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*On the Cover: Ashwini (mom) and Reanshi Shah, Type I*
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 1988. Every year we look forward to reuniting as a community at this conference and showing our support for others. As always, The Conference and Researcher Meeting run alongside each other. This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it. The interactions between the researchers and families at this one conference are extremely special. The annual conference also provides the children an opportunity to make new friends and have a great time. 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 and greet & family fun fest, teen and adult social activities, a dance party and many opportunities to connect and interact with families and receive first hand updates from the researchers.

We are anticipating another great conference, with over 1,400 expected attendees.

The Hilton Anatole in Dallas, Texas has been carefully selected to meet the needs of the SMA Community for the 2018 Annual SMA Conference.

You must complete your conference registration with Cure SMA prior to reserving your room at the Hilton Anatole for the special room rate of $199.00 per night plus tax.

The Hilton Anatole is an elegant hotel with exceptional dining, an art collection of over 1,000 pieces and the JadeWaters resort pool complex which includes a leisure pool, beach entry family pool, lazy river, cabanas and a splash zone with two waterslides. If you are looking to explore the city, the hotel’s spectacular location places you just minutes from local Dallas sports venues, restaurants, museums, art galleries and vibrant neighborhoods.

In the evening, relax with any of the numerous spa services at the V Spa located on the 2nd Floor at The Verandah Club, or visit the JadeWaters pool complex for Friday and Saturday family movie nights.

Guest rooms are non-smoking and offer some great features like a mini refrigerator, coffee station, room safe and free WIFI service.

The Hilton Anatole also offers a sophisticated, urban dining experience with restaurants such as SER Steak & Spirits and Media Bar + Grill, as well as the trendy Gossip Bar. Enjoy daily breakfast with an open-air café ambiance at Terrace, or grab a quick bite at Counter Offer, the 24-hour grab-and-go deli.
SMA CONFERENCE GOALS

- To welcome newly diagnosed families into the SMA community.
- To help build an SMA community and help keep that community strong and unified.
- To educate SMA families and provide updates on medical issues, research and clinical trials.
- To allow networking and data sharing between researchers, families and patients.
- To educate researchers on the latest research advancements.
- To attract the best researchers to the SMA field and encourage collaborations and investments.
- To promote cross-disciplinary dialogue among academic, clinical and industrial researchers.

NEWLY DIAGNOSED CONFERENCE PROGRAM

Thanks to generous funding provided by the Erin Trainor Memorial Fund, Cure SMA will cover 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 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 SMA families and researchers.

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

Please visit www.curesma.org/get-involved/conference for more conference information.
Thank you to everyone who attended the 2017 Annual SMA Conference at Disney’s Contemporary Resort in Orlando, FL; it was truly a magical weekend of community, research and fun!!

We are happy to announce that, including on-site registration, there were around 2,100 attendees in total – our largest conference ever! It was such an honor to have SMA families, clinicians and researchers attend the conference from 30 countries across the globe.

For those who were not able to attend, check out our Facebook page to watch the Opening General Session and a few of the Family Friendly Poster Session presenters. Conference photos will also be available soon.

CONFERENCE OVERVIEW

The Conference began on Thursday with the Newly Diagnosed Program for SMA families recently diagnosed. This special session is an orientation that helps introduce new families to the conference.

Thursday evening’s Meet & Greet is a wonderful tradition at the Annual SMA Conference and welcomed new families, returning families, clinicians and researchers. It was a great opportunity for all attendees to join together in a fun and relaxed setting before the start of conference workshops.

The Meet & Greet features 30 interactive games that kids and families are encouraged to rotate around to activities like Bozo Buckets, Treasure Chest, Lollypop Tree and many more. The games were adapted for all children to enjoy and win great prizes. Candy and tattoo stations filled with goodies and fun for all were additional Meet & Greet favorites. There was even an opportunity to get your picture taken with the iconic Disney Characters, Mickey and Minnie Mouse.

The Relay Race is also a highlight of the conference, as both SMA researchers and kids line up to see who can cross the finish line first, with one rule: researchers have to race in a manual wheelchair. Conference attendees stand on the edge of the course cheering on the racers. Children always cross the finish line first, leaving the researchers far behind!

TEEN SOCIAL AND ADULTS WITH SMA RECEPTION

Thursday evening’s Teen Social, sponsored by AveXis and the Jacob Isaac Rappoport Foundation, was an invaluable opportunity for SMA teens and siblings to connect with one another and share their experiences.

Similarly, the Adults with SMA social, sponsored by the Dhont Family Foundation, brought SMA adults together in a fun, social environment where cocktails were enjoyed.
and good times were had by all. A special thanks to the Dhont Family Foundation for providing scholarships to all SMA adults attending the conference.

**WORKSHOPS**
Friday and Saturday offered families informative workshops and sessions. There were over 50 workshops during the conference, covering the latest information on critical care and support including Yoga Therapy for Adults and Teens with SMA, Healing the Grieving Heart – Part I, Family Readiness for Emergencies, and Writing Our Stories: A Workshop for Parents. Presentations from some of the workshops and sessions offered are available on the Cure SMA website.

**SYMPOSIUMS** SPONSORED BY AVEXIS AND BIOGEN
Two of the leading companies in SMA research, AveXis and Biogen, sponsored family breakfast and lunch symposiums. This allowed families to ask important questions and connect with the companies in a relaxed and open setting.

**CHILDREN’S PROGRAM**
This year’s Children’s Program was extraordinary and another huge success! Thanks to our incredible volunteers, all of the children had a fabulous time. Children stayed busy with countless arts and crafts projects, fun toys, entertainment, movies, video games, build a bear stations, and much more.

**RESEARCHER Q & A**
The Researcher Q & A Session, held on Saturday afternoon, allowed families to hear directly from SMA researchers about the latest research developments. Leading experts in SMA answered families’ questions. A video from this informative session will be available on the Cure SMA website in the coming weeks.

**IT’S A WONDERFUL LIFE**
The conference concluded with the special session, It’s a Wonderful Life. A panel of SMA adults took the stage to answer questions from the audience about their experiences, and shared encouraging messages of perseverance, joy, hardships and growth.

**UPDATES FROM THE CONFERENCE**
As part of the Annual SMA Conference, we announced the launch of a new grassroots advocacy campaign to implement newborn screening for SMA in all 50 states. Missouri State Representative Becky Ruth, sponsor of Missouri’s first-in-the-nation law instituting newborn screening for SMA, was the featured speaker at the newborn screening symposium, which marked the launch of this campaign. A recording of this symposium, online advocacy tools for connecting with elected officials, and a guide to setting up an SMA Awareness Month advocacy visit will be available in the coming weeks.

An updated SMA drug pipeline was also released during conference. The update includes 19 active programs, including one approved therapy, 14 pharmaceutical partners and 5 programs in clinical trials.

Lastly, at the Sunday morning closing session, we announced the location and dates for our 2018 Annual SMA Conference. Mark your calendars for June 14 – 17, 2018, as we make our way south to the Hilton Anatole Hotel in Dallas, Texas!

**THANK YOU**
The Annual SMA Conference would never be possible without the assistance of our fantastic volunteers. Whether setting up decor, working in the Children’s Program, preparing and running games at the Meet & Greet, inflating hundreds of balloons, moving boxes, or any of the other countless jobs, our volunteers got the job done and were absolutely amazing.

We extend our thanks to the sponsors and exhibitors who generously supported the 2017 conference. A special thanks to Biogen for their generosity as the presenting sponsor of this year’s conference.

**SEE YOU IN DALLAS!**
Meet and Greet
Researcher Relay Race
SPONSORED BY THE JACOB ISAAC RAPPOPORT FOUNDATION AND AVEXIS
Adult Social

SPONSORED BY THE DHONT FOUNDATION
Workshops
The Family Friendly Researcher Poster Session

The Family Friendly Researcher Poster Session brought together families and researchers, and encouraged families to move around to the different posters to ask questions and learn about the specific projects the researchers were presenting.

At the 2017 Annual SMA Conference, 30 presenters, representing different SMA research projects, were showcased. All of the clinical stage drug programs for SMA were included among the presenters, plus a variety of basic research projects and clinical care research projects.

Family Friendly Researcher Poster Session Posters Included:

Utz Fischer, PhD and Oliver Gruss, PhD from the University of Wuerzburg, Germany and the University of Bonn, Germany on “Regulation of SMN by post-translational modifications”.

Christine Beattie, PhD and Hao Le, PhD from Ohio State University on “The effect of low SMN on motoneuron development”.

Antoine Clery, PhD and Frederic H.T. Allain, PhD form ETH Zurich on “Splicing regulation in SMA”.

Marie-Therese Khairallah, PhD and Chris Lorson PhD from University of Missouri on “Dr. StrangeCell or: How I learned to stop worrying and love the astrocyte”

Marc-Olivier Deguise and Rashmi Kothary PhD from Ottawa Hospital Research Institute on “Is the immune system affected in SMA patients?”

Stacy Rudnicki, MD from Cytokinetics on “Clinical Trial Update of CY 5021: CK-2127107, an activator of skeletal muscle, for the potential treatment of Spinal Muscular Atrophy”.

Vanessa Christie-Brown from SMA Europe on “Disease impact on general well-being and therapeutic expectations of European Type II and Type III spinal muscular atrophy patients”.

Richard Finkel, MD and Kelly Wydronkowski from Nemours Children’s Hospital on “Implementation of SOC guidelines at Nemours”.

Tariq Rahman, PhD from Nemours/A.I. DuPont Hospital for Children on “WREX exoskeleton for children with SMA”.

Andreanne Didillon, Fatima Mostefai, Amir Haghandish from University of Ottawa on “SMN and friends: What we can learn from SMN interactors?”
Hikari Tanaka, Nikki McCormack, Mahlet Abera PhD, and Barrington Burnett PhD from the Uniformed Services University of the Health Sciences and The F. Edward Hébert School of Medicine on “Uncovering ways to slow SMN destruction”.

Lisa Belter, Jill Jarecki PhD, Kenneth Hobby, Cynthia Jones PhD, Suzanne Cook PhD, and Sandra Reyna MD from Cure SMA on “Cure SMA membership: Findings from the 2017 membership survey”.

Jackie Glascock PhD, Jill Jarecki PhD, Megan Lenz, and Kenneth Hobby on “Cure SMA newborn screening for spinal muscular atrophy: Federal and state implementation”.

John Jae Hong Park from Johns Hopkins University School of Medicine on “SMN expression is developmentally regulated in human spinal cord and muscle”.

Remy Bordonne, PhD from IGMM-CNRS on “Identification of the protective mechanism of a SMN modifier gene using S. pombe as model organism”.

Geoffrey Laff, PhD from Spotlight Innovation Inc. on “STL-182, an orally-available small molecule that stabilizes SMN protein”.

AveXis, Inc. on “AveXis: Committed to the development of therapies for patients affected by rare and life-threatening neurological diseases”.

Sangeeta Jethwa MD, Karl Yen , Ksenija Gorni MD PhD, Anne Marquet PhD, Tim Seabrook PhD, Jeppe Buchbjerger, Bertrand Verwee, Michael Ostland PhD, Parul Houston MD, Paulo Fontoura MD, John Baird PhD, Karen Chen PhD, Mary-Frances Harmon, Nikolai Naryshkin PhD, Sergey Paushkin PhD, Omar Khwaja MD from F.Hoffmann-La Roche Ltd on “Roche SMA drug program for SMA”.

Arnab Chatterjee, PhD from Calibr on “Innovative screening approaches to ID the next generation of SMN-inducing compounds”.

Arthur Burghes, PhD from The Ohio State University on “Sequence changes effecting severity of SMA and development of combined treatments”.

T Seabrook PhD, K Gelblin, K Gorni MD PhD, T Wiese MD, A Marquet PhD, C Czech PhD, D Kraus, D Trundell PhD, B Verwee, I Schwerszen MD, K Rucinski, N Gusset, S Braun PhD, J Baird PhD, K Chen PhD, M Harmon, N Naryshkin PhD, S Paushkin PhD, S Jethwa MD, from F.Hoffmann-La Roche Ltd on “Patient partnership in SMA”.

Jean Giacomotto, PhD from The Queensland Brain Institute on “Innovative genetic approach to recapitulate and study SMA in the zebrafish”.

Lawrence Charnas, MD, PhD and Emilie Voltz, PhD from Novartis on “Safety and efficacy findings in the first-in-human trial of the oral splice modulator branaplam in Type 1 spinal muscular atrophy (SMA) after two years”.

Yongchao Ma PhD from Northwestern University / Lurie Children’s Hospital of Chicago on “Targeting mitochondria, the powerhouse in motor neurons, for treating SMA”.

Chad Heatwole, MD from University of Rochester on “Gathering your opinion on treatment effectiveness: The creation and use of the SMA-Health Index”.

Kimberly Long, PhD, Micah Webster, PhD, Adriana Donovan PhD, Karen S. Chen PhD, Kelly Howell PhD, Sergey PaushkinPhD Ramzi Khairallah PhD, Karen O’Shea PhD, Nagesh Mahanthappa PhD, and Alan Buckler PhD from Scholar Rock on “Scholar Rock’s novel approach to strengthening muscle in SMA patients”.

Christine DiDonato, PhD, Kristin Kroschell, PhD, Nancy Kuntz MD, and Vamshi Rao MD from Anne & Robert H. Lurie Children’s Hospital/ Northwestern University. “The Lurie Children’s Hospital experience: Spiranza dosing across the age spectrum, quantitative assessment of function and efforts towards molecular markers and new therapies”.

Jacqueline Montes, PT, EdD, NCS and Sally Dunaway Young, PT, DPT from Pediatric Neuromuscular Clinical Research Network (PNCRN) on “Clinical Observations of Fatigue & Endurance in Spinal Muscular Atrophy”.

Stephen J Kolb, MD, PhD from The Ohio State University, Wexner Medical Center on “NeuroNEXT SMA Biomarker Study. Will present the natural history study and emphasize the importance of these types of studies when placebo arms in trails are not ethical”.

Our thanks to Biogen for their generosity as the presenting sponsor of the 2017 Annual SMA Conference
Cure SMA Evening in the Magic Kingdom

SPONSORED BY BIOGEN
Type I Social

SPONSORED BY THE JACOB ISAAC RAPPOPORT FOUNDATION AND AVEXIS
Jennifer Miller Smith, Aaron Smith and Family – The Smith family has put much time and effort to make conference an unforgettable experience for everyone involved! They’ve given their time, resources, contacts and ideas, all out of their support and desire to see this conference be as successful as possible. They jump at the chance to assist us in any way they can. We are so incredibly grateful to have such an amazing family behind us every step of the way. The conference keeps growing and reaching new heights and we would like to thank Jennifer, Aaron, Katie, Zach, Madison and the rest of their family for helping us bring the SMA community together once a year, during this special weekend.

We would also like to thank everyone at Expo CCI, especially Richard Curran, who sponsor and donate all of the amazing signs and banners throughout the meeting space. The professionalism that Expo brings to our conference through their impressive signs and banners, that decorate the entire hotel, is absolutely outstanding! We cannot thank each and every one of you enough for volunteering your services and expertise to make this conference a wonderful experience!
The Annual SMA Researcher Meeting is the largest research meeting in the world specifically focused on SMA. This year we had a record-setting 470 attendees. The goal of the meeting is to create open communication of early, unpublished data, accelerating the pace of research. The meeting also furthers research by building collaborations—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.

The 2017 Researcher Meeting was a huge success; we would like to thank all of our attendees for making this year so memorable!

Special Session on Fatigue and Muscle Weakness in SMA

The SMA field has made major therapeutic advances with SMN enhancing approaches. The greatest impact from this therapeutic approach will likely require early treatment. Therefore, the goal of this session was to discuss novel points of intervention that could result in greater motor and muscle function at more advanced stages of SMA. This session also discussed fatigue and muscle weakness from a clinical and biological perspective, breaking down the potential role of the different components of the motor unit.

This session was moderated by Thomas Crawford, MD, Professor of Neurology and Pediatrics, Johns Hopkins School of Medicine.

SMA Therapy Development

Our community recently celebrated the approval of Spinraza, our first-ever FDA approval of a treatment for SMA. Like Spinraza, many of the other programs in the drug pipeline work to address this loss of SMN protein by replacing SMN1 or by modulating SMN2, the low-functioning SMA “backup gene.” These are called “SMN-based” or “SMN-enhancing” approaches. There are also a number of systems, pathways and processes that are affected in SMA, and there may be additional ways to treat SMA in these other areas—collectively referred to as “non-SMN” approaches. These non-SMN approaches include drugs that work on the muscles and nerves. Research strongly suggests that a combination of SMN-enhancing and non-SMN approaches is the best route to treat all ages, stages, and types of SMA.

This highly anticipated closing session featured nine talks on drugs in preclinical or clinical development, and was moderated by Douglas Kerr, MD, PhD, MBA, Cure SMA board of directors.
Clinical Research Studies for SMA

The goal of this session was to present the results of important studies that could influence clinical trial design or drug development. The talks in this session focused on outcome measures for clinical trials, comparing qualitative and quantitative muscle MRI measures, fatigability, electrical impedance myography and newborn screening for SMA.

