2015 Annual SMA Conference
KANSAS CITY, MO AT THE WESTIN CROWN CENTER
The Westin Kansas City at Crown Center
Crown Center, 1 E Pershing Rd,
Kansas City, MO 64108
(816) 474-4400

For any questions please see us at the Registration Area.

WORKSHOP SESSIONS

**Session #1**
- Sharing your Type I Experience
- Sharing your Type II Experience
- Sharing your Type III Experience
- Adults with SMA – Roundtable
- Grandparents Talk it Out Type I
- Grandparents Talk it Out Type II & Type III

**Session #2**
- What it Means to be a Teen on Wheels
- Genetics and Reproductive Options for SMA Families
- Healing the Grieving Heart – Part 1
- Fundraising 101 – Planning and Executing
- Medical Management of Adults with SMA
- Standing, Walking and Mobility: Decision Making and Options
- Dad’s Time: A Workshop for Fathers Only

**Session #3**
- Breathing Basics and Care Choices for Type I
- Getting the Most Out of What You Eat! Nutrition for Oral Feeders
- Orthopedic Management
- Driving and Community Mobility
- How to Be a Healthy Parent
- Keepsake Creation: Grieving Through Art
- Life Care Planning

**Session #4**
- Family Readiness for Emergencies
- Breathing Basics for Type II and Type III
- Healing the Grieving Heart – Now What? Part II
- Dating, Relationships, and Sexuality
- Yoga Therapy
- Personal Care Assistants—How to find, hire and manage your PCA’s
- Recognizing and Managing Pain in SMA

**Session #5**
- Tube Feeding and SMA: Recommendations and Practices
- Life After School: What’s Next?
- Writing Our Stories: A Parent’s Workshop in Healing Through Creativity
- The Role of PT in your Child’s Life—Being a part of a Therapy Team
- Ethics in Research
- Toy Adaptation
- Transition to Adulthood
# Conference Summary

## Thursday, June 18th – Newly diagnosed families only
- **Newly Diagnosed Program (For Newly Diagnosed Families Only)**
- **Newly Diagnosed Meet & Mingle**
- **Session A – Type I**
- **Session B – Type II & Type III**
- **Session C – Grieving**

## Thursday, June 18th – All conference attendees
- **7:30am – 5:30pm**
  - Registration Open for all Conference Attendees
- **6:30pm – 8:30pm**
  - Meet and Greet/Family Fun Fest for all conference attendees
- **7:30pm – 10:00pm**
  - Teen Social
- **7:30pm – 10:00pm**
  - Adults with SMA Social

## Friday, June 19th
- **7:30am – 5:30pm**
  - Registration Open
- **7:30am – 5:00pm**
  - Exhibitors
- **7:30am – 8:45am**
  - Continental Breakfast
- **9:00am – 10:15am**
  - General Session
- **10:30am – 12:30pm**
  - Workshop Session #1
- **12:30pm – 2:00pm**
  - Lunch Break
- **2:00pm – 3:30pm**
  - Workshop Session #2
- **6:00pm – 7:30pm**
  - Family Friendly Research Poster Session
- **7:30pm – 9:30pm**
  - Family and Researcher Dance Party

## Saturday, June 20th
- **7:30am – 5:15pm**
  - Registration Open
- **7:30am – 5:15pm**
  - Exhibitors
- **7:30am – 9:00am**
  - Continental Breakfast
- **9:00am – 10:30am**
  - Workshop Session #3
- **10:45am – 12:15pm**
  - Workshop Session #4
- **12:15pm – 1:30pm**
  - Lunch Break
- **1:30pm – 3:00pm**
  - Workshop Session #5
- **3:15pm – 5:15pm**
  - General Session / Researcher Q & A
- **6:45pm – 9:30pm**
  - PJ Party/Movie Night

## Sunday, June 21st
- **7:30am – 9:00am**
  - Family Breakfast Buffet
- **9:15am – 11:30am**
  - Closing General Session – It’s a Wonderful Life Panel

**Note:** Children’s Program rooms open during meeting times only.
Dear Families, Researchers, Care Providers and Friends,

Welcome to The 2015 Annual SMA Conference. This year our meeting will be different from those we have held in the past. Although at its core it will also be unchanged from our first conference 27 years ago.

We will be covering several new and often complicated topics this year. Clinical trials and FDA approvals, and even insurance coverage, are now critical and urgent issues for us to address. In addition, patient care is becoming increasingly important for us as we look forward to an era of approved drug treatments. These new issues are all due to our success in advancing the SMA drug pipeline.

We now have 7 clinical trials testing new SMA therapies, with 18 total programs in various stages of drug development and 15 companies investing in SMA drug programs. Your individual strength and our strength as a community have made all this possible!

This year we have a new clinical trials educational package for all conference attendees, and we have changed the schedule so that our usual closing session Q&A panels with researchers have been moved to Saturday afternoon. These panels will focus on issues such as inclusion/exclusion criteria and expanded access, along with reviewing the specific new drug clinical trials that are now in process.

The core of this conference though has always been about, and will be again this year, our community coming together to support one another. As families, researchers and care providers all with the same goal, we come together in the same place to share information and hope.

On Friday we will be announcing an exciting new role for a familiar face in our community. And somewhat “unimaginatively” we will need to return to places from the past for both of our conferences in the next 2 years, which we will announce to you all on Sunday. We will be ending our conference this year with our traditional It’s a Wonderful Life panel, along with a celebration of our community, chapters and families.

Very best,

Kenneth Hobby
President.
Dear Families, Researchers & Clinicians,

Welcome to the 2015 SMA Conference! As you know, the mission at Cure SMA is leading the way to a world without SMA. This conference is one way we try to do that. Importantly, the conference is being held simultaneously with the 19th SMA Researcher Meeting, where leading scientists from around the world congregate to discuss ways to combat SMA.

We encourage all of you to engage the researchers. The researchers value these interactions and often say things like, “The value of meeting with researchers, physicians and parents all in one place is immeasurable. Parents see how much we care, physicians see how much work is required for drug development, and researchers see how much their hard work is appreciated.”

Our hope is that the specific goals for the Researcher Meeting, which are listed below, result in a more motivated, collaborative, and productive SMA research community!

- To provide an open event for all SMA scientists to share new research data
- To facilitate collaboration among different scientists, such as basic, clinical, and industrial researchers
- To integrate new researchers and drug companies into the SMA community
- To motivate researchers with direct interactions with families and patients

We have about 250 scientists registered for the 19th SMA Researcher Meeting, with 110 presentations planned. Importantly, about a dozen different pharmaceutical and biotech companies are participating this year, and we now have more than half-a-dozen drug candidates for SMA entering clinical trials. There is real industry interest in SMA drug development today!

We have two research events scheduled for families and researchers to interact. The first is our third annual Family and Researcher Poster Session, during which researchers will explain their projects one-on-one. Second, on Saturday afternoon, we have planned a research update session to explain the clinical trials process and to provide status updates on SMA drug programs.

I look forward to meeting many of you over the next several days and to sharing exciting new research breakthroughs.

All the best,

Jill Jarecki, PhD
Research Director
Cure SMA

**SMA DRUG PIPELINE: JUNE 2015**

This year, we are funding research with more breadth, depth, and diversity than ever before. This chart shows the drugs and therapies that are currently in the pipeline for SMA, including a few that are just steps away from potential FDA approval.

<table>
<thead>
<tr>
<th>ORGANIZATION/DRUG NAME OR APPROACH</th>
<th>IND = Investigational New Drug</th>
<th>FDA APPROVAL</th>
</tr>
</thead>
<tbody>
<tr>
<td>Isis/Biogen/ISIS-SMNRx</td>
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<td>Genentech/Roche/Olesoxime</td>
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<td>AveXx/NW/Gene Therapy</td>
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<td>Roche/PTC/RO7600</td>
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<td>Novartis/LM107</td>
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<td>Repligen/Quinazoline</td>
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<td>Cytenklin/Otis/Adelis/CK-217107</td>
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<td>Neustem/Motor Neuron</td>
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<td>Paratek/Tetracycline</td>
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<td>BioBrick/Pharma/Small Molecule</td>
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<td>Sunnyside/CNS/Gene Therapy</td>
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<td>Genethon/Gene Therapy</td>
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<td>CALIBR/Small Molecule</td>
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<td>OSU/UM/Morpholine ASD</td>
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<td>Indiana U/Small Molecule</td>
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<td>RaNA Therapeutics/IncRNAs</td>
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<td>AurimMed Pharma/Small Molecule</td>
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<td>Harvard/Small Molecule</td>
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**IND = Investigational New Drug**
**NDA = New Drug Application**

**BASIC RESEARCH SEED IDEAS**

**PRECLINICAL: DISCOVERY**

**CLINICAL DEVELOPMENT**

**FDA APPROVAL**
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.
Win an iPad!

Fill out the conference survey for your chance to win an iPad

The success of our conference can only be improved by the feedback that we receive from you! Please take the time to provide any comments by filling out our conference survey, regarding this year’s conference. You can find a hard copy of your survey located in your conference folder. Please drop your completed surveys into the many bins throughout the meeting space or at the Registration Area.

All Participants who complete the survey by 10:30am on Sunday, June 21st, will have their name entered into a raffle. The winners of the raffle will receive a brand new iPad! All completed surveys will also be entered into a drawing for a chance to win a trip, including airfare generously donated by ISIS Pharmaceuticals, to either The 2016 or The 2017 Annual SMA Conference (the locations will be announced on Sunday). The winners will be drawn and announced on Sunday, June 21st at the Closing General Session/It’s a Wonderful Life.

You can also view and fill out the survey on-line at https://www.surveymonkey.com/s/2015AnnualSMAConference

Everyone at Cure SMA would like to thank Isis who provided us with two Apple iPad’s and funding to help a family attend an upcoming conference, for the survey drawing. Their generosity will make these SMA families extremely happy and grateful!
## Recommended Specialized Workshop Session Tracks

### Type I

<table>
<thead>
<tr>
<th>Session</th>
<th>Subject</th>
<th>Room</th>
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</thead>
<tbody>
<tr>
<td>Session 1: Friday 10.30am to 12.30pm</td>
<td>Sharing your Type I Experience</td>
<td>Century Ballroom B</td>
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<tr>
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<td>Grandparents Talk it Out Type I</td>
<td>Roanoke</td>
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<tr>
<td>Session 2: Friday 2.00pm to 3.30pm</td>
<td>N/A</td>
<td>Roanoke</td>
</tr>
<tr>
<td>Session 3: Saturday 9.00am to 10.30am</td>
<td>Breathing Basics and Care Choices for Type I</td>
<td>Roanoke</td>
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<tr>
<td>Session 4: Saturday 10.45am to 12.15pm</td>
<td>N/A</td>
<td>Roanoke</td>
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<tr>
<td>Session 5: Saturday 1.30pm to 3.00pm</td>
<td>Toy Adaptation</td>
<td>Roanoke</td>
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### Type II

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<tr>
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<td>Sharing your Type II Experience</td>
<td>Century Ballroom A</td>
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<tr>
<td>Session 1: Friday 10.30am to 12.30pm</td>
<td>Grandparents Talk it Out Type II and Type III</td>
<td>Mission</td>
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<tr>
<td>Session 2: Friday 2.00pm to 3.30pm</td>
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<td>Mission</td>
</tr>
<tr>
<td>Session 3: Saturday 9.00am to 10.30am</td>
<td>Getting the Most Out of What You Eat! Nutrition for Oral Feeders</td>
<td>Century Ballroom A</td>
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<tr>
<td>Session 4: Saturday 10.45am to 12.15pm</td>
<td>Breathing Basics for Type II and Type III</td>
<td>Mission</td>
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<td>Session 5: Saturday 1.30pm to 3.00pm</td>
<td>Toy Adaptation</td>
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### Type III

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<tr>
<td>Session 1: Friday 10.30am to 12.30pm</td>
<td>Sharing your Type III Experience</td>
<td>Liberty</td>
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<td>Session 1: Friday 10.30am to 12.30pm</td>
<td>Grandparents Talk it Out Type II and Type III</td>
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<td>Session 2: Friday 2.00pm to 3.30pm</td>
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<td>Session 5: Saturday 1.30pm to 3.00pm</td>
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### Grieving

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<tr>
<td>Session 1: Friday 10.30am to 12.30pm</td>
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<td>Penn Valley</td>
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<td>Session 2: Friday 2.00pm to 3.30pm</td>
<td>Healing the Grieving Heart - Part 1</td>
<td>Penn Valley</td>
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<tr>
<td>Session 3: Saturday 9.00am to 10.30am</td>
<td>Keepsake Creation: Grieving Through Art</td>
<td>Penn Valley</td>
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<tr>
<td>Session 4: Saturday 10.45am to 12.15pm</td>
<td>Healing the Grieving Heart II - Now What?</td>
<td>Westport</td>
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<td>Session 5: Saturday 1.30pm to 3.00pm</td>
<td>Grieving Siblings</td>
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### Teens

<table>
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<tr>
<td>Friday 9.00am to 10.15am</td>
<td>Kids Talk it Out (10 &amp; Older)</td>
<td>Shawnee</td>
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<tr>
<td>Friday 9.00am to 10.15am</td>
<td>Siblings Talk it Out (Ages 12 - 17)</td>
<td>Westport</td>
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<tr>
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<td>N/A</td>
<td>Roanoke</td>
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<tr>
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<td>What it means to be a Teen in Wheels</td>
<td>Shawnee</td>
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<tr>
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<td>Medical Management of Adults with SMA</td>
<td>Liberty</td>
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<tr>
<td>Session 3: Saturday 9.00am to 10.30am</td>
<td>Driving and Community Mobility</td>
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### Teens (cont.)

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<tr>
<td>Session 4: Saturday 10.45am to 12.15pm</td>
<td>Dating, Relationships and Sexuality</td>
<td>Century Ballroom B</td>
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<tr>
<td>Session 5: Saturday 1.30pm to 3.00pm</td>
<td>Personal Care Assistants - How to find, hire and manage your PCA’s</td>
<td>Liberty</td>
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<tr>
<td>Session 5: Saturday 1.30pm to 3.00pm</td>
<td>Life After School: What’s Next?</td>
<td>Mission</td>
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<tr>
<td>Special Event: Thursday 7.30pm to 10.00pm</td>
<td>Transition to Adulthood</td>
<td>Penn Valley</td>
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<td>Special Event: Thursday 7.30pm to 10.00pm</td>
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### Adults

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<td>Session 2: Friday 2.00pm to 3.30pm</td>
<td>Adults with SMA Roundtable</td>
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<td>Special Event: Thursday 7.30pm to 10.00pm</td>
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### Kids

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<td>Session 5: Saturday 1.30pm to 3.00pm</td>
<td>Kids Talk it Out (Ages 6 - 9)</td>
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<td>Special Event: Thursday 7.30pm to 10.00pm</td>
<td>Siblings Talk it Out (Ages 5 -11)</td>
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### For All

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<th>Special Event: Thursday 6.30pm to 8.30pm</th>
<th>Subject</th>
<th>Room</th>
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<tr>
<td>General Session: Friday 9.00am to 10.15am</td>
<td>Opening Meet and Greet</td>
<td>Century Ballroom</td>
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<td>Special Event: Friday 6.00pm to 9.30pm</td>
<td>Welcome and Opening Remarks</td>
<td>Century Ballroom C</td>
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<tr>
<td>General Session: Saturday 3.15pm to 5.15pm</td>
<td>Family Friendly Posters and Dance Party</td>
<td>Century Ballroom</td>
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<td>Special Event: Saturday 6.45pm to 9.30pm</td>
<td>Researcher Q &amp; A Panels</td>
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<td>General Session: Saturday 10.30am to 12.30am</td>
<td>PJ Party and Movie Night</td>
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<tr>
<td>General Session: Sunday 9.30am to 11.30am</td>
<td>Closing Session and Wonderful Life Panel</td>
<td>Century Ballroom C</td>
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<td>Dad’s Time</td>
<td>Liberty</td>
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<td>How to be a Healthy Parent</td>
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<td>Life Care Planning</td>
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<td>Orthopedic Management</td>
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<td>Recognizing and Managing Pain in SMA</td>
<td>Century Ballroom A</td>
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<td>Yoga Therapy</td>
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<td>Ethics in Research</td>
<td>Century Ballroom A</td>
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</table>
Cure SMA thanks our valued sponsors for their generous support of the 2015 Annual SMA Conference. These organizations provide the SMA community with hope for breakthroughs that will strengthen bodies, extend life, and eventually lead to a cure.

PLATINUM SPONSORS

Biogen

CONVENTION CONTRACTORS INC.
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fighting spinal muscular atrophy in memory of our shooting star

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The Jacob Isaac Rappoport Foundation and Shaina & Adi Rappoport

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

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

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

The Jacob Isaac Rappoport Foundation was created in memory of their Shooting Star, Jacob, who had SMA type I, Jacob was born December 27th, 2001 and was an alert and happy baby. When he was nearly four months old, Jacob was diagnosed with Spinal Muscular Atrophy. Soon after Jacob’s diagnosis, Adi and Shaina quickly connected with Cure SMA and many other SMA families. Cure SMA would like to extend a heartfelt thank you Shaina and Adi Rappoport and the Jacob Isaac Rappoport Foundation for all of the money they have raised over the years; the dinners they have sent; the cleaning services and packages of toys they have provided for hundreds of SMA families; the luncheons & receptions they have hosted; the funding, support and guidance they have provided and the conference; and the countless hours they have spent lending emotional support to parents of newly diagnosed children. Thank you to the entire Rappoport family, friends and relatives for your continued support throughout the years!
**Event Milestones**

**15th Annual Concert for a Cure**

The 15th Annual Concert for a Cure was held on Saturday, May 9, 2015, at the Diablo Country Club in Danville, CA and has raised over $1.54 million. This memorable evening was made especially rewarding for donors and families when Dr. John Day, Stanford Professor for Neurology, Pediatrics, and Pathology, highlights the exciting advancements in SMA and the ISIS clinical trials, and Robert Blum, CEO of Cytokinetics.

