<table>
<thead>
<tr>
<th>Topic</th>
<th>Page</th>
</tr>
</thead>
<tbody>
<tr>
<td>2016 Annual SMA Conference Info</td>
<td>4</td>
</tr>
<tr>
<td>Family Support</td>
<td>32</td>
</tr>
<tr>
<td>Legislative Updates</td>
<td>35</td>
</tr>
<tr>
<td>Clinical Trial Updates</td>
<td>38</td>
</tr>
<tr>
<td>Fundraising and Chapter Updates</td>
<td>82</td>
</tr>
</tbody>
</table>
VISION AND MISSION STATEMENT

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

OUR VALUES

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

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

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

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

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

Determination
Our work is not done until we have a treatment and cure, and we’ll remain strong in our fight no matter what challenges come our way.

The production of this newsletter was supported by a grant from Biogen.

On the Cover: Kiley McClay, SMA type II and Eleanor Bolton, SMA type II

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

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

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

Change of address
Send changes, including ZIP code to: info@curesma.org or call 800.886.1762 or mail to: Cure SMA, 925 Busse Road, Elk Grove Village, IL 60007

©2016 Cure SMA. All rights reserved.
No portions of this publication can be reprinted without written consent from Cure SMA.

Cure SMA does not support or endorse any particular treatment or therapy. Information contained in this newsletter should not be used as a substitute for consultation with a qualified healthcare professional.
We are very excited to announce that Dr. Mary Schroth has joined us as our medical professional education consultant. She will be working with us a few days each month, depending on her clinic schedule, as Mary remains deeply committed to working with her patients. Mary will also continue to chair our Medical Advisory Council.

Mary will oversee our patient care area, which includes resources like our care series booklets. However, we expect that her expertise will be most beneficial as she helps us advise and educate other medical professionals on SMA. In fact, this will be her primary area of focus as she works with us.

Our research progress means that care is more important than ever. While we remain focused on ultimately developing a cure for SMA, it’s likely that the first step will be treatments that slow or even stop disease progression, thus extending lifespan for those with SMA.

As more and more individuals live longer and longer, we’ll need more and more doctors who understand the complexities of SMA, and can provide proper care for our community. Mary’s experience and dedication will be key in helping us prepare for and meet this next challenge.

If you’re interested in connecting your healthcare provider with our educational opportunities, send us an email at info@curesma.org.
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 meet & greet and carnival, teen and adult social activities, 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 and the Research Conferences 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 annual conference also provides the children an opportunity to make new friends and have a great time. We are expecting another great attendance of over 1,400 attendees.

The Disneyland Hotel in Anaheim, CA has been carefully selected to meet the many needs of Cure SMA as the 2016 destination for the Annual SMA Conference. The Disneyland® Hotel first opened in 1955 as the place to be and be seen. More than 50 years later, the hotel that started it all is back, re-imagined for a new millennium. You’ll enjoy the hotel’s 975 newly renovated guest rooms which are non-smoking and offer great features – like refrigerators, coffee makers, flat-screen TVs and wall safes—as well as, complimentary Internet (wired and wireless) in every room, and complimentary weekday newspapers available on every hotel floor. After a day of meetings, indulge in outstanding restaurants and lounges (like the one-of-a-kind Trader Sam’s—Enchanted Tiki Bar!), relax in new Downtown Disney® District—all just steps from your room.

You must complete your conference registration with Cure SMA prior to reserving your room at The Disneyland Hotel for the special room rate of $157 per night.

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 (ETMF) is to generate substantial funds to be able to provide conference scholarships, allowing newly diagnosed SMA families/individuals the opportunity to attend the Annual SMA Conference. Our goal is to allow as many newly diagnosed SMA families as possible to attend and experience the benefits of this amazing conference. The meeting gives families the opportunity to gather critical care and daily living information early after diagnosis, learn directly from experienced SMA physicians and 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 and 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 share the details of your new and existing products with several SMA 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

SPECIAL CONFERENCE THEME PARK TICKETS

Come for the conference, and stay for the magic! Make the most of your free time with special Disney Meeting and Convention Theme Park tickets. Annual SMA Conference attendees are eligible for advance purchase of specially priced discounted Disney Meeting and Convention Theme Park tickets.
5th Annual Hope on the Hill Congressional Dinner Advances SMA Advocacy

On December 1, more than 120 people from the SMA community—including government, and industry partners—gathered for the 5th Annual “Hope on the Hill” Congressional Dinner. Several Members of Congress attended the dinner, including Speaker of the House Paul Ryan, Congressman Sean Duffy, Congressman Richard Hudson, Congressman Bill Huizenga, Congressman Erik Paulsen, Congressman David Schweikert, and Congressman Greg Walden.

The evening included remarks from Speaker Ryan, Congressman Walden (OR), and Cure SMA Board Member, Greg Zerzan.

As more SMA drugs reach late stage clinical trials, we remain focused on educating, involving, and engaging all three groups in our community: the patient and family group, the government and regulatory group, and the biotech and pharmaceutical companies that are developing and testing SMA drugs. We recognize that we need the whole community working together in order to develop safe and effective treatments for SMA and get them approved as quickly as possible.

The Hope on the Hill event allows us to further our current advocacy work (see links below)—including our FDA Engagement Initiative, the Voices of SMA, 21st Century Cures/Innovations, and more—as well as learn from successful advocacy efforts such as the passage of the Ensuring Access to Clinical Trials Act.

Learn more about the Congressional Dinner:
http://www.curesma.org/news/5th-annual-hope-on-the-hill.html
http://www.curesma.org/news/5th-annual-hope-on-the-hill-recap.html

Learn more about our current advocacy work with these extra links:
Our Industry Partners

Our thanks to the corporate and industry partners who made this evening a success.

**Discovery Sponsors**
- Biogen
- Quest Diagnostics

**New Direction Sponsors**
- Genentech
- National Association of Broadcasters (NAB)

**Benefactor Sponsors**
- AveXis
- CTIA
- McGraw Hill
- PHRMA
- PNC Financial

**Hope Sponsors**
- Goldman Sachs Group
- Altria
- Biotechnology Industry Organization (BIO)
- Comcast
- Cytokinetics
- Fluor Corporation
- Independent Insurance Agents and Brokers of America
- Viacom

**Supporting Sponsors**
- The Cypress Group
- Drinker Biddle and Reath
- iHeartMedia (previously Clear Channel)
- Intercontinental Exchange
- National Multi Housing Council
- PG&E
- PricewaterhouseCoopers
- Sarepta Therapeutics
- CME Group
- FIA

**Friends of Cure SMA**
- Assurant Inc.
- Eastman Chemical Company
- Kevin MacMillan
- U.S. Bank
The 2015 Annual SMA Conference Was a Huge Gathering for the Midwest

The 2015 Annual SMA Conference was the 2nd largest conference to date and the largest attendance for any Midwest conference with over 1,400 families, researchers, and professionals in attendance!

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

The Family portion of the Annual SMA Conference began Thursday, June 18th with the Newly Diagnosed Program where families, who were diagnosed from 2014 to present, were able to attend this special session prior to all of the other families’ arrival. It was a great way to slowly introduce new families to the conference as well as other families and professionals on a much smaller scale. This program included:

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

While the conference was underway for families, the 19th Annual SMA Researcher Meeting was being held at the same time and is the largest SMA researcher conference in the world. Cure SMA organizes the conference and financially underwrites the meeting by covering hotel, travel, and registration for research presenters. This was our largest research meeting by far with more than 320 researchers that attended from around the world. These researchers represent institutions, biotech, and pharmaceutical companies from all over. The personal connections made between families and researchers means so much to everyone who attends.
Highlights from the 2015 Annual SMA Conference Meet and Greet

The 2015 Annual SMA Conference, held in Kansas City, Missouri at the fun Westin Kansas City at Crown Center, began for all attendees on Thursday evening with our signature kick-off event, the Meet and Greet & Family Fun Fest. We welcomed new families, returning families, researchers, medical professionals, and friends from all over the world.

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

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

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

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

To see photos from the 2015 Annual SMA Conference, please visit the Cure SMA Facebook page: https://www.facebook.com/cureSMA/
Researcher Relay Races
Family Friendly Research Poster Session

At the 2015 Annual SMA Conference, the third Family Friendly Research Poster Session was held. During the event, 32 research groups presented their scientific findings. This unique event allowed for one-on-one interactions among the 1,400 families and researchers in attendance to share information. Both scientists and families seemed to really value the opportunity.
The Family Friendly Research Poster Sessions

**POSTERS INCLUDED:**

- **Wei-Shi Yeh, PhD, of Biogen** presenting on The Experiences of Individuals and Families Affected by Spinal Muscular Atrophy (SMA) and Their Views on Living with SMA.
- **Allan Kaspar, PhD, of AveXis** presenting on Treating Type I SMA with Gene Therapy: chariSMA.
- **Anne Marquet, PhD, Omar Khwaja, MD, John Baird, PhD, Nikolai Narayshkin, PhD, Sergey Paushkin, PhD, Karen Chen, PhD, Irene Gerlach, PhD, of F. Hoffman La Roche, PTC Therapeutics, and SMA Foundation** presenting on Oral Compound Increases Full Length SMN2 mRNA in Humans.
- **Emilie Voltz, PhD, from Novartis Institutes for Biomedical Research** presenting on LmOto for Treatment of Type I SMA.
- **Shoutian Zhou, PhD, from California Institute of Biomedical Research** presenting on Finding Drugs that Keep Motor Neurons Alive.
- **Caroline Woo, PhD, from RaNA Therapeutics** presenting on A Novel Epigenetic Approach to Treating SMA.
- **Jinsy Andrew, MD, from Cytokinetics** presenting on Fast Skeletal Troponin Activators Applied to SMA.
- **Christian Lorson, PhD, from the University of Missouri** presenting on Anti-sense Oligonucleotides in SMA: Design and Function.
- **Arthur Burghes, PhD, from The Ohio State University** presenting on Biomarkers that Predict Successful Therapy in SMA.
- **Natalia Rodriguez Mueala, PhD, from Rubin Laboratory at Harvard University** presenting on Studies on SMA Patient Motor Neurons Produced from Induced Pluripotent Stem Cells.
- **Kristina Bowyer from Isis Pharmaceuticals** presenting on Developing an Antisense Drug for the Potential Treatment of SMA.
- **Katherine Klinger, PhD, Alison McVie-Wylie, PhD, from Genzyme Corporation** from Progress Towards AAV-SMN Gene Therapy For Spinal Muscular Atrophy.
- **Yimin Hua, PhD, from the Krainer Laboratory at Cold Spring Harbor Laboratory** presenting on Mechanism of Action of ISIS-SMN1Rx.
- **Chien-Ping Ko, PhD, and Chiara Mazzassetto, PhD, from University of Southern California** presenting on Motor Circuit Defects and Repair in SMA Model Mice.
- **Sara Custer, PhD, and Anne Reitz, PhD, from the Androphy Laboratory at Indiana University** presenting on SMA Therapeutics and Biological Insights into Motor Neuron Biology.
- **Thomas Blaettler, MD, of F. of Hoffman La Roche** presenting on SMA and Roche: Our Commitment to Patients and Their Families.
- **Chad Heatwole, MD, from the University of Rochester** presenting on Most Important Symptoms in Adult SMA: A View From the Patient's Perspective.
- **Francesco Lotti, PhD, from Columbia University** presenting on Modifications of the SMN Complex: Do They Have a Role in SMA Pathogenesis?
- **Min Jeong Kye, PhD, from the University of Cologne** presenting on mTOR and Protein Synthesis in SMA.
- **Linda Lowes, PhD, from Nationwide Children's Hospital** presenting on Measuring Motor Ability in Children Can be All Fun and Games.
- **Monique Émond Phht, MSc from Centre de Réadaptation Marie Enfant of CHU Ste-Justine** presenting on Breath Stacking and Postural Therapy: An Optimal Combination to Improve Respiratory Function in Children with SMA Type II and III?
- **Walter Troung, MD, from Gillett Children's Hospital** presenting on The Effects of Assisted Standing on Bone in Children with SMA.
- **Diane V. Murrell, LCSW, from Texas Children's Hospital** presenting on The Role of Clinical Social Work in Support of Families with Children with SMA Type 1.
- **Deborah S. Boroughs, RN, MSN, from BAYADA Pediatrics** presenting on Bridging a Care Delivery Gap for Family Caregivers of Children with SMA Type I and Type II.
- **Rebecca Hurst Davis, MS, RD, CSP, CD, from the University of Utah** presenting on Glucose Tolerance and Fasting in SMA Type II Pilot Study Results and Next Steps.
- **Lindsay Murray, PhD, from the University of Edinburgh** presenting on What's Makes Motor Neurons Vulnerable in SMA.
- **Hong Liu, PhD, from the Kothary Laboratory at the University of Ottawa** presenting on The Contribution of Multiple Cell Types to SMA.
- **Barrington Burnett, PhD, from Uniformed Services University of the Health Sciences** presenting on Muscle Contribution to SMA.
- **Ke-Jun Han, PhD, and Chang-Wei Liu, PhD, from the University of Colorado** presenting on SMN Ubiquitination Regulates its Localization.
- **Christine DiDonato from Lurie Children's Hospital of Chicago** presenting on Pre-clinical Studies in Mild SMA Mouse Models.
- **Summer Laboratory from Johns Hopkins University** presenting on Understanding the Pathology of Human SMA.
- **Matthew Halanski, MD, from the University of Wisconsin** presenting on Respiratory and Radiographic Effects of Growing Rods in Children with Spinal Muscular Atrophy.
Family & Researcher Dance Party

Photo Booth
Children’s Program

This year’s Children’s Program was such an interactive and exciting time, had by all! Thanks to so many of our wonderful volunteers who helped make this weekend incredibly special for everyone, especially all of the children. The kids stayed engaged by participating in an array of arts & crafts, countless toys, movies, Wii video games, special entertainment guests, and so much more!
Cure SMA Thanks the Sponsors for the 2015 Annual SMA Conference
Cure SMA thanks all of the sponsors for their generous support of the 2015 Annual SMA Conference. These sponsors are partners in our community who are critical to the success in the battle against SMA. These partners contributed to scholarships and family assistance programs for the 2015 Conference and for travel and lodging expenses for the SMA Researcher Meeting. Without their support many patients, families, medical professionals, and researchers would never be able to attend and join together in this fantastic meeting.
Thank You

Thank you to all of the following companies who so generously donated items for our 2015 Annual SMA Conference. We are grateful that you cared enough to help support this great cause by donating items or services for our events.

This conference does not happen without a tremendous amount of work and support as well as kind and generous donors like you.
Cure SMA Thanks our Exhibitors for Attending the 2015 Annual SMA Conference

Cure SMA thanks all of the exhibitors for their generous support of the 2015 Annual SMA Conference. Exhibitors who attend the Annual SMA Conference were able to promote their products to SMA families, Researchers, and Medical Care Providers from all over the United States. These exhibitors are partners in our community who are critical members to helping us find a cure and/or treatment for SMA.

**PLATINUM EXHIBITORS**

Ki Mobility

**GOLD EXHIBITORS**

Bio-Rad Laboratories, Inc.
Convaid

Neotech Products
Percussionaire
Permobil
PromptCare
Thomashilfen North America
Triumph Mobility Inc. / Panthera AB

**SILVER EXHIBITORS**

Hill-Rom
LC Technologies
Numotion
Pacific Rehab Inc.
RespirTech
Sundance Enterprises, Inc.

**BRONZE EXHIBITORS**

BayB Brand Products
Disable the Brand
Electromed, Inc.
Make-A-Wish

---

**Save the date!**

**2016 Annual SMA Conference**

which will be held in Disneyland, Anaheim, CA June 16 – 19, 2016!

If you are interested in being added to our mailing list to receive exhibitor opportunities for the 2016 Annual Conference, please email exhibitor@curesma.org.
Special Thanks
The Jacob Isaac Rappoport Foundation and Shaina and Adi Rappoport

Cure SMA is extremely grateful for the continued support from the Jacob Isaac Rappoport Foundation. For the past 14 years Shaina and Adi and their foundation have helped to provide a wonderful opportunity for type I families at each conference, to come together for a special type I event. They have also been sponsoring our wonderful and amazing Children’s Program where with out this program, the conference really would not be able to exist. From entertainment, to crafts and toys, to helping with funding travel for the volunteers who run this program, every aspect of the Children’s Program is essential and important to the conference, Cure SMA and the families.

Last year a new and exciting event was started for just teens to come together and have just teen time during the conference. Thanks to the Rapoports again this year as we will hold the 2nd annual Teen Social and encourage all of our teens to take this time to connect with others in their age group!

Cure SMA would like to extend a heartfelt thank you Shaina and Adi Rappoport and the Jacob Isaac Rappoport Foundation for all of the money they have raised over the years; the dinners they have sent; the cleaning services and packages of toys they have provided for hundreds of SMA families; the luncheons and receptions they have hosted; the funding, support and guidance they have provided and the conference; and the countless hours they have spent lending emotional support to parents of newly diagnosed children. Thank you to the entire Rappoport family, friends, and relatives for your continued support throughout the years!

Jennifer and Aaron Smith, Richard Curran, and Expo

A very special thank you to the generosity and support of Expo Convention Contractors, Jennifer and Aaron Smith, and Richard Curran. They have given countless hours by recruiting donations for the conference, helping with conference layouts and diagrams, raising awareness, and so much more! The professionalism that Expo brings to our Annual Conference, through their impressions signs and banners which decorate the entire hotel, is absolutely outstanding! For the sixth year in a row, Expo has donated their expertise and signage, which never ceases to amaze all of the conference attendees. They do fabulous work and everyone at Cure SMA cannot thank them enough for volunteering their services to make the conference so great. Thank you to the whole team at Expo, Jennifer and Aaron Smith and family, and Richard Curran, for all that you do to help make this conference such a huge success year after year. Your donation of signs, banners, help, and willingness to keep improving this conference is so greatly appreciated!
Our Conference Bubble

Blog post by Rachel Sepe, mom to Scarlett, SMA type II

We’ve been home two weeks from the bubble, as I like to call it, or otherwise known as the Cure SMA conference. This is an annual conference for families, doctors, and researchers to come together to support and educate each other as we journey through this condition called SMA. I say it is a bubble because for three days we feel like we are on this island or commune where everything is normal and everyone has SMA. Hearing suction machines going off in meeting sessions doesn’t even make people bat an eye. Watching your toes every second of the day because another child in a power chair is whizzing past you becomes normal. Asking really direct and personal questions of complete strangers becomes normal because that stranger just revealed something you have never heard of before or shed some light on an issue we are struggling with. It is a reunion with families I talk with daily but see once a year and it is a bittersweet meeting with those families who are new and are walking the path we walked four conferences ago. There is nothing like this weekend and I cry every year when we have to go and even now thinking back on it makes me sob again because it is also the only time of the year where Scarlett is 100% normal.

The Cure SMA directors and staff go out of their way to make sure every moment of the conference is planned with family friendly activities and meaningful sessions. The event begins with a family funfest, or carnival as my kids call it. There are wheelchair races and accessible games. Scarlett this year took off without us playing one game after the next. She was a big girl this year and she knew it. During the following two days, Mike and I attended sessions just for type II families learning from young adults with SMA about what our future holds. We also sat through amazing orthopedic sessions with the best orthopedics for SMA discussing our surgical options and sessions on equipment, which reminded us of our options for bracing, seating, and standing. For new families, they can learn from leading pulmonologists on respiratory care, which is the most critical aspect of care, in my opinion, when you are new.

Our kids have a blast at the conference and have their friends they look forward to seeing. Isabella has found a group of tween girls. Some have SMA, and some are siblings like her who are dealing with younger brothers and sisters like Scarlett. She is already emailing girls she spent the weekend with and I hope they all remain connected. Gavin has his best buddy Oscar by his side the whole time. Oscar has SMA like Scarlett and lives in New York. For the boys, the conference really doesn’t begin until they both are there. Scarlett has met a lot of girls her age and spent the weekend being with her friends while also tagging along with Isabella’s group. The last night of the conference was most special to her. Her buddy, Ella, was at the conference as well. The girls met when they were very young and have been connected since. The final night is a PJ movie night so the girls called it their sleep over and lay side by side talking and giggling. Such a small thing, but in Scarlett’s world, it is a normal moment other girls get to experience.

We are so grateful to Cure SMA for bringing us all together and for taking the time to educate us all on best practices and keeping us up to date on research. There is such hope for the future of SMA and hope for Scarlett.

See more at: http://wishesforscarlett.com/2015/07/07/kansas-city#sthash.0pdgD0Gj.dpuf
Quotes from Around the Community on the 2015 Annual SMA Conference

“Thanks to you for inviting us and for the great organization. It was a really good experience and looking forward to the next!” – Emilie Voltz, PhD, Novartis

“I wanted to thank you for a truly wonderful conference. I’ve been trying and trying to think through how to eloquently thank you for all of your work. I think the best way I can is to let you know that throughout the course of Biogen’s meetings – I consistently heard the same thing from families: “this conference changed (or saved) our lives”. The experience of meeting with families throughout last week was incredible and I left the conference feeling very inspired and moved by the many stories I heard over the course of the week. I am so happy that I have the opportunity to work with your world-class team. Thank you for all that you do.” – Gillian S. Mullins

“Thanks for hosting what I think was a great meeting, it was my first SMA meeting and I thoroughly enjoyed it.”
– Dr. Ewout JN Groen
University of Edinburgh

“Just to say huge congratulations on organizing another superb event last week. Everything ran like a well-oiled machine and it was great to have the opportunity to meet up with the ever-broadening group of people who are now involved in SMA.”
– Joanna Mitchell
Chief Executive, The SMA Trust

“I wish to thank you for your support and to congratulate for the amazing organization of the meeting.”
– Giovanni Baranello, University of Milan

“I enjoyed the conference immensely.”
– Claudia A. Chiriboga, MD, MPH
Columbia University

“Thank you for the chance to participate in the panel presentation. It was a great opportunity [and exposure] for me to be included with such a distinguished group of investigators. I thought the whole conference was great, and I really enjoyed hearing the updates on the drug trials and learning more about newborn screening.”
– Allison D. Ebert, PhD
Medical College of Wisconsin

“Just to say huge congratulations on organizing another superb event last week. Everything ran like a well-oiled machine and it was great to have the opportunity to meet up with the ever-broadening group of people who are now involved in SMA.”
– Joanna Mitchell
Chief Executive, The SMA Trust

“I wish to thank you for your support and to congratulate for the amazing organization of the meeting.”
– Giovanni Baranello, University of Milan

“I enjoyed the conference immensely.”
– Claudia A. Chiriboga, MD, MPH
Columbia University
Hi Cure SMA,

I want to personally thank you for your invitation to exhibit at the 2015 Annual SMA Conference. I have been attending shows of all sorts for over 30 years, as a therapist, equipment supplier, and a manufacturer. This was by far, one of the greatest events I have attended.

It is very obvious that everyone in your organization truly has the families and their affected family member(s) as their main focus.

I look forward to working with your organization again in the future.

Thank you again.
Sincerely,
Sally Mallory, PT, ATP
Convaid

Hi Cure SMA,

I wanted to thank you for putting together such a remarkable conference. I’m not sure I can express what it meant to me. The best I can do is to say I left the meeting feeling inspired... and touched by the stories of the families I spoke with throughout the 3 days. The meeting was unlike anything I’ve attended in my 25 year career working in the pharmaceutical field. The families are clearly thankful for the work you do, and several commented that they look forward to this meeting each year. Although I’ve been with Biogen for 15 years, I took this specific job last year because I thought it would be an opportunity to do something very meaningful, but this was more than I could have expected.

Thank you,
Mike Poirier
Vice President, Program Leadership & Management
Biogen

My husband Matthew & I attended the Cure SMA National conference in June in Kansas City this year. It was our first conference, and we want to THANK YOU from the bottom of our hearts for all the information, connections, and support we learned from this conference! We have a daughter (4) with SMA type III, and a son (2) with SMA type II. Our oldest daughter is unaffected, and was able to attend the siblings talk it out workshop. Thank you for all you did to make it a must-do on our yearly calendar. It was wonderful!

Thank you,
Mike Poirier
Vice President, Program Leadership & Management
Biogen

Hi Cure SMA,

Conference for us a new diagnosed family was very moving and truly an aMAISYng experience. We will be forever grateful to Cure SMA for the connections and education we gained from a care, support, and research perspective attending on behalf of our beautiful Ms. Maisy. We were absolutely moved by EVERYTHING & EVERYONE!

One of my favorite quotes reads...

Thank you very much again for organizing such a wonderful meeting!

Best wishes,
–Xin Zhao
PTC Therapeutics. Inc.

While we were certainly soaking information in everyday one thing clearly resonated throughout conference for me and that was each of the Cure SMA staff personally and professionally genuinely care about ensuring new and returning families felt connected and supported for each beautiful child or adult with SMA. That feeling and atmosphere created at conference by Cure SMA positively changed me forever. While it was a lot of information and I likely forgot some of what was said the very poignant feelings I experienced attending daily will remain with me for a lifetime.

There aren’t enough kind words that would even begin to capture and represent the incredibly meaningful work the Cure SMA staff does day in and day out to support families affected by SMA. Please feel free to share this message with those in your organization as it is clearly evident to us that the Cure SMA staff is behind our Ms. Maisy forever too. Simply aMAISYng! ;-) We are already looking forward to conference next year.
Over the weekend, I got to attend the annual Spinal Muscular Atrophy (SMA) Conference. This was my first time being able to go (because it was held at the Westin Hotel in Kansas City about 20 minutes from my apartment), and while I feared that I was overly excited and had created far too lofty expectations, it turns out that I had no reason to worry. The conference, which was the second most attended conference ever for “Cure SMA,” not only met my high expectations, but surpassed them in every way. I had a phenomenal time, met some amazing people, and learned a lot about how to live with this horrible disease. In addition, I got the opportunity to share some of my own experiences about creating a successful life with SMA, as a member of a panel discussion that was held on the final day of the conference. After spending the first few days of the conference getting to meet and talk with some of the inspiring individuals trying to figure out how to give their disabled children and families a fulfilling life (much like my parents did many years ago), I felt extremely honored to get to pass on what I have learned from battling this disease for almost 30 years. This event touched me and impacted my life in so many ways that there is no way that I could fit everything I want to share with you in one post. So, in order to make sure that I adequately discuss all of the important things I learned, I will be writing several articles about the conference over the next week or two. Today you get the first of these posts, which discusses the first lesson that was passed on to me, and it was given to me before I had even made it into the hotel.

Before I get into the first part of my experience at the conference and the lessons I learned, I have to first tell you a bit about how well-run and efficient this event was. I cannot say enough about how great of a job “Cure SMA” did in making this weekend a success. If you have ever organized, worked at, or even just attended a large, multi-day conference, you know that events like this are incredibly difficult to run and often seem a little chaotic. There are just a lot of moving parts to keep track of both before and during an event of this magnitude, and no matter how much you plan and prepare for potential obstacles, there are always going to be unexpected issues that come up along the way. While I am sure that the SMA conference was no different, and it certainly had its own share of problems that popped up, from the outside as an attendee you would never have known. Everything, from the parking accommodations to the audio/video equipment to the scheduling of the various workshops, was carried out masterfully. The “Cure SMA” employees and the volunteers at the conference did an amazing job of keeping things running smoothly (and even more impressively, on time), and I believe I speak for everyone who attended when I say “thank you” to these awesome individuals. It is only through their abilities to prepare for everything they could and overcome the unexpected obstacles that arose (two skills that all of us with SMA have had to master and appreciate), that this year was such a success.

All of the time and energy that the “Cure SMA” staff put into organizing the conference is what made this event such an incredible experience. Like I said, while I have been to academic conferences and such over the years, this was my first SMA Conference. In fact, this was my first conference of any kind that focused on disabilities, so I was not sure what to expect before I arrived. It had been several years since I had been around lots of other people in wheelchairs, and I had never been around this many other physically disabled individuals, so I knew that this would be different from any event I had ever been to. During the days leading up to the conference I kept wondering what it would be like at the hotel. I kept thinking about silly, little things like how long the wait for an elevator would be at a hotel completely booked by people in wheelchairs. Or how loud a room filled with hundreds of ventilators and suction machines would be. These are obviously trivial things that would have no impact on how good the conference was, but for whatever reason these are the things I thought about (the elevator line was long, but no one was in a hurry). Thinking about these logistical challenges and such only fueled my intrigue and anticipation for the conference, and even though I had no idea what to expect, I was excited and ready for this adventure, which I knew would be full of surprises and new experiences.

The first sign that this was going to be different from anything I had ever experienced occurred before I had even entered
As we drove up to the Westin and turned into the circle drive by the front entrance to unload me and valet my car, all you could see was this caravan of conversion vans with wheelchair lifts. They were lined up on either side of the driveway in two long lines about 10 cars deep, and everywhere you looked you saw families packing up medical equipment, suitcases, and wheelchairs into their vehicles. You would think that this sort of controlled chaos and slow-moving progress, especially when you are trying to get on the road, would have made everyone involved irritated and grumpy, but despite the mayhem everyone I saw had a smile on their face. No one was blaring their car horn because they could not get out, complaining to the parking attendants about the accommodations, or arguing with other drivers for being slow. In fact, it was quite the opposite. Everyone was smiling, patiently waiting their turn, and even helping other families pack up their belongings. This was an unexpected, but heartwarming, sight to behold, and it illustrated one of the many important things I took away from the conference.

It took me a while to fully appreciate what this scene had to teach me, but after giving it some thought, I realized the powerful lesson that was being presented to me that day in the circle drive. On any other day, at any other event, a parent holding up a whole line of traffic to take care of their disabled child, who needs you to drop everything to give them a suction, would be met with car horns, foul language, and any number of colorful hand gestures. On this day, at this event though, this same action was met with nothing but patience, smiles, and understanding. No one was upset, and the reason for this is understanding. They were not upset, because two weeks, two months, or a year ago they were in the same situation. They know the frustration and embarrassment that comes with inconveniencing a whole group of people, because this awful disease has erupted yet again to make your life difficult. They know what it is like to have to burden others close to you, or even strangers, to make sure your disabled child has the best life possible. They know the struggle that comes with having a family that is affected by SMA. They understood.

This knowing that everyone around you understands what you have to go through can be a very powerful thing. In addition to witnessing this idea outside of the hotel, I also got to feel its effects first-hand throughout the conference. It was so refreshing to be able to introduce myself to someone and not have to explain who my nurse was, why I carry a suction machine, or anything else about my disability, because they already understood. Inside that hotel, for the first time ever in my life, I was “normal.” It is difficult to put into words, and I do not know if this will make sense, but experiencing what it feels like to be the “normal” one was an extremely refreshing and empowering thing. While I did not feel abnormal or misunderstood in general, nor did I think about how nice it would be to be “normal” very often, getting to know what it is like to just be Scott had a major impact on me. I was the exact same person at the conference that I was the weekend before, but the way I felt about who I was completely changed as a result of being in this environment of understanding. It helped me to better recognize and appreciate the fact that even though my SMA is a part of my identity, it does not define me any more than my gender, ethnicity, or religion do. It is only the way that others (as well as myself) view and understand my disability that it impacts my life. Getting to experience what “normal” feels like and gaining this insight into who I am is something that I will always remember, and I am certain that I will lead a happier life because of it.

Witnessing the controlled chaos outside the hotel and experiencing what it is like to be “normal” for a brief time illustrated the powerful effects that understanding can have on our lives. Watching an entire driveway full of vehicles patiently wait for someone to move without any signs of anger or frustration shows you how much your own understanding of what others are going through can influence your thoughts and actions. Not only that, but having a strong sense that others understand you, and the obstacles you face, can also have an enormous impact on your life. The common experience of battling a disease like SMA changed a situation that would typically be filled with misunderstanding, awkwardness, and frustration into one filled with nothing but empathy and compassion. You will be surprised at how often this phenomenon occurs throughout your life, if you really think about it. Keep this story in mind the next time you angrily flip someone “the bird” for holding up traffic or get upset when someone inconveniences you. Remember that you cannot always see or understand the struggle that others go through, and that the lens through which you view a situation makes a big impact on your feelings. Try to be more understanding of those around you, and you will eventually find that you have a better understanding of yourself as well. If we can all just be a little more understanding of one another, just imagine how much happier the world could be.
The SMA Researcher Meeting, held each year as part of our Annual SMA Conference, is an anticipated event in the SMA research community. This meeting has a tangible impact on achieving our mission of a world without SMA.

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

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

The meeting is also the main forum in the SMA scientific community for integrating new researchers and drug companies as efficiently as possible.

Finally, and most importantly, the conference motivates SMA researchers by allowing direct interactions with families and patients living with SMA, through events like our Meet and Greet, Family Friendly Researcher Poster Session, and various panel discussions. These are unique and special experiences for all involved.
19th Annual SMA Researcher Meeting

The goal of the SMA Researcher Meeting is to create open communication of early, unpublished scientific data, accelerating the pace of research. The meeting also furthers research by building productive collaborations. Importantly, since the meeting is part of our Annual SMA Conference, researchers have the chance to interact with, learn from, and educate families affected by SMA.

**New Clinical Trial Materials Debut**

As more SMA drugs progress through clinical trials, Cure SMA has been working to address the increasing need for accurate information.

The centerpiece of these education efforts is our new booklet, *Learning About Clinical Trials*. Developed with expert reviewers, this booklet explains the clinical trial process. Released to families at the conference, it is now available on our website, along with the updated SMA drug pipeline, and a list of trials currently recruiting.

