



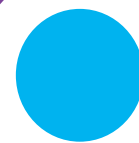
Make today a  
breakthrough.



# 2018 ANNUAL SMA CONFERENCE

JUNE 14<sup>TH</sup>-17<sup>TH</sup>, 2018

HILTON ANATOLE HOTEL IN DALLAS, TEXAS



## Dear Families,

SMA is changing, and our research, care and support programs are expanding, but our commitment and values as a community will remain the same as they have always been.

We are here to lead the way to a world without spinal muscular atrophy. We fund and direct comprehensive research that drives breakthroughs in treatment and care, and we provide families the support they need for today. The FDA approval of the first ever treatment for SMA in December 2016 was a major step toward that goal. As a community, we've celebrated that long-awaited breakthrough. But we recognize that there remains more to be done to achieve our mission.

There is a need for continued research investment. It will likely take a combination of therapies to achieve the greatest possible effect for each and every SMA patient at every age and stage of the disease. And, as SMA changes with approved treatments, care will become even more important for our community. We have implemented our SMA Care Center Network and will continue to add Care Centers across the US to improve care and outcomes. We are expanding our reach and providing new customized local support programs.

We're expecting 1,600 attendees at this year's conference, including over 400 researchers and 110 clinicians from 160 organizations. There will be 56 family workshops, 130 research presentations and 55 care presentations given during the meeting.

This event is generously supported by 19 sponsors and 25 exhibitors. Our special thanks to Biogen as the Presenting Sponsor of the 2018 Annual SMA Conference. Through these incredible sponsors, we are able to provide assistance for travel or registration to 729 attendees, in addition to subsidizing food and other costs for all!

During the scientific sessions, researchers from industry and academia will meet to create open communication, accelerating the pace of research. During the care meeting, multidisciplinary clinicians will share knowledge to optimize care. On the family side, we have workshop tracks that serve all of the different groups in our community. We have many scheduled, and informal and fun, events and opportunities for us all to connect and network.

Please reach out to any of us if there is anything we can do to make this conference as meaningful for you as possible.

All the best,



**Kenneth Hobby**  
President



**Colleen McCarthy O'Toole**  
Vice President, Events  
& Family Support Dept.



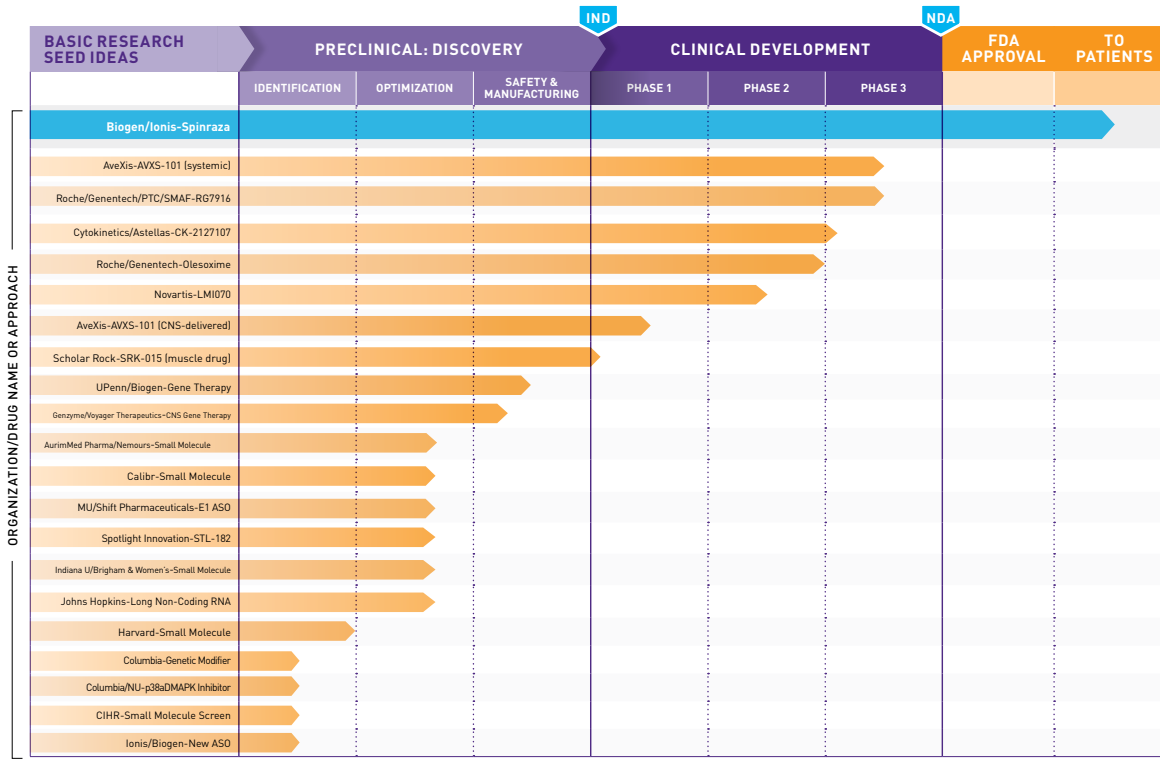
**Jill Jarecki, PhD**  
Chief Scientific Officer



**Mary Schroth MD**  
Chief Medical Officer

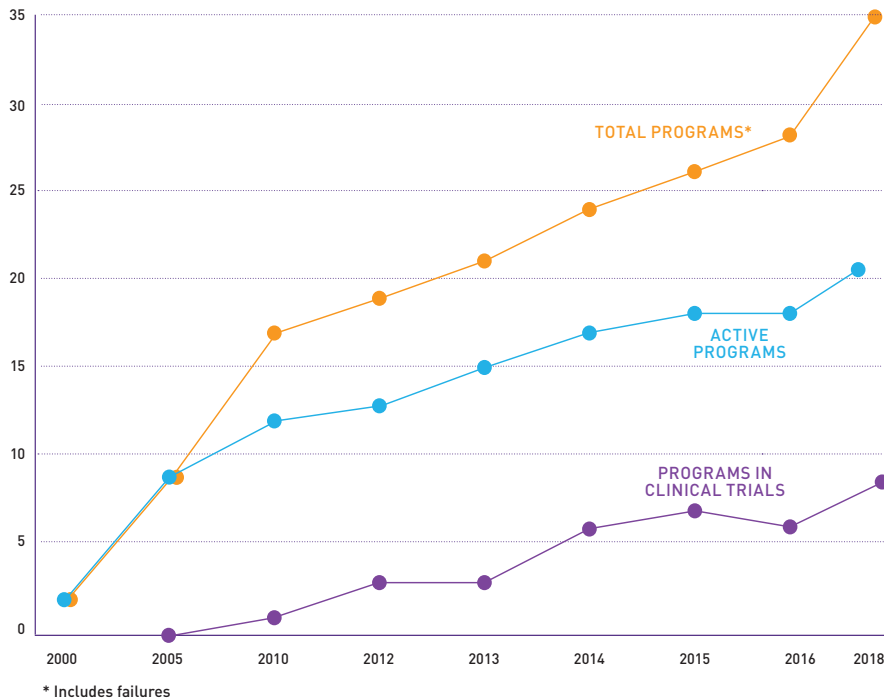


# SMA DRUG PIPELINE



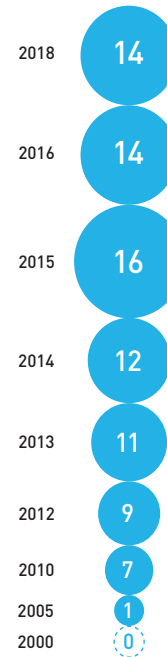
IND = Investigational New Drug    NDA = New Drug Application    Last updated: May 2018

## HOW THE PIPELINE HAS GROWN



\* Includes failures

## NUMBER OF COMPANIES INVESTING IN SMA DRUG PROGRAMS



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## VISION AND MISSION STATEMENT

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



Cure SMA is dedicated to the treatment and cure of spinal muscular atrophy (SMA)—a disease that takes away a person's ability to walk, eat, or breathe. It is the number one genetic cause of death for infants.

Since 1984, we've directed and invested in comprehensive research that has shaped the scientific community's understanding of SMA. We are currently on the verge of breakthroughs that will strengthen bodies, extend life, and lead to a cure. We have deep expertise in every aspect of SMA—from the day-to-day realities to the nuances of care options and until we have a cure, we'll do everything we can to support and advocate for those affected by the disease.

Learn more about how you can help us reach a treatment and cure at

[www.cureSMA.org](http://www.cureSMA.org)

## 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.



# SATURDAY IS CONFERENCE T-SHIRT DAY!

Please wear your conference t-shirts, which you received at registration, to show support and awareness of our SMA community!



Our thanks to Biogen for their generosity as the Presenting Sponsor of the 2018 Annual SMA Conference.

## 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.



# WORKSHOP SESSIONS

## Workshop Session #1

- Sharing your Type I Experience
- Sharing your Type II Experience
- Sharing your Type III Experience
- Adults with SMA Talk it Out
- Grandparents Talk it Out Type I
- Grandparents Talk it out Type II & Type III

## Workshop Session #2

- Ethics and Realities in SMA Research and Emerging Therapies
- Breathing Strong All Day Long: BiPAPs and Ventilators
- Medical Management of Adults with SMA
- Yoga Therapy for SMA
- What it Means to be a Teen on Wheels
- Getting the Most Out of What You Eat! Nutrition for Oral Feeders
- Healing the Grieving Heart – Part I

## Workshop Session #3

- Making Choices to Optimize Care and Quality of Life
- Aquatic Physical Therapy for Fun & Function, Teens and Adults
- Genetics and Reproductive Options for SMA Families
- The Role of Therapy (PT and OT) in your Child's Life – Being Part of the Team!
- Keepsake Creation: Grieving Through Art
- Driving and Community Mobility
- Dad's Time: A Workshop for Fathers Only – Type II & III
- Dad's Time: A Workshop for Fathers Only – Type I

## Workshop Session #4

- Aquatic Physical Therapy Question & Answer Session
- Orthopedic Management
- Tube Feeding and SMA: Recommendations and Practices
- Laughter Yoga for Caregivers and Grieving Family Members
- "I Need to Cough": Ways to Keep Your Lungs Clear
- Care for the Caregiver
- Personal Care Assistants—How to Find, Hire and Manage your PCAs

## Workshop Session #5

- Aquatic Physical Therapy for Fun & Function, Children with Supported Head
- An Approach to Exercise in Individuals with SMA
- Navigating the Special Education Process: Setting Our Kids up for Success
- Yoga Therapy for Teens and Adults with SMA
- Independent Living with SMA Panel – Tips & Tricks for Living with SMA
- Family Readiness for Emergencies
- Care for the Caregiver

## Workshop Session #6

- Aquatic Physical Therapy for Fun & Function, Children with Independent Head Control
- Special Plans for a Special Life
- Healing the Grieving Heart – Now What? Part II
- Top Tips on Traveling Around the World
- Finding a Job, Interviewing and How to Ask for Modifications Panel
- Fundraising 101
- Oral-Motor Exercises for Speech Clarity and Feeding

# Thursday June 14<sup>th</sup>, 2018

## Newly diagnosed families only

<b>1:00pm – 3:15pm</b>	Newly Diagnosed Program ( <i>For Newly Diagnosed Families Only</i> )
	Newly Diagnosed Meet & Mingle

## All conference attendees

<b>7:30am – 5:30pm</b>	Registration Open for all Conference Attendees
<b>6:00pm – 8:30pm</b>	Meet and Greet/Family Fun Fest for all Conference Attendees
<b>7:30pm – 10:00pm</b>	Teen Social   Sponsored by AveXis and Jacob Isaac Rappoport Foundation
<b>7:30pm – 10:00pm</b>	Adults with SMA Reception   Sponsored by Dhont Family Foundation

# Friday June 15<sup>th</sup>, 2018

<b>7:30am – 5:30pm</b>	Registration Open
<b>7:30am – 5:30pm</b>	Exhibitor Tables Open
<b>7:30am – 8:45am</b>	Family Attendee Breakfast Items
<b>9:00am – 10:15am</b>	General Session
<b>10:30am – 12:30pm</b>	Workshop Session #1
<b>12:30pm – 2:00pm</b>	Family Lunch with Symposium   Sponsored by Biogen
<b>2:00pm – 3:30pm</b>	Workshop Session #2
<b>3:45pm – 5:15pm</b>	Workshop Session #3
<b>6:30pm – 8:00pm</b>	Family Friendly Research Poster Session
<b>7:30pm – 9:00pm</b>	Family and Researcher Dance Party

# Saturday June 16<sup>th</sup>, 2018

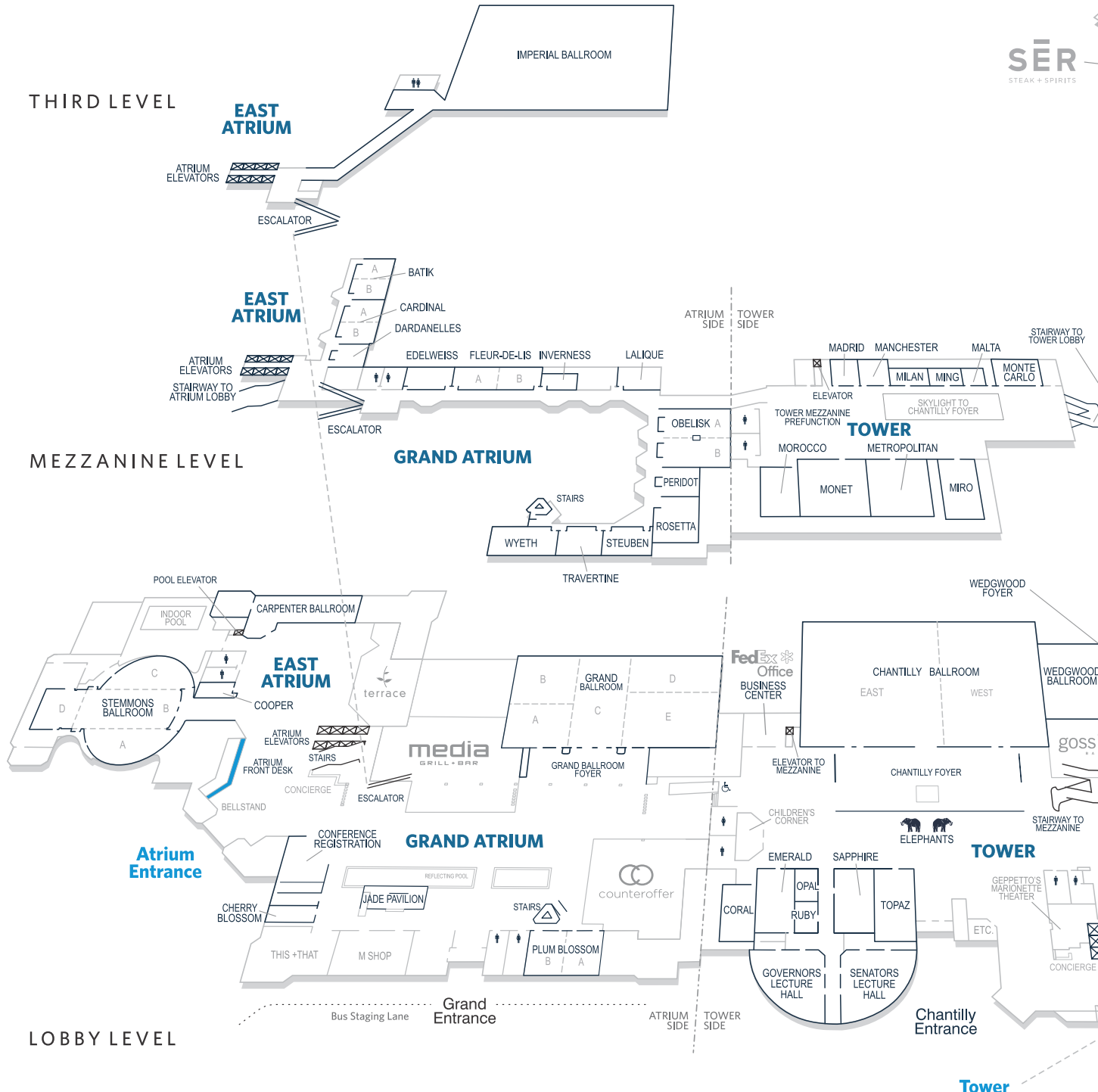
<b>7:30am – 5:30pm</b>	Registration Open
<b>7:30am – 5:30pm</b>	Exhibitor Tables Open
<b>7:15am – 8:30am</b>	Family Attendee Breakfast Items
<b>8:45am – 10:15am</b>	Workshop Session #4
<b>10:30am – 12:00pm</b>	Workshop Session #5
<b>12:00pm – 1:30pm</b>	Family Lunch with Symposium   Sponsored by AveXis
<b>1:30pm – 3:00pm</b>	Workshop Session #6
<b>3:15pm – 5:15pm</b>	Researcher Q & A Session
<b>6:30pm – 8:30pm</b>	PJ Party & Movie Night
<b>7:30pm – 10:00pm</b>	Roll-a-Palooza, Live Music Event   Sponsored by Adyn's Dream

# Sunday June 17<sup>th</sup>, 2018

<b>7:30am – 8:45am</b>	Family Breakfast with Symposium   Sponsored by Genentech/Roche
<b>9:00am – 11:15am</b>	Closing General Session – It's a Wonderful Life Panel

**Note:** Children's Program rooms open during meeting times only.










# Wed.

June 13<sup>th</sup>, 2018

5:00pm – 7:30pm	<b>Registration Open for all Conference Attendees</b>	Registration
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# Thurs.

June 14<sup>th</sup>, 2018



7:30am – 5:30pm	<b>Registration Open for all Conference Attendees</b>	Registration
1:00pm – 3:15pm	<b>Newly Diagnosed Children’s Program</b> Sponsored by JIRF 	Stemmons Ballroom
1:00pm – 3:00pm <i>Drop off opens at 12:30pm</i>	<b>Newly Diagnosed Program</b> (For Newly Diagnosed Families Only)	Obelisk A & Obelisk B
1:00pm – 2:30pm	<b>Introduction to Cure SMA and the Community</b>  Kenneth Hobby, President Colleen McCarthy O’Toole, Vice President, Events & Family Support Mary Schroth, MD, Chief Medical Officer Jessica Clark, Senior Programs Manager, Events & Family Support  <b>Parent Welcome</b>  Danyelle Sun Al Freedman  <b>Understanding the Genetics and Disease</b>  Tom Crawford, MD  <b>Understanding Treatment and Trials</b>  Rob Graham, MD  <b>Life After Diagnosis – Parents Share Their Journey</b>  David Sereni, Grieving Danyelle Sun, SMA Type II & Type III Kevin O’Brien, SMA Type III Al Freedman, SMA Type I	Obelisk A
2:30pm – 3:15pm	<b>Newly Diagnosed Meet &amp; Mingle Reception</b>	Obelisk B




6:00pm – 8:30pm	<b>Meet &amp; Greet</b> Includes Researcher Relay Race and Games	Grand Ballroom
7:30pm – 10:00pm	<b>Teen Social</b> Sponsored by AveXis and JIRF 	Carpenter Ballroom
7:30pm – 10:00pm	<b>Adults with SMA Reception</b> Sponsored by Dhont Family Foundation	Plum Blossom

**Fri.**

June 15<sup>th</sup>, 2018

7:30am – 8:45am	<b>Family Attendee Breakfast Items</b>	Grand Ballroom C
7:30am – 5:30pm	<b>Registration and Check-in</b>	Registration
7:30am – 5:30pm	<b>Exhibitor Tables Open</b>	East Atrium
9:00am – 12:30pm <i>Drop off opens at 8:30am</i> & 2:00pm – 5:15pm <i>Drop off opens at 1:30pm</i>	<b>Children’s Program</b>   Sponsored by JIRF 	Stemmons Ballroom B, C, D
9:00am – 12:30pm <i>Drop off opens at 8:30am</i> & 2:00pm – 5:15pm <i>Drop off opens at 1:30pm</i>	<b>Baby Room</b>   Sponsored by JIRF 	Stemmons Ballroom A
9:00am – 10:15am	<b>General Session Opening Remarks</b>	Grand Ballroom D & E
9:00am – 10:15am	<b>Kids Talk it Out (Ages 6 – 9)</b> Al Freedman, PhD, SMA Dad and Child Psychologist Angela Wrigglesworth, Elementary Education Teacher, SMA Adult  This facilitated session will provide SMA-affected children (ages 6 - 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.) Pre-registration is required and will be on a first come, first serve basis.	Plum Blossom



<p>9:00am – 10:15am</p>	<p><b>Sibling Talk it Out (Ages 12 to 17)</b>                  Katlyn O'Brien, Sister of a SMA Young Adult                  Traci O'Brien, Sister of SMA Young Adult                  Kelli Blume, Cousin of SMA Young Adult</p> <p>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 SMA family siblings and other family members. 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!</p>	<p>Fleur-de-lis</p>
<p>10:30am – 12:30pm</p>	<p><b>Workshop Session #1</b></p>	
	<p><b>Sharing your Type I Experience</b>  <b>Sharing your Type II Experience</b>  <b>Sharing your Type III Experience</b>  <b>Adults with SMA Talk it Out</b>  <b>Grandparents Talk it Out Type I</b>  <b>Grandparents Talk it out Type II &amp; Type III</b></p>	<p>Carpenter Ballroom                  Grand Ballroom A &amp; B                  Obelisk                  Plum Blossom                  Batik                  Cardinal</p>
<p>12:30pm – 2:00pm</p>	<p><b>Family Lunch with Symposium: Biogen Lunch and Learn</b>   Sponsored by Biogen </p>	<p>Grand Ballroom C, D, E</p>
<p>2:00pm – 3:30pm</p>	<p><b>Memory Boxes for Siblings: Creating a Lasting Treasure (for children only)</b>                  Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>Mourning the loss of a brother or sister 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 siblings an opportunity to make a memory box that will support the memories of their sibling and also learn about other creative legacy ideas to do on their own.</p>	<p>Edelweiss</p>