This session was moderated by Dr. Kathryn Swoboda, MD, Cure SMA scientific advisory board.

Regulation of Splicing and SMN Protein Expression

Individuals with SMA do not correctly produce survival motor neuron (SMN) protein at high enough levels, due to a genetic mutation in the SMN1 gene. All patients with SMA have at least one copy of a low-functioning “backup gene” called SMN2. SMN2 cannot prevent SMA because it is misspliced, meaning it primarily produces a shortened, less functional SMN protein. However, when SMN2 is correctly spliced, it is able to produce some fully functional SMN protein. Understanding how to promote the correct splicing of SMN2 is valuable for therapeutic development, as is understanding when, where and why SMN protein expression is needed in the body.

This session was moderated by Dr. Arthur Burghes, PhD, Cure SMA scientific advisory board.

Identification of Candidate Therapeutic Targets and Disease Modifiers

Research has revealed that a number of systems, pathways and processes are affected in SMA. The presenters in this session are investigating different aspects of SMA, looking for new ways to treat the disease by targeting other areas. Topics presented included: identifying certain RNA pathways that may be disrupted in SMA; dysregulation of bodily processes and systems; animal models of SMA; and discordance in disease severity. Ultimately, these approaches could be used in combination with approaches that address the underlying genetics of SMA, giving us the best chance of a comprehensive, effective treatment. This is particularly important as we seek to develop treatments for all ages, stages and types of SMA.

This session consisted of two parts and was moderated by Drs. Samuel Pfaff, PhD and Adrian Krainer, PhD, both members of the Cure SMA scientific advisory board.

To read complete summaries of these sessions: Visit www.curesma.org/news and search “2017 SMA Researcher Meeting Summary.”
Quotes from Around the Community on the 2017 Annual SMA Conference

The whole congress and particularly the magical evening at Disneyland was a really memorable experience. So nice to see such a camaraderie between the families and the Cure SMA enthusiasts and a few medical colleagues who got their priorities right.

One of my greatest hopes was that I would live to see a curative treatment for the severe neuromuscular diseases of childhood and it did look in recent years that Duchenne was leading the field.

But quite miraculously it has been the severe end of the SMA spectrum that has shown the most remarkable results and leapfrogged ahead, thanks also to some of the remarkable basic work with the SMA mice.

-Dr. Victor Dubowitz

Thank you so much for helping us get to go to the SMA conference. It was mind blowing. It also was nice to be around other families who understand SMA. My favorite part was being able to go and listen and also speak about losing our daughter. I don’t really talk about it much and it just really helped me know we are not alone and to show our little boy Chance, he was an inspiration to others. Thank you so much again, and remember you reap what you sow. You sowed a good seed here.

-The Lang Family

Thank you for the chance to participate in the exceptional meeting you all put together. The privilege of being there really struck me after returning from Orlando, as I was reading a 1995 paper by Tom Crawford. His introduction is so elegant: “The current vigor of SMA research stands as an outstanding illustration of how insight from multiple levels—from epidemiology, clinical trials, and individual patient investigations to pathology, biochemistry, classic genetics, and now molecular genetics and cell biology—contribute to and draw strength from one another.” Twenty-plus years later, I’m in Orlando listening to him and many of the others who perfected that illustration and figured out how to save children from death and disability. It was a remarkable experience. Best regards, Robert Vogt

Congratulations again on another very successful conference. Back in San Francisco and re-dedicating to our mission to advance new treatments for patients and families fighting the fight against SMA. Thank you again for including us in your program and your community and affording us the opportunity to be so inspired by the courage and hopeful optimism of the many wonderful people who are battling SMA.
Wishing you all the best,
Robert Blum

I am once again struck by what a great scientific meeting this is, and the willingness of all to share study updates is truly exceptional.

Stacy Rudnicki, MD
Thank you so much for selecting Walt Disney World to be your host this year! We see a lot of conferences come and go and certainly there are connections made with each one of them but Cure SMA is really something special and will long be remembered in the hearts of our cast.

- A cast member at Walt Disney World

We attended the 2017 conference and it just ended yesterday. I had to email immediately to say thank you to all involved. Emily is type III and functions well but this meeting is so important to us. She is twenty-four and this was only our third meeting. We wish we had attended more in the past. The financial help you give makes it possible for us to attend. Not just the scholarship but the breakfast, lunch, goodie bag, etc. this is very much appreciated.

- The Pitlik Family

What a wonderful opportunity it was, being part of your conference. Your organization seems to have built an incredible community of families and professionals. Thank you for your invitation and for the opportunity.

-Nicole Thonn

Thank you very much for the invitation to participate at the panel discussion. It was a very positive experience for me to interact directly with the families. Congratulations for the organization of this amazing conference.

Kind regards,
Jean-Paul Pfefen

I truly enjoyed being a part of the event, and honestly have not been involved in such a well-organized meeting, where the volunteers knew exactly what was expected of them, and felt needed throughout. I had a wonderful time getting to know the children over the few days I was there, and am in awe of the wonderful work that Cure SMA does related to research as well as supporting and educating the families. Thanks for letting me be a part of it this year!

-Amy Swiatek

Thank you for another incredible conference. This means so much to our family. We are so grateful for this community!

Sally Bittner Bonn, David Merulla and Oscar

I wanted to thank you all for your role in helping my family make it to the Cure SMA conference this year! The newly diagnosed conference scholarships went a long way to making this trip very memorable for me and my family. We all had such an amazing time!

Thank you again,
Valerie White

Just to say congratulations again on yet another great conference and also to thank you for including us in the industry collaboration meeting last Wednesday. It was encouraging to hear about the significant achievements and progress across the various work streams. Thanks and well done again to everyone involved!

Best wishes,
Joanna Mitchell

I wanted to say thank you and congratulations for a fantastic conference and for the opportunity for Roche/Genentech to be part of the activities - the family friendly poster sessions, the panel discussion and all of the individual events we organized. From a personal perspective, I also wanted to reiterate how grateful I was for the kids program - this was fantastic for me personally to help take care of my daughter while I worked and beyond this, I saw how fantastic this was for the families who attend. This program allows them to maximize the learnings and networking through the conference while their children are taken care of so brilliantly. It was great to catch up with all of the families and all of you in Orlando.

Kind regards
Sangeeta Jethwa

It was a great experience for me. All the knowledge acquired during the conference will be transferred to the daily clinical practice in the public and pediatric hospital where I work.

Kind Regards,
Hilda Verónica Araoz, PhD

It was a pleasure to meet all of you and discuss many important topics. The meeting was well organized and had significant impact for patients, families and all of us. Thanks again for your leadership!

Thanks,
Sukumar Nagendran, MD
Thanks to the support of our generous sponsors of the 2017 Annual SMA Conference, Cure SMA was able to bring together over 2,100 researchers, SMA families and healthcare professionals to network, learn and collaborate. This opportunity offers a unique experience to work in partnership with one another to enhance groundbreaking research and provide families the support they need for today.
Victor Dubowitz Honored at 2017 Researcher Meeting for Early Contributions to the SMA Field

During the 2017 SMA Researcher Meeting, we honored one of our 2017 conference champions, Dr. Victor Dubowitz. Dr. Dubowitz’s early contributions to the SMA field were seminal and have been critical to our understanding of SMA pathology and clinical presentation.

Dr. Dubowitz, originally from South Africa, is a neurologist, professor emeritus at the Imperial College, London. He has authored countless books and research papers about neurological disorders in children, including SMA. Upon completion of both a MD and PhD, he became the Chair of Pediatrics and Neonatal Medicine at Hammersmith. There he rapidly established an internationally recognized pediatric center for muscle disease with a primary emphasis on the clinical management of patients and their long-term follow-up. In particular, he worked to characterize the relationship between age of clinical onset in SMA and severity. This work led to the naming of what is now known as SMA Type 2 as Dubowitz disease.

In addition to his clinical work, Dr. Dubowitz has made numerous additional contributions to the scientific community. Among them he established the multidisciplinary journal *Neuromuscular Disorders*, of which he remains Editor-in-Chief, and founded the World Muscle Society, of which he is the standing president. The Dubowitz Neuromuscular Centre at the Great Ormond Street Hospital for Children in London bears his name.

To honor his lasting contributions to the field, Dr. Arthur Burghes (Ohio State University) presented Dr. Dubowitz with a plaque on behalf of Cure SMA. Dr. Burghes, who has developed many animal models for SMA, received his PhD under the oversight of Dr. Dubowitz. During the award, Dr. Burghes discussed the very significant and lasting influence Dr. Dubowitz had on his career personally, as well as his impact on the entire SMA community.
Every year, the Jacob Isaac Rappoport Foundation goes above and beyond to ensure that our Annual SMA Conference is a tremendous success. They sponsor the amazing Children’s Program, which makes the conference enjoyable for kids and families alike. Without all of the volunteers, the many crafts, entertainment and activities that are provided during the Children’s Program, the conference would not be able to happen; it is essential and incredibly important to our Annual SMA Conference.

Shaina, Adi and their foundation also sponsor a few other fun activities for families throughout the conference.

They provide a special evening for just teens to gather together in their own space and connect with one another. 2017 was the third year of the Teen Social; It has been a huge success and always has a fun entertainment component as well!

The Type I Reception is a special event for type I families to come together each year and would not be possible without the support from the Jacob Isaac Rappoport Foundation. Now in its 16th year, this reception has been an integral part of the conference and for Type I families.

The Rappoport’s support each and every year for the Annual SMA Conference is so greatly appreciated. Always looking for ways to make the experience the best for everyone, the Rappoorts know how important the conference is for families and want it to be as successful as possible.

Additionally, Shaina and Adi sponsor all Newly Diagnosed Type I Care Packages that are sent out to any newly diagnosed Type I family who contacts Cure SMA. These care packages are filled with great, helpful toys and ideas for newly diagnosed families.

The Jacob Isaac Rappoport Foundation was created in memory of their Shooting Star, Jacob, who had SMA Type I. Jacob was born December 27th, 2001 and was an alert and happy baby. When he was nearly four months old, Jacob was diagnosed with Spinal Muscular Atrophy. Soon after Jacob’s diagnosis, Adi and Shaina quickly connected with Cure SMA and many other SMA families. 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 thousands of SMA families; the luncheons and receptions they have hosted; the funding, support and guidance they have provided to 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!
2017 National Partners

Thank you to our National Partners for their continued support of Cure SMA! Without this support, many of our events and programs wouldn't be possible.

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SMA Community’s Voice Heard “Loud and Clear” at the Patient Focused Drug Development Meeting with the FDA

On April 18, 2017, the SMA community—families, clinicians, researchers, industry and regulators—gathered for a Patient-Focused Drug Development (PFDD) Meeting with the FDA.

As part of the reauthorization of the Prescription Drug User Fee Act (PDUFA), the FDA is required to gather community feedback more systematically, through events such as this PFDD meeting, and incorporate that feedback into decisions made on drug development.

The goal of the PFDD meeting was to provide the FDA with an overview of the impact SMA has on individuals and families, and of the expectations and priorities for current and future treatments. This meeting with the SMA community is only the second externally led PFDD meeting to be granted.

“We want to make sure we understand the impact of the disease and what patients prioritize in the treatment of their disease. Those are two specific areas of extreme importance to us,” said Billy Dunn, MD, director of the Division of Neurology Products in the FDA’s Center for Drug Evaluation and Research, during his opening remarks.

“What we hear today will help us to think about clinical trial design, what outcome measures to use in clinical trials, what really matters to patients, and how we as regulators should think about the balance of risks and benefits for patients with SMA,” said Wilson Bryan, MD, director of the Office of Tissues and Advanced Therapies in the FDA’s Center for Biologics Evaluation and Research. “Most of our medical education comes from patients. This [meeting] gives us as regulators the opportunity and the privilege to continue our education by listening to you.”

The Impact of SMA

Twenty different panelists, representing both patients and caregivers of all types, ages and stages of SMA, testified as part of the meeting. Each round of panelist testimony was followed by a period of facilitated discussion and polling questions. Over 400 individuals participated, including in-person and webcast audiences.

“Your voice, which we heard today loud and clear and in great detail, helps FDA as we perform our public health mission and as we evaluate and approve new drug applications,” said Jonathan Goldsmith, MD, associate director for rare diseases in the Office of New Drugs in the FDA’s Center for Drug Evaluation and Research.

Some of the key themes, as summarized by Dr. Goldsmith, included:

- The diagnostic journey, particularly for individuals with SMA type II or III
- The impact of respiratory complications in SMA
- The impact of the loss of the ability to swallow in SMA type I
• The importance of mobility issues
• Difficulties with the activities of daily living
• The impact of fatigue, weakness and muscle pain
• The complications and benefits of surgical intervention
• The challenges of managing complicated medical care at home
• The impact of sometimes prolonged hospitalizations
• The impact of frequent medical visits, including transportation of patient and equipment
• The impact on the family, including social isolation and mental health issues
• The importance of equipment, such as ventilators for home use
• The use of computer technology, including for communication
• Advancements in robotics, wheelchairs and other assistive technology
• The community’s views on new treatments and those under development
• Individual and collective thought processes when a new treatment comes out
• How individual families make individual decisions to best fit their unique needs

Despite the often difficult burden of SMA, Dr. Goldsmith also praised the community for “your commitment and love for your children; your courage and determination as adults, older children and teens; and how you maintain hope and unity.”

Expanding the Community

Both the panelists and FDA speakers recognized the importance of maintaining a strong and unified community in SMA, and of expanding that community to include the industry researchers who are developing drugs, and the regulators who will review and make decisions on the approval of those drugs.

“I also want to thank you for fostering the strong sense of collaboration that is helping to bring new therapies for SMA to patients and families,” concluded Dr. Goldsmith. “The spirit of this organization is really an example to other communities about how you do drug development, how you raise money, how you invest that money in trying to advance things from the laboratory to the clinic, how you give out research grants…and how you get interest from regulated industry.”

“I think it’s the folks with SMA, I think it’s the families of patients with SMA, I think it’s those of you who champion SMA that deserve all the applause in the world,” said Dr. Dunn. “Each step of the way the SMA community has been a distinct pleasure. Thank you for allowing us to be part of your community.”

Thank You

Thank you to all the FDA members who attended, and particularly to Drs. Dunn, Bryan and Goldsmith for providing the opening and closing remarks. Thanks also to Dr. John Day for providing an opening overview of SMA, and to James Valentine, JD, MHS, for moderating the discussion.

Thank you to our panelists for their incredible generosity with their time and their stories: Gina Cannady, Debbie Cuevas, Scott Ellis, Kristen Farrell, Christine Getman, Grace Grutter, Kelly Jankowski, Patti Kemp, Rio Landa, Kristen Lasko, Jungin Angie Lee, Kathryn McBride, Christina Murray, Brad Nunemaker, Kevin Schaefer, Danyelle Sun, Hugo Trevino, Lyza Wiesman, Brynne Willis and Jessica White. Thanks also to the in-person and webcast audiences for their valuable and thoughtful contributions.

Thank you to the members of the SMA Industry Collaboration for their support of this meeting: Astellas, AveXis, Biogen, Novartis, Genentech/Roche, Cytokinetics, Ionis, MDA and the SMA Foundation.
Last fall, Cure SMA announced a Newborn Screening initiative with the goal of adding SMA to the Recommended Uniform Screening Panel (RUSP). The RUSP is a list of disorders the Secretary of the Department of Health and Human Services (HHS) recommends to states for their state universal newborn screening programs.

Earlier this year, Cure SMA and our SMA Newborn Screening Coalition partners Biogen and AveXis announced the completed submission of a proposal to add spinal muscular atrophy to the federal Recommended Uniform Screening Panel (RUSP).

After evaluating the submission based on several criteria, the Advisory Committee on Heritable Disorders in Newborns and Children announced in May that it had unanimously accepted SMA into the evidence review process for the RUSP. A final decision on whether to recommend SMA will be issued in February 2018.
Newborn Screening Ongoing Efforts – One State at a Time

While a successful RUSP application will strongly support our case for national implementation, the decision to screen for SMA ultimately rests with each individual state.

Missouri became the first state to pass legislation in July. Senate Bill 50, sponsored by Representative Becky Ruth, Missouri is a great example of how our community can move forward on newborn screening while continuing work on the RUSP.

This early victory energized our families to additional efforts at the state level. This fall, families in Arizona, Texas, Indiana, Illinois, Maryland, New York, Ohio, California, Utah, Arizona, and Pennsylvania, engaged their elected officials on the importance of newborn screening.

Our families in Texas were featured in an article about its state program. In Utah, families testified before the state and proposed to expand newborn screening; as a result, legislators will add additional funding to support the program next year. Legislation related to newborn screening has been introduced in Illinois and Ohio. Newborn Screening Advisory Committees in Minnesota and Maryland are also considering adding SMA to their panels.

Thank you to all our families who continue to be advocates in your state. For more information and to get involved in our advocacy efforts, please email advocacy@curesma.org.

Kelly and Jeff Eakin, Brandi and Michael Akins, Jonathan, Kristen and Max Lasko, Morgan Lenz and Kenneth Hobby made advocacy visits in Washington D.C. to support SMA newborn screening at the federal level.

