

# DIRECTIONS

FALL 2018



**cure**  
**SMA**

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# AveXis Files for FDA Approval of AVXS-101 for Spinal Muscular Atrophy Type I

AveXis, Inc., a Novartis company, announced that they have filed for FDA approval of AVXS-101, a gene therapy that replaces the survival motor neuron 1 (SMN1) gene, which is missing or mutated in individuals with SMA.

This first filing is for intravenous (IV) delivery of gene therapy. Based on typical practices, the expectation is that the FDA approval for IV delivery will closely match the IV clinical trial participants, who were SMA type I infants under nine months of age.

In addition, AveXis is currently testing intrathecal (IT) delivery of AVXS-101 in clinical trials. IT delivery would allow older and bigger patients to receive the therapy. Once these clinical trials are complete, AveXis will determine whether the data supports a separate BLA filing for IT delivery.

“We congratulate AveXis and Novartis on reaching this milestone,” said Kenneth Hobby, President of Cure SMA. “The IV clinical trial data shows that a one-time dose of gene therapy has the potential for a transformative impact on this life-threatening disease. We look forward to a rapid approval by the FDA, and then future positive results from the ongoing intrathecal clinical trials.”

## Next Steps

The filing that AveXis announced today is called a Biologics License Application, or BLA. The FDA will first review the application to make sure it is complete. After that review, the FDA will then evaluate the application to determine that AVXS-101 is safe and effective when given via IV delivery.

A final decision is expected in the first half of 2019.

## Cure SMA Supports 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. Adeno-associated virus serotype 9 (AAV9) has 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 IV delivery, and injection directly into the CSF, a process known as IT delivery. The IV delivery approach is currently under review for approval by the FDA.

IT delivery of 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. IT delivery of gene therapy is currently being tested in clinical trials.

## AveXis Community Statement on FDA Filing for SMA Type I

In October, AveXis provided a community statement on their FDA filing of AVXS-101 for SMA Type I.

Dear SMA Community,

AveXis, a Novartis company, is pleased to let you know that we have submitted regulatory applications for AVXS-101 in the U.S., Europe and Japan for use in infants with SMA Type 1. This is an important and exciting initial step toward the potential approval of AVXS-101, a gene replacement therapy candidate for the treatment of the underlying root cause of SMA.

Data from our pivotal Phase 1 study conducted in SMA Type 1 formed the primary basis for these submissions. Based on the data included in the applications, our expectation is that the initial label will be for intravenous (IV) use of AVXS-101 for infants with SMA Type 1, as IV dosing has only been studied in clinical

trials in infants. Our clinical development program is designed to help us understand the safety and how well AVXS-101 works in a broad set of pediatric patients with SMA. For example, our study of AVXS-101 in SMA Type 2 (STRONG) is ongoing, and data from that study will help determine the final study design for the planned study in children up to 18 years of age (REACH). Please see the table below for more information and updates on our planned and ongoing studies.

## Next steps for AVXS-101 regulatory applications:

- **U.S.** – The U.S. Food and Drug Administration (FDA) will make the decision of whether to accept our Biologics License Application (BLA) for AVXS-101 within the next 60 days. If accepted, we anticipate the FDA will take six months to review the BLA and make a decision on whether or not to approve AVXS-101. This enables a potential approval for AVXS-101 in the U.S. in the first half of 2019. We will provide an update on anticipated approval timing if and when the BLA has been accepted for review.
- **Europe** – Similarly, the European Medicines Agency is evaluating our Marketing Authorization Application (MAA) for AVXS-101 and will determine its acceptability within the next couple of weeks. If accepted, the European Medicines Agency will have seven months to review the MAA and make a decision on whether or not to approve AVXS-101. This enables a potential approval for AVXS-101 in Europe in mid-2019.
- **Japan** – In mid-September we initiated the pre-application review period of the Japanese New Drug Application (J-NDA) for AVXS-101 with the Ministry of Health, Labour and Welfare (MHLW). Upon completion of the pre-review, we anticipate the application submission of the J-NDA to MHLW will be completed by the end of this year and will have a six-month review period once accepted. This enables a potential approval for AVXS-101 in Japan in the first half of 2019.

AveXis is excited by this progress, and we want to thank everyone in the community for your ongoing interest in AVXS-101. We especially want to thank the patients and families who have participated in our clinical trials – it is because of you that we are where we are today, and we are incredibly grateful.

We look forward to continuing to keep you updated. In the meantime, if you have any questions please contact us at [medinfo@avexis.com](mailto:medinfo@avexis.com).

Sincerely,

The AveXis Team

## Frequently Asked Questions

### 1. When will AVXS-101 be approved by the FDA?

- We are pleased to have submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA). The FDA will make the decision of whether to accept the BLA for review within the next 60 days. If accepted, we anticipate the FDA will take six months to review the BLA and make a decision on whether or not to approve AVXS-101.
- This enables a potential approval of AVXS-101 in the U.S. in the first half of 2019. We will provide an update on anticipated timing if and when the BLA has been accepted for review.

### 2. What is a BLA? What does it mean for a BLA to be “accepted”?

- A Biologics License Application (BLA) is a request to the FDA for permission to market a biologic product in the U.S. The application includes specific information about the product, including pre-clinical and clinical data and manufacturing information.
- The FDA first does a cursory review to ensure the BLA submission package is complete and contains all of the appropriate information. At that time, they “accept” the file and the review period begins.

### 3. If AVXS-101 is approved by the FDA, who will be eligible to receive it?

- The pivotal Phase 1 study in SMA Type 1 formed the primary basis for the BLA submission. Based on the data included in the application, our expectation is that the initial label will be for intravenous use of AVXS-101 for infants with SMA Type 1.
- Our clinical development program is designed to help us understand the safety and how well AVXS-101 works in a broad set of pediatric patients with SMA. See table below for more information and updates on those studies.

### 4. What is the latest update from the clinical development program?

- AveXis is pleased with the advances we have made in our clinical development program for AVXS-101. Recently both the Phase 1 STRONG trial in SMA Type 2 and Phase 3 STRIVE trial in Type 1 were fully enrolled. Additionally, the Phase 3 STRIVE-EU trial was initiated in Europe.

- Please see the chart below for more details on the clinical development program.

### 5. When will updated clinical trial data be announced?

• We expect to have robust data from our AVXS-101 clinical development program at the American Academy of Neurology annual meeting in early May 2019, including from the Phase 1 STRONG study in SMA Type 2, Phase 3 STRIVE study in SMA Type 1, as well as data from our study of pre-symptomatic SMA patients known as SPRINT.

### 6. Are you planning to seek approval of AVXS-101 in other regions?

• Yes, we are currently working with the health authorities of several additional countries to understand their requirements for filing a new drug application.

These health authorities will use the information collected in our clinical studies to help determine whether AVXS-101 could be approved in their respective country

### 7. Who should I contact to see if my child is eligible for a clinical trial with AVXS-101?

• For more information regarding eligibility criteria for our clinical trials, please view the listings on ClinicalTrials.gov or [www.studysmanow.com](http://www.studysmanow.com). You may also contact us at [medinfo@avexis.com](mailto:medinfo@avexis.com).

## Overview of AVXS-101 Clinical Development Program (as of October 2018)

Study Name	Where	Who	Administration	Status
	U.S.	<ul style="list-style-type: none"> <li>20 patients with SMA Type 1</li> <li>Less than six months of age</li> </ul>	Intravenous (IV) infusion	Enrollment complete
	Europe	<ul style="list-style-type: none"> <li>30 patients with SMA Type 1</li> <li>Less than six months of age</li> </ul>	IV	Enrolling – Italy and UK currently activated
	Global	<ul style="list-style-type: none"> <li>At least 27 patients with two or three copies of <i>SMN2</i></li> <li>Less than six weeks of age and pre-symptomatic</li> </ul>	IV	Enrolling – U.S., Canada and Australia currently activated
	U.S.	<ul style="list-style-type: none"> <li>27 infants and children who are symptomatic with the bi-allelic deletion of <i>SMN1</i> and three copies of <i>SMN2</i> without the <i>SMN2</i> genetic modifier</li> <li>Older than six months and less than five years old</li> </ul>	Intrathecal (IT) injection	Enrollment complete
REACH	Global	<ul style="list-style-type: none"> <li>Data from STRONG (the first study of AVXS-101 delivered through IT injection) will help determine the final study design</li> </ul>	IT	Planned

# 2018 Annual SMA Conference Recap



**Thank you to everyone who attended the 2018 Annual SMA Conference at the Hilton Anatole in Dallas, Texas; it was truly a weekend filled with community, connections, research and fun!**

Including on-site registration, there were around 1,800 attendees in total – our second largest conference ever! We were so honored to have SMA families, clinicians and researchers attend this conference from all over the world, including individuals attending from over 30 countries.

The 4-day weekend was packed full of workshops and special sessions. The Newly Diagnosed Program kicked off the conference on Thursday, with the Opening General Session welcoming all attendees on Friday. Saturday's Researcher Q&A panel was once again a great way for families and individuals to hear directly from SMA researchers. Finally, the It's A Wonderful Life panel of adults with SMA ended the weekend on a high note. Relaxing and enjoyable events such as the Family PJ Party and Movie Night were also conference favorites!

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

We extend our thanks to the sponsors and exhibitors who generously supported the 2018 Annual SMA Conference. A special thanks to Biogen for their generosity as the Presenting Sponsor of this year's conference. Lastly, at the Sunday morning Closing General session, we announced the location and dates for our 2019 Annual SMA Conference. Mark your calendars for Friday, June 28 – Monday, July 1, 2019, as we head back to the magical Disneyland Resort in Anaheim, California!

# Meet and Greet

The 2018 Annual SMA Conference began for all attendees with our signature kick-off event, the Meet and Greet! This is a Thursday evening tradition where we welcome new families, returning families, clinicians, and researchers from all over the world.

It is always a special time for all attendees to gather in a fun setting prior to the start of the conference workshops. Everyone at the conference comes together to begin a weekend filled with new friendships and connections, learning the most up-to-date information, and being a part of this wonderful and caring community.

One of the main components that makes the Meet & Greet such an enjoyable event are the 30 games lined up around the edges of the room. Kids and families rotate around to the many games ranging from Bozo Buckets, a Treasure Chest, the Lollipop Tree, and so many more. The games are adapted for all children to enjoy and win great prizes. There was even a candy station and tattoo station, filled with goodies and fun for all!

**"I wanted to thank you for everything over the years. You all are the backbone of this organization - always smiling, always helping and so committed to improving the care and treatment of people affected by SMA." -Karen Patterson**



# Researcher Relay Race



"You and your team put on a great conference! Thank you for the opportunity to be there, meet the families, the Cure SMA staff, and learn about SMA and all the incredible work your organization has done and is doing in support of the SMA community. I truly enjoyed meeting the families and the children and learning about their journeys. The closing session was particularly insightful into the lives of people living with SMA." -Beatriz Duque Long

The popular Researcher Relay Race is always a highlight at the Meet and Greet, as researchers and kids race head-to-head to see who can cross the finish line first, with one rule: that the researchers have to race in a manual wheelchair!

All of the attendees stand along the perimeter of the course cheering on the racers to see who will be announced as the winner- The kids always cross the finish line first, leaving the researchers behind in the dust!



# Workshops

A variety of informative workshops and sessions are offered to all families on Friday and Saturday, led by some of the leading SMA experts in the U.S. as well as other families affected by SMA. There were over 50 workshops offered during the conference this year, covering the latest information on critical care and support.

Presentations from some of the workshops and sessions offered are available on the Cure SMA website, <http://www.curesma.org/get-involved/conference/>.



“Thanks for a great meeting as always! I must say that this was one of the best Genetics Workshops!” -Louise Simard





# Dance Party



This year at the Annual SMA Conference, we were so excited to bring back an evening of dancing for families and researchers alike! The Family and Researcher Dance Party showcases all of the attendees' best dance moves as they groove to the DJ's tunes and dress up with glow sticks, light-up glasses and other fun!

This event is a highlight for all, where everyone can let loose!

**"This week I have been the happiest I have ever felt. I've realized my worth, my purpose, and found my joy all from being with an amazing and inspiring group of people that I love dearly. Find your joy and fly with it." -Savannah Bedenbaugh**



# The Family Friendly Researcher Poster Session



The Family Friendly Researcher Poster Session brings together families and researchers. Families are encouraged to move around to the different posters created by researchers. This unique event allows for one-on-one interactions between the 1,800 families and researchers in attendance, and learn about the specific projects being presented.

At the 2018 Annual SMA Conference, 30 presenters representing different SMA research projects were showcased. All of the clinical stage drug programs for SMA were included among the presenters, along with a variety of basic research projects and clinical care research projects as well. To view the list of presenters, please visit <http://www.curesma.org/news/2018-ffps-presenters.html>

## Family Friendly Researcher Poster Session Posters Included:

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

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

**Antoine Clery, PhD and Frederic H.T. Allain, PhD from ETH Zurich** on "Splicing regulation in SMA".

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

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

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

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

**Richard Finkel, MD and Kelly Wydrnkowski from Nemours Children's Hospital** on "Implementation of SOC guidelines at Nemours".

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

**Andreanne Didillon, Fatima Mostefai, Amir Haghandish from University of Ottawa** on "SMN and friends: What we can learn from SMN interactors?".

**Hikari Tanaka, Nikki McCormack, Mahlet Abera PhD, and Barrington Burnett PhD** from the **Uniformed Services University of the Health Sciences and The F. Edward Hébert School of Medicine** on “Uncovering ways to slow SMN destruction”.

**Lisa Belter, Jill Jarecki PhD, Kenneth Hobby, Cynthia Jones PhD, Suzanne Cook PhD, and Sandra Reyna MD** from **Cure SMA** on “Cure SMA membership: Findings from the 2018 membership survey”.

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

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

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

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

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

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

**Rosangel Cruz, Kenneth Hobby, Jill Jarecki, PhD., Megan Lenz, and Lisa Belter** from **Cure SMA** on “Externally-led patient focused drug development meeting for spinal muscular atrophy”.

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

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

“I just want to extend my gratitude for this scholarship and wanted to say that I learned a lot from Cure SMA conference. I am looking forward to future conferences and I hope to add to the body of research for this diagnosis.”  
-Chintan A. Pancholi-Parekh

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

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

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

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

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

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

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

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

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

***Our thanks to Biogen for their generosity as the presenting sponsor of the 2018 Annual SMA Conference.***



# Family PJ Party and Movie Night



“Thank you so much for putting on such a wonderful conference again this year! It is such a highlight every year for our family. Thanks again for everything.”

-Lindsay Deshotels



“The conference was INCREDIBLE. Thank you SO much for your help enrolling us in the newly diagnosed program. We will definitely be going every year!”

-Amanda Scafuto



# Roll-a-Palooza



Thanks to funding and suggestion from the Adyn's Dream Foundation, at this year's Annual SMA Conference, we were able to offer a new opportunity for families. On Saturday evening, we held a Roll-a-Palooza live concert event, which featured two local bands from Austin, Texas.

The idea behind this event was to offer families and kids the opportunity to experience a live concert event, full of dancing, refreshments, snacks and mingling. It was a great evening for all!



“I wanted to say thank you to Cure SMA and the Dhont family for offering the scholarship program. I haven't been to the conference in many years. I'm really looking forward to meeting new people and hearing about all the latest research developments. Thanks again.”

-Suzanne Ammons



SPONSORED BY ADYN'S DREAM



# Adults with SMA Reception

The Dhont Family Foundation generously sponsored a special evening for adults with SMA and their families on Thursday evening. The reception offered adults with SMA the opportunity to meet and spend time together in a relaxed and friendly social setting, which included refreshments and appetizers.

SPONSORED BY THE DHONT FAMILY FOUNDATION



“Thank you for everything! I am grateful for the opportunity, the event and the attention. Congratulations, once again you’ve made a great conference.”  
-Patricia Souza

# Teen Social

Teenagers ages 11 – 17 were invited to a special teens-only event during the Meet and Greet!

This event was created to provide a space for teenagers to interact and make lasting connections among their peers in a friendly, social setting. There was an activity for the teens to participate in, as well as snacks and refreshments. Special thanks to the Jacob Isaac Rappoport Foundation and AveXis for sponsoring this fun event!



SPONSORED BY THE JACOB ISAAC RAPPOPORT FOUNDATION AND AVEXIS





# 22<sup>nd</sup> Annual SMA Researcher Meeting



The SMA Researcher Meeting is the largest research meeting in the world specifically focused on SMA, and seeks to create open communication of early, unpublished data to accelerate the pace of research. The meeting also furthers research by building collaborations—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers. This year, 530 researchers attended the meeting—a record number!

The SMA Researcher Meeting runs alongside the family conference but is held separately for researchers to engage with each other. We then provide opportunities throughout the conference for both the researchers and families to come together for events such as the Meet and Greet, Family Friendly Researcher Poster Session, Family and Researcher Dance Party, the Researcher Q & A session and more. The interactions from both sides of the conferences are so special and is what makes the Annual SMA Conference such a unique and valuable weekend for everyone involved.

To view the highlights and summaries specifically from the SMA Researcher Meeting, please visit the SMA Researcher Meeting page <http://www.curesma.org/research/for-researchers/research-conference/>.



“I just wanted to let you guys know that the conference was amazing (again). It is just incredible that you all pull this off year after year. The logistical expertise required to make this happen are mind blowing and everything was flawless.”

-Spencer Perlman



“Thank you for a wonderful conference. I learned so much and met so many wonderful people!”

-Debra Patrizi

# Clinical Care Meeting

We were pleased to introduce the inaugural Cure SMA Clinical Care Meeting, which was held on Saturday afternoon in Dallas, TX. This meeting is the third component to the Annual SMA Conference; we now have the researcher meeting, the family meeting, and the clinical care meeting all happening in one place at the same time, making each new connection and interaction that much more valuable.

The goals of this meeting were to provide an opportunity for SMA health care providers across various disciplines to share knowledge, discuss improved care for SMA, foster clinical care research collaboration across care centers, promote interactions between new clinicians and leaders in clinical care, build the SMA clinical care community, and promote conversations between families and patients living with SMA, clinicians, researchers and industry partners.

Over 120 health care providers who diagnose and care for people affected by spinal muscular atrophy were able to participate this year. This included physicians, nurse practitioners, physician assistants and nurses, pediatric and adult specialists including neurologists, pulmonologists, rehabilitation medicine, orthopedic surgery, palliative care, physical and occupational therapists, speech and language pathologists, nutritionists, social workers, respiratory therapists, and trainees in the above disciplines. We look forward to continuing this exciting opportunity.



**“Thank you so much for the wonderful opportunity you afforded me to attend the Cure SMA conference in Dallas, TX the other week. It was an excellent opportunity to network and learn from other clinicians as well as interact with some of our amazing SMA families. I look forward to seeing what more the future holds for our wonderful SMA families. Thank you again for your generous financial support/sponsorship enabling me to attend the conference. I greatly enjoyed this learning and networking opportunity with our SMA families, clinicians, and pharmaceutical supporters.”**

**-Vanessa M. Yount**

# Children's Program



This year's Children's Program was extraordinary and another huge success! Thanks to our incredible volunteers, all the children had a fabulous time. Kids stayed busy with countless arts and crafts projects, toys, live entertainment, exciting movies, fun activities, video games, build-a-bear stations, and so much more.

The Annual SMA Conference would never be possible without the assistance of our fantastic volunteers! From helping with set up, working in the incredible Children's Program, preparing and running games at the Meet & Greet, inflating hundreds of balloons, to any of the other countless jobs that needed to be done, our volunteers were absolutely amazing.



**"I appreciate the BIG organizational commitment the entire conference is, as well as the little things. Well done Cure SMA!" -Anne DeMark Thompson**



The **first** treatment for children and adults with spinal muscular atrophy (SMA)

# WHAT COULD SPINRAZA UNLOCK?



**Sofia**  
age 2.5  
Infantile-onset (Type 1) SMA  
treated with SPINRAZA

The effectiveness of SPINRAZA was evaluated in a well-controlled clinical study of 121 individuals with infantile-onset (Type 1) SMA aged 30 to 262 days and supported by clinical experience across a broad range of ages, SMA types, and level of function.

## Achieving motor milestones

When treated with SPINRAZA, individuals with infantile-onset SMA showed improvements in milestones that are rarely, if ever, achieved in untreated infants.

## Safety considerations

It is recommended that your healthcare provider perform blood and urine testing before starting SPINRAZA and before each dose to monitor for signs of bleeding or kidney damage.



