

DIRECTIONS

COMMUNITY NEWSLETTER | SPRING 2023



cure
SMA

LETTER FROM KENNETH

I always enjoy seeing the excitement in our community as we plan to meet in-person at the Annual SMA Conference, which is already less than two months away! Our 2023 Conference is on track to be our biggest yet, and I look forward to seeing many familiar faces, and many new members of our community as well, since we'll be on the East Coast for the first time in six years.

Many of us look forward to the updates on recent progress with research, treatments, and care at the conference. But equally important is the excitement and the power that comes from seeing our community all together at such a scale. Opening the doors and seeing everyone flood into the huge ballroom, which is now necessary for our opening Meet and Greet, is the highlight of my year.

In addition to the conference, I'm also encouraged by all of you who are attending, building teams, and fundraising for spring in-person Walk-n-Rolls. Walk-n-Roll is Cure SMA's most important nationwide fundraising event program, and the funds we raise are critical to furthering progress in research, treatments, and care for individuals with SMA. If there wasn't a spring event in your area, there's sure to be one during our fall Walk season.

We have so much amazing progress to highlight when we get together in-person. From three approved powerful treatments with close to three quarters of affected individuals in the U.S. on at least one, to newborn screening across 99% of the U.S., to more clinical trials happening than ever before especially focused on combination approaches, we continue to make massive strides in a short amount of time.

However, our work is not over. We need your help to fund more basic research to develop more treatments that work in different ways, more combination therapies, and more opportunities to restore and increase strength. We need to double our number of Care Centers and provide more access to high quality care. We need to remove any barriers to living independent, successful, fulfilling lives.

You can help us by participating in a Walk-n-Roll or other fundraising event. Come along and attend a local Summit of Strength. Together, we create a better future for everyone living with SMA. And together, we will celebrate our progress in just a few short weeks in Orlando.

In the meantime, I hope you'll enjoy our spring issue of Directions, which is full of exciting updates, fundraising highlights, and stories from all of you, our SMA community.
Thank you!



Kenneth Hobby
President, Cure SMA

TABLE OF CONTENTS

4 Community Mailbag

Page

8 Community Spotlights

Page

10 Guest Articles

Page

22 Cure SMA Updates

Page

34 Event Updates

Page

The production of this newsletter is supported by
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Letters from the Community



"We received Isla and Aiden's packages from Cure SMA today and wanted to say thank you! They loved opening up all the toys and the excitement of getting something delivered to them. Our older daughter loves art and will enjoy the coloring books and play dough too."

Although this diagnosis is scary and uncertain, we feel so welcomed and cared for by the community."

~ Lambert Family



"Thank you for all those therapeutic toys for the well-being of our children. Thank you Cure SMA for this surprise!"

~ Ramos Family

"We received your gifts yesterday. We are overwhelmed and grateful. Thank you so much! It is beyond our expectation. We also clearly know, without your support and funds, there would be no Spinraza® or Evrysdi®, or Zolgensma®. Those are the true gifts from you and the donators and the researchers."

~ Hu Family



"Rylie and I wanted to reach out and thank you for the teen support packages she received. The items included have already been so helpful in her day-to-day life and have expanded her ability to be independent in day-to-day tasks. We are so incredibly grateful that this is something you offer and for the independence you have helped provide."

~ Erbacher Family



"Thank you for that information packet, the information is what our neurologist recommended as well."

I also wanted to thank you for the package of gifts for Ellie. That was so thoughtful."

~ Pierce Family

"Nolan wants to say thank you for the wagon! He is loving it and we appreciate it so much!"

~ Lange Family

"I just wanted to say a big thank you to those who put together the care packages. It's evident that there was a lot of thought put into them because each item is extremely helpful to anyone who has SMA. The electronic items, like the Echo and Wi-Fi plugs, can truly be lifesavers, but the smaller items like the back scratcher and jar opener make life so much easier. I also appreciate the fleece sheepskin, as I did not know about them and the benefits of using them."

