

DIRECTIONS

COMMUNITY NEWSLETTER | WINTER 2023

See inside for Annual Conference Art Exhibition winner!



The Joy of Giving!

RESEARCH >> INDEPENDENCE >> ACCESS

Look inside to see how a generous gift can
help support Cure SMA today!



**cure
SMA**





LETTER FROM KENNETH

Our Annual SMA Conference in Orlando this summer was our biggest conference ever with 3,200 attendees. It was an honor to have individuals with SMA and their families, healthcare professionals, researchers, and industry partners from over 45 countries across the globe join us. In this issue of Directions, you'll find a section dedicated to the best of the Annual Conference, from our opening Meet and Greet to recaps of new activities this year. We hope to see you June 6–9, 2024 in Austin, Texas!

During the summer, you may have also heard about the Erin Trainor Memorial Fund. The Erin Trainor Memorial Fund was started by Barbara and Gene Trainor who lost their five-month-old daughter, Erin, to SMA Type 1. In the lead up to the Annual Conference and during the Opening Session, the Erin Trainor Memorial Fund generously matched all gifts made to the Cure SMA Care Center Network in an effort to add new Care Centers. Thanks to all of you who donated, we raised nearly \$300,000, matched by the Trainors to \$600,000!

We have some key areas to focus on over the coming months. One of these is new funding for basic research. This year, Cure SMA has increased our total funding going towards basic research grants by an additional \$250,000 over last year. We're focusing on advancing research to discover new non-SMN therapies and combination treatment approaches. Our goal is to gain the knowledge and understanding to help restore muscle strength and function.

There are several phase 3 clinical trials in our initial round focusing on combination and optimization approaches, that are now reaching full enrollment. This will be an important year ahead as we gear up once again as a community to effectively complete trials and prepare for regulatory approvals, followed by access. Additionally, we have new clinical trials starting up with even more new approaches against SMA.

As we look toward the future with research and new combination treatments, it is just as important to focus on addressing the very real needs the SMA community has right now through our support and advocacy efforts. These efforts include a recent successful Hill Day in Washington, D.C., and our practical local support programs, including our newly diagnosed and teen and adult care packages and our Summit of Strength events.

If you would like to help us continue to expand access to care and treatments, advance breakthroughs in basic research, and support the daily needs of our SMA community, one of the best ways to do so is by contributing to our year-end campaign. Check out the back cover and inside pages of this issue for details.

With your support, we will create a better future for everyone living with SMA.

Thank you!

Kenneth Hobby
President, Cure SMA

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The production of this newsletter was supported
by a grant from Genentech.



Letters from the Community



"We received the package and Celebi loves everything that was in it! Thank you so much for all that you guys do. We really appreciate all the info and the support."

~ The Remillard Family

"Just had to say a big thank you. This put the biggest smile on Leland's face!! And the biggest tears in my eyes."

~ The Ianno Family



"I received a parcel today. Amelia and I enjoyed everything very much. Most of all, Amelia liked the toy dog, she called him Ben."

~ The Mishchenko Family

"We received the wagon and Owen is already loving it. We want to thank you very much for it and again for the care package of toys we received a few weeks ago. Thank you again for all the resources and please feel free to keep me informed of anything else you may think of. "

~ The Rachal Family



"Thank you for sending the care package so quickly. It's one of my favorite things that Cure SMA does and is an incredible way to walk with families at the beginning of their journey with SMA - inspiring celebration and joy in the midst of the overwhelm."

Little man loves the sheep skin. What a cool gift!

~ The Harris Family



"I can't thank you enough for the information and incredible box of adaptive gifts and toys for Evie! Your generosity overwhelmed us and made us cry!"

May God bless you and all you do for people like our Evie and our family!"

~ The Bradford Family



"Just wanted to send you another photo of Lily. She is doing amazing in her wheelchair. We are still so grateful for this loaner. It has given her so much independence and freedom. She zips around in this thing all the time and I almost can't get her out of it some days. We decorated her chair for our most recent family photos and she loved showing off her skills."

She continues to make progress every day in her mobility and we are so encouraged by all the support!"

~ The Aherns Family



"Thank you so much for the care package you sent to Matias, he is so happy with all the toys."

~ The Aguayo Family



"We wanted to say thank you again so much for the information packet and care package. I know Wrenley will have so much fun with all these toys! The support we've received these last few weeks has been amazing and we are so grateful."

Lots of love!
The Million Family

If you would like to submit a photo or story to be included in a future issue of *Directions*, please email communitysupport@curesma.org.



20 years of friendship
Gab & Jenn



Asher P.



Seda K.



Archie J.



Evie B.



Caleb N.



20 years of friendship
Josh & Marley

If you would like to submit a photo or story to be included in a future issue of *Directions*, please email communitysupport@curesma.org.



Owen R.



Jamie H.



Richárd R.



Matias A.



The Julien Family



The Aherns Family



Carina C.



Luca C.

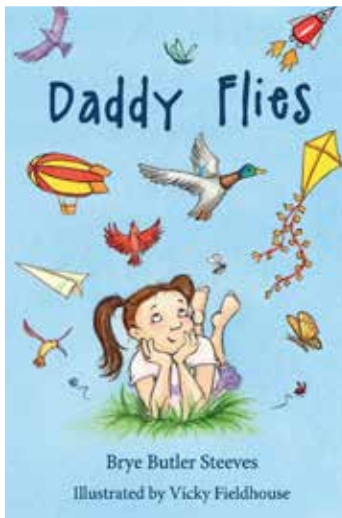
Donations



Cure SMA was thrilled to receive a shipment of lightweight maracas donated in honor of Jack Resendez! These maracas are specially made and aren't available for purchase in stores so a special thanks to the Resendez family for this wonderful donation to our newly diagnosed care packages!



Cure SMA was thrilled to receive a large shipment of newly diagnosed care package items such as playdoh kits, modeling clay and walkie talk toys donated in honor of Caleb Neal's first birthday! We would like to thank the Neal family and their friends for this wonderful donation and for organizing this effort in lieu of gifts to honor Caleb's 1st birthday this past February!



Cure SMA was excited to receive a donation of the book "Daddy Flies" to our newly diagnosed care packages in honor of what would be Liv Harlow McDonald's sixth birthday on March 7. This generous donation was made by Liv's grandparents Debbie and Richard Butler and is extra special as the book was written by Liv's Aunt Brye Butler Steeves.

Thank You!



Community Spotlight:

Holly Ianno and Family

Leland Ianno is eight years old, but he's an old soul with a deep curiosity, a love of people, and a passion for history. His mom, Holly, says "Leland is fascinated by historical times but also the history of the people around him. He wants to know everything, and he'll sit and have a conversation with you for hours and you feel like you're talking to a much older person."

While Leland has a love of learning about others, he and Holly have their own remarkable story to tell, which includes but is certainly not limited to their experience with SMA.

Holly says, "Leland had been in physical therapy since his early childhood due to toe walking, however, his pediatrician and physical therapist couldn't put a finger on why he had lower body weakness."

As Leland progressed in age, Holly couldn't shake the feeling that something wasn't quite right. She says, "I was on Instagram and saw a post with a video showing a child who got up off the floor using his hands. I told my husband that it looked just like how Leland did when he got off the ground, however I had no idea this could be an indication of SMA."

As Holly continued to look into what might be happening with Leland, she became pregnant with a baby girl in 2019 and sadly lost the baby in 2020 at 18 weeks and six days. Her obstetrician suggested genetic testing, which Holly and her husband went forward with.

Holly says, "Everything for my husband and I looked great and healthy, so we thought, why not?" They were surprised when the testing results came back showing that both Holly and her husband are carriers of SMA.

Despite this information, they hadn't yet connected this information back to Leland. Holly says, "Of course when you hear about SMA you look it up. When I originally looked into SMA after finding out I'm a carrier, I was just looking at SMA Type 1 and I thought, oh Leland doesn't have that. I didn't know at that time that there are different types of SMA."

"Fast forward, and I became pregnant again and was advised to have an amniocentesis. In that process I was on Instagram searching hashtags and that's when I looked at my husband and told him I thought Leland had SMA. My husband is very big on research, and he began doing his own research and we both came to the conclusion that Leland may be Type 3."

"We called Stonybrook Hospital in Long Island, New York and luckily they were able to get us an appointment the next day. About a month later we received test results that showed that Leland indeed had SMA Type 3."

While determining Leland's diagnosis took more than seven years, life since that day has been a whirlwind. Holly says, "We received his diagnosis a week before we were moving and leaving New York, so panic set in. I thought, I'm leaving New York with all this healthcare. What will we do?"

"Luckily, Stonybrook helped us find a clinic 15 minutes from our new house and the doctor, Dr. Honeycutt, is one of the only doctors in Greenville, South Carolina, that treats SMA."

Since Leland's diagnosis was so recent, Holly says, "We're still in the learning process and I think we'll forever be in the learning process. I'm in the mentality of taking it a day at a time because I don't know any other way to handle it."

"I say to my son, you're going to have such power in other places of life. It may not be running, or it may not be playing soccer or baseball, but using your voice and being who you are is what is going to make you powerful. And that's the same for me as his mom. I'm going to be his voice forever."

When asked what he would say to other kids with SMA, Leland says, "Remain strong and don't stop doing things you love because you have SMA. Keep being strong."

MOVING AHEAD

**Evrysdi keeps
working so we can
keep reaching**



What is Evrysdi?

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

Important Safety Information

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

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

Tell your healthcare provider about all the medicines you take.

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

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

The most common side effects of Evrysdi include:

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

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

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

Please see accompanying brief summary for additional Important Safety Information.

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

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 **Evrysdi**
risdiplam 60 mg
powder for oral solution

LIFE IN ACTION

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

What is EVRYSDI?

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

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

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

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

How should I take EVRYSDI?

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

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

Taking EVRYSDI

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

Reusable Oral Syringes

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

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

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

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

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

How should I store EVRYSDI?

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

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

General information about the safe and effective use of EVRYSDI.

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

What are the ingredients in EVRYSDI?

Active ingredient: risdiplam

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

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EVRYSDI® (risdiplam)

Distributed by:

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For more information, go to www.EVRYSDI.com or call 1-833-387-9734.

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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 and Adult Support Package

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

www.curesma.org/SupportPackage



Teen and Adult Independence Assistance Package

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

www.curesma.org/IndependencePackage



LifeVac Support Program

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

www.curesma.org/LifeVac



Medical Alert Bracelet

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

www.curesma.org/ResponderPHR

SUPPORT PACKAGES AND PROGRAMS



Annual SMA Conference Sponsorship

The Adults with SMA Sponsorship offers adults with SMA ages 18 and older, as well as one caregiver, waived registration fees, a stipend for travel, and one hotel room for three 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 four-day conference.

www.annualsmaconference.com/agenda.html



Adults with SMA In-Person Socials

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

Email communitysupport@curesma.org for more details!



Adults with SMA Virtual Socials

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

Email communitysupport@curesma.org for more details!



Educational Webinars and Panels

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

www.youtube.com/c/CureSMA/playlists



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



Community Spotlight: Dee and River Rolle

"There is an endearing tenderness in the love of a mother to a son that transcends all other affections of the heart," said Washington Irvine. When watching River Rolle wriggle in his mother Dee's lap while he sweetly places his hand on her chin and looks into her eyes, there is nothing more apparent than the love and affection between them.