Find out how SMA360° can help with insurance and treatment logistics at [SPINRAZAsupport360.com](https://SPINRAZAsupport360.com)

## INDICATION

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

## IMPORTANT SAFETY INFORMATION

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

**The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**For additional Important Safety Information, please see brief summary of full Prescribing Information to the right.**

This information is not intended to replace discussions with your healthcare provider.



**Lauren**  
age 20  
Later-onset (Type 3) SMA  
treated with SPINRAZA



**Bill**  
age 48  
Later-onset (Type 3) SMA  
treated with SPINRAZA

Individual results may vary based on several factors, including severity of disease, initiation of treatment, and duration of therapy.

### IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

#### USES

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

#### WARNINGS

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

#### COMMON SIDE EFFECTS

- **The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome (headache related to the intrathecal procedure).
- Serious side effects of complete or partial collapse of a lung or lobe of a lung have been reported.

**Talk to your healthcare provider about any side effect that bothers you or that does not go away.**

#### OTHER INFORMATION

SPINRAZA is a medication that should be administered as an injection into the lower back (a procedure called intrathecal injection) by, or under the direction of, an experienced healthcare professional.

#### QUESTIONS?

The risk information provided here is not comprehensive. To learn more, talk about SPINRAZA with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at [www.spinraza.com](http://www.spinraza.com) or 1-844-4SPINRAZA (1-844-477-4672).

#### MANUFACTURED FOR

Biogen  
Cambridge, MA 02142

revised 05/18



# 2018 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.

## National Premier Partners



## National Platinum Partners



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## National Silver Partner



## Resources and Communications Partners





# Newborn Screening

## SMA Newborn Screening Advancements

The first FDA approval of a therapy for SMA has created an opportunity for our community to move forward on another of our long-term priorities: newborn screening. SMA is the leading genetic cause of death for infants, but we have an historic opportunity to change that through newborn screening and early treatment.

Currently, the average infant with SMA type I is not diagnosed until five months of age. This means that these babies are not receiving treatment during the critical early weeks and months of life, when research suggests it may be most effective.

In February, the federal Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) recommended that newborn screening for SMA be implemented nationwide. This recommendation was subsequently approved by Health and Human Services Secretary Alex Azar in July. Cure SMA was there at every step in the process, submitting the nomination and supporting it from acceptance to vote.

This is an important first victory as we work toward our goal of having every baby born in the United States screened for SMA, but much remains to be done. Each state must decide individually whether to implement SMA newborn screening.

Seven states—Illinois, Indiana, Minnesota, Missouri, New York, Utah and Ohio—have already adopted this recommendation. Three others—Georgia, Massachusetts and North Carolina—have begun pilot screening, and at least a dozen more are currently considering action.

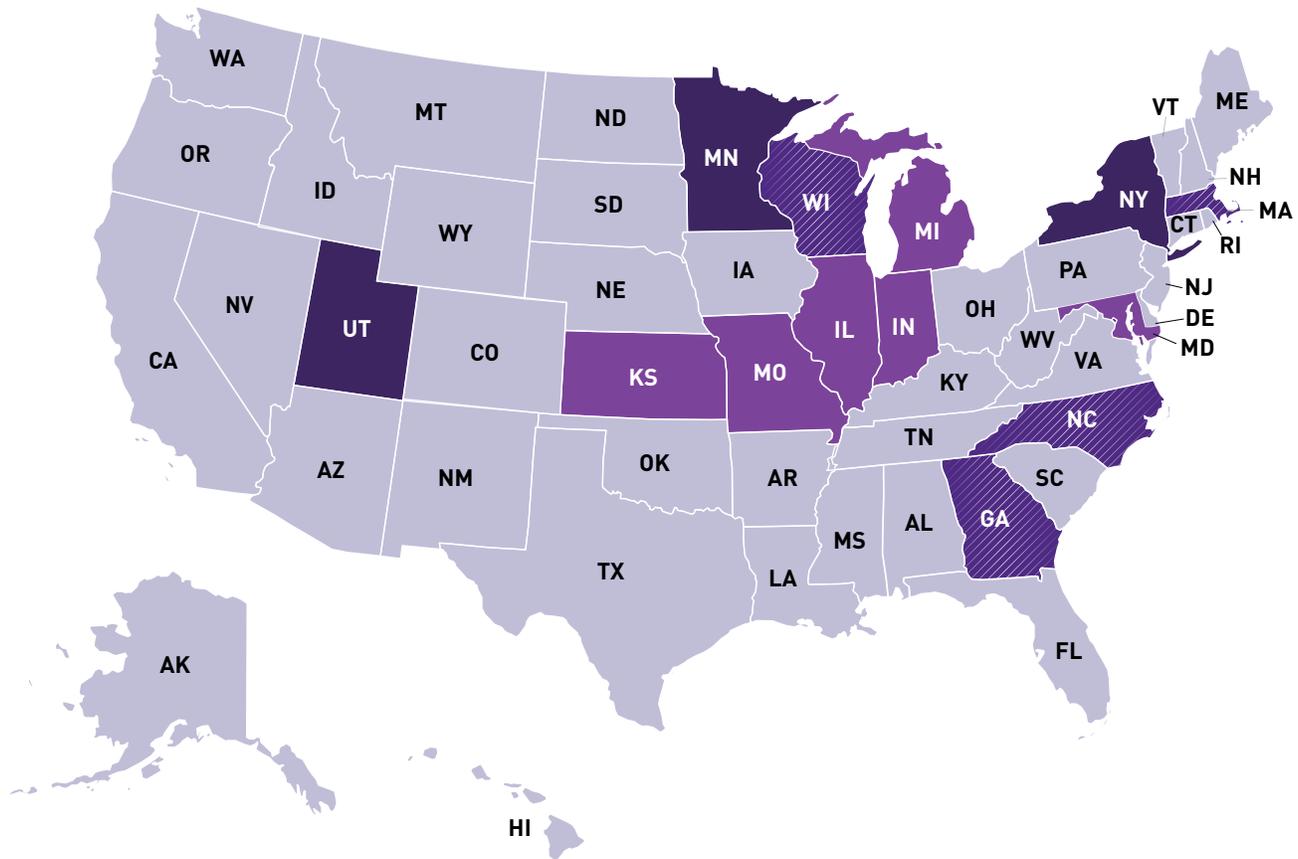
## Cure SMA's Approach to Newborn Screening

The complexities of newborn screening require a sophisticated, multifaceted approach. With this in mind, Cure SMA has been working on newborn screening through several different avenues, bringing our full resources to ensure that babies born with SMA have the opportunity to receive early treatment for maximum effectiveness.

## Advocacy

We are working with family advocates, clinicians, legislators and administrators, to encourage speedy implementation of SMA newborn screening. This process requires resources in all 50 states.

We also remain engaged at the federal level, ensuring that federal newborn screening programs are adequately funded, particularly those designed to help states begin screening for newer conditions like SMA. This year, thanks to our advocacy, funding for the Health Resources and Services Administration (HRSA) Heritable Disorders Program and the CDC's newborn screening quality assurance program both increased significantly. We also secured increases in funding for biomedical research at the National Institutes of Health and the Department of Defense's research programs, which include SMA.



## Family Support

Families that receive an SMA diagnosis through newborn screening are another example of how rapidly the experience of SMA is changing. While they will face many of the same issues as families that receive a symptomatic diagnosis, they will also have their own unique experiences and decisions to make.

We're already working to address this, developing educational materials that will help families who receive a diagnosis through newborn screening understand their options and make the decisions that are best for their child and their family.

## State Implementation

Once states begin screening for SMA, they will need to quickly connect families with treatment options near them, direct them to centers experienced in SMA care, educate families on next steps, and follow up to make sure babies are reaching the best outcomes.

That's why we're building partnerships with states as they prepare to screen, educating them about our family support programs and our SMA Care Center Network.

By working with states, we'll help make sure families get timely treatment and care, connect with our community, and receive the resources they need at every step of the way. In addition, Cure SMA made grants to four states to help cover start-up costs for SMA screening, thus avoiding a common barrier to speedy implementation of screening.

## Research

Clinicians who care for babies identified through newborn screening must venture into uncharted waters. With that in mind, Cure SMA convened a working group of SMA clinicians to set a standard for how babies with SMA will be guided toward appropriate treatment. This work was published in the *Journal of Neuromuscular Diseases* in April.

Cure SMA is also building a newborn screening data portal. This portal will be used to collect information on outcomes for these babies, building an evidence-based standard of care for those identified through newborn screening.

# Clinical Trial and Drug Program Updates

## New Clinical Trial Data Released

Over the past few months, several companies released new data from their programs. The data released covers the entire SMA population—from infants to adults—and includes different therapeutic approaches, including those that target the underlying genetic cause of SMA and those that work on the muscles affected by SMA.

The release of this data will help further the development of treatments for all types, ages and stages of SMA.

## Cytokinetics Update on CK-2127107



Cytokinetics, Inc., and Astellas Pharma Inc., recently provided an update for their collaborative skeletal muscle program, including clinical trial updates for reldesemtiv in SMA.

In June, Cytokinetics announced data from a Phase 2 double-blind, randomized, placebo-controlled clinical study in patients with SMA which was designed to determine potential pharmacodynamic effects of a suspension formulation of reldesemtiv following 8 weeks of oral dosing in each of two cohorts of 36 patients with Type II, Type III, or Type IV disease. Secondary objectives were to evaluate the safety, tolerability and

pharmacokinetics of reldesemtiv. The study showed statistically significant concentration-dependent increases in changes from baseline in Six Minute Walk Distance (6MWD), a sub-maximal exercise test of aerobic capacity and endurance.

The study also showed statistically significant increases for Maximal Expiratory Pressure (MEP), a measure of strength of respiratory muscles. Other assessments, including the Hammersmith Functional Motor Score - Extended, (a functional motor scale that was assessed in the development program that led to the FDA approval of the first therapy for patients with SMA), Revised Upper Limb Module, Timed Up-and-Go, Forced Vital Capacity, and the SMA Health Index (SMA-HI), a patient reported outcome measure (PROM) developed to comply with FDA standards for PROMs, did not demonstrate differences between reldesemtiv versus placebo. Adverse events were similar between groups receiving reldesemtiv and placebo.

Cytokinetics recently convened an expert advisor meeting to discuss the Phase 2 clinical study of reldesemtiv in patients with SMA and received encouraging and constructive feedback as well as recommendations to inform potential next steps.

Cytokinetics will be seeking a Type C regulatory interaction with the FDA this year regarding the acceptability of 6MWD as an endpoint for a potential registration program for reldesemtiv in patients with SMA.

## Cure SMA Funding for Combination Therapies

The clinical trials for CK-2127107 (reldesemtiv) came about because of early seed funding from Cure SMA, supporting research focused on the potential application of these types of skeletal muscle activators to SMA. 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.

## Genentech Update on Risdiplam (RG7916) Genentech

Genentech, a member of the Roche Group, recently announced interim clinical data from the dose-finding parts of the pivotal FIREFISH and SUNFISH studies investigating risdiplam (RG7916) in spinal muscular atrophy (SMA).

In the FIREFISH study in Type 1 SMA, six out of 14 infants (43 percent) were able to sit (with or without support), including three (21 percent) who achieved unassisted stable sitting after eight months of treatment. In addition, four infants (29 percent) demonstrated rolling to the side; seven (50 percent) kicking and six (43 percent) achieved upright head control. These milestones were assessed according to the Hammersmith Infant Neurological Examination (HINE) Module 2 and are key secondary endpoints in the confirmatory part of FIREFISH.

Updated analyses of the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), a scale developed to assess motor function in infants with Type 1 SMA, demonstrated that eight out of 14 infants in FIREFISH (57 percent) achieved a score of 40 or above at their eight-month visit. Typically, an infant with Type 1 SMA does not demonstrate any motor improvement and can decline during this time period.

In Part 1 of the SUNFISH study in Type 2 and 3 SMA, SMN protein median increases of greater than two-fold, as measured in blood, were seen after 12 months. A very broad patient population aged between two to 24 years was included, ranging in functional status from weak non-ambulant to strong ambulant, and with varying degrees of scoliosis from none to severe. Twenty-one patients initially received lower doses of risdiplam for at least 12 weeks. Sixty-three percent of patients experienced an improvement in MFM over baseline of three points or more after one year. Such improvements were seen

both in patients under 12 years old (76 percent; n=17) and over 12 years old (46 percent; n=13). Follow-up is ongoing for the confirmatory Part 2 portions of both the FIREFISH and SUNFISH studies.

## About Risdiplam

Risdiplam is an investigational, oral medicine that is systemically distributed and designed to increase SMN protein levels in the central nervous system (CNS) and throughout the body. It is designed to help the SMN2 gene produce more functional SMN protein, to better support motor neurons and muscle function. Roche and Genentech are leading the clinical development of risdiplam in collaboration with the SMA Foundation and PTC Therapeutics. Risdiplam is currently being evaluated in three multicenter trials in people with SMA:

- **FIREFISH** – an open-label trial in infants aged one to seven months with Type 1 SMA
- **SUNFISH** – a double-blind, placebo-controlled trial in children and young adults (two to 25 years old) with Type 2 and 3 SMA
- **JEWELFISH** – an open-label exploratory trial in people aged 12–60 with Type 2 or 3 SMA who have been previously treated with SMN-targeting therapy as part of a clinical study

A new trial, RAINBOWFISH in pre-symptomatic SMA, will be initiated by early 2019.

You can read more about all of these studies by visiting [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

## Scholar Rock Update on SRK-015



Scholar Rock, a biotechnology company based in Boston, announced that it has completed dosing of the first cohort and is continuing to enrollment of healthy volunteers in its Phase 1 clinical trial of SRK-015. SRK-015, a selective inhibitor of the activation of myostatin, is the company's lead antibody drug candidate. Scholar Rock believes it has the potential to be the first muscle-directed therapy to reverse or prevent further muscle atrophy in patients with spinal muscular atrophy (SMA) and could be used alone or in conjunction with the current standard of care.

The placebo-controlled, double-blind Phase 1 clinical trial will evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of single- and multiple-ascending doses of intravenous SRK-015 in healthy adult volunteers. Assuming the successful completion of the Phase 1 trial, the company plans to initiate a Phase 2 proof-of-concept trial in the first quarter of 2019 to evaluate the efficacy and safety of SRK-015 in patients with later-onset SMA as a monotherapy or in conjunction with an approved SMN upregulating therapy.

## Biogen Update on SPINRAZA



In October, Biogen, Inc., announced new interim results from NURTURE, an ongoing open-label, single-arm efficacy and safety study of SPINRAZA in 25 pre-symptomatic infants with SMA.

The interim analysis evaluated survival and respiratory intervention rates in infants (n=25) who were genetically diagnosed with SMA and began treatment in the presymptomatic stage of the disease. As of May 2018, all patients in the study were alive and none required tracheostomy or permanent ventilation. Additionally, 22 of the 25 participants were able to walk either with assistance or independently according to the motor milestone standard of the World Health Organization and all 25 were able to sit without support.

The motor skills of study participants were also evaluated using the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), an assessment which considers 16 different types of movement to create an overall score between zero and 64. The mean CHOP INTEND scores were 62.6 for study participants with three copies of the SMN2 gene and 61.0 for those with two copies of the gene.

All NURTURE study participants were 14 months or older at the time of the analysis. Participants included infants with two copies of the SMN2 gene (n=15) who are likely to develop SMA Type 1, and infants with three copies of the SMN2 gene (n=10) who typically develop SMA Type 2 or 3. No new safety concerns were identified.

## Cure SMA Provides Seed Funding for Spinraza

From 2003 to 2006, Cure SMA provided the very first research funding needed to begin investigation into the therapeutic approach behind SPINRAZA. We would like to thank and acknowledge Cold Spring Harbor Laboratory (CSHL) and the University of Massachusetts Medical School for generating critical intellectual property for the program that was licensed to Ionis Pharmaceuticals. We specifically thank Drs. Adrian Krainer, Yimin Hua and colleagues at CSHL for years of dedication to and hard work on the preclinical development of Spinraza for SMA, and Drs. Ravindra Singh and Elliot Androphy for their work funded by Cure SMA in originally identifying the SMN1 gene sequence, which is the sequence targeted in Spinraza. To learn more about SPINRAZA, visit [www.curesma.org/spinraza](http://www.curesma.org/spinraza).



The Cure SMA Support Package for teens and adults is filled with helpful items that have been recommended by adults living with SMA. Many of these items included in the package have been found to make activities of daily living easier for those with SMA.

They also help those living with SMA to gain independence with certain tasks they might not have been able to do on their own.

## CURE SMA TEEN AND ADULT SUPPORT PACKAGE

If you are a teen or adult with SMA and are interested in receiving a Cure SMA support package please visit [www.events.curesma.org/supportpackage](http://www.events.curesma.org/supportpackage) to request one at no charge.

# Community Spotlight: Kimberly Hill

The below NDEAM Spotlight was written by Kim Hill, Disability Integration Specialist at the Oklahoma Department of Emergency Management, who also lives with SMA Type II.

Hello! My name is Kimberly or Kim Hill. I am a 34-year-old from Oklahoma with SMA Type II. I am trached and use a ventilator full time and I can only use my left thumb myself. I can speak but I am difficult to understand, so I communicate mostly through text on my computer. I have always had a hopeful and defiant spirit, but I never really planned for college or getting a job because I always conquered life one challenge at a time. This attitude got me a Bachelor of Arts Degree from Oklahoma State University and I am one thesis short of a Master of Science degree from Oklahoma State University. The Department of Rehabilitation Services paid for school and my assistive technology.

I work part-time from home for the Oklahoma Department of Emergency Management as their Disability Integration Specialist. Emergency management includes the preparedness, response, mitigation, and recovery aspects for dealing with an emergency or disaster within a community. My job is to make emergency management in Oklahoma more inclusive of people with disabilities and access and functional needs. Oklahoma has quite a few natural disasters, so it keeps me busy.

Like most college students, I was not sure what I wanted to do with my life. I liked government and helping people, so I got a B. A. in political science and I did some volunteer work in research for a few electoral candidates. All of that got me nowhere in finding a job. I even got a job coach which was both incredibly frustrating and amusing. I was getting really discouraged with her and finding a job, but I knew there had to be something I could do from home to help people, so after the May 2013 Oklahoma tornadoes I started volunteering for social media emergency management and I loved it. I highly recommend starting out as a volunteer just to see what you enjoy doing and you can make connections there as well.

Because my political science degree was not as useful as I thought it would be, I went back to school and started studying fire and emergency management online. I had a classmate who worked for the Oklahoma Department of Emergency Management and I asked him if they had anyone working with people with disabilities after disasters and they did not. My classmate told his boss about me and after a chance meeting with his boss, who was then the director of OEM, I had my dad tell the director that I wanted to help him with the disability community and that I would volunteer or intern for him because he needed someone like me.

Surprisingly, he agreed, and my position was created.

Because I can only use my left thumb and a switch to control my computer, I must utilize assistive technology to do everything. My workplace bought me a GoPro camera for when my team is out in the field after a disaster so I can go with them. I bought a Beam+ Robot before I actually started working there and now it lives in my office so I can attend meetings and such. If you haven't seen one, it's like a big FaceTime on wheels. I have been very lucky in that my coworkers have been great and very inclusive of me in everything that they do. It could be because causing a ruckus when people are not inclusive is part of my job, though! :-)

It's bizarre, but the most challenging part of me getting a job as a person with a disability was getting Medicaid to follow their own laws! When I first started trying to get a job, DHS kept telling me that I would lose my Medicaid benefits just by working and that is NOT true. I had to school DHS on the federal law 1619B, which allows you to keep Medicaid while working. I can't make a lot of money, but I can work and keep Medicaid benefits. The amount of money you can earn and keep Medicaid is different in each state so always do your own research and never take DHS' word as gospel.

My advice for anyone looking for a job is to find something you enjoy doing and do well, make connections wherever you are, be bold, and never be afraid to ask if you can help with whatever it is. You never know unless you ask! What I really want to get across is that if I can go to school and get a job with only one working thumb, ANYONE can if they want to and believe that they have something to offer!

Thank you to Kim for sharing her experiences and perspective in navigating higher education and employment with Cure SMA!