**Six Programs Participate in Drug Discovery Panel**

During a special panel discussion, representatives from six drug programs in clinical trials updated families. This session also included informational presentations from Dr. Jill Jarecki, our research director, and Dr. John Kissel.

Four of the six programs—gene therapy, ISIS-SMNRx (now called IONIS-SMNRx), RG7800, and LM1070—treat the underlying genetics of SMA. The other programs, Olesoxime and CK-2127107, work to protect the muscles and nerves.

**Pharmaceutical Companies Release New Data From Clinical Trials**

The SMA Researcher Meeting also provides companies with a worldwide platform to announce new results. Several of our pharmaceutical partners released new clinical trial data at our SMA Researcher Meeting. These include:

- Ionis Pharmaceuticals provided an update on children with SMA enrolled in the open-label extension (OLE) study for the Phase 2 study of ISIS-SMNRx (now called IONIS-SMNRx).
- Cytokinetics released data from their Phase 1 study of CK-2127107, a skeletal muscle activator. Cytokinetics, in partnership with Astellas, has announced a Phase 2 study will begin later in 2015.
- BioBlast announced positive preclinical proof-of-concept results of its possible SMA drug.
- Nationwide Children’s Hospital updated their Phase 1 trial of gene therapy.

**Updates from the 2015 SMA Researcher Meeting**

Following are introductions to the researcher meeting sessions. Complete summaries of all can be found on our Cure SMA website, in the news section. [http://www.curesma.org/news/](http://www.curesma.org/news/)

**Emerging Trends in Motor Neuron Pathobiology**

We recognize that the research in SMA and in related motor neuron disorders share similar challenges: to know WHAT cells to protect, WHEN we need to protect them, and HOW we can identify new potential therapies. This session brought in scientists who work in other motor neuron disorders to explore connections to SMA.

**Newborn Screening**

Data generated in mouse models of SMA suggest that drug delivery is most effective when given early. This session explored how the SMA research community is pursuing newborn screening for SMA.

**Clinical Research**

As SMA drugs move closer to FDA approval, an important goal is to represent the patient perspective on topics like the impact of SMA, what constitutes meaningful change, and risk/benefit analysis. The clinical research session explored this, as well as biomarkers and clinical trial outcome measures.

**Drug Discovery**

The 2015 SMA Researcher Meeting closed with a much anticipated session on SMA drug development. Eight talks were given, four focused on drug programs at early stages in the SMA pipeline, and four focused on drugs being tested in clinical trials.

**SMA Pathology**

Electrical current must flow from sensory neurons to the motor neurons in the spinal cord and then to the muscle itself to promote muscle contraction. The session discussed the formation of these circuits, as well as how to measure their electrical flow and function.

**MN Partners and Therapeutic Targets, Parts 1 and 2**

Individuals with SMA do not correctly produce survival motor neuron (SMN) protein at high enough levels. There may be ways to treat SMA other than increasing SMN levels. The drugs targeting alternative pathways could be used in combination with SMN-enhancing therapies.
Seven Individuals and Families Recognized for Their Contributions to the Community

Each year, while much of our community is gathered together, we use the Annual SMA Conference to recognize several individuals, families and foundations who have provided special support to the Annual SMA Conference and the SMA community.

Here are this year’s awardees. On behalf of the entire community, thank you for all you do!

**Shaina and Adi Rappoport and Family**

The Jacob Isaac Rappoport Foundation was started by Shaina and Adi Rappoport in memory of their shooting star, Jacob. For 14 years, they have provided incredible support to the conference, sponsoring the Type I Reception, the Children’s Program, and the Teen Social. The Foundation also sponsors Type I Care Packages throughout the year.

**Meredith and Patrick Woodruff and Family**

Meredith and Patrick Woodruff started the Miller McNeil Woodruff Foundation in honor of their son, Miller, who lived 87 days before passing away from SMA type I. Thanks to their generosity, many families from their home state of Arkansas were able to receive a scholarship to the 2015 Annual SMA Conference.

**Barbara and Gene Trainor and Family**

The Trainor family started the Erin Trainor Memorial Fund in memory of their daughter, who passed away in 1994 from SMA type I. The Erin Trainor Memorial Fund has raised over $710,000 toward the family’s initial goal of $1 million. The ETMF generously provides conference scholarships for newly diagnosed families.

**Richard Curran, Aaron Smith and Jennifer Miller-Smith, and Expo CCI**

Through Expo CCI, Richard Curran, Aaron Smith and Jennifer Miller-Smith have gone above and beyond in donating all the signage for our Annual SMA Conference for several years now. They help with conference layouts, signage needs, special requests, and they assist in setting up and organizing the signage while on site.

**Jill Jarecki, PhD**

Jill Jarecki, PhD, has now been Cure SMA’s research director for 10 years. Under Jill’s leadership, Cure SMA has funded $26 million in research grants, the SMA drug pipeline has doubled in size—from 9 to 18 programs—and the number of companies investing in SMA drug development has grown from 1 to 15. Jill’s expertise, leadership, and passion have helped make all of these advances possible.

**Richard Rubenstein**

Richard Rubenstein is chair of the Cure SMA Board of Directors. His passion, dedication, leadership, and compassion are crucial to the health and growth of our organization. We especially recognize the added time and effort he has put in the last several months in preparation for the conference.

**Mary Schroth, MD**

Dr. Mary Schroth chairs the Cure SMA Medical Advisory Council, which is responsible for many of the workshops and events at each Annual SMA Conference. In addition, Mary provides key leadership to other support & care programs, such as the care series booklets. At this year’s conference, we announced that Mary is stepping into a new role as Cure SMA’s medical professional education consultant.
Cure SMA Medical Advisory Council Meets in Chicago

Members of Cure SMA’s Medical Advisory Council (MAC) gathered in Chicago during early November, for two days of meetings to set the agenda for the care of SMA individuals and families.

Our MAC is chaired by Mary Schroth, MD, a leading pulmonologist, and professor of pediatrics. The MAC is one of the most highly respected bodies of SMA medical and clinical experts in the US, including 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 focuses on educating families, healthcare providers, and the public about SMA; expanding SMA standards of care; and translating positive research results into clinical practice.

Clinical Care Research Grants

One of the top items on the MAC’s agenda was the awarding of up to $150,000 in new clinical care grants. Clinical care research is part of Cure SMA’s comprehensive research funding strategy. Cure SMA funds 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.

Earlier this year, we opened up a new clinical care research request for proposals (RFP). A clinical care RFP is an invitation for scientists to submit their best ideas for projects that address the clinical, psychological or social aspects of SMA. They explain what they want to study, how they plan to study it, and why they think it will help those affected by SMA.

During the meeting, the MAC evaluated all proposals to see which projects are the most intriguing, which have a well constructed study plan, and which match up with the most pressing unanswered questions about SMA. The best proposals will then receive a clinical care grant from Cure SMA.

Setting the Care Agenda for SMA

Also during these meetings, the MAC worked on setting the agenda for the next 3-5 years of care for SMA. Currently, several pilot studies in care (many funded by Cure SMA) are ongoing or have recently been completed.

The MAC used these meetings to chart out next steps with the information that has already been learned from these pilot studies. They will look to scale these up, to see what impact the discoveries might have on the wider SMA population, and lead toward defining and improving the standard of care.

The MAC also set out goals and plans for how this evidence and data will be used to educate medical professionals.

Our thanks to the MAC for their dedication and expertise.
Cure SMA received a special delivery of fun items that have been donated to our Newly Diagnosed Care Package program! Thank you so much to the Wick Family for this wonderful donation in memory of their daughter Alison’s birthday.

These beautiful afghans will be going into our care packages. They were made by Joyce Fitch, in honor of her grandson Benjamin Carpenter, to bring comfort and warmth to the children who receive them. Thank you!

Cure SMA recently received a donation of lightweight handmade toys from Annette Bonelli, in memory of her grandson Jack. Jack’s favorite toy was an owl, so Annette included an owl ribbon on each one of the toys! These toys will be added to our newly diagnosed care packages, and we would like to thank Annette for her generous donation!

Kris and Michele Foell generously donated these books for SMA care packages, in memory of their daughter. Eva loved these very special books, and reading them helped both her and her parents through some difficult and stressful situations. Thank you to the Foell’s for such a thoughtful, lovely gift.
Cure SMA recently received a donation of beautiful handmade blankets and hats for our newly diagnosed care package program. These were donated in honor of Ellie Stevenson via “The Belsy Project.” Thank you for your generosity!

Cure SMA recently received a generous donation of blankets and stuffed animals for our newly diagnosed care packages. We would like to thank Khrystal Davis for her generous donation! Many of the blankets and stuffed animals Khrystal donated were zebras, and zebras symbolize a rare disease, which is why she selected them.

Cure SMA recently received a generous donation from the Irie family, in honor of their daughter Nawai’s 1st birthday. Nawai’s mom, Wendy, said the items they donated include things they received in their care package and found helpful, things they use daily, and things that Nawai enjoys doing. The Irie’s hope these items will help fellow SMA families smile.

Cure SMA recently received a generous donation of toys in honor of Ahnya Dixon’s 2nd birthday. Ahnya’s family organized a birthday party in honor of Ahnya, and collected toys for our newly diagnosed care packages. Cure SMA would like to thank Ahnya’s family and her party guests for this donation!

Save the date!
Thursday, June 16th - Sunday, June 19th, 2016

2016 Annual SMA Conference
Anaheim, CA,
at the Disneyland Hotel
The SMA drug pipeline has grown dramatically in just over a decade. Of the 18 programs in the pipeline, seven of these are in clinical trials, and two of those are in Phase 3 clinical trials.

Reaching and achieving FDA approval is a major hurdle in our path to a treatment and cure. And, consistent with our strategy of pursuing many different therapeutic avenues at once, we may face this hurdle many times.

When the FDA evaluates a drug for approval, they must weigh many different factors, including the quantity and quality of evidence of safety and effectiveness, potential benefits of a treatment versus the potential risks, the impact the treatment will have on the patient community, and more. This is why it is so crucial that we build strong relationships with the FDA and other regulatory bodies. We want to be sure the voice of our patient community is heard when the FDA considers these drugs for approval.

The Cure SMA FDA Engagement Initiative

Earlier this year, families and representatives from the Cure SMA community met with the FDA as part of our ongoing FDA Engagement Initiative. The goal of these most recent meetings was to further expand the FDA’s understanding of the priorities of our patient community in drug development.

During the meetings, patients voiced opinions on their assessment the most important/burdensome aspects of SMA on patients and their families, what would be meaningful change in SMA patients’ everyday lives, and the overall impact that an approved treatment would have for our patient community.

PFDD and PDUFA

Every five years, Congress must renew Prescription Drug User Fee Act (PDUFA), which is the law that allows FDA to collect fees from drug manufacturers to fund the new drug approval process and other related activities. When PDUFA was reauthorized in 2012, known as PDUFA V, a stronger emphasis was placed on patient input in the drug development process. This resulted in the creation of the Patient-Focused Drug Development (PFDD) Initiative, to more systematically gather patients’ perspectives on their condition and available therapies to treat their condition.

As part of this commitment, FDA is holding at least 20 public meetings over the course of PDUFA V, each focused on a specific disease area, as well as offering participation in other community-wide meetings. In late 2014, Cure SMA submitted a formal request to be granted one of these meetings. If selected, a PFDD meeting for SMA would be one way to provide our patient community with an opportunity to impact the future of SMA drug development.

Past and Future Activities

Prior steps in our FDA Engagement Initiative have included:

- The SO-SMART Workshop. During the 2014 Annual SMA Conference in Washington DC, the National Institutes of Health (NIH) hosted a forum to discuss the appropriate outcome measures for clinical trials, using SMA as an example population. 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.
- Focus groups with families and organizations within the SMA community to learn more about the patient point of view on drug development. A total of nearly 100 people participated, including individuals with SMA, parents, and clinical experts. The results showed the difficulties of living with SMA, from the long process of finding a diagnosis to the wide-ranging psychosocial effects of coping with SMA. The results also showed that even very small improvements in functional ability could have enormous benefit, while small losses in functional ability could translate to devastating effects on the family. Our thanks to our co-funders of the focus groups: SMA Foundation, Isis Pharmaceuticals, and Biogen.
- Two comprehensive surveys: one of parents whose children have participated in a clinical trial, and one of parents whose children have not. The participator survey focused on motivations, hopes, and communication when parents have children with SMA participating in clinical trials. 53 parents of children with SMA who participated in a clinical trial in the last ten years completed the survey. A total

continued on next page
of 222 parents whose children have never participated in a trial took the second survey. This survey looked at the reasons why parents of children with SMA have not involved their children in clinical trials. Our thanks to Parent Project Muscular Dystrophy (PPMD) for partnering with us on the two parent surveys.

• A keynote session with Richard Moscicki, MD, at our 2014 Annual SMA Conference. Dr. Moscicki is Deputy Director for Science Operations at the FDA’s Center for Drug Evaluation and Research.

Cure SMA has also been working with other government and regulatory bodies through events like our Congressional Dinner and SMA Day on the Hill. Rep. Fred Upton, who chairs the Energy and Commerce Committee, was one of the congressional guests at our 2014 Hope on the Hill Congressional Dinner. Chairman Upton leads the 21st Century Cures Initiative, which was created to investigate how the government can help “accelerate the discovery, development, and delivery of promising new treatments and cures for patients.” At a hearing to discuss the release of the most recent discussion draft, Chairman Upton cited the SMA community—including sisters Brooke and Brielle Kennedy, from his home state of Michigan, who have SMA type II—as part of the inspiration for his support of 21st Century Cures.

2015 Annual SMA Conference
During the 2015 Annual SMA Conference, we shared more of the initial results from the above activities, and discussed future plans for our FDA Engagement Initiative.

At the conference, our moderated panel on SMA clinical trials included John Whyte, MD, MPH, Director of Professional Affairs and Stakeholder Engagement, Center for Drug Evaluation and Research, FDA, as well as panelists from Ohio State University, Nemours Children’s Hospital, Genzyme Corporation, The Hastings Center, Cytokinetics, Nationwide Children’s Hospital, Biogen, F. Hoffmann-La, Isis Pharmaceuticals, and Novartis.

Our thanks to the many families, organizations, and staff at the FDA that have been our partners in this effort. Their willingness to share their time, energy, and experiences is crucial to our success.

FDA Engagement Update: The Voices of SMA
Thanks to our vibrant community, August was one of our largest and best awareness months ever, with unprecedented levels of public awareness and outreach for SMA.

One of these opportunities to convert awareness into action was our “Voices of SMA” project—an outreach to the FDA in order to educate them about:

1. Our community’s priorities in drug development
2. The impact of SMA on patients and their families’ daily lives, and
3. What would amount to meaningful change in SMA patients’ everyday lives

As we expected, our community came through with dozens of stories. We created The Voices of SMA, a booklet summarizing the key themes, and highlighting those same themes through selected excerpts from the stories. This booklet, along with a complete collection of all the stories submitted, was distributed to all the top decision-makers at the FDA.

The Voices of SMA is just one of the critical next steps in our FDA Engagement Initiative. To access The Voice of SMA booklet, visit: http://www.curesma.org/documents/research-documents/voices-of-sma-booklet.pdf
Cure SMA has announced a new partnership with the District Policy Group, a boutique lobbying, public policy, and advocacy practice, to advocate for several key policy areas on behalf of the SMA community.

With seven drug programs now in clinical trials, the Patient-Focused Drug Development emphasis of PDUFA V, and the progress toward passage of 21st Century Cures, now is the opportune time for us to maximize our influence with government and regulatory bodies, in order to speed our path to a treatment and cure for SMA.

When we changed our name to Cure SMA last year, a primary goal was to broaden our community to attract even more people to be a part of our mission. Families remain at the heart of what we do, and we are focused on accelerating momentum toward a treatment and cure.

The partnership with the District Policy Group provides a unique opportunity to continue our expansion by bringing key influencers in the FDA, Congress, and state and local legislatures into our community.

Advocating for Change

The District Policy Group will be working with us on five major areas:

- FDA Engagement Initiative. Pursue timely and safe approval of SMA drugs by the FDA, and making sure the patient perspective is properly represented to the FDA during these discussions.
- Newborn Screening Program. Gain broad support for newborns to be automatically screened for SMA.
- Legislative priorities. Advance key initiatives such as the 21st Century Cures Initiative.
- Grassroots advocacy. Focus on both local and national opportunities for our community to get involved.
- Medical reimbursements. Find opportunities within Medicare, Medicaid, and the Affordable Care Act for more families to obtain the equipment and care they and their children need.

About District Policy Group

The District Policy Group is a bipartisan boutique lobbying practice within Drinker Biddle & Reath that has deep experience – and a long record of success – in advancing health care clients’ interests before the legislative and executive branches of government at the federal level. The District Policy Group team, comprised of a dozen lobbyists, public policy specialists, and grassroots coordinators, has extensive expertise in government relations, advocacy, Capitol Hill policy and politics, federal agency rulemaking, grassroots organizing, social media, and coalition building. The District Policy Group is particularly skilled at assisting not-for-profit patient advocacy organizations in understanding the political and policy landscape, and with that information, helping clients in developing creative legislative and regulatory strategies, achieving their public policy goals, and operating effective grassroots and grasstops advocacy programs. In addition, the team assists clients in building and maintaining direct, close, and productive relationships with Members of Congress, Congressional staff, federal agency officials, and key thought leaders and stakeholders in the health policy arena.

House of Representatives Passes Ensuring Access to Clinical Trials Act

The House of Representatives has passed the Ensuring Access to Clinical Trials Act of 2015 (EACT). This bill already passed the Senate in July, and will now go to President Obama to be signed into law.

This bill allows individuals to receive up to $2,000 as compensation for participating in a clinical trial, without those funds counting as income for the purposes of Medicare and SSI eligibility. If this bill is signed into law, those affected by rare diseases like SMA can participate in a clinical trial, and their compensation for travel and related costs won’t change their eligibility for Medicare or social security benefits. This removes a critical barrier to participation and paves the way for trials to move forward more quickly and efficiently.

Cure SMA is pleased to be one of nearly 75 organizations who advocated with the House for the passage of this law, along with the Cystic Fibrosis Foundation, NORD, and PPMD.
Clinical and Regulatory Webinar and Extended Q&A Now Available

On November 9, 2015, Cure SMA hosted a one-hour webinar on clinical trials and regulatory topics. Jill Jarecki, Research Director at Cure SMA, was joined by a panel of three drug development experts to discuss clinical trial design, expanded access, NDA processes, and more.

The panelists included Tim Franson, MD, is Chief Medical Officer at YourEncore, Thomas Murray, PhD, is President Emeritus at The Hastings Center, and Tim Miller, MD, is VP & Head, Medical Affairs, North America Rare Diseases at Genzyme.

The webinar included a Q&A session, and an extended Q&A was published, post-webinar to cover many of the topics time did not permit discussing.

Extended Q&A

Is it possible that in the Phase 3 trial there are two different doses and placebo investigating? And is it possible that this information is secret?

Phase 3 trials are typically conducted as blinded-randomized placebo controlled trials. Therefore, some patients are given drugs and some are given a sham treatment or a placebo. This means that the drug company, the site principal investigator (PI), and the patient do not know whether they are receiving drug or placebo. This trial design is considered to be the gold standard in determining the true benefit of a drug, as it has been shown that significant percentage of benefit in clinical trial can come from a placebo effect. Please see page 15 of our clinical trial booklet for more information on placebos.

Considering the obvious and established deterioration of SMA patients, why wasn’t the natural history of SMA used instead of placebo/sham controls?

Many studies have shown the placebo effect is real and significant. Thus, placebo controlled studies are considered the gold standard for drug development. However, good natural history data can be used in registration trials, for example. In addition, natural history data can and has been used to reduce the number of individuals who are placed in a placebo group in a clinical trial—for example, a 2:1 treatment to placebo ratio.

Another consideration is the fact that SMA natural history data has been changing over the last decade. The National Institutes of Health (NIH) has funded the largest and most well controlled natural history study in infants under six months of age, which will be completed over the next few months. Once the results become available in 2016, that will open up more opportunities to use this data.

I was hoping to find out more about the results from the trials being conducted now. Can that be discussed?

Cure SMA regularly posts updates on clinical trials, including information on new trials, recruitment, and results. Please visit our news section and click on “Clinical Trials” in the right sidebar. http://www.curesma.org/news/

What is the probability that drug trials will open to patients with SMA type III?

While there are currently no trials open to SMA type III patients, Cytokinectics recently announced that they will open a trial in 75 teens and adults with SMA type II, III, or IV—both ambulatory and non-ambulatory. We will post additional details as soon as they become available, as well as any updates on future trials for all types of SMA.

When is the FDA is going to be satisfied with the cure?

The FDA needs to see robust data to be convinced the benefit of a drug outweighs the risk in specific patient populations. The best possible measure for this is a blinded, randomized placebo-controlled trial. However, there are mechanisms to speed this up, such as accelerated approval, where drug approval can be sought from a surrogate marker or from Phase 2 trial data. Often this data would need to be more robust than sought from placebo-controlled results of a Phase 3 trial.
In addition, drug sponsors are continually speaking with the FDA about when their data warrants a NDA submission for marketing approval. In fact, designations like fast-track and breakthrough are intended to give drug sponsors greater access to the FDA for such ongoing discussions, in the cases of rare or orphan diseases.

The FDA will also look at information from families and individuals affected by SMA to determine the impact that a potential treatment will have. Treatments may slow disease progression, stop it, or reverse it. The FDA will take into account the community’s input on how each of these outcomes could affect their daily lives.

Because the pathophysiology of SMA is well known and increasing/correcting the SMN2 protein is a known target for drug therapy, would increased measurements of patients’ cerebrospinal fluid (CSF) SMN2 protein combined with obvious and measurable clinical improvements not suffice for required FDA drug approval criteria?

Surrogate markers can be used as primary endpoints for accelerated approval, provided it is proven that the marker correlates with disease severity. SMN fits many of those considerations, but several major questions remain unanswered, making its use as a surrogate challenging right now. Researchers need to learn more about when and where SMN is needed, particularly in patients who are already showing symptoms of SMA. This is one of the many reasons why basic research is so important, even with multiple drugs already in clinical trials. Several ongoing basic research projects are working to answer these critical questions about SMN protein.

At what point would the FDA consider not filing a NDA unethical?

Timing and data requirements for FDA submission are generally negotiated with the FDA before hand for rare diseases. In fact, this is particularly true if a drug program has a fast-track or breakthrough designation, as Dr. Franson discussed during the webinar. These designations provide for many more discussion points with the FDA, and greater collaboration on what needs to be in a NDA and when enough data has been collected to support the NDA filing.

Can orphan status help expedite reviews/approvals for a therapy/drug?

Having orphan status gives a drug candidate priority review by the FDA at the time when the new drug application (NDA) for marketing approval is submitted to the FDA. This shortens the NDA review process at the FDA from 10 months down to six months.

Does Cure SMA have a projected timeline for all of the compounds in phase 3 that would outline the FDA review/decision of each compound and supply chain of molecule if small molecule or large molecule?

The figure on page 13 of our booklet, *Learning About Clinical Trials*, explains in more detail each phase of the drug development/clinical trials process and how long each of those phases might take. In addition, 2-3 times per year, Cure SMA releases an updated drug pipeline showing the stage of each program. Taken together, these pieces can provide a rough estimate of when a given program might reach NDA status.

However, it’s important to keep in mind that many things can either speed up or slow down the process for a drug. And, perhaps most important of all, only 10% of drugs that begin the clinical trial process will reach the NDA stage. In fact, 40% of drugs with good Phase 2 data will fail in Phase 3. The continued progress of a drug will depend on the data that is generated through the clinical trials.

As far as expanded access use, is it limited to the type of patient population currently being studied in a clinical trial, or can it be applied to a broader patient population that may not have met inclusion criteria of a trial?

It depends on the type of expanded access protocol (EAP) being sought. Some EAPs are for one patient only. Others are for a group that has been tested in clinical trials. And still others are for a wider group, including those who may have been excluded from a clinical trial in the past. The pharmaceutical company and the FDA will look at a number of factors. Has the drug been proven safe, or might there be unknown side effects for other populations that have not yet been tested in clinical trials? Can they manufacture enough of the drug to serve a wider group? Are the clinical trials complete, or might a wider EAP jeopardize enrollment in ongoing Phase 3 trials? The 

continued on next page
Roche Announces New Clinical Trial of RG7916

Roche, along with partners PTC Therapeutics and the SMA Foundation, recently provided the following update.

The collaboration between Roche, PTC Therapeutics and the SMA Foundation would like to provide you with an update on the advance of our SMN2 splicing modifier programme. This programme is separate to the Roche-led olesoxime programme.

In April 2015, we shared with you that the Moonfish clinical study, investigating SMN2 splicing modifier RG7800 in people with SMA, was placed on clinical hold. This was a precautionary measure after an unexpected safety finding was identified in an animal study exploring long term treatment with RG7800 at exposures above those observed in the Moonfish study. The Moonfish study remains on hold as we continue investigations to understand these findings.

We are pleased to share with you that we have moved forward with development of a second SMN2 splicing modifier. This new investigational medicine, RG7916, will now advance to its very first clinical study in healthy individuals in January 2016, which will provide important information about the safety profile, pharmacokinetics (what the body does to the medicine) and effects of RG7916 in healthy individuals. RG7916 has different characteristics relative to RG7800, which may influence how the medicine interacts with the body and this will be evaluated in the study.

We have received questions on the inclusion of Itraconazole in this study. Itraconazole is an approved medicine which will be used as a tool to help understand the metabolism, or break down, of RG7916 in humans. This is common practice for this type of study.

Please visit Clinicaltrials.gov if you would like to read more about this clinical study.

Information from this study will help us to compare the two SMN2 splicing modifiers RG7800 and RG7916 and decide how the programme advances further. We look forward to providing you with an update about this in the first half of 2016.

Our commitment to helping the SMA community remains strong as we continue to pursue new medicines for the treatment of SMA.

In collaboration with our partners, PTC Therapeutics and the SMA Foundation, we would like to thank you for sharing your experience of living with SMA; you inspire us every day.
Cytokinetics Announces New Details on Upcoming Phase 2 Trial

In early January, Cytokinetics opened enrollment for a Phase II study testing CK-2127107 in teens and adults with SMA type II, III or IV. Cure SMA is excited to see the continued progress of this drug into the next phase of clinical trials, and particularly excited to see this trial focused on teens and adults. This latest trial announcement speaks to two of Cure SMA’s primary goals: pursuing a breadth of treatment options, and ensuring we have treatments for all types, all ages, and all stages of SMA.

The clinical trial is designed to assess the effect of CK-2127107 on multiple measures of muscle function in both ambulatory and non-ambulatory patients with SMA, a severe, genetic neuromuscular disease that leads to debilitating muscle function and progressive, often fatal, muscle weakness. In collaboration with Astellas, Cytokinetics is developing CK-2127107 as a potential treatment for people living with SMA and certain other debilitating neuromuscular and non-neuromuscular diseases and conditions associated with skeletal muscle weakness and/or fatigue.

The primary objective of this double-blind, randomized, placebo-controlled clinical trial is to determine the potential pharmacodynamic effects of a suspension formulation of CK-2127107 following multiple oral doses in patients with type II, type III, or type IV SMA. Secondary objectives are to evaluate the safety, tolerability and pharmacokinetics of CK-2127107. The trial will enroll seventy-two patients in two sequential, ascending dose cohorts (two cohorts of 36 patients each, half ambulatory and half non-ambulatory). Each cohort will be stratified by ambulatory versus non-ambulatory status to receive CK-2127107 dosed twice daily for 8 weeks.

“Initiating this first Phase 2 trial of CK-2127107 represents a major step forward given our interests to serve the many adolescents and adults who are living with SMA, a disorder with few treatment options,” said Robert I. Blum, Cytokinetics’ President and Chief Executive Officer. “We look forward to working closely with the investigators and clinical trial sites to evaluate the effects of our next-generation skeletal muscle activator, which we believe holds promise for the potential treatment of patients battling this devastating disease.”

Save the date!
Thursday, June 16th - Sunday, June 19th, 2016

2016 Annual SMA Conference  Anaheim, CA, at the Disneyland Hotel
Cure SMA Releases New Clinical Trial Care Series Booklet

As more and more SMA drug programs progress through clinical trials, there is an increasing need for accurate and unbiased information on clinical trials from our community. Cure SMA has been working to address this through education efforts targeted to the specific needs of our community.

The centerpiece of these education efforts is our new care series booklet, Learning About Clinical Trials. Developed with a panel of expert reviewers, many of whom are members of our Medical Advisory Council, this booklet introduces and explains the clinical trial process.

While a number of general resources on clinical trials exist, these do not address the unique challenges and opportunities of SMA. Importantly, our booklet fills the unmet need for clinical trial information that directly addresses SMA and the common questions in our community.

Unique Challenges and Opportunities

These crucial issues include:

• The SMA treatments currently in development work on a number of different therapeutic approaches. Some target the underlying genetics, and some would protect nerves or muscles in order to slow or stop the disease. With these different approaches being tested on different ages, stages, and types of SMA, researchers face complex decisions in designing and evaluating clinical trials—decisions such as who is included in trials and how change is measured.

• SMA is considered an “orphan disease.” For orphan diseases, the number of patients enrolled in each clinical trial phase is often smaller than the numbers for non-orphan diseases. Orphan diseases are also eligible for special incentives from the FDA to encourage or speed up drug development.

• Because many of those affected by SMA are very young, SMA trials must consider additional factors when testing on infants or children.

• Because SMA is a life-threatening disease that does not yet have an approved treatment, the question of expanded access is more critical than it might be for other diseases.

This care series booklet is now available online, and will be part of a clinical trial information packet that will be distributed at our Annual SMA Conference. Throughout the remainder of this year, we’ll continue to release new materials to aid the entire community.

To download this care series booklet, please visit: http://www.curesma.org/documents/research-documents/clinical-trials-care-series-booklet.pdf

Ongoing Educational Efforts

In addition to these education efforts, here are a few of the other ways we’re working with our community on clinical trials:

• We’re working closely with the FDA, to set the stage for potential FDA approval of the drugs currently in development.

• We’ve updated our website to include a list of trials currently recruiting participants.

• We’ve released an updated version of the SMA drug pipeline, showing all 18 drug programs currently in development, including the seven now in clinical trials.

• We regularly post updates on clinical trials in our news section. These news updates are also typically shared as part of our monthly e-news.

Have a question about clinical trials or a suggestion of other information that might be helpful? Send us an email at info@curesma.org.
New Gene Therapy Results Announced at World Muscle Congress

Nationwide Children’s Hospital/AveXis announced new results from their ongoing Phase 1 trial of systemic gene therapy for SMA during this year’s World Muscle Congress in the United Kingdom. Dr. Jerry R. Mendell, Director of the Center for Gene Therapy at The Research Institute at Nationwide Children’s Hospital (NCH), made the presentation.

Dr. Mendell, lead investigator in the study, reported preliminary observations from the ongoing study including survival data which indicated that none of the patients in this study have reached an “event”, which is defined as death or until a patient requires at least 16 hours of continuous respiratory assistance for at least two weeks in the absence of an acute reversible illness. Additionally, Dr. Mendell described the motor function improvement observed in all patients, as measured by The Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), a test developed to measure motor skills of patients with SMA type I. All patients in this study showed improvement in CHOP-INTEND scores, with modest improvement in the low-dose treatment group and greater improvement in the mid-dose treatment group.

Dr. Mendell stated that AVXS-101 appears to be generally safe and well tolerated in the patients studied to date.

Cure SMA Funds Multiple Gene Therapy Approaches

Beginning in 2010, Cure SMA made a series of grants to Nationwide Children’s Hospital 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. Dr. Brian Kaspar and Dr. Mendell 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).

Currently, two approaches are being studied: an injection into a vein, known as systemic delivery, which is the treatment being tested in this current trial, and delivery directly into the cerebrospinal fluid (CSF), a process known as CSF-delivered gene therapy.

In total, Cure SMA has granted $845,000 for gene therapy, including support for both the systemic program and the CSF program.

Novartis Launches Phase 2 Clinical Trial

In early June, Novartis published a paper in the journal Nature Chemical Biology, detailing promising results in their program to develop an orally available SMA drug that corrects SMN2 splicing. In testing, the drug increased SMN protein levels and extended survival in a severe mouse model of SMA.

Novartis is currently testing this drug in a Phase 2 clinical trial at several European sites. The open-label trial is enrolling infants aged six months or less with SMA type I, who also meet other inclusion/exclusion criteria. Complete information on this clinical trial can be found on the FDA’s clinical trial registry. Available here: https://clinicaltrials.gov/ct2/show/NCT02268552?term=spinal+muscular+atrophy&recr=Open&rank=12

Individuals with SMA don’t produce SMN protein at high enough levels, due to a mutation in the SMN1 gene. Individuals with SMA do have one or more copies of the SMN2 gene, which also produces SMN protein, but most of the protein produced by SMN2 lacks a key building block.

Cure SMA has identified four possible therapeutic approaches for treating SMA, one of which targets the SMN2 gene. The goal of this approach is to correct the gene splicing, meaning SMN2 could produce a complete protein, or to prompt SMN2 to make more protein. The Novartis program is one of several programs pursuing this approach.
Dear members of the SMA community,

During the past few months, we have received many questions from members of the SMA community about ISIS-SMNRx and when it might be widely available to people with SMA. We are deeply moved by your stories and recognize the urgent need within the SMA community for patients and families living every day with this devastating disease. We wanted to provide you our thoughts on key issues related to the ISIS-SMNRx program. We understand that words alone do not give you the comfort and peace of mind you deserve, but we hope this letter will provide answers to some of your questions.