2:00pm – 3:30pm	<p><b>Workshop Session # 2</b></p>	
	<p><b>Ethics and Realities in SMA Research and Emerging Therapies</b>            Rob Graham, MD            Vanessa Battista, RN, MS, CPNP            Tom Murray, PhD</p> <p>A "cure" for everyone? 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, access, and distributive justice for newly approved therapies.</p>	Obelisk
	<p><b>Breathing Strong All Day Long: BiPAPs and Ventilators</b>            Oren Kupfer, MD            Peter Schochet, MD</p> <p>Individuals with SMA are at risk for difficulty breathing due to muscle weakness. This workshop will discuss why the muscle weakness causes difficulty breathing during sleep, illness and after surgery, and how to know when breathing support machines such as BIPAP should be considered. We will talk about the modalities available to use at home and in the hospital, as well as strategies to help your care team support respiratory health at home. There will be opportunities after the presentation to ask general questions about breathing complications and to share experiences that have improved the health of those with SMA. We hope to have the whole pulmonary team there for this interactive session.</p>	Carpenter Ballroom
	<p><b>Medical Management of Adults with SMA</b>            Tina Duong, MPT, PhDc            Bakri ElSheikh, MBBS, FRCP</p> <p>This workshop will focus on three key areas: 1) Standards of Care related to the adult medical and rehabilitation management of SMA in the current natural history landscape. 2) We will discuss challenges related to obtaining Spinraza treatment, present experience, and expectations. 3) We will also discuss the latest research associated with other combination therapies that may be effective in adults with SMA. Specific attention will be aimed toward the importance of integrating, coordinating, and directing one's own care. The session is ideal for families and participants who are transitioning to adult clinics or adults with SMA.</p>	Plum Blossom B



	<p><b>Yoga Therapy for SMA</b> Anne Buckley-Reen, OTR/RYT</p> <p>The workshop will introduce you to the benefits of yoga for a SMA child. Anne will discuss the physical, emotional, respiratory, cognitive and immunological benefits of yoga. The workshop will include sharing a yoga experience with a SMA family. The workshop will conclude with a review of a typical yoga routine which includes circle of song, eye exercises, postures, progressive relation and sound breath.</p>	<p>Grand Ballroom A &amp; B</p>
	<p><b>What it Means to be a Teen on Wheels</b> Angela Wrigglesworth, Elementary Education Teacher, SMA Adult</p> <p>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.</p>	<p>Plum Blossom A</p>
	<p><b>Getting the Most Out of What You Eat! Nutrition for Oral Feeders</b> Rebecca Hurst Davis, MS, RD, CSP, CD, CNSC Stacey Tarrant, BS, RD, LDN Laura Watne, MS, RD, CSP</p> <p>This workshop is designed to address the nutritional needs of people with SMA who eat by mouth. The first half 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 nutrient deficiencies and diet modifications for people with SMA. You will also learn the signs/symptoms of feeding issues/intolerances and when it might be necessary to supplement intake. We will discuss potential changes in nutrition recommendations related to Spinraza use. The rest of the work shop will break out into round table discussion groups led by a registered dietitian for 1) Nutrition challenges specific to: 1) Adults with SMA and 2) Children with SMA.</p>	<p>Batik</p>
	<p><b>Healing the Grieving Heart – Part I</b> Fred Troutman, RN, PhD David Sereni, DPT</p> <p>The healing process, Parts 1: 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.</p>	<p>Dardanelles</p>



3:45pm – 5:15pm	<b>Workshop Session # 3</b>	
	<p><b>Making Choices to Optimize Care and Quality of Life</b></p> <p>Vanessa Battista, RN, MS, CPNP Carolyn Long, MSW</p> <p>Individuals and families of children living with SMA are required to make many decisions regarding care. Improvements in healthcare, as well as advancements in technology, are providing individuals with SMA better quality of life. For some, determining what interventions make sense can be challenging, as some decisions may lead to unexpected outcomes. In this workshop, we will consider options presented to families and potential consequences that may affect quality of life. We will also identify signs of pain, and explore various modalities to treat pain in individuals living with SMA..</p>	Lalique
	<p><b>Aquatic Physical Therapy for Fun &amp; Function, Teens and Adults</b></p> <p>Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS</p> <p>Bring your swim suits and head to the pool for an active 90 minutes with a team of aquatic physical therapists. Our focus will be on improving functional strength and skills in a dynamic environment. Games, stretching, toys and balance will all be discussed and demonstrated during our session. We look forward to seeing you in the water.</p>	Indoor Pool
	<p><b>Genetics and Reproductive Options for SMA Families</b></p> <p>Harvey J. Stern, MD, PhD, FACMG, FAAP Louise R. Simard, PhD Melissa Gibbons, MS, CGC</p> <p>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 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 5q-SMA will be discussed including Preimplantation Genetic Diagnosis (PGD) with in vitro fertilization (IVF). A short update on research for non-5q-SMAs will also be presented.</p>	Batik



	<p><b>The Role of Therapy (PT and OT) in your Child's Life – Being Part of the Team!</b>                  Karen Patterson, PT, PCS, MS                  Anne Thompson, OT</p> <p>The goal of this session is to explore the general physical, occupational and speech therapy needs of children with SMA geared toward those families who may be newer to navigating this world. We will discuss how to create your team and different therapy options. 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 and how to work with your team to develop functional goals. We will also discuss some basic body mechanics to help the caregiver. A large amount of time will be devoted to encouraging an open discussion with all participants with opportunities to share experiences and frustrations and to answer questions you might have.</p>	<p>Cardinal</p>
	<p><b>Keepsake Creation: Grieving Through Art</b>                  Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>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 parents 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.</p>	<p>Edelweiss</p>
	<p><b>Driving and Community Mobility</b>                  Anne Hegberg, MS, OTR/L</p> <p>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.</p>	<p>Plum Blossom B</p>







	<p><b>Dad’s Time: A Workshop for Fathers Only – Type II &amp; III</b></p> <p>Kevin O’Brien, Father of a SMA type III daughter Ryan Wolff, Father of a SMA type II daughter</p> <p>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.</p>	Carpenter Ballroom
	<p><b>Dad’s Time: A Workshop for Fathers Only – Type I</b></p> <p>David Sereni, Father of SMA type I son Al Freedman, Father of SMA type I son</p> <p>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.</p>	Fleur-de-lis
6:30pm – 8:00pm	<b>Family Friendly Researcher Poster Session</b>	Grand Ballroom A, B, C
7:30pm – 9:00pm	<b>Family and Researcher Dance Party</b>	Grand Ballroom D & E



**PLEASE NOTE  
WEAR SUNSCREEN AND KEEP HYDRATED!**

As many of you know, June in Texas is a very hot time. We strongly encourage everyone to take the proper precautions when outside for any amount of time, by applying sunscreen and covering your body as much as possible to avoid excessive sun exposure. Also, with the high temperatures and humidity, drink plenty of water to remain hydrated in this heat!

7:15am – 8:30am	<b>Family Attendee Breakfast Items</b>	Grand Ballroom A, B, C
7:30am – 5:30pm	<b>Registration and Check-in</b>	Registration
7:30am – 5:30pm	<b>Exhibitor Tables Open</b>	East Atrium
8:45am – 12:00pm <i>Drop off opens at 8:15am</i> & 1:30pm – 5:15pm <i>Drop off opens at 1:00pm</i>	<b>Children’s Program</b>   Sponsored by JIRF 	Stemmons Ballroom B, C, D
8:45am – 12:00pm <i>Drop off opens at 8:15am</i> & 1:30pm – 5:15pm <i>Drop off opens at 1:00pm</i>	<b>Baby Room</b>   Sponsored by JIRF 	Stemmons Ballroom A
8:45am – 10:15am	<b>Workshop Session #4</b>	
	<b>Aquatic Physical Therapy Question &amp; Answer Session</b>  Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS  The pool offers great benefits for individuals of all ages with SMA. Come spend some time out of the water to hear the whys and hows of aquatic exercise for you or your family member. We will discuss reasons to and not to use the water, what to look for in aquatic facilities, safety, equipment and exercises. This is a great time to come and see the equipment, mostly dry, and in one place to get the creative juices flowing for how some items might be adapted to be just perfect for your needs. Plenty of time will be available for attendees to ask questions of our three physical therapists in attendance.	Cardinal
	<b>Orthopedic Management</b>  Brian Snyder, MD, PhD Samuel Rosenfeld, MD  This workshop will focus on life with SMA as it pertains to maintaining the best possible orthopedic management. It will also address the orthopedic complications of SMA. Children with SMA often have musculoskeletal impairments that interfere with mobility, function, and efficiency, and can contribute to restrictive pulmonary disease. Orthopedic intervention can improve or stabilize these impairments and help prevent deterioration in function. Orthopedic surgical procedures can correct hip instability and scoliosis, facilitate orthotic management, and accommodate the demands of continued growth. Musculoskeletal integrative medicine is important in normal development, especially in the child with SMA.	Fleur-de-lis



	<p><b>Tube Feeding and SMA: Recommendations and Practices</b></p> <p>Rebecca Hurst Davis, MS, RD, CSP, CD, CNSC          Stacey Tarrant, BS, RD, LDN          Laura Watne, MS, RD, CSP</p> <p>This workshop will discuss the nutritional challenges and unique nutritional needs of people with SMA who are tube fed. In the first half of this session, you will learn about common nutrition issues, growth expectations and goals, and recommendations for calories, protein, fat, vitamins, minerals and fluid. We will discuss specialized diets including blenderized and commercially available real food formulas and supplements. Our discussion will also include information about how Spinraza use may change nutritional needs. The second half of the workshop will break out into round table discussion groups each led by a registered dietitian.</p>	<p>Lalique</p>
	<p><b>Laughter Yoga for Caregivers and Grieving Family Members</b></p> <p>Linda Shively, SMA Parent</p> <p>When we have significant stress in our life, especially after receiving a diagnosis, being a caregiver, or experiencing a death, it may be difficult to find reasons to laugh, we may not give ourselves permission to laugh, or we may think we're not supposed to laugh. Laughter, like crying, can be a very healing emotional release.</p> <p>This interactive workshop is a safe place to laugh and will demonstrate that you do not need humor or comedy to laugh. Thankfully, the body receives the same physical and emotional benefits from simulated and real laughter. Together, we will use laughter as exercise combined with yoga breathing. Playful movements along with eye contact may turn simulated laughter into real laughter.</p> <p><b>Benefits you will receive include:</b></p> <ul style="list-style-type: none"> <li>• Increase blood flow and oxygen in your body</li> <li>• Enhance uplifting endorphin levels</li> <li>• Reduce pain</li> <li>• Stimulate the immune, digestive, and cardiac systems</li> <li>• Stretch and help relax tight muscles</li> <li>• Help reduce stress levels</li> </ul> <p>Come ready to move, laugh, and have fun (all exercises can be adapted to various physical abilities). No prior yoga experience required. Wear regular clothes as long as you can move and breathe easily. We will end with a meditation to center ourselves after the laughter. Participants say that afterwards they feel better both physically and emotionally. Come laugh with us!</p>	<p>Plum Blossom</p>




	<p><b>“I Need to Cough”: Ways to Keep Your Lungs Clear</b></p> <p>Richard Kravitz, MD Jane Taylor, MD, MSCR</p> <p>In this workshop, we will discuss how cough works and why it is important, why cough strength is diminished in SMA, how we can measure cough strength, and how we can help cough function and limit respiratory infections and hospitalizations. We plan to discuss the medical literature, share our experiences, and hear from the audience during a question and answer session at the end of the presentation. Bring your questions and experience to this interactive session we hope to have our whole pulmonary team there!</p>	<p>Batik</p>
	<p><b>Care for the Caregiver</b></p> <p>Carolyn Long, MSW Vanessa Battista, RN, MS, CPNP Jennifer Lemisch, MA, ATR-BC, LPC Diane Murrell, LCGC</p> <p>Caring for a loved one with SMA can be emotionally and physically draining, and finding time for self-care is challenging. Come learn various techniques for taking care of yourself in this workshop, which will be available for two sessions. Each method has its own workstation, so you can come and go as you please. At the time of print, we have art therapy, aromatherapy, yoga, nutrition for wellness, and centering beads, though more stations may be added. We hope you find a method that works for you.</p>	<p>Obelisk</p>
	<p><b>Personal Care Assistants—How to Find, Hire and Manage your PCAs</b></p> <p>Paula Barrett, SMA Parent Carolyn Barrett, SMA Adult</p> <p>This workshop is designed to give you the tools you that will need in order to hire your own personal care assistants. What to look to 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.</p> <p>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.</p>	<p>Carpenter Ballroom</p>



<p>10:30am – 12:00pm</p>	<p><b>Kids Talk it Out (Ages 10 &amp; Up)</b>          Angela Wrigglesworth, Elementary Education Teacher, SMA Adult          Al Freedman, PhD, SMA Dad and Child Psychologist</p> <p>This facilitated session will provide SMA-affected children (Ages 10 &amp; Up) 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.) Pre-registration is required and will be on a first come, first serve basis.</p>	<p>Plum Blossom</p>
<p>10:30am – 12:00pm</p>	<p><b>Workshop Session # 5</b></p>	
	<p><b>Aquatic Physical Therapy for Fun &amp; Function, Children with Supported Head Control</b>          Jennifer Martyn, PT          Krista Torseth, PT, DPT          Kendra Paker, PT, DPT, PCS</p> <p>Bring your swim suits and head to the pool for an active 90 minutes with a team of aquatic physical therapists. Our focus will be on improving functional strength and skills in a dynamic environment. Games, stretching, toys and balance will all be discussed and demonstrated during our session. We look forward to seeing you in the water.</p>	<p>Indoor Pool</p>
	<p><b>An Approach to Exercise in Individuals with SMA</b>          Tina Duong, MPT, PhDc          Leslie Nelson, MPT, PhDc, OCS          Anne Stratton, MD</p> <p>The goal of this session is to provide education on exercise guidelines and options to support an active lifestyle in participants of all ages and types of SMA. Information will be based on current exercise literature in SMA and expert clinical opinion. This session will help you better understand the basic physiology of exercise and how it may impact you. To ensure the session encompasses a wide range of physical abilities, we will also provide alternatives and modifications for all levels of fitness as well as discuss relevant medical considerations and precautions prior to initiating an exercise program. We will help provide options and resources for various forms of group and individual exercise. The focus of this session will center around personalized exercise to meet your personal activity goals. This session is ideal for families and participants interested in learning more about initiating and progressing exercise activity throughout the lifespan.</p>	<p>Cardinal</p>




	<p><b>Navigating the Special Education Process: Setting Our Kids up for Success</b></p> <p>Kimberly Cook, SMA Parent Rachel Sepe, SMA Parent Tina Lewis, SMA Parent</p> <p>Special education can be intimidating for parents entering a whole new world of acronyms, timelines, and IEP goals. Knowing what you can expect for your child and asking for appropriate services and accommodations can be difficult. Join our session to learn the ins and outs of special education. Hear the stories of three students and the accommodations and services that benefited them. Question and answer session to follow.</p>	<p>Carpenter Ballroom</p>
	<p><b>Yoga Therapy for Teens and Adults with SMA</b></p> <p>Anne Buckley-Reen, OTR/RYT</p> <p>The workshop will introduce you to the benefits of yoga for adults and teens with SMA. Anne will discuss the physical, emotional, respiratory, cognitive and immunological benefits of yoga. The workshop will include sharing a yoga experience with a SMA family. The workshop will conclude with a review of a typical yoga routine which includes circle of song, eye exercises, postures, progressive relaxation and sound breath, stress reduction, enhance energy and endurance, maintain joint mobility, and more!</p>	<p>Batik</p>
	<p><b>Independent Living with SMA Panel – Tips &amp; Tricks for Living with SMA</b></p> <p>Melissa Milinovich, SMA Adult Stevie Hopkins, SMA Adult</p> <p>Adulting is a challenge some days; adulting with SMA is an even bigger challenge on most days! Come meet a group of adults who are successfully navigating life with SMA while living independently, having careers, families, and more. These adults have offered to share their stories, trials and triumphs, as well as try to answer the burning questions of how to live the life of an independent adult while having SMA.</p>	<p>Lalique</p>



**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.

	<p><b>Family Readiness for Emergencies</b></p> <p>Rob Graham, MD Jennifer Miller-Smith, SMA Mom</p> <p>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.</p>	Fleur-de-lis
	<p><b>Care for the Caregiver</b></p> <p>Carolyn Long, MSW Vanessa Battista, RN, MS, CPNP Jennifer Lemisch, MA, ATR-BC, LPC Diane Murrell, LCGC</p> <p>Caring for a loved one with SMA can be emotionally and physically draining, and finding time for self-care is challenging. Come learn various techniques for taking care of yourself in this workshop, which will be available for two sessions. Each method has its own workstation, so you can come and go as you please. At the time of print, we have art therapy, aromatherapy, yoga, nutrition for wellness, and centering beads, though more stations may be added. We hope you find a method that works for you.</p>	Obelisk
12:00pm – 1:30pm	<p><b>Family Lunch with Symposium: Explore Gene Replacement Therapy  </b> Sponsored by AveXis </p>	Grand Ballroom A, B, C
1:30pm – 3:00pm	<p><b>Siblings Talk it Out (Ages 5 to 11)</b></p> <p>Katlyn O'Brien, Sister of a SMA Young Adult Traci O'Brien, Sister of SMA Young Adult Kelli Blume, Cousin of SMA Young Adult</p> <p>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 SMA family siblings and other family members. 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!</p>	Plum Blossom





<p>1:30pm – 3:00pm</p>	<p><b>Workshop Session # 6</b></p>	
	<p><b>Aquatic Physical Therapy for Fun &amp; Function, Children with Independent Head Control</b>                  Jennifer Martyn, PT                  Krista Torseth, PT, DPT                  Kendra Paker, PT, DPT, PCS</p> <p>Bring your swim suits and head to the pool for an active 90 minutes with a team of aquatic physical therapists. Our focus will be on improving functional strength and skills in a dynamic environment. Games, stretching, toys and balance will all be discussed and demonstrated during our session. We look forward to seeing you in the water.</p>	<p>Indoor Pool</p>
	<p><b>Special Plans for a Special Life</b>                  Richard Rubenstein, Esq.</p> <p>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.</p>	<p>Fleur-de-lis</p>
	<p><b>Healing the Grieving Heart – Now What? Part II</b>                  Fred Troutman, RN, PhD                  David Sereni, DPT</p> <p>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.</p>	<p>Edelweiss</p>
	<p><b>Top Tips on Traveling Around the World</b>                  Srin Madipalli, SMA Adult</p> <p>Travel entrepreneur and Airbnb Product Manager for accessibility, Srin Madipalli, will be running a session to share some of his top travel tips based on his extensive experience of travelling around the world!</p>	<p>Carpenter Ballroom</p>




	<p><b>Finding a Job, Interviewing and How to Ask for Modifications Panel</b></p> <p>Kimberly Hill, SMA Adult          Jaclyn Greenwood, SMA Adult          Carolyn Barrett, SMA Adult          Brad Nunemaker, SMA Adult          Brynne Willis, SMA Adult</p> <p>Come meet a diverse panel of successful adults with SMA as they discuss how they have obtained and sustained employment. Topics to be covered include the many aspects of the employment process from job searching, applying, interviewing, asking your employer for workplace accommodations, assistive technology for the workplace, and maintaining benefits (such as Medicaid) while working. The panel will share their personal experiences and helpful tips they have gained. This workshop will also include a question and answer session.</p>	Lalique
	<p><b>Fundraising 101</b></p> <p>Erin Kelly, Cure SMA Staff          Amber Snyder, Cure SMA Staff</p> <p>Join us for this fun and informative workshop that puts the <b>FUN</b> in <b>FUN</b>draising! We'll tackle topics like how to effectively make an ask, best practices for building sponsorships for any fundraising event, and ideas for local fundraising in your community. From forming a team at a Walk-n-Roll to planning a fundraiser with a local fitness studio – there are plenty of ways to make fundraising to support Cure SMA fun and easy for the whole family.</p>	Cardinal
	<p><b>Oral-Motor Exercises for Speech Clarity and Feeding</b></p> <p>Renee Roy Hill</p> <p>This workshop is for participants who are having difficulty with oral control for feeding, speech, saliva control and/or oral rest posture. During this session, attendees will learn how using a muscle-based approach to oral function can assist in maintaining and improving motor function, reducing fatigue. This approach focuses on assessing the underlying motor system and developing a plan based on current function and can be used with all types of SMA and function levels.</p>	Batik
<b>3:15pm – 5:15 pm</b>	<b>Researcher Q &amp; A Session</b>	Grand Ballroom D & E
<b>3:15pm – 3:35 pm</b>	<p><b>Welcome and Update on Cure SMA Research Activities</b></p> <p>Jill Jarecki, PhD, Chief Scientific Officer, Cure SMA</p> <p>Dr. Jarecki will discuss the scope and goals for Cure SMA research investments.</p>	



Sat.




