



ANNUAL SMA CONFERENCE

#2022SMACONFERENCE

JUNE 16-19, 2022 | ANAHEIM, CA

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PROGRAM GUIDE



WELCOME LETTER

Welcome to the 2022 Annual SMA Conference. It's good to be back! We are excited to have our whole community back together in-person.

This conference will be both a catch up and a look-forward. We will catch up, after two missed years, on connections with friends and collaborators. And we will catch up on all the amazing success and progress that we have had in SMA in spite of the recent pandemic and isolation.

We have reached the milestone of 97% of newborns in the U.S. being screened, followed by rapid treatment. We have three powerful approved therapies, with almost 70% of all in our community on a treatment. And we have more clinical trials in process now than ever before!

As a community, we celebrate these breakthroughs, all the while recognizing that there remains more to be done. We are excited to be back at our conference to look forward and keep moving forward. SMA has changed and we are expanding our research, care, and support programs while our community values remain the same.

It is important now to optimize the impact of the therapies and develop combination approaches and additional therapeutic targets to treat all ages, stages, and types of SMA, and to focus on restoring and building strength. As we are now able to return to in-person activities for our SMA community, including conferences and fundraising events, we are very excited to be able to restart basic research funding. Funding and leading the 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 do.

One of our important initiatives is our SMA Care Center Network. The network is expanding to 30 centers this year, and we will continue to add local care centers across the U.S., to guide our understanding of how SMA is changing, how care is being delivered, and to identify best practices. We are increasingly shifting our programs to provide customized and local support and advocacy, to follow where our treatments and improved care are being delivered.

We're expecting a record number of attendees at this year's conference. There will be 49 family workshops, 32 research presentations, and 15 care presentations given during this event.

This conference is generously supported by ten sponsors and 17 exhibitors. Our special thanks to Biogen, Genentech, and Novartis Gene Therapies as the Presenting Sponsors of the 2022 Annual SMA Conference. Through these sponsorships, we are able to provide all of these incredible workshops and events, as well as the assistance for travel and registration costs for so many attendees!

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. For individuals impacted by SMA, we have workshop tracks that serve all of the different groups in our community. And, as always, we look forward to connecting and networking with each other through scheduled events and informal conversations.

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

Sincerely,



Kenneth Hobby
President



Colleen McCarthy O'Toole
Vice President, Community Support



Mary Schroth, MD
Chief Medical Officer



Jackie Glascock, PhD
Vice President, Research

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



OUR VALUES

Innovation

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

Balance

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

Collaboration

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

Respect

There is no "right way" to live with a disease like spinal muscular atrophy. Every person's experience is different, and it's every family's right to decide what SMA means for them.

Compassion

Thanks to the Cure SMA community, no person is ever alone in facing this disease. We offer unconditional support to people affected by SMA and communicate openly and honestly, giving them clear and accurate information.

Determination

Our work is not done until we have more treatments and a cure, and we'll remain strong in our fight no matter what challenges come our way.

WELCOME!

We are honored to have SMA individuals, families, researchers, and clinicians attend this conference from all over the world! There are individuals attending from 29 countries, including: Argentina, Australia, Brazil, Canada, Costa Rica, Egypt, France, Germany, Georgia, Iceland, Ireland, Italy, Mexico, Morocco, Nepal, Netherlands, New Zealand, Norway, Panama, Pakistan, Paraguay, Spain, Switzerland, Taiwan, Turkey, Uganda, United Kingdom, United States, and Uruguay.



As of May 11th, 2022

Celebrating Our 33rd Conference This Year!

2022



1988



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, DC
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, DC
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
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

SPONSORS

Thank you to our generous sponsors for their support of the 2022 Annual SMA Conference. These partnerships offer a unique opportunity to enhance groundbreaking research and provide individuals with SMA and their families the support they need today.

PRESENTING SPONSORS



VISIONARY SPONSOR



PLATINUM SPONSORS



GOLD SPONSORS

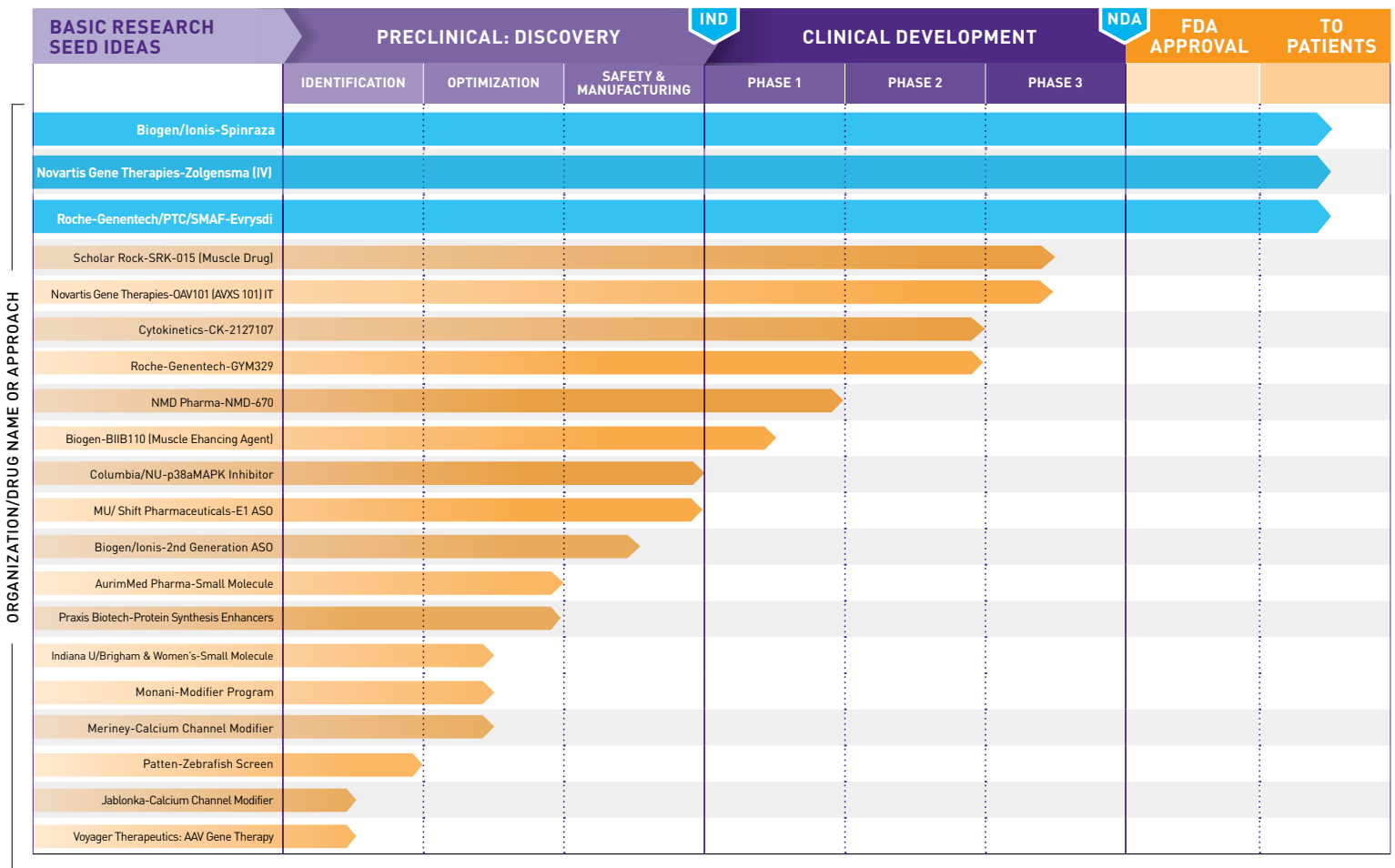


SMA DRUG PIPELINE

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: November 2021



THE IMPORTANCE OF CONTINUING TO INVEST IN BASIC RESEARCH

- In the past two decades, Cure SMA has invested \$15 million in funding for 128 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 disease, there remains significant unmet needs, especially for older, symptomatic individuals with SMA.
- 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 and help restore strength and function.

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

- **Non-SMN targets.** We're looking for other systems, pathways, and processes that can serve as the basis for an SMA treatment. We're 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're 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 recently launched a request for proposals for new basic research grants. These grants will be focused on the previously described key areas of research. By continuing 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. These grants will be announced later this fall. Please be on the look out for information about these grants in upcoming Cure SMA publications.

WHAT IS THE CURE SMA FUNDING MODEL?

The Cure SMA research-funding model is firmly based on the philosophy of expert and independent review and oversight of research projects. The Cure SMA strategy consists of having expert advisors review, select, and then oversee the research projects that we fund. This system has many advantages. Primarily, it ensures that Cure SMA funds 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. It is often referred to as “peer review”, which means scientists working in similar areas judge each other’s work.

Never a Better Time to Invest in SMA Research!

Due to the generous support of Richard and Jane Nunemaker, gifts to these peer-reviewed research commitments will be matched up to \$250,000! Your investment in SMA research will focus on ways to enhance muscle strength and function. Your investment may also have an impact on nerve muscle connections and regenerating nerves.

And your gift will be matched up to \$250,000. Thank you to the Nunemaker’s for bringing this research closer to reality with their gift. If you want your gift match, write “Research Match” on the check memo or on the donation form.

And thank you for your continued support.



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!

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



twitter.com/cureSMA



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



facebook.com/cureSMA



youtube.com/user/FamiliesofSMA1



www.linkedin.com/company/families-of-sma



CONFERENCE SUMMARY

WEDNESDAY, JUNE 15, 2022

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

THURSDAY, JUNE 16, 2022

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

4:30PM – 6:00PM NEWBORN SCREENING MEET & MINGLE *Sponsored by Novartis Gene Therapies*
for families identified through newborn screening

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

8:30PM – 10:30PM DAD'S NIGHT OUT **for dads only**

FRIDAY, JUNE 17, 2022

7:00AM – 5:30PM REGISTRATION OPEN

7:30AM – 8:45AM ANNUAL SMA CONFERENCE BREAKFAST WITH SYMPOSIUM
Sponsored by Biogen

7:30AM – 5:30PM EXHIBITOR TABLES OPEN

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

10:45AM – 5:15PM ADULTS WITH SMA LOUNGE *Sponsored by Biogen*
for adults with SMA and their attendants

11:15AM – 12:30PM WORKSHOP SESSION #1

12:30PM – 1:30PM ANNUAL SMA CONFERENCE LUNCH

1:45PM – 3:15PM WORKSHOP SESSION #2

3:30PM – 5:00PM WORKSHOP SESSION #3

FRIDAY, JUNE 17, 2022 CONTINUED

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

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

7:30PM – 10:00PM TEEN SOCIAL **for all teens ages 12 – 17**

8:00PM – 10:00PM ADULTS WITH SMA RECEPTION sponsored by Biogen
for adults with SMA and their attendants

8:30PM – 10:30PM MOM'S NIGHT OUT **for mom's only**

SATURDAY, JUNE 18, 2022

7:30AM – 4:30PM REGISTRATION OPEN

7:30AM – 4:30PM EXHIBITOR TABLES OPEN

7:30AM – 8:45AM ANNUAL SMA CONFERENCE BREAKFAST WITH SYMPOSIUM
Sponsored by Genentech

9:00AM – 10:30AM WORKSHOP SESSION #4

9:00AM – 4:15PM ADULTS WITH SMA LOUNGE Sponsored by Biogen
for adults with SMA and their attendants

10:45AM – 12:15PM WORKSHOP SESSION #5

12:15PM – 1:15PM ANNUAL SMA CONFERENCE LUNCH

1:15PM – 2:45PM WORKSHOP SESSION #6

3:00PM – 4:15PM WORKSHOP SESSION #7

4:30PM CURE SMA EVENING AT THE PARK

SUNDAY, JUNE 19, 2022

7:30AM – 8:45AM ANNUAL SMA CONFERENCE BREAKFAST WITH SYMPOSIUM
Sponsored by Novartis Gene Therapies


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

WORKSHOP SESSIONS

WORKSHOP SESSION #1

- Aquatic Physical Therapy Question & Answer Session
- Ethics, Angst, Excitement and Realities of SMA Treatment and Research
- Using Muscle-Based Exercises for Speech Clarity and Feeding
- Women's Health in SMA
- Therapeutic Yoga for Adults and Teens with SMA
- "Talk it Out" for Kids with an SMA-affected Parent (Ages 6- 17)

WORKSHOP SESSION # 2

- Taking Charge of Your Health: The Transition from Pediatric to Adult Care

Sponsored by Genentech
- The ABLE Act: EnABLE Savings for Life
- Change Your Brain - Change Life: The number one tip from a Disability Life Coach
The number one tip from a Disability Life Coach
- Pediatric Therapeutic Yoga for SMA
- Spouses and significant others of adults with SMA sharing your experience
- Healing the Grieving Heart – Part I
- Aquatic Physical Therapy for Fun & Function, Children with Supported Head Control
- Using Our Breath for Big Emotions – Mindfulness for Kids

WORKSHOP SESSION # 3

- Medical Management of Adults with SMA
- The Role of Therapy PT and OT in your Child's Life – Being Part of the Team!
- I Need to Cough, I Need to Breathe, I Need to Sleep
- Grandparents Coffee and Mingle
- Aquatic Physical Therapy for Fun & Function, Children with Independent Head Control
- Adults with SMA Relationships Panel – Share Your Experience
- Memory Boxes for Siblings: Creating a Lasting Treasure (for children only)

WORKSHOP SESSION #4


- Orthopedic Management
- Driving and Community Mobility

Sponsored by Genentech
- Keepsake Creation: Grieving Through Art
- Unpacking Clinical Trials
- Young Adults Talk it Out (Ages 18+)
- Genetics and Reproductive Options for SMA

WORKSHOP SESSION # 5

- Navigating the Special Education Process: Setting Our Kids up for Success
- An Approach to Exercise in Individuals with SMA
- Special Plans for a Special Life: Estate Planning with Supplemental Needs Trusts
- Hold It No More: A Panel Discussion on Suprapubic and Mitrofanoff Catheterization Options
- Benefits of Adaptive Recreation and Cycling
- Aquatic Physical Therapy for Fun & Function, Teens and Adults
- Healing the Grieving Heart – Now What? Part II

WORKSHOP SESSION # 6

- Siblings Talk it Out (Ages 5 to 11)
- Service Dogs: Safety, Independence, Confidence
- How to Build Your Presence and Advocate Through Writing & Social Media

Sponsored by Genentech
- The Caregiver's Toolbox (Essentials)
- A Lifetime of Good Nutrition
- What it Means to be a Teen on Wheels

WORKSHOP SESSION # 7

- Kids Talk it Out (Ages 6 – 10)
- SMA and Dating/Sex Roundtable Discussion
- Early Scoliosis Management in SMA
- University of Utah TRAILS Program: Adaptive Sports and Novel Adaptive Equipment
- Finding Hope After Loss: Helping Parents and Children Cope
- Emergencies – Anticipation, Prevention and Readiness in the Community

ADDITIONAL SESSIONS

- Kids Talk it Out (Ages 11 – 17) Friday, June 17th
9:00am – 10:30am
- Siblings Talk it Out (Ages 12 – 17) Friday, June 17th
9:00am – 10:30am



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!



SPECIAL EVENTS



MEET & GREET

THURSDAY 6:00PM - 8:30PM

GRAND BALLROOM

KICK OFF TO THE CONFERENCE!

Meet Disney Characters: Micky & Minnie, Tiana & Louis, Lilo & Stitch, Winnie the Pooh & Tigger

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



FAMILY FRIENDLY RESEARCHER POSTER SESSION

FRIDAY 6:30PM - 8:30PM

GRAND BALLROOM

ROTATE AROUND TO OVER 30 POSTERS PRESENTED BY RESEARCHERS

Meet Disney Characters: UP's Carl & Russell, Mike Wazowski & Sully, Woody, Jessie, Buzz Lightyear, Pluto



PJ PARTY AND MOVIE NIGHT

FRIDAY 7:30PM - 9:30PM

ADVENTURE LAWN

MOVIE FOR KIDS AND ADULTS ALIKE!

CURE SMA EVENING IN THE PARK

SATURDAY 4:30PM

DISNEYLAND PARK ENTRANCE TICKET TO BEGIN AT 4:30PM

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

FOR YOUR CHANCE TO WIN AN APPLE WATCH OR
2023 CONFERENCE SCHOLARSHIP



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

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

All participants who complete the surveys by 10:30am on Sunday, June 19th, 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, June 19th at the Closing General Session/It's a Wonderful Life. All participants who complete the surveys by 10:30am on Sunday, June 19th, 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 2023 Annual SMA Conference (the locations will be announced on Sunday), or a brand new Apple Watch!