First, we want to assure you we are doing everything in our power to advance ISIS-SMNRx though development, and hopefully approval, as quickly as possible. While the drug development process can be frustratingly slow, especially to patients and families touched by a disease like SMA, the development program for ISIS-SMNRx has progressed much faster than most. An early and important step in moving this investigational drug forward was receiving Fast Track designation from the FDA. Fast Track is designed to facilitate the development and expedite review of investigational drugs for approval to treat serious conditions such as SMA. This designation allows us more frequent meetings with the FDA throughout our clinical program to help us gain insight and guidance from the agency as we work toward a regulatory filing. Our hope is these more frequent discussions offer the type of guidance that will result in fewer delays and quicker reviews. The communications with regulators has been ongoing and we continue to engage in regular and productive discussions with health authorities in the US and across the EU to understand the most expeditious path forward for ISIS-SMNRx.

In addition to Fast Track designation, there are several additional regulatory approaches in the U.S. that are designed to expedite the investigational drug review process. These include Accelerated Approval, Breakthrough Therapy, and Priority Review. We have and will continue to explore all of these approaches and pursue every option that may accelerate the possible approval of ISIS-SMNRx. However, we have to continue the well-controlled Phase 3 clinical trials to collect the data necessary for regulators to make decisions about the safety and efficacy of ISIS-SMNRx.

A question many parents and patients with SMA ask is how to interpret the open-label, Phase 2 clinical trial results to date. Like all of you, we are encouraged by the results. However, the current open-label data, while exciting and supportive of continued research, are not from definitive randomized well-controlled trials. We remain committed to executing the Phase 3 pivotal studies as quickly as possible to build the body of evidence needed for evaluation by regulators in the U.S. and around the world.

We are driven in our work on ISIS-SMNRx by the acute awareness that patients and families are waiting for access to treatment. Our current focus is to advance our ongoing well-controlled Phase 3 studies which will be required for approval. We believe that this approach is the best path to making this investigational drug available to the SMA community in the shortest period of time for the greatest number of patients. Patients and families frequently ask us about access to this investigational drug prior to regulatory approval and the timeline associated with opening an Expanded Access Program (EAP). This is among the most difficult questions we receive and unfortunately, there are easy no answers.

Our goal is to hopefully make ISIS-SMNRx available to patients once we are confident that doing so will not risk slowing the collection of the data necessary for regulators to make a determination about the safety and efficacy of ISIS-SMNRx and once we are able to end the well-controlled study arm. Making ISIS-SMNRx available earlier in the process could jeopardize potential approval and ultimately access for patients.
ADDITIONAL UPDATES

Biogen Announces EMBRACE, a New Phase 2 Clinical Trial

Biogen, in partnership with Ionis Pharmaceuticals, has announced a new Phase 2 clinical trial, called EMBRACE.

EMBRACE is a multi-center, Phase 2 clinical study evaluating the safety and exploratory efficacy of the investigational drug, ISIS-SMNRx, (ISIS 396443) in patients with infantile or childhood-onset Spinal Muscular Atrophy (SMA). The study is a double-blind, randomized, sham-procedure controlled study, designed to examine the safety and exploratory efficacy of ISIS-SMNRx in approximately 20 patients with SMA over a 14 month period.


Ionis Releases Encouraging Results from Phase 2 Study

Ionis Pharmaceuticals provided an update on its ongoing open-label Phase 2 clinical study of ISIS-SMNRx in infants with type I spinal muscular atrophy (SMA). Previously the company reported data from this study on event-free survival, measures of muscle function and assessments of developmental milestones. The data reported now shows continued increases in median event-free survival and muscle function scores as well as achievement of developmental milestones.


Ionis Announces Additional Trial Results at 2015 Annual SMA Conference

During the 2015 Annual SMA Conference, Ionis Pharmaceuticals, Inc. provided an update on children with spinal muscular atrophy (SMA) who have completed their open-label, Phase 2 multiple-dose study of ISIS-SMNRx and are continuing to receive treatment in an open-label extension (OLE) study. Consistent with earlier observations, increases in muscle function scores and additional motor function tests were observed in children treated with ISIS-SMNRx. Ionis is currently collaborating with Biogen to develop and commercialize ISIS-SMNRx to treat patients with SMA.


Ionis Pharmaceuticals Announces SHINE, an Open-Label Extension Study

Ionis Pharmaceuticals, Inc. announced that it has initiated an open-label extension study, SHINE. The SHINE study provides ISIS-SMNRx to infants and children with spinal muscular atrophy (SMA) who have completed their participation in the Phase 3 ENDEAR and CHERISH studies and are eligible to participate in SHINE. All patients in the SHINE study will receive a 12 mg dose of ISIS-SMNRx every four months for infants who completed ENDEAR or every six months for children who completed CHERISH.


Sincerely,
Biogen and Ionis Pharmaceuticals
SHARING PHOTOS

Graeme Mooney
Charli Nave
George Cornelius
Combs
Abella Runkle
Abigail Reyes
Harrison Bradshaw
Finley Anderson

Charles Durell II
Graham Vollmer
Arianna Fischer
Bear Bonner
Graham Hennings
SHARING PHOTOS

Julissa Magana

Jace Humphrey

Isaac Kimmel

Jordyn Turner

Jamie Halpenny

Jace Cruz

Julissa Magana
Cure SMA-funded researchers Rebecca Hurst Davis and Kathy Swoboda have published a paper, “Responses to Fasting and Glucose Loading in a Cohort of Well Children with Spinal Muscular Atrophy Type II” in the journal Pediatrics. Elizabeth A. Miller and Ren Zhe Zhang also co-authored the paper.

This study was funded by an initial $50,000 Cure SMA clinical care research grant to Dr. Swoboda. Children with SMA sometimes develop insulin resistance or glucose intolerance, meaning they cannot properly regulate their blood sugar. The goal of this pilot project was to further understand glucose, insulin, and other responses to fasting and glucose loads in people with SMA.

The study found that children with SMA type II defined as obese using objective variables are at increased risk for impaired glucose tolerance regardless of whether or not they visually appear obese. However, further studies are needed to determine the prevalence of impaired glucose tolerance and tolerance for fasting within the broader heterogeneous SMA population and to develop appropriate guidelines for intervention.

Future Studies
In 2014, Cure SMA made a second $50,000 grant to Ms. Hurst Davis to continue research in this area. This funding will build on the results of this paper. Future studies will be used to better understand what is happening clinically in individuals with SMA, as well as to devise strategies for ways to treat patients who are insulin resistant/glucose intolerant.

Clinical Care Research
Clinical care research is part of Cure SMA’s comprehensive research funding 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.

Earlier this year, we opened up a new clinical care research request for proposals (RFP). A clinical care RFP is an invitation for scientists to submit their best ideas for projects that address the clinical, psychological or social aspects of SMA. They explain what they want to study, how they plan to study it, and why they think it will help those affected by SMA.

Currently, those proposals are being reviewed by our Medical Advisory Council. They will carefully review all proposals to see which projects are the most intriguing, which have a well constructed study plan, and which match up with the most pressing unanswered questions about SMA. The best proposals will then receive a clinical care grant from Cure SMA.

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.
SMA Drug Pipeline

Our approach to research is focused on continually expanding the pipeline of potential drugs and therapies for SMA, cultivating new approaches and advancing promising ideas. The drug candidates shown below represent all four of our therapeutic approaches. Every year, the pipeline gets broader and deeper. We’ve seen enormous growth over the past decade, and we expect that growth to continue in the coming years as more projects approach FDA approval.

### HOW THE PIPELINE HAS GROWN

![Graph showing the growth of the SMA drug pipeline from 2000 to 2015.]

- **TOTAL PROGRAMS**:
  - 2000: 1
  - 2005: 5
  - 2010: 10
  - 2015: 25

- **ACTIVE PROGRAMS**
  - 2000: 1
  - 2005: 4
  - 2010: 9
  - 2015: 20

- **PROGRAMS IN CLINICAL TRIALS**
  - 2000: 0
  - 2005: 2
  - 2010: 5
  - 2015: 10

*Includes failures

### NUMBER OF COMPANIES INVESTING IN SMA DRUG PROGRAMS

- 2015: 16
- 2014: 12
- 2013: 11
- 2012: 9
- 2011: 7
- 2010: 6
- 2005: 1
- 2000: 0

Last updated: June 2015.
Cure SMA Funds $1.85 Million in New Research Grants in 2014-2015

Cure SMA has announced a total of $1.85 million in new research funding. Our model is designed to fund both a breadth and a depth of projects, in order to continuing building up both a breadth and depth of programs in the drug pipeline. This includes funding for:

- Basic research, to investigate the biology and cause of SMA, in order to identify the most effective strategies for drug discovery, and to develop tools that facilitate SMA research.
- Drug discovery, to convert basic research ideas into practical new drug candidates.
- Clinical research, to develop the tools to allow clinical trials to be conducted in all types and stages of SMA.
- Clinical care, to understanding issues like breathing and nutrition that impact current quality of life for those affected by SMA.
- 19th Annual SMA Researcher Meeting, to bring together the leading SMA scientists, clinicians, and drug developer together to develop SMA research strategies.

If you missed any of our funding announcements, catch up on them in our news section on our website. Each announcement includes a profile of the researcher who is receiving the grant, and explains more about their projects and how they might benefit the SMA community.

Basic Research
- $140,000 to Sara Custer, PhD, at Indiana University
- $140,000 to Francesco Lotti, PhD, at Columbia University
- $140,000 to Mustafa Sahin, MD, PhD, at Boston Children’s Hospital
- $80,000 to Linda Lowes, PT, PhD, at Nationwide Children’s Hospital*
- $140,000 to Chad Heatwole, MD, at the University of Rochester*

Drug Discovery
- $330,000 to California Institute of Biomedical Research and their collaborators, led by Peter Schultz, PhD
- $445,000 to Brian Kaspar, PhD, at Nationwide Children’s Hospital

Clinical Care
- $50,000 to Nilesh Mehta, MD, at Boston Children’s Hospital
- $50,000 to Walter Truong, MD, at Gillette Children’s
- $50,000 to Deborah Boroughs, RN, MSN, at BAYADA Home Health Care
- $50,000 to Rebecca Hurst Davis, MS, RD, CSP, CD, at University of Utah
- $50,000 to Martin Lemay, PhD, at Université du Québec à Montréal
- SMA Researcher Meeting
- $200,000 to bring the leading SMA researchers together from around the world

*The grants to Dr. Heatwole and Dr. Lowes, both of which deal with clinical trial outcome measures, were jointly funded by basic research and clinical research grant money.

We thank the following families and foundations for their generous contributions toward these grants: The Jacob Isaac Rappoport Foundation toward Drs. Custer, Sahin, Lotti, and Kaspar; The Miller McNeil Woodruff Foundation toward Drs. Lowes and Kaspar; The Michael and Chandra Rudd Foundation toward Drs. Kaspar and Schultz; The Spinal Muscular Atrophy Research Team toward Dr. Heatwole; FSMA Canada toward Dr. Lemay.
Cure SMA, along with collaborators from Biogen and the SMA Foundation, have published a paper in the journal BMC Neurology.

Over the past year, we’ve completed 16 focus group sessions and 37 interviews in the US with 96 participants including: 21 with individuals with SMA; 64 parents of individuals affected by SMA; and 11 clinicians who specialize in the care of SMA patients. The goal of this project was to document the perspective of individuals and families living with SMA.

Key Themes
Three key themes emerged from the responses collected from these projects.

1. confronting premature death
2. making difficult treatment choices
3. fearing the loss of functional ability
4. coming to terms with lost expectations
5. fatigue and stress
6. stigma
7. limitations on social activities
8. independence
9. uncertainty and helplessness
10. family finances

Advocacy with the FDA
An important goal at Cure SMA is to add the patient voice to the drug development process. This paper is another step in this process, building on our Voices of SMA project that was released in September of 2015. As part of the PFDD (patient-focused drug development) initiative, both the stories from Voices of SMA and the findings from the focus groups will become part of the review process if and when an SMA drug is brought forward for approval.

Published peer-reviewed journal articles like this are one of the most important ways for researchers to share information. Published results allow the experiments and results to be reviewed and vetted by other scientists and drug companies, who may then use these as the basis for further drug development. This paper will help further incorporate the patient perspective into current and future SMA drug development research, by sharing it with the FDA and other drug companies.
Collaborative Ways Cure SMA is Driving Research Results

Much as families in our community often work together to start a chapter, organize an event, or raise awareness of SMA, researchers must work together in order to find the fastest and most effective path to a cure for SMA. In addition to providing funding for ongoing SMA research, Cure SMA provides several opportunities for researchers to collaborate and advance new breakthroughs.

Cure SMA Funded Journal Articles

One of the most important ways for researchers to share information is through published, peer-reviewed journal articles. Published results allow the experiments and results to be reviewed and vetted by other scientists, who may then use these as the basis for further experiments.

In 2014, 16 new research publications were released as a result of Cure SMA-funded projects. And five additional publications were released in just the first few months of 2015. The researchers behind these five most recent publications received a total of $663,000 in grant money from Cure SMA.

The SMA Researcher Meeting

Published journal articles are not the only way researchers can collaborate. Often, researchers will discuss data even earlier in the process, to share exciting results and to get input and feedback to refine experiments and improve results. The SMA Researcher Meeting, held as part of the Annual SMA Conference, is the largest platform in the world for SMA researchers to share this early, unpublished data with fellow scientists.

At the SMA Researcher Meeting, researchers can share information both through formal presentations, and through side meetings and building of collaborative partnerships. This allows us to continue accelerating the pace of research, bringing new information, new breakthroughs, and—eventually—new treatments to the community.

Recent Cure SMA-Funded Journal Articles

2015

2014
Please visit the news section of our website for a full list of articles published in 2014. [http://www.curesma.org/news/](http://www.curesma.org/news/)
Cure SMA, the leading organization dedicated to the treatment and cure of spinal muscular atrophy, recently provided an additional $445,000 grant toward a gene therapy for SMA at Nationwide Children’s Hospital.

Brian Kaspar, PhD, principal investigator in the Center for Gene Therapy at Nationwide Children’s, and his team have made promising strides toward therapies to treat SMA. This new award brings Cure SMA’s total funding for this program to $845,000: $745,000 for the CSF program and $100,000 for the systemic gene therapy program.

The number one genetic cause of death for infants, 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.

**Advancing Therapeutic Approaches**

Gene transfer may increase SMN levels by using a viral vector to deliver the SMN1 gene to affected cells. Dr. 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 therapy: an injection into a vein, a process known as systemic delivery which is currently in clinical trials, and delivery directly into the cerebrospinal fluid (CSF), a process known as CSF-delivered gene therapy. The CSF-delivery project in SMA is a translational funding initiative to the Kaspar Lab at Nationwide Children’s Hospital provided by a collaborative funding initiative by The National Institutes of Health-National Institute of Neurological Disorders and Stroke and Cure SMA.

“We are excited about the milestones that have been reached thus far with Cure SMA funding, and look forward to seeing progression of the gene therapeutic program with our new round of funding to Dr. Kaspar. There is great potential for CSF-delivered gene therapy to be further advanced for patients with SMA,” said Jill Jarecki, PhD, research director, Cure SMA.

The new funding will now support regulatory filings with the FDA to begin a new clinical trial, studies to define dosing levels for a clinical trial focused on CSF-delivered gene therapy, and support screening potential patients for the trial and evaluating their responses to the therapy.

“We are excited about our progress to advance a CSF route of delivery to human clinical trials for SMA and we are grateful for the continued support from Cure SMA,” said Dr. Kaspar. “We were able to demonstrate remarkable survival rates with normal motor functions in our SMA animal models, and found significant targeting of motor neurons throughout the brain and spinal cord,” Dr. Kaspar explained. These results were published in a November 2014 article in Molecular Therapy, prompting the additional funding support from Cure SMA.

**Advancing Treatment for the Entire Community**

“A critical long-term goal of the Cure SMA drug discovery approach is to help identify treatments for SMA patients of every age, disease type, and stage. One of the most compelling aspects of CSF-delivered gene therapy is the promise it shows for reducing the amount of drug required for larger and older patients. This could eventually make the treatment accessible to a wider population,” said Dr. Jarecki.

The technology for both systemic and CSF-delivered gene therapy has been licensed to AveXis, a clinical stage biotechnology company.

Beginning in 2010, Cure SMA made a series of grants to Dr. Kaspar to study gene therapy, also called gene transfer. 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.

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.
Cure SMA President Kenneth Hobby was a featured panelist at Partnering for Cures, a conference convened by the Milken Institute’s FasterCures initiative. At the event, over 700 research innovators—including senior government officials, patient advocates, scientists, major investors, industry CEOs and philanthropists—gathered to discuss some of the most common barriers to speeding up innovation in medical research, and how those barriers can be collaboratively overcome.

Kenneth was joined by Christopher Austin (Director, National Center for Advancing Translational Sciences, National Institutes of Health), Annie Kennedy (Senior Vice President, Legislation & Public Policy, Parent Project Muscular Dystrophy), Todd Sherer (CEO, The Michael J. Fox Foundation for Parkinson’s Research), and moderator Louis DeGennaro (President and CEO, Leukemia & Lymphoma Society).

The panel was part of the “Disruptors’ Academy,” a series of town-hall sessions on how organizations can thoughtfully and creatively disrupt old patterns and find new ways to move medical innovation forward. The panel addressed questions including: How do foundations prioritize among the competing opportunities to invest their time and resources, from continuing to support basic science to investing in start-up companies through influencing the approval and reimbursement process? Does the recent focus across the ecosystem on patient-centeredness provide a unique opportunity? What capacity needs to be built in the sector?

The panel also discussed the current landscape and future opportunities for organizations like Cure SMA, as they partner with scientists, pharmaceutical companies, and regulatory agencies, working to bring a drug all the way from a basic research seed idea up through FDA approval.

Cure SMA’s Approach to Innovations

Cure SMA’s model is based on two primary objectives: aggressive yet strategic investment in all aspects of the research process, and the creation of partnerships between the patient and family community, researchers and pharmaceutical and biotech partners, and regulatory agencies like the FDA—all with the goal of developing safe and effective treatments for SMA.

Earlier this year, Cure SMA joined the TRAIN project within FasterCures. The goal of TRAIN is to connect disruptive innovators in the disease research space with the vital resources, tools and relationships to catalyze development of new therapies and cures. Through the network, innovation in one disease area is translated to another in order to achieve treatment breakthroughs for all.

Our work with TRAIN gives us the opportunity to both learn from and share our expertise with other organizations who have that same commitment to innovative solutions.

Our thanks to FasterCures and the Partnering for Cures team for putting on such a great event, and for inviting Cure SMA to be a part of it. And thank you to our fellow panelists for sharing their expertise as well.
Cure SMA Joins The Research Acceleration and Innovation Network

Cure SMA has become a participating organization of The Research Acceleration and Innovation Network (TRAIN), a program of FasterCures, bringing the total number of venture philanthropies in TRAIN to 80.

FasterCures established TRAIN in 2005 to connect disruptive innovators in the disease research space with the vital resources, tools and relationships to catalyze development of new therapies and cures. Through the network, innovation in one disease area is translated to another in order to achieve treatment breakthroughs for all.

“We’re excited to join with so many organizations doing important, cutting-edge work in disease research,” said Kenneth Hobby, president of Cure SMA. “We look forward both to learning from our partners in TRAIN, and to sharing our expertise with them wherever we can. We believe that collaboration is one of the key ways that we can make progress toward our goal of a treatment and cure for SMA, and joining TRAIN reflects that commitment.”

TRAIN organizations—such as the Cystic Fibrosis Foundation (CFF), Michael J. Fox Foundation for Parkinson’s Research and the Leukemia & Lymphoma Society—share a singular focus on, and a significant stake in, getting promising therapies from the laboratory bench to the patient’s bedside as rapidly as possible. CFF’s partnership with Vertex Pharmaceuticals, which produced the game-changing drug Kalydeco, has captured the public’s attention in recent years. Many other foundations are pursuing similar pathways to patient benefit, including CureDuchenne and Parent Project Muscular Dystrophy, which are also investing in companies with new therapeutics, and the Multiple Myeloma Research Foundation, which is paving the way to precision medicine for the patients it represents.

Collectively, TRAIN participants fund nearly $1 billion in medical research annually, and more than three-quarters of them are partnering with biopharmaceutical companies to advance their collective goal of getting new and better treatments to patients faster. While the organizations that participate in TRAIN are individually unique, they all demonstrate an ongoing commitment to accountability, collaboration, research effectiveness, resource building and patient centeredness.

“TRAIN’s patient-driven foundations are applying entrepreneurial philanthropy to de-risk translation and commercialization of research in their respective disease areas, and are responsible for some of the most transformative ideas in R&D,” said Kristin Schneeman, program director at FasterCures.

Save the date!
Thursday, June 16th - Sunday, June 19th, 2016

2016 Annual SMA Conference Anaheim, CA, at the Disneyland Hotel
Cure SMA,

Thank you so much for her package she loves everything in it. She wanted to open everything right away and is cuddling with her homemade blanket. The binder is PERFECT. Again thank so much.

Erin Petrie of Nevada

Cure SMA,

We found out about your organization when our son Henry was diagnosed with type I at 6 months old. Henry died last month, just over a year old.

We want to thank you for all you have done for our family. We were fortunate to have chapter leader, David Sereni living in our town. The Sereni’s were incredibly supportive and probably the single biggest comfort we had while caring for our son.

The care package, but specifically the wagon and the car bed were invaluable. Through all that Cure SMA gave us, we were able to feel a little less isolated in a heartbreaking situation.

Thank you again.
Best,
Tara & Theo Csavas of California

Cure SMA,

I have received the wagons. This really amazing! I would not have thought of such a great idea myself. Thank you so much!

Regards,
Tanping of California

Cure SMA,

We received the care package last night and were overwhelmed. There were so many great toys for our girl; I know she is going to love them all. We also just looked at each child’s picture and name and cried. Every child is a blessing and such a special gift from God. It helped us feel like we are not alone. Please convey our appreciation to those who have so generously given to make the care package possible.

Thank you for what you do.
Jenny Pope of Texas

DIRECTIONS Winter 2015

FAMILY LETTERS

Cure SMA:

Words cannot express the gratitude our family feels with this care package. When Justin was diagnosed we knew life was going to change and we would need support. Your organization has provided a wealth of educational materials, answered many of our questions and most importantly empowered us to be the best parents and advocates we can be for our son.

We are thankful for all your organization has done and the kind families whom have embraced us as their own. Hope is a word that is often used, but has many meanings to different people. The cure SMA family has brought a new meaning to us through your generosity. We have hope that our family though challenged will provide a quality of life for our son that will bring him happiness.

We have hope that all those who will come after us will have a cure. We have hope that the essence of your organization’s work will change the world for a better place. Most of all we have hope that the future holds endless possibilities for people to show true love and compassion.

Thank you,
The Larez Family of Washington
Good Afternoon,

My husband and I are so overwhelmed by the package we received today. We just found out about our daughter’s diagnosis a week ago, and I still don’t think we know how to feel. The items and information and letters from all of you are appreciated more than we can say! She has type III and really doesn’t show a whole lot of symptoms besides when she climbs or jumps, so it is really hard to wrap my head around the fact that things might progress later since it is not very evident now. I’m sure that will take awhile to get used to. Anyway, every thing was amazing. The play-doh was a HUGE hit. Jordyn LOVES, LOVES play-doh, so some extra colors were just so exciting for her. And as you can see in the pictures, the box comes in handy as a toy as well. This is such a heart warming thing to receive a week to the day after we got a positive diagnosis. I just cannot say thank you enough! The smasheroo book is awesome too. I had tears the whole time I was reading it. I know this will be a book we will read to her many times.

Again, we cannot thank you all enough for the support and information. I’m not sure what the future holds for us, but I know we have enough support and strength to get through anything. Thank you THANK YOU again!

Please see the attached pictures of Jordyn and her items.

Misti Turner of Louisiana

Cure SMA,

Thank you so much for all the information and care package! I wasn’t expecting all of that. It was a nice surprise and we really appreciate it. The girls were so excited to open that big box and see what was in it! I see some of the information is different than what I knew from before. That is good to know because in a couple weeks, the girls and I will be talking to some classes at the technical school here in town. This will be our 4th year talking to students. We like to get the word out about SMA (especially to the nursing students for some possible future home health nurses). We explain what SMA is and tell everyone what abilities Trinity and Carrigan have rather than just disabilities. We give our personal story and background. We think it is important to let people know that kids in wheelchairs are normal people, too. We get out and about all the time and like the girls to feel included. We tell people that we do what other families do, just with modifications and extra time. I would like to mention the Cure SMA website if anyone would like to know more about it.

Thanks again!
- Sharon of Minnesota

Hi Cure SMA,

Just wanted to let ya’ll know that the care package was a wonderful gift. We received the care package at the end of a really difficult day of traveling to appointments and it gave us all a good note to end our day on! We are so very thankful.

Have a great day,
Dominick of Louisiana

Dear Cure SMA:

We received the package yesterday. Thank you so much! It’s such a blessing in hard times. We have been looking at what clinical trails my son may qualify for and your breakdown helped tremendously! And all the information answers so many questions.

We cannot thank you enough for everything. We were blown away by the care package and all the support and information. You are the most amazing support group we’ve ever heard of!
We have been spreading info to others around us Thank you again for such support in such a difficult time. We are feeling hope. Thank you so much! We really are blown away by all of your support. You guys are angels. Thank you again and again!

Anastassia Kobernik of Washington

Cure SMA,

Thank you so much for the amazing care package. Harrison loves the baby Einstein ocean thing and the silky. The package touched mine and Chadwick’s hearts. It’s nice to know we’re not alone in this and at this devastating time.

Thanks and God bless.
Love the Bradshaws of Utah

_______
Dear Cure SMA,

Thank you for the wonderful care package! Can I admit that it took until this weekend to open the box? I have had the box for over a month....or is it two? I guess it is part of my denial (I don’t WANT my son to have SMA!). I know opening the box doesn’t make my son have SMA - but....I don’t know....I guess in opening it I have to admit to myself that he does have SMA. I even walked by the box multiple times a day with the other kids asking what it was :) Now that I have opened the box it is a blessing! Even the notebook is wonderful to organize the LARGE stack of paperwork I have accumulated! THANK YOU so very much for all the wonderful and thoughtful toys - Bryan is going to really enjoy playing with them.

The wagon came yesterday! The kids were SO excited! THANK YOU so very, very much for blessing Bryan and our family with this amazing wagon!

THANK YOU again!
Sandy of Michigan

Cure SMA,

Really I don’t even know how to thank you enough for the huge care package of toys for Travis! I was not expecting all of that lol. He loves all of it and we read the book last night!

Honestly we appreciate it so much.
Hannah Barry of Ohio

Hi Cure SMA!

Thank you so much for reaching out. I’m so happy to have found Cure SMA! We received the care package on Friday and it was awesome! I cried - it was so sweet and generous and Sadie LOVES the new seat that was in there!

Again, thank you SO much for everything. We really appreciate it!

Jessica Newsome of Pennsylvania

Cure SMA,

Thank you SO much! The support we have gotten from all of you at Cure SMA has been such a comfort!

Kristina DeFazio of Maryland

Cure SMA,

We are so overwhelmed by the generosity of the care package you sent for our precious Graham. All of the toys and things I would have never thought of- it’s all so perfect. Even the handmade quilt, how thoughtful and special. The positioners will be extremely helpful, as will the bath mat as we were really starting to struggle with that.

I look forward to reading more through the material in the info packet. I’m sure I’ll have questions. I can’t seem to say thank you enough. It is just so wonderful to know we have support and aren’t facing this alone.

Best,
Adrienne of Indiana
Dear Cure SMA,

Tyler received his package, we used it as a tool to tell him his diagnosis. We told him why he was having trouble with his legs, what it was called “SMA” and that there are lots of other children like him and people working very hard who care about all these children, then showed him the large box that came in the mail with all sorts of things that they thought he would like. Three days later we had our first SMA clinic appointment which lasted about 4 hrs. He was so happy to be able to bring things and entertain himself with the items from his care package.

Thank you so much!
Kim Hansen of Massachusetts

Cure SMA,

We just received the care package! Absolutely amazing!

How incredibly thoughtful and generous. A little hard to see things donated in memory since it’s a reminder of the reality but what a beautiful way to pay it forward.

So excited to use these new toys. The notes are helpful too as to how each item adds benefit. Blown away by the support. Again, thank you!

Thank you for the car bed too.
Jenny Arnold of Maine

Cure SMA,

We are at a loss for words. This disease is so isolating but I cannot even begin to describe the mental and emotional lift you provided for my entire family with that care package. CJ loves everything but especially his hand sewn blanket. He lays on it and plays with his dinosaurs and watches movies with it too. My two older kids have been wearing their SMA bracelets and refuse to take them off. What an amazing organization. You are a huge blessing to us, thank you thank you thank you!

Pictures to come as soon as I can get everyone to cooperate! :)
Melissa of New York

Hi Cure SMA,

We received the information booklets and care package. It is beyond words how amazing that care package is! It moved us to tears! Thank you so much for reaching out to us! I was getting overwhelmed and scared going through information on the Internet and then read the information you sent to us and it was great. I felt educated, but not scared or overwhelmed. These last few weeks have been a whirlwind.

Once again thank you!
Tiffani of Texas

Thank you Cure SMA. I don’t know how I would have managed without your help and support. This is my daughter Lucrezia and she is very happy with the Ball toy from the care package she received. Thank you for everything.

Trish Arroyo of New York
A Bobcat Named Madison

MIAMI GARDENS, Fla. - En route to the NAIA National Championship, the St. Thomas University Women’s Basketball team has had a special member of the team all season long, inspiring players and coaches alike. As a result, the Bobcats have put together a 27-4 record, regular season and conference tournament Sun Conference championships, and a 12-game winning streak heading into the national tournament.

Madison Smith, an eight year old girl from Pembroke Pines, Fla., was diagnosed with a relatively unknown genetic neuromuscular disease called Spinal Muscular Atrophy (SMA) on October 2, 2007, two days shy of her first birthday. SMA is a group of inherited diseases that destroy the nerve cells called motor neurons controlling voluntary movements such as crawling, walking, head and neck control and swallowing. As a result, Madison has never walked or crawled and is in an electric wheelchair.

Through a program facilitated by Team IMPACT, Madison joined the St. Thomas Women’s Basketball team as an “Honorary Bobcat” at the start of the season. Since joining the team, Madison has not missed a home game and even travelled to some regular season road games with the team.

During games, Madison takes part in pre-game introductions, sits with the coaching staff during the game, and attends team meetings during halftime and postgame. Her chemistry with the Bobcats has grown with each experience, inspiring the team to greater heights throughout the season.

This week, Madison and her father Aaron Smith, are with the Bobcats in Sioux City, Iowa for the NAIA Division II Women’s Basketball National Championship. Just like she has all season, Madison will be with the Bobcats every step of the way.

“Madison’s experience being an honorary teammate of the Bobcats is hard to put into words other than perfect,” said Jennifer Miller-Smith, Madison’s mother and President of the South Florida Chapter for Cure SMA. “Madison has truly loved every aspect of this experience from bonding with the team, respecting the coaches, learning the game of basketball, and feeling like a true part of something special. The staff at St. Thomas University Athletics and the Women’s basketball team are very special people and that trickle-down effect of how they treat Madison has made this experience even better. We are thankful to everyone involved and to Team IMPACT for making this happen.”

Every player on the team has contributed greatly to this cause and formed a personal relationship with Madison. As a team, the Bobcats have helped raise awareness for Cure SMA, even recording a SMash SMA video that went viral on social media channels. Freshman guard Emma Grimes, the team’s player liaison for the overall project, was recently presented with The Sun Conference’s Champions of Character award for her service.

The Women’s Basketball team’s commitment toward creating an extraordinary experience for Madison is the latest example of STU’s commitment to the NAIA Champions of Character program. The program’s core values of integrity, respect, responsibility, sportsmanship and servant leadership were all on display during this project.
Maybe Mom was Right

By Shane Burcaw, A Special to the Morning Call

“Sometimes we just have to do things we don’t want to do.”

This simple phrase — spoken by my mother at least a billion times throughout my childhood — has been on my mind a lot lately. She used to unleash it whenever my brother or I complained in that whiny tone of voice that only annoying little kids can create.

“Do we have to eat our vegetables?”

“Do we have to go to school tomorrow?”

“Do we have to take a bath? It has only been three days!”

She whipped out this phrase so often that I began to resent it. As I grew up, I developed a healthy sense of stubbornness that caused me to resist things that authority told me I had to do. Don’t get me wrong, I wasn’t a misfit. But I found ways to avoid undesirable activities.

Most of the time, my stubbornness came out when faced with commands regarding the treatment of my disease. I resisted physical therapy, I resisted wheelchair changes, I resisted a feeding tube, and most recently, I resisted the use of my BiPAP machine.

A BiPAP (which stands for bilevel positive airway pressure) is a device intended to help people with weak lung muscles breathe overnight. Sounds pretty important, right? Well, about five years ago, my muscle disease progressed to the point where doctors became deeply concerned with my ability to breathe while I slept.

A sleep study confirmed this: My lungs basically took the night off as soon as I shut my eyes.