# CURE SMA FAMILY SUPPORT APP IS NOW AVAILABLE FOR YOUR MOBILE DEVICE!



Cure SMA is excited to announce the launch of a new mobile app for the SMA community. The Cure SMA Guide app is a family support program that takes a new approach to provide useful tools and information related to SMA care, to use at home and on-the-go. The app is now available to download from your Apple or Android device. The app contains important information about SMA, including all of the SMA Care Series booklets, and acts as a quick way to have SMA care information available at your fingertips.

## Access important information such as:

- Downloadable PDFs of all SMA Care Series booklets, in both English and Spanish
- Resources for physicians, nurses, therapists, and other healthcare providers
- Local chapter contact information
- General medical and emergency recommendations and tools
- Much more!

The Cure SMA Guide is your portable source for SMA care and support.

Access valuable information at the touch of a button. Share resources with your family, friends, and care providers.



Please download our app directly from the Apple Store or Google Play Store for Android devices.



Cure SMA would like to thank Genentech for generously funding a grant to support the Cure SMA Guide app resource for the SMA community!

# Drug Discovery Grants

## \$300,000 Grant Awarded to Kevin Hodgetts, PhD, The Brigham and Women's Hospital



Cure SMA has awarded a \$300,000 preclinical drug discovery grant to Kevin Hodgetts, PhD, at the Brigham and Women's Hospital, for his project, "Pre-Clinical Development of LDN-5178 for the Treatment of SMA."

This grant will be conducted by two academic research teams working together to identify new treatments for SMA. The two teams are led by Prof. Kevin Hodgetts at the Laboratory for Drug Discovery in Neurodegeneration (LDDN) at Brigham and Women's Hospital, an affiliate of the Harvard Medical School, and Prof. Elliot Androphy at Indiana University.

The two academic research teams work together on the optimization of novel small molecules as activators of SMN2 protein. With this funding, they will continue the development of their lead series of compounds that increase the half-life and accumulation of normal SMN protein. These lead compounds have been shown to increase SMN protein in brain and extend survival and motor function in a mouse model of SMA.

In this proposal, the research teams will perform pre-clinical safety and toxicity studies on their lead molecules to ready them for Investigational New Drug (IND) submission.

## \$300,000 Grant Awarded to Umrao Monani, PhD, Columbia University



Cure SMA has awarded a \$300,000 preclinical drug discovery grant to Umrao Monani, PhD, at Columbia University, for his project, "Restoring function at the NMJ: A novel means to treat SMA."

A drug directed at SMN enhancement, Spinraza, has recently become available but is unlikely to benefit all patients with maximal effectiveness. The objective of this project is to validate a novel genetic factor, besides SMN up-regulation, that could serve as a new target for complementary treatments for SMA.

Dr. Monani and his team will use viral vector technology (gene therapy), to determine if the genetic factor can be reliably delivered to SMA mice and attenuate severe disease. Secondly, the team will determine if the factor is equally potent in its effects across mouse models of differing SMA severity and at different points of time.

These experiments will serve as proof of concept studies to validate moving into IND enabling studies and human trials. The greater the number of ways one can combat SMA, the more likely the entire population of SMA patients will benefit.

## Drug Discovery Funding

These grants to Dr. Hodgetts and Dr. Monani are part of \$600,000 in new translation drug discovery funding. This grant was generously funded by a donation made to Cure SMA anonymously in honor of William N. Kanehann. We are grateful for this amazing donation in memory of Billy's life.



## Cure SMA Provides Funding and Recruitment for the NeuroNEXT SMA Biomarker Study

A recent paper published in the *Journal of Contemporary Clinical Trials Communications* titled, "Recruitment & retention program for the NeuroNEXT SMA Biomarker Study: Super Babies for SMA!" highlights the challenges faced when recruiting infants with SMA into the NeuroNEXT study and how the study leaders were able to overcome those challenges with the help of advocacy groups, such as Cure SMA.

Recruitment and retention of research participants are challenging and critical components of successful clinical trials and natural history studies. Infants with spinal muscular atrophy (SMA) are a particularly challenging population to study due to their fragile and complex medical issues and, until 2016, a lack of effective therapies. Recruitment of healthy infants into clinical trials and natural history studies is also challenging and sometimes assumed to not be feasible.

In 2011, the NeuroNEXT group initiated a two-year, longitudinal natural history study of infants with SMA and healthy infant controls to provide data to assist in the analysis and interpretation of planned clinical trials in infants with SMA.

Prior to the finalization of the clinical protocol, the study collaborated with Cure SMA. Cure SMA representatives provided feedback on the draft recruitment materials prior to the study going live. Once enrollment was open, Cure SMA actively worked to share and inform the SMA community of the recruitment materials and messages.

Every new patient registered with Cure SMA received a welcome packet of general SMA information that included the SMA Biomarker study brochure during the enrollment period. Cure SMA also circulated quarterly updates to ensure that people did not forget about the study. Furthermore, Dr. Stephen Kolb, the study's Principal Investigator attended the annual, international Cure SMA family meeting as an outlet to share study data directly with the families affected with SMA.

To help reduce the financial burden of participation in the trial for families and optimize reliability of study measures, Cure SMA provided funds to reimburse families for travel expenses as well as general funding for the study.

### Enhancing Our Understanding of SMA Natural History

The NeuroNEXT study is an example of how development of a proactive, thematic and inclusive recruitment and retention plan that effectively communicates the rationale of a clinical study and partners with patients, advocacy groups and the local communities can effectively recruit participants in vulnerable populations. The success of the study has contributed to the interpretation of clinical trials in this population and to the approval of the first FDA-approved medication for SMA and promises to contribute to numerous clinical trials involving the study of motor function in infants.

This study is critical because of its definitive and well-controlled data on the natural history of SMA type I. It showed that motor function decreased rapidly in SMA infants, whereas it rapidly increased in the unaffected infants. In addition, by eight months of age, babies with two copies of SMN2 had typically reached an endpoint of either death or usage of ventilation (BiPap or a tracheotomy) for more than 16 hours a day.

This further reinforces the fact that early intervention is especially critical in SMA type I. It will also support continued efforts to add SMA to state newborn screening panels, as that represents the most effective way to ensure early treatment for those affected by SMA.



## Heather Tomko Crowned as Ms. Wheelchair USA 2018

The guest blog post below was written by Heather Tomko, sharing her experience in the Ms. Wheelchair USA 2018 pageant. She is the reigning Ms. Wheelchair USA in addition to being a public health researcher and masters student. Heather is a lifelong Pittsburgh resident and lives with SMA Type II.

Being in a pageant is not something that I ever pictured myself doing. That's not to say that I've never been attracted to the glitz and the glamour of it, but I never knew that it was something that was even an option for me. But when one of my friends e-mailed me the link for Ms. Wheelchair USA, it was something that I instantly knew I had to do.

Applying for the state-level of the pageant involved filling out an application, getting letters of recommendation sent in, and crossing my fingers and waiting! When I got the exciting news that I had been chosen as Ms. Wheelchair PA USA and I'd move on to Nationals, the reality started setting in. There were events and appearances to be at, sponsorships to obtain, dresses to buy, and hotel rooms to book! Looking over the schedule for the week was overwhelming and exciting at the same time.

Before I knew it, pageant week had arrived. The week itself was a bit of a whirlwind – the other nine women and I made appearances all around Stow, Ohio while we got to know each other. It was a little surprising how quickly we all bonded, and a culture shock when the pageant was over and we all had

to go back to our “normal” lives. It sounds very cliché, but I could have imagined any of the women winning the crown, but I am incredibly excited to have the chance to make my mark.

I was diagnosed with SMA Type II when I was around a year old, and I've been using a wheelchair for as long as I can remember. That means that for as long as I can remember, there have been things that are inaccessible to me. I'm almost 30 years old now, and accessibility-wise, I feel like not much has changed in those 30 years. This is what I'm hoping to change during my year's reign as Ms. Wheelchair USA. I want to work towards making accessibility a consideration from the outset, rather than an afterthought, so that people with SMA (and other disabilities) have greater access to what everyone else does.

To follow along with me during my reign, you can “like” my official Facebook page – “Heather Tomko – Ms. Wheelchair USA 2018.”

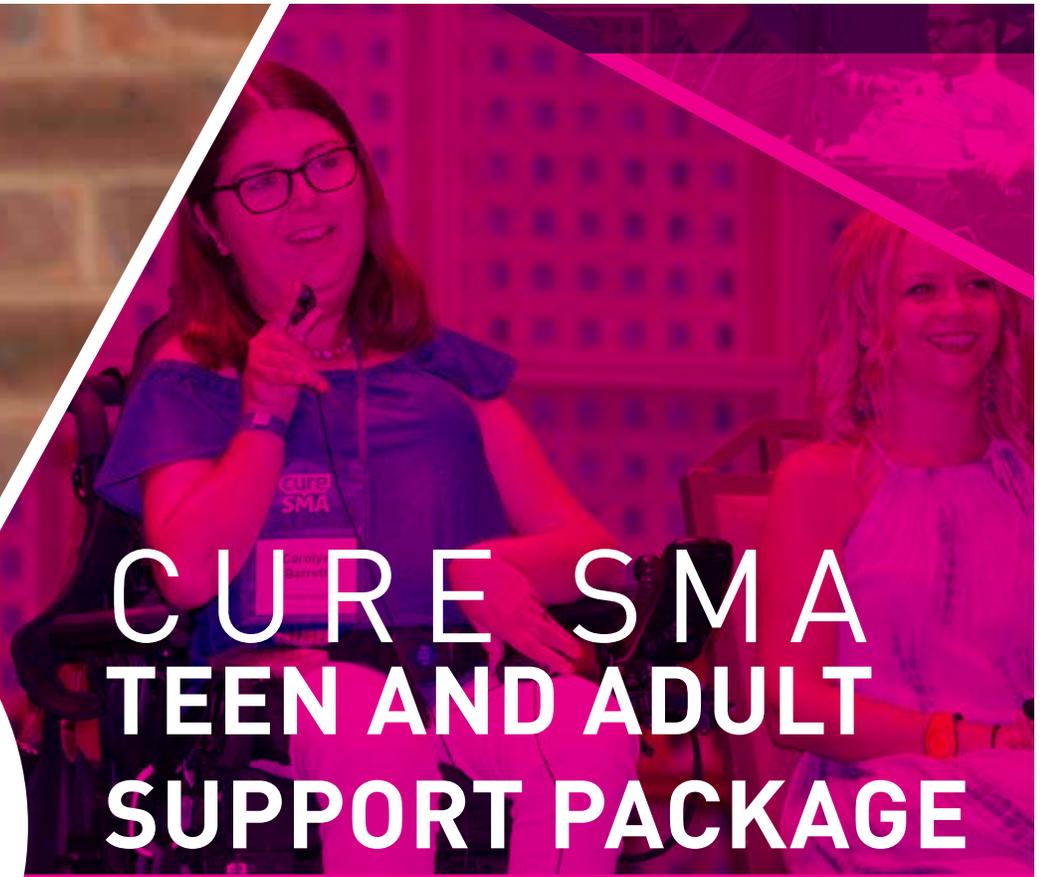
### Follow Heather and Ms. Wheelchair USA on social media!

**Blog:** The Heather Report

**Twitter:** @TheHeatherReport + @MsWheelchairUSA

**Instagram:** @theheatherreport + @mswheelchairusa

**Facebook:** The Heather Report, Heather Tomko - Ms. Wheelchair USA 2018 + Ms. Wheelchair USA



# CURE SMA TEEN AND ADULT SUPPORT PACKAGE



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They also help those living with SMA to gain independence with certain tasks they might not have been able to do on their own.

If you are a teen or adult with SMA and are interested in receiving a Cure SMA support package please visit [www.events.curesma.org/supportpackage](http://www.events.curesma.org/supportpackage) to request one at no charge.

# Thank you!



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

Whether you made an advocacy visit, attended an MLB awareness game, sent in photos or videos, or simply shared messages on social media, we appreciate the community effort to spotlight SMA and look forward to next year.



**Make today a breakthrough.**



# Cure SMA Summit of Strength

Attendance and feedback have been extremely positive for this new program.

The Summit of Strength Program is a local event uniting the SMA community around treatment, care, and support. This new program, consisting of 16 one-day mini conferences held in 16 cities across 15 states, kicked off in July and has continued to grow with great success! The Summit of Strength is a day of community and learning; it brings together families in their local areas to discuss local SMA care, support topics, research, advocacy, and more.

This Summit of Strength is intended for adults and teens with SMA, parents, grandparents, and caregivers. The average attendance so far has been 80 attendees, and the attendance keeps growing.

## These local summits have been scheduled in the following cities:

- |                            |                                  |
|----------------------------|----------------------------------|
| Cincinnati, Ohio           | Boston, Massachusetts            |
| Little Rock, Arkansas      | Phoenix, Arizona                 |
| Philadelphia, Pennsylvania | New York, New York               |
| Orlando, Florida           | Milwaukee, Wisconsin             |
| Birmingham, Alabama        | Anaheim, (Southern) California   |
| Baltimore, Maryland        | Chicago, Illinois                |
| Austin, Texas              | St. Louis, Missouri              |
| Raleigh, North Carolina    | Palo Alto, (Northern) California |

## Some attendee feedback...

*“The presenters were excellent and having a chance to connect with others living with SMA was so helpful, especially since we haven’t been able to go to conference for several years.”*

*“Really appreciate having a local event to meet other families, learn about various topics and share information.”*

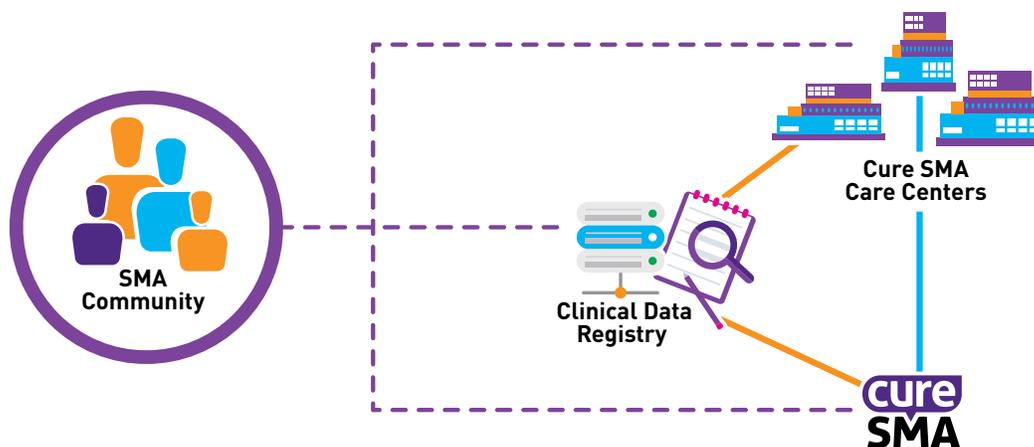
*“All the topics were well chosen and pertinent to the needs of the audience.”*

*“Very important for the community - Great job.”*

*“It was excellent and very helpful and informative.”*

For more information, please contact [familysupport@curesma.org](mailto:familysupport@curesma.org) or visit [www.CureSMA.org](http://www.CureSMA.org).

We thank AveXis for sponsoring this initiative, as well as all the wonderful speakers who have presented at each Summit of Strength.



## SMA Care Center Network and Clinical Data Registry Launched

In October, Cure SMA the launch of our SMA Care Center Network. The SMA Care Center Network is the centerpiece of our efforts to address the changing landscape of SMA. The goal of the SMA Care Center Network is to develop an evidence-based standard of care that will improve the lives of all those affected by SMA.

Individuals affected by SMA have complex medical needs. This requires a team approach, with neurologists, pulmonologists, physical and occupational therapists, nutritionists, orthopedists, and others working together to treat the individual. Just as the clinical team takes a multi-faceted approach to treating the individual, Cure SMA takes a multi-faceted approach to improving care for all those affected by SMA.

With the advent of approved therapies for SMA, care is more important than ever before. As treatments extend lifespan, the number of individuals living with SMA will increase. This means we need more doctors and healthcare providers trained in SMA care, particularly for the teen and adult SMA communities. And with new treatments, the experience of SMA is rapidly shifting, and healthcare providers need to adapt quickly to these changing needs.

The network will also help expand access to approved SMA treatments, increase the number of sites for SMA clinical trials, and help accelerate and enhance insurance coverage and reimbursement.

### More About the SMA Care Center Network

In this initial launch phase, we will begin with 16 geographically diverse centers, to ensure we collect information to understand

the needs of our entire community. In subsequent years, additional care centers will be added to the network as our community's needs continue to grow.

The first four of these 16 centers are Arkansas Children's Hospital, Little Rock, AR; Gillette Children's Specialty Healthcare, St. Paul, MN; University of Rochester Medical Center, Rochester, NY; and the University of Utah Primary Children's Hospital, Salt Lake City, UT. The remaining 12 centers will be announced through the end of the year.

Member sites will collaboratively collect patient care data through the clinical data registry. This data will help answer questions about the impact of SMA and develop strategies for optimal care for those affected by SMA.

From this analyzed broad body of data, we will determine a standard of care and best practices, and then share that with local doctors and healthcare providers across the country. This will allow all with SMA to benefit from the most cutting-edge information on care.

The SMA Care Center Network will also increase the number of sites that offer access to approved SMA treatments, by providing doctors with readily available data to guide proper administration of current and future approved treatments.

Over the course of the next three years, Cure SMA will invest \$9.1 million in the development of the SMA Care Center Network. And the SMA Care Center Network is just one piece of our growing efforts in care, which include the SMA Clinical Care Meeting, held each year as part of the Annual SMA Conference, Cure SMA care series booklets, and our physical and occupational therapy mentoring programs, funded by Cure SMA.



# cure SMA 2019 ANNUAL SMA CONFERENCE

**Friday**  
 June 28th, 2019  
**TO Monday**  
 July 1st, 2019

## DISNEYLAND HOTEL IN ANAHEIM, CALIFORNIA

Every year, Cure SMA sponsors a conference to bring together the leading SMA researchers, clinicians, and families living with SMA. Cure SMA has been hosting the Annual SMA Conference since 1988. The weekend is filled with a wide variety of workshops, keynote sessions with leading researchers, a family-friendly poster session, a memorable children's program, a meet & greet with a family fun fest, teen and adult social activities, a dance party, and a Disneyland Night at the Park, as well as many opportunities to connect and interact with families and receive first hand updates from the researchers.

We look forward to reuniting as a community at this conference and showing our support for others. As always, the Family, Research, and Clinical Care Conferences run alongside each other. This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it. The interactions between the families, researchers, and clinicians at this conference are extremely special. The annual conference also provides the children an opportunity to make new friends and have a great time. We are expecting another great attendance of over 1,500 attendees.

Disneyland Hotel, Disney's Paradise Pier Hotel, and Disney's Grand Californian Hotel & Spa in Anaheim, California have been carefully selected to meet the needs of the SMA Community for the 2019 Annual SMA Conference.

You must complete your conference registration with Cure SMA prior to reserving your hotel room, for the special room rate of \$234 at the Disneyland Hotel, \$218 at Disney's Paradise Pier Hotel, and \$300 at Disney's Grand Californian Hotel & Spa per night, plus tax.

Disneyland Hotel, where the majority of the conference will take place, is a whimsical hotel with exceptional dining, special Disney character visits, outdoor movie nights, a complete fitness center, and a pool complex which includes two retro-themed pools, two whirlpool spas, cabanas, and two monorail waterslides. You are also just steps away from the vibrant Downtown Disney District and numerous other Hotels of the Disneyland Resort area, including Disney's Paradise Pier Hotel and Disney's Grand Californian Hotel & Spa.

Relax with any of the luxurious spa services at the Mandara Spa, which is conveniently located at Disney's Grand Californian Hotel & Spa, listen to live acoustic music nightly at Trader Sam's Enchanted Tiki Bar, take a "Happiest Hotel on Earth" guided historical tour, and test out your Disney knowledge at Disneyland Park with a trivia sheet that you can request from Guest Services. And always keep an eye out for Disney characters visiting these activities!

Guest rooms are sophisticated and fanciful, in true Disney style. They are non-smoking and offer some great features like a mini refrigerator, coffee station, room safe, and free WIFI service.

Disneyland Hotel offers a fine dining experience at Steakhouse 55, along with themed eateries like Tangaroa Terrace, which features casual island meals, and Goofy's Kitchen, which is delightful all-you-care-to-enjoy buffet that includes a visit from Goofy, himself! Grab quick bites and morning refreshments at the Coffee House, and enjoy legendary libations with friends and family at Trader Sam's Enchanted Tiki Bar or The Lounge at Steakhouse 55 after a busy day at conference.