Mary and Joe McHale founded the 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, SMA type III, was diagnosed with SMA. Both Danny and Ariana will turn 15 years old this year.

Thank you!

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

**15th Annual New England Chapter Cure SMA Walk-n-Roll**

The New England Chapter hosted their 15th Annual Cure SMA Walk-n-Roll on Saturday, May 16, 2015 at DCR Wompatuck State Park in Hingham, MA. Silvia Murphy, Stacey Farrell, and Don Norton teamed up to organize this walk that brought together about 700 participants – including 30 families affected by SMA!

Many families and friends came together from throughout New England to raise money for a cure for SMA. We owe much thanks to the volunteers and business partners who helped with this event. A team from Biogen, who are researching treatments for SMA, participated and made a generous sponsorship donation to the event! Many of the volunteers have been coming for five to ten years, and they keep the whole event running smoothly. We are very grateful to the local businesses and friends who donated most of the supplies, refreshments, and raffle items. We hope to see everyone again next year!
Event Milestones

10th Annual Steven’s Walk to Drum Out SMA

This year marked the 10th Annual Steven’s Walk to Drum Out SMA! The walk was held on Saturday, May 9, 2015, at the Newton Lake Park in Haddon Township, NJ, in honor of Steven Potter, SMA type III. It was a beautiful day filled with family, friends, neighbors, the local fire department, the local Walgreens, Hair Cuttery, a live band, and some new faces! There were wonderful Chinese auction baskets, donated by family members, friends, many companies, and BAYADA. This year’s walk also had a 50/50 raffle, and the winner was extremely excited!

Many wonderful people came together to help solicit several different companies for these auction items. All of the participants and volunteers have made it possible to continue to support SMA and run Steven’s Walk year after year, especially Chrissy Dougherty and Trish Rudisill.

A very special thank you to Mike, Terri, Steven, and Matthew Potter for all of your dedication and time to host this event for ten years. You all have truly made a difference for Cure SMA!

10th Annual Greater New York Walk-n-Roll to Cure SMA

This year marked the 10th Annual Greater New York Walk-n-Roll. The day was filled with great activities for participants of all ages, including a 50/50 raffle and face painting for the children to enjoy! The rain held out just long enough for a beautiful walk, which was held in memory of Jack Bonelli, SMA type I.

We would like to give a special thanks to all of our amazing teams, participants, and donors for making our 10th Annual Walk a HUGE success. Due to everyone’s efforts, we were able to raise almost $33,000!

The support of all of this year’s participants and donors will help fund critical SMA research programs that will lead to a treatment and cure for SMA. These funds will also provide important family support and patient care programs.

An extra special thanks to all of our teams for participating in this year’s event: Cuevas-Goldstein, Fight for Own, For The Love of Max, Friends of Julia, JUST Walking for a Cure, Nicholas & Tyler’s Fight Against SMA, Olivia Barbarino, Sweet Baby Jack, Team BAYADA, Team Dylan, Team Farmingdale, Team Juliana, Team Ross, Team Rummel/Morovich, and Team Tansey.

A very special thanks to Debbie Cuevas and all of the volunteers for your time and energy put into organizing this amazing event. We look forward to next year!
Event Milestones

10th Annual Grant Sheppard Memorial Scramble

On Friday, August 22, 2014, the 10th Annual Grant Sheppard Memorial Scramble for SMA was held at Hickory Hills Country Club in Chilton, WI. This golf event is in memory of Grant Thomas Sheppard, SMA type I.

The golfers and volunteers had a fantastic day filled with lots of fun and laughter. Everyone had a great time golfing, at the silent auction, and bucket raffles, helping to raise money for Cure SMA. In total, the event has raised $100,000 over the past 10 years.

A major thank you to Scott, Lisa, Peter, and Lily Sheppard for all of their hard work in planning this event year after year! With your dedication to organizing this event each year, we are that much closer to finding a treatment and cure for spinal muscular atrophy.

Through cutting-edge science and medicine, Biogen discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders.

Biogen proudly supports CureSMA and the 2015 Annual SMA Conference, bringing together researchers and families dedicated to finding treatments for SMA.

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Proud Sponsors of the 2015 Annual SMA Conference

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Our conference has received grant support from the following governmental groups. We thank them for their fantastic support that has helped bring together the SMA research community along with our families.

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.

Registration and attendance at, or participation in, the Annual SMA Conference and related functions constitutes attendee’s authorization to Cure SMA’s use and distribution of attendee’s photo/image/likeness and videotape/voice recordings in any medium, including Cure SMA’s websites and Cure SMA’s pages on social networking platforms (e.g., Facebook), for use in editorial, educational, promotional, and advertising purposes, for the solicitation of contributions, and for any other purpose in furtherance of the Association’s purposes and objectives, without payment to the attendee. Such authorization is binding upon the attendee as well as the attendee’s heirs, executors, administrator, and assigns.
Special Thanks

Jennifer Miller Smith, Aaron Smith and Family

Your generosity has been felt throughout the duration of this year’s conference planning. You have all put in countless hours of your time by getting donations for the conference, helping us with the conference layouts, raising awareness throughout your community, supporting other families, and so much more! Our appreciation towards your entire family is felt by everyone the Cure SMA National Office. Thank you so many times over!

Expocci – The professionalism that Expocci brings to our conference through their impressive signs and banners, which decorate the entire hotel, is absolutely outstanding! For the sixth year in a row Expocci has donated their expertise and signage, which never cease to amaze us. This organization does fabulous work and we cannot thank them enough for volunteering their services to make this conference great! Thank you to Expocci, their employees, Richard Curran and Jennifer & Aaron Smith.
EXPO

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EXPO
Supports CURE SMA
in hopes to finding a treatment and cure for
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The Erin Trainor Memorial Fund

The mission of the Erin Trainor Memorial Fund is to provide Conference Scholarships allowing newly diagnosed SMA families/individuals the opportunity to attend The Annual SMA Conference.

The Erin Trainor Memorial Fund (ETMF) will generate substantial funds which will be used for conference scholarships. In addition, ETMF will provide increased awareness of Conference benefits to individuals affected by SMA, corporate partners and the medical community.

The Erin Trainor Memorial Fund is a restricted endowment governed by the Board of Directors of Cure SMA to support the future Newly Diagnosed SMA families. Conference scholarships will be awarded when the fund reaches a minimum of $1 million.

-Gene Trainor

Over $713,000 has already been raised, illustrating forward progress toward achieving the initial $1 million goal.

Please Note

During this conference we would like to strongly encourage our conference attendees to take proper sanitary actions. Please remember to wash your hands frequently so we can keep the spread of germs to a minimum. If you or a family member are feeling under the weather, we advise that you not attend workshops or the children’s program. There are hand sanitizers throughout the conference so please take advantage of those.
SCOTT, KAREN, GENNA & MATTHEW GELLER

Proudly Support the

2015 Annual SMA Conference

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**Meet & Greet**

Thursday 6:30pm

Kick off to the Conference!

- Researcher Relay Race
- Family Fun Fest/Carnival

Century Ballroom

**Family Friendly Research Poster Session**

Friday 6:00pm

Rotate around to over 30 posters presented by researchers

Century Ballroom

**Family and Researcher Dance Party**

Friday 7:30pm

Dance the night away

Century Ballroom

**PJ Party and Movie Night**

Saturday 6:45pm

Family Movie for kids and adults

Century Ballroom

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**Food items included for all registered attendees:**

- **3 Breakfasts:** Friday, Saturday and Sunday
- **2 Boxed Lunches:** Friday & Saturday
- **Light Appetizer/Snacks:** Thursday at the Meet and Greet
- **Light Appetizers/Snacks:** Friday evening at the Family Poster Session
- **Popcorn:** Saturday evening at the PJ Party and Movie Night
Wednesday, June 17th

3:00pm – 9:00pm  Registration Open for all Conference Attendees  Roanoke Foyer

Registration Area Sponsored by astellas

Thursday, June 18th

7:30am – 5:30pm  Registration Open for all Conference Attendees  Roanoke Foyer

Registration Area Sponsored by astellas

11:30am – 4:15pm  Newly Diagnosed Children’s Program  Pershing Place

11:30am – 4:15pm  Newly Diagnosed Baby Room  Pershing Place East

12:00pm – 4:00pm  Newly Diagnosed Program (For Newly Diagnosed Families Only)  Century Ballroom A

Introduction to Cure SMA and the Community

Overview of Conference and Goals
Kenneth Hobby, President
Colleen McCarthy O’Toole, Senior Director
Spencer Perlman, Cure SMA Legislative

Understanding the Genetics and Disease
Tom Crawford, MD

Understanding Clinical Trials
Rob Graham, MD

Life After Diagnosis – Parents Share Their Journey
Barbara Trainor, Grieving  Angel Wolff, SMA Type II
Justin Kuester, SMA Type I  Danyelle Sun, SMA Type II & III
Natalie Gibbs, SMA Type II & III
# Thursday, June 18th

<table>
<thead>
<tr>
<th>Time</th>
<th>Event Description</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>1:15pm – 2:30pm</td>
<td>Newly Diagnosed Program Meet and Mingle Reception</td>
<td>Shawnee-Mission</td>
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<tr>
<td>2:30pm – 4:00pm</td>
<td>Newly Diagnosed Breakouts</td>
<td>Century A, Century B, Westport</td>
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<td></td>
<td><strong>Session A – Type I – Optimal Care for SMA</strong></td>
<td>Century Ballroom B</td>
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<tr>
<td></td>
<td>Mary Schroth, MD, Chair of Cure SMA MAC</td>
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<td></td>
<td>Garey Noritz, MD</td>
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<td></td>
<td>Rebecca Hurst Davis, MS, RD, CSP, CD</td>
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<td>Vanessa Battista,</td>
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<td>Karen Patterson</td>
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<td><strong>Session B – Type II/III – Optimal Care for SMA</strong></td>
<td>Century Ballroom A</td>
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<tr>
<td></td>
<td>Kristin Krosschell, MA, PT, PCS</td>
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<td>Richard Kravit, MD</td>
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<td>Richard Shell, MD</td>
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<td>Brian Snyder, MD,PhD</td>
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<td>Erin Seffrood, MS, RD, CSP, CD</td>
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<td>Susan Apkon, MD</td>
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<td>Natalie Gibbs, Moderator</td>
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<td><strong>Session C – Grief and Loss (Support Session)</strong></td>
<td>Westport</td>
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<td>Richard Rubenstein</td>
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<td>Fred Troutman</td>
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<td>David Sereni</td>
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<td>6:30pm – 8:30pm</td>
<td>Meet &amp; Greet, includes Family Fun Fest / Carnival &amp; Researcher Relay Race</td>
<td>Century Ballroom</td>
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<tr>
<td>7:30pm – 10:00pm</td>
<td>Teen Social</td>
<td>Mission</td>
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<td>7:30pm – 10:00pm</td>
<td>Adults with SMA Social</td>
<td>Liberty</td>
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</tbody>
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**Conference wristbands must be worn during the entire conference.**

You will not be admitted into any conference event or workshop without this conference wristband.

See page 58 for Speaker Bios
Meet and Greet
Thursday June 18, 6:30 pm to 8:30pm • Century Ballroom

Meet us in the
Century Ballroom

6:30pm – 8:30pm
Meet & Greet and Family Fun Fest
Conference welcome and introductions and carnival games and prizes for all children

6:45pm
Researcher Relay Race

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we proudly support cure sma

congratulations on another successful conference
Children’s Program Entertainment Schedule

Friday June 19th • Pershing Place

Morning: 9:00am- 12:30pm
11:30am-12:15pm  Clowning Juggling Show- “If Only My Bag were Empty”...Bliepey the Clown is headed somewhere. Where? We’re not exactly sure, but his bag is slowing him down. Bliepey and his audience will discover that all sorts of cool things are hidden in that bag. Bliepey and a bevy of volunteers will juggle, dance, spin plates, and frolic their way toward, wherever it is Bliepey was headed.

Afternoon: 2:00pm-3:30pm
2:15pm-3:00pm  Juggling Show- Erik J Pratt of Drops Unlimited Entertainment presents his most dangerous show ever! He is a clown at heart, so danger might not mean what you think it does. Or maybe it will. This juggling show will include ropes, giant beanbag chairs, audience volunteers, and at least one wobbly table.
3:00pm-3:30pm  Balloon Twister

Saturday June 20th • Pershing Place

Morning: 9:00am- 12:15pm
10:00am-11:00am  Tea Party- Any children interested in joining the Tea Party are also welcomed to bring their dolls.
11:15am-12:15pm  Clement McCrae Puppet Shows- Enjoy this mostly marionette puppet show is a celebration of the lush cornucopia of cultures in the music, costumes, and dance of the marionettes and other puppets. This variety puppet show is quick paced and filled with all sorts of fun that will appeal to all audiences – for the young and old.

Afternoon: 1:30pm-5:15pm
2:30pm-3:30pm  Ventriloquist- Kevin brings a unique blend of ventriloquism, comedy, and fun for ALL ages.
4:00pm-5:00pm  The Rockin Rob Show- a completely interactive and educational concert for young children and families, delivered by way of music, movement, magic & variety.

Parents please have your phones on and handy in case one of the Children’s Program volunteers needs to call you.

Sponsored by

No Food Allowed in Children’s Program
Please do not bring any food into the children’s program since some of the children attending have severe food allergies.
Thank You to All of the Conference Children’s Program Volunteers

Each and every one of our volunteers has a major impact on the atmosphere and success of our conference, and every year their impact radiates throughout the weekend! Their long days of hard work provide families with the reassurance that their children are well cared for and are having fun, while they attend workshops, learning the latest information on SMA. The enthusiasm they provide for the kids in the children’s program, during the carnival games, the Dance Party and at our other events, is contagious with each event. Our volunteers take great pride in helping to ensure that at each conference they attend, families leave with the feeling that this was the best conference yet. Without you all, our conference would be no where near what it encompasses today. Thank you for everything!

We would like to especially thank the following volunteers who return year after year, leaving their jobs and families at home to help SMA families:

**VOLUNTEER FOR 26 YEARS**

George Ghorbanian,
Chicago Police Officer

George has volunteered at the conference since the Children’s Program started back in 1990. The 2015 Annual SMA Conference will be George’s 26th consecutive conference!!

**VOLUNTEER FOR 24 YEARS**

Patti Slojkowski
Dental Hygienist
Patti has two teenage children.

**VOLUNTEER FOR 21 YEARS**

Steve Smith
Physical Therapist
Steve has 3 daughters, ages 1 - 10.
Volunteer for 17 Years

Kelly Milito
Hair Stylist
Kelly has 4 children, ages 12 - 19.

Kelly Basso
Surgical Consultant
Kelly has 3 teenage and adult children.

Volunteer for 14 Years

Peg Bailey
Nurse

Mary Blume
Hospital Administrative Assistant
Mary has 2 adult children.

Traci O’Brien
Recent Grad School Graduate in Marketing

Theresa O’Brien
General Manager at Gerber Collision

Volunteer for 7 Years

Jessica Schmitke
Onsite Health Coach/Coordinator

Hugo Trevino
Recent College Graduate majoring in Spanish & Translation Studies

Caitlin Trainor
Recent college Graduate with a degree in Psychology

Grace Trainor
College Student studying Exercise and Movement Science

Eileen Venedam
College Student studying Business

Annie Venedam
**VOLUNTEER FOR 6 YEARS**

- **Doug Placko**
  - Senior Account Executive
  - Volunteer for 6 years

- **Patrick Hogan**
  - High School History & Psychology Teacher

- **Melissa Peyton**
  - 1st Grade Teacher

**VOLUNTEER FOR 5 YEARS**

- **Joy Martin**
  - Physical Therapist
  - Joy has 3 children, ages 6, 8 and 10.

- **Donna Budil**
  - Preschool Director
  - Donna has 2 kids, ages 12 and 15.

- **Brian Blume**
  - Accountant
  - Brian has 2 adult children.

