cure SMA-New Jersey/Delaware Chapter
**DELAWARE**

**2014 Delaware Marathon**

On Mother’s Day, over 40 runners participated in the Delaware Marathon and over $15,500 was donated to Cure SMA. This year, we had many small events leading up to the Marathon. Three Dover, DE area schools, Fairview, South Dover and East Dover, held Zumba classes for the students, and all proceeds were donated towards our event. In addition, Holy Cross Elementary School held a tag day for four students who ran on marathon teams, Pierce Cheslock, in honor of his sister Eden, and Jake, Blaise and Isobel Moyer, in memory of their brother Steven. Over $600 was collected. Also, a special thank you to Gary Knox, owner of Forneys Too in downtown Dover, for his very generous matching gift donation.

A special thanks to all of our runners: Carrie Apter, Michael Apter, Kelsey Barry, John Cheslock, Nicole Cheslock, Curt Clouser, Andrea Collins, Cristina Colon, Tina Dombroski, Jeanette Foor, Dean Holden, Kim Holden, Morgan Howell, Cara Hughey, Ted Janusz, Lauren Janusz, Alex Janusz, Zoey Janusz, Dave Keane, Nicole Keane, Jennifer Kelly, Kenny Kelly, Joe Leighton, Jason Moyer, Jessica Moyer, Isobel Moyer, Blaise Moyer, Amy Reville, Erica Richard, Luis Rios, Jennifer Rios, Ethan Robinson, Laurie Shahan, Sarah Sharp, Joan Smith, Tanya Spiezio, Alyssa Sweeney, Philip Wandless, and Deb Yingling!

**Hanna’s Hope for a Cure 1st Annual Softball Tournament**

Our chapter was blessed to have a new family, the Warfields, host a softball tournament in Georgetown, DE in honor of their daughter Hanna, SMA type II, and donated over $6,000 for Cure SMA! A special thanks to Gary and Ashley Warfield for organizing this event!

**Coastal Carwash Fundraiser**

Coastal Carwash held a fundraiser in Dover, DE and 10% of proceeds over three days of car washes were contributed to Cure SMA.

**NEW JERSEY**

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

On June 6th, 2014, Cocktails for a Cause took place at the Hilton Sky Room in Hasbrouck Heights, NJ. Friends and families gathered for a night of cocktails, hors d’oeuvres, a live band, DJ and fabulous raffle prizes. Everyone was in the company of great friends who share our vision of finding treatments and a cure for SMA in memory of Ryan Reilly, SMA type I.

The event was very effective, raising over $41,000 for Cure SMA! Thank you to everyone who joined Ryan’s Buddies in an effort to raise awareness about SMA. A special thank you to Lori Zorat, Glenn Rosenbower and Wendy Rosenbower for organizing this event, which will take place every June in honor of Ryan’s birth month.
9th Annual Steven’s Walk to Drum Out SMA
The 9th Annual Steven’s Walk to Drum out SMA was held on May 3rd, 2014 at Newton Lake Park in Haddon Township, NJ. It was a beautiful day filled with family, friends, neighbors, our local fire department, our local Walgreens, Hair Cuttery, a live band (JAM), some new faces. We had wonderful chinese auction baskets, donated by family members, friends, many companies and BAYADA. We had a 50/50 and the young man that won donated all the money back. We have many wonderful people in our lives to help solicit many different companies for these auction items. Our family and good friends help us continue to support SMA and run Steven’s Walk. This Walk is very important to all of us. We are working hard to find a cure or treatment, hopefully in our son’s lifetime. Steven has SMA type III and is a junior in high school.

With Steven heading into his senior year of high school next year, we are planning a bigger and better Steven’s Walk! We are planning on making some fun changes for this exciting time in his life. This will be the 10th Annual Steven’s Walk. Most of these people and companies have been with us since the first year. It’s going to be even more special to us. This year Steven’s Walk raised $13,000 for Cure SMA.

Mike and Terri Potter
Hadden Township, NJ

7th Annual Cure SMA Walk-n-Roll in honor of Katherine Santiago
For seven years, the Walk-n-Roll in memory of Katherine Santiago, SMA type II, has been held in Millburn, NJ. For seven years, our small event has grown into an annual Walk that I hope is making a difference in the SMA community. This year, we raised over $7,000 for Cure SMA and more importantly, raised awareness for SMA. At every walk, good weather and company have always shown through. And every year, our walk has introduced us to new SMA families, which remind us every day that we should continue working towards discovering a cure for SMA.

As our walk continues, it brings new changes and new goals. Our founder, Allie Mazzella, has gone to college and starting this year, has turned over the role of main organizer to me, although she continues to help in so many ways.

After hosting the walk with the help of my friends, I can’t wait to do it again and again. With my friends Abi, Michael, Justin, Evan and Allie along side of me, I will uphold this event and hope to run it for another healthy six years (until I go to college). I find it a humbling experience, something that not everyone has the opportunity to do. Meeting newcomers who have no idea what SMA is and walk to raise awareness of the disease; greeting Walk-n-Roll veterans whose support is greatly appreciated, and finally hearing from SMA families how much they look forward to our walk has increased my confidence that these Walk-n-Rolls do make a difference.

Julia Santiago, 7th Grader
Millburn, NJ
2014 New Jersey Marathon
The 2014 New Jersey Marathon took place on April 27th, 2014 in Oceanport, NJ. Cure SMA and Team Bilodoze SMA teamed up to run in the 2014 NJ Marathon and Half Marathon in memory of Billy Kanehann. The Marathon took runners through diverse neighborhoods including Oceanport, Monmouth Beach, Long Branch, Allenhurst, Asbury Park, Loch Arbour, and Ocean Grove. Thank you to Katherine Kanehann for organizing this great event and raising over $7,000 for Cure SMA!

