

# 2019 ANNUAL SMA CONFERENCE

JUNE 28TH - JULY 1ST, 2019

DISNEYLAND HOTEL IN ANAHEIM, CALIFORNIA



**cure**  
**SMA**

Make today a  
breakthrough.

## Dear Families,

We are leading 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 Spinraza in December 2016 and the approval of Zolgensma last month were major steps toward that goal. As a community, we've celebrated those long-awaited breakthroughs, all the while recognizing that there remains more to be done.

SMA is now changing. Individual needs and even our whole community are changing. We are expanding our research, care and support programs, while our commitment and community values remain the same as they have always been.

As therapies are approved, research and care are more important than ever. We need to understand and optimize the impact of these therapies, and develop combination approaches and additional therapeutic targets to treat all ages, stages, and types of SMA, especially for teens and adults.

One of our most important initiatives in this area is the SMA Care Center Network. The network now has 14 centers, and we will continue to add care centers across the US, to improve care and results, and to increase access to new therapies.

We are increasingly shifting our programs to provide customized and local support. Examples of this include the 36 local Summits of Strength that we will have conducted across the country by the end of this year, and over 900 new adult support packages that we have delivered. During the conference, we look forward to sharing more with you about these initiatives and addressing the key new and exciting challenge for us: choices.

We're expecting a record number of 2,500 attendees at this year's conference. Crucially, this is comprised of larger numbers than ever across all groups in our community – families, newly diagnosed, adults and over 700 researchers and clinicians from 160 organizations. There will be 65 family workshops, 165 research presentations and 70 care presentations given during the meeting.

This conference is generously supported by 18 sponsors and 16 exhibitors. Our special thanks to AveXis, Biogen, and Genentech as the Presenting Sponsors of the 2019 Annual SMA Conference. Through these sponsorships, we are able to provide assistance for travel or registration to over 1,100 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 and network to optimize care and grow our SMA community of healthcare providers. On the family side, we have workshop tracks that serve all of the different groups in our community. And, as always, we look forward to connecting and networking with each other through scheduled events and informal conversations.

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

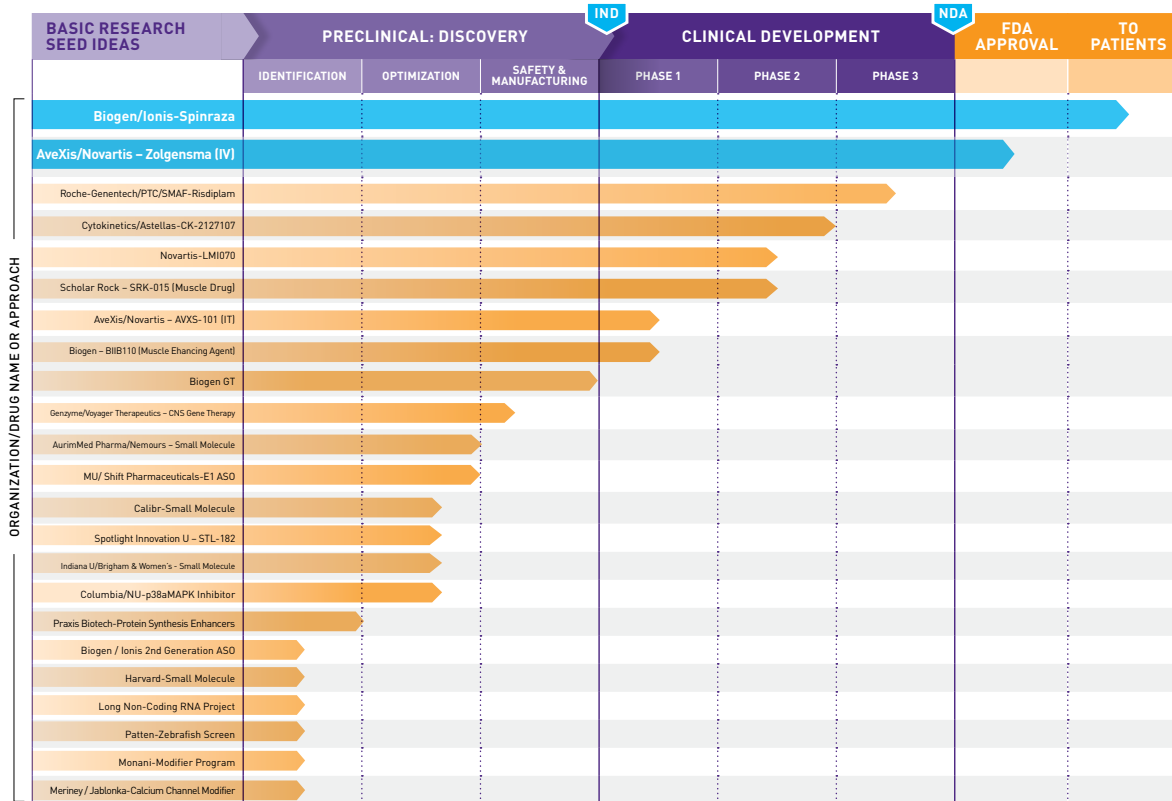
**Jill Jarecki, PhD**  
Chief Scientific Officer

**Mary Schroth MD**  
Chief Medical Officer



## SMA DRUG PIPELINE

We're funding and directing research with more breadth and depth than ever before. We know what we need to do to develop and deliver new therapies, which could also work in combination, to reach our goal of treatments for all ages and types. And we're on the verge of further breakthroughs that will continue to change the course of SMA for everyone affected, and eventually lead to a cure.

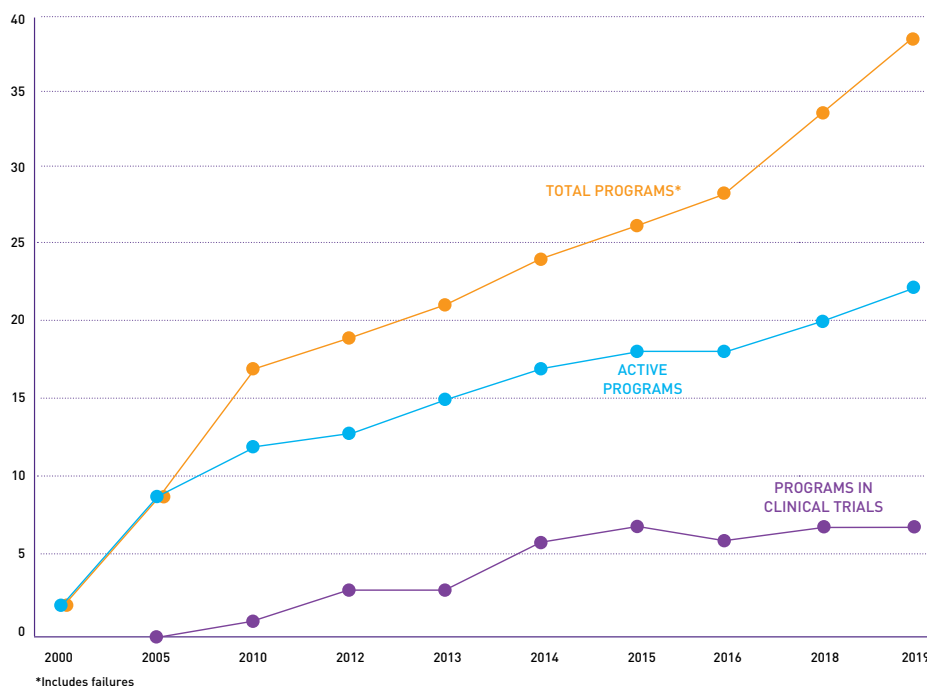


IND = Investigational New Drug

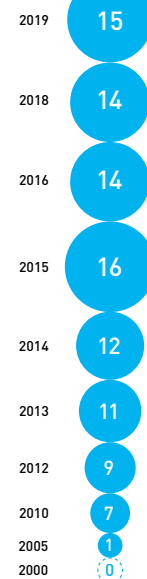
NDA = New Drug Application

Last updated: June 2019

### HOW THE PIPELINE HAS GROWN



### NUMBER OF COMPANIES INVESTING IN SMA DRUG PROGRAMS



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Cure SMA is dedicated to the treatment and cure of spinal muscular atrophy (SMA).

Since 1984, we've directed and invested in comprehensive research that has shaped the scientific community's understanding of SMA.

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 children and families affected by the disease.

Learn more about how you can help us reach more treatments and a cure at [www.cureSMA.org](http://www.cureSMA.org).

## 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.



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.

## OUR VALUES

### Innovation

Our commitment to more treatments and a 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 more treatments and a 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 more treatments and a cure, and we'll remain strong in our fight no matter what challenges come our way.





# SUNDAY 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!



# WORKSHOP SESSIONS

## Workshop Session #1

- Aquatic Physical Therapy Question & Answer Session
- Ethics and Realities in SMA Research and Emerging Therapies
- Breathing Strong All Day Long: BiPAPs and Ventilators
- Genetics and Reproductive Options for SMA Families
- The Role of Therapy (PT, OT and SP) in your Child's Life – Being Part of the Team!
- Power Soccer...More than just a Sport!
- Driving and Community Mobility
- Spouses and Significant Others of Adults with SMA – Sharing your Experience

## Workshop Session #2

- Getting the Most Out of What You Eat! Nutrition for Oral Feeders
- Healing the Grieving Heart – Part I
- Supporting the Emotional Health of SMA Affected Family Members
- Oral-Motor Exercises for Speech Clarity and Feeding
- Relevant Assistive Technology Tools for individuals with SMA
- Aquatic Physical Therapy for Fun & Function, Teens and Adults

## Workshop Session #3

- Dad's Time: A Workshop for Fathers Only – Sitters, Standers & Walkers
- Dad's Time: A Workshop for Fathers Only – Non-sitters
- Mom's Time: A Workshop for Mothers Only – Sitters, Standers & Walkers
- Mom's Time: A Workshop for Mothers Only – Non-sitters
- Keepsake Creation: Grieving Through Art
- What it Means to be a Teen on Wheels
- Medical Management of Adults with SMA

## Workshop Session #4

- Orthopedic Management
- Tube Feeding and SMA: Recommendations and Practices
- Family Readiness for Emergencies
- "I Need to Cough": Ways to Keep Your Lungs Clear
- Adults with SMA – Sharing Your Experience
- Finding Hope: Helping Parents and Children Cope with Loss
- Care for the Caregiver

## Workshop Session #5

- Aquatic Physical Therapy for Fun & Function, Children with Supported Head Control
- An Approach to Exercise in Individuals with SMA
- Therapeutic Yoga for Adults and Teens with SMA
- Special Plans for a Special Life
- Care for the Caregiver
- A Parent's Guide: How to Change your Child's Behavior
- Fundraising 101: Case studies in success with securing sponsorships, developing committees, and making your Cure SMA event special.

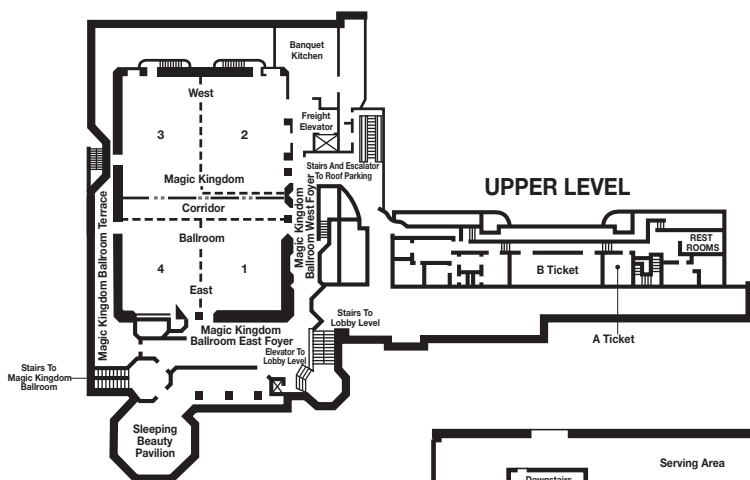
## Workshop Session #6

- Aquatic Physical Therapy for Fun & Function, Children with Independent Head Control
- Navigating the Special Education Process: Setting Our Kids up for Success
- Healing the Grieving Heart – Now What? Part II
- Top Tips on Traveling Around the World
- Pediatric Therapeutic Yoga for SMA
- Young Adults Talk it Out (Ages 18+)
- Finding a Job, Interviewing and How to Ask for Modifications Panell
- Unpacking SMA Clinical Trials

## Workshop Session #7

- Making Choices to Optimize Care and Quality of Life
- Personal Care Assistants—How to Find, Hire and Manage your PCAs
- Rehabilitation care considerations in the new era of SMA
- Grandparents Talk it Out – Non-sitters
- Grandparents Talk it Out – Sitters, Standers & Walkers
- Managing Stress in a World That Isn't Always Accessible
- Advocacy for the SMA Community

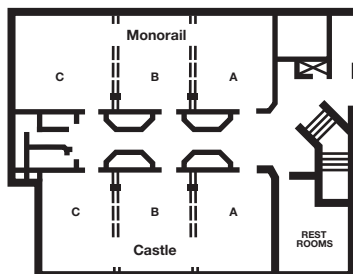
FANTASY TOWER



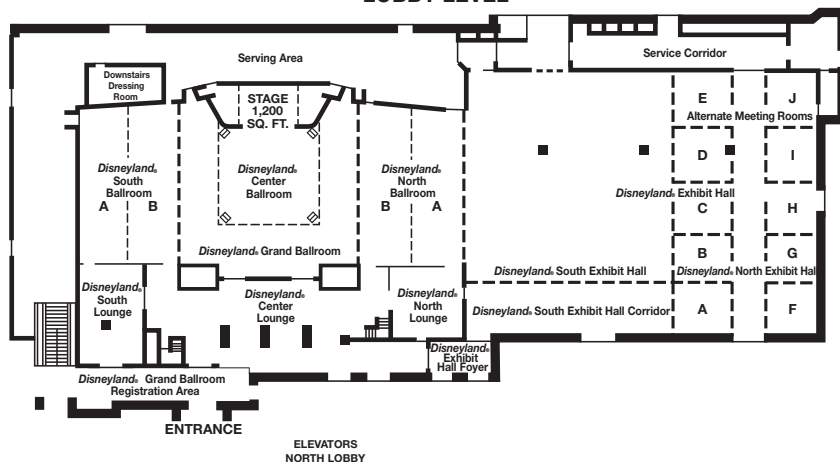
# Disneyland Hotel

## CONVENTION CENTER

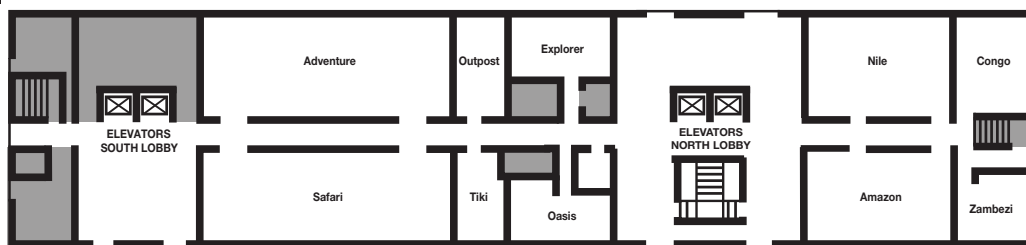
LOWER LEVEL



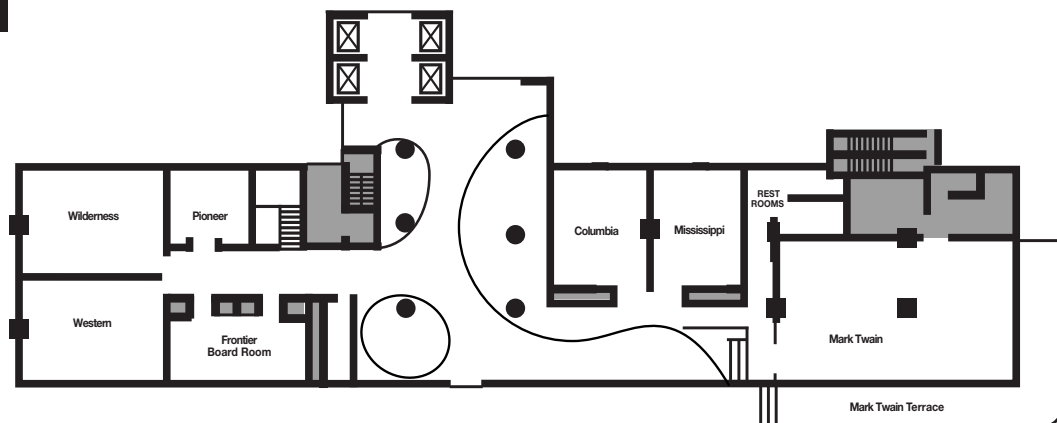
LOBBY LEVEL



ADVENTURE TOWER



FRONTIER TOWER



# Friday

June 28<sup>th</sup>, 2019

## Newly diagnosed families only

<b>1:00pm – 3:20pm</b>	Newly Diagnosed Program <i>(For Newly Diagnosed Families Only)</i>
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## All conference attendees

<b>7:00am – 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 Jacob Isaac Rappoport Foundation
<b>7:30pm – 10:00pm</b>	Adults with SMA Reception   Sponsored by the Dhont Family Foundation and Biogen

# Saturday

June 29<sup>th</sup>, 2019

<b>7:00am – 5:30pm</b>	Registration Open
<b>7:30am – 5:30pm</b>	Exhibitor Tables Open
<b>7:15am – 8:30am</b>	Family Breakfast with Symposium   Sponsored by Genentech/Roche
<b>9:00am – 11:00am</b>	General Session
<b>11:15am – 12:30pm</b>	Workshop Session #1
<b>12:30pm – 1:45pm</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 – 8:30pm</b>	Family Friendly Researcher Poster Session
<b>7:30pm – 9:30pm</b>	PJ Party and Movie Night
<b>8:00pm – 10:00pm</b>	Adults with SMA Reception   Sponsored by The Dhont Family Foundation and Biogen





# Sunday

June 30<sup>th</sup>, 2019

7:30am – 4:30pm	Registration Open
7:30am – 4:30pm	Exhibitor Tables Open
7:30am – 8:30am	Breakfast Items
8:45am – 10:15am	Workshop Session #4
10:30am – 12:00pm	Workshop Session #5
12:00pm – 1:15pm	Family Lunch with Symposium   Sponsored by AveXis
1:30pm – 3:00pm	Workshop Session #6
3:15pm – 4:30pm	Workshop Session #7
4:30pm	Cure SMA Evening at the Park   Sponsored by AveXis

# Monday

July 1<sup>st</sup>, 2019

7:30am – 8:45am	Family Breakfast Item
9:00am – 11:15am	Closing General Session – It's a Wonderful Life Panell

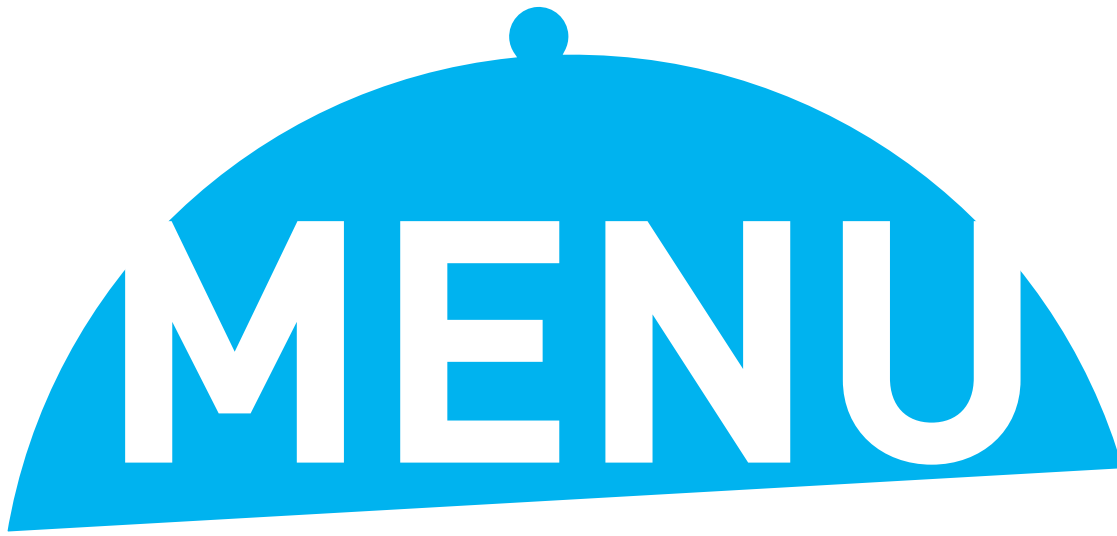


## SMA CONFERENCE GOALS

- 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.



