



2023 ANNUAL SMA CONFERENCE



PROGRAM GUIDE

JUNE 29 – JULY 2, 2023 | ORLANDO, FL

#2023SMACONFERENCE

WELCOME LETTER

Welcome to the 2023 Annual SMA Conference—our first conference on the East Coast in six years!

This year's conference is our largest ever, and the excitement and power that comes from seeing our community all together at such a scale is unmatched.

We will recognize and celebrate all the progress our community has made, while also looking forward towards our goals for the future.

We have three powerful SMA treatments, with three quarters of all affected individuals in the U.S. getting access. There are more clinical trials happening than ever before! We have newborn screening across 99% of the country and hope to be celebrating 100% soon. And we are seeing progress with diagnosing and treating SMA around the world. While we recognize these breakthroughs, we are also cognizant of the work we must continue to do. The SMA landscape has changed significantly over the last six years, and we are expanding our research, care, and support programs accordingly. It is important now to optimize the impact of our current therapies and to develop new combination approaches and additional therapeutic targets to focus on restoring and building strength. As we continue to get back to in-person activities for our SMA community, including fundraising events, we are very excited to be able to continue our basic research funding. Leading early research on understanding the disease and identifying treatment targets has led to the significant changes and outcomes that are now occurring. But our community has new needs and there is more work for us to fund.

One of our important new initiatives is our SMA Care Center Network. Care Centers guide our understanding of how SMA is changing, and how care is being delivered at the local level and help us identify best practices. We currently have 29 Care Centers across the U.S. with the goal to double that in the coming years.

In addition to the record attendance this year, we also saw a 35% increase in the number of research and care abstracts submitted to the Annual SMA Research and Clinical Care Meeting. There will be more than 30 research and care presentations, in addition to two poster sessions during the meeting. We look forward to our joint researcher and community member events like the Meet and Greet on Thursday evening and Family Friendly Researcher Poster Session on Friday.

During the scientific sessions, researchers from industry and academia will meet to create open communication, accelerating the pace of research. During the care meeting, multidisciplinary clinicians will share knowledge to optimize care and grow our SMA community of healthcare professionals. On the individual and family side, we have over 60 workshop sessions 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.

This conference is generously supported by 12 sponsors and 19 exhibitors. Our special thanks to Genentech and Biogen as the Presenting Sponsors of the 2023 Annual SMA Conference. Through these sponsorships, we are able to provide all of these incredible workshops and events, as well as assistance for travel and registration costs for so many attendees!

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

Sincerely,



Kenneth Hobby
President



Colleen McCarthy O'Toole
Senior Vice President, Community Support



Mary Schroth, MD
Chief Medical Officer



Jackie Glascock, PhD
Vice President, Research

Index

Conference Workshop
Page 18

Special Events
Page 19

Conference Menu
Page 26

Conference Agenda
Page 33

Children's Program
Entertainment Schedule
Page 54

Speaker Bios
Page 73

Exhibitor Map
Page 127



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 organization'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 MISSION

Cure SMA leads the way to a world where everyone impacted by spinal muscular atrophy is empowered to lead independent, successful, and fulfilling lives. We strive to create a community where every individual is heard and feels welcomed. Cure SMA provides practical support programs for our community and advocates for their needs. We fund and direct comprehensive research that drives breakthroughs in treatment and we advance access to high quality care. We will not stop until we have a cure.

OUR VALUES

Innovation

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

Balance

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

Collaboration

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

Respect

There is no “right way” to live with a disease like spinal muscular atrophy. Every person's experience is different, and it is 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 with SMA and their families, 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 will remain strong in our fight no matter what challenges come our way.

ILLNESS AND EMERGENCY INFORMATION

IF YOU HAVE AN ILLNESS OR HEALTH EMERGENCY WHILE AT THE ANNUAL SMA CONFERENCE PLEASE SEE BELOW:

If you are experiencing a life-threatening emergency, please call 911.

If you are not feeling well, please take care of yourself. We also ask that you avoid others at the Annual SMA Conference until you are feeling well.

If you or someone you are caring for experiences any of the following, please go to an Emergency Department. Please take all personal medical equipment with you.

- Difficulty breathing
- Low oxygen saturations
- Persistent fever
- Worsening of symptoms despite implementation of illness protocols
- Not able to keep fluids in
- Equipment failure
- Injury and/or possible broken bones

WHERE TO GO IF AN EMERGENCY OCCURS:

Pediatric Care:

**Nemours Children's Hospital
Emergency Department**
6535 Nemours Parkway
Orlando, FL 32827
407-561-4000

**Nemours Children's Health
Urgent Care**
750 Centerview BLVD
Kissimmee, FL 34741
407-850-3497

Adult Care:

**AdventHealth Celebration Hospital
Emergency Department**
400 Celebration Place
Celebration, FL 34747
407-303-4000

**AdventHealth Centra Care
Lake Buena Vista Urgent Care**
12500 South Apopka Vineland Road
Orlando, FL 32836
407-934-2273

Please note that there are multiple healthcare providers in the Orlando area.

Additional information is available at the Registration Desk.



SATURDAY IS CONFERENCE T-SHIRT DAY!



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

Cure SMA would like to thank our Sponsors!

Presenting Sponsors



Diamond Sponsor



WELCOME!

We are honored to have SMA families, researchers, and clinicians attend this conference from all over the world! There are individuals attending from 46 countries, including: Argentina, Australia, Austria, Bangladesh, Belgium, Brazil, Bulgaria, Canada, Chile, China, Colombia, Costa Rica, Czech Republic, Ecuador, Finland, France, Georgia, Germany, Hungary, Iceland, India, Ireland, Italy, Japan, Jordan, Malaysia, Mexico, Morocco, Netherlands, Norway, Pakistan, Panama, Paraguay, Peru, Poland, Portugal, Puerto Rico, Slovenia, Spain, Switzerland, Taiwan, Turkey, United Kingdom, United States, Uruguay, and Vietnam.



As of April 26, 2023

Celebrating All The Years!

2023



2023 - Disney's Yacht and Beach Club Resort	Orlando, Florida
2022 - Disneyland Hotel	Anaheim, California
2021 - Virtual SMA Conference	At Home
2020 - Virtual SMA Conference	At Home
2019 - Disneyland Hotel	Anaheim, California
2018 - Hilton Anatole Hotel	Dallas, Texas
2017 - Disney World Contemporary Resort	Orlando, Florida
2016 - Disneyland Hotel	Anaheim, California
2015 - Westin and Sheraton Hotels	Kansas City, Missouri
2014 - Gaylord National	Washington, D.C.
2013 - Disneyland Hotel	Anaheim, California
2012 - DoubleTree-Bloomington	Bloomington, Minnesota
2011 - Disney World Swan and Dolphin Resort	Orlando, Florida
2010 - Marriott-Santa Clara	Santa Clara, California
2009 - Hyatt Regency Cincinnati	Cincinnati, Ohio
2008 - Beantown-Boston Park Plaza	Boston, Massachusetts
2007 - Moving Forward-Hyatt Regency Woodfield	Schaumburg, Illinois
2006 - Surfin' SMA-Mission Valley Marriott	San Diego, California
2006 - International SMA Research Meeting	Montreal, Canada
2005 - Together for a Cure-Marriott Downtown	Philadelphia, Pennsylvania
2004 - Connect for a Cure-Hyatt Regency Woodfield	Schaumburg, Illinois
2003 - SMA Takes the Hill-Hyatt Regency Capital Hill	Washington, D.C.
2002 - Greatest Conference Ever-Hyatt Regency Woodfield	Schaumburg, Illinois
2001 - Magnificent Milestones-Fairmont Chicago	Chicago, Illinois
2000 - Conference of the Century-Hilton St Louis	St. Louis, Missouri
1999 - Countdown to a Cure-Hyatt Regency	Milwaukee, Wisconsin
1998 - SMA: Past, Present and Future-Hyatt Regency	Denver, Colorado
1997 - New Horizons... Making Progress-Wyndham	Itasca, Illinois
1995 - MGM Grand-Las Vegas	Las Vegas, Nevada
1995 - International SMA Research Meeting	Amsterdam, the Netherlands
1994 - The Clarion Plaza Hotel	Orlando, Florida
1992 - Pheasant Run Resort	St. Charles, Illinois
1990 - Pheasant Run Resort	St. Charles, Illinois
1988 - The First SMA Conference-Hyatt Regency Chicago	Chicago, Illinois

1988



SPONSORS

Thank you to our generous sponsors for their support of the
2023 Annual SMA Conference.

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PLATINUM SPONSORS



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SILVER SPONSOR

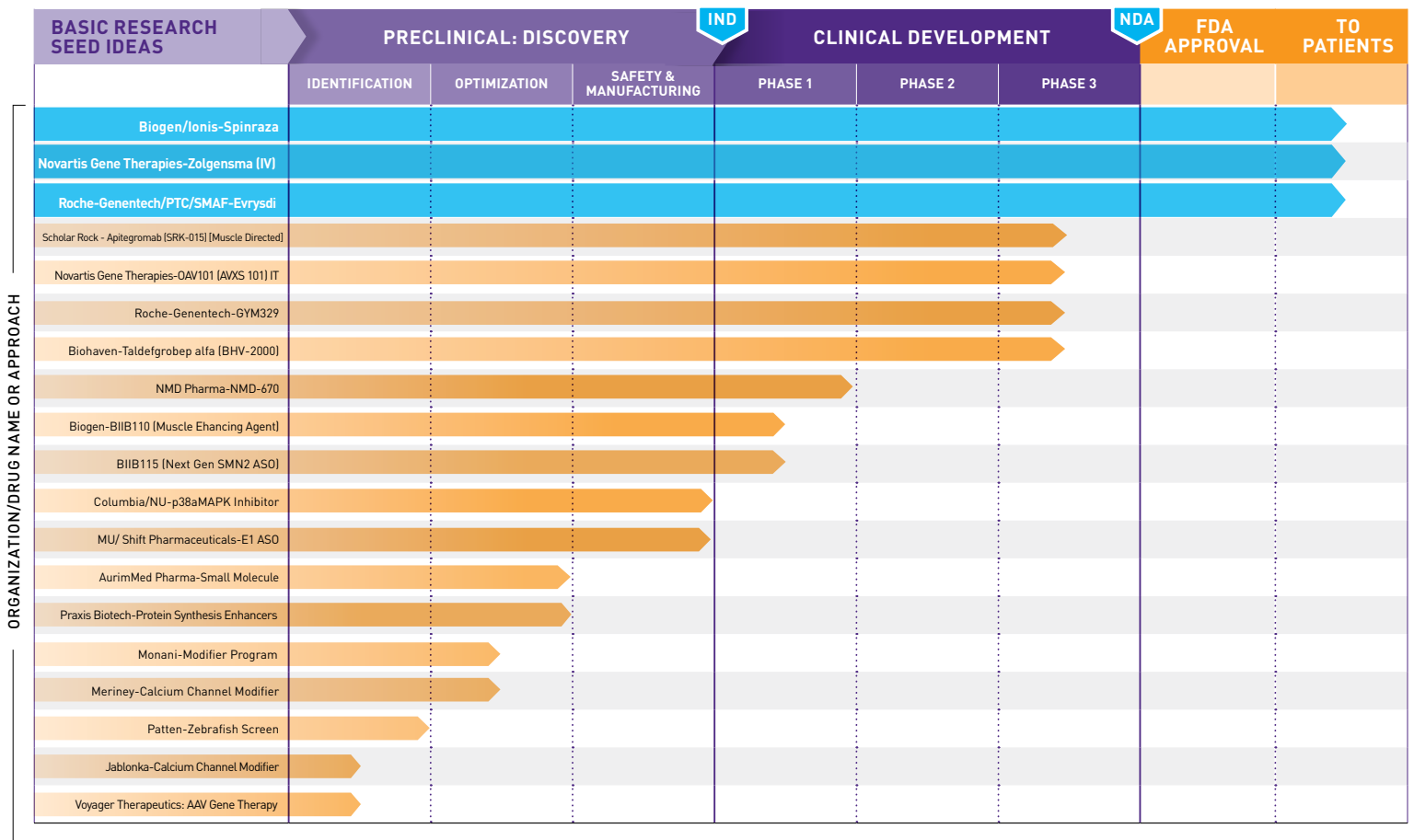


SMA DRUG PIPELINE

We are 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, or even combinations of therapies, to reach our goal of treatments for all individuals with SMA. And we are on the verge of further breakthroughs that will continue to change the course of SMA.

The Cure SMA Drug Pipeline is one of the primary ways we evaluate the success of our research program. It identifies the major drug programs in development and tracks their progress from basic research through U.S. Food and Drug Administration (FDA) approval and beyond. The Cure SMA Drug Pipeline identifies several possible treatment targets:

- Replacement or correction of the faulty *SMN1* gene.
- Modulation of the low functioning *SMN2* back-up gene.
- Muscle protection to prevent or restore the loss of muscle function in SMA.
- Neuroprotection of the motor neurons affected by loss of SMN protein.
- Newer approaches that identify additional systems and pathways affected by SMA.



IND = Investigational New Drug

NDA = New Drug Application Last updated: March 2023



THE IMPORTANCE OF CONTINUING TO INVEST IN BASIC RESEARCH

- In the past two decades, Cure SMA has invested over \$15 million in funding for 133 basic research grants that have furthered our understanding of SMA
- Cure SMA provided early funding for basic research projects that resulted in the development of our now approved genetically targeted drugs
- While the currently approved therapies dramatically alter the disease, there remains significant unmet needs especially for our older and the symptomatically diagnosed SMA population
- Continued basic research ensures a robust pipeline to now identify potential therapeutic avenues that will work in different ways and in combinations to address these needs. These next approaches will help to restore strength and function

WHAT ARE THE KEY AREAS OF RESEARCH WE WANT TO UNDERSTAND?

- Non-SMN targets. We are looking for other systems, pathways, and processes that can serve as the basis for an SMA treatment. We are asking how we can best measure the effectiveness of these non-SMN approaches
- How to best utilize both SMN-enhancing approaches and non-SMN approaches. We are asking how these different approaches can be used in combination to provide treatments for all ages and stages of SMA
- The role of SMN protein and when and where it is needed. We're investigating what critical functions it performs, where it is lacking in the body tissues of those with SMA, and how quickly it needs to be replaced to provide benefit for those with SMA

HOW IS CURE SMA COMMITTED TO CONTINUED BASIC RESEARCH?



- Cure SMA awarded 5 new basic research grants at the beginning of this calendar year for a total of \$500,000. These grants were featured in our recent Compass publication
- We are funding even more basic research grants in the year ahead. These grants will be focused on the above key areas of research. These grants will be announced later this fall. Be on the lookout for information about these grants in upcoming Cure SMA publications
- We are continuing to increase our funding level. This round we will award \$750,000, for an increase of \$250,000 over the previous cycle. By continuing to increase our investment into basic research, we can help ensure that we find answers to the key questions that will drive the next generation of therapeutic development



WHAT IS THE CURE SMA RESEARCH 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 nonprofit groups. It is often referred to as peer review, which means scientists working in similar areas judge each other's work.



Thank You to the Concepcions

We are very pleased to announce a pledge of \$1 million from the Concepcion Family Foundation. This gift will be fulfilled over a ten-year period and is dedicated to the funding of basic research grants. We thank the Concepcions for their generous gift, commitment to the Cure SMA basic research funding program, and for partnering with us to work toward our mission of funding and directing comprehensive research that drives breakthroughs in treatment, until we have a cure.

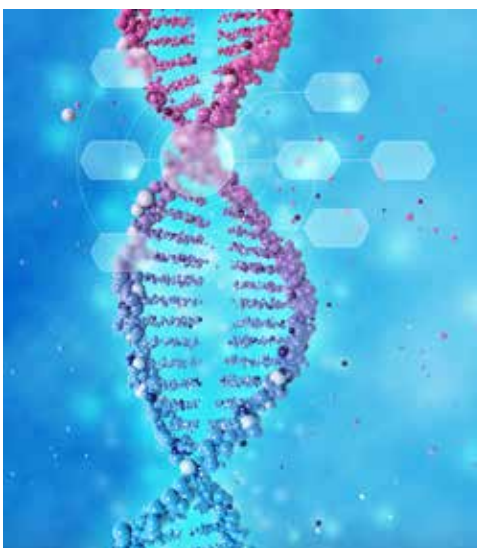
Richard and Jane Nunemaker Match

Special thanks to Richard and Jane Nunemaker for partnering with Cure SMA in our quest to invest in research that will focus on ways to enhance muscle strength and function as well as investigating nerve muscle connections and the regeneration of nerves. **To achieve these goals, the Nunemakers have generously offered to match all gifts up to \$250,000.**

To join the Nunemakers in investing in the future of SMA by making a donation to support our basic research efforts, please visit:



donate.curesma.org/Nunemaker

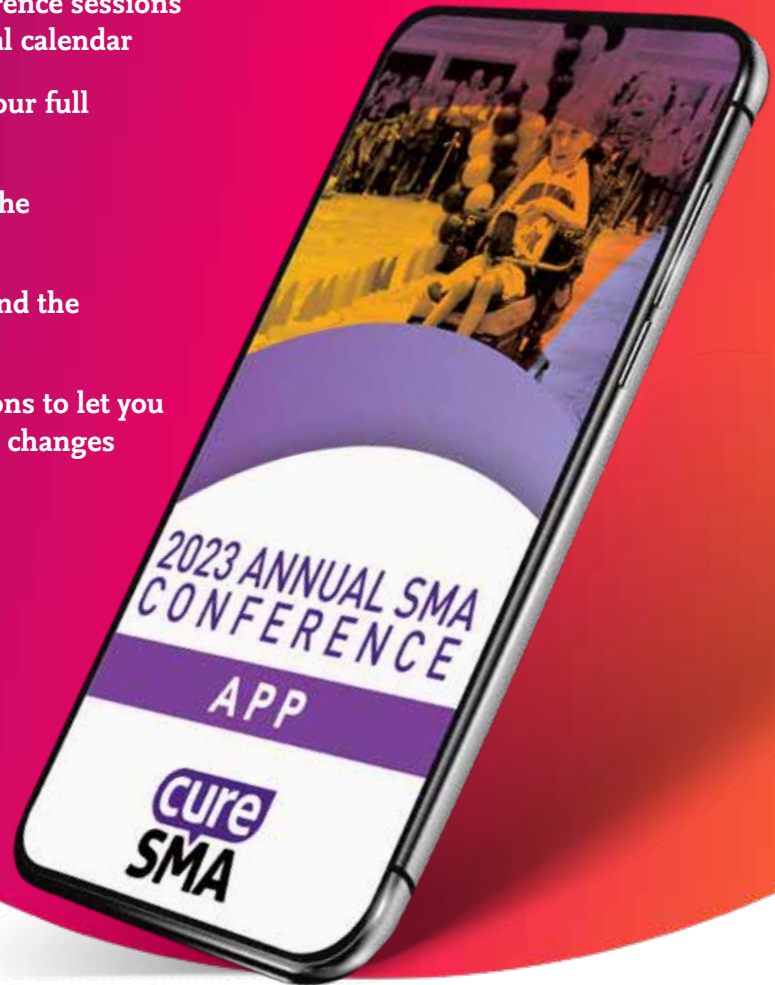


Annual SMA Conference App!

The 2023 Annual SMA Conference App is the perfect tool to keep you up to date on everything happening at conference!

FEATURES:

- Ability to add conference sessions to your own personal calendar
- A virtual edition of our full conference booklet
- Information about the Children's Program
- Maps of all hotels and the convention center
- Real time notifications to let you know of any session changes



Search Annual SMA Conference and download in the app store today!

2022 State of SMA is here

– pick up your copy today!

The report showcases outcomes from Cure SMA's three databases:

- The Membership Database with patient-reported outcomes on almost 10,000 affected individuals worldwide that also includes data from the annual Community Update Survey
- The SMA Clinical Data Registry (CDR) containing electronic medical record (EMR) sourced data from over 800 patients receiving care from US-based SMA Care Center Network sites
- The SMA Newborn Screening Registry (NBSR) with parent-reported outcomes from over 65 babies with SMA identified through statewide SMA newborn screening

Highlights from the report included:

- Demographics of the SMA Community
- Prevalence of SMA type and SMN2 copy number
- Age of SMA diagnosis
- Impact of newborn screening
- Motor function
- Quality of life
- Use of SMA treatment
- How SMA is changing over time
- ...and much more!

Funding for the SMA Care Center Network was provided in part by the Erin Trainor Memorial Fund and the Tyler William Orr Memorial Fund



Last Year's State of SMA Report

If you have not already received a copy of the 2021 State of SMA, please visit the conference registration desk, scan the QR code, or email research@curesma.org to request one at no charge.



Cure SMA is thankful for all the members of the SMA community who have generously shared their data. Their willingness to share details about how SMA impacts their families and daily lives allows us to advance our understanding of this disease. It lays the foundation for continued progress on behalf of our community. Cure SMA is grateful for the support and funding provided by the Cure SMA Real World Evidence Collaboration (RWEC), and the Cure SMA Industry Collaboration (SMA-IC) for research initiatives. Cure SMA is also grateful to the SMA Care Center Network (CCN) for their commitment to improving care for people with SMA and contributing consented patient data.

NEWBORN SCREENING

NEWBORN SCREENING FOR SMA

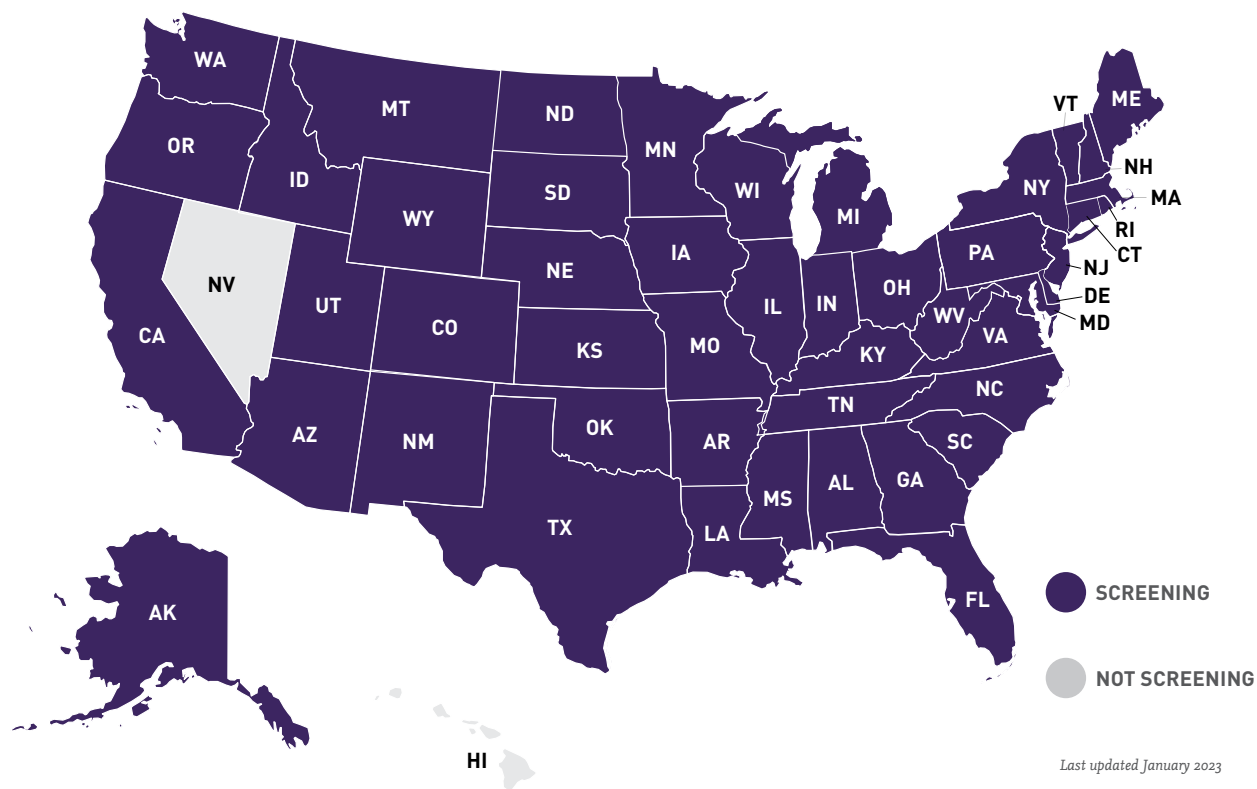
We are almost there!

As of March 2023, 48 states and Washington, D.C. screen newborns for SMA through a blood test. This means that 99% of all infants born in the US are now screened for SMA!

This is a tremendous accomplishment by the SMA community through sharing your stories, showing up to meetings, and educating decision makers about how newborn screening can save and improve lives. Congratulations!

But we will not stop until we reach 100%. Cure SMA remains focused and committed to adding Nevada and Hawaii, the final two states that have not implemented SMA newborn screening.

48 States Currently Screen for SMA | 99% of Newborn Babies in the U.S. are Screened



About Newborn Screening

Early diagnosis and treatment are key in ensuring the best possible outcomes for a baby with SMA. The most effective way to do this is by screening every newborn for SMA through their state's newborn screening program. The federal government recommends which conditions to screen for, while each state is expected to implement the screening of new disorders based on its local processes.

For more information, go to: www.curesma.org/newborn-screening-for-sma/



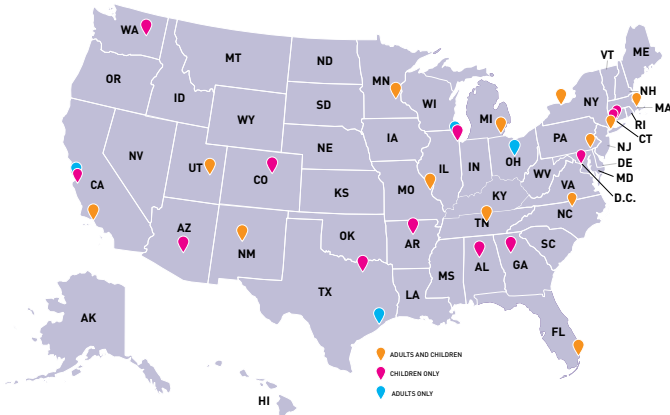
Calling on parents and caregivers of children with SMA identified by SMA Newborn Screening

We ask for your help! Cure SMA wants to learn about the impact of newborn screening on children with SMA over time. Please follow the QR code and complete the survey on behalf of your child. The information collected provides guidance about time to diagnosis, time to treatment, and early symptoms if present. We thank you in advance!

CURE SMA CARE CENTER NETWORK

GOAL: To provide the best healthcare for people with SMA, including offering new therapies, and to gather and disseminate new knowledge to advance the SMA standard of care for pediatric and adult persons.

To establish best care, Cure SMA has partnered with hospitals across the U.S. to form the Cure SMA Care Center Network. These Centers are geographically diverse and represent a cross section of care for individuals with SMA. Nearly half of these centers provide care to both pediatric and adult patients, four centers provide care focused on adults and 12 centers provide care focused on children. We are not done! With your support Cure SMA plans to expand the SMA Care Center Network from 29 to 60 centers across the U.S.



ADULT and PEDIATRIC CENTERS

- Boston Children's Hospital, Boston, MA
- Columbia University, New York, NY
- Connecticut Children's Medical Center, Hartford, CT
- Duke University Medical Center, Durham, NC
- Gillette Children's Specialty Healthcare, St. Paul, MN
- The Children's Hospital of Philadelphia, Philadelphia, PA
- The University of Michigan, Ann Arbor, MI
- University of California, Los Angeles (UCLA), Los Angeles, CA
- University of Miami, Miami, FL
- University of New Mexico, Albuquerque, NM
- University of Rochester Medical Center, Rochester, NY
- University of Utah, Program for Inherited Neuro Disorders, SLC, UT
- Washington University/St. Louis Children's Hospital, St. Louis, MO

SMA Clinical Data Registry

The SMA Clinical Data Registry (CDR) is central to our work to collect data to then improve healthcare for people with SMA. Each SMA Care Center Network site consents patients to allow the center to submit information and data to the SMA CDR.

The ability to collect data on a large number of representative individuals living with SMA in a registry is powerful. With more than 900 participants, the CDR is building a dataset to document changes in SMA over time. The CDR data is analyzed to drive care improvements. The analyses will be used to set the standard of care for SMA. Standard of care is treatment that is accepted by medical experts as a proper treatment for a specific disease or condition and that is widely used by healthcare professionals. The data driven SMA standard of care guidelines will be distributed widely throughout the SMA community and to clinicians and hospitals caring for people with SMA. Keep in mind that data is more compelling than expert opinion.

Clinical Data Registry Number of Participants Over Time¹



29
Cure SMA
Care Centers

22
States and Washington, D.C.
with an SMA Care Center

900+
Participants with
SMA in the Registry

ADULT CENTERS

- Baylor College of Medicine, Houston, TX
- Northwestern University, Evanston, IL
- Stanford Health, Palo Alto, CA
- The Ohio State University, Wexner Medical Center, Columbus, OH

PEDIATRIC CENTERS

- Advocate Children's Hospital, Park Ridge, IL
- Arkansas Children's Hospital, Little Rock, AR
- Children's Healthcare of Atlanta, Atlanta, GA
- Children's Hospital Colorado, Aurora, CO
- Children's National Medical Center, Washington, DC
- Children's of Alabama, Birmingham, AL
- Phoenix Children's Hospital, Phoenix, AZ
- Seattle Children's Hospital, Seattle, WA
- Stanford Children's Health, Palo Alto, CA
- University of Texas Southwestern/Children's Health, Dallas, TX
- Vanderbilt University Medical Center, Nashville, TN
- Yale Pediatric Neuromuscular Clinic, New Haven, CT

Acknowledgements

Funding for the SMA Care Center Network was provided in part by the Erin Trainor Memorial Fund and the Tyler William Orr Memorial Fund.

Funding was also provided by the Cure SMA Real World Evidence Collaboration which includes Cure SMA, Biogen, Genentech/Roche Pharmaceuticals, and Novartis Gene Therapies.