River, meaning a large natural stream of water flowing, is a fitting name for an active boy full of energy and excitement. Dee describes River as "a typical three-year-old boy outside of him not walking." River loves to pop wheelies in his wheelchair and cruise around wearing a superhero cape. He loves to exert his independence and is firmly in the toddler, "I want to do it myself phase."

Despite his desire for independence, Dee has been River's world since his birth. "It's just been River and me. I'm a single, working parent," says Dee. "River was born in February 2020, a month before the world shut down due to COVID-19, which meant that we were almost immediately isolated. It was hard in general being a new mom and to add COVID on top of it was an extra challenge."

On top of these challenges, Dee started to notice concerning changes in River. "He was hitting all his milestones until about eight or nine months old. And he was standing at one point, and then all of a sudden he would not stand. He also never fully crawled," said Dee.

After many months of sleepless nights of worry, and meeting with a series of doctors, including pediatricians and neurologists, River was diagnosed with SMA Type 2 in December of 2021.

"We are blessed to have a wonderful neurologist named Dr. Shadé Moody at UTHealth Houston. Immediately following River's diagnosis, Dr. Moody put together a comprehensive health team and a plan of action, including a pulmonologist, an occupational therapist, and getting River treatment." Dee is incredibly thankful for Dr. Moody's quick action, as he was diagnosed a month and a half before his second birthday, narrowly making the cutoff for this treatment. River was also born just a few months before Texas instituted newborn screening for SMA.

Dee says, "After River's diagnosis, I wasn't able to stay in the shock and sadness for long because although I have a supportive extended family, it was just the two of us living in Houston, so I had to snap into it and keep us going."

However, just because Dee has kept going doesn't mean it hasn't been difficult. "I still have my moments. Even now, I have some grieving periods. But I went to the Cure SMA Annual Conference this year and being around and building community with other people and seeing how they're living their lives and fulfilling their purpose has really helped me. My family came out for the end of the conference too."

Speaking of family, Dee and River recently relocated to San Antonio, Texas to be closer to her parents so they can provide both her and River with support.

Dee and River have also built community through social media. Dee shares River's journey on his Instagram, aptly named @river_rocksandrolles. Dee says, "I created his Instagram as a healing thing for myself, but also to showcase River because I when was in the process of trying to figure out his diagnosis I stopped posting pictures of my baby. Because I'm a first-time mom, I wasn't sure if his delays were my fault, and I was being inadequate."

"Starting River's Instagram and sharing his story has been healing for me and connected me to family, friends, and supporters across the country. River's supporters are called River's Rock Stars, and they're awesome! I want to show the world my beautiful baby. Community is a beautiful thing for us."

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

2023 Annual SMA Conference Summary

Thank you to everyone who was a part of the largest ever Annual SMA Conference this past June! Over 3,200 attendees from around the globe came together for a weekend filled with networking, reconnecting, research, and fun! It was an honor to have so many people with SMA, their families, healthcare providers, and researchers attend the conference from over 46 different countries.

The four-day weekend was filled with special events and workshops. The Newly Diagnosed Program kicked off the conference on Thursday for those recently diagnosed with SMA, followed by our Opening General Session on Friday morning where all attendees were welcomed. Friday and Saturday were packed full of educational and supportive workshops, as well as the Family Friendly Researcher Poster Session. Finally, the 'It's a Wonderful Life' panel of adults with SMA ended the weekend on a high note. The Meet & Greet, PJ Party & Movie Night, and Evening at the Park were also conference highlights!

Lastly, at the Closing General Session on Sunday morning, we announced the location and dates for our 2024 Annual SMA Conference.

Mark your calendars for Thursday, June 6–Sunday, June 9, 2024, as we head to Austin, Texas at the JW Marriott Austin!

We extend our thanks to the sponsors and exhibitors who generously supported the 2023 Annual SMA Conference. A special thanks to Biogen and Genentech for their generosity as Presenting Sponsors of this year's conference.







My 2023 Annual SMA Conference Perspective: Updates and Moving Forward

By: Lyza Weisman

Numerous advancements and changes in the SMA community have occurred over the past few years. While the dynamic of the disease has been changing, the community is still here, outspoken and strong. Over 3,200 people registered for the 2023 annual SMA conference—you can bet the hallways were full of chatter, learning, and adventures, if not an entirely different form of traffic jams. More than ever, you can feel the hope in the air, including the changing diversity of ages of patients with SMA.

The adult SMA community is vibrant in ability and demonstrates that it is possible to be independent with a disability. Many workshops focused on the adult community, from sex and dating to swimming and an entirely designated Adult Lounge. Many kids with SMA have exceeded in strength and ability more than ever believed possible, thanks to the three treatments approved by the FDA. That said, sixteen other medications are currently in the pipeline.

The current treatments focus on the genetics of the disease, targeting its cause but do little towards reversing the course of the disease. Many of the drugs in the pipeline seek to change this by focusing on the muscle and motor neurons and regenerating lost muscle and strength. However, the muscle remains mysterious to scientists, given the need for more understanding of the muscle and motor neurons. Many researchers are heavily involved in seeking to learn about muscle and motor neuron lifespan and whether creating new muscles will help or if it would be more beneficial to focus on already existing muscles.

I spoke with a researcher at the annual poster board session who has been focusing on the motor neuron in patients with SMA using mouse studies. She found a unique differentiating factor between SMA and nearly all other genetic motor neuron diseases. In almost every other genetic motor neuron disease, when a motor neuron dies, or necrosis occurs, the death of the motor neuron and its dead tissue is visible. However, in SMA, the motor neuron disappears. It is like a room with a hundred people who are all drinking. You walk into the room, and you can tell there are a hundred people, even though ten people have now collapsed on the floor.

However, in the case of SMA, you walk into the room knowing it started with a hundred people, but now there are only ninety, and you have no clue where the others went. This is one example of a gap in the research that scientists are working to close to reverse the disease successfully.

Progress has been made in many areas of SMA, not only in scientific research, but mass successes have occurred in establishing newborn screening throughout the country—99% of the states have newborn screening for SMA. The only two states remaining are Hawaii and Nevada. Hawaii has approved newborn screening and is now setting things up to start newborn screening for SMA—all that is left is Nevada. It is important to note that newborn screening for SMA is only in the U.S., where approximately 10,000 individuals with SMA live. Still, it is estimated that about 250,000 people are living with SMA throughout the world, and it is them that we, as a community here, should try to help and reach out to. In the U.S., most individuals with SMA are on treatment, but globally, only 25,000 individuals with SMA are on treatment.

At the conference, I met and saw people from all over the world, and many more attending their first conference. Of course, there are newly diagnosed families, but many adults also attending conference and accessing the community and its resources for the first time. Cure SMA continues to send out care packages and be there to help the community members, and yet there is always more to do. SMA is not cured. As a community, we need to be there to help and motivate, allowing hope, perseverance, strength, and community to continue to be the backbone of SMA as the dynamic changes and the future holds multiplying possibilities for all patients worldwide.

Workshops

The largest number of workshop sessions ever were offered at this year's conference to families and individuals with SMA all day Friday and Saturday. These sessions were held by leading SMA experts as well as other individuals and family members affected by SMA.

There were over 60 workshops offered during the conference this year, covering the latest information on critical care and support. We are so grateful to each and every one of our speakers for making this amazing weekend one filled with new information, updates, and support!

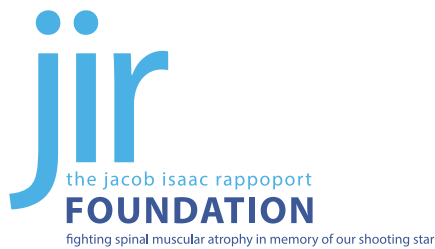


Children's Program

This year's Children's Program was another huge success! Thanks to our incredible volunteers, all the children had a fabulous time. Kids stayed busy with arts and crafts projects, toys, live entertainment, exciting movies, fun activities, video games, and so much more. A highlight was the caricature artist who drew photos for the kids to take home as conference mementos!

The Annual SMA Conference would not have been possible without the assistance of our fantastic volunteers. From setting up decor, working in the Children's Program, preparing and running games at the Meet & Greet, to countless other jobs, our volunteers are the heartbeat that keeps the conference moving.

The Children's Program is supported by the Jacob Isaac Rappoport Foundation, and their support allows this portion of the conference to be a highlight for so many families. We greatly appreciate the Jacob Isaac Rappoport Foundation sponsorship and helping to ensure the success of our Annual SMA Conference.



Meet & Greet

Each year the Annual SMA Conference officially kicks off with our signature event for everyone in the community to come together. This was our largest ever Meet & Greet and it was an amazing evening filled with fun, games, connections, and so much more!



Researcher & Clinician Relay Race

The ever-so popular Researcher Relay Race is always a highlight during the Meet & Greet, as individuals with SMA race researchers and clinicians one-on-one to see who can cross the finish line first. There is only one rule: the researchers and clinicians race in a manual wheelchair. All conference attendees lined up around the course cheering on the racers to see who won. Try as they may, the researchers and clinicians seemed to always be left in the dust!

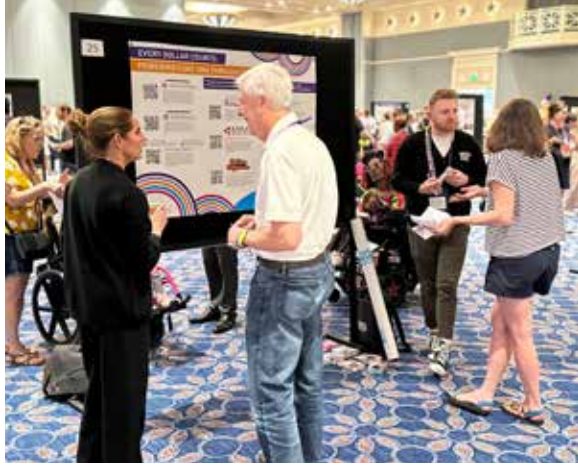


PJ Party & Movie Night

On Friday evening during the Family Friendly Researcher Poster Session, a cozy space was created for all conference attendees to dress down in pajamas and enjoy Finding Nemo on the big screen while cozying up with tasty popcorn. It was a relaxing end to the evening, after a full day of workshops.



Family Friendly Researcher Poster Session



Family Friendly Researcher Poster Session

The Family Friendly Research Poster Session allows for one-on-one interactions between families, individuals with SMA, and researchers. Cure SMA invited researchers who were attending the SMA Research Meeting to present family friendly research posters. During the Friday evening event, attendees were encouraged to rotate to different posters to ask questions and learn directly from the researchers involved in each of the projects being presented. Attendees were also able to meet Disney characters including Mr. and Mrs. Incredible, Edna Mode, Woody, Bo Peep and Lotso, and Mike and Sulley.

FAMILY FRIENDLY RESEARCHER POSTER SESSION POSTERS INCLUDED:

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

Cure SMA Evening at the Park

The Cure SMA Evening at the Park is a highlight of the conference! On Saturday, all attendees were invited to enjoy a magical evening at the EPCOT Theme Park. This fun-filled night also helped bring awareness to SMA and the entire SMA Community as guests showed up and showed off their conference t-shirts!