June 17<sup>th</sup>, 2018

Research Q&A Session at Annual SMA Conference

<p>3:35 pm – 4:25 pm</p>	<p><b>Part I: SMA Research Panel (Basic science and Pre-Clinical Drug Development, Second Generation and Combinatorial Therapies)</b></p> <p>Moderated by Jackie Glascock, PhD, Senior Scientific Program Manager, Cure SMA</p> <p>Panel of SMA Basic Scientists/Companies with Pre-Clinical Drugs in Development</p> <ul style="list-style-type: none"> <li>- Umrao Monani, PhD, Associate Professor of Pathology and Cell Biology, Columbia University</li> <li>- Charlotte Sumner, MD, Professor of Neurology and Neuroscience, Johns Hopkins University School of Medicine</li> <li>- Barrington Burnett, PhD, Associate Professor, Department of Anatomy, Physiology and Genetics, Uniformed Services University</li> <li>- Steve O'Connor, PhD, Chief Executive Officer, Shift Pharmaceuticals</li> <li>- Yung Chyung, MD, Chief Medical Officer, Scholar Rock</li> </ul>	
<p>4:25 pm – 5:15 pm</p>	<p><b>Part II: Clinical Trial Updates</b></p> <p>Moderated by Jill Jarecki, PhD, Chief Scientific Officer, Cure SMA</p> <p>Panel of Companies with Drugs in Clinical Trials</p> <ul style="list-style-type: none"> <li>- Stacy Rudnicki, MD, Senior Medical Director, Clinical Research, Neurology, Cytokinetics</li> <li>- Douglas M. Sproule, MD, MSc, Vice President, SMA Therapeutic Area Head, AveXis Inc, AveXis Inc.</li> <li>- Paulo Fontoura, MD PhD, Global Head Clinical Development Neuroscience, F.Hoffmann-La Roche/Genentech</li> <li>- Herve Jullien de Pommerol MSc, MBA, Global Program Head, Neuroscience, Novartis</li> <li>- Wildon Farwell MD, Senior Medical Director, Clinical Development, Biogen</li> </ul>	
<p>6:30pm – 8:30pm</p>	<p><b>PJ Party &amp; Movie Night</b></p>	<p>Grand Ballroom A, B, C</p>
<p>7:30pm – 10:00pm</p>	<p><b>Roll-a-Palooza, Live Music Event  </b> Sponsored by Adyn's Dream </p>	<p>Imperial Ballroom</p>

Sun.

June 17<sup>th</sup>, 2018

<p>7:30am – 8:45am</p>	<p><b>Family Breakfast with Symposium: Bringing the Patient Voice into SMA Research</b> Sponsored by Genentech/Roche </p>	<p>Grand Ballroom A, B</p>
<p>9:00am – 11:15am <i>Drop off opens at 8:30am</i></p>	<p><b>Children's Program  </b> sponsored by JIRF </p>	<p>Stemmons Ballroom B, C, D</p>
<p>9:00am – 11:15am <i>Drop off opens at 8:30am</i></p>	<p><b>Baby Room  </b> sponsored by JIRF </p>	<p>Stemmons Ballroom A</p>
<p>9:00am – 11:15am</p>	<p><b>It's a Wonderful Life/Closing General Session</b></p>	<p>Grand Ballroom C, D, E</p>

# 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. We truly see Cure SMA as a gift of hope and are overjoyed to have reached our \$1 million goal to ensure that all newly diagnosed families can attend this amazing event in perpetuity.**

*-Gene Trainor*

## Congratulations to the Trainor Family

And everyone who has supported their effort in reaching the \$1 million endowment goal of the Erin Trainor Memorial Fund (ETMF) and the initial disbursement of funds starting at last year's 2017 Annual SMA conference.



# Jacob Isaac Rappoport Foundation



Since Jacob Rappoport's diagnosis in 2001, Shaina and Adi Rappoport have dedicated themselves to Cure SMA's mission. Through their effort to create a legacy for Jacob after his death, Shaina and Adi created The JIRF (Jacob Isaac Rappoport Foundation). The JIRF generously funds some of the most popular programs at the Annual SMA Conference each year.

The JIRF sponsors the Children's Program each year so that parents can attend important workshops and lectures while their affected children are entertained and safe. The program provides fun crafts, entertainers, and activities led by an incredible group of volunteers.

The JIRF sponsors the Teen Social which is its fourth year. This event gives all teens at the conference the opportunity to gather and make connections. The Teen Social was founded by Jordan Rappoport, sister to Jacob.

Because of the Rappoport's personal experience with SMA type I, the JIRF also provides funding for the Type I Reception. The reception brings together type I families in an emotionally supportive environment to connect and share their unique experiences.

In addition to programs at the Annual SMA Conference, the JIRF also sponsors all Type I Newly Diagnosed Packages. These packages are sent to every type I family that contacts Cure SMA and includes toys and helpful ideas. The JIRF has also provided significant funding for research programs.

Thank you to Shaina and Adi, along with their children Jordan, Max, and Sam, for their continued support of Cure SMA and our SMA community!

**jir**  
the jacob isaac rappoport  
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# We're proud to sponsor Cure SMA and the 2018 Annual SMA Conference

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## Jennifer Miller Smith, Aaron Smith and Family



Jennifer Miller Smith and Aaron Smith have been involved with Cure SMA for the last 11 years. In 2007, their daughter, Madison, was diagnosed with SMA. The family immediately connected with Cure SMA to connect with other families and raise funds to support SMA research. Jennifer and Aaron generously volunteer their time to work on everything from signage to workshops to gathering in-kind donations for the conference. Thank you to Jennifer, Aaron, Katie, Zach, Madison, and the rest of this incredible family for their dedication to making our conference a success each year.

We would also like to thank everyone at Expo Convention Contractors in Miami, FL for their incredible support of Cure SMA. The company donates all the professional signage and banners displayed throughout the hotel during conference. A special thank you to Richard Curran, company owner and grandfather to Madison, for his generous support of Cure SMA.

In addition to their incredible support of the conference, Jennifer, Aaron, and Expo Convention Contractors also generously supports families and events in south Florida.



A photograph showing two pairs of hands gently holding a small green seedling with two leaves, planting it into dark brown soil. The background is a blurred field of similar seedlings.

# IMAGENE IF

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## Concert for a Cure Hits \$2 Million Milestone



The Cure SMA Concert for a Cure took place on April 21, 2018 at Diablo Country Club in Diablo, CA and reached a very exciting goal-after combining 18 years of fundraising and hard work, this special event has raised \$2 million! 320 guests joined together and celebrated at Aloha to the Cure, as we say 'hello' to a treatment for SMA, and 'so long-see you later' to Danny and Arianna who graduate high school this year. Aloha to the Cure raised over \$200,000 this year!

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

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

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

Since then, Concert for a Cure grew tremendously with the help of community support! The McHale and Dindzans Families joined together to create the largest event in the West Coast for Cure SMA! From 2002-2011, the event was held at Blackhawk Country Club in Diablo and reached their \$1 million dollar mark in 2012-Celebrate Miracles by the Millions. The fun themes such as Cruising for the Cure, UnCork the Cure, Champions for the Cure, always gave a unique feel to the special night. In 2013, Diablo Country Club became the new home for Concert for a Cure! During the evening-guests enjoy raffles, cocktail auction, balloon pop and games on the terrace before gathering together in the ballroom for dinner, a live auction and presentations by the McHale Family, SMA Club at Danny's school, and research updates about SMA by local doctors.

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

To learn more or get involved, visit [www.concertforacure.org](http://www.concertforacure.org)



# 7th Annual "Hope on the Hill" Congressional Dinner Hits \$1M Milestone



On Wednesday, November 29, 2017 more than 160 people from the SMA community - including government and industry partners – gathered for the 7th Annual “Hope on the Hill” Congressional Dinner in Washington, D.C. Several members of congress attended the dinner including Rep. Sean Duffy, Rep. Tom Emmer, Rep. Erik Paulsen, and Rep Greg Walden.

The evening included remarks from event co-chairs Greg and Shannon Zerzan, Cure SMA President Kenneth Hobby, and SMA mom Cheryl Yoder. Rep. Greg Walden, Chairman of the United States House of Representatives, Committee on Energy and Commerce gave a keynote address highlighting the recent milestones in SMA research. Rep. Walden was presented with the Cure SMA Friends and Family Award for his dedication to the SMA community and support of the “Hope on the Hill” Congressional Dinner over the last seven years.

This year’s event raised nearly \$200,000, bringing the event’s cumulative total to over \$1 million raised. The “Hope on the Hill” Congressional Dinner began in 2011 as an intimate gathering of friends and supporters. Now, heading into its eighth year, the dinner has grown into a premier event which raises funds for research and

support services for SMA. The event also raises awareness about SMA by building connections between elected officials and industry leaders, so they can work to better address issues facing the SMA community.

Cure SMA would like to say a special thank you to Greg and Shannon Zerzan for chairing this event since 2011. We also appreciate the hard work and dedication of the steering committee, volunteers and sponsors who all work tirelessly to make this event a success.

The 8th Annual “Hope on the Hill” Congressional Dinner will be held on November 27, 2018.





# Genentech

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## Proud to be a sponsor of the **Cure SMA 2018** Annual Conference

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**Researcher  
Symposium**

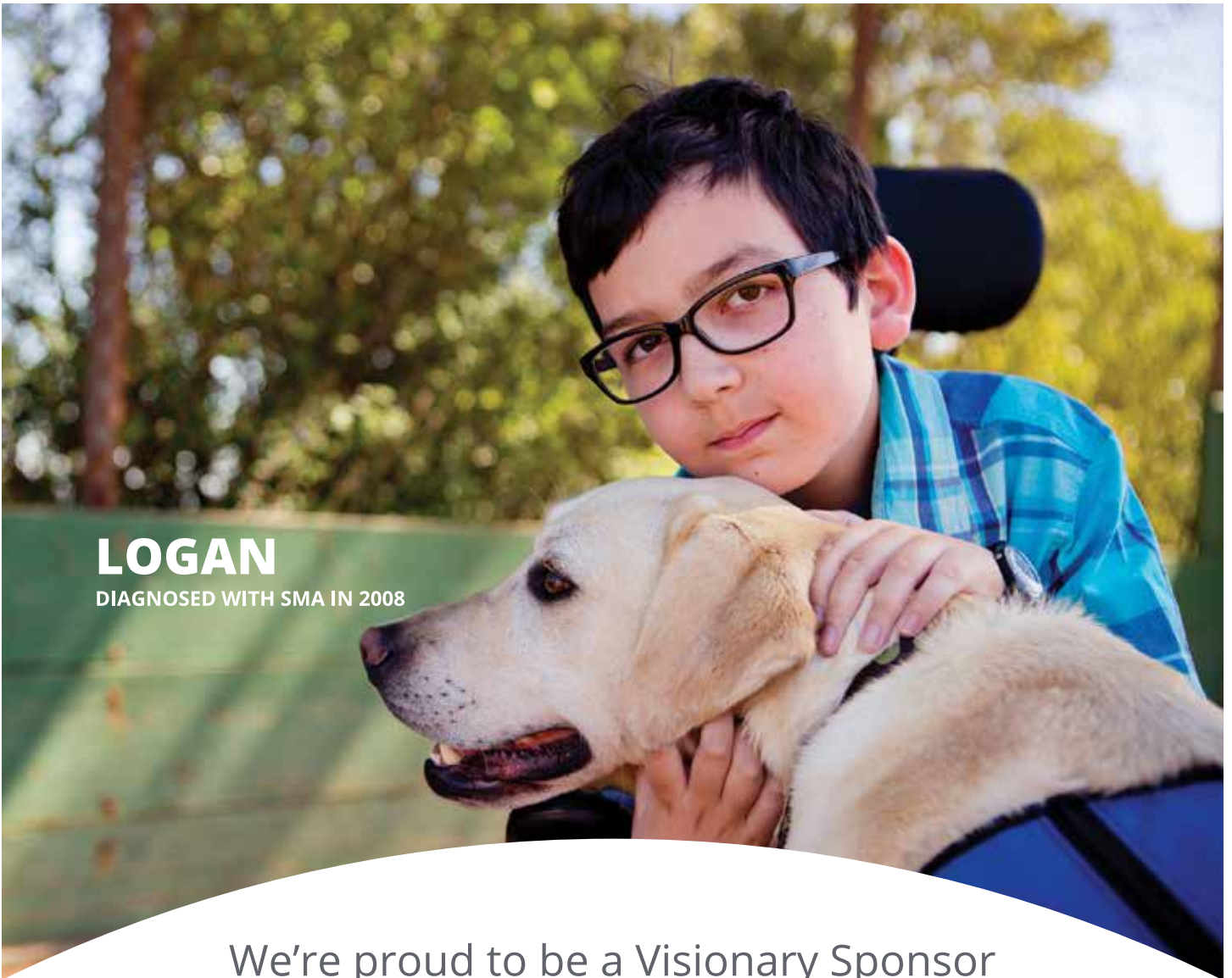
Friday, June 15th  
7:00 – 8:30 AM

**Family  
Symposium**

Sunday, June 17th  
7:30 – 8:45 AM

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## Rocky Mountain Golf Tournament

The Rocky Mountain Golf Tournament was first organized in 2003 by Gillian Faith, who hosts this annual event in honor of her nephew. In 2018 the Rocky Mountain Golf Tournament celebrated their 15th anniversary on May 21, 2018 at The Club at Pradera in Parker, CO. Over the last 15 years Gillian and her fundraising efforts have raised well \$340,000 and have helped contribute to Cure SMA making tremendous strides toward research, treatments and our program services.

Thank you to Gillian for hosting this annual event, and for your tireless work and time over the last 15 years. Congratulations on 15 years!



## 15th Annual Northern California Walk-n-Roll

The 15th Annual Walk-n-Roll was held on August 20, 2017 at Golden Gate Park in San Francisco. Dr. John Day from Stanford University Medical Center updated us on the latest SMA research, and Cure SMA President, Kenneth Hobby, thanked event organizers for their dedication and support to Cure SMA for the past 15 years! Thanks again to Linda Shively, Marge Shively, David Sereni, and Dick and Patricia Wolff; we appreciate everything you have done! The walk started back in 2003, and surpassed the fundraising goal by \$33,000 this year!

The morning started off with energetic warm up exercises and stretches before heading out on the two-mile walk. We paused at the Conservatory of Flowers for our traditional photo before proceeding to Rainbow Waterfall and back to Peacock Meadow. The fun continued in the green space area with face painting, games, lunch, and a lively raffle and juice kindly donated by Barsotti Juice Company. This year, the event raised a record **\$98,000** for Cure SMA which included over 39 teams and 400 participants!



## 15th Annual Cincinnati Walk-n-Roll

**Congratulations to the Cincinnati Walk-n-Roll for hosting its 15th annual Walk-n-Roll in Cincinnati, OH. Since its inception this event has raised more than \$1 million for Cure SMA.**

We would also like to extend a huge thank you to Ron & Janet Merkle, Marilyn Pater, Gloria Logeman, and Linda Schwabb for their key roles in this event.

The 15th Annual Cincinnati Walk-n-Roll took place on June 9, 2018 at Xavier University's Cintas Center. Attendees gathered to celebrate the amazing strides we've made this year and fundraise for future breakthroughs!

**A special thank you to all the current walk committee members: Beth and Kevin Lockwood, Bob and Elizabeth Lockwood, and Nicole, Mark and Amy Haake.**



## 15th Annual Pennsylvania Chapter Walk-n-Roll

**Congratulations to the Pennsylvania Walk-n-Roll for hosting its 15th annual Walk-n-Roll in Philadelphia, PA. Since its inception this event has raised more than \$1.1 million dollars for Cure SMA.**

The 15th Annual Pennsylvania Walk-n-Roll took place on May 20, 2018 at Shoemaker Green. Over 600 attendees gathered to celebrate the strength of our SMA community! Everyone enjoyed all the fun games, raffle, and the scenic view of the Philadelphia skyline!

**A special thank you to all the current walk committee members: Gina Cannady, Allyson Henkel, Stephanie Reese, Paula Saxton, Tara Maida, Christina Mohammed, and Karen Mcrory-Negrin.**



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

The Cure SMA Walk-n-Roll in Honor of Katherine Santiago celebrated its 10th Anniversary in May 2017 and continued to their 11th year on May 19, 2018. After Katherine passed away, the walk started off as a bat mitzvah project for Katherine's best friend, Allie Mazzella. Upon graduating high school, Allie passed the Walk down to Katherine's younger sister, Julia.

Katherine's Walk is spearheaded by Julia Santiago and her parents, Alma and Efren Santiago. Katherine's Walk has grown over the past 11 years and have raised over \$55,000 for Cure SMA. The Santiago family and Allie's hard work and dedication to fundraising for Cure SMA has helped us make tremendous strides in the SMA community – contributing to the funds needed to support ongoing research efforts and program services.

Thank you Julia, Alma and Efren Santiago and Allison Mazzella for hosting this walk each year!



## 2017 Pacific Northwest Walk-n-Roll

On August 20, 2017 the Pacific Northwest Walk-n-Roll celebrated its 10th anniversary. The walk was originally started in 2007 by Rick Jones in Seattle, who then passed it down to Alyssa Jones. Since 2016, Kelly and Russ Hargrave have spearheaded the Pacific Northwest Walk-n-Roll.

Without Kelly and Russ's tireless work and effort over the last two years, the walk would not be such the success it is today.

To date, the Pacific Northwest Walk-n-Roll has raised over **\$155,000**. It will continue onto its 11th year at Game Farm Park in Auburn, WA on August 19, 2018.

Thank you to Kelly and Russ Hargrave for spearheading this event over the last two years. We look forward to continuing the event as Cynthia Turner takes the lead.

# WELCOME!

We are honored to have SMA families and researchers attend this conference from all over the world! There are individuals attending from over 23 countries including: Argentina, Australia, Brazil, Canada, Chile, Columbia, France, Germany, Georgia, Hong Kong, Ireland, Italy, Japan, Mexico, Netherlands, New Zealand, Russia, Spain, Sweden, Switzerland, Taiwan, United Kingdom, and United States of America.



Thanks to the support of our generous sponsors of the 2018 Annual SMA Conference, Cure SMA is able to bring together over 1550 researchers, SMA families and healthcare professional to network, learn and collaborate. This opportunity offers a unique experience to work in partnership with one another to enhance groundbreaking research and provide families the support they need today.

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## Adyn's Dream supports families living with SMA through the excitement and joy of live music.

Since 2014, Adyn's Dream has raised money to provide Cure SMA scholarships, equipment, and much more for families in the SMA community by hosting concerts with some of Adyn's favorite musicians.



## Come join us on Saturday!

Join us Saturday evening in the ballroom as Adyn's Dream brings live music to the Cure SMA conference! Featuring some of Austin's finest bands, we are excited to bring one of our passions to the community we're passionate about!

[www.AdynsDream.org](http://www.AdynsDream.org)

# THANK YOU TO The Miller McNeil Woodruff Foundation!

The Miller McNeil Woodruff Foundation was founded in 2011 in memory of Miller Woodruff. Since Miller's passing, the Foundation has been dedicated to spreading awareness and helping to fund crucial research and support services for families with SMA. Every year, the Miller McNeil Woodruff Foundation also generously sponsors scholarships for families from Arkansas to attend Conference.



The Miller McNeil Woodruff Foundation

**On behalf of the entire SMA community, thank you to the  
Woodruff Family and the Miller McNeil Woodruff Foundation!**

Thank you to the support of our exhibitors who helped make the 2018 Annual SMA Conference a great success!

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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.

### Meet & Greet

Thursday 6:00pm

Kick off to the Conference!

Grand Ballroom

-Researcher Relay Race

-Family Fun Fest/Carnival

### Family Friendly Research Poster Session

Friday 6:30pm

Rotate around to over 25 posters presented by researchers

Grand Ballroom A, B, C

### Family and Researcher Dance Party

Friday 7:30pm

Dance the night away!

Grand Ballroom D & E

### PJ Party and Move Night

Saturday 6:30pm

Family Movie for kids and adults alike!

