

# DIRECTIONS

COMMUNITY NEWSLETTER | FALL 2022



RESEARCH • INDEPENDENCE • ACCESS

Cure SMA's Commitment to Our Community.

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





## LETTER FROM KENNETH

*As you'll read about in the pages of this issue,* our 2022 Annual SMA Conference was an enormous success conveying a clear desire from many members of our community to come back together in-person. We're also back and better than ever with our local Walk-n-Roll and Summit of Strength programs with more than 50 events taking place in-person this fall across the country. And we are now planning for an even bigger 2023 Conference which will be held June 29–July 2 in Orlando, Florida.

Next, I'd like to share some details about our key goals at Cure SMA. Advancing basic research is one of the cornerstones of our mission, and we're focusing now on funding new research to discover new non-SMN therapies and combination treatment approaches. Our goal is to gain the knowledge and understanding to restore strength and function.

We are also focused on key partnerships and campaigns including matched donation programs through the Erin Trainor Memorial Fund for the care centers and with Richard and Jane Nunemaker for basic research; clinical trial updates and webinars with our pharmaceutical partners; and now the Cure SMA Annual Campaign.

As we look toward the future with research, it is just as important that we also focus on the very real needs of the SMA community now, which include access to high quality care. That's why we're also working diligently to grow our Care Center Network and invest in a greater local presence. We are now up to nearly 30 active Care Centers across the U.S., halfway to our desired total.

Finally, in addition to research, treatment, and care, we are addressing opportunities to improve the daily lives of all people with SMA. Whether it is a lack of accessibility or the desire for greater independence, our support programs and advocacy initiatives make a difference today.

A connection between all these initiatives is the need for funding to help accomplish our goals. If you aren't doing so already, I encourage you to fundraise for our Walk-n-Roll program, give during our annual campaign and ask your friends and family to participate. 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 grants from Biogen and Genentech.

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# Letters from the Community



“I’m not sure whose idea it was to distribute the LifeVac devices to each SMA family, but it was the best thing ever! With expenses through the roof, I never invested in one. Then we received one from y’all. And tonight, I was so incredibly glad that I filled out that form to get one a year or so ago. I had it ready to go and tonight we had to use it on our five-year-old son with SMA Type 2. He choked on french fries. We thought it was such a safe food, but it was fast and luckily the LifeVac was quick to use and kept in an accessible spot. Thanks for looking out for us. Cure SMA does so much for us and I’m so incredibly thankful to everyone there.”

~ The Martinez Family

“Thank you so much for the lovely and helpful package! I find all the products extremely useful to help me function around my home. I’m especially enjoying the “hand grippers” as this has been an ongoing problem. I’m in the early stages of transforming my home into a “smart home” and the plugs are the first step in the process. Thank you again for the thoughtful and helpful products.”

~ Christy L.

“I wanted to let you know that my daughter received your care package for adults and teens. The one with a microphone, straws, and jar openers. We are so grateful! Thank you so much for all you have done for my family.”

~ Tori’s mom, Misty

“We received both packages and we can’t thank you enough. It has made a huge difference to us, and Violet is already loving big girl bath time.”  
Thanks again, it’s truly amazing!”

~ The Fitzgerald Family



“Recently, I requested the Teen and Adult Independence Assistance Package, Teen and Adult Support Package, and a large packet of information, and received it all promptly. I just wanted to thank you for providing these packages, as they’re full of items that people afflicted with SMA will likely find quite useful. Also, whoever packs the shipping boxes did a fantastic job. Everything arrived intact and fully functional, which is more than I can say about items for which I’ve paid handsomely. I’ll be making good use of everything in both packages, and I greatly appreciate the work you’re doing.”

~ Jennifer U.

“I recently received two of your Teen and Adult Independence Assistance Packages and I just wanted to reach out and say thank you! I love all the stuff; it will be very helpful.

Especially the Amazon Echo. I rely on technology a lot for my particular situation and daily activities, and I find these types of devices very handy for my needs. So overall I’m very happy with all the items and wanted to give you this feedback and thank you again!”

~ Haille C.

“Thank you so much for the resources you sent us as well as the incredible care package for our daughter—it brought me to tears. We are so appreciative of everything your group has done for our family over this last difficult month. We are very much looking forward to attending the conference in June and for a fun Disney weekend with our babies—it is giving us something to look forward to and be excited about during these difficult days.”

~ Ashley P.

“Thank you very much. We received the care package. It was so thoughtful, and our daughter was so excited.”

~ Stephanie W.



“The children received the gifts and are very happy about it! Thank you so much!”

~ Lavryk Y.



“Thank you for sending me Teen and Adult Support Packages. I just received them today. All the valuable items included in these support packages are useful and will make the activities of my daily living easier and more convenient. Thank you for providing information and resources to all SMA patients including me. You guys are doing a great job.”

~ Rahul M.

If you would like to submit a photo or story to be included in a future issue of *Directions*, please email [newsletter@curesma.org](mailto:newsletter@curesma.org).



Calen



Klym



Jude



Max

**Recognize the symptoms  
and save a life!**



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**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.**

**cure  
SMA**

**[SMARtMoves.CureSMA.org](http://SMARtMoves.CureSMA.org)**



# Community Spotlight:

## Joanna Whitler and Family

As a nurse practitioner, Dr. Joanna Whitler had never come across a patient with SMA, that was, until the patient was in her family. Joanna is mom to three-year-old Luke, who has Type 1 SMA and his brother, Bodhi.

“For nearly six months, Luke developed mostly “normally” but by seven months it was becoming clear that something was not quite right. He was not bearing any weight or pushing any resistance with his legs and generally seemed weak. We had him evaluated by a pediatric physical therapist (PT) and then our pediatrician. By the time we saw our doctor, Luke’s decline had started to progress quickly, and he had stopped moving his legs at all. Our pediatrician listened to my recounting, did an exam, and referred us to neurology where we were seen just two days later. After an agonizing week of waiting for tests results, we received our diagnosis: spinal muscular atrophy (SMA).”

Joanna said, “Luke was diagnosed young enough (seven months) to have significant benefit from recent treatments, but not young enough to benefit from newborn screening and beginning interventions in the first month of life or pre-symptom development. He is, as we often hear from healthcare providers, “a unique kid with few people ahead of him to learn and gather expectations from.”

“Luke received Zolgensma through a tiny vein in his foot on June 3, 2020, my 35th birthday, but the greater gift came two days later when I saw Luke’s legs move while he was on the changing table. His movement was coming back. He has made steady, sometimes slow, and sometimes rapid, progress and strength gains since.” “Evrysdi, which he started about six months after being treated with Zolgensma, seems to have spurred Luke’s strength gains along. It seems to push him out of plateaus that we more frequently experienced prior to starting the therapy. Physical therapy is a critical piece of Luke’s treatment. He participates in PT at least twice weekly where he is learning the skills and gaining the strength necessary to progress.”

Joanna, Luke, and the Whitler family’s experiences with their health care team have been wonderful and have also contributed to Luke’s progress. “Our care professionals have been incredibly receptive to our concerns and to any education we would provide.

Our neurologist and PT have been very proactive in their therapies and our other providers have been receptive to our requests. We have gotten very used to questions being answered with “we don’t know,” as there are so few folks having gone before us on this journey with SMA with the most recent treatments that there are just not clear expectations.”

“Recently, we have had more challenge in accessing support resources as he moves into preschool/school settings. There are just so few kids like Luke who are so significantly affected physically but have no cognitive delays or deficits. He often gets stuck in a unique position where he needs physical help and assistance, but most programs set up for that are focused more on kids with more global delays. Figuring out how to “mainstream” him but meet his needs is our current big challenge.”

Despite this big challenge, Luke and his family have experienced many bright spots along their SMA journey. They have an incredibly strong family support system, with Joanna’s parents helping often. They also value the support gained from the SMA community.

“I had visited the Cure SMA website briefly after Luke’s diagnosis, but our first experience was when a giant box full of incredibly thoughtful items was delivered to our front door. It was the first time that we felt like we had a community. We realized there are other people going through this and there are people that have been through this before.”

Since then, the Whitlers have gotten much more involved with Cure SMA, including attending a Summit of Strength and starting their own DIY fundraiser, “Laps for Luke” at a local elementary school. The event was originally for just family and friends but grew to 120 attendees.

“Cure SMA has changed Luke’s life, changed our life. Cure SMA has stood by us since the very beginning of this daunting diagnosis. Through care packages, educational summits, conferences and connecting us with other families, Cure SMA has provided physical and emotional support to our family and made us feel less alone. Most importantly, Cure SMA was there before we knew we needed them. They had been advocating for decades for the development of the life-saving therapies from which Luke now benefits. Because of Cure SMA, we are hopeful, even optimistic about Luke’s future. Because of Cure SMA, Luke is walking. I encourage others to join me in supporting Cure SMA so that they can continue to fight for all those affected by SMA, those that know they need their support and those yet to know.”





# Baking a Difference

**cure  
SMA**

## CALLING ALL YOUNG PASTRY CHEFS AND PHILANTHROPISTS!

We're inviting you to help us  
bake a difference for our SMA Community.

Any parent or guardian who signs up their kid  
to participate in our program will receive a free  
kit of baking tools and goodies to get started.  
Quantities are limited, so sign up today!

As kids bake and fundraise at school, in their  
neighborhoods, or anywhere else, they'll earn  
rewards and recognition along the way,  
including exclusive t-shirts, chef's aprons,  
and chef's hats.

If you have or know of kids with sweet hearts  
and a love of sweet treats who want to bake a  
difference, please share the website link with  
their guardians to get them signed up.

All kids aged 18 and under are invited  
to participate. Thank you!

**BAKERS  
SIGN UP  
HERE!**



# DELIVERING ON COMMITMENT TO ADDRESS UNMET NEEDS OF PEOPLE WITH SMA

## AN UPDATE SINCE THE 2022 CURE SMA CONFERENCE

Cure SMA has undertaken strategic initiatives since the 2022 conference related to continued research, new therapies, and quality care to help address unmet needs of the SMA community, particularly challenges faced by adults with SMA.

“The last decade in SMA led to significant treatment and newborn screening progress,” said Cure SMA President Kenneth Hobby. “The next will focus on meeting everyday living needs of individuals with SMA. And our work to lay the foundation for another decade of success to address this need is well underway.”

### Advancing New and Expanded Research in SMA

Cure SMA has taken proactive steps to ensure that foundational research in SMA continues and focuses on unmet needs, especially for our older and the symptomatic SMA population. Building upon our \$15 million in past research investments over the past two decades, Cure SMA will award new grants this fall (2022) to examine the role of SMN protein, new treatment pathways, including non-SMN approaches, and how

best to utilize both SMN-enhancing and non-SMN approaches. “Past funded research has resulted in three powerful genetically targeted treatments and a pipeline of other potential candidates, many that are in clinical trial,”

#### DID YOU KNOW?

There are more SMA drugs in clinical trials than ever before! Check out our SMA research pipeline at [www.curesma.org/sma-drug-pipeline/](http://www.curesma.org/sma-drug-pipeline/).

said Jacqueline Glascock, Cure SMA Vice President of Research. “The goal of this new research is to look at new therapeutic approaches and treatment combinations to help restore strength and function.”

Research funded by Cure SMA and through our generous supporters, such as the Richard and Jane Nunemaker Foundation, provides critical seed funding that can unleash additional grant resources through the National Institutes of Health (NIH), the world’s largest funder of biomedical research. Past NIH research, including through its National Institute of Neurological Disorders and Stroke (NINDS), has contributed to the development of the existing SMA disease-modifying therapies. NINDS identified its past SMA research as a “research highlight” in its proposed budget to Congress. To ensure that NIH maintains its SMA research focus, Cure SMA galvanized the SMA community around an advocacy campaign to educate Congress about the impact of past NIH research investments and the continued research needs of the SMA community. The SMA community’s advocacy paid off when, this summer, Congress included a provision in the NIH’s fiscal year 2023 funding bill that urged the agency to support new SMA research focused on current community needs, including fatigue, muscle weakness, and reversing damage caused by SMA.

“This congressional provision is significant as it signals to the NIH that Congress expects the agency to build from its past success to address other SMA challenges,” said Maynard Friesz, Cure SMA Vice President of Policy and Advocacy. “This action was only possible because of the advocacy and educational efforts of the SMA community.”



### SMA Community Successfully Advocates for Continued NIH Funding

A record number of Cure SMA supporters sent messages to their Members of Congress during and after the 2022 Cure SMA Conference in support of continued SMA research. Most of the messages were sent through Cure SMA’s new Advocacy Action Center. Some advocates participated in virtual meetings with their congressional offices or delivered personalized messages. For example, Melissa Gutridge of New Haven, Connecticut, and her son, Charlie (pictured here), wrote personal letters to her Representative, who chairs the U.S. House funding committee, and her Senator, who serves on the U.S. Senate funding committee. Combined with advocates across the country, their advocacy outreach, which also included attendance at a constituent event with their Senator, helped to secure the NIH SMA Research provision.