3rd Annual Best Meatball in Secaucus
The 3rd Annual Best Meatball in Secaucus event was held on April 12th, 2014 at the Immaculate Conception Church Gym in Secaucus, NJ. The Best Meatball in Secaucus event is hosted by local Knights of Columbus Group for Cure SMA and is an annual event. In total, over $4,700 was raised for SMA research! Thank you to Giovanni Recalde and Paulina Recalde for organizing this event!

Mia Spallina’s 8th Birthday Celebration
Mia Spallina’s 8th Birthday Celebration was held on January 10th, 2014 in Manahawkin, NJ. The event was a success, raising $340 for Cure SMA! A special thanks to Mia for gathering donations for her birthday this year! Keep up the great work!

Walk-n-Roll in Honor of Alexus and Zane
On April 26th, 2014, over 40 people came together to celebrate and raise money for the 2nd Annual Walk-n-Roll for Alexus and Zane at Challenge Grove Park in Cherry Hill, NJ. It was a beautiful, sunny day and was perfect for raising money to find a cure for SMA! There was face painting, food, some really great prizes for the raffle, and most of all, Alexus and Zane, SMA type II, enjoyed the day that was to honor them. The walk was held at a boundless park where the kids could enjoy playing with other kids while in their wheelchairs. The Chick-fil-A cow even came for a visit! In total, the event raised $4,490! A big thank you to some of the wonderful people who work at Bayada Home Health Care who came out to help and celebrate with us!

Stephanie Rupin
Mount Laurel, NJ

Wine Tasting Night
On behalf of the Knights of Columbus Council #5427 of Washington Township in Bergen County, NJ, I would like to give Cure SMA this donation in the name of Katie and Mike Van De Loo for your use in the fight against SMA. The donation came from the proceeds of our Wine Tasting Night held on February 8th, 2014. 160 people attended and we raised $3,885 for Cure SMA.

Regards,
George Kuzma
Township Of Washington, NJ

SMA Golf Tournament in Memory of Steven Moyer
On July 18th, Steve Moyer once again hosted his annual SMA Golf Tournament at Blue Ridge Trail Golf Club in Mountain Top, PA in memory of his grandson, Steven, SMA type I. The event raised $3,700 for Cure SMA! Keep up the great work!

Denim Day Fundraiser
Lanes Mill Sunshine is happy to contribute to Cure SMA. The Denim Day Fundraiser, held on March 26th, 2014 in Brick, NJ, raised a total of $225 as a gift in support of Salvatore Morrongiello, SMA type III. Salvatore is the son of a Lanes Mill Elementary School staff member.

Our group recognizes all the wonderful things you do to treat and possibly cure SMA. We hope that this donation will help to continue your dedication to fund cutting edge research and service all families throughout your community.

Sincerely,
Shannon Scott
Lanes Mill Sunshine
Brick, NJ
Concert for a Cure

The 14th Annual Concert for a Cure was held on May 10th, 2014 at the Diablo County Club in Danville, CA and was a tremendous success! This year’s event raised over $165,000 with a unique theme of “Uncork the Cure”. This memorable evening was made especially rewarding for donors and families when Dr. John Day, Stanford Professor for Neurology, Pediatrics, and Pathology, highlighted the exciting advancements in SMA and the ISIS clinical trials.

Guests were deeply moved by Danny McHale’s presentation, SMA type II, on a paper that he wrote for his leadership class and theme of “Do you believe in Miracles”, about the 1980 Winter Olympics US Hockey team. Ariana, SMA type III shared their experience visiting Cytokinetics, where she presented the researchers with her hand drawn picture of her getting out of her wheelchair, walking to a researcher and thanking them. Following Danny and Ariana’s presentation, Danny’s classmates from Los Cerros Middle School presented the McHale and Dindzans families with a check for $2,840. Led by Hannah Doris and Brady Martin, these friends organized lemonade stands and school bake sales so they could make their own contribution as a surprise to the McHale family. The 30 eighth graders and their siblings were a big hit as this marked their fifth year serving at Concert for a Cure selling raffle tickets, running games and encouraging donors to give generously.

Mary and Joe McHale founded the Concert for a Cure in 2001 shortly after the diagnosis of their son, Danny, with SMA type II. Nancy and Andris Dindzans joined the efforts in 2003 when their daughter, Ariana, was diagnosed with SMA type III. Both Danny and Ariana will turn 14 years old this year. Over the years this event has raised over $1.3 million to fund Cure SMA research programs.

Thank you!
The McHale and Dindzans families send out their heartfelt thank you to our friends, families, and the local community for their generosity in their continuous support of SMA families and children everywhere.
BTIG Charity Day
On behalf of the partners and employees of BTIG LLC, we are pleased to present you with this donation, which is the result of our efforts during the 12th BTIG Commissions for Charity Day held on May 13th, 2014. BTIG donates the proceeds from trading commissions earned on Charity Day to charities located around the world. The charitable organization that receives donations from BTIG’s Charity Day is selected by our clients and the celebrity guests who participate at our event.

The fundraiser raised $2,750 for Cure SMA. This donation reflects the combined efforts of our clients, employees, and guests, whose support and enthusiasm generate the successful results of Charity Day. We hope this gift will help your organization and make a difference in the lives of those who are fortunate enough to receive your assistance.

Thank you for your continued efforts to impact the lives of those you assist and to improve the world around us.