Travis, Holli and Brooklyn Hensley meeting with Senator Orrin Hatch. The Hensley’s made advocacy visits in Utah and testified before the state for a proposed expansion of newborn screening funding.
Publications From Cure SMA

At Cure SMA, we’re committed to making sure that families have the best, most accurate information about SMA and what it means for them, from day-to-day care to the changing landscape of research breakthroughs. Our care series booklets provide in-depth information on medical issues, genetics, and other topics of interest to both families and healthcare providers.

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

If you would like a hard copy mailed to you please email us at info@curesma.org or call 800.886.1762

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.
Spinraza Access and Administration Sites

Spinraza is now approved in the US, Canada, Europe, Japan, Brazil and Switzerland. Additional filings are pending in Israel, Australia, South Korea and Argentina.

In September, Cure SMA hosted a webinar updating the community on Spinraza access and administration sites.

- More than 170 commercial plans have approved Spinraza, meaning that over 85% of commercial members are part of plans that have a Spinraza policy.
- 28 state Medicaid boards have issued a Spinraza policy, covering 80% of state Medicaid members.

Approximately 70% of commercial and 60% of Medicaid policies are broad policies, meaning they have few or no restrictions based on type, age or copy number.

The remaining 40% are more narrow, and represent opportunities for continued advocacy by the SMA community.

Cure SMA Spinraza Site Administration List

In April, Cure SMA launched a list of Spinraza administration sites. This list currently includes 82 sites in 30 states (including Washington DC), and is continually updated as new sites are added.

Site managers must give their permission to have the site listed on Cure SMA’s Spinraza Site Administration List. If your local site is not listed, please email us, or ask the site coordinator to email us, at patientcare@curesma.org.

To learn more about access to Spinraza and the initiatives we’re currently working on, visit www.curesma.org/spinraza.html


Biogen recently provided the following community statement on Spinraza:

Dear Members of the SMA community,

As part of our commitment to the SMA community to provide ongoing and comprehensive communications about SPINRAZA® (nusinersen), we are pleased to share that the final results from ENDEAR, the Phase 3 study of SPINRAZA, were published in The New England Journal of Medicine (NEJM). The full manuscript, titled “Nusinersen Versus Sham Control in Infantile-Onset Spinal Muscular Atrophy,” appears in the November 2 issue of NEJM. Publishing this information is part of our efforts to provide additional details on clinical trial data that support the favorable efficacy and safety profile of SPINRAZA and support access to the therapy for those who may benefit.

The ENDEAR study is part of the largest well-controlled clinical development program conducted to date, which includes more than five years of data for SMA. Biogen and the physician community continue to collect and evaluate data through SHINE, the SPINRAZA open-label extension study, and we are planning to conduct new clinical research across different SMA populations. These efforts will provide a deeper understanding of SMA and SPINRAZA, and will support broad access over the long term.

We are incredibly thankful to the entire SMA community for their continued support, and especially the nearly 200 individuals and their families in the U.S. who have participated in our clinical studies. Their participation helped lead to the early and broad December 2016 FDA approval of SPINRAZA, allowing, as of September 2017, approximately an additional 1,200 U.S. patients to start on therapy, of which approximately two-thirds are individuals with Type 2 or 3 SMA.
However, we are keenly aware that there are many more who may benefit but have encountered challenges, including availability of local treatment sites, reimbursement delays, and for some patients, complexities arising from the intrathecal injection.

**We continue to work with stakeholders so that people who can benefit from SPINRAZA may gain access to therapy. Our current and planned activities to help overcome these challenges include:**

- Supporting the increasing number of local treatment delivery sites: There are now over 180 sites that have started dosing across the U.S. Physicians and advocacy groups are working to add more in the coming months, striving for a balance of both pediatric and adult sites.

- Educating stakeholders on outcome measures: Biogen believes that delivery of a high level of care in combination with the accurate and standardized use of measurement tools is an important part of managing SMA. We therefore continue to support SMA education initiatives for the community, including for physicians, physical therapists, and payers, with these goals in mind.

- Supporting physicians in addressing administration challenges: Biogen is actively engaging with the physician community, which has novel ideas regarding approaches to administering treatment for patients with complex cases, such as those with varying degrees of scoliosis and spinal rods/fusions. We are also working to understand how to make such innovations in spinal access available to more treatment centers in the U.S., where appropriate. While we realize there is an urgency to help find solutions, we also acknowledge this process may be lengthy, and Biogen is committed to finding the safest and most effective way to provide access to individuals with SMA.

Lastly, we recognize the ongoing efforts to add SMA to Newborn Screening (NBS) panels and we are proud to be a partner in this community-wide effort, with Cure SMA, other companies and you. We look forward to the upcoming federal decision by the Recommended Uniform Screening Panel (RUSP) in February 2018, and are pleased by the early efforts in some states, such as Missouri, Minnesota and Utah, leading to adoption of SMA in NBS panels.

One of the many things we’ve learned about the SMA community is that you are motivated and committed. Many of the challenges we originally identified when launching SPINRAZA are gradually being overcome – and that’s largely a reflection of the incredible determination of this community of providers, families and individuals with SMA. We remain a dedicated partner to the SMA community and will continue to provide updates in the future.

**Thank you,**

**The Team at Biogen**
Clinical Trial Updates

Genentech/Roche Releases Clinical Trial Update for SUNFISH (RG7916)

Genentech/Roche recently provided a community statement with clinical trial updates for SUNFISH (Type 2/3). The first patient is now enrolled in SUNFISH, as the trial advances to part two, which is designed to be a pivotal marketing registration trial.

SUNFISH is a two-part study in which Part 1 will allow selection of the dose of RG7916. Part 2 is designed to assess the effectiveness and is the pivotal registration part of the study. If positive, results may be used to support Health Authority submissions and potential approval and access to RG7916. The first group of participants enrolled into SUNFISH Part 1 has received RG7916 for more than 10 months and an interim analysis was recently presented at the International Conference of the World Muscle Society in France. In this analysis, all participants (51 people) had received RG7916 for 28 days or longer. SMN protein increased by up to two and a half times. This increase has been sustained throughout the duration of treatment (up to 250 days). To date, RG7916 remains well-tolerated at all doses and no-one has withdrawn from any RG7916 study due to drug related side effects.

The information from Part 1 has allowed Genentech/Roche to confirm the dose of RG7916 for Part 2 of SUNFISH and the first patient has been enrolled into Part 2.

If you would like additional information about the SMA program, please visit www.roche-sma-clinicaltrials.com, www.clinicaltrials.gov (search for SUNFISH) and www.clinicaltrialsregister.eu.

AveXis Announces Plan to Initiate Pivotal Trial of AVXS-101 in SMA Type 1 Using Product from New GMP Commercial Process

AveXis, Inc., a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases, recently announced the U.S. Food and Drug Administration (FDA) has notified the company that based on review of data submitted, including the potency assay, it may initiate its planned pivotal trial of AVXS-101 for patients with spinal muscular atrophy (SMA) Type 1 using the intravenous (IV) formulation produced by the company’s Good Manufacturing Practice (GMP) commercial manufacturing process. The company plans to initiate this trial immediately.

“We are pleased to reach this outcome following a thorough review by the FDA of the voluminous information we supplied to address the commitments made during the Chemistry, Manufacturing, and Controls Type B meeting in May, and are eager to initiate our pivotal trial of AVXS-101 for SMA Type 1 in the U.S. using product from our GMP process,” said Sean Nolan, President and Chief Executive Officer of AveXis. “Moving AVXS-101 back into the clinic, as planned, with product from our GMP process is a significant milestone, not only for AveXis but also for the patients we hope to serve.”

The AveXis facility is the production site to supply the pivotal and future trials and, should AVXS-101 be approved for marketing, to meet projected commercial demand. With the pivotal trial now starting, AveXis and the FDA are continuing discussions on key topics, including dosing, for intrathecal administration of AVXS-101 for the planned clinical trial in patients with SMA Type 2. An update on this program will be provided in the fourth quarter of 2017.

U.S. Pivotal Trial in SMA Type 1 (STR1VE)

The open-label, single-arm, single-dose, multi-center trial — known as STR1VE — is designed to evaluate the efficacy and safety of a one-time IV infusion of AVXS-101 of 1.1 x 10^{14} vg/kg, which is equivalent to the proposed therapeutic dose received by the second dosing cohort in the Phase 1 trial, in patients with SMA Type 1. Based on the data derived from
the company’s new analytical methods that were submitted and reviewed by FDA, it has been determined through direct test with the improved PCR method that the dose used in AveXis’ Phase 1 trial of AVXS-101 in SMA Type 1 was 1.1 x 1014 vg/kg. Additionally, extensive testing in the SMN delta 7 mouse potency assay has demonstrated the equivalence of dose response between the products produced by the Phase 1 and Phase 3 manufacturing process.

The trial is projected to be conducted at 16 sites in the United States, including: Ann and Robert H. Lurie Children’s Hospital of Chicago, Boston Children’s Hospital, Children’s Hospital Colorado, Children’s Hospital of Philadelphia, Columbia University, David Geffen School of Medicine at UCLA, Duke University, Johns Hopkins Pediatric Neurology, Nationwide Children’s Hospital, Oregon Health and Science University, Stanford University Medical Center, University of Central Florida College of Medicine, University of Texas Southwestern Medical Center, University of Utah, University of Wisconsin, and Washington University School of Medicine.

“We are appreciative of the detailed reviews and timely feedback we have received from the FDA,” said James L’Italien, PhD, Chief Regulatory and Quality Officer for AveXis. “We look forward to our end-of-Phase 1 meeting, which has been scheduled for late in the fourth quarter, to discuss next steps in the regulatory process for AVXS-101.”

To learn more about the co-primary and co-secondary efficacy outcome measures of the AVXS-101 trial, visit www.curesma.org/news/avexis-pivotal-trial.html

Novartis Releases Update on LM1070 (Branaplam) Clinical Trial

Novartis recently announced that they would resume enrollment in the ongoing study in type 1 SMA (CLM1070X2201) at their existing sites in Belgium, Germany, Denmark and Italy. CLM1070X2201 is an open-label study (all patients assigned to branaplam) where different doses are being tested in patients with type 1 SMA who are younger than 6 months of age. More information about the ongoing clinical trial is available on Novartis’ clinical trials website, on clinicaltrials.gov and the EU Clinical Trials Register.

Now that recruitment has resumed, Novartis is seeking to expand to additional sites and countries. Novartis also shared that they recently opened an Investigational New Drug (IND) application with the FDA. This means US patients are allowed to enroll in the study, and the company will notify the SMA community as US sites open.

As a reminder, branaplam is designed to be orally administered rather than via a feeding tube only. Novartis made this change – and others – in response to feedback from patients’ families and investigators.

In parallel, Novartis is also working closely with regulatory authorities to define the best way to expand branaplam clinical trial program beyond type 1 SMA and make the trials available for patients as quickly as possible. Recently the US FDA granted a “fast-track” designation in type 1 SMA.

Cytokinetics Receives FDA Orphan Drug Designation for CK-2127107 for SMA

Cytokinetics, Inc. recently announced that the Office of Orphan Products Development of the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to CK-2127107, a next-generation fast skeletal muscle activator, for the potential treatment of spinal muscular atrophy.

Orphan designation is granted to drugs and biologic products that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders. Through this designation, the FDA provides incentives for further clinical research and marketing authorization for conditions where there is a significant unmet need for effective therapies. “We are pleased that the FDA has granted orphan drug status to CK-2127107 for the potential treatment of patients with SMA,” said Fady I. Malik, Cytokinetics’ Executive Vice President and Head of Research & Development.

The clinical trials for CK-2127107 materialized because of early seed funding from Cure SMA supporting research focused on the potential application of these specific skeletal muscle activators. In 2014, Cytokinetics released encouraging data from preclinical studies conducted with our funding. The data showed this approach had positive effects in preserving muscle strength and reducing muscle fatigue, setting the groundwork for the ongoing clinical trials.

The progress of this program also highlights the importance of developing combination therapies to treat SMA. The goal is that CK-2127107 will show positive results in preserving muscle strength in human clinical trials, and may lend itself to combination with other SMA therapies, particularly those that address the SMN protein deficiency caused by the SMN1 mutation. The development of combination therapies is particularly important as we seek to treat all types, ages and stages of SMA.

For more information on clinical trial updates, visit www.curesma.org/news
Cure SMA Announces Over $5 Million in New Research and Care Funding

Cure SMA is pleased to announce $5 million in new research funding over the next 12 months. This funding will be used strategically to help accelerate research and ensure we are developing treatments for all types, ages and stages of SMA. This funding will also be used to increase patient access to treatments and improved optimal clinical care.

In total $2.7 million of this funding—over half of the $5 million total—will go directly to local support and care, while the remainder will fund basic research, newborn screening, and clinical and regulatory initiatives.

$900,000 in Continued Funding for Basic Research

Basic research investigates the causes and biology of SMA, often revealing more effective ways of making SMA drugs. Continued funding in basic research will help address questions about survival motor neuron (SMN) protein, which is not produced properly in the bodies of those with SMA, and help us identify other systems, pathways and processes that are affected in SMA. This research could then lead to the development of combination therapies for SMA, using both our knowledge of SMN protein and our knowledge of these other systems, pathways and processes. Approaches that work on these other areas could be used in combination with approaches that work on SMN levels, allowing us to develop treatments for all types, ages, and stages of SMA.

$1 Million in Continued Funding for Clinical and Regulatory Research

As the SMA drug pipeline continues to grow in depth and diversity, so does our need to address clinical and regulatory issues and continue to bring the patient voice into the process. Funding for this area will be directed toward several critical initiatives:

- Engagement with the FDA and other regulatory agencies
- Increased education and awareness to parents, families and the medical community to reduce diagnostic delays
- Expanding capacity for new clinical trial sites

Funding Priorities

As the SMA research landscape has developed and the drug pipeline has grown to include the first-ever FDA approved treatment, the needs for SMA research have also grown and developed. This $5 million investment will guide us into the next phase of SMA research and care, by funding the areas of greatest need, and where we are best positioned to make a significant difference.
$2.2 Million for Clinical Care Centers

Now that a treatment option is available, it is imperative to improve patient care and quality of life. With this in mind, Cure SMA is launching a network of Cure SMA care centers. These centers will electronically submit information to the newly created Cure SMA clinical data registry. The registry will be used to answer questions about best health care for SMA by examining care practices across centers. Centers will receive center-specific feedback to improve care at their site. The Network will begin with 10 centers and then rapidly expand. Our goal is to improve the standard of health care for everyone with SMA.

$100,000 to Train Healthcare Providers to Provide Quality Care for SMA

Cure SMA is also funding a new Therapist Mentoring Program in order to improve care in the local community for those affected. The purpose of this program is to:

- Provide information, experiences and resources for physical therapists and occupational therapists who are new to SMA, or are seeking additional experience
- Increase access to therapists and pulmonologists with the expertise to evaluate individuals with SMA

$400,000 to Improve Access to Treatments and Care

In order to increase capacity to follow, treat and evaluate those affected by SMA, Cure SMA will award $50,000 grants to nine administration sites. These grants will help sites reduce some of the practical barrier that create delays for our community in accessing treatments and care. These barriers include pre-authorization and insurance approvals, scheduling, and clinic support.

$400,000 for SMA Newborn Screening Initiatives

With a treatment option now available and data indicating that pre-symptomatic treatment improves clinical outcomes, it is vitally important that SMA be added to state newborn screening panels as quickly as possible. Resources are also needed for state public health labs in order to implement newborn screening for SMA. Adding SMA to the Recommended Uniform Screening Panel (RUSP) is a critical step toward our goal of having SMA added to newborn screening programs across the US. In May, the RUSP application was unanimously accepted into the evidence review stage, with a decision expected in six to nine months.

$100,000 for Data Projects to Support Increased Access and Approvals

For the past several years, Cure SMA has been collecting data and information on the experiences of living with SMA. Earlier this year, we released a survey for all individuals and families that have reached out over the past decade to gather how SMA has impacted their lives. In the coming months, we will release a similar survey to track any changes in the burden of SMA over the past year, now that an approved SMA treatment is available.

Thank You

The dedicated support of our community has made all of this possible. Whether families raising funds and awareness, researchers investigating new potential treatments, or pharmaceutical and regulatory partners helping us take the next steps toward future approvals – everyone has a role to play. Thank you to everyone for their hard work and dedication.
The SMA community celebrated an impactful and advocacy-driven Awareness Month in a number of ways this August. Whether hosting candlelight vigils, meeting with state and local legislatures, holding fundraisers, coordinating MLB awareness games and more, the community worked together to raise the general public’s awareness of SMA.

Advocacy Visits

On August 2nd, members of the community kicked off SMA Awareness Month by meeting with a dozen Senate and House congressional offices to advocate for issues that impact our community. The teams conducting these visits included families affected by SMA and Cure SMA staff.

One primary goal of these meetings was to advocate for necessary federal funding for newborn screening. Though implementation of newborn screening ultimately happens at the state level, the federal government also plays a key role.

Families in Texas, Illinois, Indiana, Ohio, Maryland and New York also made visits and testified to their state legislatures to advocate for newborn screening.