CONFERENCE SUMMARY

WEDNESDAY, JUNE 28, 2023

5:00PM – 7:30PM REGISTRATION OPEN FOR ALL CONFERENCE ATTENDEES

THURSDAY, JUNE 29, 2023

12:00PM – 2:20PM NEWLY DIAGNOSED PROGRAM
(For Newly Diagnosed Families Only)

ALL CONFERENCE ATTENDEES

9:00AM – 6:00PM REGISTRATION OPEN FOR ALL CONFERENCE ATTENDEES

2:30PM – 6:00PM ADULTS WITH SMA LOUNGE *Sponsored by Biogen*
for adults with SMA and their attendants

6:00PM – 8:30PM MEET AND GREET/ FUN FEST FOR ALL
CONFERENCE ATTENDEES

8:30PM – 10:00PM DOUBLE TAKE FASHION SHOW *Sponsored by Genentech*

8:30PM – 11:00PM DAD'S NIGHT OUT **for dads only**

FRIDAY, JUNE 30, 2023

7:00AM – 5:30PM REGISTRATION OPEN

7:00AM – 8:45AM BREAKFAST WITH SYMPOSIUM
by Genentech

7:30AM – 5:00PM EXHIBITOR TABLES OPEN

9:00AM – 11:00AM GENERAL SESSION OPENING REMARKS

11:00AM – 5:00PM ADULTS WITH SMA LOUNGE *Sponsored by Biogen*
for adults with SMA and their attendants

11:00AM – 5:00PM TEEN LOUNGE *Sponsored by Luke 18:1 Foundation*
for all teens ages 12 – 18

11:15AM – 4:45PM SMA COMMUNITY SELF EXPRESSION ART EXHIBIT *Sponsored by Biogen*

11:15AM – 4:45PM COMMUNITY CONNECTION AREA *Sponsored by Genentech*

11:15AM – 4:45PM NOVARTIS GENE THERAPIES ACTIVITY ROOM *Sponsored by Novartis Gene Therapies*

11:15AM – 12:30PM WORKSHOP SESSION #1

12:30PM – 1:30PM LUNCH

1:45PM – 3:15PM WORKSHOP SESSION #2

3:30PM – 4:45PM WORKSHOP SESSION #3

FRIDAY, JUNE 30, 2023 CONTINUED

6:30PM – 8:30PM FAMILY FRIENDLY RESEARCHER POSTER SESSION

7:30PM – 9:15PM PJ PARTY AND MOVIE NIGHT

7:30PM – 10:00PM TEEN SOCIAL *Sponsored by Luke 18:1 Foundation*
for all teens ages 12 – 18

8:00PM – 11:00PM ADULTS WITH SMA RECEPTION *Sponsored by Biogen and the Dhont Family Foundation*
for adults with SMA and their attendants

8:30PM – 11:00PM MOM'S NIGHT OUT **for moms only**

SATURDAY, JULY 1, 2023

7:30AM – 4:00PM REGISTRATION OPEN

7:30AM – 4:00PM EXHIBITOR TABLES OPEN

7:00AM – 8:45AM BREAKFAST WITH SYMPOSIUM
by Biogen

9:00AM – 10:30AM WORKSHOP SESSION #4

9:00AM – 4:00PM NOVARTIS GENE THERAPIES ACTIVITY ROOM
Sponsored by Novartis Gene Therapies

9:00AM – 4:00PM SMA COMMUNITY SELF EXPRESSION ART EXHIBIT
Sponsored by Biogen

9:00AM – 4:00PM COMMUNITY CONNECTION AREA
Sponsored by Genentech

9:00AM – 4:00PM ADULTS WITH SMA LOUNGE *Sponsored by Biogen*
for adults with SMA and their attendants

9:00AM – 4:00PM TEEN LOUNGE *Sponsored by Luke 18:1 Foundation*
for all teens ages 12 – 18

10:45AM – 12:00PM WORKSHOP SESSION #5

12:00PM – 1:00PM LUNCH

1:15PM – 2:30PM WORKSHOP SESSION #6

2:45PM – 3:50PM WORKSHOP SESSION #7

4:00PM CURE SMA EVENING AT THE PARK


SUNDAY, JULY 2, 2023

7:30AM – 8:45AM ANNUAL SMA CONFERENCE BREAKFAST


9:00AM – 11:15AM CLOSING GENERAL SESSION – IT'S A WONDERFUL LIFE PANEL

WORKSHOP SESSIONS


WORKSHOP SESSION #1

- Orthopedic Management in SMA
- Personal Care Assistants: From Recruitment and Hiring to Day-to-Day Management and Everything In-Between
- Taking Charge of Your Health: The Transition from Pediatric to Adult Care 
- Spouses and Significant Others of Adults with SMA Sharing Your Experience
- Aquatic Physical Therapy Question and Answer Session
- Sleep Problems Seen in SMA
- Addressing the Oral Motor System to Support Breathing, Eating and Speaking: Considerations For SMA
- Talk it Out for Kids with an SMA-affected Parent (Ages 6- 17)
- Siblings Talk it Out (Ages 12 to 17)

WORKSHOP SESSION # 2

- Dating Disabled: Navigating the Dating World as a Disabled Person
- The Role of Therapy PT and OT in your Child's Life – Being Part of the Team!
- Technology That Makes Life Easier 
- Pre-symptomatic and Early Diagnosis Experiences
- Pediatric Therapeutic Yoga for SMA
- Healing the Grieving Heart – Part I
- Aquatic Physical Therapy for Sitting/Walking Children Ages 8-13 years old
- What it Means to be a Teen on Wheels
- Siblings Talk it Out (Ages 5 to 11)


WORKSHOP SESSION # 3

- Pregnancy with SMA
- Beyond High School - Understanding the Barriers Students with Disabilities Face so We can Prepare Students to Advocate for Themselves
- Traveling with SMA 
- Financial and Estate Planning with Special Needs Trusts
- Finding Hope After Loss: Helping Parents and Children Cope
- Ethics, Angst, Excitement and Realities of SMA Treatments and Research
- Incorporating exercise into you or your child's life

WORKSHOP SESSION #4

- Unpacking Clinical Trials
- Therapeutic Yoga for Teens and Adults with SMA
- Medical Management of Adults with SMA
- Service Dogs: Safety, Independence, Confidence
- Navigating The Special Education Process: Setting our Children Up for Success In Preschool and Elementary School
- I Need to Cough, I Need to Breathe
- Aquatic Physical Therapy for Fun and Function, Sitting/walking Children - Ages 6 months to 7 years
- Memory Boxes: Create a Treasure (for children and teens only)
- Mindfulness for Stress Reduction
- The Role of the Multi-disciplinary Clinic -Why might it be helpful for those identified Pre-symptomatically

WORKSHOP SESSION # 5

- The Caregiver's Toolbox (Essentials)
- How to Build Your Presence and Advocate Through Writing and Social Media 
- SMA and Dating/Sex Roundtable Discussion
- Keepsake Creation: Grieving Through Art (for anyone)
- Transitioning to Middle School
- Genetics and Reproductive Options for SMA Families
- What Is an ABLE Account, and Should You Have One?
- Benefits of Adaptive Recreation
- Aquatic Physical Therapy for Fun and Function, Teens and Adults
- Kids Talk it Out (Ages 6 – 11)

WORKSHOP SESSION # 6

- Adults with SMA Relationships Panel – Relationships through experience
- Nutrition for Life!
- Preventing Emergencies – Daily Care, Contingencies and Response in the Community
- Women's Health in SMA
- Change your Brain – Change Your Life
- Navigating The Special Education Process: Transitioning to Middle School, High School, and College
- You're Invited: Fundraising Power Hour
- Healing the Grieving Heart – Now What? Part II
- Grandparents Coffee and Share Your Experience
- Aquatic Physical Therapy for Fun and Function, Children with Supported Head Control

WORKSHOP SESSION # 7

- Early Scoliosis Treatment in SMA
- Young Adults Talk it Out (Ages 18+)
- University of Utah TRAILS Program: Adaptive Sports and Novel Adaptive Equipment
- Plan an Ability Awareness Week at Your Elementary School
- Q and A Session with The Pulmonary Team
- Get to the Art of It: Community Art Time
- Parenting with SMA

ADDITIONAL SESSIONS

- Kids Talk it Out (Ages 12 – 17) | Friday, June 30th from 9:00am – 10:30am



MEET AND GREET

THURSDAY 6:00PM – 8:30PM

GRAND HARBOR BALLROOM

KICK OFF TO THE CONFERENCE!

Meet Disney Characters: Mickey and Minnie, Anna, Elsa and Olaf, Mirabel, Ralph and Vanellope

- Researcher Relay Race
- Fun Fest/Carnival
- Disney Characters



FAMILY FRIENDLY RESEARCHER POSTER SESSION

FRIDAY 6:30PM – 8:30PM

GRAND HARBOR BALLROOM

VISIT OVER 24 POSTERS PRESENTED BY RESEARCHERS

Meet Disney Characters: Mr and Mrs Incredible, Edna Mode, Woody and Bo Peep, Latso, Mike and Sulley



PJ PARTY AND MOVIE NIGHT

FRIDAY 7:30PM – 9:15PM

GRAND HARBOR BALLROOM

MOVIE FOR KIDS AND ADULTS ALIKE!

CURE SMA EVENING IN THE PARK

SATURDAY 4:00PM

EPCOT THEME PARK TICKET TO BEGIN AT 4:00PM

Help Kick-off the 2023 Annual SMA Conference!



We are looking forward to a great Annual SMA Conference this year and the Meet and Greet is the official kick-off event to the conference. Every registered attendee is invited to join in.

There will be games and prizes for all ages and types to participate in throughout the evening. And after we welcome everyone to conference, the ever so popular Researcher Relay Race will begin, where researchers and clinicians compete against individuals affected by SMA, in a tight race to the finish line. The catch is that the researchers and clinicians race in manual wheelchairs, and the kiddos always leave them in the dust!

Dinner will be provided during the event. Meet your favorite Disney characters and enjoy an evening of fun and community.

Meet and Greet and Fun Fest



Grand Harbor Ballroom Thursday, June 29th, 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, Anna, Elsa and Olaf, Mirabel, Ralph and Vanellope

Double Take Fashion Show | Doors Open at 8:30pm in Asbury Hall

Introducing a first-of-its-kind fashion show, with start-to-finish SMA community involvement, that aims to increase disability visibility, break down stereotypes and champion adaptive fashion.

SMA community members will be leading the program from beginning to end—conceptualizing the message, collaborating with design fellows at Open Style Lab (OSL) on garment adaptations, and walking or rolling the runway. Through this show, the SMA community is inviting the world to do a double-take, not because of their disabilities but because of their style and individuality, challenging the stereotype that people with disabilities want to blend in or erase their differences. Double Take also prompts the audience to see people with disabilities occupying a space that's often shut off to them.

Genentech
A Member of the Roche Group

Enjoy the show which runs from 9:00pm – 10:00pm. Cash bar will be available in the lobby. Thank you to Genentech for generously sponsoring this event.

ADULTS WITH SMA LOUNGE AND RECEPTION

Cure SMA is grateful for the support of the Adults with SMA Reception, sponsored by Biogen and the Dhont Family Foundation and the Adults with SMA Lounge, sponsored by Biogen.



There are many great opportunities to connect with other adults in the community at the Annual SMA Conference.

The Adults with SMA Lounge is a great space to refresh throughout the conference days.

Take a break and unwind with other adults with SMA and their attendants.

Adults with SMA Lounge in Cape Cod Hall is open:

Thursday, June 29th | 2:30pm – 6:00pm

Friday, June 30th | 11:00am – 5:00pm

Saturday, July 1st | 9:00am – 4:00pm

ADULTS WITH SMA RECEPTION

Come join other adults with SMA and their attendants for an evening of fun!

The Adults with SMA Reception, sponsored by Biogen and the Dhont Family Foundation, will be held on

Friday, June 30th from 8:00pm – 11:00pm in Asbury B, C and D.

Meet, mingle and enjoy some light snacks and a cash bar for this fun evening together!

ADULTS WITH SMA TRACK SPONSORSHIP

Thank you to Genentech for their generosity and supporting our Adults with SMA Workshop Track.

Genentech
A Member of the Roche Group

- Personal Care Assistants: From Recruitment and Hiring to Day-to-Day Management and Everything In-Between
- Taking Charge of Your Health: The Transition from Pediatric to Adult Care
- Technology That Makes Life Easier
- What it Means to be a Teen on Wheels
- Spouses and Significant Others of Adults with SMA Sharing Your Experience
- Pregnancy with SMA
- Beyond Highschool - understanding the barriers students with disabilities face so we can prepare students to advocate for themselves
- Traveling with SMA
- Dating Disabled: Navigating the Dating World as a Disabled Person
- Therapeutic Yoga for Teens & Adults with SMA
- How to Build Your Presence and Advocate Through Writing & Social Media
- SMA and Dating/Sex Roundtable Discussion
- Parenting with SMA
- Medical Management of Adults with SMA
- Aquatic Physical Therapy for Fun and Function, Teens & Adults
- Adult with SMA Relationships Panel – Relationships through experience
- Women's Health in SMA
- Navigating the Special Education Process - High School & College
- Young Adults Talk it Out
- Change Your Brain, Change Your Life
- What Is an ABLE Account, and Should You Have One?

Be sure to
check out
these sessions!

WHY LEGISLATIVE ADVOCACY MATTERS

DID YOU KNOW:

- State and federal policies impact nearly all aspects of life for individuals and families with SMA, from funding for employment and educational supports to eligibility related to healthcare and community supports.
- Cure SMA is advocating with and for the SMA community around everyday living issues including healthcare, education, employment, housing, transportation, technology, and disability rights
- More individuals and families with SMA connected with their Members of Congress during 2022 through Cure SMA's Action Center than ever before
- Thanks to SMA community advocacy, Congress added a provision to this year's federal budget related to new National Institutes of Health research in SMA



Advocate for SMA Community Legislative Priorities

Become a Cure SMA Advocate at Conference by scanning this QR code. It will only take a minute, but the impact will be great.



AIR TRAVEL REPORT

Cure SMA released a nationwide report to highlight the air travel experiences for individuals and families with SMA. The Good, (but mostly) Bad, and Ugly of Air Travel for Individuals with Spinal Muscular Atrophy report features first-person accounts of air passengers with SMA and their families and recommendations to Congress for improving the air travel experience and overall accessibility for the SMA community.

SMA community experiences featured in the report include:

- **GOOD:** The aisle chair transfer went smoothly because I was fortunate to receive a skilled attendant at the airport.
- **BAD:** I rarely feel like I'm respected by the airline staff/TSA and it's glaringly obvious that I'm a second-class citizen. It's humiliating.
- **UGLY:** On one trip, my chair was damaged to the point I couldn't function in it. It was not able to go up or down or recline which I use to breathe right. It was not able to drive far without needing a break. I was in pain for 8 months while it underwent repair.

The report will be a key advocacy tool in 2023 as Congress considers legislation on the future of aviation. Check out the report at: <https://www.curesma.org/advocacy/>

cure **SMA** **FILL OUT THE** **CONFERENCE SURVEY**

FILL OUT THE CONFERENCE SURVEY FOR YOUR CHANCE TO WIN AN APPLE WATCH OR 2024 CONFERENCE SCHOLARSHIP



The success of our conference can only be improved by the feedback we receive from you!

Please take the time to provide any comments by filling out our conference survey, and the workshop surveys. You can find hard copies of your surveys in your conference folder. Please drop your completed surveys into the many bins throughout the meeting space, at your workshops or at the Registration Area.

All participants who complete the surveys by 10:30am on Sunday, July 2nd, will have their name entered into a raffle.

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

The winners will be drawn and announced on Sunday, July 2nd at the Closing General Session/It's A Wonderful Life. All participants who complete the surveys by 10:30am on Sunday, July 2nd, will have their name entered into a raffle. All completed surveys will also be entered into a drawing for a chance to win a trip, including airfare to the 2024 Annual SMA Conference (the location will be announced on Sunday), or a brand-new Apple Watch!



You can also fill out the survey on-line at www.curesma.org/ConferenceSurvey23

Amy Marquez Scholarship

For the 2023 Annual SMA Conference, the Amy Marquez Scholarship is in its ninth year. The Amy Marquez Scholarship was started in memory of Amy Marquez, who passed away from SMA type I at the age of forty-one. Amy was an active member of the SMA community and was a wonderful source of support and motivation for many SMA families. This scholarship will be awarded each year to an adult with SMA to attend the Annual SMA Conference, in recognition of Amy's involvement within the SMA Adult community. The scholarship will cover both registration fees and hotel costs.



CONFERENCE MEALS INCLUDED WITH REGISTRATION

THURSDAY

DINNER AT MEET AND GREET:

DINNER BUFFET—SALAD, VEGETABLE LASAGNA, HOT DOGS, BURGERS, LEMONADE, ICED TEA, WATER, COFFEE, TEA, ICE CREAM SUNDAE.

FRIDAY

BREAKFAST ITEMS:

ASSORTED DANISHES AND CROISSANTS, APPLE JUICE AND COFFEE.

LUNCH:

WE WILL HAVE AN ASSORTMENT OF BOXED LUNCH OPTIONS FOR ATTENDEES, AS SUPPLIES LAST. PASTA SALAD, CHIPS, FRUIT AND A COOKIE ARE INCLUDED IN BOXED LUNCH.

Each choice is limited in number.

SNACKS AT FAMILY FRIENDLY POSTER SESSION:

LIGHT SNACKS OF MICKEY SOFT PRETZELS, MICKEY RICE KRISPY TREATS, LEMONADE AND WATER.

SNACKS AT THE PJ PARTY AND MOVIE NIGHT:

POPCORN AND REFRESHMENTS.

SATURDAY

BREAKFAST ITEMS:

ASSORTED DANISHES AND CROISSANTS, APPLE JUICE AND COFFEE.

LUNCH:

WE WILL HAVE AN ASSORTMENT OF BOXED LUNCH OPTIONS FOR ATTENDEES, AS SUPPLIES LAST. PASTA SALAD, CHIPS, FRUIT AND A COOKIE ARE INCLUDED IN BOXED LUNCH.

Each choice is limited in number.

SUNDAY

BREAKFAST ITEMS:

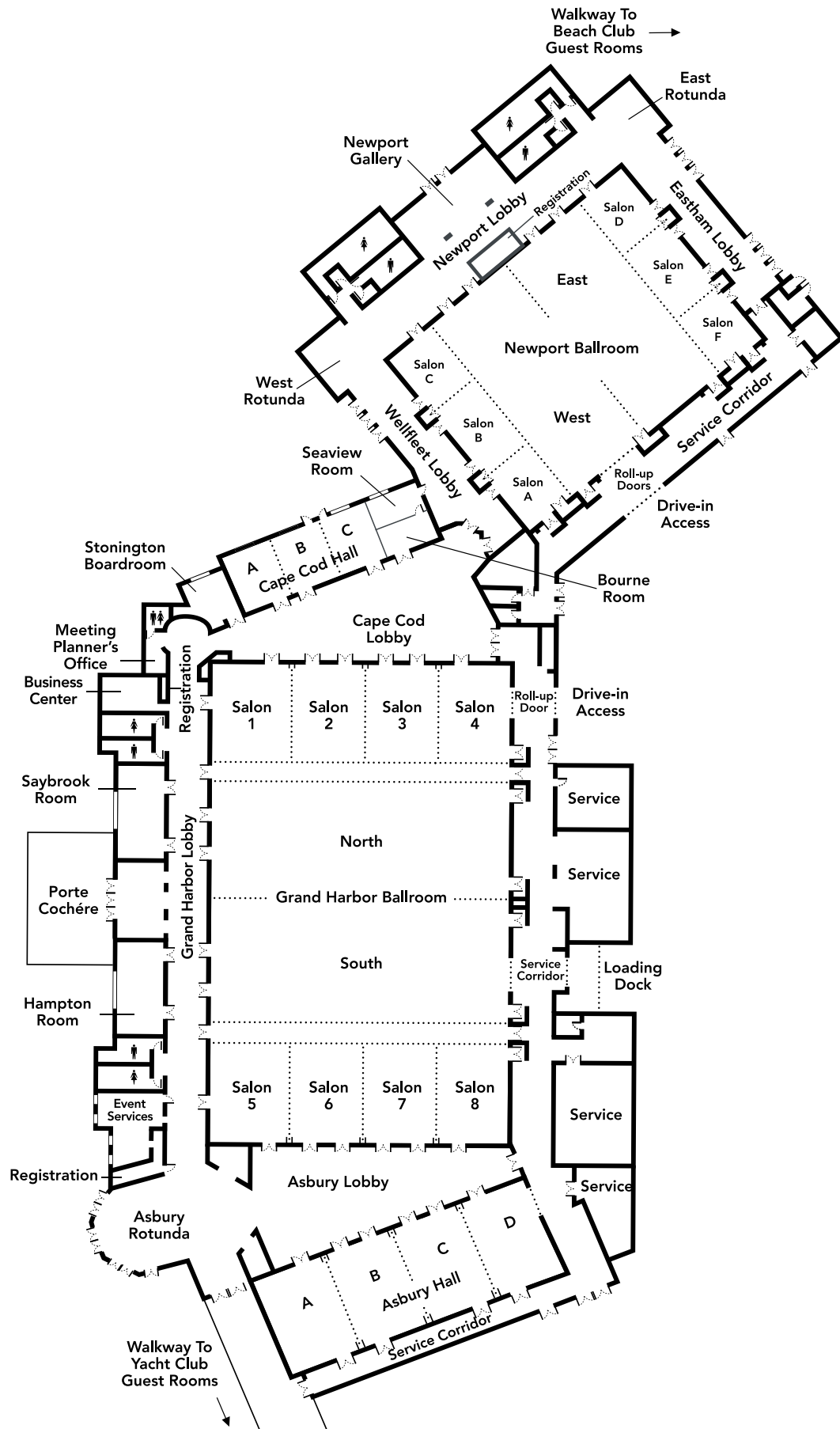
ASSORTED MUFFINS, DANISHES AND CROISSANTS, APPLE JUICE AND COFFEE.

There are many dining locations and options available throughout Disney's Yacht and Beach Club Resorts as well as all along the Board Walk. Options at Disney's Yacht and Beach Club include:

- Ale and Compass Restaurant
- Beaches and Cream Soda Shop
- Cape May Café
- Yachtsman Steakhouse
- Beach Club Marketplace
- Martha's Vineyard
- Hurricane Hanna's Waterside Bar and Grill
- Crew's Cup Lounge
- Mobile Dining through the Disney World App

UNFORTUNATELY, WE ARE NOT ABLE TO ACCOMMODATE FOR DIETARY RESTRICTIONS OR ALLERGIES.

MAP: DISNEY'S YACHT AND BEACH CLUB RESORTS



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 children are entertained and safe. The program provides fun crafts, entertainers, and activities led by an incredible group of volunteers.

In addition to programs at the Annual SMA Conference, the JIRF also sponsors all Newly Diagnosed Care Packages. These packages are sent to every newly diagnosed 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 children's program and teen social have all added wonderful components to our growing conferences!



fighting spinal muscular atrophy in memory of our shooting star
www.ourshootingstar.com

Jennifer Miller-Smith, Aaron Smith and Family



The Smith family has been an amazing asset throughout the planning of this year's 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 make this conference magical!

Expo CCI — We would also like to thank everyone at Expo CCI, especially Richard Curran, who sponsors and donates 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!



The Luke 18:1 Foundation



The Luke 18:1 Foundation was founded in 2017 in honor of Luke Stickane with the purpose of finding a cure for SMA. Since 2017, the Luke 18:1 Foundation has been a steadfast partner of Cure SMA and has granted over \$1.5 million dollars to Cure SMA to fund vital research, care, and support programs. New this year, the Luke 18:1 Foundation sponsored nearly 200 first-time attendees to attend the 2023 SMA Conference, as well as the Teen Social and the inaugural Teen Lounge.

LUKE 18:1

Cure SMA thanks the Stickane Family, Giglio Family, and the Luke 18:1 Foundation for their tireless commitment to funding a cure for SMA!

VISIT THE CURE SMA HUB!

New this year!

Join us in the Cape Cod Lobby at the Yacht and Beach Club Resorts to experience our Cure SMA Hub.



Learn more about peer-to-peer fundraising and how you can power our progress



Become a Cure SMA advocate and learn about important advocacy initiatives



Enjoy games, exclusive prizes, a raffle, and conversation and connection



It's *the* place to be at the 2023 Annual SMA Conference.

We can't wait to see you!



WALK-N-ROLL

REGISTER TODAY

FOR A WALK-N-ROLL TAKING PLACE AT A LOCATION NEAR YOU!

Register or donate today to
make an impact with supporters
across the country.



No matter how you plan to participate in our Walk-n-Roll program, this is one of the best ways to support and fundraise for people with Spinal Muscular Atrophy (SMA) in your community and beyond.

Thank You to Our National Premier Sponsors



FIND YOUR LOCAL WALK-N-ROLL
www.curesma.org/walk-n-roll-program/








WEDNESDAY, JUNE 28TH | AGENDA

5:00PM – 7:30PM	Registration Open for all Conference Attendees	Asbury Rotunda
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THURSDAY, JUNE 29TH | AGENDA

9:00AM – 6:00PM	Registration Open for all Conference Attendees	Asbury Rotunda
12:00PM – 2:20PM Drop off opens at 11:30AM	Newly Diagnosed Children's Program Sponsored by The Jacob Isaac Rappoport Foundation	Newport Ballroom
12:00PM – 2:20PM	Newly Diagnosed Program (For Newly Diagnosed Families Only)	Grand Harbor Ballroom Salons 1 and 2
12:00PM – 12:15PM	Introduction to Cure SMA and the Community **for newly diagnosed families only** Kenneth Hobby, President Colleen McCarthy O'Toole, Senior Vice President, Community Support Jessica Clark, Senior Director, Community Support	Grand Harbor Ballroom Salons 1 and 2
12:15PM – 12:20PM	Parent Welcome Al Freedman, PhD, SMA Dad Kevin O'Brien, SMA Dad	
12:20PM – 12:45PM	Understanding Treatment and Trials Rob Graham, MD	
12:45PM – 1:10PM	SMA Standards of Care Mary Schroth, Chief Medical Officer	
1:10PM – 1:35PM	Life After Diagnosis – Parents Share Their Journey Danyelle Sun, SMA Mom Amy Medina, SMA Mom Marnie Harris, SMA Mom	
1:35PM – 2:20PM	Newly Diagnosed Program Meet and Mingle Reception **for newly diagnosed families only**	Grand Harbor Ballroom Salons 3 and 4
2:30PM – 6:00PM	Adults with SMA Lounge Sponsored by Biogen **for adults with SMA and their attendants**	Cape Cod Hall
6:00PM – 8:30PM	Meet and Greet Includes Researcher Relay Race and Games Disney Characters: Minnie and Mickey, Anna, Elsa and Olaf, Mirabel, Ralph and Vanellope	Grand Harbor Ballroom
8:30PM – 11:00 PM	Dad's Night Out **for dads only**	Promenade Ballroom West **at the Boardwalk Inn Resort
8:30PM – 10:00 PM	Double Take Fashion Show Sponsored by Genentech	Asbury Hall

AGENDA | FRIDAY, JUNE 30TH

7:00AM – 5:30PM	Registration Open for all Conference Attendees	Asbury Rotunda
7:00AM – 8:45AM	Breakfast	Grand Harbor Ballroom North
7:30AM – 5:00PM	Exhibitor Tables Open	Grand Harbor Lobby and Cape Cod Lobby
7:45AM – 8:45AM	Symposium by Genentech: Community Conversation: Goals, Choices, and Life Along the Way  <small>A Member of the Roche Group</small>	Grand Harbor Ballroom North
9:00AM – 12:30PM AND 1:45PM – 5:00PM (DROP OFF OPENS AT 8:15AM AND 1:15PM)	Children's Program <i>Sponsored by The Jacob Isaac Rappoport Foundation</i> 	Newport Ballroom
9:00AM – 10:30AM	Kids Talk it Out (Ages 12 – 17) Angela Wrigglesworth, Elementary Education Teacher, SMA-Affected Adult Al Freedman, PhD, SMA Dad and Child/Family Psychologist This facilitated session will provide SMA-affected children (ages 12-17) 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. <i>**Please note, kids in this session will be escorted to the Children's Program in Newport Ballroom when the session concludes at 10:30am. Please pick up your kids in Newport Ballroom**</i>	Grand Harbor Ballroom Salon 1
9:00AM – 11:00AM	General Session Opening Remarks	Grand Harbor Ballroom South
11:00AM – 5:00PM	Adults with SMA Lounge <i>Sponsored by Biogen</i> <i>**for adults with SMA and their attendants**</i> 	Cape Cod Hall
11:00AM – 5:00PM	Teen Lounge <i>Sponsored by Luke 18:1 Foundation</i> <i>**for all teens ages 12 - 18**</i> 	Saybrook
11:15AM – 4:45PM	SMA Community Self Expression Art Exhibit <i>Sponsored by Biogen</i> 	Asbury Lobby
11:15AM – 4:45PM	Community Connection Area <i>Sponsored by Genentech</i>  <small>A Member of the Roche Group</small>	West Rotunda
11:15AM – 4:45PM	Novartis Gene Therapies Activity Room <i>Sponsored by Novartis Gene Therapies</i> 	Bourne




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.