Networking Opportunities



The Annual SMA Conference represents a wonderful opportunity to foster new connections and catch up with longtime friends. The weekend hosted a variety of opportunities for everyone in the community to meet, mingle, and connect.

Opportunities for just moms and dads to get together included the Dad's Night Out on Thursday evening and a Mom's Night Out on Friday evening. A welcoming space was created for teens at the conference this year with a new Teen Lounge sponsored by the Luke 18:1 Foundation. Teens got to craft, game, and connect all day Friday and Saturday at the conference. Then on Friday evening, the Teen Social allowed teens to participate in a fun and interactive trivia game with snacks!



Throughout the conference, adults with SMA connected in the Adults with SMA Lounge, where they held conversations and could connect with each other, all day Thursday, Friday, and Saturday. This space was sponsored by Biogen and the Dhont Family Foundation. The incredibly popular Adults with SMA Reception took place on Friday evening, offering beverages and snacks and the chance to connect in a different format for a fun night out!

Grandparents had the opportunity to enjoy coffee and mingle during the afternoon and share stories and connect with other grandparents in the SMA community, during the Grandparents Coffee & Mingle session.

With all of these different socials and networking opportunities, we hope the connections and relationships made were impactful for all!

Please reach out to our community support team email at communitysupport@curesma.org.



Research & Clinical Care Meeting

At the end of June, over 630 SMA researchers and clinicians from around the world met in Orlando, Florida, for the 2023 Annual SMA Research & Clinical Care Meeting. This annual meeting provides an opportunity for SMA scientists and clinicians to share their most recent discoveries and learnings and fosters collaborations, including cross-disciplinary knowledge sharing. The goals of this meeting are to accelerate the pace of research into SMA treatments and achieve excellence in SMA clinical care. To learn more about the sessions from this meeting, visit the latest news section of www.curesma.org.



Congratulations to the SMA Community Self-Expression Art Exhibit Fan Favorite!

“Oriental Garden” by Jocelyn Chen

2023 Annual SMA Conference attendees were able to vote for their favorite piece at the SMA Community Self-Expression Art Exhibit, which was generously sponsored by Biogen. Out of 15 paintings, pictures, and crafted pieces on display, the beautifully peaceful yet bold “Oriental Garden” was voted as fan favorite.



ARTIST: Jocelyn Chen

HOMETOWN: Collierville, TN

DESCRIPTION: Painted primarily in watercolor with acrylic paint accents, this piece is an homage to Jocelyn’s Chinese heritage. There aren’t many places in Collierville, Tennessee that have Asian influences but this serene location at the Memphis Botanic Garden has a small piece of her culture.

Thank you to Biogen for their sponsorship of the 2023 SMA Community Self-Expression Art Exhibit.



THANK YOU TO OUR CONFERENCE SPONSORS

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

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SMA Awareness Month

cure SMA BREAKTHROUGHS



Begin with You

Whether you have SMA, have a loved one with SMA, are a researcher, care provider, industry partner, or are new to the SMA community, SMA Awareness Month is a time to come together to celebrate, honor, reflect, remember, and learn. This August the theme of SMA Awareness Month was “Breakthroughs Begin with You,” which represented an opportunity for the SMA community to be in the driver’s seat leading the dialogue and efforts to foster awareness and change.

From local events to national advocacy, there were plenty of ways to get involved. Highlights from SMA Awareness Month 2023 included the following exciting campaigns and events.



PSAs and Interviews on National Radio Stations

Cure SMA partnered with iHeart Radio on national public service announcements (PSAs) that were aired in nearly 25 major markets across the U.S. We also partnered with Hubbard Radio in the Chicagoland area on PSAs and an informative interview with Cure SMA’s Chief Medical Officer, Dr. Mary Schroth.



Bilingual SMA Awareness

To extend our reach to Spanish speakers, SMA Awareness Month materials, including infographics, social media posts, and more were translated into Spanish. Look for more Spanish language content from Cure SMA coming soon, including video interviews!



Awareness Month Theme Song Our first-ever SMA Awareness Month theme song is called “Lift Me Up” and features award-winning artist, Lachi, Broadway composer Gaelynn Lea, and acclaimed songwriters April Rose, SMA community member, James Ian, and Kulick. “Lift Me Up” is a tribute to late disability rights activist Judy Heumann, aimed at spotlighting high-quality accessible art and disability within a pop culture narrative.



Candle Lighting Events Each year, our community lights candles to remember those who have passed away from SMA, and to honor the diverse people and perspectives that make up our community. The annual candle lighting occurs at sunset on the second Saturday of August. This year's candle lighting was held on Saturday, August 12, 2023.

Building and Landmark Lightings Awareness lightings at popular landmarks such as buildings, highway bridges, and entertainment venues help to bring attention to the SMA and SMA Awareness Month in a unique way. This program was started by community member, Shaakira Thomas, "To me, SMA Awareness Month is not only about ways of raising awareness, but it is also a time to honor and celebrate everyone impacted by SMA. We are all fighters and being able to hear everyone's stories is amazing. It brings me hope and strength."



Supporting SMA Research This year's SMA Awareness Month featured Research Week, and opportunity for the SMA community and supporters to learn more about and help fund basic research. The best part is, it's not too late to participate.

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 nerve muscle connections and the regeneration of nerves. Richard and Jane Nunemaker are matching all research gifts focused on these findings up to \$250,000. Your donation today will result in increased strength, mobility, and independence for people with SMA in the future.

Donate here:



Fundraising Events Some of Cure SMA's largest annual fundraising events were held in August, including three Walk-n-Roll events, a 5K, and a road race, which served to not only bring in critical funds but to increase SMA awareness locally and nationally. Be sure to visit curesma.org/fundraising-events to find upcoming events near you.

Cure SMA Staff Spirit Week The national staff team at Cure SMA planned their own fun and celebratory activities to recognize SMA Awareness Month. This included a Staff Spirit Week where team members were encouraged to sport their purple and orange gear to meetings and fundraise and participate in local events.



Shopping for Good In honor of SMA Awareness Month, Cure SMA released new merchandise in our online store, including sweatshirts, t-shirts, belt bags, tumblers, and more. Our new merch was so popular that we sold out of several items, which are now restocked. All branded items help spread SMA awareness, and a portion of each purchase you make funds research, education, support programs, and more. Visit our store at curesmaorg.myshopify.com.

Social Media Blitz Cure SMA took to social media to spread SMA awareness stories, news, and updates far and wide. We created interesting, informative, and easily sharable social media graphics and encouraged members of our community to share personal stories. If you'd like to share your story and learn more about becoming a Cure SMA social media ambassador, email us at stories@curesma.org.

August may be over, but through your support, SMA awareness continues year-round. Thank you to everyone who took the time to attend an event, donate or fundraise, light a candle, or share your story. Breakthroughs truly do begin with you!

THANK YOU



Breakthroughs Began with You During SMA Awareness Month!

Whether you made a donation, attended a Cure SMA fundraising or educational event, shared your story, or posted SMA awareness information on social media, your impact during SMA Awareness Month is felt and appreciated.

Although August is over, we hope you'll continue to help us increase SMA awareness year-round.

**Thank you for
your support and participation!**





Spot-check

Check Out Cure SMA's Spotify Playlists

Whether you're working from home, commuting to school or an appointment, or taking your dog for a stroll, odds are that you incorporate listening to music and/or podcasts into your routine.

What you may not know is now you can make Cure SMA part of that routine because we're now on Spotify!

From curated musical selections to informative podcasts featuring SMA community members, you'll find selections that are sure to keep you educated, informed, and entertained.

Perhaps you're missing Disney and the Cure SMA Annual Conference. Then check out our playlist of Disney tunes. Or maybe you're gearing up to participate in a Walk-n-Roll, Endurance, or Baking a Difference fundraising event. If so, you're in luck because we've got music to keep you motivated. We even have a kid-focused playlist that you can play in the kitchen while you bake tasty treats for our Baking a Difference program.

If podcasts are your preference, check out our 'Podcast Recommendations' playlist featuring selected episodes from Rare Mamas Rising, Once Upon a Gene, Disability Visibility, and more.



Check out all our playlists.
We can't wait for you to tune in!



Shining a Light on the Impact of Philanthropy at Cure SMA: The Joy of Giving

Life often surprises us with unexpected opportunities, and when we seize them with courage, something truly extraordinary can unfold. In the spirit of these serendipitous moments, let us acknowledge the profound impact of your generosity and the essential role philanthropy plays in our collective mission. One where everyone impacted by spinal muscular atrophy is empowered to lead independent, successful, and fulfilling lives. The “joy of giving” stands as a guiding light that not only illuminates the lives of those touched by SMA but also binds us together in this extraordinary journey.

The significance of your generosity stretches far beyond the confines of monetary giving; it is the very essence of transformation. It fuels groundbreaking research, offering scientists the resources they need to unearth new treatments and, ultimately, discover a cure for SMA. Philanthropy has given us the platform to amplify our voices, advocating for increased awareness, enhanced care, and expanded support for those navigating the challenges of SMA.

The “joy of giving” represents the boundless capacity of the SMA community for empathy and compassion. It serves as a reminder of our interconnectedness as we collectively strive for a brighter tomorrow. With your steadfast support, we can ensure that this beacon of hope shines brighter than ever before.

This year’s annual campaign is dedicated to supporting three crucial pillars: basic research, high-quality care, and practical support programs. Each of these pillars stands as a driving force for those affected by SMA, and your philanthropic efforts move them forward.



Basic Research: Your contributions fuel groundbreaking research, equipping scientists with the resources needed to unearth new treatments and, ultimately, find a cure for SMA. By investing in research, you lay the foundation for a promising future.



High-Quality Care: Your philanthropy ensures that individuals and families affected by SMA receive the highest standard of care. Your support helps enhance medical care, therapies, and interventions, improving the quality of life for those living with SMA.



Practical Support Programs: In addition to medical care, your generosity enables the implementation of practical support programs. These programs provide invaluable assistance, from access to assistive devices to emotional support, easing the burdens faced by individuals and families affected by SMA.

But philanthropy is not limited to financial contributions alone; it’s a reflection of the kindness you’ve exemplified through your actions, your advocacy, and your steadfast commitment to our shared cause. Your unwavering dedication has forged a robust network of support, extending far beyond the financial realm, offering solace and trust to those affected by SMA.

As we approach the year-end, let us pause to celebrate the transformative power of philanthropy. Your contributions, whether in the form of resources, time, or heartfelt stories, have already left an indelible mark. By sharing your experiences, your stories, and your love, you’ve not only illuminated the path toward a cure but have also nurtured a spirit of resilience and hope within the SMA community.

We extend our heartfelt gratitude for being an integral part of our journey and the very essence of CureSMA’s mission. Together, we continue to light the way forward, and together, we will triumph together.

Experience the joy of giving to the SMA community this holiday season with a donation to our year-end campaign.