Grand Ballroom A, B, C

### Roll-a-Palooza, Live Music Event

Saturday 7:30pm

Rock & roll as 2 live bands perform

Imperial Ballroom

*Sponsored by Adyn's Dream*





## Meet & Greet and Family Fun Fest

Grand Ballroom • Thursday June 14<sup>th</sup>, 6:00 pm to 8:30pm

Conference welcome, introductions, carnival games, and prizes for all children



**Conference wristbands must be worn during the entire conference.**

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

# CELEBRATING 30 YEARS!

2018



1988



<b>2018</b> - Hilton Anatole Hotel	Dallas, Texas
<b>2017</b> - Disneyworld (Contemporary Hotel)	Orlando, Florida
<b>2016</b> - Disneyland Hotel	Anaheim, California
<b>2015</b> - Westin and Sheraton Hotels	Kansas City, Missouri
<b>2014</b> - 30th Anniversary–Gaylord National	Washington, DC
<b>2013</b> - Disneyland Hotel	Anaheim, California
<b>2012</b> - DoubleTree–Bloomington	Bloomington, Minnesota
<b>2011</b> - Disneyworld Swan and Dolphin Resort	Orlando, Florida
<b>2010</b> - Marriott–Santa Clara	Santa Clara, California
<b>2009</b> - Hyatt Regency Cincinnati	Cincinnati, Ohio
<b>2008</b> - Beantown–Boston Park Plaza	Boston, Massachusetts
<b>2007</b> - Moving Forward–Hyatt Regency Woodfield	Schaumburg, Illinois
<b>2006</b> - Surfin' SMA–Mission Valley Marriott	San Diego, California
<b>2006</b> - International SMA Research Meeting	Montreal, Canada
<b>2005</b> - Together for a Cure–Marriott Downtown	Philadelphia, Pennsylvania
<b>2004</b> - Connect for a Cure–Hyatt Regency Woodfield	Schaumburg, Illinois
<b>2003</b> - SMA Takes the Hill–Hyatt Regency Capital Hill	Washington, DC
<b>2002</b> - Greatest Conference Ever–Hyatt Regency Woodfield	Schaumburg, Illinois
<b>2001</b> - Magnificent Milestones–Fairmont Chicago	Chicago, Illinois
<b>2000</b> - Conference of the Century–Hilton St Louis	St Louis, Missouri
<b>1999</b> - Countdown to a Cure–Hyatt Regency	Milwaukee, Wisconsin
<b>1998</b> - SMA: Past, Present and Future–Hyatt Regency	Denver, Colorado
<b>1997</b> - New Horizons... Making Progress–Wyndham	Itasca, Illinois
<b>1995</b> - MGM Grand–Las Vegas	Las Vegas, Nevada
<b>1995</b> - International SMA Research Meeting	Amsterdam
<b>1994</b> - The Clarion Plaza Hotel	Orlando, Florida
<b>1992</b> - Pheasant Run Resort	St Charles, Illinois
<b>1990</b> - Pheasant Run Resort	St Charles, Illinois
<b>1988</b> - The First SMA Conference–Hyatt Regency Chicago	Chicago, Illinois

# The Family Friendly Research Poster Session

Friday, June 15 from 6:30 pm to 8:00 pm

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

## POSTERS INCLUDE:

- **Kristina Gelblin from F. Hoffmann-La Roche** on “Partnering with Patients in the Roche and Genentech SMA programme”.
- **Ksenija Gorni MD PhD, Tim Seabrook PhD, and Sangeeta Jethwa MD from F. Hoffmann-La Roche** on “Roche and Genentech SMA program advancing in clinical development”.
- **Dylan Trundell PhD from F. Hoffmann-La Roche** on “Living with SMA- Individuals with SMA and their caregivers share their experiences”.
- **Kimberly Long PhD from Scholar Rock** on “Specific inhibition of myostatin activation improves muscle and bone phenotypes in models of SMA therapy”.
- **Fernanda Boulos MD and Herve Jullien de Pommerol PhD from Novartis** on “Introduction to Novartis”.
- **Douglas M. Sproule MD from AveXis, Inc.** on “Understanding Gene Replacement Therapy and the potential to help people with monogenic diseases”.
- **Zaida Alipio-Gloria from Calibr** on “Innovative screening approaches to ID the next generation of SMN-inducing compounds”.
- **Lisa Belter MPH from Cure SMA** on “Cure SMA Membership: Findings from the 2018 Membership Survey”.
- **Rosangel Cruz MA from Cure SMA** on “Benefit-Risk Survey Results for Spinal Muscular Atrophy-Informing Drug Development in SMA”.
- **Mary Schroth MD and Page Kirkpatrick MHA, MBA from Cure SMA** on “Cure SMA Care Center Network and SMA Clinical Data Registry: Using data to improve care”.
- **Jackie Glascock PhD from Cure SMA** on “Development of a Treatment Algorithm for Infants with SMA Identified Via Newborn Screening”.
- **Bakri Elsheikh MD from Ohio State University** on “Nusinersen in adults with SMA: The Ohio State Experience”.
- **Kathryn J. Swoboda MD, Jin Yun Helen Chen LGC, and Ren Zhang MPH from Massachusetts General Hospital**, on “The “SPOT SMA” Longitudinal Population Database Repository (LPDR): a tool for the SMA community and their physicians”
- **Christine DiDonato PhD from Ann & Robert H. Lurie Children’s Hospital/Northwestern University** on “Bridging Basic Science to Clinical Research and Outcomes: The Lurie Children’s Hospital experience”.
- **Elizabeth Luebbe MS from University of Rochester** on “The SMA-Health Index: A Valid Instrument to Measure Therapeutic Benefit in Clinical Trials”.
- **Stacy Rudnicki MD from Cytokinetics** on “Update of CY 5021: A phase 2 clinical trial of reldesemtiv, a fast skeletal muscle troponin activator (FSTA), for the potential treatment of spinal muscular atrophy”.
- **Hikari Tanaka from Uniformed Services University of the Health Sciences** on “Protein stabilizer enhances ASO to improve SMA phenotype”.
- **Meaghan Van Alstyne from Columbia University** on “Mechanisms of Motor Neuron Death in SMA”.
- **Oliver Gruss PhD and Utz Fischer PhD from Rheinische Friedrich-Wilhelms-Universität Bonn and University of Wuerzburg, Germany** on “Identification of cellular signalling cues regulating the SMN complex and its implications for SMA”.
- **Robin Parks PhD from the University of Ottawa** on “Can tiny bits of cells in the blood help track the progress of SMA?”.
- **Brunhilde Wirth PhD from the University of Cologne, Germany** on “Genetic modifiers of SMA help some people to remain asymptomatic; How can this knowledge help all people with SMA?”.
- **Erik Osman PhD from the University of Missouri** on “Optimization of Morpholino Modified Anti-sense oligonucleotides”.
- **Marc-Olivier Deguise from the University of Ottawa** on “Metabolic considerations: Does fat intake matter in SMA?”.
- **Allison Ebert PhD from the Medical College of Wisconsin** on “Star Wars: How SMA astrocytes hurt motor neurons”.
- **Jocelyn Côté PhD from the University of Ottawa** on “SMN & Friends: What we can learn from identifying SMN interacting partners”.
- **Arthur Burghes PhD and Corey Ruhno from Ohio State University** on “Genetic variation affecting Spinal Muscular Atrophy severity”.
- **Cera Hassinan from Johns Hopkins School of Medicine** on “Impaired motor axon radial sorting and growth is followed by a rapid phase of axonal degeneration in severe SMA”.

## Collect your Stickers !

Please use your sheet to collect stickers at each of the research posters. Rotate around to each poster and if you have collected 10 stickers, you may enter your sheet into the bin for a chance to win an Amazon Echo! The winner will be announced on Sunday at the It's a Wonderful Life Session.



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BAYADA client Mary, who has SMA, is pictured with her mom, Tara, during their local SMA Walk-n-Roll event.

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# MENU

## Conference Meals included with registration:

### Thursday

#### Dinner at Meet & Greet:

Garden salad, Texas BBQ pork, grilled chicken, cowboy baked beans, tater tots and cheese quesadillas

### Friday

#### Breakfast Items:

Sausage & egg on cheddar biscuit, egg on cheddar biscuit (no sausage), coffee and juice

#### Lunch:

Boxed lunches with assortment of turkey, ham, vegetarian, PB&J sandwiches, pasta salad, chips, fruit cup, cookie

#### Snacks:

Light snacks of pretzels, rice krispy treats, and refreshments during Family Friendly Researcher Poster Session

### Saturday

#### Breakfast:

Breakfast burritos, egg on cheddar biscuit (no sausage), coffee and juice

#### Lunch:

Boxed lunches with assortment of chicken Caesar wrap, ham, vegetarian, PB&J sandwiches, pasta salad, chips, fruit cup, cookie

#### Snacks:

Light snacks of popcorn, chocolate covered pretzels, and refreshments during the PJ Party and Roll-a-Palooza

### Sunday

#### Breakfast:

Assorted muffins, sticky pecan buns, hard boiled eggs, coffee and juice



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## SCHOLAR ROCK

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a singer*  
Lucy, 6  
spinal muscular atrophy

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# Children's Program Entertainment Schedule



## 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.

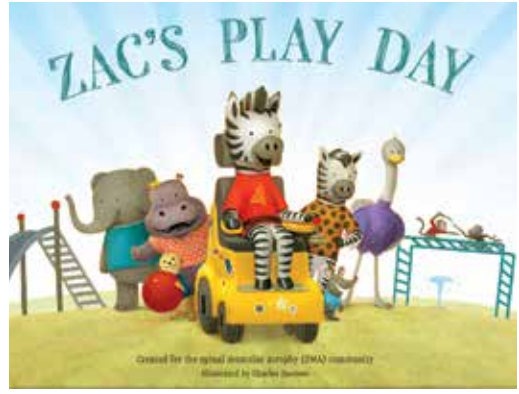
## Friday

June 15<sup>th</sup> 2018

<b>Morning:</b> 9:00am- 12:30pm	<b>Cartoon You Caricatures   9:00am- 12:30pm-</b> Stop by this fun station so caricaturist Rafael can draw a photo of you to take home as a fun conference memento!
	<b>Up, Up and Away!   11:00am- 12:00pm-</b> This spellbinding special event introduces children to the principles of air and pressure. Hot air balloons, vortex generators and even a hovercraft will help children understand the power of air.
<b>Afternoon:</b> 2:00pm-5:15pm	<b>Cartoon You Caricatures   2:00pm- 5:15pm-</b> Stop by this fun station so caricaturist Rafael can draw a photo of you to take home as a fun conference memento!
	<b>Sounds Like Science   3:00pm- 4:00pm-</b> Make waves and some noise in this fun and funky science show! The Mad Scientist will create crazy cacophony with a garbage can, a swinging sound tube and a metal pipe! Children will cheer as pickles glow and potatoes pop! What are all the kids talking about? Sounds like Science!

*\* Be sure to check out Cartoon You Caricatures so caricaturist Rafael can draw a photo of you to take home as a fun conference memento! Will be available all day Friday and Saturday during children's program hours!*





# Saturday

June 16<sup>th</sup>, 2018

<b>Morning:</b> 8:45am- 12:00pm	<b>Cartoon You Caricatures   9:00am- 12:00pm-</b> Stop by this fun station so caricaturist Rafael can draw a photo of you to take home as a fun conference memento!
	<b>SMA Storytime   10:30am- 11:00am-</b> A reading of the very first children's book on spinal muscular atrophy with Dr Robert Graham and Dr Crystal Proud! Join Biogen on Saturday, June 16 at 10:30 AM for the big debut of Zac's Play Day.
	<b>Tea Party   11:00am- 12:00pm-</b> Any children interested in joining the Tea Party are also welcomed to bring their dolls.
<b>Afternoon:</b> 1:30pm-5:15pm	<b>Cartoon You Caricatures   1:30pm-5:15pm-</b> Stop by this fun station so caricaturist Rafael can draw a photo of you to take home as a fun conference memento!
	<b>Professor Brainius   3:00pm-4:00pm-</b> The Professor Brainius Show is a high energy, age-appropriate, interactive Edu-Tainment experience! The Science experiments performed are exciting and full of magic moments for the audience.

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

Sponsored by



## 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.





## Thank You to All of the Conference Children's Program Volunteers

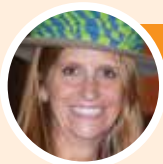
Each and every one of our volunteers has a major impact on the atmosphere and success of our conference, and every year their impact radiates throughout the weekend! Their long days of hard work provide families with the reassurance that their children are well cared for and are having fun, while they 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 29 YEARS**

**George Ghorbanian**  
Chicago Police Officer  
George has volunteered at the conference since the Children's Program started back in 1990.



**VOLUNTEER FOR 27 YEARS**

**Patti Slojkowski**  
Dental Hygenist



**VOLUNTEER FOR 24 YEARS**

**Steve Smith**  
Physical Therapist



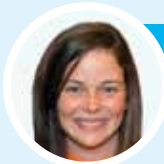
**VOLUNTEER FOR 20 YEARS**

**Kelly Milito**  
Hair Stylist



**VOLUNTEER FOR 19 YEARS**

**Jeff Harris**



**VOLUNTEER FOR 18 YEARS**

**Katlyn O'Brien**  
Elementary School Teacher



**VOLUNTEER FOR 18 YEARS**

**Kelly Basso**  
Surgical Consultant



**VOLUNTEER FOR 17 YEARS**

**Peg Bailey**  
Nurse



**VOLUNTEER FOR 8 YEARS**

**Kevin Blume**



**VOLUNTEER FOR 17 YEARS**

**Mary Blume**  
Hospital Administrative Assistant



**VOLUNTEER FOR 8 YEARS**

**Mike Graney**  
Vice President of United services



**VOLUNTEER FOR 17 YEARS**

**Traci O'Brien**  
Recent Grad School Graduate  
in Marketing



**VOLUNTEER FOR 7 YEARS**

**Lexi Basso**  
College student at Elmhurst



**VOLUNTEER FOR 9 YEARS**

**Patrick Hogan**  
High School History and  
Psychology Teacher



**VOLUNTEER FOR 7 YEARS**

**Tori Zeman**  
Occupational Therapist



**VOLUNTEER FOR 8 YEARS**

**Megan Milito**



**VOLUNTEER FOR 6 YEARS**

**Georgia Slojkowski**



**VOLUNTEER FOR 8 YEARS**

**Joy Martin**  
Physical Therapist



**VOLUNTEER FOR 6 YEARS**

**Laura Kroll**  
College student studying  
Elementary Education



**VOLUNTEER FOR 8 YEARS**

**Kelli Blume**  
School Social Worker



VOLUNTEER FOR 7 YEARS

**Danielle Kirincich**



VOLUNTEER FOR 7 YEARS

**Hayley Miller**



VOLUNTEER FOR 7 YEARS

**Collene Cahoy**



VOLUNTEER FOR 5 YEARS

**Megan Besler**  
College student at Mizzou



VOLUNTEER FOR 5 YEARS

**Leah Schneider**



VOLUNTEER FOR 4 YEARS

**Patrick Slojkowski**



VOLUNTEER FOR 4 YEARS

**Emily Kafkes**  
College Student at University of Illinois in Champaign



VOLUNTEER FOR 4 YEARS

**Sabrina Ghorbanian**  
Hair Stylist



VOLUNTEER FOR 4 YEARS

**Daniela Milito**



VOLUNTEER FOR 4 YEARS

**Michael Milito**



VOLUNTEER FOR 4 YEARS

**Raphi Milito**  
College Student at University of Iowa



VOLUNTEER FOR 4 YEARS

**Ralph Milito**  
Real Estate Agent



VOLUNTEER FOR 3 YEARS

**Maggie Harris**



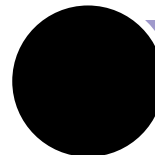
VOLUNTEER FOR 3 YEARS

**Kamryn McKenzie**



VOLUNTEER FOR 3 YEARS

**Rebeka Nekolova**



VOLUNTEER FOR 3 YEARS

**Hannah Lanasa**



VOLUNTEER FOR 2 YEARS

**Jaimie Chang**



VOLUNTEER FOR 2 YEARS

**Mae Cahill**



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Samantha Smith



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cureSMA.org



# 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 & Committee Members work exceptionally hard to make sure that all our families and friends are supported to 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!

**Richard Rubenstein** – Cure SMA is thrilled to have Richard 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. Richard most certainly puts his all into this role and is always looking forward to improving, promoting, and assisting in any way possible within the SMA community.

**The Cure SMA Chapter Officers and Committee Members** – 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 Leaders 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 solution 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 Trainor Family** – Thank you to the Trainor Family for their long standing support which they have provide to Cure SMA over the years. It is because of your generosity that Cure SMA and the Annual Conference are where we are at today!

**The Miller McNeil Woodruff Foundation and Meredith & Patrick Woodruff** – Thanks to this foundation many families from the state of Arkansas are again able to attend this amazing conference. Through their generosity, Meredith and Patrick have provided scholarships so these families have an opportunity to attend and meet other families. The Miller McNeil Woodruff Foundation began in memory of Meredith & Patrick’s son, Miller who has SMA type I.

**The Dhont Family Foundation** – We are so honored to be able to provide any adult with SMA, a scholarship for themselves and an aide to attend the conference this year, all due to the Dhont Foundation’s support and funding. Thank you for providing this opportunity to so many in the community, and for sponsoring the ever popular Adults with SMA Social!

**Adyn’s Dream Foundation** – A big thank you to the Adyn’s Dream Foundation for their amazing idea and sponsorship of our newest event, the Roll-a-Palooza. This event is a live music concert of some local bands and gives our families and the community at conference an opportunity to attend a fun and exciting concert! We also appreciate the opportunity this foundation has provided to support 6 families and help them attend the Annual SMA Conference!

**RhysStrong Foundation** – This amazing organization has helped fund 3 families to attend the conference this June! Thank you so much for making this possible for these families and for your support of the Annual SMA Conference.

**Jacob Isaac Rappoport Foundation** – Thank you to this amazing foundation for your continued years of support for newly diagnosed families, the conference and so much more. We truly appreciate your kindness and generosity.

**Jennifer Miller-Smith, Aaron Smith, Richard Curran and Expo** – We can never express our gratitude enough for all that you do to ensure that each conference is as successful as possible. Thank you for everything!

# Thank You

Each year, Cure SMA is amazed at the amount of support received from our wonderful chapters and supporters across the country.

Thanks to the dedication of our community and the ingenuity of our researchers, we now have the first-ever approved treatment that targets the underlying genetics of SMA. And we're on the verge of further breakthroughs that will continue to change the course of SMA for everyone affected—from infants to adults—and eventually lead to a cure thanks to your efforts and the funds you raise and donate.

**We truly cannot thank you enough for the time, energy, and dedication put into everything you do!**

Natasha & Ben Abruzzo

ToniAnn & Nick Accardi

Kelly & Jason Alexander

Brittany Arana

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Alexandrea Balleau & Richard Wolfe

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Kelly & Keven Coggin

Kelly Cole

Alyssa & Michiel Combs

Jared Crawford

Richard Curran

Kellie Cusack

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Olivia & Michael Enderlin

Douglas & Michele Erwin

Al Freedman

Jack & Karen Greenwood

Jaelyn Greenwood

Bonnie & Eric Grimmett

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Doug Kerr

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Jennifer Miller Smith & Aaron Smith

Tara & Jim Montague

Rick & Autumn Montoya

Gillian Mullins

Silvia & Brian Murphy

Tom Murray

Kate & Ryan Nelson

Brad & Krista Nunemaker

Jennifer & Jason Patrick

Sapna & Charles Pringle

Tara & Waymon Ragan

Steven & Erika Ragland

Megan & Tom Ramirez

Kelly & Christie Rath

Stephanie & Kareem Reese

Kristen Resendez

Whitney & Brandon Roberts

Samuel & Sharon Rosenfeld

Richard & Michele Rubenstein

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Glenn & Cynthia Schaefer

Kevin Schaefer

Misty & Keith Schaper

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David & Lisa Sereni

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Jamie & John Shish

Linda Shively

Marge Shively

Kristen Smith

Brian Snyder

Holly Sontag & Christopher Thomas

Peter Statile

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Cynthia & David Turner

Stephen & Lynne Vaudry

Ann Ward

Maggie Weldon

Silvia Wheeler

Jessica & Randy White

Kristal & Nick Wilson

Angel & Ryan Wolff

Dick & Pat Wolff

Mariano Zegarelli & Lorena Lardizabal

De Zegarelli

Shannon & Greg Zerzan

Jill & John Zmaczynski

*\*Registered as of May 10, 2018*

# Cure SMA Chapter List

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.

## Alabama

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## Alaska

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## Carolinas

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## Capital Region, satellite of the Greater New York Chapter

Amy Cunniff-Bleau, President  
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## Chesapeake

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## Cure SMA National Office

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1.800.886.1762



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# August is SMA Awareness Month

Be sure to check back at [www.CureSMA.org](http://www.CureSMA.org) for the latest news & happenings going on during the month of August

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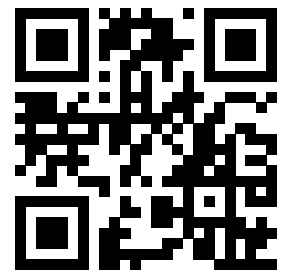
The success of our conference can only be improved by the feedback that we received from you! Please take the time to provide any comments by filling out our conference survey, and the workshop surveys. You can find hard copies of your surveys in your conference folder. Please drop your completed surveys into the many bins throughout the meeting space, at your workshops or at the Registration Area.

**All participants who complete the surveys by 10:30am on Sunday, June 17th, will have their name entered into a raffle.**

**The winners of the raffle will receive a brand new Amazon Echo!**

All completed surveys will also be entered into a drawing for a chance to win a trip, including airfare to the 2019 Annual SMA Conference (the locations will be announced on Sunday).

The winners will be drawn and announced on Sunday, June 17th at the Closing General Session/It's a Wonderful Life.



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**Carolyn Barrett**, is a 23 year old recent graduate of the Morrissey College of Arts and Sciences at Boston College. Studying Political Science, she lived on campus, worked in admissions, campus ministry and managed up to fifteen personal care assistants. She now works full time in Boston for the Boston Bar Association, taking on the city with her Service Dog Shadow. Carolyn loves to ski, spend time with friends and drive her adapted vehicle. She has been coming to the conference for as long as she can remember and is always happy to share her story and learn from others. Carolyn has SMA type II.

**Paula Barrett**, is leadership development consultant and a partner at Leadership Partners, LLC. Paula is an experienced human resources professional with a history of success leading strategic and operational initiatives. She has worked with all types and sizes of organizations. Her specialties are in performance improvement, leadership development, employee engagement, and coaching leaders.