State and Local Proclamations

Many families and chapter leaders asked their state and/or local government to issue proclamations of SMA Awareness Month in the following states/cities/counties:

- Colorado
- Michigan
- Minnesota
- Orlando, FL
- Jacksonville, FL
- Tallahassee, FL
- Pensacola, FL
- Tampa, FL
- Gainesville, FL
- Sarasota County, FL

Candlelight Vigils and Social Media Outreach

At sunset on Saturday, August 12th, the community held candlelight vigils all over the country to remember those who have passed away from SMA and to honor those living with SMA.

Niagara Falls, the Peach Bridge and the Curtis Hotel in Buffalo, New York were lit purple for the vigil and to bring awareness to SMA – a highlight of Awareness Month, courtesy of Western New York Chapter member, Bailey Walter.

Additionally, our community was vigilant and active on social media channels during SMA Awareness Month, and a few families/individuals shared with us their experiences and point of view as part of Wednesday morning videos.
Thank you!

Cure SMA would like to thank everyone who participated in SMA Awareness Month.

Whether you made an advocacy visit, attended an MLB awareness game, sent in photos or videos, or simply shared messages on social media, we appreciate the community effort to spotlight SMA and look forward to next year.
Former TCU classmates Shannon Scott Zerzan ’01 and Crystal Hecker Somers ’01 recently reconnected through social media. Their budding friendship sprang from a shared struggle.

Zerzan and Somers have young sons with spinal muscular atrophy, a genetic disease that affects the part of the nervous system that controls voluntary muscle movement. Each year between 1 in 6,000 and 1 in 10,000 people are born with the disease, which is classified into four types based on age of onset and achievement of the highest physical milestone, report the U.S. National Library of Medicine and the National Human Genome Research Institute.

“I hate that Shannon’s family has to deal with SMA,” said Somers, who lives in Humble, a Houston suburb. “But having a fellow Horned Frog I can turn to with questions, concerns or just to vent makes it so easy because we are already part of the TCU family. Now, we are part of the SMA family as well.”

“Our shared experience allows us to understand each other in a profound way.”

— Shannon Zerzan
Zerzan, who lives in Alexandria, Va., reached out to Somers a year ago when a mutual friend realized that both mothers were posting about the condition on Facebook. Eventually, the two women became close and found their mutual support invaluable.

Zerzan’s son Charlie was diagnosed in early 2010, a few months after his second birthday. “When he was about 18 months old, we noticed he was having trouble doing things at our neighborhood park that he had previously been able to do.”

Charlie developed an awkward gait. His running slowed and climbing stairs became an ordeal. A neurologist concluded that Charlie was dealing with a neuromuscular disorder, Zerzan said. “To say we felt as though we had been punched in the gut is an understatement.”

Doctors diagnosed Charlie with Type III, the rarest and mildest form of the childhood onset of the disease. His younger brother, George, does not have the condition.

Charlie participates in a clinical trial for a drug designed to treat the disease and remains healthy, Zerzan said. “He walks and runs (a little) and plays and is very familiar with the challenges placed on him by the condition.”

While Charlie tires easily, Zerzan said he makes it through most school days without using a motorized scooter. With the help of physical therapy, he has become an avid swimmer.

Somers’ son, Cooper, was 4 months old when she and her husband, Jason, noticed an issue with his legs. His pediatrician downplayed the impaired movement, but a second opinion from a different pediatrician confirmed their concerns.

An initial blood test indicated that Cooper had Type I, the most severe form of the disease.

“His pediatrician told us to take him home and love him because he could live two weeks, two months, two years,” Somers says. “Thank goodness he has fought the odds, and he has made it much longer than expected.”

Somers says that Cooper’s doctors now consider him either a weak Type II or strong Type I. He also has frequent, dangerous respiratory illnesses. In December 2012, he had pneumonia. While Cooper was in the hospital, Somers’ husband died in a car accident.

To honor her husband’s wishes, Somers enrolled their son in a public school kindergarten last year. During the school day, a nurse attends to Cooper’s needs. He gets twice-weekly physical therapy plus speech and occupational therapy sessions. At home, an assortment of medical devices helps the young boy breathe more easily. During the night, Somers must turn her son every few hours to prevent bedsores.

Yet Cooper is an active child. He participated in a special-needs baseball league in the spring, and he plays in a power chair soccer league. He also enjoys his role as big brother to Hunter, who was born after the death of Somers’ husband through in vitro fertilization with genetic screening.

Zerzan and Somers are devoted to raising awareness and hope about their sons’ genetic conditions. Zerzan and her husband, Greg, organized a Hope on the Hill congressional dinner in 2011. The annual event has raised more than $800,000 for Cure SMA, a national nonprofit organization focused on advocacy, support and research.

The Somers family is active in muscular dystrophy causes. In 2013, Cooper was featured on the Muscular Dystrophy Association’s annual telethon and served as an ambassador for the organization. Nowadays his mother works with other Houston-area mothers to organize a future fundraiser to support Cure SMA.

Somers and Zerzan plan to continue supporting each other.

“Our shared experience allows us to understand each other in a profound way,” Zerzan said. “Having a surplus of purple in our wardrobes is icing on the cake.”

Callie was born on Aug 15, 2017!

Congratulations to Rachael and Scott Rodgers of Ohio, and big brother Finn, on their new baby girl, Callie.

Congratulations!
Family Support

Fighting For Kaiden:
Cure SMA recently received a generous donation from the Fighting for Kaiden Foundation. Donated items included three EASyS strollers and two boxes of assorted items for the newly diagnosed care package program. The foundation was founded by the Defazio family in honor of their son Kaiden, who has SMA type I. We would like to sincerely thank the Fighting for Kaiden Foundation, and all their incredible supporters and donors.

Jadon's Hope Foundation:
Jadon's Hope Foundation generously donated 9 new feeder seats and 6 telescopic ramps to the Cure SMA equipment pool. Our sincerest thank you the Burks family and donors of Jadon's Hope for their continued support!

The Miller McNeil Woodruff Foundation:
The Miller McNeil Woodruff Foundation has generously donated $87,000 to Cure SMA! This gift, honoring the life of Miller McNeil Woodruff, will help fund research and advocacy initiatives that are vital to the SMA community. We sincerely thank the Woodruff family and foundation for their continued support.
Family Letters

Cure SMA,

Liv enjoying all of her goodies!
Thank you so much we really needed some fun during this difficult time.

The McDonald Family of Idaho

Hello Cure SMA,

Thank you for the WONDERFUL package! Lots of info for me and lots of toys for Lily! Thanks again!

Denise Meredith of New Jersey

Hi Cure SMA,

Thank you so much for the package. Our girl is happy, it made her day!

Fatema of California

Cure SMA,

My family and I want to thank Cure SMA for Rhydian’s gift box and wagon. He loves all of it and we are incredibly touched and grateful for the generosity and kindness. Attached are pictures with some of the goodies.

Sincerely,
Summer Gregg of Florida

Hello Cure SMA,

I want to say thank you very much for the DVD you sent me, it opened our minds. I received two days ago and I am very grateful.

So, once again thank you so much. God bless you.

With all my gratitude and affection,
Catiane Uliano of Brazil
To Cure SMA:

Thank you so much for sending such a wonderful care package for my son Aiden. Our family was devastated but with the help of curesma.org it has been a little easier. Here is a picture of Aiden and all of his goodies. Thank you again for everything!

The Lueken Family of Indiana

Dear Family Support,

My husband, kids and I would like to thank you and all the families that have generously donated the care package to our son as well as all the other families. This is very kind and touching to know that we have such a kind and loving support team behind us! Here are some pictures of “King” enjoying all his wonderful items!!

God Bless,
Mr. and Mrs. Starling of Florida

Hi Cure SMA,

Just wanted to let you know that we received the care package last night. It’s amazing! We would like to thank everyone who contributed to it, we greatly appreciate your time, effort and generosity!! It means so much to our family.

Thank you!
Lu Huang of Indiana

Dear Cure SMA,

Thank you so much for the CD. I really appreciate your kind support.

Best wishes and thank you for the work you are doing. It has a massive impact across the world.

Georgia Clarke of Australia

Cure SMA Staff,

Thank you so much for that amazing care package. Our daughter Lissa and our older daughter Renee had the absolute best time with the balloon and the pinwheel.

We can’t thank you enough,
Danielle, Josh, Renee, and Lissa Whitaker of Ohio

Cure SMA,

We are having so much fun with these new toys! Thank you!

Stephany Lawton of Washington

Dear SMA,

We would like to thank all the families who donate and to everyone from Cure SMA. Thank you for making this possible, he loves the care package!

Thank you so much,
The Zamarripa Family of Texas

Dear Cure SMA,

Thank you guys so much for everything you sent! Our sweet boy is putting it all to good use!

Jerrica Fluty of Kentucky

Cure SMA,

Our care package came today and I can’t even begin to thank you guys enough for all of these things for her.

Thank you again.
Erin Vleugels of Ohio
To Family Support:

We are so grateful for the foundation’s generosity and the parents that have donated things to be sent to other families. Some of the things in this package are things we have seen that we have wanted to get Wesley, but something always tells us not to, now we know why. Thank you guys so much!

Thanks again from the bottom of our hearts!
The Neel Family of South Carolina

Cure SMA,

We have received your care package, we are very grateful. Thank you for the books, they are very informative, as we are now learning about SMA. Takyla is also enjoying her new toys.

Thanks for what you are doing to educate and support SMA affected families.

Sincerely,
Patricia and Anthony Lynch of Florida

To Cure SMA:

Thank you so much for the wonderful care package. Nemue really had fun exploring all the toys and books and blankies. The gifts are all so thoughtful and our hearts are full of love for all of the dear children that were honored through these gifts. This has been an extremely difficult journey and we continue to struggle with coming to terms with this disease. We are grateful for your support and kindness. We are exceptionally grateful that there is a therapy available to Nemue now and appreciate all of the time and energy CureSMA has put toward this goal. From the bottom of our hearts, thank you.

Sincerely,
Carla Botschner of Florida
The other night I was hanging out with a friend from college at a downtown pub. As we sat down and ordered a couple of beers, an older gentleman at a nearby table came and sat next to us and asked me about my robotic arm. I could tell he was fascinated by the device, and he asked intelligent questions about how it functions and how I acquired it. He was a retired electrical engineer, and it’s not uncommon for people with scientific backgrounds to ask me about the arm. I honestly don’t mind this at all, as I’m more than happy to promote the company that makes the JACO arm and tell people how it’s changed my life. This man was very cordial and spoke with both my friend and me for several minutes.

Then came the kicker. As the man was leaving to return to his table, he looked at me and said that infamous line that I’ve heard all too many times: “You’re an inspiration.”

Now, anyone who uses a wheelchair or has some kind of visible disability has heard this line a million times. I even had the idea to make a drinking game out of it. If I’m out at a bar, I have to take a shot, along with whomever I’m with, every time a stranger calls me an inspiration. I haven’t tried it yet, but I can almost guarantee that it would result in my entire crowd getting wasted by the end of the night.

As to why those of us in wheelchairs and with other disabilities don’t like being called “inspirational” by people
we’ve never met, the comedian Josh Blue explains it perfectly. Blue, who has cerebral palsy and whose humor is primarily based on experiences and observations from his own life, says in his acclaimed 2012 special “Sticky Change” that when people tell us that we’re an inspiration to them, what we hear is, “Man, I would kill myself if I were you.”

It’s true. While people are trying to be nice when they use this expression, it begs the question of what is it that makes us so inspirational? Should we really be applauded for simply living our lives like everyone else? By issuing this so-called “compliment” just on the basis of our physical appearance, people are essentially implying that it would make more sense for us to stay home and be miserable. I know they don’t mean that, but if you look at the connotations of the “you’re an inspiration” phrase, that is what it boils down to.

However, there are other situations in which I don’t mind being called an inspiration. If I give a speech, reach out to someone in need, or write something that makes a positive impact on a reader, then the expression is more appropriate. In those instances, I’ve done something that warrants a compliment. Or if a friend or family member says that I inspire them (or something equally cheesy and sentimental), I don’t mind because I have a relationship with that person.

That said, when a stranger who knows nothing about me other than that I use a wheelchair and am out in public, tells me that I’m so inspirational, my natural instinct is to get annoyed and crack jokes about them. Though I deal with challenges that others don’t, I have just as many flaws, desires, quirks, and feelings as anyone else. All I’m saying is that before you go straight to putting people like me on the inspirational pedestal, get to know us first. I think you’ll find that you and I are more alike than you may have thought.

SAVE THE DATE!

FOR THE 2018 ANNUAL SMA CONFERENCE

THURSDAY, JUNE 14TH TO SUNDAY, JUNE 17TH

THE HILTON ANATOLE HOTEL IN DALLAS, TX
Loving Memories

This section is designed so it can be removed from the center of the newsletter.
In Memoriam
A’Nylah Normani Wright

This is one of the hardest, most emotionally draining things I have ever had to do. To tell the story of a beautiful angel, A’Nylah Normani Wright. LaLa was born November 3, 2015. From birth my granddaughter was always seen as a happy, spirited and loving baby who could brighten up everyone’s faces with just one smile. For those who do not know, LaLa was born with Spinal Muscular Atrophy type I. SMA is a genetic muscular disorder of which it involves the loss of nerve cells in the spinal cord causing limited muscular growth and movement. With this being said, my granddaughter was unable to care for herself and needed 24/7 assistance from her mother and family members. Even though she was born with this disease, we treated her no different then we would anyone else. We made sure that she was comfortable and a part of every event or activity we had. Even though LaLa lived a happy life, we all knew it would eventually be cut short by this disease. She was able to beat the odds and live past the life expectancy the doctors gave her. Throughout her battle, the contagious smile she offered everyone did not leave her face. In the late month of June, my daughter discovered that A’Nylah had gone into cardiac arrest and was unresponsive in her bed. While they were able to revive her, she was still left without any oxygen getting to her brain for more than fifteen minutes which resulted in her having major brain damage. The doctors could do nothing more for her so my daughter was forced to make the hard decision of bringing A’Nylah home to let nature take its course. Although her body was tired, my granddaughter still managed to keep the smile on her face to ensure those around her that no matter what she will be okay, and that she has finished her final race in life.

Sincerely,

Jasmine Lewis

In Memoriam
Terrance Rashad | Forever 2 years old
December 15, 2014 - June 9, 2017

Terrance was a smart, bright, loving prince. He loved to spend quality time with his family and play with his siblings. He has a twin sister, Teresa who also has SMA type I and three other sisters and a brother as well. He loved going outside, spending time in the sun, and the wind blowing on him. He was such a happy baby, always smiling and it was so easy to put a smile on your face when he was around. Now he is no longer suffering; he is pain free, walking, smiling, and playing with the other angels looking down over us. May he rest in peace.
In Memoriam

Dear Alexandra,

This October 24th, we celebrate your 10th birthday. As mommy promised on January 1st, 2008 (the day that you physically left this earth), we will never and have never forgotten you. Your big sister (Avery) remembers you and misses you. Your little twin sisters (Isabella Alexa and Calla Alexis) know you, talk about you all the time and miss your physical presence as well. And, you know that mommy and daddy love you so much. We think about you every single day without fail. You are still a significant and major part of our lives.

When we were on our family vacation in Hawaii, mommy was on the plane and looked out at the clouds. I kept trying to find your face through the clouds. We miss you. While talking with other families, they always say “wow, three girls, huh?” and I always respond “no, actually, four -- I have four girls.” While at the beach and seeing a family push their special needs child in a wheelchair, I imagine how different our lives would be had you lived. I just know that we would have done our best to give you and expose you to the same wonderful and exciting things in life that I see other families doing.

Alexandra, you are our angel. You keep us grounded. You look out for your sisters and family. We are so lucky that you chose us as your family and we thank God for Him allowing us to have you for as long as we did. You are our blessing. As we’ve come to learn, it’s not about how long you lived but about the lifelong impact that you’ve made on everyone who met you and on those who have subsequently come to know you. So many people have said to me, Alexandra changed my life and, as your mommy and daddy, that makes us more proud than anything.

So, as we celebrate your 10 year old birthday, we want to thank everyone who has helped us raise just north of $100,000.00. We want to congratulate Cure SMA for being so diligent in finding a cure and so proud to be a part of this historic / breakthrough year with the FDA approving the first drug to treat SMA. While we wish we had these treatments for you when you were alive, we are just so glad and honored that your memory / your legacy represents a contribution towards a treatment / cure to this terrible disease. Without you / Alexandra and other families, this momentous occasion would not be possible. In other words, your life was not lost in vein but stood for research and fundraising that resulted in this outcome so other lives would be saved. Look at you! Look what you, we, our family, friends and the Cure SMA community did together! I remember people telling me that this day would never come and, while we still have a way to go to find the perfect cure, we have come very far! We did it, Alexandra.

We love you. Happy, happy 10th birthday. We are so proud of you!! And, as promised, we will never, ever forget our Muffin Stuffin, Alexandra!!