- **Kelli Blume**
  - Graduate Student in Social Work

- **Megan Milito**
  - College student in Special Education

- **Alec Basso**
  - College student in Nursing

- **Mike Graney**
  - Vice President of United Services

- **Collene Cahoy**
  - College student in Elementary and Special Education

- **Caroline LaPelusa**
  - College student in Nursing

- **Patrick Hogan**
  - High School History & Psychology Teacher

- **Jackie Graney**
  - Director of Studio 22 Dance Studio
**VOLUNTEER FOR 4 YEARS**

- **Lexi Basso**
  - Graduate Student in Business Law

- **Tori Zeman**
  - College student in Occupational Therapy

- **Emily Yagihashi**
  - College student in Cellular & Molecular Biology

- **Kailee Breslin**
  - College student in Speech & Language Pathology

- **Danielle Austriaco**
  - College student in Business Law

- **Hayley Miller**
  - College student in International Studies

- **Maeve Gallagher**
  - College student in Communications and Public Relations

**VOLUNTEER FOR 3 YEARS**

- **Georgia Slojkowski**

- **Danielle Kirincich**
  - College student in Psychology

- **Laura Kroll**
  - College student

**VOLUNTEER FOR 2 YEARS**

- **Megan Besler**

- **Henry Guan**
  - College student in Kinesiology
Friday, June 19th

7:30am – 8:45am  |  Family Continental Breakfast  |  Century Ballroom A

7:30am – 4:00pm  |  Registration/ Merchandise Sales  |  Roanoke Foyer

Registration Area Sponsored by  

7:30am – 5:00pm  |  Exhibitor Tables Open  |  Century Ballroom Foyer & Roanoke Foyer

9:00am – 10:15am  |  General Session Opening Remarks  |  Century Ballroom C

9:00am – 10:15am  |  Kids Talk it Out (Kids Only - Ages 10 & Older)  |  Shawnee

Al Freedman, Ph.D., SMA Dad and Child
Angela Wrigglesworth, Elementary Education Teacher, SMA Adult
Rocco Arizzi, PhD, Electrical Engineer, SMA Adult

This facilitated session will provide SMA-affected children (ages 10 & older) an opportunity to talk with each other about their lives. (Note: To encourage our children to truly “Talk It Out”, this session is open to children only and requires parental consent.)

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Children’s Program - Friday

9:00AM – 12:30PM & 2:00PM – 3:30PM

Children’s Room • Pershing Place  
(ages 4 & older)

Baby Room • Pershing Place East  
(ages 3 and under – quiet room, no wheelchairs)

Sponsored by
Friday, June 19th

9:00am – 10:15am  Sibling Talk it Out (Ages 12 to 17)  Westport

Traci O’Brien, Sister of a SMA Young Adult

This workshop is for the siblings of brothers and sisters with SMA. With the unique relationship siblings have, we have much to offer one another. This workshop will create a fun and comfortable setting in which siblings can express themselves and their feelings, both positive and negative, of having a sibling with SMA through activities, games, and discussions. This workshop will be facilitated by Traci O’Brien, sibling of a SMA family member, and assisted by other adult SMA siblings. It is geared for siblings between the ages of 12-17 years old. They will learn ways to cope and manage common situations that are experienced by brothers and sisters. Dress comfortably and be prepared to make friends and have fun!

10:30am – 12:30pm  Workshop Session #1

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Location</th>
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<tbody>
<tr>
<td>10:30am – 12:30pm</td>
<td>Sharing your Type I Experience</td>
<td>Century Ballroom B</td>
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<tr>
<td>10:30am – 12:30pm</td>
<td>Sharing your Type II Experience</td>
<td>Century Ballroom A</td>
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<tr>
<td>10:30am – 12:30pm</td>
<td>Sharing your Type III Experience</td>
<td>Liberty</td>
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<tr>
<td>10:30am – 12:30pm</td>
<td>Adults with SMA – Roundtable</td>
<td>Penn Valley</td>
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<tr>
<td>10:30am – 12:30pm</td>
<td>Grandparents Talk it Out Type I</td>
<td>Roanoke</td>
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<tr>
<td>10:30am – 12:30pm</td>
<td>Grandparents Talk it out Type II &amp; Type III</td>
<td>Mission</td>
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12:30pm – 2:00pm  Lunch Break

2:00pm – 3:30pm  Workshop Session # 2

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Location</th>
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<tbody>
<tr>
<td>2:00pm – 3:30pm</td>
<td>What it Means to be a Teen on Wheels</td>
<td>Roanoke</td>
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<tr>
<td>2:00pm – 3:30pm</td>
<td>Genetics and Reproductive Options for SMA Families</td>
<td>Mission</td>
</tr>
</tbody>
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Angela Wrigglesworth, Elementary Education Teacher, SMA Adult

It is hard to be a teenager in today’s society. The combination of rapidly changing hormones, peer pressure, and educational challenges are often too much for most young people to handle. If asked to imagine all of these factors combined with a diagnosis of SMA, most would probably picture a disaster waiting to happen. But being a teen with SMA is not impossible and in fact can produce some of the most influential and positive times in a young person’s life. This facilitated session, led by former teen Angela Wrigglesworth, will help to shed light on how to overcome the difficulties of being a teenager and bring forth a smooth segue into adulthood.

Harvey J. Stern, MD, PhD, FACMG, FAAP, Genetics and IVF Institute
Louise R. Simard PhD, University of Manitoba, Canada

This workshop will provide an introduction to genetics and, in particular, SMA genetics. Our objective is to provide a better understanding of why SMA clusters in families, why SMA presents with such wide clinical diversity (severe to mild forms) and to discuss the different DNA tests available for SMA. These tests allow
Friday, June 19th

us to diagnose 5q-SMA in infants and adults, identify SMA carriers, and determine if your unborn child has SMA. Reproductive options for couples who are at-risk for having children with SMA will be discussed including Preimplantation Genetic Diagnosis (PGD) with in vitro fertilization (IVF).

**Healing the Grieving Heart – Part I**

**Fred Troutman, Ph. D.**

**David Sereni**

The healing process, Parts 1 & 2: This workshop is for those dealing with the loss of a child or family member and provides an opportunity for those attending to address their experience with bereavement and feelings of loss.

**Fundraising 101 – Planning and Executing**

**Ellen Perry, Development and Family Support Manager**

**Amber Snyder, Development and Family Support Manager**

**Sarah Rodriguez, Development and Family Support Officer**

**Heidi Roy, Development and Family Support Officer**

**Shannon O’Brien, Development and Family Support Manager**

**Jessica Clark, Development and Family Support Manager**

**Lyndsey Rice, Development and Family Support Associate**

**Krystal Bocanegra, Development and Family Support Associate**

**Allie Ziolko, Development and Family Support Associate**

**Brittany Downey, Development and Family Support Assistant**

This year’s fundraising workshop is designed for those who are beginning to fundraise and finding the event that is right for you. We will guide you through the most important steps in planning any type of event from a walk-n-roll, golf outing, gala, bowl-a-thon, marathon, and so many others in small group discussion with a staff member and Cure SMA event volunteer. We will also walk you through best practices to use when building sponsorships for your fundraising event. Be ready to have a great time and learn a lot about raising funds to support the work at Cure SMA.

**Medical Management of Adults with SMA**

**John Kissel, MD**

**Garey Noritz, MD**

This workshop will focus on two key areas: the specific and sometimes unique medical issues facing adults with SMA and the challenges and difficulties that often are faced with transitioning from predominantly pediatric to adult care. Specific attention will be directed to the importance and difficulties adults face in integrating, coordinating, and directing their own care, especially when that care is divided between clinicians. Much of the discussion will be directed by the specific needs and questions of the attendees.

**WELCOME!**

We are honored to have SMA Families and Researchers attend this conference from all over the world! There are individuals attending from over 22 countries including: Argentina, Brazil, Canada, Chile, China, Czech Republic, England, Germany, Guam, India, Israel, Italy, Japan, Netherlands, Russia, Scotland, South Africa, Spain, Switzerland, Taiwan, Turkey, and the United States.
Friday, June 19th

Standing, Walking and Mobility: Decision Making and Options  
Century Ballroom A
Karen Patterson, PT, MS, PCS  
Kristin J Krosschell, PT, DPT, MA, PCS  
Terri Carry, PT  
Susan Apkon, MD

The ultimate goal of this session is to help you advocate for your child’s optimal level of standing, walking and mobility and we will explore the decision making processes related to such. We will discuss standers and bracing to support function and/or mobility needs for the child with SMA, as well as provide an understanding of wheeled mobility options and important things to consider in your decision making. The discussion will include coverage of multiple issues including: how to decide on a type of brace, stander, or mobility device, who is an appropriate candidate, when and how to start the process, and how and when to initiate these discussions with your local therapist and clinicians. This session will be relevant for those with SMA 1,2 and 3 and their families.

Dad’s Time: A Workshop for Fathers Only  
Century Ballroom B
Al Freedman, Father of SMA Type I son  
Justin Kuester, Father of a SMA Type I daughter  
Kevin O’Brien, Father of a SMA Type III daughter

Although SMA is experienced directly by the person with the disease, all members of the family are impacted in profound ways. During this session, fathers of SMA-affected individuals will share their unique perspectives, ideas, and experiences. This session is intended for fathers of SMA-affected children or adults. Time will be allotted for all attendees to ask questions and contribute to the group discussion.

<table>
<thead>
<tr>
<th>Time</th>
<th>Event Description</th>
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<tr>
<td>6:00pm – 7:30pm</td>
<td>Family Friendly Research Poster Session</td>
<td>Century Ballroom</td>
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<td>7:30pm – 9:30pm</td>
<td>Family and Researcher Dance Party</td>
<td>Century Ballroom</td>
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The Family Friendly Research Poster Session

Friday June 19, 6:00pm to 7:30pm • Century Ballroom

The Family Friendly Research Poster Session will allow for one-on-one interactions between families and researchers. Cure SMA has invited researchers, who are also attending SMA Researcher Meeting, to present family friendly research posters during this event. During the event, families are encouraged to rotate around to the different posters to ask questions and learn directly from the researchers involved in the specific projects being presented.

POSTERS INCLUDE:

- **Wei-Shi Yeh PhD of Biogen** presenting on The Experiences of Individuals and Families Affected by Spinal Muscular Atrophy (SMA) and Their Views on Living with SMA.
- **Allan Kaspar PhD of AveXis** presenting on Treating Type I SMA with Gene Therapy: chariSMA.
- **Anne Marquet PhD, Omar Khaja MD, John Baird PhD, Nikolai Naryshkin PhD, Sergey Paushkin PhD, Karen Chen PhD, Irene Gerlach PhD** of F. Hoffman La Roche, PTC Therapeutics, and SMA Foundation presenting on Oral Compound Increases Full Length SMN2 mRNA in Humans.
- **Emilie Voltz PhD from Novartis Institutes for Biomedical Research** presenting on LM1070 for Treatment of Type I SMA.
- **Shoutian Zhou PhD from California Institute of Biomedical Research** presenting on Finding Drugs that Keep Motor Neurons Alive.
- **Caroline Woo PhD from RaNA Therapeutics** presenting on A Novel Epigenetic Approach to Treating SMA.
- **Jinsy Andrew MD from Cytokinetiks** presenting on Fast Skeletal Troponin Activators Applied to SMA.
- **Christian Lorson PhD from the University of Missouri** presenting on Anti-sense Oligonucleotides in SMA: Design and Function.
- **Arthur Burghes PhD from The Ohio State University** presenting on Biomarkers that Predict Successful Therapy in SMA.
- **Natalia Rodriguez Mueala PhD from Rubin Laboratory at Harvard University** presenting on Studies on SMA Patient Motor Neurons Produced from Induced Pluripotent Stem Cells.
- **Kristina Bowyer from Isis Pharmaceuticals** presenting on Developing an Antisense Drug for the Potential Treatment of SMA.
- **Katherine Klinger PhD Alison McVie-Wylie PhD from Genzyme Corporation** presenting on Progress Towards AAV-SMN Gene Therapy For Spinal Muscular Atrophy.
- **Yimin Hua PhD from the Krainer Laboratory at Cold Spring Harbor Laboratory** presenting on Mechanism of Action of ISIS-SMNIRx.
- **Chien-Ping Ko PhD and Chiara Mazzasette PhD from University of Southern California** presenting on Motor Circuit Defects and Repair in SMA Model Mice.
- **Sara Custer PhD and Anne Reitz PhD from the Androphy Laboratory at Indiana University** presenting on SMA Therapeutics and Biological Insights into Motor Neuron Biology.
- **Thomas Blaettler MD of F. of Hoffman La Roche, La Roche: Our Commitment to Patients and Their Families.
- **Chad Heatwole MD from the University of Rochester** presenting on Most Important Symptoms in Adult SMA: A View From the Patient’s Perspective.
- **Francesco Lotti PhD from Columbia University** presenting on Modifications of the SMN Complex: Do They Have a Role in SMA Pathogenesis?
- **Min Jeong Kye PhD from the University of Cologne** presenting on mTOR and Protein Synthesis in SMA.
- **Linda Lowes PhD from Nationwide Children’s Hospital** presenting on Measuring Motor Ability in Children Can be All Fun and Games.
- **Monique Émond Ph, MSc from Centre de Rédaptation Marie Enfant of CHU Ste-Justine** presenting on Breath Stacking and Postural Therapy: An Optimal Combination to Improve Respiratory Function in Children with SMA Type II and III.
- **Walter Troung MD from Gillett Children’s Hospital** presenting on The Effects of Assisted Standing on Bone in Children with SMA.
- **Diane V. Murrell, LCSW from Texas Children’s Hospital** presenting on The Role of Clinical Social Work in Support of Families with Children with SMA Type I.
- **Deborah S. Boroughs, RN, MSN from BAYADA Pediatrics** presenting on Bridging a Care Delivery Gap for Family Caregivers of Children with SMA Type I and Type II.
- **Rebecca Hurst Davis MS, RD, CSP, CD from the University of Utah** presenting on Glucose Tolerance and Fasting in SMA Type II Pilot Study Results and Next Steps.
- **Lindsay Murray PhD from the University of Edinburgh** presenting on What’s Makes Motor Neurons Vulnerable in SMA.
- **Hong Liu PhD from the Kothary Laboratory at the University of Ottawa** presenting on The Contribution of Multiple Cell Types to SMA.
- **Barrington Burnett PhD from Uniformed Services University of the Health Sciences** presenting on Muscle Contribution to SMA.
- **Ke-Jun Han PhD and Chang-Wei Liu PhD from the University of Colorado** presenting on SMNUbiquitination Regulates its Localization.
- **Christine DiDonato from Lurie Children’s Hospital of Chicago** presenting on Pre-clinical Studies in Mild SMA Mouse Models.
- **Sunner Laboratory from Johns Hopkins University** presenting on Understanding the Pathology of Human SMA.
- **Matthew Halanski, MD, from the University of Wisconsin** presenting on Respiratory and Radiographic Effects of Growing Rods in Children with Spinal Muscular Atrophy.
Working to develop a treatment for Spinal Muscular Atrophy
Saturday, June 20th

7:30am – 9:00am  
**Family Continental Breakfast**  
Century Ballroom C

7:30am – 5:15pm  
**Registration/ Merchandise Sales**  
Roanoke Foyer

Registration Area Sponsored by

Registration Area Sponsored by

7:30am – 5:15pm  
**Exhibitor Tables Open**  
Century Ballroom Foyer & Roanoke Foyer

9:00am – 10:15am  
**Kids Talk it Out (Kids Only - Ages 6 - 9)**  
Westport

*Al Freedman, Ph.D., SMA Dad and Child  
Angela Wrigglesworth, Elementary Education Teacher, SMA Adult*

This facilitated session will provide SMA-affected children (ages 6 to 9) an opportunity to talk with each other about their lives. (Note: To encourage our children to truly “Talk It Out”, this session is open to children only and requires parental consent.)

9:00am – 10:30am  
**Workshop Session #3**  
Roanoke

**Breathing Basics and Care Choices for Type I**

*Mary Schroth, MD  
Richard Shell, MD  
Dr. Richard Kravitz*

Respiratory complications are the greatest challenge to manage for all individuals with SMA type I. This workshop will utilize medical care providers for the basics of respiratory management and a patient and family panel to share care choices, successes, and lessons learned.

### Children’s Program - Saturday

9:00AM – 12:15PM  
& 1:30PM – 5:15PM

**Children’s Room • Pershing Place**  
(ages 4 & older)

**Baby Room • Pershing Place East**  
(ages 3 and under – quiet room, no wheelchairs)

Sponsored by
Saturday, June 20th

Getting the Most Out of What You Eat! Nutrition for Oral Feeders  
Century Ballroom A

Erin Seffrood, MS, RD, CSP,CD  
Stacey Tarrant, RD, LDN  
Rebecca Hurst Davis, MS, RD, CSP,CD

This workshop is designed to address the unique nutritional needs of people with SMA who eat by mouth. The first 30 minutes of this session will include an overview of SMA nutrition. You will learn about recommendations for calories, protein, fat, vitamins, minerals and fluid as well as common nutrition deficiencies in SMA. You will also learn the signs/symptoms of feeding issues/intolerances, when it might be necessary to supplement intake, and when it might be necessary to cut back. The rest of the workshop will break out into two round table discussion groups for 1) Adults with SMA and 2) Children with SMA.

Adults with SMA-This discussion will focus on nutrient intake, lab values, and nutrition challenges that are specific to adults with SMA during different life stages.

Children with SMA- This discussion will focus on intake, nutrient concerns, and questions related to children with SMA.

Orthopedic Management  
Century Ballroom B

Brian Snyder, MD  
Matt Halanski, MD

This workshop will focus on life with SMA as it pertains to maintain the best possible orthopedic management. It will also address the orthopedic complications of SMA.

Driving and Community Mobility  
Mission

Mary Schwartz OTR/L, CDRS

Community mobility is necessary to access school and work as well as leisure and social activities. When young, parents transport the wheelchair user. But, like any other teen, driving is a common goal wheelchair or not. This presentation will address: (1) safe securement when being transported in a wheelchair. (2) issues to be addressed prior to purchasing a vehicle for adaptation (3) current trends in adaptive driving equipment (4) the importance of an evaluation by a qualified driver rehabilitation specialist (5) funding possibilities.