Paula has a Bachelor's degree in Business from Merrimack College and a Master's degree in Business Administration from the D'Amore-McKin School of Business at Northeastern University. Paula also served as Chairman of the Board for Cure SMA for five years. Prior to this she served as Secretary of the New England Chapter for 10 years. Paula lives in Massachusetts with her husband Mike and daughter Carolyn. Carolyn has type II SMA, recently graduated from Boston College and is working full-time in Boston

**Vanessa Battista, RN, MS, CPNP**, is a Pediatric Nurse Practitioner (PNP) on 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 previously worked at the SMA Center at Columbia University Medical Center in NY, and then at the SMA Center at Children's Hospital Boston, before relocating to Philadelphia. Vanessa is a member of the Cure SMA Medical Advisory Council (MAC).

**Kelli Blume**, is a School Social Worker at Grayslake Middle School in Grayslake, IL. She loves supporting her students at school and enjoys working with students from all different backgrounds and walks of life. She received her undergraduate degree from University of Illinois Champaign-Urbana and received her master's degree in Social Work from University of Illinois at Chicago. She has helped volunteer at the Sibling Workshop during the past few SMA conferences. She looks forward to the Sibling Workshop at the conference every year, and loves getting to know the siblings of SMA.

**Anne Buckley-Reen**, is an Occupational Therapist and Yoga therapist who has been developing yoga sequences to address the needs of special populations for the past 17 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 Cure SMA Conference.

**Terri Carry, PT**, is a physical therapist at Children's Hospital Colorado. Her entire career has been as a pediatric physical therapist with special interest in neuromuscular patients. She has been the physical therapist in the Neuromuscular Clinic at Children's Hospital Colorado since it was first established more than 30 years ago. She is currently involved as a clinical evaluator for numerous clinical trials for both SMA and Duchenne Muscular Dystrophy. She also works in the inpatient rehabilitation unit as well as the NICU.

**Kimberly Cook**, co-founder of ACT, initially began her career in the private sector. It was her journey as a mother that ultimately guided Kimberly to her true passion — special education. Blessed with 2 beautiful boys — now grown adults — her eldest son was born with Spinal Muscular Atrophy. A lifelong learner at heart, Kimberly set out on a mission to gain a greater understanding of the resources available to ensure that her son would have access to the same opportunities as every other child. The challenge and reward of the journey motivated Kimberly to advocate for other families in similar circumstances through a career in special education. Kimberly's passion and energy inspires and encourages educators and students alike. Her sons each lead successful, independent lives. Both children graduated with degrees in architecture from the University of Texas at Austin. Kimberly lives the motto — providing all children, regardless of ability, the opportunity to have access to grade level curriculum in the least restrictive environment.

As a special education professional, Kimberly focuses on access to the curriculum for all learners. She serves educators, students, and parents through her work with Access Curriculum Together and as an adjunct instructor at Texas A&M University in Corpus Christi. She holds a Bachelor of Science in Advertising from the University of Texas in Austin and a Masters in Educational Administration and Mid-Management from Texas A&M University in Corpus Christi. She worked as a member of the Texas Assistive Technology Network and the Texas Low Incidence Disability Network as a consultant with the Education Service Center, Region 2. Her diverse experience includes teaching in a general education and special education environment, teaching students with dyslexia and autism, and serving as a federal programs director. Kimberly participated in Harvard Graduate School of Education's professional education course, Universal Design of Learning: New Directions for Teaching Learners with Diverse Needs, where she gained a true understanding of UDL. Kimberly's primary areas of interest include assistive technology, access to curriculum, and low incidence disabilities. Kimberly lives in Corpus Christi with her husband Kresten and dog, Cooper.

**Tina Duong, MPT, PhDc**, is a physical therapist at Stanford University in Stanford, CA. She is also a member of the Cure SMA Medical Advisory Council.



**Bakri Elsheikh, MBBS, FRCP**, is an Associate Professor of Neurology at the Ohio State University Wexner Medical Center. He is board-certified in Neurology, Neuromuscular Medicine and Clinical Neurophysiology. He is the medical director of the EMG Laboratory, the Clinical Neurophysiology Fellowship and the Adult SMA clinic at OSU. His research focus is on the outcome measures and treatment of adults with SMA.

**Albert Freedman, PhD**, 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 is a member of Cure SMA's Medical Advisory Council. Dr. Freedman's son, Jack, was born in 1995 and is affected by SMA Type I.

**Robert Graham, MD**, 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.

**Jaclyn Greenwood**, is from Southern California and was diagnosed with SMA type II at 18 months. She graduated summa cum laude from Chapman University with a BS in Molecular Biology. She went on to earn a masters of science degree in genetic counseling from the University of California, Irvine in 2012. As a certified genetic counselor, she currently works for the California Department of Public Health as a coordinator for their Prenatal Screening Program. Despite life's physical obstacles, Jaclyn lives a full life and enjoys driving a modified van, traveling, and rooting for the local Ducks hockey team. Jaclyn and her family have been involved with Cure SMA since first diagnosed in 1990 and have found tremendous support from the organization. She is excited to be a part of Cure SMA and their effort to expand their SMA adult community outreach.

**Anne Hegberg**, is the senior clinician in the Driver Rehabilitation Program at Marianjoy Rehabilitation Hospital, part of Northwestern Medicine in Wheaton, IL. She has over twenty-five years of experience working with clients of all ages with visual, motor and/or cognitive impairments. With adapted vehicles including two wheelchair accessible minivans, the needs of the clients with severe physical impairments can be met. Anne has presented locally and nationally about all aspects of driver rehabilitation. She has authored the adaptive equipment chapter in the AOTA publication "Driving and Community Mobility".

**Kimberly Hill**, is 34 years old from Oklahoma and she started showing signs of SMA Type II at 10 months old. She has a trach, uses a ventilator full time, and can only use her left thumb herself. Thanks to her family, support network, and assistive technology she has a Bachelor's degree in political science and will soon have a Master's degree in FEMA, both from Oklahoma State University. She works for the Oklahoma Department of Emergency Management as their Disability Integration Specialist. She started attending the Cure SMA Conference in 2014 and this is her first year to be a presenter, which she is excited about.

**Renee Roy Hill**, has provided therapeutic assessments and program planning for adults and children with oral placement, feeding and motor speech deficits for over 17 years. She is the owner of Crossroads Therapy Clinic in New Braunfels, TX and a member of the TalkTools® speakers bureau. Renee has been an invited speaker for ASHA state conventions and has received specialized training in speech/oral-motor/feeding therapy, Apraxia, sensory processing disorders, Hanen Courses, NDT training, TAMO therapy and PROMPT. She is the creator of the TalkTools® Schedule Board Kit, co-author of Ice Sticks, and author of the TalkTools® Apraxia Program.

**Stevie Hopkins**, a Chicagoland native, is a lifelong entrepreneur with an expansive background in the music business, finance industry and web startup world. He owns the music merchandise company Second City Prints and is the co-founder of 3E Love, a disability awareness lifestyle brand he created with his sister, Annie. Both born with Spinal Muscular Atrophy Type II, Stevie and Annie founded 3E Love in 2007 to help change society's perception of disability and provide tools for others to embrace diversity.

**Becky Hurst Davis MS, RD, CSP, CD**, is a registered dietitian working with Intermountain Health Care in Salt Lake City, UT. She graduated with her Masters of Science in Nutrition from the University of Utah. Becky is a certified specialist in pediatric nutrition as well as a certified nutrition support clinician. She has great interest in clinical research providing improved nutrition care to people with neuromuscular diseases and has been involved with Cure SMA for over 9 years.

**Richard M. Kravitz, MD**, is a 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**, 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 and professor 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 since 1986. Her work in pediatric neuromuscular care, and spinal muscular atrophy in particular, spans nearly 30 years.

**Oren Kupfer, MD**, is a Pediatric Pulmonologist and Inpatient Medical Director at the Children's Hospital Colorado Breathing Institute and Assistant Professor of Pediatrics at the University of Colorado School of Medicine. His passion is the respiratory care of children with SMA and other neuromuscular disorders, developing evidence-based clinical care guidelines for respiratory illnesses and post-operative care, and improving quality of life and survival in SMA. He is a member of the Cure SMA Medical Advisory Council.

**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 May, 2001. For the past 14 years, she has been a member of the Pediatric Advanced Care Team, the palliative care service at the hospital, working with patients and siblings. The majority of her clinical work occurs in the homes of families who are receiving home care and/or hospice treatment for their child.

**Tina Lewis**, joined the Cure SMA Community in 2007 after her son, Julian, was diagnosed with SMA at the age of 3. Immediately after her son's diagnosis, Tina became a parent advocate for her son and other children with SMA. Her continuous advocacy at the preschool, elementary and middle school levels has ensured a positive impact on her son's educational success. Tina is an active member of the development of her son's Individualized Education Plan. Educating Julian's team of physical therapists, occupational therapists, physical education teachers, classroom teachers, and administrators about his physical needs has been essential in ensuring equitable access to the learning environment. At the broader level, Tina has supported families through Cure SMA, as they work to ensure the same experiences for their own children. For the past 22 years, Tina has been an educator in the state of Maryland. As an elementary classroom teacher, reading specialist and now reading support teacher, she has a broad range of experiences working with families and students who have IEP's. Her firsthand experiences as an educator have made a direct impact on her son's progress in school. It is her passion to ensure that all children are provided an environment that maximizes their potential for learning.

**Carolyn Long, LSW**, is the bereavement social worker at The Children's Hospital of Philadelphia (CHOP). Carolyn provides clinical grief therapy to families whose children have died. She has presented extensively on Palliative Care and Bereavement topics. She has experience and knowledge both in adult and pediatric end of life. Carolyn is certified yoga instructor and practices yoga extensively. She believes yoga is an excellent way to manage stress, build physical strength and increase wellness and personal happiness.

**Srin Madipalli**, is a technology entrepreneur with SMA (Type II) who founded an accessible travel startup, Accomable, that was acquired by Airbnb. He now leads Airbnb's efforts to make the platform more accessible to travelers with disabilities. Srin has degrees from Oxford University and Kings College London; and before becoming an entrepreneur, he was a corporate lawyer at a major international law firm. Srin has travelled extensively around the world and is regarded as a leading expert on the areas accessible travel and technology.

**Jennifer Martyn, PT**, studied physical therapy at the University of Washington, graduating in 1995. Her first job at Mary Bridge Children's Hospital in Tacoma plunged her into aquatics doing a group physical therapy program for children with a variety of diagnosis. Additionally, she provided clinical based physical therapy and staffed the regional MDA clinic. In 2001 she began Wave Therapies where she works with both adults and children in a warm water environment with a focus on improving strength and range of motion with a focus on improving functional skills. When not in the water Jennifer loves being with her family, biking, kayaking, gardening and sewing.

**Melissa Milinovich**, is from Amelia, Ohio and was diagnosed with Spinal Muscular Atrophy at the age of 2 (strong type I / weak type II) 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 graduated this year from University of Phoenix with Master's of Information Systems. From being a career woman with Hewlett Packard Enterprises as an Asset Manager in the USPS Division to a single mother of her daughter, Claudia, Melissa strives to reach the high goals she has set for herself. She never allows anyone to tell her she "can't" do something; she figures out how to achieve it. Despite using a wheelchair for full-time for mobility, she lives a very fulfilling life and encourages others to do the same. In her free time, she serves on the National Board of Directors for Cure SMA, assists her daughter's school with administrative and fundraising tasks, assists her MDA office with Summer Camp, speaks at many events to advocate for the rights of people with disabilities, and is the board chairman of the Accommodations Committee for the 2017 ReelAbilities Film Festival.

**Jennifer Miller-Smith**, is the mother of three, including two disabled daughters. Katie is 22 and has Chiari 1 Malformation and Post-Concussion Syndrome. Zach is 20 and unaffected. Madison is 11 years old and has SMA type 2. Just two days before Madison's first birthday, her parents got the diagnosis that Madison had SMA type 2. Due to the deterioration of Madison's muscles, she was never able to crawl or walk. Her parents, Jen and Aaron Smith, knew they could not stand by and watch their child wither away or wait around for a cure, so they took action. Jennifer started fundraising and founded the South Florida Chapter of Cure SMA and the Gala of Hope which in 7 short years raised over 1.3 million dollars. Jennifer has spent countless hours lending emotional support to parents of newly diagnosed, educating hospitals and the medical community, as well as raising funds and awareness for SMA. She also currently runs SMA Support System, the largest online forum for Spinal Muscular Atrophy worldwide.

**Thomas H. Murray, PhD**, is President Emeritus of The Hastings Center. He served as President and CEO of Hastings from 1999 until 2012. Prior to returning to Hastings, he was Director of the Center for Biomedical Ethics at Case Western University School of Medicine and Susan E. Watson Professor of Bioethics (1987-1999). From 1996 through 2001 he served as Presidential appointee on the National Bioethics Advisory Commission and as chair of its Genetics subcommittee. He serves on many editorial boards and has been president of the Society for Health and Human Values and the American Society for Bioethics and Humanities. He served as Chair of the Ethical Issues Review Panel for the World Anti-Doping Agency. He is currently a member of the Disciplinary Tribunal and the Independent IAAF Ethics Board and Vice Chair of Charity Navigator. Dr. Murray has testified before many Congressional committees and is the author of more than 250 publications including *The Worth of a Child*. His latest book, *Good Sport*, on values, drugs and sport, was published by Oxford University Press in January. In 2004 he received an honorary Doctor of Medicine degree from Uppsala University, the Henry Knowles Beecher Award from The Hastings Center in 2012, and the Patricia Price Browne Prize in 2013.

**Leslie Nelson, PT, PhDc, OCS**, is an Assistant Professor in the Department of Physical Therapy at University of Texas Southwestern in Dallas Texas. She has a broad background in physical therapy with a focus, over the past 12 years, on infants and children with neuromuscular disorders and muscle diseases. In addition to her clinical expertise, she has been involved in both investigator-initiated grants and industry sponsored clinical trials for studies involving neuromuscular disorders. Leslie recently started serving as a mentor in the newly founded Cure SMA Therapist Mentoring Program. She is currently pursuing her PhD with a focus on functional outcomes for infants with SMA.

**Garey Noritz, MD**, is an Internist and Pediatrician at Nationwide Children's Hospital. He is the director of the Division of Complex Health Care, 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. 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 Council of the Council for Children with Disabilities of the American Academy of Pediatrics. 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.

**Bradley Nunemaker**, was diagnosed with SMA Type III when he was nine years old. He joined the Cure SMA Board of Directors in 2012 and serves as Treasurer. Brad received his BBA from the University of Michigan Business School. He is currently the Executive Vice President, Finance – Health & Wealth Solutions at Alight Solutions and is a certified public accountant. Brad previously served as Treasurer on the Board of Directors for Onward Neighborhood House, a community based not-for-profit in Chicago. He is excited to continue to build the strong community of adults with SMA and to continue to expand awareness. In April 2017, Brad was a panelist in the Patient-Focused Drug Development meeting with the FDA, and shared his experiences on the impacts of living with SMA. In addition to chairing the Finance Committee on the Board of Directors of Cure SMA, Brad also is a member of the Adults with SMA Subcommittee. Brad and his wife Krista live in Elmhurst, Illinois with their two sons. The whole family is proud to support Cure SMA.

**Katlyn O'Brien**, is a 29 year old graduate from Western Illinois University. Katlyn has a degree in Elementary Education and is currently working as a 4th grade teacher at Park School Campus in Round Lake, IL. She has also successfully supervised and coordinated the Sibling Workshop during the last few SMA conferences. Katlyn has a 29 year old sister named Shannon who is affected with SMA Type III. Having a sibling who is affected with SMA presents endless rewards and challenges. It is her goal to reach out to other siblings by allowing them the opportunity to express themselves and support each other through common experiences and interactions.

**Kevin O'Brien**, 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 34 years in both technical and sales roles. Currently he is in a sales role in the transportation sector. Kevin is the father of Shannon O'Brien, a 31 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 26 years.

**Traci O'Brien**, is an SIU and Grenoble Ecole de Management alumni. She spent 1.5 years in the Southeast of France studying International Business/ Marketing where she lived with locals and traveled in Europe and Africa. She is proud of surviving graduate school twice and for co-founding SIU's Women's Ultimate Frisbee team. A very special part of her heart is reserved for these conferences, she loves empathizing with other siblings and having dance parties with all in a different city each year! She enjoys playing Nintendo and listening to music with her big sis, Shannon O'Brien. She's been volunteering ever since being released from child care. Cure SMA is like family to her, "I am grateful to be a part of this diverse community all bonded by SMA; we get angry, we laugh, we cry, but through it all, we know we're not alone."

**Kendra Paker PT, DPT, PCS**, graduated in 2002 from the University of Puget Sound Physical Therapy program. She has been interested in aquatics since the age of 3, and was both a competitive swimmer and swim instructor before becoming a physical therapist. Kendra has worked in pediatrics for Mary Bridge Children's Therapy Services since 2003, incorporating aquatic therapy into her practice with individual clients, and served as the pool program manager for the clinic. Additionally, she has worked with an adaptive swim program for children with special needs and their parents at the local YMCA for the last 10 years. Outside of physical therapy and the pool, Kendra enjoys spending time with her family, traveling, hiking, cycling, and reading.

**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 20 years, and is a clinical faculty associate the Doctor of Physical Therapy program at the University of Wisconsin-Madison. She is pediatric physical therapist for the UW American Family Children's Hospital Neuromuscular Disease clinic, including SMA patients of all types. She also continues to be involved in research at the University of Wisconsin American Family Children's Hospital.

**Samuel Rosenfeld, MD**, is the director of the Neuromuscular Clinics at CHOC Children's 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, Fellow of the American Academy of Pediatrics, and Fellow of the American Orthopaedic Association. Dr. Rosenfeld has been on staff at CHOC Children's 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 Cure SMA. Dr. Rosenfeld is a member of the Pediatric Orthopaedic Society of North America, American Academy of Pediatrics, American Orthopaedic Association, AACPDM, ACPOC, COA, WOA, CMA, and Orthopaedic Rehabilitation Association.

**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 of: New York State Bar Association; National Society of Financial Services Professionals; National Association of Insurance and Financial Advisors; Chairman, National Board of Directors, Cure SMA; Greater New York Chapter, Cure SMA; 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, 13 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.

**Sara B. Rubinstein, CO**, has been a staff orthotist at Ann & Robert H. Lurie Children's Hospital of Chicago for eight years. Sara currently focuses most of her clinical time in multi-disciplinary clinics treating patients with myelomeningocele and various forms of neuromuscular disease. She has spoken at both the American Academy of Orthotists and Prosthetists and the Association of Children's Prosthetic-Orthotic Clinics conferences, and assists in orthotics labs at the Department of Physical Therapy and Human Movement Sciences at the Feinberg School of Medicine at Northwestern University.

**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.

**Peter Schochet, MD**, is a board certified pediatric pulmonologist who has been dedicated to the respiratory management of children with neuromuscular disease. He is a Clinical Assistant Professor of Pediatrics at UT Southwestern. He has been an active member of the neuromuscular team at Children's Health Medical Center Dallas since 1996. He is actively involved in the care of SMA patients undergoing scoliosis surgery at Texas Scottish Rite Hospital for Children. He is committed to non-invasive ventilation and management of patients with neuromuscular weakness. He has lectured on the medical management of patients with SMA. He prefers a collaborative style of medical management with open communication with team members, patients and their families.

**Mary Schroth, MD**, is the Cure SMA Medical Director and chairs the Cure SMA Medical Advisory Council. She is also a Pediatric Pulmonologist and Professor of Pediatrics at the University of Wisconsin School of Medicine and Public Health in Madison. She is co director of the UW Health American Family Children's Hospital Neuromuscular Disorder Clinic and Director of the UW Pediatric Pulmonary Center Training Grant.

**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 17 years. She specializes in providing medical nutrition therapy to infants, children, and teenagers with pulmonary and neuromuscular disorders, including patients who eat orally and those who do not. She is continuously trying to understand the role diet plays in the treatment of SMA.

**Rachel Sepe**, is the mother of three children, Isabella (13), Gavin (9) and Scarlett (7) and currently is a small business owner. Prior to Scarlett's diagnosis of SMA Type II, Rachel was an elementary school teacher with a Master's degree in Education with focuses in Health Education and English Language Learners. While teaching for 18 years, she worked with school specialists to create IEP and 504 plans for students with special needs. Currently, she lends advice and support to parents navigating the school system using both her education background as well as her daughter's journey in the public-school system. She and her husband, Mike, live in Barrington, Illinois.

**David Sereni, MPT**, is a Senior Physical Therapist and Clinical Instructor at Kaiser Permanente in Santa Rosa, California. He is also part of the Adjunct Faculty, teaching Adapted PE at Santa Rosa Junior College. He has been the President of the Northern California Chapter of Cure SMA for 18 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.

**Linda Shively**, a Certified Laughter Yoga Leader, shares the power of choosing to laugh for physical and emotional health and well-being. After the death of her almost 4-year old daughter Jessica, SMA Type I, in 2005, Linda has become passionate about inspiring others and helping people move beyond their grief to re-discover their joy on the other side. Linda has helped hundreds with their grieving process and has touched the lives of thousands of individuals, families, and organizations through her speaking, coaching, and service. Holding a B.S. in Psychobiology from UCLA and certification as a Neuro-Linguistic Programming (NLP) Master Practitioner, Linda loves learning how the brain and mind function. As a Certified Master Life and Executive Coach, she helps people get unstuck in their business and life, especially from their grief and loss, so they can live the lives they are meant to live. In 2003, Linda founded the Northern California Walk-n-Roll which has raised well over \$840,000 for Cure SMA.

