The 2017 Annual SMA Conference, now in its 29th year, is set to kick off on June 29 and continue into a weekend full of programs and events that bring families, researchers and healthcare professionals together.

This year’s conference has already surpassed the records of previous years. We’re expecting over 2,000 attendees, including almost 450 researchers from nearly 100 institutions and pharmaceutical companies. There will be a total of 52 family workshops and 130 formal research presentations.

This event is generously supported by 16 sponsors and 23 exhibitors. Our special thanks to Biogen as the Presenting Sponsor of the 2017 Annual SMA Conference. Through these incredible sponsors, we are able to provide assistance travel or registration to 710 attendees.

Advancing a Path for Current & Future Therapies and Continued Advocacy

We’ve seen significant advances in SMA research come to fruition with the approval of Spinraza. While an approved therapy offers tremendous benefit to those with SMA, there is also a need for continued research, advocacy, and investment in SMA care.

We know that collaboration between academics, industry, government and families is crucial to sustaining our current momentum. The Annual SMA Conference provides a great forum for this collaboration, and gives our community the opportunity to discuss important topics and next steps in several critical areas:

- Spinraza administration and access
- Insurance coverage and other new topics related to approved therapies
- Newborn screening and other opportunities for advocacy
- Continued research funding to treat all ages, stages and types of SMA
- Improving and implementing the standard of care for SMA
How to Get Involved

Whether you’re able to join us in Orlando or not, be sure to like us on Facebook and follow us on Twitter for conference updates.

We’ve got lots planned, including:

- A Facebook live-stream of the Family Friendly Poster Session on Friday evening at 7:00pm.
- Regular updates from the conference posted in our news section
- Live-tweeting at 2 Special Sessions
- Sharing photos on all social media channels

If you’re posting from or about our conference, be sure to use the hashtags #cureSMA and #SMAConference.

WE LOOK FORWARD TO SEEING YOU IN ORLANDO!
Spinraza Update

On December 23, 2016, our community celebrated the approval of Spinraza, the first-ever FDA approved treatment for SMA. That historic first approval—with a broad label covering all ages and types of SMA—has opened up new opportunities for us, to ensure that as many families as possible receive access to this therapy.

Currently, we are focused on two primary areas to improve access to Spinraza:

1. Advocating for full, broad insurance coverage through both commercial and government payers.
2. Expanding the number of sites involved in prescribing and delivering Spinraza.

The first item is the focus of our Coverage and Payment Policy Project. While the project covers the full breadth of treatment and care for SMA, it is specifically focused on obtaining timely coverage for new treatments as they are approved by the FDA. With that in mind, many of our current activities have focused on the approval of Spinraza, both to ensure that it is broadly covered by insurance, and to set a standard and process for decisions on future treatments.

For a complete update on this project, including the activities surrounding Spinraza, see pages 16-17 of this issue.

In addition, Cure SMA has launched a list of Spinraza administration sites. This list includes over 59 sites in 26 states (including Washington DC).

Our goal at Cure SMA is to build this list to a few hundred active centers across the US, to meet the expected needs of our whole community. We have to work together to greatly increase the number of sites involved in both delivering this first-ever new SMA therapy and providing SMA care.

Our work in expanding these sites is based on a “congressional model.” Our initial goal is to build this list to at least one site in every state, then to build it to at least one pediatric and one adult site in every state. Finally, we will work to build to multiple sites in each state, based on population.

If your local site is not on our site list, please email us, or ask the site coordinator to email us, at patientcare@curesma.org. Site managers must give their permission to have the site listed on our website.
The site list is continually updated as new sites are added. Please note that the figures and maps are current as of May 2017. We encourage you to visit www.cureSMA.org/Spinraza for the most updated maps and site list.

We also encourage you to visit www.cureSMA.org/news for regular updates on Spinraza site administration and our coverage and payment policy project. Approximately once per quarter, Cure SMA hosts webinars on the status of Spinraza access. These webinars are also announced via our news section, and on our social media channels.
Advocacy

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.”

Charting a Course for Current and Future Therapies

The PFDD meeting also presented an opportunity to assess the current landscape of SMA, with Spinraza, an approved treatment, now available, and “unambiguously, unequivocally indicated for the entire population,” according to Dr. Dunn. However, even with the approval of Spinraza, much more work remains to be done to develop a range of treatments that will cover all ages, types and stages of SMA.

In opening remarks, Dr. Dunn cited the important timing of the meeting, coming on the heels of the first-ever approved therapy for SMA. Dr. Dunn acknowledged the “unrelenting approach” of Biogen, Ionis and the SMA community in reaching the milestone of the first-ever approval, and in continuing to pursue further treatments.

“I’m excited as you all are that there’s a new treatment available,” Dr. Goldsmith added, “but I’m also excited that there’s lots of members of industry here. So I don’t think that this is the only treatment that will be available, and you’ll have other kinds of options in the coming years.”

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 with 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.
The Secretary’s Advisory Committee on Heritable Disorders in Newborns and Children (SACHDNC) announced that they have accepted spinal muscular atrophy into the review process for the Recommended Uniform Screening Panel (RUSP). The RUSP is a list of conditions that all newborns in the US are recommended to be screened for.

The RUSP application for SMA now moves into evidence review, which is a six to nine month process. Once the evidence review is completed, the committee will make a recommendation to the Secretary of Health and Human Services, who will determine whether SMA will be added to the RUSP. The review by the HHS Secretary can also take several months, meaning a final decision on the RUSP application could come in mid-2018.

“We are pleased that the committee has taken this significant first step toward recommending SMA for inclusion on the RUSP,” said Jill Jarecki, PhD, Cure SMA’s chief scientific officer. “Adding SMA to the federal guidelines would help ensure that all babies born with SMA receive the best chance for prompt, effective treatment. As the committee continues their review, we will continue our advocacy for SMA screening implementation. Thank you again to our working group for their hard work on the nomination.”

At the meeting, the committee heard testimony from the SMA community in support of the application. Dr. Jarecki testified, along with Debra Schaefer, who has had two granddaughters affected by SMA: one who passed away in 2013, and one who was diagnosed in utero and has benefited from early treatment with Spinraza.

While the RUSP application is reviewed, our community’s work on newborn screening continues in earnest. This includes:

- Creating a treatment plan for infants who test positive for SMA during the screening process. This treatment plan answers several critical questions, including which SMA types (based on SMN2 copy number) will receive drug pre-symptomatically and what symptoms would trigger treatment for others, how the medical community will ensure prompt access to treatment, and how families will be connected to a doctor who can manage the full range of care required for SMA. We plan for this project to be completed before a final decision is made on the RUSP.
- Working with Congress to ensure federal funding is appropriated for newborn screening.
• Working with state labs and the CDC to secure funding and execution of state-level pilot screening projects where needed. The information learned in these state pilots can be used to ensure a speedy, smooth implementation process, once SMA screening is adopted.

• Advocating at the state level for SMA newborn screening. Because the list of conditions on the RUSP is not mandatory, each state will make its own determination on whether SMA will be added. Further, many states have a process that allows them to adopt SMA newborn screening before a final decision is issued on the RUSP application.

Training Advocates Throughout Our Community

The decision on implementing newborn screening for SMA ultimately rests with each individual state, meaning we will need “SMA champions” in all 50 states to help advocate on behalf of our community. While a successful RUSP application will strongly support our case for implementation, in most states we do not need to wait for a decision in order to begin the process.

To help support our community’s efforts, we will be offering a number of advocacy training opportunities and materials.

• We will hold a newborn screening symposium at the Annual SMA Conference. The purpose of this symposium is to train our community to advocate for newborn screening in their respective states.

• We will be releasing an advocacy toolkit: Newborn Screening for SMA: Tools for Advocates Working to Improve Children’s Lives. The toolkit covers an overview of SMA, an introduction to effective advocacy across different levels of government and legislation, and leave-behind resources including an SMA fact sheet, a one-pager on the importance of newborn screening, and a series of key talking points for policymakers.

Thank You

The RUSP application was prepared and submitted by a Newborn Screening Working Group comprised of staff and members of the board of directors of Cure SMA, representatives from Muscular Dystrophy Association, and a panel of SMA clinicians and researchers. We thank them for their hard work in writing the application, and for their continued work as the application moves through this process.

The SMA Newborn Screening Coalition will also continue to support the RUSP application and the other aspects of newborn screening advocacy described above. Members of the SMA Newborn Screening Coalition include representatives from pharmaceutical companies Biogen and AveXis, and staff members from Cure SMA, with guidance from representatives from the District Policy Group, a DC-based public policy firm that provides advocacy support to Cure SMA.

To learn more about how you can help champion SMA newborn screening,

e-mail advocacy@curesma.org
Cure SMA Coverage and Payment Policy Project Continues to Move Forward

The Cure SMA Coverage and Payment Policy Project has two distinct but complementary tracks:

- Outreach on behalf of our community. We’re working with public and private insurers, regulators, and other stakeholders, advocating for prompt, comprehensive coverage for SMA treatments and care.

- Outreach to our community. We’re working to provide the necessary tools and information so that individuals and families can navigate this often complex area.

While the project covers the full breadth of treatment and care for SMA, it is specifically focused on obtaining timely coverage for new treatments as they are approved by the FDA. With that in mind, many of our current activities have focused on the approval of Spinraza, both to ensure that it is broadly covered by insurance, and to set a standard and process for decisions on future treatments.

Advocacy on Behalf of Our Community

The following are some of the key activities from the first quarter of 2017.

We sent letters to the CEOs and CMOs of all major insurance plans and companies. These letters address the Spinraza insurance policies released over the past several months, and are tailored to each individual company’s decision. The goal of these letters, and any subsequent meetings, is to broaden coverage for companies that have released limited policies, and to advocate for a quick and broad decision for those companies that have not yet released policies.

We also sent letters to all of the state Medicaid directors and state governors, urging coverage of Spinraza. Alongside of this, we developed SMA fact sheets specific to each state, to use in advocacy and education with individual states. In the coming months, these materials will also be used to help educate and mobilize grassroots advocates in many states.

Throughout the spring, we are meeting with various national organizations and government agencies that have a role in recommending, influencing, and otherwise determining coverage and payment policy, including the federal Centers for Medicare and Medicaid Services in Baltimore, Maryland. The goal of these meetings is to educate stakeholders on SMA and the availability of care and treatments, and to continue advocating for broad and timely coverage, under government-funded and commercial, private health plans.

In addition, throughout the spring, we also will be meeting with representatives from individual companies, associations, and entities that have influence over coverage and payment policies, including the state Medicaid programs and commercial insurance.

Representatives from Cure SMA are part of a working group on orphan drug pricing through the Institute for Clinical and Economic Review. ICER is hosted a policy summit in May. The summit will discuss of how we can best measure the impact and value of drugs developed for orphan diseases, using Spinraza as one of its key test cases. The goal is to leverage these value assessments for broader insurance coverage.
Practical Tools for Our Community

In addition, we also want to make sure that each individual and family has the tools necessary to advocate with their insurers.

In early 2017, released *Choice and Connection to Care: A Health Insurance Roadmap for People Living with Spinal Muscular Atrophy (SMA) and Their Caregivers*. This care series booklet addresses some of the broader questions that are common in our community, including questions on type of insurance, eligibility, out-of-pocket costs, nontraditional treatments, and new treatments.

You can download a copy of this booklet now from our website, or send an email to info@curesma.org to request a physical copy of the booklet.

For those affected by SMA, insurance can be a challenging subject to navigate. Those affected by SMA often rely on a unique combination of private and public insurance. What’s more, the needs of those affected by SMA can change frequently—and the options for treatment are changing too—meaning that even those with existing insurance can face uncertainty when it comes to coverage and out-of-pocket costs.

This booklet addresses some of the broader questions that are common in our community, such as:

- What are the different types of health insurance available?
- How do I know which type(s) I or my child is eligible for?
- How do I figure out what insurance may cover for me or my child?
- How do I calculate estimated out-of-pocket costs?
- What options are available for costs that may not be covered by traditional health insurance plans?
- How can I talk to insurers about insurance options and new treatments?

The booklet also includes links to additional information, a glossary of common insurance terms, and more.

We expect that this booklet will help families tackle some of the most common issues and questions. However, please note that each individual situation is different, so this booklet cannot substitute for personalized advice from a patient advocate, social worker, or other resource.

Your elected officials who represent you in Washington, D.C. have caseworkers in their regional and district offices in your communities. These caseworkers can help you work with and better understand government programs, like Medicaid, Medicare, TRICARE, and Social Security.