**To register for the Annual SMA Conference, please visit [www.cureSMA.org](http://www.cureSMA.org)**



## SMA CONFERENCE GOALS

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

### NEWLY DIAGNOSED CONFERENCE PROGRAM

Thanks to the generous funding provided by the Erin Trainor Memorial Fund, Cure SMA covers up to 4 family members' registration fees as well as one hotel room for 3 nights for the Annual SMA Conference. This program is for all families newly diagnosed since the last conference. The mission of the Erin Trainor Memorial Fund (ETMF) is to generate substantial funds to be able to provide conference scholarships, allowing newly diagnosed SMA families/individuals the opportunity to attend the Annual SMA Conference. Our goal is to allow as many newly diagnosed SMA families as possible to attend and experience the benefits of this amazing conference. The meeting gives families the opportunity to gather critical care and daily living information early after diagnosis, and learn directly from experienced SMA physicians and network with other families. This program is automatically offered to all newly diagnosed families that contact Cure SMA.

[For more information, please email familysupport@curesma.org](mailto:familysupport@curesma.org)

### GENERAL CONFERENCE SCHOLARSHIP

For families who are not newly diagnosed, we also have general conference scholarships available to help waive the registration fee costs to attend the conference. Families looking to receive a general conference scholarship are required to apply and can be placed on a scholarship waiting list. They will be notified if they are able to receive a scholarship.

[Please email conference@curesma.org for more information.](mailto:conference@curesma.org)

### SPECIAL MEETING AND CONVENTION THEME PARK TICKETS

Come for the conference and stay for the magic! Make the most of your free time with special Disney Meeting and Convention Theme Park tickets. 2019 Annual SMA Conference attendees are eligible for advance purchase of specially priced discounted Disney Meeting/Convention Theme Park tickets. Ticket information will be provided after you register for the Annual SMA Conference.

## EXHIBITOR OPPORTUNITIES

The Annual SMA Conference is a wonderful opportunity to promote your company and products to SMA families, medical professionals, and researchers from all over the world.

By hosting an exhibitor table or booth, you will be able to personally share the details of your new and existing products with several SMA families and medical professionals, as well as be prominently displayed throughout the conference and online.

**For more information, please email [exhibitor@curesma.org](mailto:exhibitor@curesma.org)**

## SPONSORSHIP OPPORTUNITIES

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

**For more information, please email [sponsorship@curesma.org](mailto:sponsorship@curesma.org)**



**Please visit [www.curesma.org](http://www.curesma.org) for more conference information.**



## Care Packages



Thank you to the Crane family for collecting and donating Isaac's favorite toy to share with others in honor of his 4th birthday!



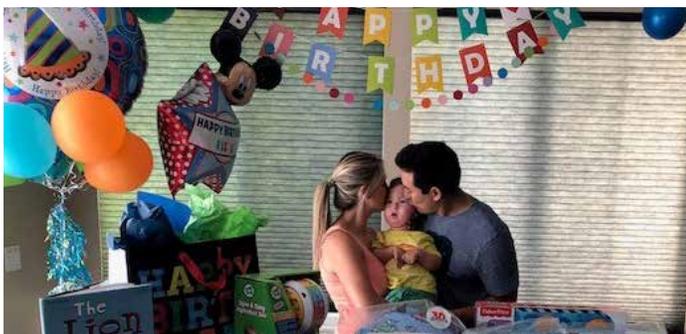
A big thank you to Sophia Salus's grandma, Pat Ross, for creating and donating an array of fun pillows for our care packages!



An assortment of great toys were collected in Hannah Costlow's honor by her family and friends, and we would like to thank you all for this wonderful donation!



The Facchini family donated numerous fantastic toys to the care package program to celebrate Charlie's 3rd birthday! Each toy is so greatly appreciated!



In honor of Tristan Andrew's 2nd birthday, a large number of wonderful toys were collected for the care package program! Thanks to all who donated!



Thank you to the Metter family who thoughtfully donated lovely handmade blankets to the care package program in honor of their daughter, Charlotte.



Thank you to the Bauss family for generously donating valuable toys and equipment for the Cure SMA care package program and equipment pool, in honor of Cason.



So many lovely toys were donated to the care package program from the month-long toy drive in honor of Remi Sizemore's 2nd birthday! Thank you to all who participated!



Homemade baby blankets were donated by Louise Chisholm, a Canadian artist from Long Island, Nova Scotia, in celebration of Bodhi Bhattarai. Louise spent her career as a pediatric nurse and has donated hundreds of these blankets to hospitals all over Canada and the U.S., including around 20 quilts to Cure SMA. Thank you, Louise, for your generous donation!



Ava Ruvalcaba's family donated an assortment of fantastic toys for the care package program in honor of her 1st birthday! Thank you to all the Ruvalcaba's family and friends!

# UNLOCK POSSIBILITIES AT A SPINRAZA EVENT

Get knowledge and resources about the first treatment for spinal muscular atrophy (SMA)



## SPINRAZA for SMA

Learn about SPINRAZA treatment from a healthcare provider (HCP)



## Treatment Journey

Hear inspirational stories and build connections with the SMA community



## Support Services

Understand what resources may be available for you



Visit [SPINRAZAevents.com](https://www.spinrazaevents.com) or call 1-888-615-4343 to sign up for the next free educational webcast or live event near you

### INDICATION

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

### IMPORTANT SAFETY INFORMATION

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

**The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**For additional Important Safety Information, please see brief summary of full Prescribing Information on the next page.**

This information is not intended to replace discussions with your healthcare provider.



Individual results may vary based on several factors, including severity of disease, initiation of treatment, and duration of therapy.

### IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

#### USES

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

#### WARNINGS

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

#### COMMON SIDE EFFECTS

- **The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome (headache related to the intrathecal procedure).
- Serious side effects of complete or partial collapse of a lung or lobe of a lung have been reported.

**Talk to your healthcare provider about any side effect that bothers you or that does not go away.**

#### OTHER INFORMATION

SPINRAZA is a medication that should be administered as an injection into the lower back (a procedure called intrathecal injection) by, or under the direction of, an experienced healthcare professional.

#### QUESTIONS?

The risk information provided here is not comprehensive. To learn more, talk about SPINRAZA with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at [www.spinraza.com](http://www.spinraza.com) or 1-844-4SPINRAZA (1-844-477-4672).

#### MANUFACTURED FOR

Biogen  
Cambridge, MA 02142

revised 05/18



# Sharing Photos







# Family Letters



**Hi Cure SMA,**

Thank you so much for the care package! Skylar loves all the toys!!!

**Brittany Phillips**



**Cure SMA,**

We are very appreciative of the care package. There are lots of useful items in it. Thank you so much!

**The Sangster Family**



**Hi Cure SMA,**

My name is Sharisse and I'm Kylee's mommy. Thank you so very much for her care package, we love everything in there. Kylee loves everything! Very thoughtful.

**Sharisse D.**

**Dear Cure SMA,**

We received the care package filled with toys for Jonathan. Thank you very much, we appreciate Cure SMA's kindness. The items are amazing and we can't wait to use them with Jonathan. We really are grateful for everything.

**Sincerely,  
Natalia Gutierrez**



**Hello Cure SMA,**

Abram received his Cure SMA goodies box last week! It was like Christmas morning! Here are a few pictures of him getting into things and enjoying them. Thank you again for such a generous gift.

**Jackie Lackey**



**The entire staff at Cure SMA,**

We can't say thank you enough for the care package. My son, Henry, especially loves the orange feather, the ball, and the toy rings as they are all light and he can grasp them. We are so thankful for all of the info you mailed out too.

**The Farkas Family**



**Hi Cure SMA,**

We are so happy to receive the care package which lifted our spirits up. I thank you, Cure SMA and the rest of the SMA families who took time to help improve our life and mindset. My family and I will continue to keep in touch and share the love and joy given to us.

**Sincerely,**

**Ferdinand, Marisa, & Mayumi Corpuz**



**Cure SMA,**

Thank you all SO much for this generous box of fun for Eli! He LOVED seeing each thing come out of the box! Especially the Play-Doh and Nerf gun! We are extremely thankful.

**Thank you,  
Nichole Guard**



**Hi Cure SMA,**

Thank you so much for the care package. Maisie already loves it!! She is especially fond of the ball. Loving having a support system.

**Thank you again!!  
Ciji Green**



**Cure SMA,**

This is to inform you that I received your valuable CD which includes your Care Series booklets plus useful information. Please accept our sincerest thanks and kindly find attached Dima's regards to you.

**Dara Hameed**

**Dear Cure SMA,**

Thank you so much for the care package you sent us shortly after Samuel's diagnosis. I am overwhelmed and overcome with emotion as I read the notes and look at all of the special items. The love and support we have received from this organization and from the other families who have been affected by this disease is second to none! We feel the love and we are so very grateful!

**Sincerely,  
Courtney Ogletree**



**Dear Cure SMA Team,**

Thank you so much for the care package that you sent out Reyna. We weren't expecting what we received, and we are blown away by the care and compassion you are showing our family. We just wanted to thank you for the help you've been providing us. Thank you all again so much for what you do!

**Sincerely,  
Luis Diaz**

**Hi Cure SMA,**

We just wanted to say thank you for the care package for Matteo. Gabriella was diagnosed with SMA, type 3 at age 4.5 years and you were there for us in my time of need. Unfortunately, my son Matteo was also just diagnosed at age 8 years. We were crushed to say the least, but you were there to support us again. Matteo was very surprised by his package. He especially loves the toy guns that shoot foam bullets, which I keep finding all over the floor. They are getting used well! Thank you again for supporting my family.

**Love,  
Jennifer, David, Gabriella and Matteo Ramirez**



**Cure SMA,**

Wow! I got our box of toys today. THANK YOU SO MUCH! I'm not a crier, but the love and support we've gotten just brings tears to my eyes. You all are doing wonderful things! Thanks soooo much!

**Thank you,  
Jenna, Frank, and Gabriel Martinez**

### Everyone at Cure SMA,

It was so incredibly kind of all of you to send such an amazing care package. It was so wonderful to see each personalized description of the goodies sent. The toys and blankets are fantastic, and the binder is something that neither my husband nor I have thought to use, and it's only made it better by the immaculate organization of it. I can honestly say that we are humbled by your generosity and support. I am truly speechless, not only by all of the thoughtful gifts, but by the evident compassion you all have.

We haven't had the opportunity to have him try everything out, as we just received the package earlier today. However, I have attached a picture of our sweet William, aka "Liam." I'm certain that you'll be able to see how much he also appreciates your generosity, support, and all around love.

We are astronomically lucky to have an organization like this to turn to. You all have helped to not only restore our faith in humanity, but to see a light at the end of this super scary tunnel life has thrown at us.

Again, we truly can't thank you enough for everything.

**Best Regards,  
Megan Fabrizi**



### Good Morning,

I cannot thank you and Cure SMA enough for sending me the Hope Car Bed. We got it just in time for her Spinraza treatment. I absolutely love it and most importantly so does Kennedy. As soon as I put her in it and strapped her in her O2 levels and BPMs were perfect. She laid awake for part of our 30 minute drive then fell asleep. And my stress was gone knowing she was safe. So again, thank you for letting me borrow it.

**Love,  
Jamie and Kennedy Stevens**

### Cure SMA is AMAZING.

It's an organization dedicated to the treatment and cure of SMA. They fund groundbreaking research and provide families like ours all the support we could ever need. When Marley was first diagnosed my best friend Julia got in touch with them. Not too long after we had a massive box of toys and things all the other SMA kids like to play with, use and to make life a little easier. Then, they sent us a car bed for Marley to make the travels back and forth to Iowa City much more comfortable for her. They hold an annual conference every year for all the SMA families to gather and just feel normal for a bit. We haven't gone yet but we want to try and make it next year. Yesterday, they sent us a BRAND NEW Panthera Micro Wheelchair that was donated. I had signed up for their waiting list months ago not sure really what to expect. This wheelchair is used by many of our friends and we have heard good things about it. Unfortunately, it is made overseas, so insurance doesn't cover it and costs anywhere from \$4k-\$6k brand new. Dillon and I were stressing about trying to buy one new or used and we got the email last week that we were next on the waiting list. AND THEN THIS SHOWED UP! Everyone in the household is very excited and grateful.

**Sincerely,  
Alisha Grauer**





## Community Spotlight: Adrienne Vollmer

In May, Governor Eric Holcomb held a signing ceremony for Indiana House Bill 1017, adopting newborn screening for SMA and SCID. Dubbed “Graham’s Bill” in honor of Graham Vollmer, the bill was largely advanced by the efforts of Adrienne Vollmer, newborn screening advocate and SMA mom. The signing was held at the Cure SMA Walk for Graham, an event organized by the Vollmer family and benefitting Cure SMA.

### Adrienne recently shared with us her advocacy experiences and advice.

**Cure SMA:** Tell us a little about your family:

**Adrienne Vollmer:** I live with my family in Westfield, IN. My husband Nick and I have been married for 11 years, and we have three beautiful children. Ben is 8, Lucy is 6, and Graham is 3. Graham was diagnosed with SMA type 1 when he was 7 weeks old.

**CS:** Why did you choose to start advocating on behalf of SMA newborn screening?

**AV:** When Graham was diagnosed, it was one of the scariest times of my life. To think that I might lose a child was something I couldn’t possibly wrap my head around. We decided at that point that we would always advocate and fight for children and their families who were going through this experience. And most importantly, I wanted to feel like I was doing something to make a difference.

**CS:** What has been your experience advocating in Indiana?

**AV:** I have been welcomed by the legislators here in Indiana with open arms. State Reps Doug Gutwein and Donna Schaibley really paved a way for me into the decision-



making process and helped give my voice a platform to stand on. For me, just knowing that I had the right people in our corner made all of the difference in the world.

**CS:** What are you most proud of in regard to your advocacy?

**AV:** Being able to have the opportunity to share Graham's story in front of the Indiana House and Senate, and having the newborn screening bill passed with 100% agreement from both sides of the aisle... these are things that I will never forget. Truly once in a lifetime opportunities for me.

**CS:** What has been the most challenging part of your advocacy experience, if anything?

**AV:** I would say that the only challenge is stepping outside of my comfort zone and really be willing to stand up for what I believe in. The newborn screening didn't directly impact my family, but it was really important to me to step up and try to make a change for those families yet to be diagnosed. Fighting for those babies that aren't born yet.

**CS:** Indiana is one of six states that have passed laws adding SMA to their newborn screening panels. Do you have advice to advocates in states where things aren't moving as quickly?

**AV:** My advice would be to utilize resources from other states. Look at states like Indiana and Missouri where these laws have successfully passed and see if you can get those legislators to make some phone calls for you. I bet they will, and I'm sure that it will help pave the way to the right people. It's all about getting in front of the right people.



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**SMA** **SMA CONFERENCE**

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**You may be gone from  
my sight, but you are never  
gone from my heart.**

**In remembrance of all of the lives  
lost to Spinal Muscular Atrophy.**



# GET INVOLVED WITH YOUR LOCAL CHAPTER

**Cure SMA has 34 volunteer chapters throughout the United States.**

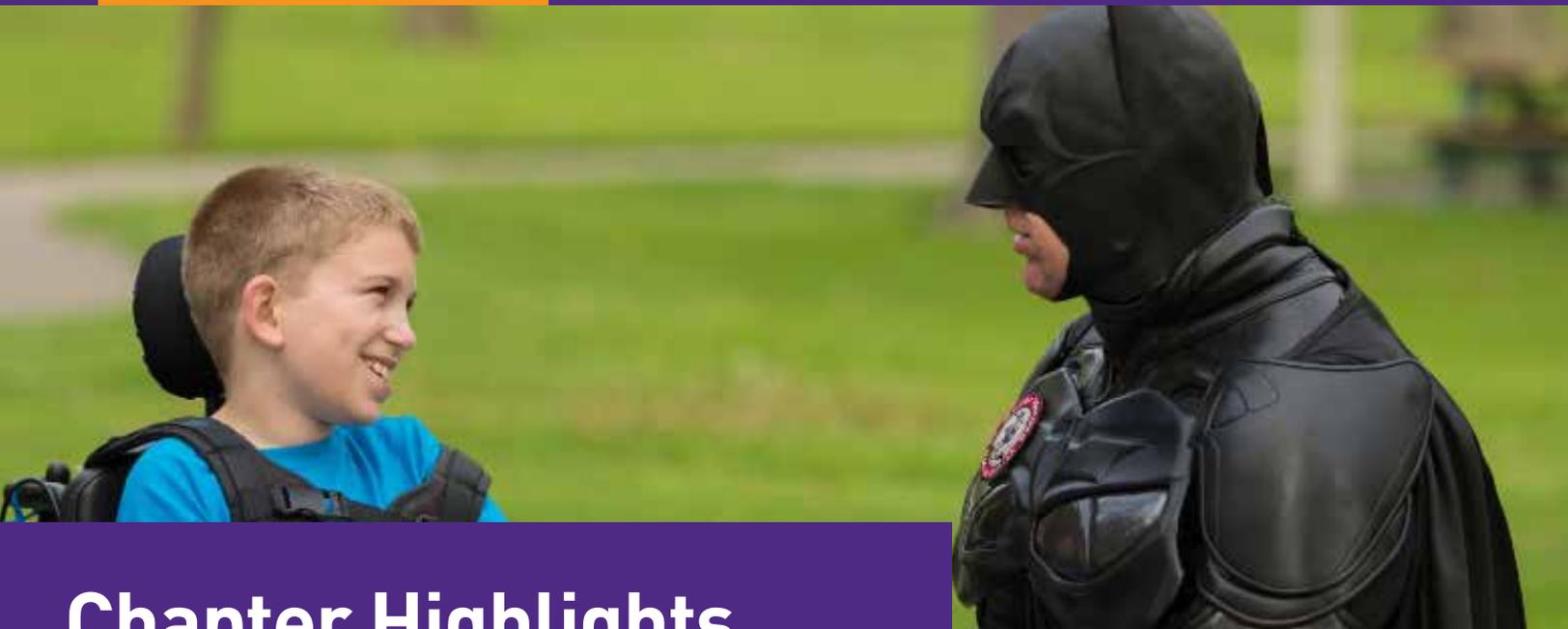
Our chapters provide support for affected families through networking, fundraising events, and advocacy. As representatives of Cure SMA, chapter leaders spread SMA awareness in their local communities and generate support for our organization.



**VOLUNTEER OPPORTUNITIES ARE AVAILABLE NATIONALLY**

Please contact [fundraising@curesma.org](mailto:fundraising@curesma.org) for more information.

Visit <http://www.curesma.org/get-involved> to find your local chapter.



## Chapter Highlights

### Nick's Kids Fundraiser to benefit Cure SMA

#### Alabama Chapter

On August 2, 2018, in Tuscaloosa, AL, Cure SMA was awarded \$1,000 from Nick's Kids Foundation to support the mission of Cure SMA. The University of Alabama Head Football Coach Nick Saban and his wife Terry fundraise year-round to be able to donate to organizations throughout the State of Alabama and the Southeast. Special thanks go to Nick's Kids Foundation for their continuous support of Cure SMA!



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## Arizona Chapter



## Byrds FORE a Cure Golf Classic

The 5th Annual Byrds FORE a Cure Charity Golf Classic took place at Stonecreek Golf Club in Phoenix, AZ, on May 5, 2018. Together with community support, the event raised **\$10,120**. The Byrds FORE a Cure golf event is in honor of twins, Kyle and Lauren Byrd, who were diagnosed with SMA in 2008.

Special thanks to Cassandra Johnson for organizing this annual event, as well as to the Byrd Family and Ken and Laurie Johnson for all the additional work they do that helps make this event such a success!

## Arkansas Chapter



## 2018 Arkansas Chapter Walk-n-Roll

The Inaugural Arkansas Chapter Walk-n-Roll was held on April 14, 2018, at Rogers Park in Rogers, AR. Despite the cold weather, over 65 participants attended and raised \$4,931 for Cure SMA. The event featured a scenic one-mile walk, family games and activities, a DJ, snacks and refreshments!

Thank you to the Chapter Leaders Kelly and Jason Alexander, DeAnna and Sean Dillon, and Sylvia Wheeler for their hard work and dedication to making this event a success. Thank you to all the amazing families, participants, donors, and sponsors for their support and generosity!