**Louise Simard, PhD**, is a Professor and Chair of the Department of Biochemistry & Medical Genetics in the Faculty of Health Sciences at the 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 2008 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 Cure SMA's booklet entitled “The Genetics of Spinal Muscular Atrophy” which has now been translated into several different languages.

**Brian D. Snyder, M.D., Ph.D.**, is Professor of Orthopaedic Surgery, Harvard Medical School and Research Professor of Bioengineering at Boston University, Department of Bioengineering. An attending orthopaedic surgeon at Boston Children's Hospital, his clinical practice focuses on congenital and acquired deformities about the hip and spine related to neuromuscular conditions as well as pediatric trauma. As a clinician-scientist funded by NIH, DoD, private foundations and industry, his translational research focuses on improving the practice of orthopaedic surgery by applying engineering principles to solve clinical problems. In recognition for his translational research, Dr. Snyder received a Kappa Delta Award from the American Academy of Orthopaedic Surgeons, the Russell Hibbs Award from the Scoliosis Research Society and the A. Clifford Barger Excellence in Mentoring Award from Harvard Medical School. Dr. Snyder is member of the board for Cure SMA.

**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).

Anne Stratton, MD, is a pediatric physiatrist (Rehabilitation doctor) at Children's Hospital Colorado. She is originally from Ohio and received her M.D. from the University of Cincinnati. She then completed dual residencies in Pediatrics and Physical Medicine and Rehabilitation at the University of Colorado, followed by a Pediatric Rehabilitation Medicine Fellowship. She has enjoyed being one of the pediatric physiatrists actively involved in the multidisciplinary Neuromuscular Clinic at Children's Hospital since 2010. She joined the Cure SMA medical advisory committee (MAC) in 2015. 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**, 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 10 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. For the past 3 years, she has been an active member of the Cure SMA Medical Advisory Council.

**Jane B. Taylor, MD, MsCR**, is a pediatric pulmonologist with a masters degree in clinical research. She joined the Department of Pediatrics at Children's Mercy Kansas City in 2009 after completing her training at Washington University in St. Louis. She is the Pulmonary Medical Director of the Multidisciplinary Muscular Dystrophy Clinic and the SMA Clinic at Children's Mercy. Dr. Taylor's research focuses on large database analysis and outcomes research. She collaborates closely with the Center for Infant Pulmonary Disorders (CIPD) and the Neonatal Home Ventilator Program at CMH. She is the site PI for the ChILD (Childhood Interstitial Lung Disease) Network. Dr. Taylor is also a board member for the local American Lung Association, advocating for pediatric lung health on both the local and national level.

**Anne DeMark Thompson, OT**, practices occupational therapy at the University of Wisconsin American Family Children's Hospital. She has specialized in pediatric occupational therapy for 25 years, working both with inpatients and outpatients ages 0-21 years of age. Anne enjoys working with children of all ages and diagnoses, and particularly enjoys being part of the multidisciplinary specialty clinics at the American Family Children's Hospital, including the Neuromuscular Disorders Clinic. Anne holds a Bachelor of Science degree in Occupational Therapy from the University of Wisconsin-Madison. She greatly values the opportunity to learn from her patients and families with SMA.

**Krista Torseth, PT, DPT**, has been at Wave Therapies since 2009, where she currently works with children and their families in the pool 2-3 days per week. She graduated from the University of Puget Sound in 2007 with her doctorate in physical therapy, beginning full time work in pediatrics, both outpatient and inpatient. Her work in aquatics began in 2008. She has extensive experience with infants, children and adolescents and extensive training in neurodevelopmental treatment and aquatics, receiving APTA's Certificate in Aquatic Physical Therapy Competency in 2015.

**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 led 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.

**Laura Watne, MS RD CSP**, is a Registered Dietitian and Board-Certified Specialist in Pediatric Nutrition. She joined the Children's Hospital Colorado multidisciplinary Neuromuscular Clinic team in 2011. Laura specializes in providing medical nutrition therapy for infants, children and adults with spinal muscular atrophy and a variety of other neuromuscular disorders. Laura has a passion for helping patients with SMA thrive and improve their quality of life through evidence-based nutrition plans. Her other areas of expertise include medical nutrition therapy for patients with spina bifida, spinal cord injury, Rett syndrome, as well as young athletes. She is excited to be a new member of the Cure SMA Medical Advisory Council.

**Brynne Willis**, is a graduate student at Johns Hopkins University pursuing a Masters in Clinical Mental Health. With this degree, she is tailoring her skills and knowledge to address the psycho-social effects of individuals and families living with chronic illness and disability. This stems from her personal experience in being diagnosed with SMA type 3 at the age of 10, which proved to be a significant challenge in childhood and adolescence. Brynne is currently conducting a psychological research study, "Self-Censoring Assessment: A Pilot Study on Individuals with Progressive Neurological Disease" in attempts to analyze the behavioral impact of living with invisible and visible disability brought on by SMA. Currently, Brynne is an intern at Safe Harbor Christian Counseling providing individual and group counseling using positive psychology as a primary intervention to mediate anxiety and stress. In her spare time, Brynne enjoys public speaking, participating in advocacy work to advance the rights of disabled individuals, shopping, and trail riding with her horses.

**Angela Wrigglesworth**, a third-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 founder of the Ms. Wheelchair Texas Foundation, was a sixteen-year member of the National Task Force on Public Awareness through MDA, and sits on the advisory 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."

**Ryan Wolff**, is father to Madison, type II (age 15) and Ella (age 11). Ryan has been attending conference since 2005 in which San Diego was his family's first conference. He enjoys helping and answering questions for SMA families, and being a part of the Annual SMA Conference each year.



# SPEAKERS

Thank you to each and every 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!



Make today a breakthrough.



## 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.



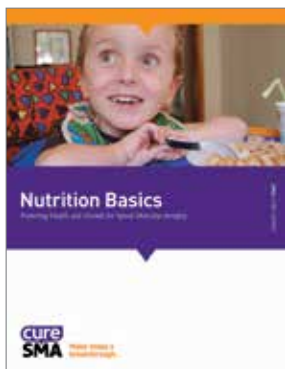
## PLEASE NOTE

### WEAR SUNSCREEN AND KEEP HYDRATED!

As many of you know, June in Texas is a very hot time. We strongly encourage everyone to take the proper precautions when outside for any amount of time, by applying sunscreen and covering your body as much as possible to avoid excessive sun exposure. Also, with the high temperatures and humidity, drink plenty of water to remain hydrated in this heat!

# Publications From Cure SMA

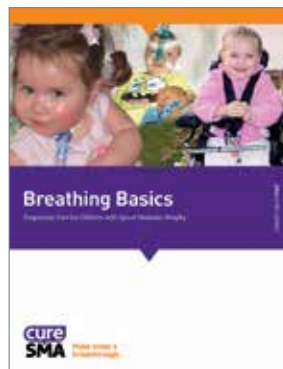
At Cure SMA, we're committed to making sure that families have the best, most accurate information about SMA and what it means for them, from day-to-day care to the changing landscape of research breakthroughs. Our care series booklets provide in-depth information on medical issues, genetics, and other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



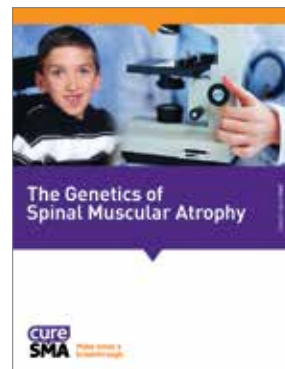
**Nutrition Basics**



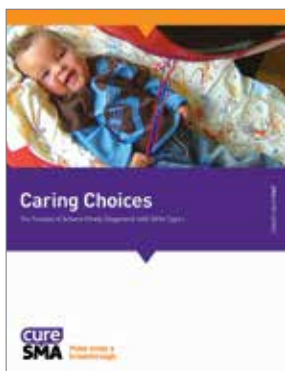
**Cure SMA Family Support and Patient Services**



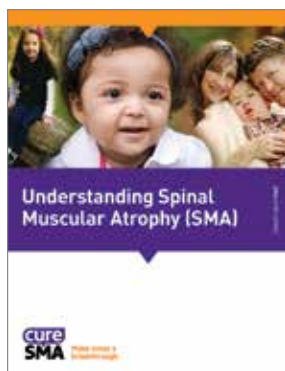
**Breathing Basics**



**The Genetics of Spinal Muscular Atrophy**



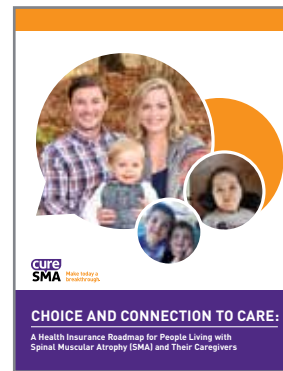
**Caring Choices**



**Understanding Spinal Muscular Atrophy (SMA)**



**Musculoskeletal System**



**Choice and Connection to Care: A Health Insurance Roadmap**

## For electronic copies:

Download this booklet from the Cure SMA web site at [www.cureSMA.org](http://www.cureSMA.org). Go to the support & care publications section on our website.

## For print copies:

Please contact the Cure SMA national office at [info@curesma.org](mailto:info@curesma.org).

**If you would like a hard copy mailed to you please email us at [info@curesma.org](mailto:info@curesma.org) or call 800.886.1762**

## Disclaimer:

*Cure SMA does not, as an organization, support or endorse any particular treatment or therapy. Information contained in this booklet is for informational and educational purposes only. All medical information presented should be discussed with a qualified physician.*



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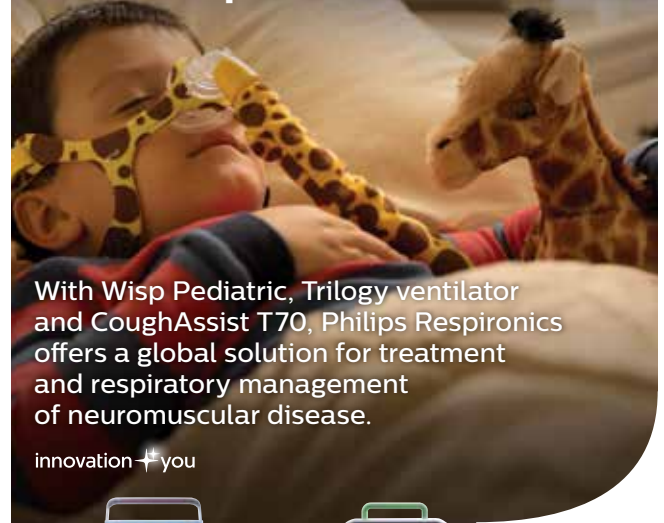
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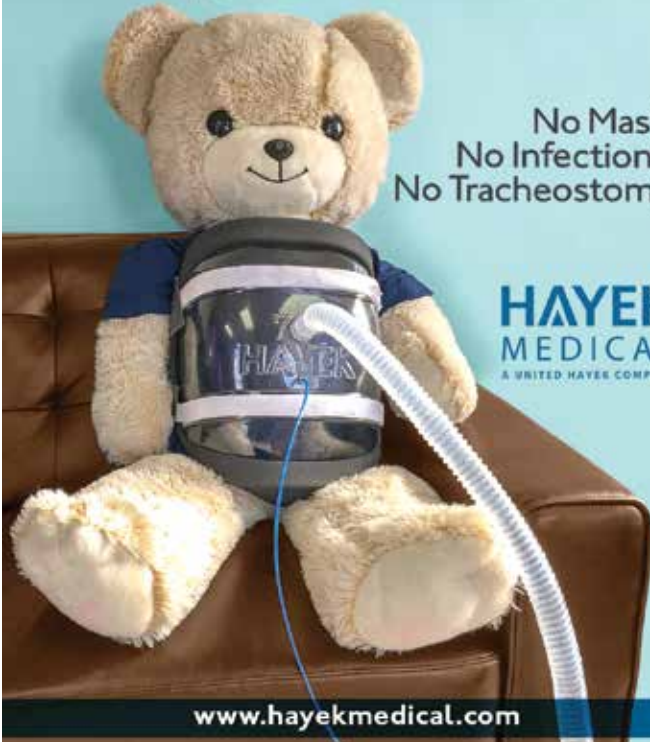


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# cure SMA 2018

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**Cure SMA Adjustable**

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

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



**Cure SMA Unisex Full Zip Hoodie | Cost: \$49**

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



**Cure SMA Unisex and Youth Baseball Tee | Cost: \$25**

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



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

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



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

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



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

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



**Cure SMA Unisex Orange V-Neck Tee | Cost: \$24**

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



**Cure SMA Grey Crew Neck Tee | Cost: \$24**

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



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

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



**Cure SMA Men's Performance Half Zip | Cost: \$49**

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



**Cure SMA Women's Performance Half Zip | Cost: \$49**

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

# Cure SMA Board of Directors

Cure SMA holds itself accountable to people with Spinal Muscular Atrophy and their families, as well as to its donors and volunteers, and also to the general public. The Board of Directors 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 M. Rubenstein – Chairman of the Board

Richard is Attorney and Financial Advisor at Strategies for Wealth based in greater New York metropolitan area. His expertise is in Financial and Estate Planning. Richard has significant experience planning for persons with special needs, and is a resource for our community lecturing at the National Conferences each year on the subject. He was born and raised in the Town of Yorktown Heights in Westchester County, New York, where he currently resides with his wife Michele and daughter Emma. His experience with SMA began February 2006 when his son Max was diagnosed with SMA. 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 the local chapter events, makes significant efforts to raise funds for the charity, and attends 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, Max's cherished memory, and his esteem for the Cure SMA community, that drives his desire and inspires him to contribute.

## DEVELOPMENT AND MARKETING COMMITTEE



## Cathy Barsotti-Dooling – Committee Member

Cathy and her husband Matt discovered Cure SMA after their daughter Annie was diagnosed with SMA when she was three and a half years old (Oct 2016). She is a graduate of UC Berkeley, with a degree in Social Science / International Relations. Cathy has over 24 years experience in sales and marketing with Royal Cruise Line (nine years), Disney (three years), Travelocity (seven years). She is currently responsible for the marketing and sales of Barsotti Family Juice Company (six years), established by her parents over 40 years ago. She is also an Ambassador for the Produce for Better Health organization. Cathy, Matt, and Annie live in Walnut Creek, CA. They are thankful for everything the Cure SMA organization is doing to advocate on behalf of patients and families. Cathy is committed to working with Cure SMA to promote awareness and raise funds to support research leading to more treatments and ultimately a cure.



## Douglas Erwin – Committee Member

Douglas and his wife, Michele discovered Families of 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. 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.



## Matt Evans – Board Member and Chair of Development and Marketing Committee

Dr. Evans is a director within the genetics research and development department of Quest Diagnostics. Matthew's first exposure to the SMA community was in 2005 through his graduate thesis research at the University of Massachusetts Medical School where he received his Ph.D. in Molecular Biology. The entirety of his graduate work was focused on SMA and understanding the mechanism of action of pre-clinical trial therapeutic targets. Upon leaving UMass Medical and starting a career with Quest Diagnostics Matthew's work evolved to enhancing the carrier and diagnostic testing experiences with SMA and other neurological disorders. Matthew with his wife Kathleen live in Central Massachusetts with their three young girls. He is thankful for all the years that he has been able to remain connected to the CureSMA Organization and the entire SMA community and excited for continued involvement.



#### **Rob Lockwood – Committee Member**

Rob and his wife Ann, son Christopher and daughter Grace live in the Cincinnati area. Rob works a district manager for a major pharmaceutical company and leads a sales team that markets vaccines that prevent vaccine preventable diseases for children, adolescents, and adults. He has worked in the pharmaceutical industry for 27 years and has worked in a variety of sales, account management, and training positions. Rob's niece Emma and nephew Nick both have SMA. His brother Kevin and sister-in-law Beth founded the OKI chapter and host the Walk n Roll along with Bob (retired from Cure SMA Board in 2017) and Beth Lockwood (Rob and Kevin's parents). The chapter has raised over 1 million dollars to help find a cure for SMA. Rob hopes to contribute to the organization and serve as an active board member.



#### **Srin Madipalli – Committee Member**

Srin Madipalli is a former London-based lawyer and disabled entrepreneur, who has Spinal Muscular Atrophy (SMA) and uses a power wheelchair. In 2011, Madipalli took six months off to go travelling but grew frustrated with the difficulties of finding accessible accommodation (he would often arrive at so-called 'accessible' vacation rentals only to find steps to the front door). He quit his City law job, put himself through an MBA at Oxford Said Business School and taught himself to code in order to build the prototype for Accomable, which was launched in 2015. In November 2017 Accomable was acquired by Airbnb. Madipalli is now based at Airbnb's San Francisco office as Accessibility Product and Program Manager.



#### **Shannon Shryne – Board Member**

Shannon is the co-founder and Executive Director of Augie's Quest – dedicated to finding cures and treatments for ALS. Augie's Quest, under Shannon's leadership, has raised over \$65 million in the past 12 years. All funds raised by Augie's Quest support the work at the ALS Therapy Development Institute, the world's foremost drug discovery center focused solely on ALS. Shannon serves as their Vice President of Strategic Partnerships and a member of their Executive Team. Shannon has 25 years experience in non-profit development and leadership with expertise in corporate engagement, major gift solicitation, prospecting and cultivating donors, fundraising, and cause marketing. She spent over 20 years with the Muscular Dystrophy Association engaging national corporate partners resulting in more than \$200M for the Association. In addition to serving on the Development and Programs board of Cure SMA, Shannon serves on the Advisory Boards of the Kevin Turner Foundation, the California Fire Fighter Foundation, and Free the Music USA.



#### **Patrick Woodruff – Committee Member**

Patrick graduated from The University of Arkansas with a Bachelor of Science degree in business administration. He is currently a VP with Bank of the Ozarks out of Bentonville, AR. Patrick and his wife, Meredith were introduced to SMA in 2011 when their second son, Miller was diagnosed with a severe case of SMA type 1. Miller only lived 87 days and since then, they started The Miller McNeil Woodruff Foundation ([www.imwithmiller.com](http://www.imwithmiller.com)). Miller's foundation has raised over \$1,000,000 since inception and has helped fund numerous SMA clinical trials and crucial research. Since the FDA approved Spinraza, Patrick's personal focus is to make sure every hospital has mandatory prenatal screenings for SMA. He is honored to be a part of the Cure SMA board and will continue to help fund advocacy and awareness initiatives, along with finding a cure.

## **EVENTS AND FAMILY SUPPORT COMMITTEE**



#### **Carolyn Barrett – Committee Member**

Carolyn Barrett works at a Boston legal non-profit, supporting their professional development department. She holds a Bachelor's of Arts in Political Science from Boston College. Carolyn is passionate about promoting the engagement of adults with SMA on the national and local level. Attending the conference for as long as she can remember, Carolyn views this weekend as one of the highlights of her year. In her free time, she enjoys driving her fully adaptive car new places, exploring the outdoors with her service dog, Shadow, and spending time with friends.



#### **Yahnatan Lasko – Committee Member**

Yahnatan (Jonathan) Lasko and his wife Kristen discovered Cure SMA after their first child Max was diagnosed with SMA type 1 in 2013. Since then, Yahnatan's passion has been at the intersection of technology and disability. He founded the SMA Adaptability online community and introduced the SMA world to ATmakers, a group that brings "makers" and STEM students together with Assistive Technology users to solve real-world problems. Yahnatan has two engineering degrees from Johns Hopkins University. He works as a computer scientist in human language technologies.



#### **Catherine Martin – Committee Member**

Catherine Martin is a Senior Vice President and Assistant General Counsel at Citi in New York. Ms. Martin joined Citi in 2013 and acts as primary legal coverage for several of Citi's credit trading businesses and specializes in derivatives and structured products. Prior to joining Citi, Ms. Martin was an associate at Latham & Watkins LLP and a member of the firm's Finance Department. Ms. Martin received a J.D., magna cum laude, from Syracuse University College of Law and is a member of the New York Bar.



#### **Mary McHale – Committee Member**

Mary McHale has served on the Board since 2007 as both a Board and Committee member in Programs and Development. Mary and her husband Joe McHale are parents to Danny, 16 years old with Type II SMA and Robby, 13 unaffected. Mary is the co-Chair of Concert for a Cure, an annual gala which has raised over \$1.8 million dollars for Cure SMA. Mary holds a Bachelor's of Science degree from the University of California at Davis in Human Development and Communication. She received her Masters of Education degree at the University of Vermont in Higher Education Administration. Mary also worked for 10 years in corporate consulting in Change Management, Organizational Development, and Strategy with Changeworks, Inc. and Accenture. Today, as a Career Advisor with Collegial Services, Mary enjoys helping clients achieve their career goals.



#### **Amy Medina – Committee Member**

Amy graduated from Marian University with a Bachelors degree in Social Work. She has worked for Fond du Lac County Department of Social Services for 8 years as a CPS social worker. Amy became involved with Cure SMA in 2011, after her first child, Mateo, was diagnosed with type 1 at just 1 month of age. Amy's second son, Javier, age 2, was diagnosed with SMA via amniocentesis and began treatment at 12 days old taking part in a clinical trial. Amy recently gave birth to a daughter, Amelia, who was also diagnosed with SMA. Amy's passion is educating others on SMA which includes training the local EMTs on SMA care and having the local newspaper write articles on SMA.