You can also fill out the survey on-line at <https://www.surveymonkey.com/r/2022AnnualSMAConference>



Amy Marquez Scholarship

For the 2022 Annual SMA Conference, the Amy Marquez Scholarship is in its eighth 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 individuals with SMA. 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.





ADVOCACY IS A TEAM SPORT

Get Involved. Tell Your Story. Advocate for SMA Priorities.

The voices of individuals with SMA and their families are incredibly important when reaching out to lawmakers about issues important to the SMA community. Help break down barriers and promote independence of children and adults with SMA by becoming an advocate and advancing Cure SMA's *Greater Independence* legislative agenda:

1. **Access to Healthcare and Treatment:** promote the development of and timely access to SMA treatments, equipment, and other health-related services.
2. **Transition to Adulthood:** improve the transition from youth to adulthood for individuals with SMA and their families.
3. **Community Living:** promote independent community living for all individuals with SMA.
4. **Financial Independence:** promote financial independence and economic self-sufficiency for individuals with SMA.
5. **Disability Rights:** promote accessibility, equality of opportunity, and full integration and participation in all aspects of life for children and adults with SMA.

Start Your Advocacy While at the Cure SMA 2022 Conference!

Make a difference at the Cure SMA 2022 conference by scanning the QR code to send an advocacy message to your Members of Congress. While at the conference, educate your elected officials about SMA and the issues important to the SMA community.



It's easy and will only take a minute!
Be a part of something big.

CURE SMA LAUNCHES NEW ADVOCACY TOOL FOR SMA COMMUNITY



Cure SMA's new Advocacy Action Center makes it easier for individuals with SMA and others to contact state and federal officials on SMA community priorities from your phone or computer. Check out the new online advocacy tool and stay informed about opportunities to engage on issues important to children and adults with SMA and their communities. Check it out at: www.curesma.org/advocacy/actioncenter

www.CureSMA.org/Advocacy • email advocacy@curesma.org

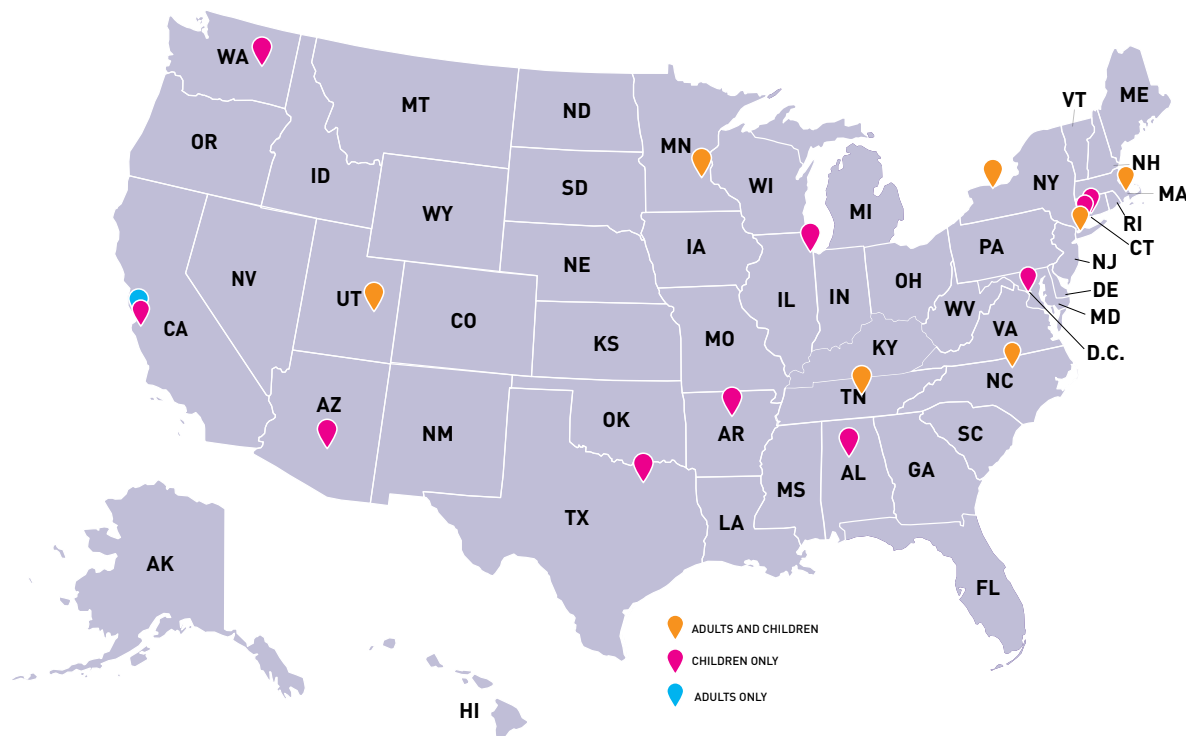
CURE SMA CARE CENTER NETWORK

The Cure SMA Care Center Network consists of 18 hospitals geographically dispersed throughout the U.S., representing SMA care for children and adults. These Care Centers—a collection of clinics partnering with Cure SMA—provide multidisciplinary care for people with SMA and contribute consented SMA patient electronic health record data to the SMA Clinical Data Registry.

More than 700 SMA patients are in the SMA Clinical Data Registry to date, with 73% pediatric and 27% adult SMA representation. The SMA Care Center Network will be expanding in 2022 to 29 centers! These additional centers will allow for increased adult representation. In addition, the Cure SMA Care Center Network aims to continue to recruit Care Centers with the goal of 2,500 SMA patients in the registry.

The Cure SMA Care Center Network's overall goal is to educate healthcare providers, and develop an evidence-based standard of care that will improve the lives of all those with SMA. The network and registry will achieve the following objectives:

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



Advocate Children's Hospital, Park Ridge, IL
 Arkansas Children's Hospital, Little Rock, AR
 Boston Children's Hospital, Boston, MA
 Children's of Alabama, Birmingham, AL
 Children's National Medical Center, Washington, DC
 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
 Phoenix Children's Hospital, Phoenix AZ
 Stanford Health, Palo Alto, CA
 Stanford Children's Health, Palo Alto, CA
 Seattle Children's Hospital, Seattle, WA

University of Rochester Medical Center, Rochester, NY
 The University of Utah, Program for Inherited Neuromuscular Disorders, SLC, UT
 University of Texas Southwestern/Children's Health, Dallas, TX
 Vanderbilt University Medical Center, Nashville, TN
 Yale Pediatric Neuromuscular Clinic, New Haven, CT

NEW CARE CENTERS COMING SOON! Visit www.curesma.org/sma-care-center-network/

CONFERENCE MEALS INCLUDED WITH REGISTRATION

THURSDAY

DINNER AT MEET & GREET:

DELI DINNER BUFFET—BUILD YOUR OWN DELI SANDWICHES, WATER.

FRIDAY

BREAKFAST ITEMS:

ASSORTED BAGELS & CREAM CHEESE, ORANGE JUICE AND COFFEE.

LUNCH:

BOXED LUNCHES HAVE AN ASSORTMENT OF CHOICES FOR ATTENDEES, AS SUPPLIES LAST. EACH ATTENDEE CAN HAVE 1 OF THE FOLLOWING SANDWICHES: TURKEY, HAM, VEGETARIAN, PB&J, OR A SOUTHWEST SALAD. 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 AND WATER.

SNACKS AT THE PJ PARTY AND MOVIE NIGHT:

POPCORN AND REFRESHMENTS.

SATURDAY

BREAKFAST ITEMS:

ASSORTED DANISHES & CROISSANTS, ORANGE JUICE AND COFFEE.

LUNCH:

BOXED LUNCHES HAVE AN ASSORTMENT OF CHOICES FOR ATTENDEES, AS SUPPLIES LAST. EACH ATTENDEE CAN HAVE 1 OF THE FOLLOWING SANDWICHES: TURKEY, HAM, VEGETARIAN, PB&J OR A CAESAR SALAD. PASTA SALAD, CHIPS, FRUIT AND A COOKIE ARE INCLUDED IN BOXED LUNCH. *Each choice is limited in number.*

SUNDAY

BREAKFAST ITEMS:

ASSORTED BAGELS & CREAM CHEESE, ORANGE JUICE AND COFFEE.

There will also be food carts available outside of the Disney Meeting Space for attendees to purchase food and beverages.

There are many dining locations and options available throughout the Disneyland Resorts and Downtown Disney. Options at the Disneyland Hotel include:

- The Coffee House
- Trader Sam's
- Tangaroa Terrace
- Goofy's Kitchen
- Mobile Dining through the Disneyland App

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

Erin Trainor Memorial Fund



For the past several years, the mission of the Erin Trainor Memorial Fund has been able to provide conference scholarships allowing newly diagnosed SMA families/ individuals the opportunity to attend The Annual SMA Conference. The Erin Trainor Memorial Fund (ETMF) has generated substantial funds sending hundreds of newly diagnosed families to conference.

“Erin would have been 27 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.”



In the past five 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 97% of all births, with the remaining wrapping up in 2022. This means that infants can now be diagnosed with SMA very early before symptoms appear, offering the pathway to early treatment. We are so proud of the impact our friends and family have had on increasing the quality of life for children like our daughter Erin. Had these treatments and screenings been available for Erin, our outcome would have been quite different indeed. 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.



Because of their commitment to assure that every newly diagnosed person has the best and finest care, the Erin Trainor Memorial Fund will match all gifts made to the Care Centers up to \$350,000 for a total of a \$700,000 investment into the network in order to make SMA treatments more accessible across the country. To learn more about this matching opportunity, please email Brett.kinley@curesma.org.

ERIN TRAINOR MEMORIAL FUND

Thank you to the Trainor Family

And everyone who has supported the newly diagnosed conference scholarship program and are now in this effort to support the opening of additional multi-disciplinary Care Centers across the country.

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 & Family



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

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





MEET & GREET AND FUN FEST

Grand Ballroom Thursday June 16th, 6:00pm – 8:30pm

CONFERENCE WELCOME

Introductions, carnival games, and prizes for all

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RESEARCHER RELAY RACE 7:00pm

.....

MEET DISNEY CHARACTERS

Meet Mickey & Minnie, Tiana & Louis, Lilo & Stitch,
Winnie the Pooh & Tigger





WEDNESDAY, JUNE 15TH | AGENDA

5:00PM – 7:30PM	Registration Open for all Conference Attendees	Disneyland Ballroom Registration Hallway
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THURSDAY, JUNE 16TH | AGENDA

9:00AM – 6:00PM	Registration Open for all Conference Attendees	Disneyland Ballroom Registration Hallway
12:00PM – 2:20PM Drop off opens at 11:30pm	Newly Diagnosed Children's Program <i>Sponsored by The Jacob Isaac Rappoport Foundation</i>	North Exhibit Hall
12:00PM – 2:20PM	Newly Diagnosed Program <i>(For Newly Diagnosed Families Only)</i>	South Ballroom
12:00PM – 12:15PM	Introduction to Cure SMA and the Community <i>**for newly diagnosed families only**</i> Kenneth Hobby, President Colleen McCarthy O'Toole, Vice President, Community Support Jessica Clark, Director, Community Support	South Ballroom
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 Carolyn and Rory Philstrom, SMA Parents	
1:35PM – 2:20PM	Newly Diagnosed Program Meet and Mingle Reception <i>**for newly diagnosed families only**</i>	
2:30PM – 6:00PM	Adults with SMA Lounge <i>Sponsored by Biogen</i> <i>**for adults with SMA and their attendants**</i>	
4:30PM – 6:00PM	Newborn Screening Meet & Mingle <i>Sponsored by Novartis Gene Therapies</i> <i>**for families identified through newborn screening**</i>	Sleeping Beauty Pavilion
6:00PM – 8:30PM	Meet & Greet Includes Researcher Relay Race and Games <i>Disney Characters: Mickey & Minnie, Tiana & Louis, Lilo & Stitch, Winnie the Pooh & Tigger</i>	Grand Ballroom
8:30PM – 10:30 PM	Dad's Night Out <i>**for dads only**</i>	Magic Kingdom Ballroom 1

AGENDA | FRIDAY, JUNE 17TH

7:00AM – 5:30PM	Registration Open for all Conference Attendees	Disneyland Ballroom Registration Hallway
7:30AM – 8:45AM	Annual SMA Conference Breakfast	South Exhibit Hall
7:30AM – 5:30PM	Exhibitor Tables Open	North Lounge, South Lounge & Center Lounge
7:45AM – 8:45AM	Annual SMA Conference Symposium, Sponsored by Biogen: Perspectives on SMA 	South Exhibit Hall
9:00AM – 12:30PM & 1:45PM – 5:00PM (DROP OFF OPENS AT 8:30AM & 1:15PM)	Children's Program <i>Sponsored by The Jacob Isaac Rappoport Foundation</i> 	North Exhibit Hall
9:00AM – 12:30PM & 1:45PM – 5:00PM (DROP OFF OPENS AT 8:30AM & 1:15PM)	Baby Room <i>Sponsored by The Jacob Isaac Rappoport Foundation</i> 	North Exhibit Hall
9:00AM – 10:30AM	Kids Talk it Out (Ages 11 – 17) Angela Wigglesworth, Elementary Education Teacher, SMA-Affected Adult Al Freedman, PhD, SMA Dad and Child/Family Psychologist This facilitated session will provide SMA-affected children (ages 11-17) an opportunity to talk with each other about their lives. To encourage our children to truly "Talk It Out", this session is open to children only and requires parental consent. <i>**please note, kids in this session will be escorted to the Children's Program in North Exhibit Hall when the session concludes at 10:30am. Please pick up your kids in North Exhibit Hall**</i>	South Ballroom
9:00AM – 10:30AM	Siblings Talk it Out (Ages 12 to 17) Katllyn O'Brien, Sister of an Adult with SMA Traci O'Brien, Sister of an Adult with SMA This workshop is for the siblings of brothers and sisters with SMA. With the unique relationship siblings have, we have much to offer one another. This workshop will create a fun and comfortable setting in which siblings can express themselves and their feelings, both positive and negative, of having a sibling with SMA through activities, games, and discussions. This workshop will be facilitated by SMA family siblings and other family members. It is geared for siblings between the ages of 12-17 years old. They will learn ways to cope and manage common situations that are experienced by brothers and sisters. Dress comfortably and be prepared to make friends and have fun! <i>**please note, kids in this session will be escorted to the Children's Program in North Exhibit Hall when the session concludes at 10:30am. Please pick up your kids in North Exhibit Hall**</i>	Adventure, in the Adventure Tower
9:00AM – 11:00AM	General Session Opening Remarks	Center Ballroom
10:45AM – 5:15PM	Adults with SMA Lounge <i>Sponsored by Biogen</i> <i>**for adults with SMA and their attendants**</i> 	Steakhouse 55



PLEASE NOTE

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

11:15AM – 12:30PM

Workshop Session #1

	<p>Aquatic Physical Therapy Question & 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>	Adventure Lawn
	<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 community. 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>	Center Ballroom
	<p>Using Muscle-Based Exercises for Speech Clarity and Feeding Renee Roy Hill, MS, CCC-SLP, COM@CLC</p> <p>This workshop is for participants who are having difficulty with oral control for feeding, speech, saliva control and/or oral rest posture. During this session, attendees will learn how using a muscle-based approach to oral function can assist in maintaining and improving motor function, reducing fatigue. This approach focuses on assessing the underlying motor system and developing a plan based on current function and can be used with all types of SMA and function levels.</p>	North Ballroom A
	<p>Women's Health in SMA Christina Han, MD Perry Shieh, MD</p> <p>This workshop will highlight the importance of routine gynecologic care for women with SMA and the management of common gynecologic problems. 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 for a woman with SMA is a multidisciplinary approach in a tertiary facility with familiarity in managing neuromuscular disorders.</p>	North Ballroom B
	<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>	South Ballroom
	<p>"Talk it Out" for Kids with an SMA-affected Parent (Ages 6- 17) Angela Wrigglesworth, 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>	Safari, in the Adventure Tower

