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.

On the Cover: Evan Vaudry, SMA Type II
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.

In February 1994, Barbara and Gene Trainor lost their five-month-old daughter Erin to SMA type I. Throughout the past 20 years, the Trainor family, friends, and colleagues have raised funds for Cure SMA through annual events such as the Chesapeake Crab Feast and the Chesapeake Charity Golf Classic. In addition, they have provided comfort to numerous families by forming the Chesapeake Chapter in Baltimore, Maryland.

This led them to establish the Erin Trainor Memorial Fund, to honor their daughter’s memory by bringing newly diagnosed families together during the Annual SMA Conference and strengthening the SMA community. The Trainors have set an ambitious goal: to raise $1 million through the Erin Trainor Memorial Fund.

Thanks to the dedication of the Trainors, and the generosity of our donors, they recently surpassed the 2/3rd mark on the way to their goal, with over $667,000 raised.

The Erin Trainor Memorial Fund exists to provide scholarships for newly diagnosed SMA families to attend The Annual SMA Conference. Not only will the ETMF allow families the opportunity to participate in this three-day event, but it will also provide each with the opportunity to gather critical care and daily living information from experienced health care professionals as well as other families living with a similar diagnosis.

“We truly see Cure SMA as a gift of hope. This gift has blossomed over the years through supporters like you,” say Gene and Barb Trainor.
Every year, Cure SMA sponsors a conference to bring together the leading SMA researchers, clinicians, and families living with SMA. Cure SMA has been hosting the Annual SMA Conference since 1989. The weekend is filled with a wide variety of workshops, keynote sessions with leading researchers, a family-friendly poster session, a memorable children’s program, a family fun fest and carnival, teen and adult social activities, a dance party, and many opportunities to connect and interact with families and receive first-hand updates from the researchers.

Every year we look forward to reuniting as a community at this conference and showing our support for others. As always, the family conference and the researcher meeting run alongside each other. This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it. The interactions between the researchers and families at this one conference are extremely special. The annual conference also provides the children an opportunity to make new friends and have a great time. We are expecting another great turnout of over 1,100 attendees.

The Westin Kansas City at Crown Center has been carefully chosen as the 2015 destination for the Annual SMA Conference. The hotel is encompassed by Hallmark’s Crown Center, which offers 85 acres of shops, restaurants, and attractions. Each of the 724 guest rooms is equipped with a mini-fridge, coffee maker and 37” flat panel TV along with other modern amenities. The hotel offers a variety of restaurants along with 24-hour room service. For your convenience there is complimentary wireless Internet in the lobby and public areas along with premium internet available in the guest rooms. Bring your suit along to enjoy the outdoor heated swimming pool.

You must first register for the conference before you can reserve your discounted hotel room rate of $135 per night.

Known as the City of Fountains and home to great jazz music and barbeque, Kansas City is an urban town with Midwestern charm, there are many things to do for all ages. The Westin Kansas City at Crown Center is in the heart of it all. The Crown Center’s various shops, restaurants and theatres encircle the hotel. Some of the many attractions include: The Science City at Union Station, The LEGOLAND® Discovery Center, SEA LIFE Aquarium, Kemper Museum of Contemporary Art, The Power and Light District and many more. Come on along and enjoy the sites and sounds of Kansas City.

To register for the Annual SMA Conference, please visit www.cureSMA.org.
NEWLY DIAGNOSED CONFERENCE PROGRAM

Thanks to the generous funding provided by the Erin Trainor Memorial Fund, Cure SMA covers the registration fees for the Annual SMA Conference for all families newly diagnosed since the last conference. The mission of the Erin Trainor Memorial Fund is to generate substantial funds to be able to provide conference scholarships, allowing newly diagnosed SMA families/individuals the opportunity to attend the Annual SMA Conference. Our goal is to allow as many newly diagnosed SMA families as possible to attend and experience the benefits of this amazing conference. The meeting gives families the opportunity to gather critical care and daily living information early after diagnosis, to learn directly from experienced SMA physicians, and to network with other families. This program is automatically offered to all newly diagnosed families that contact Cure SMA.

For more information, please email familysupport@curesma.org

EXHIBITOR OPPORTUNITIES

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

For more information, please email exhibitor@curesma.org

SPONSORSHIP OPPORTUNITIES

The Annual SMA Conference does not happen without a tremendous amount of work and support. Thank you for your consideration and for making a difference in SMA research and for those affected by SMA. Sponsorship is a way of establishing a deeper association and positive brand awareness with the SMA families and researchers.

For more information, please email sponsorship@curesma.org or call 800.866.1762

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

SMA CONFERENCE GOALS

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

Make today a breakthrough.
## 2015 Annual SMA Conference

**WESTIN KANSAS CITY AT CROWN CENTER, KANSAS CITY, MO • JUNE 18– JUNE 21, 2015**

### Wednesday, June 17th

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<tr>
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### Thursday, June 18th

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<td>Registration Open for all Conference Attendees</td>
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<tr>
<td>12:00pm – 4:00pm</td>
<td>Newly Diagnosed Program (For Newly Diagnosed Families Only)</td>
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<td>Newly Diagnosed Meet &amp; Mingle</td>
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<td>Session A – Type I</td>
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<td>Session B – Type II &amp; Type III</td>
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<td>Session C - Grieving</td>
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<tr>
<td>6:30pm – 8:30pm</td>
<td>Meet and Greet/Family Fun Fest for all conference attendees</td>
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<td>7:30pm – 10:00pm</td>
<td>Adults with SMA Social</td>
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<td>Teen Social</td>
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<td>7:30am – 5:00pm</td>
<td>Exhibitors</td>
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<tr>
<td>7:30am – 8:45am</td>
<td>Continental Breakfast</td>
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<td>9:00am – 10:15am</td>
<td>General Session</td>
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<td>10:30am – 12:30pm</td>
<td>Workshop Session #1</td>
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<td>12:30pm – 2:00pm</td>
<td>Lunch Break</td>
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<td>2:00pm – 3:30pm</td>
<td>Workshop Session #2</td>
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<td>6:30pm - 9:30pm</td>
<td>Researcher Poster Session and Dance Party</td>
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### Saturday, June 20th

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<td>7:30am – 5:15pm</td>
<td>Exhibitors</td>
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<td>7:30am – 9:00am</td>
<td>Continental Breakfast</td>
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<td>9:00am – 10:30am</td>
<td>Workshop Session # 3</td>
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<td>10:45am – 12:15pm</td>
<td>Workshop Session # 4</td>
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<td>1:30pm – 3:00pm</td>
<td>Workshop Session # 5</td>
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<td>3:15pm – 5:15pm</td>
<td>General Session /Researcher Q &amp; A</td>
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<td>6:45pm – 9:30pm</td>
<td>PJ Party/Movie Night</td>
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### Sunday, June 21st

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<td>7:30am – 9:15am</td>
<td>Continental Breakfast</td>
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<td>9:30am – 11:30am</td>
<td>Closing General Session – It’s a Wonderful Life Panel</td>
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Cure SMA Hosts SMA Researcher Meeting this June

The Annual SMA Conference consists of two separate events that run in parallel. The first is focused on families affected by SMA, who gather for workshops, general sessions, networking, and fun events such as a dance party, movie night, and more. The goal is to lend each other support and strength while learning about the latest advances in research and care.

The SMA Researcher Meeting

The second event is the SMA Researcher Meeting, the largest research meeting in the world specifically focused on SMA. During this event, researchers from industry and academia meet to create open communication of early, unpublished scientific data, accelerating the pace of research.

The meeting also furthers research by building productive collaborations—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.

These collaborations have produced many tangible results. For example, the SMA Researcher Meeting has been the site of multiple advisory meetings for the drug ISIS-SMNRx. This drug is now in Phase 3 clinical trials, the first drug developed to treat the underlying cause of SMA to advance to late-stage clinical trials.

Other collaborations have included partnerships between academic researchers and industry researchers.

Uniting the SMA Community

In addition to providing leadership in the SMA research field, holding the SMA Researcher Meeting alongside the family conference allows us to offer unique opportunities for families and researchers to come together to learn from and collaborate with each other.

This includes:

- A meet-and-greet event featuring the much loved “researcher relay race”
- A family-friendly research poster session, during which researchers present their findings in a way that is accessible to families and other lay audiences
- A panel discussion and Q&A with industry experts at the closing keynote session of each conference

Sharing New Discoveries

While the SMA Researcher Meeting attendance is for professional scientists only, we also publish summaries of the most exciting new discoveries after each year’s meeting. Typically, these are posted on our news site as well as in print via an issue of Compass, our research publication.

If you want to get on the mailing list for Compass, send us an email at info@curesma.org.
Cure SMA Provides Record Number of Resources to Families Worldwide

Along with funding spinal muscular atrophy research, Cure SMA provides thousands of families with vital family support and resources to help them live active, engaged and hopeful lives. Cure SMA is a resource for stable, unbiased support, offering understanding and resources when they are most needed.

In just the past year:

- We were contacted by over 400 newly diagnosed SMA families.
- We shipped nearly 1,300 packets of information to families affected by SMA.
- We sent 150 CDs, filled with SMA information, to families in other countries across the globe.
- We sent almost 2,500 support items to SMA families. Some of these include Newly Diagnosed Care Packages, wagons, and information for both families and healthcare providers.
- We provided 300 pieces of medical equipment to SMA families across the United States.

The Mission of Family Support

Our family support and patient care departments work with families in these and many other ways, such as:

- Supplying SMA information to all newly diagnosed families who contact us, helping each to understand and manage the disease better.
- Sending care packages of toys that have been recommended for children affected by SMA.
- Housing a shared medical equipment pool, available to families at no cost.
- Hosting the Annual SMA Conference, the world’s largest gathering of families, medical professionals, and leading researchers from around the world.
- Offering local community support through our vast volunteer network.
- Publishing care series booklets, Directions (our biannual family newsletter), and other important publications.
- Providing personal support via telephone or email.

Families affected by SMA turn to Cure SMA for information, guidance, encouragement, and most importantly, hope. We are committed to providing families the support they need for today, while we fund the research that will lead us forward to a treatment and a cure for SMA.

To contact the family support department, please email familysupport@curesma.org.

CURE SMA RECEIVES GENEROUS DONATION FROM JADON’S HOPE FOUNDATION

Thanks to Jadon’s Hope Foundation, the Cure SMA equipment pool received 10 new Special Tomato Feeder Seats!

After learning that there was a waiting list for feeder seats in the Cure SMA equipment pool, Kristin Burks, co-founder and president of Jadon’s Hope Foundation, reached out to her board members and got approval to purchase new feeder seats for the Cure SMA equipment pool.

Thanks to this generous donation all of the families who were on the waiting list have now received a feeder seat.

Thank you to Kristin and Tony Burks, and the entire board of Jadon’s Hope Foundation for their continued support of Cure SMA. As a result of their generous donation, the Cure SMA equipment pool continues to grow, allowing us to provide much needed equipment to SMA families across the country.
Bugenske Family Donates $17,000 to Cure SMA Equipment Pool

On Saturday, October 25, 2014, Leo’s Pride and over 600 participants ran and walked a 5K or 1 mile in support of Leo Bugenske and Cure SMA. In addition to the race, they also celebrated Leo’s first birthday with a beautiful cake.

From the proceeds, the Bugenskes donated $17,000 to purchase eight EasyS Strollers for the Cure SMA equipment pool. The Bugenske family was inspired by their own experience of receiving an EasyS Stroller from Cure SMA. The stroller keeps Leo comfortable on the go, and also holds his medical equipment.

“We started with local parks and familiar places, and now Leo has been all over South Carolina. He went to the beach twice this summer, and went to the mountains this fall. He is truly living. He is getting out and appreciating all the beauty that exists. This all began because of the stroller provided by Cure SMA,” his family said.

In addition to the hundreds who came out for the event, a generous anonymous donor matched each dollar raised. “We are so blessed to have such amazing support from our family, friends and community. They have all come together and been so wonderful to us. We feel their love for Leo and for us.

“Leo has changed many lives. He is such a blessing and we are so thankful to have a support network that not only recognizes what a blessing he is, but actively passes the light he shines to others. They have made this donation possible and we are so grateful for that.”

Our thanks to Meredith, Adam, and Leo Bugenske, and all those who supported this event.

Cure SMA is so grateful to have received a donation of handmade baby quilts donated by Becky Shelton for our Newly Diagnosed Care Package Program! These baby quilts will be put into the Type I Care Packages, in honor of her great-grandson Greyson Braune. Thank you Becky for these wonderful additions to our care packages!

Kinetic Sand Donated for Care Packages

We are incredibly grateful to have received a large shipment of Kinetic Sand Activity Kits to be included in the Newly Diagnosed Type II Care Packages in honor of Jayce’s second birthday!

Jayce’s parents, Jenna Gomez and Corey Clark, received a care package when they contacted Cure SMA after Jayce’s diagnosis. Since receiving the care package, Jayce discovered kinetic sand and absolutely loves it! “It is so easy for him to grab, and he loves how it falls through his fingers when he squeezes. It’s one of the few things that really get his hands moving,” said Jenna.

Wanting to give back to the care package program, Jenna and Corey sent out invitations to all their family and friends to attend Jayce’s second birthday party and asked every guest to bring a box of kinetic sand so they could donate them to Cure SMA. Thanks to all their generous guests, they ended up collecting almost 50 boxes of kinetic sand.

We would like to extend our sincerest thanks to Jenna Gomez and Corey Clark, as well as all of their generous guests who have made such a wonderful donation. Happy birthday, Jayce!
Hi Cure SMA,

Levi loves the sheep skin blanket! He has me lay it out on the floor so he can lay on it! I am so grateful for everyone at Cure SMA! You guys have been so wonderful and so helpful through all of this! Thank you so much for all that you guys do!

Cassie Eastman of Nevada

Dear Cure SMA,

We just wanted to thank everyone for all the help we are getting. We truly appreciate it. It’s been really hard and overwhelming since we noticed our son not moving his arm or legs and after getting the diagnosis that just made it worse. The only good thing about this is that we now know that we are not alone and that Cure SMA is giving us so much help. It really gave our mind and hearts some peace to know that there are people that are willing to help us, especially our son, and even though we can’t do much to fix it we can do our best to make him happy and comfortable. We truly appreciate all the families that donated all the stuff to help our little boy feel comfortable and for the toys, and for the helpful hints. It really means a lot to us. Thank you..

Thank you once again,
Laura Lopez of Illinois

Thanks Cure SMA.
I think Jace liked the items in his SMA package even better than his Christmas toys!

The packet and website have been extremely useful and have turned us on to many events and resources already.

Thank you so much.

Sincerely,
Ben Dorer of Michigan

Cure SMA,

We received both today. Just in time! My husband is installing the car bed as we are heading to Indianapolis for a sleep study tonight, and Isaac loves his new toys! Especially the feather, pinwheel, and light up aquarium. My husband and I were both moved to tears by all of the items. The little tags with the names of the other SMA babies just touched our hearts and made us feel not so alone. We are not the first ones to walk this road, but the journey is easier because of people like them and you. The liquid pillow is so perfect too!

God bless,
The Kimmels of Indiana

Cure SMA,

Thank you so much for the information, amazing care package, and use of the shower chair and base! I cannot tell you how much we appreciate it. We will happily return the favor to others in the future!

Sincerely,
Kelsey and the Lucardie family (Noah) of Ohio
Dear Cure SMA,

We received our care package and are very delighted. Thank you so much for the generous gifts. It was like Christmas all over again! It was so touching to see all of the families that have donated for this cause & that have already gone through what we are facing. We appreciate all of the support.

Thank you,

Nawai Irie & Family of Hawaii

Hi Cure SMA,

Thank you so much for sending this all to us. It has so many special and sweet things inside. We were both very honored and amazed by how thoughtful it was. Knowing each item was donated in honor of someone affected by SMA. It feels nice to be able to connect and hear other families’ stories. We are grateful for whatever services you can provide to help us get started in transitioning. We too would eventually like to get involved in spreading awareness about SMA. Thank you again!

Tarrah Osmulski of Virginia

Helpful Hints

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

SMA Support System is a Family Support Group, ran through Facebook for individuals affected by SMA, including Parents, Researchers, Doctors, Therapists & Nurses, Equipment & Care Specialists, Individuals living with SMA, Newly Diagnosed Families & MORE! All of Our Members are there to Connect on a Variety of Topics Surrounding Spinal Muscular Atrophy, from basic research, to clinical trial opportunities, to palliative care choices, medical inquiries, advice requests from the community as a whole and many more topics. We have grown from an original 200 members to Over 3100+ Members in less than 2 years, have Members in over 10 Countries, and have coordinated family care connection throughout the SMA Community Worldwide!

Have Questions? New to SMA? Ask to Become a Member Today!

SMA Support System
https://www.facebook.com/groups/386426768116105/

-Admin, Sierra Kulas, Port Crane, NY

Thank you

We wanted to take a moment to recognize Rosemary Francis, who has been volunteering with Cure SMA for over three and a half years! Thank you so much for everything you do, Rosemary! You have helped our organization in so many ways, and we all really appreciate your hard work!
“He will live until about 4 years old.”

Our doctor told us this when our son, Alex, was officially/unofficially diagnosed with Spinal Muscular Atrophy (SMA). Officially, because the doctor had experience with children born with SMA; unofficially, because the blood work hadn’t yet come back confirming it. In a weird way, we thought ourselves lucky because the websites and local doctors knew that the majority of children born with SMA type I die before their second birthday. We thought: We just doubled that!

SMA is a form of Muscular Dystrophy. It affects the way a person crawls, sits, walks, breathes and swallows. About one in 50 people are genetic carriers. SMA can affect any race or gender. As time goes on, a child diagnosed with SMA will get weaker and lose any and all muscle function.

What would I tell myself at the time of diagnosis that I now know?

Doctors don’t know everything.

Doctors cannot possibly know when someone will die because they often base their assumptions on what has happened in the past — not with the current research and knowledge that may still be forming.

Parents of children with the same diagnoses as your child know a lot. Ask them everything you can — no question is a stupid question when it comes to your child.

Children are fighters and have a way of proving us wrong.

Have faith.

Alex will be 12 years old in February. He’s alive, goes to a regular school, has friends, loves everyone he meets and is a happy, curious, lovable kid.

Don’t get me wrong, his life (or ours for that matter) has not been an easy one. He’s been hospitalized three times but each time has been shorter than the last. He’s had ear infections, colds lasting up to a month, pneumonia, paraflu, strep throat, C. diff, bacterial pneumonia, staph infection, a collapsed left lung, a fractured right femur, pneumonia again, sinus infection, back surgery and a fractured left femur.

We know what to do when he gets sick and how to treat him at home. We have doctors in our corner who listen to us (and him) when needed. We also know things can change at any time and we must be prepared. We need to keep germs away because they’re the enemy. It’s not so much getting sick, it’s the inability for his weak body to fight the germs. We try not to keep him in a bubble. He needs to experience life.

We were initially told to stay away from the Internet for information. Some information you receive is correct and some is not. Some can be dark and scary. If I had to give advice to a parent of a newly diagnosed child with special needs, it would be this:

• Have doctors in your corner who are willing to listen to you and your child — and if they don’t have the answers you need, know they will find it for you. They must be able to reach out to other doctors who specialize in the care you’re looking for. They must realize that they don’t know everything. If you don’t have a doctor where you live, find one elsewhere who knows what they’re talking about.

• Connect yourself with parents who are also on the same journey as you and ask them questions — but know that even though the diagnosis is the same, they may do and say things you might not be comfortable with. You need to weigh your options with your beliefs and the needs of your family. Unfortunately, I’ve come across quite a few parents who are angry and they may lose sight of what is important. Everyone deals with the stress of a child with special needs in their own special way. If you don’t understand where they’re coming from, you can respect that and move on.

• I’d suggest seriously considering enrolling your child in a trial study if it’s available. This is how the researchers find cures and/or treatments. This is how you can help future children and their families who may receive the devastating news. You may see your child as a guinea pig, but if you don’t investigate the possibilities, who will?

• Have a little faith – chose your path to help you get through this. Miracles do happen.

• Love your child with all your heart and soul, and let them feel it.

• Try and have some time for yourself, your significant other and other children if you can. You don’t even have to leave the house. See if you can get someone to help you out just to give you a little reprieve.

• If you’re on information overload, take a step back and regroup.

• We do the best we can with what we know and what will get us through this journey.
Why I Want Your Children to Ask Questions About My Disability

By Alyssa Silva, this post originally appeared on Living With Spinal Muscular Atrophy, http://alyssaksilva.com/

There was this time a few years back now, when two of my friends and I went out for ice cream. Just like any other summer night in a small town, the place was packed, so I told them I’d go park myself at a picnic table before anyone else grabbed it. On my way over to the table, I saw a little girl crying. No, actually, she was wailing in her mother’s arms. Just as I was approaching the table, which unfortunately was right next to this less than cheerful little girl, she screamed, “Mommy! Ugly!” and pointed... Right. At Me. And just like that, they quickly gathered their belongings and headed straight for their car.

I couldn’t believe it. There I was, minding my own business and anxiously awaiting my delicious milkshake that was swirling in the blender, and this kid just blurted out “ugly” in the midst of her hysterics. But the part that hit me the hardest was not her reaction. It was her parents’ reactions. Although this situation was a bit extreme and only happened once (kids don't always say the truth, right?), I’ve always dealt with incessant stares and inquisitive children whose parents don’t always handle these encounters appropriately. It’s time we change that.

Let’s begin by pointing out the obvious. I’m different and look different. I find it completely normal that a child gets curious. In fact, I believe their curiosity is a great thing because without being curious and never asking, they’ll never know. With that being said, when a parent hurries their child along when they say something along the lines of, “Why is she like that?” nothing good will come from it because nothing will be solved. Unanswered questions can often lead to misconceptions, and that’s where we fall into problems. Contrary to silly metaphors, curiosity never actually killed the cat.

Although there are people who, one might argue, don’t handle these types of situations well, there are people who know exactly what to say to their little ones and, over the years I, too, have learned the appropriate way to handle when children ask questions. If you ever find yourself in a situation aforementioned, here are a few helpful hints to remember.

1. **If you see us interacting and conversing with our peers out in public, chances are we will be happy to answer your child’s questions.** I’m happy to give the “little kid version” of my disability, which is that my legs don’t work, and I have to use this pretty cool chair to get around. I’ve learned that it works really well, and there’s a great possibility that the child will then begin focusing on my chair and not so much on me.

2. **Sometimes, a child may say something directed towards a person with a disability that embarrasses a parent, but it’s important to not act irrationally.** For example, “Why does she look like that?” or “Why is she so little?” or, my least favorite, “ugly” are just a few phrases I’ve heard in the past. Instead of getting upset, take a quick second to answer your child. Even if you don’t know the person’s story, a simple “That’s how she was born” will suffice. Going back to my first tip, if your child continues to ask questions, why not approach us and ask? I promise we don’t bite.

3. **Kids learn by example, so be aware of your actions.** Negative reactions will ultimately result in negative outcomes. However, being responsive in a positive manner will teach children that it’s OK to be interested in these types of matters. Encouraging children to ask questions will eventually teach them to be more accepting and less prejudiced towards people with disabilities.

4. **Remember that, on the inside, we are just like everyone else!** Ask questions, and don’t be afraid to tell us what’s on your child’s mind. Asking us questions is just like having small talk with the person waiting behind you in line. We will share what we want to share in hopes of enlightening your child. Most of us want to help in better educating the world about disability and the fact that our disabilities don’t define who we are.

I was once out to lunch when a mother approached me with her son. She said, “My son has been asking me all sorts of questions about you, and I told him he can ask you personally. Do you mind?” I was ecstatic. Even though the boy quickly became quiet and shy, I was able to give a little bit of insight on my disability, and his questions were answered. That’s one less child who will be feeling perplexed the next time a four-wheeled machine whizzes by him out in public. I can only hope more people will do the same in the future to help change the way disabilities are viewed in a child’s eyes.
Looking for Room to Grow

By Jeff Auter, this article originally appeared in the February 2015 issue of Mobility Management.

Traditionally, children with spinal muscular atrophy (SMA) have been issued strollers with full recline capability as a mobility option — strollers such as the EaSyS or Kimba with full recline and full leg extension capability. These, however, do not have much growth potential for children past 7 years of age, after which these children have to use reclining manual wheelchairs as their only option.

In February 2014, Addison, age 8 ½, came into National Seating & Mobility’s Milwaukee office for an evaluation to replace her current EaSyS stroller. She met with Sean Auter and me to determine what could be done to grow her existing stroller base until a new wheelchair option could be decided upon. She was 55” in height and weighed 50 lbs. The parents had done a very creative job in extending the EaSyS support platform using PVC pipe tubing to accommodate Addison’s height. When Addison was in public, her nurse walked in front of her, as her head stuck out 23” longer than the stroller’s front casters. The nurse did this to prevent people from walking into Addison during outings.

DEFINING A SUCCESSFUL SYSTEM

Her parents reported that they did not wish to pursue getting a reclining wheelchair, as the frame would be too tippy backwards, and the chair would be too long to fit into transport vans or attend school. Also, Addison would have no way to come up in space to see her surroundings, as her hip angle did not match that of a reclining wheelchair. In other words, she could not sit in an adult wheelchair and have only her backrest be brought upright.

Addison also required mounting places for her respiratory and suction equipment. There was no real place to attach a transit tie-down system onto a conventional reclining wheelchair. There was really no way to make enough space on an existing reclining wheelchair to accommodate her equipment.

At evaluation in February, Sean and I took body measurements and determined Addison needed a support system at least 57” in length that allowed her to lie down totally flat, but bend slightly at her hips and knees. This system had to be no more than 26” wide to fit onto the stairlift at school, which she attends daily. The system needed to have adult push handles that could angle-adjust for the heights of the different people pushing while Addison was lying completely flat. Imagine a hospital gurney, but with push handles that rose above it — like those you would see on a tilt-in-space wheelchair like the Ki Mobility Focus CR or Quickie IRIS.

The new system also had to allow for growth in length and changeable positions of her hip and knees. Several years ago, Sean and I would have gone into our back warehouse where our fabrication shop is and modified a wheelchair frame ourselves to fit someone like Addison and the requirements she has. We would have done welding and custom fabrications to hand-make a system for Addison. But in today’s world, working with a manufacturer makes things serviceable and allows duplication in the future on a worldwide level. We drew up a couple of stick figures showing Addison from a top and a side view. We identified the angles that we needed to be adjustable and also gave the dimensions for this to be created. Most manufacturers told us that a conventional reclining wheelchair was the only option, but they did have limited customization, which came at great cost.

NEW CONFIGURATION POSSIBILITIES

In came Tom Whelan and Ki Mobility. They were excited to work with me on this, and together, we felt their Ki Focus rotational chair would be an excellent possible platform for a totally new seating system.

Tom came up with the idea of using their angle-adjustable links at the front and back of the frame and hip/knee/
foot with telescoping tubes to allow for lower-extremity growth. My idea was to take the Focus and turn it backward so when the unit is tilted, it actually raises the entire platform forward into a more upright position. This allows Addison to view her entire world as much as she can tolerate.

Push handles were placed on the front of the chair as well as the back, which allowed the chair to be pushed from either end. Both sets of handles also were designed with angle adjustability in mind. By doing so, we save on the overall length of the chair. When not pushing from the front, those handles are folded down. This saves on storage space, and also prevents Addison’s head and feet from accidental impact. This took care of the frame.

What we had to do next was address her lying-down position and supportive surfaces, but keep in mind relative space to put her respiratory equipment and other supplies. Tom suggested we work with the Pete Cionitti from Therafin Corp. Sean made templates out of cardboard from the measurements we took and had them sent to Therafin. Pete and the Therafin team were able to quote us foam cushions and storage platforms to attach to the frame of the chair. We had the custom Ki Focus shipped to Therafin for installation of the positioning components.

**READY FOR THE FUTURE**

Once all of this work was completed, the unit arrived back at National Seating in Milwaukee. Sean had Addison come in and adjusted the center of gravity of the frame, allowing it to fit into the family’s van and maximize the stability of the unit.

We then adjusted the seating system. There are two armrests that fold down with Therafin lateral pad hardware, allowing the chair to be 26” wide at most to fit onto the school lift. They fold back up to allow Addison a comfortable position for her arms. The hip angle is adjustable, which compensates for hip and knee flexion contractures. The system has lateral pads along her thighs to allow us to control position of her lower extremities; these are telescoping to allow for growth. Only a new mattress will be needed in the future to accommodate for growth, as the frame does extend and cover for growth.

As you can see, there is plenty of room under the platform where Addison lies to allow for her storage of equipment, and the area is easy to access as well. The chair easily fits in the family vehicle, as well as at home and school. She is totally protected, comfortable and can safely explore the world.

I have been working as an RTS for 40 years and typically had to make things like this myself. It is great to know that companies like Ki Mobility and Therafin are there to assist us as ATPs to provide our patients with these unique solutions in this challenging world, especially when it comes to getting things like this funded. Addison has been using this new system for the past six months with great success, not only for her but for her family and caregivers as well. Seeing the smiling faces of the family and smiling faces of children like Addison has kept me continually involved over the past 40 years.
The Disability Visibility Project recently came across the work of Vicki Jurney-Taylor, writer, disability advocate and accessibility consultant. She is spearheading an online campaign requesting Congress to require the airline industry to provide spaces on commercial flights for passengers who must remain seated in their wheelchairs for health and safety reasons.

Below is an interview with Vicky the Disability Visibility Project did:

What motivated you to start this petition?

This project is very near and dear to my heart since I cannot sit in a standard airline passenger seat due to my disability. Many individuals, like me, lack the strength to support their necks or torsos, or it is very painful for them to sit anywhere except in their wheelchairs. Also, I have hundreds of stories from people who have been injured while being transferred by airline personnel who were not trained, and of their wheelchairs being damaged by baggage handlers.