How to Be a Healthy Parent  
Liberty

Vanessa Battista, RN, M.S., CPNP, CCRC  
Pam Mosher, MD, MDIV

There is no “perfect roadmap” for the path to living a healthy life as a parent of a child/adolescent with SMA. How does one find moments of strength, sustenance, humor, and nourishment, while not striving for perfection, and also discover ways to set healthy boundaries? Join us for this informal workshop where we will explore ways to cope with not only the joys, but also the responsibilities and fears, that can accompany your roles as parent, spouse, sibling, employee and friend while having a child/adolescent with SMA.
Saturday, June 20th

**Keepsake Creation: Grieving Through Art**

*Jennifer Lemisch, MA, ATR-BC, LPC*

Mourning the loss of a loved one with SMA is ongoing and can be a difficult process. Expression through grief activities often assists in restoration and healing for those experiencing the loss. This workshop will provide bereaved family members an opportunity to make a keepsake object that will support the memories of their child and also learn about other creative legacy ideas to do on their own.

**Life Care Planning**

*Richard Rubenstein, Esq.*

Legal and financial planning that parents must undertake to protect their family can be difficult and confusing. It is vital for all parents to have an estate plan in place, more so for parents of children with disabilities. Find out how to assure that money you set aside for your child with special needs does not jeopardize government benefits your child is entitled to receive. Richard will give a general overview of the basics of estate planning and the differences between the various types of Special Needs Trusts. Learn how to prepare a letter of intent, an important and often overlooked document. A document you must create to provide all of the specialized knowledge required, preparing the person you chose to care for your child in your absence; this document also helps that person interpret your hopes and desires for your child’s future. Finally, Richard will discuss various techniques to advocate on behalf of your child in order maximize resources from insurance companies and government agencies. Parents must go through these difficult steps to ensure a well planned future for their special child.

**Family Readiness for Emergencies**

*Al Freedman, Ph.D., SMA Dad*

*Rob Graham, MD*

*Justin Kuester, SMA Dad*

Targeting all types of SMA, this workshop means to prepare families, young adults, and other providers for emergency situations encountered by those with SMA. Through shared experiences from parents and hospital providers, we hope to anticipate challenges, establish contingency plans, and identify resources to optimize community and hospital based emergency care. Open discussions about system barriers, professional perceptions and biases, misunderstandings of SMA, and practical issues are important to avert problems and advocate effectively when emergencies occur.

**Breathing Basics for Type II and Type III**

*Mary Schroth, MD*

*Richard Shell, MD*

The respiratory complications in children with SMA type II and SMA type III and options for intervention will be discussed including difficulty coughing and possible problems during sleep and what you can do to help your child’s breathing. We plan to discuss our experience and hear from the audience as we discuss interventions and work towards preventative and proactive care.
Saturday, June 20th

Healing the Grieving Heart – Now What? Part II

Fred Troutman, Ph.D.
David Sereni

This workshop is a continuation of Part 1. For those dealing with the loss of a child or family member and provides an opportunity for those attending to address their experience with bereavement and feelings of loss.

Dating, Relationships, and Sexuality

Danielle Sheypuk, PhD, Psychologist and Model, SMA Adult

Dating and the establishment of romantic relationships rank very high in life’s priorities however, this is not frequently recognized as such in the disabled community. In fact, society, media included, seems to ignore the idea that people with disabilities have the same emotional needs and desires regarding relationships, sex, and dating as the rest of the population.

Dr. Danielle Sheypuk, an expert in the area of dating and sexuality among people with disabilities, will present a two-part workshop on the topic. The first half of the workshop will highlight the importance of dating and romance, discuss readiness to date, and also provide some dating how-tos among other topics. This part of the workshop will be open to teens and adults with SMA, significant others as well as parents and caregivers.

Part Two of Dr. Sheypuk’s workshop will address issues of sexual intimacy including exploring techniques/positions, sensate focus, surrogate partner and PCA-facilitated sex, as well as create a private space for an uncensored Q&A. This part of the workshop is only open to adults with SMA and their partners who are 21 years of age and older.

Yoga Therapy

Anne Buckley-Reen, OTR/RYT, FORKIDS OT

The workshop will introduce you to the benefits of yoga for a child, teen or adult with SMA. Anne will discuss the physical, emotional, respiratory, cognitive and immunological benefits of yoga and share her work with an SMA family. The workshop will include a basic chair practice and conclude with a modified floor routine (for those interested). The yoga practice includes postures, eye exercises, progressive relaxation, and breathing and singing exercises.

Recognizing and Managing Pain in SMA

Vanessa Battista, RN, M.S., CPNP, CCRC
Pamela Mosher, MD, MDIV
Gina Santucci, RN, MSN, APRN-BC

Symptoms of pain in SMA may be difficult to recognize and therefore go untreated affecting quality of life. Having an understanding of the complex nature of pain is helpful in determining what pain management modalities are most effective for your child. This workshop will focus on defining pain pathways, identifying signs of pain, and exploring currently available modalities to treat pain in a child living with SMA.
SMA Conference Day 2: Saturday, June 20th

**Personal Care Assistants—How to find, hire and manage your PCA’s**

*Liberty*

*Paula Barrett, SMA Parent*

*Carolyn Barrett, SMA Adult*

This workshop is designed to give you the tools you will need in order to hire your own personal care assistants. What to look for? Where to look? How to describe your needs? In answering these questions and more, we'll strive to help you learn how to recruit, interview and screen PCA applicants. This workshop is designed for anyone that is looking to bring PCA's into their life for the first time or young adults who may be looking to independently manage their team of PCA's, but all are welcome. In addition, we will foster a dialogue on how parents can help their children learn to slowly manage their PCA's on their own. We'll encourage questions and share personal anecdotes as a way to shed light on this process.

12:15pm – 1:30pm  **Lunch Break**

1:30pm – 3:00pm  **Sibling Talk it Out (Ages 5 to 11)**

*Westport*

*Traci O'Brien, Sister of SMA Adult*

This workshop is for the siblings of brothers and sisters with SMA. With the unique relationship siblings have, we have much to offer one another. This workshop will create a fun and comfortable setting in which siblings can express themselves and their feelings, both positive and negative, of having a sibling with SMA through activities, games, and discussions. This workshop will be facilitated by Traci O'Brien, a sibling of a SMA family member, and assisted by other adult SMA siblings. It is geared for siblings between the ages of 5 – 11 years old. They will learn ways to cope and manage common situations that are experienced by brothers and sisters. Dress comfortably and be prepared to make friends and have fun! Preregistration is required.

1:30pm – 3:00pm  **Workshop Session # 5**

**Tube Feeding and SMA: Recommendations and Practices**

*Roanoke*

*Erin Seffrood, MS, RD, CSP,CD*

*Stacey Tarrant, RD, LDN*

*Rebecca Hurst Davis, MS, RD, CSP,CD*

This workshop will discuss the nutritional needs for individuals with SMA who are fed by tube. We will discuss appropriate growth, recommendations for calories, fat, vitamins, minerals, and fluids, and signs/symptoms of feeding issues and intolerance. The second part of the workshop will be an opportunity to discuss actual tube feeding practices among different families and clinics.

**Life After School: What’s Next?**

*Mission*

*Stevie Hopkins, SMA Adult*

You or your child is about to graduate from high school or college...What's next? Living with SMA as an adult can add extra challenges but that doesn't mean it can't be fun and successful. Having SMA doesn't prevent you from having social and personal aspirations in terms of education, work, dating, sexuality,
travel and independent living. Learn from Stevie’s life journey and take part in an interactive discussion that will help you plan for you or your child’s future. Stevie doesn’t know it all, so others who want to share their experiences are welcome to pitch in! Please know that this discussion will not be sugar coated and topics could be of PG 13+ nature.

**Writing Our Stories: A Parent’s Workshop in Healing Through Creativity**  
*Penn Valley*

_Sally Bittner Bonn, writer, literary teaching artist, SMA mom_  
_David Merulla, writer/musician, healthcare worker, SMA dad_

Being the parent of a child with SMA brings unique emotional challenges. With so much focus on our kids’ needs, our own needs as parents and caregivers are often overlooked. Creative writing can be a healing and enriching way to tackle some of these difficult emotions and address these needs. Studies show that writing through trauma has a positive impact both emotionally and physiologically. This hands-on workshop will offer a series of writing exercises and strategies for parents to explore, with the goal of emotional release and healing in mind. In addition, participants will receive a resource guide full of creative ideas to try at home. No previous writing experience necessary.

**The Role of PT in your Child’s Life – Being a part of a Therapy Team**  
*Liberty*

_Kristin J Krosschell, PT, DPT, MA, PCS_  
_Karen Patterson, PT, MS, PCS_  
_Terri Carry, PT_

We will explore the overall general Physical Therapy needs of children with SMA. We will provide and share examples of treatment ideas that you can also share with your local therapists. We will discuss evidence-based interventions to help your child maximize their function.

**Ethics in Research**  
*Century Ballroom A*

_Wynne Morrison, MD, MBE_  
_Rob Graham, MD_  
_Vanessa Battista, RN, M.S., CPNP, CCRC_

Beyond the “hope and hype” of research, there are ethical, emotional, and practical considerations for the individual with SMA and their family. This session will review factors that may influence decision-making. Extensive time will be allotted for open discussion of research benefits and challenges.

**Toy Adaptation**  
*Century Ballroom B*

_Shannon & Justin Kuester, SMA Parents_

The adapting toys workshops will be a hands-on how-to session. You will learn to adapt a toy by physically adapting one yourself with guidance and instruction. Each participating family will take home a toy they have adapted (Limit one toy per family on a first-com, first serve basis).

**Transition to Adulthood**  
*Shawnee*

_Melissa Milinovich, Hewlett Packard, Mother, SMA Adult_

This workshop will feature two individuals with SMA telling their personal stories of how they successfully transitioned to independent living, college and then employment. Information and resources will be
provided on personal care assistance services (including managing a staff of personal care attendants),
different forms of assistive technology used to increase independence, and ways to maintain needed
benefits (such as Medicaid) while working

3:15pm – 5:15pm  Researcher Q & A Session  Century Ballroom C

3:15 pm  Welcome and Update on Cure SMA Research Activities. Jill Jarecki, PhD, Research Director, Cure SMA.
Dr. Jarecki will discuss the scope and goals for Cure SMA investments in basic, drug discovery, and clinical research.

3:30 pm  The Complexity of Clinical Trials for SMA.
Introductory Talk on Clinical Trials for SMA.  John T. Kissel, MD, Chair, Department of Neurology, The Ohio State University.

3:45pm to 4:30 pm  Moderated Panel Discussion on Complexity of SMA Clinical Trials.
- John T. Kissel, MD, Chair, Department of Neurology, The Ohio State University (Moderator).
- Richard Finkel, MD, Division Chief, Division of Neurology, Nemours Children's Hospital
- Katherine Klinger, PhD, Sr. Vice President, Genetics and Genomics and Presidential Fellow, Genzyme Corporation
- Thomas H. Murray, PhD, Senior Research Scholar, President Emeritus, The Hastings Center
- John Whyte, MD, MPH., Director of Professional Affairs and Stakeholder Engagement, Center for Drug Evaluation and Research, FDA

4:30pm to 5:15 pm  Panel on SMA Drugs in Development
- Jinsy Andrews, MD, Director of clinical Research and Development, Cytokinetcs
- Jerry R. Mendell, MD, Director, Center for Gene Therapy, The Research Institute at Nationwide

Saturday, June 20th
Saturday, June 20th

Children’s Hospital The Research Institute at Nationwide Children’s Hospital, Columbus Ohio

- Wildon Farwell MD, MPH. Medical Director, Neurology Early Clinical Development, Biogen
- Irene Gerlach, PhD, Project Team Leader Rare Diseases, F. Hoffmann-La Roche
- Eugene Schneider, MD, Executive Director, Clinical Development, Isis Pharmaceuticals
- Emilie Voltz, PhD, Clinical Trials Leader in Neurosciences, Translational Medicine, Novartis Institutes for Biomedical Research
- Thomas Blaettler, MD, Global Clinical Development Team Leader, F. Hoffmann-La Roche

6:45pm – 9:30pm   PJ Party and Movie Night   Century Ballroom

Sunday, June 21st

7:30am – 9:00am   Family Breakfast Buffet   Century Ballroom A & B

9:15am – 11:30am   It's a Wonderful Life/Closing General Session   Century Ballroom C

We will close the conference with this amazing panel comprised of adults with SMA who will share their life’s journey and advice on living with SMA. This general session will leave you inspired and in awe of their accomplishments. Don’t miss this! Teens are welcomed and encouraged to attend as well!

Announcing conference survey winners for iPads!
At Astellas, we believe we can make a real impact on the future.

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BAYADA Pediatrics is proud to support

Cure SMA

With a broad range of services and a team of pediatric professionals who are committed to keeping children with SMA safe at home, BAYADA provides:

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

This conference does not happen without a tremendous amount of work and support as well as kind and generous donors like you.
Thank You
BioMarin

At BioMarin, we are inspired and driven by the patients who receive our therapies, and we will continue our efforts to help more patients suffering from rare diseases who have unmet medical needs.

We are dedicated to making a meaningful impact in the lives of patients suffering from rare genetic diseases that are often underserved and ignored.

For more information, please go to www.bmrn.com

Please Note

During this conference we would like to strongly encourage our conference attendees to take proper sanitary actions. Please remember to wash your hands frequently so we can keep the spread of germs to a minimum. If you or a family member are feeling under the weather, we advise that you not attend workshops or the children’s program. There are hand sanitizers throughout the conference so please take advantage of those.

Swimming Pool

Please take extra caution around this year’s hotel pool and Jacuzzi. There will be NO lifeguard on duty. Parents, please keep an eye on your children at all times, anyone entering the pool facility will be swimming at their own risk.

Disclaimer: The acceptance of Exhibitors and Sponsors does not constitute or imply endorsement by Cure SMA of any company, product or service. Cure SMA accepts no responsibility for any claims made by any outside party.
August is SMA Awareness Month

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

Susan Apkon, MD, Dr. Apkon is the Director of Rehabilitation Medicine at Seattle Children’s Hospital in Seattle Washington. She is an Associate Professor of Rehabilitation Medicine at University of Washington. She is the Director of the Neuromuscular Clinic at Seattle Children’s and cares for children with spinal muscular atrophy in this multidisciplinary clinic. As a rehabilitation physician, her goal is to improve the functional abilities of children with SMA and work to improve their quality of life. As a board-certified pediatrician, she also helps primary care doctors throughout the Pacific Northwest with the overall medical management of children with SMA.

Rocco Arizzi, was born in Stratford, New Jersey in December 1973 as the fourth of four children. He was diagnosed with Spinal Muscular Atrophy (later confirmed as type II) at about age three. When he was four years old he was selected as the Muscular Dystrophy Association’s Goodwill Ambassador for the state of New Jersey and shortly thereafter began a two-year reign as National Goodwill Ambassador. His family moved to San Antonio, TX when he was in kindergarten. At age 16, Rocco was one of 200 Texas high school students chosen to attend the Texas Academy of Mathematics and Science, a two-year early-entry college program at the University of North Texas. He went on to attain a Bachelor of Science in Electrical Engineering at the University of Texas at San Antonio. While working as an adjunct algebra instructor and tutor at a local community college, he attained a master’s degree in Electrical Engineering from Florida Atlantic University in Boca Raton. Then he received a PhD in Electrical Engineering from The Catholic University of America in Washington, DC. Rocco is currently 39 years old and resides in Barrington, NJ. He is employed as an electrical engineer at the Naval Surface Warfare Center in Philadelphia and is also an adjunct Physics professor at Drexel University in Philadelphia.

Carolyn Barrett, is 21 years old and a rising senior at Boston College where she’s studying Political Science. Carolyn has SMA type II and a retired service dog at home. Carolyn has been attending the conference for as long as she can remember and looks forward to sharing her SMA experience with others.

Paula Barrett, is an experienced human resources professional that works as a consultant for small and medium size businesses. Her diverse experience includes recruitment and retention, compensation management, performance management, training and organizational development. Paula also just completed a five-year term as Chair for the Board of Cure SMA. Prior to this she was secretary of the New England Chapter. Paula and her husband Michael have a daughter Carolyn who has SMA type II and is a student at Boston College.

Vanessa Battista, RN, MS, CPNP, CCRC, currently works as a Pediatric Nurse Practitioner (PNP) with the Pediatric Advanced Care Team (PACT) at The Children’s Hospital of Philadelphia. She holds a B.A. in psychology from Boston College, a B.S. and M.S. from Columbia University School of Nursing, and a certificate in Pastoral Ministry from the Boston College School of Theology and Ministry. Initially, Vanessa worked as a clinical research coordinator, RN, and then PNP at the SMA Research Center at Columbia University Medical Center in NY, and then practiced as a PNP at the SMA Center at Children’s Hospital Boston, before relocating to Philadelphia. Vanessa’s interests lie in providing interdisciplinary care to children/adolescents and families living with chronic conditions. She lectures and teaches locally and nationally and serves on various advisory boards. She is also a member of the Cure SMA Medical Advisory Council (MAC).