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## Conference Meals included with registration:

### Friday

#### Dinner at Meet & Greet:

Deli Dinner Buffet, lemonade and water

### Saturday

#### Breakfast Items:

Assorted cereals with milk, assorted muffins, juice and coffee

#### Lunch:

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

#### Snacks at Family Friendly Poster Session and PJ Party:

Light snacks of Mickey pretzels, Mickey rice krispy treats, popcorn and refreshments

### Sunday

#### Breakfast:

Assorted cereals with milk, bagels with cream cheese, juice and coffee

#### Lunch:

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

### Monday

#### Breakfast:

Assorted cereals with milk, cheese and roasted vegetable frittatas, juice and coffee

**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****Friday 6:00pm - 8:30pm**

Kick off to the Conference!

Meet Disney Characters:

Micky & Minnie, Donald & Daisy, Wreck it Ralph

Grand Ballroom

**-Researcher Relay Race**

**-Family Fun Fest/Carnival**

**-Disney Characters**

**Family Friendly Researcher Poster Session****Saturday 6:30pm - 8:30pm**

Rotate around to over 30 posters presented by researchers

Meet Disney Characters:

Woody, Jessie, Buzz Lightyear, Pluto

Grand Ballroom

**PJ Party and Movie Night****Saturday 7:30pm - 9:30pm**

Family Movie for kids and adults alike!

Center Ballroom

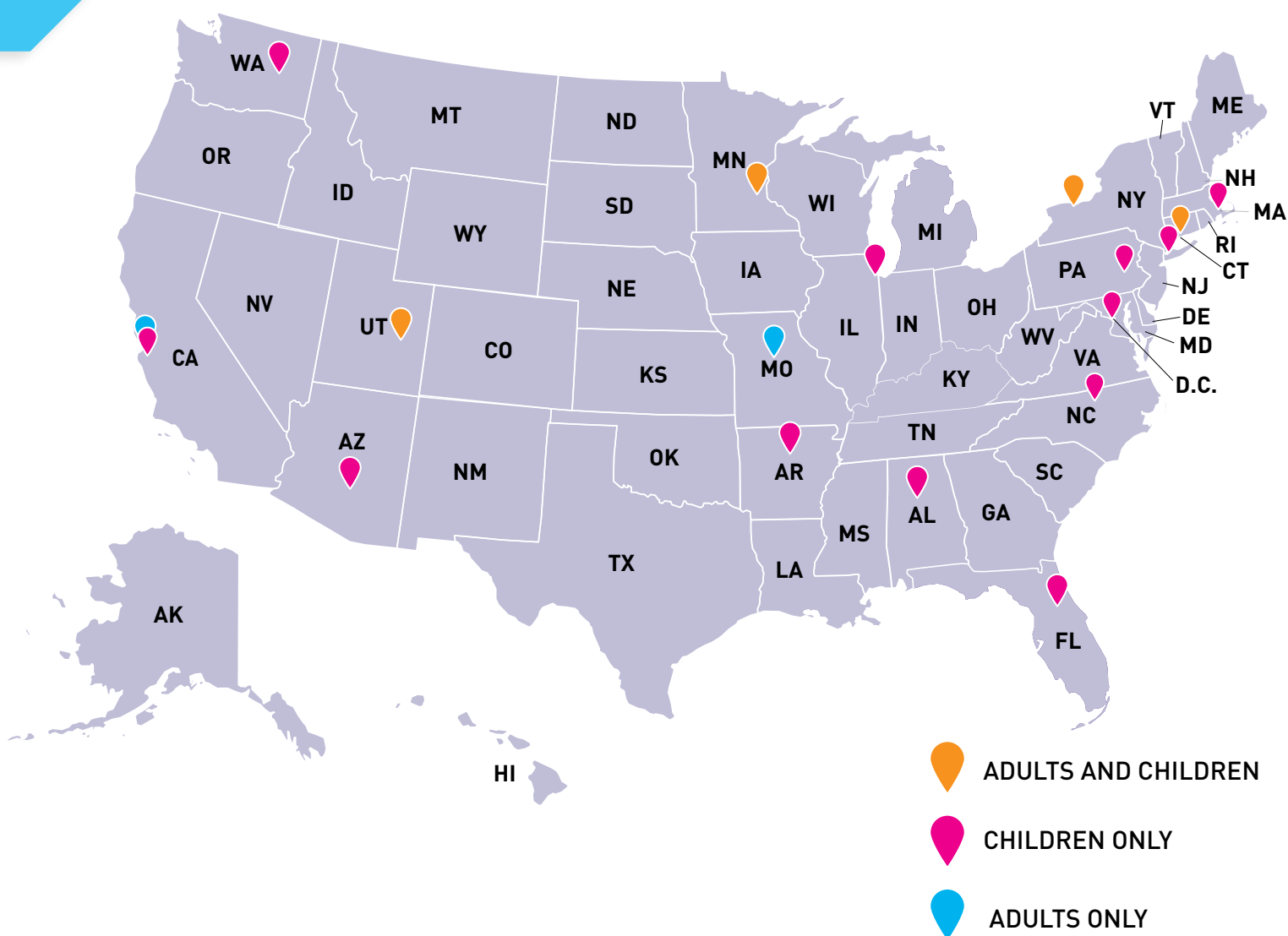
**Cure SMA Evening in the Park****Sunday 4:30pm**

Disney Entrance Ticket to begin at 4:30pm

# CURE SMA CARE CENTER NETWORK

The Care Center Network is made up of clinics who are partnering with Cure SMA to share consented electronic health record data with the SMA registry to achieve the following GOALS:

1. Quality improvement of SMA clinical care and disease management leading to creation of evidence to support a robust standard of care for SMA
2. Standardize care across the US to facilitate more rapid therapeutic development
3. Expand clinical care center capacity to deliver new therapies to individuals with SMA, increase patient access to new treatments, and increase the number of sites for SMA clinical trials
4. Resource for local patient services and family support and regional healthcare providers

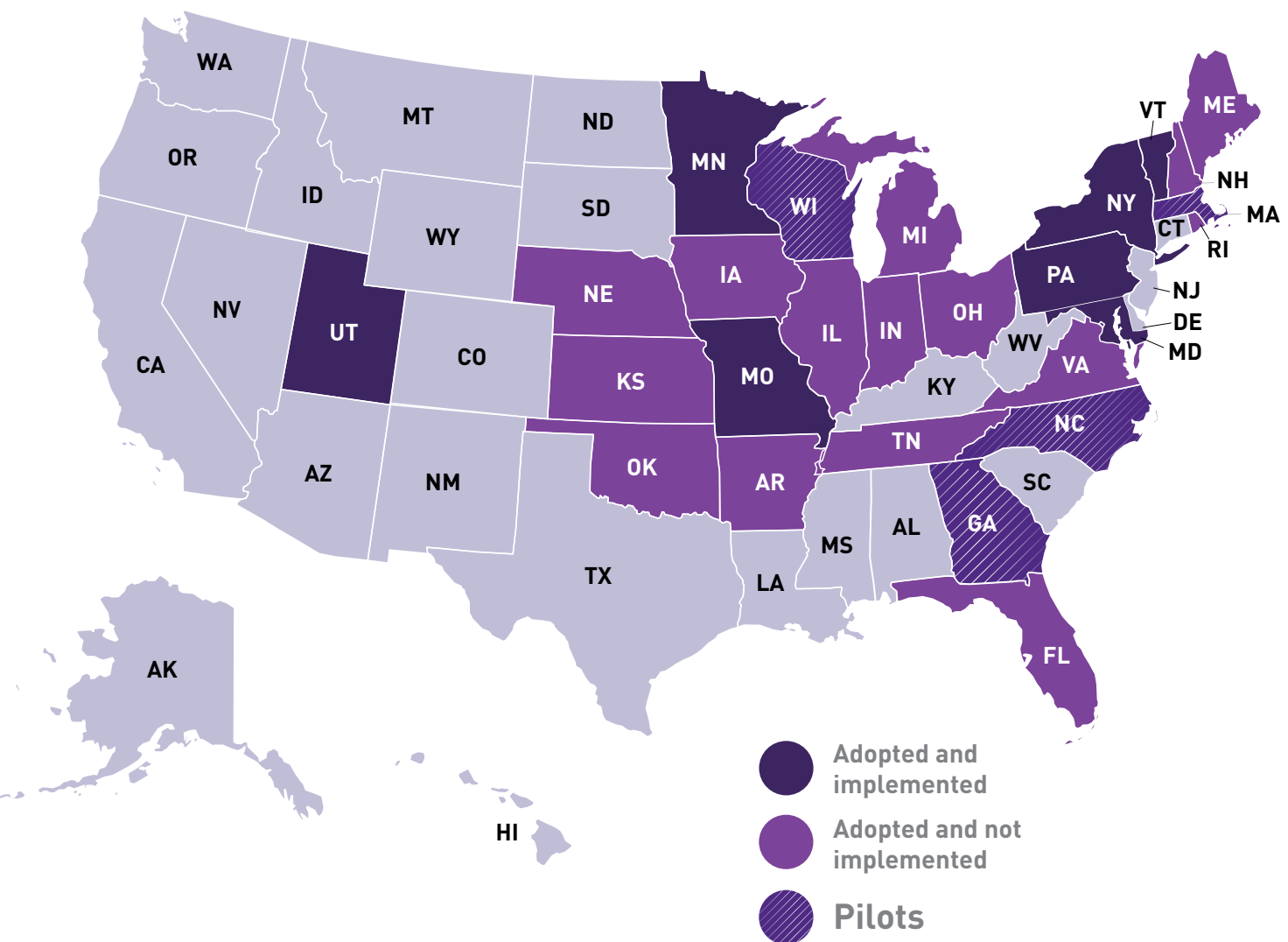




# NEWBORN SCREENING PROGRAMS

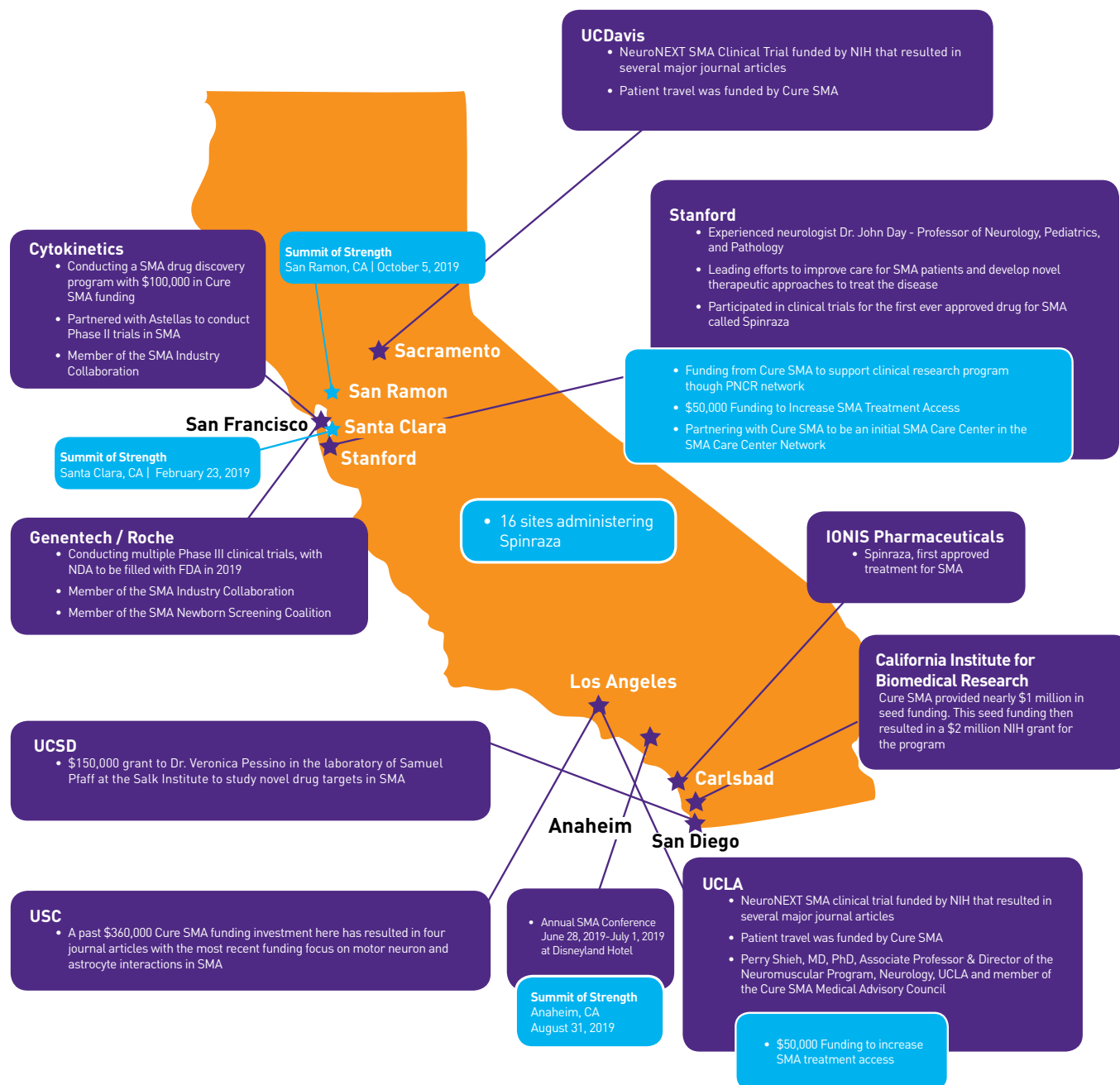
Updated as of June 1, 2019

Early diagnosis and treatment are key in the fight against SMA. The best way to do this is through screening every newborn for SMA through their state's newborn screening program. Each state decides what conditions to screen for in these tests. Cure SMA has been working to ensure that every state screens for SMA, and thanks to the hard work of our families and advocates, we have made tremendous progress. As of June 1, 2019, seven states are permanently screening every infant, which are colored in dark purple. The fifteen in light purple have taken action to include SMA in their testing, but are implementing their systems for doing so. The striped patterns are for states running temporary programs, called pilots, that will be used to help states set up permanent programs.



# CALIFORNIA PROGRESS HIGHLIGHTS

Newborn Screening for SMA adopted through state legislation and will be implemented by 2020





# MEET & GREET AND FAMILY FUN FEST

Grand Ballroom Friday June 28th, 6:00pm – 8:30pm

## CONFERENCE WELCOME

introductions, carnival games, and prizes for all

## RESEARCHER RELAY RACE 7:00pm

## MEET DISNEY CHARACTERS

Meet Mickey and Minnie, Donald and Daisy, and  
Wreck-it Ralph

**Thurs.**June 27<sup>th</sup>, 2019

5:00pm – 7:30pm	<b>Registration Open for all Conference Attendees</b>	Disneyland Ballroom Registration Hallway
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**Fri.**June 28<sup>th</sup>, 2019

7:00am – 5:30pm	<b>Registration Open for all Conference Attendees</b>	Disneyland Ballroom Registration Hallway
1:00pm – 3:20pm	<b>Newly Diagnosed Program (For Newly Diagnosed Families Only)</b> Sponsored by Erin Trainor Memorial Fund	South Ballroom B
1:00pm – 3:20pm Drop off opens at 12:30pm	<b>Newly Diagnosed Children's Program</b>	North Exhibit Hall
1:00pm – 2:25pm	<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  <b>Parent Welcome</b> Al Freedman Danyelle Sun  <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 Al Freedman, SMA Type I Danyelle Sun, SMA Type II & Type III Kevin O'Brien, SMA Type III Amy Medina, SMA Type I	South Ballroom B



2:35pm – 3:20pm	<b>Newly Diagnosed Meet &amp; Mingle Reception</b>	South Ballroom A
6:00pm – 8:30pm	<b>Meet &amp; Greet</b> <b>Includes Researcher Relay Race and Games</b> Disney Characters: Mickey & Minnie, Donald & Daisy, and Wreck-it Ralph	Grand Ballroom
7:30pm – 10:00pm	<b>Teen Social</b> Sponsored by JIRF 	Adventure
7:30pm – 10:00pm	<b>Adults with SMA Reception</b> Sponsored by Biogen and the Dhont Family Foundation  	Mark Twain

**Sat.**June 29<sup>th</sup>, 2019


7:00am – 5:30pm	<b>Registration Open for all Conference Attendees</b>	Disneyland Ballroom Registration Hallway
7:15am – 8:30am	<b>Family Breakfast with Symposium, Sponsored by Genentech/Roche:</b> Join us for Breakfast: A Conversation with Genentech and Special Community Guests 	South Exhibit Hall
7:30am – 5:30pm	<b>Exhibitor Tables Open</b>	North Lounge, South Lounge & Center Lounge
9:00am – 12:30pm & 2:00pm – 5:15pm Drop off opens at 8:30am & 1:30pm	<b>Children's Program</b> Sponsored by JIRF 	North Exhibit Hall
9:00am – 11:00am	<b>General Session Opening Remarks</b>	Center Ballroom
11:15am – 12:30pm	<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.) This workshop will be on a first come, first serve basis.	North Ballroom B

Sat.

