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: Monique, Shiloh, Amanda and Amante Spiteri
Biogen announced on October 28, that its New Drug Application (NDA) for nusinersen, an investigational treatment for spinal muscular atrophy (SMA), has been accepted by the U.S. Food and Drug Administration (FDA) for Priority Review, and that the company’s Marketing Authorization Application (MAA) has been validated by the European Medicines Agency (EMA). Nusinersen had previously been granted Accelerated Assessment status by the EMA’s Committee for Medicinal Products for Human Use (CHMP). The regulatory review process for these applications has now been initiated in the U.S. and EU. Both the Priority Review and Accelerated Assessment designations can reduce the standard review time. If approved, nusinersen would be the first therapy for SMA, a leading genetic cause of infant mortality.

Starting in 2003, Cure SMA provided the seed funding needed to begin investigation into this therapeutic approach. The intellectual property generated with our funding was then licensed to Ionis Pharmaceuticals to create nusinersen. Biogen intends to market nusinersen under the brand name SPINRAZA. This name has been conditionally accepted by the FDA and the CHMP and will be confirmed upon approval.

“The FDA and EMA have acknowledged the potential for nusinersen to address the urgent need for an effective SMA treatment by granting special status to the applications, and FDA has shared that they plan to act early on our NDA under an expedited review,” said Michael Ehlers, M.D., PhD, executive vice president, head of Research and Development at Biogen. “We are now focused on working with the agencies to hopefully bring this investigational treatment to the SMA community as quickly as possible.”

The regulatory filing packages in the U.S. and EU are based on data that demonstrate the clinically meaningful efficacy and favorable safety profile of nusinersen from multiple studies. These include the results from the interim analysis of ENDEAR, the Phase 3 study evaluating nusinersen in infantile-onset (most likely to develop Type 1) SMA, as well as open-label data in other patient populations. The ENDEAR interim analysis demonstrated that infants receiving nusinersen experienced a statistically significant improvement in the achievement of motor milestones compared to those who did not receive nusinersen. Data from the other endpoints analyzed were also consistently in favor of the treated infants. Nusinersen was generally well-tolerated, with a favorable safety profile. No adverse events (AEs) were considered related to nusinersen.

**Leading the Way on Regulatory Issues**

When the FDA evaluates a drug for approval, they must weigh many different factors, including the quantity and quality of evidence of safety and effectiveness, potential benefits of a treatment versus the potential risks, and the impact the treatment will have on the patient community. While we wait for the NDA to be reviewed, we won’t sit back. We are actively working to continue our ongoing efforts to make sure that our community’s voice is heard during this entire process.

This includes our upcoming Patient-Focused Drug Development Meeting with the FDA, newborn screening initiatives, coverage and payment projects, and many more initiatives designed to make certain that regulators, payers, and industry partners understand the impact of SMA, and create solutions that will address the areas most important to us.

**Thank You**

This is a historic moment that many in our community have worked for tirelessly. We extend our deepest gratitude to all our families, supporters, donors, and partners who have contributed to this milestone.

Cure SMA 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 Dr. Adrian Krainer and his colleagues at CSHL for years of dedication to and hard work on the preclinical development of nusinersen for SMA, and Drs. Ravindra Singh and Elliot Androphy for their work funded by Cure SMA in originally identifying the ISSN1 gene sequence, which is the sequence targeted in nusinersen. In addition, Drs. Krainer and Androphy are long standing members of the Cure SMA Scientific Advisory Board.
On September 26, 2016, Biogen and Ionis Pharmaceuticals provided the following community statement regarding the completion of their NDA filing for nusinersen.

Dear members of the SMA community,

Today we have achieved a crucial step in the pathway to approval of nusinersen. We have completed the submission of our New Drug Application (NDA) to the Food and Drug Administration (FDA) and we will submit our application to the European Medicines Agency (EMA) in the coming weeks.

Once the regulatory agencies receive an application, a validation or review period begins, in which they will review the submission to ensure the application is complete and sufficient to proceed. We are grateful to the FDA for their close collaboration throughout our development program, and most recently, their willingness to work with us on a “rolling submission” of our application. This rolling submission provided us with the opportunity for increased dialogue with the FDA over the past two months and the ability to make the documents available to the FDA as soon as possible. Additionally, the EMA’s Committee for Medicinal Products for Human Use (CHMP) recently granted Accelerated Assessment to nusinersen, which can reduce the standard review time in the EU. We appreciate both regulatory agencies’ collaboration with us during this important time of application submission and review.

We are also incredibly thankful to the entire SMA community for your continued support. We still have additional milestones to complete before a potential approval and the final approved label, but we are getting closer. A product label will instruct physicians on the use of an approved treatment and the final product label is ultimately decided by each regulatory authority representing a country or region where approval is being sought. For each regulatory submission we are providing all of the data we have to date, which includes data from patients with pre-symptomatic, Type 1, Type 2 and Type 3, and we are seeking a broad label for the treatment of SMA. Again, if approved the final label is decided by the regulatory agencies and is based on their assessment of the data we provide to them. We know there will continue to be many questions such as the timelines of the approval process, the potential label if nusinersen is approved, and about our ongoing plans for expanded access. We remain committed to transparent and timely communications and will continue to provide updates as the program moves forward.

Sincerely,

Biogen and Ionis
More About Spinraza

SMA is caused by a mutation in the survival motor neuron gene 1 (SMN1). In a healthy person, this gene produces a protein—called survival motor neuron protein or SMN protein—that is critical to the function of the nerves that control our muscles. Without it, those nerve cells cannot properly function and eventually die, leading to debilitating and often fatal muscle weakness.

All patients with SMA have at least one copy of survival motor neuron gene 2 (SMN2), often referred to as the SMA “backup gene.” Due to a splicing error, most of the SMN protein made by SMN2 is missing an important piece, called exon 7.

Antisense drugs are small snippets of synthetic genetic material that bind to ribonucleic acid (RNA), so they can be used to fix splicing errors in genes such as SMN2. Spinraza is antisense oligonucleotide that targets SMN2, causing it to make more complete SMN protein.

Background on Spinraza

From 2003 to 2006, Cure SMA provided the seed funding needed to begin investigation into this therapeutic approach. 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 Dr. Adrian Krainer and his colleagues at CSHL for years of dedication to and hard work on the preclinical development of nusinersen for SMA, and Drs. Ravindra Singh and Elliot Androphy for their work funded by Cure SMA in originally identifying the ISSN1 gene sequence, which is the sequence targeted in spinraza.

Spinraza was first known as IONIS-SMNRx, then nusinersen.

After the preclinical work was complete, Ionis Pharmaceuticals partnered with Biogen to launch a number of clinical trials testing spinraza. Biogen is now responsible for all spinraza development, regulatory and commercialization activities and costs.

Clinical Trials

Three pivotal trials tested spinraza:

- **ENDEAR**, a placebo-controlled trial testing spinraza in infants with SMA type I
- **CHERISH**, a placebo-controlled trial testing spinraza in children with SMA type II
- **NURTURE**, an open-label trial testing spinraza on infants genetically diagnosed with SMA but not yet showing symptoms.

On August 12, 2016, Biogen and Ionis announced that spinraza had met its primary endpoint in an interim analysis of the ENDEAR trial. As a result, the trial was ended early and all participants were transitioned into SHINE, an open-label extension trial. Also as a result of these findings, Biogen initiated a New Drug Application (NDA) in the US, as well as international regulatory filings. They also announced plans for an expanded access program for individuals with SMA type I.

On November 7, 2016, Biogen and Ionis announced that spinraza had also met its primary endpoint in an interim analysis of the CHERISH trial. As a result, the trial was ended early and all participants are being transitioned into SHINE, an open-label extension trial. Biogen will also be sharing this new data with the FDA and other regulatory groups around the world.

NURTURE is currently recruiting participants.

Timeline of Events

- **2003 - 2006**: Cure SMA makes $500,000 in seed grants to fund the therapeutic approach that led to spinraza.
- **July 2010**: Ionis (then known as Isis Pharmaceuticals) licenses the intellectual property to begin development of spinraza.
- **December 2011**: Ionis initiates a Phase 1 clinical trial of spinraza.
- **January 2012**: Biogen and Ionis enter into a partnership agreement to continue developing spinraza.
- **April 2013**: Ionis begins testing spinraza in Phase 2 clinical trials.
- **August 2014**: Ionis and Biogen launch ENDEAR.
- **November 2014**: Ionis and Biogen launch CHERISH.
- **March 2015**: Biogen and Ionis launch NURTURE.
- **August 12, 2016**: Biogen and Ionis announce their intention to initiate regulatory filings for spinraza, after the drug meets its primary endpoint in an interim analysis of ENDEAR.
- **September 26, 2016**: Biogen and Ionis announce that they have completed their rolling submission to the FDA and EMA.
- **October 28, 2016**: Biogen and Ionis announce that the FDA has accepted their application with priority review.
- **November 7, 2016**: Biogen and Ionis that spinraza also met its primary endpoint in an interim analysis of CHERISH.

A decision from the FDA on spinraza is expected in December 2016 or the first quarter of 2017.
How You Can Help Us Shape the Future for Those Affected by SMA

As more SMA drug programs progress through trials and reach new stages of the regulatory process—including Biogen’s recent NDA filing—there is an increasing need for us to address these clinical and regulatory issues and bring the patient voice into the process. We are also focused on the issues that will impact access to a treatment once it is approved by the FDA. These include advocating for broad labels, addressing payers to ensure that SMA treatments are covered by private and public insurers, and promoting newborn screening, so that, in the future, anyone born with SMA can immediately begin receiving treatment.

At this very critical time, we need to make certain that regulators, payers, and industry partners understand the impact of SMA, and create solutions that will address the areas most important to us.

Many of you have asked how you can participate in this process. The following are just a few of the ways you can get involved.

**Patient-Focused Drug Development Meeting**

Cure SMA is excited to announce that the SMA community has been granted a Patient Focused Drug Development Meeting with the FDA. At the Patient Focused Drug Development (PFDD) Meeting, individuals and families from throughout our community will have the opportunity to testify directly to the FDA. The meeting is tentatively scheduled for spring 2017. We will pass along the exact date when it is finalized. We will also be announcing a number of ways that our community can participate in this meeting.

The testimony at this PFDD meeting will also be used to create a Voice of the Patient report. This report will become part of the review process for all SMA treatments that are submitted the FDA.

**To learn more about all of these initiatives:**

Visit www.cureSMA.org/news and click on “Advocacy” on the right hand side of the page.  
Send us an email at info@curesma.org to request more information.  
Watch our Facebook page at www.facebook.com/cureSMA.  
Check out the Advocacy section of this newsletter, which begins on page 44.
Community Update Survey

For the past several years, we’ve been working to collect data and information on our community’s experiences, goals, hopes, and challenges. We know that the voice of our community is powerful. By sharing our stories, we can communicate our priorities to the FDA and regulators, provide insight into daily life with SMA and the ongoing medical challenges, and help ensure favorable insurance coverage for the new treatments that we expect to come forward in the near future and beyond.

That is why we’re preparing to launching a community update survey covering a number of areas that are relevant to the real-world experiences of living with SMA.

We are encouraging every family in our community to complete this survey. This survey will open in December. Check out www.cureSMA.org/news for more information.

Community Education Modules

With the recent developments in the SMA drug pipeline, we have planned a series of educational modules to address the topics, issues and questions that will be most critical as we move forward. All modules will include an interactive learning opportunity, such as a webinar, as well as booklets, handouts, and other materials that our community can review and refer back to. Webinars will be recorded and posted online for those who are not able to participate during the live session.

Our first webinar was held Tuesday, December 6. This webinar covered the NDA process, featuring panelists Doug Kerr, Global Neurology Development Lead at Shire and member of the Cure SMA Board of Directors, and Jeanne Ireland, Principal of Ireland Strategies and former Senior Advisor to the Commissioner at the FDA.

Our second webinar will cover FDA Interactions, including the upcoming Patient-Focused Drug Development Meeting. This webinar is tentatively scheduled for February 2017. A final date as well as registration will be announced in early 2017.
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 and greet & family fun fest, teen and adult social activities and many opportunities to connect and interact with families and receive first hand updates from the researchers.

Every year we look forward to reuniting as a community at this conference and showing our support for others. As always, the Family and the Research Conferences run alongside each other. This is the largest conference in the world for those affected by SMA, and also for those involved in providing support and care for SMA patients. There is no other program like it. The interactions between the researchers and families at this one conference are extremely special. The annual conference also provides the children an opportunity to make new friends and have a great time. We are expecting a record breaking attendance of 1,800 attendees.

Disney's Contemporary Resort is an ultra-modern hotel with award-winning dining, spectacular views and dazzling pools. Whether you’re staying in the iconic A-frame Contemporary tower or the nearby Garden Wing, you can walk to Magic Kingdom main gate or catch the Resort Monorail.

Take your pick from 2 pools conveniently located along the shore of Bay Lake. You may take the complimentary Resort Monorail to Magic Kingdom Park and with just one transfer take it to Epcot. In the evening, enjoy a nighttime water parade as the Electrical Water Pageant lights up the lagoon.

Guest rooms are non-smoking and offer some great features like a mini refrigerator, coffee makers, pack n play crib a room safe as well as internet - free wifi service.

Disney’s Contemporary Resort also offers an abundance of restaurants such as the California Grill, Chef Mickey’s, and the Wave… of American Flavors, and also some quick service restaurant’s like the Contempo Café, Contemporary Grounds and The Sand Bar.

**SPECIAL CONFERENCE THEME PARK TICKETS**

Come for the conference, and stay for the magic! Make the most of your free time with special Disney Meeting and Convention Theme Park tickets. Annual SMA Conference attendees are eligible for advance purchase of specially priced discounted Disney Meeting/convention Theme Park tickets.

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

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

For more information, please email familysupport@curesma.org

EXHIBITOR OPPORTUNITIES

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

For more information, please email exhibitor@curesma.org

SPONSORSHIP OPPORTUNITIES

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

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

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

SMA CONFERENCE GOALS

• 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 and research and clinical trials.
• To allow networking and data sharing between researchers and families and patients.
• To educate researchers on the latest research advancements.
• To attract the best researchers to the SMA field and encourage collaborations and investments.
• To promote cross-disciplinary dialogue among academic, clinical, and industrial researchers.
The 2016 Annual SMA Conference was a huge success! The weekend long event at the Disneyland Hotel was full of programs and events that brought together families and researchers.

The conference kicked off with the annual Meet & Greet and Researcher Relay Race, and closed with the, “It’s A Wonderful Life” Q&A panel – and the days in-between offered a variety of programs and events for all attendees.

Over 1,500 attendees, including 300 researchers from nearly 100 organizations and pharmaceutical companies, gathered in Anaheim, CA, to take part in this year’s Annual SMA Conference—making this our largest conference ever!

The 2016 Annual SMA Conference allowed us to build on our progress and the important milestones that our community has already reached, and to chart a course to meet our goals in the years ahead.

Thank You to Biogen, the Presenting Sponsor of the 2016 Conference

Cure SMA would like to thank Biogen for their generosity as the presenting sponsor of the 2016 Annual SMA Conference. Biogen also went above and beyond to provide all families attending the conference with twilight tickets to Disneyland on Saturday evening, where they could experience the Paint the Night Parade. The parade was filled with floats, music, and characters, and attendees lined the street in matching Cure SMA conference t-shirts to enjoy an evening in the park and help spread awareness for SMA.

“Without the generosity and support of Biogen, it would not be possible to bring the entire SMA community together on this scale,” said Kenneth Hobby, president of Cure SMA. “In nearly 30 years of hosting this conference, this will be one of our largest ever, which is fitting. We’ve broken new ground over the past several years, through our efforts in research, family support, and advocacy. We’re excited to continue expanding that, using this conference as a springboard to new initiatives that we have planned over the next several years.”

“The Cure SMA annual meeting provides a unique and vibrant opportunity for leading researchers, families and advocates to learn about advances in SMA research and care and share their experiences as a unified community,” said Paula Cobb, senior vice president, Biogen Rare Disease Group. “We’re proud that our sponsorship contributed to bringing families to this special conference.
Reseacher Relay Races
Meet & Greet
This year’s Children’s Program was such an interactive and exciting time, had by all! Thanks to so many of our wonderful volunteers who helped make this weekend incredibly special for everyone, especially all of the children. The kids stayed engaged by participating in an array of arts & crafts, countless toys, movies, Wii video games, special entertainment guests, and so much more!
The Family Friendly Research Poster Session

The Family Friendly Research Poster Session brought together families and researchers, and encouraged families to move around to the different posters to ask questions and learn from the researchers about the specific projects being presented.

At the 2016 Annual SMA Conference, 30 presenters representing different SMA research projects were showcased. All of the clinical stage drug programs for SMA were included among the presenters, plus a variety of basic research projects and clinical care research projects.

POSTERS INCLUDED:

- AveXis INC. on “An innovative approach to spinal muscular atrophy: Replacement of the SMN1 gene with gene therapy”.
- F. Hoffmann-La Roche and Genentech on “Roche SMA program - our commitment to patients and families”.
- F. Hoffmann-La Roche on “An oral SMN2 splicing modifier in clinical development for patients with SMA”.
- Cytokinetics on “CK-2127107, a selective activator of the fast skeletal muscle troponin complex, for the potential treatment of SMA”.
- California Institute on Biomedical Research (CALIBR) on “Identification of drug candidates for SMA using genome-edited neuronal cells”.
- RaNA Therapeutics on “A novel epigenic approach to treat SMA”.
- Ionis Pharmaceuticals on “Developing Nusinersen: an antisense drug for the potential treatment of SMA”.
- Chien-Ping Ko PhD, Charlotte Sumner MD, and Sergey Paushkin PhD on “A new book on SMA: Spinal Muscular Atrophy: Disease Mechanisms and Therapy”.
- Novartis on “LMI070 for treatment of type I SMA”.
- Burghes Lab at Ohio State University on “Neuromuscular impact of symptomatic SMN restoration and development of assays for potency of scAAV9-SMN as well as identification of suppressors of SMA mutations”.
- The Beattie Lab at Ohio State University on “Going fishing! What fish motor neurons can tell us about SMA”.
- The Sumner Lab at Johns Hopkins University on “Long Non-Coding RNAs: Expanding SMA therapeutic targets”.
- The Cléry Lab from ETH Zurich on “Splicing regulation in SMA”.
- The Kothary Lab from Ottawa Hospital Research Institute on “Differential impact of SMA severity on disease: muscle as an example”.
- The Côté Lab from the University of Ottawa on “SMN and friends: What we can learn from SMN interactors”.
- Melissa A. Alderfer, PhD, at Nemours/Alfred I. duPont Hospital for Children on “Systematically assessing the psychosocial needs of families of children with SMA”.

Posters included:

- The Family Friendly Research Poster Session brought together families and researchers, and encouraged families to move around to the different posters to ask questions and learn from the researchers about the specific projects being presented.
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POSTERS INCLUDED:
• Tariq Rahman, MD, at Nemours/Alfred I. duPont Hospital for Children on “WREX- A dynamic arm orthosis for children with SMA”.

• The Androphy Lab at University of Indiana on “Developing novel therapies - a one-two knockout punch to SMA”.

• The Sahin Lab at Harvard University on “microRNA biogenesis in spinal muscular atrophy”.

• The Lotti Lab at Columbia University on “SMN-dependent cellular pathways and their contribution to SMA etiology”.

• Chad Heatwole, MD, at University of Rochester on “Adults Living With SMA: A Patient-Centered Assessment of the Symptoms That Matter Most”.

• Linda Lowes, PhD, at The Research Institute at Nationwide Children’s Hospital on “Tracking movement with the Microsoft Kinect”.

• The Murray Lab at the University of Edinburgh on “When Do Motor Neurons Get Sick in SMA”.

• Walter Truong, MD, at Gillette Children’s Specialty Healthcare on “The effects of assisted standing on bone in children with SMA”.

• Nilesh Mehta, MD, at Boston Children’s Hospital on “Body composition and individualized nutrition in pediatric SMA”.

• Rebecca Hurst Davis, MS, RD, CSP, CD, at the University of Utah on “Diet and blood sugar in children with SMA type II”.

• Deborah Boroughs, RN, MSN, at BAYADA Home Health Care Bayada on “Bridging a care delivery gap for family caregivers of children with SMA types I and type II”.

• Holly Peay, PhD, from Parent Project Muscular Dystrophy and RTI International on “To participate or not to participate? Decision making for SMA clinical trials”.

• Stephen J. Kolb, MD, PhD, at Ohio State University on “SMA infant biomarker study - super baby progress in 2016”.

• The DiDonato Lab at Lurie Children’s Hospital of Chicago on “Modifying SMA phenotypes in milder SMA mice”.

• Manuel Prieto Chief Technology and Innovation Officer at Marsi Bionics on “A wearable gait exoskeleton for the daily life activity of children with SMA”.

Winter 2016 DIRECTIONS
Workshops
Researcher Meeting
The 2016 SMA Researcher Meeting was a huge success! We would like to thank all of our attendees for making this year so memorable!

This special session was moderated by Cure SMA Scientific Advisory Board Member Stephen Kolb, MD, PhD (Ohio State University).

With the recent announcement that nusinersen has been submitted for FDA approval, and with other SMA drug programs progressing through clinical trials, opportunities such as this panel, “The Changing Landscape of SMA,” are critical to the next phase of SMA research and care.

Dr. Kolb began by asking the group to imagine a future when the first drug approvals for SMA therapies have occurred, and the implications that might have for basic research, drug development, and clinical care.

The session emphasized how to move toward effective drug treatments and improved quality of life for patients with all types of SMA and at all stages of disease progression. The session also included discussion of approval of second in class drugs (meaning new drugs that are approved after the first drug is approved), the use of combination therapies, and the importance of patient registries and standards of care.
Identification of Candidate Therapeutic Targets and Modifiers, Parts 1 & 2

Research has revealed that a number of systems, pathways and processes are affected in SMA. The presenters in these two sessions are investigating different aspects of SMA, looking for new ways to treat SMA that target other areas. Ultimately, these approaches could be used in combination with approaches that address the underlying genetics of SMA, giving us the best chance of a comprehensive, effective treatment. This is particularly important as we seek to develop treatments for all ages, stages and types of SMA.

The first session was moderated by Cure SMA Scientific Advisory Board Member Sam Pfaff, PhD.

The second session was moderated by Cure SMA Scientific Advisory Board Member Umrao Monani, PhD.

Regulation of SMN Protein Expression and Function

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

This session was moderated by Cure SMA Scientific Advisory Board Member Rashmi Kothary, PhD.

Clinical Research Studies

The goal of this session was to present the results of important studies that could influence clinical trial design or drug development. The session was moderated by Thomas Crawford, MD.

Overall, participants were favorable towards trial participation. Some barriers to trial participation included: the possibility of receiving placebo instead of drug, the need for more information about trial risks, lack of potential benefits, and the need for more information about day-to-day requirements. Parents were more likely to enroll their children due to: confidence in the trial and researcher, access to trials and to the drug post-trial, and recommended trial participation.