Sally Bittner Bonn and David Merulla, are parents to Oscar, age six, who has SMA Type II. They have spent the last five years since his diagnosis using their creativity to express and process the complex emotions that come with being the parents of a child with SMA. Sally is a published writer, performance poet, and literary teaching artist. She works part-time as the Director of Youth Education at Writers & Books, and has been leading creative writing workshops for adults and children for over a dozen years. She is writing a book about the joys and challenges of raising Oscar. David is a singer/songwriter, musician, and fiction writer who plays under the moniker Autumn in Halifax www.autumninhalifax.com. He has toured regionally on both the east and west coasts, opening for such bands as Calexico, Damien Jurado, and Tristeza. David has released several albums with Carbon Records. He works for the University of Rochester Medical Center in the Cardiology Department. In addition, Sally and David created a multi-media performance entitled Are You Able? for Rochester’s 2014 Fringe Festival. Website and blog: www.oscar-go.org

Anne Buckley-Reen, Anne is an Occupational Therapist and Yoga therapist who has been developing yoga sequences to address the needs of special populations for the past 15 years. Anne’s researched program “Get Ready to Learn: Yoga Therapy in the Classroom” is in use in over 700 NYC classrooms, 20 states and 4 countries. She is delighted to be participating again in the FSMA Conference.
Terri Carry, PT, graduated from University of Colorado school of Physical Therapy. Have worked entire career in pediatric physical therapy doing a variety of different things but have always had special interest in the Neuromuscular patients. Have worked in the Children's Hospital Colorado Muscle clinic since it was first established. Currently working on many clinical trials for both SMA and Duchenne. Excited about the possibility of treatment for these diseases.

Thomas Crawford, MD, has been interested in all aspects of SMA since 1980. He is currently at Johns Hopkins hospital in Baltimore, Maryland and continues to be involved in research and clinical care of children with SMA. He is also a member of Cure SMA’s Scientific Advisory Board.

Albert Freedman, PhD, Dr. Freedman is a child and family psychologist in independent practice in West Chester, Pennsylvania. He provides consultation and training to professionals in health care and educational settings, and frequently speaks and writes on the topic of caring for children with special needs. He has served as a member of the Family Advisory Council at the A.I. duPont Hospital for Children in Wilmington, Delaware and is a consultant with Bayada Home Health Care in Moorestown, NJ. Dr. Freedman’s son, Jack, was born in 1995 and is affected by SMA Type I.

Natalie Gibbs, Natalie has a Bachelors Degree in Public Relations from the University of Kansas. She is currently part owner of a swimming pool and outdoor furniture company in Overland Park, Kansas. Natalie has been actively involved with Cure SMA since 1999 and is currently the Kansas City Chapter area President. She has two daughters, Lauren, SMA Type III and Claire, SMA Type II. She is currently serving as the Chapter elected board member.

Rob Graham, MD, Dr. Graham, a specialist in Critical Care Medicine, is the Clinical Director for Clinical Care, Anesthesiology, Perioperative Extension (CAPE) and Home Ventilation Programs at the Children's Hospital Boston in Massachusetts. He is dedicated to both the acute and long-term care of children with SMA. Through his outpatient, home visit, and educational programs, he hopes to improve the care and quality of life for children and families with SMA.

Matt Halanski, MD, Dr. Halanski earned his medical degree from Wayne State University, Detroit, Mich., in 2001. He completed his orthopedic surgery residency at the University of Wisconsin, Madison. He completed his pediatric orthopedic surgery fellowship at Starship Children’s Health in Auckland, New Zealand. Dr. Halanski joined the orthopedic department at the University of Wisconsin in 2011, where he assumed the orthopedic care of the neuromuscular population at American Family Children’s Hospital at UW. Dr. Halanski’s interests include clinical medicine and orthopedic research. He enjoys treating all areas of pediatric orthopedics, with special interests including spinal and lower-extremity deformities. His recent research interests have focused on limiting patient morbidity by minimizing operative time and blood loss in spinal deformity surgery, development of animal models to study spinal deformity, limb growth, and corrective procedures; and resident education. He and his colleagues at UW have recently created an SMA database in hopes to answer some clinical questions surrounding the SMA population. He is board certified by the American Board of Orthopedic Surgery (ABOS), an active member of the Pediatric Orthopedic Society of North America (POSNA), and a candidate member in the Scoliosis Research Society (SRS).

Stevie Hopkins, believes that his SMA Type II is the cause of all of his brilliance and good looks. To come to this understanding, Stevie has had to overcome his fair share of challenges, depression and the loss of his sister. Now an accomplished entrepreneur and motivational speaker, he has found a new love for life. Stevie is the co-founder of 3E Love, home of the Wheelchair Heart symbol, and has work experience in the financial, insurance, and music industries. Hard work and perseverance has allowed him to travel nationwide and meet thousands of people on his journey. Stevie seeks to share his message and story with others, empowering them to live for the moment and follow their dreams.

Rebecca Hurst Davis MS, RD, CSP, CD, Rebecca is a clinical dietitian at the University of Utah’s Pediatric Motor Disorder Research Program in Salt Lake City, Utah. Her work experience consists of work in pediatric nutrition and metabolic nutrition. As a metabolic dietitian, she worked with several different metabolic disorders including PKU and fatty acid oxidation disorders. She is a board certified specialist in pediatric nutrition. Currently, she focuses on nutritional management and research for infants, children, teenagers, and adults with Spinal Muscular Atrophy (SMA). Rebecca is working to provide evidence-based nutritional research as well as to optimize the nutritional management of SMA.

John Kissel, MD, Dr. Kissel is Professor of Neurology, Pediatrics and Neuroscience at The Ohio State University Wexner Medical Center and Nationwide Children’s Hospital in Columbus, Ohio, where he serves as director of MDA and SMA Clinics. He has been on staff at The Ohio State
University since 1985 and was named chair of the Department of Neurology in July, 2015. He also holds The Gilbert and Kathryn Mitchell Chair in Neurology. Dr. Kissel has published extensively in a wide range of peripheral nerve, muscle, and motor nerve disorders, including spinal muscular atrophy. He is a Fellow of the American Academy of Neurology and a member of the American Neurological Association.

Richard Kravitz, MD, is Associate Professor of Pediatrics in the Division of Pediatric Pulmonary and Sleep Medicine at the Duke University Medical Center in Durham, North Carolina. He has a long-standing interest in caring for patients with neuromuscular weakness and helps to advocate for their needs on a local, state, and national level. He is co-director of the Duke Comprehensive Neuromuscular Clinic, where he provides pulmonary care to children and adults with neuromuscular weakness in a multi-disciplinary setting. He has co-authored several position papers on the management of the respiratory complications of patients with neuromuscular weakness. He is a member of the Cure SMA Medical Advisory Council.

Kristin J. Krosschell, PT, DPT, MA, PCS, is an Assistant Professor in the Department of Physical Therapy and Human Movement Sciences at Northwestern University Feinberg School of Medicine. Her teaching is focused in the pediatric, lifespan and neurological clinical content. She has been a pediatric physical therapist for over 31 years, and has been involved in the treatment of individuals with neuromuscular disorders throughout her career, treating individuals with Duchenne Muscular Dystrophy (DMD) and Spinal Muscular Atrophy (SMA) since her first year of practice. She is Board Certified in Pediatric Physical Therapy. Her research focus has been on outcomes development and measurement in SMA. She has been a member of the Project Cure SMA Clinical Trials Group, has participated in the International Coordinating Committee for SMA Clinical Trials (ICC) and is a member of the ICC Outcomes group and was a contributing member to the SMA Standard of Care Consensus document. She is also a member of the NIH Task Force on Childhood Motor Disorders, the Cure SMA Medical Advisory Committee, the NINDS Pediatric Neuromuscular and Spinal Muscular Atrophy Common Data Elements Working Groups, the Chicago Cerebral Palsy Research Registry project, the APTA Pediatric and Neurology Sections, and the AACPDM continuing education committee.

Justin and Shannon Kuester, were brought into the world of Cure SMA in 2006 with the birth of their daughter, Addison (SMA Type I). Justin attended Virginia Tech and holds a Bachelors Degree in Computer Science and Masters Degree in Information Technology. Justin has over 17 years of experience with both Accenture and FIS providing IT Consulting and Management services for clients in the Federal Government, Financial Services and retail business. He has been very active in the SMA community online as well as locally working with the University of Wisconsin American Family Children Hospital and Dr. Mary Schroth. Justin is very excited to serve the SMA community with a balanced view on family support and finding a cure.

Shannon is married to Justin and together they have two daughters. Haley is their oldest and she is ten. Before Shannon had kids of her own, she was a special education teacher. For a short time she taught students with learning disabilities Intro to Algebra and Algebra I. Shannon enjoys knitting, hiking and trying to sew. Shannon taught herself to adapt toys because she hated the high price tag that was attached to switch adapted toys. She has no technical background, but she does have a history of breaking computers and cell phones, so if Shannon cause a soldering iron, so can you!

Jennifer Lemisch, MA, ATR-BC, LPC, is a board certified art therapist, licensed professional counselor who has been employed at The Children’s Hospital of Philadelphia since 2001. For the past 11 years, she has been part of the Pediatric Advanced Care Team, the palliative care service at the hospital, working with patients and siblings, both in the inpatient setting and in the community doing home visits with families.

Melissa Milinovich, is from Amelia, Ohio and was diagnosed with Spinal Muscular Atrophy at the age of 2 after several misdiagnoses; however, she has never let that stop her. She graduated with honors from Wright State University with a Bachelor’s of Science in Business in 2000 and has been on the fast-track ever since. From being a career woman with the Hewlett Packard Company as an Account Delivery Lead to a single mother of her daughter, Claudia, Melissa strives to reach the high goals she has set for herself and never allows anyone to tell her she “can’t” do something. Despite using a wheelchair for mobility, she lives a very fulfilling life and encourages others to do the same. In her free time, she is the state coordinator for the Ms. Wheelchair Ohio – America program after being selected as Ms. Wheelchair Ohio 2005 and placing as the 3rd runner up at the Ms. Wheelchair America 2006 pageant. Melissa also serves on the National Board of Directors for Cure SMA.
Wynne Morrison, MD MBE, is a physician practicing pediatric critical care and pediatric palliative care at the Children’s Hospital of Philadelphia. She is a faculty member in the Department of Anesthesiology and Critical Care and the Department of Medical Ethics and Health Policy at the Perelman School of Medicine at the University of Pennsylvania, where she teaches in the medical school professionalism and ethics curriculum. Her scholarly work is in the areas of pediatric ethics, end of life care, and patient-family-physician communication. Writing poetry helps to keep her sane.

Pamela J. Mosher, M.D., M.Div., Dr. Mosher was born and raised in Boston, MA but now lives in Canada. She holds a B.Sc. in Psychology from Duke University and an M.Div. from Harvard Divinity School, where she studied comparative religion, ethics, and interpretations of suffering and death across cultures/religious traditions. She obtained her M.D. from Stanford University School of Medicine. She completed combined residency training in pediatrics, adult psychiatry, and a fellowship in child/adolescent psychiatry at the Triple Board Program at Brown University/Warren Alpert School of Medicine. Dr. Mosher formerly worked as a staff physician at the IWK Health Centre in Halifax, Nova Scotia on the pediatric palliative care service and then within the division of Child/Adolescent Psychiatry in emergency psychiatry, consultation-liaison psychiatry, and outpatient mental health. She will soon be moving to Toronto, Ontario. Dr. Mosher has longstanding interests in the psychological experiences of medically-ill children and dying children, and grief and bereavement during childhood/adolescence.

Garey Noritz, MD, is an Internist and Pediatrician at Nationwide Children’s Hospital. He is medical director of the Complex Health Care Clinic, which provides a medical home to children and adults with neurodevelopmental disabilities. He is Associate Professor of Pediatrics at The Ohio State University, and the Director of the Comprehensive Cerebral Palsy Program at Nationwide Children’s Hospital. Dr. Noritz is a graduate of Brown University School of Medicine, and a Fellow of the American Academy of Pediatrics and of the American College of Physicians. He is Board Certified in Internal Medicine, Pediatrics, Neurodevelopmental Disabilities, and Hospice and Palliative Medicine. He is active at the national level in advocating for the care of children and adults with disabilities. He is Past Chair of the Lifespan Committee of the American Academy for Cerebral Palsy and Developmental Medicine, and is on several committees with the American Academy of Pediatrics, the American College of Chest Physicians, and Cure SMA Medical Advisory Council.

Dr. Noritz’s research interests include bone health in patients with disabilities, transition of children with disabilities to adult models of care, palliative medicine, and the respiratory care of patients with neuromuscular diseases.

Kevin O’Brien, Kevin served in the US Navy for four years as an electrician aboard ship. After his discharge he then entered the corporate world, working in the telecommunications and IT industries for the past 34 years in both technical and sales roles. Kevin is the father of Shannon O’Brien, a 28 year old with SMA Type III who works at the Cure SMA National Office along with his wife Karen. Kevin and his two other daughters, Katlyn and Traci have been active volunteers for the Cure SMA organization for the past 22 years.

Traci O’Brien, is a 26 year old recent grad of SIU and Grenoble Ecole de Management. She spent 17 months in the Southeast of France studying International Business and traveling throughout Europe and Morocco. Traci is most proud of surviving Graduate school and being co-founder of SIU’s Women’s Ultimate Frisbee club team. Traci couldn’t imagine life without music or her spiritual connection to nature. She loves dancing to the beat of her own drum and playing Nintendo with her big sis, Shannon O’Brien, who has SMA type III. She has been volunteering at the conferences ever since being released from child care. Traci gets stoked about attending conferences each year where she aims to be a source of inspiration and comfort to families. She says that CureSMA is like a second family to her and remarks, “I am grateful to have this network of such amazing and diverse people working toward a common goal—to not let SMA take anymore from us and to support each other, cry and laugh together along the way.”

Karen Patterson, MS, PT, PCS, is a Faculty Associate with the University of Wisconsin Physical Therapy Program in Madison, Wisconsin. She has worked in pediatric physical therapy for 15 years, in almost every setting possible and now teaches the pediatric neurology classes for a Doctor of Physical Therapy program at the University of Wisconsin-Madison. She sees pediatric patients in the outpatient rehab center and Neuromuscular Disease clinic, including SMA patients of all types. She was the senior physical therapist for the American Family Children’s Hospital, where she worked with many children with SMA who were hospitalized. She also has been involved as an investigator with Project Cure, and currently is working on the development of a retrospective study with Dr. Mary Schroth and Dr. Matt Halanski at the University of Wisconsin American Family Children’s Hospital.
Speakers (cont.)

Spencer Perlman, is President of Washington Strategic Consulting (WSC), a public policy consulting firm that provides a full suite of federal government relations services to disease advocacy groups, non-profit health systems, public universities, corporations, and local governments. WSC delivers expert counsel and insight to clients in three major practice areas: Legislative Advocacy, Public Policy Analysis, and Grants Management & Procurement.

Spencer possesses nearly 17 years’ experience in Washington, D.C. providing government relations counsel to an array of clients and working as a senior aide to a Member of the House of Representatives. Since joining WSC in 2007, he has spearheaded several critical client initiatives concerning health care reform, health information technology, newborn and carrier screening for genetic disorders, personalized medicine, traumatic brain injury, and medical education.

Spencer joined WSC from Drinker Biddle & Reath, a prominent national law firm, where he served for two years as a Government Relations Director. Previously, Spencer spent four years as Legislative Affairs Manager at Ungaretti & Harris, a respected Chicago-based law firm. From 1997 to 2001, Spencer served as Legislative Director for former Rep. John Edward Porter of Illinois, the then-Chairman of the House Appropriations Subcommittee on Labor, Health & Human Services, and Education.

Spencer received his Bachelor of Arts degree in History and English from the University of Michigan at Ann Arbor in 1997.

Richard M. Rubenstein, Esq., as an attorney and financial advisor, and father of a child with special needs who suffered from SMA, Richard is well versed and has a heartfelt commitment to assist families who require special needs planning. Richard concentrates his legal practice in estate planning, and elder law. At Strategies for Wealth, Richard works to develop long term relationships with his clients and base them on integrity and commitment to them, their families, friends and businesses. He begins by coordinating and integrating of all aspects of their personal and business finances. His role becomes that of a trusted attorney and financial advisor providing professional counsel to afford his clients with the financial confidence necessary to maximize efficiency and effectiveness in achieving great success, wealth and peace of mind. Richard is a Member, New York State Bar Association, National Society of Financial Services Professionals, National Association of Insurance and Financial Advisors, Treasurer, National Board of Directors, Cure SMA, Greater New York Chapter, Cure SMA, NYC Area Chapter, Muscular Dystrophy Association, Town of Yorktown, Group Home Committee, and Chairman, Town of Yorktown, Board of Ethics. He currently resides in Yorktown Heights, NY with his wife Michele, 9 year old daughter Emma, and dog Ike. He makes this presentation in loving memory of Max, “my special boy”, April 24, 2005 – February 8, 2009

Gina Santucci, RN, MSN, APRN-BC, has over 25 years of professional experience in pediatrics including palliative care, critical care, staff development and teaching. For the past 10 years her focus has been providing pediatric palliative care at the Children’s Hospital of Philadelphia. She is a member of the Hospice and Palliative care Nurses Association, American Academy of Hospice and Palliative Medicine and is actively involved in publishing, research and education. She frequently lectures on pain and symptom management and developed the core curriculum for the pediatric hospice and palliative nurse.