~ Karras Family



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

~ Romero Family

"I sincerely appreciate the gift and your thoughtfulness in helping my children. What a shocking surprise!

Actually, after attending the Cure SMA Dallas Summit of Strength this past weekend, I just feel that I am not alone because Cure SMA is here for my kids.

Your gift will always remind me of your unselfish concern for others.

Thank you very much for the informational packet on SMA and toys and helpful items. What a generous gift! Thank you for adding joy for my kids. You know exactly what they wanted. Not only the gifts but also your thoughtfulness is more than just a gift.

I want to say thank you again for the invitation to Disney. What a generous gift! My kids couldn't believe when I told them about the trip.

What can I say! You just know how to make my kids feel great."

~ Abebe Family

"First and foremost, OMG, was not expecting even a quarter of what ya'll sent us. Throughout all this there are you guys and ladies, helping, doing things we didn't know we needed. That care box has meant more to us than words could ever express. It was something that sanctified we are not alone.

Jack loved everything and played with his new toys so much but out of everything that we got there was one thing that made us cry tears of joy. The quilt. It was so cute, handmade and from someone that has gone through this.

The other major helper that we would never have thought of or heard of was the sheep skin. Our son has been able to find some comfort when he lays on it especially after his first treatment. Without that and scruffy the SMA doggy friend, I don't know if we would have made it.

Here is my son, Jack opening his care package and experiencing the excitement that you all gave him the night before his first treatment. Thanks again for being just the best and most welcoming group out there. We have signed up for the Annual SMA Conference and couldn't be more thankful with the financial scholarship you all provided my family."

~ Ackerman Family



"Thank you for the care package. Abi was so happy and having so much fun with all the toys!"

~ Galicia Family



"Just wanted to share with you all Aniya in her wagon that was sent to us from Cure SMA! She wasn't able to sit without her seat for support in the wagon, but now she is fully sitting all by herself like a big strong girl!"

She enjoyed all the goodies!"

~ Weihs Family

"Thank you so much for everything! He loves them. The sheepskin mat is AMAZING, and I have the little ball hanging from his overhead toy. It's his favorite."

~ Berkebile Family

"THANK YOU SO MUCH!

We have just returned from our vacation and received the package you sent. We were brought to tears. We are so incredibly grateful. This was a true gift. She now cuddles with her soft blankets, talks to her stuffed doggie, and will continue to grow into her other toys over the next few years."

~ Aherns Family



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



Aniya



Waylon



Asher



Pearson



The Wolff Family



The Turnbull Family



Vera



Matias



Spring into Action by Doing It Your Way!

Attention all budding philanthropists—Are you ready to get your hands dirty and bring your creative ideas to life? Then join our Do It Your Way (DIY) fundraising program and let's turn this spring into a season of change.

Sign up today, start your own DIY fundraiser, and make a difference.

*Need some help sprouting some ideas?
We've got you covered.*

- Start a yard sale
- Create a community event in your neighborhood
- Start an athletic-themed fundraiser
- Host a brunch with friends and charge for bottomless mimosas
- Design and sell swag on Bonfire.com
- Host a garden party
- Organize a car wash
- Sell some home baked goods



Small Business Spotlight:

A HOLIDAY WITH HEART



What's sweeter than Valentine's Day? A Valentine's Day that also gives back to our SMA community.

For Valentine's Day 2023, Cure SMA partnered with Janelle Fiesta, an adult with SMA, to create a series of adorable, illustrated Valentine cards, with purchases benefitting our community.

Janelle is an artist, entrepreneur, and owner of Cute Loot Arts LLC, which sells creative and colorful cartoon illustrations, including stickers, enamel pins, clothing, custom commissions, and more.

Janelle founded Cute Loots in 2017. She said, "What began as a spontaneous opportunity to be an art vendor at a local convention manifested into a bigger dream. Due to this experience, Cute Loot Arts eventually came to be. Our mission is to make you happy with our cute and punny cartoon characters. Whether our art merch can make you laugh, feel inspired, smile, create a conversation, or make you go, "Oh my gosh that's so cute, I have to get them all, but I can't choose!" Cute Loots hopes that we're able to somehow brighten up your day."