## Carolinas Chapter



## 2018 Wilmington Walk-n-Roll

North Carolina

The Wilmington Walk-n-Roll took place on April 28, 2018, at Hugh Macrae Park in Wilmington, NC. This year a record breaking **\$15,940** was raised for SMA research and family support! We would like to extend a huge thank you to Sejal, Shashin and Sesha Patel for their hard work in planning this event, and their continued passion for Cure SMA and the mission.

## Zach Fest Benefit

The Zach Fest Benefit was held on May 12, 2018, in Burlington, NC. The day was filled with food, drinks, and live music. This first-time event raised a generous amount of funds for Cure SMA! Thank you to April Basden and her family for hosting this event in honor of their son Zach.

*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*



## Hayes' Heroes Evening of Hope

South Carolina

The 3rd Annual Hayes' Heroes Evening of Hope took place on February 24, 2018, at the City of Lexington Municipal Complex in Lexington, SC. The event was held in honor of Hayes Wingard who had a super time! This year's event raised more than any other year, **\$32,131!** It was a wonderful night filled with tasty food and drinks, a silent and live auction, live music and hope. We would like to thank Keri Johnson, Joyce Sireno, Kathy Cooper, Jaime Wingard, Amy Roberson, Callie Magee and Amanda Baldwin for their incredible efforts in planning such a successful event and for their amazing dedication to SMA research and family support.

## Kathy Cooper Spa Night

A huge thank you to Kathy Cooper for hosting a spa night fundraiser with friends in Lexington, SC on August 16, 2018. This event raised over \$250 for Cure SMA, particularly to use for our Newly Diagnosed care packages.

## SMA Sip and Shop

On August 30, 2018, local businesses in Lexington, SC donated 10% of their sales to Cure SMA. The businesses included The Classy Cruet Lexington, The Party Shoppe by Balloonopolis, James and Eloise Children's Boutique and Blush Boutique & Home. Funds were raised in honor of Hayes' Heroes. Thank you to Kathy Cooper and her family for facilitating this great fundraiser!

## Northern California Chapter

### Do it for Danny Walk-n-Roll

Danny McHale and his friends started fundraising for a cure for SMA back in 7th grade and haven't stopped since! His family started with bake sales and lemonade stands, which became the foundation of the Cure SMA Club at Monte Vista High School. All of the students at Monte Vista have joined together in the fight against SMA through various car washes, t-shirt sales and home run derbies. The Do it for Danny Walk-n-Roll on April 23, 2018 was another great event that raised \$3,798 and also helped to raise awareness of SMA in the community.

### Angie Lee Individual Fundraising Page

Core members of the Angie's Hope team live all over the country as freshmen at different colleges, and they decided to host an online fundraiser in April 2018. The fundraiser was a celebration of what they have accomplished in raising awareness and funds; it was an opportunity to promise to cure SMA even though they've been spread out to all different places in the world.

Anyone who donated more than \$5 received a laptop sticker designed by the Angie's Hope team! Thank you, Angie Lee and Kyra Scadden, for helping raise more than \$6,000!

*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*



## 2018 Cure SMA Concert for a Cure

The Cure SMA Concert for a Cure took place on April 21, 2018, at Diablo Country Club in Diablo, CA and reached a very exciting goal—after combining 18 years of fundraising and hard work, this special event has raised **\$2 million!** Three hundred and twenty guests joined together and celebrated at Aloha to the Cure, as we say ‘hello’ to a treatment for SMA, and ‘so long-see you later’ to Danny and Arianna who graduate high school this year. Aloha to the Cure raised over **\$210,000** this year!

Danny McHale, son of Joe and Mary, was diagnosed with spinal muscular atrophy in February 2001.

“When Joe and Mary asked what could be done for little Danny, the physician said “Nothing. Take him home and love him in the time you have left.” He wasn’t expected to see his first birthday. Unwilling to accept this prognosis, that afternoon the McHale’s called “Families of SMA” who told them “Yes, there is hope.”

Soon after, a friend of the McHale’s, learned of Danny’s diagnosis. Although she had met them only once, she really wanted to do something to help. She offered to host an evening of music and put together a few auction items, inviting friends and neighbors to join in. That night, back in June of 2001, more than 50 people came together in the Book’s backyard. They raised \$7,500 for Cure SMA to help further the research and development of a treatment.”

Since then, Concert for a Cure grew tremendously with the help of community support! The McHale and Dindzans families joined together to create the largest event in the

west coast for Cure SMA. During the evening guests enjoy raffles, silent auction, balloon pop and games on the terrace before gathering together in the ballroom for dinner, a live auction and presentations by the McHale Family, SMA Club at Danny’s school, and research updates about SMA by Cure SMA President, Kenneth Hobby.

Cure SMA would like to say a special thank you to Mary McHale, Nancy Dindzans, and Cathy Barsotti! This event wouldn’t be possible without the incredible dedication and help of all the committee planning members, event day volunteers, and sponsors!



*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*



## 2018 Northern California Walk-n-Roll

The 16th Annual Northern California Chapter Walk-n-Roll took place at a new location, Kennedy Grove Regional Recreation Area in El Sobrante on August 25, 2018. Over 425 participants and 33 teams raised **\$111,000** for Cure SMA! The day began with a fun, dance warm up and announcements by Walk Chair, Pat Wolff! Friends and families took a short walk around the beautiful park. Sandwich lunches were served following the walk, along with juice from in-kind sponsor, Barsotti Juice Co. and Sohum's Lemonade Stand! Everyone enjoyed the new picnic area and balloon artist,

face painter, and memorial area hosted by the Live RhysStrong Foundation. The day ended with a very exciting raffle!

This walk would not be possible without the help from the event committee, volunteers, and sponsors. A huge thank you goes out to David and Lisa Sereni, Linda Shively, Marge Shively, and Pat and Dick Wolff! Each year, this event grows bigger and bigger because of their dedication. Cure SMA and the Northern California Chapter appreciate everything you do!



Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.



## 2018 Wannabe Cup

Between August 2 and 4, 2018, the 20th Annual Wannabe Cup Charity Golf Tournament was held at the Silverado Resort in Napa, CA. The event included two days of golf; first and final matches with closing ceremonies and awards.

This year's event raised **\$17,093**.

The Wannabe Cup started 20 years ago in honor of Skylar Bahrenburg. The friends and family of the Bahrenburg's would gather together for the two-day long golf tournament, fundraising and giving back to the SMA community by donating their funds to Cure SMA. Since the group of 32 golfers are in California, Washington, and Oregon, this annual event rotates taking place in either Oregon or Northern California.

*A special thank you to Joe Belcher and Andy Belcher for organizing this annual golf tournament.*



## Southern California Chapter



## Southern California Walk-n-Roll

On April 14, 2018, families gathered together at TeWinkle Park in Costa Mesa for the Southern California Walk-n-Roll. The morning was filled with a walk around the park, lunch and an exciting raffle. Thanks to the following teams for helping raise \$9,595 for Cure SMA: Fight for Charlie, Princess Ava's Squad, Team Biogen, Team Kennedy, WheelyQ BBQ and Wyatt Marion! Thank you to Autumn and Rick Montoya for planning this week each year.

## Ann Katz Birthday Fundraiser

Thank you to Ann Katz from San Diego, CA, for raising \$3,363 in honor of her granddaughter, Victoria Koblentz, during the month of June!

## WheelyQ SMA Sucks



During the month of August, Collin Pollock, who has SMA, donated \$1 for each WheelyQ BBQ Sauce purchased online. Thank you, Collin, for your partnership!

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## 2018 Los Angeles Walk-n-Roll

Griffith Park was home to the Inaugural Los Angeles Walk-n-Roll on May 19, 2018! The morning started with a walk around Equestrian Trail followed by a visit by Star Wars Storm Troopers. Over 75 participants formed eight teams raising \$9,585 for Cure SMA! We are excited for next year's walk to be even bigger on May 4, 2019! Thank you to Ruby Lopez and all the volunteers who helped with this meaningful, new event!



## 2018 Fitness After Dark

The 3rd Annual Fitness After Dark in honor of John Robert Echman took place on August 24, 2018, at The Camp Transformation Center in Menifee Lakes. Thanks to Chad Burlison for hosting this exciting boot camp workout class and raising over \$1,000!

## 2018 San Diego Rock 'n' Roll Marathon

Team Cure SMA – Rock 'n' Roll Marathon Series in San Diego was held on June 2, 2018 in downtown San Diego, CA. Everyone teamed up to run the San Diego Rock 'n' Roll Marathon and Half Marathon to raise money and awareness for team Cure SMA. Thank you to our runners Brad Colenna and Jennifer Cuthbert for raising \$900 for Cure SMA!



## Surf Away with Ricochet the Dog

Every August during SMA Awareness Month, Cure SMA and IONIS Pharmaceuticals host a free assisted surfing experience lead by Ricochet the SURFice dog and professional surfers on the beautiful beaches of La Jolla Shores in La Jolla, CA. Friday, August 3, 2018, brought together many new SMA faces and families. Over 40 kids and families enjoyed lunch, a tattoo artist and games after catching some waves!

A huge thank you to Kristina Bowyer, Patrick Cauntay and Max Moore for planning this special event and an incredible thank you to Ricochet for making the day possible for those affected by SMA.

## John's Incredible Pizza Dine and Donate

On July 27, 2018, families in Southern California gathered together at John's Incredible Pizza and raised \$40 from the Dine and Donate event! Thank you to Autumn and Rickk Montoya for planning this FUN-raiser.

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## Chesapeake Chapter



## 25th Annual Chesapeake Crab Feast and Silent Auction

Maryland

This year the Chesapeake Chapter celebrated its milestone 25th Annual Crab Feast and Silent Auction on July 29, 2018, at the American Legion Hall in Towson, MD. Three hundred and fifty people came together to celebrate another remarkable year as well as to raise funds for Cure SMA. Attendees were able to participate and enjoy the silent auction, wine and dine raffle, laydown games and beer wheel. The event raised over **\$37,000** for the Chesapeake Chapter and Erin Trainor Memorial Fund. Longtime supporters came from far and wide to be part of the day and received a commemorative wine glass with a thank you inside. The note read, "Words cannot express our gratitude and sense of joy as we continue to celebrate a very effective treatment for SMA. This would not have been possible without you, and for this Cure SMA raises a glass and toasts you today as we celebrate our 25th year!" The Chesapeake Chapter looks forward to next year's 26th Annual Crab Feast on August 4, 2019.

Many thanks to the ticket captains who contributed to the sold-out event; Charles Nolan, Mike Demchak, Anthony Nolan, Stephen Nolan, Matt Brunner, Eugene Trainor, Bob Cawley, Billy Owings, Joe Maher, Patrick Farace, and Mary Kate Venedam. Special thanks to the auction item donors, crab servers and Knights of Columbus #5208 for managing the wheels and raffles.

The Erin Trainor Memorial Fund is an endowment established to provide scholarships for newly diagnosed families to attend the Annual SMA Conference. Cure SMA has been coordinating this conference since 1980 and in this time, it has become the SMA conference to attend on an annual basis for families, clinicians and researchers alike. Thank you to the event organizers for their unwavering support the past 25 years to make this a special day in memory of Erin Trainor: Barb and Gene Trainor, Beverly and Dan Venedam, and John and Katy Nolan.

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## 2018 Chesapeake Chapter Walk-n-Roll

The Chesapeake Chapter Walk-n-Roll was held on May 19, 2018 at Old National Pike Park in Mount Airy, MD. The event was a huge success surpassing the event goal and raising **\$23,560**. Thirteen teams and over 100 walkers and rollers attended and had their faces painted by Flo's Faces, listened to music played by DJ Alfie, and were well fed by Larry Jackson's "Bullhead Pit Beef."

A huge thank you to the event day volunteers and especially the event organizers: Tina and Derek Lewis, Kim Heinrich, Barbara White, Melissa Wayland and Beverly Venedam for hosting an amazing event. Your hard work and dedication is greatly appreciated. Lastly, thanks to Stacey Markel Photography for donating her time and the generous sponsors: Our Little Jewels, Starcom Construction, Johns Hopkins Homecare Group, Mobility Works and New Beginning Pediatric Rehab.

## United Parish of Bowie Walk-n-Roll

The United Parish of Bowie hosted their third walk-n-roll to benefit Cure SMA on June 9, 2018, in Bowie, MD. Attendees enjoyed food, games, a bounce house, face painting, crafts and more while raising over \$3,500 to help fund critical research and family support programs. Thank you to Matthew and Christine Bickel for organizing this event in honor of their son Kyle. Cure SMA is so grateful to the Bowie community for joining the fight to cure SMA.

## Evening of Hope for Kamdyn's Kure

The 2018 Evening of Hope for Kamdyn's Kure was held on April 27, 2018, at its new venue, DuCar Farm in Chambersburg, PA. It was a special evening with family and friends, featuring a huge raffle, silent auction, and dancing. The dinner was delicious and catered by Hempen Hill BBQ. There were over 90 guests in attendance and the event raised **\$14,622** for SMA research and family support programs! A special thank you to the event chair, Amber Hartung, who organized this fundraiser in honor of her son, Kamdyn.



In addition, the evening would not have been possible without the hardworking and passionate committee members: Christina Dull, Mallory Pellegrini, Pam King and Steph Davenport. Thank you to the volunteers and in-kind donors, who helped make the night an even greater success. Lastly, thank you to the local sponsors: Cara and Duane Sheaffer of DuCar Farm, Steve and Pam Cramer, Brad Helmuth Drywall Services, Peachy Keen, Pretty Faces by Sasha, RS Entertainment, Starr Insurance, Sweet William Floral and Scratch Catering.

## Bull Roast Fundraiser in Honor of Camden O'Rear

On April 22, 2018, at the Tall Cedars Hall in Baltimore, MD, the O'Rear family hosted a Bull Roast to raise money for Cure SMA. Thank you to Robyn and Tim O'Rear who organized this event and raised \$2,000 in memory of their son, Camden, and all the other children who have lost their battle or are still fighting SMA.

## Max's 5th Birthday Fundraiser

On August 1, 2018, in celebration of Max Lasko turning 5 years old, the Lasko family organized a fundraiser to support Cure SMA. Thank you to Jonathan and Kristen Lasko from Derwood, MD, for raising \$931 for support programs for families affected by SMA and research to help develop new treatments that will hopefully one day lead to a cure!



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## 2018 West Virginia Walk-n-Roll

West Virginia

On May 19, 2018, the Chesapeake Chapter held the West Virginia Walk-n-Roll in Star City, WV at Edith Barill Riverfront Park. Over 150 walkers and rollers from West Virginia as well as the surrounding states of Pennsylvania, Ohio, and Maryland came together to raise **\$17,573**. Thank you to the event day volunteers, raffle basket donors, and live band. A special thank you to Rose Kraftick for her talented face-painting skills, Cress Photography for capturing the day, and each team who passionately raised funds and awareness for this year's event.

Cure SMA would like to acknowledge the incredible event organizers Ashley Close, Julie Coddington, Caressa Delaney, Janice Kress, Ann Stanley and Candace Strader for putting on this wonderful event. Also thank you to the generous sponsors: Mylan Pharmaceutical, Cardinal Pediatrics, United Bank and Allan N. Karlin & Associates.

## Cycling Fundraiser for Winnie Stanley

Thank you to Ann Stanley and Candace Strader for organizing the first cycling fundraiser in the Chesapeake Chapter for SMA Awareness Month. Over 50 participants came together on August 24, 2018 at three different Zenergy Cycling locations in Morgantown, WV, Charleston, WV and Pittsburgh, PA. Cure SMA is grateful to each rider and donor for participating in this fundraising event in honor of Winnie Stanley.



## Connecticut Chapter



## Sun Life Financial Hockey Game

In August of 2017, the Sun Life work family learned the heartbreaking news that Isaac Bliven, the son of an employee, was diagnosed with SMA. In support of Isaac, Sun Life employees purchased tickets to the Hartford Wolf Pack hockey game in Hartford, CT on February 23, 2018. \$5.00 from each ticket purchased went directly to Cure SMA. Through the ticket sales and online donations from Sun Life and beyond, they raised over \$7,000 for Cure SMA and are excited for additional opportunities to assist in curing this terrible disease! Special thank you to Greg Sobin and Brett Bliven for their hard work in putting this fundraiser together for Cure SMA!

## Cubby's Run

The 7th Annual Cubby's Run was held on May 12, 2018, at the Ridgefield Rec Center Trail in Ridgefield, CT. Thanks to the hard work and dedication of Ethan Hynes, the event organizer, this walk-n-roll raised over \$4,000!

Ethan organizes this event every year in honor of his childhood friend Cubby Wax, who has SMA. Cubby now takes classes at Norwalk Community College and Western Connecticut College and is studying screenwriting and editing. He is also employed at a local movie theater where he works on his passion of writing screenplays and editing shorts. If you are interested in getting involved in the 8th Annual Cubby's Run in May 2019, please email [ethanhynes@me.com](mailto:ethanhynes@me.com).

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## 2018 Connecticut Chapter Walk-n-Roll

The Annual Connecticut Chapter Walk-n-Roll was held on Sunday, May 20, 2018 at Northwest Park in Manchester, CT. The group enjoyed a one-mile walk on a packed gravel path along the river with food, music, and raffles greeting everyone after the walk. Families, friends, coworkers, care providers, and supporters all came out to show their support for Cure SMA. There was an incredible display of generosity on the raffle tables with items from all over Connecticut. This year, the Connecticut Walk-n-Roll raised over \$4,000!

Special thanks to our event organizer Macie Tozzoli for her hard work and dedication to making this event so successful! See you in May 2019 for the next Connecticut Walk-n-Roll!

### Greater Florida Chapter

## Dare to Be Remarkable Screening

On May 1, 2018, over 40 people gathered to view a screening of Dare to Be Remarkable, a documentary about Alissa Silva, who shares her story of living with SMA. The event included a silent auction which raised over \$2,000. Thank you to Tom Dorgan for organizing this event at a local community center in The Villages, FL, to raise awareness!



## SMA Awareness Day with the Tampa Bay Rays

Over 125 friends and family members came together on August 26, 2018, to watch the Tampa Bay Rays take on the Boston Red Sox in St. Petersburg, FL. The event was held to raise awareness at the annual SMA Day with the Tampa Bay Rays. The Rays donated a portion of the ticket price raising \$670 for Cure SMA! A special thanks to Katie Kerns for organizing this fun event.



## Filipino Student Association at the University of Central Florida Bake Sale

On August 29, 2018, students in the Filipino Student Association at the University of Central Florida in Orlando, FL hosted a bake sale, raising \$100 to benefit Cure SMA. Many of the members are excited to enter the medical field and thought Cure SMA would be an ideal partnership. Thank you to Jasmine Gabriel, Philanthropy Director, for supporting Cure SMA!

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## Greater New York Chapter



### 2018 Greater NY Chapter Golf Outing

On May 21, 2018, the Greater NY Chapter Golf Outing was held at The Hamlet Golf and Country Club in Commack, NY. The golfers enjoyed beautiful weather, lunch, on-course beverages, dinner, and a raffle all while supporting an incredible cause! Thank you to everyone who attended and donated. Because of their support the goal was exceeded raising **\$41,590**. A special thank you to the Rubenstein and Sparacino families for allowing Cure SMA to host the event at the Hamlet and the Fagerland family for volunteering. Cure SMA would like to thank our event organizers Erin Bonner, Debbie Cuevas, and Catherine Martin for putting together this wonderful event.

Special thanks to the generous sponsors: Citi Group, Macy's, Latham & Watkins, Shearman & Sterling LLP, Allen & Overy, Iconic Mechanical Services Corp, and The Brookes Family.

### 2018 United Airlines NYC Half Marathon

Thank you to the three incredible runners who trained and fundraised for the 2018 United Airlines New York City Half Marathon on March 18, 2018. Cure SMA is grateful to this year's runners, Catherine Martin, Jillian Ament and Joseph Kolinsky for raising \$4,550 to support critical SMA research and family support programs!



### 2018 New York Evening of Hope

On May 2, 2018, Cure SMA hosted the Inaugural New York Evening of Hope held at Bistrot Bagatelle in New York City's Meatpacking District. It was a special evening attended by over 100 guests including unlimited beer and wine, hors d'oeuvres, a silent auction, and most importantly hope! There were speeches given by SMA researcher, Dr. Umrao Monani, from Columbia University and chair of the Cure SMA Board of Directors, Rich Rubenstein. Cure SMA is grateful to the many families who attended and helped raise **\$37,228!** A special thank you to the Kostyuchenko Family and this year's sponsors: The Geller Family, Sema4, Strategies for Wealth, International Union of Painters and Allied Trades and Institute of Culinary Education.