Mary Schroth, MD, is a Pediatric Pulmonologist and Director of the UW Pediatric Pulmonary Center Grant. She is an Associate Professor of Pediatrics at the University of Wisconsin School of Medicine and Public Health in Madison. She has been instrumental in developing a leading respiratory care program for children with neuromuscular disease and, with colleagues, in developing a multidisciplinary neuromuscular clinic. She played a pivotal role in the development of the Consensus Statement for Standards of Care in SMA. Dr. Schroth also chairs the Cure SMA Medical Advisory Council.

Erin Seffrood, MS, RD, CSP, CD, is a pediatric dietitian at the American Family Children’s Hospital in Madison, Wisconsin. She has been working in pediatric nutrition for 15 years. She specializes in providing medical nutrition therapy to infants, children, and teenagers with all types of spinal muscular atrophy. She is actively working to further understand the role diet plays in the treatment of SMA.

Mary Schwartz OTR/L, CDRS
David Sereni, is a senior physical therapist at Kaiser Permanente in Santa Rosa, California. He is also part of the adjunct faculty pool at Santa Rosa Junior College. He has been the president of the Northern California Chapter of CureSMA for 16 years. He lost his son Matthew to type I SMA in January of 1999. He has been involved with the “Grief and Loss” and the “Healing the Grieving Heart” workshops since his first conference in 1999. He has also been active in the Newly Diagnosed program for the past several years.

Richard Shell, MD, is a Pediatric Pulmonologist and Associate Professor in the Center for Respiratory Health at Nationwide Children’s Hospital and The Ohio State University in Columbus, Ohio. He is involved in the SMA and Neuromuscular Clinics and is very proactive in educating patients and families, as well as the medical staff, about the disease. He has been actively involved in research surrounding the care of patients with neuromuscular disease focusing on the respiratory complications of these diseases. He is also a member of the Hospice and Palliative Care Team and Ethics Committee, where he helps families consider very difficult decisions. He is a member of the Cure SMA Medical Advisory Council.

Danielle Sheypuk, PhD, promotes her very real message that people with disabilities are sexy, datable, educated, and glamorous—and that they are integrating more successfully than ever into the social scene. After a recent explosion of media attention when Dr. Sheypuk became the first model in a wheelchair to “walk” the runway at New York Fashion Week Spring 2014, her impact on the disabled community and on the way society views this population has been tremendous and universal. Holding a Ph.D. in Clinical Psychology from The New School for Social Research in New York City, Dr. Sheypuk is widely regarded as a “sexpert” and leading commentator on the psychology of dating, relationships, and sexuality for the disabled population. She has established a successful and innovative private therapy practice that employs Skype-based treatment, which she engineered specifically to make it easier for people with disabilities to attend sessions. In May 2015, Dr. Sheypuk appeared in her first TEDx Talk, originating from Barnard College at Columbia University. The topic was the changing culture of American sexuality, and the mainstreaming of people with disabilities as potential romantic and sexual partners in everyday life.

Louise Simard is a Professor and Chair of the Department of Biochemistry & Medical Genetics in the Faculty of Medicine at University of Manitoba in Winnipeg (Canada). She has been involved in SMA research since 1990 and her laboratory has made important contributions in the area of SMA genetics and translating this knowledge to DNA diagnostic applications. Her laboratory was among the first groups to demonstrate that SMN protein is very abundant in growth cones which are structures at the tip of axons that are migrating towards their target muscle. More recently, Dr. Simard has been involved in SMA clinical trials to measure SMN mRNA as a potential “biomarker” in blood samples of SMA children and adults receiving Valproic Acid. Dr. Simard has been actively involved in Cure SMA and Muscular Dystrophy Canada. In 2006 she received the Dr. David Green Award which is presented to a scientist who has made a significant contribution to Muscular Dystrophy Canada in the areas of neuromuscular research or the advancement of care of clients with neuromuscular diseases and in 2009, Dr. Simard was one of Cure SMA’s 25 Voices of SMA. In 2012, Dr. Simard was awarded the Queens “Diamond Jubilee” medal for her contributions to Muscular Dystrophy Canada and their clientele. Dr. Simard is the primary author of FSMA’s booklet entitled “The Genetics of Spinal Muscular Atrophy” which has now been translated into several different languages.

Brian Snyder, MD, is an Associate Professor of Orthopaedic Surgery, Harvard Medical School and Board Certified Pediatric Orthopaedic surgeon on staff at Children’s Hospital where his clinical practice focuses on spinal deformity related to congenital and neuromuscular etiologies, hip dysplasia and other acquired deformities about the hip, cerebral palsy and pediatric trauma. He organizes the orthopaedic resident upper and lower extremity fractures, basic orthopaedic biomechanics and the biomechanics of fracture healing. He is also the Director of the Orthopedic Biomechanics Laboratory at the Beth Israel Deaconess Medical Center. The Laboratory is a multi-disciplinary core research facility for Harvard University, Harvard Medical School, MIT, and the Harvard Combined Orthopaedic Residency Program. The OBL focuses on basic and applied research in musculoskeletal biomechanics including: bone mechanics; characterization of bone microstructural morphology and bone structure-property relationships; biomechanics of bone fracture, fracture healing and fracture treatment; pre-clinical evaluation of novel osteodynamic agents effect on bone biomechanics in animal models; etiology and prevention of pathologic fractures in osteoporosis and metastatic cancer; kinematics of
normal, injured and artificial joints; biomechanics of spinal injury and spinal instrumentation; validation of objective functions for optimized, computer assisted surgical planning of osteotomies about the hip for the prevention and treatment of osteoarthritis.

**Harvey J. Stern, MD PhD, FACMG, FAAP**, is currently the Director of Reproductive Genetics and the Preimplantation Genetic Diagnosis program at the Genetics & IVF Institute in Fairfax, Virginia. He is board certified in Medical Genetics and Pediatrics and has subspecialty certification in clinical, biochemical and molecular genetics. Dr. Stern is a member of many professional organizations including the American Society of Reproductive Medicine, American College of Medical Genetics and European Society of Human Reproduction and Embryology (ESHRE).

**Stacey Tarrant**, is a clinical nutrition specialist at Boston Children’s Hospital in Boston, Massachusetts. She has been providing medical nutrition therapy and nutrition counseling to families of children with spinal muscular atrophy for the past 7 years within Boston Children’s Hospital multidisciplinary SMA clinic. Her other specialty areas include dietary therapy for children with inborn errors of metabolism, including fatty acid oxidation disorders, and the ketogenic diet for children with intractable epilepsy. She is currently collaborating on a research study to better understand the body composition of children with SMA.

**Barbara Trainor**, has been involved with Cure SMA for over 20 years. Barbara created a major event fundraising manual for Cure SMA in an effort to help others create their own fundraising events. In 2003 Barbara took over as Chapter Coordinator for Cure SMA and led tremendous growth in both the fundraising and support functions of the Cure SMA Chapters. Barbara was elected to the national board of Cure SMA in March 2002. Barb continues to be the coordinator for The Annual SMA Conference. Through her tireless efforts, Barb has helped the conference grow into the annual highlight for the entire SMA Community. This is a conference that has true impact on the lives of families, patients, researchers and doctors. This meeting has been a major influence on bringing researchers into the SMA field and establishing successful collaborations that have moved us forward towards a treatment and cure. Barb has done all this alongside running the Cure SMA Chesapeake Chapter, which has now raised over $2.2 Million for SMA research and support.

**Fred Troutman, PhD**, is a retired Air Force Colonel and a nurse educator from Portland, Oregon. The death of his son, Jonathan, brought him to the self-help bereavement group, The Compassionate Friends, and lead to his study of how we manage the losses in our lives. He is interested in nonprofit service organizations and presents classes and workshops on loss, change and bereavement. Fred has attended Cure SMA conferences for a number of years.

**Angela Wrigglesworth**, a fourth grade teacher from Houston, holds an undergraduate degree from Texas A&M University and a master’s degree in special education from the University of St. Thomas. She is the coordinator of the Ms. Wheelchair Texas Foundation, is a member of the National Task Force on Public Awareness through MDA, and sits on the board of the Camp for All Foundation. Angela enjoys speaking about living with Spinal Muscular Atrophy Type II and sharing John Wooden’s philosophy that, “Things turn out best for those who make the best of the way things turn out.”

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**Speakers (cont.)**

Thank you to each and every single speaker who essentially enables this conference to exist year after year. These individuals volunteer their time to help educate the community on the latest information for Spinal Muscular Atrophy. Thank you for your time and expertise each year!
Disclaimer: The acceptance of Exhibitors and Sponsors does not constitute or imply endorsement by Cure SMA of any company, product or service. Cure SMA accepts no responsibility for any claims made by any outside party.
Everyone at Cure SMA would especially like to thank the following people:

The Cure SMA Board Members - Cure SMA is very fortunate to have such a dedicated group of individuals who represent the SMA Community. Our Board Members work exceptionally hard to make sure that all of our families and friends are supported the best of their ability. They help this community come together by devoting their efforts to the mission of Cure SMA. We would like to thank each and every one of you for everything you do!

Rich Rubenstein – Cure SMA is thrilled to have Rich as the Chair on the Cure SMA Board. We are all thankful for his passion, dedication and all that he has brought to this organization. Rich most certainly puts his all into this role and is always looking forward to improve, promote and assist in any way possible within the SMA Community.

The Cure SMA Chapter Officers – Our organization is incredibly lucky to have a wonderful support system throughout the U.S., who make up all of our Chapters. What these Chapter Officers provide is the guidance, support and knowledge that keep families connected and informed. Each and every one of these individuals represents Cure SMA in the best way possible and without them we would not be the community we are today.

The Cure SMA Medical Advisory Council Members – We are fortunate to have one of the most highly respected bodies of SMA medical and clinical experts in the U.S. as a part of this community. The Medical Advisory Council members are constantly brainstorming new ideas to raise awareness about SMA in the medical community, provide the best care for SMA patients and their families, and much more. These wonderful and knowledgeable professionals volunteer their own time, away from their clinics and families, to organize the conference workshops and educate families on many different medical aspects of SMA. We are all extremely grateful for the support and knowledge you all bring to Cure SMA.

The Cure SMA Scientific Advisory Board Members – The members of the Scientific Advisory Board annually review all applications for research grants and also help plan the program for the Annual Researcher Meeting. We would like to thank the members of the SAB for their expertise. We are incredibly thankful for their hard work and commitment and for ensuring that the “best” SMA research is funded by Cure SMA. The Cure SMA Translational Advisory Council Members – These individuals play an important role in the steps between early academic research to clinical and medical stages in the process of finding a cure for SMA. Translational research is the critical middle step that takes research ideas and makes them into practical solutions for patients. We extend a sincere thank you to all members of the TAC for taking their time on this challenge and role of supporting Cure SMA.

The Kansas City Chapter Officers and Members – The Kansas City Chapter was ecstatic when we announced that the Annual SMA Conference would be right in their backyard and they have carried that excitement leading up to the start of the conference! Thank you to all who have reached out to lend a hand and have been promoting this conference throughout your state!

Dr. Mary Schroth – Thank you so much to Dr. Schroth for everything you do for families and this organization! The conference, our care series booklets and so many of our programs would not happen without your help and hard work. We truly appreciate you and all that you do!

Evan Hill – Evan Hill Design has been extremely helpful this past year and for so many years before with all of our designs, booklets and constant updates. Thank you for your patience and all of the wonderful work you do!

The Miller McNeil Woodruff Foundation and Meredith & Patrick Woodruff – Thanks to this foundation many families from the state of Arkansas were again able to attend this amazing conference because of the scholarship opportunities they provided. Many of these families would have been unable to attend the conference without Meredith and Patrick’s generosity and help. The Miller McNeil Woodruff Foundation began in memory of Meredith & Patrick’s son, Miller, who has SMA type I.

Sweet Baby Zane and Hillary & Keith Schmid – A special thank you to Sweet Baby Zane and Hillary & Keith Schmid for sponsoring the Adults with SMA Social so that adults with SMA have the opportunity to spend time together and connect with each other!

The Angel Baby Foundation and John & Jamie Shish – The Angel Baby Foundation has graciously helped fund scholarships for families to attend the Annual SMA Conference this year! We cannot thank John & Jamie Shish and the Angel Baby Foundation enough for all that they have done for Cure SMA!
Thank You

Each year, Cure SMA is amazed at the amount of support received from our wonderful chapters and supporting families across the country. Through your efforts and the funds you raise and donate, you allow us to provide crucial family support programs and advance research programs to develop a treatment and cure for spinal muscular atrophy. We truly cannot thank you enough for the time, energy, and dedication put into everything you do!

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Cure SMA Board of Directors

Cure SMA holds itself accountable to its donors and volunteers, people with Spinal Muscular Atrophy and their families, as well as to the general public. The Board of Trustees is a diverse group of volunteer individuals, who bring a variety of experiences and expertise to the governance of Cure SMA. The boards’ responsibility is to direct and carry out the vision, mission, and goals of the organization.

Richard Rubenstein, Esq., Chair

Richard is Attorney and Financial Advisor at Strategies for Wealth based in Rye Brook, NY. His expertise is in estate planning, elder law, planning for persons with special needs and business succession planning. He was born and raised in the Town of Yorktown Heights in Westchester County, New York, where he currently resided with his wife Michele and daughter Emma. His experience with SMA began February 2006 when his son Max was diagnosed with the illness. Since Max’s death in February of 2009 he has not wavered in his resolve to contribute to the Cure SMA community. He participates in nearly all of the local chapter events, makes significant efforts to raise money for the charity and attends all of the national conferences. He has a heartfelt desire to be involved with Cure SMA as a board member, and though he never expects to achieve complete peace of mind regarding Max’s death, it is his love for Max and his cherished memory that drives his desire and inspires him to make a contribution.

Robert Lockwood, Secretary

Bob Lockwood graduated from the University of Cincinnati with a bachelor of science degree in psychology and retired in September 2008 from Wyeth Pharmaceuticals after 36 years in sales and sales management. He has been a member of the OKI Cure SMA Chapter since its inception in 2003 and has two grandchildren, Emma and Nicholas Lockwood with SMA type I. The OKI Chapter has raised over $1 million in that period of time. Bob is passionate about raising awareness and finding a cure for SMA. As one of the newer members of the Board, Bob looks forward to serving and fulfilling the vision of Cure SMA.

Bradley A. Nunemaker, Treasurer

Brad was diagnosed with SMA Type III when he was nine years old. Brad received his BBA from the University of Michigan Business School. He is a certified public accountant and is currently the Chief Financial Officer of Aon Hewitt Consulting. Brad previously served as Treasurer on the Board of Directors for Onward Neighborhood House, a family oriented not-for-profit in Chicago. He hopes to use his prior experiences to help Cure SMA and to continue to build the strong community of adults with SMA. Brad and his wife Krista live in Elmhurst, Illinois with their two sons. The whole family is proud to support Cure SMA.

Rocco Arizzi

Rocco was diagnosed with Spinal Muscular Atrophy (later confirmed as type II) at about age three. When he was four years old he was selected as the Muscular Dystrophy Association’s Goodwill Ambassador for the state of New Jersey and shortly thereafter began a two-year reign as National Goodwill Ambassador. His family moved to San Antonio, TX when he was in kindergarten. At age 16, Rocco was one of 200 Texas high school students chosen to attend the Texas Academy of Mathematics and Science, a two-year early-entry college program at the University of North Texas. Rocco holds a PhD in Electrical Engineering from The Catholic University of America. He is currently employed as an electrical engineer in Philadelphia and is an adjunct physics professor at Drexel University.

Corey Braastad, PhD

Douglas Erwin

Douglas and his wife, Michele discovered Cure SMA in 2007 after their son, Greyson was diagnosed with the illness. They joined the Greater NY Chapter, and have tried to make a difference at the local level by offering support and hope to other families, as well as raising money and awareness through annual fundraisers including the NYC Half Marathon. Douglas was honored to join the board of Cure SMA in 2009, and his goal is to make a difference in the organization, with all efforts some day culminating in a CURE for the disease. In his professional life he is a Vice President of Design at the Phillips-Van Heusen Corporation, which owns labels such as; Calvin Klein, Tommy Hilfiger, Izod, and Bass. Part of the success of each of these companies is that they are Brands which have a following. Douglas wants to use his professional experience to help turn Cure SMA into a Brand which registers in people’s hearts and minds, and makes them not only aware of the disease, but also makes them care and want to contribute to help our cause.
Cure SMA Board of Directors

Natalie Gibbs

Natalie has a Bachelors Degree in Public Relations from the University of Kansas. She is currently part owner of a swimming pool company in Overland Park, Kansas. Natalie has been actively involved with Cure SMA since 1999, and she is past president of the Kansas City Chapter. She is currently on the Family Advisory Board at Children’s Mercy Hospital in Kansas City, and is past president of the Board. Natalie, and her husband, Tim has two teenage daughters, Lauren, SMA Type III and Claire, SMA Type II.

Justin Kuester

Justin Kuester and his wife, Shannon were brought into the world of Cure SMA in 2006 with the birth of their daughter Addison (SMA Type I). He attended Virginia Tech and holds a Bachelors Degree in Computer Science and Masters Degree in Information Technology. Justin has over 16 years of experience with both Accenture and FIS providing IT Consulting and Management services for clients in the Federal Government, Financial Services and retail business. He has been very active in the SMA community online as well as locally working with the University of Wisconsin American Family Children Hospital and Dr. Mary Schroth. Justin is very excited to serve the SMA community with a balanced view on family support and finding a cure.