With the globalization of our society, there is an increased need for EVERYONE to be able to fly...not just those who can sit in the seats of airplanes. It’s time to update the Air Carrier Access Act (ACAA) of 1986 and allow people who need to remain in their wheelchairs for health and safety reasons to do so while flying.

For people who are not familiar with traveling in an electric wheelchair, what are the kinds of preparations a person should take in order to fly?

First of all, when making the reservation, deal directly with the airlines and communicate your needs and make your reservations early to insure that you get one of the airline’s “aisle wheelchairs” to use while boarding and departing the airplane. Also, request to be pre-boarded. Get the name of the person with whom you spoke in case you have problems the day of your flight. Do not make your reservations online. Secondly, if your headrest, joystick, seat cushions, and foot rests are easy to remove, do so. Keep them with you at all times, as those are the parts that are most frequently damaged or lost. Finally, label your wheelchair with your name, destination, home address, and telephone number where you can be reached in case it gets lost. Disabled travelers in the U.S. can order a free booklet from the U.S. Department of Transportation outlining information for traveling with a wheelchair by telephoning PVA Distribution Center at: 888-860-7244 (Order No. 2100-16).

What’s your advice if someone experienced discrimination while boarding and exiting the plane as a disabled person? What can someone do if baggage staff damaged a person’s wheelchair?

Never fly alone. Either take your most experienced attendant or someone equally knowledgeable of your specific needs in case something happens. Airlines do drop the ball, so be proactive. Communicate your needs from the moment that you first make your reservations. And, research your rights as a passenger. These rights are found at:

http://www.friendshipcircle.org/blog/2012/06/05/air-travelers-with-disabilities-here-are-your-rights/

What have your worst and best experiences been as an airline passenger who is a wheelchair user?

My worst experience was when I flew from Phoenix to San Antonio, and the airline lost my seat cushion. My best experience was flying from Travis Air Force Base in California to Honolulu on a military Med-Evac transport plane in my wheelchair secured by a four point tie down system.

What do you say to wheelchair users who want to fly but are afraid to? What kind of advice would you give them?

Always travel with someone. Even if they do not have a disability, they can help keep you safe. Airlines do drop the ball, so be proactive. Communicate your needs from the moment that you first make your reservations. And, research your rights as a passenger. These rights are found at:

http://www.friendshipcircle.org/blog/2012/06/05/air-travelers-with-disabilities-here-are-your-rights/
In both cases, ask to speak with the airline’s Complaint Resolution Officer (CRO) immediately. You cannot be discriminated against boarding an airplane unless your disability would endanger the health or safety of other passengers, or transporting the person would be a violation of FAA safety rules. Keep in mind this can apply to someone who airline personnel determine is unable to assist themselves. Generally, airline personnel may not ask what specific disability the person has, but they can ask questions about a person’s ability to perform specific air travel-related functions, such as boarding, deplaning, or walking through the airport. Admitting that you have difficulty with one or more of these tasks also does not allow the airline to deny you travel. Rather, the airline must be able to tell you how the safety of all passengers would be affected by your presence on the flight. In the event that your wheelchair is lost or damaged, the CRO will assist you in filing a formal complaint, and the replacement or damages will be covered at the airlines expense.

**What is your goal for this petition? What kinds of changes would you like to see in the transportation industry for people who use wheelchairs in providing better access?**

The goal for the Airline Accessibility Petition is to bring awareness to the issue and to get Congress and the President to amend the ACAA to allow people who need to remain seated in their power wheelchairs for health and safety reasons to do so. In turn, it is hoped that the major airline companies will modify their airplanes so that on one side of the first row of seats may be removed as needed to provide space for wheelchairs that are secured with the Qstraint restraining system. The Federal Aviation Administration is very open to making this a reality; however, the Department of Transportation and the Airlines themselves need to be convinced. Plus, formal crash testing must be done, so this will be quite an involved process before wheelchair users can be allowed to fly in their chairs. Bringing public awareness to the issue is key.

### Airline Accessibility Petition

http://petitions.moveon.org/sign/wheelchair-access-on?source=c.em&r_by=8151355

Facebook group for the Airline Accessibility Petition

https://www.facebook.com/groups/917232951626161/

Native Texan, Vicki Jurney-Taylor grew up in a military family and has lived in Oklahoma, Florida, Puerto Rico, Louisiana, California, and Hawaii before returning to Texas to attend the University of Texas at San Antonio (U.T.S.A) to study English and then received a graduate degree in Rehabilitation Counseling at the University of North Texas. Additionally, she has earned an Advanced Certification in Nonprofit Management from U.T.S.A. Her educational and employment background in the disability advocacy field, coupled with her personal experience as a person who was born with Spinal Muscular Atrophy, has uniquely qualified her as an advocate and strategist for systems change with regard to inclusion and accessibility in the areas of independent living, employment, recreation, transportation and travel, worship, education, government, technology, communications, and consumerism.

Most recently, Vicki has been working as a Community Organizer with the nonprofit organization, All Wheels Up, Inc., whose mission is to advocate for equality in air travel for those in a wheelchair for mobility and safe seating. Utilizing her skills as a grassroots organizer and as a communications specialist, she has drafted a petition which has more than 18,000 signatures to date, as well as created a social media buzz, requesting Congress to require the airline industry to provide spaces on commercial flights for passengers who must remain seated in their wheelchairs for health and safety reasons.

Follow Vicki on Twitter: @VickiJurney
Entrepreneur with SMA Starts Accessible Car Company

By Carol Sowell, article originally featured in Quest MDA Magazine

Stacy Zoern liked the wheelchair-accessible Kenguru electric car from Hungary, so she started a Texas company to produce them.

Stacy Zoern has entered yet a new phase in her already eventful life — this time at 25 miles per hour.

When Zoern, 32, went in search of a vehicle she could drive with muscles severely weakened by spinal muscular atrophy (SMA), she looked everywhere. Her Internet search led her to Hungary and the Kenguru — pronounced “kangaroo” — a small, electric-powered vehicle that can be operated by a driver in a wheelchair. There was nothing like it anywhere else.

She was so impressed that she not only made plans to buy a Kenguru, but she went into business with the company that makes them.

The current Kenguru requires upper body strength to drive, but a model is in the works for those with limited upper body strength.

Knowing that such a vehicle would “really improve the quality of life for a lot of people,” she started Community Cars Inc., based in her hometown of Austin, Texas. Last year, her company merged with Kenguru Services KFT, and in January, the new company unveiled the first vehicle manufactured at its new plant in Pflugerville, just north of Austin.

Transportation is an important aspect of independence for Zoern. “I have to rely on people for everything. I can’t get dressed by myself, I can’t get out of bed, I can’t get to the restroom on my own. So to be able to leave my home and run errands, just to get out and go for a drive, it’s going to be awesome,” she said.

Right now she has a VW Eurovan with a lift, but it isn’t outfitted for her to drive. “My friends, family and caregivers all have to take me everywhere.”

**Kenguru still being adapted**

Zoern's own Kenguru is still in the future. The Hungarian model is made with handlebars like those of a motorcycle, which, she explains “is for people who have normal upper body strength, like people with spinal cord injuries in manual chairs.”

Community Cars is designing a second model that can be driven with either a joystick or a very small 5-inch-diameter steering wheel.

This, she said, is “the kind that people with muscular dystrophy will more than likely need. If you can operate an electric wheelchair you can operate the vehicle.”

Zoern has vivid daydreams of her life with the Kenguru: “Here’s what I imagine for myself and other people in an urban setting, or even in the suburbs. It’s for community driving, kind of like a moped. I imagine being able to go to my favorite little Mexican restaurant, or go to a doctor’s appointment on my own. Or go the movie theater, meet my friends there, and they don’t have to cart me around all the time. It will give me the freedom that I’ve never had before.”

At this time, the company has orders for vehicles from France and the United Kingdom. The first of the 200 vehicles they plan to build this year will go to fill those orders in Europe.

Zoern and her 13-person staff are seeking out a dealership in Texas that can serve the United States. The vehicle, which will retail for about $25,000, is licensed for street driving in this country but not for highways.
Childhood battles, adult victories
Zoern’s dreams of independent transportation fit in with a lifetime of breaking through obstructions and finding her way despite them.

As detailed in her 2006 autobiography, I Like to Run, Too, she has battled obstacles from the public school system, the university dormitory arrangements, Texas social services agencies, airlines and cruise ships, the general public and more. For example, because she required transportation from the special education program throughout her school years, she was required to take frequent examinations to measure her intellectual capacity, despite her superior grades and placement in a gifted program.

Entrepreneur Stacy Zoern
The fight and curiosity she developed during childhood and college has served her well in her new business venture. Beginning by contacting people she knew and widening her circle from there, she has secured $1.75 million for Community Cars from “a couple of angel investors” and other private sources.

She was surprised to find public and commercial sources of funding virtually closed to her.

“It’s almost impossible for a startup to get off the ground in this economic climate,” she found. “We have this amazing project that everybody’s so excited about, but traditional lenders won’t even talk to you unless you have two to three years of cash flow. A green car for people with disabilities seems like a no-brainer, but we went to the Department of Transportation, the Department of Energy, the Small Business Administration,” all with no luck.

One helpful source was PeopleFund, a nonprofit organization based in Austin that helps startups.

Fulfilling her dreams
The new skills and knowledge she’s acquiring every day are thrilling to Zoern.

Her new life without a law firm salary requires some sacrifices. She earns some money from contracting to do legal work, and sometimes pays herself a salary from the new company. “I make a little bit here and there, enough to survive. You have to take risks and sacrifice if you want to do something meaningful.”

One sacrifice is to “stop spending money. I don’t go shopping any more. I’m eating cereal for dinner,” she said with a laugh.

Not only is the Kenguru energy-efficient and liberating, it also offers a cost-saving option for people with disabilities. “For someone in an electric wheelchair, it’s this at $25,000 or a $100,000 van. That’s finally going to be an option.”

Her new venture allows little time for a social life. But she was gratified when several friends attended the company’s grand opening in January to show their support. Soon, in her new Kenguru, she’ll be able to join them at restaurants or the movies or their homes, without having to ask anyone for a ride.
“You must work out to be able to lift her.”

“Ugh, you are getting so heavy.”

“You should watch what you eat or no one will be able to lift you.”

“How can you lift her, you’re so small!!!”

These are just some examples of what I have heard on a regular basis from those outside of my family since my early teenage years due to the fact that I used a wheelchair for mobility and needed assistance with basic living tasks such as using the restroom. When the comments first started from the school nurse, I tried my best to ignore them and blow them off. Yes, she was helping me to the restroom but she was old so maybe she was just blowing smoke, right? In order to avoid hearing these comments, I started limiting my restroom breaks to once a day at school which meant I also had to limit how hydrated I was during the day. This wasn’t the best solution but in my teenage mind, it made sense. However, as time went on through high school and the comments did not stop, I unconsciously started my battle with anorexia. I was already limiting how much I drank during the day so limiting my food became just as easy. I became a master of “fake eating” by taking a bite or two, smearing the food around my plate, and claiming to be full. I became a master at making my Mom believe I had eaten elsewhere when it was time for a meal at home. I became a master at not eating. With high school being a stressful time for every teenage girl, my weight loss was easily explained away as stress induced. My Mom worried about me but I was good at explaining away everything and easing her concern. Once I went away to college, it became even easier for me to not eat because no one was around to hold me accountable. I became aware of how much calories were in everything and limited my intake as much as I possibly could. Instead of gaining the “freshman 15”, I lost weight and I wasn’t planning on gaining it back. I had become aware of what I was doing by not eating and I actually had no problem with it. The comments by those who assisted me had stopped and I was feeling okay with myself. I wasn’t planning on gaining any weight back until I realized that I was so sick of always sitting awkwardly while my friends ate and I was sick of worrying about calories. I finally decided that it was time to talk to someone about this internal struggle and realized I had become an anorexic. I also realized that the comments which had driven me to this dark disorder had stopped not because of my weight loss but because I had different people helping me with my needs. I had peers helping me, not old women who shouldn’t have been assisting me in the first place.

Over the next few years of college, I slowly gained enough weight to be healthy and thought I had conquered the illness. Little did I know that it was something I would battle my entire life. After college, I began my career and moved to a brand new city where I had to hire all new assistants. Unfortunately, the pool of people that I had to choose from was very small and I had to take what assistants I could find at the time until others came my way. This left me with hiring a woman who was 15 years older than me which I was not very happy about but she could do the job so I dealt with it, at least for a while. Shortly after hiring her, the comments about how “heavy” I was started coming from her and she became rather insistent that I needed to get a lift so she could care for me. At this time, I weighed about 112 lbs and using a lift was something that was not an option as it caused a great deal of pain for me. It wasn’t long before the familiar tendencies of counting calories and not eating came back; this time, though, I had discovered diet pills. I had no idea what they were doing to my body, I just knew they were helping me to drop weight fast. Eventually, I landed myself in the hospital with my organs shutting down because I wasn’t even eating enough calories to sustain basic body functions such as breathing. The physical pain I felt was unbearable but the emotional pain of watching my Mom in distress was even worse. Eventually, I recovered and once again started the long path of trying to conquer my anorexia; this time, though, I knew it was something I would always battle.

Fast forwarding to 5 years later, I was now married and we were expecting our first child. Being a mother was always something I had dreamed of and wanted, I just was never sure it would happen for me due to my disability. The joy I felt was overwhelming and
the weight gain that would come with the pregnancy never even crossed my mind. It didn’t cross my mind until the scale at the doctor’s office became my enemy. For the first several months, I had terrible morning sickness so I did not really gain any weight as my own weight was dropping while I was gaining baby weight. However, around month 5, my weight started going up and I was horrified. Logically, I knew that I had to gain weight and gaining weight was a good sign for the baby. My enemy anorexia, though, tried to convince me otherwise. By the time month 6 rolled around, I broke down in tears when I was weighed and my OB/GYN made the decision that I, nor my husband, wasn’t to be told what I weighed at each appointment and I was completely fine with this. I wanted a healthy baby and I logically knew that I absolutely had to eat to help the baby grow. For the rest of my pregnancy, I forced myself to eat as much as my body told me to eat and, for the only time in my adult life, I tried my best to not worry about calories or my weight. However, once my healthy baby girl arrived, my enemy returned with a vengeance as I tried desperately to lose the baby weight.

Present day, I am at a healthy 110 lbs (healthy for my height and my disability) and try to keep my weight right around there. My battle with calories and weight is a daily struggle but I also have a good support team who assists me when I start to feel those terrible feelings. Strangers still make comments when they see me being lifted and I work hard to just ignore those comments as they are not coming from people I deal with on a daily basis. I will always struggle with anorexia and I am aware of this; however, I think that this could have been avoided if people thought about what they were saying before they said it. Anorexia is not fun and I think those in the healthcare field who assist those with disabilities have no idea what their comments can do to a person, especially to a teenage girl. As a mother, I am very careful about what I say to my daughter and what others say to her. She is perfectly healthy but I am still cautious about her developing a poor body image. I think our society places too much emphasis on being ‘thin’ or having a ‘model’ body; I don’t want her falling into the trap of anorexia as well.

Congratulations to the Lee Family, of North Carolina, on the birth of their new little boy, Asher!

Congratulations to Andreia, Paulo and big sister Arianna Fernandes, on the birth of their new baby boy, Mason Alexandre, born on December 8, 2014!

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.
Our Clinical Trial Story

By Amanda Camp, SMA Mom

There are so many words or phrases that can describe the feelings of being a part of a clinical trial for a disease that has no treatment or cure. This picture represents just a few words that first came to the minds of parents who are currently participating in the ISIS clinical trial. I wish I could say my first emotion was “hope” or “lucky” or “blessed” but honestly it was “fear”. Thankfully for our son’s life, we didn’t stop at fear.

Afraid.

My fear didn’t come from what I knew the disease would do to my son. It came from the fear of not knowing what terrible things an experimental drug could do. The what ifs paralyzed me. I couldn’t help but imagine the craziest things. What if it affected his brain function? What if it caused a third eyeball to grow or some other outlandish bodily disfigurement (I know how silly)? What if it killed him instantly? It was hard to overcome the fear. I know it sounds ridiculous to say, but in some ways I was less afraid of what I knew the disease would do to my son and more afraid of the unknown adverse affects of the drug.

Lucky.

My thinking was completely one-sided until the night we met the first of many SMA families we would grow to love. We walked into a house full of adorable baby pictures. A little girl named Brooklynn had been born beautiful and perfect. But as you skinned over the framed pictures throughout the house you could see the devastating affects of this disease. Brooklynn went from looking like a completely healthy child to a frail and physically contorted child in less than 5 years. Her spine was curved more than 50%; her feet disfigured. As we ate dinner together, I watched Brooklynn’s frail arms lift a plastic fork as if it were a 10 pound weight. Brooklynn’s parents and grandparents shared with us their journey in the last 5 years. And then the moment that I will never forget, and possibly the moment that solidified Asher’s fate, Brooklynn’s grandmother began to cry and she said, “We would give anything to get Brooklynn into the clinical trial. She has lost so much and we don’t want to see her lose anymore. You are lucky.”

Hope.

Jeremy and I walked away that night knowing that our decision wasn’t only about our son, it was about all of the children with SMA. We felt in our hearts that we owed it to the SMA community to set aside our fears and start thinking about the hope that was possible with the ISIS drug. We knew what the disease would do and we saw a glimpse of it first hand in Brooklynn’s life, but now we were ready to see what the drug could do if it meant helping quality of life, or better yet, giving life to thousands of children, including our own son.

Gratitude.

Thankfully our new found courage and optimistic hope was nurtured by the wonderful doctor heading the clinical trial site at Nemours Children’s Hospital. It would not have been possible for us to put our son’s life in the hands of an experimental drug and complete strangers if not for Dr. Finkel. He spent hours with us explaining so much about the disease, the drug, and testing and the hope for our son and all SMA children. Honestly, if he wasn’t so patient, knowledgeable and caring we wouldn’t have felt so confident with our decision to enter Asher into the trial. Good doctors make a difference.

Amazed.

Asher was seven months old at the time he entered the trial. At four months old he lost his ability to push up from his belly. He never sat up, rolled over or crawled. In fact, Asher’s legs didn’t move at all. He had moderate head control and was able to move his arms. When you held Asher it was like holding a bean bag - mushy and unsupportive. Within three months of starting the clinical trial Asher started rolling from his back to belly. A month later Asher was able to lift his legs. Within six months, shortly after his 1st birthday, he was able to sit unassisted for almost a minute. Less than a year in the trial Asher was able to maneuver his first manual wheelchair, stand in a stander, and walk in a walker. Asher has not plateaued or declined in any physical abilities and has only continued to gain strength. We have been amazed by his progress.

Encouraged.

It didn’t take long before many people in the SMA community learned of Asher’s progress and the progress of several children in the ISIS clinical trial. Families soon began
contacting us to help decide if they should put their child’s fate in the hands of the ISIS clinical trial. With every email, phone call, and Facebook message we encouraged families and helped ease some of the same concerns and fears we had. Many families had to struggle through religious, moral and ethical beliefs to make a decision. Although helping a family decide what to do for their child was overwhelming at times, we were glad we could help families move toward hope and be encouraged by promising effective treatments such as ISIS.

**Surprised.**

We weren’t ready for what happened next in our life as clinical trial participants. As we delighted in the progress with our son and many other children in the trial, we had to painstakingly watch the decline and death of many children not in the trial. So many families had children who did not qualify for the trial.

In some cases, families with two children affected by SMA were only able to get one child in the trial and not the other. It has been heartbreaking to watch families wait to get a drug that can help their babies now.

**Anxious.**

Clinical trials take a long time and require a lot of resources. We understand this now more than ever. Another SMA parent made the perfect acknowledgement when he said, “I feel the deep sense of gratitude to the families, advocates, researchers and financial donors who have done the monumental work to raise SMA from the most hopeless of childhood diseases to the one with the greatest prospects for treatment in our lifetime.” As we anxiously wait for the phases of the ISIS trial to successfully complete, we hang on to the hope that help is on the way for all SMA children.

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**Your voice is powerful.**

Communispace is conducting a research project looking to speak with caregivers of a family member with Spinal Muscular Atrophy. **Your participation can increase our understanding of your experiences and challenges** as well as the unmet needs of families affected by SMA.

http://mayweask.com/sma/
Cytokinetics and Astellas Announce New Phase 2 Clinical Trial

Cytokinetics and Astellas jointly announced plans to begin a Phase 2 clinical trial in 2015 in patients with spinal muscular atrophy. The trial will test CK-2127107, a muscle activator. CK-2127107 will be the seventh SMA drug program to advance to clinical trials.

Cure SMA provided funding to Cytokinetics for research focused on the potential application of these types of skeletal muscle activators to SMA. Earlier this year, Cytokinetics released encouraging data from preclinical studies conducted with our funding. The data showed this approach had positive effects in preserving muscle strength and reducing muscle fatigue.

“Under the supervision of Dr. Fady Malik, we performed preclinical research relating to skeletal muscle activators. That was funded by Cure SMA, who provided grant funding around which we did some very impressive preclinical research published in recent years. That also provided a catalyst for our interest to pursue this in a Phase 2 trial,” said Robert Blum, CEO of Cytokinetics.

“I recently met with members of the senior management team at Cytokinetics. We discussed the preclinical research relating to skeletal muscle activators which had previously been funded by Cure SMA and the potential for this research to inform the development of new medicines for patients with SMA,” said Jill Jarecki, PhD, Cure SMA Research Director. “I was especially impressed by Cytokinetics’ passion and commitment to pursue improved treatment options for patients with SMA. We are very pleased that Cytokinetics and Astellas have now announced that they will initiate a Phase 2 clinical trial of CK-2127107 in patients with SMA in 2015 and look forward to results from that trial which we hope may extend the translational findings of the initial work funded by our organization.”

In non-clinical models of spinal muscular atrophy, a fast skeletal muscle troponin activator has demonstrated increases in submaximal skeletal muscle force in response to neuronal input and delays in the onset and reductions in the degree of muscle fatigue. CK-2127107 has been the subject of five completed Phase 1 clinical trials in healthy volunteers, which evaluated safety, tolerability, bioavailability, pharmacokinetics and pharmacodynamics.

This announcement highlights several strengths of our research funding model:

• We provided early seed funding to provide supportive evidence that this approach could potentially be useful in SMA. Now, two major pharmaceutical companies—Cytokinetics and Astellas—are planning to embark on a Phase 2 clinical trial in SMA.

• We invested in a variety of different approaches, attacking SMA from all sides. Other programs currently in clinical trials, such as gene therapy and ISIS-SMNRx, address the genetics of SMA. CK-2127107 may address the muscle weakness and fatigue that are caused by SMA. Ultimately, these complementary approaches could be used together.

Please visit our research section for more information on our research model.

Save the date!
Thursday, June 18th - Sunday, June 21st, 2015
2015 Annual SMA Conference Kansas City, MO
Isis Expands Phase 3 Clinical Trials

Isis recently announced that they have tested the first patient in CHERISH, a multi-center Phase 3 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.

CHERISH is the second Phase 3 study for ISIS-SMNRx. In September, Isis announced the launch of ENDEAR, a Phase 3 clinical trial to study ISIS-SMNRx in infants with SMA type I.

Additional Phase 2 Studies

In addition, Isis announced today that Biogen Idec, their clinical partner, will be conducting two additional studies which could begin in the first half of 2015:

- **NURTURE** will be a Phase 2 clinical study evaluating ISIS-SMNRx in up to 25 pre-symptomatic newborns that are genetically predisposed to the disease.
- **EMBRACE** will be a Phase 2 clinical study evaluating safety and exploratory efficacy of ISIS-SMNRx in approximately 20 patients with infantile or childhood-onset SMA. This study will bridge the gap in a small subset of patients that do not meet the age and inclusion criteria of the current Phase 3 studies ENDEAR and CHERISH.

As the ongoing trials progress, Isis and Biogen will be evaluating their clinical program and communicate any changes to our plans at that point in time.

More information on both ENDEAR and CHERISH can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) or [www.smastudy.com](http://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, on our cureSMA.org website.

Biogen Announces NURTURE, a New Phase 2 Clinical Trial

Biogen, in collaboration with Isis Pharmaceuticals, has announced that they will soon open recruiting for NURTURE. This is a multi-center, Phase 2 clinical study evaluating the efficacy of the investigational drug, ISIS-SMNRx (ISIS-396443), in pre-symptomatic newborns that have a genetic diagnosis of Spinal Muscular Atrophy (SMA).

The purpose of the NURTURE study is to evaluate the efficacy of the drug (whether early treatment with ISIS-SMNRx, before the signs of the disease are evident, could delay or prevent the development of the disease and its symptoms) and to further investigate the safety and tolerability of ISIS-SMNRx.

The NURTURE study will last about 2.5 years and is expected to enroll up to 25 infants from multiple participating clinical centers throughout the world. In the NURTURE study, all participants in the trial will receive the investigational drug ISIS-SMNRx.

The study will be conducted at multiple clinical centers worldwide (United States, Europe, The Middle East, South America and Asia Pacific). A complete list of study related criteria for this trial can also be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT02386553).

Ongoing Clinical Trials

In addition to this Phase 2 NUTURE study, Isis has two ongoing Phase 3 clinical trials testing ISIS-SMNRx.

- **ENDEAR** is testing the use of ISIS-SMNRx in infants who have SMA type I and are already showing symptoms.
- **CHERISH** is testing the use of ISIS-SMNRx in children ages 2-12 who have SMA type II.

A fourth study, EMBRACE, is planned. This will be a Phase 2 clinical study evaluating safety and exploratory efficacy of ISIS-SMNRx in approximately 20 patients with infantile or childhood-onset SMA. This study will provide data from a small subset of patients that do not meet the age and other criteria of the current Phase 3 studies ENDEAR and CHERISH. This study is expected to begin in the first half of 2015.
Cure SMA Welcomes New Medical Advisory Council Committee Members

We are excited to welcome our new members to our Medical Advisory Council. Welcome to:

- Julie Parsons, MD
- Nancy Kuntz, MD
- Perry Shieh, MD, PhD
- Randal Richardson, MD, MMS
- Elizabeth McNally, MD, PhD
- Anne Stratton, MD
- Oren Kupfer, MD
- Samuel Rosenfeld, MD
- Diane Murrell, LCSW
- Stacey Tarrant, BS, RD, LDN
- Terri Carry, MS, PT

Our MAC is chaired by Mary Schroth, MD, a leading pediatric pulmonologist, and professor of pediatrics, and includes many clinical experts in specialties such as pulmonology, neurology, physical therapy, sociology, physical medical and rehabilitation, orthopedics, cardiology, palliative care, pediatric critical care, and nutrition.

The MAC is one of the most highly respected bodies of SMA medical and clinical experts in the US, setting the agenda for proactive, creative, and collaborative leadership on issues that improve the quality of medical care for those affected by SMA. The MAC focuses on educating families, healthcare providers, and the public about SMA; expanding SMA standards of care; and translating positive research results into clinical practice.

Cure SMA would like to thank our outgoing MAC members—Drs. John Grayhack, John Kissel, Richard Finkel, and Kenneth Silver—for their expertise and years of service on the MAC.

Medical Advisory Council Gathers for Fall 2014 Meetings

Members of the Cure SMA Medical Advisory Council (MAC) held two days of meetings, November 7-8, at the Cure SMA National Office.

The MAC is one of the most highly respected bodies of SMA medical and clinical experts in the US, setting the agenda for proactive, creative, and collaborative leadership on issues that improve the quality of medical care for those affected by SMA. The MAC focuses on educating families, health care providers, and the public about SMA; expanding SMA standards of care; and translating positive research results into clinical practice.

Led by Dr. Mary Schroth—a leading pediatric pulmonologist, associate professor of pediatrics, and chair of the MAC—the meeting covers topics including:

- Medical Professional Education: MAC members are discussing potential professional education opportunities to offer to medical professionals around the country to learn about SMA.
- Clinical Care Grants: MAC members are evaluating nine clinical care grant applications to determine which grants will receive funding from Cure SMA.
- Annual SMA Conference: MAC members are evaluating survey results and discussing new workshops, possible speakers, and long term conference goals.
- SMA Care Series Booklets: MAC members are reviewing current care series booklets and looking at adding additional topics.

Our thanks to Dr. Schroth and all the MAC members for dedicating so much of their time and expertise to the SMA community.
Cure SMA has awarded a $50,000 clinical care grant to Rebecca Hurst Davis in the lab of Dr. Kathy Swoboda, for her project focusing on blood sugar levels in SMA.

Children with SMA sometimes develop insulin resistance or glucose intolerance, meaning they cannot properly regulate their blood sugar. This project will measure how the blood sugar of individuals with SMA type II responds to fasting and to different types of foods.

Research Profile

Principal Investigator: Rebecca Hurst Davis, MS, RD, CSP, CD
Institution: University of Utah
Project Title: Blood glucose monitoring and diet of children with SMA type II
Objective: This study will further assess blood sugar in 12 children with SMA type II in their home environment. The specific aims are to:

1. Monitor continuous blood sugar levels of children with SMA type II including participants from previous glucose study.
2. Evaluate and determine the role diet (a typical and a low carbohydrate diet) plays in the corresponding continuously monitored blood sugar levels.

Research Strategy

Participants with SMA type II will have a continuous blood sugar monitor placed during clinical research visit. Once participants are back at home they will record everything they eat and drink for 4 days including time and amount. One day will include a low carbohydrate diet prepared in the home. At the beginning of the 5th day participants will remove the blood sugar monitor and mail data recorder back to the researchers.

Significance of the Project

This study is needed to further understand blood sugar responses to fasting and different types of foods in a larger number of people with SMA type II in the home environment. This information will be used to better understand what is happening clinically in our patients as well as to devise strategies for ways to treat patients who are insulin resistant/glucose intolerant.