Love,

Mommy, Daddy, Avery, Isabella and Calla

PS. Your cousin, DJ and Baby Rylie send their love and your cousin/ Kameron holds you deep in his heart, as just left for college. Give Grandpa and Papa Avery a big hug. We miss them too. Grandma, Grammy and Gramps send big, big kisses, hugs, chocolate cake and balloons.
Dearest Angel Alexandra,

You were only physically with us for 2 1/2 short months, but you have continued to bless us in a very real way for ten years and counting. While just a tiny infant when you left us, you were one of the strongest persons that we have ever known. You fought hard to stay here, but even while your body was failing you graced us with the most amazing gift. Through all the pain you experienced, you managed a beautiful smile from time to time. But, it was your steady, attentive gaze that gifted us with a remarkable sense of understanding, comfort and peace. The memory of that impressive gaze and peaceful, courageous countenance captured our heart and soul and links us through eternity.

We like to think that at age ten you are riding horses with Grandpa Nelson, building bird houses and fishing with Papa Avery, shopping with Aunt LaWanda, barbecuing with Papa Meigs and singing and dancing with Grandma Meigs in heaven.

We love you so much, Sweetie Pie. We thank God for blessing us with the forever gift of you.

Grammy and Gramps

This is birthday #10 Sweepea!! Can you tell me where the time went? In “my” mind, I still relive the undeniable peace and love I saw on your face “every” time I had the pleasure of holding you. Your soft whimper was a rare experience whenever you wanted a position change or a feeding.

I loved you on sight and never got enough of seeing you, holding you. One of the “proudest” moments of my life is when I got to “escort” you to the Essence Magazine set where your mom was being photographed. You were SO sweet, not a whimper and the sweetest face the world had ever seen. Everyone on the set could barely wait for the shoot to end, so they could come see you! :)

You were such a wonderful gift and I found myself pleading with God to make everything alright for you -- for us -- so you would be here on earth as long as anyone else.

Thank you for gracing our lives with your angelic presence -- even for two plus months. Happy “10 year old day,” Sweepea -- hugs, kisses and love “always” for your brief stay. NO ONE was ready to let you return to Him. But only He knows what was best for us all and we must thank Him for “every” gift He gives -- especially the gift of my Sweepea -- Alexandra Nina Meigs.

Give Grandpa Nelson a hug for me.

Blessings and love “always,”

Your Grandma Dorothy Nelson
Care Packages

These stacking cups were donated in honor of Audrey Wright’s birthday!

Thank you to the Brady Corporation for organizing a toy drive to support our newly diagnosed care package program.

These handmade blankets were donated in honor of Charlotte Facchini.

These toys were collected and donated in honor of Remi Sizemore’s 1st birthday!

Donation made in honor of Bryce and James Eakin.

This fun and lightweight play foam was donated in honor of Lincoln Wade Pray!

These fun dinosaurs were donated in honor of Lydia and Gabriel Peters!
Donation in honor of James Brown’s second birthday.

These wonderful items were donated in honor of Emma Wiley’s birthday!

Assorted toy donation in honor of Connor Gaddy.

Donation in honor of Zailynn Mars.

Assorted toy donation from the Fighting for Kaiden Foundation.

Donation in honor of Alison Wick’s birthday.

Donation in honor of Saydee Smith’s birthday.

Handmade toys in honor of Lincoln Wade Pray. These were donated from the Young Women Organization of the Church of Jesus Christ of Latter-day Saints in Lubbock, TX.

Donations made in honor of Graham Hennings’s birthday!
SHARING PHOTOS

Allori Uriegas
Audrey Lawton
Austin Starling

Bryce and James Eakin
Chance Lang

Declan Buchanan
Ezra Zamarripa
Ellie Carver

Max Lasko and Merrick Akins
Willow Kidwell
Takyla Lynch
SHARING PHOTOS

Lucy Zahn

Fiorena Fuentes and Jennifer Miller Smith

Ruby Sun

Rhydian and Lachlan Gregg

Sofia Marin

Winnie and Charlie Stanley

Matthew Kaczor

Ivy and Cody King

Kirra Lee
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.

<table>
<thead>
<tr>
<th>Alabama</th>
<th>Greater Florida</th>
<th>New Mexico</th>
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<tbody>
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<td>P.O. Box 7918</td>
<td>18885 State Road 54 #115</td>
<td>505.353.1128</td>
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<tr>
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<td>Lutz, FL 33558</td>
<td>Natasha Abruzzo, President</td>
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<td>205.979.6493</td>
<td>727.388.1888</td>
<td><a href="mailto:newmexico@curesma.org">newmexico@curesma.org</a></td>
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<td>Jennifer Patrick, President</td>
<td>Audra Butler, President</td>
<td><a href="mailto:greaterfl@curesma.org">greaterfl@curesma.org</a></td>
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<td>P.O. Box 200532</td>
<td>4 Rutland Avenue</td>
<td>540 Talbot Ave</td>
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<td>Anchorage, AK 99520</td>
<td>Rockville Centre, NY 11570</td>
<td>Santa Rosa, CA 95405</td>
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<td>907.720.5019</td>
<td>516.214.0348</td>
<td>707.571.8990</td>
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<tr>
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<td>Debbie Cuevas, President</td>
<td>David Sereni, President</td>
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<td>LeClaire, IA 52753</td>
<td>Metuchen, NJ 08840</td>
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<td>602.314.4902</td>
<td>515.986.2181</td>
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<td>479.737.7650</td>
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<td>Family Support Co-Chairs</td>
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<td>Presidents</td>
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<tr>
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<td></td>
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<th>Kansas City</th>
<th>Pacific Northwest</th>
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<td>252-303-0014</td>
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<td>P.O. Box 82</td>
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<td></td>
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<td>215.605.7856</td>
<td>P.O. Box 941</td>
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<tr>
<td>Holly Schafer, Vice President</td>
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<td>Fridley, MN 55432</td>
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<td>Rick &amp; Autumn Montoya,</td>
<td>Kate LaChapelle, President</td>
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<td>Knoxville, TN 37921</td>
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<td>Jennifer Miller Smith, President</td>
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<td>716.984.4233</td>
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<tr>
<td>Jessica White, Family Support Chair</td>
<td><a href="mailto:Virginia@curesma.org">Virginia@curesma.org</a></td>
<td>Mary Boguhn, Family</td>
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<td><a href="mailto:virginia@curesma.org">virginia@curesma.org</a></td>
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<th>Washington (Pacific Northwest)</th>
<th>Wisconsin</th>
<th>Western New York</th>
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<td>P.O. Box 82</td>
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<td>P.O. Box 444</td>
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<tr>
<td>Olalla, WA 98359</td>
<td>Franklin, WI 53132</td>
<td>North Tonawanda, NY 14120</td>
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<td>253.857.9355</td>
<td>414.324.0499</td>
<td>716.984.4233</td>
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<tr>
<td>Rozzie McClay, Family Support Co-Chair</td>
<td>Kate LaChapelle, President</td>
<td>Mary Boguhn, Family</td>
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<td><a href="mailto:pacwest@curesma.org">pacwest@curesma.org</a></td>
<td><a href="mailto:wisconsin@curesma.org">wisconsin@curesma.org</a></td>
<td>Support Chair</td>
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<tr>
<th>Cure SMA National Office</th>
<th>955 Busse Road</th>
<th>1-800.886.3762</th>
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<tr>
<td>Elk Grove Village, IL 60007</td>
<td>elkgrovevillage.curesma.org</td>
<td><a href="mailto:canoe@curesma.org">canoe@curesma.org</a></td>
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Chapter Highlights

Nick Saban’s Giveaway Luncheon

Alabama Chapter

On August 3, 2017, Nick’s Kids Foundation donated $1,000 to the Alabama Chapter of Cure SMA. Nick’s Kids Foundation was established by Coach Nick Saban and his wife Terry to raise awareness and resources for organizations throughout the Southeast. We are grateful for Coach Nick and Ms. Terry Saban’s continued support of Cure SMA over the last 10 years! We are also very appreciative of Alabama Chapter President, Jennifer Patrick and her family for attending this event to represent Cure SMA.

Arizona Chapter

Good Sam RV BBQ Showdown

The Arizona Chapter was thrilled to be selected as this year’s beneficiary of the proceeds from tickets sold at the Good Sam RV BBQ Showdown at Phoenix International Raceway in January 2017!

In January 2017, volunteers and SMA families sold tickets to the public for sample tastings from the competing barbecue teams. We had great fortune with the weather and with ticket sales, earning our chapter over $3,400! A BIG thanks goes out to the event coordinators, Angel Wolff and Stacie Smith, Mike Reimann, Arizona BBQ Catering, and all our volunteers!
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

Arizona Chapter Continued

Byrds FORE A Cure

The 4th Annual Byrds FORE A Cure scramble at Stonecreek Golf Club in Phoenix on May 6, 2017, was a great success! Together with community support, we raised over $12,000 for SMA research! It was a day of windy weather but also some great golf. A special thank you to Cassandra Johnson for spearheading this event! Also, a special thank you to the Byrd Family and Ken & Laurie Johnson for their generous prize donations and to our photographer, Bo Buchanan!

Cure SMA would also like to thank the generous local sponsors: AZ Barbeque Catering, Ron Ginter ChFC with Ameriprise Financial, Team 323 Realty, S.E.E.K Arizona, The Byrd Family, The Landscape Guy, Texas BBQ House, Kidz-R-Kool Dentistry, and Premier Development, as well as all of the golfers and volunteers who helped make the day possible!

We look forward to planning the 5th year of this event in 2018, so make sure to check the event calendar at CureSMA.org for any updates!

Arkansas Chapter

Family Day

On May 21, 2017, the Arkansas Chapter hosted its first SMA Family Day at Conway Human Development Center Park! Families joined together to make new friends while fishing, playing on the accessible playground and enjoying refreshments.

Carolinas Chapter

Cure SMA Casino Night in Honor of Gray’s Gang

North Carolina

The 3rd Annual Cure SMA Casino Night for Gray’s Gang took place on February 25, 2017, in Charlotte, NC. It was a great way to celebrate Gray’s senior year of high school. Local high schoolers participated in a casino-like experience that offered roulette, blackjack, poker, and craps. Not only did the kids enjoy a night packed with games, music and friends, they also had the opportunity to win awesome raffle prizes, and $19,240 was raised for Cure SMA!

Thank you to the Dougherty Family and Gray’s Gang for their hard work on this event and dedication to Cure SMA. A special thank you to Abbie Dougherty for chairing this event!

Fuddruckers Awareness Dinner

On Saturday, August 5th families and friends of the Arizona Cure SMA chapter gathered for dinner at Fuddrucker’s. This annual event is a great way for families to connect and help kick off SMA Awareness Month.
Charley–Walker Foundation New Year’s Eve Fundraiser

On December 31, 2016 the Charley-Walker Foundation donated $20,000 from their New Year’s Eve fundraiser in honor of Charlotte Swing. A huge thank you to Elizabeth Rogers for your generosity and amazing support for Cure SMA!

Raleigh Rock ‘n’ Roll Marathon & Half Marathon

This year’s marathon and half marathon took place on April 1st and 2nd in Raleigh, NC. It was a day filled with perseverance and hope. The Cure SMA team raised a total of $6,303.10 for SMA, which surpassed their original goal of $2,500! Thank you so much to our amazing runners, Nelson Amador, Kim Boyd, Kevin Chan, Evelyn Guerra, Greg Mitchell, Jeff Mitchell, Justin Mitchell, Kelsie Mitchell, Lisa Mitchell, Sarah Mitchell, Valerie Mitchell, Kelly Morrison, Kevin Morrison, Su Stone, Sophia Su, and Mimi Chan!

Hayes’ Heroes Present a Cure SMA Evening of Hope

The 2nd Annual Hayes’ Heroes Present a Cure SMA Evening of Hope, held in Lexington, SC took place on April 1, 2017. Family and friends gathered in honor of Hayes Wingard to raise over $24,880 for Cure SMA. Guests enjoyed appetizers, silent and live auctions, as well as an inspiring video featuring the families’ of Hayes Wingard and Leo Bugenske.

Thank you to Kathy Cooper and Joyce Sireno for all of their hard work, and a special thank you goes out to Keri Cooper for chairing this year’s event.

Northern California Chapter

Do it for Danny Walk-n-Roll

Danny McHale and his friends started fundraising for a cure for SMA back in 7th grade and haven’t stopped since! His family started with bake sales and lemonade stands, which became the foundation of the Cure SMA Club at Monte Vista High School. All of the students at Monte Vista have joined together in the fight against SMA through various car washes, t-shirt sales and home run derbies. The Do it for Danny Walk-n-Roll on April 23, 2017 was another great event that raised $3,798 and also helped to raise awareness of SMA in the community.
The 17th Annual Concert for a Cure was held on May 13, 2017 at the Diablo Country Club in Danville, CA and it was a tremendous success! This year’s event raised over $157,000 with the unique theme of #RoundUpForACure. 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 ISIS clinical trials.

The 40 plus high school students and their siblings were a huge hit as this marked their seventh 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. In 2003, Nancy and Andris Dindzans, joined in the efforts when their daughter Ariana, was diagnosed with SMA Type III. Both Danny and Ariana will turn 18 years old this year. Since this event began in 2001, it has raised over $1.7 million to fund Cure SMA research programs.

The McHale and Dindzans families send out their heartfelt THANK YOU to their friends, families, and local community for their generosity in their continuous support of SMA families and children everywhere!
2017 Cure SMA 530 Rust Car Show

The inaugural Cure SMA 530 Rust Car Show in Orland, CA, held in honor of Radyn and Thoryn Vaughan, was held on July 27, 2017. This great day of family entertainment raised $3,000. The day also featured an antique and vintage car show, food, vendors, raffle and more.

Thanks to Michelle Tedrick for hosting this event!

Southern California Chapter

Score4Sky

Score4Sky held their second adaptive sport event on January 14, 2017 in Newport Beach, CA, at Sage Hill School. The founder of Score4Sky, Spencer Bahrenburg, taught the group basketball skills with modifications.

Thank you, Spencer, for helping raise $650 for Cure SMA!

2017 Surf Away SMA with Ricochet

Cure SMA and IONIS Pharmaceuticals continued SMA Awareness month by hosting a free assisted surfing experience lead by Ricochet the SURFice dog and professional surfers on the beautiful beaches of La Jolla Shores in La Jolla, CA. On August 15, 2017, General Calhoun, John Cave, Riley and Bailey Sommerville, Neil Turner, Avery and Zane Wheeler, Bryce Young, Caleb Kobayashi, and Benjamin Lou surfed with Ricochet. What an incredible experience each our surfers had that day.

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

The Southern California Walk-n-Roll in Costa Mesa on April 8, 2017, was a blast! The morning started off with teams gathering at the start line for a great walk around the beautiful park. Over $13,000 was raised for Cure SMA!

Thank you to our supporters who assisted our teams: Familia Diego, Hammer Time, Matthew's Super Troopers, Neil's Giant Leaps, Princess Ava's Squad, Suzie's Angels, Team Caleb STRONG!, Team CoPo, Team Kennedy, Team Maelee, and Team Melissa.

Thank you to Rickk and Autumn Montoya for planning this great event!
3rd Annual Southern California Gala of Hope

On May 6, 2017, over 180 guests gathered at the Ritz-Carlton Laguna Niguel in Dana Point, CA for the 3rd Annual Southern California Cure SMA Gala of Hope. This incredible evening raised over $169,000 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 help find a cure for SMA.

The evening consisted of cocktails and hors d’oeuvres on the Promenade Lawn with a silent auction and live performance by James Ian, 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: National Premier Sponsors: Biogen and AveXis; Platinum Sponsors: Ionis Pharmaceuticals, Leaf Communication Consulting, Moreno Foundation and all other sponsors, in-kind donors, and media sponsors! Thank you to Nikki Reyes-McIntosh and the planning committee for all their support and dedication. This event was a huge success, thanks for your hard work!
San Diego Rock n’ Roll Marathon

Team Cure SMA participated in a Rock ‘n’ Roll Marathon Series held in San Diego on June 4, 2017, in downtown San Diego, CA. Everyone teamed up to run the San Diego Rock ‘n’ Roll Marathon and Half Marathon to raise money and awareness for team Cure SMA. In total, Team Cure SMA raised over $5,000 to find a cure for SMA.

Thank you to our runners, Angela King, Carli Katz, Gigi Dryer, Howard Katz, and Scott Koblentz!

24th Annual Chesapeake Crab Feast and Silent Auction

A special thanks to all friends, SMA families, and coworkers who came together at the American Legion Hall in Towson, MD on August 6, 2017 to bring awareness and raise money for Cure SMA. The event raised over $35,000 for the Chesapeake Chapter and the Erin Trainor Memorial Fund. Over 350 people enjoyed the delicious crabs & beer, bidding on auction items and playing the wheels. This event would not be possible without our crab servers, many of whom were friends and family of the Lewis Family and Gabrielle Trainor. In addition, thanks to The Knights of Columbus of Glyndon, MD who continue to show their devoted commitment to Cure SMA each year with the volunteers who run the wheels. Another thank you, to all who donated to the wonderful auction. And finally, thank you to Barb and Gene Trainor, John and Katy Nolan, and Bev and Dan Venedam who have continued to make the Crab Feast a huge success the past 24 years.