11:15AM – 12:30PM


Workshop Session #1



	<p>Orthopedic Management in SMA Brian Snyder, MD, PhD Anne Stratton, MD</p> <p>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.</p>	<p>Grand Harbor Ballroom South</p>
	<p>Personal Care Assistants: From Recruitment and Hiring to Day-to-Day Management and Everything In-Between Paula Barrett, MBA Carolyn Barrett, MS, adult with SMA</p> <p>This workshop, designed for SMA parents, adults, and teens, will begin with a brief overview on how to get started when hiring and managing you or your child's personal care assistants for the first time. What to look for? Where to look? How to describe your needs? How to train someone on your specific needs? How to transition care management from parents to teens? We'll then dive into common questions, issues, and circumstances that come up while managing complex PCA care (among everything else!). The workshop will conclude with a dialogue on unique challenges, opportunities, and everything in between that comes up when hiring and managing PCA care as an individual with SMA (including recruiting during a tight labour market).</p> <p>While this workshop is designed for individuals 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, all are welcome.</p>	<p>Asbury Hall C and D</p>
	<p>Taking Charge of Your Health: The Transition from Pediatric to Adult Care <i>Sponsored by Genentech</i></p> <p>Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN, FPCN Bakri Elsheikh, MBBS, FRCP Sarah Stoney, MSW, LSW Diane Murrell, LCSW</p>  <p>This workshop will focus on the following aspects of transition from pediatric to adult healthcare: Understand the importance of why transition; identify some of the transition skills needed before transferring to adult care; a single clinic study on Advance directives; and, an adult provider's perspective on adult clinics and differences you might anticipate. This session is ideal for parents, adolescents, and young adults who are preparing transitioning from pediatric to adult care.</p>	<p>Asbury Hall B</p>
	<p>Spouses and Significant Others of Adults with SMA Sharing Your Experience 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. This session will allow attendees to connect with others in this similar walk of life!</p>	<p>Asbury Hall A</p>
	<p>Aquatic Physical Therapy Question and Answer Session Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS</p> <p>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.</p>	<p>Grand Harbor Ballroom Salons 7 and 8</p>

AGENDA | FRIDAY, JUNE 30TH

	<p>Sleep Problems Seen in SMA Richard Kravitz, MD</p> <p>Individuals with SMA are at risk for various sleep disorders. Whether it is caused by their underlying muscle weakness or due to normal childhood development, families and individuals with SMA need to be aware of these disorders so that they can be brought to the attention of their primary care providers and SMA care team. There are various diagnostic tools that can be utilized in establishing the etiology of these sleep problems. Both medical and behavioral interventions are available that are useful in the management of sleep disorders. With the arrival of SMN modulators, the standard of care is changing, and individuals with SMA and families need to be aware of the options now available to help provide for a good night's sleep. In this workshop, we will discuss how SMA impacts sleep and what options are available to optimize both the individual's and their families' sleep so that all can be well rested.</p>	Grand Harbor Ballroom Salons 5 and 6
	<p>Addressing the Oral Motor System to Support Breathing, Eating and Speaking: Considerations For SMA Renee Roy Hill, MS, CCC-SLP, COM@CLC</p> <p>Development of the oral motor system is crucial to the development of the skills, breathing, eating and drinking, and is often affected with some types of SMA, sometimes later in life. Current research also shows that the development of the oral structure is impacted by how the structures are used in function and function is impacted by how the structure has developed. The earlier these concepts are considered, the better chance we can help guide development of these systems. This presentation will discuss normal development, considerations for persons with SMA and what can be done at any age and stage with the purpose of developing the best possible oral rest posture, nasal breathing, eating, and speaking.</p>	Grand Harbor Ballroom Salons 3 and 4
	<p>Talk it Out for Kids with an SMA-affected Parent (Ages 6- 17) Angela Wigglesworth, Elementary Education Teacher, SMA-Affected Adult Al Freedman, PhD, SMA Dad and Child/Family Psychologist</p> <p>This facilitated session will provide children with an SMA-affected parent (ages 6-17) 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.</p>	Grand Harbor Ballroom Salon 1
	<p>Siblings Talk it Out (Ages 12 to 17) Katlyn O'Brien, Sister of an adult with SMA Traci O'Brien, Sister of an adult with SMA</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 and discussions. This workshop will be facilitated by SMA family siblings and other family members. It is geared for siblings between the ages of 12-17 years old. They will learn ways to cope and manage common situations that are experienced by brothers and sisters. Dress comfortably and be prepared to make friends and have fun!</p>	Grand Harbor Ballroom Salon 2
<p>12:30pm – 1:30pm Lunch Grand Harbor Ballroom North</p>		
<p>12:30pm – 1:30pm Lunch for Florida Chapter Porte Cochere</p>		
<p>1:45pm – 3:15pm Workshop Session #2</p>		
	<p>Dating Disabled: Navigating the Dating World as a Disabled Person Maylan Chávez, BS in Psychology, adult with SMA Michael Hazel, Film Major, adult with SMA</p> <p>This workshop will discuss and dismantle the happiness and heartbreak of dating as a disabled person. Two adults with SMA will be exploring the obstacles, challenges, logistics, and stigmas surrounding the dating world in an open, round-table type discussion with all attendees. Ages 16+ are highly encouraged to attend. The workshop will be answering questions, sharing experiences, anonymously and in person, for those who have doubts, need advice, or simply want encouragement and uplifting with navigating dating.</p>	Asbury Hall B

<p>The Role of Therapy PT and OT in your Child's Life – Being Part of the Team! Jennifer Hubbell, OTR/L Leslie Nelson, PT, PhD, OCS Anne Stratton, MD</p> <p>The goal of this session is to explore the general physical and occupational 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, as well as how different teams may approach therapy considerations. 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>	Asbury Hall A
<p>Technology That Makes Life Easier: <i>Sponsored by Genentech</i> Kevin Schafer, Writer, Podcaster, adult with SMA Tyler Dukes, DevOps Manager, adult with SMA Jenna Coburn, Social Media Manager, adult with SMA Jose Flores, Corporate Speaker, #1 Best-Selling Author, Global Motivator, Workshop Coordinator, adult with SMA Amber Bosselman, Certified Life Coach, adult with SMA</p>  <p>In this panel discussion, five adults with SMA will discuss all things relating to technology and assistive devices that help make life easier for individuals with SMA. The panellists will dive into how they use assistive technology in their personal and work lives. They will share information on the different devices on the market and how to navigate funding those devices. The presentation will be followed by an audience Q and A.</p>	Asbury Hall C and D
<p>Pre-symptomatic and Early Diagnosis Experiences Danyelle Sun, MSW, SMA Mom, Social Work Senior Manager at Cure SMA Diane Murrell, LCSW Marnie Harris, SMA Mom Darlisha Barnes, SMA Mom</p> <p>If you're a family who has received an SMA diagnosis either in-utero, through newborn screening, or some other early diagnosis avenue, please join us in this session. You will have an opportunity to hear stories from others who can relate to your experience and to make connections with other parents, too. Professionals in the SMA community who support newly diagnosed families will also provide support, answer questions, and speak to the unique place you may find yourself in with an early or pre-symptomatic diagnosis.</p>	Grand Harbor Ballroom Salons 3 and 4
<p>Pediatric Therapeutic Yoga for SMA 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. The workshop will include sharing a yoga experience with a SMA family. The workshop will conclude with a review of a typical yoga routine which includes circle of song, eye exercises, postures, progressive relation and sound breath.</p>	Grand Harbor Ballroom Salons 7 and 8
<p>Healing the Grieving Heart – Part I Fred Troutman, RN, PhD David Sereni, DPT Natasha Abruzzo, RN</p> <p>The healing process, Part 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>	Grand Harbor Ballroom Salons 5 and 6
<p>Aquatic Physical Therapy For Sitting/Walking Children (Ages 8 – 13 years old) Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS</p> <p>Bring your swimsuits and head to the pool for an active 90 minutes with a team of aquatic physical therapists and other kids of similar age. 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. An adult must be in the water with each child. We look forward to seeing you in the pool.</p>	

	<p>What it Means to be a Teen on Wheels 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 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>	Grand Harbor Ballroom Salon 1
	<p>Siblings Talk it Out (Ages 5 to 11) Katlyn O'Brien, Sister of an Adult with SMA Traci O'Brien, Sister of an Adult with SMA</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 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>	Grand Harbor Ballroom Salon 2
3:30pm – 4:45pm Workshop Session # 3		
	<p>Pregnancy with SMA Annah Mobus, CMAA/CPC, AAPC, Adult with SMA Type 1, Mother of 1 Megan DeJarnett, Adult with SMA Type 2, Mother of 2 Jennifer Perkins, Adult with SMA Type 3, Mother of 2</p> <p>The workshop will introduce you to the joys, experiences and challenges faced of pregnancy for adults with SMA. Three mothers will discuss the physical, emotional, respiratory, economic, and societal challenges of enduring pregnancy with Spinal Muscular Atrophy. The workshop will include questions and answers segments and experience compilations on topics. The workshop will be broken into pre-pregnancy, the 3 trimesters, and after. The workshop will also provide written segments and contact info from speakers for those who want it.</p>	Grand Harbor Ballroom Salon 1
	<p>Beyond High School – Understanding the Barriers Students with Disabilities Face so We can Prepare Students to Advocate for Themselves Jessica Keough, Founder and President of Faith Above My Ability</p> <p>Jessica Keough, special education teacher, advocate, and champion for PA Act 69 (Medical Assistance for Workers with Disabilities with job success) will share her post-secondary journey. She'll also share practical steps for student centered transition planning, advocacy tips, and ways parents can empower students to navigate the systemic challenges of adult life.</p> <p><i>For ages 13 and up, working professionals with SMA, parents of teens who have IEPs.</i></p>	Grand Harbor Ballroom Salons 7 and 8
	<p>Traveling with SMA <i>Sponsored by Genentech</i></p> <p>Kevan Chandler, Author, Speaker, Founder of We Carry Kevan and Adult with SMA</p> <p>Cory Lee, Accessible Travel Writer, Influencer, and Adult with SMA</p> <p>Sarah Bellish, Manager of Policy and Advocacy at Cure SMA, and Adult with SMA</p> <p>Has the travel bug bitten you, but you're not sure how to make traveling with SMA a reality? Or maybe you're an experienced traveler, but have some questions for our panel of travel experts? Our panelists will answer all your questions about air travel, how to find an accessible hotel, how to find caregivers for your travels, and much more. No matter what your experience is when it comes to traveling, you will undoubtedly be wanting to book your dream trip by the end of this informative session.</p>	Asbury Hall C and D 
	<p>Ethics, Angst, Excitement and Realities of SMA Treatments and Research Rob Graham, MD Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN, FPCN</p> <p>A cure for everyone? Beyond the hope and hype of research, there are ethical, emotional, and practical considerations for the individual with SMA and their family. This session will review factors that may influence decision-making. Extensive time will be allotted for open discussion of research benefits and challenges, access, and distributive justice for newly approved therapies.</p>	Grand Harbor Ballroom Salons 3 and 4

	<p>Financial and Estate Planning with Special Needs Trusts Richard Rubenstein, Esq., RICP®</p> <p>Legal and financial planning 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 the 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. He will also 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>	Asbury Hall B
	<p>Incorporating Exercise Into You or Your Child's Life Leslie Nelson, MPT, PhDc, OCS Anne Stratton, MD Jennifer Hubbell, PT</p> <p>The goal of this session is to provide a broad overview of 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 and experiences. This session will help you better understand the possibility of different forms of exercise and ways to partner with your healthcare team when formal therapy sessions may not be the best option. 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>	Grand Harbor Ballroom Salons 5 and 6
	<p>Finding Hope After Loss: Helping Parents and Children Cope Audra Perry Butler, SMA Parent</p> <p>When a family losses 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>	Grand Harbor Ballroom Salon 2
6:30pm – 8:30pm	<p>Family Friendly Researcher Poster Session Includes Disney Characters: Mr and Mrs Incredible, Edna Mode, Woody and Bo Peep, Latso, Mike and Sulley</p>	Grand Harbor Ballroom
7:30pm – 9:15pm	<p>PJ Party and Movie Night</p>	Grand Harbor Ballroom Salons 5 - 8
7:30pm – 10:00pm	<p>Teen Social Sponsored by Luke 18:1 Foundation <i>**for all teens ages 12 - 18**</i></p> <p style="text-align: center;"><i>LUKE 18:1</i></p>	Asbury Hall A
8:00pm – 11:00pm	<p>Adults with SMA Reception Sponsored by Biogen and The Dhont Family Foundation <i>**For adults with SMA and their attendants**</i></p> <p style="text-align: right;">   </p>	Asbury Hall B, C and D
8:30pm – 11:00pm	<p>Mom's Night Out <i>**for moms only**</i></p>	Promenade Ballroom West <i>**at the Boardwalk Inn Resort</i>

SATURDAY, JULY 1ST | AGENDA

7:30am – 4:00pm	Registration Open for all Conference Attendees	Asbury Rotunda
7:00am – 8:45am	Breakfast	Grand Harbor Ballroom North
7:30am – 4:00pm	Exhibitor Tables Open	Grand Harbor Lobby and Cape Cod Lobby
7:45am – 8:45am	Symposium by Biogen: Personal Journeys with SMA and Clinical Trial Results 	Grand Harbor Ballroom North
9:00am – 12:00pm and 1:15pm – 4:00pm (Drop off opens at 8:30am and 12:45pm)	Children's Program <i>Sponsored by The Jacob Isaac Rappoport Foundation</i> 	Newport Ballroom
9:00am – 4:00pm	Adults with SMA Lounge <i>Sponsored by Biogen</i> <i>**for adults with SMA and their attendants**</i> 	Cape Cod Hall
9:00am – 4:00pm	Teen Lounge <i>Sponsored by Luke 18:1 Foundation</i> <i>**for all teens ages 12 - 18**</i> 	Saybrook
9:00am – 4:00pm	SMA Community Self Expression Art Exhibit <i>Sponsored by Biogen</i> <i>**Meet the artist during lunch from 12:00pm – 1:15pm**</i> 	Asbury Lobby
9:00am – 4:00pm	Community Connection Area <i>Sponsored by Genentech</i> 	West Rotunda
9:00am – 4:00pm	Novartis Gene Therapies Activity Room <i>Sponsored by Novartis Gene Therapies</i> 	Bourne
9:00am – 10:30am Workshop Session #4		
	Unpacking Clinical Trials Thomas Crawford, MD Perry Shieh, MD Julie Parsons, MD Three 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.	Grand Harbor Ballroom South

	<p>Therapeutic Yoga for Adults and Teens with SMA Anne Buckley-Reen, OTR/RYT, FORKIDS OT Peyton Tansey, Adult with SMA</p> <p>The workshop will introduce you to the benefits of yoga for adults and teens with SMA. Anne and her Co-host Peyton will discuss the physical, emotional, respiratory, cognitive and immunological benefits of yoga. The workshop will include a chair practice modeled by Peyton and all are invited to participate. The workshop will conclude with a review of a typical yoga routine which includes circle of song, eye exercises, postures, progressive relaxation and breathing exercises. Learn about the many benefits of yoga practice for SMA including stress reduction, balanced energy, joint mobility, respiratory support and more!</p>	Asbury Hall C and D
	<p>Mindfulness for Stress Reduction Tara Davenport, SMA Parent and Mindfulness Teacher</p> <p>Mindfulness has been shown in study after study to help reduce stress and improve quality of life. But why does it work and how? In this workshop you will learn practical, easy to access ways of implementing mindfulness in your own life, along with an understanding of why it works. Join us as we discuss and learn how to use this practice.</p>	Grand Harbor Ballroom Salon 1
	<p>The Role of the Multi-disciplinary Clinic – Why might it be helpful for those identified Pre-symptomatically Leslie Nelson, PT, PhD, OCS Anne Stratton, MD Carolyn Kelley, DPT, PCS Danielle Forrest, OTR, OTD</p> <p>The goal of this session is to identify the general components of a multi-disciplinary clinic and how the various clinicians and members of the team may be beneficial to you as you navigate the process of guiding the care for your child that was identified with SMA pre symptomatically. We will discuss the potential for guidance regarding rehabilitation, including orthopedic considerations as well as physical and occupational therapy needs of children with SMA in the context of early identification and treatment. We will also discuss how you might take the recommendations provided from your multidisciplinary team and incorporate them into your larger care team (treating therapists and even team members within the school and community settings). 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 specific questions you might have.</p>	Asbury Hall A
	<p>Medical Management of Adults with SMA Bakri Elsheikh, MBBS, FRCP Sally Dunaway Young, DPT</p> <p>This workshop will focus on three key areas: 1) Standards of Care related to the adult medical and rehabilitation management of SMA in the current natural history landscape. 2) We will discuss challenges related to obtaining treatments, present experience, and expectations. 3) We will also discuss the latest research associated with other combination therapies that may be effective in adults with SMA. Specific attention will be aimed toward the importance of integrating, coordinating, and directing one's own care. The session is ideal for families and participants who are transitioning to adult clinics or adults with SMA.</p>	Grand Harbor Ballroom Salons 7 and 8



PLEASE NOTE


June in Florida has the potential to rain, heavy at times, on and off, mostly in the afternoon. Please be prepared when heading outside, to grab ponchos, umbrellas, rain gear, etc. to stay safe and dry!

SATURDAY, JULY 1ST | AGENDA

	<p>Service Dogs: Safety, Independence, Confidence KateLynne Steinke Jones on the Job</p> <p>Anybody that has typed service dog into a search engine knows that it can become overwhelming and confusing easily; distinguishing fact from fiction is difficult unless you already know what you're looking for. This workshop will cover all things service dog related, from what a service dog is and does to acquisition options and handler rights. Join KateLynne and her service dog, Jones, for a presentation full of accurate information and plenty of time to have all your questions answered. KateLynne has first-hand knowledge and experience with multiple schools, different breeds and genders, and has a vast amount of working dog knowledge she's ready to share with you. You'll leave with the knowledge you need to continue with your service dog journey, whichever path you choose, and end up with the dog you deserve to have by your side as your safe and helpful partner. Please join us, no matter where you are in the service dog process, and see what assistance and tasks are possible that could help you in your daily life.</p>	Grand Harbor Ballroom Salons 5 and 6
	<p>Navigating The Special Education Process: Setting our Children Up for Success In Preschool and Elementary School Tina Lewis, Parent of a Child with SMA, and Education Specialist</p> <p>As you embark on the journey to send your child to preschool or elementary school, this session will focus on what you can do to set your child up for success in their very first educational experiences. Through my own personal and professional experiences, I will discuss the essentials for ensuring equitable opportunities for your child. We will discuss the specifics of developing an Individualized Education Program (IEP), a Section 504 Plan, as well as considerations for services and accommodations that will ensure success for your child!</p>	Grand Harbor Ballroom Salons 3 and 4
	<p>I Need to Cough, I Need to Breathe Oren Kupfer, MD Peter Schochet, MD Jane Taylor, 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. Individuals with SMA are at risk for difficulty breathing due to muscle weakness. We will also discuss why the muscle weakness causes difficulty breathing during 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. Bring your questions and experience to this interactive session and we hope to have our whole pulmonary team there!</p>	Asbury Hall B
	<p>Aquatic Physical Therapy for Fun and Function, Sitting/walking Children – Ages 6 months to 7 years Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS</p> <p>Bring your swimsuits and jump in the pool for an active 90 minutes with a team of aquatic physical therapists and other kids of a similar age. 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. A parent or caregiver are required to be in the water with each child. We look forward to seeing you in the water.</p>	
	<p>Memory Boxes: Create a Treasure (for children and teens only) Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>Grieving a loss young is not an easy process and a different experience for everyone. This workshop will provide bereaved children/teens an opportunity to create a box to hold memories of their loved one. Art making provides an outlet for expression of feelings and children/teens will be encouraged to share together while creating their box. Children/teens are welcome to bring photos of their loved ones to use. There will also be a printer available to print a few photos.</p>	Grand Harbor Ballroom Salon 2

10:45am – 12:00pm

Workshop Session # 5

	<p>The Caregiver's Toolbox (Essentials) Wendy Godfrey (ST Wellness)</p> <p>In this session, we will explore simple and effective ways for caregivers to practice their own self-care daily to help minimize stress and improve wellness.</p>	Grand Harbor Ballroom South
	<p>How to Build Your Presence and Advocate Through Writing and Social Media <i>Sponsored by Genentech</i></p> <p>Kevin Schaefer, Writer, Podcaster, and Adult with SMA Allie Williams, Adult with SMA Shane Burcaw, Adult with SMA Brianna Albers, Author, Advocate, and Adult with SMA Kevan Chandler, Author, Speaker, Founder of We Carry Kevan and Adult with SMA LaMondré Pough, Adult with SMA Jose Flores, Corporate Speaker, #1 Best-Selling Author, Global Motivator, and Workshop Facilitator Adult with SMA</p> <p> <i>A Member of the Roche Group</i></p> <p>In this workshop, moderator Kevin Schaefer speaks with several published authors and influencers in the SMA community. These panelists talk about their writing, storytelling techniques, social media platforms and how their work coincides with disability advocacy. They also share tips for getting your work out in the world and the importance of representation in media. An audience Q and A will follow the main conversation.</p>	Asbury Hall C and D
	<p>SMA and Dating/Sex Roundtable Discussion Shonna Counter, Adult with SMA</p> <p>It's amazing how many people are under the impression people with disabilities don't... can't?... aren't interested in?... Sex! Maybe even you, yourself, have wondered... how?... If?... Can I? How do I? – Where can I meet someone? How do I date? When do I mention my disability? Can I even have children? How will I take care of them?</p> <p>This session will be a group discussion featuring everyone's input. Since SMA is such a broad-spectrum disorder, one person cannot have all the answers to every question. Let's come together and collaborate with each other to have frank discussions and offer up ideas and suggestions of our own successes (or warnings about failures) to open up the possibilities for new romantic connections.</p> <p>WARNING: this session is suited for those who are of an appropriate age for dating/discussions about relationships and sexuality. There will likely be non-traditional ideas discussed and we ask that you attend with an open, non-judgmental disposition.</p>	Asbury Hall B
	<p>Keepsake Creation: Grieving Through Art <i>(for anyone)</i> Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>Grieving a loved one is not an easy process and is a different experience for everyone. This workshop will provide participants an opportunity to create keepsakes and share memories of their loved one while art making. Participants are encouraged to bring photos of your loved one for use in the workshop. There will also be a printer available to print a few photos if brought digitally.</p>	Grand Harbor Ballroom Salon 2
	<p>Transitioning to Middle School Mimi Chan, SMA Parent Brady Chan, middle schooler with SMA Rio Landa, SMA Parent Mateo Landa, middle schooler with SMA Dany Sun, MSW, SMA Parent and Social Work Senior Manager, Cure SMA Ruby Sun, middle schooler with SMA</p> <p>The transition to middle school can be both exciting and daunting for any student. Add in accommodations needed for a student with SMA and it can result in parents and students feeling overwhelmed with all the changes. This workshop will help you and your tween prepare to transition to middle school by discussing topics such as logistics, physical education accommodations, using a locker, transitioning from classroom to classroom, and much more! Come hear from a panel of parents and middle schoolers with SMA as they share their experiences, ask questions, and share your insight, too. Together, we can all make the transition to middle school a smooth one!</p>	Grand Harbor Ballroom Salons 7 and 8

SATURDAY, JULY 1ST | AGENDA

	<p>Benefits of Adaptive Recreation Ed Bronsdon</p> <p>Adaptive Recreation provides individuals with and without disabilities with opportunities to participate in recreation, sport and leisure activities. These activities may enrich your quality of life, build community and inclusion, enhance self-esteem, help you to learn new skills, and foster fitness through fun. This workshop will provide a mix of information, demonstration and inspiration for accessing and personally realizing the benefits of adaptive recreation.</p>	Grand Harbor Ballroom Salons 5 and 6
	<p>What is an ABLE Account, and Should You Have One? Mark Solomon (Financial Guide for Special Needs Families)</p> <p>This presentation will be educational. Mark will address what an ABLE Account is, eligibility for you or your child, the effect on benefits, and best practices: Qualified Disability Expenses, Savings Options, and Do you need to purchase in your state? Following the presentation will be a Q and A.</p>	Asbury Hall A
	<p>Genetics and Reproductive Options for SMA Harvey J. Stern, MD, PhD, FACMG, FAAP Melissa Gibbons, MS, CGC</p> <p>This workshop will provide an introduction to the genetics and inheritance of SMA. Our objective is to provide a better understanding of the role SMN1 and SMN2 play in SMA and to discuss the different genetic testing options 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. The wide range of reproductive options for couples who are at-risk for having children with 5q-SMA will be discussed including Preimplantation Genetic Testing (PGT-M) with in vitro fertilization (IVF), Sperm/Egg Donation, Chorionic Villus Sampling, Amniocentesis, Newborn Screening and at birth diagnostic testing.</p>	Grand Harbor Ballroom Salons 3 and 4
	<p>Aquatic Physical Therapy for Fun and Function, Teens and Adults Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS</p> <p>Bring your swimsuits and head to the pool for an active 75 minutes with a team of aquatic physical therapists. Our focus will be on improving functional strength and skills in a dynamic environment. Come try equipment that may be new to you and see how it affects your success and independence in the water. Games, stretching, toys and balance will all be discussed and demonstrated during our session.</p>	Admiral Pool
	<p>Kids Talk it Out (Ages 6 – 11) Angela Wrigglesworth, Elementary Education Teacher, SMA-Affected Adult Al Freedman, PhD, SMA Dad and Child/Family Psychologist</p> <p>This facilitated session will provide SMA-affected children (ages 6-11) 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.</p>	Grand Harbor Ballroom Salon 1
12:00pm – 1:00pm	Lunch	Grand Harbor Ballroom North
1:15pm – 2:30pm	Workshop Session # 6	
	<p>Adults with SMA Relationships Panel – Relationships through Experience Kyla Pollock and Collin Pollock Brittany Bingman and Steve Bingman Jenna Coburn and Tanya Vega Angela Wrigglesworth and Justin Titcombe</p> <p>The workshop will introduce you to spouses and partners of those with SMA. This session is intended for the adults with SMA and their spouses, partners, and significant others for a discussion on navigating relationships with SMA. You will hear from panelists that come from different backgrounds, sexual orientations, and generations, so that we can broaden the audience even further. The discussion may include topics such as love, sex, caregivers, self-care, family life, finances, careers, and other relationship topics through experiences. Audience questions are welcomed and encouraged.</p>	Grand Harbor Ballroom South

	<p>Nutrition for Life! Rebecca Hurst Davis, MS, RD, CSP, CD, CNSC Stacey Tarrant, BS, RD, LDN Laura Watne, MS, RD, CSP</p> <p>In this workshop, we will discuss nutrition for people with SMA throughout their lifetime, from birth to adulthood, whether food is eaten by mouth, provided by tube-feeding, or a mixture of both. We will address the unique nutritional needs of infants, toddlers, school-aged children, teenagers, and adults. We will also discuss how disease-modifying therapies have impacted nutrition recommendations.</p>	Asbury Hall C and D
	<p>Preventing Emergencies – Daily Care, Contingencies and Response in the Community Rob Graham, MD</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>	Asbury Hall B
	<p>Women’s Health in SMA Lorelei Thornburg, MD</p> <p>This workshop will highlight the importance of routine pre-conception care and reproductive planning for people with reproductive potential with SMA. Given the unique obstetrical issues confronted by patients with SMA, we will highlight the importance of identifying specific risks encountered during pregnancy in addition to providing recommendations to improve maternal and fetal outcomes. A vital component of successful pregnancy with SMA is a multidisciplinary approach in a tertiary facility with familiarity in managing neuromuscular disorders.</p>	Asbury Hall A
	<p>Change Your Brain - Change Your Life Amber Bosselman, Certified Life Coach, adult with SMA</p> <p>Living with a disability is hard, because there are many circumstances in our lives that we can’t control. Learning how to respond with intentional thinking creates power and control in the areas of your life that feel messy. Come learn from a certified disability life coach and leave with a skill you can start implementing right now.</p>	Grand Harbor Ballroom Salons 7 and 8
	<p>Navigating The Special Education Process: Transitioning to Middle School, High School, and College Tina Lewis, Parent of a Child with SMA, and Education Specialist</p> <p>As your child strives for greater independence in middle, high school and college, this session will focus on what you can do to set your child up for success as they transition into these settings. Through my own personal and professional experiences, I will discuss the essentials for ensuring equitable opportunities for your child. We will discuss the specifics of transition planning, tools for independence, and disability services typically available for students who plan to attend college.</p>	Grand Harbor Ballroom Salons 5 and 6
	<p>You’re Invited: Fundraising Power Hour Jesse Aynes, Vice President, Development and Events Samantha Edidin, Director, Development and Corporate Engagement Kyle Houlihan, Director, Development and Events Alyssa Zavislak, Sr. Manager, Development and Events</p> <p>Be our guest and come learn how to supercharge your fundraising efforts! This session will feature tips, tricks, and best practices for fundraising success delivered to you by Cure SMA community members. Are you someone interested in hosting an electric and powerful fundraising event? Or maybe you’re a Walk-n-Roll or Endurance team captain and want to turn up the dial on your fundraising? Perhaps you want to amplify an existing event experience? Whether you’re new to fundraising, or are well-versed in this area, this session is for you!</p>	Grand Harbor Ballroom Salons 3 and 4
	<p>Healing the Grieving Heart – Now What? Part II Fred Troutman, RN, PhD David Sereni, DPT Natasha Abruzzo, RN</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.</p>	Grand Harbor Ballroom Salon 1

SATURDAY, JULY 1ST | AGENDA

	<p>Grandparents Coffee and Share Your Experience Moderated by Sylvia Wheeler, Grandparent</p> <p>Grab some coffee while you join other grandparents who have a grandchild affected by SMA. Share stories, photos and make new connections. This session is intended for grandparents of those who have SMA.</p>	Grand Harbor Ballroom Salon 2
	<p>Aquatic Physical Therapy for Fun and Function, Children with Supported Head Control Jennifer Martyn, PT Krista Torseth, PT, DPT Kendra Paker, PT, DPT, PCS</p> <p>Put on your swimsuits and head to the pool for an active 75 minutes with a team of aquatic physical therapists. Our focus will be on improving functional strength and skills in an environment designed to maximize independent movement. Games, stretching, toys, and balance and will all be discussed and demonstrated during our session. An adult must be in the water with each child. We look forward to seeing you in the pool.</p>	
<p>2:45pm – 3:50pm Workshop Session # 7</p>		
	<p>Early Scoliosis Management in SMA Brian Snyder, MD</p> <p>This workshop will focus on care of early scoliosis with SMA including monitoring and management considerations and strategies.</p>	Asbury Hall A
	<p>Young Adults Talk it Out (Ages 18+) Angela Wigglesworth, Elementary Education Teacher, SMA-Affected Adult 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.</p>	Asbury Hall C and D
	<p>University of Utah TRAILS Program: Adaptive Sports and Novel Adaptive Equipment Melissa McIntyre, DPT</p> <p>TRAILS is an adaptive sports program at the University of Utah that focuses on the recreation, wellness, and education needs of people with complex physical disabilities. Since its creation in 2005 by Dr. Jeffery Rosenbluth and Tanja Kari, TRAILS has spearheaded the creation of novel adaptive equipment that allows athletes to participate and compete in sports that historically would have been unavailable to them. This workshop will briefly go over the history of TRAILS, followed by a discussion of programming and available equipment. We will conclude by discussing current outreach efforts and ways to get involved.</p>	Asbury Hall B
	<p>Plan an Ability Awareness Week at Your Elementary School Cathy Barsotti, SMA Parent Dany Sun, MSW, SMA Parent and Social Work Senior Manager, Cure SMA</p> <p>This workshop will introduce you to an Abilities Awareness Week curriculum that you can implement at your child's school, in partnership with your child's school team. Cathy and Dany will discuss ways to implement a week of activities and experiences for students to learn about a wide range of disabilities others experience. An Abilities Awareness Week program can help to spread awareness, teach empathy, and model ways to include everyone in play and learning. You will be provided with a framework to present to your child's school and steps to take to implement the week's activities.</p>	Grand Harbor Ballroom Salons 7 and 8

	<p>Q and A Session with The Pulmonary Team Oren Kupfer, MD Peter Schochet, MD Jane Taylor, MD Richard Kravitz, MD</p> <p>Over the last few years, disease-moderating medications have become available to treat patients with SMA. These agents have positively altered the day-to-day living of children and adults with SMA and are improving the prognosis of this illness. Respiratory and sleep changes are amongst the benefits seen. In our 2023 Annual Cure SMA Family conference, the pulmonologists and sleep physicians who serve on the MAC are holding sessions on airway clearance, respiratory support, optimizing sleep health and an open free flowing QandA session. Come meet our pulmonologists and be ready with your questions, as our experts look forward to answering them while giving you updates on what to expect in the coming years.</p>	Grand Harbor Ballroom Salons 5 and 6
	<p>Parenting with SMA Collin Pollock Annah Mobus Carli Hamilton</p> <p>Join a few panelists for a conversation about the rewards and challenges of being a parent living with SMA.</p>	Grand Harbor Ballroom South
	<p>Get to the Art of It: Community Art Time Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>It's time for some creative fun time! All are welcome! Participants will be encouraged to create mixed media art and will be provided with choices for making a signboard, decorative boxes, and other keepsake items. Take a break from the day for some art time and create a memory that will last a lifetime.</p>	Grand Harbor Ballroom North
4:00pm	Cure SMA Evening in the Park	EPCOT

SMA Community Self Expression Art Exhibit in Asbury Hall

Members of the SMA community were invited to share their unique pieces of art, and this exhibition will be open for attendees to enjoy on Friday, June 30th from 11:15am – 4:00pm.