Clinical Care Funding

This grant to Ms. Davis and Dr. Swoboda is part of $225,000 in clinical care funding. We fund clinical care research to understand the issues that affect daily life for people with SMA, from breathing to nutrition, and to improve their quality of life today. We’ve profiled each of the researchers who’ve received a grant, and have shared how their work can benefit those affected by 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.
Cure SMA has awarded a $30,000 clinical care grant to Deborah Boroughs, RN, MSN, for her project focusing on caregiver training.

Individuals with SMA have complex and unique medical needs. However, parents and other in-home caregivers often don’t receive the information and training they need, especially when the individual is discharged from the hospital. This project will provide caregiver training, and evaluate the effectiveness of this training in improving care for individuals with SMA.

Research Profile

Principal Investigator: Deborah S. Boroughs, RN, MSN

Institution: BAYADA Home Health Care

Project Title: Bridging a care delivery gap for family caregivers of children with SMA type I and type II

Objective: An educational gap currently exists, where, when, and how new knowledge is delivered to family caregivers of children with complex health needs at home. Family caregivers of children with SMA types I and types II often discover that few, if any, formal continuing education opportunities exist in the community. We propose to bridge a learning gap for family caregivers that may develop after the child is discharged from the hospital. Skill enhancement and practice opportunities are expected to increase caregiver confidence and skill level and to decrease the number of emergency room visits, re-hospitalizations, and accidental deaths at home.

Research Strategy

BAYADA Pediatrics has developed a community-based family caregiver training to provide on-going training and preparation for proper emergency response by family caregivers of children with SMA living at home. Outcomes of this proposed pilot program will be analyzed using the Framework Method. Important qualitative data gleaned from the project will be published in a white paper that can be used to develop a nationwide program to extend the training family caregivers receive in the hospital to accessible and convenient education at home in the community.

Significance of the Project

To our knowledge, nowhere in the United States is there a program for continuing education in the community for family caregivers of children with SMA. The hypothesis, aims, methods, measurements and predictions for this project are innovative, unique, and designed to yield beneficial effects. We anticipate that on-going and convenient family caregiver training will empower the caregivers of children with SMA with greater confidence and skill mastery and will lead to improved clinical outcomes that enhance and extend the lives of children with SMA across the nation.

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.
Cure SMA has awarded a $50,000 clinical care grant to Dr. Walter Truong, for his project focusing on musculoskeletal issues in SMA.

Children with SMA who cannot stand on their own often use standers as part of a therapy program. As the name might suggest, these support children in a standing position and are thought to improve bone strength. Dr. Truong’s work will be measuring how effective this equipment is in improving bone strength, and how long children with SMA should use a stander.

**Research Profile**

*Principal Investigator:* Walter Truong, MD  
*Institution:* Gillette Children’s Specialty Healthcare  
*Project Title:* Effects of standing on non-ambulatory children with spinal muscular atrophy  
*Objective:* The goal of this small study is to use thin platforms that fit between a child’s feet and the footboard of a stationary assisted stander, in order to measure the force that goes through the legs of children with spinal muscular atrophy as they are supported in their stander. This project, and a future larger study that includes hundreds of children from many different children’s hospitals, will help determine whether using standers actually make bones stronger so that they don’t fracture so easily in these children.

**Research Strategy**

Nine children with spinal muscular atrophy will use their standers for one month in the same way as they have used them before this study, but will then increase the amount of time they use their standers over the next year. The special platforms will measure the force in the children’s legs as they stand, and a special scan like an x-ray will measure the strength of the children’s bones resulting from this increased standing to see if more standing makes stronger bones.

**Significance of the Project**

Children with spinal muscular atrophy who cannot walk often use standers to help them put weight on their legs so that their bones become stronger and do not break so easily. But, doctors are not sure if using these standers really strengthens a child’s bone, so this study will help show whether or not standers actually work, and if they do, how long should children with spinal muscular atrophy use the standers in any given week.
We’re excited to announce that we have awarded $50,000 clinical care grant to Dr. Nilesh Mehta, for his project focusing on nutrition in SMA.

Some individuals with SMA are at risk for undernutrition, which means they are not receiving enough nourishment. Others are at risk for overnutrition, which means they are consuming too much. Many factors contribute to these risks, so it can be difficult for doctors and families to strike the right balance. Dr. Mehta will be investigating techniques to measure each individual’s unique needs, creating a nutrition prescription tailored for them.

Research Profile
Principal Investigator: Nilesh Mehta, MD
Institution: Boston Children’s Hospital
Project Title: Role of Individualized Metabolic Measurement in Children with Spinal Muscular Atrophy.
Objective: Both over and undernourishment can pose significant problems related to breathing, strength and endurance, for individuals with SMA. The proposed study aims to examine the role of novel approach where nutritional prescriptions are based on measured calorie requirements (metabolic testing) and therefore individualized for each child. Our study will also describe the muscle mass and fat mass in subjects and examine the accuracy of a portable device that allows these measurements to be made regularly and therefore allow a more detailed evaluation of the patient’s growth and body composition.

Such a method of assessing and prescribing nutrition tailored to each individual has been shown to be beneficial in other patient groups, but has never been tested in children with SMA. Children with SMA type II and III are vulnerable to malnutrition and could benefit from a nutrition therapy that would maintain their muscle mass and strength. This group is already faced with challenges to receiving the optimal amount of feeds. This is an area that has been neglected.

Significance
Results will help identify the prevalence of malnutrition, suboptimal energy intake (actual energy intake in relation to measured energy requirement), and body composition. The findings will a new paradigm based on measured requirements, and improve nutrition related outcomes.

Clinical Care Funding
Clinical care research is the fourth prong in our research strategy. We fund clinical care research to understand the issues that affect daily life for people with SMA, from breathing to nutrition, and to improve their quality of life today.

The evidence-based clinical care projects we’re funding can improve the standard of care for those with SMA, educate medical professionals, and point the way toward new innovations.

Earlier this year, we asked scientists to submit their proposals for clinical care research projects. Our Medical Advisory Council then met to evaluate each proposal, and selected the most promising ones for funding.

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.
Cure SMA has awarded a $50,000 clinical care grant to Martin Lemay, PhD, for his project focusing on posture and breathing. Breathing difficulties are one of the most common complications with spinal muscular atrophy, and also one of the most serious. In many cases, poor posture contributes to these complications. Dr. Lemay’s project will use specific posture training exercises and a specific breathing technique, to see if the combination might alleviate breathing difficulties.

Meet Martin Lemay

Who are you?

I am a kinesiology professor at Université du Québec à Montréal and a researcher in pediatric rehabilitation at Marie Enfant Rehabilitation Center (CHU Sainte-Justine). I am currently the director of the Movement and Cognition Laboratory. I have expertise and knowledge in exercise science, movement analysis and diseases affecting movement such as SMA. I will be the lead investigator of this project and I will be collaborating with a multidisciplinary team combining a physiotherapist (Monique Émond), a movement analysis specialist and researcher (Louis-Nicolas Veilleux), and a respiratory therapist (Josée Albert) from different backgrounds.

How did you first become involved with SMA research?

In 2009, clinicians and researchers of our rehabilitation center have conducted a preliminary study evaluating the impact of postural therapy specific to trunk muscles in ten children with neuromuscular diseases (including 3 children with SMA type II and III). The 8-week therapy showed very promising results, especially in children with SMA type II and III. Following the therapy, children showed increased abdominal strength that in turn permitted stronger expiration. Lower pelvic tilt, a more symmetric posture in quiet sitting as well as better chest mobility were also observed and resulted in better breathing. Overall, improvement in respiration, posture and trunk mobility significantly improved quality of life. Postural training has been used in the last few years in our rehabilitation center with great success. However, this approach has yet to be validated with a large number of patients with SMA.

What is your current role in SMA research?

We are committed to developing new and sensitive evaluation tools as well as innovative and effective treatments for children with SMA. We have recently evaluated the impact of aquatic training and showed improvements in respiratory function, strength and range of motion of children with SMA. We have also recently identified markers of fatigue in children with neuromuscular diseases such as SMA that could be used to better monitor the effects of fatigue in these children. Eventually, we would like to create and to validate a questionnaire for monitoring fatigue in children with SMA.

What do you hope to learn from this research project?

Children with SMA often have an inappropriate posture which affects breathing. The main objective of the study is to evaluate the impact of a 12-week postural training specific to trunk muscles combined with breath stacking (a technique to help keep lungs clear of secretions) on respiratory function of children with SMA type II and III.

How will this project work?

Respiratory parameters, posture, strength and quality of life will be measured immediately before and after the intervention (postural training + breath stacking).

What is the significance of your study?

A new effective therapeutic approach could be proposed to clinicians and children with SMA.

Clinical Care Funding

This grant to Dr. Lemay is the final grant to be announced of $230,000 in new clinical care funding. We fund clinical care research to understand the issues that affect daily life for people with SMA, from breathing to nutrition, and to improve their quality of life today. Use the links below to see profiles of the other researchers who’ve received a grant, and to learn how their work can benefit those affected by SMA.
SHARING PHOTOS

Madisyn Mevissen

Madison and Ella Wolff

Oscar Merulla-Bonn

Madison Wolff

Kyler, Hayden and Jake Smelser

Lilee Ford with Tobe

Maeve, Bridget and Shannon Abradles

Nora Gooden

Lucy and Will Butler

Peter and Lucy Henkel

Levi Eastman

Peter and Lucy Henkel
SHARING PHOTOS

Tongtong Jiang

Victoria Koblentz  Stella from Europe

Ray and Ethan Fantel

Sydney Utzat

The Kulas Kids

Sam LiVigni

Rachel, Jake and Katherine Saxton
When Cure SMA first started funding research thirty years ago, we had to build up from virtually nothing. It’s no easy task. Drug development relies on volume to be successful—just one or two options are not enough. The vast majority of drug candidates end in failure. To arrive at just one FDA-approved drug for SMA, we need to start with thousands of compounds in the pipeline. On average, only 10% of drugs that make it as far as clinical development will ultimately receive FDA approval.

Getting the funding and attention we need to find a treatment and cure for SMA is an uphill battle. Our research strategy is about volume, balance, and leverage—promoting a breadth of options, pursuing a diversity of approaches, and positioning SMA as an attractive investment for companies.

Though challenges remain, we’re seeing tangible results from our approach. Through strategic investments, we’ve grown the pipeline to an unprecedented level of breadth and diversity. Fifteen years ago, we had just two potential drugs in the beginning stages of preclinical discovery. Five years ago, we had 12. Going into 2015, we have 17, including seven in clinical trials—just steps away from FDA approval.

We’ve created a new booklet, Advancing Research Toward a Treatment and Cure, to help introduce the research process. The last few months have seen several exciting announcements of new research grants and new clinical trials. As these announcements are made, we’re evaluating where each of them fits into the big picture of research, and how each might contribute to our overall goal of a treatment and cure for SMA. This booklet explains more about that big picture.

Download the PDF: http://www.curesma.org/documents/research-documents/2015-research-overview.pdf
An important goal of our research funding strategy is to share scientific findings with the broader scientific community. Scientists who receive Cure SMA funding often publish their findings in peer-reviewed journals. This means that other scientists can learn from their results, which will pay dividends across the wider landscape of SMA research—allowing us to multiply the impact of our funding.

In 2014, Cure SMA-funded research led to the publishing of sixteen journal articles. Journal publishing is competitive, and only the best and most intriguing results are published. Congratulations to all these authors on their accomplishment. Thank you for helping us move SMA research forward.

2014 Cure SMA-Funded Journal Articles


Cure SMA Funds 26 Research Projects in 2014

Our strategic research approach includes four areas—basic research, drug discovery, clinical trials, and clinical care—working together toward our vision of a world without SMA. Cure SMA has invested $57 million in research since 1984, with $35 million in the past decade alone. In 2014, we provided funding to 26 different research projects across these four areas.

Our Research Model

Basic Research

Basic research is the first step in developing a treatment and cure for SMA. Basic research projects investigate the biology and cause of SMA in order to identify the most effective strategies for drug discovery. In three decades of basic research funding, we’ve awarded 79 grants for a total of nearly $10 million.

Drug Discovery

Drug discovery converts what we have learned about the causes and biology of SMA into new drug candidates that can be tested in clinical trials. There are now 17 drugs in the SMA drug pipeline, with 6 in clinical trials. Cure SMA has been involved in over half of all SMA drug programs and has invested over $19 million since 2000.

Clinical Trials

Clinical trials test drug candidates for safety and effectiveness. Cure SMA has invested $6.5 million to conduct and prepare for clinical trials. Now, because of our successes, government and industry fund the majority of SMA clinical trials.

We continue to work with clinical partners on trial readiness and recruitment, building on experience and testing protocols we have developed. We also continue to earmark a portion of our research funding for clinical trials, so that we can opportunistically invest where our dollars will be most effective.

Clinical Care

Clinical care research is the fourth prong in Cure SMA’s research. We fund clinical care research to understand the issues that affect daily life for people with SMA, from breathing to nutrition, and to improve their quality of life today. 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.

Looking Ahead

Cure SMA will be committing another $1.8 million to new research funding over this year, including approximately $1 million in funding to be announced in the next three months.

Though there’s great promise in the research landscape, there’s also a pressing need for continued and growing investment. With the size of our community and the strength of our connections, we’re able to direct research at unparalleled scale and efficiency. We look forward to announcing our new funded projects, many of which will build on the successes of this year’s funded projects.

2014 Funded Projects

Basic Research

1. Regulation of HDAC5 phosphorylation by Cdk5 in SMA. Yong-Chao Ma, Ph.D., Northwestern University.
2. Motor axon development in SMA. Charlotte Sumner, M.D., Johns Hopkins University.
3. The role of glia cells in SMA. Chien-Ping Ko, Ph.D., University of Southern California.
4. Arginine Methylation as a Regulator of SMN Activities in Motoneurons. Jocelyn Côté, Ph.D., University of Ottawa (with funding from Canada).
5. The role of vehicle coat protein alpha-COP in new models of SMA. Sara Custer, Ph.D., Indiana University.
6. The when and where requirements of SMN in mild SMA. Christine DiDonato, Ph.D., Northwestern University.
7. To Characterize the Role of SMN Protein in Myoblast Fusion. Barrington G. Burnett, PhD at Uniformed Services University of the Health Sciences.
8. Multi-Center Electrophysiological Evaluation of Clinically Relevant Phenotypes in SMA Mouse Models. Laurent Bogdanik, PhD & Cathleen Lutz, PhD at The Jackson Laboratory.
9. Astrocytes and Oxidative Stress in SMA. Allison Ebert, PhD at the Medical College of Wisconsin.
Swiss drugmaker Roche has agreed to buy privately-held Trophos. Roche, the third largest pharmaceutical company in the world, will now bring its resources to help move olesoxime forward. Olesoxime is a neuroprotectant being developed to treat spinal muscular atrophy.

At the 2014 Annual SMA Conference, Dr. Eric Dessaud of Trophos presented results of a Phase 2 study of olesoxime. These results found the drug showed a beneficial effect on the maintenance of neuromuscular function and also helped reduce medical complications associated with the disease.

Trophos has just completed a pivotal clinical trial and is preparing to file an New Drug Application (NDA) with the FDA. At Cure SMA, we continue to leverage partnerships with government and regulatory sectors, in the hopes of moving drugs like Trophos toward FDA approval in the most efficient way possible.

Trophos has just completed a pivotal clinical trial and is preparing to file an New Drug Application (NDA) with the FDA. At Cure SMA, we continue to leverage partnerships with government and regulatory sectors, in the hopes of moving drugs like Trophos toward FDA approval in the most efficient way possible.

This includes our Hope on the Hill event, hosting FDA and other government speakers at our Annual SMA Conference, coordinating joint meetings like NINDS SO-SMART, and participating in formal FDA meetings.
This is an exciting time for the entire SMA community, as we celebrate another year of increasing momentum toward our goal of a treatment and cure for SMA.

It’s also an exciting time for us at Cure SMA, as we’re looking forward to announcing over $1 million in new research grants in the next three months—part of our $1.8 million commitment to research over this fiscal year. In total, we’ve invested more than $57 million in SMA research.

We’re marking these milestones with the release of an updated SMA Drug Pipeline. The pipeline is a chart that shows each of the current SMA drug programs, and it is one of the primary ways we evaluate the success of our research program.

The pipeline tracks:

• The stages of each individual drug program, so we can see their progress.

• The breadth of programs, so we can ensure we are attacking SMA from all sides and pursuing diverse therapeutic approaches.

• The pharmaceutical and academic partners involved in SMA drug discovery, to be sure that we are building a broad community of support.

**Important Results**

This latest version includes a number of important results:

• We have 17 active programs, more than ever before.

• We have 12 pharmaceutical partners working with us, more than ever before.

• We will have six programs in clinical trials by the end of 2014, more than ever before.

• We have two programs in Phase 3 clinical trials, more than ever before.

• One of those programs—Isis Pharmaceuticals—is the first-ever Phase 3 trial for a drug developed specifically to treat the underlying cause of SMA.

• Cure SMA has provided funding for over half of the drug programs currently in the pipeline.

**Future Goals**

Researchers often test thousands of compounds to produce one drug for clinical trials. And of drugs that do make it clinical trials, only 10% will be successful. Drug development is a huge undertaking, which makes it all the more important for us to carefully track our progress.

In addition to sustaining the progress we’ve already seen, we are working to build our cumulative pipeline from 24 programs to over 30 total programs. The cumulative pipeline is the total number of active programs (currently 17) plus the number of failures (7).

A lot of hard work has already been done, and we have great hope for the near future. But more work remains to achieve our first approved treatment for SMA, and then to extend that treatment to every age and type of SMA.

With the support of our community, we know that together, we can cure SMA.
Cure SMA Scientific Advisory Board Meets in Washington D.C.

In November, Cure SMA’s Scientific Advisory Board (SAB) met in Washington, D.C.

Our Scientific Advisory Board is a recognized group of experts in SMA and motor neuron biology. They provide strategic and practical guidance in shaping our research programs.

This meeting focused on two main priorities: awarding up to $700,000 in new basic research funding, and planning the 2015 SMA Researcher Meeting.

Basic Research Funding

Basic research is the critical first step in the research process. It investigates the causes and biology of SMA, often revealing new and more effective ways of making drugs. Earlier this year, we issued a basic research request for proposals, which is an invitation for scientists to submit their best ideas for SMA basic research.

This meeting is the culmination of the SAB’s evaluation process for these proposals. They’re looking to see which projects have a well-constructed study plan, and answer the most pressing 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.

In early 2015, we will award up to $700,000 in new funding to the best proposals, based on the SAB’s evaluations.

SMA Researcher Meeting

The SMA Researcher Meeting is held each June as part of our Annual SMA Conference. Part of the SAB’s role is to set the agenda for the SMA Researcher Meeting. They’ll determine which topics are most timely and relevant for SMA researchers.

Society for Neuroscience Meeting

Why Washington, D.C.? In addition to being a center for research, industry, and government, Washington, D.C. is also the site of this year’s Society for Neuroscience Annual Meeting, which will be held over the weekend.

Jill Jarecki, Cure SMA’s research director, and several of our SAB members stayed through the Society for Neuroscience meetings to network with other researchers, share their own findings, and attend presentations.

Our thanks to the SAB for all their hard work and expertise.

Updates on Pharmaceutical Companies Investing in SMA Drug Development

Over the past decade, one of the most important advances in SMA drug development has been the number of pharmaceutical companies investing in treatments for SMA. Ten years ago, we had just one company. Now, we have a dozen. These additional companies bring additional resources and expertise that will get us to treatments and a cure faster.

In the past several weeks, there have been several notable developments in this area:

1. Astellas invested in Cytokinetics to advance CK-2127107, a muscle drug, into Phase 2 clinical trials.
2. Roche agreed to purchase Trophos. Roche, the third largest pharmaceutical company in the world, is now using its resources to help move olesoxime forward.
3. Roche Venture Capital (VC) invested in AveXis, which is currently working on a gene therapy treatment. AveXis also received funding from Deerfield Management, a healthcare investment firm, as part of that same round of financing.
4. Pfizer and Repligen announced plans to dissolve their partnership effective the end of April. Cure SMA is currently working with the groups involved to learn more about future plans and options for the program. A further update will be provided on this as soon as we have definitive information, which will likely happen in late April.

Our SMA drug pipeline tracks the progress of all the individual drug programs, as well as the different companies who are investing in those programs. The most recent version of this pipeline was released at the end of last year. We’ll be releasing an updated version later this spring, reflecting these developments in the SMA drug programs.

Questions on the drug pipeline or on how we’re working with these pharmaceutical partners? Send us an email at info@curesma.org.
Cure SMA has awarded a $140,000 research grant to Francesco Lotti, PhD, at Columbia University, for his project, “Role of Sumoylation in SMN Function and SMA Pathology.”

Survival motor neuron (SMN) protein is critical to the function of the nerves that control our muscles. We know that individuals with spinal muscular atrophy don’t correctly produce this protein at high enough levels, so researchers are working to sharpen our knowledge of this important protein and how it impacts those with SMA. Through their research, we’re learning more about how the SMN protein functions and how other cells and tissues respond to the loss of SMN protein.

Dr. Lotti’s project will look specifically at modifications that are made to SMN protein after it is created (called post translational modifications or PMTs) to see what effect they might have.

**Meet Francesco Lotti**

**Who are you?**

Modeling human disease to understand pathological mechanisms has been the focal point of my scientific career. To date, I have studied the mechanisms that regulate gene expression, with a particular emphasis on target identification and design of therapeutic interventions. To reach this goal, I employ cell and animal models as well as a wide range of biochemical, molecular and cell biological methodologies.

**How did you first become involved with SMA research?**

Since 2003, I have been studying SMA and the functions of the disease gene product SMN. As a postdoc in the Dreyfuss’ laboratory at the University of Pennsylvania, I contributed to the discovery that SMN deficiency results in changes to the cell’s splicing machinery, particularly to a cellular complex called small ribonuclear proteins. More recently as a Junior Faculty at Columbia University, I identified splicing events caused by the loss of SMN that are essential for motor neuron function, and I have directly linked their disruption to dysfunction in animal models of SMA. These findings revealed disruption of splicing as one mechanism contributing to SMA pathology.

**What is your current role in SMA research?**

A prominent conundrum in SMA research is how SMN deficiency in all tissues leads to selective neuronal dysfunction. My work focuses on the downstream events on discovering the changes that occur in RNA splicing due to the loss of SMN. Beyond providing fundamental insights into RNA metabolism and motor neuron biology, this effort aims to unravel the molecular defects underlying the loss of SMN and to develop appropriate therapeutic strategies based on these discoveries.

**What do you hope to learn from this research project?**

The identification of biological pathways that regulate SMN function is critical in revealing strategies for SMA therapy. However, little is known about the modifications to the SMN protein after it is made (called post translational modifications or PMTs) or how PMTs control SMN function. This project will investigate the hypothesis that PMTs of the SMN protein regulate SMN cellular functions.

**How will this project work?**

The team will investigate the requirement of PMTs in SMN protein function using well-established cell and mouse model systems of SMA. Our goal is to determine whether PMTs are required for the SMN protein to work correctly.

**What is the significance of your study?**

Successful completion of this project will reveal the role of PMTs in the regulation of SMN biology. In addition, to the importance to unraveling novel regulatory networks that control fundamental cellular processes, importantly this project also has the potential to link PMTs of the SMN protein to SMA pathology.
Cure SMA has awarded a $140,000 research grant to Chad Heatwole, MD, at the University of Rochester for his project, “Development of a Clinically Relevant Outcome Measure for SMA Therapeutic Trials.”

As more spinal muscular atrophy drug programs progress to clinical trials, it becomes critically important to develop ways of accurately measuring whether or not the treatment is successful. These “outcome measures” must be developed for individuals of all different types, ages, and severity of SMA.

Dr. Heatwole’s project will focus on developing a reliable method for adults with SMA to report on clinical trial outcomes that are important to them. Along with this grant to Dr. Heatwole, our current round of funding includes a previously announced grant to Dr. Linda Lowes, who is studying outcome measures in infants with SMA type I.

Our goal in supporting these different projects is to ensure that well-designed trials can be conducted in all SMA populations. This project is supported by funding from The Spinal Muscular Atrophy Research Team (SMART), Buffalo, NY.

Meet Chad Heatwole

Who are you?

I am a member of the neuromuscular faculty at the University of Rochester Medical Center with 14 years of clinical experience treating and managing patients with neuromuscular disease. I currently lead an international network for the design and creation of disease-specific outcome measures and am the CEO and founder of The Neuromuscular Institute of Quality-of-Life Studies and Outcome Measure Development. My work has led to the development of multiple patient relevant outcome measures for use in drug labeling trials.

How did you first become involved with SMA research?

As a neurologist with subspecialty training in neuromuscular medicine I care for a variety of patient’s with neuromuscular disease; some have spinal muscular atrophy. There is a need for therapeutic advances in SMA. The development of the infrastructure to better study and evaluate SMA promising therapeutics is essential in preparation for successful clinical trials in SMA. There is a clear need to develop quality outcome measures that are reliable, valid, relevant, responsive to clinical change, and capable of capturing a patient’s insight on their health status. This need, and more importantly the desire to better provide for my SMA patients, has led me to become involved in SMA research.

What is your current role in SMA research?

While my prior research has extensively focused on other neuromuscular disorders, this will be our first research initiative specifically focused on SMA. I look forward to becoming a part of the SMA research community and utilizing the experience of our research team to develop useful disease-specific instruments for the benefit of SMA patients.

What do you hope to learn from this research project?

The objective of this project is to develop, validate, and utilize a reliable, responsive, and patient-meaningful disease-specific patient reported outcome measure for SMA clinical trials. Patient-reported outcomes are typically required by the FDA in pivotal drug trials.

How will this project work?

This research will:
• Utilize a large cross-sectional study to identify those symptoms that are most important to SMA patients;
• Develop and validate a reliable, responsive, and patient-meaningful patient reported outcome measure for SMA patients; and
• Implement this instrument in SMA clinical trials and in clinic settings as a means to track patient-meaningful responses to treatment.

What is the significance of your study?

At the completion of our work the SMA research community will have a valid outcome measure to aid in therapeutic assessment that will encourage therapeutic development for adult SMA patients.

Basic Research Funding

This grant to Dr. Heatwole is part of $640,000 in new basic research funding that we’ve recently announced. Please see below for links to other recent announcements.

Basic research is the first step in our comprehensive research model. We fund basic research to investigate the biology and cause of SMA, in order to identify the most effective strategies for drug discovery. We also use this funding to develop tools that facilitate SMA research.
Cure SMA has awarded an $80,000 research grant to Linda Lowes, PT, PhD, at Nationwide Children's Hospital for her project, “Development of An Innovative Outcome Measure to Define Disease Progression in SMA type I for Use in the Home or Clinic.”

As more spinal muscular atrophy drug programs progress to clinical trials, it becomes critically important to develop ways of accurately measuring whether or not the treatment is successful. These “outcome measures” must be developed for individuals of all different types, ages, and severity of SMA. Otherwise well-designed trials become difficult to conduct in all SMA populations.

Dr. Lowes’ project will test a new way of measuring results in infants with SMA type I. This measurement could be used in the home by parents, if the child isn’t able to make regular visits to a clinic.

**Meet Linda Lowes**

*Who are you?*

I received my physical therapy degree from The Ohio State University and my PhD from Drexel University. My co-Investigator, Lindsay Alfano and I are both currently researchers at Nationwide Children's Hospital in Columbus, Ohio.

*How did you first become involved with SMA research?*

I work with an amazing group of physicians and researchers in the Center for Gene Therapy at Nationwide Children's Hospital. I provide the outcome measures for the Center's current gene therapy study in SMA type I.

*What is your current role in SMA research?*

Matching the correct outcome measure to the changes you expect to see during a clinical trial is crucial or you may reject a potential treatment because you weren’t quantifying the correct changes. SMA is such a unique disease that we wanted to develop an assessment tool specifically for measuring abilities in infants, children and adults with spinal muscular atrophy. We are working on a software suite that measures movement abilities in infants, people who can stand and those who are in a wheelchair.

*What do you hope to learn from this research project?*

This project will develop an outcome measure to help advance clinical trials in infants with SMA type I that can be used in the clinic or the patient’s home.

*How will this project work?*

The project team will utilize Microsoft Kinect to record the infant's movement. Investigators will visit the homes to instruct families on how to use our system, teaching them to record the baby’s movement, once a month for 12 months.

*What is the significance of your study?*

It is difficult for fragile infants with SMA to participate in clinical trials. At home testing would ease the burden on the infant and family.

**Basic Research Funding**

This grant to Dr. Lowes is part of $640,000 in basic research funding. We are profiling each of the researchers who’ve received a grant, and are sharing how their work can benefit those affected by SMA.

Basic research is the first step in our comprehensive research model. We fund basic research to investigate the biology and cause of SMA, in order to identify the most effective strategies for drug discovery. We also use this funding to develop tools that facilitate SMA research.
Cure SMA has awarded a $140,000 research grant to Mustafa Sahin, MD, PhD, at Boston Children's Hospital for his project, "mTOR and Protein Synthesis in SMA."

Individuals with spinal muscular atrophy don't produce survival motor neuron (SMN) protein at high enough levels. We know that motor neuron cells stop working correctly and die when there is not enough SMN protein, but we need a greater understanding of what's going wrong in SMA. What is the exact timing when defects occur, and what other cell types are affected by low SMN levels?

Dr. Sahin's project will look specifically at a cellular pathway, called mTOR, which does not function properly when SMN levels are lowered.

Meet Mustafa Sahin

Who are you?