That’s super-duper unhealthy and highly dangerous. Not getting enough oxygen overnight can cause a myriad of serious health issues, especially for people with spinal muscular atrophy. My doctors ordered me to begin using a BiPAP overnight.

Being the stubborn 18-year-old that I was at the time, I decided that using a BiPAP was not on my agenda. It was uncomfortable, and even worse, uncool to be strapped to a machine every night.

I wasn’t waking up feeling crappy, so why bother with this new routine? Furthermore, using the BiPAP felt like my disease was getting the best of me, which I wanted to avoid at all costs.

Time passed (as it tends to do), and the BiPAP sat in my room, collecting dust. My refusal to use it even became a sort of bragging chip that I flaunted to elevate my own status as a person who was conquering his disease. If you look back at the first blog post I ever wrote, I poked fun at how awesome I felt for not needing my BiPAP, despite doctors’ insistence that I use it.

As I said, that was about five years ago. My lungs have deteriorated further since then, and I still wasn’t using my BiPAP. Clearly, I was being an idiot. But in the past month I have started to recognize my immaturity. Here is how that happened:

It began with my dreams. Lately, my dreams have been increasingly vivid and graphically horrifying, not so much monsters and demons, but unsettling depictions of people I know in desperate situations — dark images of drowning and falling and being trapped. Each morning I’d awaken in a panic, feeling like death was hovering somewhere close, just out of sight, but present and almost palpable.

Physically, my condition was also worsening. Several nights last month I had waken in the middle of the night with significant phlegm in my lungs, my chest heaving as if I wasn’t breathing.

On top of all this, I was simultaneously going through a rough stretch of time on an emotional level, ending a long relationship with someone I loved deeply. The result of the breakup was plenty of alone time to ponder my future, my goals, my shortcomings and more specifically, my developing complications with breathing overnight.

Something needed to be done. That was obvious. But giving in to the BiPAP felt like such a step backward. I didn’t want to admit I was getting worse.

Luckily for me, the universe must’ve seen me floundering about like an atrophied fish out of water, because it connected me with someone who has shaken me from my daze and inspired me to begin taking better care of myself.

Her name is Ida, and she works as an intensive care nurse in Philadelphia. I met Ida after she reached out to offer assistance as a volunteer for my nonprofit. Since then, we have become best friends in a short span of time.

From day one of our friendship, Ida has gently pushed me to give the BiPAP another try. I met her prodding with the same stubborn disposition that I’ve always had. But eventually she wore me down, and I decided to try it for an hour while watching Monday Night Football.

The rush of beautiful air into my lungs caught me off guard. A few jagged coughs escaped as my lungs, which had been lying mostly dormant for so long, suddenly came alive. Wow, so this is what it feels like to actually breathe! The tempo of my breathing evened out, and for two hours I sat in total relaxation, letting the machine fill my lungs with glorious oxygen.

As of today, I’m working diligently toward using the BiPAP throughout the night, and I could not be any happier to know that I’m doing my body such good.

All of us have those things that we should be doing, but aren’t. Whatever your reasoning is, I challenge you to put aside your stubborn opposition, and just give it a try. Using my BiPAP is not comfortable or fun, but it’s important, and it’s going to help me live a lot longer than I would if I kept up my childish defiance.

As my mom would say, “Sometimes we just have to do things we don’t want to do.”

Shane Burcaw is a Bethlehem local using humor to change the world. His column about life and disability in the Lehigh Valley will appear occasionally in The Morning Call. Contact him: shane@burcaw@gmail.com. Twitter: @shaneer28 and his Instagram: shaneburcaw
Alumna Becomes Advocate, Inspiration For Those With Disabilities

Columbian College graduate Alexa Dectis will pursue law degree on West Coast.
By James Irwin

Her classmates were milling around the National Mall, their black graduation robes glistening slightly from an on-again, off-again drizzle that accompanied the early summer heat. Alexa Dectis, meanwhile, was backstage, getting laptop recommendations from Apple CEO Tim Cook.

“He gave me advice on which one I should buy for law school,” Ms. Dectis said of her conversation with Mr. Cook, the George Washington University Commencement speaker. “He suggested the new gold MacBook that had just come out.”

An hour earlier, as part of the on-stage Commencement exercises, Ms. Dectis, B.A. ’15, had helped present Carole M. Watson, Ph.D. ’78, with an honorary degree from the university. Now, she was chatting up Mr. Cook about which new computer she should purchase. For the former child actor-turned aspiring lawyer, it was a fun moment from a special day, one made possible after years of overcoming a genetic disorder that robs her of motor strength.

‘I knew I wanted to come here’

The conversation with Mr. Cook was about function. Ms. Dectis lives with type II spinal muscular atrophy. Her body cannot regrow stem cells after they die, causing her to progressively weaken. Lightweight hardware is important for her, she told Mr. Cook. She also explained that Apple’s accessibility features had been helpful during her time at GW. His recommendation of the new MacBook was about weight and utility.

“He told me the new 12-inch MacBook was just over two pounds and contained all the power and storage I needed to succeed as a law student,” Ms. Dectis said.

It was information she valued. Ms. Dectis has spent most of her life finding ways to work through and around her spinal muscular atrophy. As a child, she could walk. Today, she can’t stand.

The disorder makes just about everything difficult. But Ms. Dectis sees it as a hurdle, not a deterrent. At age five, shortly after she began to lose strength in her legs, she began a decade-plus long career as a child actor, starring on Sesame Street and appearing on Nick Jr. It sparked an interest in the entertainment industry that continues this day.

She grew up in Allentown, Pa., and applied to 18 colleges. Ms. Dectis enrolled at GW because of the university’s disability support services.

“I realized how wheelchair-accessible it was and how the [Office of] Disability Support Services could accommodate me and help me succeed,” she said.

“The moment I got to GW, I knew I wanted to come here.”

Supporting students with disabilities

Mounted on the inside wall of the Office of Disability Support Services’ street-level office in Rome Hall is an encased Rawlings Adirondack Pro baseball bat. A metallic plaque wrapped around the top of the barrel bears a simple message: “NEVER Stop Going To Bat For Your Students.”

Susan McMenamin has been promoting that culture of advocacy and empowerment for students with disabilities at GW for 11 years, first as the associate director of DSS and now as its director, following the 2014 retirement of longtime director Christy Willis. In addition to providing equal access to people with disabilities, she said, DSS has a complementary mission to establish a climate of inclusiveness. DSS-registered students at GW have increased from around 500 in 2004 to more than 900 in 2014.

Ms. McMenamin met Ms. Dectis the spring prior to her freshman year.

“Her family was extraordinarily proactive in recognizing the full spectrum of accommodations and services she would require,” Ms. McMenamin said. “Those were important steps in ensuring she would have an equal opportunity to be successful here.”
The accommodations began, as they often do, with housing and broadened to include personal care and technology-based support. Incorporating voice-command assistance to help with writing assignments and exams were added as Ms. Dectis’ motor issues became more problematic, Ms. McMenamin said.

“Changes in her functioning meant we were always redesigning the plan,” she said. “The key to Alexa was there was nothing about her individual circumstances that she ever outwardly suggested was a roadblock—I say outwardly because I know she has struggled. But she was always able to maintain that positive outlook, which I found highly commendable.”

An advocate for inclusiveness

DSS also encouraged Ms. Dectis to apply for the Mei Yuen Hoover Scholarship, established in 1999 by its namesake, a 1945 alumna, to provide support for students with disabilities who wish to study at the Columbian College of Arts and Sciences. Seventy-one students have benefitted from Hoover scholarships since it was created.

It made everything a little easier, Ms. Dectis said.

“I have so many other things that I have to worry about in my life,” she said. “The scholarship made it possible for me to attend GW—it allowed me to pay for some of the accommodations I need. Because my tuition is lower, I can afford to live independently in a city, despite having a physical disability. From there I could do really incredible internships and take classes with renowned professors.”

Ms. Dectis thrived as a communications major. Classes with adjunct professors Cheryl Wood and Betty Van Iersel, she said, provided her with practical skills. A course on persuasion taught by Clay Warren, the Chauncey M. Depew Professor of Communication, challenged her to think critically. Internships with Discovery Communications, SiriusXM Radio and Clear Channel—secured through the Center for Career Services and Career and Internship Fair—opened her to opportunities in the media and entertainment industries. She even secured a small role in the 2013 movie, “Admission,” starring Tina Fey, Paul Rudd and Nat Wolff.

Through DSS, Ms. Dectis also became a campus public figure for students with disabilities. She served on the office’s speakers bureau, a group of DSS students who meet with administrative, advising and student groups—including the Board of Trustees and career services staff—to share their stories and promote education of working with disabilities. She was a finalist for the role of student speaker at Commencement.

“Alexa came to be known as a student who had a real willingness to serve as a spokesperson for how we can create the kind of climate at this university that is fully inclusive and respectful of people with disabilities,” Ms. McMenamin said. “She was the kind of student here who, I think, became a role model, and she was somebody who many of us on staff really looked up to, for her determination, positive outlook and her drive and commitment.”

Going west

On May 17, Ms. Dectis took her prestigious spot on stage at Commencement, reserved for students who introduce honorary degree recipients. Three months later, she completed her internship in the talent management department at Discovery and then flew to Los Angeles. On Monday, she began her first day of classes at Chapman University’s Fowler School of Law, which she hopes is the first step to securing a career on the West Coast as an entertainment lawyer.

It’s been a busy summer.

“It’s like a hurricane,” she said.

During her final few weeks in Washington, she spent time reflecting on her GW experience. She also finally purchased that new 12-inch MacBook. Mr. Cook’s assessment, she said, was accurate.

“I can maneuver it with ease, and utilize several other additional accessibility features integrated in the system, including the sensitivity settings for the trackpad and the ‘speech’ feature,” she said. “Thanks to another ‘Only at GW’ moment, I had an encounter with Tim Cook and received personal advice that will contribute to my success.”

Providing more students with access to college—and to enhanced academics and career opportunities once they get there—is a central tenet of Making History: The Campaign for GW, a $1 billion philanthropic effort that will bring the university into its third century. Today, nearly two-thirds of all undergraduate students receive need- or merit-based financial assistance.
On August 12, 2015 Lilee Ford had what she has deemed “the best day” of her life. She got to hang out with the special effects wizards at SynapseFX on the set of one of her favorite TV shows - SyFy channel’s Z Nation. She was able to observe the artists expertise and even got to help apply make up to a zombie extra! She also got to meet a number of cast and crew members. These wonderful people showed their support when told of SMA Awareness month and graciously posed for photos with Lilee’s “CureSMA.org” sign.

Diagnosed with SMA type II in 2004, Lilee had been showing signs of progression at age 15 months when her development suddenly ‘hit a wall.’ Many doctors and tests later, the call was received on December 20, 2004 telling me of her diagnosis and the prognosis statement available at that time. I was devastated. I knew nothing of this Spinal Muscular Atrophy except having seen some children featured on the MDA telethon just two months earlier that resonated with me for an unknown reason.

Cure SMA, formerly Families of SMA at the time, was a life saver for me after a dear friend gifted me with a computer and internet access so that I could connect with support and information.

Conservation of strengths and limited germ exposure became my prime goals with Lilee’s care. Her unrelated diagnosis of developmental delays and learning disabilities including dyslexia would come later but, coupled with her limited physical activity, has lead to Lilee being the creative, self teaching, film loving young lady she is today.

Her appreciation of make-up began when she was quite young. She started with ‘fun makeup’ for little girls and would apply to herself and her dolls faces. Then she became interested in cosmetology and started collecting an actual makeup kit. She made up herself and started on the neighbor kids, soon teaching herself how to create fake bruises and cuts by mixing eyeshadows and using lipsticks for blood. Growing up in a family of film buffs, Lilee had early exposure to many film classics. With her older siblings, Newt and Adam, leading the way, Halloween became the event of the season and Lilee’s get-ups became more and more intricate as her interest in science fiction/horror special effects makeup grew. And her resolve to go to an effects makeup school to be a professional make up artist has also grown. This is what Lilee would like to be when she grows up.

This last year, Lilee and our family began watching the TV series Z Nation and were instant fans of the show and it’s talented cast and the quality of the zombie effects. The production films in nearby Spokane, WA and an internet friendship with one of the cast members lead to being allowed to visit the set for the special day. Since the visit took place during the month of August Lilee and I decided to ask if they would support the SMA community and they were all happy to do so. Lilee loved experiencing make up artistry for the day and as it is what she wants to do in her life, it’s what I hope and pray for her to be able to do to the fullest. Support of Cure SMA and the research for treatment medications and a cure is what we ask of all who are a part of our lives and the lives of those in the SMA community. A BIG Thank You to those who stand with us!

SyFy Channel began airing Season 2 of Z Nation on Sept 11.

Submitted by: Annie Ford of Washington
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.
August is SMA Awareness Month. Two months ago we hadn’t heard of it; now we are very aware of SMA. As the month draws to a close, we are planning our two month old infant’s funeral.

Gabriel Anthony Allen, named after a mighty angel and a peaceful grandfather, was born June 24, 2015. It was a wonderful pregnancy, with a first kick at 11 weeks then more from 14 weeks on. Joanna felt like she could be pregnant forever. There were a few days at 38 weeks where Joanna had to do kick counts but he always passed them, and she chalked it up to being so distracted at work she missed the smaller movements of a term fetus running out of room. Aaron and Joanna explained pregnancy to their first child, Eleanor, who was 2 and a half years old; Eleanor was fascinated by a pregnancy picture book. They told her that the baby would come when the weather was hot; she would teach the baby to eat food, to talk, and dance like her.

Joanna’s coworkers and family practice patients asked her what she was having: boy or girl? And twenty times a day, she replied, “It doesn’t matter, as long as it’s healthy.” And she meant it.

His due date was June 24, 2015: Labor was induced but unmedicated, and went as smoothly as labor could possibly be. Joanna even was able to help deliver Gabriel up onto her tummy and hold him, just as she had hoped. He took to nursing quickly with a strong suck. Perfect.

Mighty Gabriel.

Later the nurses were talking to each other about their assessment: “He’s a bit floppy.” But he passed everything else. He gripped a finger nicely, though delayed by a second. He bent his elbows, but didn’t lift up his arms at the shoulder. He looked to Joanna like a baby with that post-feeding content relaxed posture— all the time.

Gabriel’s doctor and multiple residents checked him over. When it didn’t resolve within 24 hours (pitocin effect would have worn off by then) we met a neonatologist and ordered simple screening labs. Joanna wanted to rule out anything that could be fatal in the first few days of life. The labs were normal, and both parents breathed a sigh of partial relief. A larger panel was sent out and after two extra days in the hospital with EKG and respiratory monitors that beeped for false positives an MRI of the brain he looked weak but stable. Aaron’s mom recalled that he was “floppy” at birth and while developmental milestones were delayed, he eventually caught up and walked at 18 months. The family hoped that Gabriel would develop the same, and went home to enjoy their new family life.

Joanna, Aaron, Eleanor and Gabriel found a new rhythm. Gabriel disliked being in bassinet at night, and we took turns holding him all night. He slept contentedly. During the day the family went outside for walks, played at the beach near home, or laid on the lawn under the tree canopy. They loved skin-to-skin time and holding him close in the hug of the Moby wrap. Gabriel had his first doctor’s visit, talked about their hospital story and Aaron’s floppy start and resolution. There was hope that everything would resolve, and we would watch Gabriel roll, walk, and talk, then grow up to be tall, dark and handsome, with a magical wonder about the world and fantastic sense of humor. He would be a mechanic, painter, photographer, musician, doctor or lawyer; it didn’t matter, so long as he was happy.

At two weeks old, Gabriel had the first cyanotic episode while nursing and he choked on let down; it lasted 10 seconds and involved only the lips. Joanna requested an echocardiogram from their doctor, but was told to watch more. She was watchful and anxious. Both parents were sleep deprived and worried about him. The next night she struggled to keep him latched for a feeding over an hour; a floppy baby who wouldn’t nurse could be sign of something very wrong. But his subsequent feedings were good, and she remained watchful.

Four days later, he had another brief episode of lip cyanosis, not involving feeding. Joanna brought him to the doctor’s for a weight check, and he was growing an ounce a day and voiding and stooling well. An echo was ordered. They went home and had normal family time. They brought him to picnics and showed him proudly to neighbors, who gasped at how beautiful he was.

Gabriel’s story took a turn that night: Tuesday, July 14. He was held in a sling with mom; he grunted and when mom looked down, his whole face was blue. Joanna looked down, his whole face was blue and he was working to breathe. She lifted him up and coached him, “c’mon honey, turn pink.” It took 30 seconds. She laid him down on the bed and watched: his abdomen was working hard, he had retraction, and his chest was sucking in with each breath. They brought him to the ER, and were whisked into the trauma room with 5 people waiting for them.

In Memoriam
Gabriel Anthony Allen
June 24, 2015 – August 27, 2015
It was recommended he be evaluated at the Children’s Hospital 2 hours away; while waiting, Gabriel’s vitals were normal, and his breathing improved, and he nursed again. “Won’t it be funny,” they thought, “when he was sent home again after all this?” But as the transport team arrived, Gabriel had another cyanotic episode, with oxygen saturation of 88% and retractions. This was scary. They kissed him and he left with the helicopter team.

Aaron and Jo drove in the early morning hours on to meet Gabriel in the PICU. He seemed to be doing better and was able to breast-feed and swallow ok, so they went upstairs to the Step down Floor. They continued feeding and nursing, touching and talking to him on the bed. He was weaned off oxygen and IV fluids stopped. But overnight he struggled again to nurse only 10 minutes, and then refused even the bottle feeds. He looked so tired, Joanna worried he was dying then.

Joanna and Aaron talked to a hospitalist, neurologist, and geneticist. There was a possible diagnosis of Spinal Muscular Atrophy, and that test was best to get first before anything else. Aaron grieved normal development, and worried that he might be permanently weak for his lifetime. Then Gabriel had another cyanotic episode after an NG feeding, and was moved back to the PICU for rest on a noninvasive ventilator. He got a mask and a bionic helmet.

The next day he was more awake, and he was given a temporary nasojejunal feeding tube, but still getting breastmilk. Eleanor visited her brother today, and more family over the next few days. He got a bath, and spent a little time off the ventilator and he slept on his tummy a while, then on mama; they both loved it.

The next day was Monday; a big day, a tough day. Joanna and Aaron talked to six groups (pretty much their whole team). They discussed respiration, ventilation, muscle weakness, and the differential diagnosis. They discussed SMA, how the spinal nerves are missing a gene and a protein that keep the nerves alive. Without the nerves, the muscles weaken and die. Aaron realized then that Gabriel’s life would not likely be decades but years, or months. He grieved. They also talked about treatment options for the respiratory issues: he might be able to use a nasal cannula, but he would eventually need a ventilator all the time, and we could choose to have a tracheostomy performed. There is so much to have to think about for Gabriel’s comfort and care. Their brains hurt, but their hearts hurt more.

The next few days, Aaron spent as much time with Gabriel as he could. He was desperate to get all the awake time, which we called “eyeball time.” They talked to multiple specialty doctors and techs every day, for planning. They couldn’t discuss the difficult choices of ventilator or trach-- just couldn’t do it until they had a diagnosis. It was crisis time, and Joanna and Aaron could handle only the immediate moment. The future, with possible SMA or something worse and possibly death in the short term, was too tough. They sent Eleanor to stay with her grandma an hour away, in order to concentrate on Gabriel. This was tough on all of them.

Joanna and Aaron were told Monday that the blood test might take 2 weeks or more; that seemed so long and tortuous, but at the same time they didn’t want to lose the safety of not knowing the scary future. Then Friday, the lab test came back confirming SMA. The doctors were as kind about the diagnosis as possible. Joanna and Aaron cried, knowing that their son would not live decades, but years or possibly only months. Their little one could feel and hear and see, but would lose movement and speech. Joanna grieved, and wanted at least to hear his voice and cooing, and see a smile. He was only 4 weeks old.

They talked to doctors about a drug trial with great potential; the Columbus study is a treatment that actually inserts the new SMN1 gene in the DNA. Joanna and Aaron talked with Dr. Mendell, who explained the trial, its side effects and the inclusion criteria. Gabriel needed so much support from the ventilator that he may not qualify; even if they could get him there, he couldn’t get the treatment until August 24th because another child was getting the treatment.

The FDA requirements for the study stated they could treat only 1 child every 3 weeks. Gabriel would have to get off the ventilator and wait another month. If he could.

There was another study, in Chicago; it was a Phase 2 study, which meant Gabriel might get a placebo. But he was excluded because his symptoms started within a week of birth. Two hopes, now down to half a hope.

During the third week of their hospital stay, Gabriel started doing “sprints” off the ventilator; he breathed on a high flow nasal cannula that gave him CPAP to keep his throat from obstructing with breathing. He got up to 4 hours at a time off the ventilator. But he became...
Gabriel's eyes shone when his uncle
recognized. Aaron and Joanna lived for
little. He watched his parents with open
eyes, and lifted an eyebrow to show
recognition. Aaron and Joanna lived for
those little eyebrows. Family came by,
and Gabriel's eyes shone when his uncle
played bicycle kick. He wanted to play,
like any other baby. He had colick and
hated to poop, like any other baby. He
just happened to also have SMA and
respiratory failure.

He started having some very scary
episodes of oxygen desaturation, once
while Aaron was holding him, minutes
into a sprint on the high flow nasal
cannula. The respiratory therapist and
nurse brought him quickly to his bed
and gave him a few puffs of positive
pressure ventilation with the bag and
suctioned his mouth and transitioned
him back to the nasal mask. Aaron shook
and cried. But the respiratory therapist
insisted Aaron hold Gabriel; “you cannot
be afraid to hold your own son.” No
more sprints for Gabriel.

A few days later, after we changed code
status to "no code," he had an episode
while friends were in the room; we
were afraid he was dying then, but it
passed. And a third episode even while
his mask was on, because of a possible
mucus plug. Oxygen would drop and his
heart raced, which we knew because of
those monitors; they constantly beeped
and alarmed, and ruled our lives. It was
traumatic.

Gabriel started chest percussion therapy
and had no more acute desaturations.
But he couldn't take home his current
mask and ventilator. There was no
mask made small enough for our little
Gabriel's face: babies used a nasal mask
in the NICU, but didn't take it home;
and older children used masks for sleep
apnea, but not all the time. There was
just not a market for infant nasal masks.
It was so frustrating! Ten very smart
pulmonary specialists and techs talked,
and put together an adapted mask, but
Gabriel failed with increased respiratory
rate and poor ventilation; his carbon
dioxide level went high in less than 5
hours. We despaired that Gabriel would
live out his lifetime at the hospital.

Two days later, we tried a different
mask, and it fit right, and could support
Gabriel's own breaths. He actually
seemed more comfortable. This was our
fourth week in the hospital. The team
shifted gears to getting us home; we met
with multiple specialists every day, this
time to plan home nursing, hospice care,
and home equipment. Aaron and Joanna
learned to do respiratory and oral care,
so we could nurse him at home. Our
moods were a little higher, as we got
closer to home.

On August 13, after 30 days in the
hospital, we went home. We said
goodbye to doctors, therapists, nurses
and others who had cared for Gabriel.
He left in an ambulance, and was met by
home nurses and equipment providers
who set us up in his new bedroom. He
had a line of windows that faced the
woods. His pack and play was flanked by
comfortable chairs. We added artwork
to the walls. And our monitors helped
watch his oxygen, heart, and breathing.

At first we were anxious; Gabriel stayed
mostly in his crib, and Eleanor was kept
in the other room to avoid pushing
buttons or pulling important cords.
Our nurses came 24-7 to help us. As
time went on we calmed down, and we
welcomed Eleanor to Gabriel's bedside
to hold his hand, talk to him and sing
"Twinkle Twinkle." He loved it. We
read books to both children. We sang
and played music for him. We wanted
Gabriel's life to be one of quality, even if
it was short.

We became braver. We shifted his crib
equipment six feet to the back door
and brought him out to the back deck
on his pillow. The baby was outside! He
looked at trees, and at his parents who
made silly faces. A few days later, we
put him in a wagon given to us by Cure
SMA and brought him outside the house
for an hour under the trees, where he
had rested with Joanna and Eleanor five
weeks before. We had a photography
session for precious family photos. And
after a week home, we brought Gabriel
in his wagon across the street to our
beach, to rest in the breeze. Eleanor played in the water as kids are supposed to do in the summer. We all breathed.

Gabriel had a few good days. He watched us with open eyes and lifted eyebrows. He was especially intrigued by a bright yellow smiley face balloon. He moved his arms more than he had before and almost held a crinkly ribbon ball. He smiled at a nurse who wiped his lips, and they played a game with it. Precious memories.

The next day, the weather turned cold and cloudy, and Gabriel’s respiratory rate increased. He was working harder to breathe, and was anxious. We used morphine to calm the air hunger, and he rested. But he was visibly using his abdominal muscles more, and this meant his diaphragm was getting weaker. The ventilator supported breaths that it could sense, but he was moving so little air that there were more and more breaths that were not supported. He worked harder. He spent two days mostly asleep, and needed medication for anxiety and air hunger. He had episodes of low oxygen and heart rate. He looked pale and tired. He seemed to be suffering. We listened, with sad but loving hearts.

The next day was clear, and warmer, and sunny. He was 9 weeks old, and we had been home for two weeks. We went outside under the trees. He was sleeping and remained so until the end. We were visited by bugs and slugs and a woodpecker, and even a hummingbird so close we could hear its wings. We played our favorite songs for him. He stayed with us for the whole afternoon, breathing slowly. And as the afternoon faded, so did he. He died in our arms, loved for every moment of his two months of life.

No child should die in his infancy; it’s not fair that a beautiful perfect boy would die after only 9 weeks of life. But genetics isn’t fair. No parent should have to bury their infant. We grieve, open and honestly, for the first words he didn’t say, the learning about the world he couldn’t do, and for the man he would have grown up to be. We will forever miss our Mighty Gabriel.

His short life has meaning. He has taught us how to love deeply, and to live in the moment as a pure experience. He has touched family and friends and friends of friends. They hold their children closer, and are speaking to their families of love and life. Poems and songs will spread the love. And he will, hopefully, inspire others to work on treatments for SMA, so that no other person should die of it, no child be affected by it, and no more parents know this loss.

Joanna Nigrelli, mother of Mighty Gabriel
“Special Child”  
By: Sharon Harris

You weren’t like other children  
And God was well aware  
You’d need a caring family  
With love enough to share.  
And so he sent you to us  
And much to our surprise  
You haven’t been a challenge  
But a blessing in disguise.  
Your winning smile and laughter  
The pleasures you impart  
Far outweigh your special needs  
And melt the coldest heart  
We’re proud that we’ve been chosen  
To help you learn and grow  
The joy you have bought to us  
Is more than you can know  
A precious gift from heaven  
A treasure from above  
A child who’s taught us many things  
But, most of all, “Real Love”.

Adisyn Grace Cavey  
April 25, 2008 - December 4, 2014

Love you so much baby girl.  
Enjoy those beautiful wings.

Cure SMA,  

Please accept this donation in loving memory of our daughter Emily Coddington. Emily had Type I SMA and earned her angel wings in February 2010. Emily would have celebrated her 6th birthday on 5/4. We are proud to be a part of the Cure SMA Family and appreciate all you do for SMA families.

Sincerely,  
Julie & Terry Coddington of Pennsylvania

In Memorian  
Ta’Bria Neosha Collier  
November 28, 1994 – May 19, 2014
Artist Shirks Fame To Invent Tools That Allow Kids With Disabilities To Paint

When Madison was first diagnosed with spinal muscular atrophy, her doctor didn’t know a whole lot about the genetic condition. She flat-out told Madison’s fearful parents that their baby wouldn’t make it to her 2nd birthday.

“That was pretty tough,” Jennifer Miller-Smith, Madison’s mom, told The Huffington Post.

Seven years later, while the second-grader relies on a wheelchair and faces the disease’s degenerative effects, Madison is “thriving,” her mom proudly shared. A lot of that is thanks to Dwayne Szot, an artist who has committed his career to inventing tools that enable kids with disabilities to paint, draw, blow bubbles — pretty much do anything any typical child gets to do.

Before Madison met Szot, an innovator based out of a small fishing town in northern Wisconsin, the 8-year-old often felt frustrated and helpless. While she wanted more than anything to play with her friends, she was often relegated to the sidelines due to her condition.

SMA causes the body’s muscles to weaken over time, making it impossible to perform such simple tasks as flipping a switch. Those with SMA type 2, like Madison, will never be able to walk or stand up, according to the U.S. Library of National Medicine.

But when Madison met Szot at an SMA conference in Los Angeles two years ago, her world opened up in a way she had always hoped, but wasn’t sure was possible.

Since the late 1980s, when Szot unveiled the first edition of his painting wheelchair, the artist has spent his days building upon his current inventions and developing new ways to engage with kids with limited physical ability.

“What I do in the studio is create a means for a full completeness of experiences,” Szot told HuffPost at an event in west Miami in April. “It’s not just about mark making. It’s about that opportunity to experience and enjoy life to its fullest.”

Szot knew from the time he was a child in the foster system in the Midwest that he would pursue a career in art. But it was one that wouldn’t involve fame or fortune.

“I knew growing up that I was never going to be this kind of art guy who put paintings on the wall in a museum,” Szot said. “I wanted to be the kind of art guy who made something that was going to create social change — that was going to make a difference. And there’d be a usefulness to what I did as an artist.”

Szot was particularly inspired by his foster siblings with disabilities, and how they adapted together to make their everyday routine work.

He recalled how he and the other kids were always late for the school bus. To help his sister with cerebral palsy get there just a bit faster, he started dragging her along in a wagon.

It was those childhood experiences, and simple adaptations, that inform his work today.

Szot, for example, first developed his art roller with a National Endowment for the Arts grant nearly 30 years ago. It involves attaching PVC pipe and a print plate to the base of a walker or a wheelchair. After it’s filled up with paint, the user just rolls and can create a massive mural.

He uses similar technology for the Walk Chalk and Roll, which allows kids in wheelchairs to draw on the sidewalk with chalk.

“It taught our kids that they can do sidewalk chalk and they can create these magnificent paintings and such, with just a little bit of adaptability,” Miller-Smith said of Szot’s tools. “Now that we connected something to [Madison’s] wheelchair — now she can do it.”

When he’s not toiling in his workshop, Szot takes his tools on the road, both around the U.S. and abroad, to show children with a range of conditions that they no longer need to live their lives as bystanders.

Szot’s inventions have taken him as far as Saudi Arabia and Mexico. But this year, his workshops are all based in the U.S. He’s making stops in Detroit, Chicago and Portland, Maine, among other major cities.

This past spring, Szot set up shop at the Patricia & Phillip Frost Art Museum at Florida International University, which allowed Madison to reconnect with the man who changed her life on her own turf.
Together with Miami-Dade Department of Cultural Affairs, the Children's Trust and All Kids Included, the event invited 200 kids, both those with disabilities and without, to play together using Szot's tools.

For parents, participants and museum staff seeing Szot’s work for the first time, the experience was eye-opening.

“I use the word ‘genius’ very rarely,” Jordana Pomeroy, the museum’s director, told HuffPost. “And I think it’s very appropriate in describing the work that [Szot] does with kids with physical challenges.”

Newly diagnosed families that are just beginning to grasp what their children's conditions mean for the long term felt particularly hopeful.

Kaden, 14 months old, was diagnosed with SMA about half a year ago. He’s never crawled or rolled over and will never walk.

Just playing with a toy is a challenge for him since he has to use nearly every muscle to prop himself up and keep himself from falling over, his mom, Katie Myers, said.

But after watching Kaden spend the afternoon painting murals and playing with an adaptive kite, Myers said she felt reassured about her baby’s prospects.

“Being able to see how much he loves life and loves the world, and wants to be a part of the world – it changes our whole perspective,” Myers said. “Despite the situation he’s been given, the world is his.”

Dear Cure SMA,

Johnny Macfarlane’s & Ticker Ba-Aye’s newest bundle of joy, Porter, came into our world six months ago on Halloween 2014. She was and is so beautiful and so sweet! Her full head of dark curly hair framed her large brown eyes and made your heart melt when you held her.

As months past, Ticker and Johnny began to notice that Porter’s development seemed to be slower than what they remembered with her older brother, Forrest. Their pediatrician also began reporting that her motor skills were lagging behind other infants her age. Porter was having trouble holding her head up, moving her arms and legs, or just rolling over.

Ticker and Johnny decided it was time to have their little girl checked out to see what they could do to help her. It was then that they received the devastating news that Porter most likely had Spinal Muscular Atrophy (SMA) type I. We had never heard of SMA, and were overwhelmed to learn that SMA type I is the most severe form of this genetic disorder and is evident in infants within the first 6 months of life. It is caused by a loss of specialized nerve cells, called motor neurons, in the spinal cord and the part of the brain that is connected to the spinal cord. Affected infants are developmentally delayed; most are unable to support their head or sit unassisted. Children with Type I have breathing and swallowing problems that may lead to choking or gagging. Their life expectancy is shockingly no more than 2 years. Without an onslaught of medical interventions (breathing/feeding tubes etc...) she’s expected to live to no more than a year.