To read complete summaries of any of these sessions:

SMA Therapy Development

The highly anticipated closing session covered drug development for SMA. Seven talks were given about drugs both in preclinical and clinical development. Currently, there are 18 drugs in the SMA drug pipeline, with six of them in clinical trials.

This session was moderated by Cure SMA Scientific Advisory Board Member Adrian Krainer, PhD.
Our thanks to Biogen for their generosity as the presenting sponsor of the 2016 Annual SMA Conference.
Cure SMA thanks all of the sponsors for their generous support of the 2016 Annual SMA Conference. These sponsors are partners in our community who are critical to the success in the battle against SMA. These partners contributed to scholarships and family assistance programs for the 2016 Conference and for travel and lodging expenses for the SMA Researcher Meeting. Without their support many patients, families, medical professionals, and researchers would never be able to attend and join together in this fantastic meeting.
Thank you to Our 2016 Annual SMA Conference Exhibitors!

Cure SMA would like to thank the following exhibitors who have generously supported our 2016 Annual SMA Conference. Exhibitors who attend the Annual SMA Conference were able to promote their products and services to SMA families! These exhibitors are partners in our community who are critical members to helping us find a treatment and a cure for SMA. Thank you again for your support! This one-of-a-kind event would not be possible without your support!
If you are interested in being added to our mailing list to receive exhibitor opportunities for the 2017 Annual Conference, please email exhibitor@curesma.org.

Save the date!

2017 Annual SMA Conference

which will be held in Disney World, Orlando, FL June 29 – July 2, 2017!
Thank you to the whole team at Expo, Jennifer and Aaron Smith, Richard Curran and all of their family and friends. Their gracious donation to our Annual SMA Conference each year is truly amazing! All of the signage, banners and designs are donated to help spread awareness throughout the conference venues and provide this conference with the extra special boost of professionalism and the feeling of community during the conference days.

If you can believe it, Jennifer and Aaron go above and beyond this incredible donation by dedicating so much of their time to helping support other families throughout the country, raising awareness about SMA, and all of the additional help in ensuring that the Annual SMA Conference is a special week for everyone who attends. Your impact to this amazing conference is felt by those who travel from all over the world to attend each year!

We can never thank the Smith family and their friends and family for the fabulous work they provide through the years!

The Jacob Isaac Rappoport Foundation and Shaina and Adi Rappoport

An amazing 15 years of incredible support has been so generously provided by the Jacob Isaac Rappoport Foundation. Shaina, Adi and all their family and friends have gone above and beyond for newly diagnosed families, Cure SMA and the Annual SMA Conference.

From the entire SMA Community, we extend our sincerest thanks in the variety of ways you have touched and contributed to so many. Cure SMA truly would be no where near who we are today without this wonderful family and their efforts!

The Jacob Isaac Rappoport Foundation has provided dinners for families, cleaning services and large boxes of toys have been sent to hundreds of families, and they have also provided invaluable support and resources for families throughout the years. In addition to all of this amazing generosity, the Jacob Isaac Rappoport Foundation contributes greatly to the success of our Annual SMA Conference. They have hosted a special luncheon and reception for families each year, provided new opportunities for teenagers to mingle together at the conference with the Teen Social, and have really transformed the Children’s Program into such a special and fun time for kids. Shaina and Adi have sponsored the Children’s Program by providing entertainment, crafts and toys, helping to fund travel for many of the volunteers who come each year to help out and this program is such a vital component to making the Annual SMA Conference such a highlight each year! Thank you to the entire Rappoport family and friends for your amazing support!
At Cure SMA, we’re committed to making sure that families have the best, most accurate information about SMA and what it means for them, from day-to-day care to the changing landscape of research breakthroughs. Our care series booklets provide in-depth information on medical issues, genetics, and other topics of interest to both families and healthcare providers.

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

For print copies:
Please contact the Cure SMA national office at info@curesma.org.

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

Disclaimer:
Cure SMA does not, as an organization, support or endorse any particular treatment or therapy. Information contained in this booklet is for informational and educational purposes only. All medical information presented should be discussed with a qualified physician.
Quotes from Around the Community on the 2016 Annual SMA Conference

“Congratulations on a tremendous conference (my first time attending). Exceptional presentations/sessions and engagement! Looking forward to continued collaboration with you.”

–Diana M Slowiejkgo, PharmD, PhD, Genetech

“Thank you once again for organizing such a wonderful conference. It was a pleasure to be a part of it. As always, I learned a lot, made new connections, and have a renewed focus for my SMA research.”

–Dawn S. Chandler, PhD
The Ohio State University School of Medicine

“It was a really good meeting this year. We made some really good collaborations out of it, so it was very productive for my group scientifically as well.”

–Greg Matera, PhD
Professor, University of North Carolina

“Going to the conference was an invaluable asset for our family as newly diagnosed. We were scared and overwhelmed, and while those feelings aren’t gone, we know now that we are not alone and we feel much more equipped for the future. It was really, really good for us to be around so many people who know exactly where we are coming from, and I would recommend that any newly diagnosed family try to go!

It was an amazing experience. One that we will not forget. I have to admit I was a little overwhelmed seeing all types of SMA kids and adults but I felt like for the first time in my life that I wasn’t alone in this.

It was a life changing experience. I cannot imagine not attending another one from here on out!”
Hats off to you for an absolutely phenomenal Cure SMA meeting. I attend close to 15 separate medical meetings a year. Your meeting stands above all others as the best venue for scientific and clinical updates AND helps my heart grow larger. Last night was one of the most positive professional experiences I’ve ever had.

– Sarah Kulke, MD
Cytokinetics

You guys at Cure SMA did an awesome job (which has been what we’ve experienced both times we’ve attended), making it educational and an awesome memory for the families! A big THANK YOU to everyone for all the time, energy, and work that you give to make it happen. I was able to talk with some of the volunteers, and thank them in person too. When you step back and realize all the people involved it is really impressive. I’m sure you all know how much the conferences mean to all of us, but just wanted to make sure you heard it from my family as well! I was really thankful that Hannah was able to meet other children, and was so happy with the experience of her first conference.

– Charlotte Shepherdson

This past 2016 Cure SMA conference marks the first ever National conference I have attended. I do not regret many things in my life, but not going to these annual events earlier remains one of them. They are chock full of information that tailors to the individuals experience with SMA. The supportive environment makes it easy to connect and share with other affected individuals. I personally found the workshops beneficial and was a great way to connect to other people experiencing the same issues I face on a daily basis. The Cure SMA team did a great job in emphasizing that doctors, drug representatives, and staffing are here for you. They are here to listen, educate, and discuss real life issues that affect you everyday. These conferences are a sure-fire way to get involved and support the fight for SMA, and I am excited to see what the next conference holds in store!

– Brynne Michelle Willis

I would like to thank you and Cure SMA for your kind invitation to the Cure SMA meeting. I greatly appreciated your attention and your warm invitation to present our genomics work on SMA. I was very impressed and happy by the progresses in therapeutics in SMA.

Best wishes,
Judith Melki, MD, PhD

Thank you once again for a fantastic meeting and all the enthusiastic work you put into everything.

– Prof. Dr. Brunhilde Wirth
Head Institute of Human Genetics
University of Cologne

“Congratulations on another amazing conference! It is such a special, inspiring and (last but not least) informative meeting and I can’t thank you enough for everything you do to make it a success.

In many ways I feel like the joy experienced through the eyes of the SMA children and families carries us through the year. It definitely fuels the work of everyone at Biogen and we are privileged to have been a part of the Disneyland conference.”

– Gillian S. Mullins
Biogen
Paint the Night Parade & Evening in the Park
Family Support

These beautiful blankets were donated to our newly diagnosed care package program in honor of Charlotte Facchini. All of the blankets were handmade by Charlie Marie’s great grandma and great Aunt Jojo. We would like to thank Charlotte’s family for making this wonderful donation to Cure SMA!

A donation of musical touch pads and rattles will be included in our newly diagnosed care packages that were so graciously given to Cure SMA! This donation was made in honor of Ava Adams’ first birthday. A huge thank you Ava’s family and friends for these wonderful items!

A large shipment of items arrived at the Cure SMA National Office, in honor of what would have been Aria Johnston’s first birthday. Aria’s family and friends collected three large boxes of toys, that were recommended by other SMA parents, for our newly diagnosed care packages. We would like to thank Aria’s family for their wonderful donation to Cure SMA!

Cure SMA received a wonderful donation of assorted toys that were sent in by Caitlin Candelari. Caitlin donated these items in honor of Graham Leatham’s birthday. We would like to thank Caitlin for making this generous donation to Cure SMA!

Thank you to the Wick Family for the adorable pillow pets! Cure SMA received a donation of pillow pets from the Wick family which were given in honor of their daughter Alison’s birthday. The pillow pets will be added to our newly diagnosed care packages.
Cure SMA is so grateful to have received a special delivery of stacking cups that were donated by Pamela Wright, in honor of her granddaughter Audrey Quynh Wright. These will be included in the SMA Type I Care Packages that will be sent to newly diagnosed families when they first contact Cure SMA. Thank you Pamela for your wonderful donation to Cure SMA!

Joyce Bruno & Sherlene Baird are two of the sweetest ladies! Both of these ladies have been coming in to our office one day a week for the past 2 years to volunteer their time. They sit in the kitchen and put labels on newsletters and other odds and ends jobs and they brighten up our day with their beautiful smiles and friendly nature. We are so grateful to have such kind and dedicated volunteers. Thank you, Joyce and Sherlene!

We would like to give a special thank you to Patricia Kerster for donating items to our care package program! These assorted items were recommended by other SMA parents and were donated in honor of Jake and Kate Saxton.

A special donation came to Cure SMA in honor of Dashiel Stanton. The donation included counting books and crocheted hats. Dash’s mom, Amanda, donated the counting books because Dash loves to poke the bubbles in the book, so she wanted other kids to enjoy it too! Dash’s grandmother, Patty, crocheted the colorful hats for newly diagnosed families. We would like to thank Amanda and Patty for their wonderful donation to Cure SMA!

A very sincere thank you to Graham Vollmer’s grandmother who hand knitted adorable hats for newly diagnosed families. This generous donation was made in honor of Graham Vollmer’s first birthday.

Cure SMA received a generous donation from Steven and Gloria Kimmel, in honor of their son Isaac. Isaac loved to be read to, so his parents sent an assortment of books to be included in our newly diagnosed care packages so other families can enjoy reading together. We would like to thank Steven and Gloria for their wonderful donation to Cure SMA!

2017 Annual SMA Conference
which will be held in Disney World, Orlando, FL June 29 – July 2, 2017!

“Save the date!”

“If you are interested in being added to our mailing list, please email exhibitor@curesma.org

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The Bradshaw family honored their son, Harrison, over Christmas by filling his stocking with things that they wanted to donate to our newly diagnosed care package program. Harrison’s brother and sister thought that filling up his stockings for others affected by SMA was a great way to remember Harrison during the holiday season. We would like to thank the Bradshaw family for their wonderful donation to Cure SMA!

The Brady Corporation hosted a toy drive for their employees and collected toys and donations to help fund type II and type III care packages that are sent to newly diagnosed families as soon as they contact Cure SMA. Thanks to the generosity of their employees, and a match from their Employee Volunteer Program, The Brady Corporation was able to donate 17 care packages to support our newly diagnosed care package program! Thank you to The Brady Corporation for their wonderful donation to Cure SMA!

The Garrison family lost their son, Cooper, in July to SMA. Cooper was five months old. To honor Cooper’s 1st birthday, his family planned a virtual birthday party and requested that friends and family members donate items for our newly diagnosed care package program. We would like to thank Cooper’s family and friends for their incredibly generous donation!

Cure SMA received two special donations in honor of Bennett Morea’s third birthday. Thank you so much to Karin and Nathan Morea for donating the lightweight rattles and o-balls in memory of their son. And thank you to Janie Mellas for the “You Are My Sunshine” books donated in memory of her grandson. These wonderful gifts will be added to our Newly Diagnosed Care Packages and will be sent to newly diagnosed families as soon as they contact Cure SMA.

Cure SMA received a wonderful donation from the Crane family, in honor of their son Isaac’s 2nd birthday. The Crane family donated the book “The Gruffalo” to our newly diagnosed care package program. Isaac loves to read, and for the past several months, his favorite story has been “The Gruffalo.” He loves the illustrations, the words, and to hear people’s voices bring the characters to life. The Crane family hopes other children will enjoy this book as much as Isaac has! Thank you to the Crane family for your wonderful donation to Cure SMA!

We would like to thank Carol Bixler for making a wonderful donation to Cure SMA in honor of her great-grandson Luke Bertch. Carol’s donation included handmade blankets for our newly diagnosed care packages.

The Garrison family lost their son, Cooper, in July to SMA. Cooper was five months old. To honor Cooper’s 1st birthday, his family planned a virtual birthday party and requested that friends and family members donate items for our newly diagnosed care package program. We would like to thank Cooper’s family and friends for their incredibly generous donation!
A very special thank you to Ashleigh Pray who donated playfoam and a foam head donut to help protect the head, neck, and ears from pressure related issues in honor of her son Lincoln. These items will be added to our newly diagnosed care packages.

The Fighting for Kaiden Foundation donated bath chairs, strollers, and toys, to be included in type II and III care packages. The Fighting for Kaiden Foundation was founded by the Defazio family in honor of their son Kaiden, who has SMA type I. Cure SMA would like to thank the foundation and all their incredible supporters and donors.

Cure SMA received a generous donation of toys and other items from Ionis! These items were collected through a toy drive organized by Ionis during the holiday season. These items will be added to our newly diagnosed care packages, and we would like to thank Ionis for their wonderful donation to Cure SMA!

We would like to thank Tony and Monica Perez who so generously donated dry erase calendars, blankets, and chapstick. This donation was made in honor of Tony and Monica’s daughter, Bellamay Perez which will be added to our newly diagnosed care packages.

Jadon’s Hope Foundation has generously donated 10 new feeder seats to the Cure SMA equipment pool. To learn more about this donation and about the Cure SMA equipment pool, visit our news section.

Cure SMA, The Shakespeare family has made a generous donation in memory of their son Mathis. The Shakespeare family provided a handful of cat toys for our newly diagnosed care packages.

These handmade blankets have been donated in honor of Ellie Stevenson from the Bellsy Project to be included in the newly diagnosed care packages. Thank you for all you do for SMA and the families affected.

Love,
Kate Stevenson (Ellie’s Mom)

These glow sticks were donated in honor of Elke, by her parents Anton and Stephanie Kliewer. Elke loves little glow sticks and she sleeps with one every night. Since they are “light” in two ways (light in weight and provide light), the family thought it would be a great addition to our SMA type I care packages. Thank you to the Kliewer family!

Cure SMA received a generous donation from the Live RhysStrong Foundation in honor of Rhys Santiago. Their donation included Crayola Twistable Slick Stix crayons that will be added to our newly diagnosed care packages. We would like to thank the Live RhysStrong Foundation for their wonderful donation to Cure SMA!
Each month a new box arrives at the Cure SMA National Office. As each new box is opened an array of colors and designs pop out from within the box. Every quilt that is taken out of these boxes radiates the love, compassion and dedication of Montana Grandma. Since her granddaughter was diagnosed almost seven years ago, this quilt project created a way to reach out to other families who have also received a SMA diagnosis. “I felt the pain of my son, TJ, and daughter-in-law, Jaime, as they faced this incredible ordeal of living with SMA. Through them I realized many other families were going through the same pain. So, I decided the way to help was to make a quilt for each child.” Each quilt is carefully hand crafted with love from Cindy Bobolz, aka Montana Grandma, in honor of her beautiful Nora, and arrives at a newly diagnosed families’ house in their care package, once they connect with Cure SMA.

Cure SMA cannot thank Montana Grandma enough for the many years of contributing her time, fabric, craftsmanship and passion so that other families and members of this community can be wrapped in the assurance that they are not alone in this journey.

Montana Grandma is currently retired and living in a small town in the mountains of Montana. She has been sewing for over 50 years and quilting for over 35 years.

If you have received a quilt from Montana Grandma and would like to share your comments with her, please check out this special link that was created by Nora’s parents, TJ and Jaime, to allow everyone in the SMA Community an opportunity to connect with Montana Grandma, www.goodentree.com/quilts.
Dear Cure SMA,

Words cannot express how blown away we were when we received Shepard’s care package from y’all. It was like Christmas morning for him! He squealed with delight as we unpacked it and wanted to play with everything all at once. He loves sleeping on his sheepskin. That has been Mama’s favorite too since it helps Shepard sleep better and more comfortably both at nap time and bedtime. We are new to the world of SMA, but of all the diagnoses out there this one has got to have the best support network! We are comforted knowing that there are so many loving families out there that understand what we’re going through and so willing to support/help in anyway. Thank you again!

Love,
Sara, Eric, and Shepard Blanchone

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Dear Cure SMA,

Thank you so much for the box of toys and also for lending us an SMA friendly car seat. Our son especially likes the feather and the fox mirror. We recommend the Lamaze Soft Chime Garden; it’s our 5 month olds absolute favorite toy.

Thank you again for not only the toys and car seat but the overall support you have provided our family since our son’s diagnosis. You are an amazing organization.

The Sweet Family

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Dear Cure SMA,

I can not begin to thank your organization enough for the generous package of presents. We all cried when we opened the box. We were exhausted from being 21 days in the hospital and seeing the baby suffering with all the treatments. We came home and opened the box and saw all the donations from different babies with SMA and it was very moving. What a generous group of families to reach others that just began the process of understanding SMA.

Sincerely,
Marlena Nord of Minnesota
Hi Cure SMA,

We received the information packet and the care package... it was more than I could have ever imagined. My family and I are beyond grateful that we don’t have to walk this journey alone.

Thanks again for the wonderful package and the continued support. It really means the world to me.

Lindsay Deshotels of Texas

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I cannot thank CureSMA enough for all the resources and support you’ve sent during our first month. I really do not know how we would have managed without it.

The care package you sent of toys and books and blankets is overwhelming, but it’s already helping William play more actively. He loves the rattle and the O-ball.

Sending love from our family to our new, extended CureSMA network.

Thank you!
Kelly Jankowski of Pennsylvania

---

Cure SMA,

Thank you so much for the care package. Izabella is so happy and I am so happy too; thank you guys for all your love and dedication to us. God bless you!

Erika Moya of New York

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Dear friends at Cure SMA,

Wow! What an incredibly wonderful gift you have given us today. We were so surprised at all the amazingly generous gifts that were sent in the care package for our little Charlotte. You have absolutely touched our hearts and I can’t tell you how much we really appreciate seeing all the little pictures attached to the toys donated by the generous parents that are also going through what we are going through. You have brought tears to my eyes, tears of joy today. Thank you for helping us see how truly kind people can be and realize that we are not alone.

Charlotte is just absolutely enjoying everything and we are overjoyed with this little seat as it is the perfect support for her.

Thank you again so very much. Words can’t even express.

God bless.
Amanda Metter and Charlotte Rose Metter
Cure SMA,

Thank you so much for the amazing packet and care package! Our entire family from LA to Washington was in tears to see the generosity and love sent to our daughter by people and families who understand our needs and can relate to our struggles. In a time that seems so unstable, it brought some much needed optimism. Thank you again! We feel so blessed, and perhaps for the first time in a long time, we don’t feel alone.

Marilee Tweedy of Oregon

Cure SMA,

I just wanted to say how thankful and appreciative we are for the wonderful gifts that were sent. I cannot tell you enough how grateful I am to be welcomed into such an amazing organization! I was not expecting a box full of toys, blankets, a swing, and even a seat! Again, thank you all so much!

Sincerely,
Alyssa Moran of New York

Wow, we received all of the info and box of toys!
Thank you so much, I was envisioning a few toys and a list of toys. We truly appreciate all of the donations and information!
Saydee loves her new toys
Thank you again,
The Smiths of New York

Cure SMA Family,

We wanted to thank you all for the wonderful care package that was sent to our son. He is now able to grab and play with the smaller toys. We are so grateful for you all that put this package together and to the other families that helped as well.

Sincerely,
Tina and Torence and our son Malachi of Ohio

Dear Cure SMA,

Words cannot express the joy Kate felt and the gratitude we felt when her care package arrived. She was so excited and kept saying “My presents! My presents!” She loves puzzles, coloring, and fuzzy blankets. The news of her diagnosis has not been easy, but it is comforting to know that there is a support group like CureSMA to help. Thank you all so much!

Sincerely,
Brian and Elizabeth Veit of Missouri

Wow, we received all of the info and box of toys!

Thank you so much, I was envisioning a few toys and a list of toys. We truly appreciate all of the donations and information!

Saydee loves her new toys

Thank you again,
The Saiz Family of California
AveXis Announces Single-Arm Design for Pivotal Study of AVXS-101

AveXis, Inc., a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases, announced that the planned pivotal study of AVXS-101 in spinal muscular atrophy (SMA) Type 1 will reflect a single-arm design, using natural history of the disease as a comparator, and enroll approximately 20 patients. This update is based on the receipt of the minutes following the Type B meeting with the U.S. Food and Drug Administration (FDA) held on September 30, 2016.

In addition to evaluating safety, the planned program is expected to evaluate achievement of motor milestones, specifically patients’ ability to sit unassisted, as well as an efficacy measure defined by the time from birth to an “event,” defined as death or requiring at least 16 hours per day of ventilation support for breathing for greater than two weeks in the absence of an acute reversible illness, or perioperatively.

At the Type B meeting and in the meeting minutes, the FDA acknowledged the company’s rationale for a single-arm pivotal study and provided a number of constructive suggestions to help optimize such a trial design. The FDA also indicated its preference for a design with co-primary endpoints consisting of a measure of developmental milestone achievement (such as sitting unassisted) along with a clinically meaningful measure of survival (such as time to an “event” as described above). Based on FDA’s suggestions as well as other expert input, AveXis continues to evaluate a number of the details of the trial design. More specific information will be made available at the time the study is initiated, which is expected in the first half of 2017.

“We believe the Type B meeting had a positive tone, with FDA offering a number of constructive suggestions which we believe will better enable implementation of a pivotal study design that is most appropriate for the patients suffering from this devastating disease,” said Sean Nolan, President and Chief Executive Officer of AveXis. “With the feedback needed from the FDA to move forward with our pivotal trial, we plan to proceed as expeditiously as possible to begin the study in the first half of 2017.”

With regard to the ongoing Phase 1 trial of AVXS-101, the FDA stated the following in the meeting minutes: “We strongly recommend that at the completion of the study, you request an end-of-Phase 1 meeting to evaluate the adequacy of data to support future product development, including a discussion of whether the data from the Phase 1 study might provide the substantial evidence necessary to support a marketing application.”

The company’s strategy with the SMA Type 1 program is to complete the ongoing Phase 1 trial and, in parallel, execute on the single-arm pivotal trial, while continuing collaborative discussions with the FDA regarding the most expeditious pathways for FDA approval of AVXS-101.