Caseworkers, also sometimes called constituent services representatives, are in the local community offices of federal elected officials specifically to help constituents navigate requests or problems relating to federal agencies and programs. The use of their services is free and available to anyone who lives in the elected official’s state (for U.S. Senators) and in the district (for U.S. House Representatives).

Individuals and families affected by SMA can use these caseworkers to help with public insurance denials or delays relating to any aspect of treatment and care. This includes coverage decisions for Spinraza.

How can a Congressional caseworker help?

- Inform you of access to care, providers, and services in your area and around your state as well as the eligibility requirements and enrollment processes for insurance coverage provided by Medicare and Medicaid and the eligibility for Veterans benefits and Social Security Disability Insurance.
- Direct you to the appropriate persons within federal agencies who can help provide additional answers for your request or appeals.
- Serve as an advocate on your behalf to appeal adverse decisions made by Medicare, Medicaid, TRICARE, Social Security, and the Veterans Administration.

How do I access these resources?

- Look up your Representative or Senators or call (202) 224-3121 to find who represents you in Washington, D.C.
- Call the Washington office or appropriate local office and briefly indicate your request for a caseworker and the specific issue that requires their assistance.
- Representatives’ offices can sometimes respond more quickly to casework requests than Senators’ offices due to the smaller population served, as they serve a district while Senators’ offices serve the entire state.
- Caseworkers may require you to submit materials to assist them with your request. Electronic communication is ideal as mail takes 2-3 weeks to reach offices due to security screenings.
- Offices may also ask you to complete a release form so they can work on your behalf.
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.

For print copies:
Please contact the Cure SMA national office at info@curesma.org. 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.
AveXis, Inc., a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases, reported topline results from the Phase 1 trial of AVXS-101 in spinal muscular atrophy (SMA) Type 1. The company also reported financial results for the fourth quarter and full year ended December 31, 2016, recent corporate highlights and upcoming milestones.

"The completion of our Phase 1 clinical study of AVXS-101, the first ever gene therapy studied for the treatment of SMA Type 1, is an exciting and eagerly awaited milestone, and we are quite pleased with these data," said Sean Nolan, President and Chief Executive Officer of AveXis. "The past few months have been productive for AveXis, and we look forward to continuing the momentum with several upcoming corporate catalysts, including the planned Type B CMC meeting with the FDA, as well as ongoing collaborative discussions with regulatory authorities in the United States and Europe to explore the most expeditious pathways for marketing approval of AVXS-101."

Topline Results from the Phase 1 Trial of AVXS-101 in SMA Type 1

- No New Treatment-related Safety or Tolerability Concerns Identified: As of January 20, 2017, AVXS-101 appeared to have a favorable safety profile and to be generally well tolerated, with no new safety or tolerability concerns identified.

- No New Events and 15 of 15 Patients Event-Free at 13.6 Months, including 12 of 12 Patients in Proposed Therapeutic-Dose Cohort: As of January 20, 2017, 12 of 12 patients (100%) in the cohort of patients who received the proposed one-time therapeutic dose of AVXS-101 (Cohort 2) had reached 13.6 months of age event-free, where the expected event-free survival rate based on natural history of the disease is 25%. The median age at last follow-up for Cohort 2 was 20.2 months, with the oldest patient at 31.1 months of age.

- Rapid and Sustained CHOP INTEND Improvements Above Baseline: As of January 20, 2017, mean increases from baseline in CHOP INTEND scores of 7.7 points in Cohort 1 and 24.7 points in Cohort 2 were observed, reflecting improvement in motor function. In Cohort 2 there were mean increases in CHOP INTEND of 9.8 points one month after gene therapy and 15.4 points three months following gene therapy.

- Cohort 2 Patients Consistently Achieved and Maintained Key Developmental Motor Milestones: As of January 20, 2017, 11 of 12 patients (92%) in Cohort 2 achieved head control, nine of 12 patients (75%) could roll a minimum of 180 degrees from back to both left and right, and 11 of 12 patients (92%) could sit with assistance. For the end-of-study assessment, AveXis evaluated three validated and well-established measures of sitting unassisted for periods of increasing duration. Nine of 12 patients (75%) could sit unassisted for at least five seconds,
seven of 12 patients (58%) could sit unassisted for at least 10 seconds and five of 12 patients (42%) could sit unassisted for 30 seconds or more. Two patients could walk independently, and each had achieved earlier and important developmental milestones such as standing with support, standing alone and walking with support.

Upcoming Clinical Trials of AVXS-101 in SMA Type 1

AveXis is planning to initiate two new clinical trials in the first half of 2017:

• A single-arm, Phase 3 trial testing systemic delivery of AVXS-101 in infants with SMA type 1—the same delivery method being tested in the current trial

• A Phase 1 trial testing CSF-delivery (intrathecal injection) in children with SMA type 2.

Cure SMA Funds Multiple Gene Therapy Approaches

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

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

Currently, two approaches are being studied: an injection into a vein, known as systemic delivery, and delivery directly into the cerebrospinal spinal fluid (CSF), a process known as CSF-delivered gene therapy. CSF-delivered gene therapy has shown promise for reducing the amount of drug required for larger and older patients. This could eventually make the treatment accessible to a wider population.

In total, Cure SMA has granted $845,000 for gene therapy, including support for both the systemic program and the CSF program. Using the data generated with our funding for CSF delivery, Dr. Kaspar and his team were able to secure a $4 million grant from NINDS in 2013, to develop this delivery approach for human clinical trials in SMA.
Genentech Roche provided the following community statement with clinical trial updates for SUNFISH (Type 2/3), FIREFISH (Type 1) and JEWELFISH.

Dear SMA community,

At Genentech (A Member of the Roche Group) we are committed to addressing the urgent needs of people living with SMA. We are happy to share this update on our two investigational oral molecules in development: olesoxime and RG7916, and the three trials with RG7916 that are currently recruiting: SUNFISH (Type 2/3), FIREFISH (Type 1) and JEWELFISH (non-naive Type 2/3).

Olesoxime

- Olesoxime is an orally administered compound that may maintain mitochondrial function and support the continued function of cells
- The European Medicines Agency (EMA) and US Food and Drug Administration (FDA) have recommended that we generate additional benefit/risk data in a Phase III study
- We are considering EMA and FDA feedback on potential plans for a Phase III study with olesoxime and will keep you updated in the coming months

RG7916

- RG7916 is an SMN2 splicing modifier that is taken orally and distributes widely throughout the whole body
- It is in clinical development in collaboration with PTC Therapeutics and the SMA Foundation
- RG7916 has received Orphan Drug Designation from the FDA

What clinical trials with RG7916 are currently recruiting?

Three clinical trials with RG7916 are currently recruiting participants: SUNFISH, FIREFISH and JEWELFISH. Potential participants would need to meet all the inclusion criteria before enrollment in any RG7916 trial.

SUNFISH

Aim: To assess the safety and efficacy of RG7916 in patients with Type 2 or 3 SMA.

- Part 1 will assess how safe and well tolerated RG7916 is at 2 different dose levels
- Part 2 will assess the efficacy and safety of RG7916 at the dose selected from Part 1
Who could enroll? Children and young adults (2-25 years old) with Type 2 or 3 SMA.

Current status: Planned enrollment is complete for Part 1 Dose level 1 (low dose) of RG7916 in 10 adults and adolescents (Group A), and in 9 children (Group B). Part 1 Dose level 2 (higher dose) is currently enrolling patients.

Safety review: The safety of study participants is a priority for us. As pre-planned in the study protocol, a Safety Monitoring Committee reviews all safety information from all SUNFISH participants. After reviewing the safety information from the lower dose given to group B in Part 1, the Committee endorsed SUNFISH to advance as planned.

Future development: Part 2 is expected to begin in the second half of 2017, including additional countries and sites.

**FIREFISH**

Aim: To assess the safety and efficacy of RG7916 in infants aged 1 to 7 months with Type 1 SMA.

- Part 1 will assess the safety and efficacy of RG7916 at 2 different dose levels
- Part 2 will assess the efficacy and safety of RG7916 at the dose selected from Part 1

Who could enroll? Infants aged 1 to 7 months with Type 1 SMA.

Current status: The first infant was dosed with RG7916 in December 2016, and the study is progressing in Europe. FIREFISH Part 1 will open in additional countries and sites, including the US, in the coming months.

Safety: The safety of study participants is a priority for us. As pre-planned in the study protocol, a Safety Monitoring Committee reviews all safety information from all FIREFISH participants.

Future development: Part 2 is expected to begin in the second half of 2017, including additional countries and sites.

**JEWELFISH**

Aim: To assess the safety and tolerability of RG7916.

Who could enroll? Patients with SMA who have previously received an SMN2-targeting therapy (either as part of the MOONFISH study with RG7800 or a study with another SMN2 targeting therapy).

Current status: The first patient has received their first dose at a US site.

Safety: Safety of study participants is a priority for us. As pre-planned in the study protocol, a Safety Monitoring Committee reviews all safety information from all JEWELFISH participants.

Future development: Further screening and enrollment of patients at sites across the US and Europe is planned.

**How can I or my family find out how to participate in SUNFISH, FIREFISH or JEWELFISH?**

Please contact your physician if you think you or a family member could be suitable for one of these trials.

You can also visit [www.roche-sma-clinicaltrials.com](http://www.roche-sma-clinicaltrials.com) to read more about our program.

Additionally, your local patient group may have more information and resources. For a list of organizations go to: [www.smafoundation.org/about-sma/sma-organizations-worldwide](http://www.smafoundation.org/about-sma/sma-organizations-worldwide)

**How can I find out more?**

You can read more about SUNFISH, FIREFISH and JEWELFISH at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and [www.clinicaltrialsregister.eu/](http://www.clinicaltrialsregister.eu/)

We will continue to share updates and developments about our studies.

If you have any questions, or would like to discuss this further, please contact sangeeti.jethwa@roche.com
Cytokinetics Receives Orphan Drug Designation

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.

CK-2127107 Clinical Trial Currently Recruiting

In March, Cytokinetics announced the opening of recruitment for a second cohort of their Phase 2 trial of CK-2127107. This Phase 2 double-blind, placebo-controlled clinical trial will enroll ambulatory and non-ambulatory patients 12 years of age and older, with SMA type II, III or IV. The trial will measure the drug’s effects on muscle strength and function, including respiratory assessments.

For additional information about the CK-2127101 clinical trial, visit www.clinicaltrials.gov.

Cure SMA Funding for Combination Therapies

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.
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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Last summer, Cure SMA, with help from independent SMA experts, our scientific advisory groups and Medicine and Science Committee in our Board of Directors, created a strategic research plan to guide us into the next phase of SMA research. A major part of this strategic plan is expanding our longstanding investment in basic research. Basic research is the first step in our comprehensive research model. Basic research investigates the causes and biology of SMA, often revealing more effective ways of making SMA drugs.

Individuals with SMA don’t produce survival motor neuron (SMN) protein at high enough levels due to a mutation in the survival motor neuron 1 (SMN1) gene. Much of the early research into SMA has focused on increasing SMN production, either by replacing or correcting SMN1 or by modulating SMN2, the low-functioning SMA “backup gene.” Many of these SMN-based approaches (also called “SMN-enhancing” approaches) are already being tested in clinical trials. Spinraza, the only treatment currently approved for SMA, is also an SMN-enhancing approach.

But research has also revealed that a number of systems, pathways and processes are affected in SMA, and there may be additional ways to treat SMA that work on these other areas. These types of treatments are often referred to as “non-SMN” treatments or approaches.

And perhaps most crucially, these non-SMN approaches could be used in combination with SMN-enhancing approaches, allowing us to attack SMA from all sides and giving us the best chance of a comprehensive, effective treatment.

The grants in this current round of funding go toward both SMN and non-SMN approaches, reflecting our commitment to developing combination therapies to best treat all ages, stages and types of SMA.

More About Our Basic Research Grants

$140,000 grant to Arthur Burghes

$140,000 grant to Arthur Burghes at Ohio State University, for his project “Identification of SMA modifiers and deletion/duplication junctions in the SMA region.”

Dr. Burghes’ project will seek to identify genes that could account for the differences in disease severity between siblings, as well as to help identify targets for therapeutic intervention in SMA.
$140,000 grant to Jocelyn Côté

Dr. Côté’s project will seek to explain how SMN regulates translation, a newly discovered function, and how understanding this process may yield novel therapeutic targets for the treatment of SMA.

$75,000 grant to Chris Lorson

Dr. Lorson and his team are specifically focused on astrocytes, cells known to support motor neurons in the brain and spinal cord, and their influence on lower motor neuron susceptibility in SMA.