They are rejoicing in their daughter’s life and are committed to making her as happy and pain free as possible for as long as they have her. Ticker has written: “I wanted to let y’all know and reach out asking that if it’s within your limits of comfort that you keep her in your thoughts. While there is no cure I do believe in miracles and if nothing else I believe in the power of people and their thoughts to raise the vibration of any given soul to a place of peace and light. She’s just the sweetest, happiest, most perfect little bundle of love and I couldn’t feel more blessed to know and have her as my daughter. She’s already taught me so much and no doubt will continue to... thank y’all so much...”
SHARING

Lylah’s Arms: GVSU students 3D print device for child with SMA

By Dana Chicklas, Fox 17 West Michigan

A one-year-old girl in Hudsonville is building the strength to move her arms in new ways with the help of two mechanical engineering students at Grand Valley State University who built what they call an “Angel Arms Exoskeleton,” or “Lylah’s Arms.”

Holly Gritter told FOX 17 News that her daughter Lylah was born with spinal muscular atrophy or SMA, known as the number one genetic killer in children two years old and under, with one in 40 people carrying it.

Gritter describes SMA as similar to arterial lateral sclerosis or ALS, but in children. Lylah was born at 39 weeks with no signs of any trouble until at about three months old she began to lose strength fast.

Lylah turns two this August, and her mother calls her smart, sassy, and determined. For Lylah to color and hold her blue crayon on her own is something that gives her family hope.

“For her to say, ‘I color,’ — okay, you can color,” said Gritter, “it’s just such a great feeling, especially from a year ago when we didn’t think she was even going to survive.”

A strong-willed little girl, Lylah can move her hands and forearms. But since April, after two graduate GVSU mechanical engineering students created “Angel Arm Exoskeleton” for Lylah, she is strengthening muscles she could not use on her own before.

GVSU students Joseph Kissling and Brooks Schaefer tailored the exoskeleton to Lylah and then used a 3D printer to build the exoskeleton for about $300, with FDA-approved plastic and parts from stores. That would be extremely expensive using any other technique.

“Instead of making it very specific, very complicated, we took a step back and simplified it,” said Kissling. “We made it so that anybody can get hold of it, anybody can use it.”

Kissling and Schaefer said they plan to make their design available for anyone in need to plug-in their own measurements and then help them find a 3D printer to make the exoskeleton at an affordable price.

“For me, it was a reaffirmation of what you’ve really decided to do with your life, why you’ve decided to deal with all the ugly parts of math,” said Kissling. “At the end, you really want to help people.”

Lylah’s family is forever thankful for her independence.

“We say a lot, ‘Never give up.’ As parents, that’s what you do for your children,” said Gritter. “Whatever we can do to help her and make her feel like everybody else, that’s what we do. For her to have her freedom of mobility is our future for her: drive a wheelchair, to use her arms.”

“We really are thankful for every single day,” said Gritter. “Every day we feel lucky that we’re still together as a family.”

Lylah’s family continues to seek to raise awareness of SMA. You can keep in touch with them through their Facebook page: Prayers for Lylah.

GVSU students Kissling and Schaefer entered the 5 x 5 Nights competition for a grant, which they said they would use to get their product to market.
The Dindzans Family
The Koblentz Family
The Gonzalez Family
The Barrett Family
The Ahmad Family
The Meyers Family
The Freitas Family
The Bullock Family
Candle Lighting

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

One of the highlights of SMA Awareness month is the SMA Candle Lighting. The Annual SMA Candle Lighting was held on Saturday, August 8th. Many families and SMA organizations around the country participated by lighting a candle at sunset to remember those who have lost their battle with SMA and to honor those with SMA who are still here fighting everyday! Here are all the great pictures that were posted to the Cure SMA Facebook page!
SMA AWARENESS MONTH
Candle Lighting 2015
Cure SMA Welcomes the North Jersey Chapter

Cure SMA is pleased to announce the creation of a new chapter in northern New Jersey, serving families and supporters with zip codes starting with 07, 085, 086, 087, and 088.

Led by chairpersons Kara Hartnett (family support), Anna Pham (fundraising), and Zainab Jaffar (communications), the chapter is excited to begin helping families throughout their area, and fundraising to support research to find a treatment and a cure for SMA. The chapter is already hard at work on their first North Jersey Chapter Walk-n-Roll, to be held in June 2016.

With the addition of the North Jersey chapter, Cure SMA now has 34 chapters throughout the United States, serving our 110,000 members and supporters. Chapters provide a base of community and support for families affected by SMA, whether sharing practical advice or simply having someone nearby who understands what you are facing. Chapters often work together to organize Walk-n-Rolls or other fundraising events. Chapters also keep their members informed of opportunities for local advocacy and awareness.

If you’re in northern New Jersey and want to get involved in the new chapter, email northjersey@curesma.org. If you’re in another area of the US, check out our chapter page to find the chapter closest to you. If you’re interested in starting a chapter or have any other questions about chapters, email fundraising@curesma.org.

Cure SMA Welcomes the Virginia Chapter

Cure SMA is pleased to announce the creation of a new chapter in Virginia. This chapter will also serve residents of Washington DC.

Led by chairpersons Jessica White (family support), Jim Ferry (fundraising), and Kyle and Laura Derkowski (communications/awareness), the chapter is excited to begin helping families throughout their area, and fundraising to support research to find a treatment and a cure for SMA. The chapter is already hard at work, and held the first Virginia Chapter Walk-n-Roll in September.

With the addition of the Virginia chapter, Cure SMA now has 34 chapters throughout the United States, serving our 110,000 members and supporters. Chapters provide a base of community and support for families affected by SMA, whether sharing practical advice or simply having someone nearby who understands what you are facing. Chapters often work together to organize Walk-n-Rolls or other fundraising events. Chapters also keep their members informed of opportunities for local advocacy and awareness.

If you’re in Virginia or Washington DC and want to get involved in the new chapter, email virginia@curesma.org. If you’re in another area of the US, check out our chapter page to find the chapter closest to you. If you’re interested in starting a chapter or have any other questions about chapters, email fundraising@curesma.org.

Thank You!

Thank you to Kara, Anna, Zainab, Jessica, Jim, Kyle, Laura, and all the chapter officers who work tirelessly on behalf of our community.
Thank You to Jessica Moyer and Family

A note from Rich Rubenstein, Kenneth Hobby, and the entire Cure SMA team:

Cure SMA has had the honor to have Jessica Moyer and her family as one of our volunteer chapter leaders for almost 14 years. Jessica has been critical to the success of our organization, thanks to her efforts locally and nationally. Cure SMA would not be where we are today without her support, guidance, dedication and compassion.

When her son, Steven, was diagnosed with SMA type I back in 2001, Jessica started the South Jersey/Delaware Chapter to provide support, awareness and resources to Cure SMA. Jessica has always gone above and beyond in her role as President of the chapter by visiting families when newly diagnosed or when admitted into the hospital, speaking with families for hours on the phone or speaking at the Annual SMA Conference on how to care for the caregiver. She’s also helped fund teddy bears for the Children’s Program at conference and continues to raise awareness in many ways throughout her community.

In addition to these amazing outpourings of support, Jessica, her family, and friends have worked tirelessly to help raise almost $2 million for SMA research and family support. They have hosted numerous events including their annual Swing for a Cure Golf Tournament, Zumbathons, cocktail and dinner events, and the Delaware Marathon. She and her family—Jason, Isobel, Blaise and Jake—have also personally supported every event throughout the chapter.

After this amazing tenure of service as the South Jersey/Delaware Chapter President, Jessica will be stepping down to focus on her family. Though Jessica will no longer be leading the chapter, she will continue her support by organizing annual fundraising and awareness events, as well as providing support to grieving families and serving as the type I contact for newly diagnosed families within the chapter. Thank you to Jessica, and to all of her family and friends, for helping lead us closer to a treatment and cure for SMA!

Gratefully,
Rich Rubenstein, Chair of the Board of Directors
Kenneth Hobby, President

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.
Make Planning a Fundraiser Easy and Fun

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

Merchandise
- Charcoal Cure SMA T-shirt $20
- Purple Cure SMA T-shirt $20
- Oversized Cure SMA Magnet $4
- White Cure SMA T-shirt $20
- Cure SMA Beach Towel $20
- Cure SMA Bracelet $2
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.

Alabama
P. O. Box 71918
Tuscaloosa, AL 35407
205.979.6493
Jennifer Patrick, President
alabama@curesma.org

Alaska
PO Box 200632
Anchorage, AK 99520
907.720.5019
Meghan Lowber, President
alaska@curesma.org

Arizona
PO Box 43861
Phoenix, AZ 85080
602.314.4902
Angel Wolff, President
arizona@curesma.org

Carolinas North & South
PO Box 30157
Winston-Salem, NC 27130
252.303.0014
Jennifer Lee, President
carolinas@curesma.org

Capital Region, satellite of the Greater New York Chapter
Amy Cunniff, President
gnycapitalregion@curesma.org

Chesapeake
PO Box 354
Cockeysville, MD 21030
410.371.7946
Tina & Derek Lewis, Presidents
chesapeake@curesma.org

Connecticut
PO Box 124
Rowayton, CT 06853
Mary Ellen Barrelle, Treasurer
connecticut@curesma.org

Georgia
PO Box 1249
Mableton, GA 30126
404.217.3130
gorgia@curesma.org

Greater Florida
18865 State Road 54 #115
Lutz, FL 33558
727.368.1888
Audra Butler, President
greaterfl@curesma.org

Greater New York
4 Rutland Avenue
Rockville Centre, NY 11570
631.676.6474
ToriAnn Accardi, Family Support Chair
greaterny@curesma.org

Illinois
PO Box 684
Grayslake, IL 60030
847.373.3762
Janet Schoenborn, President
illinois@curesma.org

Iowa
PO Box 326
Johnston, IA 50131
iowa@curesma.org

KANSAS CITY
P.O. Box 18485
Raytown, MO 64133
816.529.1834
Kristal Wilson, President
kansas@curesma.org

Louisiana
PO Box 1189
Prairieville, LA 70769-1189
225.937.0725
Krista Scurria, President
louisiana@curesma.org

Michigan
PO Box 500
Ada, MI 49301
800.886.1762
Holly Schafer, Vice President
michigan@curesma.org

Minnesota
PO Box 32813
Ponca, MN 55422
Jonathan Schwerk, President
minnesota@curesma.org

New England
PO Box 2902
Woburn, MA 01888
781.476.2174 / 877-256-9111
BJ Mirabile, President
newengland@curesma.org

New Mexico
PO Box 90845
Albuquerque, NM 87199
505.353.1128
Natasha Abruzzo, President
newmexico@curesma.org

Northern California
540 Talbot Ave
Santa Rosa, CA 95405
707.571.8990
David Sereni, President
ncalif@curesma.org

North Jersey
PO Box 86
Metuchen, NJ 08840
Kara Hartnett, Family Support Chair
northjersey@curesma.org

North Texas
5575 LaJolla Dr.
Frisco, TX 75034
972.414.0897
Michele Erwin, President
northtexas@curesma.org

OKI (Ohio, Kentucky & Indiana)
PO Box 541012
Cincinnati, OH 45254
513.753.8222
Kevin & Beth Lockwood, Presidents
oki@curesma.org

Pacific Northwest
P.O. Box 82
Olalla, WA 98359
253.857.9365
Russ Hargrave, President
pacwest@curesma.org

Pennsylvania
PO Box 721
Bangor, PA 18013
866.657.8113
Rocky Mountain
PO Box 941
Morrison, CO 80465
877.591.4023 (toll free)
Karen McKoy-Negrin, President
pennsylvania@curesma.org

Southern California
12611 Cardinal Avenue
Garden Grove, CA 92843
714.390.5179
Rick and Autumn Montoya, Presidents
scalif@curesma.org

South Florida
PO Box 268122
Weston, FL 33326
954.444.5937
Jennifer Miller Smith, President
southfl@curesma.org

South Jersey & Delaware
PO Box 538
Medford, NJ 08055
Nicole Cheshlock, Vice President
southernsey@curesma.org

Tennessee
PO Box 7025
Nashville, TN 37201
865.945.7616
Sarah Bogess, President
tennessee@curesma.org

Texas
PO Box 1242
Liberty Hill, TX 78642
512.498.4934
Kelly Coggin, President	
texas@curesma.org

Utah
435.901.4661
Shane Barber, President
utah@curesma.org

Virginia
PO Box 1695
Woodbridge, VA 22195
571.991.0604
Jessica White, Family Support Chair
virginia@curesma.org

Western New York
PO Box 444
North Tonawanda, NY 14120
716.884.4233
Mary Boguhn, Family Support Chair
wny@curesma.org

Wisconsin
PO Box 320516
Franklin, WI 53132
414.324.0499
Kate Vogedes, President
wisconsin@curesma.org

Cure SMA National Office
925 Busse Road, Elk Grove Village, IL 60007, 1.800.886.1762
CHAPTER UPDATES

Arizona Chapter

10th Annual Arizona Walk-n-Roll
The 10th Annual Arizona Walk-n-Roll was held on November 15, 2015 at the Steele Indian School Park in Phoenix, 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 $17,000! 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, the Occupational Therapy students from A.T. Still University and nursing students from Grand Canyon University. It was a great day despite the rain for families to connect and allow the children/young adults to hang out, play games and enjoy each others company. Thank you to everyone for coming out and supporting this event.

A special thank you to Angel Wolff and her committee for another wonderful walk for Arizona!

2nd Annual Cure SMA Byrd’s FORE a Cure
The 2nd annual Byrd’s FORE a Cure took place on April 25, 2015 in Dewey, AZ, at the Prescott Golf and Country Club for a successful day of increasing awareness of SMA, fundraising and great golf! Prizes were awarded to the first and second place teams. We are grateful for our wonderful sponsors and the local restaurants, golf courses, retail stores, businesses and the Diamondbacks for their incredible support of our Arizona Chapter of Cure SMA. With their help, and the generosity of all our golfers and dinner attendees, Byrd’s FORE a Cure raised over $4,500 to Cure SMA! See you next May!

A Night at the VIG
In September, 2015, the Vig in Phoenix, AZ, hosted a Cure SMA Awareness evening for Cure SMA. The fundraiser allowed you to bring in the flyer to the Vig and they donated a portion of the proceeds from the evening to Cure SMA. In total, over $500 was raised for SMA research. Thank you to Cassandra and Stacie for organizing this event.

SMA Dinner and Candle Lighting
The SMA Dinner and Candle Lighting ceremony was held in August at Fuddruckers 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!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
The 2015 Cure SMA Casino Night with Gray’s Gang was a blast and raised $26,543! 130 High Schoolers attended and tried their hand at blackjack, Texas Hold’em, craps and roulette on April 25, 2015. They enjoyed music by the Myers Park High School band Shaggy Rogers and the Scooby Snacks. The kids munched on lots of yummy snacks and Chick-fil-A nuggets donated by Chick-fil-A and had their fill of great soft drinks donated by Snapple and Dr. Pepper. The goal was to get as many raffle tickets as possible to try to win some great prizes including a yoga package, golf package, bowling/gaming package, restaurants package, gas cards, and more! There was also a photo booth to capture the memories of the night. The fundraiser was a huge success with the high schoolers and everyone is already anticipating next year’s Casino Night!

DECA Club

On October 20, 2015, the DECA Club of Myers Park High School raised over $4,500 for Cure SMA in honor of Gray Dougherty! The students planned the entire event, held in Charlotte, NC, and was a huge success! Thank you to the DECA Club and the Dougherty Family for their generous support.

Island Independence 5K - Charley Walker Team

Thank you to all of the runners that participated in the 2015 Island Independence 5K in Topsail, NC on July 4, 2015 who fundraised for Cure SMA! Thanks to the dedication of this group of runners, over $4,100 was raised in honor of Charley Swing and Walker Herring Taylor. Special thank you to Elizabeth Rodgers for all her hard work!

Cure SMA Walk-n-Roll in Honor of Loving Logan

The 2015 Cure SMA Walk-n-Roll in Honor of Loving Logan was held on March 21, 2015 in Smithfield, NC. The event is hosted each year by Dana Grimstead and Layne Moore in memory of Logan Moore, SMA type I. Thank you to Dana and Layne for hosting another successful fundraiser and raising over $3,300 for Cure SMA!

Pure Give: Cure SMA

Pure Barre Greenville NC, a barre workout studio in Eastern North Carolina, held a fundraiser on May 2nd, 2015 for Cure SMA. Our first ‘pure give’ class was done in honor of one of our clients, Jody Swing, who has a three-year old daughter, Charley, with SMA type I. PureGive is a nationwide fundraising initiative that Pure Barre studios across the country hold to give back to the community.

On May 2, 2015 women from Eastern NC joined us for an hour long class in which all profit was collected as a donation for cure SMA. This all day event included a raffle, dance party in our studio for local kids, and even an appearance by the Kona ice truck. Kona Ice donated 25% of their profits from the day to us as well. Our staff was overwhelmed and humbled by

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
the amount of people who showed up to support the Swing family. Charley herself even came to join in on the fun. As a community, we raised a total of $2,457 in one day. All of this profit was donated directly to cure SMA in Charley’s name. This event was so much fun to plan and be a part of, and we hope that our donation helps to get researchers a few steps closer to finding a cure for SMA.

2015 Cure SMA Golf in Honor of Alex Blair
Thank you to Kathie Clark and her team at The Chef’s Palette in Cary, NC for hosting the 2015 Cure SMA Golf in Honor of Alex Blair! The event held at Devil’s Ridge Country Club in Holly Springs, NC on October 19 raised $2,261 for Cure SMA.

2015 Cure SMA Hope Campaign
Thank you to the Chan Family of Raleigh, NC for hosting a virtual Hope Campaign Fundraiser during August 2015 for SMA awareness month! Special thanks to Mimi Chan for spreading SMA awareness, and inspiring friends and family to donate by sharing her son Brady’s journey with SMA. Over $2,000 was raised to support Cure SMA!

Stroller Warriors T-Shirt Fundraiser
Thank you to Stephanie Geraghty for hosting The Stroller Warriors ® Virtual 5K for Cure SMA in August 2015. The 5K and t-shirt sale is held each year in honor of Cole Geraghty of Jacksonville, NC. This year the event raised almost $2,000.

SOUTH CAROLINA
2015 Cure SMA Go Miller Go 5K
The 2015 Cure SMA Go Miller Go 5K held on September 12, 2015 in Charleston, SC, was a tremendous success! Despite the rain nearly 450 participants and 21 teams came out to James Island County Park to raise funds and spread SMA awareness.

In its second year, the Go Miller Go 5K raised nearly $50,000! Thank you to Lindsay and Eason David for all of their hard work on this event in honor of their son, Miller.

Go Leo Go 5K
Congratulations to the Bugenske Family and all of Leo’s Pride on the success of the 2nd Annual Go Leo Go 5K! Over 550 participants gathered in South Carolina on October 31, 2015. Because of the success of this event, held in honor of Leo Bugenske, over $82,700 was donated to Cure SMA to support research programs and the equipment pool.

This year’s race marked an incredible milestone for Leo – his second birthday! Special thanks to Adam, Meredith, and Leo Bugenske for their generous support of Cure SMA.

The Medical University of South Carolina’s Occupational Therapy Class of 2015
The Medical University of South Carolina’s Occupational Therapy Class of 2015 a donation of $381.49 to Cure SMA. Throughout the 2.5 years as Occupational students, the Class of 2015 worked diligently to fundraise for causes that were close to our hearts. This donation is in honor of two special SMA warriors, Leo Bugenske of Chaplin, SC, and Miller David of Charleston, SC, among with all the other families, friends, and fighters that are affected by SMA.

Patee Tamric, OTS
Charleston, SC

3rd Annual Rex’s Ride Motorcycle Run
Thank you to John Clark for hosting the 3rd Annual Rex’s Ride Motorcycle Run on June 6, 2015 in Clover, SC. Over $1,500 was raised in memory of Rex Clark!
Chesapeake Chapter

MARYLAND
22nd Annual Crab Feast & Silent Auction

The Chesapeake Chapter held its 22nd Annual Crab Feast & Silent Auction on August 2, 2015. Over 400 people filled the Towson American Legion in Towson, MD, and enjoyed plentiful crabs and games of chance. The event raised over $59,000 for Cure SMA, with the auction itself raising over $8,400! To date the Crab Feast alone has raised over $600,000 for Cure SMA.

Beverly Venedam, Vice President
Chesapeake Chapter

2015 Chesapeake Chapter Walk-n-Roll

The 2015 Chesapeake Chapter Walk-n-Roll was held on Saturday, April 25, 2015 at the Old National Pike Park in Mount Airy, MD. Participants enjoyed a one mile walk throughout the beautiful park followed by an afternoon with lunch, face painting, balloon art and games. Through the generous support of 20 walk-n-roll teams and our event sponsors: Our Little Jewels, Dectis Painting, Stacey Markel Photography and the Chesapeake Chapter Community, the event exceeded its fundraising goal by raising over $56,000! A special thank you to Kim Heinrich and Amber Hartung for sharing their personal stories at the event. We also would like to extend a huge thank you to Tina and Derek Lewis, Beverly Venedam, Kim Heinrich and Melissa Wayland for organizing another successful event. We are looking forward to 2016!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Our Little Jewels

The 8th Annual Our Little Jewels Golf Outing was held on Friday, September 25, 2015. There were 124 golfers who played at The Links at Challedon in Mount Airy, MD. Many thanks to the 15 volunteers who helped make the outing a success. The luncheon after golf was catered by Scittino’s Italian Market. Awards were given to the foursomes that finished in 1st, 2nd, 8th, 16th and 24th place. The event raised over $35,000 with a net profit of $21,000. The money raised was used to support Cure SMA, the Baltimore Muscular Dystrophy Association, and the Bennett Blazers Sports Program for Physically Challenged Children, the MD Council for Special Equestrians, the Kids Korral Therapeutic Horseback Riding Center and the Mary Mac Foundation.

Jim Lewis
Ellicott, MD

De Matha Catholic High School

On Friday October 2, 2015 DeMatha Catholic High School raised over $8,000 for Cure SMA. The high school, known for its powerhouse athletic and music programs, should also be recognized for its commitment to serving others. DeMatha is an all-boy, Catholic high school located in Hyattsville, MD. The school has a walk-a-thon every October to help raise money for charities and non-profit organizations. This year, DeMatha decided to raise money for Cure SMA in an effort to support faculty member Matthew Bickel’s son, Kyle, and others who suffer from SMA. Kyle is in the sixth grade and has SMA type III. Kyle is part of the swim and dive teams at his local summer pool and also enjoys playing basketball. Mr. Bickel and his family were overwhelmed by DeMatha’s tremendous financial and emotional support.

DeMatha Catholic High School
Hyattsville, MD

Primo Pasta Kitchen 2015, Family Festival

On September 10, Primo Pasta Kitchen in Pasadena, MD hosted its inaugural Family Festival to benefit Cure SMA, and raised over $3,000! The event included activities for people of all ages and abilities, a silent auction and a chance to raise awareness in a big way as guests smashed color-filled water balloons in an aerial-filmed #SMAshSMA video. Inspired by the story of Micah Biello, SMA type I, Primo plans to continue the new tradition next fall.

Bryna Colley
Baltimore, MD

Annapolis Mothers of Multiples

Annapolis Mothers of Multiples selected the Cure SMA Chesapeake Chapter as the recipient of their Charity Fundraising effort during their annual Fall Consignment Sale. This very popular event attracts families and expectant mothers from the Anne Arundel County area. A Cure SMA awareness and information table was available for this two-day event. Special thanks to Cadet Girl Scouts, Lily McCullough and Joelle Garcia from Troop 1448, for raising...
additional funds at the Bake Sale table. The total donations raised were $1,117. The opportunity to spread awareness to local families, priceless.

Kimberly Martin-Heinrich  
Chesapeake Chapter

**John Hopkins Home Care Group**

John Hopkins Home Care Group has chosen Cure SMA as one of four charities employees will have the option of supporting in 2016. With an early fundraiser in the fall of 2015, John Hopkins Home Care Group has already raised over $1,000 for Cure SMA! A special thank you to Kim Heinrich for advocating for Cure SMA to be one of these four charities.

**WEST VIRGINIA**

**Candle Lighting**

An SMA Candle Lighting ceremony was held on Saturday, August 8, 2015 in West Virginia. Thank you to Steph Leasure and her family for organizing this ceremony and raising awareness for SMA.

---

**Connecticut Chapter**

**Cubby’s Run**

Cubby’s Run 2015 was another huge success. Overall we raised another $27,000—putting us at a grand total of just about $100,000 for the four years we have been holding this event. Cubby SMA type I, is still doing great and we are looking forward to another successful year. This year will be held at the same location, the Ridgefield Rec. Center on May 7th. If you'd like to join us or donate, feel free to contact ethanhynes@me.com.

Ethan Hynes  
Ridgefield, CT
Connecticut Chapter cont.

Shine's Annual Cure SMA Cut-a-Thon
Shine’s Annual Cure SMA Cut-a-Thon was held on Sunday, August 23, 2015. It was a beautiful day filled with fantastic families and children of all ages having their haircut to support a great cause. Erin Simmons, Shine’s Owner, has held this Cut-a-Thon in honor of Cubby Wax, 16, since he was in elementary school. The back to school cuts’ proceeds are given to Cure SMA to help find a cure!

As always, it was a lively event filled with laughter and everyone left with a great style. All the stylists wore purple beads and gave out purple Cure SMA bracelets, which happens to be Cubby’s favorite color! Shine would like to thank Cure SMA for all their help in planning a great event and to all of Ridgefield, who always gives generously to support Cubby, Shine, and Cure SMA.

Georgia Chapter

Cure SMA Tennis Tournament in Honor of Peyton Zimmermann
Mike and I decided we wanted to do a fundraiser in memory of our daughter Peyton not long after she passed away. Like any parent, we want our child to be remembered, to have left behind a legacy. Usually our children have a lifetime to do that, but Peyton had less than 13 months. Despite that, she touched more lives than we could have dreamed in such a short amount of time, and now we feel it’s our responsibility to continue her legacy.

There are so many families that have SMA angels and SMA warriors. We’ve lost so many kids this year, it is just heartbreaking. The more people that know about SMA, the sooner we will find a cure. We plan on continuing the tennis tournament as long as we are in Georgia and hope that we reach more and more people each year. This year we had about 65 participants and raised over $14,000 that was donated to Cure SMA. This was because we had several generous business sponsors and many personal donations. From the bottom of our hearts, thank you to our sponsors and our participants for supporting us in our journey to support SMA families and find a cure.

Stacey Zimmermann
Grovetown, GA

Morning Minyan
Morning Minyan raised $282 during the August’s Cure SMA Awareness Month. These funds were donated in the name of the Morning Minyan Congregation of Shalom in Orange, CT. Congregant’s grandchild is affected by SMA. Morning Minyan is happy to support Cure SMA’s efforts and programs.

Rock ‘n’ Roll Savannah Marathon & ½ Marathon
For the first time ever, Team Cure SMA runners participated in the Rock ‘n’ Roll Savannah Marathon & Half Marathon in Savannah, GA on November 7, 2015. Thank you to our dedicated group of runners: Heather Little, Jeffrey Little, Skyler Russell, and Stacey Wells. Over $3,000 was raised for Cure SMA!

Cure SMA Day with the Atlanta Braves
The Georgia Chapter hosted the Cure SMA Day with the Atlanta Braves on July 18, 2015. Thank you to Anami and Tim Lehmann for their hard work planning this day and selling tickets. More than 30 people attended and $1,100 was raised for Cure SMA!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
**CHAPTER UPDATE**

Greetings from the Greater Florida Chapter!

Stretching from Sarasota to Pensacola, the Greater Florida Chapter covers a large and diverse geographic area, spanning hundreds of miles and serving many SMA families. In 2015, we worked together to achieve our chapter’s biggest fundraising year yet, and we are looking forward to what we will accomplish in 2016!

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!

---

**2015 Greater Florida Walk-n-Roll**

Since our inaugural event in 2009, the Cure SMA Greater Florida Chapter has hosted a sponsored walk each November to help raise funds for SMA research and family support programs. The 2015 Greater Florida Walk-n-Roll was held on November 14, 2015 at Lake Parker Park in Lakeland, FL, raising just over $33,000 for Cure SMA! It was our largest Walk-n-Roll to date, with 20 fundraising teams and more than 250 people in attendance. Our highest fundraising team, Mama Needs A Miracle, contributed a record-breaking $8,200 to the event, and our biggest team, Team 2 Angels, had 33 members!

This year’s theme was “Lights, Camera, Cure SMA,” and we tried to bring the movies to life at the event. Our friends from the MUCH Foundation, the 501st Legion, the Suncoast Ghostbusters, and A Dream Come True Entertainment made sure that our favorite movie characters from Darth Vader to Indiana Jones and everyone in between was there. Outback Steakhouse and Bay Street Bistro treated us to a red carpet worthy lunch. It really was a magical day – right out of the movies! Thank you to all of our sponsors, donors, volunteers, and participants for making the 2015 Greater Florida Walk-n-Roll such a great success!

---

**23rd Annual Maluko Golf Tournament**

The 23rd Annual Maluko Golf Tournament took place on October 26, 2015 at Carrollwood Golf & Country Club in Tampa, FL. With more than 100 golfers hitting the links, the tournament raised $25,000 to help fund SMA research and family support programs! For the past 17 years, event organizers 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. This golf tournament is one of our chapter’s largest fundraisers every year, and we are so thankful for the efforts of the Maluko’s, who are an amazing group of friends!
The Village Early Learning Center Costume Crusade

The Village Early Learning Center, owned by Joe, SMA type III, and Susan Miller, and The Village Family Foundation host incredible community events at the pre-school, with the proceeds donated to charity. The Costume Crusade, held on October 30, 2015 was the place for kids of all ages to show off their favorite Halloween costumes. It’s also known for the annual wheelchair race against Mr. Joe, with Asher Camp dressed as the Wizard of Oz, winning the bragging rights for 2015! The Millers generously donated $2,000 from the Costume Crusade to Cure SMA – we can’t thank them enough for their community spirit and their ongoing support of our organization!

SMAsh SMA at the Oakstead Fall Festival

The National Elementary Honor Society at Oakstead Elementary School in Land O’ Lakes, FL, hosted a SMAsh SMA Booth at their Fall Festival on October 23, 2015. For a small donation, students got a chance to smash eggs to smithereens, just like we hope to do to SMA! The booth was organized by Oakstead parent Sally Miller, in honor of Brogan Post, SMA type I, and raised $400 for Cure SMA. Thank you so much to Sally and all of the students who made the booth a success!

LEGOLAND Florida Ticket Offer

For the 4th consecutive year, LEGOLAND Florida offered reduced price general admission tickets to chapter members in August and September, generously donating half of the sales proceeds back to the chapter! Thank you to LEGOLAND Florida for your $600 donation to our cause and for your continuous support!

Painting for Andy

Andy Butler should have celebrated his 6th birthday in January 2015. On Sunday, January 25, his mom, Audra Butler, organized a “Painting with a Purpose” fundraiser to celebrate his memory instead. Ten friends and family members picked up their brushes to paint, “Colorful Firefly Jar,” a picture that captures the hope, light, and joy that Andy brought to this world. Painting with a Twist in Trinity, FL hosted the event and donated a portion of their proceeds, totaling $225, to the chapter.

SMA Awareness at Keiser University

Thank you to Robyn Silva, SMA type II, for raising SMA awareness at Keiser University in Lakeland, FL. Each month, the university chooses a different organization or cause to highlight, and in July 2015 they chose Cure SMA, donating $88 to our cause!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Clearwater Marine Aquarium Outing
On May 3, 2015, 13 families in our chapter came together to visit the Clearwater Marine Aquarium and to meet its most famous inhabitant, Winter the Dolphin. The aquarium staff provided our group of 55 people with complimentary General Admission tickets, which allowed entry to both the aquarium facility and Winter’s Dolphin Tale Adventure in downtown Clearwater. A big thank you to the amazing staff at the Clearwater Marine Aquarium and Laurie Sore, our chapter’s Vice President, for making this amazing event happen!

End of Summer Family Outing
Three of our chapter’s families met for some fun in the sun at Tampa Waterworks Park on September 20, 2015. The kids and adults alike had a blast at this accessible downtown park with a splash pad!

SMA Awareness Month
Thank you to the following cities and counties across the state for formally and publicly proclaiming August 2015 as Spinal Muscular Atrophy Awareness Month: City of Lakeland, City of Pensacola, City of Tampa, Manatee County, Orange County, Pinellas County, Polk County, and Sarasota County. We appreciate the support of our local communities!

Spring Family Picnic
Ten families made of more than 50 people came together on March 14, 2015 at Wall Springs Park for a day of fun in the sun! Families drove from as far as Sarasota, Lakeland, and Orlando to attend the picnic. All of the kids enjoyed playing at the accessible playground, and the parents enjoyed getting to know each other. We also were thrilled to celebrate Asher Camp’s second birthday, which was the same day as the picnic, with a special Spiderman cake!

Greater New York Chapter

2015 TCS New York City Marathon
Congratulations and huge thanks to all of our amazing runners who participated in the 2015 TCS New York City Marathon on November 1, 2015 and helped raise over $37,000! This race was held on a beautiful day in New York and took runners through all five boroughs of the city.

Thank you to the following Team Cure SMA runners for all of their hard work, both training and fundraising: Chuck Breuker, Tammy Breuker, Jordan Faith, Danielle Ford, Tiffany French, Andrew Mayer, Joseph Mayer and Meghan Shevlin.

11th Annual Greater New York Chapter Walk-n-Roll
This year marked the 11th Annual Greater New York Chapter Walk-n-Roll which was held on Sunday, October 4, 2015 at the Long Beach Boardwalk in Long Beach, NY. The day was filled with great activities for participants of all ages including a 50/50 raffle, face painting and the chance to catch up with SMA families from all over the area. This year’s walk was held in memory of Pranav Mahadevan, SMA type I.