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

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



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

Individuals with SMA and Their Families Advocate on Capitol Hill



More than 110 adults with SMA and families of children with SMA from across the country traveled to Washington, DC to educate their Members of Congress and their staff about SMA and the unmet research and everyday living needs of the SMA community. In a single day, the group of Cure SMA Advocates met with 60 U.S. Senate offices and 82 U.S. House offices to advocate for new SMA research at the National Institutes of Health, accessible air travel, and other SMA community priorities. We were especially grateful that U.S. Senator Mike Braun of Indiana, U.S. Senator Tim Kaine of Virginia, and U.S. Senator Elizabeth Warren of Massachusetts reserved time in their schedules to meet directly with the SMA community. Thank you to all Cure SMA Advocates who attended Cure SMA's 2023 Hill Day or have taken an advocacy action during 2023. Your advocacy matters!





Research Update

Cure SMA Publishes Thought Leader Perspectives about the Future of SMA Biomarker Research

An important part of Cure SMA's core mission is to carry out and support research that will improve the quality of life for all people with SMA. This summer, we published a research article titled, "**Identifying Biomarkers of Spinal Muscular Atrophy for Further Development.**" This article appeared in the July 12, 2023, issue of the peer-reviewed research journal, the *Journal of Neuromuscular Diseases*.

KEY TAKEAWAYS

- Cure SMA recently published a research article that describes the findings of the SMA Biomarkers Multidisciplinary Working Group, a group of SMA researchers and healthcare providers who met in 2020
- Through a series of surveys and conversations, the working group determined which of the currently studied SMA biomarkers is likely to be the most useful in SMA research and care
- The group decided that a protein called "neurofilament (NF)" is best suited for continued validation as a biomarker for SMA
- Then, the working group outlined a plan for the validation of NF for widespread use as an SMA biomarker

Cure SMA Convenes Working Group

In 2020, Cure SMA brought together 11 scientists and healthcare professionals who specialize in SMA research and care to form the SMA Biomarkers Multidisciplinary Working Group. Over the past two decades, researchers have performed many studies on molecules and measurements that may be good biomarkers for SMA. However, before a biomarker is widely accepted for use, scientists must validate it by performing rigorous tests to make sure it meets certain standards.

Therefore, the goals of the SMA Biomarkers Multidisciplinary Working Group were:

- To review what was known so far about potential SMA biomarkers
- To determine which biomarker is likely to prove the most valuable in ongoing SMA research and care
- To outline a plan for future research that will support the validation of the selected biomarker

WHAT IS A BIOMARKER?

A biomarker is a molecule or measurement that is a sign of what is happening in the body. Biomarkers can be used to diagnose illness, predict disease severity, select appropriate treatment, or measure treatment response.



SMN2 copy number is a good example of a biomarker for SMA. Everyone has at least one copy of the *SMN2* gene, and many people have two, three, or more copies. The number of *SMN2* genes a person with SMA has is important because the *SMN2* gene can function as a back-up for the missing *SMN1* gene. Because the *SMN2* gene can also produce a small amount of SMN protein, the more copies of *SMN2* a person with SMA has, the less severe the disease is likely to be. For this reason, *SMN2* can be used as a biomarker to help determine SMA severity.

Although *SMN2* copy number can help doctors predict SMA type, a person's *SMN2* copy number is fixed and does not change with disease progression or treatment. For this reason, other biomarkers are needed to measure changes that occur over time during SMA research and clinical care.

Working group members were first provided with a reading list of articles describing research studies on the most promising SMA biomarkers. These “candidate” biomarkers included genetic factors, proteins, imaging techniques, and methods that measure electrical activity in muscles and nerves. Working group members were also invited to add articles to the reading list for the group to review.

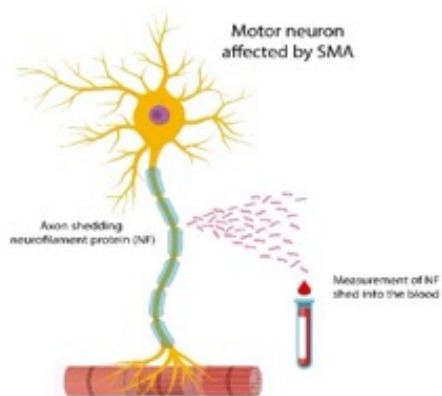
After the working group members had time to review the reading list, their input through a series of surveys and phone calls. In the first three surveys, working group members were asked to rank candidate SMA biomarkers in terms of which were likely to be most useful. The second three surveys asked more detailed questions about the biomarkers the group had ranked highest. In the last three surveys, working group members were asked for input on the most important gaps in knowledge about these biomarkers, and how those gaps could be filled. They also weighed in on which SMA biomarkers might be most appropriate for persons of certain ages.

Working Group Findings

The working group determined that a protein called “neurofilament (NF)” has the most potential for widespread use as a biomarker in SMA research and clinical care. NF is found in the axons of healthy neurons, where it plays an important role in maintaining axon structure. In many diseases that affect neurons, the NF protein is released from axons as neurons start

to die. Therefore, changes in NF levels can help track not only disease progression but also responses to treatments that slow or halt disease.

NF can be detected in both blood and cerebral spinal fluid (CSF). In addition, a lot is already known about NF because it has been studied as a biomarker for several neurological diseases, including SMA. For these and other reasons, the working group chose NF as the most promising biomarker to develop for SMA research and clinical care. They also decided which research questions about NF still need to be answered. Finally, the working group outlined which types of experiments would be helpful in answering these questions and validating NF as an SMA biomarker.



This illustration was adapted from the University of Basel, Hospital Basel.

Impact of Findings

The U.S. Food and Drug Administration (FDA) has approved three drugs for the treatment of SMA. About 70% of people living in the U.S. with SMA have received one or more of these treatments. In addition, researchers continue to study many [more drugs that may be useful in treating SMA](#). Some of the new drugs being researched may be used in combination with currently approved treatments to give people with SMA the best possible quality of life. As the number of approved SMA drugs increases, and more people are treated with different combinations of SMA drugs, developing biomarkers that inform research and healthcare decisions is more important than ever.

Advocacy Update

Cure SMA Conference Attendees Identified Their Top Advocacy Priorities



A record-number of adults with SMA and families with children with SMA attended the 2023 Cure SMA conference this summer. Along with attending workshops and participating in the conference activities detailed in this issue of Directions, conference attendees also had opportunities to raise awareness about SMA and the advocacy issues that mattered most to them.

At Cure SMA's new interactive conference exhibit (known as "The Hub"), individuals with SMA and their families identified their top advocacy priority and posted it on a large "What Do You Advocate For?" banner visible to all conference attendees. The banner quickly filled with post-it notes identifying housing, transportation, healthcare, air travel, SMA research, and disability rights as being among the top priority areas for the SMA community.

In addition, conference attendees were able to act on these important issues directly onsite through Cure SMA's Advocacy Action Center. To learn more about the advocacy priorities of the SMA community (including issues identified at conference) and advocacy engagement opportunities, visit Cure SMA's web site at <https://curesma.org/advocacy>.

Final Two States Aiming for Early 2024 SMA Newborn Screening Implementation

Currently, about 99% of all babies born in the U.S. are screened for SMA. The final two non-screening states are Hawaii and Nevada. However, that could change soon! Hawaii and Nevada officials have told Cure SMA that they are both working toward implementation dates in the first quarter of 2024. While state plans may change, we are hopeful that we'll hit our goal of universal screening of SMA in all 50 states sometime in early 2024. If we achieve this milestone, it will be a result of the education and advocacy efforts led by the SMA community. Thank you! Your advocacy is making difference.



Reach out to Cure SMA Advocacy at advocacy@curesma.org if you have any questions or visit <https://curesma.org/advocacy> for more information.

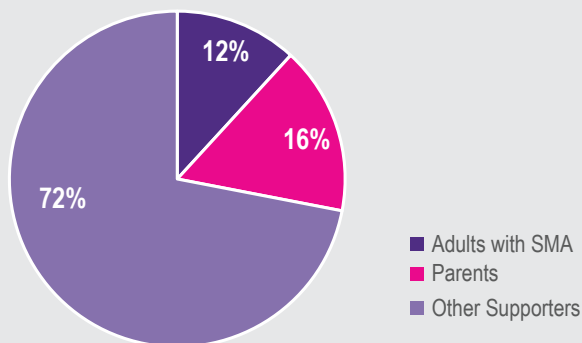


ADVOCACY ACTION CENTER

Cure SMA'S Advocacy Community: Growing, United, & Active: Why You Should Become an Advocate

Cure SMA Advocates

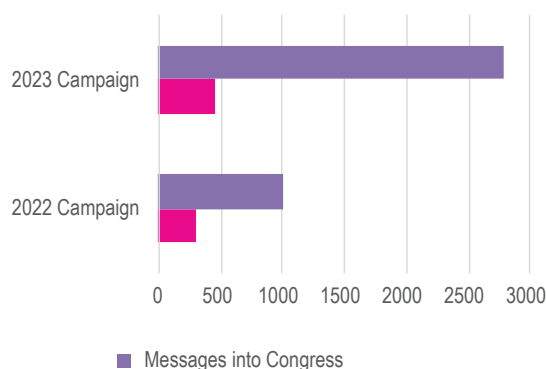
[Cure SMA Advocate (noun): a person with spinal muscular atrophy, their family members, friends, or other SMA community supporters who join Cure SMA's Advocacy Program to learn about and advocate for SMA community priorities.]



As of September 1, 2023

The recent growth in Cure SMA Advocates—which has increased by 138% since Cure SMA launched its new online advocacy tool—has led to greater levels of advocacy engagement and resulted in legislative and regulatory wins for the SMA community.

Cure SMA upgraded its online advocacy tool in 2022 to make it easier for individuals with SMA, their families, and others to advocate in support of everyday living priorities of the SMA community. Since, the number of Cure SMA Advocates has grown to 3,000, including Advocates in 433 of the 435 U.S. congressional districts and all 50 states, the District of Columbia, and Puerto Rico.



More Cure SMA Advocates = Greater Advocacy Engagement & Impact

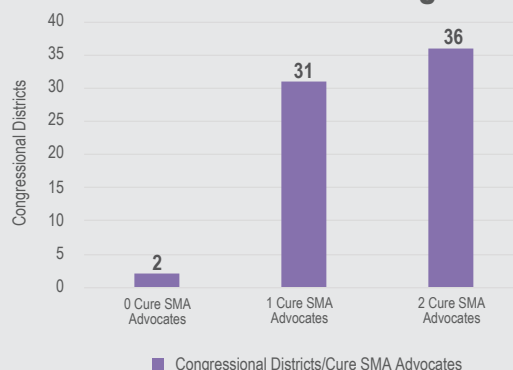
Advocacy engagement and impact have grown with the increase in Cure SMA Advocates. Cure SMA's most active advocacy campaign in 2022 was in support of new SMA research at the National Institutes of Health (NIH). Cure SMA Advocates sent more than 1,000 individual messages to Congress through the campaign. The engagement was successful as Congress included a provision in last year's NIH budget highlighting the unmet needs of individuals with SMA and the importance of funding new SMA research. The most active advocacy campaign thus far in 2023 was around accessible air travel.

The campaign has already surpassed 2,700 messages, reaching 454 of the 535 Members of Congress. Because of the SMA community's increased level of engagement, nearly every U.S. Senator and Representative heard directly from a constituent about the challenges individuals with SMA face when traveling by air. By comparison, the 2022 SMA research campaign reached only half of Congress.