Melissa Milinovich

Melissa is a 35-year old woman from Amelia, Ohio who was diagnosed with Spinal Muscular Atrophy at the age of 2 after several misdiagnoses but has never let that stop her. She graduated with honors from Wright State University with a Bachelor’s of Science in Business in 2000 and has been on the fast-track ever since. From being a career woman with the Hewlett Packard Company as an Account Delivery Lead to a single mother of her daughter, Claudia, Melissa strives to reach the high goals she has set for herself and never allows anyone to tell her she “can’t” do something. Despite using a wheelchair for mobility, she lives a very fulfilling life and encourages others to do the same. In her free time, she is the state coordinator for the Ms. Wheelchair Ohio – America program after being selected as Ms Wheelchair Ohio 2005 and placing as the 3rd runner up at the Ms. Wheelchair America 2006 pageant.

Steve Ragland, Esq.

Steven discovered Cure SMA on the afternoon of February 13, 2008 – the day his then-20-month-old son Logan was diagnosed with SMA type II. The information and support offered during that time was invaluable and after the shock of diagnosis subsided, Steven and his wife got involved in the Northern California chapter of Cure SMA – meeting a wonderful network of families and fellow-travelers. Steven joined the Board of Cure SMA in 2009. In the professional realm, Steven is a partner in the San Francisco, California law firm Keker & van Nest LLP, where he specializes in complex civil and criminal litigation. Prior to his career as a lawyer, Steven worked in the Washington, D.C. non-profit realm and also taught political science to high school students. Steven received his B.A. in government from Lehigh University in Bethlehem, Pennsylvania and hid J.D. from American University, Washington College of Law in Washington, D.C. He lives in San Francisco with his wife, his son Logan and Logan’s big sister.

Adi Rappoport, Esq.

Adi is a shareholder with the Gunster law firm – residing in the firm’s West Palm Beach office. Adi represents business owners, businesses, charities, wealthy families, and corporate executives. His practice includes all areas of federal and state taxation, estate planning, tax exempt organizations, tax controversies, mergers and acquisitions and general corporate law. Adi is a Board Certified Tax Attorney. He received his LL.M. degree in Taxation, his Juris Doctor degree with honors and his Bachelor of Science degree in Accounting with highest honors all from the University of Florida. Adi has written and lectured on various tax topics relating to business taxation and estate planning.

Shaina and Adi Rappoport are the co-founders of The Jacob Isaac Rappoport Foundation, Inc. Shaina and Adi are the proud parents of four children, a daughter Jordan and three sons, Jacob, Max, and Sam. The Rappoport’s son Jacob died in 2002 from Spinal Muscular Atrophy. The Rappoport have formed The Jacob Isaac Rappoport Foundation in his memory. The Jacob Isaac Rappoport Foundation is dedicated to raising money for finding a cure or treatment for Spinal Muscular Atrophy and to provide support to affected children and their families.

Barbara Trainor

Barb has been involved with Cure SMA for over 20 years. Barb, along with her husband Gene, established the Chesapeake Chapter back in 1993. Since then the chapter has raised nearly $3 million for SMA research and support. In 2003 Barbara took over as Chapter Coordinator for Cure SMA and led tremendous growth in both the fundraising and support functions of the Cure SMA Chapters. Barbara was elected to the national board of Cure SMA from March 2002 to 2010. She joined the board again in 2012. Barb has been many years coordinating The Annual SMA Conference. Through her tireless efforts, Barb has helped the conference grow into the annual highlight for the entire SMA Community. More recently, she and her husband Gene have established the Erin Trainor Memorial Fund to provide scholarships for newly diagnosed families to attend the annual SMA conference. She attended the University of Maryland where she received her Bachelor of Science degree in Business Marketing. Barb resides in San Francisco with her husband Gene and two daughters Caitlin and Grace.
Cure SMA Board of Directors

Gregory Zerzan, Esq.

Greg Zerzan and his wife Shannon have two children, one of whom has SMA. Greg is an attorney in Washington, DC. He has held several positions in both the public and private sectors, including as counsel to various congressional committees and as an official in the United States Treasury. The family lives in Alexandria, Virginia.

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Cure SMA Medical Advisory Council (MAC)

Mary K. Schroth, MD, Chair
Dr. Schroth is the Director of the Pediatric Pulmonary Center Grant at the American Family Children's Hospital, University of Wisconsin School of Medicine and Public Health in Madison. She is also a Professor of Pediatric Pulmonology on the faculty at the University of Wisconsin. She has been instrumental in developing a leading respiratory care program for children with neuromuscular disease and, with colleagues, in developing a multidisciplinary neuromuscular clinic. She played a pivotal role in the development of the Consensus Statement for Standards of Care in SMA. As a specialist in SMA respiratory care and an educator, Dr. Schroth is a leader in the SMA community and with Cure SMA.

Susan Apkon, MD
Dr. Apkon is the Director of Rehabilitation Medicine at Seattle Children’s Hospital in Seattle Washington. She is a Professor of Rehabilitation Medicine at University of Washington. She is the Director of the Neuromuscular Clinic at Seattle Children’s and cares for children with spinal muscular atrophy in this multidisciplinary clinic. As a rehabilitation physician, her goal is to improve the functional abilities of children with SMA and work to improve their quality of life. As a board-certified pediatrician, she also helps primary care doctors throughout the Pacific Northwest with the overall medical management of children with SMA.

Vanessa Battista, RN, MS, CPNP, CCRC
Vanessa Battista, RN, MS, CPNP, CCRC is a board certified Pediatric Nurse Practitioner (PNP) and is currently a member of the Pediatric Advanced Care Team (PACT) at The Children’s Hospital of Philadelphia. She holds a B.A. in psychology from Boston College, a B.S. and M.S. from Columbia University School of Nursing, and a certificate in Pastoral Ministry from the Boston College School of Theology and Ministry. Vanessa has been caring for children and families living with SMA for the past ten years and has participated in various aspects of clinical research. As a PNP, Vanessa worked at the Pediatric Neuromuscular Center/Spinal Muscular Atrophy Research Center at both Columbia University Medical Center and The Children's Hospital of Boston. Vanessa's interests lie in providing interdisciplinary care to children/adolescents and families living with chronic conditions. She lectures and teaches nationally and internationally and serves on various advisory boards. She is currently an investigator on an FSMA funded research study on nutritional management and research for individuals with SMA.

Terri Carry, PT
Graduated from University of Colorado school of Physical Therapy. Have worked entire career in pediatric physical therapy doing a variety of different things but have always had special interest in the Neuromuscular patients. Have worked in the Children’s Hospital Colorado Muscle clinic since it was first established. Currently working on many clinical trials for both SMA and Duchenne. Excited about the possibility of treatment for these diseases.

Thomas Crawford, MD
Dr. Crawford has been interested in all aspects of SMA since 1980. He is currently at Johns Hopkins hospital in Baltimore, Maryland and continues to be involved in research and clinical care of children with SMA. He is also a member of Cure SMA’s Scientific Advisory Board and Medical Advisory Council.

Albert Freedman, PhD
Dr. Freedman is a child and family psychologist in independent practice in West Chester, Pennsylvania. He provides consultation and training to professionals in health care and educational settings, and frequently speaks and writes on the topic of caring for children with special needs. He has served as a member of the Family Advisory Council at the A.I. duPont Hospital for Children in Wilmington, Delaware and is a consultant with Bayada Home Health Care in Moorestown, NJ. Dr. Freedman has two children, Jack (age 20, SMA Type 1) and Cara (age 15, unaffected).

Robert Graham, MD
Dr. Graham, a specialist in Critical Care Medicine, is the Clinical Director for Clinical Care, Anesthesiology, Perioperative Extension (CAPE) and Home Ventilation Programs at the Children's Hospital Boston in Massachusetts. He is dedicated to both the acute and long-term care of children with SMA. Through his outpatient, home visit and educational programs, he hopes to improve the care and quality of life for children and families with SMA.

Rebecca Hurst Davis MS, RD, CSP, CD
Rebecca is a clinical dietician at the University of Utah's Pediatric Motor Disorder Research Program, bionutritionist for the Center for Clinical and Translational Science, and instructor for the Division of Nutrition at the University of Utah in Salt Lake City, Utah. Her work experience consists of work in pediatric nutrition and metabolic nutrition. As a metabolic dietitian, she worked with several different metabolic disorders including PKU and fatty acid oxidation disorders. She is a board certified specialist in pediatric nutrition. Currently, she focuses on nutritional management and research for individuals with neuromuscular disorders.
Cure SMA Medical Advisory Council (MAC)

**Richard Kravitz, MD**

Richard Kravitz, MD, is Associate Professor of Pediatrics in the Division of Pediatric Pulmonary and Sleep Medicine at the Duke University Medical Center in Durham, North Carolina. He has a long-standing interest in caring for patients with neuromuscular weakness and helps to advocate for their needs on a local, state, and national level. He is co-director of the Duke Comprehensive Neuromuscular Clinic, where he provides pulmonary care to children and adults with neuromuscular weakness in a multi-disciplinary setting. He has co-authored several position papers on the management of the respiratory complications of patients with neuromuscular weakness as well as spoken nationally and internationally on these topics. He is a member of the Cure SMA Medical Advisory Council.

**Kristin J. Krosschell, PT, DPT, MA, PCS**

Dr. Krosschell is an Associate Professor in the Departments of Physical Therapy and Human Movement Sciences and Pediatrics at the Feinberg School of Medicine, Northwestern University, in Chicago Illinois. As a pediatric physical therapist she has been both a clinician and researcher, with a focus on infants and children with neuromuscular and neurological disorders and muscle diseases. She is a frequent speaker at the SMA Annual Conference and has worked closely with Cure SMA for many years. Her work in pediatric neuromuscular care, and spinal muscular atrophy in particular, spans nearly 30 years.

**Nancy Kuntz, MD**

*Nancy Kuntz, MD*

Nancy Kuntz, MD is a board certified child neurologist with additional board certifications in Pediatrics, Neurodevelopmental Disabilities, Electrodiagnostic Medicine and Autonomic Medicine. Dr. Kuntz joined the Departments of Pediatrics and Neurology at Northwestern Feinberg School of Medicine in December 2009 and is Medical Director of the Mazza Foundation Neuromuscular Disorders Program and the MDA Clinic at Lurie Children’s. Before relocating to Chicago, Dr. Kuntz was in the Department of Neurology at Mayo Clinic Rochester where she did her training in child neurology and clinical electromyography and developed experience treating neuromuscular disorders in children. Dr. Kuntz is interested in the development of less invasive methods of neuromuscular diagnosis in children and serves as site Principal Investigator in a number of clinical treatment trials for neuromuscular disorders in children.

**Oren Kupfer, MD**

Dr. Kupfer is a board-certified pediatric pulmonologist. He completed pediatric pulmonary training at Children’s Hospital Colorado and the University of Colorado School of Medicine in 2013. He is dedicated to respiratory care of children SMA, developing evidence-based clinical care guidelines for respiratory illnesses and post-operative care, and improving quality of life and survival in SMA. He lives in Denver, CO with his wife and three young children.

**Elizabeth McNally MD, PhD**

*Elizabeth J. Ward Professor, and Director, Center for Genetic Medicine*

Elizabeth McNally directs the Center for Genetic Medicine (CGM) at Northwestern’s Feinberg School of Medicine. Dr. McNally is a cardiologist who specializes in caring for the cardiac complications of neuromuscular disease. Dr. McNally’s research is on inherited forms of heart disease and muscle disorders that affect children and young adults. Dr. McNally serves as an advocate through her work with the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, and the American Heart Association.

**Diane Murrell, LCSW**

*Diane V. Murrell, LCSW*

Diane V. Murrell, LCSW, is a licensed clinical social worker in the neurology division at Texas Children’s Hospital in Houston, Texas. She works in the Blue Bird Circle Clinic with families who have children with a chronic illness or disability and is the dedicated social worker for the muscular dystrophy association clinic. She is also the author/illustrator of three children’s books and maintains a blog on the TCH web site that addresses concerns related to bullying, inclusion and the development of empathy. Her interests in research are from the perspective of social work and include autism, spinal muscular atrophy, inclusion, bullying and the psycho social impact of diagnosis-related loneliness on a parent and/or a child’s quality of life. In addition, Diane founded and is the volunteer manager for the Houston Fireballs, the first power soccer program in Texas. This program seeks to provide a normal sports experience for those in power wheelchairs (www.houstonfireballs.com).

**Garey Noritz, MD**

*Garey Noritz, MD*

Garey Noritz, MD is an Internist and Pediatrician at Nationwide Children’s Hospital. He is medical director of the Complex Health Care Clinic, which provides a medical home to children and adults with neurodevelopmental disabilities. He is Associate Professor of Pediatrics at The Ohio State University, and the Director of the Comprehensive Cerebral Palsy Program at Nationwide Children’s Hospital.

Dr. Noritz is a graduate of Brown University School of Medicine, and a Fellow of the American Academy of Pediatrics and of the American College of Physicians. He is Board Certified in Internal Medicine, Pediatrics, Neurodevelopmental Disabilities, and Hospice and Palliative Medicine. He is active at the national level in advocating for the care of children and adults with disabilities. He is on the Executive Committee of the Council on Children with Disabilities of the American Academy of Pediatrics, and is past Chair of the Lifespan Committee of the American Academy for Cerebral Palsy and Developmental Medicine. In 2013, he was awarded “Physician of the Year” by Nationwide Children’s Hospital.

Dr. Noritz’s research interests include bone health in patients with disabilities, transition of children with disabilities to adult models of care, palliative medicine, and the respiratory care of patients with neuromuscular diseases.
Dr. Noritz was the lead author of the American Academy of Pediatrics’ recent clinical report, “Motor Delays: Early Identification and Evaluation”, which advises Pediatricians to institute screening for motor problems in early childhood.

Julie A. Parsons, MD

Associate Professor of Pediatrics and Neurology, University of Colorado Children’s Hospital Colorado

Julie A. Parsons, M.D. earned her BA in Human Biology and Psychology at Stanford University, and has been with the University of Colorado School of Medicine since completing the Child Health Associate Program in 1979 earning a Master’s of Science degree. After practicing as a pediatric physician assistant for five years, she earned her MD and completed her Pediatric Internship, Residency, Child Neurology residency and Neuromuscular Fellowship at University of Colorado. Dr. Parsons was in private practice from 1993 to 2000, and then joined the faculty at University of Colorado School of medicine where she is an Associate Professor of Pediatrics and Neurology and the Child Neurology Residency Program Director. Dr. Parsons was named the inaugural Haberfeld Family Endowed Chair in Pediatric Neuromuscular Disorders. She is co-director of the multidisciplinary MDA clinic at Children’s Hospital Colorado, which she has attended since 1993.

Karen Patterson, MS, PT, PCS

Ms. Patterson is a Faculty Associate at the Doctor of Physical Therapy Program at the University of Wisconsin-Madison where she teaches the pediatric neuroscience classes. She has worked in pediatric physical therapy for 15 years, in almost every setting possible. She continues to practice and is a senior physical therapist for the UW American Family Children’s Hospital where she sees children with SMA in the AFCH Neuromuscular Disease Clinic and in the UW Pediatric outpatient clinic. Ms. Patterson has also previously participated as an Investigator with Project Cure, and is currently involved with the establishment of a clinical database for SMA at UW.

Randal Richardson, MD, MMS

Randy Richardson is a neuromuscular neurologist at Gillette Children’s Specialty Healthcare in St. Paul, Minnesota. He sees children and adults with pediatric-onset neuromuscular disease and is an avid electromyographer.

Samuel Rosenfeld, MD

Dr. Rosenfeld is the director of the Neuromuscular Clinics at CHOC Childrens Hospital. He is a graduate of the Pennsylvania State University School of Medicine. Dr. Rosenfeld completed his Orthopaedic Surgery Residency at the University of California, Irvine and Fellowship in Children's Orthopaedics and Rehabilitation at Rancho Los Amigos National Rehabilitation Center. Dr. Rosenfeld is Board Certified by the American Board of Orthopaedic Surgery, and is a Fellow of the American Academy of Orthopaedic Surgery. Dr. Rosenfeld has been on staff at CHOC Childrens Hospital and clinical faculty at the University of California, Irvine since 1983. Dr. Rosenfeld is on the Professional Advisory Committee of the Spina Bifida Association, as well as the Medical Advisory Committee for CureSMA. Dr. Rosenfeld is a member of the Pediatric Orthopaedic Society of North America, American Academy of Pediatrics, AACPDM, ACPOC, COA, WOA, CMA, Orthopaedic Rehabilitation Association, and the American Medical Association.

Richard Shell, MD

Dr. Richard Shell is a Pediatric Pulmonologist and Associate Professor in the Center for Respiratory Health at Nationwide Children's Hospital and The Ohio State University in Columbus, Ohio. He is involved in the SMA and Neuromuscular Clinics and is very proactive in educating patients and families, as well as the medical staff, about the disease. He has been actively involved in research surrounding the care of patients with neuromuscular disease focusing on the respiratory complications of these diseases. He is also a member of the Hospice and Palliative Care Team and Ethics Committee, where he helps families consider very difficult decisions.