The exhibition will also be open on Saturday, July 1st from 9:00am-4:00pm, with a special opportunity to meet the artists from 12:00pm-1:15pm.



This self-expression art exhibit is generously sponsored by Biogen.

SUNDAY, JULY 2ND | AGENDA

7:30am – 8:45am		Breakfast		Grand Harbor Ballroom North
9:00am – 11:15am (drop off opens at 8:30am)	Children's Program <i>Sponsored by The Jacob Isaac Rappoport Foundation</i>		Newport Ballroom	
9:00am – 11:15am	It's a Wonderful Life/Closing General Session		Grand Harbor Ballroom South	

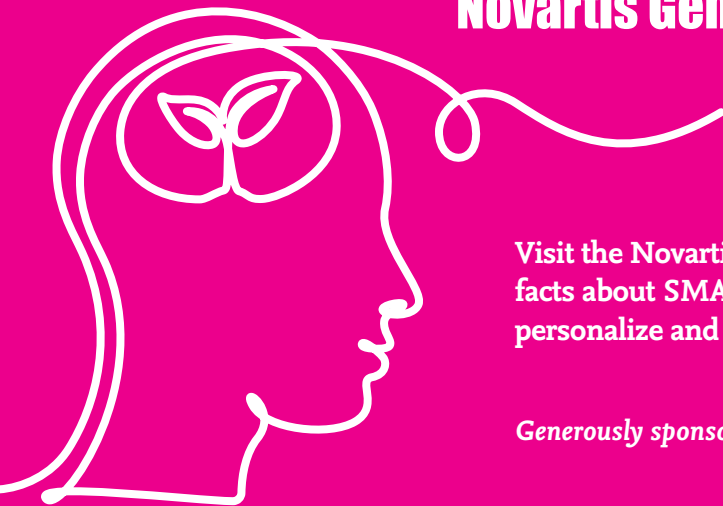


PLEASE NOTE

WEAR SUNSCREEN AND KEEP HYDRATED!

As many of you know, June in Florida 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 the heat!

Novartis Gene Therapies Activity Room | in Bourne



Visit the Novartis Gene Therapies Activity Room to learn facts about SMA, and to receive a pair of mickey ears to personalize and bedazzle to your liking.

Generously sponsored by Novartis Gene Therapies.



FAMILY FRIENDLY RESEARCHER POSTER SESSION

FRIDAY, JUNE 30 6:30PM – 8:30PM IN GRAND HARBOR BALLROOM

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

POSTERS INCLUDE:

- **TOWARDS A COMBINED THERAPY FOR SMA BASED ON NUSINERSEN AND VALPROIC ACID**
Alberto Kornblihtt, PhD, University of Buenos Aires and CONICET, Argentina
- **THE CURE SMA CARE CENTER NETWORK**
Alesa Monk, MBA, Cure SMA
- **THE ROLE OF MUSCLE IN SMA**
Ameet Khara, PharmD; Scott Bayer, PhD; Nathalie Kertesz, B.S., PhD, Scholar Rock
- **PROSPECTIVE MONITORING OF MUSCULOSKELETAL FINDINGS IN PATIENTS WITH SMA**
Anne Stratton, MD, Scott Miller
- **ONE RING TO RULE THEM ALL: ARE SM-RING ASSEMBLY FUNCTIONS CENTRAL TO THE DEVELOPMENT OF SMA?**
Anton Blatnik, PhD, The Ohio State University
- **ON THE ROAD TO RNA TRAFFICKING TO UNLOCK THE SECRETS OF SMA**
Florence Rage, IGMM, Univ Montpellier, CNRS, Montpellier, France
- **A TRIAL TO EVALUATE THE EFFICACY AND SAFETY OF TALDEFGROBEP ALFA IN AMBULATORY AND NON-AMBULATORY PARTICIPANTS WITH SPINAL MUSCULAR ATROPHY**
Lindsey Lee Lair MD, Biohaven
- **A CROSS-SECTIONAL EXAMINATION OF THE SMA COMMUNITY USING THE CURE SMA 2022 COMMUNITY UPDATE SURVEY**
Lisa Belter, MPH, Cure SMA
- **USING MICE TO UNDERSTAND NEUROMUSCULAR JUNCTION RECOVERY FOLLOWING TREATMENT IN SMA**
Lyndsay Murray, PhD, University of Edinburgh, UK
- **CURE SMA: SUPPORTING NEW SMA RESEARCH AT NIH THROUGH LEGISLATIVE ADVOCACY**
Maynard Friesz, Cure SMA
- **DEVELOPMENT OF A 12-TIER FUNCTIONAL SCALE FOR SPINAL MUSCULAR ATROPHY: A NEW COMMON LANGUAGE FOR ALL**
Meghan Moore Burk PT, DPT, Children's Hospital Colorado
- **LONG-TERM FOLLOW-UP STUDIES OF INTRAVENOUS AND INTRATHECAL ONASEMNOGENE ABEPARVOVEC GENE THERAPY IN SYMPTOMATIC AND PRESYMPTOMATIC SPINAL MUSCULAR ATROPHY**
Melissa Wigderson, Novartis Gene Therapies
- **UNLOCKING THE POTENTIAL OF PGE2 TO INCREASE MUSCLE STRENGTH IN SMA**
Micah Webster, PhD, Epirium Bio, San Diego, CA
- **A NEW CLINICAL STUDY AND UTILIZATION OF MODERN TECHNOLOGIES TO EVALUATE THE FERTILITY OF MEN WITH SPINAL MUSCULAR ATROPHY (SMA)**
Natan Bar-Chama MD, Reproductive Medicine Associates of New York, The Mount Sinai Hospital New York, NY
- **DEVELOPMENT OF AN INTERNATIONAL SMA BULBAR ASSESSMENT FOR INTER-PROFESSIONAL ADMINISTRATION**
Sally Dunaway Young, PT, DPT, Stanford University
- **THE ADULT SMA TREATMENT AND RESEARCH PROGRAM AT THE OHIO STATE UNIVERSITY**
Sarah Heintzman, APRN-CNP, FNP-C, The Ohio State University
- **WHAT'S NEW IN THE SMA CLINICAL DATE REGITRY? A 2023 UPDATE**
Sarah Whitmire, MS, Cure SMA
- **ELECTRICAL SIGNALS IN SMA**
Saravanan Arumugam, PhD, Department of Medical Physiology and Biophysics, School of Medicine, University of Seville, Spain
- **REAL-WORLD EXPERIENCE WITH RISDIPLAM TREATMENT**
Sheila Shapouri, PharmD, MS, Senior Health Economist, Genentech
- **IMPACTING EQUITABLE ACCESS TO INFORMATION AND PROGRAMS AMONG SPANISH SPEAKING SMA COMMUNITY MEMBERS**
Sundip Ravel, PharmD, Biogen
- **GRIEF COURSE IN BEREAVED PARENTS OF CHILDREN WITH SPINAL MUSCULAR ATROPHY**
Tara A. Lavelle, PhD, Assistant Professor, Tufts Medical Center, Boston, MA
- **ROCHE/GENENTECH CONTINUED EFFORTS TO ADVANCE CARE IN SMA**
Travis Dickendesh, PhD, Principal Medical Science Director, Genentech
- **BIOGEN'S SMA CLINICAL RESEARCH PROGRAM**
Wendy Agnese, PharmD, Biogen
- **THE SMA DAILY LIFE STUDY: AN EXPERIENCE SAMPLING STUDY EXAMINING PATIENT-CENTRIC OUTCOMES IN THE DAILY LIVES OF INDIVIDUALS LIVING WITH SMA**
Yasemin Erbas, SMA Europe
- **EVERY DOLLAR COUNTS: POWERING CURE SMA THROUGH FUNDRAISING**
Jesse Aynes, Sam Edidin, Kyle Houlihan, Cure SMA



**MEET SOME OF YOUR
FAVORITE DISNEY CHARACTERS
AT THE POSTER SESSION!**



Saturday Evening at the Disney Theme Park

You are invited to join us on Saturday for an evening of fun at the EPCOT Theme Park. All registered attendees will receive a complimentary park entrance ticket for anytime after 4:00pm.

Cure SMA Conference Photo at 5:00PM!

Cure SMA would like to thank our Sponsors!

PRESENTING SPONSORS:



DIAMOND SPONSOR:



TITLE SPONSOR:





**Cure SMA thanks The Dhont Family Foundation,
leaders in scientific and social research investment,
for their continued support for those living with SMA,
their families and caregivers.**

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!



2023 SUMMIT OF STRENGTH

PROGRAM



A LOCAL EVENT UNITING THE SMA COMMUNITY AROUND TREATMENT, CARE, AND SUPPORT

Summits are one a day educational events, crafted to provide people of all ages and types of SMA and their caregivers the opportunity to network and learn about the latest advances in treatment, care, advocacy, and support.

January 21 | Walnut Creek, CA

February 4 | Atlanta, GA

February 18 | Dallas, TX

March 4 | NYC, NY

March 25 | Baltimore, MD

April 22 | Ann Arbor, MI

April 29 | Portland, OR

May 13 | Cincinnati, OH

May 20 | Mystic, CT

June 3 | Minneapolis, MN

July 22 | Chicago, IL

August 12 | Boston, MA

August 19 | Seattle, WA

September 9 | Birmingham, AL

September 23 | Orlando, FL

October 21 | Charleston, SC

November 4 | Houston, TX

November 11 | Anaheim, CA

November 18 | Miami, FL**

December 2 | New Orleans, LA

December 9 | Phoenix, AZ

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

***The November 18, Miami, FL, SOS will be available in both English and en Español.*

Summit of Strength Webinar Series

Cure SMA offers a variety of educational online content specifically tailored for individuals and families living with SMA. Check out our YouTube channel at youtube.com/@CureSMA for all of these valuable webinars!

Visit our website to register for these events, or check back for any changes in schedule:
summit-curesma.donordrive.com

Cure SMA would like to thank our 2023 Summit of Strength Sponsors

National Presenting Sponsors



National Platinum Sponsor



National Visionary Sponsors



For more info or questions please email communitysupport@curesma.org | 800.886.1762 | curesma.org



CHILDREN'S PROGRAM ENTERTAINMENT SCHEDULE



No Food Allowed in Children's Program

Please do not bring any food into the children's program since some of the children attending have severe food allergies.

FRIDAY JUNE 30TH, 2023

CHILDREN'S PROGRAM MORNING SESSION

9:00AM – 12:30PM

9:00AM – 12:30PM

Cartoon You Caricatures Stop by this fun station so a caricaturist can draw a photo of you to take home as a fun conference memento!

11:15AM – 12:15PM

Fire and Ice Show Everyone will be dazzled and entertained as they interact with our Mad Scientist! Foggy dry ice storms, spectacular fireballs, and even a special Mad Science burp position will amaze audiences as they learn about chemical reactions, dry ice and the states of matter!

CHILDREN'S PROGRAM AFTERNOON SESSION

1:45PM – 4:45PM

1:45PM – 4:45PM

Cartoon You Caricatures Stop by this fun station so a caricaturist can draw a photo of you to take home as a fun conference memento!

2:30PM – 3:30PM

Superheroes of Science Show It's a Bird! It's a Plane! It's SUPERHERO SCIENTISTS! This show is jam packed with the best heroes of Science- Tesla, Bernoulli... while we explore the Science behind superhero powers! Watch as we make Superman fly and make a storm indoors!

SATURDAY JULY 1ST, 2023

CHILDREN'S PROGRAM MORNING SESSION

9:00AM – 12:15PM

9:00AM – 12:15PM

Cartoon You Caricatures Stop by this fun station so a caricaturist can draw a photo of you to take home as a fun conference memento!

11:00AM – 11:30AM

Bubble Show Mr. Keith your Bubbleologist will perform a 30-minute, interactive show featuring rainbow bubbles, giant bubbles, small bubbles, bubbles inside of bubbles, smoke (vapor) bubbles and Mr. Keith's friend Snapper. All this happens while sharing some of the fascinating Science behind bubbles.

CHILDREN'S PROGRAM AFTERNOON SESSION

1:15PM – 4:00PM

1:15PM – 4:00PM

Cartoon You Caricatures Stop by this fun station so a caricaturist can draw a photo of you to take home as a fun conference memento!

2:00PM – 3:00PM

Circus Magic Show Jacki Manna presents this Circus style show which is corny, wacky, wild, crazy, silly, funny, engaging, and side-splitting with zany puppets, amazing magic and circus tricks like plate spinning.

SUNDAY JULY 2ND, 2023

CHILDREN'S PROGRAM MORNING SESSION

9AM – 11:15AM

9AM – 11:15AM

Cartoon You Caricatures Stop by this fun station so a caricaturist can draw a photo of you to take home as a fun conference memento!

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

Sponsored by





HOW DO BATHROOM BREAKS WORK?

Every time any child has to use the restroom a parent will be contacted via text message or phone call to come take their child to the restroom. Volunteers are **not allowed** to escort any children to the bathroom. Parents please have your phones handy so you can be reached for toileting breaks.

CAN I LEAVE MY CHILD WITH SMA WHO REQUIRES BREATHING ASSISTANCE IN THE CHILDREN'S PROGRAM?

No, since the children's program is run by volunteers they are not responsible for any medical care for children with SMA. A child who requires any breathing support, cough assist or suction cannot be left in the Children's Program without a nurse or caretaker.

CAN MY CHILD CHECK THEMSELVES IN AND OUT OF THE CHILDREN'S PROGRAM?

If your child is 12 years of age or older and you sign the consent on their registration form then they are allowed to check themselves in and out of the children's program.

WHAT DO VOLUNTEERS ASSIST WITH THROUGHOUT THE DAY?

Volunteers supervise and assist children by playing games, watching movies, doing arts and crafts, etc. Volunteers are not responsible for any medical care, medicine disbursement, bathroom breaks/diaper changes, feeding responsibilities or wheelchair/stander transfers. Parents or their caretakers will be notified via cell phone or text message to assume those responsibilities no matter the type of SMA the child has.

IF I AM NOT LEAVING MY CHILD ALONE IN THE CHILDREN'S PROGRAM DO I STILL HAVE TO FILL OUT A REGISTRATION FORM?

Yes- we ask that every child who enters the room (with or without a parent or caretaker) please fill out the registration form so we have contact information in case of emergency.

CAN I BRING FOOD OR DRINKS INTO THE CHILDREN'S PROGRAM?

No, food and drinks are not allowed into the children's program since some of the children attending have severe food allergies.

WHEN IS THE CHILDREN'S PROGRAM OPEN?

The children's program is open during workshops only so please make sure to pick up your children as soon as your workshops end during the morning and afternoon sessions!

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.



CLINICAL AND REAL-WORLD STUDIES SHOW



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

*Pivotal trials did not include adult patients with spinal muscular atrophy (SMA).

IS POSSIBLE

Results from SPINRAZA (nusinersen) trials

Later-onset SMA: The CHERISH clinical trial studied 126 nonambulatory individuals aged 2 to 9 years with later-onset SMA. At 15 months, the average score for people taking SPINRAZA showed a 3.9-point improvement in overall motor function as measured by the Hammersmith Functional Motor Scale–Expanded (HFMSE) versus a 1.0-point decline in the untreated control group.

Early-onset SMA: The ENDEAR clinical trial studied 121 children aged 7 months and younger with early-onset SMA. At 13 months, 51% of children taking SPINRAZA were motor milestone responders compared with 0% of untreated children. Additionally, there was a 47% reduced risk of mortality or permanent ventilation in the SPINRAZA group versus the untreated group.

- A responder is a child who had at least a 2-point increase in ability to kick, or at least a 1-point increase in categories like head control, rolling, sitting, crawling, standing, or walking

Presymptomatic SMA: In the ongoing NURTURE supportive, open-label (no control group) study of 25 infants aged 6 weeks and younger who had not yet shown symptoms of SMA, 100% were alive without permanent ventilation after at least 14 months.

Real-world evidence

In an independent, observational study of individuals aged 16 to 65 years with later-onset SMA taking SPINRAZA, the average improvement in overall motor function, as measured by HFMSE, was 1.73 at 6 months (124 individuals), 2.58 at 10 months (92 individuals), and 3.12 at 14 months (57 individuals). While some adults experienced stabilization or significant improvement in motor function, some adults did not.

- Limitations included: no untreated control group for comparisons, all patients received SPINRAZA, observational studies are valuable but not as strong as pivotal studies

INDICATION

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

IMPORTANT SAFETY INFORMATION

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

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

The most common side effects of SPINRAZA include lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome. These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

Please see Important Facts about SPINRAZA on the next page and full Prescribing Information on SPINRAZA.com.

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

Talk to your doctor about the possibilities with SPINRAZA.



LAUREN // AGE 20
ART ENTHUSIAST
TREATED WITH SPINRAZA



STEPHEN // AGE 39
FATHER
TREATED WITH SPINRAZA



CARLEE // AGE 11
BAKER
TREATED WITH SPINRAZA

Come visit us at the SPINRAZA booth and say hi!

13,000+

individuals have been treated with SPINRAZA worldwide[†]

From 3 days to 80 years old,^{†‡}

there's someone from almost every age group who has taken SPINRAZA

4,600+ adults

have been treated with SPINRAZA worldwide[†]

[†]Based on commercial patients, early access patients, and clinical trial participants through May 2022.

[‡]Pivotal clinical studies of SPINRAZA included patients aged 3 days to 16 years at first dose, but they did not include sufficient numbers of subjects aged 65 years and older to determine whether they respond differently from younger patients.

IMPORTANT FACTS ABOUT SPINRAZA[®] (nusinersen)

USES

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

WARNINGS

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

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

COMMON SIDE EFFECTS

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

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

OTHER INFORMATION

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

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

QUESTIONS?

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

MANUFACTURED FOR

Biogen, Cambridge, MA 02142

THANK YOU TO OUR VOLUNTEERS



Thank You to The Conference Children's Program Volunteers

Without this amazing group of individuals, our conference would not be possible. The level of enthusiasm each volunteer provides throughout the day in the children's program and during the evening events is endless and even contagious! Every volunteer has a major impact on the success and atmosphere 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 all while they attend important workshops to learn about the latest information on SMA. Our volunteers take great pride in helping to ensure families leave with the feeling that this was the best conference yet! Thank you to every single volunteer who helps make this conference possible for everything you do!

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 33 YEARS

George Ghorbanian
Chicago Police Officer



VOLUNTEER FOR 31 YEARS

Patti Slojkowski
Dental Hygienist



VOLUNTEER FOR 28 YEARS

Steve Smith
Physical Therapist



VOLUNTEER FOR 24 YEARS

Kelly Milito
Hair Stylist



VOLUNTEER FOR 23 YEARS

Kelly Basso
Electrologist



VOLUNTEER FOR 22 YEARS

Katlyn O'Brien
Teacher

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 22 YEARS

Mary Blume
Caregiver for Grandkids Mason and Ava



VOLUNTEER FOR 21 YEARS

Noreen Reilly
Data Analyst



VOLUNTEER FOR 21 YEARS

Traci O'Brien
Marketing Consultant



VOLUNTEER FOR 16 YEARS

Julie Smith
IT Director



VOLUNTEER FOR 14 YEARS

Jackie Staples
Event Planner



VOLUNTEER FOR 13 YEARS

Patrick Hogan
High School Teacher and Coach



VOLUNTEER FOR 13 YEARS

Jackie Graney
Studio 22 Dance Director



VOLUNTEER FOR 12 YEARS

Megan Milito
Dance Teacher



VOLUNTEER FOR 12 YEARS

Joy Martin
Physical Therapist



VOLUNTEER FOR 12 YEARS

Donna Budil
Preschool Director



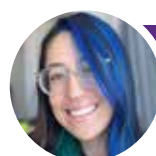
VOLUNTEER FOR 11 YEARS

Kayla Forsythe
Registered Nurse



VOLUNTEER FOR 11 YEARS

Lexi Basso
Registered Nurse



VOLUNTEER FOR 11 YEARS

Hayley Miller
Senior Solution Engineer



VOLUNTEER FOR 11 YEARS

Jennifer Walsh
Business Systems Analyst



VOLUNTEER FOR 10 YEARS

Georgia Slojkowski
Elementary Orchestra Teacher



VOLUNTEER FOR 9 YEARS

Patrick Slojkowski
Associate Scientist

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 9 YEARS

Megan Besler
Financial Analyst



VOLUNTEER FOR 8 YEARS

Raphi Milito
Sales Specialist



VOLUNTEER FOR 8 YEARS

Michael Milito
Project Engineer



VOLUNTEER FOR 8 YEARS

Ralph Milito
Real Estate Agent



VOLUNTEER FOR 8 YEARS

Daniela Milito
Chef



VOLUNTEER FOR 8 YEARS

Sally McCarthy
Retired Nurse



VOLUNTEER FOR 7 YEARS

Alanna Woods



VOLUNTEER FOR 7 YEARS

Michele Rubenstein
Assistant Teacher at Therapeutic Preschool



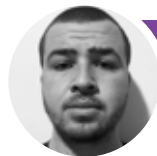
VOLUNTEER FOR 7 YEARS

Emma Rubenstein
Student at Marist College



VOLUNTEER FOR 7 YEARS

Rebeka Nekolova
Freelance Editor



VOLUNTEER FOR 7 YEARS

Jack Jomarron
Policy Analyst



VOLUNTEER FOR 6 YEARS

Samantha Smith
Occupational Therapy Student



VOLUNTEER FOR 6 YEARS

Isabella Hernandez
Student at Depaul



VOLUNTEER FOR 6 YEARS

Cara Freedman
Research Assitant at NORD



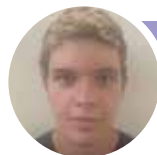
VOLUNTEER FOR 6 YEARS

Katie Dieckhoff
Early Head Start Teacher



VOLUNTEER FOR 5 YEARS

Anne Wallo
NBA Entertainer and Realtor



VOLUNTEER FOR 5 YEARS

Will Reilly
Student

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 5 YEARS

Morgan Ramirez
Student



VOLUNTEER FOR 2 YEARS

Molly Ramirez
Student at NWU



VOLUNTEER FOR 5 YEARS

Julia Kafkes
Student



VOLUNTEER FOR 2 YEARS

Emily Pehoski
Board Certified Behavior Analyst



VOLUNTEER FOR 4 YEARS

Sarah Grimson
Retail Manager



VOLUNTEER FOR 2 YEARS

Steve Paoletti
Law Student and U.S. Army Reserve Soldier



VOLUNTEER FOR 2 YEARS

Alison Williams
Student at the University of Missouri



VOLUNTEER FOR 2 YEARS

Dolores O'Tooles
Support worker and Special Needs Assistant



VOLUNTEER FOR 2 YEARS

Ryan Tribe
Student at Illinois Wesleyan



VOLUNTEER FOR 2 YEARS

Sally O'Toole
Student



VOLUNTEER FOR 2 YEARS

Chelsea Staples
Veterinary Assistant



VOLUNTEER FOR 2 YEARS

Daisy Ortiz
Server



VOLUNTEER FOR 2 YEARS

Cathy Staples
Orthodontal Assistant



VOLUNTEER FOR 2 YEARS

Jack Olech
Student



VOLUNTEER FOR 2 YEARS

Hannah Smith
Student at Drake



VOLUNTEER FOR 2 YEARS

Lexi Nickols
Social Worker



VOLUNTEER FOR 2 YEARS

Dave Richmond
Property Manager

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 2 YEARS

Kay McGovern
Private Investigator



VOLUNTEER FOR 2 YEARS

Terry McGovern
Sales Specialist



VOLUNTEER FOR 2 YEARS

Stacey McDonogh
Student Social Care Worker



VOLUNTEER FOR 2 YEARS

Bryan Lopez
Mortgage Specialist



VOLUNTEER FOR 2 YEARS

Nick Lazic



VOLUNTEER FOR 2 YEARS

Kristi Lazic
Registered Nurse



VOLUNTEER FOR 2 YEARS

Stacy Hernandez



VOLUNTEER FOR 2 YEARS

Lola Hernandez
Student



VOLUNTEER FOR 2 YEARS

Jim Graf
Warehouse Lead



VOLUNTEER FOR 2 YEARS

Annie Graf
Paraprofessional



VOLUNTEER FOR 2 YEARS

Logan Dimitriou
Student



VOLUNTEER FOR 2 YEARS

Carter Cotton
Student



VOLUNTEER FOR 2 YEARS

Shelby Cavanaugh
Student



VOLUNTEER FOR 2 YEARS

Lily Carlson
Student



VOLUNTEER FOR 2 YEARS

Eric Berghoff
Student



VOLUNTEER FOR 2 YEARS

Lena Aherns
Student



Kelly Basso | VOLUNTEER FOR 23 YEARS

The 2023 Annual SMA Conference marks a huge milestone for one of our longest returning volunteers, Kelly Basso! Kelly volunteered at her first conference 23 years ago and has been involved ever since! Every year she takes time away from her own family and grandkids to travel and support the Children's Program. You may even recognize her as a familiar face at the check-in desk as she has become a regular there ensuring everyone gets checked in and checked out! Kelly is the true definition of a dedicated volunteer. Everyone at Cure SMA would like to thank Kelly for all her hard work and dedication over the last 23 years!



Mary Blume | VOLUNTEER FOR 22 YEARS

The 2023 Annual SMA Conference also marks a big milestone for another one of our long-standing volunteers, Mary Blume! Mary volunteered 22 years ago at her first conference and has been involved ever since! Each year Mary takes time away from her family and watching her own grandchildren, Mason and Ava, to come out and volunteer in the Children's Program. For those with younger kids, you may recognize her as a familiar face in the Baby Room since she has become a regular there every year! She is a great representation of a dedicated volunteer that we are so lucky to have! Everyone at Cure SMA would like to thank Mary for her dedication and service over the last 22 years!

MOVING AHEAD

A **community** going
places **together**



Join us for a community symposium

FRIDAY, JUNE 30 | 7:45AM-8:45AM ET

- Hear first-hand perspectives from people taking **the only oral, non-invasive, at-home therapy for SMA**
- Review the science of SMA and **how Evrysdi works**

Stop by our exhibit booth

FRIDAY, JUNE 30 | 7:30AM-5:00PM ET

SATURDAY, JULY 1 | 7:30AM-4:00PM ET

What is Evrysdi?

Evrysdi is a prescription medicine used to treat spinal muscular atrophy (SMA) in children and adults.

Important Safety Information

Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant, as Evrysdi may harm your unborn baby. Ask your healthcare provider for advice before taking this medicine
- are a woman who can become pregnant:
 - Before you start your treatment with Evrysdi, your healthcare provider may test you for pregnancy
 - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping Evrysdi
 - **Pregnancy Registry.** Talk to your healthcare provider right away if you become pregnant while taking Evrysdi. Ask about registering with the Evrysdi Pregnancy Registry, which was created to collect information about your health and your baby's health. Your healthcare provider can enroll you in this registry by calling 1-833-760-1098 or visiting www.evrysdipregnancyregistry.com
- are an adult male. Evrysdi may affect a man's ability to have children (fertility). Ask a healthcare provider for advice before taking this medicine
- are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby

Tell your healthcare provider about all the medicines you take.

You should receive Evrysdi from the pharmacy as a liquid. If the medicine in the bottle is a powder, **do not use it.** Contact your pharmacist for a replacement.

Avoid getting Evrysdi on your skin or in your eyes. If Evrysdi gets on your skin, wash the area with soap and water. If Evrysdi gets in your eyes, rinse your eyes with water.

The most common side effects of Evrysdi include:

- For later-onset SMA: fever, diarrhea, rash
- For infantile-onset SMA: fever; diarrhea; rash; runny nose, sneezing, and sore throat (upper respiratory infection); lung infection (lower respiratory infection); constipation; vomiting; cough

These are not all of the possible side effects of Evrysdi. For more information on the risk and benefits profile of Evrysdi, ask your healthcare provider or pharmacist.

You may report side effects to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at 1-888-835-2555.

Please see accompanying brief summary for additional Important Safety Information.

Talk with your doctor about Evrysdi
or visit **Evrysdi.com** to learn more



Patient Information
EVRYSDI® (ev-RIZ-dee)
(risdiplam)
for oral solution

What is EVRYSDI?

- EVRYSDI is a prescription medicine used to treat spinal muscular atrophy (SMA) in children and adults.

Before taking EVRYSDI, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. If you are pregnant, or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. EVRYSDI may harm your unborn baby.
- are a woman who can become pregnant:
 - Before you start your treatment with EVRYSDI, your healthcare provider may test you for pregnancy. Because EVRYSDI may harm your unborn baby, you and your healthcare provider will decide if taking EVRYSDI is right for you during this time.
 - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping EVRYSDI.
 - **Pregnancy Registry.** There is a pregnancy registry for women who take EVRYSDI during pregnancy. If you become pregnant while receiving EVRYSDI, tell your healthcare provider right away. Talk to your healthcare provider about registering with the EVRYSDI Pregnancy Registry. The purpose of this registry is to collect information about your health and your baby's health. Your healthcare provider can enroll you in this registry by calling 1-833-760-1098 or visiting <https://www.evrysdipregnancyregistry.com>.
- are an adult male planning to have children: EVRYSDI may affect a man's ability to have children (fertility). If this is of concern to you, make sure to ask a healthcare provider for advice.
- are breastfeeding or plan to breastfeed. It is not known if EVRYSDI passes into breast milk and may harm your baby. If you plan to breastfeed, discuss with your healthcare provider about the best way to feed your baby while on treatment with EVRYSDI.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider, including your pharmacist, when you get a new medicine.