A Way to Cure SMA Maryland

The Camino de Santiago is one of the most famous pilgrimage walks in the world. Many thousands walk it each year and all walk for their own personal journey. To walk the entire passage from St. Jean Pied de Port to Santiago de Compostela is a 490 mile journey. From April 22 to June 4, 2017, Barb and Gene Trainor took this pilgrimage both in memory of their daughter Erin and in thanksgiving and celebration of the first FDA approved treatment for SMA. Since 1994, Barb and Gene have passionately dedicated their lives to help with the support of families who live with this disease and for researchers who tirelessly work for a cure.

Thank you to the Trainor family and all those who supported them on their journey. Through the support of their friends and family, Barb and Gene raised $57,950 for the Erin Trainor Memorial Fund to provide scholarships for newly diagnosed SMA families to attend The Annual SMA Conference.
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

Gala of Hope for Kamydn’s Kure

The Gala of Hope for Kamydn’s Kure was held on April 7, 2017 at Green Grove Gardens in Greencastle, PA. The night entailed cocktail hour with the South Western High School string octet, dinner, dancing, and a red carpet photo booth. There was a silent and live auction made possible by the generous donations of family, friends and local businesses. The night was a huge success and a wonderful time was had by all who attended. Nearly $21,000 was raised to support Cure SMA!

The event was hosted by Travis and Amber Hartung in honor of their 5 year old son, Kamydn who was diagnosed at 13 months old. A special thanks to the family, friends and volunteers who dedicated their time to help make the gala amazing.

Lastly, thank you to the local sponsors: Steve & Pam Cramer, Brad Helmuth Drywall Services, Cumberland Valley Dental Care, RS Entertainment, DJ Rello, DuCar Farms, and Scratch Catering.

2017 Chesapeake Chapter Walk-n-Roll

On April 22, 2017, 16 teams of walkers and rollers gathered in Mount Airy, Maryland, at Old National Pike Park to raise money to support the ongoing efforts of Cure SMA. Over 150 people registered for the event that was organized by Cure SMA’s Chesapeake Chapter. Despite the rain, many attendees completed the one-mile loop around the park, had their faces painted by “Flo’s Faces,” listened to great music played by DJ Alfie, and were well fed by Larry Jackson’s “Bullhead Pit Beef.” The annual walk-and-roll was again a great success and raised more than $15,000. Additional thanks must be offered to Stacey Markel who donated her time to photograph the teams and event (staceymarkelphotography.com). Our gratitude goes out to all of the teams who attended and made it special: Johns Hopkins Homecare Group, Mica’s Milers, Team Aiden James, Team Allison, Team Camden O, Team Emmy, Team Erin Go Bragh, Team Jase, Team Kaiden, Team Mayse, Team Nora, Team Olivia, Zooming 4 Zoey, Mia’s Millers, Team Morgan and Team JLew.

Bull Roast Fundraiser in Honor of Camden O’Rear

On April 22, 2017 at the Tall Cedars Hall in Baltimore, MD, over 250 attendees came out to support the Bull Roast in honor of Camden O’Rear. Thank you to everyone who donated, helped out with the event, as well as all those who attended the benefit Bull Roast for SMA. A special thanks to Ashley O’Rear-Hardeesty, Dan Hardeesty, Theresa Cox, Sandy Hughes- O’Rear, Amanda Warren, Lissa Gotti, Shawn Taylor, Jamie Bentkowski Swann, Jack Atkins & Kanye Twitty, and Liz Johnson for all you did. Without all of your help, the event would not happen. It was a successful day with many laughs and some tears as well. We can’t wait until next year!

Finally, thank you to Robyn and Tim O’Rear, for organizing this event, and raising $2,250 in honor of their son, Camden, and for all the other children that have lost their battle, or continue to fight SMA.

On Sunday April 23, 2017, over 250 attendees came out to support the Bull Roast in honor of Camden O’Rear. Thank you to everyone who donated, helped out with the event, as well as all those who attended the benefit Bull Roast for SMA. A special thanks to Ashley O’Rear-Hardeesty, Dan Hardeesty, Theresa Cox, Sandy Hughes- O’Rear, Amanda Warren, Lissa Gotti, Shawn Taylor, Jamie Bentkowski Swann, Jack Atkins & Kanye Twitty, and Liz Johnson for all you did. Without all of your help, the event would not happen. It was a successful day with many laughs and some tears as well. We can’t wait until next year!

Finally, thank you to Robyn and Tim O’Rear, for organizing this event, and raising $2,250 in honor of their son, Camden, and for all the other children that have lost their battle, or continue to fight SMA.
On May 20, 2017 the Chesapeake Chapter held its second annual West Virginia Walk-n-Roll in Star City, WV, raising nearly $20,000. Over 150 walkers participated in the walk, with several families walking in memory of their children lost to SMA and many walking in support of their children living with SMA. People came from across West Virginia as well as the surrounding states of Pennsylvania, Ohio, and Maryland. The attendees enjoyed a beautiful, sunny morning filled with music, face painting, a raffle, and most importantly, coming together to make connections and friendships with other SMA affected families.

Thank you to all the volunteers and participants who made the event a success, especially the walk committee; Caressa Delaney, Julie Coddington, Ashley Close, Janice Kress, and Autum Johnson. We are looking forward to the 2018 Walk-n-Roll!

Steamboat Landing Bingo Fundraiser

Steamboat Landing Camp Ground in Milton, DE had another successful Bingo this year on August 5, 2017. Thank you to all those who attended and to the owners of Steamboat Landing, Rusty and Linda Pride, for making this event possible and raising $2,000 in honor of Olivia Calvert.

Orioles SMA Awareness Day

Thank you to everyone who came out to watch the Orioles take on the LA Angels on August 20, 2017! It was a beautiful day at Camden Yards and the Chesapeake Chapter raised over $200 in support of Cure SMA.
CHAPTER UPDATES

Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

Connecticut Chapter

6th Annual Cubby’s Run

The 6th Annual Cubby’s Run was held on May 6, 2017 at Ridgefield Recreation Center Trail in Ridgefield, Connecticut. Thanks to the hard work and dedication of Ethan Hynes, the event organizer, this Walk-n-Roll raised over $12,500!

Ethan organizes this event every year in honor of his childhood friend Cubby Wax, who has SMA. Cubby has graduated high school and is moving on to better things at college this year! Thanks to all of our supporters, our total fundraising is over $120,000! We’re excited to continue and push our total even higher next year.

We hope to see everyone again in May 2018 for the 7th Annual Cubby’s Run! If you have any questions or want to get involved, please email ethanhynes@me.com.

2017 Connecticut Chapter Walk-n-Roll

The Connecticut Chapter Walk-n-Roll was held on May 21, 2017 at Northwest Park in Manchester, CT. It was two figure eight loops that lead walkers through Union Pond, the playground and baseball fields. Families, friends, coworkers, doctors, and nurses all came out to show their support. There was an incredible display of generosity on the raffle tables with items from all over Connecticut. The turnout more than doubled from the 2016 Walk-n-Roll and this year raised over $5,500!

Special thanks to our event organizer Macie Tozzoli for her hard work and dedication to making this event successful! Looking forward to seeing everyone again next year!

Greater Florida Chapter

SMA Awareness Day with the Tampa Bay Rays

Friends and family members came together on August 5, 2017 to watch the Tampa Bay Rays take on the Milwaukee Brewers and to raise SMA awareness at the annual, “SMA Day with the Tampa Bay Rays.” The Rays donated a portion of the ticket price to Cure SMA, which totaled $350!
On February 24, 2017 over 200 guests gathered at the Ivy Room in Chicago, IL for the 2nd annual Cure SMA Chicago Gala of Hope. This incredible evening raised over $103,000 in support of Cure SMA. This fun event brought together families, researchers, doctors, business leaders, as well as generous supporters to raise funds to find a treatment and cure for SMA. The evening featured cocktails, hors d’oeuvres, a silent auction, live auction, and live entertainment. Comedian Brett Walkow was the MC and there was a live performance from the band Shout Out, who kept everyone out on the dance floor all night! A special thank you to our title sponsor AveXis for their generous support of the 2nd annual Chicago Gala. Cure SMA would also like to thank the other sponsors who generously supported this event: Biogen, Ivy Room, Aon, Angel Baby Foundation, Amy Lenahan, and Atlas Forms & Graphics Inc. Thank you to all of the committee members and volunteers for their hard work, dedication and commitment to the success of this event, especially Maria Marusich and the O’Brien family and friends.
In 2007, 3rd grader Kyra, wanted to help her friend Angie, by raising money to find a cure for her disease, SMA, so they formed Angie’s Hope. It all started with a penny drive and since then they have hosted multiple events such as garage sales, dinner parties and big ball soccer tournaments to raise funds for a cure.

This past year for their 10th annual event, when they were seniors in high school, they hosted an exciting “PROMise to Cure SMA” gala in Naperville, IL. The “PROM” featured music, hors d’oeuvres, a silent auction, raffle, and lots of dancing!

It was a wonderful celebration of what they have accomplished over the past 10 years through Angie’s Hope with raising awareness and funds, and in total they raised an incredible $55,000!

What started as a penny drive with a goal to raise $200 resulted in a decade long, community led effort that would raise $250,000 with their 10th year event! After 10 years of hard work these teens are celebrating amazing advancements in research and they are all graduating from high school this year with the knowledge that when they work together they can make a difference!
**11th Annual Illinois Chapter Walk-n-Roll**

The Illinois Chapter hosted the 11th Annual Illinois Chapter Walk-n-Roll on June 11, 2017, at Independence Grove in Libertyville, IL. The sun was shining all day and we were very fortunate to enjoy a beautiful day for this fun-filled event to raise awareness and funds for SMA!

Over 400 people gathered at the event for a day of fun! Everyone enjoyed the walk, DJ, face painter, balloons, and a t-shirt art station.

We would like to give a special thanks to all of our amazing teams, participants, sponsors and donors for making our 11th Annual Illinois Walk a huge success. Due to everyone’s efforts over $49,000 was raised and a total of 22 teams participated! Congratulations to Team Taylor for being the top fundraising team and for having the largest team with 47 walkers!

Cure SMA would like to give a big thank you to the walk’s event organizers, Janet Schoenborn and Danielle Plotke! They help make this event happen and made it such a great day and opportunity for all of the Illinois family and friends to come together.

A special thank you to Cynthia Annel, Jodi Garvey, Maria Marusich, Susan Pavlow, Tina Krajewski and everyone else who helped plan this year’s event. We hope to see everyone at the 12th Annual Illinois Chapter Walk-n-Roll!

**2017 Links for Leani Golf Outing**

Links for Leani was held May 22, 2017, in Quincy, Illinois at the Cedar Crest Country Club. A team of five people helped with the planning and even more helped the day of the event, including several who came from out of town to do so. Cedar Crest Country Club gave us a great deal on golf and lunch. Many companies and individuals sponsored with donations and prizes. Over 20 teams of 4 participated.

It was a fun day. Leani got to be there and was, of course, the star of the day. $8,000 was raised, which was split between Cure SMA and Leani’s parents to help with their expenses.

I am so grateful to all who made this happen, including Shannon O’Brien and Cure SMA who continue to be such great support to Leani, Nichole and Dwight.

Ron Fritts- Quincy, IL

Cure SMA would like to thank everyone at AveXis for their continuous and generous support of our organization and the SMA community.

**AveXis SMA Awareness Month Fundraising Challenge**

Throughout SMA Awareness Month, AveXis created a fundraising challenge for their employees.

During this challenge AveXis employees were able to raise $15,000 to support Cure SMA! Thanks to a generous match from AveXis their donations were doubled and they helped us raise $30,000 for Cure SMA!

Cure SMA would like to thank everyone at AveXis for their continuous and generous support of our organization and the SMA community.

**2017 Bank of America Shamrock Shuffle 8K**

Members of Team Cure SMA ran the Bank of America Shamrock Shuffle 8K on April 2, 2017, in Chicago, Illinois. This was the second year that Cure SMA participated in the run as an official charity partner. Together, the runners raised $2,065 to help Cure SMA! Cure SMA would like to thank all of our runners; Jessica Colella, Melonie Behrens, Traci O’Brien and Laura Jacobs 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 August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

2017 Cure SMA Day with the Chicago Cubs

The Illinois Chapter had a great night on August 2, 2017, at Wrigley Field in Chicago celebrating SMA Awareness Month at the 2nd annual Cure SMA Day with the Chicago Cubs. There was an incredible turnout with 250 family members and friends coming together to raise awareness for SMA and to cheer on the Chicago Cubs! We are looking forward to doing this event again next year!

Father/Daughter Dance in Memory of Ava

Ashten Pottenbaum hosted a father/daughter dance on March 3, 2017, in memory of Ava Adams who gained her wings in 2016. Ava’s parents are Ben and Krista Adams of Vail, IA and her proud sister is Grace. There were over 90 girls, about 70 fathers/uncles/brothers/friends etc. attended the dance, and roughly 150 spectators attended the Grand March. The attendees were able to make princess crowns, participate in a limbo contest, register for raffle items, enjoy cookies and juice, and danced the day away! Many local businesses, family members, and community members made generous donations to Cure SMA. The dance raised over $3,500!

2017 Evening of Hope in honor of Miracle for Madi

The 2nd Annual Evening of Hope in Honor of Miracle for Madi took place on March 1, 2017, at the Rogalski Center in Davenport, IA. This event is in honor of Madi Ramirez, SMA type III. The dinner event featured a silent and live auction, a sip ‘n sparkle, a step and repeat for guests to take pictures, a plated dinner, and dueling pianos to raise money for Cure SMA.

The event was a huge success, raising over $22,000 for Cure SMA!

A big thank you to Megan Ramirez and her planning committee for organizing this event and making it such a great success!

2017 St. Louis Walk-n-Roll

The 2017 St. Louis Walk-n-Roll took place on May 21, 2017. Many SMA families gathered together to support the event and enjoyed food, music, and a 5K walk and run around Tower Grove Park in St. Louis, MO.

The event was a great success, raising over $15,000 in its second year! Thank you to everyone who supported and attended the St. Louis Walk-n-Roll! Thank you to Janet Hutchinson and Julie Gutierrez for helping organize this walk!
Gracie’s 6th Annual Steak Dinner

Gracie’s 6th Annual Steak Dinner for SMA took place on April 29, 2017, in memory of Gracie DeGraffenreid, SMA type I. Friends and families from Eugen, MO gathered to raise awareness of SMA and funds for Cure SMA in Gracie’s memory. Thanks to the generosity of everyone who attended, $7,713 was raised for Cure SMA. Thank you to Sherry DeGraffenreid for planning this event in memory of Gracie every year!

SMA Awareness Day with the Portland Sea Dogs

The families in Maine had great day at Hadlock Field on August 6, 2017, celebrating SMA Awareness month! More than 20 friends and families attended the game. It was great to have both long time families and some new friends gather for some fun and awareness of SMA. Thank you to everyone who made this 4th annual event a success! We are looking forward to doing it again in 2018 and hope you can join us!

2017 Evening of Hope Singer-Songwriter Night

On April 27, 2017, the Louisiana Chapter hosted the 2nd annual 2017 Evening of Hope Singer-Songwriter 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 songwriters Irene Kelley and Clay Bordreaux. Guests from all over the area attended the event which raised more than $28,000 for SMA research and family support programs.

A special thank you to Krista Scurria for chairing the event, as well as our dedicated committee members, amazing volunteers and generous sponsors. Your support made this night possible and continues to lead Cure SMA closer to a cure for SMA.

3rd Annual Nico’s Night Out

On April 29, 2017, the 3rd Annual Nico’s Night Out Gala was held at the Double Tree in Bedford, Massachusetts. Over $22,500 was raised that evening for SMA research! Attendees enjoyed a fabulous evening of dining, dancing, raffles, and a silent auction. After dinner, attendees were treated to a performance by a Frank Sinatra impersonator, a staple of this event year after year!

A special thanks to Susie Sapienza, the event organizer who has worked tirelessly for several years now to make this event a yearly success! Also, thank you to all of our sponsors, attendees, and volunteers who all helped make this day possible! Save the date for our 4th Annual Nico’s Night Out Gala on April 28, 2018!
Chapter Updates

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17th Annual New England Chapter Walk-n-Roll

The New England Chapter held their 17th Annual Cure SMA Walk-n-Roll on May 20, 2017, at DCR Wompatuck State Park in Hingham, Massachusetts. Team Murphy, in memory of Cianan and Cecilia; the Farrell family, in honor of Team Aileen; and Team Norton, in honor of Owen; as well as more than twenty other teams and affected families came together. In total, there were over 500 participants who raised over $125,000!

This event is not only a fundraiser for Cure SMA, but also an annual reunion of SMA families from throughout New England. Teams that raised over $10,000 this year included: Team Murphy, Mac’s Pack, Kelsey’s Krew, and Walking for William. Other big fundraisers were Wizards of OZ, Team Biogen, Team Norton, Chloe’s Club, Team Aileen, and Team Tyler. Teams that raised over $1,000 included Mirabile Mania, Team Tamara, Addison Barrett, Angelica Marie, Team Evan, and Team Melanie Lee. Also participating were Mikaela’s Marchers, Glen’s Gang, Team Rose, Team BraeKer, Team Rogocup, Powell, Team Ian, and Believe in Bodhi. Other SMA individuals also participated or were represented by loved ones.