12:30pm – 1:30pm

Annual SMA Conference Lunch

South Exhibit Hall


1:00pm

James Ian SPACES Performance

South Exhibit Hall

1:45pm – 3:15pm

Workshop Session #2

	<p>Taking Charge of Your Health: The Transition from Pediatric to Adult Care <i>Sponsored by Genentech</i> Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN, FPCN Bakri Elsheikh, MBBS, FRCP Sarah Stoney, MSW, LSW</p> <p> <i>A Member of the Roche Group</i></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>	North Ballroom A
	<p>The ABLE Act: EnABLE Savings for Life Jeanie Geraci</p> <p>This presentation will be educational and interactive. Jeanie will address what you need to know regarding The Able Act in your state and the Pros/Cons of having an Able Account. Topics of discussion will be: What is an Able Act, are you or your child eligible, the impact on other resources, how much money are you allowed, what type of accounts are allowed and how to set up an Able Account.</p>	Center Ballroom
	<p>Change Your Brain - Change Your Life The number one tip from a Disability Life Coach Amber Bosselman, 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 to learn and leave with a skill you can start implementing right now.</p>	South Ballroom
	<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 a seated and floor yoga practice demonstrating beneficial postures for SMA. We will practice a basic routine includes circle of song, eye exercises, postures, progressive relation and breathing exercises. Modifications and adaptations will be shared.</p>	North Ballroom B
	<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. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	Magic Kingdom Ballroom 1
	<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>	Magic Kingdom Ballroom 4
	<p>Aquatic Physical Therapy for Fun & Function, Children with Supported Head Control 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. Our focus will be on improving functional strength and skills in a dynamic environment. Games, stretching, toys and balance will all be discussed and demonstrated during our session. We look forward to seeing you in the water.</p>	Disneyland Hotel Center Pool

	<p>Using Our Breath for Big Emotions – Mindfulness for Kids Tara Davenport, SMA parent Claire Miles, SMA patient (x-linked)</p> <p>Caregivers and children: join us at this interactive workshop where we learn ways to use our breath to help us deal with big emotions. Tara and her ten-year-old daughter, Claire, share mindfulness ideas that can help when emotions begin to become overwhelming. The hope is to teach caregivers and children together so later we can remind each other of these fun techniques. This is best for children who are old enough to stay engaged for an hour of listening and participating in structured activities together. All types of SMA are welcome as we will discuss many different ideas of how to do these activities – even if we need assistance with breathing. Adults who are kids at heart are also welcome to join us!</p>	Safari, in the Adventure Tower
<p>3:30pm – 5:00pm Workshop Session # 3</p>		
	<p>Medical Management of Adults with SMA Tina Duong, MPT, PhDc Bakri Elsheikh, MBBS, FRCP</p> <p>This workshop will focus on three key areas: 1) Standards of Care related to the adult medical and rehabilitation management of SMA in the current natural history landscape. 2) We will discuss challenges related to obtaining 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>	South Ballroom
	<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. 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>	Center Ballroom
	<p>I Need to Cough, I Need to Breathe, I Need to Sleep Richard Kravitz, MD 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 sleep, illness and after surgery, and how to know when breathing support machines such as BIPAP should be considered. We will talk about the modalities available to use at home and in the hospital, as well as strategies to help your care team support respiratory health at home. There will be opportunities after the presentation to ask general questions about breathing complications and to share experiences that have improved the health of those with SMA. Bring your questions and experience to this interactive session we hope to have our whole pulmonary team there!</p>	North Ballroom A

<p>3:30PM – 5:00PM</p>	<p>Grandparents Coffee and Mingle Join fellow grandparents as you “mingle” together. Grab some coffee and meet other grandparents. Share stories, and photos and make new connections.</p>	<p>Magic Kingdom Ballroom 1</p>
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AGENDA | FRIDAY, JUNE 17TH

	<p>Aquatic Physical Therapy for Fun & Function, Children with Independent Head Control 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. Our focus will be on improving functional strength and skills in a dynamic environment. Games, stretching, toys and balance will all be discussed and demonstrated during our session. We look forward to seeing you in the water.</p>	Disneyland Hotel Center Pool
	<p>Adults with SMA Relationships Panel – Share Your Experience Kyla Pollock & Collin Pollock Rachel Stewart & Susan Stewart</p> <p>A spin off of the spouses and partners of those with SMA. This session is intended for the adult with SMA and their spouses, partners, and significant others to join and share their experiences and engage in discussion. Time will be allotted for all attendees to ask questions and contribute to the group discussion.</p>	North Ballroom B
	<p>Memory Boxes for Siblings: Creating a Lasting Treasure (for children only) Jennifer Lemisch, MA, ATR-BC, LPC</p> <p>Mourning the loss of a brother or sister with SMA is ongoing and can be a difficult process. Expression through grief activities often assists in restoration and healing for those experiencing the loss. This workshop will provide bereaved siblings an opportunity to make a memory box that will support the memories of their sibling and also learn about other creative legacy ideas to do on their own. Participants are welcome to bring a few 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>	Adventure, in the Adventure Tower
6:30pm – 8:30pm	<p>Family Friendly Researcher Poster Session Includes Disney Characters: UP's Carl & Russell, Mike Wazowski & Sully, Woody, Jessie, Buzz Lightyear, Pluto</p>	Grand Ballroom
7:30pm – 9:30pm	<p>PJ Party and Movie Night</p>	Adventure Lawn
7:30pm – 10:00pm	<p>Teen Social</p>	Safari
8:00pm – 10:00pm	<p>Adults with SMA Reception Sponsored by Biogen</p> 	South Ballroom
8:30pm – 10:30pm	<p>Mom's Night Out **for moms only**</p>	Sleeping Beauty Pavilion

FAMILY FRIENDLY RESEARCHER POSTER SESSION

FRIDAY JUNE 17 FROM 6:30PM TO 8:30PM

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





POSTERS INCLUDE:

- **UPDATES ON THE DEVELOPMENT OF THE SPINAL MUSCULAR ATROPHY: PERSON-REPORTED OUTCOME (SMA-PRO): A CAREGIVER AND SELF-PROXY PERFORMANCE MEASURE FOR CHILDREN AND ADULTS WITH SMA**
Amy Pasternak, PT, DPT, ATC, CSCS, Boston Children's Hospital, Boston, MA
- **APITEGROMAB CLINICAL TRIAL UPDATES**
Blair Orr, PhD, George Nomikos, MD, Jagdish Patel, PhD, Scholar Rock
- **RESILIENT: A NEW SMA CLINICAL TRIAL WITH A DUAL ACTING ANTI-MYOSTATIN**
Clifford Bechtold, M.S., Lia Donahue, M.A., Biohaven Pharmaceuticals, Inc
- **A NEW MUSCLE-DIRECTED TREATMENT APPROACH FOR SMA**
Didier Bagnol, PhD, Epirium Bio
- **SMN PROTEIN CONTROLS RNA MOLECULES: WHAT DO WE KNOW AND WHAT DO WE TRY TO FIND OUT**
Dmytro Morderer, PhD, Mayo Clinic
- **MANATEE: ROCHE AND GENENTECH CLINICAL STUDY OF GYM329 (RO7204239) IN COMBINATION WITH RISDIPLAM TREATMENT IN PEDIATRIC PATIENTS WITH SMA**
Donald Daudt, PhD, Genentech, South San Francisco, California
- **NEWBORN SCREENING FOR SMA**
Elizabeth Kichula, MD, PhD, Childrens Hospital of Philadelphia
- **HOW DO GLIA IMPACT MOTOR NEURON FUNCTION IN SPINAL MUSCULAR ATROPHY?**
Emily Welby, PhD, Allison Ebert, PhD, Medical College of Wisconsin
- **NMD670, A CHLORIDE ION CHANNEL INHIBITOR AS A NOVEL APPROACH TO IMPROVING SKELETAL MUSCLE FUNCTION FOR SPINAL MUSCULAR ATROPHY**
Eva R. Chin, PhD, NMD Pharma
- **DEVELOPMENT OF A COMPETENCY-BASED EDUCATIONAL PROGRAM FOR SPINAL MUSCULAR ATROPHY PHYSICAL THERAPISTS (STEP-IN)**
Jacqueline Montes, PT, EdD, Columbia University Irving Medical Center
- **THE SPINAL MUSCULAR ATROPHY PATIENT AND CAREGIVER-REPORTED HEALTH INDICES: DISEASE SPECIFIC OUTCOME MEASURES OF HOW INDIVIDUALS WITH SMA FEEL AND FUNCTION**
Jamison Seabury, BS, University of Rochester Center for Health + Technology
- **SMA FAMILIES FILMING INFANTS LEARNING MOVEMENT (SMA FAMILIES FILM) USING A SMARTPHONE APP AT HOME**
Kristin J Krossschell, PT, DPT, PCS, Northwestern University Feinberg School of Medicine, Chicago, IL
- **NEW RESEARCH AVENUES FOR THE TREATMENT OF SMA**
Laxman Gangwani, MTech, PhD, Texas Tech University Health Sciences Center, El Paso, TX
- **EVALUATING THE SMA CAREGIVER EXPERIENCE**
Lisa Belter, MPH, Cure SMA
- **HOW DO MOTOR NEURONS RECOVER AFTER TREATMENT IS GIVEN?**
Lyndsay Murray, PhD, University of Edinburg
- **TRANSITION PLAN IMPLEMENTATION AND PROVIDER ENGAGEMENT FOR ADULTS AFFECTED BY SPINAL MUSCULAR ATROPHY (SMA)**
Lynn Colegrove, BS, MBA, Cure SMA
- **IMPLEMENTATION OF SPINAL MUSCULAR ATROPHY NEWBORN SCREENING ACROSS THE U.S.**
Mary Schroth, MD, Cure SMA
- **THE SMA BULBAR SURVEY: WHAT WE'VE LEARNED AND FUTURE DIRECTIONS**
Maureen A. Lefton-Greif, PhD, CCC-SLP, BCS-S, Johns Hopkins School of Medicine, Baltimore, MD
- **BULBAR FUNCTION IN PATIENTS WITH SPINAL MUSCULAR ATROPHY TYPE 1**
Nicole LaMarca, DNP, MSN, CPNP, PMHS, Novartis Gene Therapies, Inc., Bannockburn, IL, USA
- **WHAT POWERS CURE SMA?**
Pam Swenk, Chief of Staff, Cure SMA
- **THE SAFETY AND EFFICACY OF SPINRAZA IN INFANTS AND CHILDREN**
Sarah Jennison Sr. Director of Family Access, Rare Disease Biogen, Biogen
- **UNDERSTANDING THE DIAGNOSTIC AND TREATMENT JOURNEY FOR INDIVIDUALS WITH SMA**
Sarah M Whitmire, MS, Cure SMA
- **TUBE FEEDING FORMULAS USED BY PATIENTS WITH SMA: EXPERIENCE FROM 3 INSTITUTIONS**
Stacey Tarrant, RD, LDN, Boston Children's Hospital
- **A NEW SMALL MOLECULE THAT IMPROVES NEUROMUSCULAR STRENGTH IN SPINAL MUSCULAR ATROPHY MODEL MICE**
Stephen Meriney, PhD, University of Pittsburgh
- **SPINRAZA PIVOTAL DATA IN LATER-ONSET AND INDEPENDENT, OBSERVATIONAL, DATA IN ADULTS**
Sundip Raval, PharmD, Executive Director of Rare Disease Marketing Biogen, Biogen
- **COPI: A CARGO TRAIN HELPING TO BUILD NEURONS**
Timra Gilson, PhD, Elliot Androphy, MD, Indiana University School of Medicine
- **RAINBOWFISH: ROCHE AND GENENTECH CLINICAL STUDY OF RISDIPLAM TREATMENT IN INFANTS WITH GENETICALLY DIAGNOSED, PRESYMPTOMATIC SMA**
Travis L. Dickendesher, PhD, Genentech, South San Francisco, California



**MEET
DISNEY CHARACTERS**

SATURDAY, JUNE 18TH | AGENDA

7:30am – 4:30pm	Registration Open for all Conference Attendees	Disneyland Ballroom Registration Hallway
7:30am – 8:45am	Annual SMA Conference Breakfast	South Exhibit Hall
7:30am – 4:30pm	Exhibitor Tables Open	North Lounge, South Lounge & Center Lounge
7:45am – 8:45am	Annual SMA Conference Symposium, Sponsored by Genentech: Progress in SMA: Perspectives on Life, Choices and Taking Action  <small>A Member of the Roche Group</small>	South Exhibit Hall
9:00am – 12:15pm & 1:15pm – 4:15pm <small>(drop off opens at 8:30am & 12:45pm)</small>	Children's Program <i>Sponsored by The Jacob Isaac Rappoport Foundation</i> 	North Exhibit Hall
8:45am – 12:00pm & 1:15pm – 4:15pm <small>(drop off opens at 8:30am & 12:45pm)</small>	Baby Room <i>Sponsored by The Jacob Isaac Rappoport Foundation</i> 	North Exhibit Hall
9:00am – 10:30am	Workshop Session #4	
	Orthopedic Management Brian Snyder, MD, PhD Samuel Rosenfeld, MD Anne Stratton, MD <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>	Center Ballroom
	Driving and Community Mobility <i>Sponsored by Genentech</i> Anne Hegberg, OTR/L, CDRS, CDI <p>Community mobility is necessary to access school and work as well as leisure and social activities. When young, parents transport the wheelchair user. But, like any other teen, driving is a common goal wheelchair or not. This presentation will address: (1) safe securement when being transported in a wheelchair. (2) issues to be addressed prior to purchasing a vehicle for adaptation (3) current trends in adaptive driving equipment (4) the importance of an evaluation by a qualified driver rehabilitation specialist (5) funding possibilities</p>  <small>A Member of the Roche Group</small>	North Ballroom B
	Keepsake Creation: Grieving Through Art Jennifer Lemisch, MA, ATR-BC, LPC <p>Mourning the loss of a loved one with SMA is ongoing and can be a difficult process. Expression through grief activities often assists in restoration and healing for those experiencing the loss. This workshop will provide bereaved parents an opportunity to make a keepsake object that will support the memories of their child and also learn about other creative legacy ideas to do on their own. Participants are welcome to bring a few 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>	Adventure, in the Adventure Tower

	<p>Unpacking Clinical Trials Edward Smith, MD Thomas Crawford, MD Perry Shieh, MD</p> <p>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.</p>	South Ballroom
	<p>Young Adults Talk it Out (Ages 18+) 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 young adults (ages 18+) an opportunity to talk with each other about their lives.</p>	North Ballroom A
	<p>Genetics and Reproductive Options for SMA Families 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>	Adventure Lawn

<p>9:00am – 4:15pm</p>	<p>Adults with SMA Lounge <i>**for adults with SMA and their attendants**</i> Sponsored by Biogen</p>	Steakhouse 55
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<p>10:45am – 12:15pm</p>	<p>Workshop Session # 5</p>	
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	<p>Navigating the Special Education Process: Setting Our Kids up for Success Kimberly Cook, SMA Parent Tina Lewis, SMA Parent</p> <p>Special education can be intimidating for parents entering a whole new world of acronyms, timelines, and IEP goals. Knowing what you can expect for your child and asking for appropriate services and accommodations can be difficult. Join our session to learn the ins and outs of special education. Hear the stories of three students and the accommodations and services that benefited them. Question and answer session to follow.</p>	North Ballroom B
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	<p>An Approach to Exercise in Individuals with SMA Tina Duong, MPT, PhDc Leslie Nelson, MPT, PhDc, OCS Anne Stratton, MD Jennifer Hubbell, PT</p> <p>The goal of this session is to provide education on exercise guidelines and options to support an active lifestyle in participants of all ages and types of SMA. Information will be based on current exercise literature in SMA and expert clinical opinion. This session will help you better understand the basic physiology of exercise and how it may impact you. To ensure the session encompasses a wide range of physical abilities, we will also provide alternatives and modifications for all levels of fitness as well as discuss relevant medical considerations and precautions prior to initiating an exercise program. We will help provide options and resources for various forms of group and individual exercise. The focus of this session will center around personalized exercise to meet your personal activity goals. This session is ideal for families and participants interested in learning more about initiating and progressing exercise activity throughout the lifespan.</p>	South Ballroom
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SATURDAY, JUNE 18TH | AGENDA