For Valentine's Day, Janelle brought her creative talents to Cure SMA, creating custom illustrations for a set of Valentine cards that were perfect for school parties and Valentine's Day gift exchanges. These fun and festive cards were sold in kits that included Cure SMA stickers, youth bracelets, and printable coloring book pages.

We plan to continue this tradition annually so we can keep to spreading both love and SMA awareness.

Cure SMA is interested in partnering with other small businesses like Cute Loot Arts. Are you a small business owner with products, services, or talents that you think would be of interest to our #SMACommunity?

Email stories@curesma.org.





Visit the **NEW** CureSMA.org!

The SMA landscape has changed dramatically over the last several years and we're thrilled to announce that our website is now reflective of these exciting advancements and our evolving community.

Here's what you can expect when you visit our new online home:

- A New, Contemporary Look
- Updated and Enriched Content
- Streamlined Menu and Navigation
- Improved Information Searches
- Translates to Multiple Languages
- Photos, Videos, & Stories from You—Our SMA Community
- Fun Ways for You to Get Involved



We'd love to hear your questions or feedback. Contact us at stories@curesma.org and a member of our team will be in touch. Explore more and visit CureSMA.org today!



Caring is Caregiving

By Annie Heathcote

Sometimes an unexpected opportunity, paired with a leap of faith, can become something incredible. A while back, a friend reached out to me about an opportunity to compete for the title “Ms. Wheelchair Wisconsin 2023,” a title that would enable me to advocate at an even larger scale. I applied, got accepted, and had to write and memorize a two-minute speech. Competition day was fun, but intense! It included four rounds of judging, answering multiple disability-related questions, and then giving my speech on my platform “Caring Is Caregiving.” To my delight, I was crowned with the title. As a result, I’ve been interviewed and able to appear or speak at 18 events so far, and I’m looking forward to doing more. I’m so thankful for this title which allows me to impact lives by volunteering and traveling throughout Wisconsin to spread disability awareness! The Ms. Wheelchair America organization has helped women like me share our voices and facilitate positive change wherever we go. I’m looking forward to competing for the national title of “Ms. Wheelchair America 2024” later this year.

Have you experienced trouble finding caregivers? Is the process daunting? Throughout our nation, the crisis of the lack of caregivers is rarely discussed, yet it affects many significantly. With wage competition and our culture becoming more individualistic, it leaves the largest minority group, people with disabilities, vulnerable. According to The Long-Term Care Workforce Crisis 2022 Report, there was an increase in caregiver vacancies at an alarming rate of 27.8% last year alone. So, what are we, as people with disabilities or caregivers, supposed to do amid this crisis? Here are a few tips I’ve learned from experience that could also help you.

First, get the word out that you need help. When you’re ready to find caregivers that are non-family members, it’s essential always to be looking. Sometimes you can find caregivers through job postings on social media or job sites like Indeed, and Care.com, or even your local newspaper. I’ve also found some of my best caregivers in the most unexpected ways. Whether through an outing, church, or social media, once you talk to and let people know that you need care to live your life, they are more inclined to give the job a chance. I don’t need nursing care, so I’m okay with hiring anyone willing to listen, learn, and respect my routine and who I am.

Secondly, if you’re going through an agency to fund your care, the process can be strenuous. Getting the necessary documents to the care agencies and setting up the payroll system to make the job a success can be complicated. Unfortunately, there’s no way to reduce the confusing 20+ page application, but something you can do is mark it up to say what the pages mean and where to sign to simplify it for them. Then, once submitted, make sure you communicate when they’re approved and schedule dates to train them. Once they’re all set to go, it’s important to ease them into the process, especially if they’ve never done caregiving before. Something I found helpful is to have one of my well-trained current caregivers train the new person. I have the new caregiver watch the first shift of the care routine, then during another shift do half of what they’re comfortable with, and then another time do the whole routine while the other caregiver watches. It not only makes them feel comfortable, but it makes me feel safe while they use my different equipment, and we talk through preferences in my care routine.