Cure SMA Funds Multiple Gene Therapy Approaches

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

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

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

In total, Cure SMA has granted $845,000 for gene therapy, including support for both the systemic program and the CSF program. Using the data generated with our funding for CSF delivery, Dr. Kaspar and his team were able to secure a $4 million grant from NINDS in 2013, to develop this delivery approach for human clinical trials in SMA.
AveXis Reports Interim Data from Ongoing Phase 1 Clinical Trial of AVXS-101 at the World Muscle Society Congress

AveXis, Inc., a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases, today provided an update on interim data from the ongoing Phase 1 trial of AVXS-101 in spinal muscular atrophy (SMA) Type 1 as of September 15, 2016. The data were presented by Jerry Mendell, M.D., director of the Center for Gene Therapy at The Research Institute at Nationwide Children’s Hospital, at the 21st International Annual Congress of the World Muscle Society in Granada, Spain. Earlier this year, AveXis received breakthrough designation from the FDA for AVXS-101, the first SMA program to receive that designation.

For the first time, interim data from the trial were presented that highlighted patient achievement of key motor development milestones as of September 15, 2016. Two-thirds of patients in Cohort 2 (the proposed therapeutic dose) had achieved the ability to sit unassisted, including one patient whose achievement of this milestone was confirmed after September 15. In Cohort 2, 11 of 12 patients achieved head control, 7 of 12 patients could roll over completely and 11 of 12 patients could sit with support. Two patients are now walking independently, including one whose achievement of this milestone was confirmed after September 15. These two patients each achieved earlier and important developmental milestones such as crawling, standing with support, standing alone and walking with support.

“To date, the majority of patients who received the proposed therapeutic dose of AVXS-101 have achieved key milestones and two-thirds of these patients can sit independently—a fact completely inconsistent with the known disease course, as children with untreated SMA Type 1 will never sit unassisted,” said Sean Nolan, President and Chief Executive Officer, AveXis. “We are encouraged by these interim data, and continue to work diligently to bring this gene therapy to the children suffering from this devastating condition.”

Roche Launches New Phase 2 Clinical Trials

In September of 2016, Roche officially launched two Phase 2 clinical trials testing RO7034067 (also known as RG7916) in response to favorable results from a Phase 1 study in healthy volunteers. RO7034067 is an orally available drug that aims to correct the splicing of SMN1, the SMA “backup gene.” One trial (Firefish) will be enrolling approximately 50 infants with SMA type 1 at sites across the US and Europe. Firefish is an open-label study consisting of two parts: an exploratory dose finding part (Part 1) for 4-weeks and a confirmatory part (Part 2) of RO7034067 for 24-months.

The second trial (Sunfish) will be enrolling individuals aged 2-25 with SMA type II or III. This trial will recruit nearly 200 individuals across 34 sites in the US, Canada, Europe and Australia. Sunfish is a randomized, double-blind, placebo-controlled study consisting of two parts: an exploratory dose finding part (Part 1) of RO7034067 for 12-weeks and a confirmatory part (Part 2) of RO7034067 for 24-months.

Clinical development of RG7800, a related compound, remains on hold. Individuals who were previously enrolled in “Moonfish,” the clinical trial testing RG7800, will have the opportunity to transition into a separate open-label study testing RO7034067.

Novartis Releases Community Update on the Study of LMI070

Novartis recently provided a community update on clinical trials for LMI070. This information is effective as of July 25, 2016:

“In May, we shared with you news of the difficult decision to pause enrollment for our study of LMI070 for the treatment of Type 1 Spinal Muscular Atrophy (SMA). This decision was made because results from an animal study, using daily dosing for a year compared to weekly dosing in the human study, showed unexpected injuries to the peripheral nerves and spinal cord, testes, and blood vessels in the kidney.

We would like to bring you up to date on what has been happening since then.

In addition to pausing enrollment, we also lowered the dose of LMI070 for enrolled patients in an abundance of caution. About eight weeks after this reduction, we received confirmed reports of worsening of motor skills in some patients varying in degree of severity. We are extremely saddened to note that one patient died following an upper respiratory infection with contribution of increasing respiratory muscle weakness.

Following these reports, and with the approval of the Data Monitoring Committee (DMC), a team of independent experts who monitor the trial’s safety results, we have decided to offer patient’s families the option to return to the original dose. This has allowed parents and investigators to make the best decision for each individual patient.

We remain committed to the safety and wellbeing of children in this trial. We hope to resume enrollment in the study when we have fully evaluated the new toxicology findings, the additional monitoring of patients, and the feedback from the regulatory authorities, investigators, and DMC members.

We will continue to provide updates as new information is available.

The Novartis Team”
SMA Community is Granted a Patient Focused Drug Development Meeting with the FDA

Cure SMA is excited to announce that the SMA community has been granted a Patient Focused Drug Development Meeting with the FDA. At the Patient Focused Drug Development (PFDD) Meeting, individuals and families from throughout our community will have the opportunity to testify directly to the FDA.

The PFDD meeting will include testimony on a variety of topics important to our community, including:

- The impact of SMA on daily life
- What would amount to meaningful change in SMA patients’ everyday lives
- Benefit/risk
- Our priorities in drug development
- Our preferences and expectations for an improved treatment

The testimony will also be used to create a Voice of the Patient report. This report will become part of the review process for all SMA treatments that are submitted the FDA.

The meeting is tentatively scheduled for spring 2017. We will pass along the exact date when it is finalized. We will also be holding a webinar in early 2017 on FDA Interactions. The webinar will cover how to participate in the PFDD meeting as well as other opportunities for engagement with the FDA.

Leading the Way in FDA Engagement

Earlier this year, Cure SMA, along with our partners at MDA and the SMA Foundation, gathered the information and prepared a formal request for this PFDD meeting. We thank our partners for their hard work in making this meeting possible.

In addition, we will be announcing a new community survey in later this year. This survey will cover a number of areas that are relevant to the real-world experiences of living with SMA. Through this survey, we will collect and distribute data to support the testimony presented at the PFDD meeting.

At this very critical time, many of you have asked us how you can participate in the process. Participating in this survey and, if you are able, participating in the PFDD meeting, will provide a tangible and measurable impact for our whole community.
Cure SMA Announces Newborn Screening Initiative in Partnership with MDA

Twice this year, Cure SMA has had the opportunity to testify before the federal Advisory Committee on Heritable Disorders on the need for newborn screening for SMA. This committee, part of the Health Resources and Services Administration (HRSA), an agency of the U.S. Department of Health and Human Services, determines which conditions will be added to the Recommend Uniform Screening Panel (RUSP). This is a panel of diseases the federal government recommends to the states for newborn screening.

In May, Spencer Perlman, a Cure SMA board member, testified about the importance of newborn screening. Earlier this month, Shannon Zerzan, a mom of a son with SMA, also testified on behalf of Cure SMA. Both testimonies focused on the need to reduce diagnostic delays, the progress of the SMA drug pipeline that has brought us to the cusp of approved treatments, and the importance of early administration of that treatment.

One primary goal of this testimony was to lay the groundwork for our Newborn Screening Initiative, launched earlier this year in partnership with MDA. The initiative includes a working group comprised of members of Cure SMA’s Board of Directors, staff, Scientific Advisory Board, and external SMA experts.

Working Toward Newborn Screening for SMA

Once a treatment is approved by the FDA, newborn screening for SMA would allow individuals to begin receiving treatment immediately, even before showing symptoms.

In addition, adding SMA to newborn screening would eliminate the diagnostic delays that many families face. Research has shown that these delays range from an average of 3.6 months after symptom onset for those with SMA type I, up to an average of 43.6 months (more than 3 ½ years) for those with SMA type III.

Because the FDA review of a treatment can take 8-12 months, and because the process for adding a condition to the RUSP is quite complex and requires significant data packages in itself, it is important that the necessary work on newborn screening take place alongside the FDA review of a treatment.

This initiative is a continuation of Cure SMA’s longstanding work in this area. In 2008, Cure SMA and other partners from the SMA community submitted an application to have SMA added to the RUSP. In response, the committee requested additional data from statewide pilot screening programs. This data, along with the continued progress of the SMA drug pipeline, will be crucial in moving the application forward.

How Newborn Screening is Implemented for SMA

In order to have a condition added to the RUSP, the committee must have pilot screening data from one or more state labs, validating the diagnostic test and its predictive capacity. The committee must also see that there is an approved treatment a disease, and that there is evidence that pre-symptomatic treatment is effective. Once a condition has been added to the RUSP, screening must also then be implemented on a state-by-state basis.

Cure SMA, MDA, and the advisory group have already begun work on several important markers that must be reached in order to have SMA added to the RUSP. These include:

- Testifying before Advisory Committee on Heritable Disorders
- Ensuring that proper state pilot screening is taking place, in order to support the application
- Compiling and preparing this pilot data as it is collected
- Creating relationships at the federal and state level
- Preparing the formal application to have SMA added to the RUSP
- Working with Congress to secure the appropriations needed to fund newborn screening for SMA

Eliminating Barriers to Treatment

Our primary goal is to get a treatment approved for as many people as possible, as quickly as possible. And once that treatment is approved, our goal is to make sure that treatment is administered in a way that will provide the most benefit to our community—from newborns to adults.

Newborn screening would eliminate the barrier of diagnostic delays, which can prevent individuals from receiving timely treatment. In addition, a number of initiatives we are working on—including initiatives on insurance/coverage and ensuring broad drug labels—will also help remove barriers to treatment access for all ages, stages, and types of SMA.

In the coming months, we will provide additional updates on all of these initiatives, including newborn screening. In addition, many of these topics, as well as opportunities for community participation, will be covering in our upcoming series of educational modules.
2016 Update on SMA-FDA Interactions

The SMA drug pipeline has grown dramatically in just over a decade. Of the 18 programs in the pipeline, six of these are in clinical trials, and several of those are in Phase 2 and Phase 3 clinical trials.

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

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

The Cure SMA FDA Engagement Initiative

In continuation of Cure SMA’s FDA Engagement Initiative, representatives have met regularly with FDA and other patient stakeholder groups to discuss patient perspectives and the importance of patient input in the drug development process. These meetings have been possible in part due to FDA’s work on the Prescription Drug User Fee Act (PDUFA) and the Patient-Focused Drug Development Initiative (PFDD).

PFDD and PDUFA

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

As part of this commitment, FDA pledged to hold at least 20 public meetings over the course of PDUFA V, each focused on a specific disease area, as well as offering participation in other community-wide meetings.

Voices of SMA

Late last year, Cure SMA set out to create a personal and effective tool with which to educate FDA staff as part of the PFDD Initiative. Through dozens of stories from the SMA community, Cure SMA created The Voices of SMA booklet, which described SMA drug development priorities, the impact of SMA on patients and their families’ daily lives, potential progress for patients, and our preferences and expectation for improved treatment.

The booklets, which were enthusiastically received by members of FDA leadership will be used to inform the agency during the review process when an SMA drug is brought forward for approval. In response to the Voices of SMA, Janet Woodcock, MD, Director of the FDA’s Center for Drug Evaluation and Research, shared, “Thank you. This is very moving and informative. We have to salute the courage of the families dealing with this terrible condition—they are continuing to fight! We have heard from other patient groups, who have serious debilitating illnesses, that improvements that would be ‘small’ in a healthy person can be extremely meaningful if one’s function is severely compromised.”

PDUFA Reauthorization

As we are nearing the end of PDUFA V, Cure SMA began working with FDA and other stakeholder groups at the end of 2015 to work towards reauthorization of the law that is set to expire in September 2017. As part of the process to reauthorize this law, the FDA held monthly meetings with stakeholders, patient groups, health care professionals, and academic experts to discuss ways to improve the drug review process. These meetings were concluded in March and were used to inform the agreement between FDA and industry that will be sent to Congress in the Fall of 2016 for reauthorization next year.
Cure SMA is committed to addressing the needs of individuals and families affected by SMA. In order to accomplish this goal, we’ve been working for the past several years to collect data and information on the experiences of living with SMA, as well as our community’s goals, hopes and challenges.

This information can be collected via quantitative means such as data, surveys, and questionnaires, or qualitative means such as patient and family stories.

The Importance of Comprehensive Data from Our Community

Data collected from our community can be used in many ways. For example:

- Shaping the direction of clinical trials for SMA, guiding our pharmaceutical partners to recruit participants efficiently, and designing trials with endpoints that are clinically meaningful to quickly determine whether the treatments are effective.

- Communicating our community’s goals and priorities to the FDA and other regulators, such as our community’s viewpoints on benefit/risk and meaningful change in SMA patients’ lives.

- Providing insight into daily life with SMA and the ongoing medical challenges. This information can be used by healthcare providers to better define the standard of care, and target interventions for the greatest impact.

- Revealing aspects of SMA that are poorly understood, leading researchers to develop new tools, measurements and screening methods to better understand and measure these aspects.

Exploring Patient and Family Experience

In early 2016, we expanded our ongoing data collection efforts by launching a survey tool for those newly diagnosed with SMA. The goal of this survey is to increase our understanding of SMA at the time of diagnosis.

Though participation in this survey is voluntary, we encourage all families to complete it within six months of diagnosis. Towards the end of 2016, we will announce a second survey targeted toward individuals and families not newly diagnosed. Participants will be asked to update their information on a yearly basis. Our goal is to create a database that will demonstrate the impact of SMA over time.

By sharing your story and how SMA has impacted your lives, you will help the scientific and research communities create answers that address these real-world concerns, and accelerate therapy development for SMA.

Past Data Collection Efforts to Share our Community’s Experience

Particularly over the past three years, Cure SMA has been working on a number of fronts to ensure our community’s experiences are properly represented. These include:

We completed a survey project consisting of focus groups and interviews with 96 participants including: 21 with individuals with SMA; 64 parents of individuals affected by SMA; and 11 clinicians who specialize in the care of SMA patients. The goal of this project was to document the perspective of individuals and families living with SMA and to assess whether the currently used trial outcomes are meaningful to patients.

Cure SMA, along with collaborators from Biogen and the SMA Foundation, used this survey information to publish a paper in the journal BMC Neurology, reporting on patient and family experiences. In addition, a second paper from these focus groups is currently under review for journal publication. The second paper will focus on defining meaningful change in drug development, and determine critical parameters for measuring change from the patient perspective.

Parent Project Muscular Dystrophy (PPMD) and Cure SMA collaborated in two separate survey projects on clinical trials. One project focused on individuals whose children have participated in a clinical trial, including their motivations, hopes, and communication. The second focused on individuals whose children have not participated in a clinical trial, and the reasons for this lack of participation.

We brought the Voices of SMA project before key FDA leaders. Last year, we called upon our community to submit their experiences of living with SMA. Guided by themes identified through various data collection projects, we created The Voices of SMA, a booklet summarizing and highlighting these key themes through selected excerpts from your stories. This booklet, along with every submitted story, was distributed to all the top decision-makers at the FDA. As part of the PFDD (patient-focused drug development) initiative, these stories will influence the review process when an SMA drug is brought forward for approval.
At the December 2015 meeting, Cure SMA testified before FDA. Below is a portion of that testimony, which also included introductory remarks on SMA and Cure SMA.

Cure SMA will continue to actively participate in these PDUFA meetings and advocate for the issues of importance to our community.

Cure SMA Testimony Before the FDA

“We strongly urge the FDA to ensure that user fee agreement funds are utilized to strengthen and incorporate the patient voice throughout all stages of the drug development and approval processes, including increased representation on FDA Advisory Committees. The FDA’s inclusion of patients with rare diseases on Advisory Committees and Panels will ensure that the interests of patients with rare diseases are adequately represented.”

“Additionally, we support the inclusion of the patient’s viewpoint throughout the drug development process. There must be an increased emphasis to incorporate the patient voice earlier in the development process. The FDA also must actively strive to understand the needs of SMA patients across types and the disease’s impact on parents and caregivers.”

“We also are hopeful that strong incentives are maintained for orphan drugs. We support the Orphan Products Grants Program and the expansion of the program to allow for additional funding for clinical research which tests the safety and efficacy of drugs in rare diseases or conditions.”

“We must also ensure that the user fee agreement provides adequate funding for the FDA. Ample funding helps to facilitate the drug review process. Moreover, sufficient funding will allow for expedient review of drugs and biologics.”

“We also strongly support the FDA’s hiring of internal experts on orphan diseases. The Food and Drug Administration Safety and Innovation Act, signed into law in July of 2012 added a provision that included consultation with external experts on rare diseases, and the 21st Century Cures Act would lift certain salary caps at the FDA, which would encourage the hiring of experts in rare disease areas. However, should this legislation fail to come to fruition, we urge this issue be addressed in PDUFA reauthorization legislation.”

“In conclusion, on behalf of Cure SMA, thank you for allowing us to present our views on the reauthorization of the Prescription Drug User Fee program. We look forward to continued discussions as the process moves forward.”
Clinical Trial of CK-2127107 in Patients with Spinal Muscular Atrophy

CK-2127107 is a novel fast skeletal muscle troponin activator being developed as a potential treatment for people living with SMA and certain other debilitating neuromuscular and non-neuromuscular conditions associated with muscle weakness and/or muscle fatigue. CK 2127107 is liquid form taken twice a day by mouth.

This first clinical trial of this investigational drug in patients with spinal muscular atrophy (SMA) is a Phase 2, double-blind, randomized, placebo-controlled, multiple dose study of the investigational agent, CK-2127107, in patients with SMA, Types II, III, or IV, ages 12 and older. It is designed to assess the effect of 8 weeks of dosing of CK-2127107 on measures of muscle function and fatigue in both ambulatory and non-ambulatory patients with SMA. The plasma concentration of CK-2127107 will be measured at selected time points and the plasma concentrations obtained may be used to understand the response to different concentrations. The clinical trial is sponsored by Cytokine, a biopharmaceutical company in South San Francisco, California, in collaboration with its partner Astellas, a pharmaceutical company in Tokyo, Japan.

In this clinical trial, eligible patients with SMA will randomly be assigned to receive either CK-2127107 or placebo, and neither the participant nor the site Investigator will know which study treatment the patient is receiving. The primary objective of the study is to evaluate measures of skeletal and respiratory muscle function. In addition, safety, tolerability and levels of CK-2127107 will be monitored.

The following sites are currently recruiting patients. As additional sites are added this list will be updated on www.clinicaltrials.gov:

United States, California
Pediatric Neuromuscular Clinic Stanford University
Palo Alto, California, United States, 94304
Contact: Angelica Martinez, (650) 725-4341, ammarti1@stanford.edu

United States, Kansas
University of Kansas Medical Center
Kansas City, Kansas, United States, 66160
Contact: Ayla McCalley, (913) 945-9937, amccalley2@kumc.edu

United States, Maryland
Johns Hopkins Hospital Institute for Clinical and Translational Research Pediatric Clinical Research Unit
Baltimore, Maryland, United States, 21287
Contact: Anges Kind Rennie, (443) 287-6294

Cure SMA Releases 2016 Updates from Roche / Genentech

At the Annual SMA Conference, representatives from programs Roche/Genentech gave an update on Olesoxime:

Roche and Genentech are committed to addressing the urgent and unmet needs of the SMA community for an approved therapy. We are working to bring treatment options to people and families affected by the disease as soon as possible. We are currently evaluating the clinical development of two investigational medicines with different approaches in SMA – olesoxime and an SMN2 splicing modifier.

Since March 2015, Roche has been assessing and advancing the clinical development and manufacturing of olesoxime to meet health authority requirements and to hopefully gain market authorization to provide olesoxime to patients with SMA.

Roche has also consulted the US and EU health authorities on the adequacy of the clinical evidence currently available for olesoxime to establish the benefit/risk profile of this investigational medicine for the treatment of SMA. As a result, Roche will be conducting a Phase 3 study of olesoxime in people with Type 2/3 SMA. We will start the study as soon as possible.

People and families affected by SMA remain important partners to Roche and Genentech; we will continue to provide timely and relevant updates.
In early 2016, Global Genes published, “From Molecules to Medicines: How Patients Can Share Their Voices Through the Drug Development Process.” This toolkit was the product of a collaboration between Global Genes and leaders in advocacy and government, including Cure SMA. Work from Cure SMA was used as case studies in five different areas: encouraging research, funding research, providing data, participating in trials, and participating in committees.

The world of drug development is rapidly changing, and one of the most fundamental changes is the increased participation of patients, their families, and their caregivers throughout the process. Historically, pharmaceutical companies and regulatory agencies included participation from patients at limited points during drug development, most often when a drug was nearing approval. Patients’ and caregivers’ knowledge was not routinely included at key points such as targeting early research, assessing the benefits and risks of new therapies, developing regulatory guidelines, or designing clinical trials.

However, this is changing. This new toolkit, which is part of a series on drug development, highlights how the patients—including those in the SMA community—are using their voices to drive conversations, research and drug development to yield significant advances for their disease communities.

Thank you to our community, whose dedication and persistence have become an example for the rest of the advocacy world to emulate. Our thanks also to Global Genes and the other collaborators on this project, for inviting us to be part of the important work being done in rare disease advocacy.

SAVE THE DATE!

2017 Annual SMA Conference
which will be held in Disney World, Orlando, FL June 29 – July 2, 2017!

If you are interested in being added to our mailing list to receive exhibitor opportunities for the 2017 Annual Conference, please email exhibitor@curesma.org.
Sharing Photos

Izabelka Kostan

Kalen and Kyan Kulas

Kate Veit

Layla Villamor

Lillian Lee

Lily Parlier

Louie Rascon
Cure SMA Announces $2.5 Million in New Planned Research Funding

At the 2016 Annual SMA Conference, Cure SMA announced $2.5 million in new planned research funding over the next 12 months. This funding will be used strategically to help accelerate research and ensure we are developing treatments for all types, ages, and stages of SMA.

Funding Priorities

As the SMA research landscape has developed and the drug pipeline has grown, we recently undertook a systematic review of our research funding priorities. Through conversations with independent SMA experts, our scientific advisory groups, and the newly formed Medicine and Science Committee in our Board of Directors, Cure SMA has created a strategic research plan to guide us into this next phase of SMA research. This strategic research plan identifies the areas of greatest need and where we are best positioned to make a significant difference.

Continued Funding for Basic Research

Basic research investigates the causes and biology of SMA, often revealing more effective ways of making SMA drugs. Continued funding in basic research will help address questions about survival motor neuron (SMN) protein, which is not produced properly in the bodies of those with SMA, and help us identify other systems, pathways and processes that are affected in SMA.

This research could then lead to the development of combination therapies for SMA, using both our knowledge of SMN protein and our knowledge of these other systems, pathways and processes. Approaches that work on these other areas could be used in combination with approaches that work on SMN levels, allowing us to develop treatments for all types, ages, and stages of SMA.