$75,000 grant to Stephen J. Kolb

Dr. Kolb and his team will create a large animal model of newborn infants with SMA, and use this model to understand the changes seen in motor neurons, potentially providing insights into our understanding of the timing of motor neuron degeneration.

$75,000 grant to Yong-Chao Ma

Dr. Ma’s project will use a combination of genetic, biological, and biochemical approaches to investigate how increased Cdk5 protein activity leads to motor neuron degeneration, and why this protein displays increased activity when SMN proteins are low.

$140,000 grant to Alberto Kornblihtt

Dr. Kornblihtt and his team will seek to identify cellular mechanisms and drugs that affect epigenetic, or nongenetic, features of the SMN2 gene, potentially increasing production of full-length SMN protein from the SMN2 gene.

$140,000 grant to Oliver Gruss & Utz Fischer

Drs. Gruss and Fischer and their teams will analyze the impact of SMN protein on circuits that control many aspects of a cell’s activity, known as cellular signaling networks, to understand the details of how SMN protein works in the cell and the role SMN plays in overall cell activity.
I am a mother, a rare patient advocate, a supporter of the arts, and a project manager for technical services on the communications team and I have been with Cisco for 15 years. My passion is connecting people and ideas together to make a positive impact in the community.

Five years into my career, Cisco acquired WebEx giving employees robust benefits. Little did I know how invaluable those benefits would become in the years ahead. In fact, many of Cisco’s benefits offered to employees provided an important safety net for our family especially health insurance, health savings accounts, profit sharing and family assistance tools. Many thousands of newly diagnosed rare families are left out of government assistance programs and are forced to fend for themselves or fund raise.

Life can and often does change in an instant. That’s what happened to me with the birth of my only daughter, Malena. At the age of fifteen months, I realized things weren’t quite normal in her development when I discovered Malena had missed her major milestones of crawling and walking.

After seeing the neurologist and waiting two months for the results of her genetic test, Malena received a diagnosis of Spinal Muscular Atrophy (SMA). I was devastated when I learned that SMA was the number one genetic killer of children under the age of two. Individuals with SMA have difficulty performing essential functions of life, like crawling, walking, breathing, and swallowing. I asked myself, now what?

Over time, giving of one’s time began to take on a deeper meaning. I find it is easier to give; I also learned to ask for help. Deciding to openly share my journey gave me the courage to fight SMA. I did not want to hide from it.

In 2008, I took a red-eye to Boston to attend my first medical conference hosted by Families of SMA (now named Cure SMA), and that is when my real journey began. When I heard Kenneth Hobby, the President speak, he reminded me of Cisco’s strategic leadership style. Like Cisco, Cure SMA developed a long-term vision and purpose in which to lead, which helped family members navigate the unfamiliar world of clinical trials and drug development for rare orphan diseases.
I was grateful to have found them. I knew partnering with Cure SMA would save my daughter's life. I was proven right. The standard of care Cure SMA provided helped me navigate the difficult healthcare decisions early in my daughter's physical development. The protocol helped Malena rank in the top 10 percent of her peers regarding her health status. SMA does not affect a person's ability to think, learn, and build relationships with others.

Malena is a vibrant fifth grader. She loves all things fashion. Malena likes to spend time with her friends and attending Golden Empire Elementary. She enjoys acting and wants to become a Director someday. Since the age of two, Malena uses a power chair to get around. Life for Malena is busy. Her schedule includes visits to an interdisciplinary team of specialists, but she never complains.

Malena recently recovered from a left femur fracture and underwent Scoliosis surgery during the summer. Malena is cognitively brilliant but physically fragile. A simple cold can put her in the hospital if she does not follow her respiratory regime. Malena uses several pieces of respiratory equipment to keep her lungs clear, and she also makes time for weekly physical therapy to keep her muscle flexibility. Malena has become a great advocate for her health over the years. We are proud of her grace, kindness and determination to stay independent.

Through the strategic partnership with Cure SMA, surprising outcomes started to develop. I decided to raise funds for the nonprofit as a way to pay it forward to the larger community. I had never fundraised before, but it was important to try. I contacted the local Parks and Recreation team, and the City of Sacramento helped me make meaningful introductions to mentors. It paid off.

Together we raised over $10,000 for Cure SMA during our first Walk-n-Roll. I realized the power of partnerships, community, and self-belief. For several years, the annual October event gave me a positive way to deal with a rare condition and acted as a point of focus during some difficult years. The positive impact the event had on the family and friends who attended was profoundly therapeutic.

I realized I had managed to raise awareness and break my preconceptions of what I could achieve as an individual. People care; give them a reason.

At present, I have had to take a break from hosting the Walk-n-Rolls to balance Malena's needs with my own. I now focus on raising awareness in strategic ways by engaging the business community, and broadening my reach by attending conferences and events hosted by CureSMA and other rare organizations like Global Genes whenever feasible.

Throughout Malena's treatment Cisco has supported me by giving me their trust, flexibility to work from home when I've needed to, and more importantly empowering me to take risks and continue to strive for more—in a way saying, "we believe in you because you make things happen." I am proud to work for a company who stands behind their employees and also encourages us to give back. To me, that says a lot about who they are. As I spend time volunteering, I know it is valued and that gives me the courage to never give up. It’s a great feeling to #LoveWhereYouWork.

Since writing this story, Team Malena, Team Cisco, and the local community have raised over $50,000 towards a cure. The first drug to treat Spinal Muscular Atrophy, Spinraza received approval status from the FDA on December 23, 2016. We are ecstatic! Our hope is Malena will start treatments in 2017, so she can keep the strength that she currently has and improve her quality of life. A real game changer.
Family Letters

Cure SMA,

Thank you for donating everything to us! It warmed our hearts to see all the things our Christopher could hold and play with. I love watching him experience feeling and seeing different objects! We weren’t expecting any of it. We never would have thought to get him some of the things that families donated. He loves it all! It is greatly appreciated!

Samantha Moser
of Pennsylvania
Everyone at Cure SMA,

We want to thank you from the bottom of our heart for the beautiful gift for our son, Matthew. He had so much fun opening presents that our hearts melted.

We don’t know how to thank you because we know that simple words are not enough.

God bless you all
The Kaczor family of Illinois

To Cure SMA:

I cannot thank you all enough for the care package. It honestly brought us all to tears seeing the excitement on Adrian’s face looking through everything. I’m sending along a few pictures because he was so excited it was hard to get a good one!

Thank you so much,
Cassie Daniels & Adrian Diaz of Massachusetts

To Cure SMA,

Yesterday I received the relative packet you sent out. There looks to be a lot of useful information in it. I am excited to read about SMA and what we can do as family to help Jordy. Also, Jordy received his care package yesterday as well. Wow, just wow! There are so many toys and other items in there I could not believe it. Thank you to you and everyone who donated the items. It is so heart-warming to know that people out there care and are willing to help when a child and his family’s life changes so suddenly. I will continue to use the resources and contacts in the package and hope the rest of my family will do the same.

Jessica McCoy of Kansas

Dear Cure SMA,

Thank you so much, this was a big surprise for all of us even my other kids where so excited. You guys put so many smiles on our faces yesterday and we thank you all. Rico wanted to take everything to his school to show everyone.

We all are a family fighting for a cure to help our children and loved ones with SMA.

Maria Aguirre of Arkansas

Thank you so much for the big gift box that was a huge help for us with helping the baby to have toys he is able to better play with.

Veronica Murillo of Texas

Thank you CureSMA, Madison had so much fun opening and showing off her ‘love box’. Thank you!

Sharrall L Phillips of Georgia
Hi Cure SMA,

I just wanted to thank you and your team for the care package and car bed that was sent out to the family. They are so overwhelmed with joy and are very thankful for Cure SMA. I have attached a picture of Ava Francis with her awesome goodies. She has responded very well with the toys and enjoys being in her car bed.

Again, thank you so much for all of your help. You guys are truly amazing!

Charlene Santoyo of California

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Cure SMA,

We want to thank you so very much for the care package. The items from all the families have brought so many tears to my eyes. One of our favorites was the otteroo. I was actually going to order her one when we got home from the hospital but much to our surprise there was one already here. I think is going to be a wonderful tool for her physical therapy and it doesn’t help that she absolutely LOVES the water! Thanks again for everyone’s kindness.

Much love,
Molly Strohm and family of Missouri

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Cure SMA!

Parker received a very large box today! Thank you so much for the many, many goodies that were donated through SMA. Parker was so excited to see everything. He had so much fun with all of the toys! He especially loved the pinwheel and balloon, of course! :) It means a lot to us to have such a supportive community and organization behind us. We look forward to working with Cure SMA in any way we can.

Thank you!
Allie Royal of Florida
I am so honored to have been asked to be apart of the CureSMA family, and to have this opportunity to connect with all of you! I want you all to ask yourself this question, what is better than being yourself?? The answer is, NOTHING! Be proud of who you are, where you came from and all that you represent! Embrace your past, present and welcome what the future brings. We all have things that make us different; it is what makes the world that we live in such an incredibly beautiful place. When I was 18 months old I was diagnosed with Spinal Muscular Atrophy; this affects the strength of my muscles and prevents me from being able to walk. Although my SMA stops me from being able to walk, I dare it to try and stop me from dreaming and conquering my goals. I want you to be empowered and to dare your obstacles from stopping you. There is nothing or no one that can get in the way of you being who you are and who you’re meant to be.

If something seems difficult or out of reach, does that mean you can’t do it? Absolutely not, all it means is that you have to work a little harder and be creative. Never be afraid of doing something a different way if it helps you turn your dreams into reality. In the world we live in, filled with technology and science, there literally is a way to make the impossible, possible!

I believe that there are 5 rules to living the healthiest and happiest you that you can! It doesn’t matter whether you have a disability, if you are rich or poor, if you live in the city or the mountains or even if you’re just a cat yawning on the couch. If you follow these guidelines, you will not only be able to conquer any obstacle that comes along, but you will lead a life that is your own. That is something that no disability or situation can take away from you.

How to achieve the healthiest YOU in any situation

By Jacquelynn Cowles
#1. Support.

I would be lost if it wasn’t for my family, and friends that have become family. I was brought up without excuses; I was encouraged to push through and overcome anything that was thrown at me. No matter how difficult something might’ve seemed, I knew if they were there, everything would be okay. Having a support system in place is something that every individual needs. I don’t care if you are the strongest person in the world or the weakest, everyone needs someone who will be there for them for the highs and the lows. The only way a support system works, is if you let them in. Educate them on what is going on and be open to them about how you are feeling and what you need. When my parents found out that I was diagnosed with SMA they leaned on the support of family to create a support system that was not only for them but for me as I grew up. Educating my family on the fact that I will be able to do anything that I want and that we all needed to join together to push forward as a united front to the world.

When creating your support team, a few things that you want to remember are:

- Choose people who love you exactly the way you are.
- Choose people who will believe in you and your dreams even when you are too afraid to believe in yourself.
- Communicate and educate. Don’t assume that people know how you feel and what you are going through, no matter what it is or who they are.

#2. Mind.

We need to feed our mind and soul the same way that we need to feed our bodies. OK maybe not the same way, I don’t think our mind wants that piece of pizza as much as our stomachs (or maybe it does?). For me, meditation is a great way to not only gather my thoughts, but a way to remove stress and anxiety. When I first moved away to college I had a ton of anxiety. It was the first time that someone other than my family was taking care of me, my first time being away from home and I honestly didn’t know how to process everything at one time. I was introduced to meditation and after some practice (which it does take practice to get used to) I was able to get off my anxiety medication and no longer had the fear of being alone in a room without my support system right next to me.

Meditating is very unique to every person that practices. If you are new to meditating I suggest:

- Finding a quiet & comfortable place, away from distractions.
- Using a guided meditation. There are tons of podcasts and YouTube videos. I will be adding meditations to my site that you can also use.
- Set aside a time that you can commit to every day to give to yourself.

#3. Diet.

As a holistic nutritionist I believe that there is a lot of power in the food that we eat. When we power up our body with healthy food and “super” food, it is able to be at its best and heal itself from the inside out. I’m not going to lie to you, I used to be a fast food addict that was itching to get her next French fry. I had muscle pain and tremors, my body felt extra weak and I could see myself going down a rabbit hole that was nothing like Wonderland. I decided to change my habits with not only meditation but my diet! I eat mostly plant based now and pay attention to staying away from processed foods. After just one week I saw a significant difference in my body. My muscle tremors were less frequent and my muscles felt less fatigue. Could food really do this? Well, it’s been 2 years now and my body has never felt better and my immune system is at it’s prime! Now, just because you’re making healthy choices, doesn’t mean that you can’t have your favorite meal every once in a while. It just means that you make better choices for your body and that you listen to what it needs and how it reacts to what you eat and do.
Create a food plan that works for you!