The Need to Grow the Cure SMA Advocate Network and Increase Advocate Engagement

A larger, more engaged network of Cure SMA Advocates has already helped to increase awareness of SMA and the advocacy goals of the SMA community. However, to meet existing and future needs of the SMA community, more growth and engagement are needed. Currently, there are 70 congressional districts with two or fewer Cure SMA Advocates, including two with zero Cure SMA Advocates. Some of these districts are represented by committee chairs and other congressional leaders who have influence over legislation important to individuals with SMA and their families. It is critical that Cure SMA has many advocates, including adults with SMA and families of children with SMA, in each congressional district so that every Member of Congress understands how proposals they are considering will affect children and adults with SMA and their families. In addition, committee leadership regularly changes, especially after an election, making it important to have strong and active Cure SMA Advocates in every state and congressional district. Members of Congress, including those who lead committees, especially value local perspective from their constituents. This targeted local advocacy is only possible through an active advocate network.

Cure SMA Advocates Per Congressional District



We Need Everyone's Help – How You Can Help

Advocacy is a team sport. It is critical that every member of the SMA community—whether you are a person with SMA, a family member, friend, or supporter—sign up as a Cure SMA Advocate and stay engaged throughout the year. To become a Cure SMA Advocate or to check your status, please scan the QR code. If your contact information prepopulates then you may already be a Cure SMA Advocate. Otherwise, you'll need to add your address and other contact information to determine your local, state, and federal elected leaders. Once you are a Cure SMA Advocate, consider acting on an advocacy campaign through Cure SMA's Advocacy Action Center. Your action matters and will make a difference.

State of SMA Report Recap

In early July 2023, Cure SMA launched its second annual State of SMA report. The purpose of this report is to share highlights from Cure SMA's databases using data through the end of 2022, including:

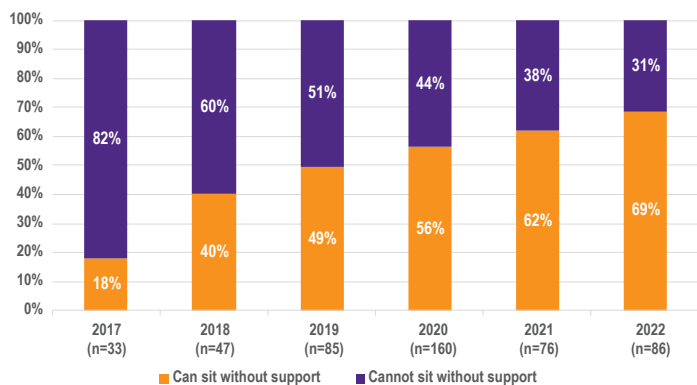
- the Membership Database with patient-reported outcomes on over 9,700 affected individuals worldwide that also includes data from our annual Community Update Survey,
- the SMA Clinical Data Registry (CDR) containing electronic medical record (EMR) sourced data for over 800 patients receiving care from U.S.-based SMA Care Center Network sites, and
- the SMA Newborn Screening Registry (NBSR) with parent-reported outcomes for over 70 babies with SMA identified through statewide SMA newborn screening.

The 2022 State of SMA report presents a current snapshot of the quickly changing landscape of SMA. More specifically, the report includes age and gender breakdown of the SMA community; employment and education of adults with SMA; prevalence of SMA type and *SMN2* copy number, use of FDA approved treatments, impact of newborn screening, and decreasing mortality rates.

Highlights from the 2022 report:

- Both children and adults are represented in Cure SMA databases. The average age was 22 years old, and 50% of individuals were 17 years or younger. The number of adults is increasing over time.
- Newborn screening has allowed for earlier diagnosis, regardless of *SMN2* copy number. Among the infants identified by newborn screening in 2022, (n=54), the reported average age of diagnosis confirmation was 0.4 months.
- From the annual Community Update Survey, the proportion of individuals with 2 *SMN2* copies that report the ability to sit without support has been increasing since 2017.

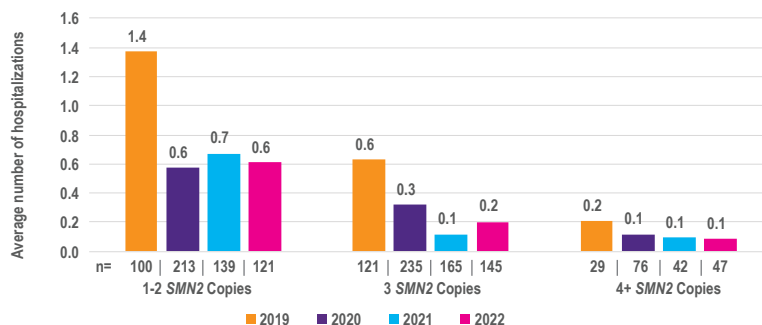
The proportion of individuals with 2 *SMN2* copies who report sitting without support has increased since 2017¹



Clinical Research & Care Update

- Additionally, participants from the annual Community Update Survey reported a reduction in the average annual rates of hospitalizations from 2019–2022.

Annual hospitalizations by *SMN2* copy number has decreased since 2019¹



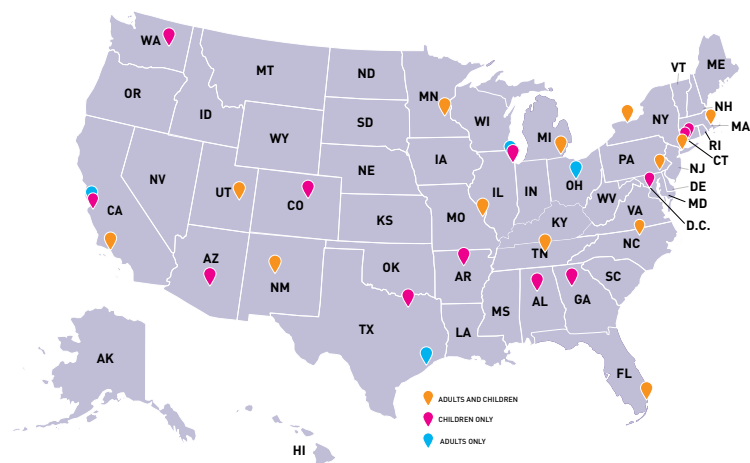
- The mortality rate of SMA in 2022 was approximately one-third of what it was in 2012. For every 100 people with SMA, the annual mortality rate decreased from 1.84 in 2012 to 0.63 in 2022.

Cure SMA hopes data from this report will foster future research, programs and development of additional therapies.

Cure SMA is thankful for all the members of the SMA community who have generously shared their data to make this report possible. Cure SMA also thanks the Care Center Network for their care of patients with SMA and work with the CDR. Cure SMA is also grateful for the support and funding provided by the Cure SMA Industry Collaboration (SMA-IC), and the Cure SMA Real World Evidence Collaboration (RWEC).



To access the 2022 State of SMA report, please visit:
www.curesma.org/cure-smas-2022-state-of-sma-report/



The Care Center Network

Thank you to the Cure SMA Care Center Network for their commitment to improving care for people with SMA and contributing consented patient data.

Since 2018, Cure SMA has partnered with hospitals across the U.S. with the goal of improving healthcare for people with SMA. Every Care Center Network site submits consented patient information and data to the Cure SMA Clinical Data Registry. This data is then analyzed to drive healthcare improvements.

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



The Cure SMA Real-World Evidence Collaboration

The Cure SMA Real World Evidence Collaboration (RWEC) was established in 2021 to leverage the experience, expertise and resources of pharmaceutical and biotechnology companies and nonprofit organizations involved in the development of SMA therapeutics to guide the future direction of real-world evidence collection and use in SMA. Funding for the development of the State of SMA was provided by the Cure SMA RWEC. Members of the RWEC include Biogen, Novartis Gene Therapies, Genentech/Roche Pharmaceuticals, and SMA Europe.



The Cure SMA Industry Collaboration

The Cure SMA Industry Collaboration (SMA-IC) was established in 2016 to leverage the experience, expertise, and resources of pharmaceutical and biotechnology companies, as well as other nonprofit organizations involved in the development of spinal muscular atrophy (SMA) therapeutics to more effectively address a range of scientific, clinical, and regulatory challenges. Current members include Cure SMA, Biogen, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, Genentech/Roche Pharmaceuticals, and SMA Europe. Funding for the research included within the State of SMA Report was provided by the 2022 SMA-IC; members include Cure SMA, Biogen, Genentech/Roche Pharmaceuticals, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, and SMA Europe.

2023 Fall Immunization Recommendations

Cooler weather serves as a reminder to explore how to keep ourselves healthy through the cold and flu season.

What you can do to protect yourself and others from winter illnesses

Avoiding others who are ill, sanitizing and/or washing hands often, covering coughs and sneezes when possible, and wearing a mask when in large groups all contribute to having a healthier winter. In addition, getting annual immunizations for flu and an updated COVID-19 vaccine will also decrease the severity of these illnesses.

A vaccination or immunization is a preparation that is most often administered through needle injections to stimulate the body's immune response against diseases. Generally, antibodies start to increase two weeks after a vaccine is given. There are many more viruses and the majority do not have a vaccine to boost the immune system.

Influenza

Influenza is a serious viral respiratory infection that is caused by influenza viruses, types A and B, which infect the nose, throat, and lungs. People with SMA, young children and people 65 years and older are at risk for serious flu complications. Typically, influenza season begins in October and peaks between December and February. Common symptoms include fever, cough, sore throat, runny or stuffy nose, headache, muscle and body aches, and fatigue. People with flu are most contagious in the first three to four days after illness begins. Influenza is spread by airborne droplets made by people with flu when sneezing, coughing, and talking that land in the nose or mouth or on the hands of others who touch their nose, mouth, or eyes. The usual time from exposure to symptoms is approximately two days.

The influenza vaccine has been shown to reduce flu symptoms and decrease the risk for serious complications. The flu shot may not prevent infection, but it will take the infection from "WILD to mild" per the Center for Disease Control and Prevention (CDC). The annual seasonal flu shot is recommended for everyone six months of age and older. Please note that the nasal spray Live Attenuated Influenza Vaccine (LAIV) is not recommended for people with SMA.

People with SMA and their caregivers and family should all receive the influenza vaccine. Common vaccine side effects include soreness, redness and/or swelling at the injection site, headache, fever, nausea, fatigue, and muscle aches that resolve in 1-2 days.

If you have SMA and you become infected with influenza, please contact your healthcare provider to discuss possible antiviral medication for influenza. This is a medication that when started early in the course of infection can decrease the duration and severity of symptoms.

COVID-19

COVID-19 is a viral respiratory infection that is very contagious, spreads very quickly and is also spread by infected respiratory droplets. COVID-19 infection symptoms are similar to other viral illnesses with the exception that loss of taste and/or smell is more often associated with COVID infection, however, this is not a consistent symptom. To determine which infection someone may have, consider testing for the viruses.



To protect against COVID-19 infection get the new COVID-19 vaccine (2023-2024 formula). Antibodies against COVID-19 wane over time. The COVID-19 vaccine has been shown to reduce symptoms and decrease the risk for serious complications. Although COVID-19 is rising across the U.S. now, the overall hospitalization rate is lower than last year thanks to immunity from vaccinations and prior infections. New COVID-19 vaccines (2023-2024 formula) were approved in September 2023 for people 12 years of age and older. For children 11 years of age and younger, the vaccines are available under an emergency use authorization similar to the initial launch of the COVID vaccine.