Yours Truly,
Scott Kovalik and Steven Starker (co-founders)
San Francisco, CA

KENTUCKY
Kentucky Walk-n-Roll
The Kentucky Walk-n-Roll was held on May 17th, 2014 at Briar Hill Park in Crestwood, KY. The Kentucky Walk-n-Roll was a huge success, raising over $8,400 for Cure SMA! Thank you to Kristen DeLuca for organizing the event. She has put a lot of hard work in to bringing back this walk. All event participants had a fantastic time, and we look forward to seeing them back again next year!

OHIO
Rock the Jet Express Docks for Cure SMA
On June 11th, 2014, Pepsi, Toledo TV station, WNWO-NBC Toledo, and Clear Channel Toledo’s KISS-FM held its 5th annual fundraiser for Cure SMA in Put-in-Bay, OH. This Jet Express fundraiser was in memory of young Will Blumensaadt of Put-in-Bay, OH who had SMA type I, and passed away just short of his first birthday in June 2005. This is one of many fundraisers held all over the nation to create awareness for SMA and help fund a cure. The Jet Express donated 100% of the proceeds to Cure SMA. Pepsi was generous and provided Pepsi products to everyone who bought a ticket on June 11th. The Jet Express Fundraiser raised $2,546 for Cure SMA! Thank you to Todd Blumensaadt for organizing this event! Keep up the great work!

The 16th Flying Pig Marathon
The 16th Flying Pig Marathon was held on May 4th, 2014 in Cincinnati, OH. Some ran the full marathon, while others completed the half marathon, 10K, 5K, or 4-person relay. Thank you to all of our runners for their hard work and fundraising efforts. The event raised over $5,000 for SMA research! Thank you to the Lockwoods for helping organize this event! Also, thank you to Zumbiel for your continued support year over year to the event and this important cause.

Return to Tiffany – Raffle
Cure SMA raffled off a beautiful “Return to Tiffany” Heart Tag Charm Bracelet and used the money to help find a cure for SMA! The fundraiser was held online and organized by Beth Lockwood from the OKI Chapter. In total, the raffle raised over $600 for Cure SMA! Thank you, Beth, for organizing this raffle and helping us find a treatment and cure for SMA!
**Pacific Northwest Chapter**

**Chapter Updates**

In February several families met for lunch and some Valentine’s Day crafts. The kids also decorated sugar cookies after lunch. In May, we had a bowling day. The kids and parents had a great time bowling and playing in the arcade. After bowling, we all went out to lunch. Everyone had lots of fun! We plan several fun outings like this a year and would love to have more families join us. Events coming up include our annual SMA Day with the Mariners, a day at the horseraces and our Walk-n-Roll.

Introducing...@FSMA_PNW via Twitter!

Twitter is a social networking service that answers the question, “What are you doing?” by sending short text messages 140 characters in length, called “tweets”, to your friends, or “followers.”

For our chapter purposes, Twitter will be a great tool to broadcast latest news and blog posts, interact with our community, and enable easy group communication.

Joshua Jones will be running the account on behalf of the chapter. His daughter Lucy has SMA type II, diagnosed at 18 months. She's currently four years old and is an amazing big sister to her little brother Lucas, who is two.

Also keep up with the chapter and like us on Facebook [www.facebook.com/FSMAPNW](http://www.facebook.com/FSMAPNW)

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**Pennsylvania Chapter**

**Lyla Mertz Foundation Fundraisers**

The Lyla Mertz Foundation, Inc. made the following donations to Cure SMA from the 4th Annual Dance Away SMA, which raised over $5,500. The Swing for a Cure Golf Tournament on September 20th, 2014, which raised over $4,000 for Cure SMA. Shelly Brown donated $700 from the Silpada Fundraiser, as well as over $300 from First Niagra Bank from their dress down fundraiser. The Team Lyla t-shirt sales raised over $50! Finally, donations in memory of Lyla Mertz, SMA type I, came out to $200, totaling over $11,000 for Cure SMA! It is our hope to continue with fundraising events until a cure is found!

Sincerely,
Steven and Jennifer Mertz
Lyla Mertz Foundation, Inc.
Kunkletown, PA
Stand Up to SMA

**Stand Up to SMA**

Stand Up to SMA was held on April 25th, 2014 at Yo Fresh Yogurt Café in Jenkintown, PA. The Stand Up to SMA fundraiser had a raffle and silent auction available, with all funds being donated to SMA research. In total, the event raised over $10,000 for Cure SMA! Thank you to Arden Rose Neff for organizing this very successful event!
Cure SMA PA Chapter 11th Annual Cure SMA Walk-n-Roll

The Cure SMA PA Chapter 11th Annual Cure SMA Walk-n-Roll took place on May 18th, 2014 at Lloyde Hall Recreation Center in Philadelphia, PA. Thank you to all of the dedicated sponsors who helped make this day happen! The fun-filled event day included activities for the whole family to enjoy! The event was a huge success, bringing out hundreds of “walkers and rollers” to help fund a cure for SMA! In total, this year’s event raised over $93,000 for Cure SMA!

Thank you to all of the amazing SMA families, participants, and donors for helping make this event possible. A special thank you to Karen McRory-Negrin, Allyson Henkel, Paula Saxton, and Tara Maida for all of your hard work in planning this event year after year! Keep up the amazing work!
Muscles for Mckenna Gala
Another very successful year for the Muscles for Mckenna Gala! This event was held on March 7th, 2014 at The Crystal Tea Room in Philadelphia, PA. The night included food, drinks, live entertainment by The Heartbeats, dancing, and a silent and live auction. This event was flawless, with a red carpet entrance, with photos courtesy of Charlie Clark Photography.