## Prioritizing the Need for Future and Combination SMA Treatments

Cure SMA has also made significant progress in educating federal regulators about the need for new SMA therapies, including treatments that can work in combination with SMN-enhancing treatments. In August, Cure SMA organized a listening session for the FDA to learn directly from adults with SMA, a teenager with SMA, and parents of children with SMA. The SMA community representatives shared their personal experiences with how current FDA-approved SMA treatments have improved their lives and described the difficulties they continue to face that they hope future SMA treatments will address. This is the second major listening session Cure SMA has organized with FDA, which has already approved three SMA treatments.



### SMA Community Shares Treatment Experiences and Future Goals with Regulators

**“While I am incredibly grateful for the progress that I have made, I am by no means satisfied. I am extremely excited about the possibility of a combination of treatments that could be even more effective than what I have experienced so far. I carefully track all the drugs that are currently in the pipeline, especially the drugs that are targeted at regenerating and enhancing muscle strength, and I am patiently waiting for the day I can add an additional therapy to my treatment and see even more gains.”** ~ Kyle Derkowski, Adult with SMA

**“If you compare my three children you will see a huge improvement of motor function. However, each of them could still benefit from another drug or dual drugs to fully increase their ability”**

~ Amy Medina, Parent of 3 Children with SMA.



**“For me a treatment that targets muscle gain would greatly improve my independence and would insure that i can continue doing the things that i love like power soccer,”**

~Wren Grabham, Teenager with SMA

## Expanding Local Access to Evidence-Based Care

Cure SMA also took a significant step forward to increase access to and improving care for children and adults with SMA. Following conference, Cure SMA announced ten new medical centers have been added to Cure SMA's Care Center Network. The focus for the Care Center Network expansion included adult care and expanding representation of the SMA community in the Care Center Network. With these new sites, there are now 28 SMA Care Centers across the country, 13 that serve adults and children with SMA, four adult-only centers, and 11 children-only centers.

**“Our care centers are collecting clinical data from SMA patients to develop an evidence-based standard of SMA care that will improve the lives of all those with SMA,”** said Dr. Mary Schroth, Cure SMA Chief Medical Officer.

“These centers will help us stay on top of health-related changes and trends across the SMA community and to update and disseminate care best practices so that we are meeting the real-time challenges faced by adults and children with SMA.”

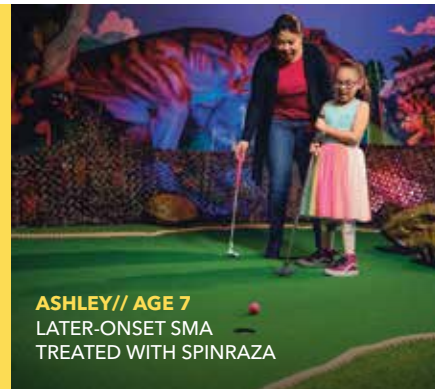
“Working together, the SMA community has been successful in first slowing, and then stopping the progression of the disease,” Hobby said. “Our target now is to reverse or cure the disease. These early actions along with the continued support and commitment of the SMA community and our partners will help to drive the next decade of success in SMA research, therapies, and care. To learn more about how you can help support or participate in Cure SMA's efforts, go to [www.curesma.org/](http://www.curesma.org/) or email [info@curesma.org](mailto:info@curesma.org).

ADVERTISEMENT

# THIS MOMENT COMES FROM WITHIN

**CARLEE // AGE 11**  
LATER-ONSET SMA  
TREATED WITH SPINRAZA

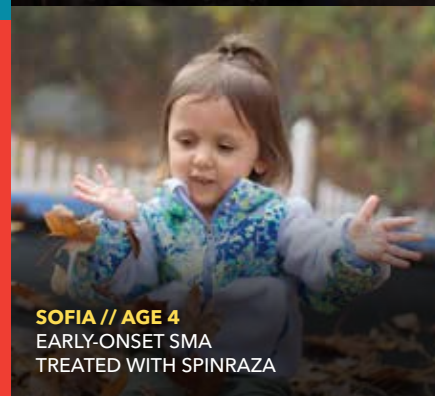
Individual results may vary based on several factors, including severity of disease, initiation of treatment, and duration of therapy.



**ASHLEY // AGE 7**  
LATER-ONSET SMA  
TREATED WITH SPINRAZA



**STEPHEN // AGE 39**  
LATER-ONSET SMA  
TREATED WITH SPINRAZA



**SOFIA // AGE 4**  
EARLY-ONSET SMA  
TREATED WITH SPINRAZA

## INDICATION

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

## IMPORTANT SAFETY INFORMATION

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

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

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

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

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

**Please see full Prescribing Information on SPINRAZA.com.**

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



**From 3 days\* to 80 years old,<sup>†‡</sup>  
there is someone from almost every  
age group who has taken SPINRAZA**

**7+ years** of clinical trial data across a range of SMA types  
**13,000+ people** have been treated with SPINRAZA worldwide<sup>§</sup>

\*Includes clinical trial patients.

<sup>†</sup>Clinical studies of SPINRAZA did not include sufficient numbers of subjects aged 65 and over to determine whether they respond differently from younger patients. Clinical studies included patients from 3 days to 16 years of age at first dose.

<sup>‡</sup>Based on commercial patients in the US (including Puerto Rico) through December 2020.

<sup>§</sup>Based on commercial patients, early access patients, and clinical trial participants through May 2022.



**Watch SPINRAZA stories**

**IMPORTANT FACTS ABOUT SPINRAZA<sup>®</sup> (nusinersen)**

**USES**

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

**WARNINGS**

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

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

**COMMON SIDE EFFECTS**

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

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

**OTHER INFORMATION**

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

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

**QUESTIONS?**

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

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# Community Spotlight:

## Michelle Tynski and Family

Child's play matters. It allows children to develop cognitive, social, emotional, and physical skills while engaging creatively. It's not just how the kids play that's important, but it also matters what they play with. Which is why Michelle Tynski, mom to nine-year-old Trace, Benjamin who passed away in 2016, and 3-year-old Zach, who has Type 1 SMA, took interest in Zach's favorite toys and the message they were conveying.

These toys included a Fisher-Price® Little People® yellow school bus in smaller, more portable, and larger, push and pull versions. Michelle was drawn to the buses because they each featured a figurine in a wheelchair. The figurines were meant to click into place in the school bus seats to be transported on an adventure.

As Zach started playing with his bus toys, Michelle quickly noticed the figurine in the wheelchair wasn't locking into place like the other ones, which lead the figurine to be knocked back and forth in the bus when it was moving. Observing this issue is what led Michelle to voice her concerns.

In January 2021, Michelle posted this message to her TikTok and Facebook accounts: "I need to address something to Fisher-Price. While I am happy and thrilled that you included figures in your toys for disabled children to be more inclusive, there is something about this toy that seems a little off. Notice how all the other characters fit into this bus nicely, and while there is space for the handicapped child, look what happens when you try to move the bus."

Michelle then displayed for her followers what happened to the little boy in the wheelchair when she moved the toy. While Michelle's TikTok and Facebook posts are generally meant for friends and family, her TikTok racked up hundreds of comments and more than 11,000 views.

Despite these views and comments, Michelle said she didn't think much of it. "I was just a mom advocating for my child." That was, until March 2021 when she received a comment on her Facebook post from Fisher-Price asking if they could connect her with a member of their

product development team.

Michelle then followed up with Fisher-Price. They thanked her for sharing her concern and said they made improvements to the toys that would be rolled out during mid-summer 2021. "They're changing their toys because I said something!" Michelle shared in a follow up video to friends, family, and other followers.

In July 2021, Michelle and Zach were sent demo toys of updated school buses and an airplane featuring the figurine in the wheelchair, who now locks securely into place for safe travel, just like all the other figurines. Fisher-Price also sent the Tynskis a newly created figurine of a little girl in a wheelchair that also neatly locks in place as a school bus or airplane passenger.

Zach took to his new toys right away and Michelle was just as thrilled. In a Facebook message she shared, "Thank you so much Fisher-Price. You have made such an impact in our home. This is what disability inclusion looks like!"

**If you'd like to purchase any of the updated Fisher-Price toys, you can find several of them on Amazon:**

- Fisher-Price Little People Big Yellow Bus, musical push and pull toy with Smart Stages for toddlers and preschool kids
- Fisher-Price Little People Josh & Mia Figures

If you purchase from Amazon, don't forget to shop with Amazon Smile! After signing in, choose Cure SMA as your selected charity and Amazon will donate a percentage of your purchase to Cure SMA. Make sure to bookmark this link to ensure that each time you make a purchase, Amazon Smile donates to Cure SMA.

Thank you to Michelle for sharing your story and for advocating for our community!

# THANK YOU TO OUR CONFERENCE SPONSORS

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

## PRESENTING SPONSORS



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# CURE SMA CONFERENCE THROUGH THE YEARS



# 2022 Annual SMA Conference Summary

Thank you to everyone who attended the 2022 Annual SMA Conference at the Disneyland Hotel in Anaheim, California. We were thrilled to be back together as a community – after two years of virtual conferences and events – for an impactful weekend filled with learning, connecting, and fun! We are happy to announce that, including on-site registration, there were over 2,100 attendees in total!

It was such an honor to have individuals with SMA, families, caregivers, clinicians, and researchers attend the conference from over 29 different countries around the world.

The four-day weekend was filled with unique 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, an ‘It’s A Wonderful Life’ panel of adults with SMA ended the weekend on a high note. The Meet & Greet, PJ Party and Movie Night, and Evening at the Park were also conference highlights!

Lastly, at the Sunday morning Closing General Session, we announced the location and dates for our 2023 Annual SMA Conference. Mark your calendars for Thursday, June 29–Sunday, July 2, 2023, as we head to Disney’s Yacht and Beach Club Resorts in Orlando, Florida!

We would like to extend our thanks to the sponsors and exhibitors who generously supported the 2022 Annual SMA Conference. A special thank you to Biogen, Genentech, and Novartis Gene Therapies for serving as Presenting Sponsors of this year’s conference.



# 2022 Annual SMA Conference Quotes from around the community

“As a first-time attendee, I truly enjoyed seeing the community come together and witness the level of detail and care that went into an enjoyable experience. I was so proud to work alongside your team supporting this important work for the SMA community.”

~ *Melissa*

“I concur that the Cure SMA staff hit a home run this year!”

~ *Nick*

“All the work that was done certainly showed and paid off. We felt welcome all the time.”

~ *Kathy*

“I loved the conference. I was profoundly impacted by seeing all of the people living with SMA and their families and friends! What a wonderful community and in the over 30 years that I have worked for pharma companies this was the best conference that I have attended!”

~ *Joe*

“The Genentech team wanted to say a huge thank you for such a well run and impactful 2022 Cure SMA meeting. Everything could not have gone more flawlessly for all participants and the content, the connections/re-connections, the education and the learnings were found in every moment of both the Researcher and Community meeting.

A true thank you for all of your continued partnership and allowing us to be a sponsor of this event!”

~ *Genentech Team*

“I just wanted to send along a quick note of gratitude for holding such an amazing conference again this year! It was wonderful seeing so many families and kiddos’ faces in person again after so long. I had an incredible time as always, learning and connecting non-stop. I personally favor the researcher poster session and wheelchair races, was blown away by Biohaven’s research and therapy development, and was heart-warmed by the social media session including positive role models with SMA. Thank you for the increased inspiration, motivation, and continued opportunity to serve the community.”

~ *Sierra*



# My Annual SMA Conference Experience

By: Jessica Keogh, M.Ed

**This was my first year attending the annual SMA conference and the experience was invaluable.**

I've lived with my disability my entire life, but until very recently my diagnosis was unknown. It's ironic that I'm coming full circle from the very first doctor who told my parents I had SMA to 32 and a half years later that I do have a very rare form of SMA called SMARD. Celebrating my "new" diagnosis at the SMA Conference was the perfect way to kick off my summer!

Meeting people in person from online community groups, who I have known online for years, was certainly the highlight. There's nothing quite like talking with people who truly get your everyday life and sharing what we like to call "life hacks" of how to do or overcome certain situations.

I was able to attend the special education and SMA session. As a special education teacher of eleven years, I attended in hopes to share hope. I wish my parents had sessions like this when I was growing up, but I'm grateful that CURESMA is providing these much needed sessions for our community.

One of my favorite sessions was the session about SMA and intimate relationships because there isn't often a space for these types of conversations. Sessions like this help our community come together so we can continue to change the narrative surrounding disability and relationships.

**Naturally, as an educator, I'm inclined to leave you with a few takeaways:**

- Get involved with our SMA community – community is everything!
- Sign up to attend the next Cure SMA conference you don't want to miss it!
- Spread the word about Cure SMA so we can continue to empower our community!