#### **Shannon Zerzan – Board Member and Chair of Event and Family Support Committee**

Shannon Zerzan is a full-time mother and volunteer who became involved with Cure SMA shortly after her eldest son was diagnosed with SMA in 2010. In 2011, she and her husband Greg co-founded the annual Hope on the Hill Congressional Dinner in Washington, D.C. They strive to educate federal policymakers on issues of importance to the SMA community and mentor families following diagnosis and as they navigate treatment options. Prior to having children, Shannon was the director of operations for a Washington, D.C. based national healthcare trade association. She has also worked on political campaigns and for a civics education non-profit. Shannon lives in Alexandria, Virginia, with her husband and two sons.

## **NOMINATING AND GOVERNANCE COMMITTEE**



#### **Nick Farrell – Committee Member**

Nick is Vice President, General Counsel and Chief Compliance Officer at Hillenbrand, Inc. – a diversified industrial company headquartered in Indiana – where he focuses primarily on securities, mergers & acquisitions, compliance and public company governance matters. Nick and his wife were introduced to SMA in February 2012, when their second daughter Blake was diagnosed with Type II SMA. And it was just days later that they were introduced to CureSMA, with the arrival of their newly-diagnosed care package. Since then they have been proud to support CureSMA in any way possible, including local chapter activity and their own annual fundraiser, which has raised over \$100,000 for Cure SMA. Nick is honored to be a part of Cure SMA and it is his mission to capitalize on the recent momentum that the organization has generated to find a cure. Nick and his wife live in Cincinnati, Ohio, with Blake and her two sisters. In addition to Cure SMA, Nick also serves on the Boards of Trustees of two Cincinnati-based nonprofit organizations devoted to serving children and adults with disabilities. Nick received his B.A. from Wake Forest University and his J.D. from Vanderbilt.



#### **Brant Imperatore – Committee Member**

Brant is a trusted public policy advisor and advocate with well over a decade of experience serving financial services companies and investors. His training in the field began as Counsel for the House Subcommittee on Capital Markets, Insurance and GSE's from 2000 to 2002 where he was responsible for managing a variety of different legislative issues including the Commodity Futures Modernization Act, Terrorism Risk Insurance Act, and Sarbanes-Oxley. Excited about the opportunity to build a best-in-class consulting firm, Brant joined Cypress in 2009 after five years running the financial services practice at a large Washington lobbying shop. In addition to his client and issue management work at Cypress, Brant is responsible for the firm's legal and compliance issues, which includes maintaining its accreditation from Integrity Research for best practices in the political intelligence space. Brant received his MBA from the Darden School of Business at the University of Virginia, where he remains active in speaking to students about the intersection of business and public policy. He has also served as a faculty member for the Practising Law Institution on hedge fund regulation and has appeared on CNBC, Bloomberg and Fox Business to discuss current policy issues. Brant lives in Virginia with his wife, Jane, and their three children.



#### **Tom Murray – Board Member and Chair of Nominating and Governance Committee**

Thomas Murray, PhD, is President Emeritus at The Hastings Center. He was formerly the Director of the Center for Biomedical Ethics in the School of Medicine at Case Western Reserve University, where he was also the Susan E. Watson Professor of Bioethics. He serves on many editorial boards and has testified before many Congressional committees. Among other current posts, he serves as Vice Chair of Charity Navigator and a member of the Ethics Board for the International Association of Athletics Federations. He has been president of the Society for Health and Human Values and of the American Society for Bioethics and Humanities. Murray is the author of more than 250 publications. His newest book is *Good Sport: Why Our Games Matter – and How Doping Undermines Them*.



#### **Steven Ragland – Committee Member**

Steven discovered Cure SMA on the afternoon of February 13, 2008 - the day his then 20-month-old son 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 Kecker Van Nest & Peters 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 taught political science to high school students. Steven received his B.A. in government from Lehigh University in Bethlehem, Pennsylvania and his J.D. from American University, Washington College of Law in Washington, D.C. He lives in San Francisco with his wife and two children.



#### **Danyelle Sun – Board Member**

Dany graduated from UW-Milwaukee with a Bachelors and Masters degrees in Social Work. She went on to work for a community non-profit for eight years, eventually managing multiple mental health programs that served both youth and adults. She currently works for Milwaukee County, serving individuals experiencing mental illness and alcohol or drug addiction. Dany became involved with the Wisconsin Cure SMA Chapter shortly after her daughter, Ruby and then son, Landon, were both diagnosed in 2013. She co-manages a widely utilized online SMA Support group. Dany's passion is advocacy and empowerment for individuals and families experiencing challenges in life, especially those within the SMA community.



#### **Brynne Willis – Committee Member**

Brynne Willis is a graduate student at Johns Hopkins University pursuing a Masters in Clinical Mental Health. With this degree, she is tailoring her skills and knowledge to address the psycho-social effects of individuals and families living with chronic illness and disability. This stems from her personal experience in being diagnosed with SMA type 3 at the age of 10, which proved to be a significant challenge in childhood and adolescence. Brynne is currently conducting a psychological research study, "Perceived Stigma and Self-Censoring Behavior: A Phenomenological Study on Individuals Diagnosed with Spinal Muscular Atrophy" in attempts to analyze the behavioral impact of living with invisible and visible disability brought on by SMA. Currently, Brynne is an intern at Safe Harbor Christian Counseling providing individual and group counseling using positive psychology as a primary intervention to mediate anxiety and stress. In her spare time, Brynne enjoys public speaking, participating in advocacy work to advance the rights of disabled individuals, shopping, and trail riding with her horses.

## FINANCE COMMITTEE



### Edmund Lee – Board Member

Edmund and his wife Kwiyoum have been blessed with their daughter, Angie, with SMA type II. They lived in Naperville, IL and with the help of their whole community have hosted an annual fund raiser in the area for 10 years called Krya's Idea Angie's Hope. The fund raiser was started by an idea from one of Angie's best friends, Kyra Scadden when they were in second grade. Since then with the help of the community have raised a little over \$250,000 for Cure SMA. Edmund works for a group purchasing organization and has an MBA from the Wharton School with a finance major and a bachelor's degree in computer science from Seoul National University. The Lee family is committed to spreading awareness about this disease and hopes to be an example of how to live gracefully despite the disease.



### Bradley A. Nunemaker – Board Member, Treasurer, and Chair of Finance Committee

Brad, was diagnosed with SMA Type III when he was nine years old. He joined the Cure SMA Board of Directors in 2012 and serves as Treasurer. Brad received his BBA from the University of Michigan Business School. He is currently the Executive Vice President, Finance – Health & Wealth Solutions at Alight Solutions and is a certified public accountant. Brad previously served as Treasurer on the Board of Directors for Onward Neighborhood House, a community based not-for-profit in Chicago. He is excited to continue to build the strong community of adults with SMA and to continue to expand awareness. In April 2017, Brad was a panelist in the Patient-Focused Drug Development meeting with the FDA, and shared his experiences on the impacts of living with SMA. In addition to chairing the Finance Committee on the Board of Directors of Cure SMA, Brad also is a member of the Adults with SMA Subcommittee. Brad and his wife Krista live in Elmhurst, Illinois with their two sons. The whole family is proud to support Cure SMA.



### Peter Statile – Board Member

Pete Statile is an accomplished commercial, operational and financial executive. His executive and Board level experience includes a wide range of public and private industrial businesses with extensive operations in the North American, European, and Asian markets. Most recently, Pete has been President of Venator Enterprises, a private advisory group, providing strategic services focused on the executive management, leadership, operational and financial challenges of middle market companies. Mr. Statile also has extensive Board-level experience that continues to this day. He is a member of The Economic Club of New York and holds a Bachelor of Science degree in Accounting from Brooklyn College, Brooklyn, NY. He currently resides in Staten Island, New York with his wife.

## SCIENCE AND MEDICINE COMMITTEE



### Corey Braastad – Board Member

Dr. Braastad is the Vice President and General Manager of Genomics at Covance Drug Discovery, part of LabCorp. Corey has completed training, performed research, and developed programs in clinical trials, pharma research support, and clinical genetic diagnostic products. Dr. Braastad is a published author who has many years of experience in senior team leadership, lab operations, and R&D. Dr. Braastad is a member of the board of directors for the Spastic Paraplegia Foundation and Cure SMA. He is a Member of: The Human Variome Project; American Society of Human Genetics; American College of Medical Geneticists; American Academy of Neurology; American Society of Cell Biologists and Radiation Research Society. He has a Ph.D. in Molecular and Cellular Biology and Biochemistry from Brown University School of Medicine. He also has his B.S. in Biology - Magna Cum Laude from University of Massachusetts at Dartmouth.



### Robert Graham, MD – Committee Member

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.





#### **Jaclyn Greenwood – Board Member**

Jaclyn is from Southern California and was diagnosed with SMA type II at 18 months. She graduated summa cum laude from Chapman University with a BS in Molecular Biology. She went on to earn a Masters of Science degree in genetic counseling from the University of California, Irvine in 2012. As a certified genetic counselor, she currently works for the California Department of Public Health as a coordinator for their Prenatal Screening Program. Despite life's physical obstacles, Jaclyn lives a full life and enjoys driving a modified van, traveling, and rooting for the local Ducks hockey team. Jaclyn and her family have been involved with Cure SMA since first diagnosed in 1990 and have found tremendous support from the organization. She is excited to be a part of Cure SMA and their effort to expand their SMA adult community outreach.



#### **Doug Kerr – Board Member and Chair of Science and Medicine Committee**

Doug is a neurologist and neuroscientist with over 120 publications in medical journals. He has led the development of several therapies for neurologic and rare genetic diseases while working in the biotechnology industry. He has participated on the boards and SABs of several non-profit organizations, including the Transverse Myelitis Association, and the ALS Association. Doug has been actively involved in gene and cell therapy programs and is on the Scientific Review Panel of the California Institute of Regenerative Medicine. In August 2017, Doug joined Generation Bio, an Atlas Venture-backed gene therapy start up company in Cambridge MA. From 2015-2017, Doug ran the development activities of all the rare neuroscience programs at Shire, including the intrathecal programs designed to halt the CNS manifestations of lysosomal storage disorders as well as a series of programs in neurodegenerative disorders and gene therapy candidates. From 2009-2015, Doug was at Biogen. Doug was the global lead for a series of programs in Alzheimer's disease, Amyotrophic Lateral Sclerosis (ALS) and Spinal Muscular Atrophy (SMA). Doug led the team that developed the drug Spinraza, now approved for SMA.

Doug received his MD degree from Jefferson Medical College in Philadelphia, and his PhD in Biochemistry and Molecular biology also from Jefferson Medical College. Doug obtained his M.B.A. with a specialization in entrepreneurship and finance and graduated from Princeton with a degree in molecular biology.



#### **Kathy Klinger – Committee Member**

Katherine Klinger is Global Head of Translational Sciences. Dr. Klinger received her BA from Trinity University, San Antonio, TX and her Ph.D. in biochemistry at the University of Texas Health Science Center. Her postdoctoral fellowship was conducted at Case Western Reserve School of Medicine, Cleveland, Ohio. Dr. Klinger is ABMG certified in medical genetics and clinical molecular genetics and is a diplomate of the American College of Medical Genetics (ACMG). She has lectured nationally and internationally, is widely published in scientific journals and has written numerous book chapters. She serves on the board of several educational and humanitarian foundations, and is active in the review process for the National Institutes of Health.



#### **Brian Snyder – Board Member**

Brian is a Board Certified Pediatric Orthopaedic surgeon on staff at Children's Hospital, where he directs the Cerebral Palsy Center. His clinical practice focuses on spinal deformity related to congenital and neuromuscular etiologies, hip dysplasia and acquired deformities about the hip, cerebral palsy and pediatric trauma. In addition, he is Professor of Orthopaedic Surgery, Harvard Medical School and Director of the Orthopedic Biomechanics Laboratory at the Beth Israel Deaconess Medical Center. Dr. Snyder has been principal investigator of NIH/NCI RO1, NIH/NIAMS R21, NASA, DOD, private foundations (Whitaker, OREF, Susan B Komen, AO/ASIF, Coulter, POSNA, and SRS) and industry sponsored grants. In addition to his basic science research, Dr. Snyder has patented a unique modular spine instrumentation system to treat spinal deformity in children and adults; co-founded a company that is developing a polymer to supplement synovial fluid that minimize cartilage wear in early osteoarthritis and is developing technology for contrast enhanced, quantitative CT imaging of cartilage in synovial joints.



### **Charlotte Sumner, MD – Committee Member**

Dr. Charlotte J. Sumner is a Professor of Neurology and Neuroscience at Johns Hopkins University School of Medicine. She received her B.A. from Princeton University and her M.D. from the University of Pennsylvania School of Medicine. She cares for patients with inherited diseases of motor neurons and peripheral nerve and co-directs the Johns Hopkins Spinal Muscular Atrophy and Charcot Marie Tooth disease clinics. Dr. Sumner's research focuses on the genetic and cellular pathogenesis of SMAs with attention to identification of novel disease genes, characterization of molecular and cellular mechanisms underlying disease pathogenesis, and preclinical development of novel therapeutics. Her work has been supported by the National Institute of Neurological Diseases and Stroke, Howard Hughes Medical Institute, and disease foundations. She is an Associate Editor of the journal *Experimental Neurology* and is the co-editor of the only comprehensive book on SMA: *Spinal Muscular Atrophy Disease Mechanisms and Therapy*. She has also been advisor to several companies developing treatments for SMA and non-profit disease foundations including the Packard Center for ALS research, Muscular Dystrophy Association, Cure SMA, and the SMA Foundation.

## **PUBLIC ADVOCACY COMMITTEE**



### **Kelly Cole – Secretary and Board Member**

Kelly Cole is responsible for overseeing the CTIA Hill team and advancing the wireless industry's priorities before Congress. Most recently, Ms. Cole ran her own consulting firm, Kelly Cole Strategies, and was a consulting counsel for Wiley Rein. She has had a highly successful career in the private sector as well as public service. She served as the Executive Vice President of Government Relations for the National Association of Broadcasters where she led the association's lobbying efforts. Ms. Cole has also served as Majority Counsel for the U.S. House Energy and Commerce Committee managing communications and Internet issues. She has a Juris Doctor from Willamette University College of Law, a bachelor of arts in Political, Legal and Economic Analysis from Mills College and spent a year studying law at the London School of Economics. Ms. Cole is a member of the bar in the District of Columbia, Washington state and Oregon. Kelly has been actively involved in raising funds for the annual Cure SMA dinner in Washington, DC since its inception.



### **Annie Kennedy – Board Member**

Senior Vice President for Legislation & Public Policy at Parent Project Muscular Dystrophy (PPMD) Focused on improving health outcomes for people living with Duchenne muscular dystrophy, Annie's work includes building strong partnerships with policy makers, federal agencies, Industry, and alliances that can serve as force multipliers in moving Duchenne community priorities forward. Current areas of emphasis include PDUFA VI, implementation of key provisions within 21st Century Cures including the Patient Focused Impact Assessment Act, MD-CARE Act implementation, engagement with the FDA and Industry around regulatory policy and therapeutic pipelines, recent AdComms for Duchenne products, a national newborn screening program, resources for adults with Duchenne, optimizing clinical trial infrastructure, and drug coverage and access issues. Annie is currently honored to collaborate with many partner organizations including service on the Board of Directors of Cure SMA, Faster Cures Patients Count Leadership Council, as Co-Chair of the National Health Council's Medical Innovation Action Team, and as a Design Team member of the NCATS/ORDR Tool Kit Project.



### **Gillian Mullins – Board Member**

Gillian has spent her career working on behalf of the biopharmaceutical industry in various positions focused on patient advocacy and external affairs. Most recently, she served as the Director of Patient Advocacy for Biogen, a biopharmaceutical company based in Cambridge, MA. At Biogen, Gillian led patient advocacy relations for the company's rare disease therapeutic areas and was a core member of the team who developed and delivered SPINRAZA, the first treatment for Spinal Muscular Atrophy (SMA). Before joining Biogen, Gillian was the Managing Director of Alliance Development at the Biotechnology Innovation Organization (BIO), a national trade association representing approximately 1,100 biotechnology companies, located in Washington D.C. There she had oversight for BIO's external relationships with allied organizations, including: patient advocacy organizations, physician and payer groups, universities, venture capital organizations and state-based bioscience trade groups. Gillian received her B.A. in Political Science from Miami University located in Oxford, OH. She is a native of Washington, D.C. and resides there with her husband John and their two daughters Keaveny & Reid.



### **Spencer Perlman – Board Member and Chair of Public and Advocacy Committee**

Spencer Perlman is a Managing Partner and the Director of Healthcare Research at Veda Partners, which launched in March 2017. He has spent nearly 20 years working in healthcare policy in the public and private sectors in Washington, D.C. and has extensive experience analyzing federal regulatory and legislative actions affecting various areas of the healthcare sector. Earlier in his career, Mr. Perlman worked with a number of patient advocacy groups, healthcare providers, and professional societies to develop and implement advocacy campaigns, analyze health care and Medicare policies for client leadership and management, and lead complex grant writing projects. Mr. Perlman served as Cure SMA's representative in Washington, D.C. from 2005 – 2014. He resides in Bethesda, Maryland, with his wife and two sons.



### **Julie Riccio – Committee Member**

Julie B. Riccio joined PricewaterhouseCoopers as a Director in the Office of Government, Regulatory Affairs & Public Policy in 2014. In her current role, Julie represents the firm and its interests before Congress, the Executive Branch and federal regulatory agencies. Julie co-chairs the Federal Legislative Task Force's (FLTF) Regulatory Working Group and participates on the FLTF's EEOC and Oversight Working Groups. Julie manages the Public Policy team's strategy to engage Members of Congress on PwC's approach to diversity. In 2015, Julie launched a PwC program to help less tenured women Members of Congress establish leadership political action committees where they can use their funds to support other women, providing needed financial support to grow the pipeline of women candidates to run for Congress. The effort, aligned with the firm's broader strategy, is an important component of the firm's continued brand differentiation in Washington. Julie also represents the firm at the National Republican Congressional Committee's Women Advisory Group meetings. Julie started her career in public policy working as a legislative aide to former House Financial Services Committee Chairman Spencer Bachus (R-AL). Julie graduated from Auburn University. She is married and has three children.

## **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.



# Medical Advisory Council



## Mary Schroth MD

Mary is the Chief Medical Officer for Cure SMA. She brings 25 years of experience as a Pediatric Pulmonologist to Cure SMA and is Professor Emeritus at the University of Wisconsin School of Medicine and Public Health. As a specialist in SMA respiratory care and an educator, Dr. Schroth is a leader in the SMA community and with Cure SMA.



## Alison Ballard, RN, CPNP

Alison is a Senior Instructor in the Department of Physical Rehabilitation and Medicine at The Children's Hospital Colorado, in Aurora, Colorado. As a pediatric nurse practitioner, she has a focus on infants and children with neuromuscular disorders. She is particularly interested in improving the quality and care of patients with Spinal Muscular Atrophy. Alison has worked closely with Cure SMA Rocky Mountain Chapter since 2012.



## Vanessa Battista, RN, MS, CPNP

Vanessa is a Pediatric Nurse Practitioner (PNP) on 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 previously worked at the SMA Center at Columbia University Medical Center in NY, and then at the SMA Center at Children's Hospital Boston, before relocating to Philadelphia. Vanessa is a member of the Cure SMA Medical Advisory Council (MAC).



## William F. Bell, Jr., BS Pharm, MBA, MSCC, R.PH.

William "Bill" Bell is currently the Director of Clinical Client Services with Southern Scripts, a Pharmacy Benefit Manager serving self-funded employer groups. After receiving his Bachelor of Science in Pharmacy degree from Duquesne University in 1992, Bill practiced in various settings, such as community, academia, and long-term care. Since 2012, Bill has been employed in the Pharmacy Benefit Management industry, serving as both a Clinical Account Manager and Formulary Pharmacist for Medicare, Medicaid, and Exchange lines of business. As the current Director of Clinical Client Services with Southern Scripts, Bill is responsible for the clinical implementation and management strategy of over approximately 350 employer groups for which Pharmacy benefits are managed. In addition to these duties, Bill oversees the clinical department, consisting of both Pharmacists and Pharmacy residents. Bill's involvement and interest with SMA began in 2009, when Maureen became a child care provider to a newborn who was diagnosed with Type III SMA a few years later.



## John Brandsema, MD

Dr. Brandsema is a Child Neurologist and Neuromuscular Section Head at The Children's Hospital of Philadelphia. His main research focus is as a clinical trialist in pediatric neuromuscular disorders and his clinical interests include SMA, myopathies and myasthenia. This is his first year on the Medical Advisory Council.



## Cindy Budek, MS, APRN, CPNP-PC/AC

Cindy is a pediatric nurse practitioner specializing in neuromuscular and pulmonary medicine at Ann & Robert H. Lurie Children's Hospital of Chicago. Her clinical background at Lurie Children's includes over 30 years of practice in pediatric critical care and pulmonary habilitation, and experience in program development for the Transitional Care Unit and home ventilation program for children with chronic respiratory and neuromuscular disorders, including SMA. In 2015 she joined the neuromuscular team in a clinical and research support role and provides pulmonary care for children with SMA in the neuromuscular clinic. Her area of interest includes improving access to high quality care and addressing quality of life and transition of care issues for children and young adults with SMA and their families.