Greater Funding for Clinical and Regulatory Research

As more SMA drug programs progress through clinical trials, there is an increasing need for us to address clinical and regulatory issues and bring the patient voice into the process. Funding for this area will be directed toward several critical projects:

- Developing regulatory approaches to patient-focused drug development
- Defining clinical meaningfulness and risk/benefit in ways significant to our community
- Holding a Voice of the Patient Meeting with the FDA
- Standardizing and training for clinical trial protocols across sites
- Educating and engaging physicians, caregivers and patients

Many of these projects will be carried out as part of a new collaborative industry consortium. Through this group, seven companies working in SMA drug development will share
information, ideas, and data, working together on projects that will benefit our community.

**Greater Funding for Patient Care Initiatives**

Cure SMA has been working for the past several years to collect data and information on the experiences of living with SMA. Earlier this year, we expanded our ongoing data collection efforts by launching a survey tool for those newly diagnosed with SMA. In the next several months, we will announce a second survey targeted toward individuals and families not newly diagnosed.

A portion of our research funding for the coming year will be used to gather these responses and create a database that will demonstrate the impact of SMA over time. This information will help the scientific and research communities create answers that address these real-world concerns, and accelerate therapy development for SMA.

The increased funding will also be used to help develop centers of excellence for SMA. Information from multiple SMA care centers will be used to create evidence regarding the best care for all those affected by SMA.

**Thank You**

The dedicated support of our community has made all of this possible. Whether families raising funds and awareness, researchers investigating new potential treatments, or pharmaceutical and regulatory partners helping us take the next steps toward approval—everyone has a role to play. Thank you to everyone for their hard work and dedication.

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**Cure SMA Releases October 2016 Update to the SMA Drug Pipeline**

In October 2016, we released an update to the SMA drug pipeline. This latest version includes:

- 18 active programs.
- 14 pharmaceutical partners.

- 6 programs in clinical trials.
- 28 programs in the cumulative pipeline total, including 10 failures to date.
- An ever-increasing breadth of potential treatment approaches to SMA.

**Effective Treatments for All Ages, Stages and Types of SMA**

As the SMA drug pipeline has expanded and grown in diversity and complexity, the need for multiple therapeutic approaches and combination therapies has become increasingly apparent.

Individuals with SMA don’t produce survival motor neuron (SMN) protein at high enough levels due to a mutation in the survival motor neuron 1 (SMN1) gene. Much of the early research into SMA has focused on increasing SMN production, either by replacing SMN1 or by modulating SMN2, the low-functioning SMA “backup gene.”

Many of the programs in the pipeline rely on these SMN-based approaches, including gene therapy and antisense oligonucleotides. And as the pipeline shows, a number are already being tested in clinical trials. In future, these or other different approaches to increasing SMN levels could be used alone or together.

As research has progressed, it has also revealed that a number of systems, pathways and processes are affected in SMA, and there may be additional ways to treat SMA that work on these other areas. And perhaps most crucially, these other approaches could be used in combination with approaches that work on SMN levels, allowing us to attack SMA from all sides and giving us the best chance of a comprehensive, effective treatment. This is particularly important as we seek to develop treatments for all ages, stages and types of SMA.

In just the last year, two new SMA programs have been added to the pipeline, both pursuing combination therapies. Both programs are funded by grants from Cure SMA.

The results are impressive, but we still have more work to do. Only 10% of the drugs that make it to clinical trials will ultimately receive FDA approval, so we need to keep building and growing the pipeline. Beyond funding the pipeline, Cure SMA is committed to advocating for the patient voice in drug development to ensure regulators understand the SMA community and its needs.

**Thank You**

The dedicated support of our community has made all of this possible. Whether families raising funds and awareness, researchers investigating new potential treatments, or pharmaceutical and regulatory partners helping us take the next steps toward approval—everyone has a role to play. Thank you to everyone for their hard work and dedication.
Dr. Chris Lorson and his colleagues have published a paper, “Optimization of Morpholino Antisense Oligonucleotides Targeting the Intrinsic Repressor Element1 in Spinal Muscular Atrophy,” in the journal Molecular Therapy.

The University of Missouri-based team is investigating ways to target SMN2, the SMA “backup gene.” Because of a genetic mutation in the SMN1 gene, individuals with SMA don’t produce survival motor neuron protein (SMN protein) at high enough levels. However, all individuals with SMA have at least one copy of SMN2, which typically produces a small amount of functional SMN protein. Lorson’s compound targets SMN2 and effectively “turns the volume up” for SMN2, allowing it to make more of the correct SMN protein.

“Our current treatment helps the body create a backup mechanism to combat the disease and extends survival in mice with SMA from just 13 days to a little over five months after only one injection at birth,” Lorson said. “This treatment helps produce the right form of SMN, the one that was only produced at very low levels before.”

In 2013, Cure SMA made a $150,000 grant to Dr. Lorson and Dr. Arthur Burghes, jointly, to fund research into this potential treatment approach. “Cure SMA was instrumental in providing the initial funding for this project. At the time, our lab knew that optimization of the sequence for our ASO was important, however, funding was not available to develop or test these new compounds. Cure SMA provided funding that allowed us to identify the optimized E1 ASO compound, screening a new panel of sequences, leading to the identification of our current lead candidate that can extend survival in SMA mice nearly 10-fold. Additionally, this work is exciting because it brings another type of chemistry to the fight against SMA – Morpholino chemistry. We are excited to continue to push this work closer to the clinic and we are grateful for the early stage funding received by Cure SMA and the families that support the SMA community,” said Lorson.

“We’re very excited about these results and the continued progress of this program,” said Jill Jarecki, PhD, Cure SMA’s chief scientific officer. “We know also that the entire SMA community shares in our excitement at seeing another promising treatment continue to move forward. Dr. Lorson’s project is part of a multi-prong strategy to attack SMA from all sides, looking at treatments that address the underlying genetics, including the SMN2 gene, and at treatments that protect the muscles and nerves. We eventually hope that these drugs can be used in combination to provide the best possible therapeutic effect.”

Dr. Lorson likewise agrees that it is unlikely a single compound will address the full gamut of symptoms. However, by combining therapies currently being researched, a better prognosis could be on the horizon, Lorson said.

The early-stage results of this research are promising. If additional studies are successful within the next few years, these compounds may be tested in human clinical trials with the hope of developing new treatments for SMA.
Sharing Photos

Briahanna Johnson

Bridget, Maeve and Shannon Abraldes

Brielle and Brooke Kennedy

Carolyn Barrett

Carter and Kyle Nunemaker

Christopher Weber

Emma Rubenstein

Eleanor, Jack and Robert Bolton

Evan Vaudry

Jake, Katherine and Rachel Saxton

Sydney Utzar
Cure SMA-funded researcher Peter Schultz has been awarded a four-year NIH grant of just over $2 million to support his research into small molecules to treat SMA. Cure SMA has funded the work of Dr. Schultz and his team at the California Institute of Biomedical Research (Calibr) through three drug discovery grants in 2012 and 2015, with the total amount of funding approaching $1 million.

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

One very promising therapeutic approach is small molecules, which are chemicals that can treat or cure a disease. The Schultz group previously discovered several different compound classes that enhance SMN protein levels. Our funding to Calibr has been focused on optimizing these compound classes.

With this experience in hand, Dr. Schultz and his team have been able to secure a new NIH grant. With the additional funding from the NIH, he will be able to carry on his work on identifying and developing small molecule drug candidates for SMA.

The Importance of Leverage

It can cost upwards of $1 billion to bring a therapy through the development process to FDA approval. This level of investment requires collaboration between academics, industry, government, and families. Cure SMA brings all of those groups together, breaking down roadblocks that might delay important developments.

One of our most powerful tools in breaking down those roadblocks is our “seed funding” approach. We provide early-stage funding for promising new ideas and novel treatment approaches. In doing so, we lower the risk and attract larger investments from industry, government, and other funding sources as drug candidates move through the process.

In addition, we are working to increase the number of new therapies in the SMA pipeline, to insure we have treatments for all ages and stages of SMA. Attracting investment from government, pharmaceutical companies, and other organizations provides the resources necessary to accomplish these goals.

The NIH grant to Calibr is another example of the impact of this seed funding strategy, along with other recent examples in gene therapy, antisense oligonucleotides, and muscle activators.

Our thanks to Dr. Schultz and the team at Calibr for all their hard work on this program. In addition, our thanks to the many generous families and donors who have supported the Cure SMA grants that have allowed us to leverage new investments in SMA research.

Funding for the Calibr program is supported by a special gift from The Michael and Chandra Rudd Foundation.
More About How Seed Funding is Yielding New Investments in Research

Gene Therapy

Through a series of grants, Cure SMA invested $845,000 in two different gene therapy approaches. In 2013, the National Institute of Neurological Disorders and Stroke (NINDS) awarded Dr. Brian Kaspar a $4 million grant, based on data generated with Cure SMA funding.

The technology for this gene therapy was then licensed to a company for clinical trials. Earlier this month, AveXis held an initial public offering (IPO), raising over $95 million from investors to move gene therapy into Phase 2 clinical trials.

Antisense Oligonucleotides (ASOs)

An ASO is a small snippet of synthetic genetic material used to modulate gene expression. In 2013, we made a $150,000 grant to Dr. Chris Lorson and Dr. Arthur Burghes to help optimize an ASO that binds to a novel sequence called E1. The E1 ASO could be used to improve the function of SMN2, the SMA “backup gene.”

Last year, Dr. Lorson announced that they were able to optimize the E1 directed ASO that increased life span in severe mouse models of SMA by four times and in intermediate mouse models of SMA by seven times. Based on that data, Dr. Lorson has recently received two additional grants, totaling $550,000, from MDA, GSF, and FightSMA, to continue moving this compound forward.

Muscle Activators

In 2013, Cure SMA made a grant of $100,000 to Dr. Jeff Jasper at Cytokinetics. This grant was intended to support the development of a small molecule that protects and activates skeletal muscles that typically weaken in SMA.

In 2014, Astellas Pharmaceuticals announced a partnership with Cytokinetics to bring this drug class (specifically CK-2127107) to clinical trials for SMA. CK-2127107 is currently being tested in Phase 2 clinical trials in teens and adults with SMA, and Astellas has invested $55 million in order to move the drug to this point.

Cure SMA Recognizes Judith Melki on the Anniversary of the Discovery of the SMN Gene

During the 2016 SMA Researcher Meeting, the anniversary of the identification of the SMN genes by Judith Melki MD, PhD (Inserm, Universite Paris) was recognized.

Genetic studies of families with a history of SMA allowed researchers to localize the region containing the gene responsible for SMA to the long arm of chromosome 5 in 1992. However, the exact gene missing or mutated in SMA patients was not identified until 1995 when Dr. Melki’s team identified the SMA gene through a technique called positional cloning. This gene, termed Survival Motor Neuron (SMN) gene, was shown to be present on chromosome 5 in two nearly identical copies. These two copies have subsequently been named SMN1 and SMN2, the SMA “backup gene.”

Knowing the SMA-determining gene paved the way for continued SMA research and the development of animal models to study the disease. These advances have ultimately led to the development of potential therapies currently in clinical trials. To honor her monumental achievement, Dr. Arthur Burghes (Ohio State University) presented Dr. Melki with a plaque on behalf of Cure SMA.
An important goal of our research funding strategy is to share scientific findings with the broader scientific community. To help accomplish this goal, scientists who receive Cure SMA funding often publish their findings in peer-reviewed journals. Published articles allow the experiments and results to be reviewed and vetted by other scientists, who may then use these as the basis for further experiments—multiplying the impact of our funding.

In 2015, Cure SMA-funded research led to the publishing of 18 journal articles, by researchers from 15 different institutions. Journal publishing is competitive, and only the best and most intriguing results are published. Congratulations to all these authors on their accomplishment. Thank you for helping us move SMA research forward.
2015 Cure SMA-Funded Journal Articles


17. SMN deficiency does not induce oxidative stress in SMA iPSC-derived astrocytes or motor neurons. Patitucci TN, Ebert AD. Hum Mol Genet. 2015 Dec 7. pii: ddw489. [Epub ahead of print]

As the SMA drug pipeline has grown in breadth, depth, and sophistication, the need for combination therapies has become increasingly apparent. Individuals with SMA don't produce survival motor neuron (SMN) protein at high enough levels due to a mutation in the survival motor neuron 1 (SMN1) gene. Much of the early research into SMA has focused on increasing SMN production, either by replacing or correcting SMN1 or by modulating SMN2, the low-functioning SMA “backup gene.” Many of these SMN-based approaches (also called “SMN-enhancing” approaches), such as gene therapy and antisense oligonucleotides, are already being tested in clinical trials.

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

And perhaps most crucially, these non-SMN approaches could be used in combination with SMN-enhancing approaches, allowing us to attack SMA from all sides and giving us the best chance of a comprehensive, effective treatment. This is particularly important as we seek to develop treatments for all ages, stages and types of SMA.

How We’re Working to Advance Treatments for All Ages, Stages and Types of SMA
This most recent round of drug discovery funding reflects the increasing importance of combination therapies. Grants totaling $604,000—$300,000 to Dr. Charlotte Sumner in collaboration with Imago Pharmaceuticals, and $304,000 to Dr. Livio Pellizzoni—will support investigation into non-SMN treatments that could eventually be used in combination with SMN-enhancing treatments.

The remaining $100,000—$50,000 each to Dr. Barrington Burnett and Dr. Kevin Hodgetts—will support investigation into SMN-enhancing treatments that work in different ways than the SMN-enhancing treatments currently being studied. This could provide yet another avenue for potential combination therapies: the use of two different SMN-enhancing approaches together to provide a stronger overall effect.

$300,000 to Charlotte Sumner, in Collaboration with Imago Pharmaceuticals
Jun N-terminal kinase (JNK) is a stress-activated enzyme (an enzyme is a special kind of protein capable of producing specific chemical changes in cell) that is known to be activated in many neurodegenerative diseases, perhaps including SMA. When activated, JNK may cause motorneurons to function improperly and die. It may also cause muscles to atrophy in SMA.

Imago Pharmaceuticals along with Dr. Sumner, have developed compounds that inhibit JNK and therefore protect neurons and muscle. The goal of this project is to test these compounds in SMA animal models to see if they improve survival, motor function, reduce neuron loss, and/or improve muscle function, both alone and in combination with SMN enhancers. The safety of these compounds will then be tested.

If these JNK inhibitors are safe and work in SMA animal models to protect either neurons and/or muscle, they will progress into studies required by the FDA to support human clinical trials.
$304,000 to Livio Pellizzoni, PhD, at Columbia University

Dr. Pellizzoni and his colleagues at Northwestern University have identified a novel cellular pathway, called p38α MAPK, that is altered in SMA and may directly contribute to how the disease develops.

For instance, SMN deficiency results in activation of p38α MAPK in mouse models of SMA. The goal of this project is to evaluate whether inhibiting this pathway may help treat SMA. An orally available p38α MAPK inhibitor is currently in advanced clinical trials for the treatment of Alzheimer’s disease and other neurological disorders. Researchers will be testing this compound in a mouse model of SMA, to see if it might also be useful in treating SMA.

The results of this project may be used to support further pre-clinical and clinical development of this drug for use in SMA.

$50,000 to Kevin Hodgetts, PhD, at The Lab for Drug Discovery in Neurodegeneration at Brigham and Women’s Hospital

Another potential way of increasing SMN levels is to increase transcription of the SMN2 gene. Transcription is the process by which the information in DNA is copied into messenger RNA (mRNA) for protein production. This process can be thought of in terms of an engine, where increasing or decreasing transcription is akin to turning a gene on or off. Dr. Hodgetts and his team are investigating two distinct series of chemical platforms, to see if they might be used to increase SMN2 transcription.

The objective of this project is to optimize a compound that already increases transcription of SMN2 to have more drug-like properties suitable for pre-clinical evaluation. They will develop acceptable formulations and improve solubility of the lead compounds to enable them to be administered more easily for mouse model efficacy studies. Dr. Hodgetts and his team are working in collaboration with Elliot Androphy’s lab. Dr. Androphy was first to identify the compounds now being studied.

$50,000 to Barrington Burnett, PhD, at Uniformed Services University of the Health Sciences

Another potential way of increasing SMN levels is to target the SMN protein directly. Dr. Burnett and his team are investigating ways to slow the degradation of SMN protein, causing it to stay around for longer length of time and effectively increasing the overall levels of SMN protein in cells.

The goal of this project is to characterize and validate a novel SMN protein modulator for possible treatment of spinal muscular atrophy. This modulator regulates the degradation of the SMA protein. The team will utilize cell-based assays and animal models to investigate safety, efficacy, and selectivity of a new compound identified using a high throughput screen that modulates SMN protein degradation. The project aims to help develop molecules that possess a unique mode of action to treat SMA.
Clinical Care Research Grants

Cure SMA Announces $100,000 in Clinical Care Research Grants

In addition to funding basic research, drug discovery, and clinical trials, Cure SMA funds research in a fourth area: clinical care. The purpose of clinical care research is to understand the issues that affect daily life for people with SMA, from breathing to nutrition, and to improve their quality of life today.

During this past year, we have announced $100,000 in clinical care funding to support two new pilot studies.

Pilot studies are often a first step when testing a new or innovative treatment option. They help researchers evaluate the feasibility of larger studies, and pinpoint ways that they can modify their approach in order to produce the most meaningful results. This information is then used to support larger and lengthier studies.

$50,000 to Melissa Alderfer, PhD, Alfred I. DuPont Hospital for Children

Cure SMA awarded a $50,000 clinical care research grant to Melissa Alderfer, PhD, at the Alfred I. DuPont Hospital for Children, for her project, “Screening for Psychosocial Risk among Families of Children With Spinal Muscular Atrophy.”

Dr. Alderfer is studying a psychological assessment screening tool. The goal of her research is to adapt this tool for use with families affected by SMA to identify potential stressors and resources within the family for coping with difficult treatment choices, fatigue and stress, limitations on independence, uncertainty, financial hardship, and other factors that often accompany a diagnosis like SMA.

Dr. Alderfer’s project is focused on improving how these psychosocial difficulties are identified and treated, in order to ensure that the needs of those affected by SMA are properly met.

$50,000 to Tariq Rahman, PhD, Alfred I. DuPont Hospital for Children

Cure SMA awarded a $50,000 clinical care research grant to Tariq Rahman, PhD, at the Alfred I. DuPont Hospital for Children, for his project, “Outcome Measures Using WREX – An Upper Extremity Exoskeleton for Children with SMA.”

An exoskeleton is a robotic system that can help support movement and/or improve range of motion for individuals who have conditions that cause muscle weakness, including SMA. This technology could assist individuals with SMA in performing activities of daily living, or help address common complications, such as contractures (a shortening of a muscle or joint).

This project will evaluate one particular exoskeleton, the WREX, to see if it provides benefit for those with SMA.
Basic Research Grants

Cure SMA Awards 10 New Grants in Basic Research

Basic research is the first step in our comprehensive research model. We fund basic research to investigate the biology and cause of SMA, in order to identify the most effective strategies for drug discovery. We also use this funding to develop tools that facilitate SMA research.

Without basic research, the SMA drug pipeline would not continue to grow and diversify. We need both a breadth and a depth of options in our quest for an effective SMA therapy. Basic research is our investment in future drug development for SMA.

Broadening the Drug Pipeline and Developing Combination Therapies

Because of a genetic mutation in the survival motor neuron gene 1 (SMN1), individuals with SMA don’t produce survival motor neuron protein (SMN protein) at high enough levels, causing motor neurons to shrink and eventually die. Prior basic research projects uncovered the SMN1 gene and the link to SMN protein. However, there are still many unanswered questions about the SMN protein. By funding research into these unanswered questions, we can develop new treatment strategies that will add to the breadth of the SMA drug pipeline, as well as develop combination therapies. A broad pipeline is particularly important as we seek to develop treatments for all ages, types, and stages of SMA.

New Grants to Broaden the Drug Pipeline

Our grant to Dr. Christine Beattie will fund research on how the loss of SMN protein affects the development of motor neurons, as well as other proteins and RNAs that might interact with SMN protein during the development of motor neurons.

Our grant to Dr. Arthur Burghes will provide resources to look at the cellular function of SMN protein, hoping to learn more specifically about what SMN does to a cell to help it function.

Dr. Jocelyn Cote has discovered that SMN plays a critical role in the regulation of protein production, called “translation.” Our grant will help further investigate the impact this has on the body.

Through a new and unique mouse model, Dr. Christine DiDonato has discovered that increasing SMN in the central nervous system does not completely alleviate the symptoms of SMA, suggesting there are also defects in the skeletal muscles. Her grant will fund additional work with this mouse model to better understand where and how SMA therapies should be targeted.

Our grant to Dr. Rashmi Kothary’s will support investigation into how SMN protein affects muscle satellite cells, which help the body respond to muscle damage.
Deepening the Drug Pipeline

All individuals with SMA have at least one copy of survival motor neuron gene 2 (SMN2), which is often called the SMA “backup gene.” SMN2 also produces SMN protein, but most of the protein produced by SMN2 is lacking a key piece, called exon 7.

Of the 18 treatments currently in development, half are focused on modulating this SMN2 gene—either by prompting SMN2 to make more protein, or by fixing the splicing of SMN2, meaning that SMN2 could produce a complete protein.

This new round of grants includes several projects that will further investigate this therapeutic approach as well more novel ones, thereby deepening our knowledge of a promising treatment avenue.

New Grants to Deepen the Drug Pipeline

Our grant to Dr. Antoine Clery will fund a project investigating different molecules that might affect the splicing of SMN2.

Axons are long projections that grow out of motor neurons, and send information to other neurons or muscles. Our grant to Dr. Charlotte Sumner will fund research into how and when motor axons are affected by the loss of SMN protein, and when in development axons can be fixed by oligonucleotides, which are small snippets of synthetic genetic material that bind to ribonucleic acid (RNA) to correct the splicing of SMN2.

Dr. Megerdith Kiledjian has identified a previously unknown protein variant that could lead to increases in SMN2 mRNA and SMN protein. His grant will fund additional work to understand this protein variant.

More About Our New Basic Research Grants

$140,000 to Dr. Antoine Clery, PhD, Ohio State University

Dr. Burghes’ project will look closely at the cellular function of SMN protein, hoping to learn more specifically about what SMN does to a cell to help it function.

Cure SMA Awards $30,000 Grant to Jocelyn Cote, PhD, University of Ottawa

Dr. Cote and his team have discovered that SMN plays a critical role in the regulation of protein production, called “translation.” This grant will allow them to further investigate the impact this has on the body. Understanding how the loss of SMN protein affects translation, along with other important processes, could reveal more about where and when the protein is needed in order to effectively treat SMA.

The grant to Dr. Cote is funded by Families of SMA Canada.

$70,000 to Dr. Christine DiDonato, Lurie Children's Hospital of Chicago

Through work with a new and unique mouse model, Dr. DiDonato and her team have discovered that increasing SMN in the central nervous system does not completely alleviate the symptoms of SMA, suggesting there are also defects in the skeletal muscles that may need to be addressed. This grant will fund further work with this mouse model to better understand where and how SMA therapies should be targeted.

$140,000 to Rashmi Kothary, MD, Ottawa Hospital Research Institute

By learning more about the different ways that loss of SMN protein affects the body, researchers may be able to identify new ways to treat SMA, or new ways to evaluate whether potential treatments are effective.