I am an Associate Professor of Neurology at Harvard Medical School, with a BS degree from Brown University, and an MD and a PhD from Yale University School of Medicine. I did a residency in both pediatrics and child neurology, along with postdoctoral research training in Developmental Neurobiology at Boston Children's Hospital. I established the Multidisciplinary Tuberous Sclerosis Program at Boston Children's Hospital, and I now direct that program. I'm also the Director of the Translational Neuroscience Center at the same hospital.

How did you first become involved with SMA research?

Along with my research in tuberous sclerosis complex (TSC), I'm also involved in SMA research. Both of these conditions have a genetic cause that is generally well understood, but both have cell biology that we don't understand very well. I want to understand the cellular mechanisms of axon guidance, and its relationship to neurological dysfunction.

What is your current role in SMA research?

Right now, I'm investigating a particular cellular pathway, called mTOR. This pathway goes awry when SMN protein is lowered. This work could identify genes that compensate for the loss of SMN protein.

What do you hope to learn from this research project?

The mTOR pathway regulates protein synthesis in neurons, but is suppressed in SMA. The current study aims to understand this defect better in order to find and develop new therapeutic routes for the treatment of SMA.

How will this project work?

The lab will apply its expertise in studying neuronal protein synthesis and its regulation to determining how it is altered in SMA. A combination of cell culture and mouse experiments will be used.

What is the significance of your study?

SMA treatment may be most successful by combining treatment types: for example, treatments that increase protein synthesis may be combined with those that increase SMN expression.

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.
Audrey Lewis founded Families of SMA, now Cure SMA, 30 years ago. Audrey recognized early on the importance of attracting new and talented researchers to SMA, with the hope that they would commit their careers to developing a treatment and cure for SMA.

Cure SMA honors Audrey’s legacy with the Audrey Lewis Young Investigator Award, which is periodically given to younger researchers working in the SMA field. The goal is to make a positive impact on the early phase of a talented researcher’s career, enabling them to focus on the SMA field. The first award was given in 2011 to Dr. George Mentis of Columbia University for his project, “SMA as a progressive synaptic disease.”

The recipient of the 2015 Audrey Lewis Young Investigator Award is Sara Custer, PhD, of Indiana University. Dr. Custer is a senior post-doctoral researcher, working towards leading her own SMA research lab in the future. She will receive $140,000 for her project, “Gene changes in a NSC-34 model of SMA.”

Individuals with SMA don’t correctly produce survival motor neuron protein (SMN protein) at high enough levels. Dr. Custer’s project will examine how these low SMN levels affect the genes in motor neurons.

Meet Sara Custer

Who are you?

I have a PhD from the University of Washington in Neurobiology and Behavior and I study hereditary neurodegenerative diseases in cell culture and mouse models.

How did you first become involved with SMA research?

I became involved with SMA after moving to Indianapolis and joining the research lab of Dr. Elliot Androphy. I had previously worked with some type II and III patients at an amazing therapeutic equestrian facility in Woodinville, WA. Equestrian therapy is great for your core and for your spirit!

What is your current role in SMA research?

I am interested in learning more about the basic biology of SMA and how other proteins can influence the health and maintenance of motor neurons. We use a combination of cell models and animal models to address these questions. The more we know about the cellular environment in SMA, the more targets we have to aim at for therapeutic intervention.

How will this study work?

Using a motor neuron cell model of SMA, we will determine the gene changes caused by low SMN and also examine the biological consequences of these changes on motor neuron biology and SMA pathology.

What is the significance of your study?

Determining the gene changes caused by low SMN protein levels in our cells should identify genes that are specifically important for motor neuron health. This will reveal new drug targets and thus new avenues for therapeutic intervention in SMA, beyond the SMN protein. These pathways could be critical to motor neuron health and may also be relevant to multiple motor neuron diseases.
Cure SMA is pleased to announce that we have extended our spinal muscular atrophy research program with the California Institute of Biomedical Research (CALIBR) with an additional $315,000 drug discovery grant. This partnership first began in 2012, when we awarded $700,000 to Dr. Peter G. Schultz and his team for their drug discovery program, “Optimization of Small Molecules that Increase SMN2 Levels for the Treatment of Spinal Muscular Atrophy.”

Survival motor neuron (SMN) protein is critical to the function of the nerves that control our muscles. Individuals with spinal muscular atrophy don’t properly produce this protein at high enough levels, due to a mutation in the survival motor neuron gene 1 (SMN1). Individuals with SMA do have one or more copies of survival motor neuron gene 2 (SMN2), the SMA “back-up gene.” SMN2 makes only a small amount of functional SMN protein, so researchers are looking for ways to prompt this gene to make more protein.

One very promising therapeutic approach is small molecules, which are chemicals that can treat or cure a disease. The Schultz group previously discovered several different compound classes that enhance SMN protein levels. Our funding to CALIBR has been focused on turning these compounds into a useable drug through a process called medicinal chemistry.

**Progress to Date**

As with all our drug discovery grants, this program is overseen by the Cure SMA Translational Advisory Council (TAC), a committee of drug discovery and SMA experts. Funding for this project is provided after achieving a series of predetermined milestones, which are reviewed and approved by a sub group of the TAC convened specifically to oversee this program.

To date, the research team has completed the following:

- They have demonstrated that compound treatment elevates SMN protein levels in a severe mouse model of SMA.
- They have identified new related compounds, called analogs, that require less drug to increase SMN levels.
- They have identified new analogs with more drug reaching the brain.

Recently, we expanded our funding of this project to include a collaborative effort with Dr. Chien-Ping Ko of the University of Southern California. Currently, Dr. Ko and the team are testing compounds for benefit in a severe mouse model of SMA. They will be assessing weight gain, motor function, survival, and motor neuron morphology at synapses in the central nervous system and at the muscle.

**Next Steps for New Funding**

The new goals of the project, which will also be funded through a series of predetermined milestones, focus on continuing to optimize compounds in order to identify a drug candidate suitable for human testing. These goals include demonstrating a survival benefit in a severe model of SMA, further enhancement of brain exposure, and optimization of safety and selectivity of the lead compounds.

These optimized compounds will hopefully lead to a clinical development candidate. The candidate would then undergo the series of studies required for an Investigational New Drug (IND) application, the first step to obtaining FDA approval for a human clinical trial.
Cure SMA Researchers Present at Society for Neuroscience Meeting

The Society for Neuroscience (SfN) Meeting is the premiere neurobiology meeting, with over 30,000 scientists in attendance each year. This year’s meeting was held November 15-19 in Washington, D.C. Two dozen presentations on spinal muscular atrophy were given at the meeting, several of which were by scientists funded by Cure SMA.

Cure SMA-funded researchers Drs. Giancomo Comi, Brian Kaspar, Wilfried Rossoll, Chien-Ping Ko, George Mentis, Christine DiDonato, Charlotte Sumner, Barrington Burnett, Ravindra Singh, and Matthew Butchbach joined other prominent SMA researchers in sharing their findings with the broader neuroscience community.

In addition to educating other researchers about SMA, this event also provides a chance for SMA scientists to interact with and learn from researchers who are working on related diseases, such as ALS.

In all, 31,263 attendees from 86 countries presented 13,837 posters—15,510 presenters total. Our thanks to the following presenters for making an impact with the wider scientific community.

**SMA Presentations:**


2. Survival motor neuron protein expression in motor neurons alters their intrinsic excitability in spinal muscular atrophy. J. LOMBARDO1, L. KONG2, C. J. SUMNER2, M. A. HARRINGTON1; 1Delaware State Univ., Dover, DE; 2The Johns Hopkins Univ., Baltimore, MD

3. Astrocytes isolated from transgenic Δ7 SMA mice have altered protein secretion. E. FORAN1, T. NGUYEN1, P. R. LEE2, C. GRUNSEICH1, J. NOFZIGER1, E. S. ARNOLD1, B. BURNETT3, K. FISCHBECK1; 1Neurogenetics Br., Natl. Inst. of Health, NINDS, Bethesda, MD; 2Nervous Syst. Develop. and Plasticity Section, Natl. Inst. of Health, NICHD, Bethesda, MD; 3Anatomy, Physiol. and Genet., Uniformed Services Univ. of the Health Sci., Bethesda, MD

4. Neuron-astrocyte interactions in synaptic activities in spinal muscular atrophy. C. ZHOU, Z. FENG, C.-P. KO; UNIVERSITY OF SOUTHERN CALIFORNIA, Los Angeles, CA


7. Design of AAV-mediated CNS-targeted gene delivery system using neuronal promoters. V. LUKASHCHUK, I. COLDICOTT, P. J. MULCAHY, B. MUSZYNSKI, K. NING, M. AZZOUZ; Univ. of Sheffield, Sheffield, United Kingdom

8. Magnetic resonance imaging-guided focused ultrasound use for non-invasive gene delivery to the spinal cord. D. WEBBER-ADRIAN1, 4, E. THÉVENOT1, 4, M. O’REILLY2, 5, W. OAKDEN2, 5, M. K. AKENS7, N. ELLIEN2, 5, K. MARKHAM1, A. BURGESS2, J. FINKELSTEIN3, 6, A. J. M. YEE2, 3, 6, C. M. WHYNE2, 3, 6, K. D. FOUST8, 6, B. K. KASPAR8, G. J. STANISZ2, 5, R. CHOPRA2, 5, 9, K. HYNYNEN2, 5, I. AUBERT1, 4; 1Brain Sci. Research, Biol. Sci., 2Physical Sci., 3Ctr. for Spinal Trauma, Div. of Orthopaedic Surgery, Sunnybrook Res. Inst., Toronto, ON, Canada; 4Lab. Med. and Pathobiology, 5Med. Biophysics, 6Dept. of Surgery, Univ. of Toronto, Toronto, ON, Canada; 7Univ. of Toronto, Toronto, ON, Canada; 8Ctr. for Gene Therapy and Dept. of Neurosci., Ohio State Univ. and The Res. Inst. at Nationwide Children’s Hosp., Columbus, OH; 9Radiology, Univ. of Texas Southwestern Med. Ctr., Dallas, TX

9. Protective role of olesoxime against wild type alpha-synuclein-induced toxicity in human neurally differentiated SHSY-5Y cells. C. GOURNÉ1, J. TRACZ1, M. GIRAUDON-PAOLI1, V. DELUCA1, M. SEIMANDI1, G. TARDIFI1, M. XIOURI1, L. STEFANIG2, 3, T. BORDET1, 4, *R. M. PRUSS1; 1TROPHOS, Marseille Cedex 9, France; 2Biomed. Res. Fndn. of the Acad. of Athens, Div. of Basic
RESEARCH UPDATES


11. SMA motor neurons show impaired mRNP complex assembly. P. G. DONLIN-ASP1, C. FALLIN1i,4, J. P. ROUANET1, M. E. MERRITT1,2, G. J. BASSELL1,3,2, W. ROSSOLL1i,2; 1Cell Biol., 2Lab. of Translational Cell Biol., 3Neurol., Emory Univ. Sch. of Med., Atlanta, GA; 4Neurol., Univ. of Massachusetts Med. Sch., Worcester, MA


17. Elucidating the degeneration of spinal motor neurons in human models of spinal muscular atrophy. C. XU1, K. DENTON1, X.-J. LI1,2; 1Neruoscience, Univ. of Connecticut Hlth. Ctr., Farmington, CT; 2The university of Connecticut Hlth. Ctr., Stem Cell Inst., Farmington, CT


19. iPSC-derived neural stem cells act via kinase inhibition to exert neuroprotective effects in SMARD1. C. SIMONE1, M. NIZZARDO1, F. RIZZO1, M. RUGGIERI1, G. RIBOLDI1, S. SALANI1, M. BUCCHIA1, E. F. RICHETTI1, F. PORRO1, N. BRESOLIN1, G. P. COMI1, *S. CORTI1; 1Dept. of Physiopathology and Transplant, 2Univ. of Milan, Milan, Italy

20. A high-throughput genome-wide RNAi screen for novel modifiers of survival of motor neuron (SMN) protein levels. E. S. ARNOLD1, R. M. GIBBS1, D. Y. KWON1, S. E. MARTIN2, E. BUEHLER2, R. HUANG1, B. REDAN2, K. H. FISCHBECK1, B. G. BURNETT3; 1Natl. Inst. of Neurolog. Disorders and Stroke, 2Natl. Ctr. for Advancing Translational Sci., NIH, Bethesda, MD; 3Dept. of Anatomy, Physiol. and Genet., Uniformed Services Univ. of the Hlth. Sci., Bethesda, MD

21. Regulation of SMN and other key pathogenetic events in Spinal Muscular Atrophy (SMA): Moving to RNA-Based treatment strategies. M. BUCCHIA1, M. NIZZARDO1, C. SIMONE1, F. RIZZO1, G. ULZI1, S. DAMETTI1, A. RAMIREZ1, E. FRATTINI1, S. PAGLIARANI1, N. BRESOLIN1, F. PAGANI1, G. P. COMI1, S. CORTI1; 1Univ. of Milan, Milan, Italy; 2Natl. Ctr. for Genet. Engin. and Biotech., Trieste, Italy

22. Motor neurons from spinal muscular atrophy patients exhibit hyperexcitability. H. LIU, J. LU, H. CHEN, Z. DU, S.-C. ZHANG; Physiol., Waisman Ctr., MADISON, WI


24. Loss of TIA1 impairs development of male reproductive organs in a mouse model of spinal muscular atrophy. M. D. HOWELL1, N. N. SINGH1, J. SEO1, E. M. WHITTLE2, R. N. SINGH1; 1Biomed. Sci., 2Vet. Pathology, Iowa State Univ., Ames, IA

Promising Results Published on Cure SMA-Funded Gene Therapy Project

A manuscript from the laboratory of Dr. Brian Kaspar of Nationwide Children’s Hospital was recently published in the journal Molecular Therapy. The paper, “Improving single injection CSF delivery of AAV9-mediated gene therapy for SMA—a dose response study in mice and nonhuman primates” is the first publication resulting from a groundbreaking collaboration between Cure SMA, the National Institute of Neurological Disorders and Stroke (NINDS), and Dr. Kaspar.

Beginning in 2010, Cure SMA made a series of grants to Dr. Kaspar to study gene therapy, also called gene transfer. Spinal muscular atrophy (SMA) is caused by a mutation in the survival motor neuron 1 gene (SMN1). Because of this mutation, the individual does not produce enough survival motor neuron (SMN) protein.

Gene transfer may increase SMN levels by using a virus, called a vector, to deliver the SMN1 gene to affected cells. Kaspar’s laboratory discovered that Adeno-associated virus serotype 9 (AAV9) had the unique ability to cross the blood brain barrier and the Blood-Cerebrospinal Fluid Barrier (CSF).

Dr. Kaspar and his team have studied two approaches for SMA: an injection into a vein, a process known as systemic delivery which is currently in Phase I/2 clinical trials, and delivery directly into the cerebrospinal spinal fluid (CSF), a process known as CSF-delivered gene therapy.

Using the data generated with Cure SMA funding on the CSF-delivery of the drug, Dr. Kaspar and his team were able to secure a $4 million grant from NINDS in 2013, to develop this delivery approach for human clinical trials in SMA.

An Innovative Collaboration Produces Results

“Development of therapies requires collaboration of academics, advocacy, industry, and government—no single party has the resources to do this alone. The collaboration between Dr. Brian Kaspar, Cure SMA, and the NIH is an exciting model in leveraging resources and expertise in the hope of accelerating therapy development for SMA,” said Dr. John Porter, PhD, Program Director at the National Institute of Neurological Disorders and Stroke.

The results of this research collaboration are the subject of Dr. Kaspar’s latest article. Using a one-time delivery of the AAV9 carrying the human SMN gene, the researchers found SMA animals, which typically die at 15 days of age, surpassed 280 days median survival, with many animals surviving past 400 days. This is a remarkable extension in survival with normal motor function. Furthermore, the group tested this delivery approach in larger species and found significant targeting of motor neurons throughout the brain and spinal cord.

Dr. Kaspar stated, “We are very pleased with the results of this study and are working diligently to advance a CSF route of delivery to human clinical trials for SMA. We are grateful for the support from Cure SMA and NINDS. We stand at an exciting juncture in SMA research and clinical translation with strong will to see effective therapies for all those with SMA.”

“We are excited to see expansion of the gene therapeutic program and the potential to advance this route of delivery to patients with SMA. The latest results supports further development of a CSF-delivered gene therapy treatment,” said Jill Jarecki, PhD, Cure SMA’s research director.

Current Clinical Trials for SMA Gene Therapy

The technology for both systemic and CSF-delivered gene therapy has been licensed to AveXis, a clinical stage biotechnology company. AveXis and Nationwide Children’s Hospital are currently collaborating on a Phase I/2 clinical trial testing the systemic delivery method in infants with SMA. The trial (NCT02122952) opened for enrollment in Columbus, Ohio in April 2014 and is currently recruiting in the dose-escalation phase of the trial.

The CSF-delivered gene therapeutic is also advancing toward clinical trials. Cure SMA is currently developing third-year project plans with Dr. Kaspar to continue moving CSF-delivered gene therapy forward. In the next few months, we will announce the specifics of those plans.

“A critical long-term goal of the Cure SMA drug discovery approach is to help identify treatments for SMA patients with every type of the disease and at every stage of disease progress. CSF-delivered gene therapy is a major step toward that goal and this approach may reduce the amount of gene therapeutic required for larger and older patients,” said Kenneth Hobby, president of Cure SMA.

Cure SMA would like to thank all those who have contributed funding 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.
RESULTS PUBLISHED ON FIRST LARGE ANIMAL MODEL OF SMA

Dr. Arthur Burghes, a Cure SMA Scientific Advisory Board Member from Ohio State University, has published a paper on the first-ever large animal model of SMA.

There are a number of unresolved research questions in spinal muscular atrophy, including whether we can correct SMN protein levels when symptoms are present, what is expected of biomarker measures, and the role of SMN on other organs and tissues.

Researchers have been studying SMA via mouse models for several years. The existing mouse models of SMA have been a tremendous asset for understanding what goes wrong in SMA and testing new drug candidates.

However, mice have some limitations when comparing them to human beings. Mice have a relatively permeable blood-brain barrier just after birth, and they also have other biological characteristics that aren’t typical of humans. A large animal model of SMA can help provide more of the information we need to assess if treatments might be safe and effective before they progress to human clinical trials.

Early results from the pig model show that high SMN levels are still needed in motor neurons even after birth, and that reducing SMN levels causes SMA symptoms. The results also show that biomarkers and tests like CMAP and MUNE, which are used to measure muscle function in individuals with SMA, show improvement with SMN restoration, even after the onset of symptoms.

Congratulations to Dr. Burghes and the rest of the authors on their accomplishment. Thank you for helping move SMA research forward!

Read the Fall 2014 Issue of Compass

The latest issue of Compass, our research publication, is now available online.

This Fall 2014 issue includes news from our 2014 SMA researcher meeting, and news from the 2014 CME conference.

Also in this issue, Cure SMA Scientific Advisory Board member Arthur Burghes, PhD, provides an update from our 2014 conference special session, “Moving Beyond SMN? Strategies to Identify Non-SMN Drug Targets for SMA.”

SMA is caused by a mutation in the survival motor neuron gene 1 (SMN1). In a healthy person, this gene produces a protein—called survival motor neuron protein or SMN protein—that is critical to the function of the nerves that control our muscles. Individuals with SMA do not produce this protein at high enough levels.

Scientists are pursuing many treatment strategies that address the loss of SMN protein. But other treatment strategies may also be effective. This special session looked at some of these other treatment strategies, including what we can learn from how other diseases are being treated.

You can download this latest issue from our research publications page, where you can also browse other past issues of Compass.
Spinal Muscular Atrophy, or SMA, is an autosomal recessive disease, which is represented by the blue and orange circles in my painting. The circles that are half of each color symbolize the carriers of the defective gene. These two circles are connected by intertwined red and blue cords that are supposed to be DNA. The strands of DNA meet in the middle of the painting and then flow outward again. They lead to four semi-circles which show different outcomes for a child with parents who are each a carrier of the defective gene. There is a 50 percent chance that the child will be a carrier which is indicated by the two half-blue and half-orange semi-circles in both bottom corners of the painting. There is a 25 percent chance that the child will be unaffected, and there is a 25 percent chance that the child will have SMA. In the background of my painting there are clocks and leaves. There are exactly 40 leaves and one of them is different from the rest of them. This symbolizes the statistic that approximately one in every 40 people are carriers of the defective gene. Lastly, the clocks are all pointing to infinity signs because many people have come to understand that the infinity symbol means empowerment and everlasting love. My painting is meant to remind people that we should enjoy every moment with our loved ones and keep working towards a cure.

By: Sonika Kohli Gohil

SMASH AWAY SMA!

50 Many Individuals in the Community were involved in the SMASH AWAY SMA Campaign! Smashing Whipped Cream and other items in their own faces, all in the name of SMA! Photos and Videos were plastered across social media, Promoting Spreading Awareness Worldwide!
Make*A*Wish Wheelchair Accessible Treehouse
Given to Kalen Kulas, who suffers from SMA Type II!

What makes this tree house special? It’s wheelchair accessible.

Make-A-Wish Foundation volunteers spent about 6-weeks building the new addition, fully equipped with a ramp...and it even looks like a castle.

Make-a-Wish President Dianne Kupperman said, “The wish granters’ job is to ensure that they are actually getting the true wish of the child. And through games and stories and role plays they get the opportunity to not only get to know that family but learn what the child’s true wish is.”

Recipient Kalen Kulas said, “I wanted somewhere for my wheelchair to get from that place to that place.”

Kalen will be able to play with his brother Kyan in their new tree house.

Kyan also suffers from the same disease and needs a wheelchair to get around.

Kalen’s Mother Sierra Kulas said, “I think for Kalen and for Kyan having a tree house is something we talked about and we kind of chatted a bit about how they wouldn’t be able to get into a traditional tree house and so seeing this happen with the ramp having them be independent in their chairs it’s absolutely amazing.”

The tree house is even insulated and has a fireplace inside.

The family says they’re grateful to see their son’s wish come true.

Here are some additional links on their Tree House:
My life long dream has been to become a stand up comedian. I have all the attributes. I am very witty, I love making people laugh, performing for people is always a joy, and I have plenty of funny stories I could pull from. So I think I’m set, stand up comedy here I come! Oh there’s just one problem, (beams chair up) I can’t stand up. I bet you were thinking this, but would never say it. You’re not the only one, and that’s ok.

Disability is usually an out-of-sight-out-of-mind issue and can be an uncomfortable topic for many. Disabilities themselves have become an almost taboo subject. One in which those on the outside don’t ask about, leaving us unable to tell. Historically, Don’t ask, don’t tell began in the military in 1994, prohibiting closeted homosexual or bisexual service members or applicants from disclosing their orientation and openly gay, lesbian, or bisexual persons from joining the military. Although this policy is now over, a don’t ask don’t tell mentality still remains today, in a different form.

So today don’t worry, because I am going to discuss ways of opening dialogue about those with disabilities, but before that we will ask the important of what undiscovered potential is out there in the often secluded community of the disabled, but first I want to discuss what happens when we don’t tell each other how we feel or questions we have.

So now you must be wondering, WHY I’m in a wheelchair or what’s wrong with me. Spinal muscular atrophy; three words that most likely mean very little to you, but the world to me. Those three words, spinal muscular atrophy, commonly known as SMA, have shaped my life for the past fifteen years. SMA is a neuromuscular recessive disease caused by a genetic defect in the SMN1 gene, which encodes SMN, a protein widely expressed in all eukaryotic cells. SMN1 is necessary for the survival of motor neurons, as diminished abundance of the protein results in subsequent system-wide muscle wasting. There are three main types of SMA. Type I being the most serve and deadly and type III being the mildest form, where most are capable of weight bearing. SMA type II is the most common genetic cause of infant death. And I am affected with type II. If the science stuff blew over your head, because sometimes it does, SMA essentially causes severe weakness in muscles. So now the elephant is out of the room, and you know why I’m in a wheelchair, let me tell you how I’m living with it.

Life in a wheelchair is unique at it’s best, and my situation is even more rare because my body may not work at to its fullest ability but my mind is always 100% there. Well maybe not 100% but….. But its definitely 99%. Wheelchair life is frustrating, fun, exciting, and challenging; but I wouldn’t have it any other way, but lets be honest, this is mostly because I’ve never know any other way. Part of what makes life in a wheelchair challenging, is peoples’ pre conceived view of people with disabilities. My disease has shaped my life, but I have done all I can so it doesn’t shape how people view me. But that doesn’t change how the general public perceives and treats people with disabilities. My frustration comes in two different forms…. 1. Lack of accessibility leading to exclusion and 2. Those who talk about me, just not to me. Personally every time I go out to the grocery store, or the mall I hear little kids asking their moms all sorts of questions about me and my wheelchair, questions like “why does she need that” or “what’s wrong with her” or my personal favorite, “why don’t I get a rolly-chair”. And yet, the majority of the time I am never asked these questions, their moms shush them or pull them away as if I’m contagious. And that hurts not only me, but their child, by not allowing them to be comfortable around someone with a prominent, and obvious disability, they have almost encouraged exclusion, and discrimination, which per the definition, “involves the group’s initial reaction or interaction, influencing the individual’s actual behavior towards the group.” Ok so the awkwardness in the room has slightly risen, because you have been in this situation, but don’t worry because I’m not blaming you or accusing you of doing something wrong because you haven’t, you’ve done what we’ve all been taught don’t ask people about there private lives, but this, this wheelchair is not my private life.

The United Nations reports 1 billion people or 15% percent of the population is living with some sort of a disability, making
it the world’s largest minority. And the statistics only go down hill from there due to many forms of discrimination. UNESCO says that 90% of children with disabilities in developing countries do not attend school. From education to the workforce, the international labour organization reports unemployment among persons with disabilities is as high as 80%, because often employers assume that persons with disabilities are unable to do the work. Overall those with disabilities are vastly underrepresented in the workplace. The most heartbreaking statistics involve violence, according to a 2004 British study those with disabilities are more likely to be victims of violence or rape, and less likely to obtain police intervention, legal protection, or preventive care. These statistics all represent a form of discrimination, called ableism, in which people prefer or favor those who are able over those who are disabled.

You might me wondering who is apart of this talented group of disabled people I brought up in my thesis, besides me of course. The “stevies” come to mind… Hawking and Wonder of course. These two pioneering men have much more in common than there name, they both suffer from maddening disabilities. Hawking is commonly referred to as the smartest man alive; yet he is practically paralyzed and communicates with the world through a computer and his eyes. Hawking has ALS, or Lou Grieg’s disease, something he has had for more than half of his life. What if because of his disability people refused to listen, or communicate with him? Our world but not be the same, and for what some petty reason that because he used a wheelchair he was incapable of anything else. Luckily that’s not how history played out, but what if the next Stephen Hawking is out there and is not being heard.

Stevie Wonder is one of the most famous Motown artists, who also happens to be blind. His life has been filled with many challenges, which involve both his race and his disability. In one of his most famous songs “Living for the City” he sings, “I hope you hear inside my voice of sorrow, And that it motivates you to make a better tomorrow. This place is cruel no where could be much colder, If we don’t change the world will soon be over. Living just enough, stop giving just enough for the city.”

Once upon a time, people with disabilities were considered to be God’s Holy Fools sent to earth especially by God to guide those already on earth. These people, with disabilities were treated with immense amounts of respect and were thought to be very special. So today lets take a few steps, I should rephrase that, lets roll into becoming more open and comfortable around those with disabilities and ask the questions you have built up in your mind, and in doing so we can eliminate the alienation, discrimination and judgments those with disabilities fear. And restore the respect and regard we once had for those with disabilities. Because in the end we are human too, and like anyone else we can do a lot it just may take a few extra steps. I sure can’t sing like Stevie Wonder, science is not my thing like Stephen Hawking, but hopefully I can become a SIT down comedian.

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.
21st Century Cures Discussion Document Released

The US House of Representatives Energy and Commerce Committee has released the 21st Century Cures discussion document. The 21st Century Cures initiative was created to investigate how the government can help “accelerate the discovery, development, and delivery of promising new treatments and cures for patients.”

This initiative is led by Rep. Fred Upton, who also chairs the Energy and Commerce Committee. Chairman Upton was one of the congressional guests at our 2014 Hope on the Hill event.

Accelerating Treatments

An estimated 70% of known diseases qualify as rare diseases, including spinal muscular atrophy. Fewer than 10% of those have an approved treatment. The cost and length of the drug approval process, combined with a high failure rate, can make it difficult to improve those statistics.

After a series of hearings, meetings, and roundtables, the committee identified several areas for improvement:

- Incorporating patients into the regulatory process
- Aiding young scientists who may help develop innovative cures
- Streamlining the regulatory process
- Modernizing clinical trials and medical product regulation

This discussion document summarizes their findings and proposed legislative solutions. As the title would suggest, the goal is to generate discussion that can further refine these proposals. Once this phase is complete, the committee will introduce formal legislation to the House of Representatives.

“These ideas represent an important milestone—a critical first step in a legislative process. Our solutions to boost cures and jobs are starting to take shape as we move from broad principles to legislative language,” Rep. Upton said in a statement released by the committee. “However, this document is far from the final product. Some things may be dropped, some items may be added, but everything is on the table as we hope to trigger a thoughtful discussion toward a more polished product. #Cures2015 is now underway. Together, we will get this done.”

How to Participate

Members of the SMA community who want to comment on the discussion document can send their feedback to cures@mail.house.gov.

Be sure to sign up for our e-news by entering your email in the “Sign up for the latest SMA information” box below. Once legislation is introduced, we’ll notify the community on critical next steps.

Advocacy Opportunity: State Implementation of the ABLE Act

On December 19, 2014 the Achieving a Better Life Experience (ABLE) Act became law. The ABLE Act amends Section 529 of the Internal Revenue Service Code of 1986 to create tax-free savings accounts for individuals with disabilities. The bill makes tax-free savings accounts available to cover qualified expenses such as education, housing, and transportation. The ABLE Act allows persons (including the parents of minor children) to set up accounts that supplement, but don’t supplant, benefits provided through private insurances, the Medicaid program, the supplemental security income program, the beneficiary’s employment, and other sources. To be eligible, individuals must have a condition that occurred before age 26 and each person may only open one ABLE account. Under current gift-tax limitations, as much as $14,000 could be deposited annually.