If you have SMA and you become infected with COVID-19, please contact your healthcare provider to discuss an antiviral medication for COVID-19 to decrease the severity of symptoms. This is a medication that is most effective when started within a few days of developing symptoms.

Respiratory Syncytial Virus (RSV)

RSV is another common viral respiratory infection that usually causes mild, cold-like symptoms during the fall and winter. However, infants, young children, older adults and people with SMA are at risk for serious complications including breathing difficulty and pneumonia that can result in hospitalization.

Adults ages 60 years and over with or without SMA should consider receiving the RSV vaccine that was approved in July 2023. Antibodies persist for 2 seasons. Please discuss the benefits and risks of this new vaccine with your healthcare provider.

To protect a young infant from severe RSV infection, people who are 32-36 weeks pregnant during September through January may receive the new RSV vaccine. This will result in maternal RSV antibodies that will transfer to the infant prior to birth and protect the infant from severe RSV illness during RSV season. Alternatively, after birth infants with SMA may receive RSV antibodies during their first RSV season either through one injection (Nirsevimab) or a series of five monthly injections during RSV season (Palivizumab). These antibodies wane over time.

In summary, please obtain your flu shot and new COVID-19 shot to help you stay healthy through the winter. Take this time to also review your illness intervention plan with your healthcare team. Please consult with your healthcare provider about your care and the above information. If you are taking part in a clinical trial, please also contact your study team. If you qualify for RSV vaccine, are pregnant or have a young child with SMA please consider and discuss RSV vaccination with your healthcare provider.

More information may be found at:

Influenza: www.cdc.gov/flu/

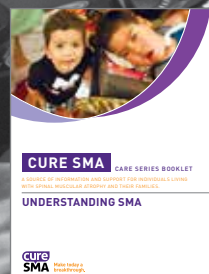
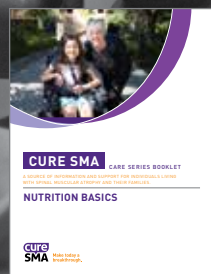
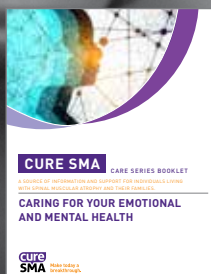
COVID: www.cdc.gov/coronavirus/2019-nCoV/index.html

RSV: www.cdc.gov/vaccines/vpd/rsv/



Cure SMA is committed to providing the entire community with the information you need to make decisions about treatment and care.

Check out the updated Cure SMA Care Series Booklets.



Find them online: www.curesma.org/care-series-booklets/

Booklets are available online and are translated into 12 languages.

**You may be gone from
my sight, but you are never
gone from my heart.**

**In remembrance of all of the lives
lost to Spinal Muscular Atrophy.**

2023 National Partners

Thank you to our National Partners for their continued support of Cure SMA!
Without it, many of our events and programs wouldn't be possible.

National Premier Partners



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Resources and Communications Partners





Big Moves for the DellaMaggoria Family

"I could talk about Lincoln all day," says Katee DellaMaggoria, mom to five-year-old Lincoln who has SMA Type 1. Lincoln was diagnosed at 11 weeks of age, had seven doses of Spinraza® and was dosed with Zolgensma® at 17 months old. Lincoln has been on Evrysdi® since he was three years old and continues to gain strength and endurance.

Katee recently finished an application for a grant for an ADA van for Lincoln and the family has been celebrating because Lincoln is a recent graduate, graduating from power wheelchair driving school!

For the last year, Lincoln has been working weekly during his physical therapy sessions practicing driving a power wheelchair. He finally passed all the requirements from driving through three doorways to going 50 feet in a straight line to even avoiding an obstacle, for which his toddler sister, Rey proved to be a good learning tool! The power chair has been ordered and Lincoln is so excited to get it and experience independence for the first time. He can't wait to race around the bases at his baseball games with his local Miracle League.



Get ready to Bake a Difference this Holiday Season with Cure SMA!

Calling all mini bakers with big hearts!
Join our sweetest campaign, where kids
whip up love in the form of cookies, cakes,
and cupcakes all in support of Cure SMA.

Baking a Difference was created by kids
for kids with sweet hearts and a love of
sweet treats. Roll up your sleeves and
roll out the dough! This holiday season
let's bake a difference together for those
affected by SMA.

Ready to be a dough-gooder?

Join Cure SMA's Baking a Difference
campaign today and register here:



cure
SMA

**Baking
a
Difference**

Have questions?

Contact us at fundraising@curesma.org
for more information.





SUMMIT OF STRENGTH PROGRAM

The Summit of Strength is a community educational program hosted by Cure SMA, crafted to provide people of all ages and types of SMA and their caregivers the opportunity to network and learn about the latest advances in treatment, care, advocacy, and support.

The Summit of Strength program will be coming to 15 cities across the United States in 2024! This will be the program's seventh year, after six successful years of 95 summits held with almost 200 incredible speakers and over 4,000 attendees.

All locations and dates will be announced in the coming months! Stay up-to-date at www.curesma.org/summit-of-strength/

This is a free program which 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 parent or caregiver.

“

“Useful and effective with powerful presentations and speeches by distinguished professionals and well-done job by team for hosting these kinds of events.”

“It was our very first time attending a summit and it was wonderful. We received so much information regarding studies. It was close to home, and we were able to meet with some SMA families we haven't seen since before pandemic. Thank you so much!”

“Loved meeting people face-to-face!”

“Grateful to have the opportunity to meet with the sponsors. After 20 years on this journey, we still learn something that helps at EVERY event. Thank you!”

”



We look forward to celebrating our 100th Summit of Strength in 2024!



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



This program is made possible by our generous donors in the SMA community. Cure SMA wishes to thank our sponsors for the 2023 Summit of Strength Program – National Presenting Sponsors, Biogen and Genentech, National Platinum Sponsor, Novartis Gene Therapies, and National Visionary Sponsor, Scholar Rock and Biohaven Pharmaceuticals.

SATURDAY, JANUARY 27, 2024

San Juan Marriott Resort 1309 Ashford Ave., San Juan, 00907, Puerto Rico



2024 SAN JUAN, PUERTO RICO

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

SUMMIT OF STRENGTH

Cure SMA invites you to attend the San Juan, Puerto Rico Summit of Strength on Saturday, January 27.

The Summit of Strength is a community educational program hosted by Cure SMA. These in-person events provide attendees the opportunity to learn from an array of national and local experts surrounding topics that are valuable to individuals of all ages and types of SMA, and their caregivers.

This is a free program which includes a meal and parking for all attendees.

Cure SMA les invita asistir el Summit of Strength de San Juan, Puerto Rico el sábado, 27 de enero

Summit of Strength es un programa educativo para la comunidad, patrocinado por Cure SMA. Estos eventos en persona proveen a los asistentes la oportunidad de aprender de varios expertos nacionales y locales sobre temas importantes a individuos de cualquier edad y tipo de AME, y a sus cuidadores.

Este es un programa gratis que incluye comida y estacionamiento a todos los asistentes.

Register here! / ¡Inscríbanse aquí! summit.curesma.org/SanJuan

WALK-N-ROLL

There will be another special event taking place after the Summit of Strength program, called a Walk-n-Roll. This is a fundraising program that brings communities together to support Cure SMA's funding of life-changing research, resources, and programming for those impacted by SMA, and is another great opportunity for community networking.

Habrará otro evento especial llamado Walk-n-Roll que tendrá lugar después del programa Summit of Strength. Este es un programa de recaudación de fondos que reúne a las comunidades para apoyar la financiación por parte Cure SMA de la investigación, los recursos y la programación que cambian la vida de las personas afectadas por la AME, y es otra gran oportunidad para establecer contactos con la comunidad.

Cure SMA wishes to thank our National Presenting Sponsors for the 2024 San Juan event, Biogen and Genentech.
Cure SMA desea agradecer a nuestros patrocinadores presentadores nacionales por el evento de San Juan 2024, Biogen y Genentech.



Biogen

Genentech

A Member of the Roche Group

For more info or questions please email communitysupport@curesma.org.
Para más información o si tiene alguna pregunta, comuníquese con communitysupport@curesma.org.



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



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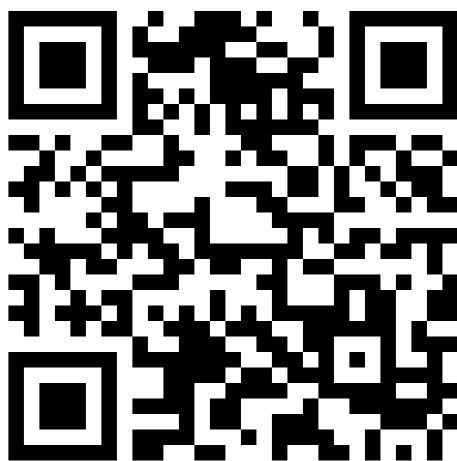
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**cure
SMA**

#CureSMA and #SMACommunity



Spring Walk-n-Roll Highlights

As of Fall 2023

**cure
SMA**

WALK-N-ROLL



Together, we raised more than

\$625,000

Thank You!

HIGHLIGHTS

Thanks to the our incredible event committees, fundraisers, team captains, and sponsors, Cure SMA had:

- 19 Walk-n-Roll events held across the country this Spring
- Over 250 Walk-n-Roll Teams who fundraised
- Participants who raised an average of \$165 each



WALK-N-ROLL CHAMPIONS CLUB

Cure SMA Spring Walk-n-Roll Champions Club Members

Maggie Moore	LeeAnn Loyd	Chelsea Braekevelt	Amy McGuinness	Allyson Henkel
Mike Enderlin	Beth Moore	Devon Decker	BarbaraJean Mirabile	Rachel Miele
Olivia Enderlin	Mary Moore	Gretchen Dorer	Brian Murphy	Jenna Plastino
Christine Hart	Jack Resendez	Aryn Manni	Silvia Murphy	Travis Hensley
Jonathan Lasko	Kristen Resendez	Caitlin Petras	Siobhan Murphy	Fiorennna Fuentes Stark
Derek Lewis	Ramiro Resendez	Karen Suber	Erik Peterson	Joanna Tewfik
Tina Lewis	Audra Butler	Michelle Werner	Emily Ruprecht	Amber-Joi Watkins
Beverly Venedam	James Geueke	David Atchison	Joe Scully	Maureen Ford
Caitlin Brown	Matthew Phelps	Laura Atchison	Erin Sheehan	Sydney Knobel-Graves
Sherry Langland	Lindsay Rodriguez	Jay Backstrom	Lynne Vaudry	Sandy Schwegel Kline
Beth Lockwood	Elizabeth Copeland	Marisa Boidi	Rose Wilson	Faith Zacheis
Lola Savage	Ceri Devine	Carrie Capone	Leah Young	Casey O'Neill
Donna Czeck	Maria Pimentel Munguia	Abigail Druffner	Christie Barnard	Winnie Stanley
Emily Czeck	Christina Reed	Lauren Gargan	Emily Farkas	Brittainy Howard
Bonnie Grimmett	Matthew Valley	Cristle Gordon	Nichole Finizio	Megan Nolan
Maria Meyer	Darlisha Barnes	Heidi Johnson	Barbara Fleming Brzycki	Jordan Salomone
Jacob Loyd	William Becker	Sandra Mahoney	Albert Freedman	

We would like to congratulate the following spring 2023 Walk-n-Roll participants for being inducted into the 2023 Cure SMA Walk-n-Roll Champions Club! This program recognizes and rewards individual participants who raise at least \$1,000 at their respective Walk-n-Roll events. Congratulations and thank you to this esteemed group of people who are leading the charge in making the world a better place for the SMA community.