We would like to give special thanks to all of our amazing teams, participants, and donors for making our 11th Annual Walk-n-Roll a HUGE success. Due to everyone’s efforts we were able to raise over $23,000!

This year’s teams included:
Accardi Boys, CDM, Fight for Owen, Friends of Julie, Love for Max, Sweet Baby Jack, Team Dylan, Team Farmingdale, Team Juliana, Team Pranav 2015, Team Ross, Team Tansey and The Florida Skylanders!

Special thanks to Debbie Cuevas and all of the wonderful volunteers for organizing this amazing event each year!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
2015 United Airlines New York City Half Marathon
The United Airlines New York City Half Marathon took place on March 15, 2015 in New York, NY. The day consisted of a great race taking runners through famous landmarks such as Central Park and Times Square. This race attracts more than 10,000 runners annually, and this year Team Cure SMA had five runners participate. Together, they raised over $7,500 for Cure SMA! Special thanks to the Lozina family for helping organize the event.

A special thanks to our runners:
Neda Azadivatan-Le, Ellie Burton, Natalie Ginor, Kara Hartnett and Joseph Kolinsky.

SMA Day with the NY Mets
On August 2, 2015, several families and friends came out to Citi Field in New York, NY in memory of David Cunniff, to cheer on the NY Mets and raise awareness for SMA. Everyone had a great day at the Modell’s Clubhouse and ran the bases on the field after the game. The event raised $6,040 this year for Cure SMA!

Special thanks to the Cunniff family and Debbie Cuevas for all of their hard work to organize this event!

Capital Region
2015 Capital Region 5K & Walk-n-Roll in Memory of Trooper Cunniff
The Greater NY Capital Region Chapter held their fourth annual 5K & Walk-n-Roll in memory of Trooper David Cunniff. We ran, walked, and rolled at the Colonie Mohawk River Park in Cohoes, NY, on Saturday, September 26, 2015. It was a beautiful day for the event and we had a great turn out helping us raise over $21,900. Our faithful attendees were there in addition to many new faces and fundraising teams. We had a great time while remembering our SMA Angels and celebrating our Warriors. Thank you to Whalen Chevrolet and Hannah Balta of HB Photography for your continued support of this event, and a BIG thank you to all our volunteers! This event would not be possible without your dedication and support.

Special thanks to Amy Cunniff and volunteers for making this event a huge success.

The Ice Fishing Tournament
The Ice Fishing Tournament was a huge success with over 120 entrants. All proceeds were in memory of Trooper Cunniff. Many members of the Cunniff family attended along with friends and even state troopers. A total amount of $3,050 was raised. Thank you to Christine from Docksider who kindly offered her restaurant to host this event.

Scrap-a-thon Raises $1,000
A group of dedicated scrapbookers got together for their annual fall retreat November 13-15, 2015 and raised $1,000. Women participated by getting pledges from family and friends for every scrapbook page completed. There was also a hilarious game of Coco Loco where teams paid $20 to participate with the entry fee going completely to Cure SMA. A big thank you to Jessica Madden of JReaM Retreats for coordinating this fundraising effort! We’ve never had so much fun raising money!
**Illinois Chapter**

**9th Annual Illinois Chapter Walk-n-Roll**

This year marked the 9th Annual Illinois Chapter Walk-n-Roll, and it was a record breaking year in both attendance and fundraising! The walk was held May 31, 2015 at Independence Grove in Libertyville, IL. Although the morning started out chilly when the walk kicked off, we were very fortunate to enjoy a beautiful day for this fun-filled event to raise awareness and funds for SMA!

Almost 700 people gathered at the event for a day of fun! Everyone enjoyed the walk, DJ, face painter, balloons, and even some karaoke.

We would like to give a special thanks to all of our amazing teams, participants, sponsors and donors for making our 9th Annual Walk a HUGE success. Due to everyone's efforts we were able to raise over **$58,000** and had a total of 30 teams participate!

Cure SMA would like to give a BIG thank you to Janet Schoenborn, the walk's event organizer and Illinois Chapter President for everything she did to help make this such a great day and opportunity for all of the Illinois family and friends to come together.

A special thank you to Danielle Plotke, Cynthia Annel, Liz Macellaro, Jake Reilly, Matt Reilly, Tina Krajewski, Amanda Colbert and everyone else who helped plan this year’s event.

We hope to see everyone at the 10th Annual Illinois Chapter Walk-n-Roll this coming May or June of 2016!

**2015 Big Ball Soccer Tournament**

Metea Valley High School’s teachers technically should have been trampled in a soccer game against their varsity soccer students; after all, the kids are younger, stronger, and faster. But at the Big Ball Soccer Fundraiser for Spinal Muscular Atrophy on September 26, 2015 the much anticipated varsity-faculty match-up ended in a draw, with both teams battling hard to keep each other at bay.

In the end, the biggest winner was the Cure SMA organization with this year’s Big Ball Soccer Tournament raising more than **$39,000** to find a cure for the disease which afflicts Metea Valley student, Angie Lee. More than 400 people turned out for the event held at Players Indoor Sports in Naperville, IL.

“The fundraiser was a lot of fun and we were so glad to be a part of it,” said faculty team captain and varsity soccer coach, Joshua Robinson. “Our soccer players are competitive kids; they played hard to try and beat us, but more importantly, to help beat SMA,” he continued. Angie Lee, Kyra Scadden, and their friends have been organizing the annual fundraiser since 2007. They have raised more than **$190,000** for SMA!

Cure SMA would like to give a special thanks to Kim Lee and her entire event committee for planning such an incredible event each year!

**19th Annual Chicago Half Marathon & 5K**

Members of Team Cure SMA ran the Chicago Half Marathon & 5K on September 27, 2015 in Chicago, IL. This is the fifth year that Cure SMA participated in the run as an official charity partner. Together, the runners raised over **$19,000** to help cure SMA! Cure SMA would like to thank all of our runners; Noel Guest, William McAllister, Jennifer Malfas, Katarzyna Kos, Melonie Wolf, Michelle Yohler, Matt Schuller, Drucilla Dence, Katlyn O’Brien, Kim Courdt and Mike Nakanant for their hard work in training to run this race as well as all their fundraising efforts!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Illinois Chapter cont.

Rebeckah Fundraiser
Our granddaughter, Rebeckah, passed away at 3 ½ years old from SMA. Every year for her birthday, family and friends honor her by making donations to various organizations. This year, her mom, Kim, ran from their home to the cemetery, over 20 miles and raised $620! Thank you for all you do for SMA families.

Julie Courdt
Peoria, IL

Fundraiser in Honor of Ryan Manfre
In April, Daniel the Prophet School in Chicago sponsored an orange and purple dress down day to raise awareness for SMA and to fundraise for Cure SMA. The fundraiser was held in honor of Ryan Manfre, SMA type III, 2nd grader at St. Daniel.

Iowa Chapter

19th Annual Beaverdale Beaverdash
The 19th Annual Beaverdale Beaverdash took place on September 19, 2015 in Des Moines, IA. It was a beautiful day and the event was once again a success! Over 300 people gathered to run, walk or roll. This year’s Beaverdash raised over $24,000 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!

Small Heroes, Might Cause Gift Registry
In 2015, Colin Schlemme, SMA type II needed to have a major surgery to correct his scoliosis, which is a secondary complication of his SMA. Amanda Schlemme created a gift registry page in honor of Small Heroes this year which raised $1,700 for Cure SMA. We look forward to this event returning in 2016!

Benefit in Honor of Cael
$1,000 was raised from a fundraiser in Rhodes, IA in honor of Cael Rudkin, SMA type II, for Cure SMA. Thank you, Chris and Joy Rudkin, for your hard work and fundraising efforts!

St. Ambrose University
St. Ambrose University, alumni and SMA parents, Megan Ramirez, Brian and Becky Manfre, teamed up with Cure SMA to “Sting Away SMA.” All athletes on campus were encouraged to attend a comedy night and to make a donation as their entrance fee. The following weekend, The St. Ambrose Bees had a home football game where they raised money by selling t-shirts, having a bake sale, and doing a 50/50 raffle. Together the event raised over $800!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.

MISSOURI

13th Annual Bommarito Z Club Car Show
The 13th Annual Bommarito Z Club Car Show took place on September 6, 2015. The event had great attendance and 55 cars. Honoring Brittany Carpenter, SMA type II, and in memory of Madeline Schmidt, SMA type I, Michael, SMA type I, and Brian Goodyear, SMA type I. Thanks to Steve and Chris Colesworthy, Jeff Polsgrove and all members of the Z Club. A total of $17,279 was raised to benefit Cure SMA.

Rock ‘n’ Roll St. Louis Half Marathon
18 runners gathered for our first year running for Cure SMA in the 2015 Rock ‘n’ Roll St. Louis Half Marathon! The runners held fundraisers and t-shirt sales to raise money to cure spinal muscular atrophy. In total, our runners raised over $9,400 for Cure SMA! Congratulations to all of our runners! Keep up the great work!

Gracie’s 4th Annual Steak Dinner for SMA
Gracie’s 4th Annual Steak Dinner took place on April 5, 2015 in memory of Gracie DeGraffenreid, SMA type I. Friends and families from Eugene, MO, gathered to raise SMA awareness and funds for Cure SMA in Gracie’s memory. Thanks to the generosity of everyone who attended, more than $7,000 was raised for Cure SMA! Thank you to Sherry DeGraffenreid for planning this event year after year in memory of Gracie.

Scare Away SMA
Over $600 was raised in honor of Matilda (Tilly) McRoberts, SMA type II, and the Derrington Family through the Wesbury Manor Hunted Forest in Missouri for the “Scare Away SMA” fundraising efforts. Thank you, Dana Derrington, for organizing such a great event! Keep up the great work!

Phi Tau Omega Sorority Trivia Night
The ladies of Delta Phi Chapter of the Phi Tau Omega Sorority in Saint Louis, MO raised $2,900 from their annual trivia night! This event is held every year in honor of Brittany Carpenter, SMA type II, who is a sophomore in high school. They have been in contact with her and her family since she was a little girl. Thank you, Kathy Goodyear, of the Delta Phi Chapter for organizing this event every year!

KANSAS

Kansas City Chapter Walk-n-Roll
Bucking tradition, we held the 15th Annual Kansas City Chapter Walk-n-Roll on September 19th, 2015 in Roeland Park, KS. The weather was perfect, so the change was worth it. We had more than 360 people sign up to support our families and raised over $46,000. It was another fun, successful event for the Kansas City Chapter. More than 19 SMA families were represented by teams of family and friends. We are looking forward to more fun events in the future!

This summer, several SMA families got together to enjoy a little pool time. It was even the first time in a pool for several of our kids.

Kristal Wilson, Raytown, MO

South Gray High School
On February 3 and 15, 2015 South Gray High School in Montezuma, Kansas raised $279 from a shooting contest fundraiser in honor of Chance Wiebe, SMA type I.
2015 Cure SMA Writer’s Night
On October 22, 2015 the Louisiana Chapter hosted the Inaugural Cure SMA Writer’s Night at Boudreaux’s in Baton Rouge, LA. The evening featured cocktails, hors d’oeuvres, dinner, a silent auction, live auction, raffle items, and a live performance by professional songwriter and traveling troubadour, Verlon Thompson. Over 110 guests attended the event which raised more than $25,000 for SMA research and family support.

A special thank you to Krista Scurria for chairing the event, as well as the dedicated Writer’s Night committee members, amazing volunteers, and generous Presenting Sponsor Baton Rouge Cardiology Center who made this night possible.

Jane Gives Back Trunk Show
Thank you to Jennifer Ecker and Matilda Jane Clothing for hosting a Jane Gives Back Trunk Show this fall to raise funds and awareness for SMA. The event, held in honor of Ella Jayne, SMA type III, raised over $400 for Cure SMA.

Kick 4 Clayton Kickball Tournament
On June 27, 2015 the Kick 4 Clayton Kickball Tournament took place at Keithville Community Park in Keithville, LA. Event participants, families, and friends enjoyed a fun afternoon of kickball followed by a picnic lunch. Thank you to Alexis Bryant for organizing the tournament, which raised over $400 in memory of her son Clayton Bryant, SMA type I.

Turkey Trot Festival Fundraiser
Every year here in Yellville, AR, we have a festival/homecoming weekend in October. For the third year, we have set up a booth to collect donations for Cure SMA. We do this in honor of our grandson, Cason Bauss, SMA type I, and our friends’ daughter, Ember Hinson, SMA type I, both of whom are SMA angels since 2013. We have our booth at Turkey Trot every year and this year we were blessed with great weather and people were generous. We gave away great prizes of wonderful gift baskets and two televisions on October 9 and 10, 2015. The donations from the fundraiser totaled over $2,100 to help all affected babies, children and adults to receive the treatments now in development, and to offer hope for parents who are carriers.

Thank you for your tireless dedication to support research as well as families. We are here in the trenches helping as much as we can!

Mary Bauss, Lisa May, Gina Brewer
Yellville, AR
Michigan Chapter

11th Annual Michigan Chapter Walk-n-Roll 2015
Thank you to all who participated in the 11th Annual Michigan Chapter Run, Walk-n-Roll that was held May 2, 2015 at Hawk Island Park in Lansing, MI. We were very fortunate to enjoy an absolutely beautiful May day for Michigan and for this fun-filled event to raise awareness and funds for SMA!

A special thank you to Linh Huynh, the walk’s event organizer, for everything she did to help make this such a great day and opportunity for all of the MI family and friends to come together. This was Linh’s first year organizing the walk and her input and help was so appreciated by everyone.

We would also like to thank Holly Schafer, who organized the walk for the previous 10 years and was still so incredibly helpful in all of the planning and day of event needs. Thank you to Ken and Cindy Armbrustmacher and all of their volunteers who helped make the day of events run so smoothly. A special thank you to Melissa House and Team Abbey for the amazing job they do fundraising each year! To the Wolfe Company and Linda and Harvey Wolfe for being such generous sponsors over the years. Also, thank you to Deb Postma for all of her help and support and to everyone who brought in all of the wonderful items for the raffle!

We cannot thank everyone, who participated, donated and helped out at the event, enough! The 5k, walk, kids fun run, face painting, and Jammin DJ’s were all such important aspects to making the walk such a great event for everyone! The 11th Annual Michigan Chapter Run, Walk-n-Roll raised over $45,208 for Cure SMA!

We hope to see you at the 12th Annual Michigan Chapter Walk-n-Roll this coming May of 2016!

Byron Center Christian Schools
Byron Center Christian Schools donated $500 in honor of Isaac Postma, SMA type I. Thank you so much for your incredible donation!

SMA Art Gala
A fun new event came to Cure SMA through Valerie Ott and Valerie Ott Photography called the SMA Art Gala. This event was created to honor Brielle and Brooke Kennedy, who both have SMA type II. For senior photos, Valerie joined forces with a few different senior girls to take their photos and set them up through this gallery and an evening out. One of the seniors babysits the Kennedy girls and suggested Cure SMA as the recipient to receive any donations from the money raised at the SMA Art Gala. Thank you to the senior girls, the Kennedy family, Valerie and everyone who attended the event, which raised $354!

Friday Fundraiser
Walk the Line to Sci-Recovery of Southfield, MI, held a “Friday Fundraiser” to help raise $200 for Cure SMA, in honor of Jace Dorer, SMA type II.

Our staff participates in a weekly “Friday Fundraiser,” and for $2 they can come to work on Fridays wearing school spirit, professional sports or any charity shirts or sweaters. This past summer after we had the pleasure of meeting Jace and his loving and supportive family, we knew that Cure SMA should be our chosen charity for the months of June through September.

We are a family owned physical therapy clinic that specializes in recovery therapy for spinal cord and brain injury and all of us at Walk the Line have been honored to show our spirit in support of a cure for SMA this past summer!

Erica Coulston, President of Walk the Line to Sci-Recovery Southfield, MI

MLB Run Challenge
This was from a pledge I made for every run scored in major league baseball on Labor Day. 147 runs x .50 = $73.50. In September, I did a video and challenged my friends. I’m going to do a NFL point challenge. I will do another video for the football challenge. Find a cure!

All the best,
John Eaton
Ann Arbor, MI

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
CHAPTER UPDATES

Michigan Chapter cont.

SMA Awareness Night at the Tiger’s Game
Thank you to everyone who attended the Tigers Game on August 23, 2015 and helped raise awareness for SMA to the Michigan Community and Tigers fans! It was a great way to spread awareness about SMA during August SMA Awareness Month!

Pontiac Summer Roundup
The Motor City POCI hosted its 2nd Annual Pontiac Summer Roundup at Bakers of Milford, MI and all proceeds from the show came to Cure SMA. “It was our pleasure to support such a worthy cause! We hope that our donation will help support the great work that your organization does. We are happy to report that we will be sending a check for $1,000 to support Cure SMA. Thank you again for your time and participation at this year’s event. I hope that our donation will help in some small way for your very important cause. Our club likes to choose a worthy cause each show to contribute to and Cure SMA was our choice this year. Understanding the issue is one thing but to help some one with the disease is quite an honor.”

Lori Woods and John Berzseny of the Motor City POCI

Thank you to Lori, John and everyone at the Motor City POCI for your support! It was a great day checking out all of the fun cars and also bringing attention to SMA! Also, thank you the Grindle Family, TJ, Jason, Ben and Lily, for helping Cure SMA become involved in this event!

Minnesota Chapter

10th Annual Ride Away SMA
Ride Away SMA is held in memory of Lindsey Anne Ronningen, SMA type I, who passed away in 2004. Each year, hundreds of people gather to raise money for Cure SMA and participate in a motorcycle ride to honor Lindsey.

Ride Away SMA began on June 27, 2015 at Peace Church in Rochester, MN, and included a pancake breakfast and silent auction. All of the participants gathered before the ride and were able to purchase merchandise and get together to honor what would have been Lindsey’s 12th birthday. As their event continues to grow, Amy, Eric, and Colleen are surrounded by so many people that continue to love and support this event.

This year, Ride Away SMA had about 235 riders that came together to support Cure SMA and the Ronningen family. The event ended at Eagles Club in Rochester, MN for another silent auction and a group dinner. Here, they announced the raffle ticket winners, silent auction winners, presented speeches thanking everyone for supporting the event and ended the evening with live music.

Overall, the 10th Annual Ride Away SMA event raised over $23,000 for Cure SMA. Thank you to Amy Allen and Eric and Colleen Ronningen for hosting such an amazing event year after year in memory of Lindsey!

15th Annual Cure SMA Minnesota Chapter Walk-n-Roll
On September 12, 2015 the 15th Annual Minnesota Chapter Walk-n-Roll took place at Silver View Park in Mounds View, MN. There were over 200 registrants who participated in this year’s walk. The walk included games, raffles and an opportunity to meet other families. We would like to thank everyone who helped out this year! Thanks to your contributions, we were able to raise $15,722 for Cure SMA! Some of our top fundraising teams were Team Andrew, who raised over $3,000, Team Bug, who raised over $1,200, and Team Nate, who raised over $1,100 for Cure SMA!

Thank you to our event organizers, and a special thank you to Patty Schwerr, who’s been volunteering with Cure SMA for over 20 years, and has helped year after year with this event! We would also like to welcome Matt Czech and Kara Forcier to the Minnesota Chapter and for being a part of the committee for next year’s walk!

NORTH DAKOTA
Jack Attack on SMA: Round 11
Jack Attack on SMA: Round 11 took place on June 6, 2015 in Fargo, ND at Lindenwood Park. The event raised over $16,000 for Cure SMA! Thank you to Kristi and Rod Gellner for hosting this event year after year in honor of Jack, SMA type III!

A special thank you to our SMA fighting champs who participate in this event year after year: Jack Gellner, Cole Pulkrabek, Ashley Wohl, Chloe & Everley Bartholome, Trish Kuemprer and in memory of our SMA angel, SJ Thomas.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.

MASSACHUSETTS
2015 New England Chapter Walk-n-Roll

The New England Chapter reached a new milestone in 2015: the 15th Annual Cure SMA Walk-n-Roll was held on Saturday, May 16, 2015, at DCR Wompatuck State Park in Hingham, Massachusetts. The Murphy family, in memory of Cianan and Cecilia; the Farrell family, in honor of Aileen; and the Norton family, in honor of Owen, teamed up to organize this walk that brought together over 600 participants and raised almost $140,000.

This event is not only a fundraiser for Cure SMA, but an annual reunion of SMA families throughout New England. The top fundraising teams this year were: Team Murphy, Mac’s Pack, Goin’ for Owen, Walking for William, and Team BraeKer; followed by Wizards of OZ, Team Aileen, Walk for Tamara, Chloe’s Club, Team Stephanie, A Cure for Katie, Team Melanie Lee, and Team Angelica. Also contributing over $1,000 each was: Team Evan, Team Addison, Glen’s Gang and Team Apperian. Also participating were: Team Ian, Vivienne for a Cure, Ella’s Entourage, ImaBeliEVA, Team Greer, and Cheeseman’s Cheerleaders. Other SMA individuals that were represented include: Maya Cutrone, Daryl Denelle, Annika Cederlund, Mark Butler, Lauren Rogowicz, and Dahrian Pimentel. We apologize if we inadvertently left anyone out!

The walk route was 1.5 miles on a paved path through beautiful New England scenery. The raffle is always a big draw – top prizes this year were lottery tickets, a Target gift card, a Lululemon gift card, and Red Sox tickets. Children enjoyed face-painting, balloons and carnival games with prizes. The favorite treats were the ice cream cups donated by H.P. Hood and the potato chips donated by Utz.

Many thanks to Biogen, the presenting sponsor, who also brought a team from the Cambridge office, and long-time sponsors Campanelli and John Hoadley Plumbing & Heating. We owe so much to the volunteers, many of whom have been coming almost since this walk first began. Norwell, MA’s Boy Scout Troop 44 worked the refreshment station once again—not just with great energy, but also very nice manners! We are also very grateful to the local businesses and friends who donated most of the supplies, refreshments and raffle items.

We hope to see everyone again on May 21, 2016! If you want to learn more about this event please contact Silvia Murphy at Silvia@curesma.org.
New England Chapter cont.

2015 Rogo Cup to Cure SMA Golf Tournament

The 3rd Annual Rogo Cup to Cure SMA Golf Tournament was held on June 1st, 2015, at the Atlantic Country Club in Plymouth, MA. The Rogowicz, Butler, Spiegel, and Scully families hold the tournament in memory of Mark Butler, SMA type I, in honor of Billy Spiegel, SMA type I, and Lauren Rogowicz, SMA type III. Once again our wonderful golfers and volunteers powered through a cold wet day! We are so very thankful for having such a great turnout once again. We can’t thank our sponsors, donors, golfers and volunteers enough for their continued support. With their support, we raised over $31,000 this year—bringing the 3 year total of over $115,000 to support Cure SMA. We are looking forward to another great event on Monday June 6, 2016, and thereafter until we finally see a cure for SMA.

Nico’s Night Out Gala

Nico’s Night Out Gala was inspired by an unfortunate diagnosis of SMA on April 25, 2011. My fiery, tenacious red head Nicolas was the heart and soul of such a successful night which raised $27,000 for the research and treatment of spinal muscular atrophy.

Our night began at the Andover Country Club, in Andover, MA. 227 guests browsed and eagerly entered raffle tickets into over 50 raffle baskets. Several items up for silent auction included a signed baseball and photo of Curt Schilling, two third row, third baseline tickets to the Red Sox, a hockey stick signed by Ray Bourque, a Tom Tom GPS watch, a one hour private plane tour of the Boston Skyline, a hot air balloon ride and a golf outing for 4 at the prestigious, private Andover Country Club. We also incorporated a different type of fundraising item which we called a “Chance Box”–the boxes were purchased for $10 each and the buyer was guaranteed to win either a bottle of wine or a $25 gift card to a restaurant. All 100 boxes were sold quickly!

The dinner was accompanied by the performances of Domenic Catino—who is our very own Frank Sinatra—and the gorgeous voice of Vanessa Salvucci. Other amazing entertainment included the sound works of Nick Scenna with DJ 827 Boston and the joy brought by the props in our very own photo booth. Along with dinner and some dancing, the guests were treated to “The Cure,” a purple signature champagne cocktail, which was also huge fundraiser for the night.

The night ended successfully, bringing a sense of purpose to all those who graciously donated, and it humbled the hearts of those guests who were completely unaware of what challenges and devastation accompany a diagnosis of SMA. Together we made a difference. Together we came that much closer to making today a breakthrough.

Jordan’s Journey Golf Tournament

On June 5, 2015 friends, family and SMA crusaders assembled at The Hillview Country Club in North Reading, MA, for a golf tournament and banquet in memory of Jordan Clapp, who passed away from SMA type I at 5 months old. During the golf tournament, participants were able to take part in different activities spread across the course, all benefiting SMA. Once the tournament was complete, the golfers joined other supporters for a banquet–complete with raffles, silent auctions, dinner and drinks. With all of the love, support and generosity from all of the sponsors and attendees the event raised over $25,000! Jordan’s Journey will continue to fight SMA and plans on hosting another event in 2016!

Falmouth Road Race

This past August, I was one of the 11,000 runners who traveled 7 miles along the hilly coastline of Cape Cod in the 2015 Falmouth Road Race. I had previously run the race a few years earlier. That August morning was different, outfitted in our purple shirts, my team of 10 was running for Cure SMA!

In March 2014, my husband and I learned the shocking news that we were SMA carriers. Like so many others, we had no family history and had never even heard of the disease that
New England Chapter cont.

would forever impact our family. We felt helpless and tried to find any way that we could turn this negative into something positive.

In our inaugural year, we had no problem recruiting a team of 10 runners eager to support the team. We had friends who had run the race before and were daunted by the task of fundraising, but who ended up well exceeding their initial goals! We had family who had never run a race before, but who tirelessly trained to run beside us. Together, our team raised over $14,500 – more than 145% of our goal!

With nearly every step, we were motivated by cheers of supporters who saw our shirts and cheered “Cure SMA”. Around mile 3, as we climbed a winding hill, someone asked “What is SMA?”, my husband Patrick shouted “SPINAL MUSCULAR ATROPHY” as he dashed by, raising awareness with each step. Around mile 6, I heard a woman wearing a Cure SMA shirt shout to me “Thank you for running.”

SMA has caused us so much pain and uncertainty. More than eighteen months after learning our carrier status, we still don't know how we'll be able to grow our family. One thing that I know for sure, though, is that we’ll continue to run every August for the Cure SMA Road Race team. We can’t wait for our team to grow each year and continue to spread awareness, and raise critical dollars to help Cure SMA.

Lin Lavalle, Belmont, MA

Enjoyed a BBQ lunch, music, prizes, raffles and great vendors! With their generous donations, we raised almost $900 for Cure SMA.

And this year, Alex, SMA type I, received two huge surprises! For the first time ever, Alex was able to enjoy a motorcycle ride—someone finally found a sidecar for him! He was then made an honorary member of the Minuteman HOG Dartmouth Chapter—and even received his own vest!

Thank you to all who helped make this year the best yet! Hope to see you all next year!

Tommy Foisy, Dartmouth, MA

NEW HAMPSHIRE

Friends of Sofia Equine Ride

We ride to fly, to feel, to touch, to breathe, to laugh, to soar, to overcome, to relax, to get away, to belong, to feel strong, to heal, to love and to be loved back. We ride to live and together, to fight to eliminate Spinal Muscular Atrophy.

Several months ago I knew nothing about SMA, until a friend’s granddaughter, Sofia, was diagnosed with this rare genetic disease. To learn that SMA takes away one’s ability to walk, eat, or breathe and that there is no cure and no treatment – I wanted to rally fellow equestrians together in an effort to show support, raise funds, awareness and most importantly, HOPE.

The Friends of Sofia’s Trail Ride was held on October 10, 2015 at Gelinas Farm in Pembroke, NH, in honor of Sofia Wylie, SMA type I. There were 50 riders who were able to choose one of three different length trails and 17 hikers who hiked the 5 mile trail. All participants were treated to an amazing lunch donated from local businesses, raffles and wagon rides! The event raised $7,262 for Cure SMA.

Robin Susi, Pembroke, NH

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
**Rhode Island**

**Rhode Island Walk-n-Roll**

October 17, 2015, was the date of the inaugural Rhode Island Cure SMA Walk-n-Roll at beautiful Colt State Park in Bristol, RI. About 130 walkers and rollers participated in a 1.5 mile walk through the park, finishing along beautiful Narragansett Bay. Snacks and a raffle followed.

Evan Vaudry’s, SMA type II, church, Four Corners Community Chapel, rallied behind him by forming Team “Chapel 4 Evan”. Chapel 4 Evan had a mini “Walk-n-Roll” one Sunday during the service (led by the Mission Board) and raised a total of $4,390. The entire walk-n-roll raised a record $24,365 for Cure SMA! We are so grateful!

We look forward to the 2nd Annual Rhode Island Walk-n-Roll on October 1, 2016.

Lynne Vaudry, Cumberland, RI

**Vermont**

**Vermont Walk-n-Roll**

Triumph, happiness and gratitude. That is what we felt after Vermont had its very first Cure SMA Walk-n-Roll on May 23, 2015. We had a successful event at Oakledge Park bordering picturesque Lake Champlain. It was successful because of the hard work of many SMA families, our families and friends, supporters from our schools and the local community and businesses sponsors.

Heidi Roy, Development Officer from the New England Chapter kept us (Susan O’Neill, Cari Kelley, Melanny Baker and Ashley Febvay) motivated and helped us with all aspects of holding a first-time event. Attendees enjoyed the walk, food and raffle items. Champ, the mascot from our local minor league baseball team, the Lake Monsters, was a hit as well. The event made $17,000, surpassing original goal of $5,000!

On a personal note, this event was bitter sweet. It was heartwarming to have so much support in putting together this event but sadly, only one of our two children were there to take part. Our son, Colin, passed away in November of 2014 from complications of SMA type 1. He was 13. Our 18 year old son, Casey, also has SMA type I and was a big part in the success of the event. He rallied some classmates and teachers, and created Team Sharks: Taking a Bite Out of SMA. Team Sharks had the largest team and was the top fundraising team, raising $4,161. Casey did this in honor of his little brother.

Looking forward to our next Walk-n-Roll and connecting with the SMA families in the area, raising funds and awareness of spinal muscular atrophy.

Sue O’Neill, Multan, VT

**New Mexico Chapter**

**2015 Bugaboo Workout of the Day**

On September 26, 2015 the New Mexico Chapter, CrossFit Albuquerque and Lululemon Athletica hosted the annual Bugaboo Workout of the Day (WOD) at Lululemon Athletica Albuquerque Uptown. The weather was perfect on event day and brought out 175 participants for a morning of workouts and awareness. The event raised over $11,000 for Cure SMA in memory of Benjamin “Bugaboo” Abruzzo III. A big thank you to Lululemon Athletica and Ben and Natasha Abruzzo for organizing this successful event!

---

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
North Jersey Chapter

4th Annual Best Meatball Contest in Secaucus
The 4th Annual Best Meatball Contest in Secaucus event was held on April 25, 2015 at the Immaculate Conception Church Gym in Secaucus, NJ. The year’s event was hosted by the local Knights of Columbus and is held in honor of Daniel Cevallos, SMA type I. In total, $7,516 was raised for SMA research! Thank you to Giovanni Recalde and Paulina Recalde for organizing this event!

Walk-n-Roll in Honor of Katherine Santiago
Last spring, our local Cure SMA Walk-n-Roll in honor of Katherine Santiago held on Saturday, May 16, 2015 in Millburn, NJ reached its 8th year. We raised over $7,000 with the support of our sponsors, friends and family. The contributions fund research and help families who are dealing with SMA. Some of the research funded by Cure SMA was awarded to Dr. Mustafa Sahin at Boston Children’s Hospital and Allie Mazzella, who started this walk 8 years ago, has joined Dr. Sahin’s research team! This year we had many activities and a raffle that those who attended were able to take part in. Activities like volleyball, face paint and contests helped contribute to the success of the event. We were able to walk one mile on a warm, spring morning. We continue to run this fundraiser with the help of Cure SMA, and our supporters. We hope to keep this walk going strong for many years to come!

Dr. Herbert N. Richardson 21st Century School Fundraiser
It was an amazing pleasure to have hosted the first ever SMA Fundraiser at the Dr. Herbert N. Richardson 21st Century School in Perth Amboy, NJ. The event took place on Friday, June 19, 2015 and it was a huge success. Each staff member wore their Cure SMA bracelets on Friday, June 19, in recognition and support of Cure SMA and raised $450 in one fun afternoon!

Lauren Goldzweig
Perth Amboy, NJ

Cocktails for a Cause
Ryan’s Buddies Foundation of Rutherford, NJ, hosted the 4th annual “Cocktails For A Cause” on July 31, 2015, at The Fiesta in Wood Ridge, NJ. This event honored the memory of Ryan James Reilly, who was born on June 19, 2011, and joined all other S.M.A. angels on March 6, 2012.

Ryan’s Buddies, a union of the Rosenbower Family and Friends, was formed to commemorate Ryan’s life, as well as to raise funds for and awareness about SMA; and other worthy causes.