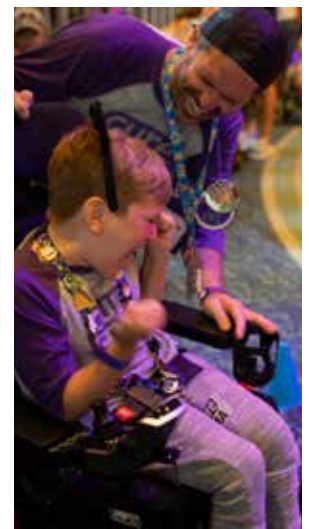
# Meet and Greet

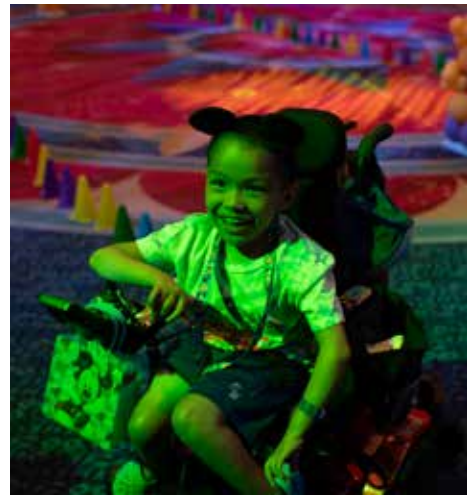
The 2022 Annual SMA Conference began with our signature kickoff event, the fun-filled Meet and Greet! This tradition welcomes new and returning individuals with SMA, families, clinicians, and researchers from all over the world.

It is a special time for all attendees to gather prior to the conference workshops. Everyone at the conference comes together to begin a weekend filled with new friendships and connections, learning the most up-to-date information, and being a part of our wonderfully caring community.

The many games that lined the perimeter of the Meet and Greet ballroom helped to make it an enjoyable event. Kids and families rotated around to play Bozo Buckets, check out a treasure chest, and much more. The games were adapted for all children to enjoy and win prizes.

Mickey and Minnie Mouse, Tiana and Louis from Princess and the Frog, Lilo and Stitch, and Winnie the Pooh and Tigger were also there to meet attendees.





# Researcher & Clinician Relay Race

The ever-popular Researcher & Clinician Relay Race was a major highlight of the Meet and Greet, as kids with SMA raced researchers and clinicians to the finish line, with adults racing in a manual wheelchair. Attendees surrounded the course and cheered on the kids to victory. Try as they may, the researchers and clinicians were left in the dust!



# Family Friendly Researcher Poster Session

The Family Friendly Researcher Poster Session allows for one-on-one interactions between families, individuals with SMA, and researchers. Cure SMA invited researchers who attended the SMA Research Meeting to present family friendly research posters. During the Friday evening event, attendees rotated to different posters to ask questions and learn directly from researchers involved in each of the projects being presented. Attendees were also able to stop by the Cuddle Corner to snuggle the therapy dogs kindly brought in by Canine Companions, and meet Disney characters including Up's Carl and Russel, Mike Wazowski and Sully from Monsters Inc., Woody and Buzz Lightyear from Toy Story, and Mickey's pal Pluto.

## Family Friendly Researcher Poster Session Posters Included:

- Updates on the Development of Spinal Muscular Atrophy: Person-Reported Outcome (SMA-PRO): A Caregiver and Self-Proxy Performance Measure for Children and Adults with SMA**  
Amy Pasternak, PT, DPT, ATC, CSCS, Boston Children's Hospital
- Understanding the Muscle-directed Approach in SMA**  
Blair Orr, PhD, George Nomikos, MD, Jagdish Patel, PhD, Scholar Rock
- Apitegromab Clinical Trial Updates**  
Blair Orr, PhD, George Nomikos, MD, Jagdish Patel, PhD, Scholar Rock
- RESILIENT: A New SMA Clinical Trial with a Dual Acting Anti-Myostatin**  
Clifford Bechtold, MS, Lia Donahue, MA, Biohaven Pharmaceuticals
- A New Muscle-Directed Treatment Approach for SMA**  
Didier Bagnol, PhD, Epirium Bio
- SMN Protein Controls RNA Molecules: What Do We Know and What Do We Try to Find Out?**  
Dmytro Morderer, PhD, Mayo Clinic
- MANATEE: Roche and Genentech Clinical Study of GYM329 (RO7204239) in Combination with Risdiplam Treatment in Pediatric Patients with SMA**  
Donald Daudt, PhD, Genentech
- Newborn Screening for SMA**  
Elizabeth Kichula, MD, PhD, Childrens Hospital of Philadelphia
- How do Glia Impact Motor Neuron Function in Spinal Muscular Atrophy?**  
Emily Welby, PhD, Allison Ebert, PhD, Medical College of Wisconsin
- NMD670, A Chloride Ion Channel Inhibitor as a Novel Approach to Improving Skeletal Muscle Function for Spinal Muscular Atrophy**  
Eva R. Chin, PhD, NMD Pharma
- Development of a Competency-based Educational Program for Spinal Muscular Atrophy Physical Therapists (STEP-IN)**  
Jacqueline Montes, PT, EdD, Columbia University Irving Medical Center
- The Spinal Muscular Atrophy Patient and Caregiver-Reported Health Indices: Disease Specific Outcome Measures of How Individuals with SMA Feel and Function**  
Jamison Seabury, BS, University of Rochester Center for Health + Technology
- SMA Families Filming Infants Learning Movement (SMA Families FILM) Using a Smartphone App at Home**  
Kristin J. Kropsschell, PT, DPT, PCS, Northwestern University Feinberg School of Medicine
- New Research Avenues for the Treatment of SMA**  
Laxman Gangwani, MTech, PhD, Texas Tech University Health Sciences Center
- Evaluating the SMA Caregiver Experience**  
Lisa Belter, MPH, Cure SMA
- How Do Motor Neurons Recover After Treatment is Given?**  
Lyndsay Murray, PhD, University of Edinburgh
- Implementation of Spinal Muscular Atrophy Newborn Screening Across the U.S.**  
Mary Schroth, MD, Cure SMA
- The SMA Bulbar Survey: What We've Learned and Future Directions**  
Maureen A. Lefton-Greif, PhD, CCC-SLP, BCS-S, Johns Hopkins School of Medicine
- Bulbar Function in Patients with Spinal Muscular Atrophy Type 1**  
Nicole LaMarca, DNP, MSN, CPNP, PMHS, Novartis Gene Therapies
- What Powers Cure SMA?**  
Jesse Aynes, Samantha Edidin, Brett Kinley, Cure SMA
- The Safety and Efficacy of SPINRAZA in Infants and Children**  
Sarah Jennison, Rare Disease, Biogen
- Understanding the Diagnostic and Treatment Journey for Individuals with SMA**  
Sarah M. Whitmire, MS, Cure SMA
- Tube Feeding Formulas Used by Patients with SMA: Experience from Three institutions**  
Stacey Tarrant, RD, LDN, Boston Children's Hospital
- A New Small Molecule that Improves Neuromuscular Strength in Spinal Muscular Atrophy Model Mice**  
Stephen Meriney, PhD, University of Pittsburgh
- SPINRAZA Pivotal Data in Later-Onset and Independent, Observational, Data in Adults**  
Sundip Raval, PharmD, Rare Disease, Biogen
- COPI: A Cargo Train Helping to Build Neurons**  
Timra Gilson, PhD, Elliot Androphy, MD, Indiana University School of Medicine
- RAINBOWFISH: Roche and Genentech Clinical Study of Risdiplam Treatment in Infants with Genetically Diagnosed, Presymptomatic SMA**  
Travis L. Dickendesher, PhD, Genentech

# PJ Party and Movie Night

On Friday evening, beneath a starry night sky on the Adventure Lawn, all conference attendees were invited to cozy up and enjoy Toy Story 4 on the big screen! It was a relaxing end to the evening after a full day of workshops.





# Cure SMA Evening at the Park

Conference attendees were invited to enjoy a magical Saturday evening together at Disneyland®. Everyone was encouraged to wear their Cure SMA Conference t-shirts to bring awareness and pride to the park. One of our favorite activities each year is to document the massive group with a group photo!



# Children's Program

This year's Children's Program was a tremendous success! Thanks to our incredible volunteers, the children had a fabulous time. Kids stayed busy with countless arts and crafts projects, toys, live entertainment, movies, video games, and other fun activities. One highlight was a caricature artist who drew pictures for kids to take home as a conference memento!

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 were amazing once again. The Children's Program is supported by the Jacob Isaac Rappoport Foundation, and their support makes the Children's Program possible.

Thank you, Jacob Isaac Rappoport Foundation for your sponsorship and enduring support.



# Networking Opportunities



Networking and fostering relationships within the Community has taken on more meaning than ever after an absence of in-person attendance for the past two years. The weekend hosted a variety of different opportunities for everyone in the community to meet, mingle and connect.

We hosted Dad's Night Out on Thursday evening and Mom's Night Out on Friday evening.

Teenagers at the conference were able to connect during our Teen Social and participate in a fun and interactive trivia game with snacks and an area devoted just to teens.

Adults with SMA had the opportunity to utilize the Adults with SMA Lounge to hold conversations and connect with each other, all day Thursday, Friday, and Saturday. This space was sponsored by Biogen and the Dhont Family Foundation. On Friday evening we held an Adults with SMA Reception, for a fun night out with beverages and snacks, and a chance to connect in a different format! Thank you to Biogen for sponsoring and supporting adults with SMA.

Grandparents had the opportunity to get a cup of joe and chat during the Grandparents Coffee & Mingle session.

This year, a new social event was created specifically for families who have been identified through Newborn Screening. The Newborn Screening Meet and Mingle event was sponsored by Novartis Gene Therapies and gave families the chance to meet in person and share their experiences with diagnosis through newborn screening.

Through all the different social events and networking opportunities, we hope the connections and relationships made will last a lifetime.



# 2023 ANNUAL SMA CONFERENCE



THURSDAY, JUNE 29TH – SUNDAY, JULY 2ND

Disney's Yacht & Beach Club Resort in Orlando, Florida

Every year Cure SMA organizes and hosts a conference to bring together 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 an Evening at the Park, as well as many opportunities to connect and interact and receive first-hand updates from 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.

Disney's Yacht & Beach Club Resort, where the majority of the 2023 Annual SMA Conference will take place, is a lakeside resort which features an array of New England-style eateries, three relaxing leisure pools, an arcade room, and Stormalong Bay – a three-acre sand bottom pool complete with a shipwreck replica, waterslides, lazy river, and a sun deck. Disney's EPCOT theme park is just a short walk away, or you can stroll down the boardwalk to visit unique shops and restaurants.

You must complete your conference registration with Cure SMA prior to reserving your hotel room, for the special room rate of \$265 per night, plus tax, at either Disney's Yacht Club Resort, Disney's Beach Club Resort, or Disney's Boardwalk Inn.

## Annual SMA Conference Goals

- To welcome newly diagnosed families and individuals into the SMA community.
- To help build an SMA community and keep that community strong and unified.
- To educate SMA families and individuals with SMA, providing updates on medical issues, research, and clinical trials.
- To facilitate networking and data sharing between researchers, clinicians, families, individuals, and patients.
- 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. Scholarship categories include the First-Time Attendee Conference Scholarship, the Adults with SMA Conference Scholarship, and the General Conference Scholarship.

If you have any questions about conference scholarships, please email [conference@curesma.org](mailto:conference@curesma.org). More information on scholarships along with ongoing conference updates can be found at [www.AnnualSMAConference.com](http://www.AnnualSMAConference.com).

## Special Meeting & Convention Theme Park Tickets

Come for the conference and stay for the magic! Make the most of your free time with special Disney Meeting and Convention Theme Park tickets. 2023 Annual SMA Conference attendees are eligible for advance purchase of specially priced discounted Disney Meeting/Convention Theme Park tickets. Ticket information will be provided after you register for the Annual SMA Conference.

Join us for the 2023 Annual SMA Conference

**TO REGISTER FOR**  
the Annual SMA Conference,  
please visit [www.cureSMA.org](http://www.cureSMA.org).



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

# MOVING AHEAD

**Now  
approved  
for more people  
with SMA**

See **What is Evrysdi** below  
and visit **Evrysdi.com** for  
more information



## 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
- 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.**

**Please see accompanying brief summary for additional Important Safety Information.**

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](http://www.fda.gov/medwatch). You may also report side effects to Genentech at 1-888-835-2555.

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

**Genentech**

A Member of the Roche Group

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**Evrysdi**<sup>®</sup>  
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.
- 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 syringes that are needed for taking your medicine and explain how to use them. Wash the syringes per instructions after use. Do not throw them away.
- Use the reusable oral syringes provided by your pharmacist (you should receive 2 identical oral syringes) to measure your or your child's dose of EVRYSDI, as they are designed to protect the medicine from light. Contact your healthcare provider or pharmacist if your oral syringes 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)
  - diarrhea
  - lung infection (lower respiratory infection)
  - vomiting
  - 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.
- 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). Please see the Discard After date written on the bottle label. (See the **Instructions for Use** that comes with EVRYSDI).

**Keep EVRYSDI and all medicines out of the reach of children.**

**General information about the safe and effective use of EVRYSDI.**

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

**What are the ingredients in EVRYSDI?**

**Active ingredient:** risdiplam

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

**Genentech**

*A Member of the Roche Group*

EVRYSDI® (risdiplam)

Distributed by:

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94080-4990

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For more information, go to [www.EVRYSDI.com](http://www.EVRYSDI.com) or call 1-833-387-9734.