June 29<sup>th</sup>, 2019

11:15am – 12:30pm	<b>Sibling Talk it Out (Ages 12 to 17)</b> Katlyn O'Brien, Sister of a SMA Adult Traci O'Brien, Sister of SMA Adult Kelli Blume, Cousin of SMA Adult  This workshop is for the siblings of brothers and sisters with SMA. With the unique relationship siblings have, we have much to offer one another. This workshop will create a fun and comfortable setting in which siblings can express themselves and their feelings, both positive and negative, of having a sibling with SMA through activities, games, and discussions. This workshop will be facilitated by 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!	Monorail A & B
11:15am – 12:30pm	<b>Workshop Session #1</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.	North Ballroom A
	<b>Ethics and Realities in SMA Research and Emerging Therapies</b> Rob Graham, MD Vanessa Battista, RN, MS, CPNP, CRRC Tom Murray, PhD  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.	Center Ballroom

	<p><b>Breathing Strong All Day Long: BiPAPs and Ventilators</b>  Oren Kupfer, MD  Peter Schochet, MD  Richard Kravitz, 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>	South Ballroom A
	<p><b>Genetics and Reproductive Options for SMA Families</b>  Harvey J. Stern, MD, PhD, FACMG, FAAP  Louise R. Simard, PhD</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>	Safari
	<p><b>The Role of Therapy (PT, OT and SP) in your Child's Life – Being Part of the Team!</b>  Terri Carry, PT,  Anne Stratton, MD  Jennifer Hubbell, PT</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>	South Ballroom B


	<p><b>Power Soccer...More than just a Sport!</b></p> <p>Karen Russo, SMA Parent</p> <p>Dominic Russo, SMA Parent</p> <p>JC Russo, SMA Adult</p> <p>Natalie Russo, SMA Adult</p> <p>Diane Murrell, LCSW</p> <p>Why it's so much more than a sport? Learn about power soccer from the perspective of Team USA players, parents who drove the sport to be nationwide and a social worker who saw how this program changes lives and built a program from her neuromuscular clinic.</p> <p>This workshop is everything you always wanted to know about power soccer. From the game itself, equipment, where it is played, cost, health benefits, how to start a team in your area, or where the current seventy-five plus teams are located! We will share with you the importance of sport in the disabled athlete's life and how this sport will benefit your child and how it can bring you closer as a family.</p>	Adventure
	<p><b>Driving and Community Mobility</b></p> <p>Brandon Lesch, OT</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 whether 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>	Amazon
	<p><b>Spouses and Significant Others of Adults with SMA – Sharing your Experience</b></p> <p>Kyla Pollock, spouse of adult with SMA</p> <p>A time for spouses and partners of those with SMA to share their unique perspectives, ideas and experiences. This session is intended for spouses, partners, and significant others to those with SMA only. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	Monorail C
12:30pm – 1:45pm	<p><b>Family Lunch with Symposium, Sponsored by Biogen:</b></p> <p>SPINRAZA (nusinersen): A Clinical Overview </p>	South Exhibit Hall
2:00pm – 3:30pm	<p><b>Memory Boxes for Siblings: Creating a Lasting Treasure (for children only)</b></p> <p>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>	Monorail A & B




2:00pm – 3:30pm	<b>Workshop Session # 2</b>	
	<p><b>Getting the Most Out of What You Eat!</b>  <b>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 workshop will break out into round table discussion groups led by a registered dietitian for nutrition challenges specific to: 1) Adults with SMA and 2) Children with SMA.</p>	North Ballroom A
	<p><b>Healing the Grieving Heart – Part I</b>  Fred Troutman, RN, PhD  David Sereni, MPT  Natasha Abruzzo, NP</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>	Monorail C
	<p><b>Supporting the Emotional Health of SMA Affected Family Members</b>  William Taft, MD, Child Psychiatrist  Diane Murrell, LCSW, Social Worker  Angela Wrigglesworth, MS, Elementary School Teacher  Al Freedman, PhD, Child/Family Psychologist</p> <p>How do children and adults cope with the experience of SMA emotionally? How can family members support the emotional needs of their SMA-affected loved ones? This panel of mental health and education professionals will share their perspectives and recommendations. Time will be allotted for Q&amp;A and discussion with attendees.</p>	Adventure
	<p><b>Oral-Motor Exercises for Speech Clarity and Feeding</b>  Renee Roy Hill, MS, CCC-SLP</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>	Safari

	<p><b>Relevant Assistive Technology Tools for individuals with SMA: Where we've come and where we are going!</b></p> <p>Hanna Eide, SMA Adult</p> <p>Dan Phillips, CCC - SLP</p> <p>Technology has become so much a part of all of our lives, that it seems to be constantly around us. It can seem overwhelming for many of us, just managing our daily lives. For individuals with motor challenges, technology can be a critical piece in independence and opening up a world of possibilities. It is often difficult to keep up with assistive technology and determine the most appropriate tools in our ever-changing tech landscape with new advancements happening every day. Hanna Eide, a 22-year-old individual with SMA, and Dan Phillips, an assistive technology professional will share their expertise and experience with using a wide variety of assistive technology tools. This workshop will explore assistive technology tools that are relevant for individuals with SMA in a wide variety of areas that include:</p> <ul style="list-style-type: none"> <li>• Augmentative and Assistive Communication</li> <li>• Digital Media tools</li> <li>• Social Media</li> <li>• Reading and Writing</li> <li>• Artificial Intelligence tools</li> </ul> <p>Hanna will share her own personal experience and journey with tools that she has implemented throughout the years as well as her recommendations for future development and needs that she continues to have, despite access to many tools. Dan will provide a wide variety of suggestions and references for a wide variety of tech tools to address the target areas. Participants will leave with a greater understanding of using current technology tools in innovative and functional ways as well as a large list of suggested tools for the future.</p>	North Ballroom B
	<p><b>Aquatic Physical Therapy for Fun &amp; Function, Teens and Adults</b></p> <p>Jennifer Martyn, PT</p> <p>Krista Torseth, PT, DPT</p> <p>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>	Disneyland Hotel Pool
3:45pm – 5:15pm	<b>Workshop Session # 3</b>	
	<p><b>Dad's Time: A Workshop for Fathers Only – Sitters, Standers &amp; Walkers</b></p> <p>Kevin O'Brien, SMA Parent</p> <p>Ryan Wolff, SMA Parent</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>	North Ballroom A

	<p><b>Dad's Time: A Workshop for Fathers Only – Non-sitters</b></p> <p>Al Freedman, SMA Parent</p> <p>David Sereni, SMA Parent</p> <p>Although SMA is experienced directly by the person with the disease, all members of the family are im-pacted 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>	Safari
	<p><b>Mom's Time: A Workshop for Mothers Only – Sitters, Standers &amp; Walkers</b></p> <p>Angel Wolff, SMA Parent</p> <p>Danyelle Sun, SMA Parent</p> <p>Although SMA is experienced directly by the person with the disease, all members of the family are im-pacted in profound ways. During this session, mothers of SMA-affected individuals will share their unique perspectives, ideas, and experiences. This session is intended for mothers of SMA-affected children or adults. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	North Ballroom B
	<p><b>Mom's Time: A Workshop for Mothers Only – Non-sitters</b></p> <p>Amy Medina, SMA Parent</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, mothers of SMA-affected individuals will share their unique perspectives, ideas, and experiences. This session is intended for mothers of SMA-affected children or adults. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	Adventure
	<p><b>Keepsake Creation: Grieving Through Art</b></p> <p>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>	Monorail A & B
	<p><b>What it Means to be a Teen on Wheels</b></p> <p>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>	Amazon

	<b>Medical Management of Adults with SMA</b> Tina Duong, MPT, PhDc Bakri ElSheikh, MBBS, FRCP 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.	Nile
6:30pm – 8:30pm	<b>Family Friendly Researcher Poster Session</b> Includes Disney Characters: Woody & Jessie, Buzz Lightyear and Pluto	Grand Ballroom
7:30pm – 9:30pm	<b>PJ Party and Movie Night</b>	Center Ballroom
8:00pm – 10:00pm	<b>Adults with SMA Reception</b> Sponsored by The Dhont Family Foundation and Biogen  	Mark Twain




7:30am – 8:45am	<b>Family Attendee Breakfast Items</b>	South Exhibit Hall
7:30am – 4:30pm	<b>Registration Open for all Conference Attendees</b>	Disneyland Ballroom Registration Hallway
7:30am – 4:30pm	<b>Exhibitor Tables Open</b>	North Lounge, South Lounge & Center Lounge
8:45am – 12:00pm & 1:30pm – 4:30pm Drop off opens at 8:15am & 1:00pm	<b>Children's Program</b> Sponsored by JIRF 	North Exhibit Hall
8:45am – 10:15am	<b>Workshop Session #4</b>	
	<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.	Center Ballroom
	<b>Tube Feeding and SMA: Recommendations and Practices</b> Rebecca Hurst Davis, MS, RD, CSP, CD, CNSC Stacey Tarrant, BS, RD, LDN Laura Watne, MS, RD, CSP 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.	North Ballroom B

	<p><b>Family Readiness for Emergencies</b></p> <p>Rob Graham, MD</p> <p>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>	South Ballroom A
	<p><b>“I Need to Cough”: Ways to Keep Your Lungs Clear</b></p> <p>Richard Kravitz, MD</p> <p>Oren Kupfer, MD,</p> <p>Peter Schochet, MD</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>	South Ballroom B
	<p><b>Adults with SMA – Sharing Your Experience</b></p> <p>Carolyn Barrett, SMA Adult</p> <p>Jaclyn Greenwood, SMA Adult</p> <p>During this session, adults with SMA will share their unique perspectives, ideas, and experiences. This session is intended for adults with SMA. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	North Ballroom A
	<p><b>Finding Hope: Helping Parents and Children Cope with Loss</b></p> <p>Audra Perry Butler, SMA Parent</p> <p>When a family loses a child, the grief is profound, and the family dynamic is instantly changed. In this workshop, we will discuss how parents can help their family navigate the grief journey, from being consumed by the loss to cherishing the memories. Created by retired psychologist Dr. Glenn G. Perry, Jr. and facilitated by SMA parent Audra Perry Butler, the workshop will examine the reactions and emotions created by grief, some of the ways that mothers and fathers grieve differently, and specific coping strategies for parents. We also will review how children process grief and give you ways to help your surviving children understand their feelings. Finally, we will provide ideas to help your family find ways to honor and remember your child, as your family moves forward and rediscovers hope.</p>	Monorail C




	<b>Care for the Caregiver</b> Carolyn Long, MSW Vanessa Battista, RN, MS, CPNP, CCRC Jennifer Lemisch, MA, ATR-BC, LPC Diane Murrell, LCGC <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>	Adventure
10:30am – 12:00pm	<b>Kids Talk it Out (Ages 11 - 17)</b> Al Freedman, PhD, SMA Dad and Child Psychologist Angela Wrigglesworth, Elementary Education Teacher, SMA Adult <p>This facilitated session will provide SMA-affected children (ages 11-17) an opportunity to talk with each other about their lives. To encourage our children to truly "Talk It Out", this session is open to children only and requires parental consent. Registration will be on a first come, first served basis.</p>	North Ballroom A
10:30am – 12:00pm	<b>Workshop Session # 5</b>	
	<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>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>	Disneyland Hotel Pool
	<b>A Lifespan Approach to Activity and Exercise</b> Tina Duong, MPT, PhDc Leslie Nelson, MPT, PhDc, OCS Anne Stratton, MD <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>	North Ballroom B

	<p><b>Therapeutic Yoga for Adults and Teens with SMA</b></p> <p>Anne Buckley-Reen, OTR/RYT, FORKIDS OT</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 teen and his family. The workshop will conclude with a seated/chair practice of a 30 minute yoga routine to facilitate stress reduction, enhance energy and endurance, maintain joint mobility, and more!</p>	South Ballroom A
	<p><b>Special Plans for a Special Life</b></p> <p>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>	Center Ballroom
	<p><b>Care for the Caregiver</b></p> <p>Carolyn Long, MSW</p> <p>Vanessa Battista, RN, MS, CPNP, CCRC</p> <p>Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>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>	Adventure
	<p><b>A Parent's Guide: How to Change your Child's Behavior</b></p> <p>Brynne Willis, SMA Adult</p> <p>Elizabeth Bondarenko, OTL/R</p> <p>Parenting is hard work! In the midst of meltdowns, outbursts, and bad behavior choices, you can feel like you're at your wits' end. This seminar equips parents with practical strategies they can implement to help their child act appropriately. Learn about the ABC's of Behavior, a specific assessment tool that helps you understand your child's actions, and behavioral modification techniques that can be used to change your child's behavior. Expand your resources and knowledge to successfully navigate the many challenges involved with improving your child's behavior.</p>	South Ballroom B

	<p><b>Fundraising 101: Case studies in success with securing sponsorships, developing committees, and making your Cure SMA event special.</b></p> <p>Erin Kelly, Cure SMA staff</p> <p>Amber Snyder, Cure SMA staff</p> <p>Join us for this fun and informative workshop that puts the FUN in FUNdraising! We'll tackle topics like how to secure sponsors for your event, develop your event committee, and make your Cure SMA event special. Whether you're new to fundraising or have been hosting an event for years, this workshop will help you take your fundraising skills to the next level.</p>	Safari
12:00pm – 1:15pm	<p><b>Family Lunch with Symposium,</b></p> <p>Sponsored by AveXis: AveXis Updates</p> 	South Exhibit Hall
1:30pm – 3:00pm	<p><b>Siblings Talk it Out (Ages 5 to 11)</b></p> <p>Katlyn O'Brien, Sister of a SMA Adult</p> <p>Traci O'Brien, Sister of SMA Adult</p> <p>Kelli Blume, Cousin of SMA 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>	Adventure
1:30pm – 3:00pm	<b>Workshop Session # 6</b>	
	<p><b>Aquatic Physical Therapy for Fun &amp; Function, Children with Independent Head Control</b></p> <p>Jennifer Martyn, PT</p> <p>Krista Torseth, PT, DPT</p> <p>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>	Disneyland Hotel Pool
	<p><b>Navigating the Special Education Process: Setting Our Kids up for Success</b></p> <p>Kimberly Cook, SMA Parent</p> <p>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>	North Ballroom B

	<p><b>Healing the Grieving Heart – Now What? Part II</b></p> <p>Fred Troutman, RN, PhD</p> <p>David Sereni, MPT</p> <p>Natasha Abruzzo, NP</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>	Monorail A & B
	<p><b>Top Tips on Traveling Around the World</b></p> <p>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 traveling around the world!</p>	Safari
	<p><b>Pediatric Therapeutic Yoga for SMA</b></p> <p>Anne Buckley-Reen, OTR/RYT, FORKIDS OT</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 for the developing child. The workshop will include sharing a yoga experience with a SMA family. The workshop will conclude with a floor yoga routine with optional modifications. This gentle practice will include a balance of supported postures, breathing exercises, a progressive relaxation sequence and a circle of song to enhance breathing and endurance.</p>	South Ballroom A
	<p><b>Young Adults Talk it Out (Ages 18+)</b></p> <p>Angela Wrigglesworth, Elementary Education Teacher, SMA-Affected Adult</p> <p>Al Freedman, PhD, SMA Dad and Child/Family Psychologist</p> <p>This facilitated session will provide SMA-affected young adults (ages 18+) an opportunity to talk with each other about their lives. Registration will be on a first come, first served basis.</p>	North Ballroom A
	<p><b>Finding a Job, Interviewing and How to Ask for Modifications Panel</b></p> <p>Kimberly Hill, SMA Adult</p> <p>Jaclyn Greenwood, SMA Adult</p> <p>Carolyn Barrett, SMA Adult</p> <p>Brad Nunemaker, SMA Adult</p> <p>Brynne Willis, SMA Adult</p> <p>Come meet a diverse panell 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 panell will share their personal experiences and helpful tips they have gained. This workshop will also include a question and answer session.</p>	South Ballroom B