More than 140 guests enjoyed an evening of cocktails, food, raffle prizes and music. We are forever grateful to the many sponsors of our event who donated both monetarily and in the form of in-kind donations and we are proud to say that the event raised over $22,000 of which $12,500 was donated to Cure SMA. We are also grateful to our family and friends who continue to support our organization. We will continue to raise funds for research and patient-care until a cure is found. For more information on Ryan’s Buddies, please visit our website at www.ryansbuddies.org

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
CALIFORNIA

Concert for a Cure

The 15th Annual Concert for a Cure was held on May 9, 2015 at the Diablo Country Club in Danville, CA and was a tremendous success! This year’s event raised over $142,000 with the unique theme of “Shoot for a Cure” under the stars. This memorable evening was made especially rewarding for donors, families and guests when Dr. John Day, Stanford Professor for Neurology, Pediatrics, and Pathology, highlighted the exciting advancements in SMA and the IONIS (formerly known as ISIS) clinical trials.

Guests were deeply moved by Danny McHale, SMA type II, and Ariana Dindzan’s, SMA type III, presentation along with special check presentation from Danny’s classmates at Monte Vista High School in Danville where they raised over $7,300. The 40 plus freshmen and their siblings were a huge hit as this marked their sixth year serving at Concert for a Cure selling raffle tickets, running games and encouraging donors to give generously.

Mary and Joe McHale founded Concert for a Cure in 2001 shortly after the diagnosis of their son, Danny, with SMA type II. Nancy and Andris Dindzans joined the efforts in 2003 when their daughter, Ariana, was diagnosed with SMA type III. Both Danny and Ariana will turn 15 years old this year. Over the years this event has raised over $1.45 million to fund Cure SMA research programs.

THANK YOU! The McHale and Dindzans families send out their heartfelt thank you to the friends, families, and local community for their generosity in their continuous support of SMA families and children everywhere.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Northern California Chapter cont.

5th Annual Cure SMA Sacramento Walk-n-Roll
On October 17, 2015 families, friends, and local supporters 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. The event consisted of face painting, balloon artist, DJ, raffles, local vendors, memory garden, incredible lunch and friendship.

Nina Daya, Charlotte Weber and Wayne Waggoner 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 $7,000 was raised this year.

13th Annual Cure SMA Northern California Walk-n-Roll
Our 13th annual Walk-n-Roll in San Francisco, CA on August 22, 2015 was a blast! We remembered our SMA angels with a beautiful memory garden and honored our SMA warriors. Dr. John Day from Stanford University Medical Center and Kenneth Hobby from Cure SMA updated us on the latest SMA research. Then we warmed up with energetic Zumba exercises before heading out on our two-mile walk through beautiful Golden Gate Park. We paused at the Conservatory of Flowers for our traditional photo before proceeding to Rainbow Waterfall and back to Peacock Meadow. The fun continued in our site with face painting, balloon creations, tattoos, nail salon, games, lunch and a lively raffle. We raised over $60,000 for Cure SMA. Thank you to our families and friends for their generous donations and to our gold sponsors: Cytokinetics, Genentech, Live Rhynstrong Foundation, and Palo Alto Medical Foundation. Thanks also to our silver sponsors Convaid, Echos, and Mobility Works. We appreciated our new relationship with LiveRamp, too. Let’s do it all again in 2016!

New Cure SMA Club!
Monte Vista High School in Danville, CA has started a new Cure SMA Club. The founding officers are Brady Martin, Natalie Couture, Hannah Doris and Julia Berg. The focus of the club is spreading awareness and fundraising activities for Cure SMA. We have had tremendous results already with 190 students joining the club. Stay tuned for more great news from Monte Vista High School.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
OKI Chapter

OHIO

2015 OKI Chapter Walk-n-Roll
The 12th Annual OKI Chapter Walk-n-Roll held on June 13, 2015 in Cincinnati, OH was a tremendous success! Everyone enjoyed the kid’s carnival, magician, balloon sculpting, raffle and music!

This year almost $80,000 was raised for Cure SMA! Thank you to our 25 amazing fundraising teams for their efforts. A special thank you to Kevin and Beth Lockwood, Bob and Elizabeth Lockwood, Nicole, Mark and Amy Haake, and our dedicated raffle committee for all of the time and dedication you put into planning the Walk-n-Roll each year.

Cassano Cares Foundation Golf Outing
Thank you to the Cassano Cares Foundation for making Cure SMA their benefitting charity for this year’s golf tournament held on September 28, 2015 in Dayton, OH. Proceeds totaling over $45,000 were given to Cure SMA in honor of Maggie and Charlie Monnin. Thank you to Chip Cassano and the Cassano Cares Foundation for their tremendous support! Special thanks to the Monnin Family for all of their help with the tournament.

Cure SMA Golf Birdies for Blake
The 2015 Cure SMA Golf Birdies for Blake raised nearly $22,000 for Cure SMA! Despite the extremely wet weather, over 100 people came out to support the outing on October 3, 2015 in Liberty Township, OH. It was the biggest Birdies for Blake fundraiser yet!

A special thank you to the whole Farrell Family for their dedication to this event, and support of Cure SMA in honor of Blake!

2015 Cure SMA Dayton 5K in Honor of Emmett Keeton
The 2015 Cure SMA Dayton 5K in Honor of Emmett Keeton held on September 13, 2015 in Dayton, OH raised over $8,700 for Cure SMA! Over 75 runners came out to EastWood Metro Park to help raise funds and SMA Awareness. A special thank you to the Rodgers Family and Keeton Family for all of their work putting this event together!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Dancing with Lima
Thank you to Marie Niese for all of her hard work on the 2015 Dancing with Lima event! The event, held in Lima, OH on October 8, 2015, brought together local celebrities, politicians, and professional dancers for a night of fun dance competition. Over $5,000 was raised to support Cure SMA!

The Jet Express Put-In-Bay Boat Line Company Ticket Sales Fundraiser
Thank you to the Blumensaadt Family for organizing The Jet Express Put-In-Bay Boat Line Company Ticket Sales Fundraiser again this year on June 6, 2015. Each year they host this fundraiser in honor of Will Blumensaadt who passed away from SMA type I in 2005. Thank you to The Jet Express and everyone who purchased tickets. Over $3,300 was raised for Cure SMA.

Children Making Change
Children Making Change, a program started by Ashley Fisher, lets children start to think about fundraising when they are younger while doing age appropriate chores/activities. At the kickoff meeting for this program, several children present local organizations that are meaningful to them. This year one of the families involved presented SMA. A friend in Pennsylvania that has two children with SMA reached out to Children Making Change. Over $2,700 was donated to Cure SMA!

Flying Pig Marathon
Thank you to all of our amazing runners that participated in the 2015 Flying Pig Marathon on May 3, 2015 in Cincinnati, OH. Because of their dedication to our mission, they raised over $1,830 for Cure SMA!

Anspach Law Fundraiser
Thank you to the Anspach Law employees at the Toledo office for fundraising for Cure SMA! Thanks to the efforts employees, and a generous match by the firm, $200 was raised for Cure SMA in November.

KENTUCKY
Cure SMA Cure Kirsten 5K/10K
The inaugural Cure SMA Cure Kirsten 5K/10K held in Paintsville, KY on October 17, 2015 was a huge success! The event, hosted in honor of Kirsten Cornett, raised over $11,000 for Cure SMA. The 5K/10K brought together the Paintsville community and helped spread SMA awareness. A special thank you to Wayne Cornett and the entire Cornett Family for their incredible work on this year’s event.

2015 Cure SMA Walk-n-Roll with Jonah Smiles
The 2015 Cure SMA Walk-n-Roll with Jonah Smiles in memory of Jonah Setser took place on September 12, 2015 at BG Crossroads Co-Op in Woodburn, KY. Thank you to Michelle Setser, Sarah Martin and all those who participated in this special event which raised $1,500!

Deacon Alexander Memorial Car Show
Thank you to the Perry Family for hosting the 3rd Deacon Alexander Memorial Car Show on August 15, 2015 in Walton, KY. The event is hosted each year in memory of Deacon Alexander Perry who passed away from SMA. Thanks to the efforts of the Perry Family and the support of their community, over $1,600 was raised!

Grimes Family
Thank you to the Grimes Family for fundraising for Cure SMA! They hosted a silent auction in memory of Annie Grimes for SMA awareness month in August and raised over $1,000.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
INDIANA

Inaugural Cure SMA 2015 Marion, IN Walk-n-Roll

Thank you to everyone who was able to join us for the first ever Cure SMA Marion, IN Walk-n-Roll, held on Saturday, August 29, 2015 at Matter Park in Marion, IN.

We had over 100 participants who joined us for a beautiful day raising awareness for SMA, fundraising and walking a 1-mile course. The weather was beautiful with lots of people gathering for a fun raffle, face painting for both kids and adults and a great DJ, with funds reaching over $7,326!

A special thank you to the Griffin Family: Jessica, Brian, Chloe and all of their family and friends who founded the walk in honor of their son, Aiden, SMA type I, who passed away from SMA in January of 2015. It was a beautiful day that brought a lot of awareness to the community for Aiden and all of those working towards a cure and/or treatment for SMA.

Thank you to Aiden’s Army for the amazing fundraising they did for this event and also to the sponsors, MGH Radiology, Richard A. Evanseck Family & Cosmetic Dentistry and Café Valley Bakery Outlet for your support!

The Cure SMA 2015 Marion, IN Walk was also nominated for the Beacon Credit Union Spotlight Project, and won 3rd place, bringing in an additional $500! Thank you to everyone who voted!

Pedal for Graham

A chilly, windy Saturday in October didn’t stop our awesome riders who participated in the Crane Cruise Bike Ride that took place in Medaryville, IN. We had 11 riders who rode anywhere from 14 miles to 45 miles to bring funds and awareness to Cure SMA for Graham Vollmer, SMA type I.

Thank you to each and everyone one of our riders for your support and help to raise over $4,705. A special thank you to Jamie Henderson who found the event to partner with and helped get the process rolling for this great event! Also, thank you to all of the kids who participated in helping to get their teachers involved in the race, from Wheatfield Elementary School!

SMA Awareness Month

Thank you to Julie Davis, proud Grandmother to Alexander Davis, SMA type II, for raising awareness throughout SMA Awareness Month in her community!

A Special Thank You

A special thank you to Michelle Palmer! We wanted to take a moment to say thank you to Michelle Palmer who has been the event organizer for the past four years for the Cure SMA Indianapolis Walk-n-Roll. Michelle, we cannot thank you enough for all of the support and hard work you put into making the Indianapolis Walk-n-Roll a great success!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
IDAHO

2nd Annual Flamingo Fun Run
The 2nd Annual 5K Flamingo Fun Run took place on September 26, 2015 in Grangeville, ID. Oakley Smith, SMA type II, a vibrant, lovable, four year-old with curly red hair is the inspiration behind the annual flamingo fun run. With incredible community support, and a lot of donated help from friends and family, the 5K raised over $7,700 toward finding a cure. Local and national businesses contributed money, donations for the raffle, and food for the participants. Thank you to the Smith family for organizing this amazing event for SMA and the Grangeville community.

WASHINGTON

8th Annual Pacific Northwest Walk-n-Roll
On September 12, 2015 the 8th Annual Walk-n-Roll took place. The day was perfect – sun and pleasant temperatures brought out over 200 people for a morning and afternoon of family fun and awareness. The Auburn Game Farm in Auburn, WA was a great venue with lots of space, playground and a covered shelter area. In addition to a wonderful day, activities consisted of a DJ, games, face painting, fabulous raffle, silent auction, lots of vendors, and lunch. Also, everyone had fun taking a turn to #SMAshSMA with water balloons on our Cure SMA smash board. Thank you to all the sponsors, generous friends and families, and committee. We raised $30,270 for Cure SMA! See you next year!

17th Annual Wannabe Cup
On August 14, 2015, the annual Wannabe Cup Charity Golf Tournament was held at the Del Mar Country Club, in Del Mar, CA. The event was filled with 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 $15,000 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 10th Annual Wreaths of Hope took place this past November in Washington. Guests at the event enjoyed making holiday greens in an old fashioned holiday barn, caroling, raffle, enjoyed homemade baked goods and hot cocoa. The event was hosted by Sybil Kuhn and raised over $1,000.

SMA Awareness Day at the Mariner’s
The 7th Annual Cure SMA Day with the Seattle Mariners in memory of Gabby Stack was held August 9, 2015. The day was enjoyed by all with friendships, family and a great day of baseball.

August SMA Awareness Month Event
On August 12, 2015 Lilee Ford, SMA type II, had what she has deemed “the best day” of her life. She got to hang out with the special effects wizards at SynapseFX on the set of one of her favorite T.V shows - SyFy channel’s Z Nation. She was able to observe the artists expertise and even got to help apply make up to a zombie extra! She also got to meet a number of cast and crew members. These wonderful people showed their support when told of SMA Awareness month and graciously posed for photos with Lilee’s “CureSMA.org” sign.

OREGON

Portland Rock ‘n’ Roll Half Marathon
The Portland Rock ‘n’ Roll Half Marathon took place on Sunday, May 17, 2015 in Portland, OR. The marathon raised over $2,125 for SMA research. A special thank you to our dedicated Cure SMA runners!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Pennsylvania Chapter

Muscles for Mckenna Gala

In its fourth year, the Muscles for Mckenna Gala raised over $110,000 in support of Cure SMA! This event was held on March 13, 2015 at The Crystal Tea Room in Philadelphia, PA. The night included dinner, drinks, live entertainment by The Heartbeats, dancing and a silent and live auction. This event was flawless with a red carpet entrance and photography by Gina Marie Photography.

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

Special thanks to Kellie Keenan for chairing this event in honor of her niece, as well as to Amy and Jim Ellixson and the wonderful committee of volunteers. Thanks to their time, energy and dedicated support this event was a huge success and continues to lead Cure SMA toward a treatment and cure.
2015 Cure SMA Pennsylvania Chapter Walk-n-Roll
The Cure SMA Pennsylvania Chapter Walk-n-Roll hosted its 12th annual event on May 17, 2015 at the Lloyd Hall Recreation Center in Philadelphia, PA. Thank you to all of the dedicated sponsors who helped make this day a huge success! The fun-filled event included activities for the whole family to enjoy! The event brought together roughly 800 walkers and rollers to help fund a cure for SMA. In total, this year’s event raised almost $95,000!

Thank you to all of the amazing SMA families, participants, and donors for helping make this event a huge success. This year’s teams included: Abby’s Army, Cousin Cruisin’ for a Cure (Kerri and Eric), Fighting the Fight, Gavin’s Corsairs, Lukie’s Tigers, Matthew’s Musclemen, Muscles for McKenna, Omar’s Soldiers, Peter’s Philadelphia Eagles, Philly SMAck Down, PromptCare, Team Bayada, Team Epic A.K.A. Captain Morgan Ahoy!, Team Jordan, Team Lyla, Team Marc, Team Saxton, Team KendalGrace and Team Jack Pack.

A special thank you to Karen McRory-Negrin, Allyson Henkel, Paula Saxton and Tara Maida for all of your hard work in planning this event year after year!

7th Annual Zane’s Run
Our 7th Annual Zane’s Run was another fantastic year! It was held on September 27, 2015 in Malvern, PA. We had our largest turnout ever with 469 participants and over 100 spectators and volunteers. This year, we were honored to have several newly diagnosed SMA families attend. While it was wonderful to connect with them, it was saddening all the same. The results of everyone’s outstanding fundraising efforts totaled $33,590! Thank you to our sponsors, volunteers, the great team of organizers, cheering squads, and all of the participants and teams which came from five different states. I know Zane was looking down smiling her sweet smile sending high fives.

Our sincerest thanks!
The Schmid Family
Malvern, PA

12th Annual Lukie’s Fall Festival
October 10, 2015 marked the 12th Annual Lukie’s Fall Festival, held at Weona Park in Pen Argyl, PA. Lukie’s Fall Festival is a fun and entertaining day, where families can come and have a great time together, while helping to raise money for Cure SMA. This year Lukie’s Fest collected $14,624 to help fund programs and treatment to find a cure for SMA. The fun day-long festival included food, games, raffles, live music, entertainment, a pie eating contest, kid’s costume parade, face painting, hay rides, clowns, balloons, mini golf, carousel rides, pumpkins, a bake sale, sand art, a Fire and Safety Program, and family fun for everyone! Lukie’s Fall Festival was founded in memory of Luke Anthony Joseph Maida, SMA type I. This annual event helps raise awareness and funds to help find a cure for SMA. It is through support and hard work of Lukie’s family, friends and community that has helped make Lukie’s Fall Fest more than a decade-long success. Thank you!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
2015 GORE-TEX® Philadelphia Marathon, Half & 8K


Special thanks to our runners: Matt Barnard, Michele Barnard, Steve Cannady, Erin Fadako, Lisa Kanehann, William Kanehann, Chelsea Mangini, Sarah Rodriguez, Dylan Saldutti, Joe Staples, Eileen Venedam and Jamie Wood.

You all ran a wonderful race and we greatly appreciate the time it took to train and fundraise for Cure SMA. Also, a big thank you to Christie Barnard for your help in organizing this year’s event!

6th Annual Dance Away SMA

The Lyla Mertz Foundation held the 6th Annual Dance Away SMA event on October 3, 2015 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. The event featured a Chinese auction, helping to bring the total amount raised to $6,000. Thank you to the Jen and Steve Mertz and their amazing volunteers for all of your hard work planning this event!

12th Annual Lily Kennedy Golf Outing

October 4, 2015 marked the 12th Annual Lily Kennedy Golf Outing at the Iron Masters Country Club in Roaring Springs, PA. Attendees enjoyed a day of golf and outdoor activities in honor of Lily Kennedy, SMA type I. Thanks to everyone’s support, the event raised $5,006 to help fund a treatment and cure for SMA. A special thanks to Heather Kennedy and Dr. Mark Lynch for all of their hard work and planning to make this outing a great success!

5th Annual Swing for a Cure Golf Tournament

The Swing for a Cure Golf Tournament held on August 9, 2015 at the Mahoning Valley Country Club in Lehighton, PA raised almost $3,000. 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 to the Mertz family for your tireless efforts in organizing such fun and successful fundraisers and for your continuous support of Cure SMA!

2015 Pittsburgh Marathon/Half Marathon

It was a great, sunny day for the 2015 Pittsburgh Marathon. Actually, perfect spectator weather! By the end of the race, it was pretty warm for the runners - but it was tolerable. Great crowd support. Heather, Tom and a couple other friends of ours were there to cheer me on (Jess couldn’t make it). I am still amazed by the wonderful support of so many friends and family to my fundraiser page. Unbelievable! I would love to see this grow next year - it would be great to become an ‘official’ charity and get more runners involved! Again - so thankful for so many supporters - many thanks! This event raised over $500!

Carol Tomko
Pittsburgh, PA

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Muscles for Mckenna Day
Special thanks to Sandy Roberts and the Buckingham Elementary School for organizing a fundraiser in honor of Mckenna Ellixson, SMA type III, on Saturday, March 16, 2015. Thanks to their efforts, $300 was raised in support of Cure SMA!

Clairview School Jeans Day Fundraiser
During the month of May, Clairview School in Greensburg, PA helped support Cure SMA by hosting a jeans fundraiser and raised $115! Thank you to Rachel Butler-Pardi for organizing this event!

Charity Basketball Game
On February 28, 2015, the Pleasant Valley Youth Association coaches held a charity basketball game to raise money for SMA. Each year the association chooses a different charity to raise money for. This year they chose Cure SMA. The game involved all the coaches in the area against each other. What a great game played by the coaches for such an amazing cause. The teams were divided by the colors orange and purple to represent Cure SMA. Children of all ages sat in the bleachers and cheered their coaches on. A very big thank you to all the coaches from the Pleasant Valley Youth Association, especially Alton Smith, Hope Smith, the Frazier family, and everyone who attended.

Christina Mohammed
Stroudsburg, PA

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 32 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.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
COLORADO

2015 Colorado Walk-n-Roll & Run 5K

The 2015 Colorado Walk-n-Roll & Run 5K was held on August 29, 2015 at Clement Park in Littleton, CO. On event day the weather was beautiful and the walk had an amazing turnout with over 400 participants. Attendees enjoyed music, face painting, a silent auction, raffles, and lunch. Thank you to all our generous friends and families, sponsors, and event day volunteers for making this year’s walk possible. Through your continued support and dedication the event raised over $78,000 this year and $362,700 since the walk began in 2007! Cure SMA would also like to extend a special thank you to our event organizer Julie Lino and her family for all of their hard work in making this year’s event the most successful yet.

12th Annual Rocky Mountain Charity Golf Tournament

The 12th Annual Rocky Mountain Charity Golf Tournament took place on Monday, May 18, 2015 at the beautiful Club at Pradera in Parker, CO. Congratulations to the 2015 tournament winners Paul Dickson, Nate Estep, Calum White, and Eric Drucker! Thank you to Gillian Faith and her family for organizing the tournament which raised over $24,000! Cure SMA would also like to thank all the players, sponsors, and event day volunteers who helped make this year’s event a success. Thank you!

BBQ and Pool Party in honor of Lexi Pacini

On August 15, 2015, Hyatt House Colorado Springs in Colorado Springs, CO, hosted a BBQ and pool party fundraiser to raise funds and awareness of SMA. Organized by Nick Kelley, the event raised over $6,500 for Cure SMA in honor of his niece, Lexi Carita Pacini, SMA type II. Thank you for your support!

Cure SMA Day with the Rockies

The Rocky Mountain Chapter had great day on August 22, 2015 at Coors Field in Denver, CO, celebrating SMA Awareness Month. More than 50 friends and families turned out for a pre-game picnic and Star Wars parade, followed by the game. It was great to have long time families as well as new ones join us for some fun and to help raise awareness. Thank you to everyone who came, and to Lyndsey Rice for her help in making this 2nd annual event a success! We are looking forward to doing it again next year and we hope you can join us.

Loree Weisman
Longmont, CO

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Southern California Chapter

CALIFORNIA

2nd Annual Southern California Gala of Hope

On October 10, 2015 over 180 guests gathered at the Ritz-Carlton Laguna Niguel in Dana Point, CA for the 2nd Annual Southern California Cure SMA Gala of Hope. This incredible evening raised over $103,000 in support of Cure SMA. The event brought together families, researchers, doctors, business leaders, philanthropists, as well as film and TV stars—all to raise funds to find a treatment and cure for SMA.

The evening consisted of cocktails and hors d’oeuvres on the Promenade Lawn with a silent auction and jazz quartet, followed by a three-course dinner, live auction, dancing and special presentations by Kenneth Hobby, President of Cure SMA and Nikki McIntosh, event chair and mother of Miles, SMA type II. The evening was a tremendous success for Cure SMA and inspired hope for all those that attended.

Thank you to the sponsors who generously supported this event: First Foundation, IONIS Pharmaceuticals, Lomonaco Design, Dr. Cary Templin, The Zenith, Western States Acquirers Association, Orange Coast magazine, Los Angeles magazine and Digital Lizard. Thank you to our dedicated committee who made this night possible: Jennifer Friedman, Amy Liepold, Myrna Liepold, Nicole Lomonaco-Sunde, Nikki McIntosh, and Autumn Montoya.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
CALIFORNIA

Reach4Sky Swimfest
On Sunday, October 18, 2015, Team Reach4Sky successfully completed the San Diego to Coronado Sharkfest swim! All 30+ team members made it safely to shore. And a nary shark was spotted in the process, although a few swimmers did report seeing a mean looking halibut near the finish line!

Team members ranged in age from 11 to 60, and in swimming skills from former state record holders to “I just hope to finish the race” novices. Our purpose was to have fun, finish safely, and raise money to help find a cure for spinal muscular atrophy. And that’s where you came in. While planning the swim, we set a goal of 400 donors and raising awareness of SMA. So far, we’ve received 266 donations, and have raised $31,065 along the way.

On behalf of Team Reach4Sky, thank you for your contribution to Cure SMA. One day, researchers WILL find a way to overcome this terrible disease, and your contribution brings us one step closer to the cure.

In his short life, Skylar had a profound impact on those who knew and loved him. We are grateful for your part in allowing us to honor his memory in such a meaningful way. Wow – what a race, what a weekend, what a wonderful way to celebrate the life of Skylar Bahrenburg!

Cure SMA would like to congratulate and thank the Bahrenburg, Matthews and Amlieck families for their hard work and dedication in planning the event. The response that was received from your family and friends is truly incredible. The funds and awareness that you raised is inspiring hope that one day we will find a treatment and a cure for Spinal Muscular Atrophy. Congratulations to all of members of Team Reach4Sky on successfully completing the Sharkfest swim!

San Diego Padres SMA Awareness Day
The 2nd annual San Diego Padres SMA Awareness Day took place on August 22, 2015 in San Diego, CA. The day brought together friends and family members for a full afternoon of tailgating, on the field experience, and a winning game by the Padres. The day raised $11,451 for SMA research. A huge thank you to Samantha Velchansky for organizing this tremendously successful outing and to Isis for hosting the tailgate. Looking forward to next year!

7th Annual Inland Empire Walk-n-Roll
On October 25, 2015 we held our 7th 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 300 participants. Throughout the day, fun was had by all with an amazing raffle, a cookout-style lunch, a DJ, performance by a drum corp 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 to raise nearly $12,000. See you next year!
Team Cure SMA – Rock ‘n’ Roll San Diego

Team Cure SMA – Rock ‘n’ Roll Marathon Series in San Diego was held on May 31, 2015 in downtown San Diego, CA. Everyone teamed up to run the San Diego Rock ‘n’ Roll Marathon and Half Marathon to raise money and awareness for Cure SMA. In total, Team Cure SMA raised over $3,600 to find a treatment and cure for SMA. Thank you, to all that came out to run and support our team! See you in 2016!

Los Angeles Rock ‘n’ Roll Marathon and Half Marathon

The Los Angeles Rock ‘n’ Roll marathon and Half Marathon took place on Sunday, October 25, 2015 in Los Angeles, CA. The marathon raised over $2,000 for SMA research. A special thank you to our dedicated Cure SMA runners!

Dinner and Donate at Ruby’s – In memory of Jaimie Ramsey

On November 18, 2015, Ruby’s Diner of Tustin, CA held a Dine and Donate night to bring community awareness to SMA. Lynn and Ade DeBlaiso hosted the event at Ruby’s where they welcomed and educated the dining guests of SMA. Overall, the event raised over $500 for Cure SMA in memory of Jaimie Ramsey, SMA type I, granddaughter of Lynn and Ade. Thank you for your continued support!

Wipe Out SMA with Ricochet the SURFice Dog

Cure SMA and IONIS Pharmaceuticals continued SMA Awareness month into September by hosting a free assisted surfing experience by Ricochet the SURFice dog and professional surfers on the beautiful beaches of La Jolla Shores. On Wednesday, September 8, 2015 in La Jolla, CA, Logan Velchansky, SMA type II, Zain El-Hallak, SMA type III, Riley Sommerville, SMA type II and Bailey Sommerville, SMA type I, surfed for the first time with Ricochet. What an incredible experience each our surfers had that day. We enjoyed lunch on the beach provided by Isis. To conclude the day, we also SMAshed Away SMA by smashing a Cure SMA sandcastle.

A huge thank you to Max Moore and Kristina Bowyer for arranging this incredible event and an incredible thank you to Ricochet and Judi for making the afternoon possible for those affected by SMA.

Southern California Summer Pool Party

On August 8, 2015 the Southern California Chapter hosted a fun-filled afternoon of swimming, pool games and cookout followed by candle lighting for SMA awareness month in Garden Grove, CA. Thank you to everyone that came out to enjoy the afternoon and see you at the next one!
12th Annual Jacob’s Run, Walk & Roll to Cure SMA

The 12th Annual Jacob’s Run, Walk & Roll to Cure SMA was held on April 12, 2015 at South Country Regional Park in Boca Raton, FL.

In total, the event raised over $105,000 for Cure SMA.

The Jacob Isaac Rappoport Foundation was founded in memory of Jacob Rappoport, SMA type I. The foundation provides funding for basic research and drug discovery programs; sponsors the Annual SMA Conference; enables Cure SMA to send care packages of appropriate toys to every newly diagnosed child with SMA type I; and funds unique programming and childcare at the Cure SMA conference. Over the years, the foundation has raised over $1.3 million.

Thank you to Adi and Shaina Rappoport for their incredible support of Cure SMA, and everything they do each year to raise awareness of SMA!
2015 South Florida Gala of Hope

The 5th Annual South Florida Chapter Gala of Hope, on November 12, 2015, was a fantastic evening to raise funds and awareness of SMA. This year’s event has already raised over $244,000, and money is still being tabulated! In its five-year history, the event has raised an incredible total of over $700,000 for SMA research and family support.

The event was founded and is hosted by Fiorenna Israel and Jennifer Miller Smith, in honor of their daughters. Madison Smith and Mia Israel both have SMA type II.

The evening entailed a night of luxury featuring a deluxe open bar by Premier Beverage, cocktail hour provided by Buddha Sky Bar and Buddha Garden, hors d’oeuvres provided by The Capital Grille, a silent auction, raffle, and live entertainment by Michael Israel. VIP guests had access to an exclusive VIP area, sushi from Dapur, premium spirits, photo opportunity with Michael Israel, and more!

A special performance by Michael Israel raised over $50,000 for Cure SMA!

Our thanks to Jack Bardakjian for his generous support of the gala. Jack accepted an award on behalf of Ferrari-Maserati of Fort Lauderdale, honoring their commitment to the Gala of Hope and Cure SMA over the past five years.

7th Annual Steven’s Swing for a Cure

On Friday May 1, 2015 over 70 golfers joined Cure SMA South Jersey/Delaware Chapter at Jonathan’s Landing Golf Course in Magnolia, DE. Thanks to our participants, sponsors and donors we raised over $16,000. A huge thank you to my mom, Joan Smith, and my sister, Justine Nichols, for all of their hard work and efforts in assembling and donating many of the baskets and raffle items. Hope you can join us for our next one!

Jessica Moyer
Magnolia, DE

10th Annual Steven’s Walk to Drum Out SMA

The 10th Annual Steven’s Walk to Drum out SMA was held on May 9, 2015 at Newton Lake Park in Haddon Township, NJ. It was a beautiful day filled with family, friends, neighbors, our local fire department, our local Walgreens, Hair Cuttery, a live band (JAM) and some new faces. We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful auction items donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA! We had wonderful Chinese auction baskets donated by family members, friends, many companies and BAYADA. We had a 50/50 raffle, and the young man that won donated all of the money back to Cure SMA!

With Steven heading to college, we decided that this would be our last year for Steven’s Walk! It has been an amazing journey hosting this event year after year, raising $16,000 in our last year, which was a huge success! Thank you to all of our dedicated volunteers, participants, sponsors and donors over the past 10 years. We’ve been truly grateful for your support!

HUGE thank you to Terri Potter and her family and friends for their 10 years of service and support of Cure SMA, and we look forward to continuing to support and work with you and your family!

2015 Delaware Marathon

On May 10, 2015 20 participants came together and took to the streets of Wilmington to run in the Delaware Marathon to support Cure SMA. The teams were organized by John and Nicole Cheslock in honor of their daughter, Eden, SMA type II. This is the fourth year that the Cheslock’s have been committed to this race for raising money to benefit SMA. Team Cure SMA raised over $15,000! Thank you to the Desroches family for organizing runners in honor of PJ, SMA type I.

Special thanks to our runners: AJ Alfieri, Charlie Alfieri, Pat Cappelaere, John Cheslock, Nicole Cheslock, Cristina Colon, Brian Cusick, Pete Desroches, Georgia DuHadaway, Dean Holden, Kimberly Holden, Cara Hughey, Erica Jones, Jennifer Kelly, Christopher Manning, Dan Reville, Erica Richard, Jonathan Richard, Allison Righter, Jennifer Rios, Luis Rios, Anoma Russum, Sarah Sharp, Deborah Smith, Stacie Smith, Jasper Sumner, Alyssa Sweeney, Mark Sylvester, Meghan Sylvester and Zara Young.

2015 CrossFit Dover 5K Walk/Run Fitness Challenge 1.5 Mile Run

The 2015 CrossFit Dover 5K Walk/Run Fitness Challenge 1.5 Mile Run was held on Friday, June 26, 2015 in Dover, Delaware raising over $6,000 for Cure SMA! Thank you to event organizer Jessica Moyer as well as Ray Parker of TriSports Events Management for all of your support and hard work in creating such an amazing event. Not only did Jessica help organize this event she was the overall champion in the 1.5 Mile Fitness Challenge!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
2015 Cure SMA Hanna’s Hope for a Cure Softball Tournament
Thank you to Gary & Ashley Warfield for hosting the 2015 Hanna’s Hope for a Cure Softball Tournament on May 1-2, 2015 in honor of their daughter Hanna Warfield, SMA type II. Eight teams came out to the Crossroads Community Church ball fields in Georgetown, Delaware to play in the tournament which raised over $3,100!

SMA Golf Tournament in Memory of Steven Moyer
The SMA Golf Tournament was held on July 12, 2015 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 $3,100. A special thanks to Steve Moyer, Steven’s grandfather, for organizing this event.

Bingo Fundraiser in honor of Olivia Calvert
The 2015 Bingo Fundraiser in honor of Olivia Calvert, SMA type II, was held on Saturday, August 15, 2015 at the Steamboat Landing Campground in Lewes, DE. The event raised $1,500! Thank you to the Calvert Family and Steamboat Landing Campground for your continued support of Cure SMA!

#SMAsh SMA Event
My office team participated in a SMAsh SMA event in our office on September 2, 2015. The all-female staff bravely donned shorts and t-shirts to smash balloons in our back parking lot. As you can imagine, it was hysterical! We recognize that SMA is not so funny and in an effort to help cure SMA, we collected $60 at this event.