- Consult your doctor before any major change.
- More plants and less process!
- Think ahead and be prepared for what the day will bring so that you can make choices that fit in with what your body needs.

#4. Activity.

It is so important to keep your body active and do things that make you happy. Happiness, is good for your heart and that amazing smile! Activity, keeps your body at its peak and keeps you healthy and strong. When I say think outside the box, it means to find a way to make your dreams reality. When I was little, any limitations that my friends and I came upon, forced us to us our imagination and to take our creativity to a new level. I wanted to skate like everyone else so we strapped skates onto my feet and I rolled around in my chair with my feet on the ground. There is a way to make every activity adaptable if we just have the imagination and courage to think outside the box! In my world there is no box and I encourage you to break open the box that’s around you.

Be active your way!

- Find an activity that makes you happy!
- Adapt it to fit your needs.
- Remember that not all activities might be easy, but that doesn't mean they are impossible.

#5. Rest.

Sleep is just as important as drinking water. Our brains need to rest in order to file all the amazing things we did during that day. Our body needs to process and rejuvenate itself so that we can wake up wide eyed and ready to rock! It's key to have a space away from technology so that we can fall into R.E.M. sleep (this is when our bodies work best). If you have a hard time sleeping, try lavender essential oils and soothing instrumental music or guided meditations for sleep.

Sweet dreams!

- Have a bedtime (even adults need a one).
- Create a soothing and comfortable environment away from technology and clutter.
- Use essential oils and meditation for rough nights.

Now, take these easy 5 steps and show the world who YOU are and what YOU can do! You might even surprise yourself.

Spread Kindness & Smile Often,

Jax XX
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.
Loving Memories
This section is designed so it can be removed from the center of the newsletter.
Lennon was born on September 19th 2015 and was diagnosed with SMA Type 1 on February 18th, 2016 at five months old. He started clinical trial treatments at St. Louis Children’s on May 11th in phase three of the Nusinersen trial. Lennon fought a courageous battle. When Lennon started the extension phase we saw such physical gain. However, Lennon got pneumonia in August and struggled in the respiratory area. Pneumonia had left its mark on him. Lennon’s spirit never wavered. Although he struggled in the respiratory area it never changed his happiness, or spirit, he simply adapted as we all do.

His spirit and warrior personality will forever be imprinted in our hearts. We lost Lennon’s physical body to SMA Type I on December 3rd 2016. Even though SMA took his body, it never took his spirit. The spirit of Lennon was so sweet, strong, bright, and a little mischievous. He took all of the treatments that he endured with grace and went through more than a lot of children and adults. He even helped do some of his treatments.

As a beautiful rare flower he bloomed, he shared his beauty and left behind his seed. The flower may be gone but the beauty will shine in the seed that he planted and left behind. We’re all better people for knowing the heart and spirit of Lennon. His beauty shines on with all who knew him.

So, please accept this donation in celebration of Lennon, our little warrior.

Sincerely,

Anthony and Peagon
We call her Mimi. August 31, 2010 was the happiest day of our life, that day Roumaissa was born. The first baby in our family; a healthy happy baby girl until 9 months old we noticed she wasn’t meeting typical milestones for a child her age, such as crawling and standing. We were concerned so we brought her to see a pediatrician. After examining her and learning more about her symptoms the pediatrician said she will never walk and she might have a neurons disease.

At 14 months old (October 17, 2011) Roumaissa was diagnosed with SMA type II. A disease that we had never heard of it. We remember that horrible day; we left that appointment sad and overwhelmed. It was very difficult at first and the hardest part was thinking about the things as a child she might not experience such as first steps, playing, going to school but we’ve adapted over the years.

Roumaissa inspired us to keep fighting the fight against SMA; every day she was strong and patient. Her sense of humor and the appearance of the wisdom beyond her age made us forget about her disease. She was a smart, lovely and beautiful girl with huge personality stuck on a weak body.

This year Roumaissa was in first grade like all the kids her age but she was very special in class not because of her disease but because she was excellent in her classroom. Her teacher was very proud of her and she said that she was beyond her grade. Roumaissa was very smart, she loved math and her dream was to be a doctor.

December 23, 2016 was the happiest day in our life when the FDA announced that it had approved Spinraza to treat spinal muscular atrophy making it the first ever FDA approved therapy for SMA. She was so excited to start the treatment as soon as possible she was living with hope that her dream will come true, the FDA approval was the best news ever and made her happy.

December 28, 2016 Roumaissa was admitted, she had trouble breathing and her oxygen level was very low but we didn’t lose hope we were fighting with her and would do anything just to keep her alive. We can’t describe the pain that our heart felt as she left this world our life completely changed when Mimi stopped breathing, Roumaissa lost her fight with SMA on December 30th 2016.

Mimi is in heaven with no worries, anger, pain and we fully trust that God will heal our hearts, There are things that we cannot control, we leave that for God’s sovereignty and we are satisfied with all that God gives us and takes from us; he knows better than us. We are nothing without God.

Roumaissa was such a blessing in our lives and to all who had the chance to share her with us and it was such an honor to have her as our daughter. We are so thankful for the pictures and videos and memories we have of our sweet girl. We had so much fun together, she was amazing. Mimi will forever be in our hearts, we love you beautiful angel.

Arioui Mohamed, Mesdoua Hassiba and Freh bengabou Asmaa
Hi,

My name is Beverly McManus, my son Damon had SMA type II. He passed away suddenly back in October 2015. I’ve attached a picture of him with all his “loot” from his newly diagnosed package. He was born on 08/23/2013. He was our surprise. He was warm, funny & extremely intelligent. He loved his family and was adored in return. He and his big brother Colin had a very strong bond even though Damon was only 2 when he passed away. He was goofy and sweet but could be very stubborn. He wanted to do everything himself. With SMA that’s fairly impossible, so we would find compromises of how much help he would accept. Anyway, he was a social butterfly like his older brother and loved being around other kids. We thought one way we could honor him for his 3rd birthday this last year was to collect PipSqueak markers to donate for the newly diagnosed packages. We received donations from friends, family and the Northern CA chapter members of Cure SMA at the Walk-N-Roll, since we celebrated both his birthdays there. Damon really liked them and they helped make it easier for him to draw since they aren’t as long as regular markers. I also had a beanie baby collection that needed a home. Damon loved the few Beanies that he had and we thought that other SMA kids would appreciate them as well, so we sent a large box with the beanie babies and pip squeak markers.

Thank you all for what you do. I truly hope that our donation blesses many families.

With gratitude,

Jason, Bev, Colin & Angel Damon McManus
August is SMA Awareness Month

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

925 Busse Road, Elk Grove Village, IL 60007 • 800.886.1762 • Fax 847.367.7623 • info@curesma.org
### Cure SMA Chapters

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

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**Cure SMA National Office**

925 Busse Road
Elk Grove Village, IL 60007
1.800.886.1762
Alabama Chapter Walk-n-Roll

The 13th Annual Alabama Walk-n-Roll was held at Cahaba Lily Park in Helena, AL on October 29, 2016. The Walk-n-Roll was a huge success and raised $35,683. We had 115 people in attendance. Everyone enjoyed the face painting, games, silent auction, and amazing food from Southern Food Service. We are grateful to all the sponsors who generously donated to the cause. Thank you to Jennifer and Jason Patrick and their amazing team of volunteers for organizing this event!

A special thank you to our teams: Baylee Grace Peterman - In Memory Of, Team Cadence, Team Carter, Team Charlotte, Team CJ, Team Hadley, and Team Logan

Hats Off to SMA

On August 13, 2016 Tammie Tubbs hosted Hats Off to SMA in memory of Aaron and Terrance Jr. Tubbs in Starkville, Mississippi. Tammie raised $259 to support Cure SMA. Thank you Tammie and all that participated.
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

### Arizona Chapter

#### 2016 Arizona Walk-n-Roll

The 2016 Arizona Walk-n-Roll was held on November 12, 2016 at the Steele Indian School Park in Phoenix, AZ. Family, friends, teams, sponsors, and vendors from the area came out to make the day a huge success. This year’s event raised over $20,000! A delicious lunch was provided by the Arizona Barbeque Association followed by an amazing raffle!

There were a variety of games and activities for individuals to take part in before and after the Walk-n-Roll. A special thank you to the volunteers from the Boys Team Charity, the Occupational Therapy students from A.T. Still University and nursing students from Grand Canyon University. It was a great day for families to connect and allow the children and young adults to hang out, play games and enjoy each others company. Thank you to everyone for coming out and supporting this event.

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

### SMA Dinner and Candle Lighting

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

### Carolinas Chapter

#### 2016 Carolinas Chapter Walk-n-Roll

The 2016 Carolinas Chapter Walk-n-Roll raised over $12,500! Many SMA families and friends gathered at Anderson Point Park in Raleigh, NC for an afternoon of fun! Everyone enjoyed the fun games and photo booth along with a moving speech by Mimi Chan about her son’s journey with SMA. Thank you to Amy Deidrick, Carrie Ann Boles, Jennifer Lee, and Bekka Mastin for organizing this event!

#### Inaugural Evening of Hope – Wine and Art Auction

The Inaugural Evening of Hope – Wine and Art Auction was held on October 1, 2016 and showcased local artists and wineries of Arizona. Friends and family gathered to enjoy cocktails, hors d’oeuvres, live and silent auction items, and an evening out. Guests were able to take home a special bottle of wine with a custom SMA label. The event raised over $8,000 for a cure for SMA. A special thank you to Bo Buchanan for spearheading this event for Cure SMA.
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

2016 Cure SMA
Go Miller Go 5K
South Carolina

The 2016 Cure SMA Go Miller Go 5K raised nearly $50,000! Lindsay and Eason David started the event in honor of their son, Miller. This year’s event, held in Charleston, SC, brought together over 350 people at James Island County Park to raise money for Cure SMA. Thank you and congratulations to the David Family on the incredible success of this event!

Our Little Jewels Golf Outing

On Friday, September 23, 2016, Our Little Jewels held its Benefit Golf Outing. The event was held at The Links at Challedon in Mt. Airy, MD. One hundred and twenty-eight golfers participated. Over $35,000 was raised for to help fund research efforts of Cure SMA. Thank you to Jim Lewis and Our Little Jewels for their support of Cure SMA!

2016 Chesapeake Crab Feast and Silent Auction
Maryland

The Chesapeake Chapter held its 23rd Annual Crab Feast & Silent Auction on August 7, 2016. Over 350 crab lovers from all over Baltimore filled the Towson American Legion Hall to enjoy 300 bushels of crabs and cold beer. In addition to picking crabs, the attendees enjoyed games of chance, silent auction, and a wine and dine raffle. This year’s event raised over $43,000 for Cure SMA! In conjunction with August being SMA Awareness month, attendees were asked to spread the word about SMA by posting during the event on social media with the Cure SMA wrist bands that were given out to the attendees.

Beverly Venedam | New Freedom, PA
Congratulations to the Arnold Family for celebrating their 20th year hosting the Annual Arnold Family Golf Outing. This year's event was held on October 10, 2016 at The Berry Hills Country Club in Charleston, WV. This golf tournament started in 1996. Kevin Arnold wanted to know what his wife, Linda, wanted for her birthday. She decided she wanted to do something for Cure SMA in honor of their son Eric. It is such an amazing milestone and the Arnold's have been so dedicated to helping us find a treatment and cure, as well as building hope within our community!

The Arnold's are grateful to their wonderful group of friends and family who have supported them throughout the years. They are a special group of people who have been with them for 20 years and are not only there for them in October but all the days in between! They are truly blessed to have their support! Eric has been an integral part of making the tournament so special. Thank you, Linda and Kevin, for hosting yet another successful golf outing and for your endless support of funding promising research for SMA!

On August 13, 2016, the Steamboat Landing Camp Ground hosted the Steamboat Landing Bingo Fundraiser in honor of Olivia Calvert from Baltimore, MD. Steamboat Landing raised an incredible $1,500 to support Cure SMA. Thank you to the participants for another successful bingo and support!