	<p>Special Plans for a Special Life, Estate Planning with Supplemental Needs Trusts Richard Rubenstein, Esq.</p> <p>Legal and financial planning that parents must undertake to protect their family can be difficult and confusing. It is vital for all parents to have an estate plan in place, more so for parents of children with disabilities. Find out how to assure that money you set aside for your child with special needs does not jeopardize government benefits your child is entitled to receive. Richard will give a general overview of the basics of estate planning and the differences between the various types of Special Needs Trusts. Learn how to prepare a letter of intent, an important and often overlooked document. A document you must create to provide all the specialized knowledge required, preparing the person you chose to care for your child in your absence; this document also helps that person interpret your hopes and desires for your child's future. Finally, Richard will discuss various techniques to advocate on behalf of your child in order maximize resources from insurance companies and government agencies. Parents must go through these difficult steps to ensure a well-planned future for their special child.</p>	Center Ballroom
	<p>Hold It No More: A Panel Discussion on Suprapubic and Mitrofanoff Catheterization Options Emily Wolinsky, Adult with SMA Rachel Stewart, Adult with SMA Andraéa LaVant, Adult with SMA</p> <p>If you're curious about living life and peeing quite freely with little interruption in your daily routine, this workshop will provide information about two options for eliminating pee math and increasing your entire sense of independence: Supra Pubic Catheters (SPCs) and Mitrofanoff procedure. You will learn from your peers directly about the pros and cons, the ins and outs, and the ups and downs of life with a SPC or Mitrofanoff.</p>	North Ballroom A
	<p>Benefits of Adaptive Recreation and Cycling Ed Bronsdon</p> <p>Many people can easily recall what it felt like to first learn to ride a bike. The struggle, the challenge – but then ultimately the achievement of being able to do something that they just didn't think would be possible, of belonging and of succeeding! Unfortunately, too many people with disabilities may not believe that they can be successful cycling...but they can! This workshop will provide a mix of information, demonstration, perspiration and inspiration for everyone involved about the benefits of adaptive cycling.</p>	Adventure Lawn
	<p>Aquatic Physical Therapy for Fun & 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 90 minutes with a team of aquatic physical therapists. Our focus will be on improving functional strength and skills in a dynamic environment. Games, stretching, toys and balance will all be discussed and demonstrated during our session. We look forward to seeing you in the water.</p>	Disneyland Hotel Center Pool
	<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 with bereavement and feelings of loss.</p>	Magic Kingdom Ballroom 4


12:15pm – 1:15pm

Annual SMA Conference Lunch

South Exhibit Hall

1:15pm – 2:45pm

Workshop Session # 6

<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, games, and discussions. This workshop will be facilitated by SMA family siblings and other family members. It is geared for siblings between the ages of 5-11 years old. They will learn ways to cope and manage common situations that are experienced by brothers and sisters. Dress comfortably and be prepared to make friends and have fun!</p>		Adventure, in the Adventure Tower
<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; distinguish 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. 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.</p>		Center Ballroom
<p>How to Build Your Presence and Advocate Through Writing & Social Media <i>Sponsored by Genentech</i></p> <p>Kevin Schaefer, Adult with SMA Allie Williams, Adult with SMA Shane Burcaw, Adult with SMA Brianna Albers, Adult with SMA Kevan Chandler, Adult with SMA</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&A will follow the main conversation.</p>		North Ballroom B
<p>The Caregiver's Toolbox (Essentials) Wendy Godfrey</p> <p>Self-care is crucial for caregivers and is often the first thing that "has to give" due to time, money, and other resources. Please join Wendy Godfrey for The Caregiver's Toolbox workshop where we will explore simple, easy, and accessible self-care strategies for you to build and start to fill your Toolbox with ways to help minimize stress and improve your wellness!</p>		Adventure Lawn
<p>A Lifetime of Good Nutrition 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. Finally, at least one young adult with SMA will speak about their unique nutritional challenges.</p>		South Ballroom




SATURDAY, JUNE 18TH | AGENDA

	<p>What it Means to be a Teen on Wheels Angela Wrigglesworth, Elementary Education Teacher, Adult with SMA</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>	North Ballroom A
<p>3:00pm – 4:15pm Workshop Session # 7</p>		
	<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 lead and moderated by Shonna but 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><i>WARNING: the conversation will be frank and is best suited for those who are of an appropriate age for dating and discussions around relationships and sexuality. There will likely be non-traditional ideas discussed and we ask that you attend with an open, non-judgmental disposition.</i></p>	North Ballroom B
	<p>Early Scoliosis Management in SMA Samuel Rosenfeld, MD Brian Snyder, MD</p> <p>This workshop will focus on care of early scoliosis with SMA including monitoring and management considerations and strategies.</p>	Center Ballroom
	<p>Kids Talk it Out (Ages 6 – 10) 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 6-10) 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>	North Ballroom A
	<p>University of Utah TRAILS Program: Adaptive Sports and Novel Adaptive Equipment Melissa McIntyre, DPT Casey Fenger, MD</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>	Adventure Lawn

AGENDA | SATURDAY, JUNE 18TH

	<p>Finding Hope After Loss: Helping Parents and Children Cope Audra Perry Butler, SMA Parent</p> <p>When a family loses a child, the grief is profound, and the family dynamic is instantly changed. In this workshop, we will discuss how parents can help their family navigate the grief journey, from being consumed by the loss to cherishing the memories. Created by retired psychologist Dr. Glenn G. Perry, Jr. and facilitated by SMA parent Audra Perry Butler, the workshop will examine the reactions and emotions created by grief, some of the ways that mothers and fathers grieve differently, and specific coping strategies for parents. We also will review how children process grief and give you ways to help your surviving children understand their feelings. Finally, we will provide ideas to help your family find ways to honor and remember your child, as your family moves forward and rediscovers hope.</p>	Safari, in the Adventure Tower
	<p>Emergencies – Anticipation, Prevention and Readiness 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>	South Ballroom
4:30pm	Cure SMA Evening in the Park	Disneyland Park
5:00pm	Cure SMA Conference Photo! Meet in front of the castle at the Disneyland Park wearing your conference t-shirts!	Disneyland Park

AGENDA | SUNDAY, JUNE 19TH

7:30am – 8:45am	Annual SMA Conference Breakfast	South Exhibit Hall
7:45am – 8:45am	<p>Annual SMA Conference Symposium, Sponsored by Novartis Gene Therapies: Gene Therapy: Targeting the Genetic Route Cause</p> 	South Exhibit Hall
9:00am – 11:15am (drop off opens at 8:30am)	<p>Children's Program Sponsored by The Jacob Isaac Rappoport Foundation</p> 	North Exhibit Hall
9:00am – 11:15am (drop off opens at 8:30am)	<p>Baby Room Sponsored by The Jacob Isaac Rappoport Foundation</p> 	North Exhibit Hall
9:00am – 11:15am	It's a Wonderful Life/Closing General Session	Center Ballroom



Saturday Evening at the Disney Theme Park

You are invited to join us on Saturday for an evening of fun at the Disneyland Park. All registered attendees will receive a complimentary park entrance ticket for anytime after 4:30pm.

We thank Biogen, Genentech, and Novartis Gene Therapies for their support and sponsorship of this wonderful Cure SMA Evening at the Park. This event will be a highlight of this year's Annual SMA Conference.

Cure SMA Conference Photo at 5pm! Meet in front of the castle at the Disneyland Park wearing your conference t-shirts!





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!

RICHARD & JANE NUNEMAKER RESEARCH MATCH



Join the Nunemaker Family in their 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. They are matching all research gifts focused on these findings up to \$250,000.

Thank you to the Nunemakers for investing in the future of SMA.

To learn more about this matching opportunity, please email Brett.kinley@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 17TH 2022

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!

11:00AM-12:00PM

Adventure Magic Show California Joe will take the kids on a magical journey around the world! The California Joe Adventure Magic Show is highly interactive with loads of audience participation. The kids get to help every step of the way!

CHILDREN'S PROGRAM AFTERNOON SESSION

1:45PM-5:15PM

1:45PM-5:15PM

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!

2:30PM-3:30PM

Comedy Magic & Juggling Variety Show With a wit faster than his hands, 'Abbit The Average' combines hilarious jaw-dropping magic with almost-nothing-dropping comedy juggling. He even tosses in some physical and prop comedy. This high-energy performance is packed with non-stop laughs and audience participation for a one-of-a-kind entertainment experience!

Sponsored by



SATURDAY JUNE 18TH 2022

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!

10:30AM-11:30AM

The Science of Magic Do you ever wonder how Magicians do some of their tricks? SPOILER ALERT! In this presentation, Great Scott the Glad Scientist breaks down several magic tricks using common scientific principals. Topics include Chemistry, Optical Illusions, Math, and Physics. Lots of laughs and audience participation.

CHILDREN'S PROGRAM AFTERNOON SESSION

1:15PM-4:15PM

1:15PM-4:15PM

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!

2:00PM-3:00PM

That's Science What is Science and what does it take to be a REAL SCIENTIST? It's not as hard as you might think! Come learn the secret through participating with Great Scott in the Scientific Method while doing fun and intriguing experiments in physics, chemistry, and properties of matter.

SUNDAY JUNE 19TH 2022

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!



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 pickup 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, so please take advantage.



THANK YOU TO OUR VOLUNTEERS



Thank You to The Conference Children's Program Volunteers

Each and every one of our volunteers has a major impact on the atmosphere and success of our conference, and every year their impact radiates throughout the weekend! Their long days of hard work provide families with the reassurance that their children are well cared for and are having fun, while they attend workshops, learning the latest information on SMA. The enthusiasm they provide for the kids in the children's program, during the carnival games, and their expertise to make this conference at our other events are contagious. Our volunteers take great pride in helping to ensure that at each conference they attend, families leave with the feeling that this was the best conference yet. Without you all, our conference would be nowhere near what it encompasses today. Thank you for everything!

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



VOLUNTEER FOR 32 YEARS

George Ghorbanian
Chicago Police Officer



VOLUNTEER FOR 30 YEARS

Patti Slojkowski
Dental Hygienist



VOLUNTEER FOR 27 YEARS

Steve Smith
Physical Therapist



VOLUNTEER FOR 23 YEARS

Kelly Milito
Hair Stylist



VOLUNTEER FOR 21 YEARS

Katlyn O'Brien
Elementary School Teacher

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 20 YEARS

Traci O'Brien
Marketing Consultant



VOLUNTEER FOR 11 YEARS

Donna Budil
Preschool Director



VOLUNTEER FOR 20 YEARS

Noreen Reilly
Data Analyst



VOLUNTEER FOR 10 YEARS

Hayley Miller



VOLUNTEER FOR 15 YEARS

Julie Smith



VOLUNTEER FOR 10 YEARS

Jennifer Walsh



VOLUNTEER FOR 13 YEARS

Jackie Staples



VOLUNTEER FOR 10 YEARS

Kelly Gibbons
Client Success Manager



VOLUNTEER FOR 12 YEARS

Patrick Hogan
High School History and
Psychology Teacher



VOLUNTEER FOR 10 YEARS

Kayla Forsythe



VOLUNTEER FOR 11 YEARS

Megan Milito



VOLUNTEER FOR 10 YEARS

Lexi Basso
Nurse



VOLUNTEER FOR 11 YEARS

Joy Martin
Physical Therapist



VOLUNTEER FOR 9 YEARS

Georgia Slojkowski
Elementary School Music
Teacher



VOLUNTEER FOR 11 YEARS

Mike Graney
Vice President of United services



VOLUNTEER FOR 9 YEARS

Laura Kroll
Elementary School Teacher

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 8 YEARS

Megan Besler



VOLUNTEER FOR 6 YEARS

Alanna Woods



VOLUNTEER FOR 7 YEARS

Daniela Milito



VOLUNTEER FOR 5 YEARS

Isabella Hernandez



VOLUNTEER FOR 7 YEARS

Michael Milito
Engineer



VOLUNTEER FOR 5 YEARS

Katie Dieckhoff



VOLUNTEER FOR 7 YEARS

Raphi Milito
Sales Specialist



VOLUNTEER FOR 5 YEARS

Samantha Smith



VOLUNTEER FOR 7 YEARS

Ralph Milito
Real Estate Agent



VOLUNTEER FOR 5 YEARS

Cara Freedman



VOLUNTEER FOR 7 YEARS

Sally McCarthy
Retired Nurse



VOLUNTEER FOR 4 YEARS

Clara Dylewski



VOLUNTEER FOR 6 YEARS

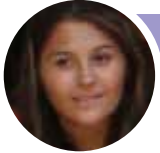
Rebeka Nekolova

THANK YOU TO OUR VOLUNTEERS



VOLUNTEER FOR 4 YEARS

Elizabeth Garvey



VOLUNTEER FOR 4 YEARS

Julia Kafkes



VOLUNTEER FOR 4 YEARS

Will Reilly



VOLUNTEER FOR 4 YEARS

Morgan Ramirez



VOLUNTEER FOR 4 YEARS

Anne Wallo



VOLUNTEER FOR 3 YEARS

Dan Melko



VOLUNTEER FOR 3 YEARS

Emma Parker



THANK YOU TO OUR VOLUNTEERS



Patti Slojkowski | VOLUNTEER FOR 30 YEARS

The 2022 Annual SMA Conference marks a huge milestone for one of our longest returning volunteers, Patti Slojkowski! Patti has volunteered at conference shortly after the Children's Program started back in 1990 making this her 30th conference! Every year she takes time away from her job as a dental hygienist as well as from her family to travel each year and support the Children's Program. Patti is the true definition of a dedicated volunteer. Everyone at Cure SMA would like to thank Patti for all her hard work and dedication over the last 30 years!

THANK YOU TO OUR VOLUNTEERS



Kelly Milito, Hair Stylist
VOLUNTEER FOR 23 YEARS

Katlyn O'Brien, Elementary School Teacher
VOLUNTEER FOR 21 YEARS



Traci O'Brien, Marketing Consultant
VOLUNTEER FOR 20 YEARS

Noreen Reilly, Data Analyst
VOLUNTEER FOR 20 YEARS



The 2022 Annual SMA Conference also marks a big milestone for a few of our long-standing volunteers! Each of them has just surpassed 20 years of devoted service to Cure SMA! Each year these wonderful ladies take time away from work and their families to come out and volunteer in the Children's Program. Everyone at Cure SMA would like to thank Kelly, Katlyn, Traci and Noreen for their dedicated service!

MOVING AHEAD

Evrysdi
keeps working
so we can
keep reaching



Join us for an engaging event

SATURDAY, JUNE 18 | 7:45AM - 8:45AM PT

- ✓ Hear directly from **community members**
- ✓ Hear directly from a **neurologist** about their experiences with **Evrysdi**

What is Evrysdi?

Evrysdi is a prescription medicine used to treat spinal muscular atrophy (SMA) in adults and children 2 months of age and older.

It is not known if Evrysdi is safe and effective in children under 2 months of age.

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
- 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.

Stop by our exhibit booth

FRIDAY, JUNE 17
7:30AM - 5:00PM PT

SATURDAY, JUNE 18
7:30AM - 4:30PM PT



Be sure to pick up a **complimentary bag**

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, upper respiratory infection (runny nose, sneezing, sore throat, and cough), lung infection, constipation, vomiting