	<b>Unpacking SMA Clinical Trials</b> John Day, MD Julie Parsons, MD Perry Shieh, MD, PhD Two treatments are approved for individuals with SMA. More treatments are in development and clinical trials. This workshop brings together a panel of 3 neurologists who conduct SMA clinical trials for a discussion of how to think about and interpret the results from SMA clinical trials and how to think about this information when making decisions about treatments.	Center Ballroom
<b>3:15pm – 4:30pm</b>	<b>Workshop Session # 7</b>	
	<b>Making Choices to Optimize Care and Quality of Life</b> Vanessa Battista, RN, MS, CPNP, CCRC Carolyn Long, MSW 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.	Safari
	<b>Personal Care Assistants—How to Find, Hire and Manage your PCAs</b> Paula Barrett, SMA Parent Carolyn Barrett, SMA Adult 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 for? Where to look? How to describe your needs? In answering these questions and more, we'll strive to help you learn how to recruit, interview and screen PCA applicants. This workshop is designed for anyone that is looking to bring PCA's into their life for the first time or young adults who may be looking to independently manage their team of PCA's, but all are welcome. In addition, we will foster a dialogue on how parents can help their children learn to slowly manage their PCA's on their own. We'll encourage questions and share personal anecdotes as a way to shed light on this process.	South Ballroom A
	<b>Managing Stress in a World That Isn't Always Accessible</b> Mary E. McNaughton-Cassill, PhD <i>This presentation will be designed for individuals and families coping with SMA</i> Stress can be conceptualized as the gap between what we have, and what we want or need. Sometimes we can change situation and thereby reduce the gap, but when there is no ready solution we often have to find creative ways to cope. Certainly, living with a disease like Spinal Muscular Atrophy (SMA) requires both creative coping, and resilience. Managing chronic health issues, care needs, pain and mobility challenges, complex insurance and health care systems, educational, career, and financial concerns, ignorance, prejudice, discrimination, and psychosocial concerns are all potential sources of stress. Although medical breakthroughs, accessibility laws, and education have all helped to make SMA more manageable, patients and their families still report significant levels of stress. This presentation will focus on understanding how stress affects us, and how we can use psychological strategies to "mind the gaps" we cannot eliminate.	Center Ballroom

	<b>Advocacy for the SMA Community</b> Jaimie Vickery, Cure SMA Staff Helena Hernandez, Cure SMA Staff <p>Advocacy is critical in making sure SMA patients and their families get the support and services they need, but it can be overwhelming and intimidating, especially in the current political environment. This session will focus on Cure SMA's current advocacy priorities, tips on effective advocacy and how to successfully navigate today's political climate</p>	Nile
	<b>Rehabilitation care considerations in the new era of SMA: Interventions, assistive devices, orthotics and strategies to improve motor function from infancy to adulthood</b> Matthew Civitello, PT Rachel Salazar, PT Meghan Moore, PT <p>Our objective is to bring awareness to available Physical and Occupational Therapy interventions particularly considering the evolving SMA phenotype as individuals with SMA are being treated with disease modifying medications. We will review the current equipment available to promote optimal positioning, increase mobility in functional positions including sitting, standing and walking, as well as strategies to improve the use of the trunk and arms. Specifically, we will discuss PROM positioning devices, seating options, standers, walkers, Dynamic Orthosis- TLSO/ SPIO/Bennik, Kinesiotaping, Serial casting and Splinting. We will highlight the approach to equipment prescription throughout the different ages and SMA types from infants to adults. We will discuss the importance of the adherence to the rehabilitation standard of care guidelines for maximal benefit from disease modifying treatments. Finally, we will promote new ideas for PT/OT treatment and encourage the patients and families to think outside the box and to get their local therapists to do it as well.</p>	North Ballroom B
	<b>Grandparents Talk it Out – non-sitters</b> Elizabeth and Bob Lockwood, SMA Grandparents <p>During this session, grandparents will share their unique perspectives, ideas, and experiences. This session is intended for grandparents. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	Monorail A & B
	<b>Grandparents Talk it Out – sitters, standers &amp; walkers</b> Pat Wolff, SMA Grandparent <p>During this session, grandparents will share their unique perspectives, ideas, and experiences. This session is intended for grandparents. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	South Ballroom B
4:30pm	<b>Cure SMA Evening in the Park</b> Sponsored by AveXis 	



Mon.

July 1<sup>st</sup>, 2019

7:30am – 8:45pm	<b>Family Breakfast</b>	South Exhibit Hall
9:00am – 11:15am Drop off opens at 8:30am	<b>Children's Program</b> Sponsored by JIRF 	North Exhibit Hall
9:00am – 11:15am	<b>It's a Wonderful Life/Closing General Session</b>	Center Ballroom





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# ERIN TRAINOR MEMORIAL FUND

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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) has generated substantial funds sending hundreds of newly diagnosed families to conference.

In addition, ETMF provides 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 announce that we have reached our \$1 million goal of the Erin Trainor Memorial Fund.**

*-Gene Trainor*

## Thank you to the Trainor Family

**And everyone who has supported this effort in reaching the \$1 million endowment goal of the Erin Trainor Memorial Fund. Because of your support, the initial disbursement of funds started at the 2017 Conference.**



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> **For more information, visit [AveXis.com](https://www.avexis.com).**

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# 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 in its fifth 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 this amazing foundation for your continued years of support for newly diagnosed families, the conference, and so much more. Your support of the type I reception, the children's program and teen social have all added wonderful components to our growing conferences!

**jir**  
the jacob isaac rappoport  
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## Jennifer Miller Smith, Aaron Smith and Family

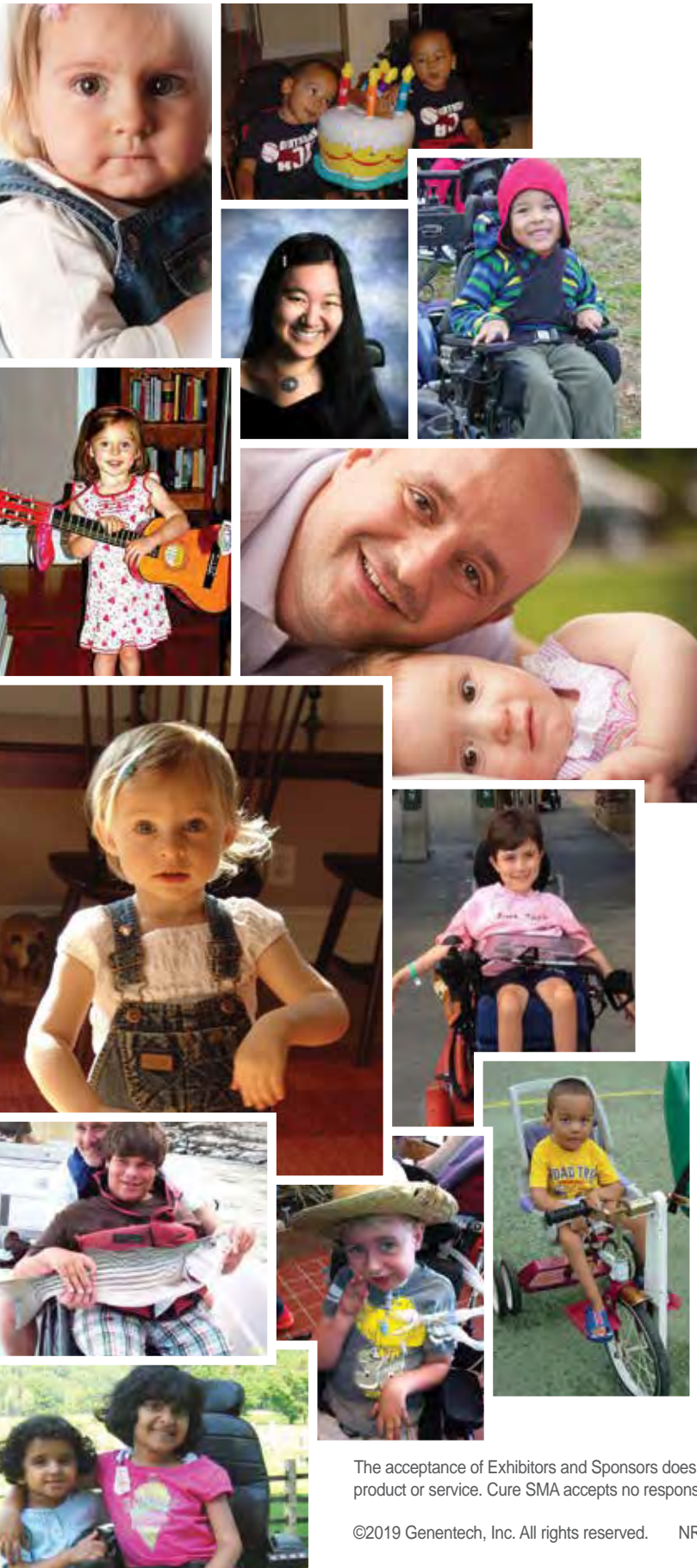


The Smith family has been an amazing asset throughout the planning of the conference. They have been so gracious these past several years for donating the signage throughout conference. We cannot thank you enough for volunteering your services and expertise to this make conference as magical as it was!

Expo CCI- We would also like to thank everyone at Expo CCI, especially Richard Curran, who sponsor and donate all of the amazing signs and banners throughout the meeting space. The professionalism that Expo brings to our conference through their impressive signs and banners, that decorate the entire hotel, is absolutely outstanding! We cannot thank each and every one of you enough for volunteering your services and expertise to make this conference great!







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# Cure SMA 2019

## Annual Conference

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**Saturday, June 29<sup>th</sup>  
7:15 - 8:30 am**

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## 10th Annual Zane's Run

Congratulations to the entire Zane's Run committee for hosting a successful 10th event! A special thank you to Hillary and Keith Schmid, the Dunlap Family, Meridith Bebee, and Carrie Kane, for their outstanding service to Cure SMA and years of dedication to the event.

Five-hundred and thirty-five people came out to Great Valley Middle School in Malvern, PA, on September 30, 2018, to celebrate the milestone 10th year of Zane's Run. It was an incredible day filled with fun for everyone!

Through this event, the Sweet Baby Zane Fund supports Cure SMA's equipment pool for purchases of car beds, bath chairs, and Panthera wheelchairs.



## 10th Annual Bugaboo WOD

Congratulations to the Bugaboo WOD event that celebrated its 10th year in 2018! On October 27, 2018, dozens of CrossFit enthusiasts came out to CrossFit Albuquerque in Albuquerque, NM, to complete a fun work-out while fundraising for Cure SMA! Thanks to the generous support of participants, nearly \$4,500 was raised for Cure SMA! A special thank you to Joe Vigil and Natasha and Ben Abruzzo for all their hard work organizing this event over the last 10 years.



## 10th Greater Florida Chapter Walk-n-Roll

The Greater Florida Chapter Walk-n-Roll celebrated their 10th anniversary in November 2018 at Fort Desoto State Park in St. Petersburg, FL, and was a huge success! Over 150 participants and 15 teams joined this yearly event to fundraise for a cure, and to celebrate the SMA community. This year more than \$23,000 was raised for Cure SMA with an overall amount of over \$200,000 over the last 10 years!

The Greater Florida Walk-n-Roll is spearheaded by the chapter leadership and supportive SMA families in the area. Audra Butler was the first event organizer and was supported over the years by Katie Kerns, Jena Maronie, Trevor Smith, Tanya Krajewski, Shawn Santos, Lisa Hoang, Asia Reiter, and Laurie Sore. Through Audra and Katie's leadership, hard work, and dedication to fundraising for Cure SMA, they have helped make tremendous strides in the SMA community – contributing to the funds needed to support ongoing research efforts and family support programs. Thank you to all the teams who have support the event over the years! Some of the teams included are: Allison Wonderland, Amiya's Circle, Andy's Army, Arms for Asher, Brooks Wolfe Pack, Chris' Charisma, Ember's Fire, Emma's Entourage, home Team for Ezra, House of K, Jaceys Journey, Joey Strong, Joie's Cure Crew, Muscle Up for Maia, Positive Vibration, Rhyidian's Team 2 Angels, Team Gabby, Team Lucas, Team Riley, TeamEd, and Tianna's Troops.

The 11th Annual Greater Florida Walk-n-Roll will take place on October 5, 2019, at Philippe Park in Safety Harbor!





## 15th Annual Western NY Walk-n-Roll

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

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



## 20th Annual Wannabe Cup

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

Between August 2 and 4, 2018, the 20th Annual Wannabe Cup Charity Golf Tournament was held at the Silverado Resort in Napa, CA. The event included two days of golf: first and final matches with closing ceremonies and awards. This year's event raised \$11,630.

Thank you so much to Joe and Andy Belcher for their tireless work organizing this annual golf tournament. Congratulations on 20 years!



## 25th Annual Chesapeake Crab Feast and Silent Auction

The Chesapeake Chapter celebrated a milestone with its 25th Annual Crab Feast and Silent Auction. It has become a staple in Baltimore, MD, a town that loves crabs. Attendees immersed themselves with blue crabs and enjoyed the silent auction, wine and dine raffle, laydown games, and beer wheel. Over the past 25 years, the Crab Feast has directly benefited the Cure SMA community by helping the Erin Trainor Memorial Fund (ETMF) generate over 1.1 million dollars. The ETMF is an endowment established to provide conference scholarships for newly diagnosed families and individuals to attend the Annual SMA Conference.

The event was created in memory of Erin Trainor, daughter of Barbara and Gene Trainor who lost her battle to SMA Type 1 in 1994, at five and half months old. The impact of her life continues 25 years later. When Erin was diagnosed, there was no treatment and no known cause of the disease. But it was always the

Trainor's focus to find hope in other ways. Cure SMA and its community brought that hope. Families and friends rallied to raise funds for research and support for families. The first event in 1994 raised only \$2,000 and gathered 100 people. This grassroots event has grown to become a tradition which now attracts over 350 people each year to celebrate and help to raise funds for Cure SMA and the ETMF.

Thank you to the event organizers for their unwavering support over the past 25 years to make this a special day: Barb and Gene Trainor with their daughters, Caitlin and Grace, Beverly and Dan Venedam with their daughters, Mary Kate, Eileen, and Annie, and John and Katy Nolan. Special thanks to Knights of Columbus #5208 for managing the wheels and raffle for 25 years! This event would not be possible without the dedication of so many family and friends who, along with Cure SMA, bring hope to so many newly diagnosed families.

# WELCOME!

We are honored to have SMA families, researchers, and clinicians attend this conference from all over the world! There are individuals attending from 29 countries, including: Argentina, Australia, Belgium, Brazil, Canada, China, Colombia, Costa Rica, France, Germany, Hong Kong, Israel, Italy, Japan, Jordan, Kuwait, Malaysia, Netherlands, New Zealand, Norway, Panama, Romania, South Africa, Spain, Switzerland, Taiwan, Turkey, United Kingdom, and the United States.



Thanks to the support of our generous sponsors of the 2019 Annual SMA Conference, Cure SMA is able to bring together over 2,000 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.

## PRESENTING SPONSORS



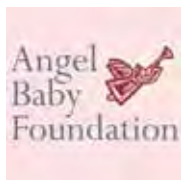
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## The Miller McNeil Woodruff Foundation!



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.



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





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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.

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

## Saturday

June 29<sup>th</sup>, 2019

<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>Properties of Matter   11:00am- 12:00pm</b> Great Scott the Glad Scientist has a presentation packed full of intriguing experiments including air, water, crazy chemistry, & dry ice. Audience participation is a must and willing volunteers are always encouraged!
<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>Forces and Motion   2:00pm- 3:00pm</b> Properties of Force and Motion is one of Great Scott's favorite presentations because he gets to bring so many of his cool science toys to play with and share as he explains the science behind them. He will use toys like Fidget Spinners, Yo-Yo's, the Wheel-O, Gyroscopes, Tops, and other and Balancing & Spinning toys to explain topics like: Isaac Newton's Laws of Motion, Centripetal and Centrifugal Force, Potential and Kinetic Energy, just to name a few.

## Sunday

June 30<sup>th</sup>, 2019

<b>Morning:</b> 8:45am- 12:00pm	<b>Cartoon You Caricatures   8:45am – 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>Fire and Ice Show   10:30am – 11:30am</b> Children will be dazzled and entertained as they interact with our Mad Scientists! Foggy dry ice storms, Eggbert the Egg, and even a special Mad Science "burp" potion will amaze children as they learn about chemical reactions, air pressure and the 3 states of matter.
	<b>Tea Party   11:30am- 12:00pm</b> Any children interested in joining the Tea Party are also welcomed to bring their dolls.
<b>Afternoon:</b> 2:00pm-4:30pm	<b>Cartoon You Caricatures   1:30pm – 4:30pm</b> Stop by this fun station so caricaturist Rafael can draw a photo of you to take home as a fun conference memento!
	<b>Dream Big   1:30-2:30pm</b> This show inspires children to GET EXCITED about science, the world around them and their futures. The science field hold so much potential and opportunities for young minds and it teaches kids that science can be found everywhere and can be part of any future they choose for themselves, Like being a great Chef, a Magician or an Astronaut and MORE!

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



# Monday

July 1<sup>st</sup>, 2019

**Morning:**  
9:00am- 11:15am

**Cartoon You Caricatures | 9:00am- 11:15am** Stop by this fun station so caricaturist Rafael can draw a photo of you to take home as a fun conference memento!