The ABLE Act still requires implementation by each State in order to establish rules for financial institutions to follow in creating options under the account. In order to provide families the opportunity to set up such accounts as soon as possible families and friends of Cure SMA are encouraged to contact your state officials and urge them to pass the necessary measures.

Action item:

1) Find the name of your state legislator (http://openstates.org/find_your_legislator/)
2) Google the name of your legislator to find their email address (the above address should also link to the legislator’s official homepage)
3) Send your legislator the following email:

“Dear (Name of Legislator):

Recently the Congress and President passed the ABLE Act into law, which helps persons with disabilities create tax free savings accounts. These accounts are critical to ensuring people with disabilities have the necessary resources to meet the unique challenges they face. In order for your constituents to access these accounts our state government needs to create the necessary regulations to implement the law. On behalf of persons with disabilities in our state I respectfully urge you to ensure our state passes ABLE Act implementing measures as soon as possible.”
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.
On April 2nd, 2014, my husband Mike and I found out we were expecting Baby #5, we couldn’t be happier! Come July of 2014 we went in for our ultrasound to reveal the sex of our bundle of joy, it was... A BOY! My husband was so excited, as we already have 3 girls; Mataya who is 14, Mya 11 and Ayla 2, and our son Rylee 3. My husband of course couldn’t wait to have another little man in the house! Mike instantly knew he wanted to name him Brody Steven, Steven being named after his father. During the ultrasound I knew something must be wrong, it lasted 2 hours! Never before had I had a ultrasound last so long. I sat anxiously, knowing the ultrasound tech could not tell me anything, so there I sat over the weekend waiting and wondering what it is they saw in my ultra sound. That following Monday I received a call at work during my lunch break, it was my Dr. saying I needed to have a follow up level 2 ultrasound as they found some abnormalities. My heart sank, every mother’s worst fear. They told me he had a right club foot, I wasn’t sure exactly what that meant either. I called my husband hysterical and so I thought!

This had been a long emotional roller coaster of tests, and ultrasound after ultrasound. I was extremely sick my entire pregnancy as well. Mike and I were relieved to hear their only concern at this point was Brody’s right club foot, this was fully treatable and not life threatening! I continued to work, but as I got closer to 7-8 months along, I was physically and emotionally drained. I eventually had to be put on bed rest and decrease my hours! October 31st came along and I was due for my final ultrasound. I was so excited! As I laid there and they were checking everything, I was feeling good! Then the Dr. said I needed to be admitted now, that I was full of fluid and Brody needed to be delivered. I was worried, of course I wanted Brody out so he was not harmed. I called my husband, he was training 2 hours away that day, just my luck. My mom came and got our youngest of 2 kids set up to be watched; she waited with me while I was connected to monitors and we waited for Mike. Once he got there I was told I needed to go to Abbott North a western hospital since it was connected to children’s for Brody. Of course United hospital was full, that is where I was supposed to deliver as my OB had rights to deliver there. Instead I had to go to Minneapolis and no longer could use my OB, I was nervous, but at the same time I was ready to meet our precious son!

We arrived at 3pm to Abbott, they checked me in, hooked me up to monitors, I was already mildly contracting. Later that night they wanted to speed up my delivery and induce me, I was put on a med to help dilate me; I continued to mildly contract throughout the night. That still wasn’t enough, and then they gave me potassium. We waited. And waited. Nothing! Little man just wasn’t ready yet, so back to another med I was put on to help contractions. Again I was playing the waiting game and nothing still! I was miserable, 2 days had passed and I was not making any changes, I was
confused, why weren’t they giving me a C-section? I mean after all I was admitted Oct 31 saying he needed to be delivered now due to the amount of fluid I had, I assumed it must not be life threatening since 2 days passed and I was still laying there waiting.

Finally the Dr. came in on November 2nd, it was decided a c-section was needed. I knew there were risks, but at the same time I was ready to meet Brody! The team prepped me for surgery, it all went so fast! I remember laying there, a blue curtain over me, my heart beating fast, and I was shaking bad! I was starting to panic as I felt to enclosed, my husband asked the Dr. to give me a med to relax. It did it’s job, I was able to relax a little, I felt nothing! I was so numb. I kept waiting to hear his cry. Nothing! I turned to my husband and said why isn’t he crying? I didn’t even know he had already been taken to the NICU. The Dr. stitched me up, they brought my baby boy in his incubator to meet me 5 minutes later. He was so tiny and precious, his eyes were wide open! He had in a tube, he needed help breathing right away; that’s why I heard no cry, of course every mothers worst fear! I was taken to the recovery room, Brody to the NICU to be monitored. I was tired but so happy he was finally here! Of course all I wanted to do was snuggle my baby boy. I was taken up to the NICU after recovery room, there was my baby boy so innocent and little as he laid in his incubator all hooked up. The Dr. said he was stable but needed to be in a breathing tube; she said something in his chromosomes, and blood tests weren’t right? I was confused I didn’t understand all the terminology she was throwing at us. I began to cry and cry, what do you mean I thought. The Dr.’s were also concerned as Brody had no movement, he didn’t move at all other than his eyes to look around. He came out with a right fractured arm as well, Brody didn’t seem to react to pain. They informed me further testing would need to be done to see why it was Brody didn’t move, didn’t cry, didn’t respond to pain.

I wanted to stay with him all night but I knew I needed to rest, it had been a long 3 days of labor and I needed to close my eyes. Brody was going to have tests done, and rest himself; Mike and I left the NICU feeling hopeful.

The next day, November 3rd, we woke up and went to see Brody. They had the neurologist meet with us, he talked to us in fancy terms, I didn’t understand. He said he was worried! Brody didn’t move and he was concerned it was something called SMA. I didn’t know what that even meant, he said it was spinal muscular atrophy type I; chromosomes tests were sent to Ohio to confirm, we had never heard of this before! The neurologist went on to say that Brody was at the worst level for this disease and would not have long. My heart again sank into my chest. What do you mean not long? He said maybe 2 weeks; Brody was breathing via the tube only and he had no muscles, he was weak and it would only progress. Brody couldn’t even make a sound.

My husband and I immediately contacted our immediate family to come meet Brody. After family had driven 2 plus hours, everyone had made it. We gathered around our baby boy and baptized him! He deserved peace and rest himself; Mike and I left the NICU feeling hopeful.

The next day, November 4th, we would let him go into his ventilator. He deserved peace and comfort! Mike and I decided that Tuesday, November 4th, we would let him go into God’s arms on November 6th. As the day went on we could see Brody getting weaker, he was already showing signs of slowing down, we wanted all of that Wednesday to hold, kiss and snuggle our baby boy. We wanted to talk to him, share stories with him, love him up all that we could. We cried and cried; I didn’t understand how it went from he’s going be okay, to I’m sorry your son will not make it. Why, why my innocent baby boy? I was mad, sad and confused, it wasn’t fair! I didn’t want to give up Brody, but I knew it would be selfish to keep him. He was ready for his angel wings, and to be at peace. That night I sat in a chair, Mike and I took turns holding him, we had to be extra careful as he had all his tubes in. I held Brody for 5 hours straight! I sang to him as we rocked in the chair, I cried and told him he will always be in momma’s heart!

The morning came, it was Nov 6th. I never anticipated this day to come so quickly, no parent does. I held Brody in that rocking chair and that morning he peacefully went in my arms. I held him tight, tears flowing down my husband and I’s face. He was gone, my baby boy! We decided to have family photos done. It was important I had these photos for my lasting memories, something I cherish so much! Our kids came into the room, peaceful music playing, Brody in his ninja turtle footies all snuggled in his blanket in my arms. The pictures were taken and I was also able to give him a bath. I snuggled him endlessly, I didn’t want to let him go, but it was time. We gave our son one last kiss and said we’ll always love you!

The next day the Dr. had called our home, said the testing was back and indeed it was SMA. I wanted to share our son’s disease and hope to help make awareness for SMA! Please help us in Brody’s name by remembering him! Thank you all for the warm heart felt wishes and prayers! Brody is our beautiful angel! Let’s help make a difference! Team Brody!

Xoxo God bless,
Mike and Kaitee Pavlich
Eighteen... and Counting

An 18th birthday is a colossal milestone. Whether it leads to college, employment, or a search for adventure, ‘18’ signifies the beginning of a new direction. New friends, new freedoms, new opportunities, new responsibilities, new dreams.

For those in the SMA family, ‘18’ takes on new meaning. For those with children diagnosed with Type I or II, an 18th birthday celebration with the guest of honor in attendance is cause for some serious hoopla. There have been several 18th birthdays lately, all with some much-deserved serious hoopla!

Many in the SMA family aren’t privy to even a first birthday, an event Randy and I took for granted after having two healthy children. When we learned Baby #3 was coming despite the fact that we’d cancelled the maternity benefits (wouldn’t need ‘em!), we shifted our middle-aged gears quickly in preparation for our new addition. Randy was going through perpetual conflicts and undermining at in his teaching/coaching situation and ended the year without a job. Likely courtesy of the constant stress, our little Jeffrey arrived earlier than planned, enabling us to enjoy him two extra weeks before the real world came knocking. I later chalked up our bonus time to angel intervention.

Jeffrey was born in May, 1997, not too long after we’d transplanted our original Fort Worth selves to (eventually) what we claimed would be our final destination - a beautiful rural area in the mountains of North Carolina. At eight weeks, my doctor brother examined his new nephew, sharing his alarming observation that Jeffrey exhibited a dull-sounding lung and no reflexes. He explained very quietly that we’d be referred by our pediatrician to a pediatric neurologist. Since one of the disadvantages of rural living is lack of medical expertise in unfamiliar situations, we were shipped off by the pediatrician the following day - ironically, Jeffrey’s well-baby appointment - to a teaching hospital 90 miles away to get input from a big-city pediatric neurologist. After almost 18 years, I think I can finally say I’ve forgiven him for being such an arrogant bearer of bad news, though I won’t forget. Nor will I forget the excruciating tests run on our baby boy to confirm the neurologist’s impression (SMA), or the absolute shock at hearing the prognosis, or the pulmonary ‘expert’ whose ineptitude three months later led to a crisis situation and a free fall to the end.

Nor will I forget the indescribable support from other SMA families, thanks to the miracle of the internet I’d never seen until the diagnosis. Before we could be hooked up here in the boonies, a friend had already found the then-Families of SMA’s guestbook and posted about our new challenge. Not long after, Annette Swetnam, mom to Betsy (Type I) and pregnant with Katie (who would also be diagnosed with Type I), called and shared some of her life with SMA. I continued connecting with other families, not comprehending at the time how critical those bonds were for my sanity. Prayer, faith, and the love and support from our own incredible families were vital as well, but the resilience and fortitude of experienced SMA families boosted me mightily for what was to come. What came was an assignment unlike anything we could have imagined.

Our sweet, happy bonus baby stayed with us for a mere 5-1/2 months. When his first birthday arrived, we didn’t celebrate. Randy and I had just begun a wild and crazy stint as bakery/sandwich shop owners in a hot tourist spot, and while I could have easily retreated to a corner and unleashed a bucket of tears, constant customer commotion kept me distracted enough to get through the day. We did, however, introduce every single customer to SMA with a flyer I’d prepared. That served as one of our earliest ways to keep Jeffrey’s memory alive.

After almost 18 years, I can say with confidence that I will never ‘get over’ our brief SMA assignment. And I’m glad. I’m thankful for the opportunities to share Jeffrey’s story with other
families, as it almost balances out - in a way - the years spent on Matthew and Katie's school stuff, sports, Scouts, driving lessons, dating, college, and weddings. I'm way out of the loop with the current SMA protocols, but some things don't change. I hope that over the years, I have provided a fraction of the same encouragement and empathy to others that was shown to me 18 years ago.

Jeffrey’s 18th birthday - on the 18th of May - will have a bittersweet touch. I'm genuinely excited when someone with SMA defies the odds, and with such encouraging advances, it seems to be happening on an increasing basis. But while I know our special little guy is in the very best place, I still wonder at times what his interests and capabilities would be today. There are some extraordinary role models in the SMA family, and I’d like to think he would have been one of them.

I would never have volunteered for SMA duty (for that matter, any kind of baby duty at my age back then!), but our family, like virtually all of you, was unwittingly hurled into it. The quality of our lives became undeniably richer because of our brief assignment with Jeffrey, and we had myriad opportunities to discover our previously untapped inner strength. The 'signs' that came when I particularly needed them and now when I least expect them make heaven seem so much closer than before. I am in awe of the stars, and God knows how much I appreciated the twinkling of a big one the night Jeffrey donned his wings.

Over the years, SMA has shifted subtly to signify so much more to me than a killer disease. Since we weren't given a choice to opt out of the assignment, I’ve pondered how my life would have been different without SMA. I wouldn’t have found Cindy Schaefer (we will surely stumble upon proof one day that we were separated at birth) and her remarkable son, Kevin, along with so many other special friends. The tenacity, creativity, dedication, and unconditional love among other SMA families is something to behold. MJ and Brenda (caregiver extraordinaire) exemplify ‘amazing.’ Many others are making a significant impact, and I’m honored to be able to witness it. SMA also provided me with the opportunity to see faith and prayer at work and how our life experiences fall into place just so. It’s been a pretty incredible reveal.

A cure for SMA would be the ultimate birthday gift for every single one of us. Meanwhile, my Pollyanna self will look at SMA as a gift that keeps on giving... way beyond 18 years.

Helen Baldwin
thejeffreyjourney.com
Cason and Ember's story is probably unique. Cason was born Feb. 4, 2013, and we thought he was just perfect. A few weeks after his birth we heard that Ember Hinson had just been diagnosed with something called Spinal Muscular Atrophy and that it would be fatal within a few months. We were devastated because my son had graduated with both of Ember's parents from our small local school with a graduating class of 70 people. Ember also has a twin sister, Darkus, who is SMA free (not sure if she has been tested for carrier or not). Well, it wasn’t long after we learned this, that we began noticing that Cason was losing any muscle tone he had at birth and began having swallowing issues and breathing issues. Well, at 2 months of age at Arkansas Children’s Hospital, we received Cason's diagnosis and were totally blown away that what is called a “rare” diagnosis was given to two babies within a few weeks of each other, with three carriers having graduated from the same small high school. Ember passed in May of 2013 at 10 months old and Cason passed June 9, 2013, at 4 months old. Both had the bright big eyes so prominent in SMA children… their only portals of communication to the world. Our life with Cason was bittersweet knowing he would not live long, but learning so much from him within 4 short months. I know you have heard this a thousand times, but he totally changed our lives. We are committed to the search for the cure and are so excited with each bit of news we hear bringing us closer and closer to that end, or at least treatment that can extend lives.

So there you have it….Frankly I wish I was still in my cocoon, never having heard of SMA. But since it has affected us so closely, I am connected to the SMA community, most of whom you would know by name I’m sure. What a wonderful loving family they are, so supportive of one another. And the babies, children and adults who are the warriors and angels of SMA are very special for sure. I’m proud to be a member of the community.

Hoping our money puts the research over the top.

Mary Bauss
Matthew A. Halanski, a Cure SMA-funded researcher from University of Wisconsin – Madison, was the lead author on, “Assessing the Needs of the SMA Population,” an article recently published in the journal Sage Open.

Drawing on responses from both families and healthcare providers, the article investigated what medical issues are most important to families affected by spinal muscular atrophy, and what medical issues healthcare providers consider most crucial.

Families identified breathing issues, impact of diet, impact of disease on the family, spinal deformity, and surgical interventions as their most important issues. Healthcare providers also identified breathing, diet, family impact, and spinal deformity as urgent needs.

This survey was conducted by Dr. Halanski and his team with support from Cure SMA. Through our Annual Conference and other forms of outreach, families affected by SMA and healthcare providers in a variety of disciplines were invited to participate.

Dr. Halanski was also the recipient of a 2013 Cure SMA clinical care grant. His project, “Spinal Muscular Atrophy: A Multicenter Multidisciplinary Assessment” will create a database of patients with SMA from multiple clinical centers. The database will be used to compare patient outcomes with the goal of improving the standard of care for individuals with SMA.

The data from this study will be used to help build the database, ensuring that the most pressing issues for families and healthcare providers are addressed in it.

“We’re very grateful for the support we’ve received from Cure SMA. Without their funding, and without the thoughtful participation of so many families, this project would not be possible,” said Dr. Halanski. “We look forward to seeing the project completed, so that we can continue to raise the level of care that individuals with SMA receive.”

Our thanks to Dr. Halanski and the rest of the authors—Drs. Karen G. Patterson, Sarah A. Sund, Linda M. Makholm, and Mary K. Schroth—for their hard work on this project, and to the families and healthcare providers who contributed to this survey.

The National Institute of Neurological Disorders and Stroke (NINDS) used the meeting as a forum to discuss the appropriate outcome measures for clinical trials, using SMA as an example population. This is critical because neurological diseases are often chronic, slowly progressive diseases with diverse populations—all of which present challenges when designing clinical trials of novel therapeutics. The three specific goals of the SO-SMART Meeting were to:

1. Have an interactive discussion about the current state of knowledge in the SMA field,
2. Present research data on the available outcome measures, and
3. Have an interactive discussion with the patients themselves on patient-related outcomes and quality of life.

The NIH asked Cure SMA to organize and lead the panel discussion on patient and caregiver perspectives on clinical trial design. In addition, the workshop participants included scientists, patients, caregivers/parents, and representatives from Cure SMA, the SMA Foundation, the Food and Drug Administration (FDA), NINDS, and private industry.

Visit the NINDS website for a summary of this session and the entire NIH-sponsored SO-SMART Meeting.
SHARING PHOTOS

Andrew and Patrick Murray

Bella Luper

Charlie and George Zerzan

Ava, Jackson and Delaney Long

Anna Rose and Joshua Scurria

Eleanor, Jack and Robert Bolton

Abby, Matt and William Johnson

Cure SMA in Brisbane Australia

Charlie Sykora

Bruin French
SHARING PHOTOS

Gray and Riley Dougherty

Ella and Madison Wolff

Hope, Michael and Noelle Willoughby

Jacqueline Kostyuchenko

Isaac Kimmel

Kaitlin Schoenbeck

Christen & Lizzy Huette

Eloise, Samantha and Jamie Pillarella

Hayden Scarbrough

Griffin Kingkiner and Anna Landre
Gwendolyn’s First Dance Recital

By Victoria Strong, Blog post from Nov. 16, 2014

She runs. She dances. She’s Super Gwendolyn!!!

It has been an enormous week for Gwendolyn. Our little girl is accomplishing so much and we are so proud of her and so grateful. Of course, last week she ran her THIRD Half Marathon! She continues attending school full time and this week happened to include some reading comprehension and spelling word testing -- and she nailed it! And on Thursday she danced in her very first dance recital -- costume and all. These are achievements to be proud of in any child. But in the world of SMA, it almost feels super human.

When Gwendolyn was diagnosed with SMA Type I, the idea of watching my baby girl in a dance recital seemed impossible. Running a half marathon? Out of the question. And while I am up on the stage helping to move her body so she can dance hip hop and Bill is pushing her wheelchair so she can run -- she is getting to do these things, things she wants to do.

Gwendolyn is in such a great place right now. She has incredible stamina. She has a determined focus which seems to further fuel her grit. And she seems especially strong. All of which continues to astound us. Where Gwendolyn is in her life right now is such a different place than we were in last February while in the PICU. And it is so far beyond what we could have imagined when she was diagnosed with SMA as a baby and the doctor said, “Take her home and love her for what limited time you have left.”

SMA makes children so profoundly physically weak. I often forget to explain the actual physical weaknesses of SMA, forgetting that the average person cannot even wrap their head around the limitations SMA creates on the body. The ability to sit up in a chair, to hold one’s head up on their own, to lift one’s arm, to grasp a pencil, to wiggle one's toes, to open the mouth and be able to form words... all taken. Children face multiple surgeries and therapies and machines and special diets and respiratory treatments several times daily and 24/7 care similar to what you’d find in the intensive care unit at a hospital... all just to help their bodies function in the same way the typical person’s body does.

But limitations are not even in our vocabulary. Pretty much anything Gwendolyn wants to try we have been able to find a way for her to have access to it. And we are not the only ones. We know so many families across the world who do nothing but create opportunities for their children to experience a life that is wild and free. Zip-lining with a breathing machine -- no problem with modifications. Riding a horse when one can’t sit up -- it can be done with help. Playing team sports in a wheelchair, yup. Surfing -- absolutely, in a different way. Ice skating, PE, cheerleading, roller skating, sledding, swimming, sailing, skiing... despite mountains of physical challenges, they are all possible!

So while SMA makes a person profoundly physically weak, they are also incredibly strong. They are brave. They are creative and charming. They are inquisitive and curious. Artistic and musical. Intelligent and daring. Just like Gwendolyn, those with SMA are vibrant little souls who are not defined by their disease. They want the world -- just like any person. They live and breathe a NEVER GIVE UP philosophy.

Soooo... back to Gwendolyn’s hip hop dance recital. It was AWESOME! One thing we know for sure -- our girl loves to dance. She was so excited about it and so proud during it. She knows every single move and darts me a “MOM!” glance if I miss a step. She has loved practicing every week with her teacher, Miss Rose, and schoolmates and she and I continued practicing the different moves at home. Our friend and fellow Washington parent, Cliff Hubbard, fixed the elevator behind the stage so Gwendolyn could have full access onto the stage. Miss G has a touch of the performance bug and so getting to have an official recital was the big crescendo to an already wonderful experience. And in costume -- double wow! And on the stage -- triple wow! And with an audience of adoring fans.
Hunter Davis met his hero “Winter the tailless dolphin”. Winter provides Hunter with a great deal of strength. He will remain in his stander, and do more physical therapy while watching Winter in Dolphin Tale. Hunter’s mother emailed the Clearwater Marine Aquarium, to let them know Hunter would visit the Aquarium in January, with a picture of Hunter watching sea lions on his third birthday. The Aquarium emailed her back, stating that they wanted to provide Hunter with a special encounter with Winter.

The Clearwater Marine Aquarium arranged for the special encounter, where many of Hunter’s family members watched. Hunter and Winter played with each other, and quickly became close friends. Although Winter is much larger than Hunter, she is gentle and loving. Hunter was completely comfortable with Winter during the encounter, and even played with her mouth. The Clearwater Marine Aquarium didn’t stop with the encounter. They videoed the experience, and interviewed Khrystal, Hunter’s mother. The Aquarium stated they make documentaries from time to time, and might include a portion of the interview. In the interview, Khrystal explained that Hunter, now three-years-old, was diagnosed Spinal Muscular Atrophy (SMA) type I at eight weeks of age, and that SMA is like ALS in babies, but is a type of Muscular Dystrophy. Hunter’s nine-year-old sister Alexandria first introduced Hunter to Winter. Alexandria learned about Winter, and said that Hunter would have to meet Winter because Hunter is like Winter. Both Winter and Hunter continue to overcome the odds. Hunter wore a shirt to the encounter made by his mother that read, “Winter has HOPE for an SMA Cure.”

The Aquarium surprised the family by sharing the video it made on Winter’s Facebook page, and the Aquarium’s Facebook page. The video went viral, and was viewed over a million times in a couple of days. The Davis family is extremely thankful to the Clearwater Marine Aquarium and Winter for bringing SMA awareness to so many people. The video can be viewed at [http://on.fb.me/1uNTb1Q](http://on.fb.me/1uNTb1Q)

-- oh yeah!!! Gwendolyn felt like she had hit Broadway!

And I loved every single second. Getting to experience this with her, dance with her, make this happen for her -- fills me with such satisfaction that the building could be crumbling around me and I would still be laser focused on Gwendolyn’s big smiling eyes.

This has definitely been a big week for our super girl. And not a moment goes by that we are not grateful for the gift of stability, strength, endurance and the opportunity for Gwendolyn to live her life doing all the many things she wants to do.

Video of Miss G Thang being so Hip Hop ::

https://www.youtube.com/ watch?v=jxS5ONWcRxM&feature=player_embedded
I would just like to submit this documentary that was made about our daughter Noel who has SMA type III. Plus an update on her condition post-surgery. The point being that we have weathered a very big storm and while there were times when we couldn’t see the storm ever subsiding, slowly and steadily things have improved and continue to do so.

In January 2014 our daughter’s pediatrician referred us to an Orthopedic Specialist because her spine was showing signs of scoliosis. She was 15 and had only been in a wheelchair full-time since age eleven. In 2013 her back was perfectly straight, so we were more than a little concerned at the change in just a year’s time.

Immediately after seeing the orthopedic doctor, we were sent to Auckland - a six-hour drive from our home, to a Spine Surgeon at Starship Children’s Hospital. The surgeon dropped a bomb on us that day and told us that Noel would need a full spinal fusion. We were absolutely blown away by this news. We asked about bracing or other methods of correction and were very bluntly told that her case was too severe for anything preventative and that if we had wanted to go that route that we should have thought of that years ago! The problem was that we didn’t even know that this was a possibility. No one warned us. But also we never came across this information ourselves and we are quite pro-active when it comes to our children’s health.

Our biggest concern other than this being a life-threatening surgery, was the possibility of mobility loss. We never got a straight answer from our doctors and there was no post operative care offered to help Noel to regain strength or to re-learn how to use her newly rigid upper torso.

The surgery itself went well. She spent two full weeks in hospital with me, her mother, by her side and her dad flying up whenever he was able. They flew her home in an air ambulance and she was still in immense pain. Physically she slowly healed, but emotionally she was traumatized not only by the surgery but also by the sudden inability to do anything for herself. At fifteen, she suddenly needed us to do everything for her and this caused her to fall into a deep depression.

Somehow in the midst of navigating these dark waters, she met her boyfriend, who has been a constant, patient and loving hero - helping her to find her way out of the darkness. He saw her through more hospitalization due to depression, so much physical pain and finally to the point she is at now - excited to live and to see what the next big adventure in life holds.

Also in the midst of all of this a documentary was made about Noel’s photography, her struggles with “being different” and her surgery. It was an amazing time and has led to so much discussion and awareness about what SMA is and how it affects families.

Noel is now 16 years old and she has been invited to exhibit her photography at our local museum in December. She is looking forward to starting Design School - but can’t go until they install a lift (that’s another story). And she is very interested in all the activity happening with CureSMA and what it could mean for the future. This is why she has pledged 10% of her proceeds of all work sold to CureSMA. I think we have about $80 to pledge so far.

Here is a link to the documentary: http://attitudelive.com/blog/dan-buckingham/documentary-noel-focus

Here is a link to her Website: http://noelratapu.com/

And here is a link to her Facebook page: https://www.facebook.com/NoelRatapuPhotography

Kind regards
Shauna Ratapu
SHARING PHOTOS

Josette Espiritu

Jonathan Valerio

Kalen Kulas

Koen Guest

Jace Dorer

Kyan Kulas
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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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.
Each year, Team Cure SMA invites a dedicated group of individuals to participate in marathons, half marathons, and other running events across the United States who share one vision: a world where spinal muscular atrophy is treatable and curable. As a member of Team Cure SMA, you have the opportunity to impact the SMA community by raising awareness and building our network of support. Dollars raised are helping to fund the research, support, and care initiated that Cure SMA will fund in 2015.

To find an upcoming race in your area please visit the Events Calendar on our webpage.

--

**Falmouth Road Race**  
Sunday, August 16, 2015  
Falmouth, MA

**2015 Chicago Half Marathon/5K**  
September 27, 2015  
Chicago, IL

**Denver Rock ‘n’ Roll Marathon**  
Sunday, October 18, 2015  
Denver, CO

**St. Louis Half Marathon**  
Sunday, October 18, 2015  
St. Louis, MO

**Los Angeles Rock ‘n’ Roll Half Marathon**  
Sunday, October 25, 2015  
Los Angeles, CA

**2015 Philadelphia Half Marathon & 5K**  
Saturday & Sunday October 30 & 31, 2015  
Philadelphia, PA

**2015 TCS New York City Marathon**  
Sunday, November 1, 2015  
New York City, NY

**2015 Rock ‘n’ Roll Savannah Marathon & Half**  
Saturday, November 7, 2015  
Savannah, GA

**San Antonio Rock ‘n’ Roll Marathon**  
Sunday, December 6, 2015  
San Antonio, TX
PHOTO 12th Annual Alabama Walk-n-roll to Cure SMA

The 12th Annual Alabama Walk-n-Roll was held in Snow Hinton Park, AL on November 1, 2014. The Walk-n-Roll was a huge success and raised over $33,500 to help find a treatment and a cure for SMA. There were over 200 attendees, and everyone enjoyed the face petting, petting zoo, games, silent auction, chili cook-off, bounce house, fire truck, ambulance, and police car. We are grateful to ALL the sponsors who generously donated to the cause. Thank you to Jennifer and Jason Patrick and their amazing team of volunteers for organizing the event.

PHOTO 9th Annual Arizona Walk-n-Roll

The 9th Annual Arizona Walk-n-Roll was held on November 16, 2014 at El Dorado Park in Scottsdale, AZ. Family, friends, teams, sponsors, and vendors from the area came out to make the day a huge success. This year’s event raised over $16,000! A delicious lunch was provided by the Arizona Barbeque Association followed by an amazing raffle!

There were a variety of games and activities for individuals to take part in before and after the Walk-n-Roll. A special thank you to the volunteers from the Boys Team Charity and the Occupational Therapy students from A.T. Still University for running the games and activities! A special highlight of the day was having an adaptive motorcycle that the children could drive their wheelchair in and get a ride around the park! It was a great time for families to connect and allow the children/young adults to hang out, play games and enjoy the beautiful, sunny weather. Thank you to everyone for coming out and supporting this event! See you next year!