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/MovingAhead to learn more



Patient Information
EVERYSID[®] [ev-RIZ-dee]
(risdiplam)
for oral solution

<p>What is EVERYSID?</p> <ul style="list-style-type: none"> EVERYSID is a prescription medicine used to treat spinal muscular atrophy (SMA) in adults and children 2 months of age and older. It is not known if EVERYSID is safe and effective in children under 2 months of age. 	<p><i>Reusable Oral Syringes</i></p> <ul style="list-style-type: none"> Your pharmacist will provide you with the reusable oral syringes 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 syringes provided by your pharmacist (you should receive 2 identical oral syringes) to measure your or your child's dose of EVERYSID, as they are designed to protect the medicine from light. Contact your healthcare provider or pharmacist if your oral syringes are lost or damaged. Once transferred from the bottle to the oral syringe, take EVERYSID right away. Do not store the EVERYSID solution in the syringe. If EVERYSID is not taken within 5 minutes of when it is drawn up, EVERYSID should be thrown away from the reusable oral syringe, and a new dose should be prepared.
<p>Before taking EVERYSID, tell your healthcare provider about all of your medical conditions, including if you:</p> <ul style="list-style-type: none"> 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. EVERYSID may harm your unborn baby. are a woman who can become pregnant: <ul style="list-style-type: none"> Before you start your treatment with EVERYSID, your healthcare provider may test you for pregnancy. Because EVERYSID may harm your unborn baby, you and your healthcare provider will decide if taking EVERYSID 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 EVERYSID. are an adult male planning to have children: EVERYSID 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 EVERYSID 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 EVERYSID. <p>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 and pharmacist when you get a new medicine.</p>	<p>What are the possible side effects of EVERYSID? The most common side effects of EVERYSID include:</p> <ul style="list-style-type: none"> For later-onset SMA: <ul style="list-style-type: none"> fever diarrhea rash For infantile-onset SMA: <ul style="list-style-type: none"> fever runny nose, sneezing, sore throat, and cough (upper respiratory infection) diarrhea lung infection rash constipation vomiting <p>These are not all of the possible side effects of EVERYSID. 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.</p>
<p>How should I take EVERYSID? See the detailed Instructions for Use that comes with EVERYSID for information on how to take or give EVERYSID oral solution.</p> <ul style="list-style-type: none"> You should receive EVERYSID 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. If the medicine in the bottle is a powder, do not use it. Contact your pharmacist for a replacement. Avoid getting EVERYSID on your skin or in your eyes. If EVERYSID gets on your skin, wash the area with soap and water. If EVERYSID gets in your eyes, rinse your eyes with water. <p><i>Taking EVERYSID</i></p> <ul style="list-style-type: none"> Your healthcare provider will tell you how long you or your child needs to take EVERYSID. Do not stop treatment with EVERYSID unless your healthcare provider tells you to. For infants and children, your healthcare provider will determine the daily dose of EVERYSID needed based on your child's age and weight. For adults, take 5 mg of EVERYSID daily. <ul style="list-style-type: none"> Take EVERYSID exactly as your healthcare provider tells you to take it. Do not change the dose without talking to your healthcare provider. Take EVERYSID 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 EVERYSID has been completely swallowed. Do not mix EVERYSID with formula or milk. If you are unable to swallow and have a nasogastric or gastrostomy tube, EVERYSID can be given through the tube. If you miss a dose of EVERYSID: <ul style="list-style-type: none"> If you remember the missed dose within 6 hours of when you normally take EVERYSID, then take or give the dose. Continue taking EVERYSID at your usual time the next day. If you remember the missed dose more than 6 hours after you normally take EVERYSID, 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 EVERYSID to make up for that dose. Wait until the next day to take the next dose at your usual time. 	<p>How should I store EVERYSID?</p> <ul style="list-style-type: none"> Store EVERYSID in the refrigerator between 36°F to 46°F (2°C to 8°C). Do not freeze. Keep EVERYSID in an upright position in the original amber bottle to protect from light. Throw away (discard) any unused portion of EVERYSID 64 days after it is mixed by the pharmacist (constitution). Please see the Discard After date written on the bottle label. (See the Instructions for Use that comes with EVERYSID). <p>Keep EVERYSID and all medicines out of the reach of children.</p> <p>General information about the safe and effective use of EVERYSID. Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use EVERYSID for a condition for which it was not prescribed. Do not give EVERYSID 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 EVERYSID that is written for health professionals.</p> <p>What are the ingredients in EVERYSID? Active ingredient: risdiplam Inactive ingredients: ascorbic acid, disodium edetate dihydrate, isomalt, mannitol, polyethylene glycol 6000, sodium benzoate, strawberry flavor, sucralose, and tartaric acid.</p> <p>Genentech <i>A Member of the Roche Group</i></p> <p>EVERYSID[®] (risdiplam)</p> <p>Distributed by: Genentech, Inc. A Member of the Roche Group 1 DNA Way South San Francisco, CA 94080-4990</p> <p>EVERYSID is a registered trademark of Genentech, Inc. M-US-00007143(v2.0) ©2021 Genentech, Inc. All rights reserved.</p> <p>For more information, go to www.EVERYSID.com or call 1-833-387-9734.</p>

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

Approved: 4/2021

OFFICIAL CURE SMA MERCHANDISE

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

Brand New Items Debuting at Conference!

Items Sneak Peek:

- Men, Women, and Youth Apparel
- Onesies
- Doggie Tanks
- Stickers
- Promo Items
- Bags
- Hats
- Drinkware
- Accessories
- Awareness Kits



Share a photo on your social media of you sporting your Cure SMA gear during this week's Conference, using the hashtag #2022SMACONFERENCE

CURE SMA MERCHANDISE PRICING AND SIZE GUIDE

Accessories:

- Awareness Kit- \$55
- Birch Ornament- \$15
- Embroidered Plush Blanket- \$25
- Fact Cards (Pack of 25)- \$5
- Hand Blown Glass Ornament- \$15
- License Plate Frame- \$20
- Push Pop Fidget Game- \$8
- Adult Reusable Face Masks (Purple, White, Black)- \$10
- Youth Reusable Face Masks (Purple, White, Black)- \$10
- Vinyl Decal- \$5
- Adult Bracelet- \$2
- Youth Bracelet- \$2
- Notecards- \$15
- Popsocket- \$10
- Temporary Tattoos (Pack of 25)- \$6
- Tuffy the Plush Puppy- \$20
- We Can Cure SMA Doggie Tank- \$20 (Xsmall-2XL)
- Luggage Tag- \$12

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

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

Drinkware:

- Purple Water Bottle- \$15
- Stainless Steel Wine Tumbler- \$20
- Travel Mug- \$15

Bags:

- Backpack- \$30
- Lunch Cooler- \$20
- Purple Zippered Tote- \$20
- Canvas Zippered Pouch- \$14
- Canvas Tote- \$20

Hats:

- Striped Pom Beanie- \$25
- Gray Adjustable Baseball Hat- \$20
- Black Performance Baseball Hat- \$20
- Gray Bucket Hat- \$20

Toddler & Youth Apparel:

- White Onesie- \$18 (6mon, 18mon & 24mon)
- Baseball Tee Onesie- \$18 (6mon, 12mon, 18mon & 24mon)
- We Can Cure SMA Onesie- \$18 (6mon, 12mon, 18mon & 24mon)
- Youth Grey Crew Neck Tee- \$24 (Small-XLarge)
- Toddler Grey Crew Neck Tee- \$24 (2T-4T)
- Youth Est 1984 Purple Jersey Tee- \$24 (XSmall-XLarge)
- Youth Baseball Tee- \$25 (Small-XLarge)
- Youth We Can Cure SMA Crewneck Sweatshirt- \$25 (XSmall-XLarge)

Adult Apparel:

- Men's Est 1984 Purple Jersey Tee- \$24 (Small-3XLarge)
- Men's White Performance Polo- \$28 (Small-4XLarge)
- Men's Performance Half Zip- \$49 (Small-3XLarge)
- Unisex Full Zip Purple Hoodie- \$49 (XSmall-2XLarge)
- Unisex Full Zip Black Performance Fleece- \$49 (XSmall-4XLarge)
- Unisex Grey Crew Neck Tee- \$24 (XSmall-4XLarge)
- Unisex Orange V-Neck Tee- \$24 (XSmall-2XLarge)
- Unisex Microfleece Full-Zip Jacket- \$49 (XSmall-3XLarge)
- Unisex V-Neck Purple Scrub Top- \$25 (Small-3XLarge)
- Unisex Baseball Tee- \$25 (Small-2XLarge)
- Unisex We Can Cure SMA Crewneck Sweatshirt- \$30 (Small-3XLarge)
- Windbreaker Full Zip Jacket- \$35 (XSmall-3XLarge)
- Women's Est. 1984 Purple Jersey Tee- \$24 (Small-2XLarge)
- Women's Purple Performance Polo- \$28 (XSmall-4XLarge)
- Women's Performance Half Zip - \$49 (Small-4XLarge)
- Women's Performance Racerback Tank- \$25 (XSmall-4XLarge)
- Women's White Reflective Performance Half Zip- \$49 (XSmall-4XLarge)

SUPPLIES ARE LIMITED

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

5 YEARS
OF POSSIBILITY

SPINRAZA[®]
(nusinersen) injection
12 mg/5 mL

CAMERON // AGE 4
EARLY-ONSET SMA
TREATED WITH SPINRAZA

LAUREN // AGE 20
LATER-ONSET SMA
TREATED WITH SPINRAZA

For those treated with SPINRAZA worldwide*— victories are personal.

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



11,000+ people
treated and counting*



From 3 days[†] to 80 years old^{‡§}
there's someone from almost every age group
who has been treated with SPINRAZA.



5 years of treating
people with SMA

*Based on commercial patients, early access patients, and clinical trial participants through September 2021.

†Includes clinical trial patients.

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

§Based on commercial patients in the US (including Puerto Rico) through December 2020.

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 full Prescribing Information on SPINRAZA.com.

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

See Important Facts About SPINRAZA on the next page.

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225 Binney Street, Cambridge, MA 02142



Attend our symposium to learn about how SPINRAZA treats presymptomatic and later onset SMA.

JUNE 17, 7:45-8:45 AM



CAMDEN // AGE 4
PRESYMPTOMATIC SMA
TREATED WITH SPINRAZA

Visit us at our booth

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



SHARE

YOUR PAST CONFERENCE EXPERIENCE

As we gear up for this years Annual SMA Conference, we wanted to share some throwback photos from past conferences! Cure SMA hosted the first Annual SMA Conference in 1988.



We love to see your throwback photos as well, so please email them to communitysupport@curesma.org for a chance to be featured in the next issue of Directions!



Tag us on social **#2022SMAConference**





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 Patient Resource Managers (PRMs) 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.
- 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. Contact the patient's doctor immediately if you see 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 occur approximately one week after ZOLGENSMA infusion. Caregivers should 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 respiratory syncytial virus (RSV) is recommended.

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 bodily waste for 1 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.



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Bannockburn, IL 60015

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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.
- 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.

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 occur approximately one week 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.

Vaccination schedule should be adjusted where possible to accommodate treatment with an oral corticosteroid. Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed during corticosteroid use. Protection against respiratory syncytial virus (RSV) is recommended.

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

Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with bodily waste for 1 month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with 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 your doctor and visit www.ZOLGENSMA.com for Full Prescribing Information.

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Novartis Gene Therapies, Inc.
Bannockburn, IL 60015

02/2022 US-ZOL-20-0121 V3



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

**we proudly support
cure sma**

**congratulations
on another
successful conference**

expo

convention
contractors



proudly supports



**Make today a
breakthrough.**

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

The Cure SMA Board and Committee Members – These are extremely dedicated individuals who represent all of the families 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 involved and passionate Chair and has already provided the organization with great guidance, passion and commitment to the entire community and Cure SMA. We are grateful for Nick's listening ear, innovation and leadership and look forward to all that he is able to provide to this community.

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

The Cure SMA Medical Advisory Council Members – We are fortunate to have one of the most highly respected bodies of SMA medical and clinical experts in the U.S. as a part of this community. The Medical Advisory Council members are constantly brainstorming new ideas to raise awareness about SMA in the medical community, provide the best care for SMA patients and their families, and much more. These knowledgeable professionals volunteer their time, away from their clinics and families, to help plan the professional Clinical Care Session, and to organize the community conference medical workshops to educate families about 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 & Patrick Woodruff – Thanks to this foundation many families from the state of Arkansas are again able to attend this amazing conference. Through their generosity, Meredith and Patrick have provided scholarships so these families have an opportunity to attend and meet other families. The Miller McNeil Woodruff Foundation began in memory of Meredith & Patrick's son, Miller who had SMA type 1.

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

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

Jennifer Miller-Smith, Aaron Smith, Richard Curran and Expo – We can never express how incredibly grateful Cure SMA is for all that you do to ensure that each conference is as successful as possible. Thank you for everything!

Natasha Abruzzo, is a mother of four including two SMA angel babies. She is a Certified Nurse Practitioner working in a women's health clinic at Presbyterian Hospital in Albuquerque, NM. She loves caring for women of all ages through all the lifespans. 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 on the board for the Albuquerque A&M Club. She has a Bachelor of Science from Texas A&M University and a Masters of Nursing from University of St Francis. Natasha is involved in the Cure SMA in New Mexico Chapter and has been involved in several Cure SMA Conferences.



Brianna Albers (she/her), is a crip cyborg storyteller living in Minneapolis-St. Paul. In 2016, she founded *Monstering*, 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.



Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN, FPCN, is 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. Prior to becoming a nurse, she was a clinical research coordinator for clinical trials in neuromuscular disease. She earned her bachelor's degree from Boston College in psychology, with minors in health sciences and faith, peace, and justice studies; her nursing degree and master's degree from Columbia University School of Nursing; and completed a certificate in Pastoral Ministry at the Boston College School of Theology and Ministry. 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. Dr. Battista is a member of the CureSMA Medical Advisory Council (MAC).



Amber Bosselman, is 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. When not busy helping other people live their best life, Amber loves to read, paint and snuggle her kitty Penny. She soaks up sunshine wherever possible and aims to spread just as much emotional sunshine to others.



Ed Bronsdon, is passionate about helping people. He believes good leaders help see and set a vision for the future and good managers help people to effectively pursue that vision. Ed has served as the executive director of the Outdoors for All Foundation since 1995. Outdoors for All is a nonprofit based in Seattle, WA that enriches the lives of children and adults with disabilities through outdoor recreation. Ed is an avid cyclist. He is a Professional Ski Instructors of America Alpine Level 3 and Adaptive Level 1 certified instructor. He is a graduate of the University of Washington and has a certificate in Rehabilitation Facilities Management from Seattle University. In his local community, Ed volunteers with the University Sunrise Rotary Club of Seattle and has been a Rotarian since 1987. Nationally, Ed volunteers with Move United (formerly Disabled Sports USA) as both a board member and instructor. Internationally, Ed volunteers with Blesma – The Limbless Veterans, a British charity, and serves as the adaptive skiing lead and liaison for Blesma's annual European Winter Sports week in France.



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

Shane Burcaw



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



Kevan Chandler, has authored several books, including a memoir of his adventures. In the 2016, Kevan and his friends took a trip across Europe, leaving his wheelchair at home, and his friends carried him for three weeks in a backpack. He now travels and speaks worldwide about his unique life with a disability. He is also the founder of the nonprofit We Carry Kevan.

SPEAKER BIOS



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

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



Shonna COUNTER, is an author, speaker, and host of the show: TooTaboo4U. Most people are uncomfortable about sex – and about disability – and especially sex & disability. Through frank conversations she's changing misconceptions – to help strengthen and empower all your personal relationships. She was diagnosed with SMA (Type 3) at age six. In 2017, she progressed to using a power wheelchair, but still walks at home. From Shonna: Part time wheelchair - full time disability advocate. Author (Men Are Like Ice Cream), storyteller, life adventurer, speaker, legal ADA advocate, performer. She has three degrees (one in marriage/sex therapy). Living life to the fullest - Eternal optimist. Always heard: "You'll never be able to..." – "You're so confident & courageous." I'm confident, that if you're courageous enough to join us - you'll enjoy this session and you'll be able to learn so much.



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



Tara Davenport, is a former civil engineer turned social work student and mother to four children, three with disabilities. She began her meditation journey 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 her mindfulness practice with other parents.



Claire Miles, is a ten-year-old with a rare form of SMA who works hard finding her own way to deal with her emotions, especially anxiety. She will sometimes jump on her mom's mindfulness bandwagon. She loves to dance, sing, act, and hang out with her dog, Nimbus.



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



Bakri Elsheikh, MBBS, FRCP, is a Professor of Neurology at the Ohio State University. He is board-certified in Neurology, Neuromuscular Medicine, and Clinical Neurophysiology. He 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.



Casey Fenger, is a Resident in Physical Medicine and Rehabilitation at the University of Utah. He received his Doctor of Medicine (MD) from the University of Utah. As part of his medical training, Casey focuses on establishing and implementing care protocols to standardize and streamline patient care. In addition to his work in the hospital, Casey has over ten years of experience in adaptive sports. One of his greatest joys is getting those with physical limitations out on the ski hill again, or for the first time.



Albert Freedman, PhD, is a child and family psychologist in independent practice near Philadelphia, PA. Al provides counseling support for children with special needs and their families, serves as a consultant to health care organizations, rare disease advocacy groups and biopharmaceutical companies, and writes and speaks widely on the topic of caring for children with complex medical conditions. 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, now 22, recently graduated from Ursinus College and is a CureSMA conference volunteer.



Jeanine Geraci-Dubs, joined Protected Tomorrows Inc., in 2021 as our Special Needs Services Coordinator. Jeanine is a true Chicagoan, studying psychology and social work at Roosevelt University. She has spent the last 15 years working and advocating in the disability community from school districts to the veteran's hospitals. With her background, along with her experience in mental health and the disability world, we are so excited to have her as part of our team. Jeanine became passionate about this line of work, having grown up with and caring for family members with special needs. She hopes to use her existing knowledge and love of learning to continue to improve others' well-being. Jeanine is currently earning her Securities Licensing (Series 65 License) in the Financial Industry. Jeanine lives in Lake County with her husband Jason and their cat Gus-Gus. In her free time, Jeanine loves to cook for her family, travel, and spend time with friends.



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



Wendy Godfrey, is a Certified Trauma Informed Social Emotional Learning (SEL) Facilitator and yoga instructor. Her passion is working with individuals, groups and organizations to promote wellness through self-awareness and simple self-care practices. Wendy was a schoolteacher for over twelve years in a high needs elementary school, allowing her the opportunity to work with special needs students and their families. Her teaching experience also led her to this passion of sharing self-care practices. She is a true believer in finding individualized, simple, and accessible ways to help you fill your "vessel" in order to provide for others. Wendy is the founder of ST Wellness which offers workshops in Self-Care focused on Caregivers, Safety, Resiliency for Teachers, and customized programs for individual organizations/fields. Wendy is very honored and excited to share with caregivers during the Caregiver Support Workshop ways to become more self-aware of stressors and develop a "toolbox" to help in de-stressing in order to promote wellness in yourself, those around you and your communities. It all starts from within.