# 2022 AWARENESS MONTH

**“Awareness is the greatest agent for change.”**

~Ekhart Tolle



## Advocacy Actions

During SMA Awareness Month and beyond, there are many ways to get involved in SMA advocacy to educate others about the priorities of the SMA community. SMA Awareness Month was a great time for individuals and families to connect with their congressional offices through our Book Your Own Meeting (BYOM) campaign and by sending Action Alerts through our Action Center. To stay up to date on current issues affecting the SMA community, sign up to be an advocate!

[www.curesma.org/advocacy-action-center/](http://www.curesma.org/advocacy-action-center/)

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.”



## 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 took place on Saturday, August 13, 2022.



## 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. The Cure SMA colors are used to represent the entire SMA community and all the hard work and efforts that are put in towards research, care, and support all year long. More than 40 buildings in more than 30 states lit up this year! This program was started by community member, Shaakira Thomas, “To me, SMA Awareness Month is not only about ways of raising



## Cure SMA Staff Involvement

In August, each staff member was sent a fun box of Cure SMA swag to get them excited about SMA Awareness Month. During the August all-staff meeting, everyone wore their purple and orange gear to display their Cure SMA pride.

Staff members also updated their email signatures and social media banners and shared their experiences serving our awesome SMA community.





## Fundraising Events

Some of Cure SMA's largest annual fundraising events were held in August, including three Walk-n-Roll events and a 5K.

The Walk for Graham was held on August 5, 2022, in Carmel, Indiana. In addition to the walk, the event included an opening ceremony, silent auction, and a raffle. It was a massive success, with over 800 attendees and nearly \$160,000 raised for Cure SMA, surpassing last year's revenue by nearly \$30,000!

The Northern California Walk-n-Roll was held on August 20, 2022, at Kennedy Grove Regional Recreation Area in El Sobrante, California. More than 300 people attended and raised over \$97,000.

The Wakefield 5K was also held on August 20, 2022, and runners raced around beautiful Lake Quannapowitt in Wakefield, Massachusetts. Entertainment included music from New England's Best DJs, finisher prizes, and a post-race raffle. Over 125 people attended and raised \$17,200 for Cure SMA.

The Colorado Walk-n-Roll took place on August 27, 2022, at Clement Park in Littleton, Colorado. 172 participants attended and raised \$36,746.



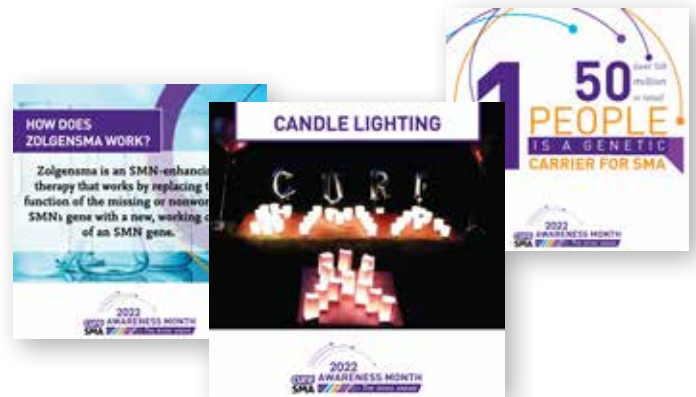
## Giving Campaigns

Individual donors are the foundation to Cure SMA's progress and without the support from thousands of people across the country, our progress would not be possible. In August, individual giving campaigns included merchandise giveaways to donors, and a Quest Diagnostics gift matching weekend, where all gifts up to \$5,000 were matched. We also received a \$5,000 donation from one of our pharmaceutical partners, Scholar Rock. If you would like to make a gift in support of Cure SMA, visit [www.donate-uresma.donordrive.com](http://www.donate-uresma.donordrive.com).



## New Merch Store Items

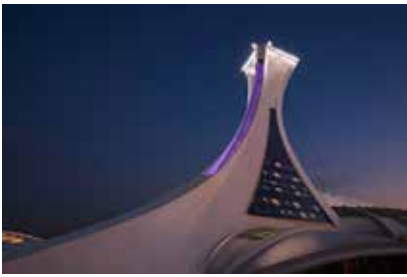
In order to make your shopping experience quick and easy, Cure SMA launched a new online merch store. For SMA Awareness Month, we released new merch including performance fleece jackets, reflective performance half-zip tops, racerback tank tops, and bucket hats. Visit <https://curesmaorg.myshopify.com/> to check out the new store. Even more new merch is coming soon!



## Social Media Blitz

More than any year before, Cure SMA took to social media to spread SMA awareness stories, news, and updates far and wide. We created infographics, stickers, and animated gifs for community members to share, and even started a TikTok! Find us on TikTok @curesmaorg. We also enlisted the help of our first-ever group of social media ambassadors who took us inside their daily lives with SMA, including navigating air travel and trying out assistive technology. 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](mailto: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 to a giving campaign, light a candle, advocate, or share your story.*



# Thank You!



**Cure SMA would like to thank everyone who participated in SMA Awareness Month.**

Whether you made an advocacy visit, attended a Cure SMA event, sent in photos or videos, or shared messages on social media, we appreciate the community effort to spotlight SMA and look forward to next year.



# Matching Gifts – The Quick and Easy Way to Increase Your Impact

Did you know that approximately \$4–\$7 billion in charitable matching gift funds go unused each year? According to Double the Donation, over 26 million individuals work for companies or organizations with matching gift programs, but only 7% ever submit a matching gift request. There is a tremendous amount of potential support that is untouched, and the good news is that these funds are often quick and easy to access.

Many companies offer corporate matching gift programs that help support charitable nonprofits and causes that their employees care about. When an employee makes a gift, they submit a matching gift request, and their employer will make a donation of their own. This approach to corporate social responsibility is a simple way for companies to support their communities and their charitable work. In many cases, the employee simply fills out a digital matching gift form and submits it to their employer. This quick process helps donors increase their impact without the need to make another contribution themselves. It's like a free donation to your favorite charitable organization.

With such a large amount of untapped funding available, Cure SMA is making it a priority to highlight the potential of matching gifts on the future of our mission and how simple they can be to secure. We encourage all our supporters to explore their employer's matching gift program, because no matter the size of the gift, anyone can double their impact in the SMA community.

To simplify the matching gift process, Cure SMA has a search tool on our website that helps our supporters check to see if their employer will match their gifts. Also, this easy-to-use tool provides all required matching gift information, including match ratios, volunteer grants, employee eligibility, instructions on how to submit a matching gift, and links to the required forms. It is a one-stop shop for all matching gift needs.

This is the perfect time to explore your employer's matching gift program and double your support for Cure SMA programs. The next breakthroughs in SMA are on the horizon and your matched gifts will help accelerate funding for basic research and new pathways for treatments, adding new sites to our Care Center Network and improved SMA care, and expanding our nationwide advocacy efforts to advance issues like accessible transportation and employment for people with disabilities.

Reach out to your employer or visit our website today to check if your gifts to Cure SMA are eligible for a corporate matching gift program. You can find our matching gift tool at [www.curesma.org/ways-to-give/](http://www.curesma.org/ways-to-give/).

Also, if you have questions about matching gifts or how you can get your employer involved in our mission, please reach out to Brett Kinley, Director, Development – Individual Giving, by phone at (847) 709-6314, or via email at [brett.kinley@curesma.org](mailto:brett.kinley@curesma.org).

**Explore your matching gift options today and double your impact on the future of SMA!**

Cure SMA continues to accelerate progress for our SMA community through our comprehensive research, advocacy, care, and support programs. Over the last year, the hard work and dedication of our supporters helped change SMA. Together, we are achieving the next breakthroughs in SMA and building a hopeful tomorrow for future generations. Below are a few highlights and outcomes from the last year.



## ADVOCACY

- Eight additional states started screening for SMA, with 98% of all U.S. newborns now being screened for SMA in 2022
- 1,006 advocacy messages were sent to Congress during the Cure SMA 2022 Conference, making it the largest federal online advocacy campaign in the history of Cure SMA



## RESEARCH

- Cure SMA solely supported the Pediatric Neuromuscular Clinical Research Network (PNCRN) of highly skilled clinical trial professionals
- Relunched our basic research funding, now focused on developing additional treatments that will target different areas of the body to help bring back strength and function



## COMMUNITY SUPPORT

- Informational packets sent – 1,238
- Pieces of equipment sent from our equipment pool – 213
- Newly diagnosed care packages sent – 226
- Teen and adult support packages sent – 525
- Total 2022 Annual Conference Attendees – 2,057



## CLINICAL CARE

- The Cure SMA Care Center Network added ten new sites (29 total)
- Over 750+ SMA patients participate in the SMA Clinical Data Registry, including both children and adults with SMA
- Over 300 Adult & Pediatric SMA physicians, researchers, clinical staff, and neuromuscular program coordinators worked collaboratively to better understand and improve standards of care for patients with SMA

# Community Spotlight:

## Amber-Joi Watkins and Family



The proverb, “It takes a village to raise a child” may be centuries old, but its relevance and meaning endure.

For Amber-Joi Watkins, her husband, Tommy Domalski, and their daughter, Céline, that village includes family and friends, as well as Céline’s pediatrician, the Children’s Hospital of Philadelphia (CHOP), her physical therapists, her friends at Cure SMA, and many others. When it became clear that Céline wasn’t meeting milestones typically seen in babies her age, this team began to form and has since changed the course of her life.

Amber-Joi said, “I’m a very active person and began to take Céline to ‘Mommy and Me Yoga’ when she was a few months old. That’s where I started to notice that she wasn’t moving like the other babies. She wouldn’t hold her head up, kick, roll, and do tummy time activities.”

Amber-Joi mentioned her concerns at Céline’s three-month pediatrician appointment. She said, “The nurse practitioner put her on tummy time, and she lifted her head.” The nurse practitioner said, “Because you’re her mommy, she’s probably not going to lift her head for you.” She told Amber-Joi, “There’s nothing to worry about. She’s perfect.”

When Céline went back to the pediatrician’s office for her six-month appointment, things began to change. “We saw a different doctor within the practice, and Céline had at that time regressed in her strength. The practitioner had previously done a rotation in an SMA clinic and noticed the signs of SMA right away. Céline had a tongue quiver, which is common with SMA, she couldn’t sit up at the time and still wasn’t rolling or kicking. Her low muscle tone was very evident, and I was told she needed to start physical therapy right away.”

Céline’s pediatrician helped her get an appointment the next day with Neurology at CHOP. Amber-Joi said, “We saw Brenda Banwell and Vanessa Battista, who does work with Cure SMA.” Right away, they had blood work taken so they could diagnose Céline. “This was the week before Christmas, so it was hard to get anything done. The lab technician knew the last truck had already arrived to pick up blood samples. He ran and caught the truck so Céline’s blood could be sent in for analysis.”

Days later, Céline was diagnosed with SMA Type 1, and six weeks after that she was approved and began to receive gene therapy at CHOP.

Today, Céline is meeting her milestones albeit delayed and lessening the concerns expressed during her initial diagnosis. Amber-Joi said, “We were warned that she would probably never walk or crawl, but that her outlook would be better than the traditional outlook, which was two years of life. So that gave us some hope, but it was also a strange and scary time.”

She said, “My husband and I took the information we were given, but we wanted to do more than give our daughter medication and wait. Being the active people we are, we wanted to start Céline with physical therapy right away, so that’s what we did.”

Amber-Joi and her husband also worked to help Céline make progress at home on her strength and mobility. They did water therapy and play in the bathtub and got Céline moving as much as possible. They also sought out childcare support that reinforced these efforts, specifically seeking nannies in school for physical or occupational therapy that understand and help to implement her routine.

“We work on motivating Céline while having fun. We do this to ensure her muscles don’t atrophy further and to allow her to rebuild some of her strength. We’ve had a lot of luck and great outcomes because she walks, crawls, goes to school without a caretaker, and more than anything else, is just a happy child.”

Currently, Céline is starting a new school year and is even in dance class. Amber-Joi said, “We don’t let SMA stop us from anything.”

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

- Protected Savings Options Comparison Chart
- Guide to Talking with Children about Disabilities
- Educator's Guide to SMA
- Sample School Letter Guide
- Travel Guide
- Adaptive Equipment List
- Driving - Becoming Licensed
- Accessible Vehicle Options and Considerations
- Home Modifications Guide
- Bathroom Accommodations
- Bathroom Equipment List
- DEI Children's Book Recommendations



To request a copy of any of these resources, please email [communitysupport@curesma.org](mailto:communitysupport@curesma.org).