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 30 YEARS**

**George Ghorbanian**  
Chicago Police Officer



**VOLUNTEER FOR 28 YEARS**

**Patti Slojkowski**  
Dental Hygienist



**VOLUNTEER FOR 25 YEARS**

**Steve Smith**  
Physical Therapist



**VOLUNTEER FOR 21 YEARS**

**Kelly Milito**  
Hair Stylist



**VOLUNTEER FOR 19 YEARS**

**Katlyn O'Brien**  
Elementary School Teacher



**VOLUNTEER FOR 19 YEARS**

**Kelly Basso**  
Surgical Consultant



**VOLUNTEER FOR 18 YEARS**

**Mary Blume**  
Hospital Administrative Assistant



**VOLUNTEER FOR 9 YEARS**

**Megan Milito**



**VOLUNTEER FOR 18 YEARS**

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



**VOLUNTEER FOR 9 YEARS**

**Joy Martin**  
Physical Therapist



**VOLUNTEER FOR 18 YEARS**

**Noreen Reilly**



**VOLUNTEER FOR 9 YEARS**

**Kelli Blume**  
School Social Worker



**VOLUNTEER FOR 11 YEARS**

**Jackie Staples**



**VOLUNTEER FOR 9 YEARS**

**Kevin Blume**



**VOLUNTEER FOR 10 YEARS**

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



**VOLUNTEER FOR 9 YEARS**

**Mike Graney**  
Vice President of United services



**VOLUNTEER FOR 10 YEARS**

**Mary Kate Venedam**



**VOLUNTEER FOR 9 YEARS**

**Donna Budil**



**VOLUNTEER FOR 10 YEARS**

**Caitlin Trainor**



**VOLUNTEER FOR 9 YEARS**

**Caroline LaPelusa**





VOLUNTEER FOR 9 YEARS

**Shannon Murphy**



VOLUNTEER FOR 6 YEARS

**Leah Schneider**



VOLUNTEER FOR 8 YEARS

**Jennifer Walsh**



VOLUNTEER FOR 5 YEARS

**Patrick Slojkowski**



VOLUNTEER FOR 8 YEARS

**Kelly Gibbons**



VOLUNTEER FOR 5 YEARS

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



VOLUNTEER FOR 8 YEARS

**Kayla Forsythe**



VOLUNTEER FOR 5 YEARS

**Daniela Milito**



VOLUNTEER FOR 8 YEARS

**Lexi Basso**  
College student at Elmhurst



VOLUNTEER FOR 5 YEARS

**Michael Milito**



VOLUNTEER FOR 8 YEARS

**Tori Cebular**  
Occupational Therapist



VOLUNTEER FOR 5 YEARS

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



VOLUNTEER FOR 7 YEARS

**Georgia Slojkowski**



VOLUNTEER FOR 5 YEARS

**Ralph Milito**  
Real Estate Agent



VOLUNTEER FOR 7 YEARS

**Laura Kroll**  
College student studying Elementary Education



VOLUNTEER FOR 5 YEARS

Sally McCarthy



VOLUNTEER FOR 4 YEARS

Jackie Beck



VOLUNTEER FOR 4 YEARS

Isabelle Austriaco



VOLUNTEER FOR 4 YEARS

Meghan Breslin



VOLUNTEER FOR 4 YEARS

Bridget Yahihashi



VOLUNTEER FOR 3 YEARS

Pam Sirt



VOLUNTEER FOR 4 YEARS

Catie Martin



VOLUNTEER FOR 3 YEARS

Jaimie Chang



VOLUNTEER FOR 4 YEARS

Kamryn McKenzie



VOLUNTEER FOR 3 YEARS

Jack Jomarron



VOLUNTEER FOR 4 YEARS

Rebeka Nekolova





VOLUNTEER FOR 3 YEARS

**Mae Cahill**



VOLUNTEER FOR 2 YEARS

**Sarah Sirt**



VOLUNTEER FOR 3 YEARS

**Isabella Hernandez**



VOLUNTEER FOR 2 YEARS

**Asia Malczak**



VOLUNTEER FOR 3 YEARS

**Nicole Wu**



VOLUNTEER FOR 2 YEARS

**Olivia Vela**



VOLUNTEER FOR 3 YEARS

**Samantha Smith**



VOLUNTEER FOR 2 YEARS

**Clara Dylewski**



VOLUNTEER FOR 3 YEARS

**Cara Freedman**



VOLUNTEER FOR 2 YEARS

**Elizabeth Garvey**



VOLUNTEER FOR 3 YEARS

**Emma Rubenstein**



VOLUNTEER FOR 2 YEARS

**Bridget Wagner**



VOLUNTEER FOR 3 YEARS

**Erin Henley**



VOLUNTEER FOR 2 YEARS

**Megan Wagner**



VOLUNTEER FOR 2 YEARS

**Julia Kafkes**



VOLUNTEER FOR 2 YEARS

**Jessica Vrioni**



VOLUNTEER FOR 2 YEARS

**Will Reilly**



VOLUNTEER FOR 2 YEARS

**Morgan Ramirez**



VOLUNTEER FOR 2 YEARS

**Caroline Young**



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**THANK YOU TO OUR VOLUNTEERS**

## George Ghorbanian | Chicago Police Officer VOLUNTEER FOR 30 YEARS

The 2019 Annual SMA Conference marks a huge milestone for our longest returning volunteer George Ghorbanian! George has volunteered at every single conference since the Children's Program started back in 1990 making this his 30th consecutive conference! Every year he takes time away from his job as a Chicago Police Officer, as

well as his family and busy life, to travel each year and support the Children's Program. George is the definition of a truly dedicated volunteer. Everyone at Cure SMA would like to thank George for all of his hard work and dedication over the last 30 years! George is not only one of Chicago's finest but clearly one of SMA's finest!





## Steve Smith | Physical Therapist

### VOLUNTEER FOR 25 YEARS

The 2019 Annual SMA Conference also marks a big milestone for another one of our long-standing volunteers Steve Smith! Steve got involved many years ago and this year marks his 25th year as a conference volunteer!

Each year Steve takes time away from his family and off of work as a physical therapist to come out and volunteer in the Children's Program. He is also a great representation of a dedicated volunteer we are lucky to have! Everyone at Cure SMA would like to thank Steve for his dedication and service over the last 25 years!





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**Cure SMA is pleased to welcome the Angel Baby Foundation as a Gold Sponsor of the 2019 Annual SMA Conference!**


**SPECIAL THANKS**  
 to the Shish Family and the Foundation for your support of this year's event.



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**Cure SMA thanks the Dhont Family Foundation for their support of the 2019 Annual SMA Conference!**

**The Dhont Family Foundation generously funds scholarships for adults with SMA (18 and over) who want to attend Conference. We are grateful for your continued partnership.**

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8

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- 4 Disneyland Park
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## AIRPORTS

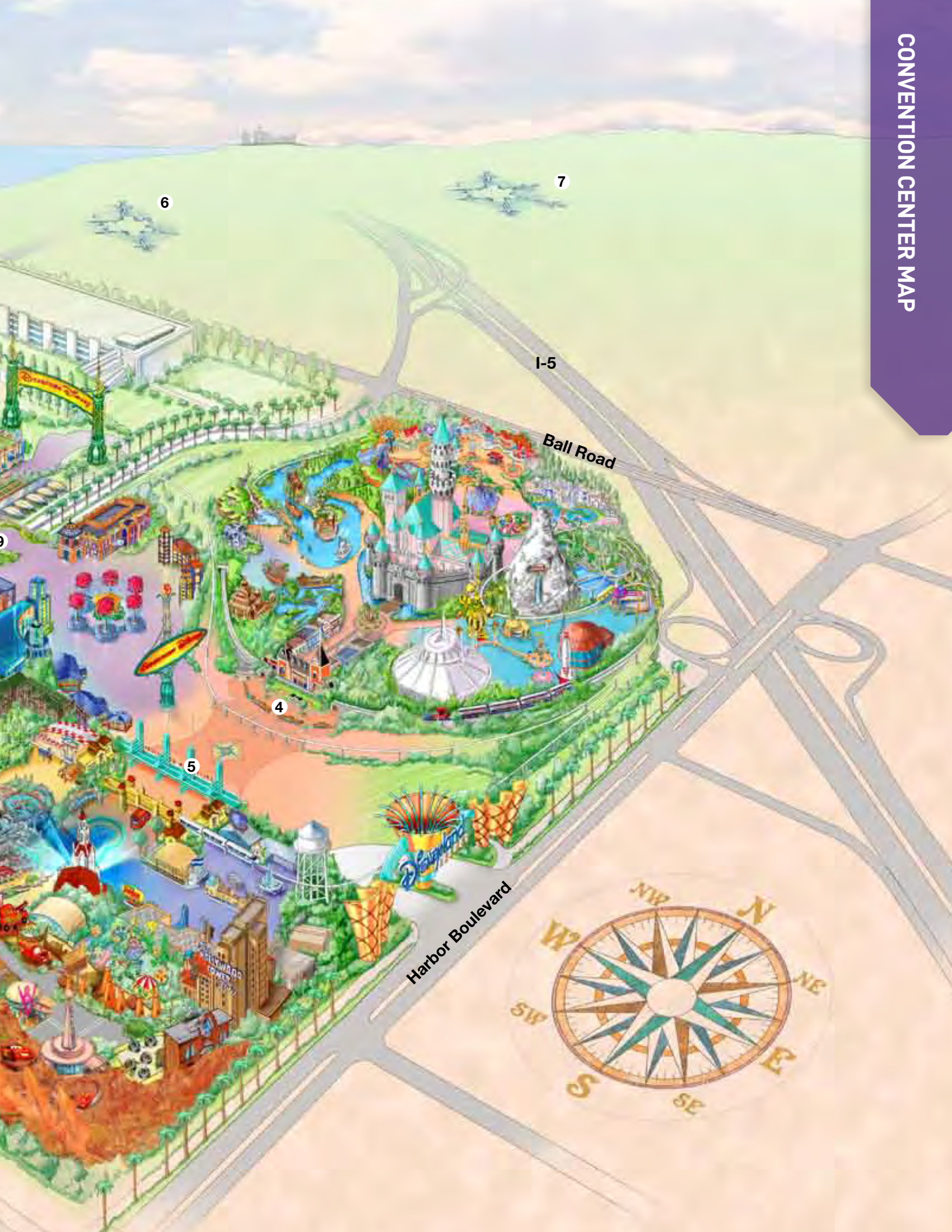
- 6 LAX – 34.5 miles from Resort
- 7 ONT – 35.5 miles from Resort
- 8 SNA – 13 miles from Resort

## OTHER

- 9 Downtown Disney District
- 10 Anaheim Convention Center







6

7

I-5

Ball Road

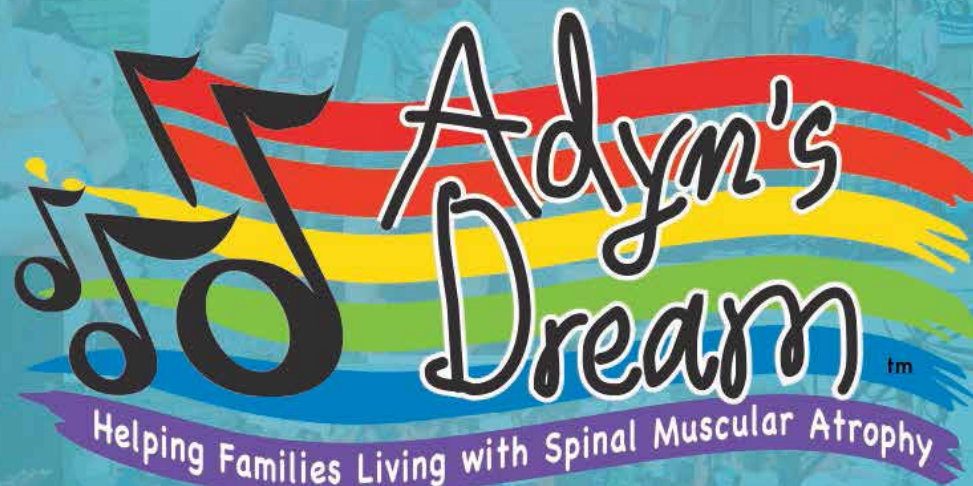
4

5

Harbor Boulevard







## 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.



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



# Sunday Evening at the Disney Theme Park

You are invited to join us on Sunday for an evening of fun at one of the Disney theme parks. All registered attendees will receive a complimentary park entrance ticket for anytime after 4:30pm.

We thank AveXis for their support and sponsorship of this wonderful Cure SMA Evening at the park.

This event will be a highlight of this year's Annual SMA Conference.



Make today a  
breakthrough.





# AT A GLANCE

**Make today a breakthrough.**



Spinal muscular atrophy (SMA) is a disease that robs people of physical strength by affecting the motor nerve cells in the spinal cord, taking away the ability to walk, eat, or breathe. **It is the number one genetic cause of death for infants.** SMA affects approximately 1 in 11,000 babies, and about 1 in every 50 Americans is a genetic carrier. SMA can affect any race or gender. But there's great reason for hope. We now have two approved treatments that target the underlying genetics of SMA. We know what we need to do to develop and deliver effective therapies. 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.

## RESEARCH



Since 1984, Cure SMA has led and invested in the research that has made today's breakthroughs possible.

With deep connections and expertise in both the patient and research

communities, we're uniquely positioned to direct funds to where they can make the greatest difference as quickly as possible.

We now have multiple FDA-approved therapies for SMA, plus 21 programs in development, including six now in clinical trials.

**We have invested more than \$80 million in research and have funded half of the ongoing new drug programs for SMA, including both FDA-approved treatments for the disease.**

## FAMILY SUPPORT AND PATIENT CARE

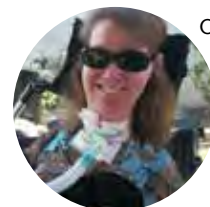


We won't stop working toward a world without SMA, but until we have a cure, we'll do everything we can to improve quality of life for children and families affected by the disease today.

**Each year, we reach nearly 4,000 families through our informational packets, newly diagnosed care packages, equipment pool, and more.**

We also reach the healthcare community through our medical professional educational opportunities and care series booklets.

## CONFERENCE AND COMMUNITY



Our Annual SMA Conference brings together researchers, healthcare professionals, and families to network, learn, and collaborate.

**The conference is the largest in the world focused specifically on SMA, and it attracts the top scientists and companies in the field.**

Today, we have more than 120,000 members and supporters, with 34 volunteer chapters throughout the country. We host over 300 fundraising and awareness events annually.



# Genentech

*A Member of the Roche Group*

**Thanks to a generous grant funded by Genentech, Cure SMA was able to provide travel stipends and hotel accommodations to adults with SMA attending the Conference this year.**



We are so grateful for this support and Genentech's partnership in the Conference.

**Thank you, Genentech!**

# The Family Friendly Research Poster Session

Saturday June 29 from 6:30 pm to 8:30 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:

- Allison Ebert, PhD from the Medical College of Wisconsin: Understanding SMN function in Different Cell Types
- Allison Mazzella from Cure SMA: Assessing Clinical Meaningfulness and Patient Experience in the SMA Teen and Young Adult Populations
- Arthur Burghes, PhD & Anton Blatnik from the Ohio State University: Suppressors of SMN Function and Modifiers of Spinal Muscular Atrophy
- Janice Wong, MD & Kristina Johnson from Biogen: The Spinraza Clinical Development Program
- Garrett Ingrando, MBA from Biogen: SPINRAZA Experience in the Real World
- Brunhilde Wirth, PhD from the University of Cologne, Germany: Genetic modifiers of SMA Help Some People to Remain Asymptomatic; How Can This Knowledge Help All People with SMA?
- Cera Hassinan from Johns Hopkins School of Medicine: Neurofilament as a biomarker for SMA
- Chad Heatwole, MD, MS-CI from University of Rochester Medical Center: Measurement of a Patient's Health Status (The SMA-Health Index)
- Geneviève Paris & Andréanne Didillon from the University of Ottawa: SMN and friends: What we can learn from SMN Interactors
- Jen Szegeida & Erin Treece, MS from Scholar Rock: Scholar Rock: SMA Muscle-Directed Therapy Advancing in Clinical Development.
- Hannah Staunton & Samuel Ewing, EngD from F. Hoffmann-La Roche: New measures of function and independence in Spinal Muscular Atrophy
- Jamie Shish from Cure SMA: Cure SMA Newborn Screening Survey and Database
- Reid Garner, Christiano R Alves, PhD, Kathryn J Swoboda, MD from Massachusetts General Hospital & Harvard Medical School: Circulating biomarkers for Spinal Muscular Atrophy
- Senam Beckley-Kartey, Marco Castillo, MD & Fani Petridis from F. Hoffmann-La Roche: Roche and Genentech Spinal Muscular Atrophy Clinical Development Program
- Kevin Kaifer, PhD from the University of Missouri: Astrocytes Contribute to Motor Neuron Degeneration in Spinal Muscular Atrophy
- Zaida Alipio-Gloria, Arnab K. Chatterjee, Jingxin Wang, Andrew To, Kristen Johnson from Calibr, a Division of Scripps Research: Innovative Screening Approaches to Identify the Next Generation of SMN-Inducing Compounds
- Frank Bennet, PhD & Kristina Bowyer from Ionis Pharmaceuticals: Antisense Oligonucleotide Strategies for SMA
- Krysta Engel, PhD from the University of Colorado: Transcriptome-Wide Interrogation of SMN-mediated RNA Localization Mechanisms in Neurons
- Laxman Gangwani, PhD from Texas Tech University: Overview and Comparison of SMN-Dependent and SMN-Independent Methods for the Treatment of Spinal Muscular Atrophy
- Lisa Belter from Cure SMA: Cure SMA Membership: Findings from the 2019 Membership Survey
- Samantha Edell, Darren T. Hwee, PhD, Stacy Rudnicki, MD, Bettina Cockcroft, MD, MBA, Andrew Wolff, MD & Lucie Vu, PhD from Cytokinetics: Results from Two Pre-clinical Studies: Reldesemtiv in Combination with Nusinersen and in Combination with SMN-C1 Shows Improved Muscle Function in Mouse Model of Spinal Muscular Atrophy
- Mary Schroth, MD, Jamie Shish, Teresa Stewart & Abigail Paras from Cure SMA: Cure SMA Clinical Care Center Network and SMA Clinical Data Registry
- Doug Sproule, MD from AveXis: Provide an overview of ZOLGENSMA indication, efficacy, safety and dosing for Caregivers
- Kathy L. Poulin, Joshua Del Papa, Hugh J. McMillan, MD, Jodi Warman Chardon, MD, Rashmi Kothary, PhD & Robin J. Parks, PhD from the Ottawa Hospital Research Institute: Biomarkers for Spinal Muscular Atrophy
- Samuel R. Rosenfeld, MD from Children's Hospital of Orange County: 20 Year Experience with Segmental Sublaminar Instrumentation for Guided Growth in Early Onset Scoliosis
- Bakri Elsheikh, MD & Sarah Heintzman, RN from the Ohio State University: The Spinraza Treatment Initiative at The Ohio State University
- Saravanan Arumugam, Michael Kahl, Zhaofa Xu & Yongchao Ma, PhD from Northwestern University: Targeting Mitochondria, the Powerhouse in Motor Neurons, for Treating SMA
- Fernanda Boulos, PhD and Svetlana Jevtic, DVM, MSc from Novartis: Branaplam (LMI070) clinical program summary
- Sibylle Jablonka, PhD & Utz Fischer, PhD from the University of Würzburg, Germany: Calcium Channels in SMA: What about the SMN complex?
- Erin Kelly from Cure SMA: The SMA Patient and Family Voice: Impacting our Community and Stakeholders Through Advocacy, Clinical Care, Research, and Industry Partnerships
- Josh Noone, PhD, Sarah Whitmire, MS, Daniel Buchenberger, MS, Rosalina Mills, Christine Pozniak, PhD & Er Chen, MPP from F. Hoffmann-La Roche: Impact of SMA on Caregivers' Daily

MEET  
DISNEY  
CHARACTERS





**Cure SMA thanks Biogen for funding a grant to provide an exclusive lounge space for adults with SMA to use while at the Conference and for supporting the Adults with SMA Reception.**



We appreciate your continued partnership  
in the Conference.