Thank you to Aaron and Ashley Close of the Chesapeake Chapter in Accident, MD who generously raised $641 with their friends and family for Cure SMA. The donation made is in memory of their son Owen Jonas Close.
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

Georgia Chapter

Rock ‘n’ Roll Savannah Marathon and Half Marathon

Runners from across the region, as well as several from across the country, came together in Savannah, GA for the Rock-n-Roll Half Marathon to benefit Cure SMA on November 6, 2016. The runners worked hard to more than double their fundraising goal in raising $12,000 to benefit the charity and its ongoing efforts. Thank you to Kari Merriken and her family for starting the first team and spreading the word throughout Savannah!

A special thank you to our runners: Emily Bryant, Kathy Cooper, Christena Dickens, Benjamin Dickens, Gina Geffre, Kyle Likens, Kari Merriken, Kanaan Merriken, Alison Morgan, Savannah Ott, Thomas Ott, April Sayers, Kelly Scardina, Lauren Wood, Brittany Young, Michael Young, Jake Young, Ruth Young, and Tom Young.

Greater Florida Chapter

2016 Greater Florida Chapter Walk-n-Roll

The Greater Florida Chapter’s 8th Annual Walk-n-Roll, held on November 12, 2016 at Lake Parker Park in Lakeland, FL was a great success! With a record number of 21 teams and 231 participants, we raised a little over $27,672, exceeding our $20,000 goal! Everyone at the Walk-n-Roll enjoyed “Quest for the Cure” themed activities and entertainment.

Thank you to all of our amazing teams, participants, and donors for making our annual walk a huge success.

An extra special thanks to our Greater Florida Board Members and Event Organizers: Audra Butler, Laurie Sore, Asia Nowothy, and Katie Kerns!

Thank you to all of our teams for participating in this year’s event: Abigail’s Avengers, Allison Wonderland, Andy’s Army, Arms for Asher, Brooks Wolfe Pack, Chris’ Charisma, Ed’s Team, Ember’s Fight, Maia’s Mission, Mama Needs a Miracle, Team 2 Angels, Team Gabby, Team Lucas, Team Tianna, The Home Team for Ezra, and Who said that.
CHAPTER UPDATES

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Maluko Golf Outing

For the past eighteen years, Chris Leto and Carlos Menendez have donated a portion of the Maluko Golf Tournament’s proceeds to Cure SMA, in honor of Tyler Hernandez and his family. On October 24, 2016, the 24th Annual Maluko Golf Tournament took place at the Carrollwood County Golf and Country Club in Tampa, FL. One hundred and twenty eight golfers participated in this year’s event, raising $25,915 for Cure SMA! A huge thank you to everyone involved with the Maluko tournament – you are amazing friends of our cause!

Hope for Henry Golf Tournament

On September 25, 2016, the annual Hope for Henry Golf Tournament was held at Hacienda Hills Country Club in The Villages, FL. The event had a sunny day of golf, which included a silent auction, raffle, a captain and crew 4-person scramble with shotgun start, and closing ceremonies and awards. The event was a great success and raised $27,642. A special thank you to Bob Chartock and his committee for organizing this annual golf tournament in honor of Henry White.

Costume Crusade at Village Early Learning Center

Complete with haunted houses, trick-or-treating, face painting, bounce house, and lots of fun, “The Village” and the Village Family Foundation hosted another great community event on October 28, 2016 in Brandon, FL raising more than $2,500 for Cure SMA! Thank you to Susan and Joe Miller for spearheading this event each year!

SMA Awareness Day with the Tampa Bay Rays

Friends and family members came together on August 21, 2016 to watch the Rays take on the Texas Rangers 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!

Jacksonville Bake Sale

In Jacksonville, FL Lily Bielling held a bake sale on behalf of her cousin, Parker Royal. She raised $249. Thank you Lily for your hard work in raising money for Cure SMA!
2016 Greater NY Chapter Walk-n-Roll

This year marked the 12th Annual Greater New York Walk-n-Roll. The day was filled with great activities for participants of all ages, including a 50/50 raffle, face painting, and a pumpkin patch for children to enjoy! It was a beautiful day at our new location, Hofstra University in Hempstead, NY. The Greater NY Walk-n-Roll was held in memory of Emily Lozina.

Thank you to all of our amazing teams, participants, and donors for making our 12th Annual Walk a wonderful day and huge success. This year’s event raised over $35,000 that will go towards funding critical SMA research and family support programs.

An extra special thanks to all of our teams for participating in this year’s event: Fight For Owen, Florida Skylanders, Friends of Julia, Sweet Baby Jack, Team Emily, Team Fab 5, Team Farmingdale, Team Juliana, Team Kiley, Team Ron, The Cofone Family, and the Philly Cheesesteaks. Thanks to Debbie Cuevas and all of the volunteers for organizing this amazing event.

2016 TCS New York City Marathon

Five incredible runners from across the country came together to run for Team Cure SMA in the 2016 TCS New York City Marathon on November 6, 2016. The runners trained hard and surpassed their fundraising goal by raising $26,985!

Special thanks to our runners: Diane Dunn, Alex Kostyuchenko, Lauralee Slaymaker, Thomas Stanley, and Sarah Yadon.

Women’s Syndicate Association Donation

The Women’s Syndicate Association raised $700 throughout 2016 to benefit Cure SMA. Thank you to Carey Brown for nominating Cure SMA to benefit from the Women’s Syndicate Association’s events.

Julie Harbey, The Women’s Syndicate Association| New York, NY

Calhoun Class of 2019 Fundraiser

Julia Lincoln raised and the Calhoun Class of 2019 raised over $250 on behalf of Julia by selling Cure SMA bracelets. Thank you Calhoun Class of 2019 and community members of North Merrick, NY for your support.

Noman Afzal- Individual Fundraiser

Thank you Noman Afzal for creating an individual fundraising page and raising $200 for the Greater New York Chapter.
Owen Schler Fundraiser

Thank you Arlene Simpson and her class at James A. Farley Middle School in Stony Point, NY for raising $200 in memory of Owen Schler. The class worked hard in December 2016 to organize a fundraiser, which will benefit the Greater New York Chapter of Cure SMA. Thank you to all who participated and generously donated to Cure SMA.

2016 SMA Awareness Day with the NY Mets

Friends and families affected by SMA had a great day at the Citi Field cheering on the New York Mets on September 25, 2016 in Flushing, NY. The Greater New York Cure SMA Chapter had a wonderful time raising awareness and went on the field to accept the game Spirit Award. After the game, the kids were able to go on the field and participate in the Mr. Met Dash where they ran and rolled around the bases! Thank you to everyone who came out to show your support.
5K & Walk-n-Roll in Memory of Trooper Cunniff | Milestone Event

On September 17, 2016, The Greater New York- Capitol Region Chapter held its 5th Annual 5K / Walk-n-Roll in Cohoes, NY. We raised $22,300 for Cure SMA and had so much fun doing it. We welcomed two newly diagnosed families into our chapter and the morning was spent making new friends and connections, while remembering those who have lost their battle. These events are always bittersweet, but it always amazes me how being with other SMA families encourages hope in all of us.

We continue to hold this event in memory of NYS Trooper David Cunniff, who founded our chapter. He made a promise to his son that our family would not stop fundraising until there was a cure. We continue to remember and honor Dave, Shawna Sullivan, Josephine Lucy, and Greyson Kelly while we work towards the day there is a cure! Special thanks to Amy Cunniff for organizing this event!
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

**Illinois Chapter**

### 2016 Chicago Half Marathon & 5K

Members of Team Cure SMA ran the Chicago Half Marathon & 5K on September 25, 2016, in Chicago, IL. This is the sixth year that Cure SMA participated in the run as an official charity partner and it was a special year as Koen Guest, was able to be a part of the race as his mom, Noel pushed him in a special stroller all 13.1 miles!

Together, the runners raised over $15,000 to help cure SMA! Cure SMA would like to thank all of our runners; Noel Guest, Koen Guest, Janet Hamilton, William McAllister, Michelle Yohler, Julia Piech, Laura Duffy, Janet Hazboun, Edward Hazboun, Emily Esposito, Nicole Esposito, Erik Hort, Ashley Golda, Alyssa Gravitt, Donnie Gravitt, Sierra Gravitt, Ron Gravitt, Jack Gonciarczyk, and Tommy Hileman for their hard work in training to run this race as well as all their fundraising efforts!

*This year’s Chicago Half Marathon will take place on Sunday, September 27, 2017*

### Illinois Garage Sale

On August 21, 2016 Maria Marusich hosted her annual garage sale in Elk Grove Village, IL and raised an incredible $1,400 to help Cure SMA! Thank you to Maria for your continuous support of Cure SMA!

### Cure SMA Day with the Chicago Cubs

The Illinois Chapter had a great night on August 29, 2016 at Wrigley Field in Chicago celebrating SMA Awareness Month at the Cure SMA Day with the Chicago Cubs. More than 80 supporters and friends came together to raise awareness for SMA and to cheer on the Chicago Cubs to victory! We are looking forward to doing it again this year on August 2, 2017, and we hope you can join us!

### Cure SMA Illinois Chapter Holiday Party

The Illinois Chapter hosted a holiday party on Saturday, December 10, 2016 at Lurie Children’s Hospital in Chicago. More than 100 family members and friends came together for an afternoon full of holiday fun! Lunch was provided and there were games, music, cookie decorating and arts and crafts available for all the kids to enjoy! We also had a special visit from Santa who came and took pictures with each kid!

*Cure SMA would like to give a special thanks to AveXis for sponsoring this fun get together for SMA families! We would also like to thank Jenny Kean and Rachel Shine from Lurie Children’s for their help in organizing this fun family event!*

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*DIRECTIONS Spring 2017*
Cure SMA 5K with Small Heroes, Mighty Cause

The 2016 Cure SMA 5K with Small Heroes, Mighty Cause was held on August 5, 2016 and raised over $35,354. The Friday evening event was filled with fun 5K walk at Cascade River View Park in Cascade, IA. The event had over seven teams participate, and over 200 racers!

A special thank you all the event sponsors, and the Schlemme Family and their support to plan this event in honor of their son Colin.

Sharing Photos

Camden Fuller
Ismael Ferrer
Lizzy Huette
Jordy McCoy
Matthew Kaczor
Kalen and Kyan Kulas
Beaverdale Beaverdash

The 20th Annual Beaverdale Beaverdash took place on September 17, 2016 in Des Moines, IA. It was a beautiful day and the event was once again a success! Over 300 people gathered to run, walk, or roll. This year’s Beaverdash raised over $18,400 for Cure SMA! Thank you to all of the volunteers and everyone who participated in the event! A special thanks to Julia Anderson and Julie Greenwood for organizing another successful event and reaching a huge 20 year milestone!

This race wouldn’t be possible without the dedication from the teams and their amazing fundraising efforts: Fight for Cael, Love for Lily, Meimann Mania, Race 4 Grace, Team Castoridae, Team Lane Walker, Walk with Scott and Walking in Memory of Caden Underwood!

Keep up the good work and keep raising funds to find a cure for SMA!
The 13th Annual Bommarito Z Club Car Show took place on September 6, 2016. The event had great attendance and 55 cars. Honoring Brittany Carpenter, and in memory of Madeline Schmidt, Michael, and Brian Goodyear. Thanks to Steve & Chris Colesworthy, Jeff Polsgrove, Janet Hutchinson, and all members of the Z Club. A total of $23,731 was raised to benefit Cure SMA.

2016 Cure SMA Neola Walk-n-Roll in honor of Maisy

The Inaugural Cure SMA Neola Walk-n-Roll in honor of Maisy kicked off on August 20, 2016 and raised over $4,000! The wonderful Saturday morning was filled with a fun 5K walk at Arrowhead Park in Neola, IA. A special thank you to the Wiggins Family and their support to plan this even in honor of their daughter Maisy.

Team Cure SMA at the 2016 Rungevity Rock ‘n’ Roll St. Louis™ Marathon & Half Marathon

This was the second year Team Cure SMA participated in the 2016 St Louis Half Marathon on October 16, 2016 in St. Louis, MO. The event raised $4,110 for Cure SMA!

A big shout out to our Team Cure SMA runners: Abby Rheinecker, Amber Platt, Brooke Kelly, Bryce Bushmeyer, Callie Bushmeyer, Kim Bushmeyer, Chuck Robbins, Stefanie Rennecker, and Whitney Robbins! Thanks for your hard work and training.