# State of SMA Report

In early June 2022, Cure SMA launched its first annual State of SMA report. The purpose of this report was to share highlights from Cure SMA's three databases:

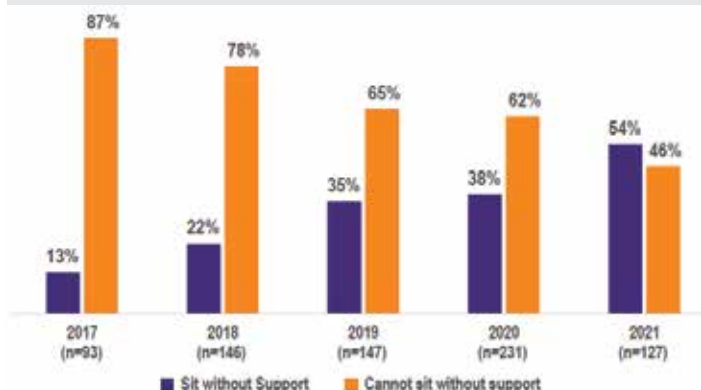
- the Membership Database with patient-reported outcomes on over 9,700 affected individuals worldwide that also includes data from our annual community update survey
- the SMA Clinical Data Registry (CDR) containing electronic medical record (EMR) sourced data for over 750 patient receiving care from US-based SMA Care Center Network sites
- the SMA Newborn Screening Registry (NBSR) with parent-reported outcomes for over 50 babies with SMA identified through statewide SMA newborn screening

The 2021 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 affected 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 report:

- Both children and adults are represented in Cure SMA databases, and the average age is 21 years old, ranging from one month to 90 years old
- The membership database shows the average reported age of diagnosis is decreasing. In the NBSR (n=52), the reported median age of diagnosis confirmation is seven days
- Within the annual community update survey, most patients with Type 1 SMA report the ability to sit without support

The proportion of individuals with SMA Type 1 who can sit without support has increased since 2017







- Additionally, participants from the annual Community Update Survey report a reduction in the average annual rates of hospitalizations across SMA types

**The average number of hospitalizations per person has decreased for all major SMA Types since 2018**



- Member-reported mortality rates in 2021 were lower than they were over the previous 12-year timespan. For every 100 people with SMA, the annual mortality rate decreased from 1.56 in 2009 to 0.55 in 2021.

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

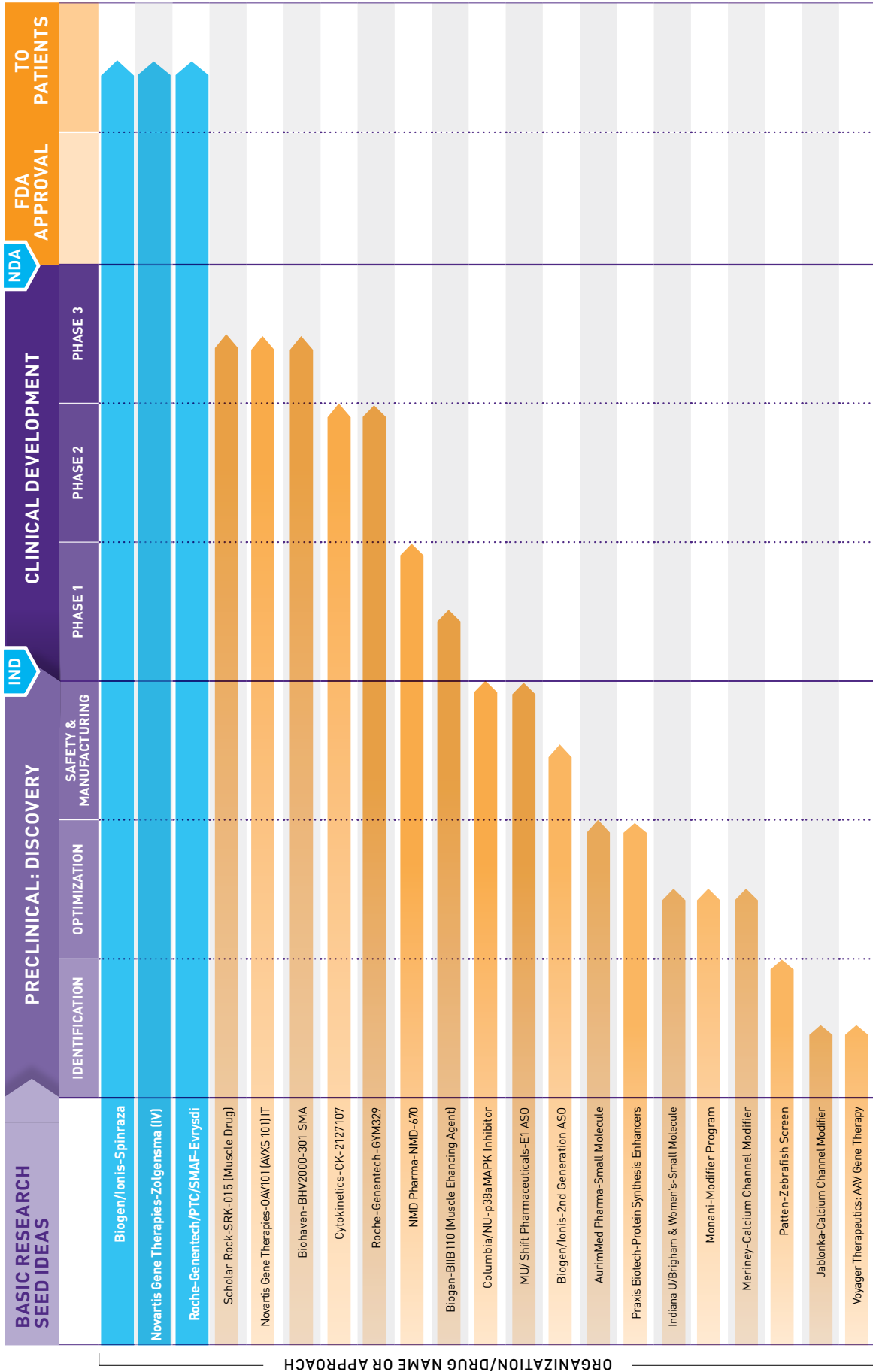
Cure SMA is thankful for all the members of the SMA community who have generously shared their data to make this report possible. Cure SMA also thanks the Care Center Network for their care of patients with SMA and work with the CDR. Cure SMA is also grateful for the support and funding provided by the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

**STATE OF SMA**  
DECEMBER 2021

To access the 2021 State of SMA report, please visit:  
<https://bit.ly/3sregpk>

# SMA DRUG PIPELINE

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



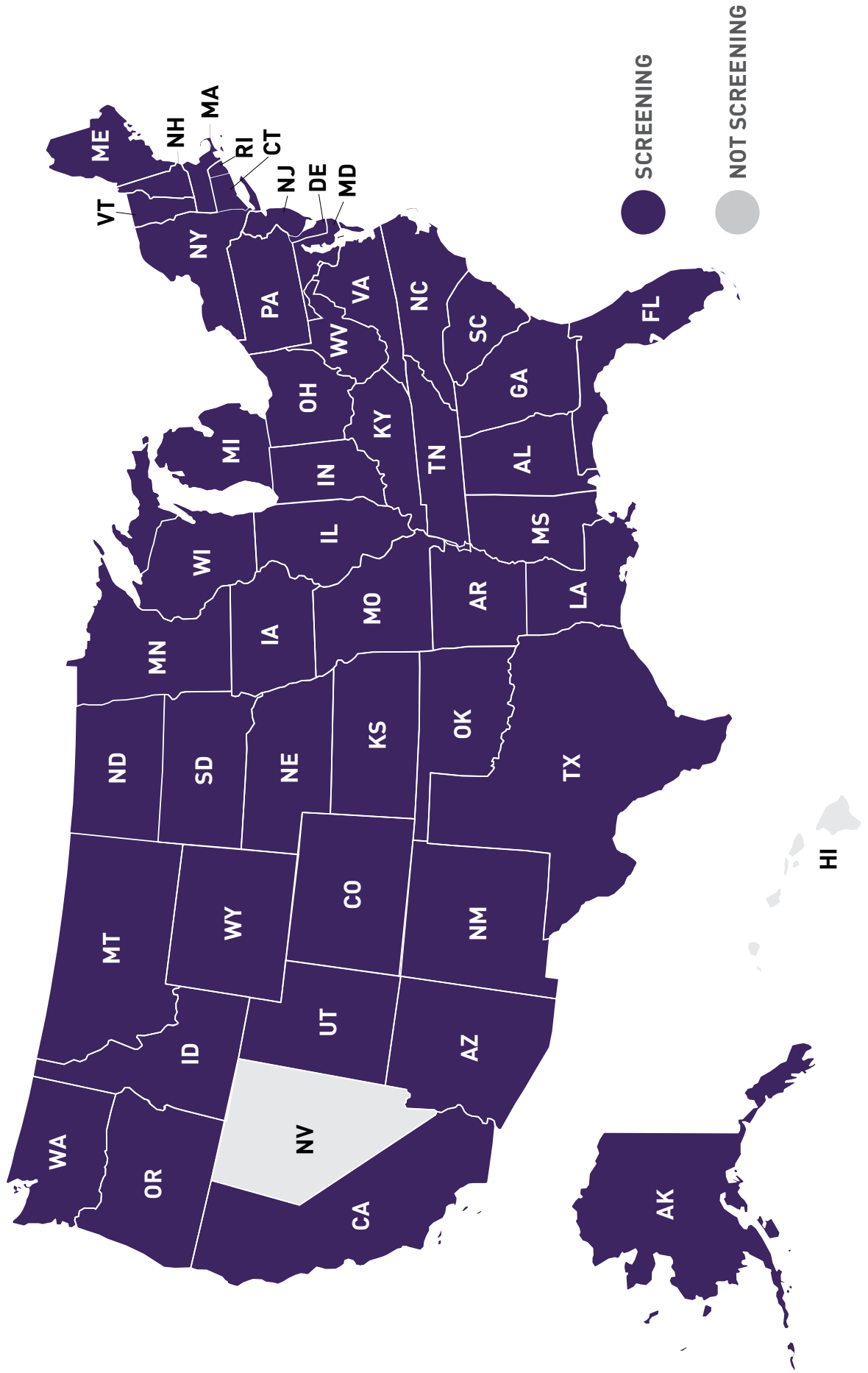
IND = Investigational New Drug

NDA = New Drug Application

Last updated: June 2022

# STATES SCREENING & NOT SCREENING FOR SMA

48 States Currently Screen for SMA | 98% of Newborn Babies in the U.S. are Screened



# Advocacy Update



## Advocacy Takes Center Stage at 2022 Conference

Cure SMA advocates from across the country elevated SMA community advocacy priorities throughout the Cure SMA 2022 Conference. Individuals with SMA and their families sent more than 1,000 advocacy messages to Congress during conference week, making it the largest federal online advocacy campaign in the history of Cure SMA. In addition, conference attendees highlighted the advocacy issues important to them at the conference selfie station.

## Advocating for Additional SMA Research

Cure SMA's advocacy campaign to educate Congress about unmet needs of individuals with SMA has paid off with significant action in the U.S. House of Representatives and the U.S. Senate.

A legislative provision that urges the National Institutes of Health (NIH) to continue research efforts in SMA focused on the needs of the SMA community was included in legislation to fund NIH. The report described how past NIH research has led to current SMA therapies and identified the importance of continued SMA research to address new challenges faced by children and adults across all ages and disease stages of SMA. Inclusion of this provision in both the U.S. House and U.S. Senate bills increases the likelihood the SMA research direction will be retained in the final funding bill, expected later this year. Thank you to everyone in the SMA community who took part in this advocacy campaign.

### U.S. House/ U.S. Senate Language

FY 2023 Labor-HHS-Education Appropriations Report (Page 156)

*Spinal Muscular Atrophy.*—The Committee commends NIH for its past research into spinal muscular atrophy (SMA) that has led to new therapies to treat SMA and also contributed toward greater knowledge and research capacity into nervous system disorders. While current SMA treatments can slow or stop future degenerative nerve damage, they are not cures, and there remains significant unmet need across all ages and disease stages of SMA. Individuals with SMA, particularly adults, the largest segment of the SMA population, face significant challenges in muscle weakness and fatigue due to degeneration that occurred prior to treatment. Individuals treated prior to clinical symptoms onset may also display unmet needs, such as bulbar impairment and gait abnormalities. The Committee urges NIH to address these unmet needs, which are common across other neurological and neuro-muscular diseases, by supporting new research into the role and function of survival motor neuron (SMN) protein, investigation into non-SMN pathways and targets capable of modifying disease, and research into how to best combine SMN-enhancing and non-SMN approaches for optimal therapeutic outcomes.