Dr. Kothary’s project will look at how SMN protein affects muscle satellite cells, which help the body respond to muscle damage.

The grant to Dr. Kothary is funded by Families of SMA Canada.

More About Our New Basic Research Grants

$140,000 to Christine Beattie, PhD, Ohio State University

Dr. Beattie's project will look specifically at how the loss of SMN protein affects the development of motor neurons, and at other proteins and RNAs that might interact with SMN protein during the development of motor neurons.
$90,000 to Antoine Cléry, PhD, ETH Zurich

Individuals with SMA have one or more copies of SMN2, the SMA “backup gene.” SMN2 produces SMN protein, but most of the protein produced by SMN2 is lacking a key piece, called exon 7.

Dr. Cléry’s project will investigate different molecules that might affect the splicing of SMN2. If the splicing of SMN2 is corrected, then it would be able produce higher amounts of complete SMN protein.

$140,000 to Charlotte Sumner, MD, Johns Hopkins University

Axons are long projections that grow out of motor neurons, and send information to other neurons or muscles. In individuals affected by SMA, these axons appear to be damaged early on in development by the loss of SMN protein caused by the mutation in the SMN1 gene.

Dr. Sumner’s project will look at how and when these axons are affected, and whether those affects can be fixed by oligonucleotides. Oligonucleotides are small snippets of synthetic genetic material that bind to ribonucleic acid (RNA). In SMA, they can be used to fix the splicing of SMN2, the SMA “backup gene,” meaning that SMN2 would be able to make a complete SMN protein. One example of this is the drug nusinersen, being developed by Biogen and Ionis Pharmaceuticals.

$140,000 to Megerditch Kiledjian, PhD, of Rutgers

Researchers believe that prompting SMN2 to make more protein could be an effective way to treat SMA. Dr. Kiledjian and his team have identified a previously unknown protein variant that could lead to increases in SMN2 mRNA and SMN protein. Understanding the basis for this could help better understand how the SMN gene is turned on and off and identify new targets for SMA drug development.
Loving Memories
This section is designed so it can be removed from the center of the newsletter.
It was January 29, 2016. I was sitting in a room, holding Alexa Kate in my arms and waiting on the pediatric neurologist to return. He had just completed a electromyography / nerve conduction study on her. She handled it like a pro. She patiently sat in my lap while the doctor performed the test. She didn’t cry; she smiled. I nursed her and her twin brother, Grant, afterward. We waited some more. Finally, the doctor returned. He walked in and spoke…”99% chance she has SMA, Spinal Muscular Atrophy, Type 1. She will never walk or talk or even crawl. She may not make it to see her 1st birthday, much less her 2nd.” I looked down at her and she smiled at me again. The room flooded with various medical staff. “This will be a catastrophic event for your family.” I glared at the doctor and screamed, “She’s going to die?” I was told to “go home and do my own research.”

My mom snuggled Grant back into his car seat. We both got up, and I carried Alexa Kate in my arms to the car. I don’t remember much of the drive back home. I do remember the anger and physical ache that came over me. The dread I felt having to call my husband and tell him her diagnosis was daunting.

January 29, 2016: Alexa Kate and Grant are 7 months old today.

I called my husband and asked him to come home from work. He knew that I knew. He immediately asked me to tell him her diagnosis over the phone. I refused. When he got home, I told him in the garage. He cried as the words left my mouth. In 10 years of marriage I had never seen him cry, much less sob. As we stood there, I told him that the diagnosis didn’t matter. She was loved. Alexa Kate was loved. She was our joy. She was happy. We would give her the best life possible.

We decided, from that moment forward, SMA was NOT going to define Alexa Kate or our family. We were not SMA. We were a happy family blessed with five precious children. Children we wanted more than anything. Children for whom we would do anything. And a sweet baby girl whom we would not be able to save, no matter what course of action we took.

Alexa Kate spent the next 3 months living life. She was never admitted to the hospital. She was never “sick”, requiring medical attention. She was not SMA. She loved life. She loved her family. Her eyes would light up at the sight of her big sisters, big brother, and baby brother. She breathed, she nursed (every single day), she smiled, she laughed, she chattered, and she had eyes as blue as the ocean water. She was perfect. She was our baby. She was pure JOY.

Alexa Kate’s life was short, much too short. However, we are better for knowing her, for loving her, and for having her. God gave us Alexa Kate. We cherished every minute with her. I would STILL choose Alexa Kate. I would STILL want Alexa Kate, even knowing that she would be born with a terminal genetic disease. I would STILL choose her. I still want her. And I will see her again one day. God’s promises are true.

Spinal Muscular Atrophy is the number one genetic cause of death in infants. SMA affects approximately 1 in 10,000 babies, and about 1 in every 50 Americans is a genetic carrier. We have no family history of SMA. We were not even aware that SMA existed. It does. SMA is real. It can happen and does happen. Live your life as if there is no tomorrow. Please remember Alexa Kate and the joy that she brought to all of us. SMA must be cured!
Tambryn's light shined so bright for her Lord and Savior that she touched the hearts of everyone she met. Through those that loved her dearly, her light will continue to shine bright and her works on earth will be remembered. We were so blessed to have her as our daughter and sister. Tambryn will forever be in our hearts.

Dance and praise God with Bubba at your side until we see you again our love.

Visit Tambryn's website to see a glimpse into our lives with dealing with SMA www.caringbridge.org/visit/tambryncampbell

Every year during the month of August our family runs the Warrior Dash in Wisconsin and we wear our shirts that on the front say See you later Ali-gator for our daughter that had SMA and on the back says SMA Awareness!

This picture is of Baylor Cain (left) and Miles Wick (right). This is for Baylor’s memory since he recently passed away. We (the Wick family) have been blessed with another healthy baby that is only a carrier to SMA. His oldest sister (Ali) we lost to SMA in July 2012. It's a small world how SMA affects you and those around you since the mother of Baylor and I are friends and were co-workers even. She was there for us through everything with Ali and now in return we have been there for her and her husband as they had a child with SMA.

Thanks,
Keri Wick
On behalf of Gabriel Allen and our whole family, Thank You,

To all of you at Cure SMA, we thank you for your support during our son’s diagnosis & hospital stay. We got home with Gabriel’s snug ride & we got outside to our yard & our lake to show him the sun & trees & feel the breeze on his face. With your support we were able to enjoy these things during his short life. 64 days full of love. We follow Cure SMA’s research updates, full of hope that treatments will be found to help others diagnosed with SMA & their families live longer, quality lives.

Losing Gabriel is hard, something we struggle with daily. But remembering our gratitude for our time with him eases the hurt & helps us live with grace.

Sincerely,

Aaron Allen, Joanna Nigrelli & Eleanor

Cure SMA:
Thank you so much for the angel statue and in general, all of the support your organization has given our family since our son’s diagnosis. Having a baby diagnosed with a terminal illness is extremely overwhelming and you made it feel like we weren’t completely alone. Our hope is that all of the money donated in Zachary’s memory will go to help other families or find a cure.

Thanks Again,
The Sweets- Jason, Amy & Ben

In Memoriam
Gabriel Allen | June 24, 2015 – August 27, 2015

In Memoriam
Zachary Sweet

In Memoriam
Owen Johnson
Spinal muscular atrophy (SMA) is a disease caused by a mutation in the survival motor neuron gene 1 (SMN1). In a healthy person, this gene produces a protein critical to the function of the nerves that control our muscles. Without it, these nerve cells cannot properly function and eventually die.

There are four types of SMA that range in severity, but all of them cause difficulties with walking, performing the activities of daily life, eating and even breathing. In its most severe form, SMA is the number one genetic cause of death for infants.

There is currently no treatment for SMA, but a number of drugs are being tested in clinical trials, and one of these will soon be presented to the FDA for approval.

August is SMA Awareness Month, so The Mighty collaborated with Cure SMA, an organization dedicated to the treatment and cure of SMA that has led and invested in research, making many of today’s breakthroughs possible. We asked our communities: “What’s one secret you want others to know about having or caring for someone with SMA?”

This is what they said:

1. “You do not need to be afraid to be around us. We are still the same family we were before the diagnosis, only more fragile. Take precautions; don’t disappear. Ask us questions about our daughter. It does not hurt our feelings. Questions help spread awareness.” — Majerie S.

2. “These children are brilliant and can communicate in fascinating ways. Just because my son can’t move, doesn’t mean he can’t get his point across! He is 2 and tells me what he wants — down to what socks he wants to wear. Learn the way SMA children communicate and you will be blown away by how they can hold a conversation!” — Autumn J.

3. “It’s not the end of the world. I am 28 with SMA type 3 and have been quite successful in life so far as a teacher. Don’t let any disability or label define who you are. There is always another way.” — Megan E.

4. “I would want others to know that caring for someone with SMA is not a burden. It’s not an awful and sad life. We live our life feeling blessed and grateful for each day. My daughter has truly been the biggest blessing in the world.” — Shellie L.

5. “It’s the hardest and most rewarding job at the same time. They are extremely smart and bright. This is my daughter. If anyone can make you smile it’s her.” — Kelsy H.

6. “Living with SMA has taught our little family to learn to love a different kind of ‘normal.’ Yes, our daughters (both with type 2) cannot walk or move around on their own, but they are vibrant and full of life! They teach us every day to enjoy the moment and never limit the possibilities.” — Robin H.

7. “Don’t limit your expectations of what is possible for your child. I used to be surprised when my son or daughter would do something I never expected they’d be able to do. Now I watch and wait to see what they’ll accomplish next,
knowing there is no limit to their potential.” — Danielle S.

8. “My wish would be that the new drug that's supposed to help cure SMA be here sooner then later.” – Melissa D.

9. “Being a caregiver isn’t giving up your time to help a child grow, but taking time to grow alongside the child.” — Chelsea K.

10. “This disease does not define my husband. He is an individual who has spinal muscular atrophy. He is strong, loving, caring, and a wonderful father. SMA is a part of him but it does not define him.” — Adolfo P.

11. “Take time to get to know my son and he’ll steal your heart just like he did mine from that first moment I saw him.” — Chris O.

12. “Caregiver fatigue is real. Self-care isn’t selfish. Keeping active and keeping social is so important in caring for my hubby of 28 years who has SMA type 3.” — Alicia L.

13. “SMA takes away what dreams I ‘thought’ I had for my son, allowing me to see the dreams that my son has and will achieve.” — Bridget C.

14. “It’s not always visible. Don’t judge. Just because I can’t do something doesn’t mean I don’t want to do it. Don’t think I’m not working as hard as I possibly can to keep the strength that I still have. I won’t give up on myself and no one else should give up on me either!” — Courtney F.

15. “I would want others to know that they do not know the meaning of true happiness until they have met someone with SMA. My son faces many physical limitations, yet he is always happy and smiling, and making others happy as well. It is impossible to be sad around him.” — Renee H.

16. “Don’t worry about breaking our kids. They are pretty tough. Also, parents and caregivers need to take the occasional break!” — Kelsey L.

17. “I have developed an amazing, special bond with my daughter. Even though she is going to be 16 years old, I still can carry her, hold her tight, tickle her and love her.” — Ruthie P.

18. “My beautiful, loving, caring, daughter is 45 years old. She is and will always be the light of my life. She has good days as well as bad, but she just never complains.” — Margaret A.

19. “I am a 19-year-old with SMA. I have moved out of home and am currently attending college at RMIT in Melbourne, Australia. It’s definitely not a death sentence.” — Harrison K.

20. “Even with SMA being a terminal diagnosis, it has taught us more about life then death. It has taught us how to love deeper, forgive quicker and be thankful for each day we are given.” — Brittney M.

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2017 Annual SMA Conference
which will be held in Disney World, Orlando, FL
June 29 – July 2, 2017!

SAVE THE DATE!

If you are interested in being added to our mailing list for the 2017 Annual Conference, please email exhibitor@curesma.org.
Capt. Tony DiGiulian has fished all over the world for everything from swordfish to marlin, but one of his most memorable trips was last week out of Hillsboro Inlet catching blue runners.

He was fishing with Madison Smith, a 9-year-old who has spinal muscular atrophy, a disease that has had the little girl in a power wheelchair since she was 2.

Madison, of Pembroke Pines, had fished in bays and lakes, and really wanted to fish in the ocean.

DiGiulian, with the help of several other people, got her and her parents offshore on Wish List with Capt. J.P. Wolf.

They tied the 37-foot Willis to one of the mooring balls off Pompano Beach, put out a chum bag and helped Madison catch runners as well as ballyhoo, lizardfish, jacks, chubs and grunts using squid, shrimp and little chunks of bonito for bait.

"To be honest with you, if someone were to give me the opportunity to only tie off to those anchor balls for the rest of my career and only take someone like Madison fishing every day, I would trade that for going marlin fishing," DiGiulian said.

"She is a tough little cookie. She doesn't want to be babied. She makes you realize our little complaints and everyday problems are tiny."

DiGiulian was working in the Pelagic booth at last month's Miami International Boat Show when Madison and her parents, Aaron and Jen, stopped to see what was new.

It didn't take long for DiGiulian to realize that he had to take Madison fishing.

"I don't know what it was," DiGiulian said. "I've been doing this for a long time with all kinds of kids, but she and I almost instantaneously struck up a friendship and talked fishing."

When the Smiths mentioned that Madison, whose website is madisonswish.com, had major surgery scheduled for April 1, DiGiulian knew he had to act quickly to get her on the ocean.

Ron Kawaja of Pelagic said he wanted to sponsor the trip. Wolf, the captain of Wish List, said it was no problem to take everyone on the boat. Tom Ackel of Bluewater Chairs provided a special fishing chair for the boat for Madison. Andy Novak of LMR Tackle donated all the bait.

The trip was a success as soon as Wish List went out the inlet.

"The highlight for Madison was getting on the ocean for the first time," DiGiulian said. "That was her dream."

"She was just blown away being on the ocean."

The fishing was phenomenal for Madison, who, when she wasn't in her fighting chair, sat on the transom while being held by one of her parents as she reeled in fish.

"She needed a little bit of help," DiGiulian said. "She can turn the handle, but it's tough for her. We helped her along, but she got the feel of the drag and the rod bending."

"She's so interested in the actual fish. She loves to touch them, her mind is so curious, and she got into kissing the fish before we released them. She was fascinated with the ballyhoo, believe it or not. She was amazed at the little orange dots at the end of their bills."

For DiGiulian, of Fort Lauderdale, that's what fishing with kids is all about.

Along with Clay Barker and artist Carey Chen, DiGiulian is one of the founders of Fish to Make a Difference. The 501c3 charity (visit fishtomakeadifference.com) enables youngsters who are fighting diseases and their families to go fishing for free with local captains.

"The highlight for me was just seeing the joy in Madison's face and the joy in her parents' face as well," said DiGiulian, who plans to get Madison back on the water. "And also exposing a child like that, who really wants to know, to the ocean and all the things that swim in it."

"She said, 'I don't care what kind of fish they are or how big they are, I just want to catch them.' It doesn't get much purer than that. Stuff like that inspires us to fish to make a difference."
Finding a Cure for Spinal Muscular Atrophy, the Number One Genetic Cause of Infant Death

Rutgers researcher targets backup gene to reduce severity of muscle wasting disease

Six-year-old Chase Kopczynski is a happy child. He smiles all the time, loves school and depending on the day you ask wants to grow up to either be a doctor, architect or Lego engineer.

Under normal circumstances such childhood characteristics would not be unusual. Chase, however, was born with a genetic neuromuscular disease that has left him in a wheelchair with limited strength in his legs, arms, hands and fingers – making everyday living for this kindergartner seem like a triathlon.

“We’ve taught him from a very young age that it is going to take longer for him to do some things so he is going to have to do them differently than others,” said his mother, Lorraine. “I think understanding this is the reason why he is such a positive little boy.”

Chase is among the 1 in 10,000 babies born each year with spinal muscular atrophy, known as SMA. The disease results in the deterioration of the nerve cells connecting the brain and the spinal cord to the body’s muscles. Eating, controlling bodily functions and even breathing for Chase and others with this debilitating disease can become an arduous chore.

Rutgers scientist Mike Kiledjian, chair of the Department of Cell Biology and Neuroscience in the School of Arts and Sciences, is one of a number of researchers looking for a way to treat the condition or cure the disease.

“It is quite sobering to see those with SMA and their families,” said Kiledjian, who recently received a $140,000 grant from Cure SMA, a patient advocacy group dedicated to funding basic research, to continue his work. “It drives you to do research in hopes of finding some treatment that will help.”

SMA is caused by a genetic mutation – inherited when both parents pass on the mutated gene – that prevents children like Chase from producing enough protein from the SMN1 gene and leads to the progressive wasting of muscles.

Kiledjian and other scientists believe a backup gene, SMN2, which produces a small amount of the protein, could be targeted for treatment. He and his laboratory team are working to find a way to increase the level of the protein produced by the backup gene so that it can takeover. Such a discovery, he said, could reduce the severity of the disease and enable patients to lead a more productive life.

“There have been major advances in the last few years and clinical trials are underway,” said Kiledjian. “But obviously, nothing is fast enough for patients and their families.”

Chase was diagnosed with SMA type 2 at 14 months. He can be placed in a sitting position but might never be able to stand or walk on his own. Unlike those with SMA type 1, the number one genetic cause of infant death in which children don’t usually survive past their second birthday, those with SMA type 2 live into their teenage years or young adulthood.
SMA type 3, the mildest form, usually doesn’t affect life expectancy. The rarest form, SMA type 4, usually surfaces in adulthood, leads to mild motor impairment and usually begins after age 35.

For Chase, even a mild cold can land the youngster in the ICU because of his weak respiratory system. To help him thrive, Chase has a G-tube placed in his tummy that delivers extra nutrition directly into his stomach to ensure that he is getting enough fluid and calories to grow. To treat his progressive scoliosis, Chase recently underwent back surgery for placement of adjustable growth rods connected to his ribs and hips that enable his body to remain upright as he grows taller.

Chase’s father, Andrew, takes him to public school in Edgewater in a modified accessible van which allows him to transport his “zoomy chair” – a motorized wheelchair that gives Chase tremendous mobility and independence while at school. He also has a health aide to help him through his day.

“He is very bright, social, outgoing and loves going to school,” Lorraine Kopczynski said about her son. “We keep him stimulated and are happy to see him happy.”

Still, she often begins and ends her day with thinking and hoping that there is a scientist who will find a way to replace the mutated gene that is causing the disease with a healthy one. If that wish is too lofty, she and other parents in her situation look to the discovery of medications that would provide their loved-ones with the physical strength that the disease has robbed from them.

“In our minds and hearts we want a cure,” she said. “No parent wants this for their child. “But every day we focus on what we have instead of what we don’t and it gives us hope.”

Chicago Area High School Helps Cure SMA

In December of 2015, Cure SMA was voted as the Maine South High School charity of choice by many of their current students. Since then, the students have been organizing fundraisers and events in support of Cure SMA.

Since December students and clubs at Maine South have organized numerous events which included, a color run, a pizza madness night, and a walk-n-stroll. The students also attended their annual Hawk Fest event where they were able to go outside and enjoy a DJ, food, and a ton of fun games which included a dunk tank, giant jenga, pie in the face, and an inflatable obstacle course to name just a few! For every activity the students participated in, they bought tickets to play and all the proceeds went into the total raised for Cure SMA!

On April 29, 2016, at their spring assembly, Cure SMA was honored to be presented with a check for Cure SMA in the amount of $32,016! Cure SMA would like to thank all the students and faculty at Maine South High School in Park Ridge, Illinois for their generous support of SMA!

We would also like to give a special thanks to Lexi Basso and her friends for entering Cure SMA into the list of possible charities to vote for. After nominating Cure SMA, Lexi and her friends then worked tirelessly to educate other students on SMA so they would vote for Cure SMA.

Thank you Lexi for spreading SMA awareness to so many of your fellow classmates and for getting them to rally around an amazing cause!
Twenty-one years ago - in 1995 - my 6-month-old son, Jack, was diagnosed with SMA. From that day forward, everything changed, especially my expectations for Jack’s future. We were told there was no cure or treatment for SMA and that Jack would not live to see his 2nd birthday. Receiving this news was very painful for our family. It was very difficult for us to find any hope.

So we took our baby home and we did our best to learn how to care for him. I learned how to use a pulse oximeter, suction machine, and cough-assist. I’ve learned all about ventilator settings, wheelchair electronics, and feeding tubes. For years, I’ve driven around with a toothbrush and a packed overnight bag in the trunk of my car, always prepared for Jack’s next hospitalization. Twenty-one years later, I feel very fortunate to have Jack, but the road we’ve travelled has been anything but smooth.

Twenty-one years ago - in 1995 - something else happened, too. Scientists discovered the gene responsible for SMA. Looking back, I didn’t focus too much of my attention on this news. I was too busy taking care of Jack and trying to come to terms with the news that he would not live very long. But when I attended my first Families of SMA conference a few months later, a palpable sense of hope was in the air. Finding the gene responsible for SMA represented a significant step forward and provided new energy for our families and researchers.

Our SMA community back then was much smaller in number, but no less committed to finding a cure. Audrey Lewis, the founder of our organization, had been working tirelessly for years to raise awareness, support our families, and to fund research. I had heard that Audrey founded our organization in 1984, together with a small group of mothers of SMA-affected children, by holding meetings in her Chicago-area home. Audrey had truly made a difference, and progress had begun to be made on the research front.

Along with that renewed sense of hope, the researchers at our 1996 conference made it clear that the road ahead to finding a treatment or cure for SMA would be long and complicated. Years would pass before the discovery of the SMA gene would lead to any kind of treatment for our children. Because waiting for a treatment or cure would be far into the future, I tucked away the idea that Jack, or any of our children, could be helped by the discovery of the SMA gene. The idea that a treatment for SMA could help our children was nothing short of a pipedream in my mind.

Twenty-one years have passed since the discovery of the SMA gene. Our Families of SMA organization is now called CureSMA. What started as a handful of parents meeting at Audrey’s house in Chicago in 1984, has grown to thousands of families around the world. An entire generation of families affected by SMA has joined our community over these two decades. And with the announcement that the FDA is fast-tracking the approval of a new treatment for SMA, the road ahead now looks and feels very different for our families. With the help of nusinersen, many SMA-affected babies and young children are maintaining, and even gaining strength, rather than losing strength. This is the new SMA.

As I write this, Thanksgiving is just around the corner. As I reflect back on the past 21 years, I feel very grateful.

---I am grateful for Audrey Lewis and the many families who supported me when Jack was newly diagnosed with SMA. I’ve done my best to thank them by “paying it forward” to other families over the years.

---I am grateful for my son, Jack, who has defied the odds and has accomplished so much in his life…. regardless of whether he ultimately benefits directly from nusinersen or any other new treatment.

---I am grateful that our collective effort to raise awareness and funds to support SMA research has led to this significant research and treatment milestone. The pipedream of two decades ago is actually becoming a reality.