The gala is in honor of Mckenna Ellixson, SMA type III. Mckenna was diagnosed with SMA two years ago. Since she was able to walk, they noticed that Mckenna had difficulty ambulating, fell frequently, and developed a slight tremor in her hands. She was involved in physical therapy, but it was not until she was evaluated at the neuromuscular clinic at CHOP that the diagnosis of SMA was discovered.

This is the second year for Muscles for Mckenna, and it turned out to be very successful! Kellie Keenan planned this gala in her honor of her niece, and raised over $116,000 for Cure SMA! A special thanks to Jim and Amy Ellixson and the committee volunteers for organizing yet another amazing event! Keep up the great work in raising awareness of SMA!
Cupcake Fundraiser in Honor of Quinn Comer
On March 22nd and April 19th, 2014, Juanita Comer held a cupcake sale in honor of her child, Quinn Comer, SMA type III, in Reading, PA. This fundraiser raised $190 for Cure SMA! Thank you to Juanita for all of her hard work in organizing this fundraiser.

Coin Canister and Bracelet Fundraiser
Our family was impacted by spinal muscular atrophy August 2012. Our son Andrew was almost one year old. As a mother my intuition and bond with my son is strong. I knew something wasn't quite right very early on, but I had no idea what. We had never heard of SMA before Andrew’s diagnosis. Facing this is not easy, and I wouldn't wish it on anyone.

Andrew is always smiling and laughing. His newest joys are teasing his sister and handing out new nicknames to the immediate family. “Guess what?” is his new favorite catch phrase and he strives to “do it by myself, all by myself”, Mr. Independent.

Our family worked together by selling bracelets to friends and family in our community, when someone needed a ride, we did not take gas money. We requested that they make a donation to the change jar for Andrew's Hope for a Cure fundraiser. We even had distant relatives in Nevada reach out and buy small amounts of bracelets to distribute out there. In our family, Andrew reminds us to be more thankful for life and not to take it for granted, he is a blessing and we are grateful for what he teaches us. When we are having bad days, he is the sunshine in our gray clouds.

At two years old, he has braved more things then most people would want to face in a life time. He battled RSV and won, he has had many blood draws, hospital visits, and people constantly intruding on his personal space, by giving him check-ups. He's had x-rays, MRI’S and Daily therapy’s and through it all he is still happy.

Our family raised $175. Our efforts were small, this was our first time fundraising. We hope to learn the ropes and go bigger next time. Thank you for the opportunity to help support our SMA Community.

With Love,
The Sonnenbergs
Easton, PA

5K for SMA
The inaugural 5K for SMA was held in Collegeville, PA on June 8th, 2014. The walk was a huge success with an attendance of almost 100 people and raised $1,251 for SMA research.

The walk was organized by Kayla Thompson in honor of her 15 year old cousin, Matthew Freitas, who has SMA type I. The goal of the walk was not only to raise money for research but also to raise awareness for the cause. Friends and family showed their support by gathering on a beautiful, and hot, Sunday morning on the Perkiomen Trail. Water, soft pretzels, homemade cookies, brownies, and cupcakes were provided for walkers when they finished. It turned out to be a fun and social event that supported a great cause!

Although it was only the first year for the 5K, it is expected to be an annual event, hopefully attracting more participants and achieving higher goals each year.

With Love,
The Sonnenbergs
Easton, PA
11th Annual Jacob’s Run Walk-n-Roll to Cure SMA
The 11th Annual Jacob’s Run Walk-n-Roll to Cure SMA was held this year on April 13th, 2014 at S. County Regional Park in Boca Raton, FL. This event became essential to local south Florida residents; the Jacob Isaac Rapoport Foundation celebrated a gathering of friends and families for eleven years in a row. In total, the event raised over $98,600 for Cure SMA.

The Jacob Isaac Rapoport Foundation was founded in memory of Jacob, Shaina and Adi Rapoport’s son and shooting star, who had SMA type I. When Jacob was four months old, Jacob was diagnosed with SMA. Soon after Jacob was diagnosed, Shaina and Adi quickly became connected with Cure SMA, as well as many other SMA families. Jacob passed away at the age of nine months. Through Jacob’s Run, Walk-n-Roll, The Foundation’s main fundraising event, his memory lives on in the hearts of hundreds of family members, friends, colleagues and local SMA community members. Thank you to Shaina and Adi Rapoport for everything you do each year to raise awareness of SMA!

Cosmo Rudd’s Birthday Fundraiser
On April 24th, 2014, Cosmo Rudd’s Birthday Fundraiser took place in Miami Beach, FL. Cosmo Rudd is affected with SMA type II-III. Thank you to Michael Rudd for organizing a fundraiser for Cosmo’s birthday and raising $120 for Cure SMA!
Southern California chapter

Team Cure SMA - Rock ‘n’ Roll San Diego
Team Cure SMA - Rock ‘n’ Roll San Diego was held on June 1st, 2014 in Downtown San Diego, CA. Everyone teamed up to run the San Diego Rock n’ Roll Marathon and Half Marathon to raise money and awareness for team Cure SMA! In total, Team Cure SMA raised over $10,000 to find a treatment and cure for SMA! Thank you, to all that came out to run and support our team!

Temecula Italian Dinner
On April 23rd, 2014, the Temecula Italian Dinner was held at Trattoria Toscana in Temecula, CA. This fundraiser was in memory of Mia Ehorn, SMA type I. The event included a silent auction and a raffle along with lunch and dinner options. In total, the event raised over $2,000 for Cure SMA! Thank you to Letizia Ehorn for organizing this event and raising awareness of SMA!