2016 Cure SMA Neola Walk-n-Roll in honor of Maisy

2016 Cure SMA Neola Walk-n-Roll in honor of Maisy

Kansas City Chapter

Annual Chapter Walk-n-Roll

We want to thank everyone who participated or volunteered for our 16th Annual Walk-n-Roll. This event could not be successful without each and every one of you. The 16th Annual Kansas City Chapter Walk-n-Roll was held on August 27, 2017 in Roeland Park, KS. We had more than 113 people sign up to support our families and raised over $43,000. It was another fun, successful event for the Kansas City Chapter of Cure SMA. More than 13 teams were formed to help reach our fundraising goal to help fund a treatment and cure. We are looking forward to more fun events in the future! Thank you to Kristal Wilson, Kim Sykora, and their committee for organizing this year’s event!

Scare Away SMA

Over $1,500 was raised in honor of Matilda (Tilly) McRoberts and the Derrington Family through the Wesbury Manor Hunted Forest in Missouri for the “Scare Away SMA” fundraising efforts. Thank you, Dana Derrington, for organizing such a great event!

Drew Derbie’s Craft Fair

Thank you to Shelly Madsen from Omaha, NE for hosting an annual craft fair in honor of their son Todd. Drew Derbie’s made $400 to help keep fighting to find a cure!
Shayla Waddell Racing Fighting Against Spinal Muscular Atrophy  

In 2016, Shayla Waddell Racing (SWR) set out to embark on their ninth year of sprint car racing in the southern plains. It was from Isaac’s courageous battle of SMA that the SWR team considered putting together a program to aide in the awareness of the disease while setting out to create a revenue source to assist medical science in finding a cure as time waits for no one. In total, Shayla raised $4,000. Thank you Shayla, Nick Newton, Brand Racing Engines, Andy Gordon, Brian & Justin Potter, Outlaw Wings, Unlimited Products, Royal Purple, Wilwood, Total Seal, Wayne Varley, Brodix, SRP Racing, JE Pistons, Maxima Racing Oils, and Mike Howard Photos are some of those who assisted in SWR’s efforts.

Talmer Bank Detroit Free Press Marathon, Half Marathon, and 5k

Our first year as a charity partner with the Talmer Bank Detroit Free Press Marathon, Half Marathon, and 5k, was a huge success! We cannot thank all of our amazing runners enough who helped us exceed our goal to raise $3,616! Cure SMA would like to thank Sarah Abraham, Kelly Forshey, Alissa Miller, Amy Kolb, Jen Gonzalez, Kristy Hetzel, Raquel Gomez, Angela Miller, Sharon Hill, Jamie Veith, and Dianna Judge who helped to raise awareness as they ran through the streets of Detroit!

We would love to have you be a part of Team Cure SMA as we partner again with the Detroit Free Press Marathon for their 40 anniversary on October 14 and 15, 2017 Join us at next year’s race!

SMA Awareness Day with the Detroit Tigers

A huge thanks you to Ben, Gretchen Jace, and Charlie Dorer for their help with the Detroit Tigers Game Fundraiser! They were able to raise $220 from the Detroit Tigers for each ticket sold for the game on September 25, 2016. It was a beautiful day for baseball and showing support for Cure SMA.

Fall Turkey Trot and Summer Yard Sale

On October 7, 2016, the community of Yellville, AK raised over $3,147 in remembrance of Cason William Bauss and Ember and Solara Hinson during the Fall Turkey Trot and Summer Yard Sale. Thank you Lisa May, grandmother of Cason, and members of the Yellville community for your support in raising these funds.
CHAPTER UPDATES

Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

Minnesota Chapter

Minnesota Walk-n-Roll

On Saturday, August 27, 2016 the Minnesota Chapter held its sixteenth Annual Walk-n-Roll at Central Park in Roseville, MN. Even though we didn’t have the best of weather with some rain and the wet-ness, we had an impressive turnout. With all the support we raised almost $38,000. We want to thank all the organizers and volunteers for helping with this event. Also thank you to all who donated silent auctions items, bake sale goodies, and prizes for the games.

A special thank you to the Minnesota Chapter leaders: Jamie Mevissen, Kara Forcier, Matt Czech, and Jonathan Schwerr. We look forward to having our next walk-n-roll at the same location.

New England Chapter

Darlin Racing Event

The Darlin Racing event was held on October 2, 2016, at Beechridge Motor Speedway in Scarborough, ME. Brian Darlin has a cousin whose daughter has SMA, and he races in the truck division all summer on Thursday nights at Beechridge. After winning the championship, he added all of his winnings for the season, and sent it to Cure SMA. He raised $2,000! Thank you, Brian, for all of your support to Cure SMA.

Portland Sea Dogs Game and SMA Awareness Day

On August 7, 2016, 30 supporters and friends came together to raise awareness for SMA and to cheer on the Portland Sea Dogs as they played against the Akron RubberDucks at Hadlock Field in Portland, ME. Cure SMA families were invited to walk around the warning track as they were introduced and Daisy Bessey threw out the first pitch of the game. Also, Ethan and Emily Bessey were later interviewed on the radio during the seventh inning. Thank you Emily, Ethan, and the rest of the Bessey family for participating in such a fun day.

Also, thank you Joel and Jenny Arnold for coordinating such a successful event.

Cure SMA 5k

On August 27, 2016, 249 participants came out in support of the 3rd Annual Cure SMA 5K at Lake Quannapowitt in Wakefield, MA. It was a beautiful and sunny summer morning, and the race field was full of runners, walkers and rollers who enjoyed a picturesque trip around the Lake. Registered participants received an awesome race t-shirt, headbands generously donated by Bondi-Bands and other goodies. The course was chip timed by Race Wire and everyone enjoyed music and event emceeing by Kevin Wood, DJ. Over $30,000 was raised for Cure SMA, and we are so grateful to all who ran, walked, rolled, strolled, supported, volunteered and donated! Finisher prizes were awarded to the top three men, top three women, and top “wheels”.

We are looking forward to the fourth Annual Cure SMA 5K on August 26, 2017 and will be excited to have even more participants and more fun this year! Thank you to Kristen Farrell for organizing this successful event.
Falmouth Road Race

The Falmouth Road Race has long been a favorite race for casual and elite runners alike, and it is now becoming an annual event for members of the Cure SMA community. On August 21, 2016, 20 runners representing Cure SMA culminated their fundraising efforts to run the seven miles in the 80+ degree heat from Woods Hole to Falmouth Heights. Together, we raised over $27,000 for Cure SMA!

Since the Falmouth Road Race Cure SMA team formed in 2015, each year we have expanded our team and our fundraising goals. The generous support of our donors has also allowed our team to grow from 10 in 2015 to 20 in 2016. To date, we have raised over $41,000, and next year our goal will be even more ambitious as we have been awarded 30 bibs for the 2017 race.

Lin LaValle, Team Cure SMA Captain

Ride for Alex

The 10th Annual Ride for Alex was held in Freetown, MA, on Sunday, September 18, 2016. We had one of the biggest turnouts to date, including the most motorcycles and raised $5,819 for Alex and Cure SMA! Alex was able to make it, which is always a wonderful thing. Everyone always loves to see him there to say hi, or even meet him for the first time, and are amazed about what an incredible boy he is. Thank you to Tammy Foisy for continuing to support Cure SMA in honor of Alex.

Boston Night of Fashion Gala

The first Boston Night of Fashion Gala took place on Saturday, October 15, 2016, at the Holiday Inn in Brookline, MA. Three local fashion designers—Samuel Vartan, Lori Kyler Christensen and Kim Pham—donated their time and talents to this inaugural event. The Boston Night of Fashion Gala was a fun night enjoyed by all and together, they raised almost $7,500! Thank you to Dan Marshall and Raj Zambre who brought their idea of a fashion show to Cure SMA a year before it became reality!

Medfield Pub Crawl

This is the third year in a row that our community rallied in the beginning of November for a fun, social event to raise money in support of our neighbors, the Johnson family. Our numbers were so high this year that we had to break off into groups as to not overwhelm our gracious hosts – the local Medfield establishments of Nosh and Grog, Avenue, Noon Hill Grille, Basil’s Restaurant, Jing’s Garden and Rock ‘n Roll Rib Joint – who welcomed us with open arms in support of such a worthwhile cause. We raised $3,775! We hope that this contribution can make a difference.

Thank you to Renee and Joe Romanowski for all that you do to help find a cure for this debilitating disease.
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

**Lowell Spinners SMA Awareness Day**

On August 21, 2016, the Lowell Spinners played the Brooklyn Cyclones at LeLacheur Park during the Lowell Spinners SMA Awareness day. The Lowell Spinners game was attended by 20 people and raised $973. Fans cheered on as Matty Davidopoulos threw out the first pitch. Thank you, Courtney Davidopoulos, for your continuous support of Cure SMA.

**Medfield High School Girls Basketball Team**

The Medfield High School Girls Basketball Team held a game on December 11, 2016, with all donations going directly to Cure SMA. They raised $632. Thank you to everyone who played and who donated.

**Fisher Cat Game & SMA Awareness day**

New Hampshire

On August 21, 2016, Cristle Gordan coordinated the Fisher Cat Game & SMA Awareness day at Manchester’s Northeast Delta Dental Stadium. $890 was raised. Thank you so much Cristle, and the baseball players who participated in this game for your continuous support of Cure SMA!

**SMAshing SMA**

Vermont

Thank you everyone who participated in the Second Annual SMAshing SMA. Held on October 15, 2016, at Dustan Field in Common, VT, participants ages 5-13 engaged in soccer clinics led by the coaches of the Girls’ and Boys’ high school varsity and smashed 40 pumpkins. They raised $165 for Cure SMA in honor Emma and Ruby Cannady. A huge thanks to Casey McGill for making this event a success.

**Rhode Island Walk-n-Roll**

Rhode Island

The 2nd Annual Rhode Island Cure SMA Walk-N-Roll took place on Saturday, October 1, 2016, in Bristol, RI. A record nine teams participated including Team Evan, Team Angelina Rose, Team Tyler and Team Roman. About 200 walkers and rollers met at Colt State Park that morning for the 1.5 mile picturesque walk, ending along Narragansett Bay. Snacks and a raffle followed. We’re so grateful to have raised $35,827! We look forward to the 3rd Annual Rhode Island Walk-n-Roll on September 23, 2017. Thank you, Lynne Vaudry, for your continuous support of Cure SMA!
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

**New Mexico Chapter**

2016 Bugaboo Workout of the Day

On September 24, 2016, the New Mexico Chapter, CrossFit Albuquerque, and lululemon athletica hosted the annual Bugaboo Workout of the Day (WOD) at lululemon athletica in Albuquerque Uptown. The weather was perfect and brought over 160 participants for a morning of workouts and awareness. The event raised over $14,000 for Cure SMA in memory of Benjamin “Bugaboo” Abruzzo III.

A big thank you to lululemon athletica and Ben and Natasha Abruzzo for organizing this successful event!

**North Texas Chapter**

North Texas Giving Day

On November 4, 2016, the Communities Foundation of Texas raised $102 dollars on behalf of Cure SMA in the North Texas Giving Day 2016. Thank you to the staff and Board of Trustees of Communities Foundation of Texas for its efforts in raising money for Cure SMA. Thank you to Michele Erwin for spearheading this effort.

**Northern California Chapter**

2016 Cure SMA Walk-n-Roll

Our 14th annual Walk-n-Roll in San Francisco on August 20, 2016, was a blast! Dr. John Day from Stanford University Medical Center updated us on the latest SMA research. Then we warmed up with energetic Zumba exercises before heading out on our two-mile walk through beautiful Golden Gate Park. We paused at the Conservatory of Flowers for our traditional photo before proceeding to Rainbow Waterfall and back to Peacock Meadow. The fun continued in our green space area with face painting, balloon creations, tattoos, nail salon, games, lunch, and a lively raffle. We raised over $62,000 for Cure SMA.

Thank you to our families and friends for their generous donations and to our sponsors: Biogen, AveXis, Cytokinetics, Sutter Health Palo Alto Medical Foundation, Live Rhythstrong Foundation, Hand & Stone Massage and Facial Spa, Convaid, Echos, and ProBed Medical USA. Thanks to our silver sponsors Convaid, Echos, and Mobility Works. Let’s do it all again in 2017!

**Kendra Gives Back Party**

On August 8, 2016, Kendra Scott and Cure SMA hosted an evening of shopping, refreshments and awareness at Kendra Scott stores in Austin and Houston. During each event Kendra Scott donated 20% of sales, raising over $1,100 for Cure SMA. Thank you to Kendra Scott for partnering with Cure SMA for this event, as well as volunteer Kirstyn Speich for all of her help on event day!
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OKI Chapter

2016 Cure SMA Golf Outing in Memory of Aubrey Grace Lyden

The 2016 Cure SMA Golf Outing in Memory of Aubrey Grace Lyden held on August 6, 2016 in Lake Milton, OH was a huge success! Thanks to the dedication of Sean and Dannete Lyden, in memory of their daughter Aubrey, $16,665 was raised. Over the last five years, the Lydens have raised over $93,000 for Cure SMA! Thank you to the Lyden Family and their incredible network of supporters for their generous support.