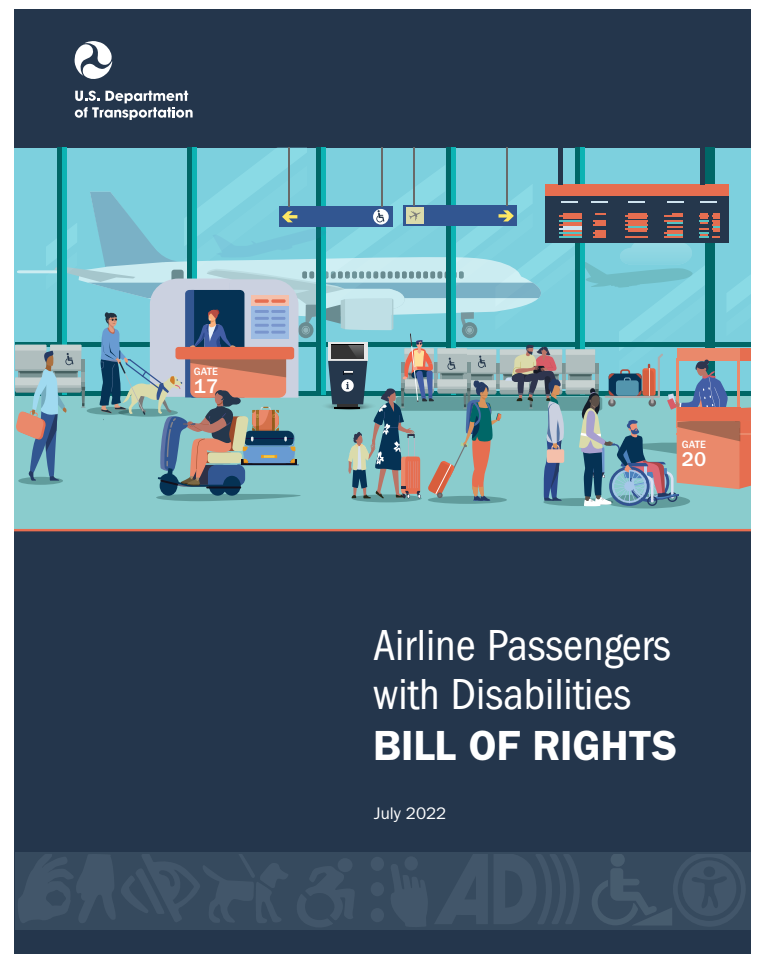
# KNOW YOUR RIGHTS

This summer, the U.S. Department of Transportation released an Airline Passengers with Disabilities Bill of Rights that outlines the fundamental rights of air travelers with SMA and other disabilities under the Air Carrier Access Act and its implementing regulation.

The Bill of Rights, which Cure SMA advocated for in legislation, will help inform air travelers with SMA of their rights when flying and help hold airlines and their contractors more accountable for upholding the law and regulations that govern air travel. The Bill of Rights includes 10 points:

1. The Right to Be Treated with Dignity and Respect.
2. The Right to Receive Information About Services and Aircraft Capabilities and Limitations.
3. The Right to Receive Information in an Accessible Format.
4. The Right to Accessible Airport Facilities.
5. The Right to Assistance at Airports.
6. The Right to Assistance on the Aircraft.
7. The Right to Travel with an Assistive Device or Service Animal.
8. The Right to Receive Seating Accommodations.
9. The Right to Accessible Aircraft Features.
10. The Right to Resolution of a Disability-Related Issue.

In addition, Cure SMA is actively advocating for other legislative changes to improve airline accessibility for people with SMA, including the goal of power wheelchair users to roll directly onto the aircraft. Check out Cure SMA's Advocacy Action Center to learn about how you can support this effort.



[www.transportation.gov/airconsumer/disabilitybillofrights](http://www.transportation.gov/airconsumer/disabilitybillofrights)

To learn more about Cure SMA Advocacy efforts, sign up to become a Cure SMA advocate, at [www.curesma.org/advocacy/](http://www.curesma.org/advocacy/). Or contact Cure SMA Advocacy Team at [advocacy@curesma.org](mailto:advocacy@curesma.org).



# Clinical Care Update

**We are excited to share that The Cure SMA Care Center Network has expanded!**

Cure SMA has added nine of 11 NEW Care Centers to the Care Center Network, bringing the total number of participating Care Centers to 29 sites across 22 states with more than 1,500 SMA patients!

**The complete list of participating care centers is as follows:**

Baylor College of Medicine	Houston, TX
Children's Healthcare of Atlanta, Inc.	Atlanta, GA
Northwestern University	Evanston, IL
The Ohio State University Wexner Medical Center, Neuromuscular Division of Neurology	Columbus, OH
The University of Michigan	Ann Arbor, MI
The Washington University   St. Louis Children's	St. Louis, MO
The University of California, Los Angeles (UCLA)	Los Angeles, CA
University of Miami	Coral Gables, FL
University of New Mexico	Albuquerque, NM

## Adult Care Centers

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

## Pediatric Care Centers

Advocate Children's Hospital, Park Ridge, IL  
 Arkansas Children's Hospital, Little Rock, AR  
 Children's Healthcare of Atlanta, Atlanta, GA  
 Children's National Medical Center, Washington, DC  
 Children's of Alabama, Birmingham, AL  
 Phoenix Children's Hospital, Phoenix AZ  
 Seattle Children's Hospital, Seattle, WA  
 Stanford Children's Health, Palo Alto, CA  
 Yale Pediatric Neuromuscular Clinic, New Haven, CT

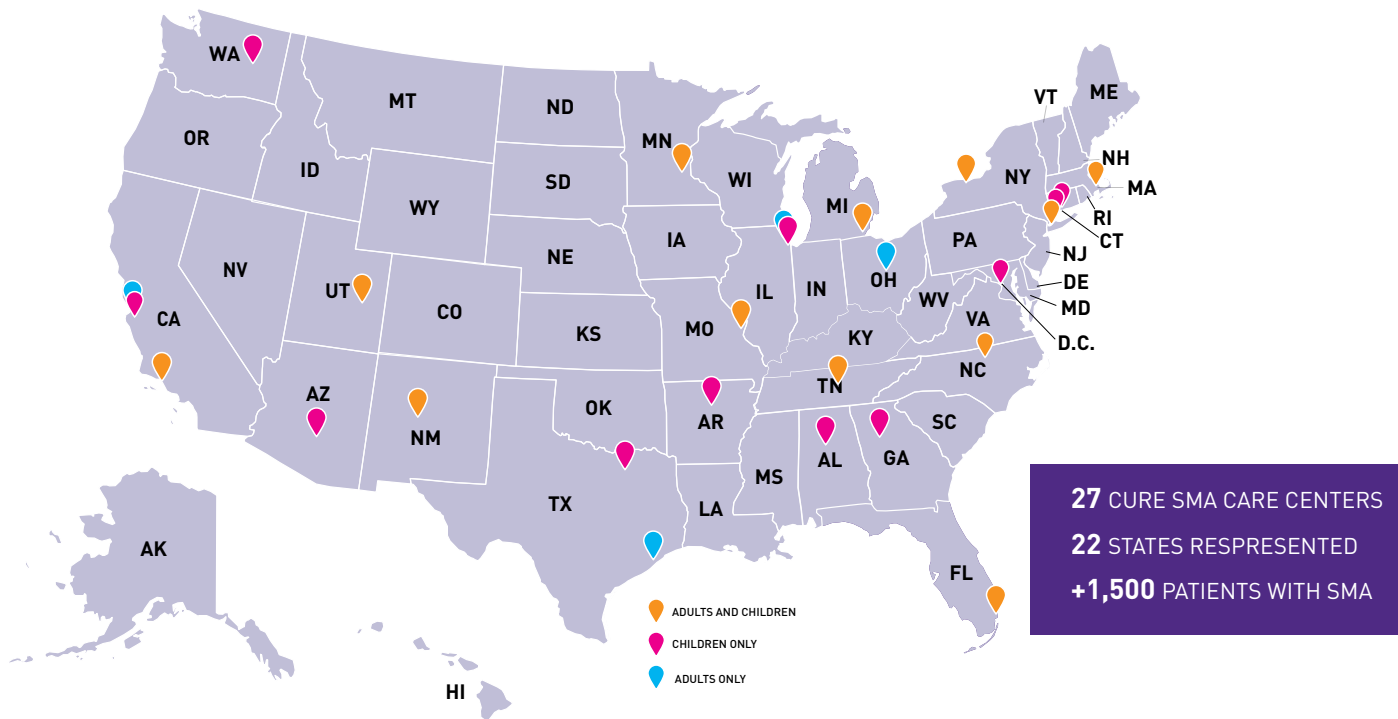
## Adult & Pediatric Care Centers

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

The Care Center Network consists of over 300 Adult and Pediatric SMA physicians, researchers, clinical staff, and Neuromuscular program coordinators that are all working collaboratively across the nation to better understand and improve standards of care for patients with SMA.

*The Network is one of the largest and fastest-growing SMA collaborations in the United States.*

The Network utilizes consented SMA patient data provided by Care Centers, SMA care providers experience and external evidence and research to define key measures to assess performance, build a robust database, create rigorous reports to identify gaps and produce a set of effective tools to improve standards of care for those with SMA. As a result, the network goal is to create high quality, safe, and efficient care with the best outcomes possible.



Visit [www.curesma.org/carecenternetwork](http://www.curesma.org/carecenternetwork) to learn more about the Network.

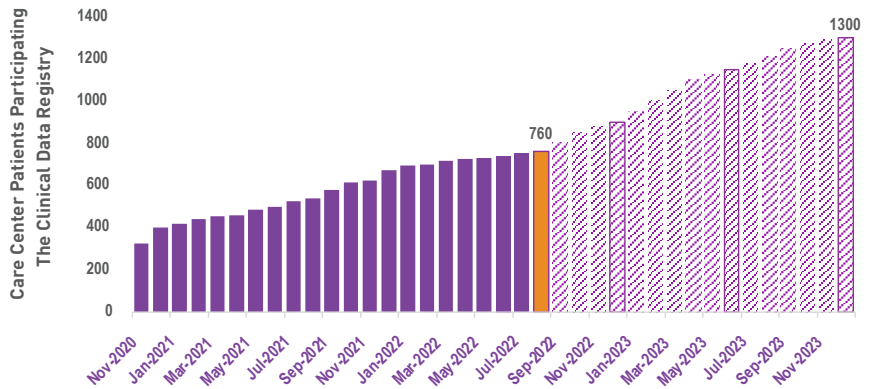
## Clinical Data Registry

The SMA Clinical Data Registry (CDR) has over 750 patients with SMA. With the recent 10-site expansion of the Care Center Network, the CDR is projected to double in size by the end of 2023. This would make the registry the largest SMA registry in the United States.

## Focusing on SMA Outcomes that Matter

In the summer of 2022, the Care Center Network added additional comprehensive data elements to the registry, allowing for SMA researchers and clinicians to better focus on areas of SMA care and real-world evidence assessing outcomes that matter most to patients and families. A few areas that we'd like to use the data to further explore include:

- Understanding change in motor function before and after initiation of SMA treatment
- Understanding the time to diagnosis and time to first treatment for infants identified via newborn screening
- Understanding time to diagnosis and time to first treatment for patients with symptomatic SMA
- Understanding the age at symptom onset and impact on treatment outcomes
- Understanding the use of multidisciplinary care received by patients with SMA



For more information about how Cure SMA is using data visit our recent State of SMA Report <https://www.curesma.org/state-of-sma-report-2022/>



# Cure SMA Programs for Adults with SMA

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

## SUPPORT PACKAGES AND PROGRAMS



### Teen & Adult Support Package

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

[www.curesma.org/SupportPackage](http://www.curesma.org/SupportPackage)



### Teen & Adult Independence Assistance Package

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

[www.curesma.org/IndependencePackage](http://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](http://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](http://www.curesma.org/ResponderPHR)

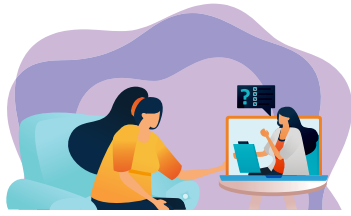
### Adults with SMA Virtual Therapy Program

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

[www.curesma.org/virtualtherapyprogram](http://www.curesma.org/virtualtherapyprogram)







## Virtual Therapy Program for Personal Care Assistants of Adults with SMA

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

[www.curesma.org/virtualtherapyPCA](http://www.curesma.org/virtualtherapyPCA)



## Annual SMA Conference Sponsorship

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

Email [conference@curesma.org](mailto:conference@curesma.org) for sponsorship application!



## Annual SMA Conference Events

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

[www.annualsmaconference.com/agenda.html](http://www.annualsmaconference.com/agenda.html)



## Adults with SMA In-Person Socials

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

[www.curesma.org/virtual-community-engagement-events/](http://www.curesma.org/virtual-community-engagement-events/)



## Adults with SMA Virtual Socials

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

[www.curesma.org/virtual-community-engagement-events/](http://www.curesma.org/virtual-community-engagement-events/)

**You  
Tube**

## Educational Webinars and Panels

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

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



Learn more about these programs at [www.CureSMA.org](http://www.CureSMA.org) and contact [communitysupport@curesma.org](mailto:communitysupport@curesma.org) with any questions!



# Community Spotlight:

Alexandra and Joe Lakhman

While sibling relationships aren't always easy, Alexandra 'Lexi' Lakhman and her brother Joe have always been close. Despite Lexi being six years older than Joe, she grew up viewing Joe as a role model, particularly for his positive outlook on life.