Learn more about Cure SMA's Walk-n-Roll events by visiting <https://walk-curesma.donordrive.com/>.

Double Your Impact: Match Your Donation Through Your Employer

Did you know you may be able to double your support for Cure SMA and the SMA community without giving a dollar more? Turn \$25 into \$50 or \$50 into \$100 through employer matching! If you or your partner works for a company that offers a matching gift program, you can ask the company to match your donation.

According to 360MatchPro, between \$4 and \$7 billion dollars in potential matching gifts to nonprofits goes unclaimed each year. Imagine what could be done with those funds! 360MatchPro says, "This is a direct result of a lack of awareness among donors. Many donors don't know their donations are eligible for a match through their employer."

Employers who offer matching programs are more common than you might think. 65% of employers offer corporate matching programs and more than 26 million people work for companies who match gifts, making it likely that you or someone you know can double your donation.



I donate to Cure SMA through my company's matching program because my donations are **DOUBLED 2X** and have a greater benefit for people with SMA. I'm proud to work for a company that increases the impact of my charitable contributions.

~ Rosa Chang Claro, Director,
Global Black Belt Sustainability, Microsoft



Want to see if you're eligible? Visit curesma.org/employer-gift-match and search your company in our online gift match tool. If your employer qualifies, you can follow the simple steps to create twice, or perhaps even three times the impact!



Winds of Change: Cure SMA Launches Pinwheels of Promise at Walk-n-Roll Events

“A pinwheel also needs wind. And with our actions and our intentions, we can be that wind. We have to be those agents of change in our communities.” ~ Josh Charles

During our spring Walk-n-Roll season, Cure SMA rolled out our new Pinwheels of Promise program, serving as a way for walk participants to recognize, remember, and reflect on their unique journeys in our #SMAcommunity.

Whether you attend a Walk-n-Roll because you live with SMA, you are remembering someone lost to SMA, or participate in support of someone with SMA, all experiences are honored at our Walk-n-Roll events in our Pinwheel of Promise Program.

Each team that attends a walk receives a pinwheel sign, which they can decorate at the event with photos and messages of hope to be displayed along the Walk-n-Roll route.

Through your participation, our goal is to build a garden of pinwheels at every Walk-n-Roll event to help us remember, honor, reflect, support, and connect across your diverse experiences and journeys with SMA. Our pinwheels are a promise to the past, present, and future of our SMA community.

All 2023 Walk-n-Roll participants who attend a walk in person will receive a commemorative Walk-n-Roll pinwheel lapel pin in one of three varieties:



Orange Pin – I have SMA



Blue Pin – I have lost someone to SMA



**Purple Pin – I support someone with SMA or
I support Cure SMA**

cure SMA DO IT YOUR WAY

FUNDRAISING

is the perfect way to host your own event

Make an impact through fundraising your way
with events as unique as those who host them!

DIY FUNDRAISING CATEGORIES:



GATHERING FOR GOOD:
COMMUNITY EVENTS



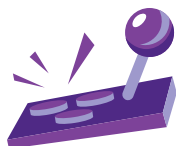
YOUTH FUNDRAISING
INITIATIVES



SLAM DUNK SMA:
ATHLETIC EVENTS



MEANING TO YOUR
MOMENTS: LIFE EVENTS



SUPPORT THROUGH STREAMING:
ESPORTS EVENTS



Scan the QR code above to visit the DIY website at: <https://www.curesma.org/diy-fundraising-program/>

Ready to get started or have a unique fundraising idea? Contact diy@curesma.org

Here are some of the recent events held by community members like you!



Pancake Away SMA

When Marnie and Trav found out their son, Davy, had SMA, they were the second family in their small group in the span of two weeks to discover their child had been born with a rare disease. Their neighbors and good friends had a daughter 3

weeks older than Davy who had recently received a CF diagnosis. In April of 2023, the Harris family and their friends decided to come together and raise money and awareness for both diseases, hosting a pancake breakfast as a joint fundraiser for Cure SMA and the CF Foundation in honor of their kids. They raised a total of \$7,000 for both organizations, and it was a great way to spread awareness of these rare diseases to with their neighbors and friends in Georgia.



Laps with Luke

In May of 2023, the 2nd Annual Laps with Luke fundraiser raised over \$12,000 at a local elementary school in North Carolina. The Whitlers invited friends, family, and community members to join them on the track to walk, run, or roll as many laps as they could! For this field day fundraiser, children were given tokens for every lap they could

complete. Each token represented \$1 that the Whitler family would donate to their fundraiser at the end of the event! Families gathered to challenge themselves on the track, do yoga, participate in strength training, complete relays, and simply play on the field—all while raising funds and awareness for Cure SMA. Luke and his family had an amazing day celebrating the community that has supported them and cheered them on throughout Luke's diagnosis of SMA.

Caring for Calvin Hog Show

Ever since his Grandpa was a little boy, Calvin's family has been in the show livestock world. When deciding on how to fundraise for Cure SMA, the family got creative and decided to host the Caring for Calvin Hog Show! Spearheaded by Calvin's parents and Uncle Kyle, the annual livestock show features concessions, virtual silent auction, and –you guessed it–lots of hogs! Support from family, friends, and the local community has allowed the Braekevelt family to not only spread awareness about SMA, but also raise over \$10,000 this past Spring for Cure SMA! The family looks forward to expanding the event in years to come, and can't wait for Calvin to participate himself!

Play For SMA



Best friends and Falmouth High School hockey co-captains Mitchell Ham and Sam Belliveau were given the opportunity to choose any topic for their senior project in Spring 2023. Because Mitchell's nephew, Isaac, has SMA Type 1, they

decided to research SMA. Once the project was turned in though, they didn't want to stop there. The duo decided to host a hockey fundraiser for Cure SMA! Mitchell and Sam rallied twenty-plus graduating seniors, lower classmen, and alumnae to play in a post-season bonus game where they could get back out on the ice for a tremendous cause. Isaac, Mitchell's biggest fan, was always ready to cheer on the Falmouth Navigators! This game was so special for Isaac, because he was able to visit the teams in both locker rooms, request songs from the DJ down (the players loved warming up to some Paw Patrol!), and greet everyone coming in for the game with Cure SMA swag, snacks, and gratitude. The arena was filled with fans including Isaac's homcare nurse, extended family, parents of players, friends, and more—all cheering and dancing. With support from the local Maine community, Mitchell and Sam raised \$4,000 for Cure SMA! Isaac is beyond grateful for the duo's efforts to support an organization that does so much for SMA kiddos like him.



Donor Spotlight:

Stickane Family



A Community's Unwavering Love: How the Stickane Family Started a Foundation to Cure Spinal Muscular Atrophy

One of the key milestones in the Luke 18:1 Foundation's journey was starting the Links 4 Luke Golf Tournament, typically held in spring each year. This event is more than just a golf tournament; it is a testament to the Stickane family and community's unwavering commitment to finding a cure for SMA.

The tournament, held at a scenic Timarron Country Club, attracts participants from all walks of life, including community members, local businesses, and individuals passionate about finding a cure for SMA. The success of the tournament year after year has been astounding. Through generous donations, sponsorships, and participant contributions, the Luke 18:1 Foundation has been able to raise an impressive \$2,000,000. In April 2023, the event was held once again, and the Foundation generously granted Cure SMA a gift of \$500,181 at the 2023 Annual SMA Conference in June this year at Walt Disney World.

This significant contribution will undoubtedly accelerate efforts to find a cure for SMA and improve the lives of those living with the disease. It is a testament to the Stickanes' unwavering determination to make a difference in the SMA community.

The Stickane family's journey from SMA diagnosis to founding the Luke 18:1 Foundation and hosting a series of successful golf tournaments and fundraising events is a powerful example of what can be achieved when love, determination, and community support come together. Their story serves as a reminder to us all that in the face of strife, we have the power to make a positive and profound impact on the lives of others.

In a world where adversity often brings out the best in people, the Stickane family of Southlake, TX stands as a shining example of unwavering love and resilience. Daniel and Nicole Stickane's journey began when their youngest son, Luke, was diagnosed with spinal muscular atrophy (SMA), a rare and debilitating neuromuscular disorder. Faced with this challenging reality, the Stickanes decided to take matters into their own hands, launching the Luke 18:1 Foundation that would not only improve their son's life but also benefit countless others battling SMA. This year, their tireless efforts culminated in two remarkable events, the Links 4 Luke Golf Tournament and Mahjong Madness Tournament, raising an astounding \$500,000 for Cure SMA and providing hope for the future.

Luke's diagnosis with SMA was a turning point for the Stickane family. SMA is a genetic disorder that weakens the muscles and hinders the body's ability to move. It is a condition that challenges not only the affected individual but their entire family. The Stickanes' journey with SMA was not easy, but their determination and love for Luke compelled them to take action.

In the face of a new way of life, the Stickanes founded the Luke 18:1 Foundation, a non-profit organization dedicated to raising awareness about SMA, funding research for a cure, and providing support to families affected by the disease. The Foundation's mission was clear: to make a tangible difference in the lives of those living with SMA.

2023 United Airlines New York City Half Marathon

In March, 8 runners took to the streets of New York to compete in the United Airlines New York City Half Marathon for Cure SMA. Together, the runners raised over \$8,000 for Cure SMA. The New York City Half Marathon is an annual half marathon road running race. It passes through and by famous landmarks such as Central Park, Times Square, and Grand Central Station.

2023 Wakefield 5K benefitting Cure SMA

The Wakefield 5K, held on August 19 at Lake Quannapowitt in Wakefield, MA, marked a historic milestone for the event. With an astounding turnout of over 120 participants, this year shattered all previous records. More importantly, the event achieved its primary goal by raising an impressive \$15,000 for Cure SMA, showcasing the incredible generosity and community spirit of Wakefield.

2023 ASICS Falmouth Road Race

In August, 20 runners participated in the ASICS Falmouth Road Race in Falmouth, MA. The Falmouth Road Race was established in 1973 and has become one of the premier running events of the summer season. Each year the race draws an international field of Olympians, elite, and recreational runners out to enjoy the iconic 7-mile seaside course. Together, these 20 runners raised over \$27,000 and counting for Cure SMA.



About Our Endurance Program

The Cure SMA Endurance Program connects runners across the country to train for endurance events and fundraise for Cure SMA. Endurance athletes—both veterans and newcomers alike—train, fundraise, and bring awareness to support Cure SMA's mission of funding breakthroughs, changing lives, and empowering the SMA community.

Have questions or interested in participating in an event? Contact fundraising@curesma.org for more information.



GOLF OUTING *Updates*

2023 Birdies for Blake Golf Outing

The 10th, and final, Birdies for Blake Golf Tournament took place on July 29, 2023, at The Sharon Woods Golf Course in Blue Ash, OH. This year's event raised over \$43,000, and since its inception, the Birdies for Blake Golf Outing has raised over \$300,000 in support of Cure SMA!