Alice Knott, Milford, DE

Carlee’s BIG SMA Shoot Out
Thank you to the Beam Family for hosting Carlee’s BIG SMA Shoot Out! The event, held on September 19, 2015 in Nashville, TN, in honor of Carlee Beam was a huge success! Thanks to the generosity of over 20 shooting teams, 100 participants, and countless volunteers, $42,700 was raised for Cure SMA!

Shooters enjoyed a fun day on the range at the Nashville Gun Club. There was a large silent auction, raffles, prizes, and lots of activities for the whole family!

2015 Tennessee Chapter Charity Golf Outing
The Tennessee Chapter Charity Golf Outing was held on May 11, 2015 at Gettysvue Country Club in Knoxville, TN. A great time was had by all at this eleventh annual event. Golfers enjoyed a day of competition, prizes, and golfing for an important cause!

Thank you to Louise Ball, Sarah Boggess, Dorri Boggess, and the Murphey Family for all of their hard work and dedication to this event and Cure SMA. This year over $13,000 was raised!
Tennessee Chapter cont.

Cure SMA Pikeville 5K in Memory of Bentley

The 2015 Cure SMA Pikeville 5K in Memory of Bentley was held on May 30, 2015 in Pikeville, TN. Thank you to Carrie Whitaker for hosting the event again this year in memory of her son, Bentley Bassamore. Thanks to Carrie’s hard work and the support of her community, over $11,100 was raised for Cure SMA!

2015 Cure SMA Nashville Walk-n-Roll

The inaugural Cure SMA Nashville Walk-n-Roll was held on October 11, 2015 at Centennial Park in Nashville, TN. Over 100 people came out to celebrate the life of Tatum Harbin. Thank you to her parents, Gary and Kayla Harbin, for all of their hard work on planning this amazing event. The Walk-n-Roll brought together families from across the state.

Thanks to the hard work of the Harbin’s and the support of family and friends, over $8,100 was raised for Cure SMA!

Benefit Motorcycle Ride in Honor of Princess Tatum

In March 2015, Gary and Kayla Harbin hosted the Benefit Motorcycle Ride near Nashville, TN, in memory of their sweet daughter, Tatum. Thanks to their hard work and the support of family and friends, over $1,460 was raised for Cure SMA!

Gospel Concert in Memory of Alana Whited

Thank you to the Whited Family for hosting a gospel concert in memory of their daughter, Alana. The concert was held at Calvary Baptist Church in Lebanon, TN on April 25, and raised $1,350!

T-Shirt Sale in Honor of Leeanna Ivy

Thank you to Cheryl Whited, and the staff and students of Ottaway Elementary School in Greeneville, TN for raising $300 at a t-shirt fundraiser in honor of Leeanna Ivy, SMA type III.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Texas Chapter

5th Annual Texas Chapter Bowl-a-Thon
The 5th Annual Texas Chapter Bowl-a-Thon was held on August 8, 2015 at Highland Lanes in Austin, TX. The event had its biggest turn out yet with over 100 participants! In addition to bowling, the afternoon included face painting, balloon animals, SMA candle lighting, and a #SMAshSMA confetti egg activity. Through the continued support of friends, family and the community the event raised over $9,800; $62,000 since it began in 2011! A special thank you to event organizer Kelly Coggin for all of her hard work in making this year’s event a huge success.

2015 Rock ‘n’ Roll San Antonio Marathon & ½ Marathon
On December 6, 2015 Team Cure SMA participated in the 2015 Rock ‘n’ Roll San Antonio Marathon and ½ Marathon in historic, downtown San Antonio, TX. We had a great turn out for our first year with 13 runners in attendance. Together, the team raised over $8,200 for Cure SMA. Congratulations and thank you to all our runners for their hard work training and fundraising!

Kendra Gives Back Party
On August 10, 2015 Kendra Scott and Cure SMA hosted an evening of shopping, refreshments and awareness at Kendra Scott stores in Austin, Dallas, Plano, Fort Worth, and Houston, TX. During each event Kendra Scott donated 20% of their sales, raising $4,300 for Cure SMA. Thank you to Kendra Scott for partnering with Cure SMA for this event, as well as all event day volunteers and guests.

2015 Ryder Davis Posse Ride
On September 5, 2015 at 7:00 AM fourteen riders left Gruene Harley-Davison in New Braunfels, TX, on the inaugural Cure SMA Ryder Davis Posse Ride. Each rode over 1,000 miles in less than 18 hours to earn Iron Butt Association SaddleSore 1000 certificates. The event was hosted in honor of Ryder Davis, SMA type I, and raised over $3,000 to fund a treatment and cure for SMA. A special thank you to Curt Carter for organizing the ride and for all those who participated this year. The event would not have been possible without your support. Thank you!

Reno Elementary Fun Run & 5K
The Reno Elementary Fun Run & 5K took place on March 28, 2015 at Springtown Reno Elementary School in Azle, TX. With support from family and friends, the event raised over $2,700. Thank you to Mistie Rivera and the Flight Crew for hosting the event and bringing SMA awareness to Springtown ISD and the local community!

SMA Awareness Presentation at TNMP
Thank you to Amy and Sydney Horak for organizing a presentation to spread awareness of SMA at Texas-New Mexico Power Company (TNMP) on September 17, 2015. As a result of their efforts, a donation of $1,185 was made to Cure SMA of behalf of Sydney, SMA type II.

Shop for a Cure
On April 26-27, 2015 Cure SMA and Talbots partnered to host Shop and Donate events at stores located in Houston and San Antonio, TX. Customers were invited to shop, enjoy refreshments, and entered to win a tote bag filled with Cure SMA merchandise. During the events each store graciously donated 10% of all sales to Cure SMA, raising $1,150. Thank you to Talbots Town & Country Village manager Chieuanh Nguyen for organizing this opportunity to spread SMA awareness and raise funds for a treatment and cure.

SMA T-Shirt Fundraiser
For their daughters’ 10th birthday, Jenny and Brad Campbell created t-shirts to celebrate the big day and spread SMA awareness. Over 100 shirts were sold in honor of twins Tambryn, SMA type I, and Braelyn, raising more than $1,000. The girls, along with family and friends, wore the brightly colored shirts on their birthday to the Texas Bowl-a-Thon held on August 8.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Virginia Chapter

2015 Richmond Walk-n-Roll

The 2015 Richmond Walk-n-Roll was held on October 25, 2015 at Midlothian High School in Midlothian, VA. We had a great crowd for this first time event! The children and adults alike enjoyed games, balloon art and face painting while listening to great music provided by a DJ. We were fortunate in having several SMA families join the fun and participate in our walk-n-roll. We exceeded our fundraising goal to a grand total of $10,870. Looking forward to making this an annual event!

Sandra Zaun
Amelia Court House, VA

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.

Virginia Chapter Walk-n-Roll & 5K

The first annual Cure SMA Virginia Chapter Walk-n-Roll & 5K held on September 19, 2015 was a huge success! More than 150 participants, supporters and volunteers were in attendance and enjoyed a beautiful day at Occoquan Regional Park in Lorton, VA. In addition to the walk-n-roll and timed 5K race, families and friends had fun with water balloons, face-painting, and a special appearance by Elsa and Anna from Disney’s Frozen. Supporters came from all across the Commonwealth of Virginia to help raise money for a treatment and cure for SMA, with some traveling from as far as Maryland, Pennsylvania, New York, Massachusetts, Ohio and Wisconsin. In total, we exceeded our fundraising goal and raised over $16,000!

Kyle Derkowski
Communications Chair, Virginia Chapter

Virginia Chapter Family Gathering

Several families from the Virginia Chapter met at Clemyjontri Park in McLean, VA on August 8, 2015. Clemyjontri Park is designed for children of all abilities to be able to play side-by-side. It has many accessible features, including a wheelchair accessible carousel. This was the first meet up organized by the Virginia Chapter. The weather was beautiful and many families enjoyed meeting each other for the first time!

Kyle Derkowski
Communications Chair, Virginia Chapter
Western New York Chapter

2015 Western New York Chapter Walk-n-Roll
The annual Western New York Chapter Walk-n-Roll was held on August 1, 2015 at Beaver Island State Park on the Niagara River in Grand Island, NY. This year we were relocated to a bigger location which was met with much approval from all attendees. The new location offered a beautiful view of the Niagara River and was perfect for the 3 mile walk.

More than 300 people helped raise over $55,000 on a day that brought families and friends affected by SMA together. Games were enjoyed by the kids, balloon animals and hats were made, SMA merchandise was sold and many good memories made. Nick from KISS 98.5 was once again our DJ and Master of Ceremony and John Litten and his cooking crew served the hot dogs.

Our basket raffle organized by Paula Orlowski and Lori Faso was a huge success! A new system was initiated to announce the winners. The computer generated system enabled the winner to be posted in record time. We had over 200 baskets so this was quite a feat.

State Senator Robert Ortt was introduced and he talked briefly about the 21st Century Cures Act. This Bill, when passed by Senate and if signed by the President will allocate much needed money for rare diseases. Michael Kracker from Congressman Chris Collins’ office also spoke. Congressman Collins is one of the writers of the 21st Century Cures Act.

Sarah Rodriguez from the Cure SMA National Office also attended and spoke regarding new developments in research.

Much thanks and credit to all that participated and helped. Save the date for next year’s Walk-n-Roll on August 6, 2016.

2015 Binghamton Walk-n-Roll
The inaugural Cure SMA Binghamton Walk-n-Roll held on Sunday, July 26, 2015 was a huge success, raising over $4,500 for Cure SMA! Our participants enjoyed a one mile walk along the beautiful paths of the Otsiningo Park in Binghamton, NY, and also enjoyed meeting the Sweet Frogs, face painting, refreshments and the chance to connect with several SMA families throughout the area.

Special thanks to Sierra Kulas and the wonderful volunteers who made this all possible!

Wellsville Country Club Pro-Am Charity Tournament
This year’s Wellsville Country Club Pro-Am Charity Tournament raised around $4,000 for community groups in western New York. The club donated $400 to Cure SMA in honor of Alex Blair, SMA type II, who is Rich Rawleigh’s nephew. Special thanks to the Wellsville Country Club for your support of Cure SMA!

Dance Away SMA
This past April, Churchville Chili High School’s executive council put on a dance-a-thon in order to raise money for Spinal Muscular Atrophy. The high school got the idea, thanks to one of the students Joe Gutberlet, SMA type III. The Dance Away SMA, kicked off at 7:00 pm and went on for four hours with students, faculty and parents of Churchville Chili school district taking part in the event. There were four themes in which participants dressed up and played games in between. Overall, the event raised over $2,500 for SMA. Kale Shiesley and family and Bailey Walter were invited to attend the event with Joe and his family.

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
Western New York Chapter cont.

Race with Dale Earnhardt Jr.
Dale Earnhardt Jr. auctioned off the race gloves that he wore in the NASCAR races during the month of August. The gloves raised over $2,000 for a cure. On October 10, 2015, Kale Shiesley and his family were invited to a meet and greet at Charlottesville Raceway in North Carolina. Kale had a face to face with Dale Jr. along with several other race drivers.

Pig Roast Fundraiser
The 5th Annual Roast SMA was held on June 13, 2015. Alex, Charli, Molly and Kale enjoyed the beautiful day. Fishing, games, good food, swimming and great fun was enjoyed by all. The pig and refreshments were again donated by Uncle Brad and Aunt Peg who hosted the event. A total of $1,460 was donated by friends and family.

ALEX AND ANI Fundraiser
On October 7, 2015 from 6:00 pm – 8:00 pm, the Western New York Chapter held their first ALEX AND ANI Fundraiser in support of Cure SMA. They raised $650 from sales during the evening as well as $235 from the raffle totaling $905 to help fund a treatment and cure for SMA. Thank you to all of the families and friends who shared their support and enjoyed an evening of shopping, refreshments, small bites and a passion for giving back to Cure SMA.

Special thanks to the Western NY Chapter for hosting this wonderful event!

Exchange Club Donation
The Erie Niagara Sunshine Exchange Club presented Karen Shiesley with a check a $500 check for Cure SMA. Thank you!

Cousins Fundraiser
This summer, Kale Shiesley’s cousins were visiting Granny’s and decided to go for a walk. They grabbed a bucket and some SMA literature and collected $247 in about an hour. Thank you for your generous donation!

21st Century Cures Act
On August 5, 2015, Congressman Chris Collins held a press conference to announce and explain the 21st Century Cures Act. This Act will delegate money to orphan diseases like SMA. It passed the House by an overwhelming majority and is waiting approval by the Senate. It is now known as the Innovation Act in the Senate. Alex Blair, SMA type I, and Kale Shiesley, SMA type II, and families were invited to attend the press conference.

Peace Bridge Lighting
The Peace Bridge, which connects the US and Canada near Niagara Falls, was lit purple in honor of SMA on August 14, 2015. The lighting was arranged by Bailey Walter, Kale Shiesley’s close friend. Next year, Niagara Falls will be lit purple on Kale’s birthday August 16, 2015. This was also arranged by Bailey
Wisconsin Chapter

2nd Annual Strike Out SMA

The 2nd Annual Strike Out SMA bowl-a-thon was held on May 16, 2015 in Wauwatosa, WI. The event was a huge success, raising over $13,400 and bringing together family and friends to bowl, play together, and raise awareness for SMA! Raffle items were graciously donated from all over Wisconsin, ranging from items from individual donors to the Green Bay Packers. Participants won awards for “High Score” and “Most Gutter Balls”! Our event has grown so quickly in just two years that we will relocate to a larger alley space next year and we cannot wait to fill the space with our families and friends to celebrate another year of coming together for a great cause! We look forward to seeing everyone at the 3rd Annual Strike Out SMA on Saturday, May 14, 2016!

Cure SMA would like to give special thanks to Danyelle Sun, Kate Vogedes and Shannon Kuester for all of their hard work planning the event and recruiting bowlers!

Brady Walk

For 5½ years, I've been an employee at Brady Corporation in Milwaukee, WI, and have participated in the Brady Walk, an annual employee event to support our community. This year, employees were given the opportunity to vote for the organizations they wanted to support during the walk. How the event works is that for each employee who signs up to walk 5 miles, Brady donates $70 toward their chosen organization. The company also hosts a food drive around the walk for the Hunger Task Force, a local Milwaukee food bank and anti-hunger advocate. For each food item contributed by employees, Brady donates $2 towards the charity that you are walking for.

Cure SMA was chosen as one of the 4 organizations that the employees wanted to support during the Brady Walk. With the help of many of my co-workers, we were able to raise $11,222 for Cure SMA. I was floored when I saw that amount written on the check. Many emotions came over me – so much pride and joy because it was all for my 3-year-old niece, Elise. She was diagnosed with Type I in February 2013 and has shown so much strength, courage and love through all of this. She is my inspiration for everything. I am so appreciative of everyone’s help to donate to Cure SMA, this organization has been so gracious to our family and I’m thrilled to have been able to give back to them and help find a cure for SMA!

Sarah Drake
Milwaukee, WI

Cure SMA would like to thank Sarah Drake for nominating Cure SMA to become one of the chosen charity partners for her company walk and for spreading awareness to her fellow employees about SMA!

5th Annual Kennady’s Dream Walk-n-Roll

The 5th Annual Kennady’s Dream Walk-n-Roll was held on September 13, 2015 in Brookfield, WI. Over 100 people gathered on a beautiful day at Fox Brook Park for a day of family fun! The day included a walk, DJ, lunch and raffle. The walk was a big success, raising over $12,300 for Cure SMA, in memory of Kennady Quinnell, SMA type I.

Everyone at Cure SMA would like to thank Erin and Corey Quinnell for all of the time and dedication they put into organizing this wonderful event each year!

Note: The amounts raised and shown are totals as of December 1, 2015 and may differ from current fundraising totals by the time you get this newsletter.
11th Annual Grant Sheppard Memorial Scramble for SMA

On Friday, August 21, 2015 the 11th Annual Grant Sheppard Memorial Scramble for SMA was once again held at Hickory Hills Country Club in Chilton, WI. We are proud to be sending you the proceeds that may be used to suit the needs of the organization. A total of $8,000 was raised during this golf outing in memory of Grant.

We would like to thank Allie Ziolko for taking the time and effort to join us and share updates with all of our contributing golfers and friends. Another big thank you to Shannon O’Brien for all her behind the scenes help with the set-up. They are both great representatives for the organization and the cause!

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

Scott, Lisa, Peter and Lily Sheppard
Sherwood, WI

Cure SMA would like to send a special thanks to Scott and Lisa Sheppard for all their hard work in planning this incredible event year after year!

Annual Hoffman Family Block Party

On Saturday, September 12, 2015, the Annual Hoffman Family Block Party and Raffle raised $591 for Cure SMA! 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. Thanks to Paula Hoffmann for organizing this event each year and for your support!

Tori Stevens Memorial Race

Tori Stevens Memorial Race was held in honor of one of racings biggest fans. Tori had spinal muscular atrophy type II and passed away at age 11. No matter how sick she was she never let it get her down; she was always up front fence side to cheer on her favorite stock car drivers with a huge smile. We had over 40 bikes and 30 backpacks, scooters and even kids’ camo recliners for children’s drawings all in Tori’s honor, also some adult prizes. A huge thank you to Hedges Racing Family and Team for their efforts! Everyone went home happy that night!! Can't wait for next year!

Schenck Jeans Day Fundraiser

Schenck, a regional CPA FIRM, has collected a $132 donation for Cure SMA organization after a recent fundraiser in its Green Bay office. They dedicated a day where employees could wear jeans for a $3 minimum donation, and our employees selected your organization as the recipient. On behalf of Schenck and all our shareholders and employees, we commend you for your work and wish your continued success.
For the love of Pete: Challenging what it means to live with SMA

Pete and his twin sister, Lucy, were born 11 years ago into our very busy family. We already had two boys, Ian and Chris, and had learned to expect kids to develop in a certain way. But by the time Pete was a few months old he moved really slowly, just his upper body, and looked really uncomfortable.

At first I was curious and just kind of Hmmmm. But when he was six months old I decided to bring it up with his pediatrician. The doctor kind of waved me away, wrote a prescription for physical therapy, and told me he was fine.

Pete wasn’t fine and neither was I. It’s miserable to suspect something is wrong with your child and not know what it is. You can’t rally around the problem, you can’t fundraise, you can’t embrace anything.

“We reacted differently. My husband lay on the floor. I called people.”

Pete lost his ability to roll over a few months later and the physical therapist recommended we see a neurologist. The doctor tested our son, who was nine months old by then, and asked us to come to her office.

I wish I could tell you otherwise, she told us. But it’s most likely spinal muscular atrophy. These babies don’t usually live past their second birthday.

Spinal muscular atrophy, or SMA, is a genetic disease that affects the motor nerve cells in the spinal cord and eventually takes away the ability to walk, eat, and breathe. It’s the number one genetic cause of death for babies.

My husband, Tim, and I reacted to the news very differently. He lay down on the floor and couldn’t move. I immediately started calling people.

“He’ll be fine. As long as he can play he’ll be happy.”

All these years later I still remember how people reacted – or maybe I only remember the comments that were helpful.

We’ll put stickers on his wheelchair, my neighbor told me. I know a child with SMA who is nine years old, another friend said. He’s going to be fine. As long as he can play he’ll be happy. It was such a relief to hear that last response, which came from the genetic counselor we met with. What she said has shaped the way we’ve raised Pete.

We've put stickers on his wheelchair and taken him parasailing, skiing, and snorkeling. He's traveled to Costa Rica and Bonaire and this year we went to Italy. He's about to enter sixth grade and has his own group of friends. He and his sister, Lucy, regularly go to the neighborhood pizzeria on their own, without me. Pete gets to enjoy many of the same privileges of being a kid as his sister and brothers do.

Recently it’s become harder for him to keep up. Pete’s having more trouble using the joystick on his power wheelchair and to bring him to Italy we had to carry an oxygen concentrator, which helps him breathe more easily, along with a ramp.

Coming up with accommodations is sometimes a headache. But we want him to live while he’s here. I hope he has a good long life but you don’t know.

“People feel good when they embrace a kid like Pete.”

I’m involved with the Philadelphia chapter of Cure SMA and we encourage anyone who’s planning to have a child to get carrier screening so they can make the right choice for their family. I know there are people who will decide not to have a child. But getting the information early also gives you time to do the research and prepare to live with a child with SMA. When I talk to people who have just gotten a diagnosis I try to give them as much hope as possible.

Having a child like Peter is humbling but I’ve learned more from being his mom than from anything else in my 46 years. So many people have contributed to his happiness. And he has given back. People feel good when they embrace a kid like Pete so his story is a happy one. Even if Pete’s life is short it has served a huge purpose.
SHARING

Strengthening the Muscle of Hope

By: Aaron “Ari” Anderson

If you’re connected to spinal muscular atrophy, you don’t have to dream of being an athlete, you are one! You all do heavy lifting, whether it is with physical or mental weights. Even though SMA has an unfair advantage by making your physical muscles weak, you can even out the playing field by strengthening your “Muscle of Hope.” This muscle can be stronger than all other muscles combined.

By sharing my unique patient perspective, I hope to bring a voice to those living with this disease as well as those who have passed away. Both are true champions in the SMA Hall of Fame, and I want to honor them, for some are gone but never forgotten. I hope my story helps to lift your burden!

My life literally began without a playbook. In 1982, there were a few individuals with SMA type II and III who had made it to adulthood, but this was hardly the case for SMA type I. In fact to this day, 90% die by the age of two. Therefore, when my parents even dared to have hope for me, when I was diagnosed with Type I at six months old…well, it had hardly been done before.

The next few years would prove how totally outmatched I was against this opponent. First, I was in ICU almost the first four years of my life from constant pneumonia. Nightly, for months on end, SMA scored a knockout punch against me every time I went into respiratory arrest. Daily, I received a body slam from vomiting food into my lungs causing even more respiratory infections. Although I was very young, the extreme nature of these events guaranteed without a doubt that I remember many of them well.

By the age of two, I received a tracheostomy and was attached to a ventilator. There was something else that had become attached to me as well though – people. People noticed that I never lost my love of life. I was intrigued by the water in those colorful balloons I played with. I was enthralled by the music of my favorite Disney movies. More importantly, after each traumatic episode, I went right back to my mischievous nature by teasing my sister when she came to the hospital or playing jokes on the nurses. Plus, my mom decided early on to “treat me as a living child, instead of a dying child.” For example, she continued to teach me things like the alphabet despite my grim prognosis.

How bleak was my prognosis? I remember one respiratory code that lasted 30 minutes, and hearing a nurse yell, “Ari, you’re scaring me!” The nurses fought back and won, even as I progressed into cardiac arrest. The nurses didn’t know that I would survive the day, much less make it to adulthood. Yet, they had a conviction that even if I didn’t survive, there was some purpose to what I was going through. Such blind faith combined with strong devotion from people who support you, can be a powerful force to get you through the dark times.

Eventually, through the use of a standing brace, the pneumonia was able to drain from my lungs. After several years of being in ICU, I was well enough to come home! Now what?

In 1986, again there was no playbook for someone with type I SMA. Since I had already outlived my life expectancy by age four, some would have said, “Just let him live a carefree life without any life-goals.” However, life is not all rainbows and roses. My mom was not going to treat me differently than my healthy sister. Even though I still had a trach, vent, and numerous other complications, by faith, I would go to traditional school and eventually college.

Many people taught me that constantly setting goals was a way to not let the muscles of hope atrophy. Once you are strong enough to lift the dead weight of hopelessness, you begin to have hope that SMA does not control your entire life. It would have been easy to just focus on preventing health crises and maintaining the status quo as long as I could. That is not how I wanted to live my life. I won’t lie. It was risky sending me to public school, (The Jungle of Germs). Precautions had to be taken. First, nurses at home and school took great care of me. Second, nurses used subtle ways to meet my medical needs, while nobody in class noticed.

When I became an adult, I thought that I had faced almost every situation life could throw at me. So far, I had beaten the odds of a deadly disease and I was active in the community. Although this was an achievement, I didn’t realize that I had
been living in an academic bubble (The Minor League). Now, unexpectedly, I needed to enter the world of political advocacy (The Major League)! In the second grade, we moved to a state where I could receive the best of nursing and physical therapy at home. As long as we stayed in this state that had superb Medicaid programs, nobody disputed my need to have these services. When I became eighteen, I found out that the world was uneducated about what it took for adults to live at home with a tracheostomy and ventilator. Just as I had to prove to my teachers that I could do my school work despite my medical challenges, I had to prove to legislators responsible for State Medicaid funding, that I could contribute to society.

Educating policy makers is a tremendous undertaking. I visit the state legislature and Medicaid many times a year. Each time I go, I travel the six hour round-trip from my house to the state capital in one day. Such draining repetition is important, because it is critical for lawmakers to remember a face instead of a blank space, when funding for life-saving programs is up for debate.

During these visits, I bring eye-popping presentations that I create myself with technology I control with my eyes! Life is breathed into these presentations when I program movement and sound effects into graphics that show what I go through daily. I frequently write to legislators and Medicaid officials. When I write, I am honest about what I need to survive. However, instead of criticism, I thank them for the hard work they do. Policy makers, under pressure, are given hope by this. I know because they write me back personally. This in return gives me hope. Such shared encouragement makes everyone equal and has taught me that policy makers are never on the opposing team.

Some people ask, “Why do you spend such long hours doing legislative work and travel so far? First, lawmakers must remember a face, when they debate the future of programs. Next, if I didn’t do all the work I do, I wouldn’t have the home care services required to do anything. I couldn’t survive in a medical facility, because I need one-on-one care to clear my lungs hourly. Also, my mom is a single parent and can’t come home to take care of me after having worked 12 hours. Each of my legislative trips is like a championship tournament, only it’s not “Win or Go Home.” I must “Win to Go Home” and survive by keeping my services. This is why I fight so hard not to lose.

Do I worry about my future place in society? During the years 2001-2003, I was scared. I was new to political advocacy. It was difficult emotionally for me to adjust to this vastly different lifestyle. Matters were more stressful because Private Duty Nursing (PDN), the nursing program I use, came indescribably close to being severely cut for adults. This was in the middle of my college years. I had always faced the challenge of getting teachers and students to see me through the lens of normalcy. During these years, my professors and classmates especially had the habit of seeing me through the lens of disability. I was facing many hardships at once.

continued on next page
I entered a state of grief. To me, life no longer held unending possibilities. For a few years, I was afraid to hope for good things. My “Muscle of Hope” had been sprained. If life or people have hurt you, it’s okay if you need to back down from certain aspects of living for a while. For me, it was a very long time before I could really trust anyone again. In other areas, I never backed down. I graduated from college in 2005 with a BS Degree in Biology, earned a Master’s Certificate in Technical Writing, and continued to work successfully with lawmakers to protect Medicaid programs for adults with chronic conditions. Despite sorrow, LIFE MUST GO ON.

It was faith that got me to be strong in hope again. God’s grace gave me the reassurance that it was ok to look forward to a brighter future. My way of looking at life is described by Dr. Danielle Sheypuk. Dr. Sheypuk is a Clinical Psychologist who has SMA type II. In Quest MDA Magazine, she says that when her clients are gloomy about the future, she tells them that such thoughts are just predictions, not unalterable facts. Sheypuk adds that along with negative events “positives are coming too. And some of these positives may surprise you.” In my own words, I say that although you were blind-sided when your best laid plans were ruined – feel great joy when your worst plans or predictions turn successful! Every stage of my life was predicted falsely. Here is how I look at the past now:

• I am thankful that I got to experience getting up before the crack of dawn like other high school and college students. I am thankful to know what it was like to cram for final exams.

• I am thankful that despite the long (and at times frustrating) hours I spend on advocacy, I get to help others in my situation, as opposed to life just being about others helping me.

Finally, let’s work together as a group of muscles in one spirit. You don’t have to be an expert with words or advocacy to help. Within you lies an even greater asset, the truth, and that is more effective than anything else! The community needs to see that we can still smile even when hooked up to tubes. If you’re loved one has passed bring pictures or videos to show lawmakers that their life did have joy and meaning. The more society sees our truth, the better the future will be for those with SMA. Plus, your tears will not go in vain, and no matter what, we will never fade into darkness.

Western New York Teen Wins Nickelodeon Halo Award

Fourteen-year-old Bailey Walter was selected from among 20,000 entrants to receive the August Nickelodeon Halo Effect Award. This award honors teens from around the country for their charitable work.

Bailey received the award for the “Let Them Dance” campaign, which she started in honor of her best friend, Kale Shiesley, who has SMA. The name was inspired by Bailey’s hope to dance with Kale at one of their school dances.

Bailey and Kale are an active part of the Cure SMA Western New York Chapter. Along with the other participants, they raised almost $60,000 through the Western New York Chapter Walk-n-Roll in August. Bailey also arranged to have Niagara Falls lit in purple in recognition of SMA Awareness Month.


Bailey and Kale were featured by The Buffalo News, The Niagara Gazette, and WKBW-TV in Buffalo. Bailey also filmed a commercial for Nickelodeon about SMA, and will donate her $5,000 prize to help find a cure for SMA.

Thank you to Bailey and Kale for all their hard work and dedication!
Sixth-grader at Caprock creates lip balm from beeswax, will compete in regional competition

By Amy Hamilton

Eleven-year-old Madelyn Swelstad of Grand Junction, CO gives a brief pitch of her business, Mad Lips, a lip balm from beeswax produced by her own bees, during the Young Entrepreneurs Academy (YEA!) graduation Thursday at Allen Unique Autos. Nine participants graduated from the Chamber of Commerce’s educational program that takes students in grades six through 12. Madelyn has been named the program’s Saunders Scholar, and she will compete in regional competition in Phoenix for a chance at up to $50,000 in scholarships.

Madelyn Swelstad is onto something.
She created a lip balm that actually works, naming her product Mad Lips.

Madelyn uses beeswax and other organic, natural ingredients to create the tubes at the Grand Junction Business Incubator Center.

But Madelyn isn’t your typical businesswoman. She’s an 11-year-old sixth-grader at Caprock Academy.

After her graduation alongside eight of her peers from the Young Entrepreneurs Academy or YEA! on Thursday night, she was headed to a middle school dance.

“I have to go home and change so I might be a couple minutes late. Fashionably late,” she said and laughed.

Madelyn is the youngest of this year’s graduating class of YEA! It’s a program through the Grand Junction Area Chamber of Commerce, which pairs business leaders with middle and high school students to create a viable business over the course of a school year. Students develop ideas, write business plans, obtain funding, pitch their ideas to investors, gain funding and register with government agencies.

Madelyn will go on to compete in a regional competition in Phoenix. If she is selected from that round and wins a future national competition in Washington, D.C., she’ll earn a $50,000 college scholarship. The prize also includes the opportunity to pitch her product with officials from Walmart and Sam’s Club and an appearance on the reality television show “Shark Tank.”

“I’d be scared to death to go on ‘Shark Tank,’ ” she said. “It’d be terrifying.”

Madelyn said being in YEA! has helped her gain confidence. She’s talked in front of a crowd of nearly 500 people.

Madelyn uses beeswax from the beehives her parents keep. If Mad Lips becomes mass-produced, she said she’ll have to find a larger source of beeswax.

Madelyn Swelstad said she was drawn to making lip balm because she loves beauty products and crafts. She has a theory that most lip balm is created to make lips drier, so people keep using the balm.

“It works for about a half-hour,” she said. “Then you have to put on more. You have to keep using it to make it feel better.”

While she’s years away from college, Madelyn said she’d like to attend “one of the big name ones.”

She loves to debate, and so she thinks becoming a lawyer might be in her future.

“For a long time that’s been something I thought I might like to do,” she said.

Madelyn Swelstad, (SMA type III), is a student in the Young Entrepreneur Academy, an extracurricular program through the Chamber of Commerce in Grand Junction, Colorado. This young entrepreneur presented her business plan and asked for startup funding in front of a panel of 14 investors in March (including the city mayor). At age 11, she is this year’s youngest Saunders Scholar recipient in her area. Using beeswax from her family’s bees, she has created a line of lip balms with interesting flavors such as mint mojito, lemon pound cake and mocha that she markets through her company, Mad Lips.

She recently returned from Phoenix after competing in the semi-finals. Madelyn is currently enrolled in an on-going ISIS clinical trial in Salt Lake City receiving intrathecal injections twice a year. Due to increased strength, her parents have had to move the basketball hoop up three feet. The Swelstad family wishes to thank Cure SMA for their involvement in advancing the science toward a cure.
Publications From Cure SMA

Nutrition Basics
Fostering health and growth for spinal muscular atrophy.

The topics in this 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 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.

Questions covered include:

- What is a gene?
- How does the gene make protein?
- How is SMA inherited?
- What is a carrier?
- What genetic tests are available to identify those with SMA or those who are carriers?
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 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)

This booklet covers the basics of SMA, including:

- Genetics
- Diagnosis
- Types of SMA
- Daily life with SMA
- And more.

And where you can go for support and guidance.

Musculoskeletal System

The goal of this booklet is to help children, families, and other professionals including nurses, physicians, and therapists understand how spinal muscular atrophy (SMA) can affect the body's musculoskeletal system.

Topics include:

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

And where you can go for support and guidance.

For electronic copies:
Download this booklet from the Cure SMA website 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.
Matthew Varney
Nicholas Knowlton
Payne
Victoria & Ella Koblentz
Victoria Koblentz
Sebastian Torres
Matthew Varney
Sebastian Torres
Ian Zurawski
Tony Hyungyu Lee
Save the date!
Thursday, June 16th - Sunday, June 19th, 2016
2016 Annual SMA Conference