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.



Christina Han, is an Associate Clinical Professor of Obstetrics and Gynecology and Division Director of Maternal-Fetal Medicine at UCLA. She is an active clinician, researcher, educator and patient advocate with a focus on advanced prenatal diagnosis, management of complex fetal anomalies, maternal comorbidities, and preconception optimization of maternal health, including for conditions like spinal muscular atrophy. She is honored and excited to participate in this year's Annual SMA Conference.



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



Jennifer Hubbell OTR/L, Jennifer is a Level II therapist in the OT/PT department of Cincinnati Children's Hospital Medical Center. She has been a pediatric therapist for 28 years and has experience in both outpatient and home health care. She has been working with families and children with SMA for 20 years and has developed a special interest in their care from diagnosis through adulthood. She has presented to peers and staff at Cincinnati Children's Hospital Medical Center as well as local schools and organizations in the areas of high-risk infants, sensory processing, childhood development and therapy in the home



Becky Hurst Davis MS, RD, CSP, CD, CNSC, is a registered dietitian working with Intermountain Health Care in Salt Lake City, UT. She graduated with her Masters of Science in Nutrition from the University of Utah. Becky is a certified specialist in pediatric nutrition as well as a certified nutrition support clinician. She has great interest in clinical research providing improved nutrition care to people with neuromuscular diseases and has been involved with Cure SMA for over 12 years.



Richard M. Kravitz, MD, MD, Pediatric Pulmonology and Sleep Medicine Physician.



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.

SPEAKER BIOS



Andraea LaVant, Andraea LaVant is a nationally and internationally sought-after disability inclusion expert. She is widely recognized for spearheading a global disability justice movement as impact producer for Netflix's Oscar-nominated film, *Crip Camp*, executive produced by President Barack and Mrs. Michelle Obama. Andraea is founder and president of LaVant Consulting, Inc. (LCI), a social impact communications firm that offers cutting-edge corporate development and content marketing for brands and nonprofits. LCI's specialty is helping brands "speak disability with confidence." Her work has been featured on *Good Morning America*, NBC, *Essence.com*, *the Root*, *Paper Magazine*, and a host of other national media. As a black, disabled woman, Andraea champions intersectionality and is committed to working toward a future where ALL people, particularly disabled people of color, are seen and valued in culture and society at-large.



Jennifer Lemisch, MA, ATR-BC, LPC, is a board-certified art therapist, licensed professional counselor who has been employed at Children's Hospital of Philadelphia since May, 2001. For the past 18 years, she has been a member of the Pediatric Advanced Care Team, the palliative care service at the hospital, working with patients and siblings. The majority of her clinical work occurs in the homes of families who are receiving home care and/or hospice treatment for their child as well as grief and bereavement counseling for siblings after death.

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



Jennifer Martyn, PT, studied physical therapy at the University of Washington, graduating in 1995. Her first job at Mary Bridge Children's Hospital in Tacoma plunged her into the world of aquatic physical therapy and she has never left. Jennifer also has experience in a variety of clinical based physical therapy settings, staffed a regional MDA clinic and currently also works with children in the ICU at a local hospital. In 2001 Wave Therapies was created 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. Jennifer received her Aquatic Physical Therapy Competency in 2015. CureSMA Annual Conference is a highlight of every summer and she is thrilled to be back with the SMA community for this year's event! When not in the water Jennifer loves being with her family, biking, kayaking, cooking and gardening.



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.



Leslie Nelson, PT, PhD, OCS, is an Assistant Professor in the Department of Physical Therapy at University of Texas Southwestern in Dallas Texas. She has a broad background in physical therapy with a focus, over the past 13 years, on infants and children with neuromuscular disorders and muscle diseases. In addition to her clinical expertise, she has been involved in both investigator-initiated grants and industry sponsored clinical trials for studies involving neuromuscular disorders. Leslie has served as a mentor in the Cure SMA Therapist Mentoring Program. She also serves on the Cure SMA Medical Advisory Committee.



Katlyn O'Brien, is a graduate from Western Illinois University. Katlyn has a degree in Elementary Education and is currently working as a 5th grade teacher at Deer Park Middle School in Lake Forest, IL. She is also working on a Masters in Educational leadership at Concordia University. She will graduate next spring from this program. She has successfully supervised and coordinated the Sibling Workshop during numerous Cure SMA conferences. Katlyn has an older sister named Shannon who is affected with SMA Type 3. Having a sibling who is affected with SMA presents endless rewards and challenges. It is her goal to reach out to other siblings by allowing them the opportunity to express themselves and support each other through common experiences and interactions.



Traci O'Brien, is a dual-masters degree graduate of SIU, where she co-founded their Women's Ultimate Frisbee club, and Grenoble École de Management, where she lived abroad for a couple of years studying International Business. She recently moved to California to pursue a marketing career in the entertainment industry (music and comedy), promote animal welfare causes, and the healing arts. A very special part of her heart is reserved for these conferences, she loves speaking to other siblings with her sister Katlyn and having dance parties with all in a different city each year! She is little sister to Shannon O'Brien, and they enjoy: playing Nintendo, listening to music, and eating pizza. Her parents also are long-time members in the community whom she's beyond words proud of... She's been a volunteer ever since being released from childcare.
Cure SMA is fam.



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



Collin Pollock, has SMA Type 3 and has been married to Kyla for 16 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 16 years to Collin, who has SMA Type 3. In addition to being her husband's full-time caregiver, she is Mom to Elyana (13) and works as VP 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. Kyla attended her first Cure SMA Conference in 2006!



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



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 20 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., as an attorney and financial advisor, and father of a child with special needs who suffered from SMA, Richard is well versed and has a heartfelt commitment to assist families who require special needs planning. Richard concentrates his legal practice in estate planning, and elder law. At Strategies for Wealth, Richard works to develop long term relationships with his clients and base them on integrity and commitment to them, their families, friends and businesses. He begins by coordinating and integrating of all aspects of their personal and business finances. His role becomes that of a trusted attorney and financial advisor providing professional counsel to afford his clients with the financial confidence necessary to maximize efficiency and effectiveness in achieving great success, wealth and peace of mind. Richard is a Member of: American Bar Association; National Society of Financial Services Professionals; National Association of Insurance and Financial Advisors; Past Chair and Board Emeritus Member, National Board of Directors, Cure SMA; Greater New York Chapter, Cure SMA; Town of Yorktown, Group Home Committee; and Past Chairman, Town of Yorktown, Board of Ethics. He currently resides in Yorktown Heights, NY with his wife Michele, 18-year-old daughter Emma, and dogs Sophie and Ripley. He makes this presentation in loving memory of Max, "my special boy", April 24, 2005 – February 8, 2009.



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



Kevin Shafer, 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.

SPEAKER BIOS



Perry Shieh, MD, PhD, Neurologist at UCLA Medical Center in Los Angeles, CA.



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.



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. 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 including: characterization of bone structure-property relationships; prevention of pathologic fractures as a consequence of metabolic bone diseases and metastatic cancer; biomechanical analysis of mechanisms of spine injury, development of a novel dual ultrasound system to non-invasively measure real time cervical spine kinematics and intervertebral disc deformation during extreme activities; development of novel contrast agents for CT and MRI and a Raman spectroscopy arthroscopic probe to evaluate the biochemical and biomechanical properties of hyaline cartilage in synovial joints affected by degenerative diseases; derivation of animal models to evaluate the natural history of early onset scoliosis, thoracic insufficiency syndrome and the efficacy of treatments to foster normal growth and development of the spine, thorax and lungs. Dr. Snyder was awarded the A. Clifford Barger Excellence in Mentoring Award from Harvard Medical School in recognition of his pedagogy, the Kappa Delta, Ann Doner Vaughan Award for Outstanding Orthopaedic Research from the American Academy of Orthopaedic Surgeons and the Russell Hibbs Award by the Scoliosis Research Society for Outstanding Basic Science Research.



Anne Stratton, MD, Assistant Professor, University of Colorado: Department of Physical Medicine and Rehabilitation, Children’s Hospital Colorado. Dr. Stratton is originally from Ohio and received her M.D. from the University of Cincinnati in 2004. She then completed dual residencies in Pediatrics and Physical Medicine and Rehabilitation in 2009 at the University of Colorado, followed by a Pediatric Rehabilitation Medicine Fellowship in 2010. Children’s Multidisciplinary Neuromuscular Clinic is one of her favorite clinics and she has a special interest in exercise tolerance and benefits in patients with muscle disease as well as skeletal changes in children with muscle disease. Clinically, she feels it is important to consider the whole patient, their lifestyle, and aspirations when making medical recommendations. She is also the mother of two young girls and enjoys doing some running in her free time.



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. After traveling as an athlete for the United States National Team, she now 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. She has first hand experience with two different agencies, two different breeds, and has learned a vast amount about all types of working dogs from former teammates and friends; and now we hope to pass that knowledge onto anyone that is interested in the subject. Jones is a seven-year-old yellow lab from NEADS World Class Service Dogs. He was bred and born at Guiding Eyes For The Blind and then decided on a career as a mobility service dog. He was raised at MCI Norfolk and spent weekends with his puppy raisers in Hopkinton. After a year and a half of intense training, he was matched with KateLynne in December of 2016....the rest is history. He now has over 80 commands that help Katelynne live an independent life no matter where the adventure takes them.



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



Harvey J. Stern, MD PhD, FACMG, FAAP, is Board-Certified in Medical Genetics and Pediatrics and has worked with SMA families for over 20 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.



Rachel Stewart (she/her), is coordinator/counselor of the WorkAbility III program, supporting community college students with disabilities in reaching their career goals. Holding a strong commitment to the disability rights movement, over the last 15 years Rachel has conducted policy work and coordinated programs aimed at increasing employment of youth and adults with disabilities. She received a M.S. in Rehabilitation Counseling from San Diego State University in 2011 and a B.A. in Psychology from UC Berkeley in 2005. Rachel is currently working on a doctorate in Educational Leadership from CSU Sacramento, and lives in Sacramento with her wife.



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 in the neuromuscular and multiple sclerosis communities.

Peyton Tansey, is an SMA adult and college student at Hofstra University. He studies Computer Science and uses and develops computers and technology to adapt to different situations both in the class and outside of it. He has been working with Ann for almost 20 years and has learned many different things together.



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 6 years, she has been an active member of the Cure SMA Medical Advisory Council



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



Krista Torseth, PT, DPT, graduated from the University of Puget Sound in 2007, with a doctorate in physical therapy. She began working in pediatrics in both inpatient and outpatient settings, beginning work in aquatics in 2008. She has extensive experience with infants, children, and adolescents with orthopedic and neurologic concerns. Krista has training in neurodevelopmental treatment, aquatics, breathing and postural control. In 2015 Krista received the American Physical Therapy Association's Certificate in Aquatic Physical Therapy. She is very excited to be in the water with kids and families for CureSMA's Annual Conference!



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



Laura Watne, MS RD CSP, Nutritionist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.



Emily Wolinsky, is an Associate Advocate of Student Accessibility Services at Austin Community College and President of NMD United, a peer-led and driven, virtual, non-profit organization, which connects her peers, adults with neuromuscular disabilities (NMDs), with resources. Emily holds two master's degrees in the fields of counseling and education and has over 25 years of experience advocating for policies and practices which support Independent Living. She uses a suprapubic catheter (SPC), which has dramatically improved her quality of life, leading her to be a self-proclaimed, "Pee Free Ambassador" who has guided and mentored countless adults through their SPC placements over the years. This is her first time attending a Cure SMA conference.



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 41-year-old Vocal Music Teacher with SMA Type 2. She is an influencer on Tiktok and Instagram where she advocates for 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. <https://www.tiktok.com/@alliewheelz>, <https://www.instagram.com/alliewh33lz81>

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 2022 Annual SMA Conference, we could not have held this event without you all.

We're proud to support the
2022 Annual SMA Conference



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

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Cure SMA thanks the Dhont Family Foundation for their support of the 2022 Annual SMA Conference!

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

Muscles for Mckenna



Congratulations and thank you to the Ellixson Family and the entire committee for The 2022 Muscles for Mckenna Evening of Hope! Their 10th annual event, and first event back in person since March 2019 due to COVID, took place on Friday, March 18, 2022 at the Crystal Tea Room in Philadelphia, PA.

Approximately 125 people enjoyed a lovely evening complete with a live and silent auction, fund-a-need, and live music. Thanks to the support of the community, sponsors, and attendees, the 2022 Muscles for Mckenna Evening of Hope raised over \$110,000 this year! We are thrilled to share that thanks to their dedication, The Muscles for Mckenna Evening of Hope has raised over one million dollars since its first event!

Thank you to the Ellixson Family for their continued support of Cure SMA!



Northern California Walk-n-Roll

We are thrilled to congratulate the Northern California community for their amazing achievement of surpassing \$1 million raised at their Annual Northern California Chapter Walk-n-Roll. The community surpassed this milestone in 2019, and in the years since have remained strong in their commitment to Cure SMA.

In 2019 alone, over 350 participants and 31 teams raised over \$100,000 for Cure SMA. This event would not be possible without the help from the event committee, volunteers, and sponsors. A huge thank you goes out to Pat Wolff, Danielle Galan, Krisie Nguyen, David and Lisa Sereni, Linda Shively, Marge Shively, and Cathy Barsotti, among the many volunteers who helped to make this impact possible!

We are excited to welcome back the Northern California Walk-n-Roll for 2022. The event will take place on August 20th at Kennedy Grove Regional Recreation Area in El Sobrante, CA as we celebrate the event's 20th anniversary. Each year, this event grows bigger and bigger because of this community's dedication. **Congratulations to the Northern California Walk-n-Roll community on their 20th Anniversary milestone and thank you for all you do!**





Elizabeth Lockwood

As a longtime supporter, advocate, event lead, sponsorship chair, and grandmother to Nick and Emma Lockwood, Elizabeth Lockwood has truly made an impact on the Cincinnati community and the overall Cure SMA community nationwide. Her dedication and passion to helping find a cure is what has helped drive her building impactful sponsorship connections within the community which has brought in over \$500,000 throughout the years, made connections for new and returning families in the community, and so much more. Cure SMA would like to thank Elizabeth for her dedication and support!

Evening of Hope: Singer-Songwriter Night

Congratulations to the Scurria Family and event committee of the 2022 Cure SMA Evening of Hope: Singer-Songwriter Night in Baton Rouge, LA! Celebrating their 5th annual event, and first event in person since April 2019, the community came together for a fabulous evening of live music, dinner, cocktails, live and silent auction, and merriment.

Thank you to the Scurria Family, 2022 Event Committee, sponsors, supporters, and attendees who made the event possible this year!





Links 4 Luke

Congratulations to the Luke 18:1 Foundation and the Stickane family as they held the 5th Annual Links 4 Luke tournament last month and celebrated over \$1,000,000 donated to Cure SMA from event proceeds. This annual event, held on Monday, May 2, 2022 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, and support the Cure SMA's at-home physical therapy webinar series.

Thank you to the Luke 18:1 Foundation and Stickane family for their continued support and partnership with Cure SMA.

LUKE 18:1

New England Walk-n-Roll

Over the past 20 + 1 (2021 would've been the 20th anniversary) years, over 350 members of the New England community have come together to raise over \$1.8 million dollars to support Cure SMA's mission. During this time Silvia Murphy has led the charge in what's become one of the top fundraising events in the nation, and for that we would like to celebrate and honor the New England community, and Silvia Murphy for their ongoing efforts in helping make today a breakthrough.



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Disclaimer: Apitegromab is an investigational drug candidate being developed and studied for SMA. The effectiveness and safety of apitegromab have not been established. Apitegromab has not been approved by the FDA or any other regulatory authority.

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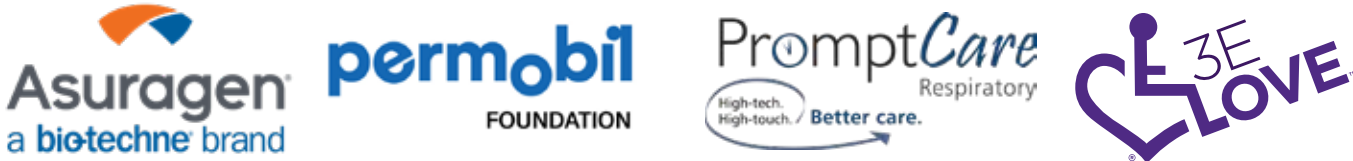
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CURE SMA BOARD OF DIRECTORS

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 volunteer individuals, 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 our organization.

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 & 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 \$200,000 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.