PHOTO 2nd Annual Bike for a Cure

Scot Raab and the Arizona Chapter held the 2nd Annual Bike for a Cure event in Flagstaff, AZ on September 27, 2014, and raised $965! Individuals were served a pasta dinner the evening before the race to get everyone carb’ed and ready for the next day. The participants rode through beautiful Fort Tuthill on the custom designed bike trail. Thank you to Scot for organizing and coordinating this event!

Note: The amounts raised and shown are totals as of February 9, 2015 and may differ from current fundraising totals by the time you get this newsletter.
P.F. Chang’s Rock ‘n’ Roll Marathon and Half Marathon
Alicia Magera ran in the P.F. Chang’s Rock ‘n’ Roll Marathon and Half Marathon in Phoenix, AZ, on January 18, 2015 for Cure SMA. Alicia ran in the 5K on Saturday the Half on Sunday and set personal records in both races. On Sunday, Alicia beat her goal by three minutes with a finish of 2:26:58.

Thank you to Alicia for her support in running on behalf of Cure SMA and raising $510 for a treatment and cure!

SMA Dinner and Candle Lighting
The SMA Dinner and Candle Lighting ceremony was held on three different dates during August in Phoenix, AZ. The event allowed families affected by SMA to come together, enjoy dinner, and celebrate their wonderful children. Thank you to everyone who attended this special event and to the members of the Arizona Chapter for all of their hard work planning this event!

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.

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**NORTH CAROLINA**

**Love, Hope & Strength Fundraiser**
The Love, Hope & Strength Fundraiser was held on Saturday, August 23, 2014 at the Flanders Gallery in Raleigh, NC. The fundraiser was held in honor of Brady Chan, SMA type II. This year, the event was held on a Saturday so that more families would be able to attend. The evening was filled with fine art, cocktails, live entertainment, and a silent auction. This year’s fundraiser brought in over $25,000 to help find a cure for SMA! Thank you to Mimi Chan and her committee, the attendees, and sponsors for organizing such a successful event!

**2nd Annual Carolinas Chapter Walk-n-Roll**
The 2nd Annual Carolinas Chapter Walk-n-Roll took place on October 11, 2014 in Anderson Point Park in Raleigh, NC. It was a fun-filled day with many SMA families and friends all coming together to help find a cure for SMA. Thanks to the generous support and participation from all attendees, the event raised over $11,000! Thank you to Jennifer Lee, Rebekka Mastin, and Carrie Ann Boles for planning this great event. Also, special thanks to Biogen Idec for joining us and to Meredith Horne for donating her photography services.

**Christmas Coffee Fundraiser for Gray’s Gang**
This year’s Christmas Coffee Fundraiser was held on December 9, 2014 in Charlotte, NC in honor of Gray Dougherty, SMA type II. The event brought in over $3,700 thanks to generous contributions from many participants. A special thanks to Kim Dunn, Betsy Little, Nell Parker, and Nina Pulliam for all of their hard work organizing this event!

**Haley Mitchell’s Memorial Gift Registry**
Haley Mitchell, SMA type I, passed away on October 26, 2010, shortly after her 13th birthday and shortly after the 12th Annual Haley Mitchell Ski-a-thon. In October of 2011, the final Haley Mitchell Ski-a-thon to honor her memory was held. This year, Jeff and Valerie Mitchell of Elizabeth City, NC raised over $5,265 in Haley’s memory through a letter writing campaign. Thank you to Jeff and Valerie for their continued support of Cure SMA!

**Stroller Warriors: T-shirt & Running Fundraiser**
The Stroller Warriors® Virtual 5K for Cure SMA is held in Jacksonville, NC, and is an opportunity for all Stroller Warriors and supporters to get involved and dedicate 3.1 miles to raising awareness for SMA. Stroller Warriors was founded by Stephanie Geraghty, a military spouse, who is directly affected by SMA. This year’s virtual 5K raised almost $1,300 to help find a cure for SMA. Thank you Stephanie for your continuous support, and for all of your hard work organizing this year’s fundraiser.

**Island Independence 5K - Charley Walker Team**
What started out as a bet between Richard Rogers and his sister-in-law Maria Holler, has turned into a way to raise money for charity. The Annual Independence Day 5K was held July 5, 2014 in Topsail, NC, in honor of Charley Swing, SMA type I, and Walker Herring Taylor. Charley, Richard and Elizabeth Rogers’ niece, was diagnosed with SMA at nine months of age. Thank you to the Charley Walker Team for raising $1,000 for Cure SMA!

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SOUTH CAROLINA

Bugenske Family’s 5K Run
On Saturday, October 25, 2014 in Irmo, SC, Leo’s Pride and over 600 participants ran and walked a 5K or one mile in support of Leo Bugenske, SMA type I, and Cure SMA. In addition to the race, Leo’s first birthday was celebrated with a beautiful cake. The Bugenskes donated $17,000 to purchase eight EasyS Strollers for the Cure SMA equipment pool. The Bugenske family was inspired by their own experience of receiving an EasyS Stroller from Cure SMA. The stroller keeps Leo comfortable, on the go, and also holds his medical equipment. In addition to the hundreds who came out for the event, a generous anonymous donor matched each dollar raised.

Our thanks to Meredith, Adam, Leo Bugenske, and everyone who supported this event.

3rd Annual Mistletoe Jam
On December 11, 2014, the Southside Smokehouse and Donny McInerney hosted the 3rd Annual Mistletoe Jam in Landrum, SC, in honor of Alexandra Genovese, SMA type II. This year’s event included local musicians, storytelling, prayers, poems, contests, auctions, and Christmas readings, which led to $6,343 in donations. The Mistletoe Jam would like to thank the following individuals for performing and/or participating: Doug and Marie Hooper, Shannon Nethery, Mike Holland, Jay and Isiah, The McInerney Family, Jamie Hartung, Natalie Taborda, Gretchen Frayseth, Don McInerney, Chris Genovese, and the Ark of Salvation Russian Choir.

Burns Family Fundraiser
Thank you to Megan Burns for organizing the Burns Family Fundraiser on July 6, 2014 in Mt. Pleasant, SC in honor of Ellie Burns, SMA type I. The event raised $250 for Cure SMA! Thank you Megan for all of your hard work organizing this fundraiser.

Go Miller Go 5K
The first annual Go Miller Go 5K was held in Charleston, SC on August 23, 2014 at James Island County Park. It quite possibly was the hottest day of the summer, but that didn’t stop a huge crowd from coming out for a fun run to raise awareness for SMA! Everyone had a great time running, walking and rolling through the picturesque, mostly shaded park, ending at the covered shed overlooking the lake for the DJ and refreshments.

The 5K was a huge success with almost 300 registrations and over 200 runners and walkers, representing seven families in the area affected by SMA. The event is held in honor of Miller David, age two, who has SMA type II. The event raised over $34,000, and we are looking forward to next year’s 5K!

Lindsay David
Charleston, SC

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MARYLAND

21st Annual Chesapeake Crab Feast and Silent Auction

On August 3, 2014 crab lovers from all over the Baltimore area came together to pick crabs to cure SMA at the American Legion Hall in Towson, MD. The event was attended by over 350 people. In addition to the crab picking, the attendees had the opportunity to participate in a silent auction and two raffles this year. The first raffle was for two football designed Adirondack chairs donated by Boot-Cat Chair Company and John Trainor and with this donation we raised over $2,300. The second raffle was for a handcrafted Baltimore Ravens quilt donated by April Hinkle. With this donation we raised over $1,000. The continued support from family, friends and faithful attendees has enabled the Crab Feast to succeed for 21 years. With their help and generosity the event raised over $56,000 to Cure SMA! All proceeds from the event support the Erin Trainor Memorial Fund.

Beverly Venedam
New Freedom, PA

Our Little Jewels 4th Annual Benefit Golf Outing

The 4th Annual Our Little Jewels Golf Outing was held at the Links at Challedon in Mt. Airy, MD on Friday, September 26, 2014 in honor of Julian Lewis, SMA type III. One hundred and twenty golfers participated in this year’s event, helping to raise over $34,000. Three of the 28 sponsors of the event helped raise over $11,000 in donations this year. Those three sponsors included the J P Blase Cooke Family Foundation, Dave and Cindy Glenn, and Brian and Cheryl Leonardi. Thank you for all of your hard work and generous donations!

Jim Lewis
Ellicott City, MD

WEST VIRGINIA

18th Annual Arnold Family Golf Outing

On October 13, 2014 at The Berry Hills Country Club in Charleston, WV, the 18th Annual Arnold Family Golf Outing took place. The golf tournament began in 1996, and is hosted by Linda and Kevin Arnold in honor of their son, Eric Arnold, SMA type II. This year’s outing brought in $7,000 to help support Cure SMA. Thank you Linda and Kevin for hosting another successful outing, and for your endless support of funding and research to help cure SMA!

Baltimore Ravens Quilt Raffle

On August 31, 2014, in Bridgeport, WV, the Chesapeake Chapter held the first ever Baltimore Ravens Quilt Raffle. The Chesapeake Chapter received this amazing, handmade Baltimore Ravens Quilt from April Hinkle in memory of her grandchildren, Jeremiah and Aavianna, who passed away from SMA type I. Congratulations to Lynne Maher on winning the quilt, and thank you to everyone who purchased a ticket or made a donation. Together, $300 was raised to help find a cure for SMA.

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Chesapeake Chapter
WASHINGTON, D.C.

Congressional Dinner – Washington, D.C.

This year’s 4th Annual Hope on the Hill Congressional Dinner in Washington, D.C. was a great success. Building on 2014’s Annual SMA Conference—also held in Washington D.C.—the Hope on the Hill Dinner brought together families, government, and industry leaders to work on shared goals like increasing awareness, advancing treatments for SMA, and improving patient care.

Held on December 3, 2014 at the Willard InterContinental Hotel in Washington D.C, the event featured keynote addresses from Rep. Greg Walden, and Kenneth Hobby, president of Cure SMA.

The event also raised $154,000 for SMA research and family support – $4,000 more than the original goal, and more than $30,000 over last year’s event.

Our thanks to Greg and Shannon Zerzan, who created and organized the event. Thanks also to our guests, the Honorable Reps. Sean P. Duffy, Scott Garrett, Erik Paulsen, Pete Sessions, Fred Upton, and Greg Walden.


The activities held as part of Hope on the Hill have already shown tangible results.

On December 3, 2014, the House of Representatives passed the ABLE Act with bipartisan support: nearly 96% voted in favor of the bill. The bill now goes to the Senate for approval. On the PPMD blog, Annie Kennedy shares a little more about the ABLE Act and what this will mean for families in our community.

On Thursday December 4, 2014, representatives from Cure SMA and Biogen Idec met with several key Senate leaders to discuss the Newborn Screening Saves Lives Act. This legislation ensures that the critically important federal newborn screening program will continue to function and identify newborns with deadly but treatable chronic illnesses. This bill had been passed by the House but had yet to be acted on by the Senate.

After those meetings, the Senate created an amended version addressing issues, such as informed consent, that are important to our community. This bill is expected to be voted on by the Senate this week, and will then be sent back to the House so they can vote to approve the amended version.

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**Connecticut Chapter**

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

Held on August 28, 2014, the SMA Kid’s Cut-A-Thon in honor of Cubby Wax, SMA type I, took place in Ridgefield, CT. The event raised $1,676 for Cure SMA. Thank you to Erin Simmons and her staff for organizing this fundraiser and for your continuous support.

**Run for SMA**

Thank you to the Yoga Shop in South Windsor, CT for organizing Run for SMA in August 2014, in honor of Emma Goldsberry, SMA type II. Teams pulled together to raise $157, and we would like to thank everyone for their contributions and support.

**Painting Party Fundraiser**

Eva Grace Foell would have turned two on December 28, but she lost her battle with SMA type I on May 1, 2014 at 16 months old. To celebrate her day, family and friends held a painting party fundraiser at a local art shop, The Art’s Desire in Torrington, CT. We completely packed the art studio and also raised awareness for SMA in our community. Thank you to Cure SMA for providing the ‘At A Glance’ pamphlet, bracelets, and balloons to round out the event. Team Eva’s fundraiser raised almost $1,000 which was donated to a local family from Milford, CT who lost their precious son Colton Owens to SMA on December 18, 2014. We were so happy to have Colton’s brave mom, attend the event and paint with us. The SMA community is one that rallies for each other during difficult times, and we are beyond grateful for everything Cure SMA does to bring us together.

Miche Foell
Litchfield, CT

**Georgia Chapter**

**SMA Day with the Braves**

The Georgia Chapter hosted the 3rd Annual SMA Day with the Braves on August 16, 2014. Families were able to take pictures with Braves girls and the famous Braves drum. The Braves beat the Oakland Athletics 4-3, and $2,269 was raised for SMA research! We are looking forward to this year’s game. We hope to see you there.

Anami Lehmann
Mableton, GA

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CHAPTER UPDATE
Greetings from the Greater Florida Chapter!

Stretching from south of Sarasota to the North Florida panhandle, the Greater Florida Chapter is committed to spreading awareness about SMA and fundraising for its cure. In 2014, we exceeded our chapter’s fundraising goal, and we are keeping up the momentum in 2015!

Every dollar raised by our chapter is used to fund SMA critical research and family support programs. Big or small, every fundraiser makes a difference! Since 2009, we’ve raised just about $320,000 in contributions to SMA research and family support programs. By December 2015, we hope to reach the milestone of $400,000 — and we are getting closer every day! Thank you for being a part of our success!

To learn more about chapter events and to keep up-to-date with our latest news, make sure to join the Greater Florida Chapter’s page 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!

Proclamations
Governor Rick Scott formally proclaimed August 2014 as SMA Awareness Month in the state of Florida! Along with the state proclamation, fourteen city and county governments also made public proclamations in support of the Cure SMA Greater Florida Chapter and SMA awareness efforts.

In the city of Lakeland, FL Asher Camp, SMA type I, and his family were recognized and accepted a proclamation before city council. In Plant City, FL Brooklynn Santos, SMA type II, and her family were honored with a similar proclamation.

Thank you to Chapter Vice President Laurie Sore for all of her work with local officials to raise so much support and awareness for our cause!

2015 Greater Florida Board Members
Our executive board of volunteers is proud to serve you! Each of us represents a different aspect of the SMA community, and we are here to offer our support. We currently are looking for a board secretary to join us. If you’re interested in learning more about this volunteer position, please contact us at 727.388.1888 or greaterfl@curesma.org.

President: Audra Butler
(Mother to Andy, SMA type I – Angel)
Vice President: Laurie Sore
(SMA type III)
Treasurer: Shawn Santos
(Mother to Brooklynn, SMA type II)
Old McMicky’s Farm Outing

On October 12, 2014, the Greater Florida Chapter was invited to spend a day at Old McMicky’s Farm in Odessa, FL as part of their 1000 KIDS program. Eight families attended this free event, and children were able to meet chickens, pigs, bunnies, and goats, milk cows, and even ride horses! It was a beautiful day filled with memories to last a lifetime! Thank you to Old McMicky’s Farm for making it possible!

If you are interested in starting a fundraising event in your area, please send an e-mail to our liaison at the Cure SMA National Office, Amber Snyder, and she will help you get started!

Thank you, Greater Florida Chapter, for all of your work to support each other and to fundraise for a cure!

Chapter Walk-n-Roll

The Greater Florida’s Chapter’s 6th Annual Walk-n-Roll, held on November 15 at Lake Parker Park in Lakeland, FL was a great success! With a record number of 21 teams and 231 participants, we raised a record-total of $28,011, crushing our $20,000 goal! Everyone at the Walk-n-Roll enjoyed superhero-themed activities and entertainment - especially the appearance by the Avengers! Following the event, we had a special online sale of our commemorative “Who’s Your Hero?” t-shirts, with all of the proceeds going to Cure SMA!

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22nd Annual Maluko Golf Tournament

For the past 16 years, Chris Leto and Carlos Menendez have donated a portion of the Maluko Golf Tournament’s proceeds to Cure SMA, in honor of Tyler Hernandez, SMA type II, and his family. On October 22, the 22nd Annual Maluko Golf Tournament took place at the Carrollwood County Golf and Country Club in Tampa, FL. One hundred and twenty golfers participated in this year’s event, raising $26,910 for Cure SMA! A huge thank you to everyone involved with the Maluko tournament – you are amazing friends of our cause!

13th Annual Costume Crusade

The Village Early Learning Center in Brandon, FL hosted the Halloween in the Halls event on October 25, 2014. Kids of all ages were treated to a bounce house, Ooey Gooey Room, Haunted Ghost Ship, Not-So-Scary Haunted House, Trick-Or-Treat Lane, and more! The Costume Crusade, held on October 31, 2014 is known for the annual wheelchair race against Mr. Joe. This year Brooklynn Santos, dressed as Anna from “Frozen,” won the bragging rights for 2014! The Millers generously donated $2,557 from The Village’s 2014 fall events to Cure SMA. Thank you to the Miller family and The Village Early Learning Center for their community spirit and their ongoing support of Cure SMA!

LEGOLAND Buddy Benefit Days Donation

As part of their Buddy Benefit Days program for charitable organizations, LEGOLAND Florida offered special park admission tickets at a deeply reduced price to Greater Florida chapter members during August and September of 2014! LEGOLAND Florida sweetened an already amazing deal by donating $15 of each ticket BACK to the chapter, contributing $1,800 to Cure SMA! Thank you, LEGOLAND Florida for your continued support!

Sarasota Mini-Golf Tournament

On November 8, 2014 organizer Timothy Enos hosted the Cure SMA Mini-Golf Tournament at Evie’s Golf Center in Sarasota, FL. Tim learned about SMA from his co-worker, Shannon Hall, whose son, Lucas DiCesare, was diagnosed with SMA type II in 2014, and he wanted to do something to help. He rallied the community around the cause, raising $1,220 at this fun event, which he promises to be the first of many! Thank you, Tim!

Donation in Honor of Maia Shockley

Holy Family Catholic Church in St. Petersburg, FL made a $1,082 donation to Cure SMA in honor of Maia Shockley, SMA type II, in July 2014. Maia attends the Holy Family School, and they are very supportive of our cause. We would also like to thank to Lisa Hoang Shockley, Maia’s mom, for working with the school to organize this generous donation.

Car Credit’s VIP Buccaneers Party

Car Credit ran a competition on their Facebook page asking people to nominate their favorite charity to win one of ten $500 donations, and Cure SMA was nominated by Victoria Rodriguez, mom to Christian, SMA type II! On Friday, January 23, 2015, Victoria and Christian, along with Shirley Lopez and the Greater Florida Chapter’s Vice President Laurie Sore,

Arms for Asher SMA Ice Bucket Challenge

Jeremy and Amanda Camp organized a Gift Registry in honor of their son Asher, SMA type I. Asher’s Ice Bucket Challenge raised a total of $615, and Asher’s mother hopes that the challenge will go viral, and even more funds will be raised to support Cure SMA.

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attended a VIP reception at One Buc Place in Tampa, FL hosted by the Tampa Bay Buccaneers and Car Credit, and accepted a $500 check for Cure SMA! The event, which was held to honor the ten winning charities, featured a live band, the Tampa Bay Buccaneers cheerleaders, and running back Bobby Rainey. Bobby took great interest in our cause, posing for pictures with Christian and also signing one of our 2014 Walk-N-Roll t-shirts! It was a wonderful opportunity to not only raise money for a cure but to also raise awareness about SMA. A huge thank you to everyone at Car Credit for their support and generosity! Together we will find a cure!

PSCU Charity Fair
On August 21, 2014, Katie, Michael, and Allison, SMA type II, Kerns represented the Greater Florida Chapter at a business fair in St. Petersburg, FL. The purpose of the fair was to educate PSCU employees about local volunteer opportunities and ways to support the community. A big thank you goes out to PSCU too for helping us raise awareness of SMA and for making a $100 corporate donation to further our cause!

Ice Bucket Challenge in Honor of Brooklynn
Thank you Shawn and Jayson Santos of Plant City, FL for raising $125 in honor of their daughter Brooklynn, SMA type II, in the SMA Ice Bucket Challenge in August 2014.

SMA Awareness Day with the Tampa Bay Rays
Almost 100 friends and family members came together on August 3, 2014 to watch the Rays take on the LA Angels and to raise SMA awareness at the annual, “SMA Day with the Tampa Bay Rays.” Allison Kerns, SMA type II, and Laurie Sore, SMA type III, were invited to meet players on the field before the game, and Brooklynn Santos, SMA type II, entertained fans during the 6th inning, “Cap Shuffle!” Chapter President Audra Butler talked about the importance of SMA awareness and the Rays support of our cause during a brief interview on Rays Radio too! The Rays also donated a portion of the ticket price to Cure SMA, which totaled $315!

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10th Annual Greater New York Walk-n-Roll to Cure SMA

This year marked the 10th Annual Greater New York Walk-n-Roll. The day was filled with great activities for participants of all ages, including a 50/50 raffle and face painting for the children to enjoy! The rain held out just long enough for a beautiful walk, which was held in memory of Jack Bonelli, SMA type I.

We would like to give a special thanks to all of our amazing teams, participants, and donors for making our 10th Annual Walk a HUGE success. Due to everyone’s efforts we were able to raise almost $31,000!

The support of all of this year’s participants and donors will help fund critical SMA research programs that will lead to a treatment and cure for SMA. These funds will also provide important family support and patient care programs.

An extra special thanks to all of our teams for participating in this year’s event:

Cuevas-Goldstein, Fight For Owen, For The Love of Max, Friends of Julia, JUST Walking for a Cure, Nicholas & Tyler’s Fight Against SMA, Olivia Barbarino, Sweet Baby Jack, Team BAYADA, Team Dylan, Team Farmingdale, Team Juliana, Team Ross, Team Rummel/Morovich, and Team Tansey. Thanks to Debbie Cuevas and all of the volunteers for organizing this amazing event.

We look forward to next year’s event!

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**2014 TCS New York City Marathon**

Congratulations and a huge thanks to all of our amazing runners who participated in the TCS New York City Marathon on November 2, 2014 and helped raise over **$28,800**! We would like to give a special thanks to Michelle Van De Loo for organizing the cheer station for this event.

Thank you to the following Team Cure SMA runners for all of their hard work, both training and fundraising: Jillian Ament, Dina Barbarino, Mandy Casamassima, Shannon Dionis, Rory Johnson, Kresimir Lozina, Katie Posillico, Nathan Rivers, and Paul Schwab.

**Cycle to Cure SMA**

Mandy Casamassima, Shannon Dionis, and first time marathoner, Kresh Lozina all ran this year’s TCS NYC Marathon on November 2, 2014 in honor of Kresh’s daughter Emily. She was diagnosed with SMA type I at eight weeks old. Together, we raised a total of $6,800 in Emily’s honor.

With sore knees, achy backs, incredibly tight quads and overwhelming gratitude, Mandy, Shannon, and Kresh

**NY Mets SMA Awareness Day**

On August 31, 2014, tons of families and friends came out to Citi Field in New York, NY in honor of Maria Verdile, to cheer on the NY Mets and raise awareness for SMA. Everyone had a great day at the Modell’s Clubhouse and got to run the bases on the field after the game. The event raised $5,480 this year for Cure SMA!

A special thanks to the Verdile family and Debbie Cuevas for all of their hard work to organize this event!

**Merrick Avenue Student Council Bracelet Sale Fundraiser**

On November 14, 2014, the Merrick Avenue Student Council in North Merrick, NY held a bracelet sale fundraiser to raise money for SMA. The members of the student council collected $205 during their fundraiser. Thank you to Julia Lincoln and her fellow student council members for supporting Cure SMA!

**Job Fundraiser**

Two years ago I initiated a monthly donation program at my job, and we pick a different organization to support each month. Whoever is interested donates $1 for that particular cause. This August was SMA month. We all dressed in the colors representing your organization on August 22, 2014 and we raised a total of $25.

Best regards,
Eileen Lai
Westbury, NY

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CAPITAL REGION

3rd Annual Capital Region 5K Run, Walk-n-Roll
The 3rd Annual Capital Region 5K Run, Walk-n-Roll was held in Loudonville, NY on September 6, 2014. This year, the event honored a very special member of the Capital Region, Trooper David Cunniff, who was the drive behind the Capital Region satellite chapter and this event. Dave had promised his son Caleb, who battles type II SMA, that he would not stop fundraising until there was a cure. So this year everyone ran, walked and rolled in Dave’s memory and to help him keep this promise.

The event was filled with a fun morning of carnival games, a 5K and Walk-n-Roll, and reuniting with other SMA families. Thanks to everyone’s fundraising efforts, they donated $25,000 to Cure SMA. A big thank you to all of the faithful volunteers who made this event possible, George Whalen of Whalen Chevrolet, Hannah Balta of HB Photography, and Amy Cunniff and the event organizers for hosting an amazing event.

September Community Kiosk Donation Bin
Thank you to members of the Greater New York Chapter - Capital Region in Lake Placid, NY for organizing the September Community Kiosk Donation Bin fundraiser! Participants helped raise $46 to help those affected by SMA.

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8th Annual Kyra’s Idea... Angie’s Hope - Cure SMA, The Ultimate Goal

On September 27, 2014, the Lee and Scadden families hosted their 8th Annual Angie’s Hope fundraiser. The soccer tournament held at Players Indoor Sports Center in Naperville, IL consisted of thirty teams of 8-12 people competing for the championship. Spectators enjoyed DJ, moon bounce, games, a silent auction, raffles, and a concession stand. Fellow students from Angie’s high school were there to support the event, and get participants and spectators excited. Throughout the night, over $32,000 was raised, causing Kyra and Angie Lee, SMA type II, to get closer to their ultimate goal of finding a cure for this disease. Over the past eight years Angie’s Hope has raised over $150,000!

Angie Lee
Naperville, IL

Lace it Up for Karleigh 5K

The first ever Lace it Up for Karleigh 5K was held on September 13, 2014, at the Macoupin County Fairground in Carlinville, IL. The event had a great turnout, and welcomed participants of all ages! The event included a cake walk, games, and raffle, prizes, and raised almost $2,182 for Cure SMA in memory of Karleigh Damm, SMA type I. Thank you to Laura Mix and Kelly Warren for all of your hard work and dedication to help organize this event!

18th Annual Chicago Half Marathon & 5K

Members of Team Cure SMA ran the Chicago Half Marathon & 5K on September 7, 2014, in Chicago, IL. This is the fourth year that Cure SMA participated in the run as an official charity partner. Together, the runners raised over $2,000 to help cure SMA! Thank you to all of our runners for their hard work in training to run this race!

Jeans Day for Charity

Thank you to HealthCare Associates Credit Union in Naperville, IL for hosting a Jeans Day for Charity in December and choosing Cure SMA as the recipient of their donations. The employees at HealthCare Associates Credit Union helped raise $121 for Cure SMA!

Lettuce Wine & Dine to Cure SMA

Cure SMA teamed up with Lettuce Entertain You Restaurants to host Lettuce Wine & Dine to Cure SMA at Saranello’s Banquets on September 26, 2014! The evening included beer, wine, appetizers, delicious desserts by Saranello’s, a live DJ and dance floor for entertainment, as well as a fun photo booth! This event was a great success and raised over $10,000 to help Cure SMA! A special thank you to Jacqueline Staples who nominated Cure SMA as her charity of choice to the Lettuce Entertain You Company and to Saranello’s for donating the venue as well as all the food and drinks for this wonderful event! Jacqueline also planned the entire event from top to bottom so thank you for all your hard work in organizing this amazing event!

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Running for Rob and SMA
This year Anne Marie Castiglioni ran the 2014 Chicago Marathon in honor of her cousin, Rob Cortes, SMA type I. Anne Marie wanted to put on her running shoes and run for something important to both her and her cousin Rob. During this experience, Anne Marie learned a great deal SMA. Rob’s Uncle Donald helped Anne Marie raise funds for this event, and with their combined efforts raised almost $1,707 in honor of her cousin. Thank you to all of the volunteers and everyone who participated in the event! With your help in raising awareness and funds, we are that much closer to finding a cure for SMA!

Small Heroes, Mighty Cause: Cascade 5K for SMA
On August 8, 2014, the Small Heroes, Mighty Cause: Cascade 5K for SMA was held at Cascade River View Park in Cascade, IA. The event, held in honor of Colin Schlemme, SMA type II, was a huge success, raising $40,000 for Cure SMA! After the run, there was a live band in the River View Park Amphitheater. A special thank you to Amanda Schlemme for organizing this event! With your help in raising awareness and funds, we are that much closer to finding a cure for SMA!

18th Annual Beaverdale Beaverdash
The 18th Annual Beaverdale Beaverdash took place on September 20, 2014 in Beaverdale/Des Moines, IA. Despite some rainy weather, the event was once again a success! Hundreds of people gathered to run, walk, or roll. The Beaverdash raised $20,316 for Cure SMA! Thank you to all of the volunteers and everyone who participated in the event! A special thanks to Julia Anderson and Julie Greenwood for organizing another successful event! Keep up the good work and keep raising funds to find a cure for SMA!
**MISSOURI**

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

We would like to thank all members of the Gateway Z Club who worked on or attended the 11th Annual Bommarito Z Club Car show, especially Jill Cummins, Steve Colesworthy, Kathy Goodyear, Rita Schmidt, and the Carpenter Family. Each year this event is in honor of Brittany Carpenter, SMA type II, and in memory of Madeline Schmidt, Brian Goodyear, and Michael Goodyear, all SMA type I. With our collective efforts, we raised over $18,000, in hopes of one day finding a cure for SMA.