Joe was diagnosed with spinal muscular atrophy (SMA) Type 2 in 2002 when there wasn't nearly as much information or as treatment options as there are today. Because of this, Joe battled illnesses throughout his childhood, and as a result, chose to be homeschooled starting in sixth grade. "Being home and growing up just being around your parents and your sister who is out in the world going to school and playing sports can be hard. I never wanted that to take a toll on his mental health," shared Lexi.

As Joe got older and started receiving more treatments, Lexi and her family investigated options to connect with their local SMA community. That's when they learned about Cure SMA. "I saw all these amazing individuals who have accomplished so much despite physical disability. I wanted Joe to see that and to know that he doesn't have to live at home and be indoors. Joe can have a dream and accomplish it and be successful."

From that moment, Lexi began volunteering as Communications Chair of the New Jersey Chapter of Cure SMA. As Communications Chair, one of the initiatives she created was a community member spotlight, highlighting individuals affected by SMA in the New Jersey area. Through this, she was able to help Joe connect with peers and learn more about strategies and options to gain independence, pursue schooling, and consider career options outside the home.

Joe is now 20 years old and entering his junior year of college at Seton Hall, studying computer science and data analytics. Ideally, he would like to become a video game designer. Last year was Joe's first time living on-campus and he says that the experience of living more independently is one of the best things to happen to him.

However, that doesn't mean that college and transitioning to a life of more independence has been easy for Joe. He faced battles receiving the accommodations he needed in a class, as well as difficulties being able to participate in social activities, like fraternity mixers, which are held in non-accessible buildings.

Initially, he thought it would be difficult to make friends but had a personal goal of becoming as involved as possible at Seton Hall. Joe quickly learned making friends was quite easy, joining clubs and on-campus podcasts, as well as becoming a peer advisor to incoming freshmen. "You make friends without even trying ... whether it's from classes or different study groups, you really just have to be yourself. The people that want to be your friend will find you," he said.

In talking to Joe, it is clear he's found a strong and supportive community for himself, something he partially credits to his fellow peer advisors, a group of 48 who are "like family". Having a support system on-campus has also proven to be imperative for caregiving purposes. During the school year, Lexi normally comes to help out once a week, but the other days he relies on either nurses or a group-chat of friends.

**"When a nurse backs out, there is no guarantee that the agency will have someone ready to step in. But from one simple text message to a group of 15 friends, someone will respond and come to help," shared Joe.**

In addition to on-campus accessibility, travel is another issue important to the Lakhman family. Joe's wheelchair is custom, and he is unable to transfer to another chair, meaning air travel isn't currently an option. This is particularly important now, as Lexi is the current title holder of Miss New Jersey USA and is gearing up for the Miss USA pageant on October 3rd.

Since Miss USA is being held in Reno, Nevada, unfortunately Joe will be unable to attend. Lexi said, "It's sad for me because he has been there supporting me at every single pageant that I've done. For him to not to be there at my final goal is really tough."

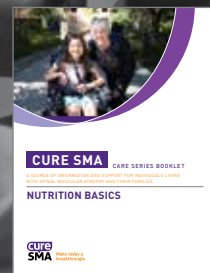
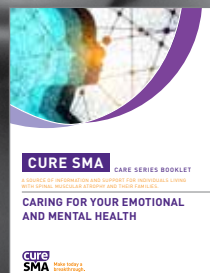
You can help by supporting Cure SMA's Greater Independence agenda and air travel accessibility initiatives.

Visit the Advocacy Action Center  
[www.curesma.org/advocacy-action-center/](http://www.curesma.org/advocacy-action-center/)



**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/](http://www.curesma.org/care-series-booklets/)**

*Booklets are available online and are translated into 12 languages.*

**cure**  
**SMA**

Make today a  
breakthrough.

**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.**

*Photo of Tonya Willingham and her daughter, Hanna*



## IN LOVING MEMORY OF MALCOLM "JACK" FREEDMAN

### April 23, 1995 - October 5, 2021

From the day he was born, to the day he was diagnosed with SMA and given a year to live, to the day he passed on, Jack taught us many important lessons. He taught us how to meet challenges and face adversity, not only with courage, but with love. Jack lived a joyful life, whether at home, at school, in the community, at camp, and even in the hospital.

My son, Jack, passed away one year ago today.

He was 26 years old.

#### **Today, I am able to look back upon Jack's life and feel a great deal of pride.**

My son accomplished a great deal in his 26 years. Jack earned excellent grades at school. He had two jobs, hundreds of friends, and a loving family. He had a remarkably positive attitude and enjoyed every single day. Jack unified everyone in the communities where he lived and went to school, bringing out the best in everyone whose path he crossed. He remembered every single birthday of every single person he met. He enjoyed music, movies, social media, outings to new places, and shopping at the mall. Jack made lifelong friends at the Pennsylvania Vent Camp, where he enjoyed ziplining, swimming, exploring nature, dancing, climbing the rock wall, and sneaking into the girl's cabins in his wheelchair. In short, Jack loved his life and I am very proud of the young man he grew up to be.

#### **Today, I look back upon Jack's life with much gratitude.**

When my son was a six-month-old baby, he was diagnosed with SMA and given a year to live. Jack surprised all of us, including his doctors, by living 25 years longer than predicted. Our 25 unexpected years with Jack were filled with both great challenges and great joy. The doctors were very honest about the medical problems Jack would encounter, and Jack faced all those challenges, and many more. But the doctors didn't tell us about the good days we would have together - and we had thousands of truly wonderful days along the way. Looking back, I feel very grateful for every single day of the 26 years we had together.

**"It's not so much what we have in this life that matters. It's what we do with what we have."**

~ Mr. Rogers

#### **Today, I look back upon Jack's life with no regrets.**

A few weeks after Jack's diagnosis in 1995, I clearly remember the moment I realized the importance of making the most of every single day with my son. I remember looking at our happy little baby and telling myself that there was only one way I could come through this experience emotionally whole. I told myself that my job as a parent was to be able to look back upon my baby's life, however long he lived, and know that we made the most of every day, every moment, and every opportunity. Upon Jack's passing, an old friend wrote to me, "You left it all on the field." Yes, we did. As I reflect on Jack's life, I am able to look back with no regrets.

#### **Today, I look back upon Jack's life as a very special gift.**

A few years ago, I began to view Jack's life very differently than I had in the early years. I came to realize that my son's rare disease was also accompanied by some unanticipated, unique, and very special gifts.

How many people do you know who can enter a room, and without saying a word, cause every other person in the room to instantly feel their big problems becoming much smaller? Jack offered others the gift of perspective.

How many people do you know who attract open-hearted, compassionate people into their lives? Jack was a magnet for people who live their lives with love, light, and joy.

How many people do you know who bring out the best in everyone around them?

How many people do you know who bring joy to everyone whose paths they cross?

In spite of all the challenges he faced - or perhaps because of them - my son influenced others in all of these very special ways. I believe he made the world a better place. Along with the rare disease called SMA came many unanticipated rare gifts, and for those gifts I will always be grateful.

#### **Today, I look back upon Jack's life with a whole lot of love.**

Jack truly loved everyone, and everyone loved Jack. As his life is celebrated, my hope is that we can all take the abundance of love Jack shared with all of us, and share that love with others, just as he did. We can all strive to "live like Jack" - with a positive attitude, appreciation for every single day, determination, unabashed joy, and a whole lot of love.

Thank you, Jack, for teaching us so many important life lessons. I was very fortunate to be your Dad.

And I will always be your Dad.

~ Al Freedman | October 5, 2022

# 2022 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



## National Platinum Partner



## National Gold Partners



## Resources and Communications Partners



# Now Open!

Cure SMA is pleased to announce the launch of our newly updated merch store! The merch store features all our most popular Cure SMA t-shirts, hats, zip-up jackets, and more, as well as new items.

In addition to new merch, the store itself has a brand-new look with improved functionality, making shopping and finding what you want a breeze.



**You can also save while you shop, with free shipping on all orders above \$35.**

**A portion of each purchase you make funds research, education, support programs, and more for the SMA community.**

*Please note the item quantities are limited, so purchase your favorites before they sell out!*

**Visit <https://curesmaorg.myshopify.com/> to check out the store.**

# SUMMIT OF STRENGTH PROGRAM



The Summit of Strength is a free educational program that provides attendees the opportunity to learn from an array of experts about topics that are valuable to individuals of all ages and types of SMA and their caregivers. We have been thrilled to reintroduce these opportunities for networking and building community in-person. This local engagement highlights Cure SMA's core values and further strengthens the bonds that make our communities so vibrant!

*This is a free program that includes breakfast, lunch, and parking for all attendees. Surveys are offered at every event and the Cure SMA team values attendee feedback in order to help us continue to build upon the value of this program.*

As of August 31, 2022, Cure SMA has hosted 64 in-person Summits of Strength and reached over 3,600 adults with SMA, families, and other caregivers across the U.S. Cure SMA also virtually hosted almost 150 experts who shared their knowledge with over 1,600 live viewers through the 22 installments of the Summit of Strength Webinar Series which took place between 2020 and 2021.

## Some of the latest feedback includes...

**“THIS is the info we want and need!”**

**“The fact of having Cure SMA to keep us informed about what is happening in innovation with medicines and for the improvement of health, is worth the effort of being together; so that we can fight for our needs. Let’s fight for people with SMA!”**

**“Everyone was engaging and relevant!”**

**“I found this event to be very educational and informative!”**

**Stay tuned for the release of the 2023 Summit of Strength dates and locations, coming soon!**

The health, safety, and well-being of the entire SMA community is important to us. Cure SMA is continually monitoring and evaluating the impact of COVID-19 in the U.S. and in-person summit events are subject to change.

If you have any questions or comments, please contact [communitysupport@curesma.org](mailto:communitysupport@curesma.org).

Visit [www.curesma.org/summit-of-strength/](http://www.curesma.org/summit-of-strength/) to register for your local 2022 Summit of Strength today!





***This program is made possible by our generous donors in the SMA community. Cure SMA wishes to thank our sponsors for the 2022 Summit of Strength Program - National Presenting Sponsors, Biogen and Genentech, National Visionary Sponsor, Scholar Rock, and National Platinum Sponsor, Biohaven Pharmaceuticals.***



# Fall Walk-n-Roll Highlights As of October 2022



## HIGHLIGHTS

# of Participants – 3,047

# of In Person Events – 21



Together, we raised more than  
**\$560,000**  
and counting!

### Our Largest Events:

- Walk for Graham: 658 walkers raising \$163,189
- Northern California Walk-n-Roll: 272 walkers raising \$98,693
- Charlotte Walk-n-Roll: 209 walkers raising \$53,937

### Events That Grew the Most:

- Raleigh Walk-n-Roll – increased 193% over prior year
- Virginia Walk-n-Roll – increased 83% over prior year
- Seattle Walk-n-Roll – increased 71% over prior year





## New England Walk-n-Roll

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



## Pennsylvania Walk-n-Roll

The Pennsylvania Walk-n-Roll took place on Saturday, May 22nd, 2023. What started off as a torrential downpour turned into a beautiful sunny day with close to 200 family and friends coming together to support Cure SMA. This year we welcomed several new families to the Pennsylvania Walk-n-Roll and came together in our new location at Villanova University. The Pennsylvania community came together for food, games, a butterfly release, a beautiful tribute speech by Al Freedman and Allyson Henkel and raised over \$85,000!

Family and friends were thrilled to be together again and truly showed and enthusiasm about all that the walk offered, our new location, and the comradery they felt from all in attendance. Thank you for all your came out or donated to support the Pennsylvania Walk-n-Roll!



## Champions Club

### Cure SMA Walk-n-Roll Champions Club Members

Allyson Perre Henkel	Melissa Feeley	Nathaniel Czeck	Sherry Langland
Silvia Murphy	Victoria Murphy	Amber-Joi Watkins	Melissa House
Heidi Johnson	Karen Dreyer	Audra Butler	Beverly Venedam
Abigail House	Benard Dorer	Krista Fitzko	Beth Moore
Sandy Klein	Rachel Miele	Doug Logeman	Katie Kerns
Siobhan M. Murphy	Joe T. Scully	William Becker	Rachael Roggie
Leah Young	Devon Decker	Luciante Salce	Jean Kinley
Brian Murphy	David Lombard	Allie Callaway Royal	The Rob Kilo Family
Tina Lewis	William Chu	Kristen Resendez	Eileen M. Brown
Beth Lockwood	Jarrett Barnes	Karime Nadia Garza	Brian Watters
Albert Freedman	Derek Lewis	Melissa Becker	William H. Henkel

We would like to thank and congratulate the following Spring Walk-n-Roll participants for being inducted into the 2022 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.