How should I take EVRYSDI?

See the detailed Instructions for Use that comes with EVRYSDI for information on how to take or give EVRYSDI oral solution.

- You should receive EVRYSDI from the pharmacy as a liquid that can be given by mouth or through a feeding tube. The liquid solution is prepared by your pharmacist or other healthcare provider. If the medicine in the bottle is a powder, **do not use it.** Contact your pharmacist for a replacement.
- Avoid getting EVRYSDI on your skin or in your eyes. If EVRYSDI gets on your skin, wash the area with soap and water. If EVRYSDI gets in your eyes, rinse your eyes with water.

Taking EVRYSDI

- Your healthcare provider will tell you how long you or your child needs to take EVRYSDI. Do not stop treatment with EVRYSDI unless your healthcare provider tells you to.
- For infants and children, your healthcare provider will determine the daily dose of EVRYSDI needed based on your child's age and weight. For adults, take 5 mg of EVRYSDI daily.
 - Take EVRYSDI exactly as your healthcare provider tells you to take it. Do not change the dose without talking to your healthcare provider.
- Take EVRYSDI 1 time daily after a meal (or after breastfeeding for a child) at approximately the same time each day. Drink water afterwards to make sure EVRYSDI has been completely swallowed.
- Do not mix EVRYSDI with formula or milk.
- If you are unable to swallow and have a nasogastric or gastrostomy tube, EVRYSDI can be given through the tube.
- If you miss a dose of EVRYSDI:
 - If you remember the missed dose within 6 hours of when you normally take EVRYSDI, then take or give the dose. Continue taking EVRYSDI at your usual time the next day.
 - If you remember the missed dose more than 6 hours after you normally take EVRYSDI, skip the missed dose. Take your next dose at your usual time the next day.
- If you do not fully swallow the dose, or you vomit after taking a dose, **do not take** another dose of EVRYSDI to make up for that dose. Wait until the next day to take the next dose at your usual time.

Reusable Oral Syringes

- Your pharmacist will provide you with the reusable oral syringe(s) that are needed for taking your medicine and explain how to use them. Wash the syringes per instructions after use. Do not throw them away.
- Use the reusable oral syringe(s) provided by your pharmacist (you should receive 1 or 2 identical oral syringes depending on your prescribed daily dose) to measure your or your child's dose of EVRYSDI, as they are designed to protect the medicine from light. Contact your healthcare provider or pharmacist if your oral syringe(s) are lost or damaged.
- When transferred from the bottle to the oral syringe, take EVRYSDI right away. Do not store the EVRYSDI solution in the syringe. If EVRYSDI is not taken within 5 minutes of when it is drawn up, EVRYSDI should be thrown away from the reusable oral syringe, and a new dose should be prepared.

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

- **For later-onset SMA:**
 - fever
 - diarrhea
 - rash
- **For infantile-onset SMA:**
 - fever
 - runny nose, sneezing, and sore throat
 - constipation (upper respiratory infection)
 - diarrhea
 - lung infection (lower respiratory infection)
 - vomiting
 - rash
 - cough

These are not all of the possible side effects of EVRYSDI. For more information, ask your healthcare provider or pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store EVRYSDI?

- Store EVRYSDI in the refrigerator between 36°F to 46°F (2°C to 8°C). Do not freeze.
 - If necessary, EVRYSDI can be kept at room temperature up to 104°F (up to 40°C) for a combined total of 5 days. EVRYSDI can be removed from, and returned to, a refrigerator. The total combined time out of refrigeration should not be more than 5 days.
- Keep EVRYSDI in an upright position in the original amber bottle to protect from light.
- Throw away (discard) any unused portion of EVRYSDI 64 days after it is mixed by the pharmacist (constitution) or if EVRYSDI has been kept at room temperature (below 104°F [40°C]) for more than a total combined time of 5 days. Discard EVRYSDI if it has been kept above 104°F (40°C). Please see the Discard After date written on the bottle label. (See the **Instructions for Use** that comes with EVRYSDI).

Keep EVRYSDI, all medicines and syringes out of the reach of children.

General information about the safe and effective use of EVRYSDI.

Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use EVRYSDI for a condition for which it was not prescribed. Do not give EVRYSDI to other people, even if they have the same symptoms you have. It may harm them. You can ask your pharmacist or healthcare provider for information about EVRYSDI that is written for health professionals.

What are the ingredients in EVRYSDI?

Active ingredient: risdiplam

Inactive ingredients: ascorbic acid, disodium edetate dihydrate, isomalt, mannitol, polyethylene glycol 6000, sodium benzoate, strawberry flavor, sucralose, and tartaric acid.

Genentech

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EVRYSDI® (risdiplam)

Distributed by:

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 A Member of the Roche Group
 1 DNA Way
 South San Francisco, CA
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For more information, go to www.EVRYSDI.com or call 1-833-387-9734.

This Patient Information has been approved by the U.S. Food and Drug Administration.

Approved: 10/2022



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Celebrate your memorable moments

Growing up with spinal muscular atrophy (SMA) looks different for every family. Your memorable moments, big and small, are worth celebrating!

Join our team of Family Ambassadors at the ZOLGENSMA booth for support, resources, and some fun, to help you find more reasons to smile every day.



Matteo, treated with ZOLGENSMA at ~1 month old, pictured at 4½ years old.

Results vary among children based on several factors, including how far SMA progressed prior to receiving treatment.

Come visit the caregiver corner at the ZOLGENSMA booth—we can't wait to see you!

Indication and Important Safety Information

What is ZOLGENSMA?

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). ZOLGENSMA is given as a one-time infusion into a vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

What is the most important information I should know about ZOLGENSMA?

- ZOLGENSMA can increase liver enzyme levels and cause acute serious liver injury or acute liver failure which could result in death.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, if the patient misses a dose of corticosteroid or vomits it up, or if the patient experiences a decrease in alertness.

What should I watch for before and after infusion with ZOLGENSMA?

- Infections before or after ZOLGENSMA infusion can lead to more serious complications. Caregivers and close contacts with the patient should follow infection prevention procedures. Contact the patient's doctor immediately if the patient experiences any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Decreased platelet counts could occur following infusion with ZOLGENSMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.

- Thrombotic microangiopathy (TMA) has been reported to generally occur within the first two weeks after ZOLGENSMA infusion. Seek immediate medical attention if the patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.

What do I need to know about vaccinations and ZOLGENSMA?

- Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed to accommodate treatment with a corticosteroid.
- Protection against influenza and respiratory syncytial virus (RSV) is recommended and vaccination status should be up-to-date prior to ZOLGENSMA administration. Please consult the patient's doctor.

Do I need to take precautions with the patient's bodily waste?

Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with patient body waste for one month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

What are the possible or likely side effects of ZOLGENSMA?

The most common side effects that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

The safety information provided here is not comprehensive. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.

You are encouraged to report suspected side effects by contacting the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch, or Novartis Gene Therapies, Inc. at 833-828-3947.

Please see the Brief Summary of the Full Prescribing Information on the next page.



IMPORTANT FACTS ABOUT ZOLGENSMA® (onasemnogene abeparvovec-xioi)

USE

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA).

- ZOLGENSMA is given as a one-time infusion into a vein.
- ZOLGENSMA was not evaluated in patients with advanced SMA.

WARNINGS

Acute Serious Liver Injury, Acute Liver Failure or Elevated Aminotransferases

- ZOLGENSMA can increase liver enzyme levels and cause acute serious liver injury or acute liver failure which could result in death.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, if the patient misses a dose of corticosteroid or vomits it up, or if the patient experiences a decrease in alertness.

Infections before or after ZOLGENSMA infusion can lead to more serious complications. Contact the patient's doctor immediately if the patient experiences any signs of a possible infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.

Decreased platelet counts could occur following infusion with ZOLGENSMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.

Thrombotic microangiopathy (TMA) has been reported to generally occur within the first two weeks after ZOLGENSMA infusion. Seek immediate medical attention if the patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.

OTHER IMPORTANT INFORMATION

Patients should be tested for the presence of anti-AAV9 antibodies prior to infusion with ZOLGENSMA.

The patient's doctor may need to adjust the vaccination schedule to accommodate corticosteroid treatment. Protection against influenza and respiratory syncytial virus (RSV) is recommended and vaccination status should be up-to-date prior to ZOLGENSMA administration. Consult the patient's doctor.

Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with patient body waste for one month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out into regular trash.

COMMON SIDE EFFECTS

The most common side effects that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

These are not all the possible side effects. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.

QUESTIONS?

To learn more, talk to the patient's doctor and you can visit www.zolgensma.com for Full Product Information.

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Novartis Gene Therapies, Inc.
Bannockburn, IL 60015

03/2023 US-ZOL-20-0121 V4

We are proud to support Cure SMA and the 2023 Annual SMA Conference.

At Scholar Rock, we aim to break scientific barriers to find meaningful therapies for those living with serious conditions, including SMA, where critical unmet needs exist.



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

The Cure SMA Board and Committee Members – This group is extremely dedicated and represent everyone affected by SMA. They are committed to maintaining the focus of our organization, while also looking for new ways that we can support individuals with SMA and families, and raise funds for SMA research and care. We thank these committed volunteers for all their ideas and efforts that go above and beyond to help the entire community.

Nick Farrell – Nick is an incredibly involved and passionate individual who puts his all into everything he does as Chair on the Board for Cure SMA. He is always looking for ways to improve, promote, and assist in any way possible within the SMA community. We are fortunate to have such a great leader on our side, and we thank him for his continued support.

The Cure SMA Chapter Leaders – Our organization is so grateful to be represented throughout the U.S., by each and every one of these dedicated and supportive individuals. Their representation of community radiates throughout the states, as they offer their time, a listening ear and their enthusiastic efforts to contribute to finding a cure for SMA. Without each of you, our organization truly would not achieve the success it has. Thank you for everything!

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 provided to Cure SMA over the years. It is because of your generosity that Cure SMA and the Annual Conference are where we are at today.

The Miller McNeil Woodruff Foundation and Meredith and Patrick Woodruff – Thank you Meredith, Patrick and this foundation for their continued years of generosity which has provided scholarships for families from Arkansas with the opportunity to attend and meet other families at the Annual SMA Conference. The Miller McNeil Woodruff Foundation began in memory of Meredith and Patrick's son, Miller who was diagnosed with SMA type I.

The Dhont Family Foundation – We are so honored to be able to provide any adult with SMA, a sponsorship that includes an aide to attend the conference this year, all due to the Dhont Family Foundation's support and funding. Thank you for providing this opportunity to so many in the community, and for sponsoring the Adults with SMA Reception!

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

Jennifer Miller-Smith, Aaron Smith, Richard Curran and Expo – We truly appreciate everything this family does for the conference year after year. Their willingness to help in any way is something Cure SMA cherishes and appreciates. Thank you for making this conference such a special event with your amazing signage, support and kindness.

Luke 18:1 Foundation – We are thrilled to be able to offer a whole new program for teens at the Annual SMA Conference this year. Thank you to the Luke 18:1 Foundation for sponsoring the teen lounge and teen social so that teens have a special place to connect and mingle and form lasting friendships.

The Cure SMA Adult Advisory Council – Thank you to the adults with SMA from across the country who serve on our Adult Advisory Council. Their participation, feedback, and experiences help to inform and influence the direction of Cure SMA programs and initiatives, including this very Cure SMA Annual Conference.



Natasha Abruzzo, FNP, RN, is a mother of four including two SMA angel babies. She is a Certified Nurse Practitioner in Albuquerque, NM. She has been a part of the HUGS committee for Presbyterian for many years which offers support to mothers who have infant loss. She is also the lead APC for her group. She is the VP for the Albuquerque A&M Club. She has a Bachelor of Science from Texas A&M University and Brookline College and a Master of Nursing from University of St. Francis. Natasha is involved in the Cure SMA New Mexico Chapter and has been involved in the National Cure SMA Conference for many years.



Brianna Albers (she/her), is a crip cyborg storyteller living in Minneapolis-St. Paul. In 2016, she founded *Monsterring*, a magazine for disabled women and nonbinary people. She consults as a patient ambassador for SMA My Way and writes the column *The Wolf Finally Frees Itself* for SMA News Today. She is currently revising *THE SAINT AND THE SPIDER*, an adult space fantasy with #OwnVoices disability representation. Find her on social media @briehalbers.



Darlisha Barnes, is the mother of two powerful girls, one who is not impacted by SMA. She is also a wife, entrepreneur, and executive coach for women leaders.



Carolyn Barrett, a Boston-based strategic communications and public relations professional whose portfolio includes major players in healthcare, higher education, and the legal field. A graduate of both Boston College (B.A. in Political Science) and Boston University (M.S. in Public Relations), she now lives on the other side of the Charles in Cambridge with her Service Dog Shadow. Carolyn loves to ski, spend time with friends, drive her adapted vehicle, and color code her PCA schedule (sometimes she has more than 15 PCAs). 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 was diagnosed with SMA type 2 at fifteen months old.



Paula Barrett, has over 25 years of human resources and leadership development experience and has worked with a variety of organizations, providing comprehensive compensation consulting, leadership development, and strategic planning consultation in the banking, healthcare, tech, and non-profit spaces. She currently manages the compensation practice at The Survey Group in Beverly, MA and previously served as Chair of the Cure SMA Board of Directors from 2008-2013 and secretary of the New England Chapter for 7 years. Paula has a Bachelor's degree in Business from Merrimack College, an M.B.A from Northeastern University, and is also certified in Everything DiSC. Paula lives outside of Boston with her husband Mike. Their daughter, Carolyn has SMA type 2.



Cathy Barsotti-Dooling, is mother to ten-year-old Annie who was diagnosed with SMA in 2016. Cathy works with her family's juice company, and also acts as PTO president at Annie's Elementary school in California. She launched the first Ability Awareness Week program at the school when Annie was in first grade. The program has been hugely successful, and she is excited to share it with you at the Cure SMA Conference



Vanessa Battista, RN, MS, CPNP-PC, a board-certified Pediatric Nurse Practitioner (PNP) and currently serves as Senior Nursing Director of Palliative Care at the Dana Farber Cancer Institute. Previously, she practiced as a PNP on the Pediatric Advanced Care Team (PACT) at the Children's Hospital of Philadelphia (CHOP), as well as the neuromuscular centers at CHOP, The Columbia University Medical Center and The Children's Hospital of Boston. Dr. Battista obtained her Master of Business Administration and Doctor of Nursing Practice from Johns Hopkins University. Her doctoral work focused on advance directives for adolescents and young adults living with neuromuscular disease.



Sarah Bellish, is the Manager of Policy and Advocacy at Cure SMA. Sarah caught the travel bug in college when studying abroad in Queensland, Australia and quickly saw a need to improve air travel for people with disabilities. In her role at Cure SMA, Sarah works to amplify the voices of the SMA community to help move the needle on issues such as housing, education, transportation, and more specifically, air travel!



Brittany Bingman, is a Licensed Master Social Worker, Wife, Mother, and Caregiver to her husband with SMA. Brittany advocates for mental health and enjoys working as a medical social worker. She is grateful for another opportunity to participate in the Cure SMA conference and share her story to help and motivate others.



Steve Bingman, is an Administrative Officer for the Department of Veterans Affairs utilizing his talents to improve the Veteran experience through communication, process improvement, and advocating for desired outcomes. Stephen is a devoted husband and proud Dad. He looks forward to sharing his experiences with SMA to help others at his second Cure SMA conference.



Amber Bosselman, a certified Life Coach who specializes in coaching people with disabilities. She brings a strong clinical background with a degree in Psychology and blends that perfectly with her practical and everyday tools she offers in life coaching. Amber has SMA type 2 and strives to be an example of what is possible as a woman of faith and business owner.

SPEAKER BIOS



Ed Bronsdon, is passionate about helping people. After 37 years of working in nonprofits benefiting people with disabilities, he recently retired from full time work. Ed is an avid cyclist, skier, and instructor. He holds Level 3 Alpine and Level 1 Adaptive certifications with Professional Ski Instructors of America. Ed volunteers with a number of community, national and international charities.



Anne Buckley-Reen, 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 research program Get Ready to Learn: Therapeutic Yoga and Mindfulness in the Classroom is in used in many school districts across the US and in Europe. She is delighted to be participating again in the Cure SMA Conference.



Shane Burcaw, is the author of several award-winning books about disability. He is the President of a nonprofit organization called Laughing At My Nightmare, and a renowned public speaker who has performed across the country from universities to elementary schools to Fortune 500 companies. He is one half of the popular YouTube channel, Squirmy and Grubs, which has gained over one million subscribers and worldwide media attention, where he and his wife document their interabled relationship.



Brady Chan, is a rising 7th grader based in Raleigh, NC. During his first year in middle school, Brady joined several after school clubs, including Project Lit reading club, Student Council, and tabletop games. He also supported the track team and was an honor roll student. Brady enjoys reading, gaming, and eating yummy food. In between relaxing, he hopes to spend this summer learning conversational French for his upcoming trip to Europe and improve on his drawing skills and chess knowledge. He's excited to meet new friends at the Cure SMA conference.



Mimi Chan, is a Chapter Representative for North and South Carolina. She is mom to Brady (SMA Type 2, 12 years old) and Lucas (non-affected, 9 years old), and has supported Cure SMA through fundraising events for over a decade. Having watched her son participate in the Spinraza clinical trial for 7 years, her personal and professional worlds collided in 2021, as she joined the clinical research industry as a marketing manager for PPD, part of Thermo Fisher Scientific. Through this role, she hopes to support advancements in medicine for other families.



Kevan Chandler, is a writer and speaks worldwide about his unique life with a disability. In 2016, he and his friends took a trip across Europe, leaving his wheelchair at home, and his friends carried him for three weeks in a backpack. Kevan is also founder of the nonprofit We Carry Kevan. He and his wife Katie live in Fort Wayne, Indiana, where they enjoy growing vegetables, making homemade bread, and reading to each other.



Maylan Chavez, is a 28-year-old Cuban born, Miami raised woman with SMA type 2. An honors student her whole educational career, she set out to major in psychology at Florida International University and soon landed in advocacy work afterwards. She is the Eyegaze Ambassador, a Cure SMA South Florida Chapter Leader, Community Editor at Rare Disease Advisor, Founder of nonprofit organization Front Row Accessible, Model for Bold Beauty Project and Raw Beauty Project, 2016 Ms. Wheelchair Southeast, and lover of life. Her goal is to advocate for inclusion in every facet so everyone can enjoy all the same experiences.



Jenna Coburn, a 22-year-old living with SMA type 2 who works as a Social Media Manager for custom wheelchair provider Access Medical Rehab. She's excited to share how she uses assistive technology in her daily life and workplace. As well as give insight to the different devices/technology available to wheelchair users.



Shonna Counter, is a Renaissance Woman: entrepreneur, author, speaker, and disability advocate. Most people are uncomfortable talking about sex – and about disability – and especially sex and disability. Through frank conversations she's changing misconceptions to help strengthen and empower your personal relationships. She was diagnosed with SMA(3) at age six. In 2017, she progressed to using a power chair, but still walks at home. From Shonna: Part time wheelchair user - full time disability advocate. Author (*Men Are Like Ice Cream*), consultant, life adventurer, speaker, and professional legal ADA advocate. She has 3 degrees (one in marriage/sex therapy). Always heard: You'll never be able to... and You're so confident and courageous. I'm confident, that if you're courageous enough to attend - you'll enjoy her session and learn/laugh so much.



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



Tara Davenport, a former civil engineer turned social work student and mother to four children, three with disabilities. She began her meditation journey over ten years ago when she realized she needed a better way to manage her stress. She brings first-hand knowledge as a parent in the trenches with children who don't function in the world in a typical way and is always excited to share about her mindfulness practice.



Megan DeJarnett, author, speaker, disability DEI educator, and inclusion advocate. Megan lives with SMA type two and is wife to her high school sweetheart, Jake and mother to her two sons, Bronx and Shai. Megan is the organizational founder of No Such Thing, and children's book author of No Such Thing as Normal and Lovely as Can Be series.



Tyler Dukes, a DevOps Manager, certified AWS Solutions Architect, and conference speaker. Professionally, Tyler leads a diverse, high-performing international team and is a conference speaker championing not only automation but diversity in the corporate landscape. Personally, Tyler, who has SMA Type 2, uses smart-home technology to live independently while spending free time with family and friends or a good show. He lives in Dallas, TX, and is also a graduate of Baylor University.



Sally Dunaway Young, DPT, a Research Physical Therapist at Stanford University, Department of Neurology and Clinical Neurosciences, in the Neuromuscular Division. She has 15 years of clinical care and research experience with children and adults diagnosed with Spinal Muscular Atrophy and other neuromuscular diseases. Throughout her career she has provided education, training, and advancements in clinical care and clinical research, focusing on outcome measures, research related to motor function, and exercise.



Bakri Elsheikh, MBBS, FRCP, a Professor of Neurology at Ohio State University. He is board-certified in Neurology, Neuromuscular Medicine, and Clinical Neurophysiology. He is the medical director of the EMG Laboratory, the Neuromuscular Medicine, and Clinical Neurophysiology Fellowships. His research focus is on the outcome measures and treatment of adults with SMA.



Jose Flores, a Corporate Speaker, Global Motivator, Mindset Disruptor, and #1 Best-Selling Author. Jose speaks all over the world using his unique story of growing up with SMA and how you can use the power of your mind to overcome anything life throws at you. His main message is to never allow your struggle to become your standard and how to dominate your life and business.



Danielle Forrest OTR, OTD, is an occupational therapist serving in the multidisciplinary neuromuscular clinic at Children's Health for the past 10 years, which has led to her passion for working with patients with SMA. She has been involved in clinical trials and is passionate about educating other therapists about this disease. She is thrilled to be speaking at Cure SMA this year.



Albert Freedman, PhD, a practicing psychologist in independent practice. Al provides counseling support for families affected by complex medical needs and serves as a consultant to rare disease advocacy organizations and biopharmaceutical companies. He is a member of Cure SMA's Medical Advisory Council. Al's son, Jack, lived with SMA for 26 years, and his daughter, Cara is currently employed at the National Organization of Rare Disorders (NORD).



Melissa Gibbons, MS, CGC, an assistant professor at University of Colorado School of Medicine and a certified genetic counselor at Children's Hospital in Colorado. She has been the genetic counselor in the Neuromuscular Clinic for 15 years and the Spinal Muscular Atrophy Newborn Screen Coordinator for Colorado and Wyoming since 2020.



Wendy Godfrey, a Resilience Strategist and the founder of ST Wellness in St. Petersburg, FL. She is a Certified Trauma Informed Social Emotional Learning (SEL) Facilitator and yoga instructor. Her passion is to help others develop resilience and self-care through self-awareness and simple, accessible practices in order to fill their own vessels to better provide for others.



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.



Carli Hamilton, has SMA Type 3. She was diagnosed at age 2 in 1994 through a muscle biopsy. She is married to Jared. Together Jared and Carli work to spread awareness for Spinal Muscular Atrophy online. Carli also works as a People Operations Specialist but her most important role is Mama. In 2019, Carli and Jared welcomed their little girl, B, into the world. The first time the Hamilton's attended a Cure SMA conference, Carli was pregnant with B. Carli loves to spend time outside with her family, read books, embroidery, Disneyland and lives off Starbucks and Dr. Pepper.



Marnie Harris, is the Director of Strategy for Shepherd Hotels, a boutique hotel franchise focused on employing adults with intellectual disabilities. She and her husband Trav are parents of two boys, 3 months and 2 years old, who both have SMA and were diagnosed through the GA newborn screen. They live in Atlanta, GA and enjoy spending time outside, being anywhere near water, and traveling.



Michael Hazel, is a 24-year old NJ/NY native who is currently majoring in Film and Television at Montclair State University. He was diagnosed with SMA Type-II at 18-months old. Michael's number one life goal is to leave the world a better place than it was before I came into it. He plans to do that through his person, artwork, and advocacy.



Jennifer Hubbell, OTR/L, a Level II therapist in the OT/PT department of Cincinnati Children's Hospital Medical Center. She has been a pediatric therapist for 29 years and has experience in both outpatient and home health care. She has been working with families and children with SMA for 21 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.

SPEAKER BIOS



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 13 years.



Carolyn Kelly, DPT, PCS., is a physical therapist at Children's Hospital Colorado. Her specialty is pediatric neuromuscular disorders. She works in the neuromuscular clinics at Children's Hospital Colorado and is a clinical evaluator for neuromuscular trials, including therapy trials for SMA and DMD. She is part of the Children's Hospital Colorado SMA newborn screening program and foot management teams. Her professional interest is early identification of children with neuromuscular conditions, early mobility and getting children and their families to participate in community programming and adaptive recreation.



Jessica Keogh, a 34-year-old woman who is a fiercely passionate special education teacher and the founder and president of Faith Above My Ability (501.C.3) As an emotional support teacher of eleven years, Jessica has a strong passion for educating, advocating for, and empowering youth with disabilities. Jessica is currently pursuing her doctorate in educational leadership and policy where her dissertation focuses on elevating voices of transition aged students with physical disabilities. Jessica is thrilled to be participating and presenting in this year's Cure SMA Conference!



Richard M. Kravitz, MD, MD, Pediatric Pulmonology and Sleep Medicine Physician. Division Chief, Pediatric Pulmonary and Sleep Medicine at the University of Virginia.



Oren Kupfer, MD, is a Pediatric Pulmonologist and Inpatient Medical Director at the Children's Hospital Colorado Breathing Institute and Associate 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.



Mateo Landa, (14) is a soon to be 8th grader at Cary Academy in Cary, NC. His favorite subjects include science, history, and math. In his free time he enjoys playing video games, training his dogs Maya and Tito, hanging out with his friends, and trying new foods!



Rio Landa, is the proud mother of Mateo (SMA Type 2) and Luci (unaffected). I'm a Cardiology Nurse Practitioner who loves to exercise and get outdoors. As a family, we love to travel, eat, and watch movies.



Cory Lee, has traveled across all seven continents and more than 40 countries as a powered wheelchair user. In 2013, he launched his travel blog, CurbFreeWithCoryLee.com, where he shares his accessible — and sometimes not so accessible — travel adventures with others. He is a four-time Webby Award winner, has won two Lowell Thomas Awards for Best Travel Blog, and he has written for publications such as Condé Nast Traveler, National Geographic, and Lonely Planet. His goal is to show other wheelchair users how to roll out of their comfort zones and see all of the beauty our world has to offer.



Jennifer Lemisch, MA, ATR-BC, LPC, is a board-certified art therapist, licensed professional counselor working at the Children's Hospital of Philadelphia since 2001. She is a member of the Pediatric Advanced Care Team, the palliative care service at the hospital, working with patients and siblings at home, providing support during hospice and end of life, and grief and bereavement counseling for siblings.



Tina Lewis, joined the Cure SMA Community in 2007 after her son, Julian, was diagnosed with SMA at the age of 3. Her firsthand experiences as a parent of a child with SMA, and as an experienced educator have made a direct impact on her ability to ensure success for her son and for other children/young adults with SMA. It is her passion to ensure that all children are provided an environment that maximizes their potential for learning.



Jennifer Martyn, PT, studied physical therapy at the University of Washington, graduating in 1995. In 2001 she started Wave Therapies where she works with both adults and children in a warm water environment with a focus on improving strength, range of motion and functional skills. Cure SMA Annual Conference is a highlight of every summer, and she is thrilled to be back with the SMA community for this year's event!



Melissa McIntyre is a physical therapist and clinical evaluator with the Utah Program for Inherited Neuromuscular Disorders (UPIN) at the University of Utah. Through UPIN, her work focuses on research and clinical care of neuromuscular disorders affecting both adult and pediatric populations. In addition to her work on multiple clinical trials. Melissa aims to minimize barriers patients face when engaging in independent recreation and physical activity regardless of their physical limitations. Melissa received her Doctor of Physical Therapy (DPT) from the University of Utah and is currently pursuing a PhD in Rehabilitation Sciences at the University of Utah.



Annah Mobus, is a MIT/HIM Specialist working as a Medical Coder. Annah is a strong type I or a weak type II. She is a mother of a delightful three-year-old boy who is a non-affected carrier of SMA. She overcomes challenges daily and wants to spread what she learns with others. She is excited to be participating in the Cure SMA Conference to spread knowledge about pregnancy and parenting, her number one learning experience so far in life.



Diane Murrell, LCSW, Social Worker in the neuromuscular clinic at Texas Children's Hospital in Houston, TX.



Leslie Nelson, 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.



Katlyn O'Brien has been an elementary school teacher for over ten years. She just completed a Master of Arts in Educational Leadership, and has aspirations to go into school administration. She has successfully supervised and coordinated the Sibling Workshop during numerous Cure SMA conferences. 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.



Traci O'Brien, is a dual-master's degree graduate, co-founded their Women's Ultimate Frisbee club at SIU, and studied International Business Marketing at GEM in France. She worked in the entertainment industry (music and comedy) in California these last few years, but just moved to the mountain region with plans to help animal welfare causes and the healing arts. A special part of her heart is reserved for these conferences as Cure SMA is fam. Her sister, diagnosed with SMA is Shannon O'Brien and they enjoy: playing Nintendo, listening to music, and pizza/movie nights!



Kendra Paker PT, DPT, PCS, graduated in 2002 from the University of Puget Sound Physical Therapy program. Kendra has worked in pediatrics for Mary Bridge Children's Hospital 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 a community-based adaptive swim program for children with special needs and their parents at the local YMCA.



Jennifer Perkins, has SMA type III with 3 copies of SMN2. She is married with two children, ages 5 and 1. She works from home as an Inventory Analyst for Target Headquarters. She has attended several Cure SMA Conferences over the years and is excited to be a speaker this year!



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 the disease, Audra struggled to cope, feeling excluded by a society that doesn't talk about grief. She has gathered coping tools over the years that she shares with other bereaved parents. Audra lives in Land O' Lakes, Florida, with her husband and their two surviving children, Lucy and Will.



Collin Pollock, has SMA Type 3 and has been married to Kyla for 17 years. Together, they own WheelyQ Barbecue where they manufacture and sell BBQ sauce and seasonings and donate a portion of profits to research into finding a cure for Spinal Muscular Atrophy. Collin attended his first Cure SMA conference way back in 1995 and made many lifelong friends that year! Collin's greatest joy is being Dad to Elyana.



Kyla Pollock, has been married for 17 years to Collin, who has SMA Type 3. In addition to being her husband's full-time caregiver, she is Mom to Elyana (14) and works as Vice President of Operations for a nationwide school staffing firm. Together, Kyla and Collin own WheelyQ Barbecue where they manufacture and sell BBQ sauce and seasonings and donate a portion of profits to research into finding a cure for Spinal Muscular Atrophy.