### **Diana Castro, MD**

Dr. Castro is a board certified Child Neurologist with additional training in Pediatric Neuromuscular Medicine and Clinical Electromyography. She trained at the University of Texas Southwestern (UTSW) from 2008 to 2011, before joining the Department of Pediatrics and Neurology as a faculty member, in September 2012. She is the Co-Director of the Pediatric MDA clinic at Children's Health in Dallas, TX and the site director for the Neuromuscular Fellowship training program at UTSW. Dr. Castro serves as site Principal Investigator and Co-Investigator in a number of clinical treatment trials for neuromuscular disorders in children. Dr. Castro is interested in advocating for patients with Spinal Muscular Atrophy and developing guidelines for better care of this population at the hospital level.



### **Jin Yun (Helen) Chen, MS, CGC**

Jin Yun (Helen) Chen, a certified genetic counselor in the Neurogenetic Unit at Massachusetts General Hospital. She sees patients in the multidisciplinary SMA clinic, and thus has gained experience in working with infants, children and adults with all types of SMA. She primarily provides genetic counseling and educational support for these families, as well as actively engaged in the SMA research studies. Prior to this, she was a genetic counselor in the Division of Genomic Diagnostics at Children's Hospital of Philadelphia where she gained expertise in variant analysis and report interpretation for both sequence variants and copy number alterations. In addition, she provided genetic counseling services in the Hearing Loss Clinic at CHOP. She received master's degree in Genetic Counseling from Brandeis University and bachelor's degree in Biochemistry from the University of Amherst in Massachusetts.



### **Shelley Coskery, RN, MSN, CRNP-AC**

Shelley Coskery is a pulmonary nurse practitioner at Children's of Alabama and started working with the neuromuscular clinic in September of 2014. As of 2017 she stepped into an outpatient full time position as the MDA Care Center Coordinator. She has an excitement for working with patients with SMA, developing access to new therapies, and serving as a site as sub-PI on a number of neuromuscular trials. This is her first year serving on the Cure SMA Medical Advisory Council.



### **Thomas Crawford, MD**

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



### **Tina Duong, MPT, PhDc**

Tina Doung, is a physical therapist at Stanford University in Stanford, CA. She is also a member of the Cure SMA Medical Advisory Council.



### **Bakri Elsheikh, MBBS, FRCP**

Dr. Elsheikh is an Associate Professor of Neurology at the Ohio State University Wexner Medical Center. He is board-certified in Neurology, Neuromuscular Medicine and Clinical Neurophysiology. He is the medical director of the EMG Laboratory, the Clinical Neurophysiology Fellowship and the Adult SMA clinic at OSU. His research focus is on the outcome measures and treatment of adults with SMA.



### **Albert Freedman, PhD**

Dr. Freedman is a child and family psychologist in independent practice in the Philadelphia area. 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. Dr. Freedman is a member of Cure SMA's Medical Advisory Council. His son, Jack, was born in 1995 and is affected by SMA Type I.



### **Melissa Gibbons, MS, CGC**

Melissa Gibbons earned her master degree in genetic counseling from the University of North Carolina Greensboro. She is an Assistant Professor at the University of Colorado/ Children's Hospital Colorado. Melissa has been a member of the Multidisciplinary Neuromuscular Clinic team at Children's Hospital Colorado for 11 years. During her time at Children's, she has provided counseling to families affected by SMA and co-founded the Neuromuscular Carrier Clinic to address the genetic education and testing needs for family members. She is a member of the Cure SMA Medical Advisory Board.



#### **Nancy Glass, MD, MBA**

Dr. Glass has been a pediatric anesthesiologist/intensivist/pain physician at Texas Children's Hospital since 1989, and is Professor of Pediatrics and Anesthesiology at Baylor College of Medicine. She currently practices pediatric palliative care at TCH, and pediatric hospice medicine at Houston Hospice, also in Houston. She sees patients in Neuropalliative Care Clinic with a pediatric neurologist, and through this relationship, began performing LPs for Spinraza injection in the neurology clinic, working with the MDA team to establish care protocols and standards. In her spare time, she is working on a Master's in Liberal Studies at Rice University, focusing on writing stories about her patient experiences.



#### **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.



#### **Jennifer Hubbell, OTR/L**

Jennifer is a Level II therapist in the OT/PT department of Cincinnati Children's Hospital Medical Center. She has been a pediatric therapist for 24 years and has experience in both outpatient and home health care. She has been working with families and children with SMA for 16 years and has developed a special interest in their care from diagnosis through adulthood. She has presented to peers and staff at Cincinnati Children's Hospital Medical Center as well as local schools and organizations in the areas of high risk infants, sensory processing, childhood development and therapy in the home.



#### **Becky Hurst Davis MS, RD, CSP, CD**

Becky is a registered dietitian working with Intermountain Health Care in Salt Lake City, UT. She graduated with her Masters of Science in Nutrition from the University of Utah. Becky is a certified specialist in pediatric nutrition as well as a certified nutrition support clinician. She has great interest in clinical research providing improved nutrition care to people with neuromuscular diseases and has been involved with Cure SMA for over 9 years.



#### **Chamindra Konersman, MD**

Dr. Konersman is an Associate Professor of Neurosciences at the University of California, San Diego (UCSD) and takes care of children and adults with SMA at UCSD and Rady Children's Hospital. She directs the multidisciplinary teams at the adult and children's hospitals and hopes to participate in more clinical trials that fundamentally alters the progression of disease and endeavors to deliver better care to these patients.



#### **Richard M. Kravitz, MD**

Dr. Kravitz is a 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**

Kristin 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 and professor 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 since 1986. Her work in pediatric neuromuscular care, and spinal muscular atrophy in particular, spans nearly 30 years.



### **Nancy Kuntz, MD**

Dr. Kuntz is a board certified child neurologist with additional board certifications in Pediatrics, 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 Pediatric Pulmonologist and Inpatient Medical Director at the Children's Hospital Colorado Breathing Institute and Assistant Professor of Pediatrics at the University of Colorado School of Medicine. His passion is the respiratory care of children with SMA and other neuromuscular disorders, developing evidence-based clinical care guidelines for respiratory illnesses and post-operative care, and improving quality of life and survival in SMA. He is a member of the Cure SMA Medical Advisory Council.



### **Khalida Liaquat, MS, LCGC**

Khalida Liaquat is a member of the CureSMA Medical Advisory Council and a licensed laboratory genetic counselor at Quest Diagnostics. Prior to joining the laboratory, Khalida served as a clinical prenatal, pediatric and adult genetic counselor in New York City. At Quest Diagnostics, Khalida specializes in molecular genetic testing for the Athena Diagnostics laboratory, focusing primarily on neuromuscular diseases. In her role in the laboratory, she works closely with clinicians in planning, testing and interpreting genetic results for SMA.



### **Diane Murrell, LCSW**

Diane 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 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 for a parent and/or child on their quality of life. In addition, Diane founded and manages the Houston Fireballs, the first power soccer program in Texas. The soccer program seeks to provide an athletic and team experience for those in power wheelchairs ([www.houstonfireballs.com](http://www.houstonfireballs.com)).



### **Leslie Nelson, PT, PhDc, OCS**

Leslie is an Assistant Professor in the Department of Physical Therapy at University of Texas Southwestern in Dallas Texas. She has a broad background in physical therapy with a focus, over the past 12 years, on infants and children with neuromuscular disorders and muscle diseases. In addition to her clinical expertise, she has been involved in both investigator-initiated grants and industry sponsored clinical trials for studies involving neuromuscular disorders. Leslie recently started serving as a mentor in the newly founded Cure SMA Therapist Mentoring Program. She is currently pursuing her PhD with a focus on functional outcomes for infants with SMA.



### **Julie A. Parsons, MD**

Dr. Parsons did her medical training at University of Colorado. She is board certified in child neurology. Dr. Parsons was in private practice from 1993 to 2000, then joined the faculty at University of Colorado School of Medicine where she is a Professor of Pediatrics and Neurology. Dr. Parsons was named the inaugural Haberfeld Family Endowed Chair in Pediatric Neuromuscular Disorders. She is principal investigator on a number of clinical trials for muscular dystrophy and spinal muscular atrophy. Dr. Parsons is Co-Director of the multidisciplinary Neuromuscular clinic at Children's Hospital Colorado.



### **Karen Patterson, MS, PT, PCS**

Ms. Patterson is a Faculty Associate with the University of Wisconsin Physical Therapy Program in Madison, Wisconsin. She has worked in pediatric physical therapy for 20 years, and is a clinical faculty associate of the Doctor of Physical Therapy program at the University of Wisconsin-Madison. She is pediatric physical therapist for the UW American Family Children's Hospital Neuromuscular Disease clinic, including SMA patients of all types. She also continues to be involved in research at the University of Wisconsin American Family Children's Hospital.



### **Silvana Ribaldo, MD**

Silvana Ribaldo, MD, is a board certified Obstetrician and Gynecologist at Columbia University Medical Center in New York City. Educated at Brown University Medical School, Dr. Ribaldo has been providing a range of gynecologic surgeries and obstetrical care since completing her residency in 2003 to patients from adolescence to menopause. Dr. Ribaldo is a Fellow of the American Congress of Obstetricians and Gynecologists and is the Medical Director of the Ob/Gyn outpatient services at the Herbert Irving Pavilion Medical Center. She is actively involved in resident and medical student teaching at Columbia University and serves on quality assurance committees to implement protocols to optimize care.



### **Samuel Rosenfeld, MD**

Dr. Rosenfeld is the director of the Neuromuscular Clinics at CHOC Children's 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, Fellow of the American Academy of Pediatrics, and Fellow of the American Orthopaedic Association. Dr. Rosenfeld has been on staff at CHOC Children's 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 Cure SMA. Dr. Rosenfeld is a member of the Pediatric Orthopaedic Society of North America, American Academy of Pediatrics, American Orthopaedic Association, AACPDM, ACPOC, COA, WOA, CMA, and Orthopaedic Rehabilitation Association.



### **Peter Schochet, MD**

Dr. Schochet is a board certified pediatric pulmonologist who has been dedicated to the respiratory management of children with neuromuscular disease. He is a Clinical Assistant Professor of Pediatrics at UT Southwestern. He has been an active member of the neuromuscular team at Children's Health Medical Center Dallas since 1996. He is actively involved in the care of SMA patients undergoing scoliosis surgery at Texas Scottish Rite Hospital for Children. He is committed to non-invasive ventilation and management of patients with neuromuscular weakness. He has lectured on the medical management of patients with SMA. He prefers a collaborative style of medical management with open communication with team members, patients and their families.



### **Richard Shell, MD**

Dr. Shell is a Pediatric Pulmonologist and Associate Professor 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. This includes involvement in the current therapeutic trials including gene therapy. 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**

Dr. Shieh 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 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 Neuro-muscular 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.





### **Edward C. Smith, MD**

Dr. Smith is a board-certified Child Neurologist with sub-specialty certification in Neuromuscular Medicine and Electromyography. He is an Associate Professor of Pediatrics at Duke University Medical Center in Durham, North Carolina where he co-directs the Duke Children's Neuromuscular Program. He provides clinical care for children and adults with SMA and is actively involved in clinical research.



### **Anne Stratton, MD**

Dr. Stratton is a pediatric physiatrist (Rehabilitation doctor) at Children's Hospital Colorado. She is originally from Ohio and received her M.D. from the University of Cincinnati. She then completed dual residencies in Pediatrics and Physical Medicine and Rehabilitation at the University of Colorado, followed by a Pediatric Rehabilitation Medicine Fellowship. She has enjoyed being one of the pediatric physiatrists actively involved in the multidisciplinary Neuromuscular Clinic at Children's Hospital since 2010. She joined the Cure SMA medical advisory committee (MAC) in 2015. 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 10 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. For the past 3 years, she has been an active member of the Cure SMA Medical Advisory Council.



### **Jane B. Taylor, MD, MsCR**

Dr. Taylor is a pediatric pulmonologist with a masters degree in clinical research. She joined the Department of Pediatrics at Children's Mercy Kansas City in 2009 after completing her training at Washington University in St. Louis. She is the Pulmonary Medical Director of the Multidisciplinary Muscular Dystrophy Clinic and the SMA Clinic at Children's Mercy. Dr. Taylor's research focuses on large database analysis and outcomes research. She collaborates closely with the Center for Infant Pulmonary Disorders (CIPD) and the Neonatal Home Ventilator Program at CMH. She is the site PI for the ChILD (Childhood Interstitial Lung Disease) Network. Dr. Taylor is also a board member for the local American Lung Association, advocating for pediatric lung health on both the local and national level.



### **Fred W. Troutman, PhD**

Dr. Troutman 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 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.



### **Laura Watne, MS RD CSP**

Laura is a Registered Dietitian and Board-Certified Specialist in Pediatric Nutrition. She joined the Children's Hospital Colorado multidisciplinary Neuromuscular Clinic team in 2011. Laura specializes in providing medical nutrition therapy for infants, children and adults with spinal muscular atrophy and a variety of other neuromuscular disorders. Laura has a passion for helping patients with SMA thrive and improve their quality of life through evidence-based nutrition plans. Her other areas of expertise include medical nutrition therapy for patients with spina bifida, spinal cord injury, Rett syndrome, as well as young athletes. She is excited to be a new member of the Cure SMA Medical Advisory Council.

# Cure SMA Scientific Advisory Board (SAB)

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.



## Jill Jarecki, PhD, Chief Scientific Officer

Dr. Jarecki oversees 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.



## Jackie Glascock, Ph.D., Senior Scientific Program Manager

Dr. Glascock serves as the coordinator of the Cure SMA Scientific Advisory Board. Prior to joining Cure SMA, Dr. Glascock studied translational therapies for SMA in graduate school at the University of Missouri.



## Elliot J. Androphy, M.D.

Dr. 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 investigates axon transport in neurons. Dr. Androphy has been a participant in multiple translational programs including antisense oligonucleotides and presently leads a multi-center small molecule approach to increase cellular levels of the SMN protein.



## 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.



## Tom Crawford, M.D.

Dr. Crawford is a 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

ataxia disorders. His primary research interests involve the basic science and clinical characterization of two important neurological disorders that affect children: SMA and Ataxia Telangiectasia.



## Stephen J. Kolb, M.D., Ph.D.

Dr. Kolb is an Assistant Professor of Neurology and Molecular and Cellular Biochemistry of The Ohio State University. His lab studies the molecular pathways underlying motor neuron disease. Dr. Kolb was also the PI of the NINDS multi-center trials for SMA biomarkers in infants.



## Rashmi Kothary, Ph.D.

Dr. Kothary is Deputy Scientific Director and Senior Scientist at the Ottawa Hospital Research Institute and a Professor at the University of Ottawa. Dr. Kothary's research focuses on studying the fundamental role of a cytoskeletal linker protein important for intracellular trafficking, investigating extrinsic

and intrinsic factors important for oligodendrocyte mediated myelination and remyelination of the central nervous system, and understanding Spinal Muscular Atrophy pathogenesis and identifying novel therapeutics for this devastating children's disease.



## Adrian Krainer, Ph.D.

Dr. Krainer is a Professor of Molecular Genetics at 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 200 published

research articles and patents. He is one of the inventors of the SMA experimental drug nusinersen.



## Umrao Monani, Ph.D.

Dr. Monani is an Associate Professor of Pathology and Cell Biology at Columbia University. His lab uses model mice to investigate the molecular and cellular basis of neurodegeneration. Dr. Monani was one of the first researchers to identify the

splicing error in SMN2 which renders it unable to compensate for the loss of SMN1.



## 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).



## Mark Rich, M.D., Ph.D.

Dr. Rich is a Professor in the department of Neuroscience, Cell Biology and Physiology at Wright State University. He completed a medial 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.



## Kathryn Swoboda, M.D.

Dr. Swoboda is physician specializing in Neurology and Genetics at Massachusetts General Hospital. She is the Katherine B. Sims Endowed Chair in Neurogenetics and the Director of the Neurogenetics Program and Co-Director of the MGH Neurogenetics

Diagnostic Laboratory. 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.

## 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 for 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 critic 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

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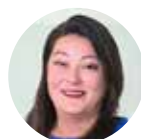
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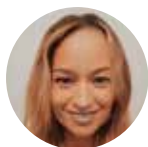
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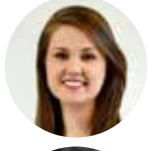
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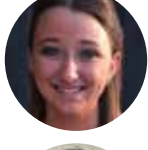
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## **FOR YOUR CHANCE TO WIN AN AMAZON ECHO**

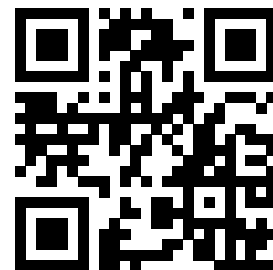


The success of our conference can only be improved by the feedback that we received from you! Please take the time to provide any comments by filling out our conference survey, and the workshop surveys. You can find hard copies of your surveys in your conference folder. Please drop your completed surveys into the many bins throughout the meeting space, at your workshops or at the Registration Area.

**All participants who complete the surveys by 10:30am on Sunday, June 17th, will have their name entered into a raffle.**

**The winners of the raffle will receive a brand new Amazon Echo!**

All completed surveys will also be entered into a drawing for a chance to win a trip, including airfare to the 2019 Annual SMA Conference (the locations will be announced on Sunday). The winners will be drawn and announced on Sunday, June 17th at the Closing General Session/It's a Wonderful Life.



You can also fill out the survey on-line at <https://www.surveymonkey.com/r/2018AnnualSMAConference>

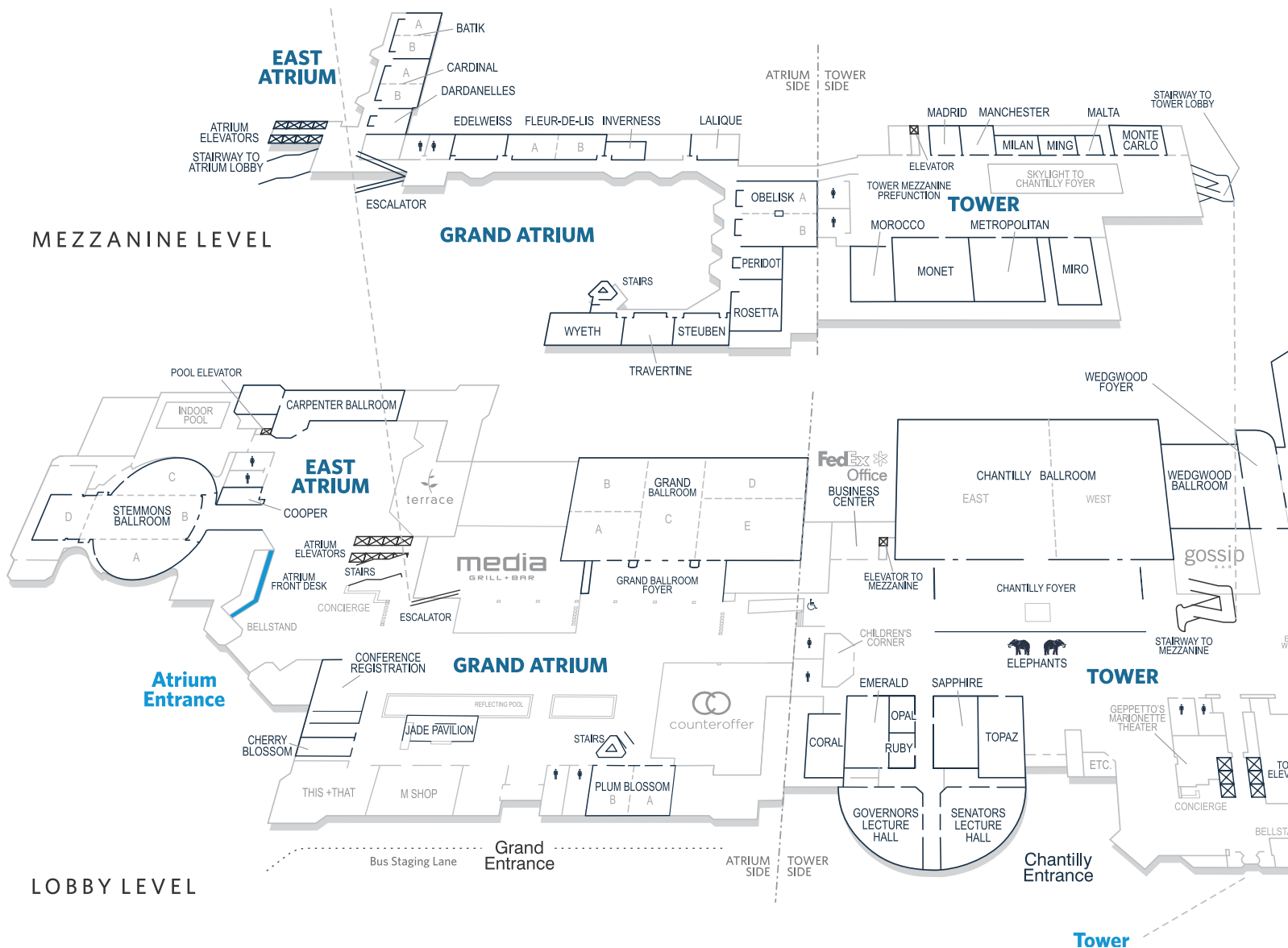


## **Amy Marquez Scholarship**

For the 2018 Annual SMA Conference, the Amy Marquez Scholarship is in its sixth 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 the SMA Adult community. The scholarship will cover both registration fees and hotel costs.

# CONFERENCE MAP

## Hilton Anatole Hotel in Dallas, Texas



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See you next year!