**Thank you, Biogen!**

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## GOLD EXHIBITORS



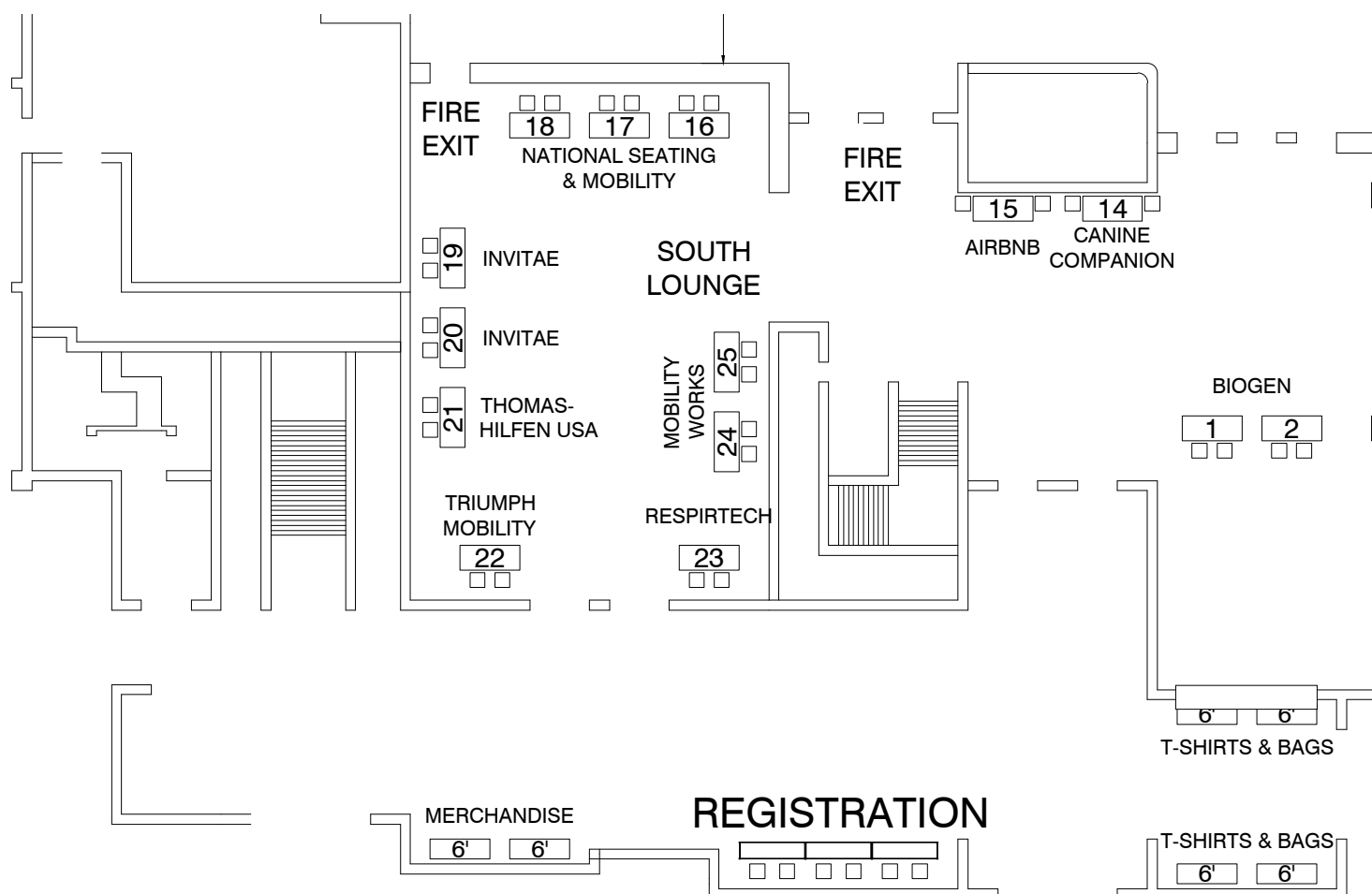


SILVER EXHIBITORS



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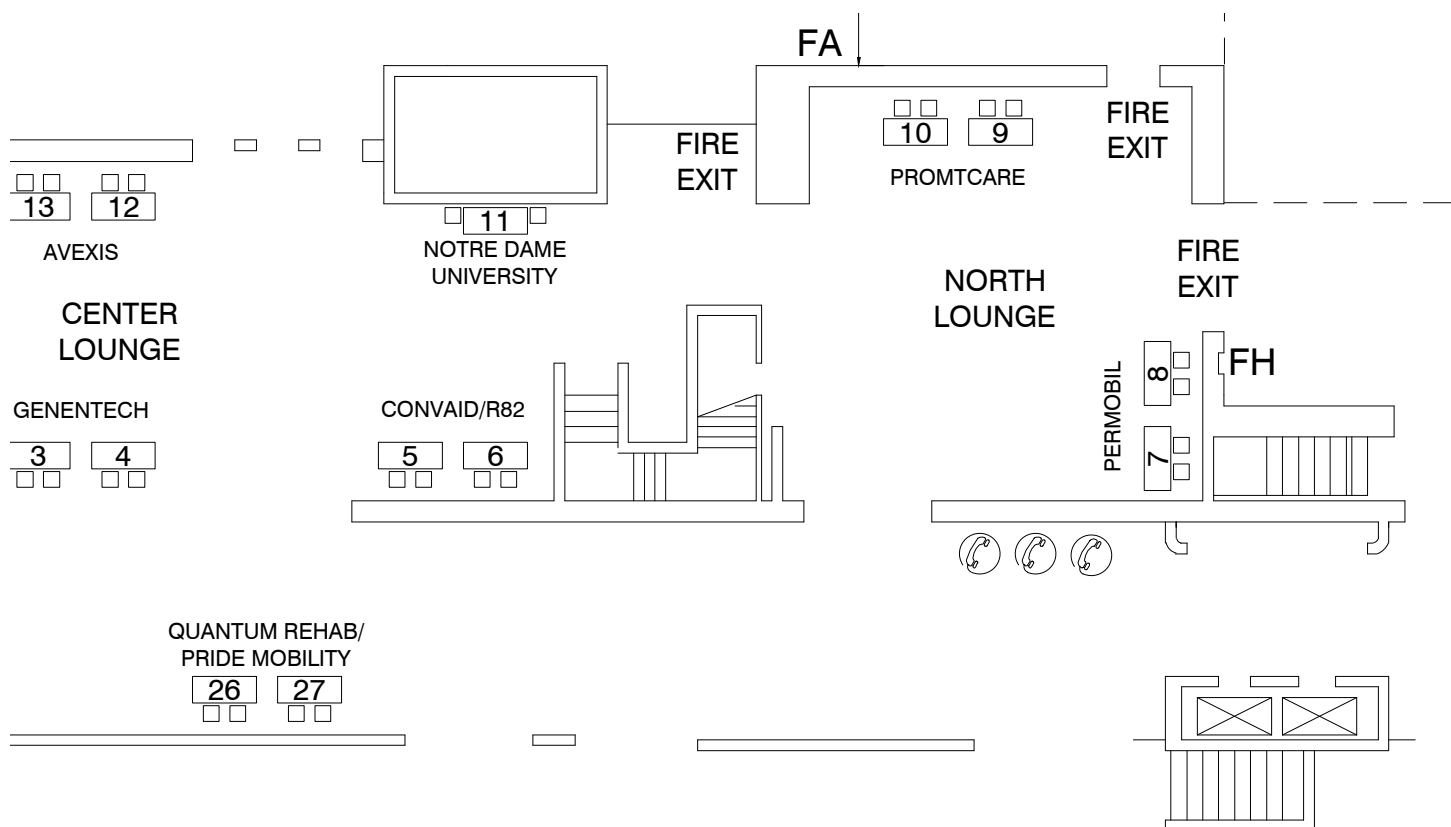
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Every day, we are motivated by people living with SMA and other diseases of impaired muscle function. They are spouses, partners, sisters, brothers, children and grandchildren. They are not defined by their disease. They are fighting with spirit, determination and courage. They amaze us. They inspire us. They are our heroes.

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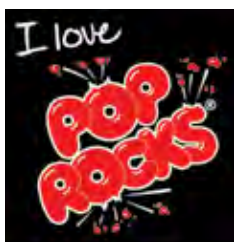
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# Recognize the symptoms and save a life!



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# 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 genetic 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  
Liz & Jim Bahrenburg  
Allie & Richard Wolfe  
Cathy Barsotti & Matt Dooling  
Kristen Bathe  
Angela & Drew Beard  
Valentina & David Berkovits  
Kelly & Jason Bertsch  
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Lardizabal De Zegarelli  
Shannon & Greg Zerzan  
Jill & John Zmaczynski



## Everyone at Cure SMA would especially like to thank the following people:

**The Cure SMA Board and Committee Members** – Cure SMA is very fortunate to have such a dedicated group of individuals, who represent the SMA community. Our Board Members work exceptionally hard to make sure that all 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 very fortunate to have such an involved and passionate Chair on the Cure SMA Board. Richard has done such a tremendous amount for this organization and as a result his passion and commitment to families and Cure SMA has lifted this organization to where it is today.

**The Cure SMA Chapter Officers and Committee Members** – Throughout the US, a variety of individuals have stepped up to help spread the values and mission of Cure SMA. Every single one of these individuals sacrifice their own time with tremendous efforts to fundraise, reach out to new families and friends, help each other and stand behind Cure SMA's mission. Additionally, they also give the faces and voices to Cure SMA that provide families with local support and guidance in learning to live with SMA. We cannot thank these dedicated individuals enough!

**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.

**Jennifer Miller-Smith, Aaron Smith, Richard Curran and Expo** – The Smith family has been an amazing asset throughout the planning of the conference. They have been so gracious these past several years for donating the signage throughout conference. We cannot thank you enough for volunteering your services and expertise to this make conference as magical as it was!

**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. Your support of the type I reception, the children's program and teen social have all added wonderful components to our growing conferences!

**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** – Cure SMA appreciates the opportunity this foundation has provided to support 3 families and help they attend the Annual SMA Conference through the scholarships they provide!

**Live 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.



# 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

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



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

## Access important information such as:

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

The Cure SMA Guide is your portable source for SMA care and support. Access valuable information at the touch of a button. Share resources with your family, friends, and care providers.



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



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

**Natasha Abruzzo, NP**, lives in Albuquerque NM. Natasha is a mom of four; first son Benjamin “Bugaboo” born in 2005 and diagnosed with SMA type I at his 2 month check-up. He lived for 6 1/2 months. Daughter, Addisyn born in 2007 and diagnosed with SMA type I at 9 days old. She lived for over 14 months. Wyatt is a healthy 11 year old and is a carrier. Bode is a healthy 9 year old and is also a carrier. Natasha started the Cure SMA New Mexico Chapter. Professionally, Natasha is a certified family nurse practitioner. She has helped with the grief and loss sessions for the past two years.

**Carolyn Barrett**, is a 24-year-old recent graduate of 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 in their Professional Development Department, 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-PC** is a Pediatric Nurse Practitioner (PNP) on the Neuromuscular Team 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 served as a research coordinator, nurse, and nurse practitioner at the SMA Centers at Columbia University Medical Center and Children's Hospital Boston. She then relocated to Philadelphia to be a part of the Pediatric Advanced Care Team (PACT), prior to returning to neuromuscular care. 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.

**Elizabeth Bondarenko**, is an OTR/L located in Baltimore, MD. She is currently working as a school-based occupational therapist, where she specializes in making schools and educational curriculum accessible to students with disabilities to maximize student participation and independence. She also has experience working with older adults with acute and chronic illness to help them live safely and independently. Elizabeth is looking forward to getting married to her fiancé in September 2019. She enjoys traveling, running, reading, and playing with her two dogs.

**Anne Buckley-Reen**, is an Occupational Therapist and Therapeutic Yoga Teacher who has been utilizing developmental yoga sequences to address the needs of special populations for the past 20 years. Anne's researched program “Get Ready to Learn: Therapeutic Yoga and Mindfulness in the Classroom” is in use many school districts across the US and in Europe. She is delighted to be participating again in the Cure SMA Conference.

**Audra Perry Butler** and her husband Alan learned about Spinal Muscular Atrophy in March 2009 when their firstborn son, Andrew Glenn, was diagnosed with the disease. After losing Andy to SMA, Audra struggled with her grief. She turned to writing to help process it and advocacy to help find a new purpose. In July 2009, Audra joined the board of the Greater Florida Chapter of Cure SMA to help increase disease awareness in her state, to provide a local network of support, and to raise funds for a cure. Most recently, she was part of the successful effort to add SMA to the state's newborn screening panel. Audra lives in Land O' Lakes, Florida, with her husband and their two surviving children, Lucy and Will.

**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.

**Matthew Civitello, MPT** is a physical therapist at Nemours Children's Hospital. He works in the neuromuscular clinic and is a clinical evaluator for various research studies involving DMD and SMA.



**Kimberly Cook**, is 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 Assistive Technology Network and the Low Incidence Disability Network in Texas.

**John Day, MD**, Professor of Neurology and Pediatrics; Director, Division of Neuromuscular Medicine  
Stanford University, Stanford Neuroscience Health Center.

**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.

**Hanna Eide**, is an individual with SMA and has been using assistive technology since she was 3. She's now pursuing a career in freelance web development after graduating from college in May of 2018. She is also an amateur photographer who specializes in animal photography and photo editing. Hanna loves sharing her experiences with technology with others so that by sharing her experiences will be helpful for anyone who is involved with someone who has a disability or has a disability themselves.

**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 Philadelphia, PA.

Al provides counseling support for children with special needs and their families, has served as a consultant to health care organizations and schools, and has authored many articles on the topic of caring for children with complex medical conditions. He is a member of Cure SMA's Medical Advisory Council. Al is the father of two adult children, Jack and Cara. Jack was diagnosed with SMA Type I in 1995.

**Melissa Gibbons, MS, CCG** is an Assistant Professor of Pediatrics at University of Colorado. She is a board certified genetic counselor who specializes in Neurogenetics and Neuromuscular Diseases at Children's Hospital Colorado. She is on the Cure SMA Medical Advisory Committee.

**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.

**Kimberly Hill**, is 35 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.

**Renee Roy Hill, MS, CCC-SLP**, 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.

**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, CNSC**, 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 10 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.

**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 Children's Hospital of Philadelphia since May, 2001. For the past 15 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 as well as grief and bereavement support after death.

**Brandon Lesch**, is an Occupational Therapist, a Certified Driver Rehabilitation Specialist (CDRS) and Illinois certified driving instructor (CDI). He has specialized in driver rehabilitation at Marianjoy Rehabilitation Hospital for more than four years and has been an occupational therapist for seven years. The driver rehabilitation program at Marianjoy is one of the largest in the United States, and addresses the needs of drivers with a wide range of disabilities including teens, clients who utilize wheelchairs and require high-tech equipment, those with low vision and the elderly.

**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.

**Amy Medina**, 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 I 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 also gave birth to a daughter, Amelia, who was diagnosed with SMA. Amy's passion is educating others on SMA which includes training local EMTs on SMA care and having the local newspaper write articles on SMA.

**Mary McNaughton-Cassill, PhD**, Dr. McNaughton-Cassill, received her Ph.D. in Clinical Psychology from the UCSD/SDSU Joint Clinical Doctoral program in San Diego. She is currently a professor of Psychology at the University of Texas at San Antonio. The courses she teaches include Abnormal Psychology, Psychology and Health, Physiological Psychology and Stress Management. In her 20+ years at UTSA, she has also served as the Associate Dean of Undergraduate Affairs, and the Interim Director of the Teaching and Learning Center and the Co-Chair of the UTSA Distinguished Teaching Academy. Awards she has won include the Chancellor's Council Teaching Award, the UTSA Student Government Distinguished Faculty Award, the Honor's College Outstanding Mentorship Award, the Howe Service to Students Award and the UT Regents' Outstanding Teaching Award and the Piper Outstanding Teaching Award. She is also a Fellow of the UT Austin System Academy of Distinguished Teachers. She does research on stress and coping, has published in a variety of academic journals, and written a book about coping with stress in the modern world entitled "Mind The Gap". She is married to UTSA Biology Professor Aaron Cassill, and has 26 year-old twin daughters.

**Jennifer Miller-Smith**, is the mother of three, including two disabled daughters. Katie is 23 and has Chiari 1 Malformation and Post-Concussion Syndrome. Zach is 22 and unaffected. Madison is 12 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 in 2009 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 is the Admin for SMA Support System, the largest online forum for Spinal Muscular Atrophy worldwide. Jennifer is also a Biogen Ambassador and Avenir consultant.

**Meghan Moore, PT, DPT, NCS** is a physical therapist at The Johns Hopkins Children's Center in Baltimore, Maryland. Meghan graduated from the joint program of the University of California San Francisco and San Francisco State University DPT program in 2013. She completed the Johns Hopkins and University of Delaware Neuro residency program in 2014. She stayed at Hopkins to work with their pediatric neurological population in acute care, outpatient settings and in clinical research. She is a board-certified Neurological Specialist. She is a faculty member on the Johns Hopkins Hospital and University of Delaware Neurologic PT Residency and Johns Hopkins Hospital and George Washington University Pediatric PT Residency. Her interests include early mobility of neuro patients in the pediatric intensive care unit, and clinical research in spinal muscular atrophy.

**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.

**Diane Murrell, LCSW**, is a licensed clinical social worker in the neurology division at Texas Children's Hospital in Houston, Texas. She works in the Blue Bird Circle Clinic with families who have children with 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 website 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, PhD, 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 13 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 has served as a mentor in the Cure SMA Therapist Mentoring Program. She also serves on the Cure SMA Medical Advisory Committee.

**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 panellist 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 32 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 32 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.

**Julie Parsons, MD**, did her medical training at the 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 Habersfeld 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.

**Dan Phillips**, is a speech and language pathologist and assistive technology specialist in northern California. He is currently the director of the Technology Resource Center of Marin, [www.trcmarin.org](http://www.trcmarin.org), a site-based assistive technology center that was created and designed by Dan in 2001 and currently serves the needs of over 4,000 students in both special and general education in Marin County, California. He has been working as an assistive technology specialist for the past 25 years. He teaches at 3 Universities in the SF Bay Area, working with special needs teachers, occupational therapists and speech pathologists.