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

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



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



[www.linkedin.com/company/curesma](https://www.linkedin.com/company/curesma)



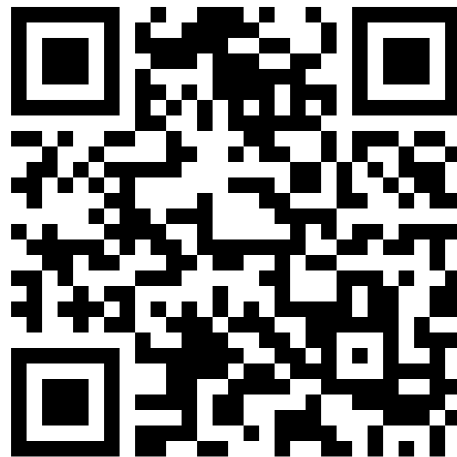
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#CureSMA and #SMACommunity



# 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



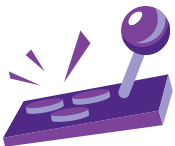
YOUTH FUNDRAISING  
INITIATIVES



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](mailto:diy@curesma.org)

# DO IT YOUR WAY Fundraising Highlights

Whether you're thinking about fundraising in honor of your birthday or a special occasion, participating in an athletic event, doing live streaming and gaming, or anything else you can think of, Cure SMA has you covered with ideas and support to get you started on your DIY Fundraising journey.

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



## Wannabe Golf Cup

The 23rd Annual Wannabe Golf Tournament was held July 7–9, 2022 at The Golf Club at Newcastle in Newcastle, WA. This two-day golf tournament rotates locations each year and is chaired by Joe Belcher, otherwise known as “The Commish” to friends, family, and volunteers. This year’s event featured opening and closing ceremonies, an auction, and a cup presentation to the Blue Team who won this year. Nearly 30 golfers attended and raised almost \$13,000 for Cure SMA.



## The Wedding of John & Kristen D’Aversa

Wedding bells are ringing for Kristen and John D’Aversa, who were married on June 17, 2022, in Woodbridge, Connecticut. In lieu of gifts, they asked their wedding guests to donate to Cure SMA, an organization that is dear to their hearts, in honor of son, Johnny who is living with Type 3 SMA. The happy couple raised over \$7,000. What a selfless way to help others during their special day!



## Surf Away SMA

Surf Away SMA is a partnership between Ionis Pharmaceuticals, Cure SMA, and Ricochet the Surf Dog. The seventh annual event took place on August 5, 2022, at La Jolla Shores Beach near San Diego, California. Nearly 20 families attended for a day of surfing, beach games, and fun.



## Goin’ for Koen

Kelly Piech, whose grandson Koen has SMA, organized this fun fundraising event in partnership with the Savannah Parrothead Club. Goin’ for Koen took place at the end of March 2022 at Cockspur Grill on Tybee Island, GA. It included a courtyard party, karaoke fun, and a reverse raffle drawing and raised almost \$20,000 for Cure SMA!



# Donor Spotlight: Lockwood Family

For more than 20 years Elizabeth, Robert, Kevin, and Beth Lockwood have dedicated a substantial amount of their time to support Cure SMA and our SMA community. As the grandparents and parents of Emma and Nick, two young adults with SMA, the Lockwood family has been participating in and spearheading events for years and helped make the Cincinnati Walk-n-Roll one of Cure SMA's largest and highest grossing Walk-n-Roll events.

Over the past two decades, the Lockwood's have been key players in rallying members of their community to come together and fundraise, successfully hosting more 13,000 attendees and raising more than a million dollars for Cure SMA!

The Lockwood's have worked endlessly to create a unique Walk-n-Roll experience that increased awareness of SMA, offered fun activities such as a petting zoo and face painting, honored and remembered those we've lost to SMA, and provided education and information on navigating life with SMA.

As chapter leaders, Elizabeth and Beth have both assisted in making meaningful and impactful connections within the community, whether it be connecting SMA families, networking with businesses, or educating the general public.

Elizabeth specifically, has spent the last 20 years going out into the community and building relationships with businesses that have since turned into over half a million dollars in sponsorship revenue! These businesses have since become invaluable partners to Cure SMA, have helped spread the word and mission of Cure SMA, and have continued support to the community year-over-year all because of her efforts.

Kevin and Robert have also dedicated several years to volunteering at the highest level of the organization. They have helped guide Cure SMA into the organization we are today. Because of Robert and Kevin's help, we have been able to fund groundbreaking research, support programs, equipment, community events, and so much more.

The Lockwood family have been key players in the success of Cure SMA and have displayed unparalleled leadership and service. Their passion, dedication, knowledge, and understanding of what our community needs and wants, and how Cure SMA can best serve, has truly helped us grow in reach, relevancy, and impact. We shine this spotlight on the Lockwood's as a thank for all that they've done, all that they do, and all that we know they will continue to do for Cure SMA and the SMA community.





# WALK-N-ROLL

**REGISTER TODAY**

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

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

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



Thank You to Our National Premier Sponsors



**FIND YOUR LOCAL WALK-N-ROLL**  
[www.curesma.org/walk-n-roll-program/](http://www.curesma.org/walk-n-roll-program/)



## 2022 Birdies for Blake Golf Outing

The 2022 Birdies for Blake Golf Tournament took place on July 31, 2022, at Green Crest Golf Club in Liberty Township, OH. This year's event raised over \$40,000 for Cure SMA! It was a beautiful day out on the green and this year's raffle was a huge hit!

*A massive thank you goes out to Mark and Nancy Farrell, Nick and Kacey Farrell, and Kate and Sam Queen for planning this wonderful event each year, and for their continued support and dedication.*

## 2022 Tee Off with the Drive in Honor of Ryan

On May 21, 2022, over 100 golfers gathered for the 2022 Tee Off with the Drive to Cure SMA in Honor of Ryan. This year's event consisted of 18 holes of golf, dinner, raffles, and fun at the Gleneagles Country Club in Lemont, IL! This event is held in honor of Ryan Manfre, an outgoing and all-around amazing 15-year-old with SMA. With the help of generous sponsors, volunteers, and golfers the event raised over \$32,000 for Cure SMA!

*We would like to give special thanks to Jeanne Emerson, Becky & Brian Manfre, and Susan and Steve Fedea for organizing this annual event, and for their continuous support!*

## 2022 Rocky Mountain Golf Tournament

The 2022 Rocky Mountain Charity Golf Tournament sponsored by Busick Insulated Glass took place on July 11, 2022, at the beautiful Pradera Golf Club in Parker, CO. Over 100 golfers attended the event which consisted of a four-person scramble, breakfast, lunch, an awards ceremony, and fun course games. With the support of this year's sponsors, donors, and players, the event raised over \$25,000 to help find a cure for SMA!

*Cure SMA would like to extend a special thank you to event organizer, Gillian Faith, and her steering committee for hosting the tournament.*

## 2022 Links for Luke Tournament

Congratulations to the Luke 18:1 Foundation and the Stickane family as they hosted the fifth Annual Links 4 Luke tournament last month and celebrated over one million dollars donated to Cure SMA from event proceeds! This annual event, held on Monday, May 2, 2022 at Timarron Country Club in Southlake, TX, benefits the Luke 18:1 Foundation, whose mission is to work together as a community to find a cure for SMA through raising awareness of SMA and early detection, funding research through fundraising events, and supporting individuals and families by connecting those impacted by SMA with meaningful programs and mentors to aid in their journey.

*Thanks to the generosity of the sponsors, players, and community, the Luke 18:1 Foundation has been able to partner with Cure SMA to fund critical research for new therapies, invest in vital equipment for the loan program, and support the Cure SMA's at-home physical therapy webinar series.*

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





## 2022 Muscles for Mckenna Gala

Congratulations and thank you to the Ellixson Family and the entire steering committee for the 2022 Muscles for Mckenna Evening of Hope! Their tenth annual event took place on Friday, March 18, 2022, at the Crystal Tea Room in Philadelphia, PA.

Approximately 125 people enjoyed a lovely evening complete with a silent auction, fund-a-need, and live music. Thanks to the support of the community, sponsors, and attendees, the 2022 Muscles for Mckenna Evening of Hope raised over \$115,000 this year! We are thrilled to share that thanks to their dedication, the Muscles for Mckenna Evening of Hope has raised over one million dollars since its first event ten years ago!

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

## 2022 Cure SMA Evening of Hope Singer-Songwriter Night

On May 13, 2022, nearly 200 guests gathered for the fifth Annual Cure SMA Evening of Hope Singer-Songwriter Night at the White Oak Estate & Gardens in Baton Rouge, LA. The evening featured cocktails, hors d'oeuvres, a silent auction, a wine pull, dinner, and a live performance by professional singer songwriters, CJ Solar and Jacob Stiefel. The evening was a huge success, raising over \$43,000 for SMA research and family support programs.

*A special thank you to Krista Scurria for chairing the event, as well as the dedicated chapter leaders, committee members, generous sponsors, and amazing volunteers, for making this day possible.*

## 2022 Chicago Evening of Hope

The 2022 Cure SMA Chicago Evening of Hope took place on April 29, 2022, at the Ivy Room in Chicago, IL. This year's event featured a casino themed evening, complete with casino games, live music, great food, a silent auction, and a raffle.

*With the help of generous sponsors, volunteers, and attendees the event raised almost \$33,000 for Cure SMA!*

# Chapter Updates



Thank you to Angel Wolff (Arizona), Ian Zurawski (Michigan), and Jessica Biggerstaff (Indiana), for working with their local Governors to officially declare August as SMA Awareness Month in their home states! Your efforts make a difference in increasing awareness of SMA.



## Chapter Socials

Summer represented the perfect time to socialize with friends both new and old! Families and individuals from Cure SMA's Arkansas, Greater New York, Minnesota, and South Florida Chapters came together for fun-filled Family Socials! In Greater New York, they enjoyed a day out at the Bronx Zoo, Minnesota threw down some friendly competition with tasty snacks at the local bowling alley, Arkansas hosted an SMA Awareness Month Movie night complete with a delicious taco bar, and South Florida had a day filled with fun and learning at the local science museum. Thank you to Erin Bonner (Greater NY), Kara Forcier (Minnesota), Fiorenna Stark and Jennifer Miller-Smith (South Florida), and Sylvia Wheeler (Arkansas) for taking the time to put together these great social events!

# RUN FOR A REASON!

Run for a reason this year and join our Cure SMA Endurance Program in a city near you!

Check out our event calendar below and sign up for a race near you! If interested in registering for an event, please contact Alyssa Zavislak at [Alyssa.zavislak@curesma.org](mailto:Alyssa.zavislak@curesma.org) for more information about running for Team Cure SMA.

EVENT	DATE
TCS NYC Marathon	November 6, 2022
Philadelphia Marathon	November 19, 2022
San Antonio Rock N' Roll Marathon	December 3, 2022
Chevron Houston Marathon	January 15, 2023
Arizona Rock n' Roll Marathon	January 15, 2023
Napa Valley Half Marathon	March 5, 2023
United Airlines Rock n' Roll Washington DC	March 18, 2023
United Airlines NYC Half Marathon	March 19, 2023
Chicago Bank of America Shamrock Shuffle	March 26, 2023
Rock n' Roll Nashville	April 22, 2023
Dick's Sporting Goods Pittsburgh Marathon	April 23, 2023
Wisconsin Marathon	May 23, 2023
Rock n' Roll San Diego	June 3, 2023

The Cure SMA Endurance Program is a nationwide series of races that brings runners together to train and fundraise in support of Cure SMA's mission of driving breakthroughs in treatment and care to provide those affected by spinal muscular atrophy (SMA) the support they need.



# Your SMA story matters!



Each person in our community has a powerful story to tell about their journey with SMA. We want to share as many stories as we can, representing all ages and stages of SMA. Your story could help another member of the community or reassure someone starting their journey.

We have three fun 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](mailto: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](http://curesma.org)
3. **Post on Social Media** Share your story in your words. Use #storySMatter and #CureSMA



Share your story, [stories@curesma.org](mailto: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.

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



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](mailto:stories@curesma.org). Digital images are encouraged!

### Change of Address

Send changes, including ZIP code to: [info@curesma.org](mailto: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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# The Giving Season is Here!

The year-end giving season is upon us, and we want to remind you that your support is more than just a gift.

When you donate to Cure SMA, you are not just making a charitable gift. You are investing in the lives of everyone with SMA. Your support...

- Helps create new treatments that increase strength for daily life at work and school.
- Funds new local expansions to our Care Center Network and improves the SMA care experience.
- Advances key advocacy initiatives like employment, affordable housing, and accessible transportation.



## Don't Miss Out!

Right now we have two amazing opportunities to help accelerate progress in the SMA community:

- The Erin Trainor Memorial Fund will match all gifts made to Cure SMA to increase the number of Care Centers across the country, up to \$350,000!
- With a generous gift from Richard and Jane Nunemaker, donations for Cure SMA basic research initiatives will be matched, dollar-for-dollar, up to \$250,000!

**Do you want to get involved with these exciting opportunities?**

Please reach out to Erin Oganessian, Vice President, Development, at [erin@curesma.org](mailto:erin@curesma.org).

Make a gift today by visiting [www.curesma.org/donate](http://www.curesma.org/donate). Or to make a gift by mail, send your donation to:  
Cure SMA 925 Busse Road, Elk Grove Village, IL 60007