---But most of all, I’m grateful that the road ahead for newly diagnosed SMA-affected children will be more hopeful than the road my son has needed to travel.

As a community, we’re all in this together. Let’s take a moment to celebrate and be thankful for this extraordinary milestone, and continue to support each other as we move forward, with hope, into the future.
Living with SMA for nineteen years has been an adventure. Most of my memories are typical for anyone my age - birthdays, vacations, graduating high school and beginning college – but a few of them are more unique, including those of the Cure SMA conferences. I’ve been to six of these conferences, including ones in Boston, Orlando, and most recently DC.

I started attending as a little kid, and spent most of the time playing with the other children in the babysitting area and going to the family events.

The volunteers always do a great job of making the experience engaging and fun for the kids.

As I got older I began attending more of the events, such as the talk-it-out workshops and some of the activities geared towards older children. During the two most recent conferences I went to, I attended many more talks and was much more active, searching for information and advice that would help me in the future. These workshops gave great insight and tips for topics such as living independently and succeeding in college.

In addition to offering advice and information, the conferences are a great opportunity to socialize with others that have lived their lives with many similar experiences.

Most of us are used to being the only ones in our social circle who know the difference between a BiPAP and a CPAP, or what a ROHO Cushion is, but at the conference these things are practically common knowledge. Not only is this a nice automatic bond between families, but also it usually leads to the sharing of stories that most would not truly appreciate.

Very few people can really understand the magic behind discovering that one surprisingly accessible park down the street, or finally finding the dream van you’ve been looking for.

Personally, I have countless positive memories of my experiences at the conferences. I remember arriving soaking wet to the opening night party in Epcot, laughing with other families and sharing tips on how to protect our wheelchairs from the downpour. I remember going into a Mexican restaurant near the conference hotel and the confused faces on the restaurant staff as group after group arrived requesting an accessible table.

I remember meeting families from across the globe, and families I had previously only spoken to online, and families that lived just a short drive from my home. For those of you reading this that have been lucky enough to attend a Cure SMA conference, I hope you have gotten as much from them as my family and I have.

For those who have not yet been, I encourage you to have a little adventure and come see what they’re all about. I feel extremely fortunate in that these conferences exist, and I am excited to see them growing in size and success each year.
My name is Monika Lemeshonok, I was born on January 27, 1986 in Belarus, city Mogilev. I was born a completely healthy child and like all the children started walking at the age of 1 year. I could stand near the crib and walk when I was holding a hand. I was a very active child, but after a few months, my mother began to notice how my legs began to weaken when I rested in a chair. My family showed me to a neurologist in the Minsk Hospital. At the age of 1.5 years, I was diagnosed with SMA type 2 (Spinal Muscular Atrophy), which was later confirmed by genetic DNA analysis. It is a terrible disease that affects nerve cells in the upper part of the spinal cord that results in atrophy of the leg muscles and back and then to the loss of movement completely. The person ceases to walk and move. Despite my disease and physical difficulties since childhood, my mother told me that you need to believe in miracles, live and enjoy life, and I’ve always believed that one day there will happen to be a miracle with a cure where I will be able to walk.

I have always admired the world and from an early age loved to draw. At first I was drawing with pencils and pens of cartoons and heroes. I really liked to draw and paint. I started with the teachers of painting in art school. From childhood, I gave my personal exhibitions of paintings in many cities in my country and abroad, and to help me in this was my mother. Many professionals and art lovers appreciated the artwork. My works are in private collections in Europe, Asia and America. In 2009 I graduated from the State University in my city, with a specialty in Fine Arts.

I paint my art works in various styles and techniques, such as surrealism, symbolism, and abstractionism. According to many professionals these paintings have rare art color.
August is SMA Awareness Month. Cure SMA has been coordinating a National Awareness Month since 1996. While our community works year-round to raise awareness of and funds for SMA, August is a great time to spotlight those efforts. In addition to our usual work—hosting and attending events, telling our stories to neighbors and friends, sharing information about SMA, using social media to raise awareness, and more—many families and chapters plan special events or efforts around SMA Awareness Month.

One of the most anticipated events of SMA Awareness Month is the SMA Candlelighting. At sunset on the second Saturday of August, the SMA community lights candles to remember those who have passed away from SMA, and to honor those who are living with SMA. This year’s Candlelighting took place on August 13, 2016.

During this year’s SMA Awareness Month, we offered a number of ways to help spread awareness for SMA on social media. Some of the ways to help spread awareness including changing your Facebook or Twitter profile picture via our Twibbon campaign, and submitting photos for our annual SMA Community Album on Facebook.

Here are just a few of the wonderful pictures that were submitted for this year’s SMA Community Album!
SMA AWARENESS MONTH

SMA SuperMenAttitude

DIRECTIONS Winter 2016
Our daughter Layla was diagnosed with SMA Type 1 (Spinal Muscular Atrophy) on May 8th 2016. SMA is a genetic disorder where people are missing an important protein gene that creates protein for their muscles. Since this gene is not there they eventually lose all muscle. Some are worse than others and it just all depends on the person. There are 3 types and they are determined based upon the age of diagnosis.

I remember that horrible day like it was yesterday. We left that appointment feeling helpless, hopeless and like our whole world was over. I kept telling her dad “I miss her so much already”....it was the weirdest feeling. Holding your little baby girl and breaking inside, missing them even though they were right there, but we were given the impression that it was only a matter of months until we lost her.

We told our moms and those closest to us that very same day and on that very same day they began researching and we were filled with hope. SMA 1, while type 1 is the most fatal it is NOT a death sentence and there are MANY things you can do to keep your little one alive, happy and healthy. Should you choose that route there are options out there.

Childrens Hospital of Oakland referred us to Stanford where they specialize in SMA. Stanford has been great. We had Laylas initial assessment with her neurologist, a speech therapist, a physical therapist, a gastroenterologist and a pulmonary doctor. In between all these appointments she started having trouble eating. She was 3 months old and had barely gained back the weight she lost from birth. We were admitted to Lucille Packard Childrens Hospital after requesting she have an NG tube placed while she awaited g-tube surgery.

During that hospital stay (a long 2.5 weeks) she started receiving breathing treatments along with cough assist session. These include a dose of albuterol to help open up her airways, a cough assist that allows her to cough followed by suctioning. I WAS VERY VERY intimidated and nervous about going home and caring for her without docs and nurses right there BUT I must say it’s not too bad.

That top part was written for her “Team Layla” page back in June...TO DATE: August 8th, 2016 she now requires 23 hours BiPap support but is now weighing at 11 lbs 4 oz. I’ve just changed her diet and am hoping she’ll start to thrive. We will be starting on the AA diet very soon. Since taking her off of Regular formula (Enfamil) she has regained a very small amount of movement in her arms, hands and feet :)

It’s been a roller coaster of emotions these last few months and I’ve had days where I thought I couldn’t handle this, my faith has been tested and I became very bitter and sad.....BUT her smile makes everything all better. She is such a sweet, beautiful and happy baby and that is going to help her in this fight.

#TeamLayla
Congratulations

Congratulations to the Nowotny Family

of Florida on the birth of their new baby girl, Marley, born on May 1st!

Congratulations to Kayla Cooper and Tyler Wingard, and big brother, Hayes

on the birth of their new addition, Judson!

Congratulations to the Brookes Family

on their new baby boy, Joaquin Javier, born on February 5th, 2016!

Congratulations to the Weaver Family

on the birth of their new baby, Winston Sage, born on October 3rd, 2016!
Hallowell teen, father hiking the Long Trail to raise awareness for Spinal Muscular Atrophy

Anthony Romano, 15, of Hallowell, and his father, Jeff Romano, are hiking 271 miles to raise money for The Hearts for Ezra Foundation, to raise awareness for Spinal Muscular Atrophy, the No. 1 genetic killer of children younger than 2 years old. The foundation was created a few years ago following the death of an 8 month old in Hallowell.

Anthony, who will be a sophomore at Hall-Dale High School in the fall, has already climbed 53 of New England’s 67,400 foot peaks. This summer, he and his father are hiking the Long Trail, which follows the Green Mountains from Quebec to Massachusetts.

To raise money, they are hoping to raise $2,713 for Hearts for Ezra: $10 for each mile of the Long Trail.

Angie Lee Receives 2016 Prudential “Spirit of Community” Award

Seventeen-year-old Jungin Angie Lee, a high school junior, has received the 2016 Prudential “Spirit of Community” Award for her home state of Illinois. The award recognizes the top middle and high school volunteers throughout the United States.

Angie and her friend Kyra formed “Angie’s Hope” in 2006, when both were in second grade. Angie, who has SMA type II, and Kyra had an idea for a penny drive that turned into a fundraiser that generated nearly $10,000 to help find a treatment and cure for SMA. Since then, they have raised almost $200,000 through a variety of events.

In 2014 and again in 2015, Angie, Kyra and their classmates held an adaptive “big ball” soccer tournament that has raised over $71,000 for Cure SMA. The inspiration for the soccer tournament came from all the way back in second grade—when Kyra first wondered why Angie couldn’t play soccer with her, and the idea for Angie’s Hope was born.

In addition to raising money for SMA research and family support, Angie also volunteers in other events throughout her community.

These efforts demonstrate “how huge a difference individuals can make when they combine efforts,” she said, and have become “a way for our small community to unite to make a change.”

Angie and the other state winners will receive an engraved medallion and a trip to Washington DC to be recognized for their efforts.

Congratulations to Angie on her award! We are very proud to have you and your community working with us to raise funds and awareness for SMA.

Thank you to CentralMaine.com, the Kennebec Journal, and the Morning Sentinel for allowing us to share this story with our community!
Family Photos

The Armbrustmacher Family
The Hoffman Family
The McHale Family
The Eide Family
The Butler Family
The Gooden Family
The Nowotny Family
The Tranby Family
The Sykora Family
The Foley Family
The McIntosh Family
The Tomko Family
The Schmid Family
The Meigs Family
The Lee Family
The Ellis Family
The Partridge Family
The Moyer Family
The Murray Family
Cure SMA Chapters

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

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The 3rd annual Byrds FORE a Cure took place on May 7, 2016 in Phoenix, AZ, at the Dove Valley Ranch Golf Club for a successful day of increasing awareness of SMA, fundraising and great golf! Prizes were awarded to the first and second place teams. We are grateful to our wonderful sponsors and the local restaurants, golf courses, retail stores, businesses and the Diamondbacks for their incredible support of our Cure SMA Arizona Chapter. With their help, and the generosity of all our golfers and dinner attendees, Byrds FORE a Cure raised $9,115 to Cure SMA! See you in May 2017!

The 2nd Annual Cure SMA Casino Night for Gray’s Gang was held on March 5, 2016. Local high schoolers participated in a casino experience that offered roulette, blackjack, poker, and craps. Not only did the kids enjoy a night packed with games, music and friends, they also had the opportunity to win pretty cool raffle prizes.

The event was a huge success and raised over $21,000 to help find a treatment and a cure for SMA. We are all looking forward to Casino Night 2017!

Thank you to the Dougherty Family and Gray’s Gang for their hard work on this event, and their dedication to Cure SMA.
Cure SMA Wilmington Walk-n-Roll

The inaugural Wilmington Walk-n-Roll held in Wilmington, NC on April 16, 2016 raised over $16,400 for Cure SMA! Shashin and Sejal Patel hosted this event in memory of their daughter, Sonya Patel. Over 100 people gathered at Hugh MacRae Park for a morning of family fun and a scenic walk around the park. Thank you to everyone who participated and made this event such a success!

Hayes’ Heroes Present a Cure SMA Evening of Hope

The inaugural Hayes’ Heroes Presents a Cure SMA Evening of Hope, held in Lexington, SC on March 12, 2016, raised $35,000! Family and friends gathered in honor of Hayes Wingard, SMA type II, to raise funds for Cure SMA. Guests enjoyed the superhero theme, silent and live auctions, and an inspiring speech by Adam Bugenske, dad to Leo Bugenske, SMA type I.

A special thank you to Keri Cooper and Angleigh Hartman for co-chairing this year’s event.

2015 Cure SMA Mistletoe Jam

Thank you to Don McInerney and Chris Genovese for hosting the 2015 Cure SMA Mistletoe Jam! Everyone gathered at the Southside Smokehouse & Grille in Landrum, SC for a night full of fun and music in honor of Alexandra Genovese, SMA type II. Thanks to their efforts $2,235 was raised for Cure SMA!

2016 Chesapeake Chapter Walk-n-Roll

On April 30, 2016, the Chesapeake Chapter of Cure SMA held its fourth annual Walk-n-Roll in Mount Airy, Maryland. The 235 attendees who made up 17 registered teams walked the one-mile loop at Old National Pike Park and raised nearly $41,000 to help the continued research efforts to find a cure for spinal muscular atrophy. Many of the walkers and rollers have attended the event multiple times, and many others were welcomed by the Chesapeake Chapter for the first time. Throughout the day, families and friends shared personal stories of having their lives affected by SMA while honoring loved ones.

The Chesapeake Chapter wants to thank Biogen, AveXis and BAYADA Home Health Care for their corporate sponsorship of the event, as well as the following local sponsors and people who donated resources, time, and support to make the fundraiser successful: Our Little Jewels, Johns Hopkins Home Care Group, Larry Jackson’s Bullhead Pit Beef, DJ Alfie Aldave, Weis Markets Mount Airy Store, Food Lion Grocery Store of Mount Airy, Starbucks of Mount Airy, Photography by Gail Brown-Niles and Robert Brown.
Gala of Hope for Kamdyn’s Kure

Gala of Hope for Kamdyn’s Kure was held on Friday, April 22, 2016 at Green Grove Gardens in Greencastle, PA. The night entailed cocktail hour with saxophonist, dinner, dancing and a red carpet photo booth. There was a silent & live auction made possible by the generous donations of family, friends and local businesses. The night was a huge success and a wonderful time was had by all who attended. Just over $20,800 was raised to support Cure SMA!

The event was hosted by Travis and Amber Hartung in honor of their 4 year old son, Kamdyn who was diagnosed at 13 months old with SMA type II.

A special thanks to the family, friends and volunteers who dedicated their time to help make the gala amazing. This event wouldn’t have been possible without their dedication and support.

Thank you to our sponsors: Biogen; Diloreto, Cosentino, Bolinger Attorneys at Law; Melanie Rotz; Brad Helmuth Drywall Services and Donkey Beats Entertainment.

United Parish of Bowie Fundraiser

On Saturday June 11, 2016, the United Parish of Bowie hosted its inaugural Cure SMA Walk and Roll, “UPB vs SMA.” It was a resounding success. Around 100 people, comprised of friends, family and church members came to support our son Kyle, who has SMA type III, and everyone else that is affected by SMA. Together we raised over $5,500. Some of the highlights of this event were the Rito Loco taco truck, Johns Water Ice, face painting, moon bounce, and other fun games.

Our family was humbled that our church sponsored this event in an effort to be more visible in the community and to help support one of its families. The support of our church and everyone that donated or walked with us really helped our son Kyle feel loved and appreciated. We are deeply thankful to everyone who participated in UPB vs SMA and look forward to hosting it again next year.

Matt Bickel | Crofton, MD

2016 Ride & Dice Motorcycle Ride in Memory of Zachary Miller

When BU Miller posted on Facebook that little Zachary, SMA type I, passed away I knew we had to do something. Although BU Miller is with a different battalion (40) than the one our Motorcycle Chapter represents (23), Unit numbers mean nothing when it comes to something like this. Plus that is what Seabee’s do - they stick together and support each other. Although at BU Miller’s initial post, I had no idea what SMA was - what I did know was the agony of burying your baby. My husband (President of NMCB-23 MC MD Chapter) and I buried our two babies, one in 1998 and one in 1999. There is no greater heartache. After talking with our Chapters members, everyone was onboard with doing something to help raise funds and awareness of SMA. Renegade Classics in Annapolis was gracious enough to host our Memorial Ride and Dice Run at their business and local businesses rallied around us and donated gift cards to be used as prizes. It was a big party - all while remembering what we were there for. We kicked off the event with a moment of silence in honor of Zachary and then the riders mounted up for a beautiful planned route through Anne Arundel and Calvert County MD.

Seabee’s NMCB-23 Motorcycle Club

Edgewater, MD

Thanks to BU Miller for raising $600 for Cure SMA!
May 7, 2016, was the inaugural West Virginia Walk-n-Roll. The event was a huge success! We had a goal of $10,000.00 and we raised almost $14,000! We had around 100 people at the event and some registered who were not actually able to attend. We had quite a few teams participate and we had one SMA affected individual who was able to attend, Alex Johnson. Alex inspired the event, which led to meeting so many more families directly or indirectly affected by SMA. Alex just turned two years old and has SMA type I.

Everything we had available at the event was donated. We are so grateful to everyone who supported and participated in the event. We surpassed our goal and barely touched our event budget. So many amazing people stepped forward to help us make this possible. We had a team of 5 organizers who worked hard to get donations, sponsorships and participants. We had a number of volunteers that helped us set up, tear down and work the registration table. Julie Coddington and John Roth donated all of the snacks and drinks. Cress Photography donated their time to photograph the event for us. Dustin McMillen came dressed as Batman and the kids loved taking photos with him! Rose Kraftick painted the kids’ faces! Hash Browns and New Grounds, a local food truck, also came and fed people and donated a portion of their proceeds to the cause. Jason Good Blues Band entertained us with live music and emceeing! So many of the teams did their own individual fundraising which allowed us to surpass our goal by thousands of dollars! Our top team, Team Owen, alone raised $5,721.54. That was over half of our goal! Allan N. Karlin & Associates, a local law firm, also sponsored the event along with Biogen, Avexis and Bayada.

I look forward to making this event annual and hopefully with the success we had in 2016, next year will be even bigger!

This event is in honor of my friend, Cubby. He is 17 years old, and about to be a senior. He is a very good student, and avid NY Mets supporter and a fan of Jim Henson and his Muppets. I got to know Cubby when we played on the same T-ball team when we were 6 years old, and we have stayed friends ever since!

2016 brought another successful year for Cubby’s Run. The 5th Annual Cubby’s Run was held on May 7, 2016, at the Ridgefield Rec Center Trail in Ridgefield, CT. Thanks to the 75+ racers, plus our vast online donations, we are proud to be able to say we have raised $23,000 this year, and over $100,000 over the past 5 years, which has all gone towards finding a cure!

Cubby’s Run will continue next year and we plan to keep our eyes forward, looking toward the $200,000 mark. More information on the 2017 race will be given out more towards the start of winter. Together we can find a cure!

Ethan Hynes | Ridgefield, CT

The Connecticut Chapter Walk-N-Roll was held on May 14, 2016 at Brooksvale Park Hamden, CT. It was a beautiful sunny day to walk the 1.5 mile tree lined loop. There was a fabulous array of donated raffle prizes, food and drink. It was great to be walking for a cure again—our last walk was in 2010! Thank you to all our family, friends, coworkers, and new faces for your support in helping to raise over $7,500 for a cure. Hope to see you all again at next year’s walk!

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

Ding’s Donates to Cure SMA

Brandon Dingwell made this pledge in honor of Eva Grace Foell. Eva was born on December 28, 2012. In March of 2013, she was diagnosed with SMA type I. That is when her parents, Kris and Miche, learned that their daughter would not live past the age of two. Despite the diagnosis, her parents vowed to give their daughter as much of a normal life as they could in the short time that she had. Every day she got dressed up and went to play with her cousin instead of being confined to her bed. In her short time on Earth, Eva taught important life lessons to those around her. From her young cousin who learned how to be loving and gentle at such a young age, to her parents and family who learned how to love like they never had before, and to learn not to sweat the small stuff. Eva brought so much love and joy in such a short period of time. On May 1, 2014, at just 16 months old, Eva Grace was taken from the earth.

Brandon Dingwell | Litchfield, CT

Ding’s Auto Parts of Litchfield, CT, generously donated 5% of its sales from the month of July to Cure SMA. They raised $4,600!

Cure SMA Tennis Tournament in Memory of Peyton Zimmermann

The second Cure SMA Tennis Tournament in Memory of Peyton Zimmermann was a huge success raising $17,000! Despite the rainy weather on April 30, 2016 over 40 players hit the courts at the Atlanta Athletic Club in Johns Creek, GA. After play, everyone gathered for a delicious dinner and moving presentation by Stacey Zimmerman. Thank you to the Zimmerman and Elmore families for their hard work in putting together this event. Special thank you to our continued sponsors and participants for their support.

Lenten Service Project in Memory of Chloe Painter

Thank you to Mrs. Ruth Patch’s 2nd grade class from Tucker, GA for donating to Cure SMA! The class did a Lenten service project where they did chores at home to raise funds in memory of Chloe Painter. Their efforts raised over $140!

Greater Florida Chapter

Chapter Update: Greetings from the Greater Florida Chapter!

Stretching from south of Sarasota to the north Florida panhandle, our chapter covers a large and diverse geographic region and includes many amazing families. We invite you to stay connected by regularly checking our Facebook page at www.facebook.com/CureSMAGreaterFlorida.

We recently welcomed Asia Nowotny to our chapter board as our treasurer. We also welcomed Katie Kerns back to our board as the chapter's secretary.

2016 Chapter Board Members

President: Audra Butler
Vice President: Laurie Sore
Secretary: Katie Kerns
Treasurer: Asia Nowotny

Are you interested in joining our chapter board or a committee?
Email us at greaterfl@curesma.org.
This year marked the return of the Greater NY Chapter Golf Outing, held on Monday, June 27, 2016 at The Hamlet Golf and Country Club in Commack, NY. This year’s event raised $23,405 in support of SMA research and family support programs. The event was a great success with 18 foursomes who golfed a beautiful day on the course and enjoyed a putting contest and 50/50 raffle. After golf, everyone joined together for a celebration dinner and raffle.

Special thanks to our sponsors: Citi, Allen & Overy, Strategies for Wealth, Cuevas & Garcia, P.A., Brookes & Gorria Search, Duane Morris, CHIPS, Joan & Felix Cutrone, The Florida Skylanders, the Martin Family and the Cuevas Family.

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

On May 10, 2016, Cure SMA held its first New York Evening of Hope in memory of Ella Savarese. Ella had SMA type 1 and passed away on May 15, 2013. The evening raised $21,789 in support of Cure SMA. This great event was hosted by City Bakery in New York City. The night included a heart-felt speech from Claudia Poernig-Savarese, Ella’s mother, as well as delectable appetizers, drinks, live entertainment and a silent auction.

As Ella’s parents stated: “Although Ella’s life was cut short at only 14 months of age, her memory is one that is carried with us every single day. This little angel touched many, many lives.”

Special thanks are extended from Cure SMA to Paul and Claudia Savarese and Julie Meriam for chairing the event as well as to Allison Ceraso, Tracy McCann, Rachel Braun, Kurt Slawitschka, and Jesse Winter of Havas Life New York for creating all of the event materials. Their time, energy and dedicated support to this event made it a huge success. Because of this event, Cure SMA is able to continue to lead in the race for treatment and a cure for those inflicted with all types of SMA.