Caregiving benefits both sides because my caregivers provide me with physical care, but I provide my caregivers with emotional care. With having over 30+ caregivers so far in my life, I’ve had lots of experience with this process. It’s something that many don’t even think about, but the more you are open to having people help you live your best independent life, the easier it gets. I’ve had the pleasure of meeting some of the most incredible people from all different walks of life, which I consider a blessing. I encourage you to follow these helpful tips to better navigate your future of welcoming caregivers to help alongside you.

Follow me on my Ms. Wheelchair Wisconsin journey to see all the exciting changes I help make for people with disabilities. Search for my Facebook page: Ms. Wheelchair Wisconsin America 2023 ~ Annie Heathcote.

Updated SMA Report is Coming....

In June 2022, Cure SMA launched its first annual State of SMA report.
The Updated SMA Report showcased outcomes 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 the annual community update survey
- The SMA Clinical Data Registry (CDR) containing electronic medical record (EMR) sourced data for more than 800 participants 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

Highlights from the report included:

- Demographics of the SMA Community
- Prevalence of SMA type and SMN2 copy number
- Decrease in the age of SMA diagnosis since 2011
- Impact of newborn screening on age at diagnosis and first treatment
- Increase in motor function among those with Type 1 SMA since 2017

The Updated SMA Report will be available starting in June 2023.
A few new outcomes will be presented in the report, including:

- Use of multidisciplinary care
- Use of SMA treatment
- How SMA is changing over time



If you have not already received a copy of the 2021 State of SMA Report, please email research@curesma.org to request one at no charge or visit www.curesma.org to download a copy.



Cure SMA wishes to thank all the members of our SMA community who have generously shared their data. Additionally, Cure SMA is 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). Lastly, Cure SMA is also grateful to the SMA Care Center Network (CCN) for their commitment to improving care for people with SMA and contributing consented patient data.



Update on the Cure SMA Industry Collaboration

Cure SMA is pleased to announce the launch of an expanded Phase 8 of our SMA Industry Collaboration. The SMA Industry Collaboration is a multi-faceted partnership that brings together pharmaceutical companies, Cure SMA, and other nonprofit organizations, to share information, ideas, and data to benefit the broader SMA community.

The goals for the next phase of the SMA Industry Collaboration include:

- Development and refinement of Clinical Trial Readiness Program resources to support effective, patient-centered management of SMA clinical research and patient evaluation.
- Distribution of survey to identify unmet needs and barriers that impact our community's ability to access and participate in SMA clinical research.
- Creation of a campaign to increase clinical trial awareness, knowledge, and access among the SMA community.

About the Cure SMA Industry Collaboration

Funding for this research was provided by the 2023 SMA-IC; members include Cure SMA, Biogen, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, Genentech/Roche Pharmaceuticals, and SMA Europe.



Understanding Peer-to-Peer Fundraising: What Is It and Why Does It Matter?

Network for Good defines peer-to-peer fundraising as “A fundraising strategy in which individual supporters host personal campaigns to collect donations from their friends, family, and colleagues on an organization’s behalf. This approach can help an organization organically expand its reach to new, previously unexplored networks of supporters.”

Peer-to-peer fundraising benefits nonprofits by expanding the organization’s reach beyond its existing contact lists and into new networks consisting of a fundraiser’s family, friends, and colleagues.. By creating peer-to-peer fundraising opportunities, nonprofits like Cure SMA exponentially increase our exposure, which creates opportunities to not only raise funds, but to increase awareness, gain new advocates, and more.

At Cure SMA, peer-to-peer fundraising represents the largest portion of our fundraising dollars brought in each year, allowing us to continue to fund breakthroughs in research and treatments for tomorrow and meaningful support programs for today.