The walk route was 1.5 miles on paved paths. Everyone appreciated the refreshments and a terrific raffle. Children enjoyed face-painting, a very talented balloon artist, and carnival games with prizes. The event was made possible by the many volunteers who helped run the registration, refreshments, raffle and game areas, and by the local businesses and friends who donate most of the supplies, refreshments, and raffle items. Special thanks goes out to Silvia Murphy for all of her hard work in making this event a success year after year!

We hope to see everyone again in May of 2018!
**2017 Rogo Cup to Cure SMA Golf Tournament**

The 5th Annual Rogo Cup to Cure SMA Golf Tournament and online fundraiser was held on June 5, 2017, at the Atlantic Country Club in Plymouth, MA. The Rogowicz, Butler, Spiegel and Scully families hold the tournament in memory of Mark Butler, in honor of Billy Spiegel, and Lauren Rogowicz. Our guests enjoyed a great round of golf, dinner, prizes, raffles, and auction items. We are so very thankful to all our sponsors, donors, golfers and volunteers for their continued support of our families and all those affected by SMA.

Special thanks goes out to the families listed above who dedicated their time and energy to making this event a success year after year. With their support, we raised over $33,000 this year, bringing the five year total to over $185,000 in support of Cure SMA. Save the date for June 4, 2018 for another great day as we work toward a cure for SMA.

**SMA Awareness Day with the Lowell Spinners**

The families in Massachusetts had great day cheering on the Lowell Spinners on August 13, 2017, as they took on the Auburn Doubledays at the fully-accessible LeLacheur Park celebrating SMA Awareness month! It was great to have both long time families as well as new ones gather for some fun and awareness-raising. Thank you to everyone who made this fourth annual event a success! We are looking forward to doing it again in 2018 and hope you can join us!

**2017 Providence Marathon Rhode Island**

On May 7, 2017, Team Cure SMA participated for the first year in the 2017 Providence Marathon in Providence, Rhode Island. We had five runners who raised over $1,000 for Cure SMA! A big thank you goes out to event organizer, Wayne Cornett, for recruiting runners and helping this event run smoothly. Congratulations and thank you to Team Cure SMA for their hard work training and fundraising!
3rd Annual Vermont Walk-n-Roll

The 3rd Annual Cure SMA Vermont Cure Walk-n-Roll took place on May 13, 2017, at Oakledge Park in Burlington, Vermont. It was a perfect day to walk along the shores of Lake Champlain while enjoying time with family and friends, meeting new people, and knowing that we were raising funds and awareness for Cure SMA. Awesome raffles, snacks and an appearance by Wally the Lake Monster rounded out the day.

A special thanks to Sue O’Neill for all of her help in putting this event together, which raised over $9,500 for Cure SMA! We are looking forward to our 4th Annual Walk-n-Roll in spring 2018!

Fifth Third River Bank Run with Team Cure SMA

It was a beautiful day to run the streets of Grand Rapids! The Fifth Third River Bank Run was a huge success! We cannot thank all of our amazing runners enough who helped us to raise $1,570! Cure SMA would like to thank Corrie Elsbrie, Jennifer Hughes, Dustin Hughes, Lisa Wilson, Paula Jonsson, and Keith Gohlke who helped to raise awareness about SMA during the race!

We would love to have you be a part of Team Cure SMA as we participate for our third year with the Fifth Third River Bank Run for their 41st race on May 12, 2018. Join us in the 5k run or community walk, the 10k run, or the infamou 25k!
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

**Detroit Pistons**

A huge thank you to the Dorer family for their help with the Detroit Pistons Game Fundraiser! For each ticket that was sold for the game on January 21, 2017, the Detroit Pistons donated $5 to Cure SMA, which help raised $175! It was a great time to enjoy basketball while showing support for SMA!

**Team Cure SMA at the Lake Minnetonka Half Marathon**

This was the second year there was a team for Cure SMA to participate in the Lake Minnetonka Half Marathon on May 7, 2017, in Wayzata, MN. The event raised over $4,535 for Cure SMA! Thank you to our runners Dan Fox, Trent Hillerud, Matt Bigelow, and Jeff Andrade who participated in the half marathon and to everyone who supported our runners!

**Donation in Honor of Isla Smith**

Thank you to Nick Schnieder, and Ross and Katie Smith for their generous contribution of $18,000 in memory of their daughter, Isla Smith.

**Andersen Windows Awareness Month Fundraisers**

Thank you to the Andersen Corporation and the Fun and Engagement Team for donating $125 and choosing Cure SMA as their charity for the month of August 2017!

**Kayley Shade Fundraiser**

At the Season Home Opener on February 17, the Sioux Falls Storm hosted a special fundraiser for Cure SMA on behalf of Kayley Shade, a Storm employee. Kayley raised $5,277 for Cure SMA from an evening of fun that included a silent auction and the opportunity to win cool prizes!
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

Greater New York Chapter

2017 Greater NY Chapter Golf Outing

The Greater NY Chapter Golf Outing was held on Monday, May 22, 2017, at The Hamlet Golf and Country Club in Commack, NY. Despite the rain, the event was a great success and raised $30,458. After the golfers braved the rain, everyone joined together for a nice dinner and raffle.

Special thanks to our sponsors: Shearman & Sterling LLP, Allen & Overy, Latham & Watkins, Lincoln Computer Services, Strategies for Wealth, Brookes & Gorria Search, the Cuevas & Martin Family, the Florida Skylanders, Joan & Felix Cutrone, the Lozina Family, and Print Café.

Huge thank you to Debbie Cuevas, Catherine Martin, Erin Bonner and Genevieve Fitzpatrick for organizing this wonderful event for Cure SMA!

Emma and Heather’s Lemonade Stand

Throughout the summer months, Emma Garrett and Heather Cuevas work hard to organize lemonade, candy and bake sales to support Cure SMA in their hometown of Rockville Centre, NY. Emma has done this for the past four summers and the last two has been joined by Heather. Both ladies are hoping the money they raise will help find a cure for this terrible disease which effects Heather’s brother Dylan. Through a lot of hard work, a good marketing plan and a nice hot sunny summer day they were able to collect and donate $500. This was the most successful sale by far and the girls hope their next sale will be even more successful.

2017 United Airlines NYC Half Marathon

Three incredible runners came together to run for Team Cure SMA in the 2017 United Airlines New York City Half Marathon to benefit Cure SMA on March 19, 2017. The runners trained hard and raised $4,185 to support critical SMA research and family support programs!

Special thanks to our runners: Jillian Ament, Kara Hartnett, and Liz Wesley.
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

### Capital Region Satellite Chapter

#### 2017 Ice Fishing Tournament

NYS Trooper David Cunniff had a vision, a goal of raising a million dollars to help find a cure for SMA. His son Caleb is affected by SMA. He and his wife Amy started and led the New York Capital Region Chapter of Cure SMA until his untimely ‘line of duty’ death in December of 2013. Amy continues to lead this chapter, and continues with his dream.

On January 28, 2017, The Docksider Restaurant on Glen Lake in Lake George, NY, held the 4th Annual Ice Fishing Tournament. $3,417 was raised by the Signal 30 Benefit Fund in honor of David Cunniff and Timothy Pratt. Our appreciation and thanks go out to Christine at The Docksider Restaurant for organizing this event, the prize donors, and all who came out to support the 2017 event.

### North Jersey Chapter

#### Dance Away SMA

On April 22, 2017, individuals gathered at the Far Hills Country Day School in Far Hills, NJ for a dance performance by In Motion and In Motion II Dance Companies to help find a cure for spinal muscular atrophy. The event raised over $3,900 to help fund Cure SMA’s research efforts and program services. Thank you to Kathie Kececi for spearheading this event year after year!

#### 6th Annual Best Meatball Contest in Secaucus

The 6th Annual Best Meatball Contest was held on April 29, 2017, at the Immaculate Conception Church Gym in Secaucus, NJ. This year’s event was hosted by the local Knights of Columbus and is held in honor of Daniel Cevallos. In total, $4,430 was raised for SMA research! Thank you to Giovanni Recalde and Paulina Recalde for organizing this event!
Chapter Updates

Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

Cure SMA Walk-n-Roll in Honor of Katherine Santiago

On May 6, 2017, the Walk-n-Roll in Honor of Katherine Santiago took place in Milburn, NJ, raising over $4,500. Katherine’s Walk began in 2008, and has been a part of my life for as long as I can remember. For the majority of my life, I’ve grown up with the SMA community. This walk was initially for Katherine - my sister, a best friend, a daughter, and a passionate Harry Potter fan.

After my sister passed away, her best friend Allie started this walk as part of her bat mitzvah project. As a shy first grader, I looked up to Allie; always wondering how I could ever run this walk with as much kindness and confidence. It is because of Allie’s confidence, kindness, and humor that the walk continues. When Allie left for college, she left the walk in my hands.

The walk has taught me so much - not just how to run a fundraiser, but how to be confident and work well with others. The walk has become part of my identity and as May rolls around each year, I become excited to share our cause with visitors.

Ten years later, Cure SMA has its first FDA approved drug, Spinraza, our walk has grown, and so have I. My family and I recently attended this year’s SMA conference as volunteers. There, I met SMA children, and children who had SMA siblings like me. As I was playing with them, I was able to create friendships and bonds with everyone there. The SMA community has never been stronger. This was my first conference in years, and for the first time in a while, I felt at home surrounded by others who all shared a common bond. We all have one goal - striving for the cure.

Julia Santiago | Milburn, NJ

Thank you to Alma, Julia, and Efren Santiago for chairing this event. Also, thank you to all the volunteers, participants, and supporters who have supported this event over the years.

Walk-n-Roll in Honor of Matthew Munch

On May 13, 2017, first graders from Shongum School in Randolph, NJ gathered with their families to walk or roll in honor of their fellow classmate, Matthew Munch. Despite the rain, over 200 participants gathered in the school gym and raised over $3,300 in support of Cure SMA. This wonderful event was organized and led by Matthew’s teacher, Mrs. Miessler; thank you!

2017 Cure SMA North Brunswick Walk-n-Roll

The Cure SMA North Jersey Chapter hosted the 2nd Annual Walk-n-Roll on June 25, 2017, at the North Brunswick Community Park in North Brunswick, NJ. The Walk-n-Roll raised over $5,400 to help find a cure for SMA. Thank you to all the dedicated teams and individual participants who helped make this day a success. The fun-filled event included activities for the whole family to enjoy, such as a balloon artist, face painting, games, and more!

Thank you to all of the amazing SMA families, participants, and donors for helping make this event possible. Big thanks to all of our volunteers who dedicated their time on event day to ensure everything ran smoothly.

A special thank you to Anna Pham, Kristen Smith, Kara Hartnett, and Dawn Cusack for all your hard work in planning this fun event! We look forward to having the 3rd Annual Walk-n-Roll in June 2018.
In its 5th year, the Deacon Alexander Memorial Car Show was held at First Baptist Church of Walton on August 12, 2017. This year we had a great turnout with almost 50 cars present and $1,200 was raised for Cure SMA! Thank you to Amanda Perry, her family, church family, and friends for making this happen every year.

In August 2017, Graves Gilbert Clinic of Bowling Green, KY chose to support Cure SMA through a t-shirt fundraiser. Employees were able to raise awareness by wearing their pink or green Annie shirts to work on Fridays. Over 300 shirts were sold and $3,251 was donated to Cure SMA in memory of Annie Grimes. A huge thank you to Eddy, Nicole, Garrett, Lainey & Emi Grimes, APRN, Jaime Duvall and the Graves Gilbert Clinic for your amazing work and support!

Jet Express hosted Will’s Day at the Bay in memory of Will Blumensaadt. The event was held on June 7, 2017, at Derivera Park in Put-In-Bay, Ohio and raised $3,000 for Cure SMA! The event featured round-trip Jet Express tickets. It was a wonderful day of family fun while raising money for SMA! Will’s father, Todd Blumensaadt said, “This is really a special event for me and the whole Jet Crew. By supporting research on this disease, hopefully we can make a difference for children and families suffering from spinal muscular atrophy.” A special thank you to Todd and the entire Jet Express Crew for their efforts and continued support!

Thank you to the Martin Family for raising $600 in honor of Lewis for Cure SMA during their bake sale in Bainbridge, OH on September 1, 2017. We truly appreciate your dedication and support!

Anspach Law Firm established fundraising efforts in support of Cure SMA in the communities of each one of their offices. Their employees made donations which were then generously matched by the firm for a grand total of $200. This year the Toledo, Ohio office chose to donate their funds to the Cure SMA – OKI Chapter. Thank you to the Anspach Law firm for their support of Cure SMA!

The Farrell family held its 6th Annual Birdies for Blake on July 29, 2017 at Green Crest Golf Course in Liberty Township, OH. There were over 100 golfers, and they raised $30,375 for SMA research and family support – their highest total ever! The day was filled with hope as attendees played an 18-hole scramble and then enjoyed dinner in honor of Blake Farrell.

Blake is a vibrant, beautiful, fun-loving 6 year-old girl who likes the same things all her friends do. She loves to dance, sing, play, laugh, and tell a joke. She loves princesses, horses, her pets, and her sisters.

Thank you to the entire Farrell family for all that you do and your continued support! We would also like to thank all of the golfers, volunteers, and sponsors who helped make this year’s event a huge success!
Congratulations to the Cincinnati Walk-n-Roll committee for raising over $1 million for Cure SMA! Reaching this incredible milestone would not have been possible without the dedication of the planning committee and the entire OKI (Ohio, Kentucky, Indiana) Chapter. This year’s event was held on May 6, 2017, inside Xavier University’s Cintas Center in Cincinnati, OH. The event was a huge success raising $84,386 for SMA research and family support! Beth Lockwood says their success can be credited to, “Having faithful people supporting us year after year to take ownership of major components such as the raffle, DJ, graphics, food, set up, registration, etc.” This event is always filled with fun family activities, lots of fundraising, and most importantly, hope. This year the kids were able to enjoy a petting zoo! The event took place inside the Cintas Center due to weather, but that didn’t stop us from having a great time.

A huge thank you to Beth & Kevin Lockwood, Bob & Elizabeth Lockwood, and Nicole, Mark and Amy Haake! Every year they spend countless hours soliciting sponsors, supporting families, and organizing logistics to make the Walk-n-Roll a success. A special thank you to Ron & Janet Merkle, Marilyn Pater, Gloria Logeman and Linda Schwabb for their important roles in this event. Thank you to Stephanie Noll for her role in putting together the beautiful butterfly tree in memory of those we have lost to SMA. We would also like to thank our raffle and food committees for all of their help! We truly appreciate all the countless teams, families, sponsors and volunteers who helped make this event a success.
We could not have asked for a better evening and turnout for the first ever Cure SMA Walk for Graham that took place on Friday evening, May 12, 2017. Not only did the number of people attending exceed our expectations of over 500 but the amount of money raised during the evening was absolutely amazing. Everyone helped to bring in over $79,000 to support all families affected by SMA and fund research!

The evening was action-packed as we started the event with a balloon release and one mile walk around the beautiful Coxhall Gardens in Carmel, Indiana. After the walkers returned from the loop there were stations all over for the kids to bop around to such things as balloon artists, a tattoo station, the nickel toss game, a book sale, a popcorn station, and kids obstacle course put on by NinjaZone. While these fun activities were going on throughout the evening, participants also enjoyed some yummy ball park food and beverages that was so generously donated by Sysco, and an array of silent auctions items ranging from a signed Colts jersey, wine baskets, golf packages, kid themes, and so many others! We had glow sticks, restaurant raffle giveaways, and goodie bags just to add a bit more to the fun!

This amazing and huge event to celebrate Graham Vollmer’s 2nd birthday would not have happened without all of the amazing support from the community, donors, sponsors, volunteers, and everyone in between.

Cure SMA would like to especially thank the amazing committee of women who helped get everything in order before, during, and after the event. You are a bunch of wonderful ladies! Also, an enormous thank you to Adrienne, Nick, Ben, Lucy, and Graham Vollmer for the time, energy, and enthusiasm you put into making this event a great time for everyone while raising awareness about SMA and funds to support so many other families in the community!

To check out the fun we had at the event look through pictures by searching #WalkforGraham or watch this great video: https://vimeo.com/217290867, and join us for next year’s evening in May 2018!

On August 10 -12, 2017, the annual Wannabe Cup Charity Golf Tournament was held at the Black Butte Golf Course in Sisters, OR. The event consisted of two days of golf including a first match and final match with closing ceremonies and awards. In its 19th year the Wannabe Cup raised over $13,100! A special thank you to Joe Belcher and Andy Belcher for organizing this annual golf tournament in memory of Skylar Bahrenburg.
On March 19, 2017, we celebrated the 5th Annual Muscles for Mckenna Gala! This year’s event was held at the stunning Crystal Tea Room in Philadelphia, PA and raised over $90,000. Each year this lively event is held in honor of Mckenna Ellixson.