Rocky Mountain chapter

COLORADO
11th Annual Charity Golf Tournament
The 11th Annual Charity Golf Tournament took place on May 19th, 2014 at Pradera Golf Club in Parker, CO. The event was a huge success, raising $25,755 for Cure SMA! We appreciate the ongoing support of the golfers who come out to play every year and love to welcome new golfers to the tournament!

Thank you to Gillian Faith for organizing this event and for all the hard work you do and raising awareness of SMA! Keep up the great work!

2nd Annual Evening of Hope Beer and Cheese Pairing
On March 13, 2014, more than 80 people gathered together to help make the 2nd Annual Beer and Cheese Pairing a great success for Cure SMA. The event was held at Wash Park Studio in Denver, CO, a quaint venue with great ambiance. Great Divide, a local brewery, provided eight beers for sampling, and the Bistro Boys Catering donated three amazing appetizers and a variety of desserts. For those who participated in the event, donated to silent auction and made monetary donations – THANK YOU! Together we raised over $8,000! We look forward to a third annual event in 2015 and hope to make the event an even bigger success.

Thank you,
Marla Marlow and Joy Spellman

Have you ever thought about starting a chapter?

WE WANT TO HEAR FROM YOU.

CHAPTERS ARE REACHING OUT TO COMMUNITIES ALL ACROSS THE COUNTRY.
Cure SMA currently has over 31 chapters in the United States, but we are looking to expand!
Support your community  |  Fundraise for research  |  Hope for families  |  Begin to make a difference today

Send an email to chapters@curesma.org to receive more information on how to start a chapter in your state.
Carlee’s BIG SMA Shoot Out

On March 29th, 2014, Kelli Beam organized Carlee’s Big SMA Shoot out event at Nashville Gun Club in Nashville, TN. At the age of only two years old, Carlee was diagnosed with SMA type II/III, a condition where her voluntary muscles keep her from walking and doing very typical tasks that we all might take for granted.

This fundraiser was put together to raise awareness and to help find a cure for SMA! In total, Carlee’s Shoot Out raised over $42,000 for Cure SMA! Thank you, Kelli Beam, for organizing this very successful event and raising awareness of SMA!

Bentley’s Smiles for Miles SMA Run, Walk-n-Roll

Bentley’s Smiles for Miles took place on May 10th, 2014 in Pikeville, TN. This event is a timed 5K run with a shorter walking or rolling component as well. The run was held throughout Pikeville, TN and started at the Pikeville main street stage on what would have been Joseph Bentley Bassamore’s 8th birthday.

Bentley passed away at just 18 months old from SMA. Bentley’s Smiles for Miles will be an annual event to raise money and awareness for much-needed SMA research and family support programs. In total, this year’s event raised over $9,000 for Cure SMA! Thank you to Carrie Whitaker for organizing this successful event!

Jellico Rubber Duck Race

The Jellico Rubber Duck Race took place on April 1st, 2014. Friends gathered to race rubber ducks to raise awareness of SMA! The fundraiser raised $500 in total for Cure SMA! Thank you to everyone in the Tennessee Chapter who participated in this fun filled event!
10th Annual Tennessee Chapter Charity Golf Outing

The 10th Annual Tennessee Chapter Charity Golf Outing was held on May 12th, 2014 at Gettysvue Country Club in Knoxville, TN. The golfers and volunteers prepared for an extra hot Tennessee day, but there was lots of excitement in the air. The morning started out with the local radio personalities Doc and Jeff from The Sports Animal promoting the event with a lot of enthusiasm from a live broadcast at Gettysvue Country Club. Jeff even joined the flight of golfers throughout the day.

During the tournament, individuals competed in closest to the pin, longest drive, and the unique and always fun Happy Gilmore putting contest, where golfers have to compete on the putting green and try to get closest to the pin by putting a golf ball with a hockey stick. This game is always a crowd pleaser, but don’t be fooled it is harder then it looks! Prizes were awarded to the three foursomes with the three lowest scores. The tournament however draws some great golfers which provided for a neck and neck competition right until the end.

Scores aside, the day included lunch, range balls, beverages, prizes, great silent auction items, raffle items, and most importantly a really fun day of golf for an important cause. This event is known for their fantastic auction and raffle items that included a full range Weber Grill with all the trimming’s, University of Tennessee swag baskets, and much, much more.

This golf outing would not be possible with out the great work of Louise Ball and Tennessee Chapter President Sarah Boggess who organized yet another successful event for their tenth year of the golf outing, as it resulted in raising over $19,800 for Cure SMA! A huge thank you to all of the volunteers for their hard work!

Compassion

Thanks to the Cure SMA community, no person is ever alone in facing this disease. We offer unconditional support to people affected by SMA and communicate openly and honestly, giving them clear and accurate information.
**Pig Roast Fundraiser**
On June 7th, the 3rd Annual Roast SMA was hosted by Brad and Peggy Shiesley in Lockport, NY in honor of Kale Shiesley, SMA type II. The fundraiser included fishing, four wheeling, frogs and lots of fun for all that attended. It was a beautiful day with a slight breeze. Everyone that attended brought a dish to share and feasted on the roasted pig. A total of $1,160 was donated. The pig and the liquid refreshments were donated by Brad and Peggy.

**Western New York Chapter**

**Joan Hornig Jewelry Show**
The Joan Hornig Jewelry Show fundraiser was held at Neiman Marcus and Julia Schwarz on May 30th, 2014. The jewelry show was hosted by Joan Hornig in Dallas, TX. The fundraiser was a huge hit, raising $9,500 for Cure SMA! Thank you, Joan, for organizing this event and for raising awareness of SMA!