Lastly, thank you to the hardworking and passionate event chair, Michele Rubenstein, and wonderful committee members, Allie Balleau, Kate McCue, Alisa Rosenfeld, the volunteers, and in-kind donors, and all who helped make this year's first event a great success.

### Birthday Celebration in honor of Tina Joy-Mari Smith

Thank you to LaToya Smith from Brooklyn, NY, for raising \$200 to help find a cure for SMA. This fundraiser was in honor of LaToya's daughter, Tina Joy-Mari Smith, who was born on August 16, 2016, to celebrate her second birthday.

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## Illinois Chapter



## 3rd Annual Cure SMA Chicago Gala of Hope

On February 23, 2018, over 250 guests gathered at the Ivy Room in Chicago, IL, for the 3rd Annual Cure SMA Chicago Gala of Hope. This incredible evening raised over **\$124,000** in support of Cure SMA. This fun event brought together families, researchers, doctors, business leaders, as well as generous supporters to raise funds to find treatments and a cure for SMA.

The evening featured cocktails, hors d'oeuvres, a silent auction, live auction, and live entertainment. Comedian Brett Walkow was the MC and the band, Shout Out, performed live and kept everyone on the dance floor all night!

A special thank you to the title sponsor, AveXis, for their generous support of the 3rd Annual Chicago Gala. Cure SMA would also like to thank the other sponsors who generously supported this event: Biogen, Ivy Room, Alight, Project Farma, Amy Lenahan, and Atlas Forms & Graphics Inc. Thank you to all the committee members and volunteers for their hard work, dedication and commitment to the success of this event, especially Maria Marusich, Jamie Shish and the O'Brien family and friends.



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## 2018 AveXis SMA Awareness Month Fundraising Challenge

Throughout SMA Awareness Month, AveXis created a fundraising challenge for their employees. During this challenge AveXis employees were able to raise \$25,000 to support Cure SMA! Thanks to a generous match from AveXis their donations were doubled, and they helped raise **\$50,000** for Cure SMA!

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



## 2018 Tee Off with the Drive to Cure SMA in Honor of Ryan

On May 19, 2018, over 115 golfers gathered for 18 holes, dinner, raffles and fun at Gleneagles Country Club in Lemont, IL, for the Tee Off with the Drive to Cure SMA in Honor of Ryan! This event is in its 6th year and held in honor of Ryan Manfre, an outgoing and all-around amazing 11-year-old with SMA. With the help of generous sponsors, volunteers and golfers the event raised over **\$38,000** for Cure SMA!

Cure SMA would like to give special thanks to Jeanne Emerson, Becky & Brian Manfre and Suzanne and Steve Fedea for organizing this annual event and for their continuous support!



## 12th Annual Illinois Chapter Walk-n-Roll

The Illinois Chapter hosted the 12th Annual Illinois Chapter Walk-n-Roll on April 14, 2018, at Independence Grove in Libertyville, IL. Despite the very cold and rainy weather, we had a wonderful turnout. A few people braved the cold and rain to complete the walk while the rest of the participants gathered under the pavilion to socialize and enjoy some hot cocoa, coffee and snacks by the fire.

We would like to give a special thanks to all the amazing teams, participants, sponsors and donors for making the 12th Annual Illinois Walk a great success. Due to everyone's efforts over **\$49,000** was raised and a total of 24 teams participated!

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

A special thank you to Cynthia Annel, Jodi Garvey, Maria Marusich, Tina Krajewski and everyone else who helped plan this year's event.

## 2018 Bank of America Shamrock Shuffle 8K

Members of Team Cure SMA ran the Bank of America Shamrock Shuffle 8K on March 25, 2018, in Chicago, IL. This was the third year that Cure SMA participated in the run as an official charity partner. Together, the runners raised \$1,310 to help cure SMA! Cure SMA would like to thank all the runners: Jessica Colella, Josie Muigai, Quinn Reilly and Connor Wilson for their hard work in training to run this race as well as all their fundraising efforts!

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## 2018 Cure SMA Day with the Chicago Cubs

The Illinois Chapter had a great night on August 2, 2018, at Wrigley Field in Chicago celebrating SMA Awareness Month at the 3rd Annual Cure SMA Day with the Chicago Cubs. Three hundred friends and families turned out for a fun night of baseball and helped spread awareness about SMA at the ballpark! Thank you to everyone who came, and the chapter looks forward to planning this event again next year!



## 2018 Cure SMA Day with the Chicago White Sox

The Illinois Chapter had a fun day at Guaranteed Rate Field on August 19, 2018, celebrating SMA Awareness Month with the Inaugural Cure SMA Day with the Chicago White Sox! Over 160 friends and families turned out for a fun day of baseball and helped spread awareness about SMA at the ballpark while cheering on the White Sox to a victory! Special thanks to the White Sox fan services staff who let all the kids with Cure SMA run the bases on the field after the game! Thank you to everyone who came, and the chapter looks forward to planning this event again next year!

## 2018 Goin' for Koen Fundraiser for Chicago Half

On August 15, 2018, friends and family of Koen Guest gathered at the Tinley Park Roller Rink to raise money for Team Goin' for Koen for the upcoming Chicago Half Marathon! Special thanks to Noel and Keith Guest for planning this fun event to raise both money and awareness for SMA.

### Iowa Chapter

## 2018 Cure SMA Evening of Hope in Honor of Miracle for Madi



The 3rd Annual Evening of Hope in Honor of Miracle for Madi took place on February 16, 2018, at the Rogalski Center in Davenport, IA, in honor of Madi Ramirez. The dinner event featured a silent auction, sip n' sparkle and pass the purse, step and repeat for guests to take pictures, a plated dinner, live music, and dancing.

The event was a huge success, raising over **\$20,726** for Cure SMA!

A big thank you to Megan Ramirez and her friends and family for organizing this event and making the evening possible! A special thank you to the generous sponsors, donors, attendees, and volunteers for their support.

## 2018 SMA Awareness Game with the Quad Cities River Bandits

On August 19, 2018, families and friends gathered at Modern Woodmen Park in Davenport, IA for a SMA Awareness Game with the Quad Cities River Bandits. The River Bandits played the Peoria Chiefs during a beautiful Sunday afternoon game. The event raised funds for Cure SMA through a portion of the ticket sales, a raffle table, dunk tank, and one of the group members got to throw out the first pitch.



Thank you to Randy and Tracy Parlier for helping organize the event and to Megan Ramirez and her family for helping fundraise! Join the Cure SMA group for another River Bandits Game in August of 2019!

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## Kansas City Chapter

Kansas



## 2018 Kansas City Chapter Walk-n-Roll

The 18th Annual Kansas City Chapter Walk-n-Roll was held on August 18, 2018, at Bishop Miege High School in Roeland Park, KS. Over 320 people and 17 teams enjoyed a one-mile and 5K walk or run, raffles, activities, a DJ, and refreshments. The day was a success, raising over **\$35,915** for SMA research and family support programs!

A special thank you to Chapter Leaders Kim Sykora and Kristal Wilson, for their hard work and dedication organizing the event! Thank you to all the generous sponsors, donors, participants and volunteers who made this year a success!

## 7th Annual Gracie's Steak Dinner

Missouri

Gracie's 7th Annual Steak Dinner took place on April 28, 2018, in memory of Gracie DeGraffenreid. Friends and families from Eugene, MO, gathered to raise awareness and funds for Cure SMA. Thanks to the generosity of everyone who attended, **\$8,101** was raised for Cure SMA. Thank you to Sherry DeGraffenreid for her continued hard work and dedication planning this event every year!



## 2018 St. Louis Walk-n-Roll

The 3rd Annual St. Louis Walk-n-Roll took place on May 20, 2018, at Tower Grove Park in St. Louis, MO. SMA families and supporters gathered together to support the event and enjoyed a scenic one mile walk and run, activities, and refreshments.

Thank you to our eight teams and over 80 participants who raised \$6,195 for Cure SMA.

A special thank you to Jan Hutchinson, Valerie Giglio, and Patrick Jue for their hard work behind the scenes! A big thank you to Kathy Goodyear and Rita Schmidt for their help on event day.



## Alpha Kappa Psi's 24th Annual Charity Golf Tournament

The Alpha Kappa Psi's 24th Annual Charity Golf Tournament was held on April 14, 2018, at the Bill and Payne Stewart Golf Course in Springfield, MO. Forty players enjoyed a great day of golf, a silent auction, and 50/50 raffle!

The event was a huge success, raising \$2,800 for Cure SMA! Thank you to Liz Veit and her fraternity for organizing this event and choosing Cure SMA as their charity this year in honor of her cousin Kate!

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## Kate the Great T-shirt Fundraiser

Thank you to the Veit family in Jefferson City, MO, for raising funds for Cure SMA! In honor of their daughter Kate's birthday in March, the Veit's held an online t-shirt fundraiser and raised \$350. Thank you for your continued support of Cure SMA!



## Ackerman Elementary School Fundraiser

Nebraska

Thank you to Ackerman Elementary School in Omaha, NE, for holding a week of fundraising in the month of March in honor of one of their students! Students donated a dollar each day to participate in the daily theme (pajama day, stuffed animal day, crazy day, hat day) ending in a "purple out," where everyone wore purple to school. They also made shirts, sent flyers home, and sent awareness videos and information to each staff member. Together they were able to raise \$1,566 for SMA research and family support programs!

## #LeviTough T-shirt Fundraiser

Thank you to the Thoene family in Ralston, NE, for raising \$550 through an online t-shirt fundraiser in the month of July, in honor of their son, Levi! Thank you for spreading awareness and fundraising for Cure SMA!

## Louisiana Chapter



## 2018 Evening of Hope Singer-Songwriter Night

On April 12, 2018, the Louisiana Chapter hosted the 3rd Annual 2018 Cure SMA Singer-Songwriter Night at Boudreaux's in Baton Rouge, LA. The evening featured cocktails, hors d'ourves, a silent auction, wine pull, dinner, and a live performance by professional singer-songwriter, Terri Hendrix with Lloyd Baines.

The evening was a huge success, raising **\$30,893** for SMA research and family support programs.

A special thank you to Krista Scurria for chairing the event, as well as the dedicated Chapter Leaders, committee members, generous sponsors, and amazing volunteers for making this day possible.

## 2018 Kiss Away SMA

Thank you to Lindsay Becton in West Monroe, LA, for raising \$765 in honor of her son, Steele Becton. Lindsay hosted her 3rd Annual Kiss Away SMA fundraiser in the month of August and donated a portion of the commission from the sales of her makeup business to Cure SMA in addition to raising awareness. Her dedication and support are greatly appreciated!

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## Michigan Chapter



## 2018 Michigan Walk-n-Roll

Thank you to everyone who participated in the 14th Annual Michigan Chapter Walk-n-Roll. Nearly 200 people gathered at Hawk Island Park in Lansing, MI, on May 5, 2018, to celebrate the strength of the SMA community and fundraise to support Cure SMA. Thanks to the support of the event's 14 teams, over **\$22,000** was raised!

A special shout out to Team Abbey House as the top fundraising team with a grand total of over \$10,000!

Thank you to the Dorer and Hunderman families for their help with this year's walk.

## 2018 Fifth Third River Bank Run

Thank you to Kim Crane who represented Team Cure SMA at the 2018 Fifth Third River Bank Run in Grand Rapids, MI on May 12, 2018! Kim's hard work in both training and fundraising paid off! She completed the 25K race and raised \$660 for Cure SMA.

## LuLaRoe Fundraiser in honor of Wade

Thank you to Steph VanLonkhuyzen of Michigan, for donating a portion of her LuLaRoe proceeds from the month of August to Cure SMA! Steph supports Cure SMA in honor of her friend's child, Wade. Thanks to the generous support of her customers \$108 was donated.

## Minnesota Chapter

## 2018 Lake Minnetonka Half Marathon & 2 Person Relay

Thank you to the 12 runners that represented Team Cure SMA in the 2018 Lake Minnetonka Half Marathon & 2 Person Relay on May 6, 2018, in Wayzata, MN. Together they raised \$6,200 for Cure SMA! Special thanks goes out to Matthew Bigelow for organizing Team Miles 4 Mary Kate and to Brian Burkart who personally raised \$1,750!



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## Chaska Middle School East Fundraiser

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Thank you to Chaska Middle School East in Chaska, MN for holding a student fundraiser in the month of March in honor of one of their students, raising \$295 for Cure SMA!

## Evie's Light T-shirt Fundraiser

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Thank you to Carissa Keister in Lakeland, MN, who held a t-shirt fundraiser in memory of her daughter, Evelyn. She raised \$140 for Cure SMA and the Minnesota Chapter Walk-n-Roll!

## Jack Attack

North Dakota

The Jack Attack on SMA: Round 13 was held on June 9, 2018, at Lindenwood Park in Fargo, ND. This year, eight teams and over 110 participants joined us on a beautiful day to support the event and raise funds for Cure SMA. Together they raised **\$14,131** for SMA research and family support programs.

A big thank you to Kristi Gellner for her hard work and dedication, organizing this event every year!



## Shop for a Cause

South Dakota

In honor of SMA Awareness Month and her birthday, Kayley Shade organized a Shop for a Cause event at Laurie Belle's Boutique in Tea, SD, on August 13 and 17, 2018. The event included a private shopping party for Kayley's friends and family, and a public shopping day to spread SMA awareness and raise funds for Cure SMA!

Thank you, Kayley, for organizing this event and helping raise \$1,200!

## Team Xavier T-shirt Fundraiser

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Thank you to the Menke family in Sioux Falls, SD, for raising \$650 through a Team Xavier t-shirt fundraiser in honor of their son, Xavier.

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## New England Chapter

Maine



## SMA Awareness Day with the Portland Sea Dogs

The families in Maine had a great day celebrating SMA Awareness Month at Hadlock Field in Portland, ME on August 5, 2018! Over 20 people turned out for the game. It was great to have both long-time families as well as new ones gather to raise SMA awareness at the ballpark. The group had fun playing catch and taking a photo on the field!

Thank you to everyone who made this 5th annual event a success!

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## 18th Annual New England Chapter Walk-n-Roll

Massachusetts

The New England Chapter held their 18th Annual Cure SMA Walk-n-Roll on May 12, 2018, at DCR Wompatuck State Park in Hingham, MA. Over 360 people and 27 teams came together to raise funds for Cure SMA and to celebrate the strength of the SMA community! This year the New England Walk-n-Roll raised over **\$106,000!**

This event is not only a fundraiser for Cure SMA, but also an annual reunion of SMA families across New England. A big thank you goes out to the three teams that raised over \$10,000: Team Murphy, Walking for William, and Kelsey's Krew. Special thanks to the three additional teams who raised over \$5,000: Hammerin Hank the Tank, Team Norton, and the Wizards of Oz 2018! Other top teams included Mac's Pack, Chloe's Club, Mirabile Mania, Team Yale, Team Angelica, Team Melanie Lee, Glen's Gang, Team Evan, Mikaela's Marchers, Team Addison Barrett, Ella's Avengers, Team H4B Boston, Team Rogo Cup, and Team BraeKer.

Thank you to all the teams who fundraised, this event would not be a success without you!

Although the weather didn't cooperate this year, everyone still had an amazing time on the walk route and enjoyed refreshments and the great raffle. Children enjoyed face-painting and carnival games created by Declan Murphy. The event is made possible by the many volunteers who help run the registration, refreshments, raffle, and game areas, and the local businesses and friends who donate most of the supplies, refreshments, and raffle items. Special thanks to Silvia Murphy for all her hard work in making this event a success in its 18th year!

We're looking forward to seeing everyone at next year's walk in May 2019!

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## New Balance Falmouth Road Race

For the 4th year in a row, Team Cure SMA joined thousands of runners in the famous New Balance Falmouth Road Race on August 19, 2018, in Woods Hole, MA. The team ran along the hilly, scenic seven-mile course in bright purple shirts, proudly raising awareness for Cure SMA. Thanks to our team's generous donors, our 19 runners raised over **\$53,000**, the most Team Cure SMA has raised yet for the Falmouth Road Race!

Special thanks go out to Lin Lavalle for all her help organizing the team and to Rachael Brown, our top runner who personally raised over \$19,000!

## 6th Annual Rogo Cup to Cure SMA Golf Tournament

The 6th Annual Rogo Cup to Cure SMA Golf Tournament was held on June 4, 2018, at the Atlantic Country Club in Plymouth, MA. The Rogowicz, Butler, Spiegel, and Scully families hold the tournament in memory of Mark Butler and in honor of Billy Spiegel and Lauren Rogowicz.



Mother nature did not cooperate this year, giving us a cold, rainy, and windy day. However, that did not deter the wonderful golfers and volunteers; there was still a great turnout! Thank you to all the golfers for coming out in the challenging weather and keeping their smiles and great attitudes all day! Cure SMA is so thankful to all of the sponsors, donors, golfers, and volunteers for their continued support of Cure SMA and all those affected by SMA. With their support, we raised over **\$44,000** this year, bringing the six-year total to over \$240,000 for Cure SMA.

A special thank you goes to Joe Scully, for his tireless efforts to organize this golf tournament and raise funds for Cure SMA!

Save the date for the 7th Annual Rogo Cup to Cure SMA Golf Tournament on Monday, June 3, 2019!



## 4th Annual Nico's Night Out Gala of Hope

On April 28, 2018, the 4th Annual Nico's Night Out Gala of Hope was held at the Double Tree in Bedford, MA. Over 200 people attended the gala and enjoyed a delicious dinner while listening to a Frank Sinatra impersonator, a Nico's Night Out tradition! Guests also enjoyed dancing, raffles, a silent auction, and new this year, a casino night. Through the incredible generosity of guests, Nico's Night Out raised over **\$29,500** for Cure SMA!

A special thanks to the event chair Susie Sapienza, who works tirelessly to make this event a success year after year. Also, thank you to all the sponsors, attendees, and volunteers who helped make this day possible!

## 2018 McKeon Golf Tournament

The McKeon family hosted their 19th and final Connor James McKeon Memorial Golf Tournament on July 21, 2018, at Pine Ridge Country Club in North Oxford, MA. Connor passed away in 2000 and his family has hosted the golf tournament in his memory for the past 18 years. Family, friends, and coworkers have supported them and each year they donate the proceeds to Cure SMA. This year over 125 golfers were in attendance; the most they have ever had! Since 2000, the Connor James McKeon Memorial Golf Tournament has raised over **\$63,000**! Thank you to the McKeon and Dumas families for all that they have done for Cure SMA over the last 19 years!

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## 5th Annual Wakefield 5K Run/Walk

On August 25, 2018, over 170 participants came out in support of the 5th Annual Wakefield 5K Run/Walk 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. Participants received an awesome blue or green race t-shirt and were automatically entered for some awesome raffle prizes, including a variety of gift certificates, prizes and baskets! The course was chip timed and everyone enjoyed music and snacks after the race. Over **\$16,000** was raised for Cure SMA. Thank you to all who ran, walked, rolled, strolled, supported, volunteered, and donated! A huge thank you goes out to Kristen Farrell, the Wakefield 5K Race Director, for all her hard work over the last six months to make this event such a success again this year.

See you next year for the 6th Annual Wakefield 5K in August of 2019!

## SMA Awareness Day with the Boston Red Sox

Families from all over New England came out to raise SMA awareness at Fenway Park in Boston, MA on August 20, 2018, as the Boston Red Sox took on the Cleveland Indians! Over 70 people were in attendance to cheer on the Red Sox and help spread awareness at the ballpark during SMA Awareness Month. The group was decked out in their Cure SMA gear from head to toe and had a great evening of baseball!



See you at next year's SMA Awareness Day with the Red Sox in 2019!



## SMA Awareness Day with the New Hampshire Fisher Cats

New Hampshire

The families in New Hampshire had a great day at Delta Dental Stadium in Manchester, NH on August 26, 2018 celebrating SMA Awareness Month! More than 15 friends and families turned out for the game. It was great to have both long-time families as well as new ones gather to raise SMA awareness at the ballpark. The Cure SMA group was able to get a pre-game photo on the field and one of the group members got to throw out the first pitch!

Thank you to everyone who made the annual event a success! Join the Cure SMA group for another Fisher Cats Awareness Game in August of 2019!