Everyone enjoyed an evening full of fun with live music by The Heartbeats. This year we were thrilled to have dancers from Emerald Isle perform Irish dances for St. Patrick’s Day! The audience enjoyed our spirited live auction, taking photos at our selfie station, and tasting our signature drink, The Irish Mckenna.

Two SMA families shared their stories during this year’s event. Jim Ellixson shared his daughter’s journey with SMA and talked about the approval of the first-ever approved treatment for SMA. Kelly Jankowski shared the impact that Cure SMA had on her family after her son’s diagnosis.

Thank you to Kellie Keenan for chairing this year’s event. A special thank you to Jim and Amy Ellixson along with the entire Muscles for Mckenna committee for making this year’s event such a success!
2017 Cure SMA Pennsylvania Chapter Walk-n-Roll

The Cure SMA Pennsylvania Chapter Walk-n-Roll hosted its 14th annual event on May 21, 2017. This year we were thrilled to host our walk at our new location at Penn Park in Philadelphia, PA. Thanks to the generous support of our 19 teams, 33 sponsors, and hundreds of supporters we raised nearly $108,000!

Everyone enjoyed our great DJ, team photo sessions, magician, games, and snacks. Together we remembered the three members of our chapter we lost this year – William Jankowski, Mason Gray, and Joseph Haughey, IV – during the butterfly release.

Thank you to all of the amazing SMA families, participants and donors for helping make this event possible.

Big thanks to our teams: Team BAYADA, Cousins Cruisin’ for a Cure, Muscles for Mckenna, The Jack Pack, Matthew’s Musclemen, Peter’s Philadelphia Eagles, Team William, Pranav, Lukie’s Tigers, Philly SMAck Down, Abby’s Army, Team Lyla, Team Kendal, Omar’s Soldiers, New HOPE 4 SMA, Team Peters, Team Saxton, Angels M I and Miracles for Marc.

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! A special thank you to Gina Cannady, Christina Murray and Stephanie Reese for helping with this year’s event.
CHAPTER UPDATES

2017 Cure SMA Pittsburgh 5K & Walk-n-Roll

The Cure SMA Pittsburgh 5K & Walk-n-Roll hosted its 2nd Annual event on April 23, 2017, at the South Park Museum Building in Pittsburgh, PA. Thank you to all of the dedicated sponsors who helped make this day a huge success! The fun-filled morning included activities for the whole family to enjoy! In total, this year’s event raised nearly $8,900!

Thank you to all of the amazing SMA families, participants, and donors for helping make this event possible. Big thanks to our teams: Cooper Can, Team Noah Jacob, and Team Krakoff.

A special thank you to Kelly Mangini and Katie Sabatos for all of your hard work in planning this fun and successful event!

Mr. Harriton Student Talent Show

Thank you to Harriton High School and the Harriton Student Government for their generous $4,000 donation to Cure SMA! Each year the students put together a production of Mr. Harriton, an annual student talent show fundraiser and beloved community event, in spring 2017. The event was a great success with sold out attendance and record fundraising.

2017 Dick's Sporting Goods Pittsburgh Marathon

Thank you to the two runners that represented Team Cure SMA at the 2017 Dick's Sporting Goods Pittsburgh Marathon on May 7, 2017! Carol Tomko and Patrick Neff raised $2,700 for Cure SMA.

Swarthmore Trailblazers Bake Sale in Memory of Owen Henry Brown

Thank you to the Swarthmore Trailblazers, a 4H Club in Swarthmore, PA, for fundraising for Cure SMA! The club held a bake sale at the town parade on July 4, 2017. The club voted on a charity to donate to and chose Cure SMA! The club was started by Amy Brown and Cindy Burkes. Amy lost her first child, Owen Henry Brown, to SMA when he was just five months old. The bake sale was a great success and raised $156!

NHS Class of 2021 Community Service Walk-A-Thon Project

Thank you to the New Hope Class of 2021 of New Hope, PA for their incredible support of Cure SMA. Students organized a Walk-a-Thon as the class' service project this year. There are two students, Jake Saxton and Matthew Riley, at the school that are affected by SMA. Thanks to the hard work of the students and their generous supporters, more than $5,000 was raised for Cure SMA! Thank you to all of the students and school staff for their hard work in making this event a success.
4th Annual Rocky Mountain Evening of Hope - Beer & Cheese Pairing

To everyone who supported the 4th Annual Beer & Cheese Pairing - thank you! The March 9, 2017, event was held at Chain Reaction Brewery in Denver, CO with 90 attendees filling the brewery to capacity. The evening raised over $9,500 and brought together many families and friends in the community. Guests received a rewarding update from Dr. Julie Parsons of Children’s Hospital of Colorado regarding Spinraza, the first-ever approved therapy for SMA. Allison and Ryan Gray also shared their story on how Spinraza has changed the outlook for their beautiful daughter living with SMA.

Guests enjoyed a variety of appetizers, as well as tastings of six craft beers brewed by Chain Reaction Brewery. The cheese pairings were hand-selected and provided by King Soopers and cheese captains Aaron Simko and Ken Sherman.

Thank you to Marla Marlow for chairing this event, as well as Zack Christofferson and Chad Christofferson, owners of Chain Reaction Brewery, and their staff, for hosting the evening. Thank you to our sponsors Biogen, AveXis, The Cash Scanlon Phillips Foundation, and Mulay Sausage, and to those who donated silent auction items, raffle items and gift cards. We sincerely appreciate your generosity and continued support of SMA families and patients everywhere.

14th Annual Rocky Mountain Charity Golf Tournament

The 14th Annual Rocky Mountain Charity Golf Tournament took place on May 22, 2017, at the beautiful Club at Pradera in Parker, CO. Congratulations to this year’s tournament winners Tyler Cowhick, Graig Sells, Eric Ostberg, and Chris Deeds! With the support of this year’s sponsors, donors, and players, the event raised more than $27,000 to fund a cure for SMA.

Cure SMA would like to extend a special thank you to event organizer Gillian Faith and her family for hosting the tournament. Your continued efforts and dedication are greatly appreciated.
South Florida Chapter

Orangetheory Burn for a Cure

On August 12, 2017, the South Florida Chapter “burned for a cure” and had a fun workout at Orangetheory Fitness Fort Lauderdale. This event raised $360 for Cure SMA. Orangetheory Fitness is a revolutionary 60-minute workout that features high-intensity interval training classes. The South Florida chapter felt good while doing good!

South Florida Family Day at the Museum

South Florida families had a great day at the Museum of Discovery and Science on August 26, 2017. Over 40 friends and families joined us for a pizza party, henna tattoos, engaged with reptiles, and more. Thank you to Jennifer Miller Smith and Fiorenna Israel for organizing this outing!

Miami Marlins Awareness Game

Family and friends gathered at Marlins Park for SMA awareness on August 13, 2017. The Miami Marlins played the Colorado Rockies during a beautiful Sunday afternoon game. The Marlins donated a portion of the ticket sales to Cure SMA totaling more than $3,000!
The 14th Annual Jacob’s Run, Walk & Roll to Cure SMA was held on April 23, 2017, at South County Regional Park in Boca Raton, FL. Over $73,000 was raised this year!

Despite the torrential rain, the event’s dedicated supporters donned rain jackets and hit the walk path. Everyone enjoyed the petting zoo, mini golf, and other fun activities.

Thank you to all of the event’s incredible fundraising teams: Team Adrian, Team BAYADA, Shannon’s Wonder Woman, Burn Fitness, Gunster, Team Jaelie, Team Biogen, Amiya’s Circle, Natalie’s Warriors for a Cure, and Team Sofie.

A special highlight of this year’s event was Adi Rappoport receiving an award recognizing his years of dedicated service to Cure SMA’s national board of directors.

Each year Adi and Shaina Rappoport organize this amazing event in memory of their son, Jacob Isaac Rappoport, who had SMA Type I. The incredible support of the Rappoport Family helps fund many of Cure SMA’s most treasured programs including: Type I Care Packages and conference activities such as the children’s program, Type I Reception, and the teen social. In addition to their generous funding, both Shaina and Adi provide support to Type I families across the country. Thank you to the entire Rappoport family for their years of dedication to both the SMA community and Jacob’s Run, Walk & Roll to Cure SMA!
On May 20, 2017, over 80 runners laced up their shoes in Pikeville, TN to run in memory of Bentley Bassamore. Everyone enjoyed the scenic run through Pikeville, and $5,225 was raised for SMA research and family support! Special thanks to all of the event’s generous sponsors and runners for making this year’s event such a success. Thank you to Carie Whitaker for her continued dedication to Cure SMA and all the work she puts into this event every year.

Cure SMA Pikeville 5K with Bentley's Smiles for Miles

On May 20, 2017, over 80 runners laced up their shoes in Pikeville, TN to run in memory of Bentley Bassamore. Everyone enjoyed the scenic run through Pikeville, and $5,225 was raised for SMA research and family support! Special thanks to all of the event’s generous sponsors and runners for making this year’s event such a success. Thank you to Carie Whitaker for her continued dedication to Cure SMA and all the work she puts into this event every year.
1st Annual North Texas Chapter Bowl-a-Thon

The inaugural North Texas Bowl-a-Thon was held on March 11, 2017 at Plano Super Bowl in Plano, Texas. The event had 12 corporate sponsors and over 80 bowlers! In addition to bowling, there was a silent auction, a face painter for the kids, and a 50/50 cash raffle in which the cash prize was generously donated back to Cure SMA!

A special thank you to event organizers, Justin Henderson and Rachel Anthony, who helped raise over $13,000 for Cure SMA, beating their original goal of $5,000!

Phi Epsilon Pi Philanthropy Project

On March 31, 2017, the members of the Phi Epsilon Pi Fraternity from the State University of New York at Buffalo who graduated in the 1960s decided to make Cure SMA their charity of choice for 2017. They collected a total of $3,600 from their members in honor of Braden Ray and Tamryn Ember Campbell, the grandchildren of a fraternity member. Thank you to Phi Epsilon Pi for their support of Cure SMA!

Talbots Make A Difference Day

On April 7, 2017, Cure SMA and Talbots partnered to host Shop & Donate events at a store located in Houston, Texas. Customers were invited to shop, enjoy refreshments and make a difference! During the event the store graciously donated 10% of all sales to Cure SMA, raising a total of $538.

A special thank you goes out to Talbots Town & Country Village manager Chieuanh Nguyen and volunteer Kelly Coggin for organizing this opportunity to spread SMA awareness and raise funds for Cure SMA. Thank you!

Lake Hill Preparatory School Powderpuff Game

In the spring of 2017, the seventh and eighth grade students at Lakehill Preparatory School held a Powderpuff volleyball game to raise money to donate in honor of a fellow student, Hannah, who has SMA. Her classmates chose Cure SMA so that they could help fund a cure for SMA. Thank you to these students for raising over $1,800 for Cure SMA!
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

2nd Annual Fayetteville 5K in Honor of Annabelle Combs

The 2nd Annual Fayetteville 5K in Honor of Annabelle Combs was held on April 22, 2017, at Oak Thicket Park in Fayetteville, Texas. With over 100 participants, the event raised over $16,000 for Cure SMA! Annabelle turned 2 years old the day before the race, making the event an extra special time to celebrate and raise awareness for SMA. A special thanks to Alyssa Combs for her hard work and dedication to make this event a success yet again!

1st Annual Houston Walk-n-Roll

The 1st Annual Houston Walk-n-Roll was held on April 29, 2017, at Traylor Stadium in Rosenberg, Texas. Even though this was the event’s first year, over 100 participants attended and raised over $10,000 for Cure SMA! Although it was a windy day in Texas, fun was had by all! The event featured a walk around the track at Traylor Stadium, a balloon artist and awards presented to the top three fundraising teams.

Thank you to all of the amazing SMA families, participants and donors for helping make this event possible, and thank you to all of our volunteers who dedicated their time on event day to ensure everything ran smoothly. A special thanks to Tracie Tanner and her family for their hard work and dedication to make this event a success!
7th Annual Texas Chapter Bowl-a-Thon

On Saturday, August 5th, 2017, the Texas Chapter held the 7th Annual Bowl-a-Thon at Mel's Lone Star Lanes in Georgetown, Texas. The location changed this year to a bowling alley with all accessible lanes and lot more fun games for the kids. The event included bowling, a silent auction, face painting, balloon art, awards, and most importantly, visiting with other families. With continued support of friends, family, and the community, the Bowl-a-Thon raised over $6,000. Special thanks to event organizer Kelly Coggin, the Davis Families, and Ostermayer Family for all their help and support. We look forward to seeing everyone again for next year’s event!

On March 11, 2017, Gabrielle Kozera and Robert & Alicia Cooper ran in the 2017 Rock ‘n’ Roll DC Marathon Series. We deeply express our gratitude to Robert and Alicia who ran in memory of their sons Jonathan and James Cooper. Thank you to our runners who raised over $1,600 for Cure SMA.

Virginia Chapter

2017 DC Rock ‘n’ Roll Marathon Series

Western New York Chapter

2017 Western NY Chapter Walk-n-Roll

The 14th Annual Walk for a Cure was once again held at Beaver Island State Park on August 5, 2017, in Grand Island, NY. It was another beautiful year at the walk along the Niagara River. Attendees enjoyed games, face painting, a raffle, refreshments, and the chance to connect with several SMA families throughout the area. This year was attended by nearly 250 people.

Thank you to Paul Boguhn for speaking about the conference. In addition, NY State Senator Rob Ort attended and added a few words of support for Cure SMA’s newborn screening efforts. A special thanks to the Western NY Chapter Leaders for another successful walk and raising over to $45,000 to help find a cure for SMA!
Note: The amounts raised and shown are totals as of August 15, 2017 and may differ from current fundraising totals by the time you get this newsletter.

Kinsley’s Day for SMA

On August 12, 2017, the 1st Annual Kinsley’s Day for SMA was held in Columbus, NY. It was a great day for family and friends to gather and enjoy games, cookout food, music and raffle. The day ended with a candle lighting ceremony in honor of SMA Awareness Month. The Weidman Family started this event to help raise money for critical SMA research in the hopes that no other family will have to say goodbye. Seventy people attended and the day raised over $1,200. Thank you to Cheryl Weidman for hosting and organizing this year’s event in memory of her daughter, Kinsley Weidman.

Wisconsin Chapter

4th Annual WI Chapter Bowl-a-Thon

The 4th Annual Wisconsin Chapter Bowl-a-Thon was held on July 29, 2017, at a new venue in New Berlin, WI. The event had a great turnout with over 100 people joining in on the family fun and raising $3,430 for Cure SMA! Thank you to our sponsors: Madaus Trucking and Darrell & Kathy Baumeister. We hope to see everyone at the 5th annual event next year!

Cure SMA would like to give special thanks to our Wisconsin chapter leaders, Amy Medina, Danyelle Sun and Kate LaChapelle for all of their hard work planning the event and recruiting bowlers!

2nd Annual Baylor Cain Golf Outing for SMA

On August 12th, 2017, we held the 2nd Annual Baylor Cain Golf Outing for SMA in honor of our son Baylor. It has been over a year since we lost our baby boy to SMA and we plan to do everything in our power to help find a cure. We had another great turnout with 155 golf participants and many more joined us for dinner. We raised over $16,000 that was all donated to Cure SMA. We thank everyone who came out this year for the continued support.

Eric & Anna Cain – Kewaskum, WI

Cure SMA would like to give a special thanks to Anna Cain and Lindsay Gitter for organizing this wonderful event and for their continuous support!
2017 Cure SMA Day with the Milwaukee Brewers

The Wisconsin Chapter had a great night on August 12, 2017, at Miller Park in Milwaukee celebrating SMA Awareness Month at the Cure SMA Day with the Milwaukee Brewers. We had a great turnout with 50 family members and friends coming together to raise awareness for SMA and to cheer on the Milwaukee Brewers! We are looking forward to doing this event again next year!

Tori Stevens Memorial Night

With 81 bikes, including one autographed by Kenny Wallace generously donated by Wild North Saloon, and 50 donated backpacks; the Tori Stevens Memorial Race had a great turn out with many smiling faces! Tori would have loved to see all of this. We love that the small town of Tomahawk can rally together and support this cause! We couldn't have pulled off this event without the help of all the businesses, racers and individuals who donated. Tori's family and Hedges Racing can't thank everyone enough! Congrats to all the winners and we hope to see you next year at the Cure SMA Tori Stevens Memorial Race at Tomahawk Speedway!! Thank you to Tomahawk Speedway for hosting our event at Tori's favorite track!! Special thanks to Linda Briner and Wendy Jarrett and the Kids for helping work the bike and fundraiser booths!

Ashley Hedges | Tripoli, WI

Cure SMA would like to thank Ashley Hedges for organizing this fundraiser each year and for her continuous support.
EVERYDAY WE ARE STRONGER

Please make a gift to the Cure SMA Year-End Campaign and help improve the lives of all people affected by SMA. With your support, we can build on our foundation of recent success and continue changing the future of SMA.

Visit our website www.curesma.org to make a gift today!

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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.
MARK YOUR CALENDARS FOR JUNE 14 – 17, 2018!

REGISTRATION IS NOW OPEN FOR THE 2018 ANNUAL SMA CONFERENCE AT THE HILTON ANATOLE HOTEL IN DALLAS, TX