The best part about peer-to-peer fundraising is that all you need to get started is your network, your personal story, and the drive to make a difference. Plus, Cure SMA is here to support and coach you along your journey and to make it easy to fundraise through our peer-to-peer fundraising events, including:

- **Walk-n-Roll** – Cure SMA hosts more than 50 Walk-n-Roll events annually, so there’s bound to be one in your area—and if not—you can start one!
- **Endurance** – Demonstrate your strength and commitment by joining Team Cure SMA for a half marathon, full marathon, 10k or 5k race.
- **Do It Your Way (DIY)** – Fundraise your way! Commemorate a special occasion, host a Facebook fundraiser, or start your own event.
- **Evening of Hope** – Volunteer on a committee or attend an Evening of Hope event. Evenings of Hope feature a night of live entertainment, opportunities to advance Cure SMA’s mission, fundraising recognition, auctions, and more!
- **Golf Outing** – Enjoy a day on the links while supporting our SMA community. You can join one of our existing tournaments or design your own golf event.

You can find out information about all of our peer-to-peer fundraising events by visiting curesma.org/fundraising-events or contact fundraising@curesma.org with questions.

Peer-to-peer fundraising is an excellent way to raise money with the help of friends and family. It offers Cure SMA and our SMA community increased exposure and funds the programs, support services, research, and advancements in care necessary to improve the lives of people with SMA. Through people, passion, and peer-to-peer fundraising, we can and will change lives!

MOVING AHEAD

Evrysdi keeps
working so we can
keep reaching



What is Evrysdi?

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

Important Safety Information

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

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

Tell your healthcare provider about all the medicines you take.

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

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

The most common side effects of Evrysdi include:

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

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

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

Please see accompanying brief summary for additional Important Safety Information.

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

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

LIFE IN ACTION

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

What is EVRYSDI?

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

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

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

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

How should I take EVRYSDI?

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

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

Taking EVRYSDI

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

Reusable Oral Syringes

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

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

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

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

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

How should I store EVRYSDI?

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

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

General information about the safe and effective use of EVRYSDI.

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

What are the ingredients in EVRYSDI?

Active ingredient: risdiplam

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

Genentech

A Member of the Roche Group

EVRYSDI® (risdiplam)

Distributed by:

Genentech, Inc.

A Member of the Roche Group


1 DNA Way

South San Francisco, CA

94080-4990

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

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Getting in the Giving Spirit with Microsoft's Giving Campaign

To support their employees' passion for giving, Microsoft matches employee donations of time and money to nonprofit organizations like Cure SMA. Each October, their fun and spirited employee Giving Campaign—a tradition since 1983—makes a significant annual impact in addition to generous giving year-round.

In October 2022, Cure SMA was fortunate enough to partner with dedicated, driven, and philanthropically inclined Microsoft employees to produce a one-of-a-kind Giving Campaign that resulted in more than \$20,000 in funds raised for Cure SMA!

The Giving Campaign charge was driven by Aparna Katyan and her husband, Mohanish, Azure Data and AI Sales Leader at Microsoft. Mohanish and Aparna are parents to two beautiful children, including their son Arjun who has SMA Type 1. Together with fellow Microsoft employees, they hosted a Giving Campaign Halloween Bash at Microsoft's Silicon Valley campus where children of employees were invited to participate in pumpkin painting, cookie decorating, robotics building, trick or treat stations, a magic show, and a costume contest.

Cure SMA was the only nonprofit invited to attend the event and hosted an information station to educate Microsoft employees and their families about SMA, as well as to collect donations. Cure SMA also got in on the fun by providing prizes for the pumpkin decorating and costume contests, along with plenty of giveaways for attendees, including custom Microsoft + Cure SMA t-shirts.

More than 1,000 people attended the event and Cure SMA looks forward to continuing to grow our partnership with Microsoft and participating in future events.