**Change for a Cure - Matthew Wallis**
This gift is in honor of Matthew Wallis, SMA type II. It is given by the Change for a Cure fund at Mark Wallis Dermatology in Longview, TX. Please use the $173 raised to fund research for a cure for SMA.

Sincerely,
The Wallis Family
Longview, TX

**Wisconsin Chapter**

**Strike Out SMA**
The 1st Annual Strike Out SMA bowling event was held on May 17th, 2014 in Wauwatosa, WI. Over 100 people enjoyed an afternoon of bowling and fun! Special thanks to Danyelle Sun, Kate Vogedes and Shannon Kuester for all of their hard work planning the event and recruiting bowlers. This year almost $7,900 was raised for Cure SMA!

**Utah Chapter**

**Jaicelyn Shakespaere - Miss Utah**
Jaicelyn Shakespaere raised awareness of spinal muscular atrophy for the Miss Emery County Platform. She informed everybody of her cousin, Mathis Shakespaere, SMA Angel, and how he had SMA type I. She told everyone about Cure SMA, and asked that people donate to the cause. She raised $521 in total for this fundraiser. A special thank you to Jaicelyn for raising awareness of SMA! Keep up the great work!
Brady Walk - Team Smiles for Elise
My sister, Jenny Sonnenberg and her husband Sam, have a 19-month-old daughter Elise who was diagnosed with spinal muscular atrophy, type I in February 2013. Through all of this, Jenny and Sam have been by Elise’s side, as well as caring for their four-year-old son, Ephraim. She has received so many wonderful things from the Cure SMA which has helped her tremendously.

My company that I work for, Brady Corporation, hosts a “Brady Walk for Community” for the past four years. We raise money for non-profits by walking up to five miles for SMA on June 20th, 2014. Brady Corp matched 100 percent of the total funds raised for each lap. Also during this, they partner with Hunger Task Force, donating $2/can. My team donated 720 cans, and with the donations and Brady match we raised a total of $5,740 for Cure SMA. I am so impressed that we got to this amount. I am so happy and ecstatic that we could give this amount of money to a wonderful charity. This is a cause so special to my heart, that it brings tears to my eyes that I can help out in a huge way with this donation.

Sarah Drake
Hartford, WI

Fundraiser in Honor of Elise Sonnenberg
CH2M HILL staff held a Bake Sale and $5 Hot Dog Lunch on June 18th, 2014, and invited the whole office complex to raise funds for the Wisconsin Chapter of Cure SMA in honor of little Elise Sonnenberg, 21-month-old daughter of Sam and Jenny Sonnenberg with SMA type I. Jenny is a transportation engineer with our firm. Despite soggy weather, we had a great turnout and raised over $1,400.

The Village Inn Dartboard Tournament
The Village Inn in Hewitt, WI hosted a dartboard tournament in February, 2014 to benefit Cure SMA. Over $220 was raised in honor of Waylon and Ellie Budtke who both have SMA type II.

Prescott High School Student Volunteer Group Fundraiser
I am the advisor for the Prescott High School Volunteer Group at Prescott High School in Prescott, WI. As we were planning our events for the year, one of our students suggested doing a project to raise money and awareness for spinal muscular atrophy. Her older sister was born with SMA and her family remembers their loss by supporting Cure SMA.

In February, that student, Emily Gordon, encouraged the group to create and sell valentines at the high school with the proceeds going to Cure SMA. Although the high school is less than 400 students, they managed to sell 100 valentines and raise $100 through this project, which met Emily’s goal.

This fundraiser resulted in $100 raised for Cure SMA. I am always encouraged when students are so touched by an event or organization, that they will spend their time and resources to garner support. This is truly a representation of good leadership and follow-through by our students. I hope it will benefit your organization well.

Sincerely,
Carrie Fisher, Volunteer Group Advisor
Prescott, WI
Make Planning a Fundraiser Easy and Fun

Fundraising Materials:

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

Merchandise

Charcoal Cure SMA T-shirt $20

Purple Cure SMA T-shirt $20

White Cure SMA T-shirt $20

Cure SMA Tote $15

Cure SMA Bracelet $2
Cure SMA,

Please accept this donation of $340.00 on behalf of Mia Spallina in memory of her cousin, Ellie Cisco

Mia Spallina raised this donation as part of her 8th Birthday Celebration. She invited her classmates to a bowling party and requested that instead of gifts and toys that they consider making a donation to Cure SMA to help families like ours. Together with her classmates, they had a fantastic night and raised a nice donation.

Our family was grateful for the amazing time and dedication you gave to my sister, Kari Ann Cisco, and her husband, Land Cisco, of Holbrook, NY. We hope our gesture will assist another family in the future.

Mia’s friends and their families also have a better awareness of SMA, the disease and your organization. I hope this keeps awareness forefront in their minds to assist you in the future.

Thank you for all you do,
Mike, Cindy and Mia Spallina

SE Wisconsin Girl Scout Troop 9052 donated $250 from their annual cookie sale to Cure SMA. The girls also learned about SMA and how it affects the daily life of their troop member Leah.

Dear Kate,

With our Girl Scout Troop 9052 2nd Grade Brownies from Pleasant View in Franklin, Wisconsin cookie earnings we want to help change the world and make a difference. This year we selected charities that affected our troop either directly (Brownie) or in-directly (siblings). Enclosed you will find a donation check for $250.00 in honor of Leah Vogedes. Thank you for taking time out of your busy day to discuss with the girls what SMA is and how it affects you and Leah.

Sincerely,
Karie Dahlen

Hi Cure SMA
Another great SMA event last Saturday!

Attached is a picture of the SMA Coin Drive coordinated by Owen’s friends at East Middle School. The coin drive raised $705.00 for Team Norton!