A huge thank you to Mark and Nancy Farrell, Nick and Kacey Farrell, and Kate and Sam Queen for hosting this wonderful event each year, and for their continued support and dedication to finding a cure for SMA.

2023 Rocky Mountain Golf Tournament

The 2023 Rocky Mountain Charity Golf Tournament sponsored by Busick Insulated Glass took place on July 10, 2023, at the beautiful Pradera Golf Club in Parker, CO. Over 100 golfers attended the event which consisted of a 4-person scramble, breakfast, lunch, awards ceremony, and fun course games. With the support of this year's sponsors, donors, and players, the event raised over \$37,000 to help find a cure for SMA!

Cure SMA would like to extend a special thank you to event organizer, Gillian Faith, and her committee for continuing to host this tournament year over year.



2023 Muscles for Mckenna Evening of Hope

Congratulations and thank you to the Ellixson Family and the entire committee for hosting the 2023 Muscles for Mckenna Evening of Hope! Their 10th annual event took place on Friday, March 17, 2023 at the Crystal Tea Room in Philadelphia, PA.

Approximately 175 people enjoyed a lovely evening complete with a live and silent auction, fund-a-need, and live music. Additionally, this year we were lucky to be able to honor three amazing nurses from CHOP who work with SMA patients. Nominated by Dr. Brandsema, these three nurses continually go above and beyond for their patients, and we were thrilled to have them as our guests.

Thanks to the support of the community, sponsors, and attendees, the 2023 Muscles for Mckenna Evening of Hope raised nearly \$115,000 for Cure SMA this year!

Thank you to the Ellixson Family and their amazing committee for their continued support of Cure SMA! We cannot wait to celebrate with them at the 2024 Muscles for Mckenna Evening of Hope being held on March 8, 2024.



2023 Cure SMA Chicago Evening of Hope

On May 5, 2023, nearly 100 guests gathered for the Annual Chicago Evening of Hope at the Ivy Room in Chicago, IL. The evening featured cocktails, casino games, food stations, silent and live auctions, a raffle and more! The evening was a huge success, raising over \$70,000 for Cure SMA.

A special thank you to Brad and Krista Nunemaker for helping with the event. We also want to express our gratitude to the dedicated community members, generous sponsors, and amazing volunteers, for making this day possible.

2023 Arizona Evening of Hope

On Saturday, May 6, 2023, the Arizona Evening of Hope took place at OSHO Brewery & Distillery in Paradise Valley, AZ. It was the first time that this event had been held since 2019 and raised over \$7,000 in just a few short months! Approximately 75 attendees joined together for an amazing time of food, drinks, silent auction items, and much more, all while supporting Cure SMA!

A huge thank you to the wonderful Arizona Chapter members for bringing back this great event, and we invite all of our Arizona community to save the date for the next Arizona Evening of Hope taking place on April 27, 2024!



Chapter Updates



Awareness Events

With August being SMA Awareness Month, several of the Cure SMA Chapters worked to plan some great events such as Dine 2 Donates, Picnics and a day at the zoo to gather other community members from their chapters and help spread awareness at the same time.

In **Arizona**, families were able to enjoy a tasty meal while also raising funds for Cure SMA at a few local restaurants across the state. Thank you to Chapter Leader Angel Wolff for organizing all these great Dine 2 Donate events!

In the **Southern California** Chapter, Community members gathered at Irvine Regional Park for a tasty cookout, as well as an SMA Awareness Month Candle Lighting Ceremony. There was a great turnout, and it was a perfect opportunity for people to meet with friends and families from across the chapter. Thank you to Chapter Leaders, Autumn & Rickk Montoya for planning such a great event!

Community Members of the **New Mexico** Chapter also gathered for a great SMA Awareness family outing at the Albuquerque Zoo! A great time was had by all exploring the zoo grounds and connecting with other families and friends from the New Mexico Chapter. Thank you to Chapter Leaders, Annie & Joe Wiseman, for planning this fun day at the zoo!

SMA Awareness Month Proclamation & Awareness Lightings

There are many ways to participate in SMA Awareness month and many members of the SMA Community took the opportunity to help spread awareness by working with local government officials to officially declare August as SMA Awareness Month. Additionally, many people across the U.S. worked with local landmarks in their hometown to light up buildings and bridges in Cure SMA's colors purple & orange to recognize the month!

In **Michigan**, Ian Zurawski has taken the time every August for many years to submit and work with the Governor to declare August as SMA Awareness Month, and in Indiana, Jessica Biggerstaff successfully worked with the Governor to also declare August as SMA Awareness month for another year! Thank you to everyone who submitted requests for SMA Awareness Month Proclamations.

Another great way to help spread awareness is with Landmark lightings. Many states have large buildings in their metropolitan downtown areas that will light up in requested colors to recognize specific communities and organizations, and many states lit up brightly in orange and purple in honor of the SMA Community. Thank you to Shaakira Thomas (Hawaii), Jessica Biggerstaff (Indiana) and all other Cure SMA Community members who took the time to organize an awareness lighting in their hometowns!

If you are interested in learning how to organize a state proclamation or awareness lighting in your home town, please reach out to Shannon O'Brien at Shannon@curesma.org



Annual SMA
Conference



2024 ANNUAL SMA CONFERENCE

THURSDAY, JUNE 6TH – SUNDAY, JUNE 9TH

JW Marriott Austin in Austin, Texas

Every year, Cure SMA sponsors a conference to bring together the leading SMA researchers and clinicians, as well as individuals and families living with SMA. Cure SMA has been hosting the Annual SMA Conference since 1988. The weekend is filled with a wide variety of workshops along with a Family-Friendly SMA Researcher Poster Session, memorable Children's Program, Meet & Greet with a family fun fest, teen and adult social activities, PJ Party & Movie Night, and other special evening events, as well as many opportunities to connect and interact and receive first-hand updates from the researchers and clinicians.

We look forward to reuniting as a community at this conference and showing our support for others. As always, the Annual SMA Conference and the SMA Research & Clinical Care Meeting run alongside each other. This is the largest conference in the world for those affected by SMA and those involved in providing support and care for SMA patients. There is no other program like it. We are expecting another great attendance of well over 2,500 attendees.

JW Marriott Austin is an elegant hotel overlooking downtown and Lady Bird Lake, with trendy indoor-outdoor eateries and numerous bars. Relax and unwind with any of the luxurious spa services at Spa by JW, located on the fifth floor. If you are looking to explore the city, the hotel's spectacular location places you just minutes from the State Capitol, University of Texas at Austin, lakefront trails, restaurants, museums, and vibrant neighborhoods.

You must complete your conference registration with Cure SMA prior to reserving your hotel room, for the special room rate of \$249 per night, plus tax. Self-parking will be \$25 per night.

SMA Conference Goals

- To welcome newly diagnosed families and individuals into the SMA community.
- To help keep our SMA community strong and unified.
- To educate SMA families and individuals with SMA, and provide updates on medical issues, research, and clinical trials.
- To facilitate networking and data sharing between researchers, clinicians, families, and individuals.
- To educate researchers and clinicians on the latest research advancements.
- To attract the best researchers and clinicians to the SMA field and encourage collaborations and investments.
- To promote cross-disciplinary dialogue among academic, clinical, and industrial researchers.

Conference Scholarships

Cure SMA also offers financial assistance to help those who are looking to attend the Annual SMA Conference.

If you have any questions about conference scholarships, please reach out to our Community Support Team by emailing communitysupport@curesma.org. For more information on scholarships along with ongoing conference updates can be found at www.cureSMA.org/annual-sma-conference/.

TO REGISTER FOR
the Annual SMA Conference,
please visit www.curesma.org

Contact Us:
Cure SMA
925 Busse Road
Elk Grove Village, IL 60007

(800) 886-1762
 conference@cureSMA.org
 www.cureSMA.org

NEW Cure SMA Merchandise!

- Belt Bag \$20
- 40oz Travel Mug \$30
- Dark Lavender Long Sleeve Tee \$30
- White Cropped Logo Crewneck Sweatshirt \$35
- Black Long Sleeve Circle Tee \$30
- Wheelchair Joystick Replacement Knob \$25



Share a photo of you sporting your Cure SMA gear to communitysupport@curesma.org and you may see it in a future publication or our next issue of Directions!

Check out these items and more available in our online store at www.curesma.org/merchandise to support our mission and raise awareness by purchasing Cure SMA merchandise!

FINAL SALE



**Annual SMA Conference Tees
was \$10 now \$5**

**V-Neck Scrub Top
was \$35 now \$10**

**Valentine's Day Goodie Bag
Bundle was \$30 now \$24**

**Reusable Face Mask
was \$15 now \$5**

**We Can Cure SMA Doggie
Tank was \$20 now \$10**

**Cure SMA Zippered
Pouch was \$14 now \$10**

**Women's Performance Polo
was \$28 now \$15**

**Men's Performance Polo
was \$28 now \$15**

Get these items while you can! Supplies are limited.

Your story matters!

Share Your Story with Cure SMA!



Whether you're new to Cure SMA or a seasoned member of our community, we want to get to know you better and learn about your story.

That's why we created three easy ways to share your story.

1. **Record a Video** Our video recording tool makes it quick and easy to record a video that we can share on social media, via emails, etc. Simply type this link into your browser and follow the prompts to record: <https://gather.video/XL22>
2. **Set up an Interview** Email stories@curesma.org and a member of the Cure SMA team will reach out to you to discuss your story. Many of these stories are featured as monthly Story Spotlights on curesma.org
3. **Post on Social Media** Share your story in your words. Use #storyeSMAtter and #CureSMA

"We are all storytellers.
We all live in a network of
stories. There isn't a stronger
connection between people
than storytelling."

~Jimmy Neil Smith



Share your story, stories@curesma.org

We can't
wait to hear
from you!

VISION AND MISSION STATEMENT

Cure SMA leads the way to a world where everyone impacted by spinal muscular atrophy is empowered to lead independent, successful, and fulfilling lives. We strive to create a community where every individual is heard and feels welcomed. Cure SMA provides practical support programs for our community and advocates for their needs. We fund and direct comprehensive research that drives breakthroughs in treatment and we advance access to high quality care.

We will not stop until we have a cure.

OUR VALUES

Innovation

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

Balance

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

Collaboration

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

Respect

There is no “right way” to live with a disease like SMA. 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 with SMA and their families, and communicate openly and honestly, giving them clear and accurate information.

Determination

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



We are a non-profit, 501(c)3 tax exempt organization. Funds will be specifically directed to scientific, educational, or literary purposes in keeping with a charitable organization. The organization is proud to provide funding to the Cure SMA newsletter and website, which provides information and networking opportunities.

Submissions

To submit articles or make other contributions to our newsletter, please contact us at: stories@curesma.org. Digital images are encouraged!

Change of Address

Send changes, including ZIP code to: info@curesma.org or call 800.886.1762 or mail to: Cure SMA, 925 Busse Road, Elk Grove Village, IL 60007

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Cure SMA does not support or endorse any particular treatment or therapy. Information contained in this newsletter should not be used as a substitute for consultation with a qualified healthcare professional.

*Cover photo: Various events from Cure SMA Community members.
Thank you to all of our SMA Community for joining us in person.*



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