We would like to extend a special thank you to Aparna and Mohanish Katyan for their leadership and for sharing their family's SMA story, and Jennifer Piellusch, Gabriella Costa, Natasha Medina, AM Gizelle Baylon for helping with event planning and execution. We would also like to thank Rosa Chang Claro, Archana Mehta, Tarunpreet Ubhi, Dhanshri More, Abhishek Dhanuka, Agil Syed, and Shailendra S. who contributed in meaningful ways to the Giving Campaign, as well as all the employees who generously donated to support Cure SMA.

Want to see if your company matches donations to Cure SMA?
Visit curesma.org/employer-gift-match/ and search your company name.



Leadership Spotlight:

Adults with SMA Advisory Council

Adults with SMA represent the largest segment of the SMA population. Building off its regular engagement with the adult community, Cure SMA formed an Adults with SMA Advisory Council in 2020 to ensure Cure SMA activities, programs, and objectives continued to focus on the needs and goals of adults with SMA.

The Adult Advisory Council consists of adults with SMA from across the country who, on a volunteer basis, meet during the year to update Cure SMA on issues impacting adults with SMA and to share their perspectives and ideas for Cure SMA plans and activities. Adult Advisory Council Members also play an important community role in engaging with and sharing information to their personal networks within the SMA community about Cure SMA events, activities, and engagement opportunities.

Cure SMA relied heavily on the Adult Advisory Council in support of the SMA community during the COVID-19 pandemic. The COVID-19 care packages and shift to virtual programming were influenced by feedback and guidance from Adult Advisory Council. Related to legislative advocacy, Adult Advisory Council members identified priorities and recommended that Cure SMA engage the entire SMA population, which became Cure SMA's advocacy agenda focused on greater independence.

Thank you to the Adult Advisory Council and all adults with SMA who have contributed their ideas and perspectives through surveys, advocacy, events, and other Cure SMA activities.

ADULT ADVISORY COUNCIL MEMBERS (2023–2024)

Carolyn Barrett | Cambridge, Massachusetts

Stephen Bingman | Mt. Juliet, Tennessee

Amber Bosselman | Cheyenne, Wyoming

Kyle Derkowski | Ashburn, Virginia

Jose Flores | Tamarac, Florida

Jaclyn Greenwood | Orange, California

Angie Lee | Santa Clara, California

Emma Lockwood | Cincinnati, Ohio

Ryan Manriquez | Davis, California

Doug McCullough | Franklin Park, New Jersey

Dan McHale | San Francisco, California

Brad Nunemaker | Elmhurst, Illinois

Peter Pingerelli | Peoria, Arizona

Collin Pollock | Garden Grove, California

Brian Ronningen | Hudson, Wisconsin

Kevin Schaefer | Cary, North Carolina

Sandy Spoonemore | Plano, Texas

Steven Verdile | New York, New York

Lyza Weisman | Longmont, Colorado

Allie Williams | Stillwater, Oklahoma



Community Spotlight:

Sarah Manuel

In Sarah Manuel's career as a school psychologist, she mentors elementary, middle and high school students as they navigate everyday challenges in life and education. Though it took time and self-acceptance, she is confident that living with SMA helps her relate to students on a deeper level.

"Just because there are limits that a diagnosis places on someone doesn't mean it reaches every aspect of your life. There's so much more that you can do than what you can't," she shared.

According to doctors, Sarah was not supposed to live past early childhood. "I like to say that I'm 40 years past my expiration date. I fully celebrate my birthday and enjoy recognizing my age because it feels like such a gift."

It is because of this perspective that Sarah chose a career path of service, where she contributes to the social, emotional, and academic development of students during their developmentally critical childhood years; she also supports adults through an adult transition program. Her focus is on identifying and advocating for kids, their families, and adults living with disabilities.

Sarah's career is driven by passion, but it was born out of necessity. "When I entered the workforce, I couldn't get a job because of the challenging economy at the time, but eventually found a role in customer service working more than 40 hours a week. It was incredibly difficult on my arms, and I lost a lot of strength. My doctor told me I had to make a job change."

She pondered what to do next. For years, she didn't feel comfortable facing her disability, but something was changing.