LaMondré Pough is a dynamic speaker, mentor, and DEIB expert. As CEO of Billion Strong, he leads an identity movement empowering people with disabilities. As an entrepreneur, LaMondré inspires audiences to embrace diversity and cultivate inclusive spaces. He also hosts the new podcast My Big Full Authentic Life and serves as Chairman of Arts Access South Carolina.

SPEAKER BIOS



Renee Roy Hill, MS, CCC-SLP, COM® CLC, has provided therapeutic assessments and program planning for adults and children with oral placement, feeding and motor speech deficits for over 25 years. She is the owner of Crossroads Therapy Clinic in New Braunfels, TX and a national and international instructor for TalkTools®. Renee has been an invited speaker for ASHA state conventions and has received specialized training in speech/oral-motor/feeding therapy to include, Apraxia, sensory processing disorders, NDT training, PROMPT, Certified Orofacial myologist and Certified Lactation Consultant. She is the creator of the TalkTools® Apraxia Program and soon to be published Flippin Funetics.



Richard M. Rubenstein, Esq., RICP, is 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. He concentrates his legal practice in estate planning, at Strategies for Wealth, Richard works with his clients to coordinate and integrate all aspects of their personal and business finances. Richard is Past Chair and Board Member Emeritus, National Board of Directors, Cure SMA. He makes this presentation in loving memory of Max, my special boy, April 24, 2005 – February 8, 2009.



Kevin Schaefer, is a writer, podcaster, and lover of all things pop culture. Diagnosed with SMA Type 2 at the age of 18 months, he shares a vast array of hilarious and eye-opening stories from his life with a neuromuscular disability in his column for SMA News Today. In addition to his columns, he works as the Associate Director of Community Content for this site's parent company, BioNews Inc. He also writes for the disability satire website, The Squeaky Wheel. Kevin is a graduate of North Carolina State University and lives with his parents in Cary, NC. People regularly mistake him for Tony Stark, on account of his intellect and advanced technological equipment.



Peter Schochet, MD, Pediatric Pulmonologist at Children's Health Pulmonary Specialists Dallas, Texas.



David Sereni, MPT, is a Physical Therapist and Staff Representative for UNACUHCP (United Nurses Association of California Union of Healthcare Professionals). He lives in Santa Rosa, California. He is also Adjunct Faculty, teaching Adapted PE at Santa Rosa Junior College. He has been the President of the Northern California Chapter of Cure SMA since 1998. He lost his son Matthew to type 1 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, Professor of Neurology and Pediatrics, University of California Los Angeles (UCLA) School of Medicine and Neuromuscular Neurologist at UCLA Medical Center in Los Angeles, CA.



Brian D. Snyder, MD, PhD, is the Maurice Mueller Professor of Orthopaedic Surgery, Harvard Medical School and Research Professor of Biomedical Engineering, Boston University School of Engineering. As a Board Certified Pediatric Orthopaedic surgeon on staff at Boston Children's Hospital, he co-directs the Cerebral Palsy Center and attends the Spinal Muscle Atrophy Clinic. His clinical practice focuses on treating congenital and acquired deformities about the hip, spine and appendicular skeleton as a consequence of neuromuscular disease and pediatric trauma.



Mark Solomon, I am a proud parent of twin 23-year-old sons, diagnosed with ASD and ADHD. I work in the field of Financial Guidance, serving the special needs community. It is my goal to provide you with impactful strategies and ideas that are simple to apply. They can change your family's financial future, and give you the hope, strength, and confidence that comes with the knowledge that you and your family can be financially secure now and into the future.



KateLynne Steinke, has been a decades long service dog user that has found great independence utilizing this amazing medical equipment after her original injury in 2010 and subsequent SCI in 2013. She travels around the country as a speaker helping potential recipients navigate the service dog process, educating the public and businesses about laws and rights, and showing the world what a service dog can do to assist their handler. Her current service dog, Jones on the Job, is an 8 year old yellow lab from NEADS World Class Service Dogs and has over 80 commands.



Harvey J. Stern, MD PhD, FACMG, FAAP, is Board-Certified in Medical Genetics and Pediatrics and has worked with SMA families for over 25 years. His special area of interest is reproductive options for SMA families and especially those who are interested in prenatal or preimplantation genetic testing for SMA.



Sarah Stoney MSW, LSW, graduated from West Chester University with a Master of Social Work in 2014, after she received her bachelor's degree in the same discipline in 2006. She joined The Children's Hospital of Philadelphia's Division of Neurology in 2014. Previously, she worked in the emergency department of Penn Medicine's Chester County Hospital and as a family counselor at The Lincoln Center for Family and Youth. At CHOP, she assists patients, and their families navigate the complex medical system, advocates with families in their schools and throughout the community and provides ongoing support to patients and families. She has a primary interest in transitioning young adults to adult medicine, and works on improving the transition process for Neurology patients.



Anne Stratton, MD, Assistant Professor, University of Colorado: Department of Physical Medicine and Rehabilitation, Children's Hospital Colorado. Dr. Stratton has been a member of the Cure SMA Medical Advisory Committee since 2015. She is one of the physical medicine and rehabilitation clinicians in the multidisciplinary Neuromuscular Clinic at the Children's Hospital Colorado, treating SMA, muscular dystrophy and other pediatric neuromuscular diseases. She has a special interest in musculoskeletal changes in children with muscle disease and in exercise tolerance and benefits in this population.



Dany Sun, is the Senior Social Work Manager at Cure SMA and an SMA parent. She is mom to Landon (SMA Type 2, 9 years old) and Ruby (SMA Type 3, 12 years old) and has been a volunteer supporter of Cure SMA since 2014, when both children were diagnosed. Dany has a master's degree in social work and worked in community mental health for 11 years before joining Cure SMA in 2019. She is passionate about advocacy, support, and the empowerment of those facing challenges in life, especially those in the SMA community. Dany lives in Southeastern WI with her children, her husband, Terence, and their two dogs Max and Coco.



Ruby Sun, is twelve years old and just finished 6th grade, her first year of middle school. In 6th grade, she has navigated time off for spinal fusion surgery and recovery, joined many different extra-curricular clubs, became a percussionist in band, and lots more. Ruby enjoys drawing, basketball (especially the Milwaukee Bucks!), and playing with fidget toys. She's excited to share all she's learned with you and to meet other middle schoolers with SMA!



Peyton Tansey, is a 22 year old college student graduating from Hofstra University with a degree in computer science. Practicing yoga since the age of 4, Peyton is delighted to share his yoga knowledge and experience with the attendees of the conference.



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 15 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 7 years, she has been an active member of the Cure SMA Medical Advisory Council.



Jane Taylor, MD, MsCR, Pediatric Pulmonologist at the UPMC Children's Hospital of Pittsburgh, associated with the University of Pittsburgh School of Medicine in Pittsburgh, PA.



Justin Titcombe, is Director of Real Estate for UFP, the largest Planet Fitness franchisee in the world. He and Angela are celebrating their 5th wedding anniversary this weekend. Justin enjoys college football, exercising and cooking.



Lorelei Thornburg, MD, is a Professor at the University of Rochester in Maternal-Fetal Medicine. She teaches nationally on both complex diseases in pregnancy, with a special focus on neurologic diseases during pregnancy.



Krista Torseth, PT, DPT, is a 2007 University of Puget Sound graduate who specializes in pediatric, aquatic physical therapy. She has extensive experience with infants, children and adolescents with a variety of diagnoses and abilities, including many with spinal muscular atrophy and Duchenne muscular dystrophy. She is excited to help you figure out how to access the aquatic environment in your own community!



Fred Troutman, RN, PhD, Nurse Educator, Professor Emeritus at Walla Walla University in Portland, OR.

SPEAKER BIOS



Tanya Vega, is a 22-year-old young adult who is experiencing all the joys of life alongside her partner, Jenna Coburn (SMA Type 2). She assists with her partner's daily life at home and in her career, but most importantly they have grown into adulthood together, by learning and exploring their relationship. She is excited to give insight and share her experiences in their relationship.



Laura Watne, MS RD CSP, is a Registered Dietitian, Board-Certified Specialist in Pediatric Nutrition and a member of the Cure SMA Medical Advisory Council. Laura specializes in providing medical nutrition therapy for infants, children, adolescents, and adults with a variety of neuromuscular conditions since 2011. Her other areas of expertise include spina bifida, spinal cord injury and Rett syndrome.



Sylvia Wheeler, is a busy grandmother of 2 (Aidyn 16 and Zoey 12). She is retired after 40+ years of working with intellectually delayed individuals and the Special Olympics with the state of Arkansas. She became involved with Cure SMA in 2012 when her granddaughter Zoey was diagnosed with type 2. Sylvia helped to start the Arkansas Cure SMA chapter in 2017 and was instrumental in lobbying the Arkansas Legislative to enact Newborn screening for SMA in 2019.



Angela Wrigglesworth, a fifth-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 SMA Type 2 and sharing John Wooden's philosophy that, Things turn out best for those who make the best of the way things turn out.



Allie Williams, is a 42-year-old vocal music teacher and disability advocate with SMA Type 2. She is an influencer on Tiktok and Instagram where she advocates for disability awareness and programs that assist disabled individuals with food and housing insecurity. She also writes articles about her life and experiences for spinalmuscularatrophy.net. Her motto is Representation Matters, and she tries to be this example every day as she shows what life is like for her on social media. @Alliewheelz

Thank you to each and every speaker who helped bring this community back together again in-person! These individuals volunteer their time to help educate the community on the latest information related to SMA. Thank you for your time and expertise for this year's 2023 Annual SMA Conference, we could not have held this event without you all.



PLEASE NOTE

WEAR SUNSCREEN AND KEEP HYDRATED!

As many of you know, June 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 the heat!

Recognize the symptoms and save a life!



EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

Motor delays or missed milestones may
signal serious conditions like SMA.

There is new hope, but it's important to act
swiftly and make SMArt Moves.



SMARTMoves.CureSMA.org

EVENT MILESTONES

Birdies for Blake



We want to send a special thank you to the Farrell Family, who in 2022 hosted not one, but two events to support Cure SMA in honor of their amazing daughter Blake!

First, was the 2022 Birdies for Blake Golf Tournament that took place on July 31, 2022 at Green Crest Golf Club in Liberty Township, OH. Golfers enjoyed a day on the links, refreshments and a great raffle while raising over \$43,000 for Cure SMA!

Then, come September 11, 2022, Kacey Farrell and her amazing committee hosted a brand new event: The Birdies for Blake LPGA Watch Party. The LPGA Tour was in Cincinnati, and the committee elected to host a LPGA Tour final round viewing party at a private residence off of the 13th hole of Kenwood Country Club. Though rain interrupted the golfer's schedules, nearly 75 people attended the event and enjoyed a fun afternoon of golf viewing, amazing food and drinks, a raffle, and a silent auction (not to mention the Bengals home opener). Through their amazing efforts, this first-time event raised \$63,000 for Cure SMA.

Thank you to Nick and Kacey Farrell, Mark and Nancy Farrell, Kate and Sam Queen, and their wonderful friends and family for spearheading these events and raising over \$100,000 for Cure SMA in 2022!

Reach4Sky



In 2022, Team Reach4Sky pledged to swim 21 miles in honor of their son Skylar's 21st birthday! Liz and Jim Bahrenburg set out on their journey with friends and family to complete 10 swims at 6 different venues across the country. A few of their stops included an open water swim in Kailua, Hawaii; a first-ever Tampa, Florida swim where they had hammerhead shark sightings; and a scenic swim under the Colorado Bridge in San Diego, CA that was followed by a small gathering of loved ones to toast Skylar's birthday. Team Reach4Sky has been hosting this swim fundraising for nearly 20 years!

This year, we want to congratulate Liz, Jim, and the rest of the Team Reach4Sky on 20 years of continued success! Thank you for all you do for the SMA community!

Links 4 Luke



Congratulations to the Luke 18:1 Foundation and the Stickane family as they held the 6th Annual Links 4 Luke tournament in April and celebrated over \$1,500,000 donated to Cure SMA from event proceeds. This annual event, held on Monday, April 24, 2023 at Timarron Country Club in Southlake, TX, benefits the Luke 18:1 Foundation, whose mission is to work together as a community to find a cure for SMA through raising awareness of SMA and early detection, funding research through fundraising events, and supporting individuals and families by connecting those impacted by SMA with meaningful programs and mentors to aid in their journey. Thanks to the generosity of the sponsors, players, and community, the Luke 18:1 Foundation has been able to partner with Cure SMA to fund critical research for new therapies, invest in vital equipment for the loan program, provide scholarships for first-time attendees at the Annual SMA Conference, and create new connections within the SMA community by launching a new technology sharing program with Cure SMA Care Center Network sites.

Congratulations to the Luke 18:1 Foundation on raising over \$1.5 million that has made a significant impact on the SMA community! Cure SMA thanks the Stickane and Giglio families for their continued support, generosity, and partnership!

Cure SMA Walk for Graham

The annual Cure SMA Walk for Graham is hosted by the Vollmer Family of Westfield in honor of their 8-year old son Graham. This special event is a way to recognize all SMA individuals and families from across the state of Indiana. The event is filled with family-friendly activities including music, food and drink, a silent auction, and a commemorative one-mile walk in Carmel, Indiana. Since 2017, Walk for Graham has raised \$618,000 for Cure SMA, and each year welcomes over 800 participants annually. Planning for this year's event is already underway and takes place on Saturday, August 5 at the Coxhall Gardens.

Congratulations to the Walk for Graham event on 5 years of continued success!



Cure SMA offers resource guides to support those with SMA and their communities. These resources cover a range of topics to enrich daily living at home and in the community. Below are some sample topics currently available, but topics covered in these guides continue to grow so stay tuned for more to come!

- Protected Savings Options Comparison Chart
- Guide to Talking with Children about Disabilities
- Educator's Guide to SMA
- Sample School Letter Guide
- Travel Guide
- Adaptive Equipment List
- Driving – Becoming Licensed
- Accessible Vehicle Options and Considerations
- Home Modifications Guide
- Bathroom Accommodations
- Bathroom Equipment List
- DEI Children's Book Recommendations
- Guide to Advertising and Interviewing PCAs
- Guide to Hiring and Maintaining PCAs
- Surgery Hospital Stay and Recovery Guide



To request a copy of any of these resources, please email communitysupport@curesma.org.

we ♥ our volunteers



OUR VOLUNTEERS ARE THE ROOT OF WHO WE ARE.

As representatives of Cure SMA, volunteers spread awareness of SMA in their local communities by supporting local events, advocating for SMA-related policies, and generating support for our organization.

We want to thank our dedicated volunteers for providing their time and efforts to further the mission of Cure SMA. Their support allows us to offer programs and services to every individual in our community, as well as continue to fund and direct comprehensive research that drives breakthroughs in treatment and care.



ARE YOU LOOKING TO VOLUNTEER WITH CURE SMA?

We are always searching for volunteers who want to make an impact in the SMA community, and there are a variety of ways to get involved:

- Become an advocate
- Join a planning committee, or volunteer on event day, at one of our 50+ Walk-n-Roll events across the country
- Engage your community and host your own event supporting Cure SMA



LEARN MORE ABOUT HOW YOU CAN GET INVOLVED!

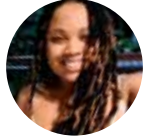


CHAPTER LEADERS

ALABAMA CHAPTER *(including Mississippi)*



Linda Russo*



Shakiira Thomas*

ARIZONA CHAPTER



Angel Wolff*



Cassandra Thompson

ARKANSAS CHAPTER



DeAnna Dillon*



Sean Dillon*



Sylvia Wheeler*

CALIFORNIA CHAPTERS

NORTHERN CHAPTER *(including Nevada)*



David Sereni, Jr*

SOUTHERN CHAPTER



Autumn Montoya*



Rickk Montoya*

CAROLINAS CHAPTER *(including North Carolina and South Carolina)*



Idalmis Vivar



Mimi Chan*

CHESAPEAKE CHAPTER *(including Maryland and West Virginia)*



Kim Heinrich



Derek Lewis*

**attending Conference*

CHESAPEAKE CHAPTER

(including Maryland and West Virginia) Continued



Beverly Venedam



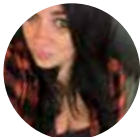
Tina Lewis*

CONNECTICUT CHAPTER

Kathryn McBride

FLORIDA CHAPTERS

GREATER AREA CHAPTER



Jennifer Smith



Katie Kerns*



Audra Butler*

SOUTHERN CHAPTER



Fiorenna Fuentes Israel*



Jennifer Miller Smith*



Maylan Chavez*

GEORGIA CHAPTER



Valerie White*



Tara Ragan*

ILLINOIS CHAPTER



Kathleen Heinrich*



Jill Zmaczynski*

**attending Conference*

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IOWA CHAPTER



Megan Ramirez*

MINNESOTA CHAPTER



Kara Forcier

Kayla Yaeger



Matthew Czech*

LOUISIANA CHAPTER

Brittany Melara

Janna Leach

NEW ENGLAND CHAPTER

(including Massachusetts, Maine, Rhode Island, Vermont, and New Hampshire)



Barbara Jean Mirabile*



Kristen Farrell



Susan O'Neill



Deodonne Bhattarai*

MICHIGAN CHAPTER



Gretchen Dorer*



Aryn Manni*

**attending Conference*

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NORTHERN CHAPTER



Alexandra Lakhman



Laura Watson*

Kristen Smith*

SOUTHERN CHAPTER *(including Delaware)*

Tara Montague

Jessica Moyer

NEW MEXICO CHAPTER



Joe Weisman*

Annie Weisman*



Natasha Abruzzo*

NEW YORK CHAPTERS

CAPITOL REGION CHAPTER



Allie Wolfe*



Amy Cunniff-Bleau

GREATER AREA CHAPTER



Erin Bonner*

WESTERN CHAPTER



Mary Boguhn

OKI CHAPTERS

(including Ohio, Kentucky, and Indiana)



Adrienne Vollmer (IN)



Holly Sontag (IN)*



Nicole Haake (OH)

**attending Conference*

CHAPTER LEADERS

OKI CHAPTERS

(including Ohio, Kentucky, and Indiana) Continued



Beth Lockwood (OH)*



Stephanie Noll (OH)



Elizabeth Lockwood (OH)*



Kevin Lockwood (OH)*



Courtney Ogletree (KY)



Kristen DeLuca (KY)*



Alex Haas (KY)*

OKLAHOMA CHAPTER



Amanda Chaffin*

PACIFIC NORTHWEST CHAPTER



Mark Courteau*

PENNSYLVANIA CHAPTER



Christina Murray



Gina Perri*



Emily Farkas*

ROCKY MOUNTAIN CHAPTER

(including Colorado, Wyoming, and Montana)



Lyza Weisman*

TENNESSEE CHAPTER



Rondi Kauffmann*



Keri Tarantino

**attending Conference*

CHAPTER LEADERS

TENNESSEE CHAPTER *Continued*



Sarah Boggess*

TEXAS CHAPTER



Kristen Resendez*



Beth Moore*



Sapna Pringle*



Kate Nelson

VIRGINIA CHAPTER *(including Washington D.C.)*



Laura Derkowski*



Kyle Derkowski*

Debra Schaefer

WISCONSIN CHAPTER



Kim Banach



Ashley Baumeister*

**CHAPTER LEADERSHIP IS A FUN AND
REWARDING WAY TO WORK WITH
THE SMA COMMUNITY.**

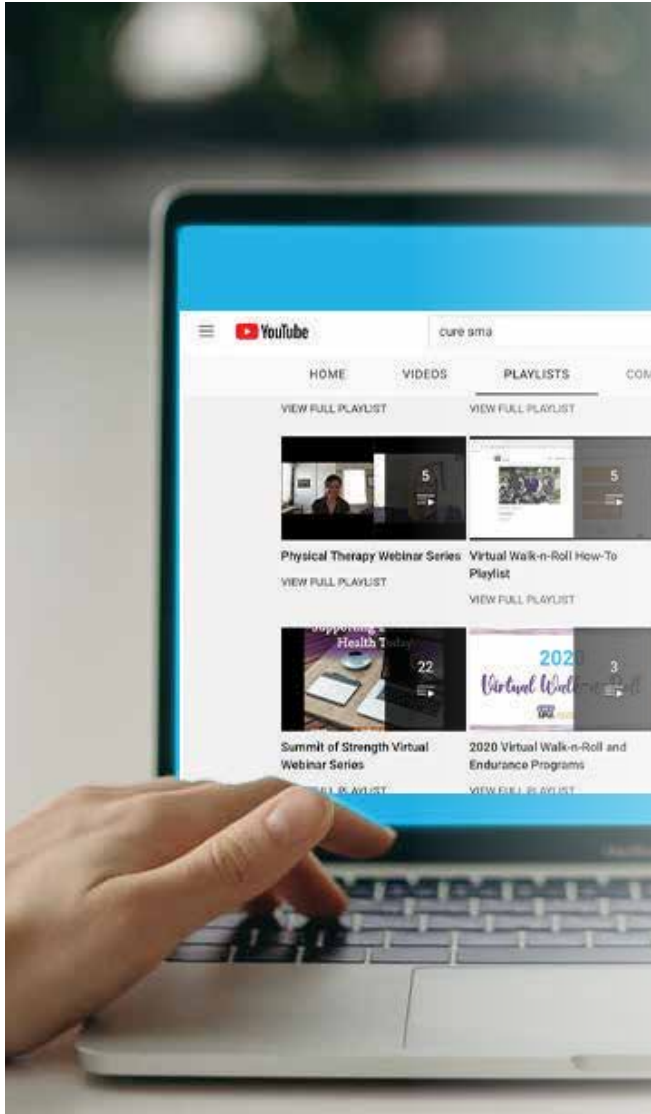
If you would like to learn more about becoming a chapter leader or looking for a fun way to connect with the SMA community, please contact Kaitly Thompson at kaitly.thompson@curesma.org

**attending Conference*



Check Out Cure SMA's YouTube Channel!

Our channel shares over 100 educational webinars that are presented by experts in the SMA community, along with insightful career and college panels led by adults with SMA, and much more!



These educational webinars cover topics such as:

- Physical therapy
- Sleep
- Mindfulness
- Nutrition
- Driving
- Pain management
- Laughter yoga
- Mental health
- Pulmonary care
- Creating a life care plan
- Role of social workers
- Yoga therapy
- Women's health
- Assistive technology
- Scoliosis management

In every playlist you will find a robust lineup of knowledgeable speakers who share valuable information and advice for people of all ages and types of SMA, as well as their caregivers. This channel is where you will find Cure SMA's most up-to-date webinars, packed with incredible information.

Take advantage of these fantastic webinars today!
www.youtube.com/c/CureSMA/playlists

Follow us on social media to stay up-to-date with news and stories!



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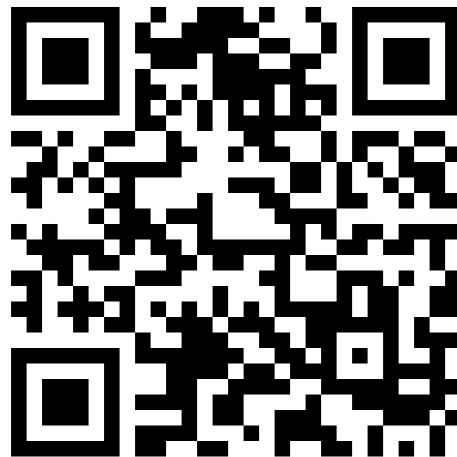
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#2023SMAConference #CureSMA and #SMACommunity

It is a priority at Cure SMA to support the adult community therefore we have created a variety of support programs. Below is information on those programs with links to apply.

SUPPORT PACKAGES AND PROGRAMS



Teen and Adult Support Package

The original package for teens and adults that is filled with helpful items that allow for increased independence and that have been recommended by other adults with SMA. Items include medical fleece ease sheepskin, Amazon Echo, telescopic back scratcher, long reacher grabber tool, gooseneck phone or tablet mount, remote control outlet switch kit, EZ-shampoo hair washing basin, neck and shoulder heating pad, and a fluidized positioner pillow.

www.curesma.org/SupportPackage



Teen and Adult Independence Assistance Package

A supplemental package for teens and adults with SMA that is filled with a new set of helpful items to help gain further independence with activities of daily living. Items include Logitech Blue Snowball Microphone, WiFi smart plugs, travel UV sanitizing wand, universal cup holder, telescopic metal straws, jar opener with base pad, and a multi kitchen tools set.

www.curesma.org/IndependencePackage



LifeVac Support Program

Through this program, a LifeVac Home Kit is provided to any individual with SMA who requests one, at no cost to this person. This is a non-powered, non-invasive, single-use only airway clearance device developed for resuscitating a victim with an airway obstruction. It is easy to use in an obstructed airway emergency and is beneficial for those who the Heimlich maneuver is not possible.

www.curesma.org/LifeVac



Medical Alert Bracelet

The Responder PHR with Medical Alert Bracelet and Keychain is offered to help identify medical needs in case of a medical emergency. Through this program, a Responder PHR (Personal Health Record) package is provided so medical personnel will have immediate access to the patient's medical records stored online, whether the patient is able to communicate for themselves or not.

www.curesma.org/ResponderPHR

SUPPORT PACKAGES AND PROGRAMS



Annual SMA Conference Sponsorship

The Adults with SMA Sponsorship offers adults with SMA ages 18 and older, as well as one caregiver, waived registration fees, a stipend for travel, and one hotel room for 3 nights for the Annual SMA Conference.

Email conference@curesma.org for sponsorship application!



Annual SMA Conference Events

Cure SMA holds workshops specifically geared toward adults with SMA, along with exclusive lounges and receptions throughout the 4-day conference.

www.annualsmaconference.com/agenda.html



Adults with SMA In-Person Socials

This program, which includes refreshments, snacks, and free parking for attendees, provides a space for adults with SMA to gather and socialize in person.

Email communitysupport@curesma.org for more details!



Adults with SMA Virtual Socials

Virtual socials offer an opportunity for adults with SMA to gather online in a friendly Zoom setting to catch up with friends and network.

Email communitysupport@curesma.org for more details!



Educational Webinars and Panels

Find informational webinars, including panels about going to college and pursuing a career, on Cure SMA's YouTube Channel.

www.youtube.com/c/CureSMA/playlists



Learn more about these programs at www.CureSMA.org and contact communitysupport@curesma.org with any questions!



Individually driven. Community inspired.

Hear from fellow life travelers living with SMA, including authors, entrepreneurs, students, partners and more, and see how they're approaching life goals and celebrating individuality.

Visit our website & follow us on Instagram to explore these stories and more

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[@smamyway_us](https://www.instagram.com/smamyway_us)



"Attitude of gratitude"
AMBER-JOI
PARENT OF DAUGHTER WITH SMA



"You can't please everyone!"
JAMES
LIVING WITH SMA



"Brighten someone else's day"
SHANE & HANNAH
SHANE IS LIVING WITH SMA

JOIN US FOR DOUBLE TAKE

A first-of-its-kind fashion show with start-to-finish SMA community involvement, that aims to increase disability visibility, break down stereotypes and champion adaptive fashion.

Thursday, June 29 | 8:30pm
Asbury Hall | Annual SMA Conference

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Thank you to the support of our exhibitors who helped make the 2023 Annual SMA Conference a great success!

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Thank You To Our Inaugural Baking a Difference Bakers!

New in 2022, Cure SMA launched a new youth fundraising initiative, Baking a Difference, which encourages the next generation of entrepreneurs and philanthropists to give back while earning rewards and having plenty of fun for Cure SMA. Kids can sell treats through fundraisers at school, in clubs or sports teams, in their neighborhoods, or anywhere else they can think of prior to or during the holiday season. From a red wagon selling treats outside a local grocery store to full-fledged bake sales, Baking a Difference raised nearly \$80,000 for Cure SMA. Across the U.S., over 200 kiddos participated in the inaugural holiday-themed campaign, and we look forward to seeing what happens this year!



Thank you to all our youth bakers (and their parents) for making our inaugural year a success! A special congratulations to the Chan Family and Chan's Chomps team for raising nearly \$65,000 in the inaugural campaign! What an incredible effort! Thanks for Baking a Difference for the SMA community!

CURE SMA BOARD OF DIRECTORS

Cure SMA holds itself accountable to people with Spinal Muscular Atrophy, their families, donors, volunteers, and the public. Our Board of Directors is a diverse group of volunteers, who bring a variety of experiences and expertise to the governance of Cure SMA. The Board's responsibility is to direct and carry out the vision, mission, and goals of Cure SMA.

OFFICERS



Nick Farrell – Chairman of the Board

Nick is Senior 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 and 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 \$320,000 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 Board of Trustees of the Convalescent Hospital Fund for Children, an arm of Cincinnati Children's Hospital. Nick received his B.A. from Wake Forest University and his J.D. from Vanderbilt.



Bradley A. Nunemaker – Treasurer

Brad was diagnosed with SMA Type III when he was nine years old. He joined the Cure SMA Board of Directors in 2012 and serves as Treasurer. Brad received his BBA from the University of Michigan Business School. He is currently the Senior Vice President, Finance – Health and 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 and advocacy. In April 2017, Brad was a panelist in the Patient-Focused Drug Development meeting with the FDA and shared his experiences on the impacts of living with SMA. In addition to chairing the Finance and Operations Committee on the Board of Directors of Cure SMA, Brad also is a member of the Adults with SMA Advisory Council. Brad and his wife Krista live in Elmhurst, Illinois with their two sons. The whole family is proud to support Cure SMA.

EXECUTIVE COMMITTEE



Robert Graham, MD – Board of Directors

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.



Allyson Henkel – Board of Directors

Allyson is a Spanish teacher and has worked as a legal and medical interpreter. Her previous work experience includes 8 years at Lord, Abbett and Co., an investment management firm, and 22 years teaching Spanish. Allyson and her husband Tim live in Rosemont, Pennsylvania. They have four children, one of whom, Peter, died from SMA at age 13. Allyson has been involved with the Pennsylvania Chapter of Cure SMA for 18 years supporting families, fundraising, raising awareness and working on advocacy. Along with other members of the Pennsylvania Chapter of Cure SMA, Allyson, Tim and their children Peter and Lucy were instrumental in securing the approval to add SMA to the newborn screening panel in Pennsylvania in 2018. The Henkel Family, through Hosts for Hospitals, hosts patients who are traveling to Philadelphia for treatment. Allyson is proud to remember and honor her son Pete and all who are affected by SMA through her work with Cure SMA.