Dan has received Honors of the Association from the California Speech and Hearing Association, the highest honor in California for speech and language pathologists, as well as Outstanding program for the Technology Resource Center. He has received three California Golden Bell teaching awards for his work within his organization for exemplary teaching. Last year, he was awarded Schoolmaster of the Year as well as Marin County Teacher of the Year; he was a top 10 finalist for California Teacher of the Year. He was awarded Best School Technology Director from Tech Advocates, a national organization and is nominated for the national award for Outstanding International Contributions by the American Speech and Hearing Association.

**Kyla Pollock**, is from Southern California and has been married to Collin Pollock, Type III, for over 13 years. The two have one daughter, Elyana (10), who is unaffected by SMA. Kyla has gradually taken on the role of her husbands full-time caregiver with the disease taking away his ability to do many of the daily activities he used to do independently. In addition to being a full-time Mother and caregiver, Kyla works as Director of School Recruitment for TherapyTravelers and is co-owner of the family business WheelyO BBQ.

**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.

**Dominic Russo**, is from Carmel, Indiana. Founding member of Power Soccer of Indy and Power Soccer Development Group. Founding President of United States Power Soccer Association, 2006 - 2018. Assistant Coach for World Cup Champions 2007 US National Power Soccer Team. 2-time National Champion coach of Sudden Impact and coached several teams in Indiana. Currently serves as the Treasurer of the Power Soccer Development Group. -Add JC Russo, is 28 years old and was diagnosed with SMA type II at 18 months. He graduated from Purdue University in 2013 with a degree in Product Lifecycle Management. After graduating, he moved to Wichita, Kansas to pursue a career at Textron Aviation. JC was the Goal Keeper for the US National Power Soccer Team in their 2007 & 2011 World Cup wins. He is a board member for the Power Soccer Development Group and helps start new teams around the country. He is also a board member for Wichita Adaptive Sports to help grow Power Soccer and other adaptive sports in Wichita.

**Karen Russo**, is the mother of two children with SMA Type II. From the time of their diagnosis she was committed to fundraising and bringing awareness to this disease. After years of fundraising, power soccer was introduced to her children and it was clear the effects this sport had on their Quality of Life. She was determined to share the sport with all that could benefit. Karen Co-founded Power Soccer of Indy (PSI) with her husband and served as President from 2006- 2018. The Russos led the birth of the US Power Soccer Association simultaneously and served on the charter board as Executive Vice President, 2006- 2012. Karen currently serves as President of the Power Soccer Development Group, who's mission continues to introduce and educate potential athletes, sponsors and the public about this incredible sport. Karen also recently joined the efforts of the American Powerchair Football Confederation (APFC) as treasurer. The APFC oversees power soccer in the North, South and Central America with the same commitment to grow the sport throughout the Americas.

**Natalie Russo**, is from Carmel, Indiana and was diagnosed with SMA type II at 18 months. She graduated from Ball State University in 2011 with a degree in Journalism. She worked for several years at a corporate media company designing websites before deciding to venture off on her own and start her own web design business. She lives an independent, full life living on her own, driving a modified vehicle and playing power soccer which has taken her all over the country and world.

**JC Russo**, is 28 years old and was diagnosed with SMA type II at 18 months. He graduated from Purdue University in 2013 with a degree in Product Lifecycle Management. After graduating, he moved to Wichita, Kansas to pursue a career at Textron Aviation. JC was the Goal Keeper for the US National Power Soccer Team in their 2007 & 2011 World Cup wins. He is a board member for the Power Soccer Development Group and helps start new teams around the country. He is also a board member for Wichita Adaptive Sports to help grow Power Soccer and other adaptive sports in Wichita.

**Rachel Salazar, PT, DPT, PCS** is a board certified clinical specialist in pediatric physical therapy and a member of the multidisciplinary team at the Spinal Muscular Atrophy Clinical Research Center at Columbia University in New York City. For the past 8 years, Rachel has been involved in the evaluation and treatment of individuals with a variety of neuromuscular disorders, primarily with a research and clinical focus on individuals with SMA. As a primary clinical evaluator, Rachel's research experience includes single and multi-center observational and clinical research studies in SMA and other neuromuscular diseases. Rachel coordinates a seating and mobility equipment clinic for neuromuscular patients, which provides evaluations and custom equipment and orthotics. She participates in regional, national, and international meetings of clinicians and researchers dedicated to advancing clinical care and research in neuromuscular conditions with a focus on range of motion, adherence to rehabilitation care considerations and motor function outcome measures.

**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.

**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.

**Perry Shieh, MD, PhD**, is associate professor of Neurology at the David Geffen School of Medicine at UCLA and Director of the Neuromuscular Division in the Department of Neurology at 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 in Brigham and Women's Hospital and Massachusetts General Hospital.

**Louise Simard, PhD**, is a Professor in the Department of Biochemistry & Medical Genetics in the Rady Faculty of Health Sciences and Associate Dean in the Faculty of Graduate Studies 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, MD, PhD**, 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.

**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.

**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).

**Danyelle Sun**, 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.

**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 11 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.

**William Taft, MD**, is a Clinical Associate Professor of Psychiatry and Pediatrics at the University of Wisconsin School of Medicine and Public Health. His practice includes inpatient consultations at American Family Children's Hospital in Madison and outpatient clinic. Over the last nine years he has worked with many patients who live with both physical and mental health challenges, including cancer, SMA, genetic and developmental disorders.

**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, RN, PhD**, is a retired Air Force Colonel and a nurse educator from Portland, Oregon. The death of his son, Jonathan, brought him to the self-help bereavement group, The Compassionate Friends, and led to his study of how we 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 member of the Cure SMA Medical Advisory Council.

**Brynne Willis**, is an LGPC located in Baltimore, Maryland. She specializes in addressing 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. Currently, Brynne is a therapist at Safe Harbor Christian Counseling providing individual and group counseling using positive psychology as a primary intervention to mediate anxiety and stress. Brynne will be attending law school at Case Western Reserve Law school in Cleveland, Ohio starting August 2019. 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.

**Angel Wolff**, is mother to 2 girls, Madison, type II (age 16) and Ella (age 12). Angel is the Chapter President of the Arizona Chapter and has been involved with the chapter for many years, supporting many families over the years.

**Ryan Wolff**, is father to Madison, type II (age 16) and Ella (age 12). 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.

**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."



## 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!



# 2019



## OFFICIAL CURE SMA MERCHANDISE

Items will be on sale at the merchandise table next to registration! Make sure to stop by and check it out while supplies last!



### ITEMS SNEAK PEAK:

- Zip up Hoodie
- Beanies
- Nursing Scrub Top
- Windbreaker Jacket
- Baseball Tees
- Performance Polos
- Baseball Hats
- Performance Half Zips
- Men's, Womens and Youth T-shirts
- Onesies
- Popsocket
- Vacuum Insulated Bottle
- Crewneck Sweatshirts
- Tote Bag



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Cure SMA is very fortunate to have such a dedicated group of individuals, who represent the SMA community. Our Board Members work exceptionally hard to make sure that all 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.

## OFFICERS



### **Richard M. Rubenstein – Chairman of the Board**

Richard is an 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.



### **Nick Farrell – Vice Chair**

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 Cure SMA, with the arrival of their newly-diagnosed care package. Since then they have been proud to support Cure SMA 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.



### **Gillian Mullins – Secretary**

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, two daughters Keaveny and Reid, and son, Quinn.



### **Peter Statile – Treasurer**

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. With over a 30-year career, many of Pete's corporate assignments involved acquisitions, divestitures, growth initiatives, and integration of diverse businesses. The integrated businesses he helped create resulted in transformational value realization. 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. For the 10 years prior to this, he held senior level positions at three companies with common private equity ownership. He was President and Chief Operating Officer of Precision Partners, a \$450M manufacturing and engineering services provider to the aerospace, defense, power generation, and transportation industries. Pete is a member of The Economic Club of New York and holds a Bachelor of Science degree from Brooklyn College, Brooklyn, NY.

## EXECUTIVE COMMITTEE

**Robert Graham, MD – Liaison of Science and Medicine**

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.

**Rob Lockwood – Liaison of Development and Marketing**

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.

**Bradley A. Nunemaker – Chair of Audit and Compliance**

Brad was diagnosed with SMA Type III when he was nine years old. He joined the Cure SMA Board of Directors in 2012. Brad received his BBA from the University of Michigan Business School. He is currently the Vice President, Finance – Health Solutions at Alight Solutions and is a certified public accountant. He is excited to continue to grow the strong community of adults with SMA and to expand awareness and research that will ultimately result in treatments and a cure for SMA. In April 2017, Brad was a panellist in the Patient-Focused Drug Development meeting with the FDA and shared his experiences on the impacts of living with SMA. Brad is the current chair of the Audit and Compliance Committee on the Board of Directors of Cure SMA, and previously served as Treasurer and Finance Committee chair. Brad previously served as Treasurer on the Board of Directors for Onward Neighborhood House, a community based not-for-profit in Chicago. Brad and his wife Krista live in Elmhurst, Illinois with their two sons. The whole family is proud to support Cure SMA.

**Spencer Perlman – Liaison of Public and Advocacy**

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.

**Danyelle Sun – Liaison of Events, Chapters, and Family Support**

Dany graduated from UW-Milwaukee with a Bachelor's and Master's degree 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.



## BOARD MEMBERS

**Corey Braastad**

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.

**Kelly Cole**

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.

**Matthew Evans**

Dr. Evans is a scientific leader within the genomics research and development department of Covance Drug Discovery, part of LabCorp. Matthew received his Ph.D. in Molecular Biology in 2011 from the University of Massachusetts Medical School. Matthew's work aimed to better understand the mechanisms of action of several novel therapeutic targets in SMA. Matthew has several years of experience in the leadership and development of genomic scientist teams in diagnostic and clinical trial product development. Matthew with his wife Kathleen live in Indiana with their three young girls. He is thankful for all the years that he has been able to remain connected to Cure SMA, the entire SMA community, and excited for continued progress towards a cure.

**Jaclyn Greenwood**

Jaclyn is from Southern California and was diagnosed with SMA type II at 18 months. She graduated from Chapman University with a BS in Molecular Biology. She went on to earn a Master's 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. Jaclyn lives an active life and enjoys driving a modified van, playing board games, traveling, and rooting for her 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**

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 startup 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.





### Annie Kennedy

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 implementation of key provisions within PDUFA VI and the 21st Century Cures Act, MD-CARE Act implementation, engagement with the FDA and Industry around regulatory policy and therapeutic pipelines, launching a national newborn screening pilot program, developing resources for adults with Duchenne, optimizing clinical trial infrastructure, and innovating around therapy valuation and access issues. Annie currently serves on the Board of Directors of Cure SMA, on the steering committee of "Transition to Care" coalition, as Co-Chair of the National Health Council's Medical Innovation Action Team, and recently served as a Design Team member of the NCATS/ORDR Tool Kit Project and on the FasterCures Patients Count Leadership Council.



### Edmund Lee

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.



### Tom Murray

Thomas Murray, PhD, is President Emeritus at The Hastings Center. He was formerly the Director of the Center for Bio-medical 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 a member of the Ethics Board and the Disciplinary Tribunal 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 300 publications. His newest book is Good Sport: Why Our Games Matter – and How Doping Undermines Them.



### Shannon Shryne

Shannon is the co-founder and President of Augie's Quest to Cure ALS. Under Shannon's leadership, Augie's Quest has raised over \$70 million to fund cutting edge ALS research. She has 25 years' experience in non-profit development and leadership with expertise in corporate engagement, 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 also serves on the Advisory Boards of the Kevin Turner Foundation and After the Impact Fund.



### Brian Snyder

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.



### Shannon Zerzan

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.



### Alexandrea Wolfe

Alexandrea (Allie) Wolfe, graduated from Russell Sage College with a Bachelor of Arts in Psychology in 2013. Her passion has always been concentrated on young children and adolescents focusing on their mental, emotional, and social growth. In the summer of 2016, Allie's son, Brooks Wolfe was diagnosed with Spinal Muscular Atrophy at 8 months of age. Thereafter, Allie realized she needed to be pro-active within the SMA community. Allie worked her way to being one of the Greater New York Capital Regions Chapter leaders where she strives to be as helpful as possible to anyone engaged with SMA. Since then, supporting the community has been a goal she works at daily.



### Carolyn Barrett

Carolyn Barrett works at a Boston legal non-profit, supporting their professional development department. She holds a Bachelor 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.



### Cathy Barsotti-Dooling

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

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 Marathon and 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 someday 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.



### Allyson Henkel

Allyson is a substitute teacher and volunteer Spanish interpreter on legal cases dealing with custody and immigration. She serves on the Hoyas Unlimited athletic board at Georgetown University. Her previous work experience includes 8 years at Lord, Abbett & Co., an investment management firm, and 15 years teaching Spanish. Allyson and her husband Tim live in Rosemont, Pennsylvania. They have four children, one of whom, Peter, was born with type II SMA. Allyson has been involved with the Pennsylvania Chapter of Cure SMA for 13 years supporting families, fundraising, raising awareness and working on advocacy. Tim, Allyson and their children Peter and Lucy, along with other members of the Pennsylvania Chapter of Cure SMA, were instrumental in securing the approval to add SMA to the newborn screening panel in Pennsylvania. The Henkel Family, through Hosts for Hospitals, also hosts patients who are traveling to Philadelphia for treatment. Allyson is proud to remember and honor her son Pete through her work with Cure SMA.



### Kelly Jankowski

Kelly Jankowski is a communications and marketing consultant at Edelman, a global public relations firm where she works with both corporate and non-profit organizations to help them articulate their purpose, protect their reputations, and tell stories about their work and products. As the head she runs programs for a dozen organizations, including PayPal, Regeneron, Yale University and Booking Holdings. When her first child, William was diagnosed with SMA, Type 1, at 12 weeks old, Kelly immediately began to work with Cure SMA and the broad network of pharmaceutical and medical providers working on treatments. William gained access to Spinraza during the early access trials, and he got stronger, but he died in May 2017, a few days after his first birthday. She and her husband Chris host several fundraisers for Cure SMA annually and are involved in chapter events nationally, in Philadelphia, and New York City. Kelly earned a BA from Duke and an MBA from the Yale School of Management. She is a member of the Milken Institute Young Leaders Circle, a participant in the Aspen Institute's Society of Fellows.



### Kathy Klinger

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.



### Yahnatan Lasko

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.



### Srin Madipalli

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.



### Catherine Martin

Catherine Martin is Head of Legal for OakNorth US, a financial technology firm focused on small business and middle market lending. Prior to this role she was Director and Associate General Counsel at Citigroup in New York where she was primary legal coverage for several of Citi's credit trading businesses and specialized 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 and Bankruptcy and Restructuring practice. Ms. Martin received a J.D., magna cum laude, from Syracuse University College of Law and is a member of the New York Bar.



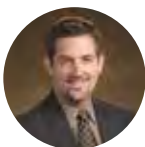
### Mary McHale

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, 18 years old with Type II SMA and Robby, 15 unaffected. Mary is the co-Chair of Concert for a Cure, an annual gala which has raised over \$2.2 million dollars for Cure SMA. Mary holds a Bachelor of Science degree from the University of California at Davis in Human Development and Communication. She received her Master 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 Coach with CS Advising, Mary enjoys helping clients achieve their career goals.



### Amy Medina

Amy graduated from Marian University with a Bachelor 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.



### Steven Ragland

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 Keker 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.



### **Charlotte Sumner, MD**

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 nonprofit disease foundations including the Packard Center for ALS research, Muscular Dystrophy Association, Cure SMA, and the SMA Foundation.



### **Patrick Woodruff**

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.





# SUMMIT OF STRENGTH

## SCHEDULE 2019



The 2019 Summit of Strength Program includes 20 single-day educational events hosted by Cure SMA. These conferences are crafted to provide information for people of all ages and types of SMA, and their loved ones and caregivers. Join us to network with other SMA families and learn from national and local experts about the latest advances in treatment, care, advocacy and support.

**THIS IS A FREE PROGRAM WHICH INCLUDES BREAKFAST, LUNCH AND PARKING FOR ALL ATTENDEES.**



The dates and cities for the 2019 Summit of Strength Program are:

- |                               |                                |
|-------------------------------|--------------------------------|
| March 2 – Dallas, TX          | August 24 – Cincinnati, OH     |
| March 9 – Fort Lauderdale, FL | August 31 – Anaheim, CA        |
| March 30 – Seattle, WA        | September 7 – Philadelphia, PA |
| May 4 – Grand Rapids, MI      | September 14 – Rochester, NY   |
| May 11 – Milwaukee, WI        | September 21 – Minneapolis, MN |
| June 1 – Kansas City, MO      | September 28 – Newark, NJ      |
| July 20 – Denver, CO          | October 5 – San Ramon, CA      |
| July 27 – Salt Lake City, UT  | October 12 – Boston, MA        |
| August 3 – Long Island, NY    | October 19 – Charleston, SC    |
| August 10 – Chicago, IL       | November 9 – Orlando, FL       |



Registration is open – Visit the Cure SMA Events Calendar at <http://www.curesma.org/get-involved/event-calendar/> to register for your local Summit of Strength today!

Thank you to the 2019 Summit of Strength Program's National Presenting Sponsors: AveXis, Biogen and Genentech/Roche. We would also like to recognize Platinum Sponsor, Scholar Rock for their support.



**A local event uniting the SMA community around treatment, care, and support**

**This is a free program which includes breakfast, lunch, and parking for all attendees.**

**For more info or questions please email [familysupport@curesma.org](mailto:familysupport@curesma.org) | 800.886.1762 | [cureSMA.org](http://cureSMA.org)**



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[twitter.com/cureSMA](https://twitter.com/cureSMA)



[facebook.com/cureSMA](https://www.facebook.com/cureSMA)



[www.youtube.com/c/CureSMA](https://www.youtube.com/c/CureSMA)

# Medical Advisory Council

The MAC is one of the most highly respected bodies of SMA medical and clinical experts in the United States, including experts representing multiple specialty areas. The MAC focuses on educating families, healthcare providers, and the public about SMA; expanding SMA standards of care; and translating positive research results into clinical practice.



## **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, MSN**

Pediatric Nurse Practitioner and Neuromuscular Care Coordinator at Children's Hospital Colorado in Denver, CO.