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## 2018 Vermont Walk-n-Roll

Vermont

The Vermont Walk-n-Roll took place in Burlington, VT, on May 12, 2018, and was a success yet again this year! This year the event had a DJ and face painting alongside the walk-n-roll, and had the usual tradition of raffles, food, fun, and camaraderie. It was great to see many familiar faces and meet new families and friends from across Vermont with the same goal: supporting Cure SMA and contributing to finding a cure for SMA. This year over **\$11,000** was raised, the event's biggest year yet! Special thanks to Sue O'Neill and Margot Vanscoy for all their hard work in making this event such a success!

The New England Chapter hopes to continue to grow the event by raising the fundraising goal and welcoming even more participants next year. See you next May for the 5th Annual Vermont Walk-n-Roll!

## North Jersey Chapter

### 2018 North Jersey Walk-n-Roll

The Cure SMA North Jersey Chapter hosted the 3rd Annual Walk-n-Roll on June 23, 2018, at Roosevelt Park in Edison, NJ. Thank you to all the dedicated teams and individual participants who helped make this day a success and helped raise **\$18,790**. This fun-filled event included activities for the whole family to enjoy, such as a balloon artist, face painting, games, and more!



A special thank you to Anna Pham, Kristen Smith, Kara Hartnett, Marianne Middlebrook and Dawn Cusack for all their hard work in planning this fun event!

## 2018 New Jersey Marathon

On April 28 and 29, 2018, 13 runners gathered in Oceanport, NJ for the 2018 Novo Nordisk New Jersey Marathon and Half Marathon and ran with Team Cure SMA. Team Cure SMA members raised \$6,457! Runners participated in the 5K, Marathon and Half Marathon. Thank you to all 13 runners for raising funds and helping to spread awareness!

## 2018 Cure SMA Walk-n-Roll in Honor of Katherine Santiago

On May 19, 2018, the Walk-n-Roll in Honor of Katherine Santiago took place in Millburn, NJ, raising \$5,106. The walk was initially for Katherine – a sister, best friend, daughter, and a passionate Harry Potter fan. However, over the last 11 years the walk has grown and is now for the countless SMA families, uniting together to get closer to a cure.

Thank you to Julia, Alma and Efrén Santiago and Allie Mazzella for holding this annual event.

## 2018 Cure SMA Walk-n-Roll in Honor of Matthew Munch and Zianna Shamsi

On May 12, 2018, the students of Shongum School and their families gathered at Shongum in Randolph, NJ to walk or roll in honor of their fellow classmates, Matthew Munch and Zianna Shamsi. Over 200 participants gathered in the school gym and raised \$4,675 in support of Cure SMA. This wonderful event was organized and led by Matthew's teacher, Mrs. Susan Miessler. Thank you, Susan, for spearheading this event!

## John's Day to Dance Away SMA

On April 28, 2018, individuals gathered at the Far Hills Country Day School in Far Hills, NJ for a dance performance by In Motion and In Motion II Dance Companies to help find a cure for spinal muscular atrophy. The event raised \$4,453 to help fund Cure SMA's research efforts and program services. Thank you to Kathie Kecci for spearheading this event year after year!

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## 2018 Best Meatball in Secaucus Competition

The 7th Annual Best Meatball Contest in Secaucus event was held on April 14, 2018, at the local Immaculate Conception Church Gym in Secaucus, NJ. This annual event is hosted by the local Knights of Columbus and is held in honor of Daniel Cevallos. Cure SMA is thankful for the \$3,120 raised for SMA research and support programs. Thank you to Giovanni Recalde and Paulina Recalde for organizing this event!

### OKI Chapter



## 2018 Cure SMA Walk-n-Roll for Graham

Indiana

The 2018 Walk-n-Roll for Graham took place on May 11, 2018, at Coxhall Gardens in Carmel, IN. This year's event exceeded all expectations by raising over **\$90,000** (and counting!) for Cure SMA and rallying together over 800 people.

Cure SMA was honored to have Governor Holcomb attend the Walk-n-Roll. At the event he signed "Graham's Bill" into law, which put permanent newborn screening for SMA in place in Indiana.

This event has something for everyone, including a beautiful walk through the trails of Coxhall Gardens, raffle items, a tattoo station, a kid's zone with endless games and prizes, a DJ, tour of a local fire truck, a balloon artist, and so much more!

This event would not be possible without all the hard work and dedication of the amazing planning committee: Melody Braun, Ashley Goldman, Lisa Hiers, Lindsay Holt, Jen Juon, Leslieanne Maggart, Caity Sams, Allison Starr, Erin Watts, and Kristen Weaver.

A special thank you to the event chairs, Adrienne and Nick Vollmer. Adrienne and Nick founded this event in honor of their son, Graham, who is affected by SMA. This family works tirelessly to support Cure SMA and the entire SMA community each day.

Many groups hosted fundraisers to support this event. Cure SMA extends a thank you to Shelby Shields and CycleBar in Carmel, IN for hosting a Cycle for a Cause fundraiser to support the Walk-n-Roll for Graham. Thank you to Wheatfield Elementary and Cathy Vollmer for hosting Silly Spray for SMA to support the event!



## 2018 Cure SMA Awareness BBQ & Candle Lighting

On August 25, 2018, the North Jersey Chapter held their 2nd Annual Cure SMA Awareness BBQ & Candle Lighting at Merrill Park in Colonia, NJ. Families from around the area came out and were able to connect, enjoy a delicious BBQ, games, and honor their loved ones at a dessert candle lighting ceremony. The event helped spread awareness in the North Jersey area and raised over \$900 for critical research and program services for families.

A special thank you to Kristen Smith and her committee for organizing this family fun event!

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## NFL Mascots vs Pee Wees Game

After attending the 2018 Walk-n-Roll for Graham, the mascot for the Indianapolis Colts NFL team was inspired by Graham Vollmer's story. This inspiration led to an exciting partnership with the Colts. Cure SMA was selected as the beneficiary for the 2018 NFL Mascots vs Pee Wees Game held in Carmel, IN on July 12, 2018.

The evening included appearances with the Colts' mascot, Blue, and his NFL mascot friends, followed by a friendly game between the mascots and local pee wee football teams.

Proceeds from ticket sales were donated to Cure SMA which totaled \$5,000!

Thank you to Graham Vollmer and his parents, Adrienne and Nick, for inspiring this partnership.



## 2018 Marion Walk-n-Roll

Thank you to Stacie Fansler and Brian and Jessica Griffin for hosting the 2018 Marion Walk-n-Roll. The event was held on July 28, 2018, at Matter Park in Marion, IN, and raised nearly \$2,300 for Cure SMA!

Everyone enjoyed the beautiful walk around Matter Park and a fun face painter!

This event was founded by the Griffin family in memory of their son, Aiden, who passed away from SMA in 2015.

## Rock the Bay

Kentucky

Rock the Bay was held on August 18, 2018, at the Kentucky Lake Subdivision, in Paducah, KY. Everyone had a great time enjoying food, drinks and live music from the band MurMur. Attendees supported the event by purchasing Rock the Bay t-shirts, burgers, hotdogs, ice cream, bubbles, and tattoos. Guests also bid on great silent auction items and entered the 50/50 raffle drawing! Over 300 people attended all in amazing support for their friends and family as well as the SMA community. This inaugural event raised a total of **\$20,860** for Cure SMA!

A huge thank you to Alex Durbin and Aaron Haas and their family and friends for hosting this wonderful event and for their continued passion and support for Cure SMA.



## 4 Annie Girl Fundraisers

During the month of August 2018, the Grimes family raised over \$3,000 by selling Annie Girl t-shirts on their website, in honor of their daughter, Annie Grimes, in Bowling Green, KY. The Grimes family partnered with employees at the Graves Gilbert Clinic and Shelby Veterinary Hospital to host Annie Girl t-shirt Fridays during the month of August. Annie Girl Cookbooks, originally created by Annie's Nana, Sheila Violette, in 2011, were also sold to raise funds. All cookbooks were completely sold out!

A huge thank you to Nicole Grimes and her family and friends for holding this fundraiser every year during SMA Awareness Month and for their continued support of Cure SMA in honor of Annie.

## Deacon Alexander Memorial Car Show

In its 6th year, the Deacon Alexander Car Show was held at the First Baptist Church of Walton, KY, on August 18, 2018. This year there was a great turn out, and \$285 (and counting!) was raised for Cure SMA! Thank you to Amanda Perry, her family, church family, and friends for making this happen every year.

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## Kentucky Derby Festival Marathon

The Kentucky Derby Festival Marathon took place on April 28, 2018, in Louisville, KY. The dedicated runners raised a total of \$1,005 for Cure SMA! A huge thank you to Chandra Landers and Natalie Marshall for running for Team Cure SMA!



## Birdies for Blake Golf Tournament

The 2018 Birdies for Blake Golf Tournament took place on July 28, 2018 at Green Crest Golf Club in Liberty Township, OH. This year was the biggest year yet, raising a total of **\$38,480** for Cure SMA! It was a beautiful day that included a scramble golf tournament, a closest to the pin competition, a raffle, and a chance to win Blake and Fiona's wheelchair art! A huge thank you to Mark and Nancy Farrell, Nick and Kacey Farrell, and Kate and Sam Queen for planning this wonderful event each year, and for their continued support and dedication to finding a Cure for SMA.



## Cleveland Walk-n-Roll Ohio

The first ever Cleveland Walk-n-Roll was a huge success! The event took place on May 19, 2018, at beautiful Lakeview Park in Lorain, OH. The event raised an impressive total of **\$22,740** for Cure SMA! It was a beautiful morning, just missing the rain, filled with family fun including a glitter tattoo artist, snacks, drinks and an extra special visit from the Superheroes to Kids in Ohio! A huge thank you to Laura Shepherd, Michelle Peppers, Vera Matic and their families for helping to plan and execute this event. Thank you to Denise Zarella for emceeing and thank you to all the dedicated volunteers!

## Will's Day at the Bay

Jet Express hosted Will's Day at the Bay in memory of Will Blumensaadt. The event was held on June 6, 2018, at Derivera Park in Put-In-Bay, OH, and featured round-trip Jet Express tickets and raised \$4,150 for Cure SMA! It was a wonderful day of family fun while raising money for SMA! Will's father, Todd Blumensaadt said; "This is really a special event for me and the whole Jet Crew. By supporting research on this disease, hopefully we can make a difference for children and families suffering from spinal muscular atrophy."

A special thank you to Todd and the entire Jet Express Crew for their efforts and continued support!



## 2018 Cincinnati Walk-n-Roll

The Annual Cincinnati Walk-n-Roll was held on the morning of June 9, 2018, at the Xavier University Cintas Center, in Cincinnati, OH. In its 15th year the event raised an amazing **\$109,471** for Cure SMA! This event is always filled with fun family activities, lots of fundraising, and most importantly, hope. This year the kids were able to enjoy a petting zoo, face painting, and lots of games! It was a beautiful morning filled with friendship, passion, and hope.

A huge thank you to Beth and Kevin Lockwood, Bob and Elizabeth Lockwood, and Nicole, Mark and Amy Haake! Every year they spend countless hours soliciting sponsors, supporting families, and organizing logistics to make the walk-n-roll a success.

A special thank you to Ron and Janet Merkle, Marilyn Pater, Gloria Logeman, and Linda Schwabb for their important roles in this event.

Thank you to Stephanie Noll for organizing a special section on our walk route in memory of those we have lost to SMA. Thank you to our raffle and food committees for all their help! All the teams, families, sponsors, and volunteers who helped make this event a success are greatly appreciated!



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## Erin Vleugels Fundraising Campaign

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Thank you to Erin Vleugels for supporting Cure SMA through her personal fundraising page! Through many generous donations from family and friends, she raised \$1,274 in memory of her daughter, Mary.

## Cure SMA Day with the Cleveland Indians

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On August 7, 2018, local SMA families in the Cleveland area attended an awareness game at Progressive Field in Cleveland, OH. Twenty-eight people gathered to watch the Cleveland Indians take on the Minnesota Twins, all while spending time with other families and spreading awareness by dressing in Cure SMA colors and gear. Thank you to the Cleveland Indians for their support as well as everyone who attended!

## Buffalo Wild Wings Dine to Donate

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On April 11, 2018, the Avon Lake, OH Buffalo Wild Wings held a dine to donate day to help raise funds for the 2018 Cleveland Walk-n-Roll. Thank you to Buffalo Wild Wings and everyone who participated for helping us raise \$275!

## Mellow Mushroom Dine to Donate

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On February 28, 2018, Mellow Mushroom in Rocky River, OH held a dine to donate night to help raise funds for the 2018 Cleveland Walk-n-Roll! Thank you to Mellow Mushroom and everyone who attended that evening for helping raise \$212!

## Doug Dietz Fundraising Campaign

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Thank you to Doug Dietz for supporting Cure SMA through his personal fundraising page! He raised \$107 in honor of his friend Sara, and in memory of her son, Fletcher.

## Pacific Northwest Chapter



## Pacific Northwest Walk-n-Roll

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Washington

On August 19, 2018, the 11th Annual Pacific Northwest Walk-n-Roll took place at Game Farm Park in Auburn, WA. It was a perfect day for a walk-n-roll, bringing together 230 participants for a morning and afternoon of family fun and awareness. Thank you to all the participants, teams, donors, and volunteers for making this year's event so successful and raising over **\$18,000**.

Thank you to this year's new organizer, Cynthia Turner, as well as to Kelly and Russ Hargrave, Jodi Pretz, Jennifer Jefferies, and Rozie McClay for helping organize the walk and secure donations.

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## Pennsylvania Chapter



## 2018 Pennsylvania Chapter Walk-n-Roll

The 15th Annual Pennsylvania Chapter Walk-n-Roll brought together more than 600 participants to the walk-n-roll to support Cure SMA. The event raised over **\$109,000!**

The cloudy skies cleared, and participants had a beautiful morning at Shoemaker Green on the University of Pennsylvania campus in Philadelphia, PA on May 20, 2018.

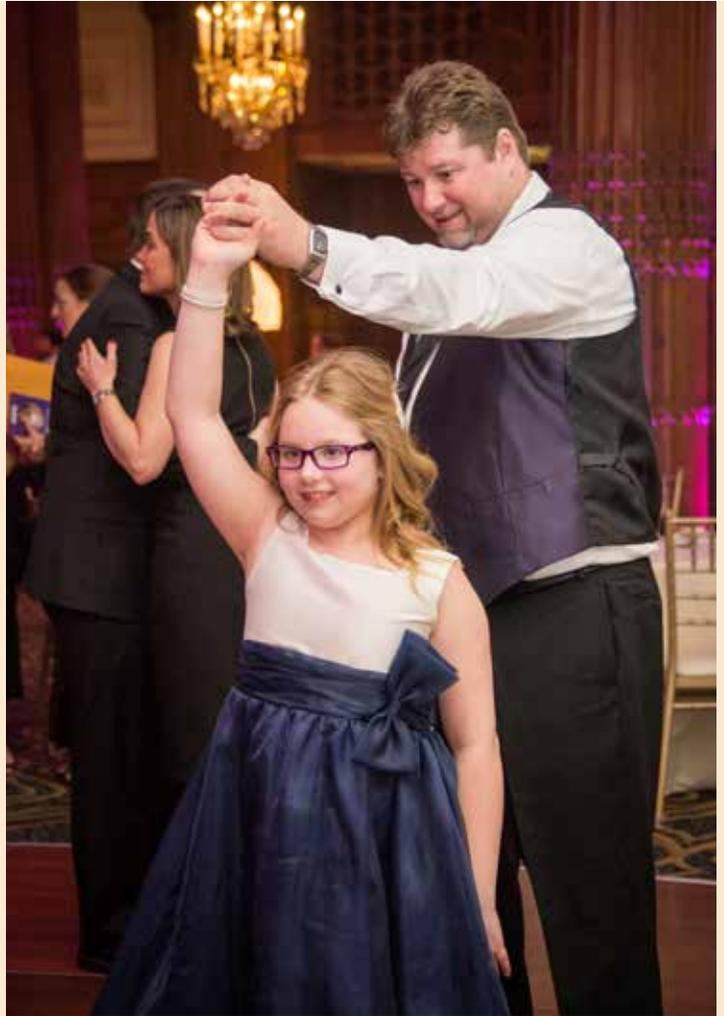
This year's Youth Citizenship Award was presented to Ian, Chris, and Lucy Henkel for their years of hard work running the games at the walk-n-roll. Man's Alternative was awarded the Corporate Partnership Award for their 15 years of consecutive sponsorship and dedication to the event.

Twenty-five teams recruited participants and raised funds to help make the event a success. Congratulations to the event's top five fundraising teams: Pete's Philadelphia Eagles, Team Hammerin' Hank, Team Saxton, Team William, and Team Madison.

Thank you to the committee members who planned every detail of this event, recruited sponsors, and inspired the local community to support Cure SMA: Gina Cannady, Allyson Henkel, Karen McRory Negrin, Tara Maida, Christina Murray, Stephanie Reese, and Paula Saxton.



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## 2018 Muscles for Mckenna Gala

The 2018 Muscles for Mckenna raised an event record of **\$120,000!** Three hundred guests gathered at the Crystal Tea Room in Philadelphia, PA on March 9, 2018 for the sixth annual event to raise funds for Cure SMA.

Guests enjoyed an evening full of fun with live music by The Heartbeats, a spirited live auction, selfie station, and great food! Guests generously raised their paddles during the Fund-a-Need portion of the evening after heartfelt presentations by the Ellixson family and Jimmy Curran about life with SMA and the importance of Cure SMA.

A special thank you to event chairs Jim and Amy Ellixson, as well as the entire committee, who made this year's event such a success: Bill & Maureen Bell, Dave & Kim Carlin, Jane & Tom Pedicone, Dennis & Melanie Ellixson, Kellie & Mike Keenan, Coleen McLaughlin, Nancy & Steve Mckenzie, Jane & Tom Pedicone, Scott & Jocelyn Setzman, and Emily & Dan Tomlinson.



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## 2018 Dick's Sporting Goods Pittsburgh Marathon

Team Cure SMA runners, Carol Tomko and Katie McMurty raised more than \$2,700 through the Dick's Sporting Goods Pittsburgh Marathon in May 2018! The pair ran in honor of Heather and Jessica Tomko of Pittsburgh, PA.



## Oxford Pharmagenesis Bake Sale

Thank you to all the employees at the Oxford Pharmagenesis office in Newhaven, PA, for raising funds for Cure SMA! During SMA Awareness Month in August 2018, the office held a bake sale and raised \$700. Thank you for selecting Cure SMA as the charity of choice!

## Rocky Mountain Chapter



## 2018 Colorado Walk-n-Roll

Colorado

On August 5, 2018, the Rocky Mountain community gathered in Littleton, CO, at Clement Park for the Annual Colorado Walk-n-Roll. This year's walk raised over **\$45,000** to help in the fight against SMA and hosted over 300 participants! Thank you to the top teams: Team Levi, Team Jack Colorado, and Hope for Hannah! Thank you also to all the sponsors: Biogen, AveXis, Bayada, Southwest Concrete Pumping, Industrial Chemicals Corporation, The Cash Scanlon Philips Foundation, Rusinak Real Estate, Freedom Mobility, and P & O Group Pediatric Specialists.

A special thank you to Michelle Pritekel, Sherri Casas, and Julie Lino for their roles in organizing and planning this yearly event.

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## 2018 Colorado Evening of Hope

The 5th Annual Evening of Hope was another great success for the Rocky Mountain Chapter. Instead of a beer and cheese pairing, a BBQ buffet and pop culture trivia night were hosted by Chain Reaction Brewery and Brain Reaction Trivia on March 15, 2018, in Denver, CO. Thank you to all the friends and families of the Rocky Mountain SMA community for their continued support.

The evening raised over \$8,700! The sponsors for the evening included the Cash Scanlon-Philips Foundation, Chain Reaction Brewery, and Mulay's Sausage. Cure SMA sincerely appreciates all the donations – in-kind, services and more – that were given to the event.

Thank you to Joy Spellman and Marla Marlow for organizing this annual event.



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## 2018 Rocky Mountain Golf Tournament

The 15th Annual Rocky Mountain Charity Golf Tournament took place on May 21, 2018, at the beautiful Club at Pradera in Parker, CO. With the support of this year's sponsors, donors, and players, the event raised **\$37,410** to help find a cure for SMA!