I will submit a corporate team match for $450.00 for Team Norton via my company’s corporate matching program.

Thanks,
John and Lena Daly

Hi All-
Two little girls in my sister’s neighborhood had a lemonade stand yesterday. They gave me the money and said ($2.00) “This is for Zane.”

Incredibly sweet! Please put into the Sweet Baby Zane fund.

Thank you!
Hillary Schmid

Hey There!
Addie, Molly, Will and our next door neighbor, Ella, made from a lemonade stand for SMA!

Thanks,
Megan Piper

-They wanted me to add that they sold cupcakes and cookies too!“

From left to right: Zach Shaw, Sean Daly, Dan Cusack, Owen Norton, Ryan Sorgi, Paul Macleod.
Absent from photo: Harrison Lee
Holy Cross Lemonade Stand

Two second graders from Holy Cross, Claire Juneau and Emily Chesser, had a lemonade stand during Dover Days in Dover, DE and donated $85 to Cure SMA.

Dear Cure SMA,

Please find enclosed $220 that our granddaughter Arien Pool raised by selling lemonade and cookies while visiting us.

Her cousin, Ayden Trammel, son of Cortney and Matt has SMA Type I.

Thanks,
Charlie and Deb LeBlanc of Kansas

Dear Cure SMA,

Me and my friend Una did a lemonade stand where we sold lemonade, strawberry lemonade, chocolate chip cookies, and necklaces raising money for SMA. So right now with the letter I sent $101 to help SMA. Thanks for helping me and SMA kids and adults.

P.S. Split some with the scientists who made and gave the SMA shot. Also give some to Dr. Chariboga and Roseangel!

Thanks!

By the way I’m pretty sure right now you’re thinking why Jacqueline hid the money in a book/box. I wanted to keep it safe, and yes, thanks to me it didn’t get away.

Bye Bye, hope you enjoy!
Jacqueline Kostyuchenko

Balance

As relentlessly as we pursue a treatment and cure, we are also strategic. We know the fastest way to a future without SMA is to take a comprehensive, unbiased approach to research and maintain a balance of optimism and realism.

Make today a breakthrough.
SHARING PHOTOS

Aiden Marie Bala

Alanis Suarez

Elena Bellavance

Hanna Warfield

Romelo Rios

Eva Kelly

Alivia Tripp

Annie Ford with Tobe

Dylan Chau

Eva Kelly

Elias Toa
SHARING PHOTOS

Gabriella Lobene

Eva Kelly

Eva Kelly

Hanna Warfield

Eve Johnson

Gabriella Lobene

Avyn Jarvis

Evan Vaudry

Diego Mojarro
FAMILY PHOTOS

The Tsolov Family

The Chan Family

The Mojarro Family

The Gitter Family

The Warfield Family

The Perge Family

The McLean Family

The Silva Family

Make today a breakthrough.

cure SMA
Cure SMA has released a brand new Care Series booklet all about The Musculoskeletal (MSK) System. The MSK system includes the muscles and bones in the body and the supporting structures like tendons (tissue that connects muscle to bone) and ligaments (connects bone to another bone). The MSK system includes the arms, legs, and spine.

This booklet is focused on helping children, families and other professionals including nurses, physicians, and therapists understand how SMA can affect the body's MSK system and is meant to serve as a resource rather than a definitive clinical management guideline.

To download the booklet, please visit http://www.curesma.org/support-care/care-publications/

The main topics of this booklet provide recommendations that vary depending on the child's type of SMA. Additional content is included on:

- Contractures on Children with SMA
- Bone Health on Children with SMA
- The Hips on Children with SMA
- The Spine on Children with SMA

This booklet was written by Susan Apkon, MD and other expert medical and family reviewers. Cure SMA cannot thank them enough for their expertise and guidance in developing this new resource.
Nutrition Basics
Fostering health and growth for Spinal Muscular Atrophy.

The topics in this new booklet cover the following important areas of SMA nutrition:

• Nutrition 101 – Mastering the Basics
• Understanding Nutrition for SMA Kids
• Assessing SMA Nutrition
• Managing Nutrition in SMA
• Facing Special Feeding Challenges
• Preventing Undernutrition or Overnutrition

Cure SMA Family Support and Patient Services
This booklet has details on the following Cure SMA programs:

1) Programs For Newly Diagnosed Families:
   • Including our special type I programs such as: Care Packages; Sheep Skin Blankets; wagons.
2) Cure SMA Equipment Pool.
3) Medical Care.
4) Daily Living.
5) Local Support.
6) How to Keep up to Date.
7) The Annual SMA Conference:
   • Including the Cure SMA Newly Diagnosed Conference Program.

Breathing Basics
This new booklet is focused on the critical aspects of respiratory care for children with Spinal Muscular Atrophy. The booklet was authored by Mary Schroth, M.D., a member of the Cure SMA Medical Advisory Council, and a leading expert on respiratory care for SMA patients.

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
• What are special needs of children with SMA type I, type II and type III?
• What respiratory equipment will you need at home?
The Genetics of Spinal Muscular Atrophy

Confused about genes, proteins, DNA and how SMA is diagnosed? Read this helpful pamphlet. It includes definitions, explanations and diagrams from genetics expert Louise Simard, Ph.D. and the Cure SMA Medical Advisory Council.

Caring Choices

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

Topics review the basics of the main care options for newly diagnosed SMA type I:

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

And where you can go for support and guidance.

Understanding Spinal Muscular Atrophy (SMA)

For electronic copies:
Download this booklet from the Cure SMA web site at www.cureSMA.org. Go to the support & care publications section on our website.

For print copies:
Please contact the Cure SMA national office at info@curesma.org.