## **Vanessa Battista, RN, MS, CPNP – Palliative Care and Quality of Life Subcommittee Chair**

Palliative Care Pediatric Nurse Practitioner at Children's Hospital of Philadelphia in Philadelphia, PA.



## **William Bell, BS Pharm, MBA, MSCC, RPh**

Director of Clinical Client Services in Chalfont, PA.



## **John Brandsema, MD**

specializes in Pediatric Neuromuscular Neurology at Children's Hospital of Philadelphia, associated with the Perelman School of Medicine at The University of Pennsylvania in Philadelphia, PA.



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

Pediatric Neuromuscular Advanced Practice Nurse at Lurie Children's Hospital in Chicago, IL.



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

Genetic Counselor at Massachusetts General Hospital in Boston, MA.



## **Thomas Crawford, MD**

specializes in Pediatric Neurology at Johns Hopkins Hospital, associated with Johns Hopkins University in Baltimore, MD.



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

Physical Therapist at Stanford University in Stanford, CA.


**Bakri Elsheikh, MBBS, FRCP**

specializes in Neurology, Neuromuscular Medicine, and Clinical Neurophysiology at The Ohio State University Wexner Medical Center in Columbus, OH.


**Albert Freedman, PhD**

specializes in Psychology in independent practice in Philadelphia, PA.


**Melissa Gibbons, MS, CGC**

Genetic Counselor at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.


**Jennifer Hubbell, OTR/L**

Occupational Therapist at Cincinnati Children's Hospital Medical Center in Cincinnati, OH.


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

Pediatric Nutritionist at Intermountain Healthcare in Salt Lake City, UT.


**Chamindra Konersman, MD**

Neurologist at Rady Children's Hospital, associated with the University of California San Diego in San Diego, CA.


**Richard M. Kravitz, MD**

Pediatric Pulmonologist and Sleep Medicine Physician at Duke University in Durham, NC.


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

Pediatric Physical Therapist at Northwestern University in Chicago, IL.


**Oren Kupfer, MD**

Pediatric Pulmonologist at Children's Hospital Colorado, associated with the University Of Colorado School Of Medicine in Denver, CO.


**Khalida Liaquat, MS, LCGC**

Genetic Counselor at Athena Diagnostics in Marlborough, MA.



**Diane Murrell, LCSW – Care Coordination, Case Management, & Nursing Subcommittee Chair**  
Social Worker at Texas Children's Hospital in Houston, TX.



**Leslie Nelson, PT, PhD, OCS**  
Pediatric Neuromuscular Physical Therapist at Children's Health, associated with the University of Texas Southwestern in Dallas, TX.



**Julie Parsons, MD – Neurology Subcommittee Chair**  
Pediatric Neurologist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.



**Silvana Ribaud, MD – Genetics, OB/GYN, & Family Planning Subcommittee Chair**  
Obstetrician and Gynecologist at Columbia University in New York City, NY.



**Samuel Rosenfeld, MD**  
Orthopedic Surgeon at CHOC Children's Hospital in Orange, CA.



**Peter Schochet, MD**  
Pediatric Pulmonologist at Children's Health, associated with the University of Texas Southwestern in Dallas, TX.



**Perry Shieh, MD, PhD – Adult Care Subcommittee Chair**  
Neurologist at UCLA Medical Center in Los Angeles, CA.



**Edward C. Smith, MD**  
Pediatric Neurologist at Duke University in Durham, NC.



**Anne Stratton, MD**  
Pediatric Physiatrist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.





**Stacey Tarrant, BS, RD, LDN**

Nutritionist at Boston Children's Hospital in Boston, MA.



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

Pediatric Pulmonologist at the Children's Mercy Kansas City, associated with the University of Missouri – Kansas City in Kansas City, MO.



**Fred W. Troutman, PhD, RN**

Nurse Educator, Professor Emeritus at Walla Walla University in Portland, OR.



**Laura Watne, MS RD CSP**

Nutritionist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.



## PLEASE NOTE

### WEAR SUNSCREEN AND KEEP HYDRATED!

As many of you know, June/July in California can be very hot during the day. We strongly encourage everyone to take 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!

# 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.


**Christine DiDonato, P.h.D.**

Dr. DiDonato is an Associate Professor of Pediatrics at the Northwestern University Feinberg School of Medicine. Dr. DiDonato has developed a translational research program for SMA. The research is multi-faceted and uses biochemistry, cell biology, molecular

biology, and animal modeling. Her team uses these approaches to decipher SMN function within nerve and muscle, two tissues affected in SMA. She also has developed a series of SMN mutations in model mice that mimic human SMN2 splicing.


**Katherine Klinger, PhD**

Dr. Klinger is Global Head of Translational Sciences at Genzyme. She 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 both nationally and internationally, is widely published in many journals and has authored

numerous book chapters. Dr. Klinger has extensive experience in translational drug development.


**Charlotte Sumner, MD**

Dr. Sumner is a Professor of Neurology and Neuroscience at Johns Hopkins University School of Medicine. In her role as a physician, she co-directs the Johns Hopkins SMA and Charcot Marie Tooth disease clinics. Dr. Sumner's research focuses on the genetic

and cellular pathogenesis of SMA with particular attention to characterization of molecular and cellular mechanisms underlying disease progression and preclinical development of novel therapeutics.

## 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



# Cure SMA Translational Advisory Council (TAC)

## OFFICIAL MEMBERS



Christine Brideau Ph.D., Vice President, In Vitro Biology, WuXi AppTec, Preclinical Drug Development



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



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



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



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



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



Peter Hodder, Ph.D., Executive Director of Discovery Research, Amgen Inc., Assay Development and High-Throughput Screening



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



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



Michael Vellard, PhD, Vice President of Research, Ultragenyx Pharmaceutical, Preclinical Drug Development



Joseph W. Lewcock Ph.D., Head of Biology Discovery, Denali Therapeutics, Preclinical Drug Development



Chien-Ping Ko, PhD, Professor, University of Southern California, SMA Biology/Neurobiology



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



Dione Kobayashi, PhD, Executive Scientist and Rare Disease Drug Developer, Myonex Therapeutics, Inc., Preclinical Drug Development

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

# Join us for a Cure SMA Walk-n-Roll



The Cure SMA Walk-n-Roll is a fun, family friendly event that supports Cure SMA's mission of driving breakthroughs in treatment and care and providing families the support they need for today. We welcome you to join us as an individual or start a team with your family, friends, co-workers, and neighbors.

The Walk-n-Roll is a fun filled day celebrating the strength of the SMA community. By supporting a Walk-n-Roll and raising money, you will help fund programs that will continue to change the course of SMA for everyone affected – from infants to adults – and eventually lead to a cure.

## WANT TO JOIN US AT OUR NEXT EVENT?



Visit [events.curesma.org/WalknRoll](https://events.curesma.org/WalknRoll)  
to find an event near you!

# Celebrating our 30th Conference this year!

2019



<b>2019</b> - Disneyland Hotel	Anaheim, California
<b>2018</b> - Hilton Anatole Hotel	Dallas, Texas
<b>2017</b> - Disney World Contemporary Resort	Orlando, Florida
<b>2016</b> - Disneyland Hotel	Anaheim, California
<b>2015</b> - Westin and Sheraton Hotels	Kansas City, Missouri
<b>2014</b> - Gaylord National	Washington, DC
<b>2013</b> - Disneyland Hotel	Anaheim, California
<b>2012</b> - DoubleTree-Bloomington	Bloomington, Minnesota
<b>2011</b> - Disney World 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

1988



**Kenneth Hobby**  
president

## RESEARCH



**Jill Jarecki PhD**  
chief scientific officer



**Mary Curry, ND**  
senior manager,  
clinical affairs



**Jacqueline Glascock, PhD**  
senior scientific program  
manager



**Rosangel Cruz, MA, BS**  
research director of  
clinical affairs



**Lisa Belter, MPH**  
senior research  
data analyst



**Allison Mazzella**  
research associate

## CLINICAL CARE



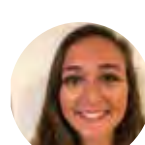
**Mary Schroth, MD**  
chief medical officer



**Teresa Stewart, MBA, BSN, RN**  
director, clinical care operations



**Jamie Shish, RD, LDN**  
director, care center  
network



**Abigail Paras**  
clinical care program  
manager



**Adrian Segura**  
senior clinical care  
data analyst

## EVENTS AND FAMILY SUPPORT



**Colleen McCarthy O'Toole**  
vice president, events &  
family support



**Shannon O'Brien**  
director, events &  
family support



**Amber Snyder**  
director, events & family support



**Jessica Clark**  
senior manager, events  
& family support



**Karen O'Brien**  
manager, events & family support



**Sara Lipscomb**  
manager, events  
& family support



**Katie Mogell**  
manager, events &  
family support



**Anna Parker**  
manager, events &  
family support



**Hannah Tolman**  
manager, events &  
family support



**Eileen Venedam**  
manager, events &  
family support





**Brittany Richter**  
senior coordinator,  
events & family support



**Alexandra Costelle**  
coordinator, events  
& family support



**Samanta Rzany**  
coordinator, events  
& family support



**Kathleen Wittmann**  
assistant coordinator,  
events & family support



**Jaki Herrmann**  
administrative associate,  
events & family support



**Alec Basso**  
warehouse associate,  
events & family support

## POLICY AND ADVOCACY



**Jaimie Vickery**  
vice president, policy & advocacy



**Helena Hernandez**  
coordinator, policy & advocacy

## DEVELOPMENT, MARKETING AND COMMUNICATIONS



**Erin Kelly**  
vice president, development



**Megan Lenz**  
director of marketing  
and communications



**Brett Kinley**  
senior manager,  
development



**Michelle Pantoja**  
marketing manager



**Alyssa Zavislak**  
development manager



**Sarah McCall**  
communications coordinator



**Tiffany Sugar**  
senior manager, graphic design

## FINANCE & OPERATIONS



**Marline Pagan, MBA**  
vice president, finance and  
operations



**Sherryl Dunning**  
accounting manager



**Alissa deRamus**  
executive assistant



**Foster Lewin**  
senior manager, database  
and systems



**Jonathan Roth**  
coordinator, data quality  
& IT support



**Jo Holland**  
data entry clerk



**Meghan Nordstrom**  
staff accountant



**Jessica Drew**  
hr generalist

# Researcher and Clinicians Registered

AS OF MAY 17, 2019

<b>Tasfia Ahmed</b> AveXis, Inc	<b>Alison Ballard</b> Childrens Hospital Colorado	<b>John Brandsema</b> Children's Hospital of Philadelphia	<b>Angela Chaves</b> Biogen
<b>Jennifer Aikin</b> AveXis	<b>Giovanni Baranello</b> UCL GOS Institute of Child Health	<b>Marie-Eve Brault</b> Skyhawk Therapeutics	<b>Juan Chavez</b> Biogen
<b>Senda Ajroud-Driss</b> Northwestern University	<b>Bart Bartels</b> University Medical Center Utrecht	<b>Karla Brigatti</b> Clinic for Special Children	<b>Helena Chaytow</b> University of Edinburgh
<b>Alisha Alaimo</b> Biogen	<b>Michela Bassano</b> Ospedale Bufalini, Cesena	<b>Sabrina Brochu</b> Biogen	<b>Loucia Chehade</b> The Ottawa Hospital Research Institute
<b>Katia Alberti</b> Fondazione Irccs Ca' granda Policlinico	<b>Vanessa Battista</b> Children's Hospital of Philadelphia	<b>Bethany Brown</b> Genentech	<b>Helen (Jin Yun) Chen</b> MGH
<b>Alberto Aleman</b> IMAC (Instituto Médico de Alta Complejida)	<b>William Bell</b> Southern Scripts	<b>Cindy Budek</b> Lurie Children's Hospital of Chicago	<b>Tai-Heng Chen</b> Kaohsiung Medical University Hospital
<b>Lindsay Alfano</b> Nationwide Children's Hospital	<b>Rakesh Bera</b> AveXis Inc	<b>Jannik Buettner</b> University of Leipzig	<b>Er Chen</b> Genentech
<b>Patrizia Allegra</b> Cytokinetics	<b>Meagan Bernatz</b> University of Utah	<b>Arthur Burghes</b> Ohio State University	<b>Jun-An Chen</b> Academia Sinica
<b>Megan Allen</b> MediTech Media	<b>Saunders Bernes</b> Phoenix Children's Hospital	<b>Laura Burke</b> Roche Products Ltd	<b>Karen Chen</b> SMA Foundation
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# CURE SMA TEEN AND ADULT SUPPORT PACKAGE

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

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

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



***SAVE THE DATE***



**NATIONAL DISABILITY EMPLOYMENT  
AWARENESS MONTH  
IS IN OCTOBER!**

Cure SMA will be participating in NDEAM activities all month long. NDEAM is a fantastic opportunity to learn about the experiences of adults living with SMA, as they navigate the successes and challenges of job searches and career paths. SMA can take away the ability to walk, eat, or breathe but thanks to new treatments and better standard of care, people with SMA are living longer and stronger. Cure SMA understands the need for more information, services and support for adults living with SMA.

Cure SMA hosted its first NDEAM webinar on October 2018. Four panellists who live with SMA shared their career journey and experiences.

Are you interested in sharing your story during NDEAM? Email us at [stories@curesma.org](mailto:stories@curesma.org)!



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[youtube.com/user/FamiliesofSMA1](https://youtube.com/user/FamiliesofSMA1)



[www.linkedin.com/company/families-of-sma](https://www.linkedin.com/company/families-of-sma)



**cure  
SMA**

# **cure** **SMA** **FILL OUT THE** **CONFERENCE SURVEY** FOR YOUR CHANCE TO WIN AN APPLE WATCH

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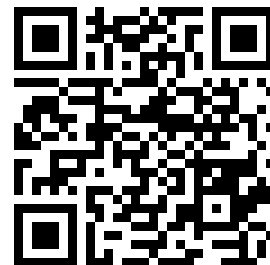


The success of our conference can only be improved by the feedback that we received from you! Please take the time to provide any comments by filling out our conference survey, and the workshop surveys. You can find hard copies of your surveys in your conference folder. Please drop your completed surveys 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 Monday, July 1st, will have their name entered into a raffle.**

**The winners of the raffle will receive a brand new Apple Watch!**

All completed surveys will also be entered into a drawing for a chance to win a trip, including airfare to the 2020 Annual SMA Conference (the locations will be announced on Monday). The winners will be drawn and announced on Monday, July 1st at the Closing General Session/It's a Wonderful Life.



**You can also fill out the survey on-line at <http://events.curesma.org/2019annualsmaconference>**



## **Amy Marquez Scholarship**

For the 2019 Annual SMA Conference, the Amy Marquez Scholarship is in its seventh 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.

# Disneyland Hotel

## HOTEL FEATURES & SERVICES

- 1 Front Desk
- 2 Guest Services
- 3 Bell & Valet Services
- 4 Rose Court Garden
- 5 Adventure Lawn
- 6 Frontier Lawn
- 7 Magic Kingdom® Lawn
- 8 Fitness Center
- 9 Guest Laundry
- 10 Business Center

## SHOPPING & RECREATION

- 11 Disney's Fantasia Shop
- 12 small world Gifts & Sundries
- 13 Monorail Pool & Slides
- 14 D Ticket Pool
- 15 E Ticket Pool
- 16 Outdoor Fireplace

## RESTAURANTS & LOUNGES

- 17 Steakhouse 55 & Lounge
- 18 Goofy's Kitchen
- 19 Tangaroa Terrace
- 20 Trader Sam's
- 21 The Coffee House

## LEGEND

- Elevators
- Restrooms
- Telephones
- ATM
- Monorail Station
- Bus Pick-Up
- Designated Smoking Areas
- Automated External Defibrillator



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## CONVENTION & BANQUET FACILITIES

### Convention Center

#### Lower Level (Entrance Near Steakhouse 55)

- Castle A-C Room
- Monorail A-C Room

### Main Level

- Disneyland® Grand Ballroom
- North, Center, & South Ballroom
- North, Center, & South Lounge
- Disneyland® Exhibit Hall

### Upper Level

- Magic Kingdom® Ballroom 1-4
- Sleeping Beauty Pavilion
- A Ticket Room
- B Ticket Room

## GUEST ROOMS

- Fantasy Tower: \_\_ 00 - \_\_ 35  
 Adventure Tower: \_\_ 36 - \_\_ 67  
 Frontier Tower: \_\_ 68 - \_\_ 99  
 (Add first two numbers for floor number)

### Adventure Tower

- Nile
- Congo
- Zambezi
- Amazon
- Oasis
- Tiki
- Safari
- Adventure
- Outpost
- Explorer

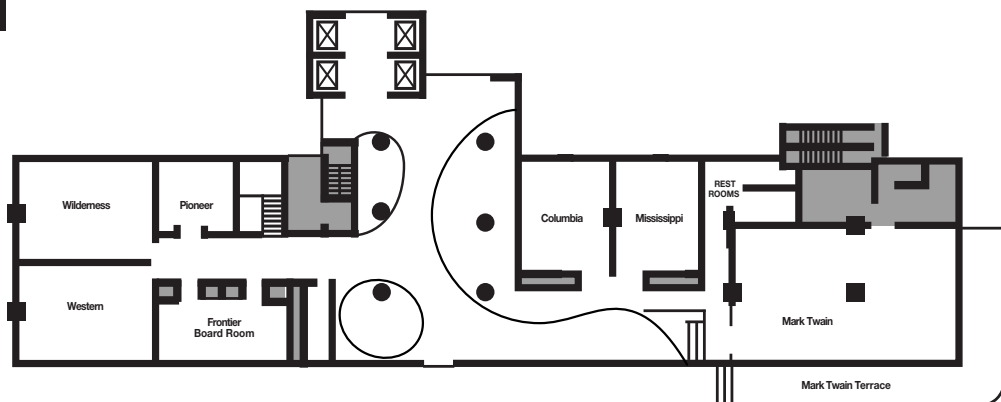
### Frontier Tower

- Western
- Wilderness
- Pioneer
- Columbia
- Mississippi
- Mark Twain
- Frontier Board Room

### Outdoor Event Areas

- Rose Court Garden
- Adventure Lawn
- Frontier Lawn
- Magic Kingdom® Lawn

## CONVENTION CENTER



**See you next year!**