Gillian Mullins – Secretary

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



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 Vice President, Finance – Health Solutions at Alight Solutions and is a certified public accountant. Brad previously served as Treasurer on the Board of Directors for Onward Neighborhood House, a community based not-for-profit in Chicago. He is excited to continue to build the strong community of adults with SMA and to continue to expand awareness. In April 2017, Brad was a panelist in the Patient-Focused Drug Development meeting with the FDA and shared his experiences on the impacts of living with SMA. In addition to chairing the Finance Committee on the Board of Directors of Cure SMA, Brad also is a member of the Adults with SMA Subcommittee. Brad and his wife Krista live in Elmhurst, Illinois with their two sons. The whole family is proud to support Cure SMA.

EXECUTIVE COMMITTEE



Robert Graham, MD – Liaison of Science and Medicine

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



Allyson Henkel – Chair of Nominating and Governance Committee

Allyson is a Spanish teacher and has worked as a legal and medical interpreter. Her previous work experience includes 8 years at Lord, Abbett & 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 17 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. The Henkel Family, through Hosts for Hospitals, hosts patients who are traveling to Philadelphia for treatment. She is proud to remember and honor her son Pete and all who are affected by SMA through her work with Cure SMA.



Kelly Jankowski – Chair of Audit and Compliance Committee

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 – Liaison of Development and Marketing

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 – Liaison of Public and Advocacy

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



Shannon Zerzan – Liaison of Community Support

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

BOARD MEMBERS



Corey Braastad – Board Member

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



Kelly Cole – Board Member

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



Jaclyn Greenwood – Board Member

Jaclyn is from Southern California and was diagnosed with SMA type 2 at 18 months. She graduated summa cum laude from Chapman University with a BS in Molecular Biology. She went on to earn a Masters of Science degree in genetic counseling from the University of California, Irvine in 2012. As a certified genetic counselor, she currently works for the California Department of Public Health as a coordinator for their Prenatal Screening Program. Despite life's physical obstacles, Jaclyn lives a full life and enjoys driving a modified van, traveling, and rooting for the local Ducks hockey team. Jaclyn and her family have been involved with Cure SMA since first diagnosed in 1990 and have found tremendous support from the organization. She is excited to be a part of Cure SMA and their effort to expand their SMA adult community outreach.



Annie Kennedy – Board Member

Chief of Policy, Advocacy, & Patient Engagement – EveryLife Foundation for Rare Diseases
Focused on improving health outcomes for people living with rare diseases by advancing the development of treatment and diagnostic opportunities for rare disease patients through science-driven public policy, Annie's work includes building strong partnerships with policy makers, federal agencies, industry, and alliances. Annie has served within the community for nearly three decades through her roles with Parent Project Muscular Dystrophy (PPMD) and the Muscular Dystrophy Association (MDA). In that time she helped lead legislative efforts around passage and implementation of the MD-CARE Act (2001, 2008, 2014), the Patient Focused Impact Assessment Act (PFIA) which became the Patient Experience Data provision within the 21st Century Cures Act (sec 3001), engagement with the FDA and Industry around regulatory policy and therapeutic pipelines, led access efforts as the first therapies were approved in Duchenne, and engaged with ICER around the development of the modified framework for the valuation of ultra-rare diseases. Annie's community roles include service on the PFDD Works coalition, the Patient Driven Values in Healthcare Evaluation (PAVE) Steering Committee, the National Health Council's PCORI Valuation Group, the Innovation and Value Initiative (IVI) Patient Advisory Committee, the National Duchenne Newborn Screening Pilot Program Steering Committee, the Institute for Gene Therapies (IGT) Patient Advocacy Advisory Council, and as a member of the NIH National Center for Advancing Translational Sciences (NCATS) Advisory Council.



Sierra Kulas – Board Member

Sierra Kulas is the parent to two children with SMA Type 2. Over the last 12 years since their diagnosis, she has held a multitude of various roles in rare disease advocacy. As a past Cure SMA chapter lead and committee member, she not only brings a breadth of professional experience but an unrelenting personal passion to make change for the SMA community. Sierra has held roles in the nonprofit sector, traveled the world as a motivational speaker telling her boys story living with and fighting against SMA, worked in pharma as the Patient Advocacy Manager for AveXis through the launch of their SMA gene therapy program, and currently sits as the Patient and Caregiver Network Chair for Medable, managing a group of 60+ patients and caregivers to advise on medical technologies in development for clinical trial.



Edmund Lee – Board Member

Edmund and his wife Kwiyoum have been blessed with their daughter, Angie, who has SMA type 2. 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 Member

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, having recently resigned to attend school with her oldest. Amy became involved with Cure SMA in 2011, after her first child, Mateo, was diagnosed with type 1 at just one month of age. Amy's second son, Javier, age six, 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 four, 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.



Tom Murray – Board Member

Thomas Murray, PhD, is President Emeritus at The Hastings Center. He was formerly the Director of the Center for Biomedical Ethics in the School of Medicine at Case Western Reserve University, where he was also the Susan E. Watson Professor of Bioethics. Among other commitments, he served on the Board and as the Vice Chair of Charity Navigator. He serves on many editorial boards and has testified before many Congressional committees. Among other current posts, 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. His newest book is *Good Sport: Why Our Games Matter – and How Doping Undermines Them*.



Shannon Shryne – Board Member

Shannon is the co-founder and President of Augie's Quest to Cure ALS, a non-profit dedicated to raising the funds and awareness urgently needed to advance cutting-edge research, fast-track effective treatments, and ultimately, find a cure for ALS. Augie's Quest, under Shannon's leadership, has raised over \$87 million since its inception in 2005, and successfully funded a potential therapeutic that is in a Phase 2 trial. Shannon has almost 30 years' experience in non-profit development and leadership with expertise in corporate engagement, major gift solicitation, prospecting and cultivating donors, fundraising, and cause marketing. She spent over 20 years with the Muscular Dystrophy Association engaging national corporate partners resulting in more than \$200M for the Association. Shannon also consults with charities including One Earth, Team Joseph, and the Foundation for Love & Acceptance. She lives in Denver with her son.



Brian Snyder, MD – Board Member

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. 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. 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 including: characterization of bone structure-property relationships; prevention of pathologic fractures as a consequence of metabolic bone diseases and metastatic cancer; biomechanical analysis of mechanisms of spine injury, development of a novel dual ultrasound system to non-invasively measure real time cervical spine kinematics and intervertebral disc deformation during extreme activities; development of novel contrast agents for CT and MRI and a Raman spectroscopy arthroscopic probe to evaluate the biochemical and biomechanical properties of hyaline cartilage in synovial joints affected by degenerative diseases; derivation of animal models to evaluate the natural history of early onset scoliosis, thoracic insufficiency syndrome and the efficacy of treatments to foster normal growth and development of the spine, thorax and lungs. He has been principal and/or co-principle investigator of several NIH/NIAMS, NASA, DoD, foundation grants (Whitaker, OREF, Susan B Komen, AO/ASIF, Coulter, POSNA, SRS). Additionally he was a permanent member of the NIH/NIAMS Tissue Engineering study panel that evaluates orthopaedic devices and biologics and represents the Pediatric Orthopaedic Society of North America (POSNA) and Scoliosis Research Society (SRS) to the FDA and Board of Orthopaedic Specialty Societies. Dr. Snyder was awarded the A. Clifford Barger Excellence in Mentoring Award from Harvard Medical School in recognition of his pedagogy, the Kappa Delta, Ann Doner Vaughan Award for Outstanding Orthopaedic Research from the American Academy of Orthopaedic Surgeons and the Russell Hibbs Award by the Scoliosis Research Society for Outstanding Basic Science Research.



Peter Statile – Board Member

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

COMMITTEE MEMBERS



Diana Castro, MD – Committee Member

Dr. Castro, a board certified pediatric neuromuscular physician who is a pioneer in research and management of patients with SMA. 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.



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 & IP teams. Prior to joining TSMC, Jay held senior executive positions in general management, strategy, marketing and sales for two 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 & 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.



Bakri Elsheikh, MD – Committee Member

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 SMA. He serves as the principal investigator on several projects on various neuromuscular diseases.



Kaleen Robinson – Committee Member

Kaleen is the Chief Talent Officer at Salo. 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.

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.



Alison Ballard, RN, MSN

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



Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN, FPCN

Board-certified Pediatric Nurse Practitioner (PNP) and Senior Nursing Director of Palliative Care at the Dana Farber Cancer Institute in Boston, MA.



William Bell, BS Pharm, MBA, MSCC, RPh

Director of Clinical Client Services in Chalfont, PA.



John Brandsema, MD

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



Thomas Crawford, MD

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



Tina Duong, MPT, PhDc

Physical Therapist at Stanford University in Stanford, CA.



Albert Freedman, PhD

Specializes in Psychology in independent practice in Philadelphia, PA.



Melissa Gibbons, MS, CGC

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



Jennifer Hubbell, OTR/L

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

MEDICAL ADVISORY COUNCIL (MAC)



Becky Hurst Davis, MS, RD, CSP, CD

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



Chamindra Konersman, MD

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



Richard M. Kravitz, MD

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



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

Pediatric Physical Therapist at Northwestern University in Chicago, IL.



Oren Kupfer, MD

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



Khalida Liaquat, MS, LCGC

Genetic Counselor at Athena Diagnostics in Marlborough, MA.



Diane Murrell, LCSW – Care Coordination, Case Management, & Nursing Subcommittee Chair

Social Worker at Texas Children's Hospital in Houston, TX.



Leslie Nelson, PT, PhDc, OCS

Pediatric Neuromuscular Physical Therapist at Children's Health, associated with the University of Texas Southwestern in Dallas, TX.



Julie Parsons, MD – Neurology Subcommittee Chair

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

MEDICAL ADVISORY COUNCIL (MAC)



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, Texas.



Perry Shieh, MD, PhD – Adult Care Subcommittee Chair

Neurologist at UCLA Medical Center in Los Angeles, CA.



Edward C. Smith, MD

Pediatric Neurologist at Duke University in Durham, NC.



Anne Stratton, MD

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



Stacey Tarrant, BS, RD, LDN

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



Jane B. Taylor, MD, MsCR

Pediatric Pulmonologist at the 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 in Denver, 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.



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)



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



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



Timothy Reilly, PhD, DABT, Director, Bristol Myers Squibb, Toxicology



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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, Head of Biology Discovery, Denali Therapeutics, Preclinical Drug Development



Charlotte Sumner, MD, Associate Professor, Johns Hopkins University, SMA Biology/Neurology



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



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GET INVOLVED WITH CURE SMA TODAY!



WALK-N-ROLL



The Cure SMA Walk-n-Roll is a nationwide fundraising event that brings communities together to support Cure SMA's funding of life-changing research, support, and programming for all those impacted by spinal muscular atrophy (SMA). What started as a grassroots initiative by individuals in their communities, has now grown into the single largest nationwide movement to bring an end to SMA. At Walk-n-Roll we celebrate fundraising achievements and recognize community members for their collective efforts to create progress for the SMA community. With over 35 events nationwide, Walk-n-Roll reminds us how strong one person can be, and how much stronger we are together.



ENDURANCE

Team Cure SMA connects runners and cyclists with Cure SMA. Right now, you can sign up to participate in a marathon, half marathon, 10K, or 5K race. Team Cure SMA race participants —veterans and newbies alike — can expect to receive exclusive endurance gear, comprehensive training programs, and fundraising guidance every step of the way.



GOLF OUTING

Prepare for a day of fun in the sun at the Cure SMA golf tournaments and outings, locally hosted in communities around the country. Each event features a wonderful day of golf, lunch and dinner, event programs, awards, course games, and more all while raising funds for Cure SMA.



cure SMA EVENING OF HOPE

The Cure SMA Evening of Hope gala and social events bring together people affected by SMA and their supporters across the country for evenings that feature cocktail hours, silent and live auctions, live entertainment, dinner programs, and much more.



cure SMA 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!



You can register yourself or a team, start fundraising and spreading the word, and we'll celebrate the impact you're making along the way!



To find your local fundraising event, please visit curesma.org/fundraise or scan the QR code.

SUMMIT OF STRENGTH PROGRAM



Summit of Strength is a free community educational program hosted by Cure SMA.

These half-day mini conferences host a wide variety of professionals who cover topics that are valuable to individuals of all ages and types of SMA, and their caregivers. Presenters are experts in their fields, with years of experience specific to SMA, and attendees have the opportunity to have their questions answered by these specialists in an intimate setting.

Cure SMA wishes to thank our sponsors for the 2022 Summit of Strength Program - National Presenting Sponsors, Biogen and Genentech, and National Visionary Sponsor, Scholar Rock.

- July 23rd – Boston Summit of Strength
- July 30th – Minneapolis Summit of Strength
- August 6th – New York City Summit of Strength
- August 13th – Philadelphia Summit of Strength
- August 20th – Austin Summit of Strength
- September 10th – San Francisco Summit of Strength
- September 24th – Baltimore Summit of Strength
- October 15th – Chicago Summit of Strength
- October 22nd – Phoenix Summit of Strength
- October 29th – Hartford Summit of Strength
- November 5th – Miami Summit of Strength
- November 19th – Anaheim Summit of Strength
- December 3rd – New Orleans Summit of Strength
- December 10th – San Antonio Summit of Strength

2022 Summit of Strength Schedule

- February 5th – Atlanta Summit of Strength
- February 19th – Orlando Summit of Strength
- March 5th – Seattle Summit of Strength
- March 19th – Nashville Summit of Strength
- May 7th – Charleston Summit of Strength
- May 14th – Denver Summit of Strength

This program includes breakfast, lunch, and parking for all attendees. Childcare and entertainment will not be available at the Summit of Strength, but children are welcome to attend with a parent or caregiver.

Register today at www.curesma.org/summit-of-strength

If you have any questions or comments, please contact communitysupport@curesma.org

August is

SMA Awareness Month



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

**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

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



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

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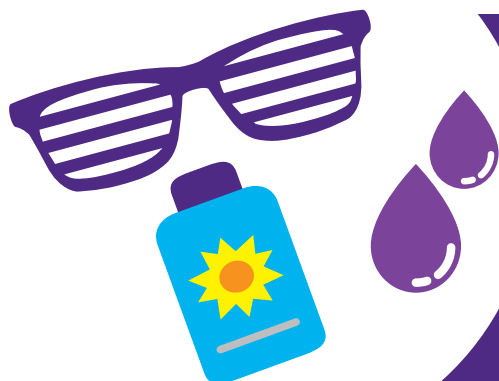
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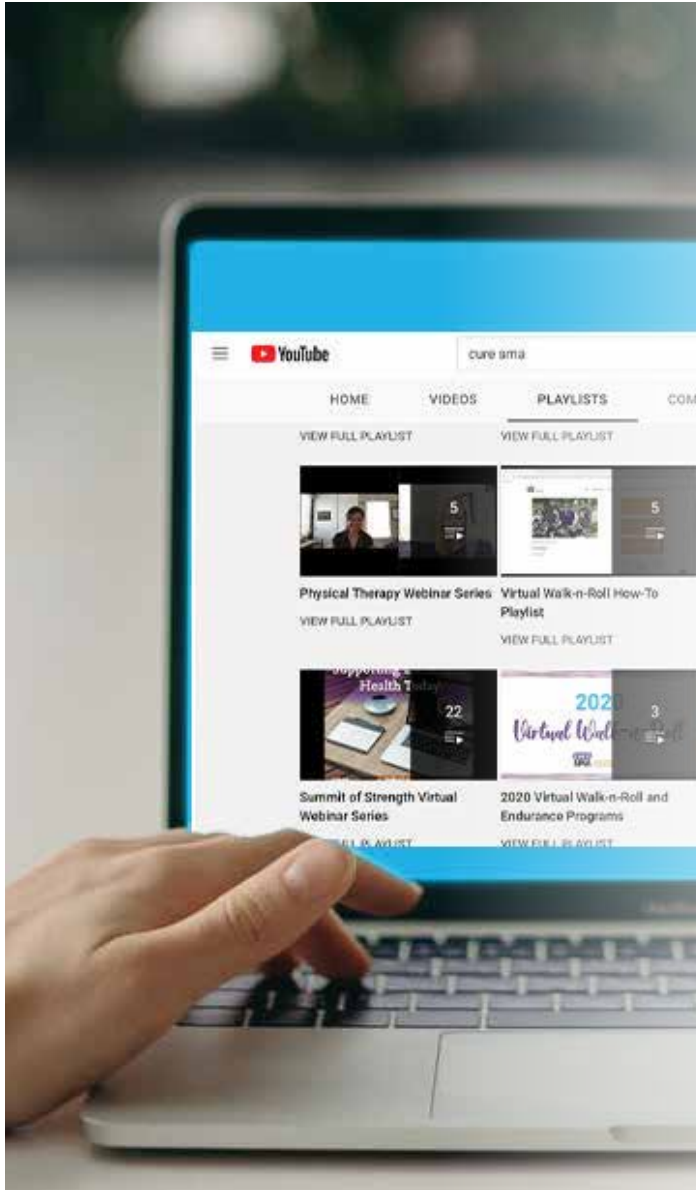
**PLEASE NOTE****WEAR SUNSCREEN AND KEEP HYDRATED!**

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!