**Fight, Cure Glow for SMA 5K/Walk**

The Fight, Cure Glow for SMA 5K/Walk, in memory of Tucker Rutledge, SMA type I, was hosted on August 8, 2014 at Viburnum High School in Viburnum, MO. Runners and walkers of all ages came together to enjoy a fun evening in the dark to help raise funds for SMA! Fun was had by all and $1,973 was raised! Thank you to Autumn Rutledge for helping to organize this wonderful event!

**Missouri Bankers Association Drawing**

This year Mark Blake held a drawing at the Missouri Bankers Association in August, 2014, and the winner was able to name the beneficiary of a charitable contribution. Yet again, Patrick Kussman was the winner of our drawing. Mr. Blake chose to donate the $100 in honor of my granddaughter Ellie Stitzer, SMA type II.

Keep up the good work, we want a cure.
Don Reynolds
Marceline, MO

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**KANSAS**

**Kansas City Chapter**

**14th Annual Cure SMA Race-n-Roll**

The Kansas City Chapter of Cure SMA held the 14th Annual Cure SMA Race-n-Roll on October 5, 2014 at Bishop Miege High School in Roeland Park, KS. More than 400 people gathered to participate in this year’s race despite the cold weather. The event raised $41,231 for Cure SMA. A big thank you to all of our sponsors and volunteers! Special thanks to Kristal Wilson and Kim Sykora for all of the work they did planning this event!

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**Kick 4 Clayton Kickball Tournament**

This year was the first ever Kick 4 Clayton Kickball Tournament. Participants made a donation of $5 and were put on one of the teams participating on event day. This was a fun filled day of family, food, fun, and kickball, created to help raise money to support the work at Cure SMA in honor of Clayton Bryant, SMA type I. Kick 4 Clayton raised $744! With your help in raising awareness, we are one step closer to finding a cure for SMA.

**ARKANSAS**

**Turkey Trot Festival Booth Fundraiser**

On a rainy weekend in Yellville, AR, we raised $850 in a booth at the Turkey Trot Festival. We would like to donate this in memory of Cason Bauss (four months) and Ember Hinson (ten months), both SMA type I. We will not stop fighting for our angels and warriors until there is treatment, or a cure, for SMA.

Mary Bauss, Yellville, AR

**Michigan Chapter**

**Cure SMA 5K - Norway, MI**

The Cure SMA 5K is an event organized in honor of Blake Sternhagen, SMA type II, to raise funds to help find a cure for SMA. The 5K had great weather and an amazing turnout. Organized by Blake's aunt, Carli Kelly, the event took place in Norway, MI on August 9, 2014. The Cure SMA 5K received amazing support from family, friends, and many new faces as well. The Cure SMA 5K raised over $30,000 to find a cure for SMA! A huge thank you to Carli Kelly for organizing this successful event! Thanks to Dennis Lynch for preparing the route for the run, and for helping the event run as smooth as possible!

**Spa-Toe-Pia Pennies for Polish Fundraiser**

The Spa-Toe-Pia Pennies for Polish Fundraiser was held in September, in Dowagiac, MI, in honor of Maggie Sue Kazlauskas, SMA type I! The fundraiser was a huge success, raising $1,000 to support Cure SMA! A special thanks to Jacqueline Hale for organizing this fundraiser!

**Jeans for Charity**

Wright & Filippis, located in Rochester Hills, MI, held a “Jeans for Charity” day on August 1, 2014 to help raise funds for Cure SMA. Each department accumulated a minimum of $3 and due to everyone’s efforts they raised $139. Thank you for your support!

**FitZone Charity WERQ Fundraiser for SMA**

The FitZone Charity WERQ Fundraiser for SMA was held on October 19, 2014, at the Antwerp Township Hall in Mattawan, MI. The fundraiser was held in honor of Brooke and Brielle Kennedy, SMA type II, and attendees enjoyed an evening of dancing, refreshments, and other fun activities, while helping to raise funds for SMA! The evening was a great success, and helped raise over $130! Thank you to Sarah Kennedy for all of your hard work planning this event!

**Chemical Bank 5K & Fun Run**

We had approximately 800 participants in this year’s 10th Annual Chemical Bank 5K and 1/2 Mile Kids Fun Run held on July 26, 2014 in Byron Center, MI. While we are please to provide a community event enjoyed by many, we are even more excited to give back to our race beneficiaries.

In celebration of our 10th anniversary we donated to a variety of organizations, including $500 to Cure SMA in honor of Sydney Potjer, SMA type I, and Isaac Postma, SMA type I. In our ten year race history, we have donated $6,750 to help find a cure for SMA.

We enjoy the opportunity to give back to the community through donations, and we are proud to continue to support the great work your organization is doing to find a cure for SMA!

Scott L. Ellison, Byron Center, MI

**Dowagiac Honor Credit Union Supports SMA**

Dowagiac Honor Credit Union in Dowagiac, MI was proud to support Cure SMA with a $500 donation. Through the Casual For a Cause program, Honor's Dowagiac branch selected Cure SMA as their 2014 charity of choice. Powered By Honor's donation is in memory of Maggie Sue Kazlauskas, SMA type I. The credit union was thrilled to support an organization dedicated to fighting SMA.
9th Annual Ride Away SMA
Ride Away SMA completed its 9th year of fundraising on June 21, 2014, raising almost $26,000. We started raising money to cure SMA nine years ago after losing our daughter Lindsey Anne Ronningen, SMA type I in 2004. (June 27, 2003 – April 27, 2004) Eric Ronningen and Amy Allen (parents of Lindsey) started this organization with the goal of finding a cure for SMA in memory of Lindsey. Ride Away SMA started as a small pancake breakfast in 2005. With the help of so many we have continued to grow adding in raffle prizes, silent auction baskets of every kind, live auction items such as vacations, and more recently the motorcycle ride and apparel sales; all of which have been a great success. A huge thank you goes out to all of our riders and donors, all of our great sponsors, and all of our great friends and family for always being there to help us!

14th Cure SMA Walk-n-Roll Minnesota Chapter
On September 13, 2014, the 14th Annual Cure SMA Walk-n-Roll took place at Silver View Park in Mounds View, MN. There were over 300 registered friends and families who participated in this year’s 5K. The Walk-n-Roll included a bunch of games, raffles, and opportunities to meet other families. During the event, there was also a remembrance held for all of our SMA angels. We would like to thank everyone that helped us! Thanks to your contributions, we were able to raise $15,184! Some of our top fundraising teams were: Team Sophie who raised $2,405, Lynne Denk who raised $2,405, and Evie’s Light who raised $2,004. Thank you to all of the amazing volunteers who made this day possible and the Minnesota Chapter board for all of their hard work planning this event!

Buffalo Wild Wings Fundraiser
Thank you to Bethany Behling, and the Minnesota Chapter, for hosting a fundraiser at Buffalo Wild Wings to raise money for Cure SMA. The fundraiser helped raise $158 to help find a cure for SMA. Thank you!

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MAINE
Sea Dogs Game & SMA Awareness Day
Families affected by SMA were offered special group tickets to attend “SMA Awareness Day” on Sunday, August 3, 2014. Prior to the game, the Portland Sea Dogs recognized all families affected by SMA in a pre-game awareness ceremony. Families also had the opportunity to parade around the warning track, and take pictures in front of Slugger, the Sea Dogs mascot, and the Portland Sea Dogs dugout. The first pitch of the game was thrown by Daisy Bessey, sister of Ezra, type I. Thank you to everyone who came out to support our families!

MASSACHUSETTS
Cure SMA 5K
On Saturday, August 9, 2014, the first Cure SMA 5K Run and Walk was held in Wakefield, MA. With almost 100 runners, and $12,264 raised, it was a successful first year! The route took runners around beautiful Lake Quannapowitt.

Francesca Di Matteo has a cousin, Cristian, type I, who lives in New Jersey and will be 10 years old in February. Through the 5K, Francesca was able to raise awareness of SMA to those running as well as to those who stopped by the Start/Finish. She’s looking forward to having larger numbers, in runners and fundraising dollars, when she hosts the 2nd Annual Cure SMA 5K Run!

8th Annual Fall Classic Cure SMA Charity Golf Tournament
We are thrilled to report that for the first time in five years it DIDN’T rain on our 8th and final Fall Classic SMA Charity Golf Tournament at the New England Country Club in Bellingham, MA, in honor of William Johnson. On October, 3, 2014, golfers gathered to play a full 18 and helped us raise over $59,000 for Cure SMA. Our family is so lucky to have run this event since 2007 raising $350,000 for the cause during our eight year run. We cannot thank the community enough for the support we have received from golfers, businesses and donors through the years. We know that the money we have raised is helping fund vital research in the quest for a cure for William.

Our family thanks you from the bottom of our hearts for your support.
Heidi & Tripp Johnson
Medfield, MA

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CHAPTER UPDATES

DIRECTIONS  Spring 2015

2014 Halloween Hustle

The Tri Sharks Triathlon Club hosted the 3rd Annual Halloween Hustle and Family Fun Run on October 26, 2014 in Shrewsbury, MA. The Halloween Hustle is a themed costume event that features a 5K for runners and a fun run for other participants. Each year our participants have more original and creative costumes, making it a fun filled day! Local volunteers, community leaders and local businesses help to make this event successful each year. Special Thanks to Melinda and Al Apgar and adorable Silas for being our fundraising stars, and to the Sohl Family for volunteering their time! We raised over $10,000 this year, and will continue to hold this event every year to support all SMA families.

The Osborn Family and Matt Pearson - President of the Tri Sharks
Cherry Valley, MA

Dance Away SMA

On Saturday, August 2, 2014, Carrie Capone, SMA type III, held the inaugural Dance Away SMA event at the Townsend VFW Hall in Townsend, MA. For many years, Carrie has had a large team for the New England Chapter Walk-n-Roll, but was unable to attend in 2014 due to another great event, her son’s college graduation! Carrie decided that this was just the push she needed to hold an event she had often thought about – a night of dancing, dinner, raffles and great friends. Carrie and her friends raised $5,510 that night, and she’s already planning great things for Dance Away SMA 2015!

Celebrate Ashleigh Bike Run & Benefit

Our third annual Celebrate Ashleigh Bike Run & Benefit took place on August 2, 2014 at The Jockey Club & Francis Farm in Rehoboth, MA in memory of our daughter Ashleigh Jacques, SMA type I. We had a bike run with several bikers that braved the rain this day. The run consisted of 50 miles, and despite the weather, we had many supporters that attended the event, which we are all very thankful for!

We had an array of delicious BBQ provided by Tennessee BBQ, live entertainment provided by “Just 2 Guys,” and we had the pleasure of meeting Queen Elsa who dazzled the children’s faces with bright colors and designs. The Bike Run & Benefit also included a craft table with angel bracelets, hair ribbons, and beads made by Ashleigh's big sister Brianna and Gramma B, a large raffle table that included great prizes, and a silent auction.

We are all thankful for $3,155 in donations and continued support throughout the years, and we look forward to hosting another fundraiser this year!

Kristen Jacques, East Taunton, MA

Victory for Violet Dance Away SMA

The Victory for Violet Dance Away SMA event was held on August 2, 2014, in Southwick, MA, In memory of Violet Clendenin, SMA type I. The event helped raise over $1,500 to help find a treatment and a cure for SMA! A special thank you to Kelly Clendenin for organizing this event!

Soccer Clinic Fundraiser

Thank you to Elizabeth Hurley for organizing the Soccer Clinic Fundraiser on June 23, 2014. This year’s fundraiser was held in honor of William Johnson, SMA type II, and raised $1,700 for Cure SMA!

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Romanowski Pub Crawl
On Saturday, November 1, 2014, the first Cure SMA Pub Crawl took place in Medfield, MA in honor of William Johnson, SMA type II. Joe and Renee Romanowski organized five local bar stops for the afternoon and evening, and friends came out to support the cause and join the fun; only a brave few made it through all five bars. The rain made for a soggy walk between destinations, but it didn’t dampen the spirit of giving as $875 was raised that day. The Romanowski’s even designed and distributed a Cure SMA commemorative beer glass for those who donated to the cause.

fitZone Charity WERQ Fundraiser for SMA
Thank you to Renee Romanowski for organizing the fitZone Charity WERQ Fundraiser for SMA on November 1, 2014, in honor of William Johnson, SMA type II. This fundraiser raised a total of $875 to help find a treatment and a cure for SMA. Thank you to everyone who participated in this event!

Lowell Spinners Game & SMA Awareness Day
There first ever Lowell Spinners Game & SMA Awareness Day was held on August 10, 2014, at the UMass Lowell Stadium in Lowell, MA! A lot of great memories were made, and the Spinners beat Williamsport 6-5, making the day even better! A special thanks to Jon Healy for helping to organize this wonderful event to help raise awareness for SMA!

8th Annual Ride for Alex
On Sunday, September 14, 2014, the 8th Annual Ride for Alex was held at the VFW in Freetown, MA. It is a day to celebrate Alex and all that he has accomplished. He enjoys meeting everyone as much as they enjoy meeting him. Over the years, this event has raised money to provide Alex with a wheelchair swing, computer equipment to help him communicate with his friends and family, and most importantly a wheelchair accessible van! Because of the van, Alex is able to go places with his family, such as trips to Maine, sledding, and hockey games just to name a few! This year’s event raised $545 for Cure SMA!

Reverites Bowling League Fundraiser
Thank you to Marie Yvonne Taglieri for organizing the Reverites Bowling League Fundraiser on December 12, 2014. The fundraiser was held in Revere, MA and raised $100 for Cure SMA.

NEW HAMPSHIRE
Fisher Cat Game & SMA Awareness Day
There was a great turnout on August 3, 2014, for the Fisher Cat Game & SMA Awareness Day at the Northeast Delta Dental Stadium in Manchester, NH! All of the attendees were able to enjoy a wonderful day of baseball, while making great memories! Thank you to Eric Lesniak for helping to organizing this successful event!

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RHODE ISLAND

5th Annual Working on Walking Golf Tournament and Dinner

Five years ago, a simple idea was created: to have a fundraiser for SMA. In the first year, we weren’t exactly sure what to expect, but as the years progressed, our event continued to grow as more and more people began showing interest in this worthy cause. Alyssa Silva, SMA type I, has been working with a team of remarkable people since this idea was born, and with each passing year, the fundraisers have become more successful and more remarkable.

On August 11, 2014, the 5th Annual Working on Walking Golf Tournament and Dinner Fundraiser took place in North Providence, RI. The morning of the fifth annual fundraiser kicked off with 104 golfers, and following the tournament was a dinner and raffle where hundreds of friends, family, and even strangers came together to show their support for SMA. With 450 people in attendance, last year’s event was the most successful fundraiser yet, bringing in over $47,000 that was directly donated to research for SMA.

We would like to acknowledge and thank the guests who participated in the night’s festivities, the wonderful group of people who worked tirelessly to put together this event, the flockers, and the businesses and people who donated to this cause. We would also like to thank Congressman David Cicilline and Congressman James Langevin of Rhode Island for stopping by. It means so much to have members from Capitol Hill showing their support for this cause. The combination of hard work, determination, and generosity is truly the recipe for something great, and we appreciate everyone’s willingness to join the SMA community in our quest to find a cure. Thank you for being so remarkable, and we look forward to seeing you again this year!

Alyssa Silva, Cumberland, RI

Northern California Chapter

12th Annual Northern California Walk-n-Roll

Plan it and they will come! Our committee did a great job of planning our Northern California Walk-n-Roll on August 23, 2014, and the people kept coming and coming! We were so pleased that we reached more families and have made new friends. We were again in Golden Gate Park, right next to the Conservatory of Flowers, in San Francisco, CA on a beautiful sunny day. On the walk and afterwards we enjoyed connecting with old friends and meeting new families. The kids and adults, enjoyed bean bag toss, badminton, face-painting and balloons characters and we all ate a delicious picnic lunch that was provided by our many sponsors. Before the day was over, we drew raffle tickets for the winners of our iPod, wine and fruit baskets, Star Wars baskets, kid baskets, SF Giants basket, Movie Night Basket, restaurant and event passes, and more. We collected almost $2,300 from the raffle alone!

Thank you to everyone who came out to our 12th Annual Walk-n-Roll and those who supported our walkers! We are thrilled that we passed our goal by raising $62,000! Let’s cure SMA!

Dick and Pat Wolff, Sunnyvale, CA

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4th Annual Cure SMA Sacramento Walk-n-Roll
On October 19, 2014, families, friends, and local supports joined together to bring awareness of SMA to the downtown Sacramento community and surrounding areas. This year’s walk took place in the William Land Regional Park on the Village Green in Sacramento, CA. Dr. John Day, MD, PhD, Professor, Neurology & Neurological Sciences and Director, Stanford Neuromuscular Disorders Program, presented updates from his research and studies. Following the walk, Dr. Day spoke individually with families and friends. The day consisted of face painting, balloon artist, raffles, lunch and friendship.

Charlotte Weber and her committee put together this amazingly successful event in honor of all SMA families and friends. Thank you to the generosity of many donors and volunteers over $4,614 was raised this year!

7th Annual Bay Area Crossword Tournament
On September 13, 2014, 60 people gathered at California State University East Bay, in Oakland, CA to participate in the 7th Annual BACFill Crossword Tournament. Thank you to the generosity of the tournament participants, $1,435 was raised for Cure SMA! We look forward to next year’s event.

Andrew Lawrence
Alameda, CA

New Mexico Chapter

6th Annual “Bugaboo” WOD
On August 23, 2014, the annual “Bugaboo” WOD took place at the Balloon Fiesta Park in Albuquerque, NM. This event was once again a huge success, raising $16,000 for Cure SMA! A special thanks to Laura Hines, Natasha Abruzzo, and their dedicated volunteers for all their hard work in organizing this event.

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OKI Chapter

OHIO

3rd Annual Aubrey Grace Lyden Memorial Golf Outing
Thank you to the Lyden Family for an excellent golf outing! The 3rd Annual Aubrey Grace Lyden Memorial Golf Outing was held on August 2, 2014 in Lake Milton, OH. They had an outstanding turnout with wonderful people, and this year’s outing raised $9,275! A special thank you to everyone that donated money and items for the auction. Dannette and Sean Lyden hold their annual golf outing in memory of their beautiful daughter Aubrey Grace Lyden, SMA type I.

Emmett’s Run, Walk, and Roll 5K
Emmett’s Run, Walk, and Roll 5K was held in Eastwood Metro Park in Dayton, OH on September 7, 2014. A big thank you to Rachael Rodgers for helping to organize this event in honor of Emmett Keeton, SMA type II. Emmett’s 5K helped raise $7,190 for SMA!

US Marshals 2nd Annual 5K and Fun Run
On October 12, 2014, the 2nd Annual US Marshals 5K and Fun Run was held in Mendina, OH! The event raised $2,180 in support of Cure SMA! A big thanks to Ryan Helfrich and the US Marshal Service for doing such a tremendous job organizing this 5K and fun run in honor of Zander and Alexis Helfrich.

Dancing with Lima
A benefit was held October 9, 2014 at the Knights of Columbus Hall in Lima, OH to benefit Cure SMA. Local celebrities were teamed up with professional dancers from area dance studios. Participating studios included The Dancer By Gina, Tanya’s School of Dance, Lyn’s Academy of Dance, and A Step Above. Dancing with Lima helped raise $5,578 for Cure SMA. Thank you to all Dancing with Lima participants, attendees, and event organizers for putting together this event in honor of Cure SMA!

Adyn’s Dream Works To Raise Money for SMA Families
It was during the long drive home from Nashville to Athens, OH, after seeing one of her favorite bands, Old Crow Medicine Show, Adyn Bucher, SMA type I, decided she was ready to start helping other SMA families. She was nine at the time. What resulted was the creation of Adyn’s Dream – a nonprofit organization built to provide money directly to SMA families for expenses such as vehicle and home modifications, medical equipment, hospital bills, travel expenses to the annual SMA conference, etc. It was incredibly important to Adyn to provide this relief to families as they directly impact a family’s quality of life and finances. Last year, Adyn’s Dream held two fundraisers. The first was a benefit show in Athens, OH that consisted of bands, raffles, and a building full of supporters! The second event was an online raffle offering tickets to the famed Ohio State vs. Michigan football game. The $675 raised during this auction was split 50/50 with Cure SMA. The winter fundraiser is getting great local support!

Brian Bucher
Athens, OH

KENTUCKY

2nd Annual Deacon Alexander Memorial Car Show
The 2nd Annual Deacon Alexander Memorial Car Show held August 16, 2014, was a success! Held in Walton, KY, the day included a bake sale, a bouncy house, a mini walk-a-thon, and a makeover booth! The car show had a large crowd, and raised over $2,000 for Cure SMA! Thanks to Amanda Perry for organizing this car show in memory of Deacon Alexander Perry, SMA type I!

Beach 2 Battleship Iron Man
Our family was first introduced to SMA in August of 2009 when our eight-month-old daughter, Sarah McColl, was taken in for genetic testing. After a short hospital stay, Sarah passed away due to complications from SMA. Sarah helped us realize the importance of family and taking time to be together. In honor of her 5th Angel Day,
OKI Chapter

I decided to try running my first ever Iron Man Distance Triathlon. In addition to running, I also I wanted to raise money in Sarah’s memory to help find a cure for SMA. So on October 25, 2014, at 7:30 am, I participated in the Beach 2 Battleship Iron Man Distance Event in Wilmington, NC. I finished that evening, and during the entire race I wore the Cure SMA bracelet that I have worn since Sarah passed. The bracelet gave me the power to finish 140.6 miles. SMA may have taken her body, but her spirit resides in my heart and life forever. Through this race, I was able to raise $1,400 in Sarah’s memory.

Mike McColl, Midway, KY

Elsa’s First Birthday

Elsa’s Aunt, Caitie Brown, has SMA type II, making this a cause very close to our hearts. I cannot imagine receiving a more fulfilling birthday gift than one that provides funding and resources to Cure SMA. Thank you to everyone who donated, and helped us raise $130 to honor Aunt Caitie.

Sean and Karianne Brown, Cincinnati, OH

INDIANA

3rd Annual OKI Chapter Indiana Walk-n-Roll

The 3rd Annual OKI Chapter Walk-n-Roll was another success! The event was held on August 30, 2014 in Indianapolis, IN at Fort Harrison State Park. Friends and family gathered to walk for SMA and enjoy games and the beautiful scenery! Thank you, Michelle Palmer, for another great year organizing this event! Over $19,710 was raised to support Cure SMA this year! Thank you to everyone who donated and attended the walk!

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.

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IDAHO

Flamingo Fun Run

The Inaugural 5K Flamingo Fun Run took place September 20, 2014, in Grangeville, ID. Oakley Smith, SMA type II, a vibrant, loveable, four-year-old with curly red hair was the inspiration behind the 5K Flamingo Fun Run. Oakley’s close family friend, Addie Lutz, a senior at Grangeville High School, was the organizer. Oakley’s favorite color is pink and she loves flamingos. If you ask her about them she will tell you, “they stand on one leg.”

As one of the high school graduation requirements, Idaho seniors are required to complete a senior project that involves at least 20 hours of work. Addie decided that a project to raise awareness of SMA in her small rural town of 3,300 would be worthwhile. With help from lots of volunteers and public officials, the inaugural 5K fun run fundraiser to benefit Cure SMA started to take shape.

With incredible community support, and lots of donated help, in the end Addie logged over 200 hours toward her senior project. The 5K Flamingo Fun Run raised $11,254 toward finding a cure. Addie attributes the success of the project to her fabulous advisory committee, the support of her and Oakley’s families, and the encouragement and assistance offered by the Grangeville community. Local and national businesses contributed money, donations for a raffles, and food for participants. Even though her senior project is complete, Addie is hopeful that this can become an annual event, and can continue to raise hope, awareness, and money to help find a cure.

“What a fabulous event! It exceeded all my expectations! It couldn’t have been better. It was a lot of work, but I met so many wonderful people who were so generous and supportive! I live in a great community! Oakley and I are looking forward to next year’s 5K Flamingo Fun Run.”

~ Addie Lutz

Addie Lutz
Grangeville, ID

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WASHINGTON

7th Annual Pacific Northwest Walk-n-Roll

On September 27, 2014, we held our 7th Annual Walk-n-Roll. The day was perfect – sun and pleasant temperatures brought out over 180 people for a morning and afternoon of family fun and awareness. The Auburn Game Farm in Auburn, WA was a great venue with wheelchair accessible paths and a covered picnic shelter. In addition to a wonderful day, activities consisted of a DJ, games, face painting, raffle, vendors and lunch. Thank you to all the sponsors, generous friends and family! Our fundraising goal was surpassed and we raised $20,000 for Cure SMA!

Kelly Hargrave
Port Orchard, WA

16th Annual Wannabe Cup

On August 16th, the annual Wannabe Cup Charity Golf Tournament was held at the Suncadia Resort in Cle Elum, WA. The event had two days of golf including a first match and final match with closing ceremonies and awards. The event was a great success and raised over $13,000 for a treatment and cure for SMA. A special thank you to Joe Belcher for organizing this annual golf tournament in memory of Skylar Bahrenburg, SMA type I.

Wreaths of Hope

The 9th Annual Wreaths of Hope took place this past December in Washington. Guests at the event enjoyed homemade baked goods and hot cocoa. The event was put on by Sybil Kuhn and raised over $1,000.

Seattle Mariners Game

It was a great day for baseball and SMA on July 27, 2014. The group had a wonderful time cheering on Mariners and enjoying a winning day for the Mariners and SMA at Safeco Field in Seattle, WA. Along with awareness, the group raised $472 to help find a cure for SMA.

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OREGON
Cole P. Randall Memorial Golf Tournament
Our family is just one of many that have received the devastating SMA diagnosis. On August 27, 2012 we joyfully welcomed Cole Parker Randall, the youngest of our three sons. Cole was a happy and seemingly healthy baby but at about five weeks old, Cole experienced some breathing difficulties and a rapid heartbeat. The following two weeks were spent in Pediatric ICU at Randall Children’s Hospital undergoing extensive medical tests that resulted in a heartbreaking, terminal, diagnosis of SMA type I. Cole was given a matter of weeks to live. We struggled to comprehend our new reality and having to break the news to Cole’s two adoring older brothers. Our only hope was to stabilize Cole to the point where he could spend his remaining days at home with his family.

Throughout it all Cole managed to smile, somehow making us all smile as well. He continually amazed those in his presence with his indomitable strength, resolve and spirit. Cole lived out his last four weeks surrounded by the love of family and friends, a mother who did not let him leave her arms and he forged a brotherly bond that will last forever. Cole passed away in our arms in the early morning of November 10, 2012. We carry with us his memory and spirit in our everyday life.

As a way to continue to honor Cole’s life and in an effort to raise awareness in our area and contribute to ongoing research, we held our inaugural golf and auction event at The Reserve Golf and Vineyards in Aloha, OR on September 27, 2014. Thanks to the amazing support of our friends, family and many local business sponsors, we were able to raise more than $74,000 to fund meaningful research through Cure SMA to help fight this devastating disease.

David & Shawna Randall
Portland, OR

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Pennsylvania Chapter

Cure SMA Gala: Sully’s Wish

Friends, family, and members of the community attended the Cure SMA: Sully’s Wish Gala on Friday, September 26, 2014 at the Children’s Museum of Pittsburgh. The fundraiser was organized by Just Ducky Tours, a local Pittsburgh attraction in honor of Sullivan Rossmiller, SMA type I. Sully is a courageous four-year-old boy living with SMA and a member of the Just Ducky Tours family. During the event, Chris D’Addario, co-owner of Just Ducky Tours and uncle of Sully Rossmiller, presented the newest addition to the Just Ducky Tours fleet, Steel City Sully in honor of his nephew.

Guests were greeted at the carnival-themed event by fire breathers, stilt walkers and silk aerialists. Attendees were moved as Lieutenant Fred Cleis and Firefighter Jim Cunningham of Pittsburgh Engine 37 presented Sully and his parents with the proclamation issued by Mayor William Peduto announcing September 26th as Sully’s Wish Day in the City of Pittsburgh.

The event raised nearly $60,000 for research and created awareness for SMA in the Pittsburgh area.

A special thanks to Christina Robertson and the rest of the volunteers who made this event a success: Victoria Cumer, Bri Feindt, Jill Greenwood, Karen Hartman, Cathy Kerec, Alyssa Knierim, Kari Kowalski, Jared Lathrop, Alex McCanna, Lauren Reinshuttle, Shelby Ursu, and Val VanHolt.

6th Annual Zane’s Run

The 6th Annual Zane’s Run was held on September 28, 2014 in Malvern, PA and raised almost $25,000 to help cure SMA! The event was held on a beautiful day and had a great turnout. Zane’s run is organized by Zane’s parents, family, and friends. The proceeds from the event go towards purchasing the New Hope Car Beds and Dream Ride car seats for newly diagnosed families, international CDs to help those understand SMA while living abroad, and other family support programs. Thank you to everyone who has helped make this event a success during the last six years!

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

Hillary and Keith Schmid
Malvern, PA

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Philly Half Marathon - Rock ‘n’ Roll Philadelphia Half Marathon

Runners from across the region, as well as several from across the country, came together in Philadelphia for the Rock-n-Roll Half Marathon to benefit Cure SMA on September 21, 2014. The runners worked hard to raise over $16,000 to benefit the charity and its ongoing efforts. The local Cure SMA Pennsylvania Chapter and the Cannady family organized the event with Sarah Rodriguez and Cure SMA, culminating in an exciting event in honor of all those living with SMA. The group successfully added to the fundraising efforts that seek to support families with SMA and help fund researchers pursuing treatments.


With many thanks for those that participated in all different ways!

Sincerely,
The Cannady Family

5th Annual Dance Away SMA

The Lyla Mertz Foundation held the 5th Annual Dance Away SMA event on October 4, 2014 at the Schnecksville Fire Co. Pavilion in Schnecksville, PA in honor of Lyla Mertz, SMA type I. The dance had a high attendance with guests enjoying dinner, dancing, and live music by the Chas Band. The event also had a Chinese auction, helping to bring the total amount raised to $6,061. Thank you to the Lyla Mertz Foundation for all of your hard work planning this event!