Disclaimer:
Cure SMA does not, as an organization, support or endorse any particular treatment or therapy. Information contained in this booklet is for informational and educational purposes only. All medical information presented should be discussed with a qualified physician.

If you would like a hard copy mailed to you please email us at info@curesma.org or call 800.886.1762
A Night to Forever Remember
By Kasey Kaler, SMA Type III

As an aspiring journalist every fiber of my being hates writing about myself.

As an aspiring journalist I want to go out and find the news, not be the news.

But, as I sit here tonight, I cannot help but write down this story about a dream coming true. First however, I’ll have to start at the beginning...

Shortly after Clint Myers’ Arizona State Sun Devil Softball squad took home the 2011 Women’s College World Series National Championship, Myers set out to find the future of his program, but instead found me.

Looking back, Coach Myers recruited me; he sold me on the type of family that ASU Softball strived to be and told me everything I could accomplish while being affiliated.

While keeping score for his son Corey’s team – also the team that my younger sister played for – Coach Myers talked of his goals of the future that would include me and I instantly wanted in.

Coach Myers had this unique ability to take care of everyone that had been affiliated with his teams at any point, and I was no exception.

He believed that no matter what, family came first and to him, family was every person affiliated with ASU Softball.

And that’s exactly what ASU Softball was and still is, even with the man who started it all at the helm for Auburn.

Thursday, February 20 was special; it was different from last year’s inaugural Spinal Muscular Atrophy (SMA) Awareness game.

The previous day the girls and I had sat around discussing SMA. SMA is a neuromuscular disease that destroys the neurons that control voluntary muscles in the body that are used for walking, head and neck control and swallowing amongst other things.

SMA is broken down into four different types based on the number of milestones that one reaches.

Personally I have Type III. I walked until I was eleven years old and ten years later I am still able to live a somewhat normal life with very minimal assistance required.

Because I am bound to a power wheelchair, I am often underestimated in terms of productivity but that was hardly the case at Farrington Stadium.

Whether you know it or not this team is very difficult to integrate oneself into. But, once you’re in, they take care of you in a way that only a family would. Differences aside, every person synonymous with Arizona State Softball has a deep-seeded love for one another that, in my opinion, defines the word team. It is something that has been instilled from day one: that they will take care of each other on and off the field.

They’re a very close-knit group, in that they struggle to let strangers in, and I was sort of thrown at them by then-Head Coach Myers.

Now, I wouldn’t trade these relationships for anything. Each and every one of them is going to do something truly great with their lives and I can only hope to be around when they do.

I’m going to grab a quote used by the one and only Bailey Wigness at this juncture:

Because it’s never been about the game…it’s about all the people in your life that make playing the game worth it. @kasey_kaler we love you! A true sun devil, a true friend, and I am blessed to have you in my life.
Midway through year three of volunteering this quote could not ring any more true to me, volunteering for ASU Softball was never about anything other than the people.

This year, the girls have clung to the people they know best and that has included me.

I could sit here and throw out how I would have loved the opportunity to play college softball alongside these amazing athletes but the reality is, all I really wanted was to be a part of a team ... and now I am.

24 girls sported bracelets (the red and black that is pictured above) in support of SMA and myself that night and watched on as I (attempted to) throw out the first pitch to Jenn Soria – also the night’s hero after a pinch-hit game-ending RBI double.

It was an unforgettable night for me and my entire family and something that I hope everyone else involved will remember for a long time after we have all since moved on.

When you talk about special teams, this team is nothing short of that, on and off the field. I can only hope to bring the happiness to them that they have brought to me in my short time with them.

There is no right way for me to express my gratitude towards them, but something wonderful happened in Tempe the other night all thanks to 24 magnificent human beings who happen to also play softball.

If you want to talk about love, below are some great examples

Kasey Kaler is The true definition of a Sun Devil. Such a beautiful person inside and out and an inspiration to me. Thank you for everything you do for us and I’m so happy last nights game was dedicated to you! Love you! #CureSMA ♥ ♥

Supporting Spinal Muscular Atrophy! thank you kasey for everything you do for us, your my inspiration! so thankful for playing last nights game for you♥️ #CureSMA #loveyou #asufamily

Truly one of the strongest girls I know and an inspiration to me! Love you @kasey_kaler ♥️ #CureSMA #KDKSmashSMA

Compassion

Thanks to the Cure SMA community, no person is ever alone in facing this disease. We offer unconditional support to people affected by SMA and communicate openly and honestly, giving them clear and accurate information.
This summer, I went to the Cure SMA conference in Washington, DC. While I was there, I went to the "Kids Talk It Out" session for kids with SMA who are 10 years old and up. This session was led by Dr. Al, a child psychologist who has a son with SMA, and Miss Angela, a teacher who also has SMA.

In this session we learned how to solve our problems with SMA, like what to say when people ask you why you're in a wheelchair. We also shared different sports and activities we were involved in and how we can help out in our community all while playing fun games.

I love the awesome games we play, but what I really love about this session is you can express your feelings about a problem or something you would like to try. Since all the people in the room have SMA, they know how you feel and maybe some of them are having the same issue, and the teachers can offer advice to you. It is also cool to hear about other activities that kids with SMA are doing, like waterskiing, horseback riding, basketball, and power soccer just to name a few.

All and all, I learned to embrace my differences with SMA and realize that I'm one of a kind. Besides, everyone knows that SMA doesn't really mean Spinal Muscular Atrophy, but “So Much Awesomeness!”

**Save the date!**

**2015 Annual SMA Conference**  Kansas City, MO

Thursday, June 18th - Sunday, June 21st, 2015