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



Cure SMA Programs for Adults with SMA

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 & 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 & 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

Adults with SMA Virtual Therapy Program

It has never been more important to take care of our mental health, as we continue to navigate through uncertain times. This new virtual therapy program is available to adults with SMA ages 18 and over within the US, to make virtual therapy more accessible. Fill out our request form to receive a complementary 30-minute live video session with a licensed therapist (up to 3 sessions maximum).

www.curesma.org/virtualtherapyprogram



SUPPORT PACKAGES AND PROGRAMS



Virtual Therapy Program for Personal Care Assistants of Adults with SMA

This new program provides personal care assistants (PCAs) of adults with SMA three complementary 30-minute live video sessions with a licensed therapist through the online therapy company, Talkspace. PCAs may be family members, friends, or hired caregivers, and are incredibly valuable members of the SMA community.

www.curesma.org/virtualtherapyPCA



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.

www.curesma.org/virtual-community-engagement-events/



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.

www.curesma.org/virtual-community-engagement-events/

**You
Tube**

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!

CONVENTION CENTER MAP

Disneyland[®] RESORT

8

HOTELS

- 1 Disney's Grand Californian Hotel & Spa
- 2 Disney's Paradise Pier Hotel
- 3 Disneyland Hotel

PARKS

- 4 Disneyland Park
- 5 Disney California Adventure Park

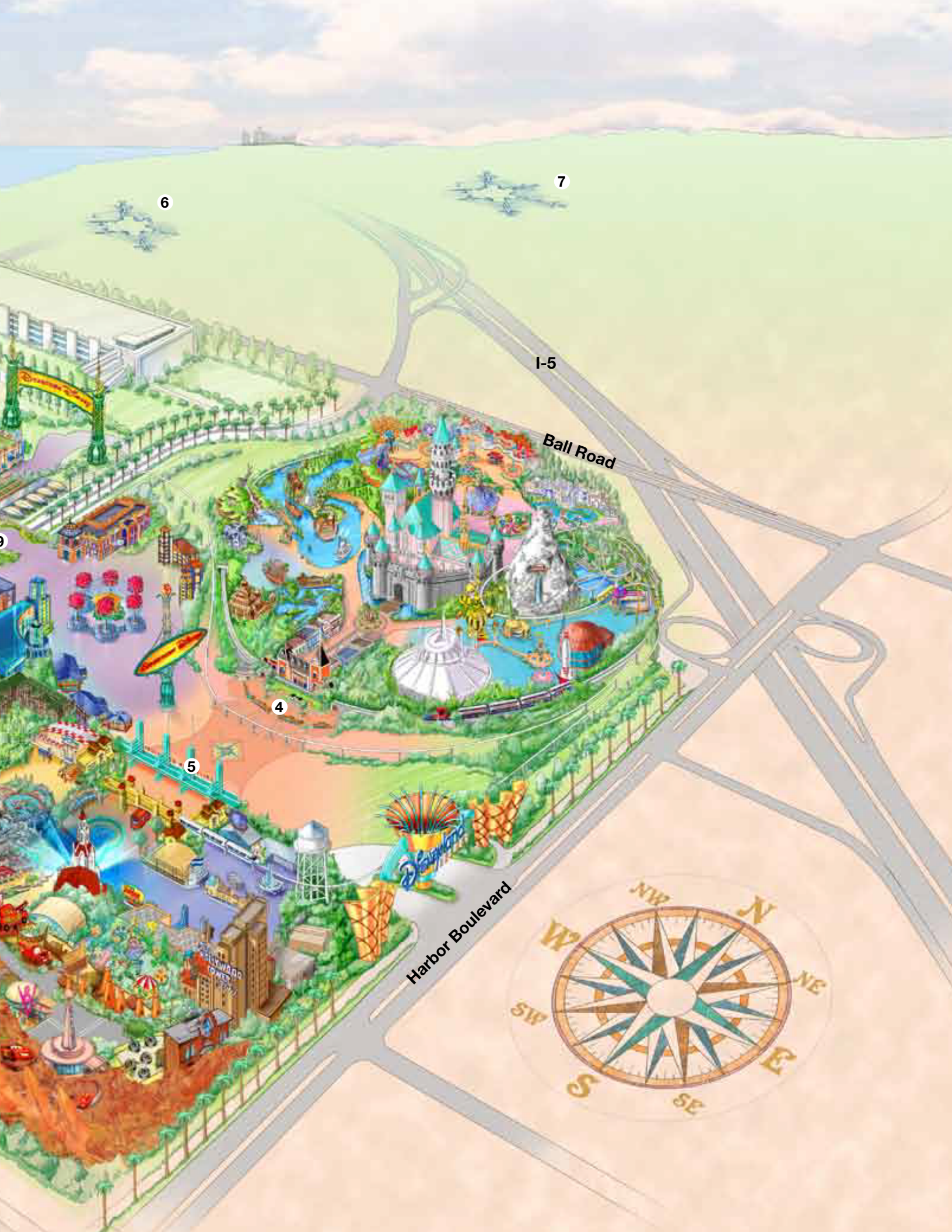
AIRPORTS

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

OTHER

- 9 Downtown Disney District
- 10 Anaheim Convention Center





6

7

I-5

Ball Road

4

5

Harbor Boulevard



Disneyland® Hotel



HOTEL FEATURES & SERVICES

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









SHOPPING & RECREATION

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

RESTAURANTS & LOUNGES

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

LEGEND

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

CONVENTION & BANQUET FACILITIES

Convention Center

Lower Level (Entrance Near Steakhouse 55)

Castle A-C Room
Monorail A-C Room

Main Level

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

Upper Level

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

Adventure Tower

Nile	Tiki
Congo	Safari
Zambezi	Adventure
Amazon	Outpost
Oasis	Explorer

Frontier Tower

Western	Mississippi
Wilderness	Mark Twain
Pioneer	Frontier Board Room
Columbia	

Outdoor Event Areas

Rose Court Garden
Adventure Lawn
Frontier Lawn
Magic Kingdom® Lawn

GUEST ROOMS

Fantasy Tower: __ 00 - __ 35
Adventure Tower: __ 36 - __ 67
Frontier Tower: __ 68 - __ 99

(Add first two numbers for floor number)



Your SMA story matters!

**cure
SMA**

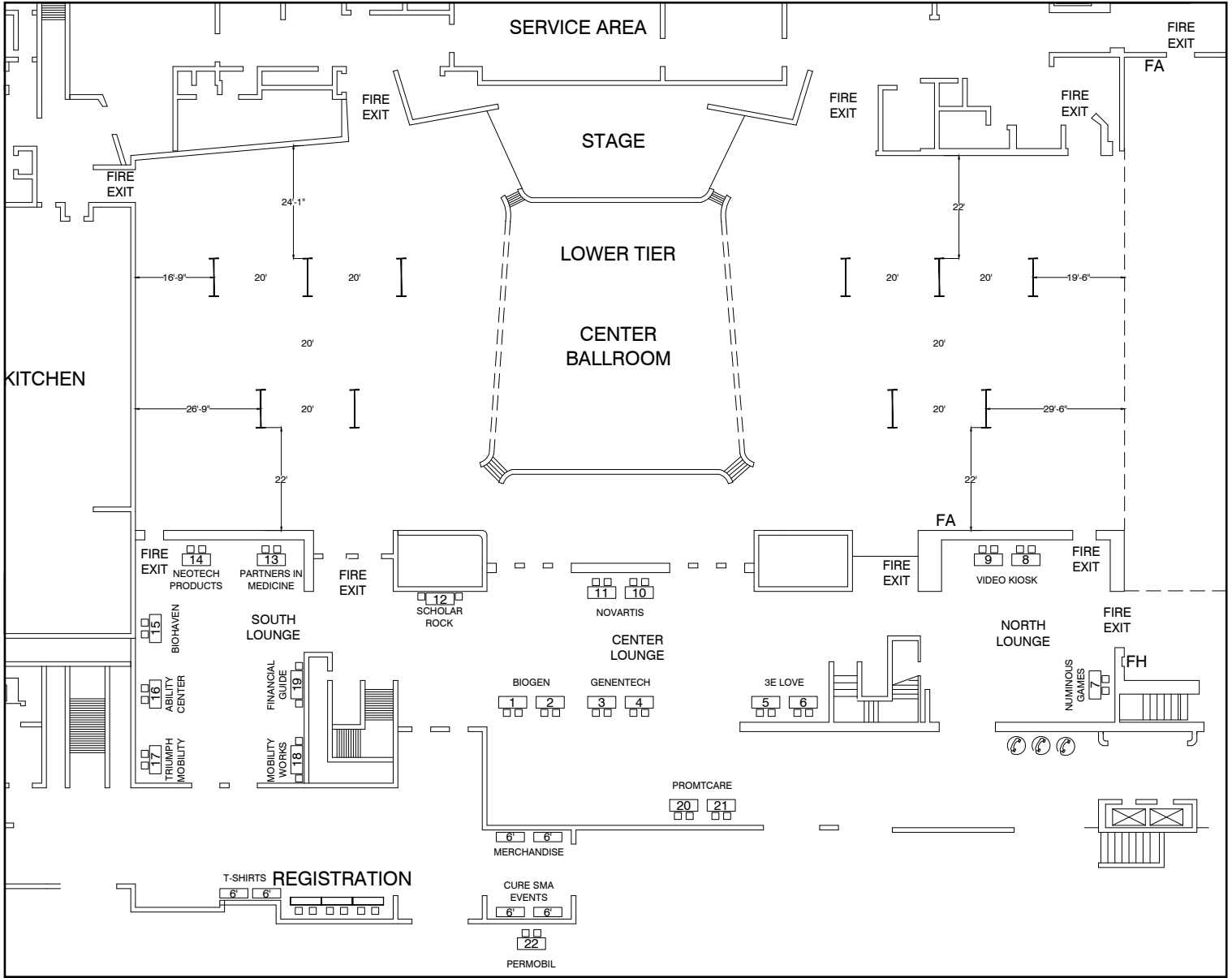
your story SMAtter

On Friday and Saturday during the conference, visit the storytelling booth in the North Lounge and record a brief video sharing your story about one or more of the following:

- Why do you feel compelled to give/donate to Cure SMA?
- What advocacy issue matters most to you and why?
- What does the Annual SMA Conference mean to you?

The videos you record will be used throughout the year to enhance fundraising campaigns, advocacy efforts, marketing communications and more.

Join us for a fun, interactive experience, entry for exclusive raffle prizes, and to share your story with the SMA community.



EXHIBITOR KEY

Ability Center | #16

Biohaven | #15

Biogen | # 1 & 2

Genentech | # 3 & 4

Financial Guide | # 19

Mobility Works | # 18

Neotech Products | # 14

Novartis Gene Therapies | # 10 & 11

Numinous Games | # 7

Partners in Medicine | #13

Permobil | # 22

PromptCare | #20 & 21

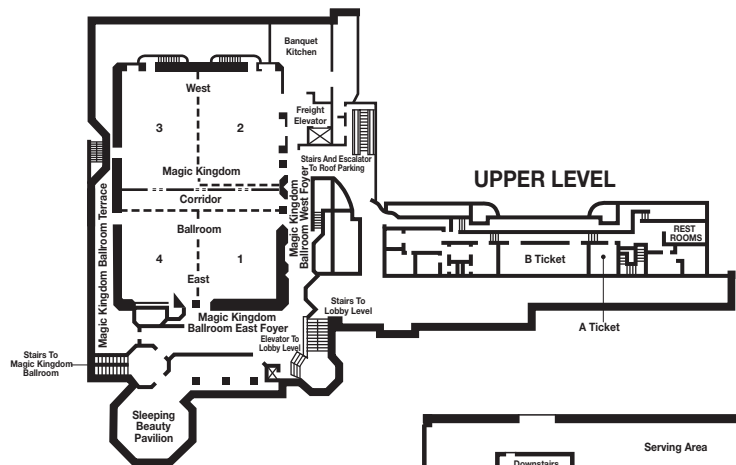
Scholar Rock | # 12

3E Love | # 5 & 6

Triumph Mobility | # 17

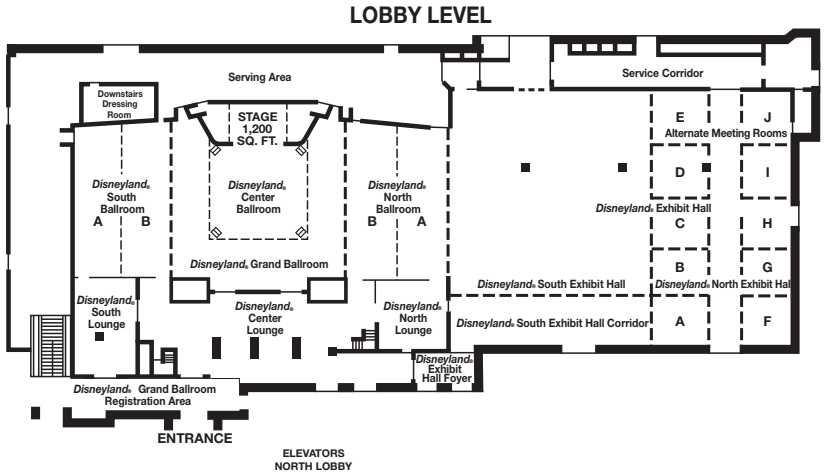
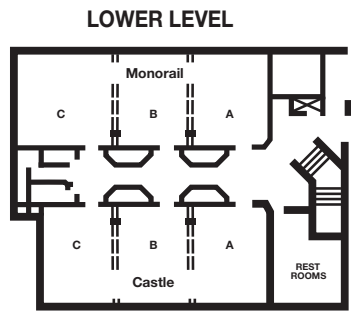
Video Kiosk | # 8 & 9

FANTASY TOWER

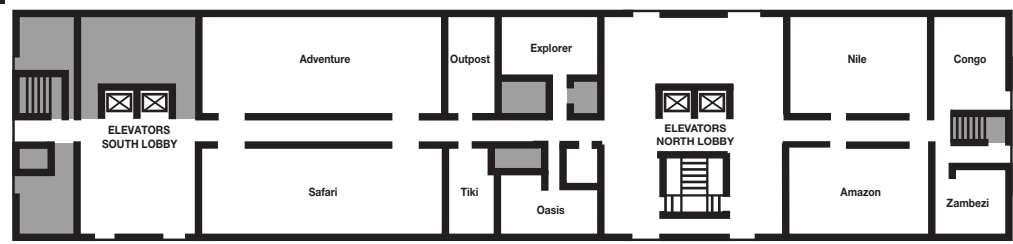


Disneyland Hotel

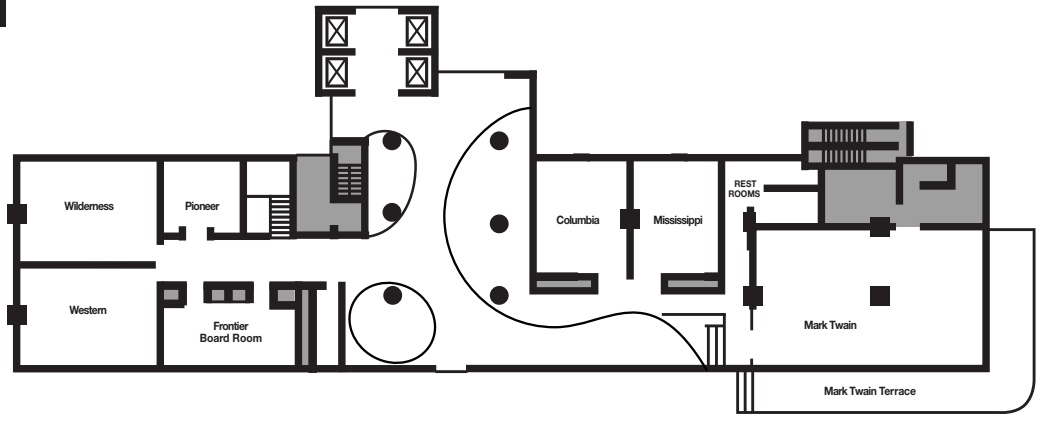
CONVENTION CENTER



ADVENTURE TOWER



FRONTIER TOWER



©Disney



SEE YOU ALL NEXT YEAR