The United Airlines New York City Half Marathon took place on March 20, 2016 in New York, NY. The day consisted of a great race taking runners through famous landmarks such as Central Park and Times Square. This race attracts more than 10,000 runners annually, and this year Team Cure SMA had 5 runners participate. Together, they raised $11,628 for Cure SMA!

A special thanks to our runners: Erin Bonner, Katie Fagerland, Carrie Gillen, Joseph Kolinsky and Molly Popolizio.
David Gershuni Giror

David Gershuni was conceived in Belgium, but avoided the terrors of the Holocaust, which overtook his Father, by being born and educated in England, and raised by his adoring mother. He received his medical degree from St. Mary’s in London, and became a world renown Orthopedic Surgeon, specializing in numerous Orthopedic procedures, including lizarov apparatus to lengthen or reshape bones, trauma, and fixing an elephant’s broken leg at The Wild Animal Park in San Diego, California. David worked, explored and adventured across the world to reach and live in Israel. After marrying Nina, a native New Zealander, they began creating their family in Israel, with the birth of their 2 daughters, Romy & Natalie. David and his family then emigrated to La Jolla, California in 1977 where his son, Daniel was born. There he had a full, stimulating life as a surgeon, clinician, teacher and researcher at the University of California, San Diego. David was diagnosed with Kennedy’s disease in 1987, and as such his thriving and productive career was greatly abbreviated. David and Nina returned to Israel in 1996, where David’s muscular degenerative disease slowly unfolded. Although numerous hospitalizations have resulted in setbacks, David continues to stay ahead of his disease, with the incredible medical care he receives and apparatuses such as wheelchair, tracheostomy and feeding port. Most importantly, David maintains a fulfilled life which centers around his wife, 3 children, 2 son in laws, 1 daughter in law and 9 grandchildren. Because of his devotion to these 16 people, David demonstrates courage, unwavering discipline, will and extraordinary determination.

Best,

Natalie (Gershuni) Ginor

Cure SMA would like to thank Natalie and her family for donating $4,000 to Cure SMA.

Maria Favara Simpson

Maria Favara Simpson Individual Fundraising

When I was five months pregnant I was told that we needed genetic testing done. There was a chance of our baby having SMA. We didn't know anything about this disease at the time. We were terrified as it was becoming new parents, and now we were being told we needed to decide if we wanted to continue with this pregnancy not knowing the results until much later. I had testing done immediately, it took months before we would be given preliminary results, and then two weeks before my delivery we received the final results. My husband Scott and I, our families and friends prayed every day. We thank God every day that our baby girl was born without SMA. We promised that we would raise money for research and aid and spread awareness for all those affected by SMA.

Maria Favara Simpson | Huntington, NY

Thank you to Maria for raising $650 so far for Cure SMA!

Gowanda High School

Class of 2015

On behalf of the Class of 2015, it is my pleasure to gift you with this monetary donation of $1,606 in the name of Zachary Degenfelder. Zack is a part of the Class of 2015 and continues to struggle daily with SMA. He is an inspiration to all his classmates.

Barbara Dempsey, Co-Advisor, Class of 2016
Gowanda High School

Knights of Columbus

Council #5427

Enclosed is a check for $1,000 from a fundraiser at the Knights of Columbus in Washington Township, NJ. This donation comes in honor of the Vanderloo family from George Kuzma, member of the Knights of Columbus Council #5427. Please continue the great work that you do.

John Pimpinella,
Columbian Club President
The partners and staff at SaxBST’s Albany, NY office raise money each month throughout the year for various charities though our Denim for Donation program. We allow employees to pay $5.00 or more to wear jeans one Friday each month to raise funds for various causes. For the month of April, we raised $58.

Carolyn Houck

### Make a Difference Club

Make a Difference Club at Putnam Valley High School and the National Honor Society donated $800 to Cure SMA in honor of Max Dash. He is their source of inspiration.

Linda Cefaloni
Putnum Valley, NY

### Ice Fishing Tournament

New York State Trooper David Cunniff had a vision and goal of raising a million dollars to help find a cure for SMA. His son Caleb has SMA type II. Him and his wife Amy started and led the New York Capitol Region Chapter Cure SMA until his untimely 'line of duty' death in December of 2013. Amy continues to lead this chapter, and continues with his dream.

One of the events held each year is an Ice Fishing Tournament on Glen Lake. In 2016, this event yielded a $2,339 contribution to Cure SMA. Our appreciation and thanks go out to Christine at The Docksider Restaurant (docksiderrestaurant.com) for organizing this event, to all the donors who provided prizes for the event, and finally to all who came and took part in the event. Most attendees gave much more than the entry fee for the cause! There was plenty of food and fun to be had by all. Most of the participants were actually out on the ice, fishing, while a few (smarter ones?) stayed inside and watched the fun from the restaurant. The 4th Annual Tournament will be held in the early months of 2017, the exact date is yet to be chosen, so watch the CureSMA.org website as the weather gets colder for the event information.

### SaxBST

The partners and staff at SaxBST’s Albany, NY office raise money each month throughout the year for various charities though our Denim for Donation program. We allow employees to pay $5.00 or more to wear jeans one Friday each month to raise funds for various causes. For the month of April, we raised $58.

Carolyn Houck
Inaugural Cure SMA Chicago Gala of Hope

On February 26, 2016 over 200 guests gathered at the Ivy Room in Chicago for the inaugural Cure SMA Chicago Gala of Hope. This incredible evening raised over $95,000 in support of Cure SMA. This fun event brought together families, researchers, doctors, business leaders, as well as generous supporters to raise funds to find a treatment and cure for SMA.

The evening featured cocktails, hors d’oeuvres, a silent auction, live auction, and live entertainment. Comedian Brett Walkow was the MC and the band Shout Out performed live and kept everyone out on the dance floor all night! This special evening not only raised significant proceeds for SMA but also additional community awareness and HOPE.

A special thank you to our title sponsor AveXis for their generous support of the inaugural Chicago Gala. We would also like to thank the other sponsors who generously supported this event: Biogen, Ivy Room, Aon, Amy Lenahan, ARI Packaging, Atlas Forms & Graphics Inc, and Connect Search. Thank you to all of our committee members and volunteers for their hard work, dedication and commitment to the success of this event, especially Maria Marusich, Sue Miranda and the O’Brien family and friends.

We hope to see you at our 2nd Annual Chicago Gala of Hope on Friday, February 24, 2017!
**10th Annual Illinois Chapter Walk-n-Roll**

The Illinois Chapter reached a new milestone this year by hosting the 10th Annual Illinois Chapter Walk-n-Roll! The walk was held May 15, 2016 at Independence Grove in Libertyville, IL. The sun was shining all day and we were very fortunate to enjoy a beautiful day for this fun-filled event to raise awareness and funds for SMA!

Almost 600 people gathered at the event for a day of fun! Everyone enjoyed the walk, DJ, face painter, balloons, and a fun t-shirt art station.

We would like to give a special thanks to all of our amazing teams, participants, sponsors and donors for making our 10th Annual Walk a HUGE success. It was another record breaking year in fundraising and the number of teams that participated. Due to everyone’s efforts we were able to raise over $59,000 and had a total of 31 teams participate!

Cure SMA would like to give a BIG thank you to Janet Schoenborn, the walk’s event organizer and Illinois Chapter President for everything she did to help make this such a great day and opportunity for all of the Illinois family and friends to come together. Cure SMA would also like to thank Danielle Plotke for helping Janet plan such an incredible event from start to finish!

A special thank you to Cynthia Annel, Liz Macellaro, Jodi Garvey, Maria Marusich, Tina Krajewski and everyone else who helped plan this year’s event. We hope to see everyone at the 11th Annual Illinois Chapter Walk-n-Roll this coming Spring 2017!
Tee Off with the Drive to Cure SMA in Honor of Ryan

The sun was shining on May 21, 2016, as we gathered for golf, dinner, raffles and fun! Glenaeles Country Club in Lemont was again the perfect venue, offering a beat-the-pro hole and other “fun-raising” opportunities on the course. This was our 5th annual event to support Ryan, an outgoing and all-around amazing 9-year-old with SMA type III. With the help of generous sponsors, volunteers and golfers we raised over $47,000 for Cure SMA!

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

Maine South High School Fundraiser

This past December, Cure SMA was voted as the Maine South High School charity of choice by many of their current students. Since then the students have been organizing fundraisers and events in support of Cure SMA. On April 29, 2016 at their spring assembly, Cure SMA was honored to be presented with a check for Cure SMA in the amount of $32,016! Cure SMA would like to thank all of the students and faculty at Maine South High School in Park Ridge, Illinois for their generous support of SMA! We would also like to give a special thanks to Lexi Basso and her friends for entering Cure SMA into the list of possible charities to vote for. After nominating Cure SMA, Lexi and her friends then worked tirelessly to educate other students on SMA so they would vote for Cure SMA. Thank you Lexi for spreading SMA awareness to so many of your fellow classmates and for getting them to rally around an amazing cause!

SAVE THE DATE!

2017 Annual SMA Conference

which will be held in Disney World, Orlando, FL June 29 – July 2, 2017!

If you are interested in being added to our mailing list for the 2017 Annual Conference, please email exhibitor@curesma.org.
37th Annual Bank of America Shamrock Shuffle 8K

Members of Team Cure SMA ran the Bank of America Shamrock Shuffle 8K on April 3, 2016, in Chicago, IL. This is the first year that Cure SMA participated in the run as an official charity partner. Together, the runners raised $2,945 to help cure SMA! Cure SMA would like to thank all of our runners; Noel Guest, Julie Pearson, Jessica Colella, Traci O’Brien and Benjamin Smith for their hard work in training to run this race as well as all their fundraising efforts!

2016 St. Louis Walk-n-Roll

The 2016 St. Louis Walk-n-Roll took place on May 15, 2016. Many SMA families got together to support its first year with food, music, and a 5K walk and run around Tower Grove Park in St. Louis, MO.

The event was a great success, raising over $35,000 in its first year! We look forward to making this an annual event! Thank you to everyone who supported and attended the St. Louis Walk-n-Roll!

2016 Evening of Hope in honor of Miracle for Madi

The inaugural Evening of Hope in honor of Miracle for Madi took place on February 19, 2016 at the Rogalski Center in Davenport, IA. This event is in honor of Madi Ramirez, SMA type III. The dinner event featured a silent and live auction, a sip ‘n sparkle, a step and repeat for guests to take pictures, and a plated dinner to raise money for Cure SMA.

The event was a huge success, raising $31,928 for Cure SMA in the event’s first year!

A big thank you to Megan Ramirez for organizing this event and making it such a great success in its first year! We look forward to next year’s event on March 3, 2017!

Phi Tau Omega Sorority Trivia Night (Delta Phi Chapter)

The ladies of Delta Phi Chapter of the Phi Tau Omega Sorority in St. Louis, MO raised $2,800 during their annual trivia night fundraiser! This event is held every year in honor of Brittany Carpenter, SMA type II. The sorority has been in contact with Brittany and her family since she was a little girl. Thank you to Kathy Goodyear, of the Delta Phi Chapter, for organizing this event every year!
Chili Cook-off Fundraiser

Each year Senior Market Sales, Inc. holds a chili cook-off in the fall, awarding the winners by donating money to a charity of their choice. With 165 employees in the Omaha office, there were 28 pots of chili and the competition was fierce! Joy Heuer was awarded first place, and selected Cure SMA as her charity of choice. We would like to thank Jill for selecting Cure SMA and donating $150 to help fund a treatment and a cure for SMA.

Lemonade Stand

Thank you to Donna Studley for raising $216 for Cure SMA in honor of Ayden Trammell. These funds were raised from a lemonade stand and parade hosted on a beautiful, sunny day in August 2015 in Phillipsburg, KS.

Central Intermediate School Fundraiser

On December 8, 2015 students from Central Intermediate School in Baton Rouge, LA donated funds from their annual wax museum fundraiser to Cure SMA. The donation was made in honor of their classmate who is affected by spinal muscular atrophy. After the event, students presented Louisiana Chapter President, Krista Scurria, with a check for $840. Thank you to Central Intermediate School for spreading awareness in your local community and raising funds for a treatment and cure of SMA.

2016 Rock ‘n’ Roll New Orleans Marathon Series

The Rock ‘n’ Roll New Orleans Marathon, Half Marathon and 10K took place on Sunday, February 28, 2016 in historic downtown New Orleans, LA. For our third year participating in the series, Team Cure SMA raised $300. Thank you to Nichole Roberts for running and fundraising for this year’s race!

Michigan Chapter 12th Annual Michigan Chapter Walk-n-Roll 2016

Thank you to all who participated in the 12th Annual Michigan Chapter Walk-n-Roll that was held May 14, 2016 at a new venue this year on Michigan State’s Campus at the Jenison Fieldhouse. We were able to utilize the indoor track, which ended up being the perfect day to walk inside to raise awareness for SMA, on the rainy May day.

A very special thank you to Linh Huynh, the walk’s event organizer, for everything she did to help make this such a great day and opportunity for all of the Michigan family and friends to come together. Linh and her family also helped bring some new and fun activities and crafts for all of the kids!

We would like to thank the Pi Alpha Phi Fraternity who volunteered for the day of the event and really helped to make the event flow! A special thank you to Melissa House and Team Abbey for the amazing job they do fundraising each year! Also thank you to the Wolfe Company and Linda and Harvey Wolfe for being such generous sponsors and supporters over the years.

We truly appreciate all of who participated, donated and helped out at the event! The one mile walk around the track, kid’s fun sprint, face painting, crafts and Jammin DJ’s were all such important aspects to making the walk such a great event for everyone! The 12th Annual Michigan Chapter Walk-n-Roll raised over $39,338 for Cure SMA!

We hope to see you at the 13th Annual Michigan Chapter Walk-n-Roll in May 2017!
**Everyone is a Bride Pub Crawl for SMA**

If you were in Kalamazoo, MI on July 23rd you may have noticed quite a few brides around the town that evening. Jamie Kneeshaw had a great idea to get more wear out of her wedding dress by raising awareness for SMA in honor of her friend, Sarah Kennedy’s daughters, Brielle and Brooke. Thank you to all who came out in your gowns, supported the local pubs and raised $455 for SMA, and thank you so much to Jamie for organizing such a fun event!

**Merry and Bright**

For the holiday season, Charity with Angelworks Photography held mini-sessions for families to get their holiday photos taken. She spread awareness about SMA the families getting their photos taken by passing out bracelets and information, and donated half of the session fees to Cure SMA in honor of Brielle and Brooke Kennedy. Through Charity’s efforts she was able to donate $455! Thank you Charity for all that you do!

**Fifth Third River Bank Run**

The 39th Annual Fifth Third River Bank Run was held on May 14, 2016 in downtown Grand Rapids and is the largest 25k in the country. Thank you to Jonathan Geenen who participated in the race and was also able to raise $330 as a part of Willow’s Runners.

**Join us on May 13, 2017 and run with Team Cure SMA!**

**Detroit Pistons**

A huge thank you to Ben Dorer for organizing the first ever SMA Night with the Detroit Pistons on March 6th with a portion of each ticket sale going to Cure SMA. Over 50 attended the game and the Detroit Pistons were able to donate $295 to Cure SMA!

**Sioux Falls Storm Fundraiser in honor of Kayley Shade**

Kayley Shade, Director of Social Media for the Sioux Falls Storm, did a fundraiser for Cure SMA where a portion of the ticket sales went to Cure SMA. They also did team t-shirts that the staff and players wore during the game and gave 100 percent of the proceeds back to Cure SMA.

Kayley, SMA type II, organized this amazing fundraiser for Sioux Falls’ indoor football team and raised over $4,400 for Cure SMA! Keep up the great work!

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

This was the first year we made a team for Cure SMA to participate in the Lake Minnetonka Half Marathon on May 1, 2016 in Wayzata, MN. The event raised over $5,000 for Cure SMA! Thank you to everyone who participated in the half marathon and to everyone who supported our runners!
Who would have thought that the New England Walk-n-Roll would still be going strong after sixteen years? With the help of twenty different teams and additional individuals honoring their SMA loved ones, we had about 500 participants and raised $120,000. In addition to other raffle favorites — such as Red Sox tickets, Amazon and Target gift cards, and the lottery basket — we had a beautiful t-shirt quilt made from the first 15 years of walk shirts, created by SMA mom Heidi Johnson. Many teams have been coming for years, making this event not just a fundraiser but a reunion. New this year was team Believe in Bodhi. Both Biogen and RaNA Therapeutics were on hand, and we were excited to welcome Dr. Swoboda, who recently joined Massachusetts General Hospital, to our New England Chapter. We have several families involved in the Boston area clinical trials and were thrilled to hear many hopeful stories (one type I baby is walking!). Thanks to the Murphy, Farrell and Norton families for organizing the event, the businesses who contribute refreshments and raffle items, and the many volunteers who make everything run so smoothly.

Silvia Murphy | Norwell, MA
Each time we receive and read one of Cure SMA’s “Directions” publications, we are heartened, inspired and hopeful. A decade ago we hadn’t even heard of spinal muscular atrophy. Roughly 8 years ago, we received the diagnosis of SMA type I from our son’s doctors at Children’s Hospital Boston. We remain hopeful that in our lifetime, there will be a treatment and cure for SMA.

In memory of our son, Ethan Michael Chitkara, we established a fund and raised money through a series of “spinathons” (indoor cycling endurance rides) as well as through the generous donations of friends and family. Today we would like to donate $31,437 to Cure SMA to contribute toward its vision of a world without SMA. Whether the funds are directed toward research, drug discovery, clinical trials or clinical care, we know that Cure SMA will put the funds to the best use to achieve its goals.

Ethan had SMA Type I and was with us for only 15 months. However, during that short lifetime, he taught us much about tolerance, patience, and strength. Through him, we were privileged to meet many incredible caregivers, doctors, nurses, therapists, and friends. We were able to see the good in so many people as we experienced this journey together with Ethan. He was a blessing, and he was our Sunshine.
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

**Vermont Chapter**

**Vermont Walk-n-Roll**

Our second year—done!! A successful and fun Cure SMA Walk-n-Roll in Burlington, Vermont, was held in May of 2016 at Oakledge Park. $15,000 was raised by family, friends, and sponsors in honor and memory of those affected by SMA.

All of the work and coordination to hold an event like this pays off on the day of the event when we see our totals racking up, and our walkers and rollers enjoying the day. It’s a wonderful feeling to be with a group of people that all know what the letters SMA stand for and know that they have come together—even if for only one day—to truly make a difference.

It’s an honor to be a part of this event and to work alongside a great bunch of SMA families, friends and business partners. We are looking forward to our 3rd walk in May 2017, and hope to raise more funds for research, awareness and family support.

Sue O’Neill | Milton, VT

**New Mexico Chapter**

**Chapter Update: Greetings from the New Mexico Chapter!**

The New Mexico Chapter is currently looking for volunteers interested in becoming more involved with the chapter. We are searching for individuals to assist with family support, events & fundraising, communications, and advocacy. If you or someone you know are interested in any of these opportunities, please contact the New Mexico Chapter at newmexico@curesma.org.

**North Jersey Chapter**

**2016 Cure SMA North Jersey Chapter Walk-n-Roll**

The Inaugural Cure SMA North Jersey Chapter Walk-n-Roll was held on June 25, 2016 at Roosevelt Park in Edison, NJ. Thank you to all of the dedicated fundraisers and sponsors who helped make this day a huge success! The fun-filled event included activities for the whole family to enjoy such as a balloon artist, face painting and games!

The event brought together roughly 300 runners, walkers and rollers to help fund a cure for SMA. In total, this year’s event raised $17,344.

Thank you to all of the amazing SMA families, participants, and donors for helping make this event possible. Big thanks to our teams: Carlos & Jeanette, JMP Supports Jayden, Lael’s Walk Stars, PromptCare, Ray’s Flamingo Flock, Team Aubrey Paige, Team Butterball, Team Jayden and Tiny Tots Superheroes.

A special thank you to Anna Pham, Kara Hartnett and Zainab Jaffer for all of your hard work in planning this fun and successful event! We’d also like to thank Diana D’Souza and all of the wonderful volunteers who made the event run smoothly!

**Walk and Roll for Micah**

Gabriella Biello hosted the “Walk and Roll for Micah” on June 11, 2016 in Millstone, NJ. She raised over $5,000! $5,115 to be exact. Her cousin Micah has SMA type I and she wanted to try to help Micah and kids like him. She didn’t know how much money she would be able to raise because she is only ten but she decided she had to give it her best shot. She planned a super fun event with lots of help from her school and groups around town.

An organization, It’s A Party, donated a giant bounce house and slide to the event. The school PTO was generous enough to let her borrow their large carnival games and thanks to Cure SMA. Gabriella had lots of prizes like bracelets and tattoos to give out to winners. Vesuvios, the best pizza place in the area, brought their food truck to the event. My aunt Keren owns Sunshine By Design and they came and did the most amazing face painting at the event.

Gabriella’s biggest ‘sponsor’ was Saker Shop Rite. She used some of the money to buy drinks and snacks to sell at the event and put the rest towards her profit. “Mr. Saker didn’t just donate to SMA he even came to the event with his family and told me I did a great job and to keep up the good work. He made me feel successful and proud of myself”.

Gabriella wants people to know that, “even if you’re young or don’t have experience you can still make a difference. Reach out to the people around you, I bet you’ll find they are eager to help! And raising awareness is important too. My brother Harry shaved “SMA”
Kellie Cusack is 17 years old. Since the fourth grade Kellie has held bake sales, lemonade stands, and bowl-a-thons with proceeds being donated to Cure SMA.

Kellie attends Vernon Township High School. She was able to speak to her classmates during their morning announcements in December 2015. She explained what spinal muscular atrophy is and how she is often hospitalized due to respiratory distress. Kellie loves to bowl and so do many of her friends. Her high school has a bowling team, so Kellie thought it would be fun to host a Bowl-a-Thon. She helped design and put up posters throughout her school. Kellie thought it would be fun to have everyone come dressed as their favorite superhero. Kellie loves Wonder Woman and Super Girl.

Then on March 5, 2016, Kellie went bowling with her friends at Sparta Lanes in Sparta, NJ with all proceeds being donated to Cure SMA. Her friend Nick (aka DJ Wheels) came and played music! Everyone was able to bowl two games and had pizza. Kellie gave out a prize to the person with the most creative costume, her friend Nina won! Kellie was able to raise close to $1,000!

Dawn Cusack | McAfee, NJ

The North Texas Chapter hosted the first Cure SMA and Dallas Mavericks game on February 28, 2016 to build community and spread SMA awareness. Attendees enjoyed meeting other SMA families while watching the Mavs take on the Minnesota Timberwolves. The event raised $420 from ticket sales. Thank you to Michele Erwin for organizing this fun family day for the chapter!

Family Day at the Dallas Zoo

On March 26, 2016 the Dallas Zoo generously waived admission for individuals and families affected by SMA. Due to the zoo’s generosity, eight North Texas Chapter families were able to visit the indoor facilities free of charge and experience the attractions together. Thank you to the Dallas Zoo for making this event possible for our local Cure SMA families!