4th Annual Swing for a Cure Golf Tournament

The Swing for a Cure Golf Tournament held on August 10, 2014 at the Mahoning Valley Country Club, Lehighton, PA raised almost $2,980! The event was held in honor of Lyla Mertz, SMA type I. The day was filled with golfing and fun for all that attended. Thank you to the Mertz family for your tireless efforts in organizing such fun and successful fundraisers and for your continuous support for Cure SMA!

11th Annual Lily Kennedy Golf Outing

October 5, 2014 marked the 11th Annual Lily Kennedy Golf Outing at the Iron Masters Country Club in Roaring Spring, PA. Attendees enjoyed a day of golf and outdoor activities, in honor of Lily Kennedy, SMA type I. Thanks to everyone’s fundraising efforts, the event raised $2,400 to help fund research and treatment to help cure SMA. A special thanks to Heather Kennedy for all of her hard work and planning to make this outing a success.

Craft Show

The Craft Show was held on September 27, 2014 in Reading, PA to help raise money to cure SMA in honor of Quinn Comer, SMA type III. A big thanks to Juanita Comer for organizing the show and helping raise $308 that will go towards SMA research!

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COLORADO

Colorado’s Cure SMA 5K Walk-n-Roll & Run
Despite a cold foggy start, on September 6, 2014 over 400 attendees enjoyed the morning with family and friends, face painting, a raffle, silent auction and the musical voices of Colorado School of Mines A Cappella groups at Clement Park in Littleton, CO.

Get ready, get set, GO! With your support, our event raised over $67,000! Thank you to all of our participants, donors, sponsors and volunteers! See you again, next year!

Julie Lino
Denver, CO

SMA Day with the Rockies
Many friends and family members came together on August 23, 2014, for an SMA Day with the Colorado Rockies at Coors Field in Denver, CO! Attendees were able to watch the Rockies beat the Brewers, while making great memories with their loved ones! Over $3,200 was raised during SMA Day with the Rockies! Thank you to Loree Weisman for helping to organize this great event!

MONTANA

Veronica St. Onge’s Birthday
At the end of August, a fundraiser was held in honor of Veronica St. Onge, SMA type I. This event was a wonderful way to celebrate Veronica, while also raising awareness for SMA. The fundraiser brought in $510, and we would like to give a special thank you to Debby St. Onge for all of her hard work planning this event in honor of Veronica.

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Inaugural Cure SMA Gala of Hope

On November 15, 2014, 140 guests gathered for the Inaugural Southern California Cure SMA Gala of Hope in Seal Beach, CA. This fantastic evening raised over $82,000 in support of Cure SMA. Thank you to those that attended, donated and supported this event. A special thanks to the event committee, Liz Bahrenburg, Nicole Lomonaco-Sunde, Nikki McIntosh, and Autumn Montoya, for all their hard work in getting this event off the ground.

One of the highlights of the evening was the presentation of the 2014 SMA Breakthrough Award to Isis Pharmaceuticals. The award was presented by Kenneth Hobby, President of Cure SMA, and accepted by Stanley T Crooke, PHD, MD, who is the founder, chairman, and chief executive officer of Isis Pharmaceuticals.

Thank you to the sponsors who generously supported this event including Isis Pharmaceuticals, Tri-State Medical Supplies, Los Angeles magazine, Orange Coast, Lee & Associates, Lomonaco Designs, Rubicon Gear, SSA Marine, Wallenius Wilhelmsen Logistics, LLC, and Corona Del Mar Physical Therapy.

Barney & Barney Foundation

On Thursday, September 18, 2014 executives from the Barney & Barney Foundation presented Cure SMA with a check for $20,000 to support spinal muscular atrophy research and family support.

Barney & Barney was started in San Diego in 1909 by two brothers. Since then, they have become one of the largest brokerages in the United States. In 2009, to celebrate their 100th anniversary, they created the Barney & Barney Foundation. The foundation has awarded over $1 million to 80 different non-profits in the last five years.

The foundation is made possible by the efforts of Barney & Barney associates and principals. Through their efforts, grant recipients are nominated, selected, and supported via donation drives, volunteer efforts and fundraising events.

Cure SMA was nominated by Tony McIntosh, who is a client executive in Barney & Barney’s Orange County office, along with is wife Nikki Reyes-McIntosh. Tony and Nikki’s son, Miles, has SMA type II.

Paul Hering, managing principal and CEO, and Arleen Lieberman, principal and Barney & Barney “Rock the Foundation” chairperson, presented the check.

Our sincere thank you to the McIntosh family, Paul Hering, Arleen Lieberman, and everyone at the Barney & Barney Foundation for their generosity.

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On October 26, 2014 we held our 6th Annual Inland Empire Cure SMA Walk-n-Roll at the beautiful Rancho Jurupa Park in Riverside, CA. This is an incredible venue for the Inland Empire Walk, which brought out over 250 participants. Throughout the day, fun was had by all with an amazing raffle, a cookout-style lunch, a DJ, and a huge area for kids activities. The morning started out a little cool and turned into a sunny warm afternoon enjoyed by all our friends and families supporting Cure SMA. Thank you to all the teams that participated and helped raise over $10,000.

A huge thank you to our sponsors: Bayada Pediatrics, Quest Diagnostics, Fusion of Ideas, Reylen Construction, LM Promotions, Great Harvest Foods and Ashley Furniture. And a special thank you to Evelyn Vasquez and her committee for another impactful walk to find a treatment and cure for SMA.

See you again next year!

On November 13, 2014, Ruby’s Diner of Tustin, CA held a dine and donate night to bring community awareness to SMA. Lynn and Ade De Blaiso hosted the event at Ruby’s where they welcomed and educated the dining guests of SMA. Overall, the event raised over $430 for Cure SMA in memory of Jaimie Ramsey, SMA type I, granddaughter of Lynn and Ade. Thank you for your continued support!

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South Florida Chapter

4th Annual Gala of Hope
On Thursday, October 2, 2014 Jennifer Miller Smith and Fiorenna Israel, two dedicated mothers of children with SMA, planned an evening to be remembered in honor of their daughters, Madison Smith and Mia Israel, SMA type II.

This year’s 4th Annual Gala of Hope is a South Florida fundraising event with one goal: To fund a cure for Spinal Muscular Atrophy. The event helped raise over $110,000.

During the evening-long event, Shaina Rappoport, mom to Jacob Rappoport, SMA type I, shared her personal story of loss and courage, inspiring the 300 attendees to donate another $20,000.

Entertainment was provided by South Florida’s own DJ Laz, TrueVibe, and Salvador LIVE!, a performance artist, break dancing-speed painter whose unique style had the partygoers in awe. Giant-sized rabbits, talking trees and trapeze artists appeared throughout the night at this Alice in Wonderland themed event. Guests were able to participate in a silent auction, and a premier raffle for the chance to win a weekend in a Maserati along with a $5,000 shopping spree at J.R. Dunn Jewelers.

Our deepest thanks to Jennifer and Fioreenna, and their husbands Aaron and Aldo (respectively), for all their hard work and creativity. Thanks also to Ferrari Maserati of Fort Lauderdale for hosting the event, The Capital Grille and Premiere Beverage & Catering for providing the food and drinks, and to Galaxy Productions for providing the evening’s entertainment.

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South Florida Chapter cont.

Home Runs that Help
This December, I participated in the 9th Annual International Power Showcase at Marlins Park stadium in Miami, FL. The Power Showcase program hosts 200 athletes from around the world providing them with the opportunity to demonstrate their tremendous talents in the Home Run Derby and All World/All-American Classic to an audience of the nation’s top scouts.

The philanthropic arm of the Power Showcase is Home Runs That Help, a community outreach initiative pairs players with organizations or individuals to raise money for their cause. In memory of my little sister, who had SMA type I, I have partnered with Cure SMA. $600 was raised in her honor, and truly, there is no amount too small and every donation helps. Thanks for your support and generosity!

Dominic Iagrossi, Jr.
Deerfield Beach, FL

South Jersey / Delaware Chapter

SMA Golf Tournament in Memory of Steven Moyer
The SMA Golf Tournament was held on July 18, 2014 in memory of Steven Moyer, SMA type I. The tournament was held at Blue Ridge Trail Golf Club in Mountain Top, PA, and included a day filled with golf, a buffet, and prizes as families came together to raise money to help cure SMA! The event was very successful, and raised almost $4,400! A special thanks to Steve Moyer, Steven’s grandfather, for organizing this event.

Dress Down Fridays
Dress Down Fridays became a routine at the Municipal Government Center last summer. The event was organized by Michael Gonnelli in memory of Daniel Cevallos, SMA type I. Thank you to all who participated in this event!

Tennessee Chapter

3rd Annual Tennessee Walk-n-Roll
The 3rd Annual Tennessee Walk-n-Roll Away SMA event was held on September 13, 2014, at the Ijams Nature Center in Knoxville, TN. Attendees participated in road rides, guided mountain bike rides and walks through the Ijams Greenway. The event also included a lot of activities for the kids such as face painting a treasure hunt, and an insect zoo! Thanks to the generous donors of this event, we were able to raise $8,516 for Cure SMA! Thank you to Sarah Boggess and Denita Guerry for organizing this event.

Texas Chapter

The 4th Annual Bowl-a-Thon
The Texas Chapter hosted their 4th Annual Bowl-a-Thon on August 9, 2014. Families came from all over Texas to enjoy an afternoon of bowling, balloon art, face painting, silent auction and FUN in Austin, TX! The event, which includes a silent auction, raised over $20,000. The event concluded with participation in the annual SMA candle lighting remembering those that have lost their battle with SMA and honoring those individuals and families that still live with SMA every day.

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

Note: The amounts raised and shown are totals as of February 9, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Kure SMA Day Craft & Vendor Fair
The 2nd Annual Kure SMA Day Craft & Vendor Fair was held on September 21, 2014 at the Harrison Street Market in Endicott, NY. Attendees were able to enjoy local crafters, vendors, food, music for all ages, door prizes, basket raffles and auctions, mascots, face painting and more! Thank you to Sierra Kulas of Port Crane, NY for coordinating this event and raising $2,140 for Cure SMA!

Kure SMA Knight with the BMETS
On August 22, 2014 the Kulas family held the very first Kure SMA Knight in Binghamton, NY. The family organized fundraiser sold 100 tickets, held a wide variety of raffles, sold t-shirts commemorating the event, and more! The Kulas family’s fun-filled night helped raised $761.

WNY SMART Walk for a Cure
Once again the Western NY Chapter of Cure SMA had a picture perfect day for the 11th Annual WNY SMART Walk for a Cure. About 350 participants walked in their bright blue walk shirts or team shirts along the walk ways in Beaver Island State Park in Grand Island, NY. Some walked, some ran, some rode bikes or scooters, and some were pushed. All agreed it was a beautiful day. The event started with a welcome by Bonnie Shiesley, Chapter President. She told the attendees about the agenda for the day, which included raffles, a basket auction, games, balloons and a clown. Large ticket items like bikes, autographed sports items, large gift certificates, along with almost 300 baskets were given out to the lucky winners.

During the event, Diane Blair, Chapter VP, did a recap of the walk history, mentioning that this event has raised almost $250,000 during its 11 year history. Participants were able to purchase Cure SMA memorabilia and look at pictures from previous events. Almost $60,000 was raised for research and family services and we would like to thank every individual that attended the 11th Annual Walk for a Cure. We look forward to another successful event next year!

Karen Shiesley
North Tonawanda, NY

Insane Inflatables
Kendall Wilson ran a 5K on September 28, 2014 in Syracuse, NY called Insane Inflatables. Insane Inflatables offered a special code that people could enter when they registered to direct part of their registration fee to a charity. Kendall had six people donate to raise $30 for Cure SMA! Her donation is in memory of Jubilee Hope Saville, SMA type I. Thank you for your contribution Kendall, every bit helps!

7th Annual Macy’s Shop for a Cause for Cure SMA
Chapter members sold $5 savings passes to be used at Macy’s stores nationwide on August, 23, 2014 to help raise awareness for SMA and funds for research. Thank you to Diane Blair of Hamburg, NY for coordinating this event and raising $25 for Cure SMA!

Note: The amounts raised and shown are totals as of February 9, 2015 and may differ from current fundraising totals by the time you get this newsletter.
10th Annual Grant Sheppard Memorial Scramble

On Friday, August 22, 2014, the 10th Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Chilton, WI. This golf event is in memory of Grant Thomas Sheppard, SMA type I.

The golfers and volunteers had a fantastic day filled with lots of fun and laughter. Everyone had a great time golfing and at the silent auction and bucket raffles helping to raise money for Cure SMA. In total, the event raised over $9,000, bringing the total for ten years to over $100,000!

A major thank you to Scott, Lisa, Peter and Lily Sheppard for all of their hard work in planning this event year after year! With your dedication to organizing this event each year, we are that much closer to finding a treatment and cure for spinal muscular atrophy.

Scott, Lisa, Peter and Lily Sheppard
Sherwood, WI

4th Annual Kennady’s Dream Walk-n-Roll

The 4th Annual Kennady’s Dream Walk-n-Roll was held on September 21, 2014 in Brookfield, WI. The day included a walk, lunch, raffle, and youth activities. The event was a success, raising over $13,400 for Cure SMA, in memory of Kennady Quinell, SMA type I. Thank you to Erin and Corey Quinell for all of the time and dedication they put into this event.

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Tori Stevens Memorial Night
In honor of a Tomahawk racers biggest fans, August 15, 2014 marked a special day at Eagle River Speedway, in Eagle River, WI as a memorial race was held in honor of Tori Stevens, who passed away November 25, 2012. Tori was an avid race fan, and some of her favorite drivers included, CJ Hedges Jr., Mike Brown, Tyler Muscloff, and Derek Eberl. During a race, you could always find her at the fence waving to her favorite drivers, making this event was the perfect way to honor Tori. Donated bikes, goodie bags, and backpacks were passed out at the race to pay tribute to the young race fan. The Tori Stevens Memorial Race included trophies in multiple classes that had her picture and name on them. Over $1,600 was raised in honor of Tori. Thank you to all of the time and effort put into making this event a success.

Annual Hoffmann Family Block Party and Raffle
The Hoffmann Family held another Cure SMA Raffle this year at the Annual Hoffmann Family Block Party on September 6, 2014 in Milwaukee, WI. The fundraiser is in honor of Jackie Hoffmann, SMA type II, and included selling raffle tickets and hanging out with friends and family as they raise money to cure SMA. This year’s event was a success, raising over $600 for Cure SMA! Thanks to Paula Hoffmann for organizing such a successful fundraiser and for all your hard work in raising funds for Cure SMA!

Peoples State Bank Fundraiser
Peoples State Bank raised $609 in memory of Kylah Ann Schulz, SMA type I, who lost her battle to SMA in August of 2011. I work with Kylah’s mother, Heather, at Peoples State Bank in Wausau, WI. We held a raffle recently and the money raised from ticket sales would be donated to the winner’s charity of choice. I won the raffle, and was thrilled to pick Cure SMA for Heather. I am so happy the winnings will be going to a cause to help find a cure for SMA!

We pray for a cure!

Dawn Borchardt
Wausau, WI

Riverview Hospital Jeans Day
Employees of Riverview Hospital in Wisconsin Rapids, WI, started SMA Awareness Month with a “Wear Jeans for SMA” fundraiser on Friday, August 1, 2014. Employees made $5 donations and received a Cure SMA bracelet, which allowed them to wear jeans to work that day.

Thanks to everyone’s participation, $415 was raised for Cure SMA!

Eau Claire Fundraiser
These checks and donations were written at Christmas by our physical and occupational therapy department. We selected Cure SMA as our organization of choice to honor Connor Holdsworth, who passed away last April from SMA. Connor’s family is from Eau Claire, WI, and chose Cure SMA as a memorial. Together we raised $275 for Cure SMA.

Peggy Murray & the Eau Claire Area School District Therapists
Eau Claire, WI

La Crescent-Hokah Elementary Fundraiser
La Crescent-Hokah Elementary fourth graders from La Crosse, WI participated in a project offered by the La Crosse Rotary Lights to celebrate the 20th year in December 2014. Area fourth and fifth graders designed barrels, and La Crescent’s fourth grade barrel was the winner. Rotary Lights awarded the winning barrel a $100 donation to the charity of their choice, and the fourth graders chose Cure SMA to receive their winnings.
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!

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

Merchandise
Dear Cure SMA friends,

Ben, now 11-years-old, was chosen as a member of US Junior Team to attend the 5th Annual World Math Team Championship in Beijing, with more than 15 countries in attendance. Majority of the students are from Asia, where they usually have much higher math and science standards. Ben received a gold medal, the only one gold medal on the US Junior Team; he ranked 13th out of 271 contestants, which is not easy since he can’t write and has to process everything in his brain, and he was tired from the long flight.

Here are some links to the UT and NBC San Diego reports on Ben before he went to Beijing:


http://www.nbcsandiego.com/video/#!/on-air/as-seen-on/Poway-6th-Grade-Math-Whiz-Headed-to-Beijing/282770661

There were a lot of major newspaper reports and major news media who followed Ben’s every step from different Chinese news media outlets. Ben was the center of opening ceremony and award ceremony. Ben also made a speech during the award ceremony. He gave a very good speech where he asked for more public attention and fair education rights to his SMA brothers and sisters. At the same time, the two big projectors played the pictures with more than a hundred SMA kids making a wish for Ben and requesting more attention for SMA.

A lot of media was involved. He also received a special award from the WMTC committee. Because of Ben, the competition has received more attention than ever. The weather in Beijing was very cold, but Ben took some opportunity to tour the Great Wall and Tian An Men Square again.

Thank you!
Jenny Huang

Lemonade Stand Fundraiser

Thank you to Addie, Molly, Will, and Ella for selling lemonade during July in honor of Cure SMA! The fundraiser raised $106 for Cure SMA. Your donation is greatly appreciated!

Lemonade Stand

Thank you to Emma Garrett for setting up a lemonade stand to raise money for Cure SMA! During August, Emma raised a total of $210 in honor of Dylan Cuevas, SMA type I. Her donation is getting us that much closer to finding a cure.
Local Long Island Girl Scout Makes a Difference One Restaurant at a Time

Zandra Genovese, a young Long Islander and Girl Scout is making a huge difference for those people who use wheelchairs for mobility on Long Island. What started as a Girl Scout's Project has quickly expanded and is gaining the interest of people and restaurants across New York State. Zandra is living with Spinal Muscular Atrophy; due to this she uses a wheelchair to help her get around. She joined the Girl Scouts to make a difference, ask anyone who knows Zandra and they will tell you she certainly has. Currently this bright, young, Long Island woman is working on achieving the Girl Scout Gold Award. This is the highest award a Girl Scout can receive. As a member of troop 2812, each girl was asked to work on a services project as part of the Girl Scouts of Suffolk County Service Unit.

For Zandra, this project was an opportunity to make Long Island communities more wheelchair accessible, restaurants especially. She created an accessibility guide for others in wheelchairs (Veterans, teens/kids, elderly, etc.). Along with the guide she also created her own rating system based on her personal experiences and opinions of the restaurants she has visited (not related to American with Disabilities Act, or ADA). This rating system is comprised of 5 categories related to wheelchair accessibility including parking, entrance, table space, bathroom space and the accommodation of the staff.

Since her project began Zandra has rated 30 restaurants in her local county in New York. Three of these restaurants belong to the DeNicola Brothers. The Ruvo and La Tavola restaurants are excited to share and help Zandra on her journey. “I have known Zandra all her life. She is a true fighter and a really inspiring, wonderful young woman.” Said Joseph DeNicola, one of the restaurants owners and friend of Zandra’s family, when asked about Zandra and her work.

After receiving the Gold Award, she plans on expanding her project to other counties and cities across New York State. Her goals do not stop there however; Zandra has expressed even wanting to expand the project across the country.

Her website, www.zrag.net allows people to view each rated restaurant, read her comments, and even view photos. Having this website allows her to have a platform where she can continuously help others in wheelchairs by sharing her experiences.

This was featured on “Heroes on Our Island”.

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.
“Tell me a story about my disease mom.” says Paul my 6 year old son. It has become part of our bedtime ritual for me to tell him a story about my childhood. He finds these stories of my youth both funny and fascinating, to imagine I was once a kid like him. Well that or he’s just trying to stall bedtime. Then he goes and drops this bomb on me.

Dear Son let me start at the beginning...

From the moment your tiny soul entered my body I knew. I knew in a way that couldn’t be explained. I had yet to take a pregnancy test, but even if I did it would have been too soon to tell. So I bided my time and a week before I even took the test I told my sister-in-law, I was pregnant! I couldn’t keep a secret. I had no doubts at all -it was a secret knowing that I can never explain, I didn’t have it with your sisters, they both took me by complete surprise. But with you everything was different from the very first moment.

You were my third and very last pregnancy and I was completely sure you would be my third little girl. I was going to name you Summer Faith. Since I am not one to wait around- I found out that you were going to be a boy around 5 months into my pregnancy. “Yup he’s got a stem!” is what the ultrasound technician told me. It took a few seconds for it to sink in what those words really meant. “What you mean it’s a boy?! Are you sure?!” I asked her. “Yup it’s for sure she assured me, you’re having a boy!” I was both thrilled and surprised and I couldn’t wait to tell your Dad. I called him in the parking lot of the Dr.’s office before I left, to tell him the news, “You’re gonna have to build that tool shed I told him because we’re having a boy!”

Fast forward 9 months and you were due to make your arrival on March 27th 2008. Both your sisters couldn’t wait to be born and they both made their arrival into this world a week before their due date. So it was decided that I would be induced at 40 weeks if you didn’t come before then- which you did not. The first thing that was different about your birth is that my epidural did not work in the way that it was intended. Every time the pain medication was pumped into my spine, my head filled up like a balloon and it felt like everything was underwater. (perhaps an omen of things to come) After 2 times of this I spoke up and told them I didn’t want any more, it wasn’t working. So as it happened I felt EVERYTHING. And to my complete and utter shock it was nowhere near as horrible as I imagined. The second thing that was different with you is that you were born crying! Both your sisters had their cords wrapped around their necks and they were silent. You came out and let us know you were there! As the nurses cleaned you up and you wailed I was silently screaming in my mind, “GIVE ME MY BABY!!!” I needed to hold and comfort you in a very primal way. After what felt like an eternity you were placed on my chest and I finally met you for the first time. My beautiful baby boy. After some talk we decided to name you after your 2 grandfathers Paul Thomas, two classic strong names. From the moment you were born you did not like sleeping alone. As all the visitors left and it was just you and I, every time I placed you in your little clear baby tub, you screamed. So that very first night, and almost every night since, you would sleep curled up on my chest peacefully and soundly.

You grew up quick, and did all the things that babies are supposed to do, when they are supposed to do them. You rolled over, sat up, walked, and then pushed stuff around incessantly all on schedule. You were my perfect little man.

You were a little over 2 years old when we were on our much anticipated family trip to Storyland in NH. You had just gotten over a fever and were fever free for the first few days before we left. After we arrived at the Christmas Farm Inn you were sitting peacefully on the couch, not joining your sisters in their exploration of our room. You were tired, very tired. It was a level of fatigue that I had never seen before in either of your sisters, ever. That was the first time the cold blade of fear pierced my heart, and I heard that faint whisper in my mind..."Something is not right."

Soon you recovered and were back to your normal self and I forgot I ever heard that voice and felt that fear. Life went on. Your Dad noticed it before I did. He thought you walked funny. “Nah” I would dismiss him “he’s only 2 he’s still learning.” But then you started falling, a lot, and with no apparent reason. It was as if your legs just gave out. Even then I still wasn’t concerned.

It was at your 3rd birthday party that everyone was commenting on your

continued on next page
“funny walk” that I started to take it seriously. So I took you to the Dr. who at that time told me it was just because you had flat feet. You would grow out of it, she told me. With that news I was completely satisfied. I had an answer, a nice easy one. At this time I was still working. Looking back I think it was because I spent most of the day away from you that I didn’t truly see the signs that were right in front of me. Very soon after your 3rd birthday I made the choice to leave work and stay home with you and your sisters. This was when the true reality of the situation really hit home for me.

As I spent my days with you it became very clear that something was definitely not right with you and it was not flat feet. Your quadriceps didn’t work. That was my first realization. You went up the stairs stiff legged and wouldn’t bend your knees, you locked them instead. Then I noticed that when you got up from the floor you did it like a baby giraffe. You would split your legs wide, lock your knees and sort of slowly get up from that position. The truth kicked me right in the stomach when we were at a party and I saw a little boy younger than you climb in and out of a sandbox with ease, then pick up a bat, assume the little half squat position to hit, and I realized you could do none of those things. Now I was concerned. I spent hours researching your symptoms on the computer and the one thing that kept popping up was Duchene Muscular Dystrophy. My heart went ice cold. It was then that I began what was to be the worst summer of my entire life.

After a few more rounds of Dr. visits, and an ER visit with what was just supposed to be a test, that turned into 4 hours of seeing every, nurse, intern and med student, that was on shift that finally we saw the Dr. who would utter these awful words, “I think its Duchene Muscular Dystrophy” he told me calmly. “We’ll take some blood and the results will be in, in a few days I’ll call you.” I couldn’t believe he just said that. There’s no way this is happening I thought to myself. Those 3 days passed quickly and I finally got the call, that it was in fact, not Duchene.

I could breathe again, you would live.

Thankfully from there I only had to endure a few more months of waiting before they finally figured it out. I was sitting in Dr. Kang’s office at Boston Children’s Hospital. It was a beautiful sunny summer day. We had just finished a muscle EMG test on you in which they would stick needles into your tiny muscles to see if the nerve impulses were intact. You screamed, loudly, as I held your tiny body trying to keep you still. He stopped soon as it became clear this was actual torture for you. As I sat there rocking your little body trying to comfort and calm you and even myself, that he said “I think Paul has SMA.” But don’t worry when you look it up it’s not Type I, I think he has Type III.” Now I’m sure he said more stuff after that but that was all I heard. As soon as I got home I found the Cure SMA website. They described the disease in simple easy terms, when I read that there were different types, I skipped ahead to the symptoms of Type III. I sat in shock as I read symptoms that described you to a T. In that moment I knew you had SMA. My heart sank as I read that there was no treatment and no cure. I wanted to stop myself but I couldn’t. Type I = fatal before the age of 2. Type II = never walk and have a shortened life span due mainly to respiratory complications.

Oh. My. God.

I had to shut it down from there I couldn’t read anymore. I don’t want to be a part of this. I don’t want to be a part of Cure SMA. I will wait until I have confirmation before I go on this webpage again. For now I still have some hope that maybe whatever is wrong is fixable. (but I’m kidding myself and I know it)

3 weeks later, I was back on the website ordering a care package of toys for you and information about SMA for me. The months after that passed in a blur. I was relieved that I had an answer. That was all I could hold on to. That feeling I had of not knowing was like I was walking around with lead in my stomach. I was heavy, all day every day. And now that I knew what the problem was it didn’t go away. I lied to everyone. I told them I was fine and that I was glad I knew what
it was. I lived in a state of disassociation for months. I was seemingly normal going about the daily tasks of life, yet I was different. My heart was broken. People would unknowingly say things to me in an attempt to be helpful that would shatter my already broken heart into even smaller pieces. Nobody understood. I felt alone and angry and scared. It stayed that way for 6 months after SMA had become a part of my vocabulary. It wasn’t until I was visiting your Auntie Sarah on my birthday that it all came out. I finally let go. I sobbed. I cried and I sobbed some more. It was a release. That was my first step on the path towards healing. After that I felt lighter. I was starting on the journey of what was to be my life. It would prove to be a very rocky and difficult terrain I was about to discover.

For a long time I felt no-one understood me or what I was going through, and it made me feel separate, resentful and angry. I felt like a lot of people had let me down but I could never explain why or how. I was changed in ways I can never explain, I was still me, but in many ways I was unrecognizable to myself. Part of me had died. But then something switched, I was awakened, and a new part of me was re-born.

It’s strange how it happened, but it was as if I finally opened my eyes to what was actually happening around me rather than buying into my own version of events. No one has let me down. I have been touched by beautiful acts of kindness all along but I was too wrapped up in anger to see any of it.

This year, the year you got your first wheelchair, an event that I thought would destroy me, but it didn’t. It was strangely uneventful, a seemingly natural progression of things to come, because this is our new life, a life in which those three little letters have changed the course of our lives forever. This year especially many people came together and in ways both big and small and moved me with their kindness- it is what finally opened my eyes and heart to the truth.

It doesn’t matter if people understand what I am going through. How could they? How could I even expect them to? What was happening was this. Whenever there was something you or I needed, we got it. Sometimes people I would never expect would humble me with their thoughtfulness. Every year people came to our events and helped without my asking. What they were all saying was this “I care. “ I support you.” And it wasn’t until this year that I finally heard them.

There are times I feel overwhelmed by the prospect of raising you. Am I equipped to help you deal with all of the challenges that you will inevitably face? Can I teach you to be a strong, independent man? Am I strong enough? Can I do this right? The future scares me and I try not to think about it. But then I think of you, right now, your perfect beautiful 6 year old self. And you say to me “I wish I could fly so you wouldn’t have to worry about my legs hurting.” And then you say “I don’t like that God made me with a disease, but I’ll go with it.”

And then I can see the truth. You are the strong one. Maybe you were sent to teach me.
Publications From Cure SMA

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.
Together
We Can

Cure SMA is our name, and it’s also our rallying cry. It’s why we keep funding research and family support. And we know it’s why our community gives so much to host events, raise awareness, lobby for policy changes, and support each other.

Together, we can cure SMA
August is SMA Awareness Month

Be sure to check back with the CureSMA.org website for the latest news & happenings going on during the month of August