Dayton Walk-n-Roll in Honor of Emmett Keeton

The Dayton Walk-n-Roll in Honor of Emmett Keeton held on September 11, 2016 raised over $12,000! Everyone gathered at Eastwood MetroPark for an afternoon of superhero themed fun. Thank you to Rachael Rodgers and the Keeton Family for organizing this event for the fourth year!

Run Run Rudolph Cake Sale

Eleven year old Luke Border baked cakes to raise money in memory of his sister, Lainie in December 2016. He and his family took orders from neighbors and ended up baking and delivering 36 “Run Run Rudolph Red Velvet” cakes to raise a total of $555 for Cure SMA. Thank you Luke, Julie, and Bret Border and all those who bought cakes to support Cure SMA.
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### Cure SMA Cure Kirsten 5K/10K

Thank you to Wayne Cornett and his family for hosting the 2016 Cure SMA Cure Kirsten 5K/10K for the second year! This year’s event held on October 17, 2016 in Paintsville, KY raised over $7,000. Local friends and runners laced up their shoes to help find a cure for SMA for Kirsten Cornett and all those with SMA.

### Christmas Craft Fair in Honor of Annie Grimes

Thank you to Garrett Grimes for his generous donation to Cure SMA! Garrett is in sixth grade and a member of the Jr. Beta Club in Bowling Green, KY. As his service project this year he made wooden block picture frames and sold them at the annual Christmas craft fair on December 8, 2016. All $850 in proceeds were donated in honor of his sister, Annie Grimes.

### Stakz Self-Serve Frozen Yogurt Fundraiser

Thank Anthony and Peagon Popovich in Bowling Green, KY for hosting a fundraiser at Stakz Self-Serve Frozen Yogurt in celebration of Lennon Taylor Popovich’s life. They raised $150 to benefit Cure SMA. Thank you to Anthony and Peagon, and to all who donated.

### Deacon Alexander Memorial Car Show

The Deacon Alexander Memorial Car Show benefitting Cure SMA was held on August 13, 2016 in Walton, KY. The event included a walk-a-thon and fun family activities. Thank you to the Perry Family for their continued support of Cure SMA in memory of Deacon!
2016 Marion, Indiana Walk-n-Roll

The Cure SMA Marion, IN Walk-n-Roll was held on Saturday, August 13, 2016 at Matter Park in Marion, IN. We had 100 participants who joined us after the rains cleared just in time for the 5k race and 1 mile walk to begin. The morning followed with a wonderful DJ, face painter for the kids, and great raffle with tons of fun items! A special thank you to Jessica, Brian, and Chloe Griffen, Stacie Fansler, Leesa Fansler, and all of their family, friends and community members who helped make this second year so wonderful! Everyone came together to raise over $7,000 for Cure SMA!

2016 Wannabe Cup

On August 11, 2016, the 2016 Wannabe Cup Charity Golf Tournament was held at the Sandpiper Golf Club, in Santa Barbara, CA. The event was full of two days of golf, including a first match and final match with closing ceremonies and awards. The event was a great success and raised over $17,000 for SMA. A special thank you to Joe Belcher for organizing this annual golf tournament in memory of Skylar Bahrenburg.

Wreaths of Hope

The 11th Wreaths of Hope took place this past November in Washington. Guests at the event enjoyed making holiday greens in an old fashioned holiday barn, caroling, raffle, enjoyed homemade baked goods and hot cocoa. The event was hosted by Sybil Kuhn and raised over $1,000.

Pacific Northwest Chapter

Flamingo Fun Run

The 3rd Annual 5k Flamingo Fun Run took place on October 8, 2016 in Grangeville, ID during the local Oktoberfest festivities. Oakley Smith, a vibrant, loveable, five year-old with curly red hair is the inspiration behind the annual Flamingo Fun Run. With incredible community support, and a lot of donated help from friends and family the 5k raised $6,000 toward finding a cure. Local and national businesses contributed money, donations for the raffle and food for the participants. Thank you to the Smith family for organizing this amazing event for SMA and the Grangeville community for their support.

Pennsylvania Chapter

Zane’s Run

The 8th Annual Zane’s Run was an incredible success raising over $52,000 for Cure SMA! Over 400 people gathered to run in memory of Zane Schmid. Each year the Schmid Family along with Meredith Bebee and the rest of their incredible planning committee work tirelessly to make this event a success! Sweet Baby Zane generously funds many Cure SMA programs including car beds, EZ-On Vests, and research. Thank you to all of the supporters of Sweet Baby Zane!

On August 28, 2016 the 9th Annual Walk-n-Roll took place. The day was perfect – sun and pleasant temperatures brought out over 200 people for a morning and afternoon of family fun and awareness. The Auburn Game Farm in Auburn, WA was a great venue with lots of space, playground and a covered shelter area. In addition to a wonderful day, activities consisted of a DJ, games, face painting, a fabulous raffle, silent auction, lots of vendors, and a food truck. Thank you to all the sponsors, generous friends and families, and great committee. We raised over $20,000 for Cure SMA! See you next year!
CHAPTER UPDATES

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2016 Rock ‘n’ Roll Philadelphia Half Marathon & 5K

Runners from across Pennsylvania came together in Philadelphia for the 2016 Rock ‘n’ Roll Philadelphia Half Marathon & 5K to benefit Cure SMA on Saturday and Sunday, September 17-18, 2016. The runners worked hard to raise over $23,798 to benefit the charity and its ongoing efforts.

Special thanks to our runners: Amy Brown, Tom Brown, Steve Cannady, Christopher Jankowski, Keith Leffler, Karen McRory-Negrin, Sarah Rodriguez and Eric Tetzlaff.

You all ran a wonderful race and we greatly appreciate the time it took to train and fundraise for Cure SMA!

Lukie’s Fest

It was another great year for Lukie’s Fest! This annual event held on October 8, 2016 raised nearly $13,000 for Cure SMA in honor of Luke Maida. The local community of Pen Argyl, PA rallied together to fundraise for treatments and a cure for SMA. Everyone enjoyed lots of fun carnival games, good food, and fall activities. Thank you to the Maida Family and Lukie’s crew for making this event such a success.

Ultimate Granite Surface Golf Outing

The 4th Annual Ultimate Granite Surface Golf Outing held on September 30, 2016 in Butler, PA was a huge success! Thanks to the generous support of the company’s employees and guests, $8,000 was raised for Cure SMA. Special thanks to Janet Bartley and the whole team at Ultimate Granite for their continued support.

2016 Cure SMA Knock Out SMA

This year marked the first Knock Out SMA event held in East Stroudsburg, PA. The event was hosted by Chris Barone in honor of Lydia Peters and Trinity VanWhy on September 24, 2016. Everyone gathered to knock out SMA karate style and raised over $8,000!

Dance Away SMA

The Lyla Mertz Foundation held the 7th Annual Dance Away SMA event on October 1, 2016 at the Schnecksville Fire Co. Pavilion in Schnecksville, PA in honor of Lyla Mertz. The dance had a high attendance with guests enjoying dinner, dancing and live music. The event featured a Chinese auction, helping to bring the total amount raised $5,400. Thank you to the Jen and Steve Mertz and their amazing volunteers for all of your hard work planning this event!
Lily Kennedy Golf Outing

October 2, 2016 marked the 13th Annual Lily Kennedy Golf Outing at the Iron Masters Country Club in Roaring Springs, PA. Attendees enjoyed a day of golf and outdoor activities in honor of Lily Kennedy. Thanks to everyone’s support, the event raised roughly $5,000 to help fund a treatment and cure for SMA. A special thanks to Heather Kennedy and Dr. Mark Lynch for all of their hard work and planning to make this outing a great success!

Annual Swing for a Cure Golf Tournament

On August 14, 2016 Steve and Jennifer Mertz hosted the 6th Annual Swing for a Cure Golf Tournament at the Mahoning Valley Country Club in Kunkletown, PA. They raised a total of $4,622. Thank you Steve, Jennifer, and all those who participated!

Dan’s Down Dog and Dash

Special thank you to Dan’s Dasher’s for organizing the 2016 Dan’s Down Dog and Dash fundraiser in honor of Peter Henkel, on Saturday, October 15, 2016 in Bryn Mawr, PA. Your generous donation of $2,500 from your event day proceeds to Cure SMA is greatly appreciated and helping to fund the most promising SMA research.
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**Rocky Mountain Chapter**

Over 450 people participated in the 10th Colorado Walk-n-Roll held on September 10, 2016 at Clement Park in Littleton, CO. Participants enjoyed face painting, temporary tattoos, cookie decorating, lunch & entertainment from the Colorado Power Rangers and Edson the Eagle from Colorado's professional soccer team, the Rapids.

We are beyond grateful for everyone who volunteered, participated, sponsored and donated to the walk. Together we raised over **$76,000**! This would not have been possible without you!

We wanted to extend a special thank you to our top sponsors & fundraising teams. Your continued support means the world to us!

Thank you to Michelle Pritekel, Sherri Casas, Julie Lino, and the Jobanputra family for their continued dedication to Cure SMA!
CHAPTER UPDATES

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2016 Rock ‘n’ Roll Denver Marathon & Half Marathon

The Rock ‘n’ Roll Denver Marathon & Half Marathon took place on Sunday, October 16, 2016 in downtown Denver, CO. For our second year participating in the Denver series, Team Cure SMA had four runners participate raising over $5,000 to find a cure for SMA. Thank you to Loree Weisman all our dedicated runners for their hard work and fundraising.

2016 Temecula Dinner- Evening Under the Stars

On December 17, 2016, the 2016 Temecula Dinner- Evening Under the Stars was held at Cottages at Temecula Creek in Temecula, CA in memory of Mia Ehorn. Participants enjoyed an incredible evening of a three course dinner, deejay, dancing, and a photo booth. The event raised $900. Thank you to Letizia Ehorn for organizing this event and your continuous support of Cure SMA.

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

On November 9, 2016, Ruby’s Diner of Tustin, CA held a dine and donate night to bring community awareness to SMA. Lynn and Ade DeBlaiso hosted the event at Ruby’s where they welcomed and educated the dining guests of SMA Overall, the event raised over $500 for Cure SMA in memory of Jaimie Ramsey, granddaughter of Lynn and Ade. Thank you for your continued support!

Cure SMA Day with the Rockies

On August 7, 2016, The Rocky Mountain Chapter had great day at Coors Field celebrating SMA Awareness month. More than forty friends and families turned out for a pre-game picnic and free concert featuring Crowder following the game. It was great to have both long time families as well as new ones gather for some fun and to raise awareness. Thank you to everyone who made this third annual event a success! We are looking forward to doing it again in 2017 and hope you can join us! Thank you Loree Weisman for coordinating this successful event.

Surf Away SMA with Ricochet the SURFice Dog

Cure SMA and lonis Pharmaceuticals hosted the 2nd Annual Surf Away during SMA Awareness month by providing a free assisted surfing experience by Ricochet the SURFice dog and professional surfers on the beautiful beaches of La Jolla Shores. On August 19, 2016 in La Jolla, CA, we had over 15 surfers come out for the first time and a few that returned from last year who surfed with Ricochet. What an incredible experience each of our surfers had that day. We enjoyed refreshments and lunch on the beach provided by lonis. A huge thank you to Max Moore and Judy Fridono for arranging this incredible event and a special thank you to Ricochet for making the afternoon possible for those affected by SMA.

San Diego Padres SMA Awareness Day

The 3rd annual San Diego Padres SMA Awareness Day took place on August 8, 2016 in San Diego, CA. The day brought together friends and family members for a full afternoon of tailgating, field experience, and a winning game by the Padres. The day raised $11,075 for SMA research. A huge thank you to Samantha Velchansky for organizing this tremendously successful outing and to lonis Pharmaceutical for their sponsorship.
South Florida Gala of Hope

On November 17, 2016 Jennifer Miller Smith and Fiorena Israel, two dedicated mothers of children with SMA, planned an evening to be remembered in honor of their daughters, Madison Smith and Mia Israel.

The 6th Annual Cure SMA South Florida Chapter Gala of Hope raised a record amount of $310,000. Total funds raised by the gala now exceed $1 million in its six year history.