CURE SMA BOARD OF DIRECTORS



Kelly Jankowski – Board of Directors

Kelly Jankowski leads the Reputation Practice, helping organizations frame the influence they want to have on their industry, their employees and society. She oversees a national team that includes specialists in integrated storytelling, content creation, crisis communications, professional services and employee engagement. Her work helps organizations rethink traditional boundaries of corporate communications to integrate data, insights and content into their reputation-building approach. She has worked with Deloitte, Gilead JM Smucker, Amazon, PayPal, Booking Holdings and Whirlpool to reinvent the way their communications, public affairs and marketing departments collaborate. A specialist in executive visibility, Kelly has managed dozens of reputation and public affairs activations at the World Economic Forum Annual Meeting and the Milken Institute Global Conference. She is a member of the University of Oregon Strategic Communication Leadership Network. In 2020, Kelly was named one of PR Week's 40-Under-40.



Rob Lockwood – Board of Directors

Rob and his wife Ann, son Christopher and daughter Grace live in the Cincinnati area. Rob works as 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 almost 30 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.



Spencer Perlman – Board of Directors

Spencer Perlman is a Managing Partner and the Director of Healthcare Research at Veda Partners, which provides federal policy expertise to institutional investors and corporations in the public and private markets since 2017. He has spent over 25 years working in healthcare policy in the public and private sectors in Washington, D.C. Earlier in his career, Mr. Perlman was health policy advisor to a Member of the U.S. House of Representatives, counseled several patient advocacy groups, healthcare providers, and professional societies in developing and implementing advocacy campaigns, and analyzed healthcare and Medicare policies for non-profit hospitals and health systems. Mr. Perlman served as Cure SMA's representative in Washington, D.C. from 2005 – 2014. He and his wife split time between Bethesda, Maryland and Kiawah Island, South Carolina; they have two adult sons.



Shannon Zerzan – Board of Directors

Shannon Zerzan became involved with Cure SMA shortly after her eldest son was diagnosed in 2010. In 2011, she and her husband Greg co-founded Cure SMA's Hope on the Hill Congressional Dinner in Washington, D.C. Hope on the Hill increases awareness in our nation's capital, where consequential regulatory and policy decisions are made that affect the daily lives and futures of individuals living with SMA. The annual event has raised nearly 2 million dollars. Shannon has delivered testimony before federal regulators on behalf of Cure SMA and works to educate legislators on issues such as accessible air travel. Now a full-time stay-at-home parent, volunteer and advocate, Shannon spends a lot of time on sidelines and in bleachers and travels the country cheering on her son's wheelchair basketball team. Prior to having a family, she was director of operations for a Washington, D.C.-based national healthcare association. She has also worked on political campaigns and for education and legal aid non-profits. Shannon lives in Southlake, Texas, with her husband and two sons.

BOARD MEMBERS



Corey Braastad – Board of Directors

Dr. Braastad retired in 2023 as the Vice President and General Manager of Genomics at Covance Drug Discovery, part of LabCorp. Dr. Braastad 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 RandD. 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. Dr. Braastad retired in 2023 as the Vice President and General Manager of Genomics at Covance Drug Discovery, part of LabCorp.

CURE SMA BOARD OF DIRECTORS



Diana Castro, MD – Board of Directors

Dr. Castro, a board certified pediatric neuromuscular physician who is a pioneer in research and management of patients with SMA. After more than ten years, Dr. Castro left academia with the objective of creating a non-profit private practice and research institute for neuromuscular conditions. The non-profit neuromuscular practice has the capacity to offer care to all neuromuscular patients, regardless their insurance situation. The research practice offers opportunities to enroll in cutting edge trials. Dr. Castro has conducted multiple clinical research trials in SMA, Duchenne Muscular Dystrophy (DMD), Charcot-Marie-Tooth (CMT), among other conditions. Being an intricate part in the development of innovative therapies for rare neuromuscular conditions is one of her biggest passions along with the strong bonds she makes with all her patients and their families. It's this special relationship with her patients that drives her to continue to search for therapeutic options for them. In addition to managing busy patient load and conducting research, Dr Castro has had many peer reviewed publications and has given multiple national and international lectures both in English and Spanish. She also sits on several national medical advisory boards.



Bakri Elsheikh, MD – Board of Directors

Bakri is an adult Neurologist who specializes in neuromuscular medicine and clinical neurophysiology. He is a professor of neurology at the Ohio State University. Currently, He serves as the director of the OSU EMG laboratory, the director of the clinical neurophysiology and neuromuscular medicine fellowships, and the director of the OSU Muscular Dystrophy Association Care Center. His clinical practice focuses on individuals with neuromuscular disorders, particularly muscular dystrophy and Spinal Muscular Atrophy (SMA). His journey with SMA started in 2004-2005, working as an investigator with his mentor Dr. John Kissel in the project Cure SMA under the umbrella of families of SMA (Now Cure SMA). He worked with others to establish the reliability and validity of several outcome measures in multicenter studies and evaluate the response to therapeutic intervention in SMA patients of all age groups. His current research focuses on optimizing care models, outcome measures, and therapeutics for adults with Spinal Muscular Atrophy. He serves as the principal investigator on several projects on various neuromuscular diseases.



Jaclyn Greenwood – Board of Directors

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 of Science degree in genetic counseling from the University of California, Irvine in 2012. As a licensed and certified genetic counselor, she currently works for the University of California, Irvine at the Chao Family Comprehensive Cancer Center as an Assistant Professor and Genetic Counselor. 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 outreach and support to the SMA adult community.



Edmund Lee – Board of Directors

Edmund and his wife Kwiyoum have been blessed with their daughter, Angie, who has SMA type II. For 16 years, they lived in Naperville, IL, where they hosted an annual fundraiser for Cure SMA with the help of their whole community. The fundraiser, called Kyr's Idea Angie's Hope, raised more than \$260,000 over a dozen years. The Lee family, now residing in California, is committed to spreading awareness about SMA and hopes to exemplify living gracefully while impacted by the disease. Edmund works for AlixPartners, a global consulting firm and also works as a venture partner at Volta Energy Technologies, a venture capital firm focused on energy storage. He received an MBA from the Wharton School with a finance major and a B.S. in computer science from Seoul National University.



Amy Medina – Board of Directors

Amy graduated from Marian University with a Bachelor's degree in Social Work. She worked for Fond du Lac County Department of Social Services for 11 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 7, was diagnosed with SMA via amniocentesis and began treatment at 12 days old taking part in a clinical trial. Amy's third child, Amelia, age 5, was also diagnosed with SMA via amniocentesis and received treatment at 11 days old through a clinical trial. Amy's passion is educating others on SMA which includes training the local EMTs on SMA care and having newspaper articles written about the importance of newborn screening and early treatment. Amy is part of a newly formed school committee to raise funds for an all inclusive playground.



Gillian Mullins – Board of Directors

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



and their three children.

Tom Murray – Board of Directors

Thomas Murray, PhD, is President Emeritus of The Hastings Center. He was formerly the Director of the Center for Biomedical Ethics in the School of Medicine at Case Western Reserve University, where he was the Susan E. Watson Professor of Bioethics. Among other commitments, he served as the Vice Chair of Charity Navigator. He has served on many editorial boards and testified before many Congressional committees. Currently he serves on the Disciplinary Commission of World Athletics. 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. Among his books are *The Worth of a Child* (1996) and, most recently, *Good Sport: Why Our Games Matter – and How Doping Undermines Them*.



Kaleen Robinson – Board of Directors

Kaleen is the Chief People Officer at Salo, a Korn Ferry Company. She has spent 35 years in HR including leadership roles at Hewitt Associates, Aon, and ALight Solutions. She graduated from North Park University with a degree in Organizational Leadership and Management. She has always been passionate about differently abled individuals, and that passion was fueled even further after the birth of her now adult twins, one with significant special needs. Through the incredibly challenging maze of medical terminology, lack of diagnosis, surgeries, and life-threatening situations they faced with their daughter, she has become an advocate for others who are differently abled. Kaleen became acquainted with SMA through a dear friend and has since been growing in her knowledge and involvement in Cure SMA. She has served on the Board of Habitat for Humanity-Lake County, IL in the past, and is an advocate for housing for all. She lives with her wife in Third Lake, IL and they have four grown children, one daughter-in-law, and three grandchildren.



Brian Snyder, MD – Board of Directors

Brian Snyder MD/PhD is the Maurice Mueller Professor of Orthopaedic Surgery, Harvard Medical School and Research Professor of Biomedical Engineering, Boston University School of Engineering. As a Board Certified Pediatric Orthopaedic surgeon on staff at Boston Children's Hospital, he co-directs the Cerebral Palsy Center and attends the Spinal Muscle Atrophy Clinic. As a translational scientist and principal investigator at The Center for Advanced Orthopaedic Studies at Beth Israel Deaconess Medical Center, his research team focuses on basic and applied research in musculoskeletal biomechanics.



Peter Statile – Board of Directors

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

COMMITTEE MEMBERS



Jay Chung – Committee Member

Jay Chung retired as head of Human Resources for TSMC North America, where he oversaw the company's multiple business units in the North America, including Sales Operations for the Foundry business, Solar business, Design Centers and Legal and IP teams. Prior to joining TSMC, Jay held senior executive positions in general management, strategy, marketing and sales for 2 large multinational corporations. He previously served as the CEO's appointed VP of Motorola's Asian Diversity Council, a board member of Monte Jade West, a technology and entrepreneurial development non-profit and is currently the Secretary of the board at Able Works, a Bay Area non-profit dedicated to helping marginalized youths and young adults achieve economic equity and financial freedom. Jay is fluent in English, Mandarin and Fujian. He has travelled globally, lived and worked in several locations in the US and Asia. He holds a Bachelor of Engineering (Electrical) degree from the National University of Singapore and has completed an Advanced Management Program at the University of Hawaii.



Marc Ginsky – Committee Member

Marc is Chief Operating Officer and General Counsel at Medable - a clinical trial technology company whose mission is to bring effective therapies to patients faster. Prior to working at Medable, Marc spent five years as the Chief Operating Officer of the Cystic Fibrosis Foundation where he oversaw a number of functions including their field and fundraising groups. Marc began his career as an attorney focused on corporate transactions and after 10 years in private practice joined Covance, a large clinical research organization where he spent 18 years (9 years as an attorney and 9 years leading their market access division). Marc is excited to contribute to the Cure SMA mission in any way that he can. Marc lives in Bethesda, Maryland, with his wife and has 2 adult daughters.

CURE SMA BOARD OF DIRECTORS



Brianna Gross, MS, LCGS – Committee Member

Brianna is a genetic counselor at Children's Hospital of Philadelphia (CHOP) who specializes in neuromuscular disorders. Prior to being a team member of the Neuromuscular Clinic at CHOP, Brianna graduated with a MS in Genetic Counseling at Long Island University- Post, after receiving her BS in Integrative Neuroscience at The State University of New York at Binghamton. She is passionate about helping patients and their families understand the role of genetics and genetic diagnosis in their medical care.



Mark Moore – Committee Member

Mark C. Moore is a corporate restructuring and litigation attorney at Foley and Lardner LLP that specializes in the representation of debtors in complex chapter 11 bankruptcy cases. He graduated in 2010 from SMU Dedman School of Law and in 2007 from Baylor University with a degree in economics. He and his wife Beth have two children with SMA Type 1, William, who was born in 2011 and passed away in 2019, and Mary, who was born in 2015 and was one of the earliest presymptomatic recipients of Spinraza through clinical trials. They also have two other children, Charlotte (born 2009) and Beckham (2021). He is extremely excited to be a part of Cure SMA and joined the Financial and Operations Committee in 2022.



Karen McRory-Negrin – Committee Member

Karen is an attorney who has spent her career serving the public at the Philadelphia District Attorney's Office and the Pennsylvania Office of Attorney General. As an Assistant DA, Karen criminally prosecuted child sexual and physical assault, domestic violence and elder abuse cases. Karen was hosted by the U.S. Embassy in Bangkok to train Thai prosecutors and probation officers on juvenile justice. As a Senior Deputy Attorney General, she enforced global and state businesses for violating the civil rights of consumers. She previously served on the Board of the Make-a-Wish Foundation and the Philadelphia Senior LAW Center. Karen and her husband Rich have four children. Their family joined the SMA community at the 2001 Cure SMA Conference, after their third child Abigail was diagnosed with Type 1 SMA. They launched the Cure SMA PA Chapter in 2003 and with many other committed families, raised over \$1 million in revenue. After 16 years of service in the PA Chapter, they relocated to Chicago, IL where Karen continues to honor Abigail's legacy.



Joe Wiseman – Committee Member

Joe lives with his family in Albuquerque, New Mexico, where he practices family law and estate planning. Joe and his wife, Annie, have four children. Their youngest child, Quinn, was diagnosed with SMA in 2019.

MEDICAL ADVISORY COUNCIL (MAC)



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.



William Bell, BS Pharm, MBA, MSCC, RPh

Director of Clinical Client Services in Chalfont, PA.



John Brandsema, MD

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



Thomas Crawford, MD

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



Tina Duong, MPT, PhD

Physical Therapist, Sr. Research Scientist Stanford University in Palo Alto, CA.



Albert Freedman, PhD

Counseling Psychologist in independent practice at Freedman Counseling Associates, Philadelphia, PA.



Melissa Gibbons, MS, CGC

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



Jennifer Hubbell, BS, OTR/L

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



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

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.

MEDICAL ADVISORY COUNCIL (MAC)



Richard M. Kravitz, MD

Pediatric Pulmonologist and Sleep Physician at the University of Virginia in Charlottesville, VA.



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

Professor and Pediatric Physical Therapist at Northwestern University Feinberg School of Medicine, associated with Ann and Robert H. Lurie Children's Hospital in Chicago, IL.



Oren Kupfer, MD

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



Khalida Liaquat, MS, LCGC

Genetic Counselor at Athena Diagnostics in Marlborough, MA.



Leslie Nelson, PT, PhD

Neuromuscular Physical Therapist at University of Texas Southwestern and Children's Health in Dallas, TX.



Julie Parsons, MD

Pediatric Neurologist at Children's Hospital Colorado, associated with the University of Colorado School of Medicine in Aurora, CO.



Samuel Rosenfeld, MD

Orthopedic Surgeon at CHOC Children's Hospital in Orange, CA.



Peter Schochet, MD

Pediatric Pulmonologist at Children's Health Pulmonary Specialists in Dallas, TX.



Perry Shieh, MD, PhD

Neurologist at UCLA Medical Center in Los Angeles, CA.



Anne Stratton, MD

Pediatric Physiatrist at Children's Hospital Colorado, associated with the University of Colorado School of Medicine in Aurora, CO.

MEDICAL ADVISORY COUNCIL (MAC)



Stacey Tarrant, BS, RD, LDN

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



Jane B. Taylor, MD, MsCR

Pediatric Pulmonologist at the UPMC Children's Hospital of Pittsburgh, associated with the University of Pittsburgh School of Medicine in Pittsburgh, PA.



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 School of Medicine in Aurora, CO.

SCIENTIFIC ADVISORY BOARD (SAB)



Jackie Glascock, PhD, Vice President of Research

Dr. Glascock oversees 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, MD

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, PhD

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, MD

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, MD, PhD

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.



Adrian Krainer, PhD

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 drug Spinraza.



Umrao Monani, PhD

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 in SMA. 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, PhD

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 muscular 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).



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 the molecular and cellular mechanisms underlying disease progression and preclinical development of novel therapeutics.



Rashmi Kothary, PhD

Dr. Kothary is a Professor in the Department of Cellular and Molecular Medicine at the University of Ottawa. He is also the Deputy Scientific Director of the Ottawa Hospital Research Institute. His current research focuses on investigating extrinsic and intrinsic factors important for oligodendrocyte mediated myelination and remyelination of the CNS (in the context of Multiple Sclerosis) and understanding Spinal Muscular Atrophy pathogenesis and identifying novel therapeutics.



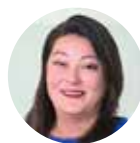
Katherine Klinger, PhD

Dr. Klinger is Global Head of Transitional 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.

TRANSLATIONAL ADVISORY COUNCIL (TAC)



Christine Brideau, PhD, Head of In Vitro Pharmacology, Deerfield Discovery & Development, LLC, Preclinical Drug Development



Dione Kobayashi, PhD, Head of Therapeutics, Amplify, Mass General Brigham, Preclinical Drug Development



Arthur Burghes, PhD, Professor, The Ohio State University, SMA Biology



Timothy Reilly, PhD, DABT, Chief Development Officer, HotSpot Therapeutics, Inc., Toxicology



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



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



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



Jasbir Singh, PhD, President, Jasin Discovery Solutions, Medicinal Chemist



Joseph W. Lewcock, PhD, Chief Scientific Officer, Denali Therapeutics, Preclinical Drug Development



Charlotte Sumner, MD, Professor, Johns Hopkins University School of Medicine, SMA Biology/Neurology



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



Michael Vellard, PhD, Chief Scientific Officer, Home Biosciences, Preclinical Drug Development

ADULTS WITH SMA ADVISORY COUNCIL



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Jaclyn Greenwood



Amber Bosselman



Jose Flores



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Kyle Derkowski



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Collin Pollock



Ryan Manriquez



Dan McHale



Sandy Spoonemore



Doug McCullough



Stephen Bingman



Emma Lockwood



Steven Verdile



Kenneth Hobby, President

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**Jacqueline Glascock, PhD,
Vice President, Research**



**Jessica Tingey, Manager,
Scientific Writer**

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Mary Schroth, MD, Chief Medical Officer



**Alesa Monk, Senior Manager,
Care Center Network**



**Mary Curry, ND, Vice President, Clinical
Research and Care**



**Sarah Whitmire, Director, Clinical
Registry Data Analytics**



**Lisa Belter, MPH, Vice President,
Research Data Analytics**



**Erica Jorgensen, Associate Manager,
Clinical Care Operations**



**Jennifer Mangers Deans, Director, Clinical Care
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**Lauren Eisenman, Manager,
Clinical Affairs**



**Erin Welsh, Specialist,
Data Analyst**

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**Colleen McCarthy O'Toole, Senior
Vice President, Community Support**



**Jessica Clark, Senior Director,
Community Support**



**Shannon O'Brien, Vice President,
Community Support**



**Karen O'Brien, Senior Manager,
Community Support**



**Sara Kwon, Senior Manager,
Community Support**



**Danyelle Sun, Senior Manager,
Social Work**

CURE SMA STAFF

COMMUNITY SUPPORT CONTINUED



**Kaity Thompson, Manager,
Community Support**



**Kathleen Wittmann, Manager,
Community Support**



**Alicia Metzger, Specialist,
Community Support**



**Jaki Herrmann, Specialist,
Community Support**

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Policy and Advocacy**



**Sarah Bellish, Manager,
Policy and Advocacy**

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**Alyssa Zavislak, Senior Manager,
Development and Events**



**Samantha Edidin, Director, Development
and Corporate Engagement**



**Melanie Lindsay, Manager,
Development and Events**



**Kyle Houlihan, Director,
Development and Events**



**Audra Gordon, Specialist,
Development and Events**



**Erin Lacey, Manager,
Development and Events**

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Marketing and Communications**



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Content Marketing**



Tiffany Sugar, Director, Design



**Darby Sampson, Specialist, Social
Media Content**



**Zach Galati, Manager,
Digital Marketing**

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**Chris Almborg, Vice President,
Compliance**

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**Marline Pagan,
Chief Operating Officer**



**Amber Ewert Snyder, Senior
Director, Database Management**



**Angela Taylor, Senior Director,
Human Resources**



**Larry Einhorn, Director,
Information Technology**



**Melissa Gonzalez,
Director, Finance**



**Rick Pagan, Manager,
Facilities and Logistics**



**Vanessa Jackson,
Specialist, Gift Processing**



**Alissa deRamus,
Executive Assistant**



**Jeanette Figueroa,
Revenue Accountant**

JOIN THE MOVEMENT WITH CURE SMA TODAY!



Make your mark against spinal muscular atrophy (SMA) to fund ground-breaking research initiatives, essential community support programs, vital care initiatives, and crucial advocacy actions for our SMA community!

Only with the loyal support from our community of event organizers, donors, supporters, and volunteers, can Cure SMA continue to achieve milestones that positively impact the lives of those with SMA.

At Cure SMA, you can make an immediate impact by joining a Walk-n-Roll, attending a Special Event, participating in an Endurance race, hosting your own Do-It-Yourself Fundraiser, or even securing corporate sponsorships. However you choose to fundraise for Cure SMA, you are making your mark by advancing our mission to find a cure!



WALK-N-ROLL

The Cure SMA Walk-n-Roll is a nationwide fundraising program that brings communities together to support Cure SMA's funding of life-changing research, resources, and programming for those impacted by spinal muscular atrophy (SMA). No matter how you plan to participate in our Walk-n-Roll program, this is one of the best ways to fundraise for people with SMA in your community and beyond. With nearly 50 Walk-n-Roll locations nationwide, it's as easy as 1, 2, 3:

- 1. Join the Movement** by creating a team, joining a team, or registering as an individual at a local Walk-n-Roll in your area.
Don't have a Walk-n-Roll nearby? Join our 2023 Walk-n-Roll USA location and participate virtually!
- 2. Ask for Support** by sharing your story and participation with family and friends. Haven't fundraised before? No worries – we can help with coaching, resources, and the tools you need for success!
- 3. Celebrate with Us** by sharing your success and the impact you've made for those with SMA together!

ENDURANCE

The Cure SMA Endurance Program connects runners and cyclists across the country to train for endurance events and fundraise for Cure SMA. Endurance athletes—both veterans and newcomers alike—train, fundraise, and bring awareness to support Cure SMA's mission of funding breakthroughs, changing lives, and empowering the SMA community. You can sign up to participate in a local marathon, half marathon, 10K, or 5K race, and earn exclusive Team Cure SMA Endurance gear all while making a difference for those with spinal muscular atrophy (SMA).



GOLF OUTING

Participants in a Cure SMA Golf Outing enjoy a day on the links while supporting Cure SMA and our spinal muscular atrophy (SMA) community. Each event features a wonderful day of golf, lunch and dinner, event programs, awards, course games, and more all while raising funds to support research, programs, and services for those with SMA. Whether you plan to join one of our already existing tournaments or design your own, Cure SMA is here to help you make your golf tournament memorable.



EVENING OF HOPE

Cure SMA Evening of Hope events celebrate progress and look towards the future of SMA by bringing together our SMA community and supporters. Evening of Hope events feature a cocktail hour, dinner, live entertainment, impactful news and updates, auctions, and more! If you don't see an Evening of Hope event in your local area, but are interested in creating an event, Cure SMA is here to support your efforts!



DO IT YOUR WAY

FUNDRAISING

Cure SMA Do It Your Way Fundraising empowers you to make an impact for the SMA community, your way. Whether you're thinking about fundraising in honor of your birthday or a special occasion, participating in an athletic event, creating a memorial page, or starting your own creative fundraiser, we've got you covered! From virtual stream-a-thons to kickball tournaments to essay writing contests and everything in between, Cure SMA has seen the SMA community get extra creative, and you can join in the fun!

READY TO MAKE YOUR MARK AGAINST SMA AND FUNDRAISE TODAY?



Find your local fundraising event at www.curesma.org/fundraising-events/ or scan the QR code to get started!



If you have any questions, please reach out to the Cure SMA Development and Events team at fundraising@curesma.org

OFFICIAL CURE SMA MERCHANDISE

Items will be on sale at the merchandise table next to registration in the Asbury Rotunda at the Yacht & Beach Club Convention Center. Make sure to stop by and check it out while supplies last!

7 Brand New Items Debuting at Conference!

- 2023 Conference Sticker
- Belt Bag
- We Can Cure SMA Cropped Grey Sweatshirt
- Dark Lavender Long Sleeve Tee
- White Cropped Logo Crewneck Sweatshirt
- Black Long Sleeve Circle Tee
- 40oz Travel Mug



Items Sneak Peak:

- Men's, Women's, and Youth Apparel
- Onesies
- Cooler Bags
- Blankets
- Stickers
- Promo items
- Bags
- Hats
- Drink ware
- Accessories
- Awareness Kits
- Candles

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

Accessories:

- Adhesive Cell Phone Wallet
~~\$10~~ **NOW \$6**
- Cookie Cutter Set
~~\$12~~ **NOW \$8**
- Car Magnet
~~\$6~~ **NOW \$5**
- Embroidered Plush Blanket
~~\$25~~ **NOW \$20**
- License Plate Frame
~~\$20~~ **NOW \$15**
- Push Pop Fidget Game
~~\$8~~ **NOW \$5**
- Adult & Youth Reusable Face Masks (Purple, White, Black)
~~\$10~~ **NOW \$5**
- Vinyl Decal
~~\$5~~ **NOW \$1**
- Adult & Youth Bracelet
~~\$2~~ **NOW \$1**
- Notecards
~~\$15~~ **NOW \$10**
- Popsocket
~~\$10~~ **NOW \$5**
- Tuffy the Plush Puppy
~~\$20~~ **NOW \$15**
- Valentine's Goody Bags
~~\$30~~ **NOW \$20**
- Wheelchair joysticks
~~\$25~~ **NOW \$20**

Stickers:

- Circle Sticker
- Est 1984 Sticker
- Logo Sticker
- Black Square Sticker
- Blue Square Sticker
- Light Purple Square Sticker
- Orange Square Sticker
- Pink Square Sticker
- Purple Square Sticker
- CURE Sticker with SMA Repeating

NEW - 2023 Conference Sticker

****All stickers on sale for \$1 each!**

Drinkware:

- NEW** 40oz Orange Travel Mug
~~\$30~~ **NOW \$25**
- Purple Water Bottle
~~\$15~~ **NOW \$10**
- Stainless Steel Wine Tumbler
~~\$20~~ **NOW \$15**

Bags:

- NEW** Black Belt Bag
~~\$20~~ **NOW \$15**
- Large Cooler Bag Backpack
~~\$45~~ **NOW \$35**

Hats:

- Gray Adjustable Baseball Hat
~~\$24~~ **NOW \$20**
- Black Performance Baseball Hat
~~\$25~~ **NOW \$20**
- Gray Bucket Hat
~~\$20~~ **NOW \$15**

Toddler & Youth Apparel:

- Baseball Tee Onesie(6mon, 12mon, 18mon & 24mon)
~~\$18~~ **NOW \$15**
- We Can Cure SMA Onesie(6mon, 12mon, 18mon & 24mon)
~~\$18~~ **NOW \$15**
- Youth Grey Crew Neck Tee (Small-XLarge)
~~\$24~~ **NOW \$20**
- Toddler Grey Crew Neck Tee (2T-4T)
~~\$24~~ **NOW \$20**
- Youth We Can Cure SMA Crewneck Sweatshirt (XSmall-XLarge)
~~\$25~~ **NOW \$20**

Adult Apparel:

- Men's White Performance Polo (Small-4XLarge)
~~\$28~~ **NOW \$15**
- Men's Performance Half Zip (Small-3XLarge)
~~\$49~~ **NOW \$40**
- Unisex Black Performance Full-Zip Fleece (XSmall-4XLarge)
~~\$49~~ **NOW \$45**
- Unisex Grey Crew Neck Tee (XSmall-4XLarge)
~~\$24~~ **NOW \$15**
- Unisex Orange V-Neck Tee (XSmall-2XLarge)
~~\$24~~ **NOW \$15**
- Unisex Microfleece Full-Zip Jacket (XSmall-3XLarge)
~~\$49~~ **NOW \$40**
- Unisex We Can Cure SMA Crewneck Sweatshirt (Small-3XLarge)
~~\$30~~ **NOW \$25**
- NEW** Unisex Dark Lavender Long Sleeve Tee (Small-2XLarge)
~~\$30~~ **NOW \$28**
- NEW** Unisex Black Long Sleeve Circle Tee (Small-2XLarge)
~~\$30~~ **NOW \$28**
- Windbreaker Full Zip Jacket (XSmall-3XLarge)
~~\$35~~ **NOW \$25**
- Women's Purple Performance Polo (XSmall-4XLarge)
~~\$28~~ **NOW \$15**
- Women's Performance Racerback Tank (XSmall-4XLarge)
~~\$25~~ **NOW \$20**
- Women's White Reflective Performance Half Zip (Small-4XLarge)
~~\$49~~ **NOW \$45**
- NEW** White Cropped Logo Crewneck Sweatshirt (Small-2XLarge)
~~\$35~~ **NOW \$30**
- NEW** We Can Cure SMA Cropped Crewneck Sweatshirt (Small-2XLarge)
~~\$35~~ **NOW \$30**
- Unisex Full Zip Purple Hoodie (XSmall-2XLarge)
~~\$49~~ **NOW \$40**

SUPPLIES ARE LIMITED

Make sure to stop by and check it out while supplies last!

Researchers and Clinicians Registered

AS OF MAY 3, 2023

Belen Abbruzzese
Novartis

Emanuela Abiusi
Università Cattolica del Sacro Cuore

Megan Abraham
Genentech

Alicia Ademi
Novartis Gene Therapies

Bassil Ahmed
University of Missouri

Kristen Alianello
Ann and Robert H. Lurie Children's
Hospital of Chicago

Marion Alleyne
Consultorios Medicos Paitilla

Carol Alochio
AAME

Renalli Alves
Promover

Christiano Alves
MGH

Judann Ambrose
Scholar Rock

Elliot Androphy
Indiana University School
of Medicine

Agnes Araujo
Novartis

Kara Arps
Monroe Carell Jr. Children's
Hospital at Vanderbilt

Saravanan Arumugam
Department of Medical Physiology
and Biophysics, School of Medicine,
University of Seville, Spain

Angela Avellaneda
Johns Hopkins

Hana Azizi
Columbia University
Medical Center

Marcello Azolino
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Scholar Rock

Maria Balch
The Ohio State University
Wexner Medical Center

Corrie Ball
Accredo

Natan Bar-Chama
The Mount Sinai Hospital

Zoe Barlow
Biohaven

Jason Barlow
Novartis Gene Therapies

Erin Barnobi
Scholar Rock

Aline Barros
Private

Kaitlin Batley
UT Southwestern

Scott Bayer
Scholar Rock

Clifford Bechtold
Biohaven

Anna Becklund
Genentech

William Bell
Southern Scripts

Lisa Belter
Cure SMA

Reena Berman
Novartis Gene Therapies

Kira Bernard
Seattle Children's Hospital

Roberto Bernardo Escudero
Novartis

Saunder Bernes
Phoenix Childrens

Debbie Berry
Nemours Children's Hospital

Diana Bharucha-Goebel
Children's National Hospital

Laura Blasco Pérez
VHIR - Vall d'Hebron Research Institute

Anton Blatnik
The Ohio State University

Beltran Borges
University of California San Francisco

Sandrine Boueilh
CHU de Québec/ CIUSSS CN

Julie Bourassa
University of Ottawa

Melissa Bowerman
Keele University

John Brandsema
The Children's Hospital of Philadelphia

Day Breen
Erlanger Children

Stacey Broughton
Children's Respiratory and Critical Care
Specialists, P.A.