Corran’s First Birthday

Thank you to the Pate Family for fundraising in memory of their son, Corran, and donating $100 to Cure SMA. A note from Corran’s father, David Pate: “Corran was diagnosed with SMA type I in April of 2015. March 10th, 2016 would have been Corran’s first birthday. This fundraiser is dedicated to celebrating the wonderful boy he was and to help find a cure to help other children not suffer from SMA.”

Bren Truesdale, Jr.’s Birthday

Thank you to Joan Truesdale, for donating $50 in memory of her son Bren and his birthday this year.

5th Annual Best Meatball Contest in Secaucus

The 5th Annual Best Meatball Contest in Secaucus event was held on April 30, 2016 at the Immaculate Conception Church Gym in Secaucus, NJ. This year’s event was hosted by the local Knights of Columbus and is held in honor of Daniel Cevallos. In total, $5,245 was raised for SMA research! Thank you to Giovanni and Paulina Recalde for organizing this event!
Northern California Chapter

Concert for a Cure

The 16th Annual Concert for a Cure was held on May 14, 2016 at the Diablo Country Club in Danville, CA and was a tremendous success! This year’s event raised over $165,000 with the unique theme of the “Olympics” being in Rio this summer. This memorable evening was made especially rewarding for donors, families and guests when Dr. John Day, Stanford Professor for Neurology, Pediatrics, and Pathology, highlighted the exciting advancements in SMA and the IONIS clinical trials.

Guests were deeply moved by Danny McHale and Arianna Dindzan’s presentation along with a special check presentation from Danny’s classmates at Monte Vista High School in Danville where they raised over $6,000. The 40 plus high school students and their siblings were a huge hit as this marked their seventh year serving at Concert for a Cure selling raffle tickets, running games and encouraging donors to give generously.

Mary and Joe McHale founded Concert for a Cure in 2001 shortly after the diagnosis of their son, Danny, with SMA type II. Nancy and Andris Dindzans joined the efforts in 2003 when their daughter, Ariana, was diagnosed with SMA type III. Both Danny and Ariana will turn 15 years old this year. Over the years this event has raised over $1.6 million to fund Cure SMA research programs.

THANK YOU! The McHale and Dindzans families send out their heartfelt thank you to our friends, families, and local community for their generosity in their continuous support of SMA families and children everywhere.
Thank you to the Cassano Cares Foundation for their incredible support of Cure SMA. The foundation hosted a golf tournament in Dayton, OH on September 28, 2015 to benefit Cure SMA. Cassano Cares donated over $45,000 in honor of Maggie and Charlie Monnin.

Thank you to the Cassano Cares Foundation, Cassano’s Pizza King, Chip Cassano, and the Monnin family for their incredible support!

The 5th Annual Cure SMA Birdies for Blake Golf Outing was a huge success! July 30, 2016 turned out to be a beautiful day at the Green Crest Golf Course in Liberty Township, OH. Over 100 golfers hit the green, and raised over $22,000 for Cure SMA! This amazing event has raised nearly $100,000 over the past five years. A special thank you to the entire Farrell family for all of their hard work on this event and dedication to Cure SMA in honor of Blake Farrell!

The inaugural Norwalk Walk-n-Roll was a huge success raising over $6,400 for Cure SMA! Hannah Barry started this event in honor of her son, Travis, who has SMA. Participants enjoyed a morning full of fun at Whitney Field in Norwalk, OH on June 5, 2016. There were lots of fun games and music, and tasty snacks for everyone.

Thank you to all 29 teams that participated in the 2016 OKI Chapter Walk-n-Roll. It was a morning full of fun on June 11, 2016 at the Cintas Center in Cincinnati, OH. Everyone enjoyed the face painting, balloon artist, fantastic raffle, and heartfelt butterfly release in memory of those that we have lost.

Thanks to the support of generous donors and sponsors and the continued efforts of our dedicated fundraising teams over $74,600 was raised for Cure SMA! A special thank you to the Walk-n-Roll planning committee for making this such a successful event: Bob and Elizabeth Lockwood, Kevin and Beth Lockwood, Amy Haake, and Mark and Nicole Haake.

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**OKI Chapter continued**

### Will’s Day at the Bay

Thank you to everyone at Jet Express for hosting Will’s Day at the Bay on June 8, 2016 in Put-in-Bay, OH. This annual event is held in memory of Will Blumensaadt who passed away in 2005. Thanks to the participation of the local community, $4,600 was raised for Cure SMA! Special thanks to the Blumensaadt family for their continued dedication to our organization.

### Joshua McKee Bullock Cure SMA Sporting Clays Invitational

Thank you to the family of Joshua McKee Bullock for putting on an amazing event! The inaugural Joshua McKee Bullock Cure SMA Sporting Clays Invitational was held on June 25, 2016 in Medina, OH. Participants enjoyed a day of shooting sporting clays, and raised over $3,500 for Cure SMA! Special thanks to Jarrod Rapalje for spearheading this fundraiser!

### 2016 Kentucky Derby Festival Marathon

Team Cure SMA raised nearly $5,000 through our first year as a charity partner with the Kentucky Derby Festival Marathon. Participants ran either the marathon or half marathon on April 30, 2016 in Lexington, KY. Thank you to all of our runners: Bryon Bartley, Shleebra Bartley, Wayne Cornett, Whitney Harris, Andrew Harris, Ashley Hawkins, Leslie Leitch, Ally Underwood, Carie Whitaker, and Emily Whitaker.

**Pacific Northwest Chapter**

### Graham’s First Birthday

For Graham Vollmer’s first birthday party in May, his friends and family had a great time celebrating his special day and sweet Graham. Thank you to all of Graham Vollmer’s friends and family for also raising $300 for Cure SMA and for the adorable knit hats that were hand made for our care packages!

### Family Picnic in Tacoma

The Pacific Northwest Chapter got a jump start on SMA Awareness month by hosting a picnic for SMA families in Tacoma, Washington, on July 30, 2016 at Wright Park. The day was full of fun as everyone enjoyed a shared picnic, meeting new friends, the playground, spray ground and feeding the ducks. The picnic was held during the “Ethnic Fest” where hundreds attended and many people asked about Cure SMA, so we were able to spread the word.

A special thank you to Charlotte Shepherdson for organizing this great annual event for the SMA community in the Washington area.
In its fourth year, the Muscles for Mckenna Gala raised over $95,000 in support of Cure SMA! This event was held on March 18, 2016 at The Drexelbrook Grand Ballroom in Drexel Hill, PA. The night included dinner, drinks, live entertainment by The Heartbeats, dancing and a silent and live auction. This event was flawless with a red carpet entrance and photography by Henry Leong.

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

Special thanks to Jim Ellixson for chairing this event in honor of his daughter, as well as to Amy Ellixson and the wonderful committee of volunteers. Thanks to their time, energy and dedicated support this event was a huge success and continues to lead Cure SMA toward a treatment and cure.
2016 Cure SMA Pennsylvania Chapter Walk-n-Roll

The Cure SMA Pennsylvania Chapter Walk-n-Roll hosted its 13th annual event on May 15, 2016 at the Lloyd Hall Recreation Center in Philadelphia, PA. Thank you to all of the dedicated sponsors who helped make this day a huge success! The fun-filled morning included activities for the whole family to enjoy! The event brought together roughly 600 walkers and rollers to help fund a cure for SMA. In total, this year’s event raised almost $90,000.

Thank you to all of the amazing SMA families, participants, and donors for helping make this event possible. Big thanks to our teams: Abby's Army, Angels MI, CHOP RT’s, Cousin Crusin for a Cure (Kerri & Eric), Flash Redfearn, Jeremiah Fighting the Fight, Fighting SMA Together: Lydia Love Bug & Gabie Baby, Lukie’s Tigers, Matthew’s Musclemen, Miracles for Marc, Muscles for Mckenna, Omar’s Soldiers, Peter’s Philadelphia Eagles, Philly SMack Down, Sweet Baby Zane, Team BAYADA, Team Jordan, Team Lyla, Team Marc, Team Saxton, The Jack Pack.

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

2016 Cure SMA Pittsburgh 5K & Walk-n-Roll

The Cure SMA Pittsburgh 5K & Walk-n-Roll hosted its inaugural event on April 17, 2016 at the South Park Museum Building in Pittsburgh, PA. Thank you to all of the dedicated sponsors who helped make this day a huge success! The fun-filled morning included activities for the whole family to enjoy! The event brought together roughly 200 runners, walkers and rollers to help fund a cure for SMA. In total, this year’s event raised $15,487!

Thank you to all of the amazing SMA families, participants, and donors for helping make this event possible. Big thanks to our teams: FIT4MOM, Colton Can, Cooper Can, Hope for Brian, Walk 4 Joey & Tina.

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

2016 DICK’S SPORTING GOODS Pittsburgh Marathon

The DICK’S SPORTING GOODS Pittsburgh Marathon took place on May 1, 2016 in Pittsburgh, PA. The day consisted of a great race taking runners over five different bridges to cross the three main rivers that run through the city as well as 13 different neighborhoods. In our first year of this race, Team Cure SMA had 4 runners participate. Together, they raised $7,796 for Cure SMA!

A special thanks to our runners: Stephen Becker, Carson Gross, Patrick Neff and Carolyn Tomko!

Casual 4 A Cause

Special thank you to the staff at Central Bucks School District for hosting the Casual 4 A Cause fundraiser on April 11, 2016 in Buckingham, PA in honor of Mckenna Ellixson, SMA type III. Together they raised $230 for Cure SMA and the Muscles for Mckenna Gala.

Danikka Pons

Thank you to everyone who participated in our Cure SMA walk! We were able to raise $200 for the Cure SMA organization.

13th Annual Rocky Mountain Charity Golf Tournament Colorado

The 13th Annual Rocky Mountain Charity Golf Tournament took place on Monday, May 23, 2016 at the beautiful Club at Pradera in Parker, CO. Congratulations to this year’s tournament winners Tyler Cowhick, Graig Sells, Eric Ostberg, and Chris Deeds! With the support of this year’s sponsors, donors and players, the event raised more than $24,000 to fund a treatment and cure for SMA.

Cure SMA would like to extend a special thank you to event organizer Gillian Faith and her family for hosting the tournament. Your continued efforts and dedication are greatly appreciated.
2016 Evening of Hope Beer and Cheese Pairing

On March 10, 2016 the Cure SMA Rocky Mountain Chapter hosted the Third Annual Evening of Hope Beer and Cheese Pairing in Denver, CO. The event featured a custom tasting menu of 6 beers crafted by local merchant Chain Reaction Brewing Company, 6 specially paired artisanal cheeses and some exciting raffle and silent auction items. Over 90 guests attended the event which raised more than $11,800 for SMA research and family support!

A special thank you to Marla Marlow for chairing the event, as well as the Chain Reaction Brewing Company crew and King Soopers team for donating their time and services to make this year’s event a great success. Your support made this night possible and continues to lead Cure SMA closer to a treatment and cure for spinal muscular atrophy.

Quilt Raffle Fundraiser

Thank you to Teresa Friede for hosting a quilt raffle in honor of her son, Brandon Friede, Cara Wherley, and Veronica St. Onge. The raffle raised $1,140 for Cure SMA. Congratulations to the winner of the one-of-a-kind quilt, Laree Morris.

Team Cure SMA – Rock ‘n’ Roll San Diego

The Rock ‘n’ Roll Marathon Series San Diego was held on June 5, 2016 in downtown San Diego, CA. Everyone teamed up to run the San Diego Rock ‘n’ Roll marathon and half marathon to raise money and awareness for Cure SMA. In total, Team Cure SMA raised over $7,142 to find a treatment and cure for SMA. Thank you, to all that came out to run and support our team! See you in 2017!

Southern California Chapter

Score4Sky

Score4Sky held their first adaptive sport event on January 30, 2016 in Newport Beach, CA, at Sage Hill School. The founder of Score4Sky, Spencer Bahrenburg, taught the group modified basketball skills. The kids enjoyed the afternoon and the snacks.

Spencer Bahrenburg is a senior at Sage Hill and founded Score4Sky in memory of his brother, Skylar Bahrenburg. Skylar made a huge impact on his life. Ever since Spencer was a kid, he loved sports. When it came to sports, Spencer and Skylar, knew everything from players to commentators. Skylar never let SMA stop him from playing the sports he loved. Sure, he had to improvise things a little, but he was still playing sports like basketball, soccer and baseball. Thus, with the help of classmates, Score4Sky was created.

Inaugural Cure SMA Orange County Walk-n-Roll

The Inaugural Orange County Walk-n-Roll in Costa Mesa on April 30, 2016, was a blast! We began the day with all the teams gathering and heading to the start line for a beautiful walk around the various ponds and rock formations and ended with a wonderful lunch provided by a local taco cart business. All the kids had a wonderful time playing on the accessible playground and enjoyed an adaptable sport, basketball, by Spencer Bahrenburg who founded Score4Sky.

Thank you to everyone who supported our teams: Miles for Miles, Team Sal, Mr. Kennedy, Abel’s Ambler, All for Aubs, Familia Diego, Team Travis, Wheels2Independence, Giant Leaps for Neil and Convaid Trekkers.

Thank you to the Southern California Chapter for hosting the Inaugural Walk-n-Roll and raising $10,871!
South Florida Chapter

Jacob’s Run, Walk & Roll to Cure SMA

Jacob’s Run, Walk & Roll to Cure SMA was held on April 3, 2016 at South County Regional Park in Boca Raton, FL. Over $100,000 was raised this year!

Hundreds gathered to remember Jacob and all those that we have lost to SMA, and celebrate the lives of those affected by SMA and how far we have come in SMA research. Everyone enjoyed the raffle, bounce houses, petting zoo, and video games at the event.

The Jacob Isaac Rappoport Foundation was founded in memory of Jacob Rappoport by his parents, Adi and Shaina Rappoport. To date, the foundation has raised over $1.4 million dollars for SMA research and support programs.

The foundation generously provides funding for SMA research programs, care packages for children with SMA type I, and programs at the Annual SMA Conference like the Teen Social, Type I Reception, and Childcare Program.

Thank you to Shaina and Adi for their incredible support of Cure SMA and all that they do for the SMA community.
On April 3, 2016 eighty women from The Bridges in Delray Beach Florida gathered together for a Mah Jongg tournament to help raise money for Cure SMA. It was a great time for a great cause and over $650 was raised! Thank you to Lysa Oakner for all of her hard work putting together this event.

On May 8, 2016, 19 participants came together and took the streets of Wilmington to run in the Delaware Marathon to support Cure SMA. The teams were organized by John and Nicole Cheslock whose daughter Eden is affected by SMA. This is the fifth year the Cheslock’s have been committed to this race and raising money to benefit SMA. Team Cure SMA raised $3,973!

Special thanks to our half marathon runners: Nicole Cheslock, Jessica Moyer, and Alyssa Sweeney!

Special thanks to our 4-person and 8-person relay teams: John Cheslock, Christina Colon, Brian Cusick, Michael Forester, Dean Holden, Kimberly Holden, Aaron Janusz, Alex Janusz, Lauren Janusz, Zoey Janusz, Blaise Moyer, Anoma Russum, Sarah Sharp, Kirsten Wolflington, Zara Young and Debbi Zarek.

Over 80 runners gathered in Pikeville, TN on May 21, 2016 to run in memory of Bentley Bassamore. Everyone enjoyed the scenic run through Pikeville. Thanks to the generous support of sponsors and runners, over $10,000 was raised for Cure SMA! Thank you to Carie Whitaker for her continued dedication to Cure SMA and all the work she puts into this event every year.

Thank you to The Medford Nova Care Staff of Medford, NJ. They donated $900 in honor of William Chernets for Cure SMA!

Thank you to the Whited Family for hosting a garage sale in honor of Alana Whited in Lebanon, TN in April 2016 and raising $350 for Cure SMA!
Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.

Texas Chapter

2016 Fayetteville 5K & Walk-n-Roll in honor of Annabelle Combs

The inaugural Fayetteville 5K & Walk-n-Roll in honor of Annabelle Combs took place on May 7, 2016 at Oak Thicket Park in Fayetteville, TX. Despite heavy flooding the week prior to the 5K & walk, the weather was beautiful on event day with the sun shining and clear blue skies. Over 220 participants attended raising more than $14,000 for SMA research and family support services.

We would like to extend a special thank you to event organizer Alyssa Combs, as well as all event day volunteers, sponsors, and donors. The event would not be possible without your support. Thank you!

Utah Chapter

2016 Utah Walk-n-Roll & 5K in memory of Harrison Bradshaw

The 2016 Utah Walk-n-Roll & 5K in memory of Harrison Bradshaw was held on March 12, 2016 at Liberty Park in Salt Lake City, UT. Several SMA families along with friends and the local community came out to enjoy the activities and entertainment. Over 115 participants attended the event raising more than $3,500 for SMA research and family support services.

We would like to extend a special thank you to our event organizers, Mary Jane Wallin, Heidi Hewitt, and Stephanie Bradshaw, as well as all event day volunteers, sponsors, and donors. The walk and 5K would not be possible without your support. A big thank you also goes out to Club Rock for providing an action-packed live performance during the event.

Virginia Chapter

Talbots Make a Difference Day

On April 15, 2016, Cure SMA and Talbots partnered to host a fundraising event in Houston, TX. Customers were invited to shop, enjoy refreshments, and learn about spinal muscular atrophy. During the event Talbots donated 10% of sales to Cure SMA, raising $580 in just four hours. A special thank you goes to Talbots Town & Country Village manager Chieuanh Nguyen for organizing this opportunity to spread SMA awareness and raise funds for a treatment and cure. Thank you!

Grace Messana Fundraiser

A special thank you to Putnam Valley High School in Putnam County, NY, for donating $800 to the Cure SMA Virginia Chapter in honor of Maxwell Dash, SMA type II. Several clubs were involved in this effort including the Make a Difference Club, the National Honor Society, and the Future Teachers’ Club. Students held several bake sales, pizza sales and various fundraisers to raise these monies. Max’s grandmother, who is a teacher at PVHS, has participated in SMA walks and has raised awareness in her school district and community. The students are thrilled to be able to help such a worthy cause and participate in the challenge of helping to find a treatment and cure.
**2016 Rock ‘n’ Roll DC Marathon**

The 2016 Rock ‘n’ Roll DC Marathon took place on March 12, 2016 in Washington D.C. The day consisted of a great race taking runners past six major landmarks throughout the D.C. area including the Lincoln Memorial, White House, Smithsonian Museum and more! In our first year of this race, Team Cure SMA had 3 runners participate. Together, they raised $2,808 for Cure SMA!

A special thanks to our runners: Christian Daubert, Karissa Holcombe and Catherine Jeon!

**Saxatones**

Thank you to Emily Ressler and Nora Gensler of the Georgetown Saxatones in Washington, D.C. for donating $539 to Cure SMA. This donation was made possible through proceeds from their annual benefit concert, Spring Sing!

**Western New York Chapter**

**2016 Binghamton Walk-n-Roll**

The 2016 Cure SMA Binghamton Walk-n-Roll was a huge success and raised over $6,000! We walked in honor of Kinsley on July 16, 2016. Kinsley’s parents formed Team Kinsley, our largest fundraising team for the event! Along with the Sweet Frogs, Chuck E Cheese and Ballwinkle from the Binghamton Mets, we kicked off our walk in style following the ever anticipated Hula Hoop Contest! Thanks to each and everyone of you who attended, walked, volunteered and donated, we have surpassed our Goal this year! This event would not have been possible without you! We look forward to seeing you next year!

**Wisconsin Chapter**

**Brady Walk**

For six and a half years, I’ve been an employee at Brady Corporation in Milwaukee, Wisconsin, and have participated in the Brady Walk, an annual employee event to support our community. Employees are given the opportunity to vote for the organizations they want to support during the walk. How the event works is that for each employee who signs up to walk five miles, Brady donates $70 toward their chosen organization. The company also hosts a food drive around the walk for the Hunger Task Force, a local Milwaukee food bank and anti-hunger advocate. For each food item contributed by employees, Brady donates $2 towards the charity that you are walking for.

Cure SMA was chosen as one of the four organizations that the employees wanted to support during the Brady Walk. With the help of many of my co-workers, we were able to raise over $12,700 for Cure SMA. I have been amazed that over the past three years, Brady has been able to donate $29,700!

I work hard to get a chance to spread the awareness of SMA for my four year old niece, Elise. She was diagnosed with SMA type I in February 2013 and has shown so much strength, courage and love through all of this. She is my inspiration for everything. I am so appreciative of everyone’s help to donate to Cure SMA, this organization has been so gracious to our family and I’m thrilled to have been able to give back to them and help find a cure for SMA!

Sarah Drake | Milwaukee, WI

Cure SMA would like to thank Sarah Drake for nominating Cure SMA again to become one of the chosen charity partners for her company walk and for spreading awareness to her fellow employees about SMA!

**2016 Pig Roast Fundraiser**

The 6th Annual Roast SMA was held on June 11, 2016. Alex, Charli, Molly and Kale enjoyed the beautiful day. Fishing, games, good food, swimming and great fun was enjoyed by all. The pig and refreshments were again donated by Uncle Brad and Aunt Peg who hosted the event. A total of $825 was donated by friends and family.

Note: The amounts raised and shown are totals as of July 31, 2016 and may differ from current fundraising totals by the time you get this newsletter.
3rd Annual Strike Out SMA

The 3rd Annual Strike Out SMA Wisconsin Chapter Bowl-a-Thon was held on May 14, 2016 in Milwaukee, WI. The event had a great turnout with over 100 people joining in on the family fun and raising over $8,000 for Cure SMA! Thank you to our sponsors: Valor Development, MillerLaw, S.C., LuLaRoe by Kate & Dany, and Madaus Trucking. Special thanks to our number one volunteer, Laura Dolezar and Northwestern Mutual. There were also great raffle prizes from the Milwaukee Brewers, Green Bay Packers, Milwaukee Bucks and many other local businesses. We hope to see everyone at the 4th Annual event next year!

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

In Loving Memory of Gail Rose Meyers

Gail was a proud grandma to Kennady Quinnell, SMA type I. She frequently would shop for toys and items that would help Kennady feel independent despite her physical limitations. She thought the care packages that Cure SMA sent out to families affected by SMA were a blessing. Inside these packages are light weight toys and items that focus on the strengths children with SMA can enjoy even if their physical abilities may limit movement. There were so many items that helped us focus on the special abilities Kennady had, which gave Gail the inspiration to find even more to bring Kennady when she would visit her. Through Kennady’s online memorial Gail raised $695 towards SMA research and family support programs. May her memory be carried on through supporting items that give other SMA children independence and joy.

SAVE THE DATE!

2017 Annual SMA Conference

which will be held in Disney World, Orlando, FL June 29 – July 2, 2017!

If you are interested in being added to our mailing list for the 2017 Annual Conference, please email exhibitor@curesma.org.
SAVE THE DATE!

2017 Annual SMA Conference

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