Perry Shieh, MD, PhD

Perry Shieh, MD, PhD, is Associate Professor of Neurology at the David Geffen School of Medicine at UCLA and Director of the Neuromuscular Division in the Department of Neurology at Ronald Reagan UCLA Medical Center in Los Angeles, California. He is also the program director of the Neuromuscular Medicine Fellowship Training Program at UCLA. He received his MD and his PhD in Neuroscience from Johns Hopkins University in Baltimore, Maryland. He completed residency training in neurology at Stanford University Hospital and fellowship training in clinical neurophysiology/EMG at Brigham and Women’s Hospital and Massachusetts General Hospital.

Dr. Shieh is a 2-time winner of The Golden Hammer Teaching Award from the Department of Neurology, UCLA School of Medicine. He is a member of the American Academy of Neurology and a Fellow of the American Academy of Neuromuscular and Electrodagnostic Medicine. His recent research has been published in clinical journals such as Annals of Neurology, Neurology, Nature Genetics, and JAMA.

Dr. Shieh lectures both nationally and internationally. His most recent speaking engagements covered chronic inflammatory demyelinating polyneuropathy (CIDP); treatments for genetic neuromuscular diseases; and the diagnosis of neuromuscular disorders using next-generation sequencing. Dr. Shieh’s principal clinical interests include spinal muscular atrophy, muscular dystrophy, inflammatory myopathy, myasthenia, electromyography, and muscle histopathology.
Cure SMA Medical Advisory Council (MAC)

Brian D. Snyder, MD, PhD

Brian D. Snyder, M.D., Ph.D. is a Board Certified, fellowship trained, Pediatric Orthopaedic surgeon on staff at Boston Children’s Hospital where his practice focuses on musculoskeletal deformity related to neuromuscular diseases affecting the spine, hip and appendicular skeleton. He is an Associate Professor of Orthopaedic Surgery at Harvard Medical School and conducts research for the Center for Advanced Orthopaedic Studies at Beth Israel Deaconess Medical Center. The Laboratory is a multi-disciplinary core research facility associated with the Harvard Medical School and the Departments of Bioengineering at Harvard University, Massachusetts Institute of Technology and Boston University Graduate School of Engineering. His research has focused on improving the practice of Orthopaedic surgery by applying quantitative engineering analysis to clinical problems affecting the musculoskeletal system, especially bone and joint structure and function in neurological disorders and metabolic bone disease.

Anne Stratton, MD

Dr. Stratton is originally from Ohio and received her M.D. from the University of Cincinnati in 2004. She then completed dual residencies in Pediatrics and Physical Medicine and Rehabilitation in 2009 at the University of Colorado, followed by a Pediatric Rehabilitation Medicine Fellowship in 2010. She is now one of the pediatric physiatrists actively involved in the multidisciplinary Neuromuscular Clinic at Children’s Hospital Colorado where she has enjoyed practicing the past 5 years. Clinically, she feels it is important to consider the whole patient, their lifestyle, and aspirations when making medical recommendations. She is also the mother of two young girls and enjoys doing some running in her free time.

Stacey Tarrant, BS, RD, LDN

Stacey is a clinical nutrition specialist at Boston Children’s Hospital in Boston, Massachusetts. She has been providing medical nutrition therapy and nutrition counseling to families of children with spinal muscular atrophy for the past 7 years within Boston Children’s Hospital multidisciplinary SMA clinic. Her other specialty areas include dietary therapy for children with inborn errors of metabolism, including fatty acid oxidation disorders, and the ketogenic diet for children with intractable epilepsy. She is currently collaborating on a research study to better understand the body composition of children with SMA.

Fred W. Troutman, PhD

Fred Troutman, RN, PhD is a retired Air Force Colonel and a nurse educator from Portland, Oregon. The death of his son, Jonathan, brought him to the self-help bereavement group, The Compassionate Friends, and led to his study of how we survive the losses in our lives. Nationally he presents workshops on loss, change and bereavement and is active in international humanitarian public health efforts with children. Fred is a regular presenter at CureSMA conferences. He has one surviving son and three grandchildren.

THE AMY MARQUEZ SCHOLARSHIP

For the 2015 Annual SMA Conference, the Amy Marquez Scholarship is in its third year. The Amy Marquez Scholarship was started in memory of Amy Marquez, who passed away from SMA type I at the age of 41. Amy was an active member of the SMA community and was a wonderful source of support and motivation for many SMA families. This scholarship will be awarded each year to an adult with SMA to attend the Annual SMA Conference, in recognition of Amy's involvement within SMA adult community. The scholarship will cover both registration fees and hotel costs.

Fill out the conference survey for your chance to win an iPad
The Scientific Advisory Board was established in 1986. Through the Board’s invaluable expertise, grant applications to Cure SMA are reviewed and evaluated. The members of the SAB annually review all applications for research grants, using a procedure similar to that used at the NIH.

Cure SMA Scientific Advisory Board (SAB)

Elliot J. Androphy, M.D.
Dr. Elliot J. Androphy is Kampen-Norins Professor and Chair of the Department of Dermatology of Indiana University School of Medicine and was formerly Vice Chair of the Dept. of Medicine at UMass Medical School. His laboratory has studied the genetics of SMA, discovered the role of exon 7 splicing in the SMA back-up gene SMN2, and currently examines axonal transport in motor neurons. Dr. Androphy has been a participant in multiple translational programs including antisense oligonucleotides, tetracyclines, and presently leads a multi-center drug discovery program for SMA that focuses on small molecule SMN enhancers. Dr. Androphy joined the SAB in 2012.

Arthur H. Burghes, Ph.D.
Dr. Burghes is a Professor of Molecular and Cellular Biochemistry at the Ohio State University and an expert in the field of SMA biology. His laboratory focuses on the molecular understanding of genetic neuromuscular disorders, in particular SMA. Dr. Burghes developed the first animal model of SMA and demonstrated that high copy numbers of the SMN2 gene can rescue the SMA mouse. Dr. Burghes joined the SAB in 2002.

Tom Crawford, M.D.
Dr. Crawford is an Associate Professor of Neurology and Pediatrics at the Johns Hopkins School of Medicine. He is co-director of the MDA clinic for Neuromuscular Disorders. His practice involves general child neurology with a principal interest in caring for children with neuromuscular, neuromotor, and ataxic disorders. His primary research interests involve the basic science and clinical characterization of two important neuromuscular disorders that affect children: SMA and Ataxia Telangiectasia. He joined the SAB in 2002.

Douglas Kerr, M.D., Ph.D.
Dr. Kerr is Director, Experimental Neurology at Biogen Idec, where he oversees clinical trials for small molecule drug development in ALS. Dr. Kerr was formerly the Director of the Johns Hopkins Transverse Myelitis Center. He has investigated neural stem cells as a potential tool for functional recovery in patients with motor neuron disease, such as SMA. Kerr joined the SAB in 2005.

Adrian Krainer, Ph.D.
Dr. Krainer is a Professor at the Cold Spring Harbor Laboratory. His research interests include unraveling the mechanisms controlling pre-mRNA splicing, including in genetic diseases such as SMA. He is a leading expert in this area with over 100 research articles published to date. Dr. Krainer joined the SAB in 2005.

Jill Jarecki, Ph.D., Cure SMA Research Director.
Dr. Jarecki serves as the coordinator of the Cure SMA Scientific Advisory Board. Prior to joining Cure SMA, Dr. Jarecki studied neuromuscular development in graduate school at Yale University and was a post-doctoral fellow at Stanford University. Most recently she was a senior scientist at Invitrogen Corporation and also Vertex Pharmaceuticals, where she led a drug discovery program to identify potential small molecule therapeutics for SMA.

Rashmi Kothary, Ph.D.
Dr. Kothary is an Associate Director and Senior Scientist at the Ottawa Hospital Research Institute and a Professor at the University of Ottawa. Dr. Kothary works on understanding the importance of the cytoskeleton in neuromuscular disorders such as SMA. Dr. Kothary has a keen interest in modeling disease pathology in mice and has developed an intermediate mouse model of SMA. He also holds the University Health Research Chair in Neuromuscular Disorders. Dr. Kothary joined the SAB in 2010.

Samuel Pfaff, Ph.D.
Dr. Pfaff is an Investigator of the Howard Hughes Medical Institute and Professor at the Gene Expression Laboratory at the Salk Institute for Biological Studies in La Jolla, California. The Pfaff laboratory focuses on the development of motor neurons. Of special interest to him is how motor neurons develop and make connections between the spinal cord and muscles in the body, and how these connections relate to spinal cord injuries and to degenerative diseases such as ALS (Lou Gehrig’s disease), spinal muscle atrophy and post-polio syndrome. In 2009, his group at the Salk Institute was awarded a $11.5 million grant by the California Institute for Regenerative Medicine (CIRM) for translational research focusing on developing a novel stem-cell based therapy for Amyotrophic Lateral Sclerosis (ALS). Dr. Pfaff joined the SAB in 2011.

Mark Rich, M.D., Ph.D.
Dr. Rich is an Associate Professor in the Department of Neuroscience, Cell Biology, and Physiology at Wright State University. He completed a medical fellowship in neuromuscular disease and spends about 10% of his time with neuromuscular patients in clinic. His research laboratory specializes on synapse physiology at the neuromuscular junction in mice. Dr. Rich joined the SAB in 2010.

Kathryn Swoboda, M.D.
Dr. Swoboda is an Associate Professor of Neurology and Pediatrics at the University of Utah School of Medicine, and Director of the Pediatric Motor Disorders Clinic at Primary Children’s Medical Center in Salt Lake City, Utah. The primary focus of her work is to better understand the pathophysiology contributing to muscle weakness in children with SMA and to help facilitate the rapid translation of new therapies for treatment trials. She joined the SAB in 2002.

See website for full bios.
Cure SMA Scientific Advisory Board (SAB)

The Importance of Independent Scientific Oversight in Selecting and Managing SMA Research Projects

WHO ARE THE CURE SMA ADVISORS?
Cure SMA has three key advisory teams, which include:
- The Scientific Advisory Board (SAB) who oversee basic research funding
- The Translational Advisory Council (TAC) who oversee drug discovery funding
- Medical Advisory Council (MAC) who oversee patient support and clinical care funding

WHAT ARE THE MAIN FUNCTIONS OF OUR ADVISORS IN RESEARCH FUNDING?
- Advise on the best scientific strategy for our funding
- Generate the Request for Proposals (RFPs)
- Review proposals
- Help organize the SMA Research Group Meeting and CME Meeting
- Develop success benchmarks our funded projects
- Serve on management teams for our funded projects

WHAT IS THE CURE SMA FUNDING MODEL?
The Cure SMA research-funding model is firmly based on the philosophy of expert and independent review and oversight of research projects. The Cure SMA strategy consists of having expert advisors review, select, and then oversee the research projects that we fund. This system has many advantages. Primarily, it ensures that Cure SMA funded the most promising research. In addition, funded projects are then run in a professional manner with the guidance of world-class experts. This type of system is the gold standard for effective scientific funding worldwide and is used extensively by both governments and non-profit groups. It is often referred to as “peer review”, which means scientists working in similar areas judge each other’s work.

WHAT SPECIFIC ADVANTAGES DOES THE PEER REVIEW SYSTEM OF EVALUATION GIVE?
People with similar training are in the best position to judge research.
One of the most obvious is that the members of our advisory boards are leading experts in SMA, who have many years of scientific training. Scientific feasibility of projects is often based on very detailed technical issues, which are always not obvious to the untrained eye. Research is so highly specialized that peers with similar expertise are in the best position to criticize and understand each other’s projects. Even on our SAB, Cure SMA has carefully chosen advisors with distinct sub-specialties (mouse work, motor neuron biology, etc.) to assess each type of specific project.

Our advisors rank and prioritize many different projects against each other.
One of the main roles of our advisors is to rank many different projects. This is particularly important because in a typical year Cure SMA assesses about 50 different research projects for SMA for funding each year. Here, small differences in scientific approach can be crucial in a project’s success or failure. Peer review allows Cure SMA to rank projects against each other more effectively with the end result being only the best projects are chosen.

Our advisors help manage funded projects.
Our scientific advisors also oversee the progress of funded projects. This aspect is the most key in the drug discovery/development area. Typically these projects are assessed by a series of pre-determined benchmarks. Our scientific advisors help devise the benchmarks for projects and also decide when benchmarks are met. Second, our advisors are a key resource for project teams, by providing technical expertise, tools and reagents. Finally, one of the hardest but most essential roles of our advisors is to end projects that have reached insurmountable hurdles.

HOW DOES THE CURE SMA FUNDING MODEL OF EXPERT OVERSIGHT IMPACT YOUR GIVING?
Importantly, our scientific funding model of project selection and oversight by independent experts does not prevent our donors from having a voice in what Cure SMA funds. Earmarked donations to particular projects are possible at Cure SMA and also still allow for project assessment by independent experts in the field. Direct donations to researchers themselves do not provide this security. Non-restricted donations to Cure SMA are important too and allow our advisors to prioritize research funding freely.
Cure SMA Translational Advisory Council (TAC)

OFFICIAL MEMBERS

Brian Pollok, Ph.D., Principal, Rapidan BioAdvisors, Assay Development

Lee Rubin, Ph.D., Director of Translational Medicine, Harvard Stem Cell Institute, Neuronal Assay Development and High-Throughput Screening

Christine Brideau Ph.D., Executive Director, In Vitro Pharmacology, Merck & Co., Preclinical Drug Development

Jim Inglese, Ph.D., Head, Laboratory of Assay Development & Screening Technology (ADST), National Center for Advancing Translation Sciences, Assay Development and High-Throughput Screening

Peter Hodder, Ph.D., Senior Director, Lead Identification, The Scripps Research Institute, Scripps Florida, Assay Development and High-Throughput Screening

Michael Vellard, Ph.D., Principal Scientist, BioMarin, Preclinical Drug Development

Joseph W. Lewcock, Ph.D., Group Leader, Department of Neurobiology, Genentech, Inc., Preclinical Drug Development

Arthur Burghes, Ph.D., Professor, The Ohio State University, SMA Biology

Charlotte Sumner, M.D., Associate Professor, Johns Hopkins University, SMA Biology/Neurology

Peter Grootenhuis, Ph.D., Senior Director, Vertex Pharmaceuticals, Medicinal Chemistry

Timothy Reilly, Ph.D., DABT, Director, Bristol Myers Squibb, Toxicology

John Porter, Ph.D., Program Director, NINDS, Neuromuscular Disease

Jasbir Singh, Ph.D., President, Jasin Discovery Solutions, Medical Chemist

See website for full bios.

Thank you to our Medical Advisory Council, Scientific Advisory Board and the Translational Advisory Council for their expertise, advice and dedication to our cause.
Cure SMA Staff

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Heidi Roy, officer (New England Territory)  Amber Snyder, manager (Central Territory)

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Amy Kephart, specialist (Patient Care)

FINANCE

Sue Kovach, finance and administration director  Sherryl Dunning, accounting manager

OPERATIONS

Megan Lenz, communications manager  Judi Smith, IT manager
Cure SMA Merchandise

Stop by the registration area and pick up your Cure SMA merchandise. Special pricing just for the Annual SMA Conference!

Charcoal Cure SMA T-shirt $15
Purple Cure SMA T-shirt $15
White Cure SMA T-shirt $15
Cure SMA Towels $15
Cure SMA Bracelet $2
Cure SMA Magnets $2
Cure SMA Tote $12
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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Featured Attractions

LEGOLAND® Discovery Center
For kids, ages 3-10, it’s like jumping into the biggest LEGO® box, ever! With more than 2 million LEGO bricks, an interactive laser ride and iconic Kansas City LEGO landmarks. LEGOLAND is the ultimate family entertainment. For information, call 816-471-4386, or visit legolandincoverycenter.com/kansas-city.

SEA LIFE Kansas City Aquarium
Already a Kansas City favorite, SEA LIFE is swimming with exotic marine life. See fish of all sizes including sharks, rays and a sea turtle. For information, call 816-471-4386, or visit visitsealife.com/kansas-city.

Hallmark Visitors Center
Explore more than 100 years of fascinating Hallmark history. See how artisans craft cards and products, and browse original Keepsakes. Open Monday – Saturday. Free admission. Call 816-274-3613 or visit hallmarkvisitorscenter.com.

Crown Center Ice Terrace
Skate on Kansas City’s only public, outdoor rink – Open daily, November through mid-March. Group rates and season passes available. For information and hours, call 816-274-8411.

Crown Center Showplace
Free interactive exhibits year-round. Plus, the Showplace is home to Santa during the Christmas season. For exhibit information, call 816-274-8444, or visit crowncenter.com.

Kaleidoscope
Kaleidoscope is a free, hands-on art studio, where children and their families make their own wonderful creations. Sponsored by Hallmark. For session times, call 816-274-8300, or visit hallmarkkaleidoscope.com.

Musical Theater Heritage at Crown Center
Kansas City’s own professional theater company specializes in Musical Theater productions. MTH, with its large casts and live music, has become a local theater favorite for its intimate and engaging concert-style productions of book musicals, and cabaret shows. For ticket information, call 816-221-6987, or visit mtkc.com.

The Coterie
Recognized nationally, The Coterie is a professional not-for-profit theatre that offers families and young adults unique theatre experiences that last a lifetime. For ticket information, call 816-474-6552, or visit thecoterie.org.
Shopping Directory
The Westin Kansas City at Crown Center
Crown Center, 1 E Pershing Rd,
Kansas City, MO 64108
(816) 474-4400

For any questions please see us at the Registration Area.