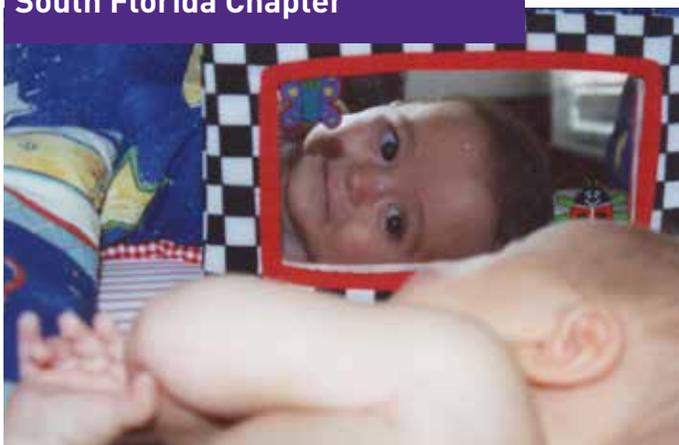
The Rocky Mountain Golf Tournament was started 15 years ago in honor of the organizer, Gillian Faith's nephew, Daniel McCulloch. Over the last 15 years, Gillian and her fundraising efforts have raised over \$340,000 and have helped contribute to Cure SMA making tremendous strides toward research, treatments, and program services.

Cure SMA would like to extend a special thank you to event organizer, Gillian Faith and her family for hosting the tournament.



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## South Florida Chapter



### Shooting Star Campaign to Cure SMA

Thank you to Adi and Shaina Rappoport and The Jacob Isaac Rappoport Foundation (JIRF) for raising **\$47,553** through the Shooting Star Campaign to Cure SMA! For the past 15 years, The JIRF has hosted Jacob's Run, Walk-n-Roll.

"Our greatest wish to be able to watch our son grow up in our home cannot come true. However, every year we meet children and their families who desperately cling to that wish," said Shaina Rappoport. "At Jacob's funeral, we promised him that we would honor his memory by dedicating ourselves to finding a cure for the disease that took his life, and to helping families whose lives have been shattered by this disease. For the past 15 years, the Foundation has had a significant impact on the progress toward a treatment and a cure and has made a difference in the quality of life of thousands of SMA babies."

This year the Rappoport family launched this campaign to celebrate Jacob's memory and raise funds for treasured Cure SMA support programs. The generous donations made to this campaign for Cure SMA will fund Type I Care Packages and programs at the Annual SMA Conference including the Children's Program, Type I Reception, and Teen Social.

Cure SMA is so grateful for the continued support of the Rappoport family and the JIRF. Thank you for your dedication to Cure SMA and the SMA community!



### Bayview Elementary Community Compassion Day for SMA

On March 6, 2018, and August 31, 2018, Bayview Elementary in Fort Lauderdale, FL celebrated Compassion Day in honor of Mia Fuentes-Israel. Students supported Cure SMA by donating \$1 to wear purple to school on those days. Donations to Cure SMA totaled \$800. Thank you to Mia and Fiorenna Fuentes-Israel, as well as Bayview Elementary, for supporting Cure SMA!



### SMA Awareness Month's Mom's Night Out

On August 18, 2018, the South Florida Chapter hosted a Mom's Night Out at the Modern Sixties Wine Lounge in Fort Lauderdale, FL. Moms enjoyed fun drink specials and wine raffles. There was a special SMA Awareness Month Raffle for a Michele Watch that kicked off at this event, raising \$650. Special thanks to Fiorenna Fuentes-Israel and Jennifer Miller-Smith for organizing this fun night out!

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## SMA Awareness Day at the Museum

For SMA Awareness Month, families were invited for an afternoon out at the Museum of Science and Discovery in Fort Lauderdale, FL, on August 11, 2018. More than 30 people enjoyed pizza, a hands-on animal presentation, and the museum exhibits. Thank you to Jennifer Miller-Smith and Fiorenna Fuentes-Israel for planning this great outing for the SMA families!



## SMA Awareness Month's Restaurant Partnership

For SMA Awareness Month, Cure SMA South Florida Chapter partnered with JT's Sports Bar in Pembroke Pines, FL to spread SMA awareness. JT's Sports Bar donated 15% of all proceeds on August 25, 2018, from 5 – 11 p.m. which totaled \$400!

The Michele Watch Raffle continued at this event, raising an additional \$150. Thank you to JT's Sports Bar for supporting Cure SMA and to Jennifer Miller-Smith for forming this partnership!



## Nova Southeastern University PED's Club Volleyball Fundraiser

On April 6, 2018, Nova Southeastern University's PED's Club, a health professions student association, raised \$200 for Cure SMA! Over 40 students participated in this event and they chose Cure SMA because of their desire to support organizations benefiting pediatrics. Thanks to PEDS Club and the Dr. Pallavi Patel College of Healthcare Sciences at Nova Southeastern University for their support of Cure SMA!

### South Jersey/Delaware Chapter

## 2018 Coastal Delaware Running Festival

Delaware

On April 22, 2018, two runners joined together at Dewey Beach in Delaware to run in support of Cure SMA at the Coastal Delaware Running Festival. Members from Team Cure SMA ran in the half and full marathons and together raised \$1,180!

Thank you to the runners, donors and the Coastal Delaware Running Festival for having Team Cure SMA!

*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*

## Texas Chapter



## 2018 Fayetteville 5K & Walk-n-Roll in Honor of Annabelle

The 3rd Annual Fayetteville 5K in Honor of Annabelle and Jude Combs was held on April 21, 2018, at Oak Thicket Park in Fayetteville, TX. With over 125 participants, the event raised over **\$13,000** for Cure SMA! The 5K race celebrated Annabelle who turned three, and new baby brother, Jude! Thank you to Alyssa Combs for another great event and the several sponsors for supporting the race!



## 2018 SMASH Dash

The 3rd Annual SMASH Dash was on August 11, 2018, in Fair Oaks Ranch, TX. Two hundred and forty-seven participants either ran a timed or untimed 5K and raised **\$10,000** for Cure SMA. Thank you to the Hastings family for planning this great event in honor of Kadence!



## 2018 Texas Bowl-a-Thon

The Texas Chapter held the 8th Annual Bowl-a-Thon at Mel's Lone Star Lanes in Georgetown, TX, on August 4, 2018. The event included bowling, a silent auction, face painting, balloon art, awards, and most importantly, visiting with other families. With continued support of friends, family, and the community, the bowl-a-thon raised over **\$7,300**. Special thanks to event organizer Kelly Coggin, Davis family, and Ostermayer family for all their help and support. Cure SMA looks forward to seeing everyone again for next year's event!



## 2018 Houston Walk-n-Roll

The Houston Walk-n-Roll was held on April 28, 2018, at Traylor Stadium in Rosenberg, TX. In the second year, over 75 participants attended and raised over **\$7,000** for Cure SMA! The event featured a walk around the track at Traylor Stadium, snacks, yard games and awards presented to the top three fundraising teams. Congratulations to Team Cooper, Team Evan and Maya's Miles!

Thank you to all the participants and teams that came out on a sunny and warm Texas day: Heavenly Angels, KIKO, Maya's Mile, Team Braemar, Team Cooper, Team Evan, Team Kayleann, and Team Nadia!

A special thank you to Tracie Tanner and her family for their hard work and dedication to make this event a success!

*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*

## Virginia Chapter



## Ukrop's Monument Avenue 10K – Helping Andrew Cure SMA

On April 14, 2018, Team “Fighter Family & Friends of Andrew” participated in the Ukrop's Monument Avenue 10K in Richmond, VA. Thank you to Carice Smith for organizing this incredible fundraiser and Team Andrew for raising **\$12,175** to support the groundbreaking research taking place to help Andrew and all those fighting SMA.

A special thank you to each team member for running, jogging, and walking in honor of their amazing friend, Andrew Creighton, and his 25-year fight against SMA: Carice Smith; Caryn, Jenna, Lindsey and Kenley Martin; Josh Montagnino; Danis Goins; Mike, Nancy (Wright), and Erin Collins; Liz Glover; Amy Couillard; Penny Dupuis; Don and Gail Raikes; and Jason, Jessica, Alyssa and Nick Montagnino.

## 2018 DC Rock ‘n’ Roll Marathon Series

On March 10, 2018, Robert & Alicia Cooper ran in the 2018 Rock ‘n’ Roll DC Marathon Series in Washington, D.C. for the second year in a row. Most of the funds raised were through Alicia's business, Tina Lane Events, by donating 10% back of each client's contract. Thank you to the Coopers for raising \$1,574 in memory of their sons, Jonathan and James Cooper, and continuing the fight to cure SMA.



## SMA Awareness Day with the Nationals

Thank you to everyone who came out to watch the Washington Nationals take on the San Francisco Giants on June 10, 2018, in Washington, D.C. Despite the rainy day, over 40 people came out to support Cure SMA. This was the first Nationals game made possible through their community grant ticket program. Thank you to Kim Heinrich and Laura Derkowski for organizing this day for families and individuals affected by SMA and bringing together the Virginia and Chesapeake Chapters.

*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*

**Western New York Chapter**



## 15th Annual Western New York Chapter Walk-n-Roll

This year the Western New York Chapter celebrated its milestone 15th Annual Walk-n-Roll. It was once again held in Beaver Island State Park in Grand Island, NY, on Saturday, August 4, 2018. The walk had another year of beautiful, sunny weather along the Niagara River. Attendees enjoyed games, face painting, refreshments and the chance to connect with several SMA families. In addition to a hugely successful raffle organized by Paula Orlowski and Lori Faso! Once again, John Litton and his hot dog crew and DJ Nick Pickolas came out to support this milestone event. Both have attended and supported this walk-n-roll from the beginning.

This year's walk had 170 attendees and raised **\$35,000** and counting to help find a cure for SMA. A special thank you to the top sponsors Drive Against Diabetes, Counsel Financial, Erie and Niagara Insurance, Jim Harszlak Foundation, Orchard Park Pediatrics, National Exchange Club, and JL Automotive. Lastly, Cure SMA deeply expresses gratitude to the walk committee for their continued efforts in supporting the Western New York Chapter and families: Karen Shiesley, Mary and Paul Boguhn, Ron and Lori Faso, Joanne Hallmark, Joanne Kwarcianny, Diane Blair, Paula Orlowski, and Heidi Samson.

Much thanks and credit to all who participated and helped. Save the date for next year's walk-n-roll on August 3, 2019.

*Note: The amounts raised and shown are totals as of August 15, 2018 and may differ from current fundraising totals by the time you get this newsletter.*

## Kinsley's Day for SMA

On August 11, 2018, Cheryl Weidman hosted the 2nd Annual Kinsley's Day for SMA in Columbus, NY. It was a fun filled day with games, a raffle, food, and a bonfire. Many stayed to participate in the candle lighting ceremony for SMA Awareness Month. Cure SMA would like to thank Cheryl Weidman for organizing this event in memory of her daughter, Kinsley Weidman, and raising \$1,254 to support Cure SMA.

## Pig Roast

The 2018 Pig Roast for Western New York Cure SMA was held on June 9, 2018 in Lockport, NY. It was hosted by Uncle Brad Shiesley and took place in his backyard. There was fishing, canoe rides, small farm animals to pet, and lots of food. Families and friends of Cure SMA brought snacks, salads, and desserts to go along with the roasted pork. It was a great afternoon, enjoyed by all. Molly Terry won the raffle for a granted wish and chose to go to Jellystone Water Park! Donations were made by those who attended, and a portion of the proceeds were donated to Cure SMA.



### Wisconsin Chapter

## Iceman Darts Tournament

The Icemen Darts Tournament held between July 27 and 29, 2018, in Waukesha, WI was a huge success raising over \$8,000 for Cure SMA! Participants enjoyed a fun weekend of fierce dart tournaments while raising funds.

Cure SMA extends a heartfelt thank you to Russ Nelson for his hard work and dedication making this event a success and for choosing to support Cure SMA. A special thank you to Tim McCarthy for nominating Cure SMA to be a beneficiary of this event in honor of his son, Braden.

## 2018 Wisconsin Chapter Bowl-a-Thon

The Wisconsin Chapter Bowl-a-Thon held on May 5, 2018 in New Berlin, WI, was a great success! The event had its biggest turnout to date with 114 bowlers and raised nearly \$6,000.

A special thank you to Kim Banach, Ashley Baumeister, Kate LaChapelle, and Amy Medina for all their hard work planning this year's event and making it such a success.



## 2018 Tori Stevens Memorial Night

Thank you to Ashley Hedges for once again planning the Tori Stevens Memorial Night to support Cure SMA! This year's event took place on August 11, 2018. Each year Ashley rallies the town of Tomahawk, WI together at the Tomahawk Speedway to remember her dear friend, Tori. Through the generosity of the local community, over \$900 was raised for Cure SMA!



## Cure SMA Day with the Milwaukee Brewers

This year's Cure SMA Day with the Milwaukee Brewers on August 26, 2018, had the largest turnout to date with 92 tickets sold! Cure SMA supporters gathered at Miller Park in Milwaukee, WI, and watched the Brewers take on the Pittsburgh Pirates.

Thank you to everyone who attended the game and helped spread SMA awareness. A special thank you to Kim Banach, Ashley Baumeister, Kate LaChapelle, and Amy Medina for helping make this day a success!



# cure SMA 2018

CURE SMA MERCHANDISE



[www.curesma.org/merchandise](http://www.curesma.org/merchandise)



**Cure SMA Adjustable**

**Baseball Hat | Cost: \$20**

This new Cure SMA hat comes in charcoal gray and has a low-profile unstructured fit. The front of the hat features the Cure SMA logo embroidered in the lower right corner and cureSMA.org embroidered on the back. The back of this baseball hat features an adjustable fabric strap with tri-glide buckle that allows you to customize your fit.



**Cure SMA Unisex Full**

**Zip Hoodie | Cost: \$49**

Style meets comfort with this popular new unisex full zip hooded sweatshirt! This trendy purple hoodie has a new Cure SMA circle design screen printed with white ink on the front left chest and enlarged on the back allowing you to spread SMA awareness everywhere you go! This design is printed on ringspun cotton and polyester which guarantees softness and comfort.



**Cure SMA Unisex and Youth**

**Baseball Tee | Cost: \$25**

Join the Cure SMA team with our new baseball tee! You and your little one will be ready for game day with this comfy athletic baseball tee available in heather gray featuring vintage purple 3/4 sleeves. This vintage jersey features a large number 50 on the back of the tee which represents the 1 in 50 people who carry the gene causing SMA. Available in both adult and youth sizes!



**Cure SMA Onesie | Cost: \$18**

Our Cure SMA Onesie lets our newest family members show their support for SMA! Made of 100% combed ring spun cotton for a natural softer feel. It's reinforced with three-snap closures to stay closed after hours of playtime.



**Cure SMA Vinyl Decal | Cost: \$5**

Make a statement and show your support by displaying the Cure SMA logo on any window or flat surface with this white vinyl decal. Made of vinyl and applied with transfer paper, this decal is easy to apply. There is no sticky background surface, just a 5"x3.7" white decal that you can apply anywhere. Easy to remove, this decal will not damage your paint like a sticker would.



**Cure SMA Popsocket | Cost: \$10**

The Cure SMA Popsocket can be used in a number of ways. It can be used as a collapsible grip which provides a secure way to hold your phone. It can also be used as a stand or to wrap up your headphone cords to store them neatly. The Cure SMA logo also allows you to spread awareness wherever you go!



**Cure SMA Unisex Orange**

**V-Neck Tee | Cost: \$24**

You won't want to take off this super soft and trendy new Cure SMA tee featuring a uniquely soft orange v-neck tri-blend fabrication and a modern fit for both men and women. The front of the shirt has our Cure SMA logo screen printed in bright white ink on the front left chest and the back features a unique new design highlighting our name "CURE" with SMA in small print repeating inside throughout the block lettering.



**Cure SMA Grey Crew**

**Neck Tee | Cost: \$24**

You won't want to take off this super soft and trendy new Cure SMA tee featuring a uniquely soft grey tri-blend fabrication available in both toddler, youth and adult sizes. The front of the shirt has our Cure SMA logo screen printed in bright white ink on the left chest and the back features a unique new design highlighting our name "CURE" with SMA in small print repeating inside throughout the block lettering.



**Cure SMA Fidget Spinner | Cost: \$6**

The Cure SMA Fidget Spinner is a fun new toy to add to your kid's collection! It is made of strong and durable plastic with steel ball bearings in the center for smooth spinning. Spinners can also be used to relieve stress, lower anxiety, eliminate boredom and increase focus. The Cure SMA logo also allows you to spread awareness wherever you go!



**Cure SMA Men's Performance**

**Half Zip | Cost: \$49**

Comfort and function describes our new Cure SMA performance half zip pullover! Made of moisture-wicking material, this performance shirt is designed to keep you dry and comfortable all day long! Reflective details on the right sleeve and lower back add to this unique style. Also available in both women's and men's sizes!



**Cure SMA Women's Performance**

**Half Zip | Cost: \$49**

Comfort and function describes our new Cure SMA performance half zip pullover! Made of moisture-wicking material, this performance shirt is designed to keep you dry and comfortable all day long! Reflective details on the right sleeve and lower back add to this unique style. Also available in both women's and men's sizes!



**Men's Performance Polo**

| Cost: \$28

Comfort and function describes our new Cure SMA Men's Performance polo. These polos are known for superior breathability and are made of moisture-wicking material, designed to keep you dry and comfortable. This white polo has our Cure SMA logo screen printed in purple ink on the front left chest.



**Women's Performance**

**Polo** | Cost: \$28

Comfort and function describes our new Cure SMA Women's Performance polo. These polos are known for superior breathability and are made of moisture-wicking material, designed to keep you dry and comfortable. This purple polo has our Cure SMA logo screen printed in bright white ink on the front left chest. This women's fit provides a feminine curve and princess seams which add shape and a more tailored fit.



**Men's Est. 1984 Purple Jersey Tee** | Cost: \$24

Join the Cure SMA team with our new football jersey style t-shirt! This trendy crew neck tee has white stripes on the sleeves and a cover stitched yoke to give a sporty look with exceptional comfort. This unique design comes in a vintage purple color and has three white stripes across the chest highlighting our name Cure SMA and the year we were established. Also available in women and youth sizes!



**Women's Est. 1984 Purple Jersey Tee** | Cost: \$24

Join the Cure SMA team with our new football jersey style t-shirt! This trendy V-neck tee has white stripes on the sleeves and a cover stitched yoke to give a sporty look with exceptional comfort. This unique design comes in a vintage purple color and has three white stripes across the chest highlighting our name Cure SMA and the year we were established. Also available in men's and youth sizes!



**Youth Est. 1984 Purple Jersey Tee** | Cost: \$24

Join the Cure SMA team with our new football jersey style t-shirt! This trendy crew neck tee has white stripes on the sleeves and a cover stitched yoke to give a sporty look with exceptional comfort. This unique design comes in a vintage purple color and has three white stripes across the chest highlighting our name Cure SMA and the year we were established. Also available in men's and women's sizes!



**Cure SMA Windbreaker Full Zip Jacket** | Cost: \$35

This lightweight packable windbreaker jacket has water resistant 100% polyester fabric with zip front closure. It's soft to the touch, versatile, and great for layering. This windbreaker packs easily and is great for all weather conditions. This charcoal and black lightweight jacket has our Cure SMA logo screen printed in bright white ink on the front left chest. It also features a scuba neck, mesh lined 3 panel hood, and welt pockets.

**cure**  
**SMA** **2018**  
CURE SMA MERCHANDISE

[www.curesma.org/merchandise](http://www.curesma.org/merchandise)

## VISION AND MISSION STATEMENT

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

## OUR VALUES

### Innovation

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

### Balance

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

### Collaboration

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

### Respect

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

### Compassion

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

### Determination

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

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

On the Cover: Baumeister family



**Make today a breakthrough.**

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

### Submissions

To submit articles or make other contributions to our newsletter, please contact us at: [newsletter@curesma.org](mailto:newsletter@curesma.org).

Digital images are encouraged!

Send your digital pictures to: [newsletter@curesma.org](mailto:newsletter@curesma.org).

### Change of address

Send changes, including ZIP code to: [info@curesma.org](mailto:info@curesma.org) or call 800.886.1762 or mail to: Cure SMA, 925 Busse Road, Elk Grove Village, IL 60007

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**MAKE AN IMPACT!**

**HELP CURE SMA TRANSFORM TOMORROW >>**

- Join us and add your voice to ours so that newborn screening for SMA is adopted nation-wide for earlier diagnosis of the disease.
- We must continue expanding our local programs, such as our SMA care packages and our new Summit of Strength program, so that we meet the shifting needs of the SMA community.
- It is imperative that everyone helps us invest in the SMA drug pipeline and provide more treatment options for all people affected by SMA.

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