Laurey Brown
Lurie Children's Hospital

Stephen Brown
Johns Hopkins University

Arthur Burghes
The Ohio State University

Barrington Burnett
Uniformed Services University of the
Health Sciences

Bryan Burnette
Vanderbilt University Medical Center

Russell J. Butterfield
University of Utah

Alessandra Calado
DONEM / RARUS

Marco Capogrosso
University of Pittsburgh

Meeta Cardon
University of New Mexico

Tara Carlin
Novartis

Kürşat Bora Çarman
Eskişehir Osmangazi University

Elice Carneiro Batista
Hospital Albert Einstein

Andrea Carruth
Childrens National Hospital

Terri Carry
Childrens Hospital Colorado

Alex Casdin
Epirium Bio

Diana Castro
Neurology Rare Disease Center

Maria Grazia
Cattinari FundAME

Özge Çetin
Keele University

Stefanie Chan
Genentech

Courtney Chase
Genentech

Emily Chee
Novartis Gene Therapies

Jun-An Chen
Academia Sinica

Tai-Heng Chen
Kaohsiung Medical University Hospital

Karen Chen
SMA Foundation

Martin Cheung
The University of Hong Kong

Claudia Chiriboga
Columbia University Medical Center

Lidia Choniawko
Stanford University

Matthew Civitello
St. Jude Children's Research Hospital

Peter Claus
SMATHERIA gGmbH – Non-Profit
Biomedical Research Institute

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Biohaven Pharmaceuticals

Laura Comley
University of Edinburgh

Caroline Conlan
University of Pennsylvania Perelman
School of Medicine

Kelly Copeland
Genentech

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Lurie Children's Hospital

Itzel Córdova
Novartis

Becky Cornacchia
Johns Hopkins Hospital

Shelley Coskery
Children's of Alabama

Mar Costa Roger
Vall d'Hebron Hospital

Nicholas Cottam
Delaware State University

Thomas Crawford
Johns Hopkins University

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Roche

Fernando Cruz
Novartis

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Mary Curry
Cure SMA

Thuy-Tien Dang
Children's Mercy Hospital

Basil Darras
Boston Children's Hospital

Donald Daudt
Genentech

Jarred Daugherty
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PLEASE JOIN US

FOR AN INSIGHTFUL AND INFORMATIVE PRESENTATION:

Personal Journeys with SMA and Clinical Trial Results sponsored by Biogen.

Hear personal journeys from a teenager and an adult on their SPINRAZA treatment experiences, and discover pivotal data about later-onset SMA and observational data in adults.



Maya P.
Patient Ambassador

Keishla R. - R
Patient Ambassador

Nassim Rad, MD
Neuromuscular Medicine,
Neurorehabilitation
UW Medicine/University of
Washington Medical Center

Dave Hernandez-Trujillo
Family Access Lead
South Region/Puerto Rico
US Rare Diseases - SMA

July 1, 2023 7:45 AM - 8:45 AM

We look forward to seeing you for breakfast at the Yacht Club during the Cure SMA Conference!

INDICATION

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

IMPORTANT SAFETY INFORMATION

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

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

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

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

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

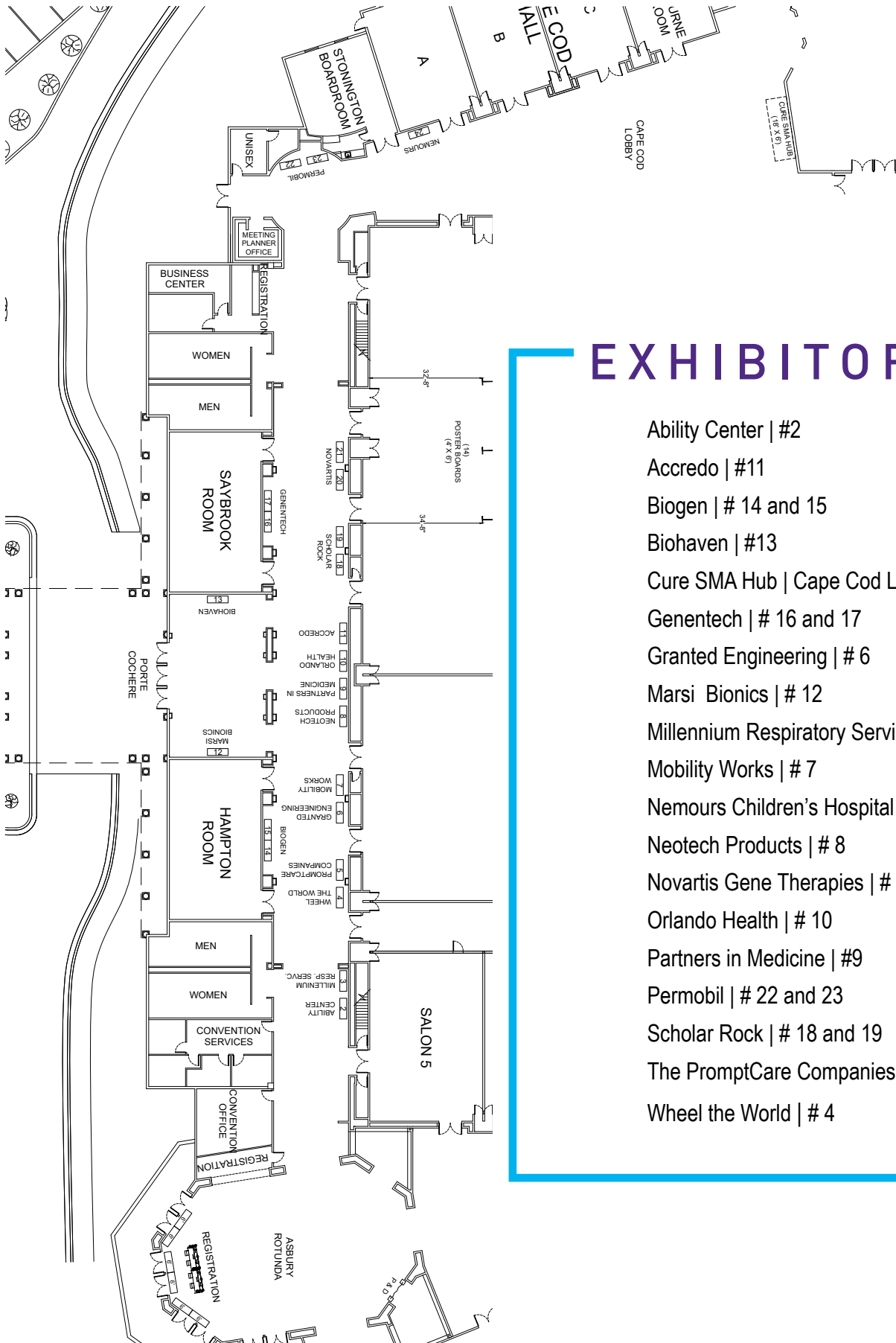
Please see Important Facts on the previous page and full Prescribing Information at [SPINRAZA.com](https://www.spinraza.com).

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

Attention healthcare professional attendees:

In accordance with the Sunshine Act reporting requirement, meals served at this event are considered transfers of value and will be reportable. Please review your local and state guidelines around promotional programs to ensure compliance.

Please note: This is not a CME presentation. CME credit will not be awarded for attendance at this presentation. Speakers are being compensated at fair market value for presentations in connection with this program.



EXHIBITOR KEY

- Ability Center | #2
- Accredo | #11
- Biogen | # 14 and 15
- Biohaven | #13
- Cure SMA Hub | Cape Cod Lobby
- Genentech | # 16 and 17
- Granted Engineering | # 6
- Marsi Bionics | # 12
- Millennium Respiratory Services | # 3
- Mobility Works | # 7
- Nemours Children's Hospital | # 24
- Neotech Products | # 8
- Novartis Gene Therapies | # 20 and 21
- Orlando Health | # 10
- Partners in Medicine | #9
- Permobil | # 22 and 23
- Scholar Rock | # 18 and 19
- The PromptCare Companies | # 5
- Wheel the World | # 4

GETTING AROUND DISNEY WORLD AT THE 2023 ANNUAL SMA CONFERENCE

Enjoyment at a Disney World Resort stretches out across hotels, parks, shopping, and every kind of fun you can imagine! It can feel overwhelming finding your way around all that fun but thankfully Disney has several convenient transportation options for you! Remember to plan and allow a little more time than you think you'll need, especially given the many wheelchair users that will be being accommodated during the Annual SMA Conference weekend. Wait times may be longer than you anticipate so make sure to plan and if you have any questions, don't hesitate to ask a Disney Cast Member!

As you plan to move from one activity to another throughout the conference, keep the following in mind:

- Conference session meeting space is located off the lobby of the Yacht Club, in the Yacht and Beach Convention area.
- Mom's and Dad's Nights Out are located at the Board Walk Inn Hotel. To get there, you can either walk around the lake using the boardwalk or take the boat across. Once you arrive at the hotel, head upstairs to the main lobby area, either by stairs outside or the elevator up to the lobby. Head out through the lobby and follow the sidewalk around to the convention space, following the signs to the meet-up space.
- The quickest route to get to the Cure SMA Evening in EPCOT Theme Park is by walking along the board walk to the park. You can take the boat over as well. Please note that when you are entering Epcot this way, you are entering at the back of the park, there are less security lines, so it might take a little longer to get through the lines and enter the park.

You can truly travel by land, air, and water when you're at Disney World! Search by the keyword below on the Disney World website (<https://disneyworld.disney.go.com>) to find more information about each type of travel. Make sure to also download the Disney World app. There's a helpful transportation navigation tool at the bottom of the menu screen.

Friendship Boats

Friendship Boats, you will catch the boats from the hotels, down by the lake on the boat deck.

- Friendship Boats typically begin running at 8:30 a.m. and end operations 60 minutes after park closing.
- Approximate transportation times vary, especially at park closing or if making multiple stops.

Routes include:

- Disney's Hollywood Studios to Swan Resort, Dolphin Resort, Yacht Club Resort, Beach Club Resort, Boardwalk Inn, and to Epcot.
- Epcot to Boardwalk Inn, Yacht Club Resort, Beach Club Resort, Swan Resort, and Dolphin Resort.

GETTING AROUND DISNEY WORLD AT THE 2023 ANNUAL SMA CONFERENCE

Disney Skyliner

Another fun way to travel is on the Disney Skyliner! Head towards Epcot and you will be able to catch Skyliner before you enter Epcot. You can also use this to get to Hollywood Studios. Also note that you do not need a park ticket to use the Skyliner so feel free to take it even for just a fun ride with a great view!

Bus/Shuttle Transportation

Bus may often be the most direct mode of transportation (and is free for resort guests) but make sure to consider that wheelchair accessible space on buses is limited so make sure to plan and allow for extra time.

Minnie Van

The adorable Minnie vans are operated through the Lyft app and while there is a cost to them, are a direct option as you travel around Disney World. As with any Lyft trip, the cost will fluctuate depending on the demand for the service. Accessible vans are available, and you can request one through the Lyft app.

Getting From the Yacht and Beach Club to



Magic Kingdom

- Pick up the Magic Kingdom Park bus from the front of the resort.
- Walk to Epcot (or take the Skyliner), take the Monorail to the Ticket and Transportation Center, then switch to the Magic Kingdom Park Monorail from there.

Epcot

- Walk – a 10-minute walk.
- Friendship Boat – about 20-minute ride. Pick up the boat at the end of dock by lighthouse and board on the left side of the lighthouse.

Hollywood Studios

- Friendship Boat – about 20-minute ride. Pick up the boat at the end of dock by lighthouse and board on the right side of lighthouse.
- Walk – a 15-to-20-minute walk.

Animal Kingdom

- Animal Kingdom Park bus, which picks up in front of resort.

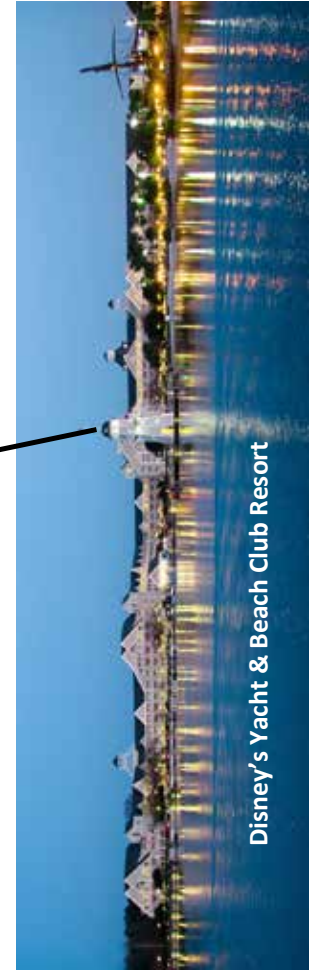
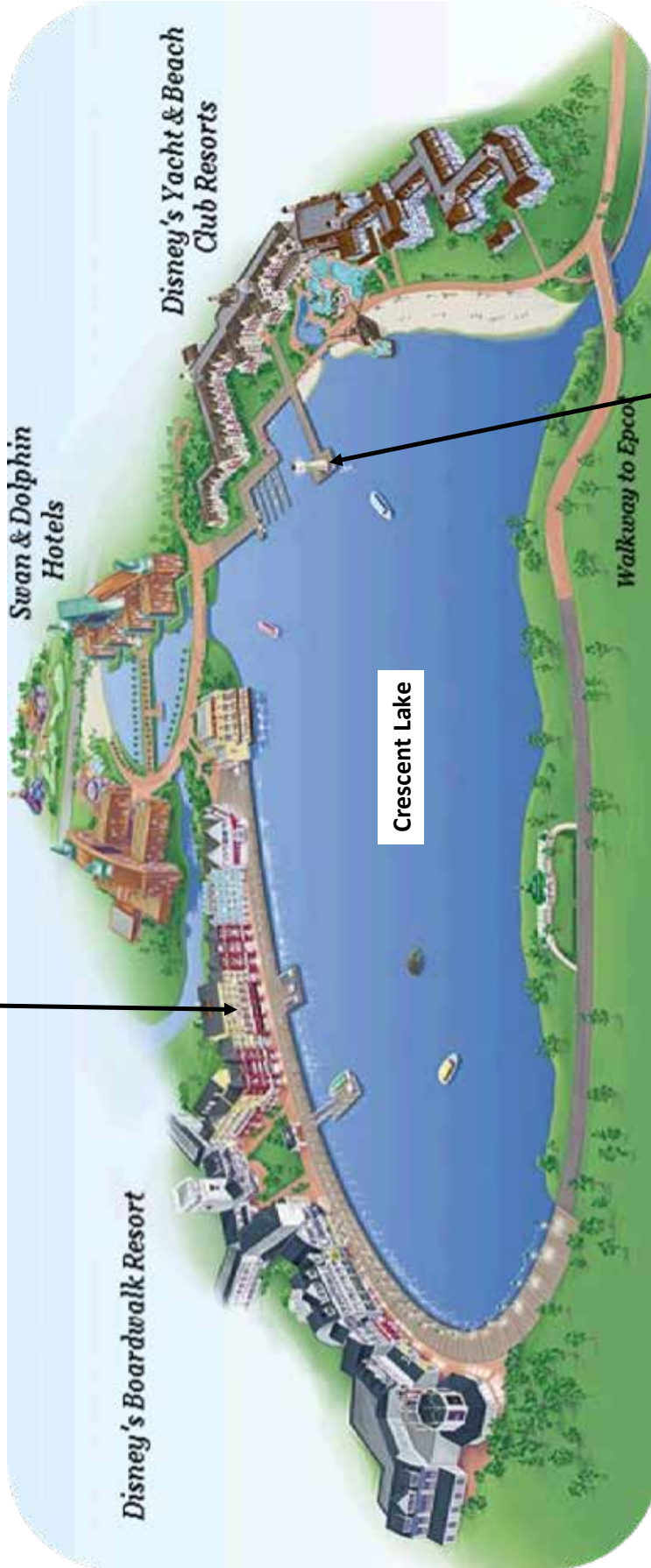
Disney Springs

- Go to the bus area to pick up a bus. Routes run every 15-20 minutes and signs will indicate when the next bus arrives.

MAP: EPCOT RESORTS

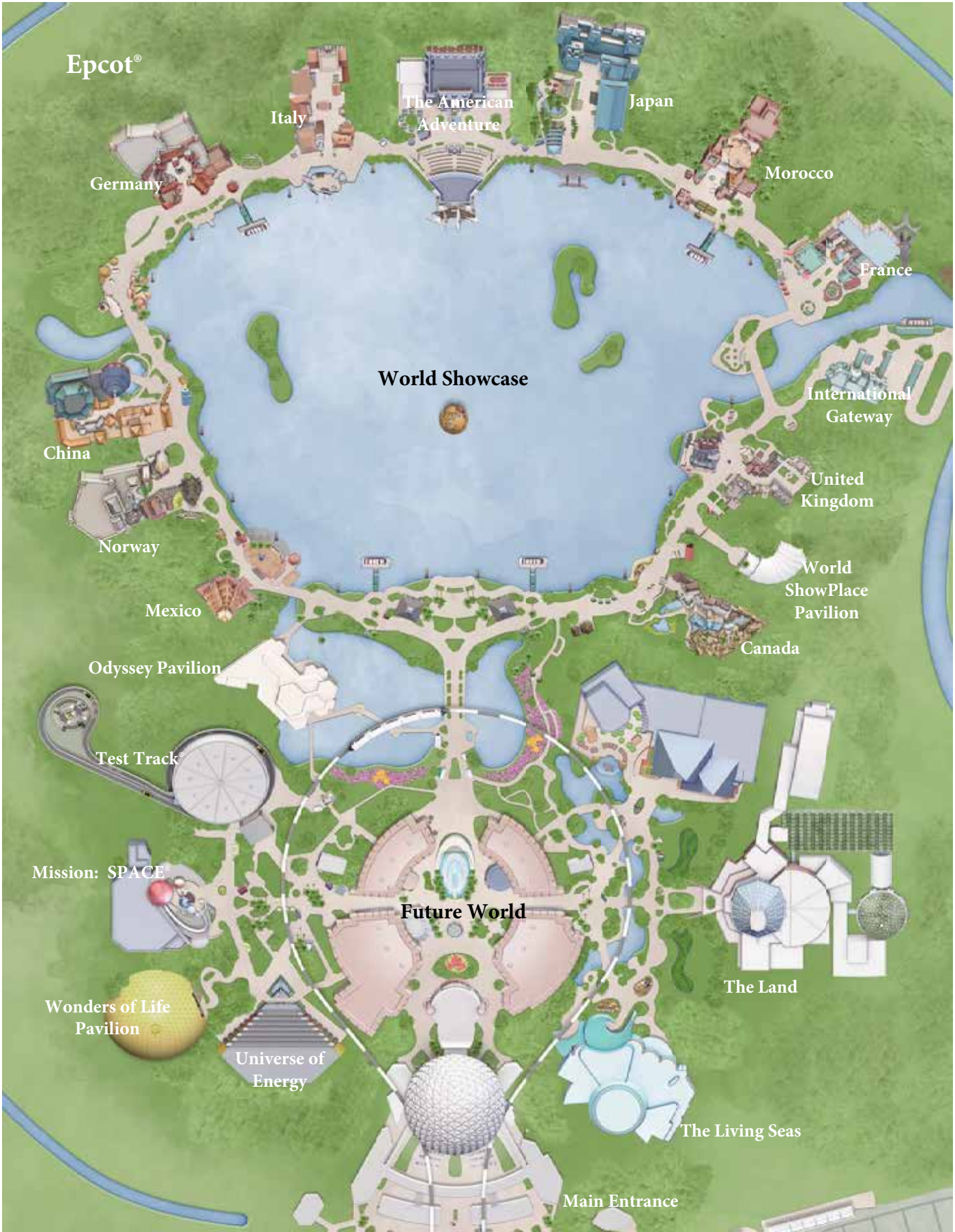


Disney's Boardwalk Resort



Disney's Yacht & Beach Club Resort

MAP: EPCOT THEME PARK





Guest Services

- A** Lobby/Concierge
- B** Disney Vacation Club® Information Center
- C** Resort Airline Check-In

Dining

- D** The Market at Ale & Compass
- E** Ale & Compass Restaurant
- F** Ale & Compass Lounge
- G** Crew's Cup Lounge
- H** Yachtsman Steakhouse
- I** Beaches & Cream Soda Shop
- J** Hurricane Hanna's Waterside Bar & Grill
- K** Martha's Vineyard
- L** Cape May Cafe
- M** Beach Club Marketplace

Shopping

- D** The Market at Ale & Compass
- M** Beach Club Marketplace

Recreation

- N** Stormlong Bay and Fitness
- O** Ship Shape Massage, Salon and Fitness
- P** Lafferty Place Arcade
- Q** Seaside Retreat
- R** Campfire
- S** Bayside Marina

- Bus Stops
- Disney's Magical Express® Bus Stop
- Laundry
- Automated External Defibrillators
- Elevators
- Designated Smoking Locations
- Dining Reservations Recommended (Call 407-WDW-DINE for more information.)

- Running Trail (0.8 mile)
- Route to Convention Center
- Wi-Fi is available in many areas throughout our Resorts including Guest rooms, main lobbies and feature pools. (Coverage may vary.)



Room.....
Building.....
Floor.....
 If you have questions, please call 407-934-3649 or visit the Front Desk.

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Timon and Pumbaa from "The Lion King" are part of our problem-free safety philosophy. Keep an eye out for their safety reminders throughout the Walt Disney World® Resort. To learn more visit disneywildaboutsafty.com

Service animal relief areas located in grassy areas adjacent to parking lots.



Guest Services

- A** Lobby/Concierge
- B** Disney Vacation Club® Information Center

Dining & Entertainment

- C** Carousel Coffee (2nd Floor)
- D** Belle Vue Lounge (2nd Floor)
- E** Flying Fish
- F** AbracadaBar
- G** Trattoria al Forno
- H** Boardwalk Pizza Window
- I** Boardwalk Deli
- J** Boardwalk Ice Cream
- K** Boardwalk Joe's Marvelous Margaritas
- L** Big River Grille & Brewing Works
- M** Jellyrolls
- N** Atlantic Dance Hall
- O** Leaping Horse Libations

Shopping

- P** Screen Door General Store
- Q** Disney's Character Carnival
- R** Trimmles & Threads

Recreation

- S** Inn Leisure Pool
- T** Luna Park Pool
- U** Luna Park Crazy Play Area
- V** Muscles & Bustles Health Club
- W** Side Show Games Arcade
- X** Community Hall
- Y** Villas Leisure Pool

- Bus Stop
- Laundry
- Automated External Defibrillators
- Mobile Food & Beverage Ordering
- Mobile Dining Reservation Check-In & Walk-Up List
- Elevators
- Designated Smoking Locations

- Running Trail (0.8 mile)
- Disney Pin Trading Destination

Service animal relief areas located in grassy areas adjacent to parking lots.

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Walt Disney World 50
 Discover classic favorites and exciting adventures across The Most Magical Celebration on Earth. To learn more about the 50th Anniversary celebration, visit disneyworld.com/50.

WALKWAY TO EPCOT™ INTERNATIONAL GATEWAY AND DISNEY SKYLINER
 Running Trail continues around Crescent Lake

SEABREEZE POINT

CRÉSCENT LAKE

BOAT TRANSPORTATION TO EPCOT AND DISNEY'S HOLLYWOOD STUDIOS

INN

1290-1353
 2289-2353
 3287-3323
 4287-4311
 5287-5311

1201-1215

2266-2282
 3201-3285
 4201-4285
 5261-5285

CONFERENCE CENTER

Floor _____
 Room _____

If you have questions, please call 407-939-5101 option 1 or visit the Front Desk.

MAIN ENTRANCE

1068 - 1136
 2068 - 2136
 3068 - 3136
 4068 - 4136
 5068 - 5095

VILLAS
 1001 - 1048
 2001 - 2066
 3001 - 3066
 4001 - 5066

WALKWAY TO TENNIS COURTS AND DISNEY'S HOLLYWOOD STUDIOS

Trimm and Pumbaa from Disney's The Lion King are part of our problem-free safety philosophy. Keep an eye out for their safety reminders throughout the Walt Disney World® Resort. To learn more visit disneyboardsafety.com.



Show Your Badge+ Program 2023

Enjoy all that our property has to offer with your name-specific meeting or convention identification badge. We have assembled an amazing array of deals that are available for guests enjoying Central Florida as part of an organized group. Simply show your badge or this digital/printed flyer in any of the venues listed below to unlock your discounts or benefits!

Dining

- **Chicken Guy!**: 10% off food purchase (excludes alcohol)
- **City Works Eatery and Pour House**: Buy one beer on tap and receive one free beer of equal or lesser value up to \$9
- **Earl of Sandwich**: 10% off food purchase and non-alcoholic beverages
- **House of Blues Restaurant & Bar**: 10% off food & non-alcoholic beverages
- **Joffrey's Coffee & Tea Company**: 20% off your purchase and a FREE Ripple Art Print on select beverages (exclusions apply)
- **Paddlefish**: 20% off food and non-alcoholic beverages
- **Planet Hollywood**: 15% off food purchase and non-alcoholic beverages
- **Raglan Road Irish Pub and Restaurant**: 10% off food and merchandise
- **STK Orlando**: 20% off purchase of food or non-alcoholic beverages (excluding tax & gratuity). Not valid on happy hour, discounted items, other promotions/offers, or on holidays. One offer per table. No cash value
- **Terralina Crafted Italian**: 20% off food and non-alcoholic beverages
- **The Polite Pig**: 10% discount on food and non-alcoholic beverages. (Cannot be combined with any other offers or discounts)
- **The Spice & Tea Exchange**: \$1 Off Any 16oz Iced or Hot Tea To Go
- **Wetzel's Pretzels**: 20% off entire purchase
- **Wine Bar George**: "Buy one, get one" complimentary glass of wine on tap. Choice of Moscato, Vezzi or Sabine. One per person per visit, per badge. Valid with food purchase. Not valid on holidays or at The Basket

Shopping

- **Basin**: 15% off entire purchase (exclusions apply)
- **Crystal Arts by Arribas Brothers**: 10% discount (exclusions apply)
- **Edward Beiner**: 15% off for purchases above \$150 (exclusions apply)
- **Fabletics**: 25% off entire purchase
- **House of Blues**: 10% off entire purchase in the Gear Shop. Exclusions apply
- **Jo Malone London**: 10% discount
- **Levi's®**: 15% off entire purchase
- **M&M's**: 15% off after spending \$40 or more
- **Ron Jon Surf Shop**: 10% off total purchase
- **Sugarboo & Co**: 10% off total purchase (exclusions apply)
- **UNOde50**: 10% off entire purchase (exclusions apply)
- **Vera Bradley**: 15% off total purchase (exclusions apply)

All offers available to guests with a current meeting/convention identification badge. No other forms of identification will be accepted. Offers are valid from January 1, 2023 until December 31, 2023 and are always subject to change at any time and without notice. Offers are not valid on prior purchases and cannot be used on gift cards. Offers may not be sold, bartered or used in conjunction with any other sales, promotions or discounts. Offers exclude tax and gratuity. Offers are limited to one per person, per day. See location for more details.

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August is SMA Awareness Month!

While we work year-round to raise awareness and funds for SMA, August is our time to shine a brighter spotlight on SMA and the SMA community.

Join Cure SMA in preparing for the biggest #SMAAwarenessMonth yet!

Here are the best ways to get ready for August:

- Our annual candle lighting takes place on Saturday, August 12th. Purchase your Cure SMA candle at curesmaorg.myshopify.com
- Do you have a story you'd like to share about your SMA journey? Or are you a social media superstar who'd like to help us create interesting and engaging social media content? Contact stories@curesma.org and let us know how you'd like to help
- Become a Cure SMA advocate and act on advocacy issues. Sign up at: www.votervoice.net/CureSMA/Register



Be sure to check out www.curesma.org/sma-awareness-month for all the latest #SMAAwarenessMonth updates!



Today, your donation can have twice the impact! Help us double the number of Care Center sites and the Erin Trainor Memorial Fund will match your donation.



In the past six years, there have been three powerful treatments approved that are making a significant difference for the SMA Community, and currently about 70% of all those with SMA are on one of these treatments. In addition, Newborn Screening for SMA has been implemented in 99% of all births, with the remaining wrapping up in 2023. This means that infants can now be diagnosed with SMA very early before symptoms appear, offering the pathway to early treatment.

All the changes in the disease state are wonderful for people with SMA; many are walking, sitting up, living longer. And as they live longer and more independent lives, they will need access to local care and support to assure a strong quality of life. SMA is NOT yet cured. There is much left to do and care centers are vital for access to care locally.

There are currently 29 local Care Centers in the Cure SMA Network but so many more are needed. **In honor of all the things we had hoped for Erin, we are committing to raising \$350,000 in order to support the opening of additional multi-disciplinary Care Centers across the country. And we will match every dollar, so Cure SMA will be able to put \$700,000 toward this important initiative.** Our goals are ambitious, yet critical to make a difference in the lives of children and adults with SMA.

Erin would have been 29 years-old this year. While we still feel the loss, we are so proud of the work that has been done and excited to see new care centers address so many children and adults with their various needs. For parents who receive a diagnosis now, their outcomes are hopeful, and we have made a renewed commitment to ensure they stay that way by increasing the Care Center Network of Cure SMA.

We still hold true to our original commitment to help make a difference to families diagnosed with SMA. Everything we did with our amazing network of contacts helped make significant differences in treatments approved for SMA and for that we all should be proud. Now we can do the same to ensure rapid local access, and care for all those impacted by SMA. Together with you, our supporters, we can continue to make a difference. Please make your generous gift of support today.

“For more than ten years, the Erin Trainor Memorial Fund had an immeasurable impact bringing newly diagnosed families to the Annual SMA conference. We still hold true to our original commitment to help make a difference to families diagnosed with SMA. For parents who receive a diagnosis today, several therapies offer a pathway to early treatment. Their outcomes are hopeful, and we are making a renewed commitment to ensure they stay that way. Beginning in 2022, the ETMF commit its resources to help increase the Care Center Network of Cure SMA. This network will give patients with SMA the best care possible.”

– Gene Trainor

**Double Your Impact
and Donate Today**

**ERIN TRAINOR
MEMORIAL FUND**