The event, hosted at Ferrari-Maserati of Fort Lauderdale, featured a deluxe open bar, cocktail hour entertainment by Alex Fox, and live and silent auctions. Internationally renowned artist, Michael Israel, performed a live-art show that raised $97,000.

The evening also showcased an inspiring video that featured Fiorena and Jennifer, as well as Shaina Rappoport and Lymari Bergollo. The video captured the mothers’ individual stories along with messages of hope for a future without SMA.

Our deepest thanks to Jennifer and Fiorena, and their husbands Aaron and Aldo, for all their hard work and creativity. Thanks also to Ferrari Maserati of Fort Lauderdale for hosting the event, The Capital Grille, Buddha Sky Bar, and Premiere Beverage & Catering for providing the food and drinks, and to Galaxy Productions for providing the evening’s entertainment.
Cure SMA Day with the Miami Marlins

It was a great day for baseball and SMA on August 14, 2016. The South Florida Cure SMA Chapter had a wonderful time cheering on Marlins and enjoying a sunny day at the Marlins Park in Miami, FL. Along with awareness, the group raised $625 to help find a cure for SMA.

Kendra Scott Fundraiser - Sips, Sweets, and Jewels

The South Florida Chapter kicked off Holiday Shopping at Kendra Scott on December 1, 2016. The evening raised over $185 and was an evening filled with shopping, raffles, and refreshments. Thank you to Kendra Scott from Mizner Park for hosting this holiday soiree.

2016 Cure SMA Nashville Walk-n-Roll

Thank you to the Harbin Family for all of their hard work organizing the 2016 Cure SMA Nashville Walk-n-Roll! The event held on October 8, 2016 in Nashville, TN raised $11,641. Everyone had a great time with all of the fun games and activities! Thank you to the Harbin family for organizing this event in memory of their daughter, Tatum.

Texas Chapter Bowl-a-Thon

The 6th Annual Texas Chapter Bowl-a-thon was held on August 13, 2016 at Highland Lanes in Austin, Texas. In addition to bowling, the afternoon also included face painting, balloon animals, and SMA candle lighting. Through the continued support of friends, family, and the community the event raised over $6,500! A special thank you to event organizer Kelly Coggin for all of her hard work in making this year’s event a huge success.
CHAPTER UPDATES

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On October 7, 2016, Cure SMA and Talbots partnered to host Shop & Donate events at a store located in Houston. 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 $411. A special thank you goes out to Talbots Town & Country Village manager Chieuanh Nguyen for organizing this opportunity to spread SMA awareness and raise funds for a treatment and cure.

Talbots Make a Difference Day

On October 7, 2016, Cure SMA and Talbots partnered to host Shop & Donate events at a store located in Houston. 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 $411. A special thank you goes out to Talbots Town & Country Village manager Chieuanh Nguyen for organizing this opportunity to spread SMA awareness and raise funds for a treatment and cure.

2016 Ryder Davis Posse Ride

On November 12, 2016, 13 people participated in the 2nd Annual Cure SMA Ryder Davis Posse Ride. These enthusiastic riders rode across the state of Texas (from New Braunfels to El Paso) and back to show their support and raise money/awareness for SMA. Several earned Iron Butt Association “Saddlesore 1000” certificates by riding more than 1,000 miles in less than 24 hours. The event concluded on Sunday with a meet-n-greet with Ryder Davis and his family. This year more than $3,000 was raised to fund a treatment and cure for SMA. Cure SMA and the Davis Family would like to extend a big “thank you” to all who participated!

2016 Rock ‘n’ Roll San Antonio Marathon & Half Marathon

On December 3, 2016, Team Cure SMA participated for the second year in the 2016 Rock ‘n’ Roll San Antonio Marathon and Half Marathon in historic, downtown San Antonio, TX. For our second year we had one runner who personally raised over $750 for Cure SMA! Congratulations and thank you to Teri Speece for her hard work training and fundraising!

2016 Virginia Chapter Walk-n-Roll

On November 12, 2016, 13 people participated in the 2nd Annual Cure SMA Ryder Davis Posse Ride. These enthusiastic riders rode across the state of Texas (from New Braunfels to El Paso) and back to show their support and raise money/awareness for SMA. Several earned Iron Butt Association “Saddlesore 1000” certificates by riding more than 1,000 miles in less than 24 hours. The event concluded on Sunday with a meet-n-greet with Ryder Davis and his family. This year more than $3,000 was raised to fund a treatment and cure for SMA. Cure SMA and the Davis Family would like to extend a big “thank you” to all who participated!

The 2nd Annual Cure SMA Virginia Chapter Walk-n-Roll surpassed all expectations! With nearly 250 in attendance at Westfield High School in Chantilly, VA, participants more than doubled last years total and raised over $35,000! The super hero themed event featured appearances by Captain America, Ironman, and even a Storm Trooper. Families and friends from across the region enjoyed beautiful weather for the one mile walk and a fun filled day of activities like face painting, hair painting, caricature drawing, raffle prizes, water balloons and even a food truck.
Jogging for Joshua

A special thank you to Nathan and Bayley Bullock who hosted the 1st annual Jogging for Joshua fundraiser in Virginia Beach, VA on November 5, 2016. The event was held in memory of their son Joshua Bullock on his birthday. Joshua passed away in January 2016. The Bullock’s friends and family raised $7,149 for Cure SMA.

2016 Rock ‘n’ Roll Virginia Beach Half Marathon

Thank you to Jackie True for raising $250 in the 2016 Rock ‘n’ Roll Virginia Beach Half Marathon on September 3, 2016 in honor of her granddaughter, McKinzie Snead.
On Tuesday, November 29, more than 160 people from the SMA community— including government and industry partners— gathered for the 6th Annual “Hope on the Hill” Congressional Dinner.

Several Members of Congress attended the dinner, including Reps. Sean Duffy, Bill Foster, Jeb Hensarling, Bill Huizenga and Erik Paulsen. Other honored guests included U.S. Secretary of Agriculture Tom Vilsack.

The evening included remarks from Cure SMA Board Member Greg Zerzan, Cure SMA President Kenneth Hobby and a keynote address by Whole Foods Co-CEO Walter Robb.

On November 29, more than 160 people from the SMA community—including government, and industry partners— gathered for the 6th Annual “Hope on the Hill” Congressional Dinner.

As more SMA drugs reach late stage clinical trials and FDA approval, we remain focused on educating, involving, and engaging all three groups in our community: the patient and family group, the government and regulatory group, and the biotech and pharmaceutical companies that are developing and testing SMA drugs.

Through this event, we hope to build momentum to increase awareness of our mutual goals in advancing treatments for SMA and improving patient care with government representatives and agencies.

The Congressional Dinner is one of the centerpiece events for our important legislative goals. The event has raised **$850,000** for SMA research and family support!

Learn more about the upcoming 7th Annual “Hope on the Hill” Congressional Dinner at events.curesma.org/CongressionalDinner.
The 13th Annual Walk for a Cure was once again held in Beaver Island State Park on August 6, 2016. In the beautiful setting along the Niagara River, the event was attended by close to 400 people. $63,000 was raised to help find a cure.

Joe Gutberlet, our type III chapter officer, spoke about his experiences at the SMA conference in June. He shared his knowledge of living with SMA with many other teens at the conference. Diane Blair spoke of the progress of the clinical trials and the hope of approvals soon to come. Orange shirts were distributed to walkers upon registering, SMA literature and wrist bands handed out, raffle tickets sold, friends reacquainted and caught up with latest news. Once again the basket raffle was a great success thanks to Paula, Lori and Alice. We had bikes, sporting goods, iPod, money tree and other great items including baskets and gift cards.

Between the guest speakers and the raffle, team pictures were taken and hot dogs and refreshments were served.

This year’s walk was dedicated to the memory of Justin Heim who lost his battle in March 2016. Thank you to Karen Shiesley and all the volunteers that make this Walk-n-Roll possible!

I finished my first marathon in 5 hours and raised over $500 for Cure SMA. It was really hard after the first twenty miles, so I had to dig deep to keep going. I’m very happy to say I did it and to say that I helped SMA in the process. I hope a cure will be found soon for my cousins, Kalen and Kyan Kulas, and for all the children battling this disease.

Charity by Design hosted the Charmed by Charity event on October 27, 2016 in Buffalo, NY at Alex and Ani, LLC. Charmed by Charity and community members in Buffalo raised $286.50 on behalf of Cure SMA. Thank you to all who participated.
Baylor Cain Golf Outing for SMA

On October 9, 2016, the 1st Annual Baylor Cain Benefit for SMA golf outing was held at Hon-E-Kor Golf & Country Club in Kewaskum, WI. The golf outing was held in honor of our son, Baylor Christian. The motto of the golf outing was that we would never stop fighting to cure SMA, because we told Baylor, on many occasions, that no matter what, we would never stop fighting for him. There was a total of 54 teams consisting of 4 people each (216 people) that golfed and many others that joined us for dinner and raffles. The day consisted of lunch, raffles, silent auctions, live auction, 50/50 raffle, dinner, and many fun games along the course. We had so much support from the community and many generous donations. This year’s event was a huge success and raised over $23,000 to donate to Cure SMA.

A special thanks to our friends who helped coordinate the event. To our family’s who have continued to support us. To everyone that helped make the golf outing a success by donating and volunteering their time. And to our local community, for their generosity and continuous support of our family and other families affected by SMA. Cure SMA would like to give a special thanks to Anna Cain and Lindsay Gitter for organizing this event and for their continuous support!

2016 Cure SMA Florence 5K/ Mini Golf Tournament in honor of Blake

On August 21, 2016, families and friends gathered in Florence, WI for a 5k and Mini Golf Tournament in honor of Blake Sternhagen. Over 200 people gathered on a beautiful day at Keyes Lake Campground for a day of fun. After the race attendees got a chance to meet former Green Bay Packer player Gilbert Brown. This wonderful event raised $25,857 to find a cure for SMA! Cure SMA would like to give a special thanks to Blake’s aunt, Carli Kelly for organizing this event.
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

**Tori Stevens Memorial Night**

This year the Tori Stevens Memorial Race was held twice on June 10, 2016 and August 13, 2016 in honor of one of racings biggest fans in Tripoli, WI. Tori passed away at age eleven. No matter how sick she was she never let it get her down; she was always up front fence side to cheer on her favorite stock car drivers with a huge smile. Special thank you to Ashley Hedges for organizing these fun events and for raising $1,590 to help Cure SMA.

**JL Auto Donation**

Special thank you to John and Terri Litten for organizing the JL Auto Donation Jar Fundraiser during the month of August in honor of Kale Shiesley. Together they raised $101 for Cure SMA. Thank you John and Terri for supporting Cure SMA.

**Kennady’s Dream Walk-n-Roll**

On October 9, 2016 we held our 6th Annual Kennady’s Dream Walk-n-Roll for SMA at Fox Brook Park in Brookfield, WI. With everyone’s help, we were able to raise $14,484! We were thrilled with the attendance and generosity of our participants and sponsors. We had a beautiful day filled with lots of love from friends, family and supporters. Money raised went towards wagons for care packages, and to Cure SMA for research, education, and to continue to support families.

We are forever thankful and grateful for the support we have received through our Cure SMA community after our daughter, Kennady. We are honored to give back and help other families affected by spinal muscular atrophy.

Thank you to Shannon and Cure SMA in helping us with this last year’s walk and giving us hope, support, and information in our journey. Thank you to Kennady’s Dream Team for helping us in another successful year of fundraising. We hope to see you all again next year! We love and miss you, Kennady. You forever changed our lives and we will work to continue to change other lives in your memory. Everyone at Cure SMA would like to thank Erin and Corey Quinnell for organizing this event year after year and for their continuous support!

**Grant Sheppard Memorial Scramble for SMA**

On Friday, August 26, 2016 the 12th Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Chilton, WI. A total of $9,000 was raised during this golf outing in memory of Grant. Cure SMA would like to send a special thanks to Scott and Lisa Sheppard for all their hard work in planning this amazing event year after year and for their continuous support!
Congratulations to the White Family of Georgia, on the birth of their new son, Jack Ericsson, born on January 6, 2017!

Thank You to Our Volunteers

Each year, Cure SMA puts on over 300 local events, the Annual SMA Conference, and sends out hundreds of care packages and other resources. Without our wonderful volunteers, the success of these events would not be possible. Volunteering empowers people to find their purpose, to take their passion and turn it into meaningful change. Thank you to all of our chapter leaders, event organizers and families for their hard work and dedication to Cure SMA. We truly couldn't do it without you!
**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.

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On the Cover: Mateo Landa, SMA type II and Lucas Chan
August is
SMA Awareness Month

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