"While I wasn't exposed to many people with a disability in my small town, when I got face-to-face with another disabled person, no matter what disability it was, it was like looking in a mirror. And I didn't like it. I preferred to be in denial," she said.

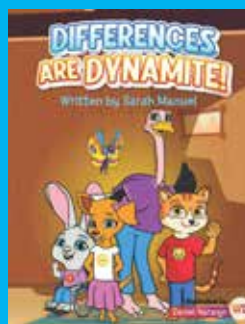
Eventually Sarah's mindset shifted. "I realized that I was made different for a reason. I went from turning away from other people with disabilities to really embracing my experience and advocating and working with kids with disabilities."

A big part of Sarah's shift has been a focus on mindset, which is something she works to instill in her students. "My world right now is surrounded by students with disabilities. The ones that tend to do best in life are the ones that have the mindset or mental attitude that they can go out and do whatever they want to do. Now, it may look different, but they can still achieve their goals and have a meaningful, fulfilling, purpose-driven life."

In addition to her work with students, Sarah's purpose-driven life has included marriage and having a child. Although she was advised by her doctor not to carry a pregnancy, she and her husband made the choice that for them, the risk was worth the potential reward. She now has a happy, healthy 12-year-old son that brings plenty of energy and excitement to her life.

Raising her son and being part of the broader school community network that helps 'raise' or support students is incredibly meaningful to Sarah. It takes a village to raise a child, and she has a message for parents of her students and parents and caregivers of children with SMA.

"I want to give parents and caregivers the permission to dream for your child and allow your child to dream fully and freely. I think when you experience a diagnosis, your world gets really small because you don't know what's going to happen, or what reality is going to be like. Nobody, even my parents, expected me to get married, have a career, and have a child."



In addition to staying busy as a mom and school psychologist, Sarah is also a children's book author. Her book, *Differences are Dynamite!*, follows six animal friends as they discover their differences and how to celebrate those differences. It's available for purchase on Amazon. Search 'Differences are Dynamite! by Sarah Manuel.'



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

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

January 21 | Walnut Creek, CA

February 4 | Atlanta, GA

February 18 | Dallas, TX

March 4 | NYC, NY

March 25 | Baltimore, MD

April 22 | Ann Arbor, MI

April 29 | Portland, OR

May 13 | Cincinnati, OH

May 20 | Mystic, CT

June 3 | Minneapolis, MN

July 22 | Chicago, IL

August 12 | Boston, MA

August 19 | Seattle, WA

September 9 | Birmingham, AL

September 23 | Orlando, FL

October 21 | Charleston, SC

November 4 | Houston, TX

November 11 | Anaheim, CA

November 18 | Miami, FL**

December 2 | New Orleans, LA

December 9 | Phoenix, AZ

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

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

Summit of Strength Webinar Series

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

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

Thank you to the National Presenting Sponsors for the 2023 Summit of Strength Program: Biogen and Genentech.



Thank you to the National Platinum Sponsor for the 2023 Summit of Strength Program: Novartis.



Thank you to the National Visionary Sponsors for the 2023 Summit of Strength Program: Biohaven and Scholar Rock.





2023 ANNUAL SMA CONFERENCE



THURSDAY, JUNE 29TH – SUNDAY, JULY 2ND

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

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

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

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

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.

TO LEARN ABOUT THE ANNUAL SMA CONFERENCE,
please visit www.cureSMA.org.



2023 ANNUAL SMA CONFERENCE

THURSDAY, JUNE 29TH – SUNDAY, JULY 2ND

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



Adults with SMA Sponsorship

This 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. This program is automatically offered to all adults with SMA ages 18 and older. Cure SMA will reach out to those who qualify.

First-Time Attendee Scholarship

Thanks to the generous funding provided by the Luke 18:1 Foundation, Cure SMA covers the costs of up to four (4) family members' registration fees, as well as one (1) hotel room for three (3) nights of the Annual SMA Conference. This program is for any individual with SMA who has never attended the Annual SMA Conference previously, and their immediate family.

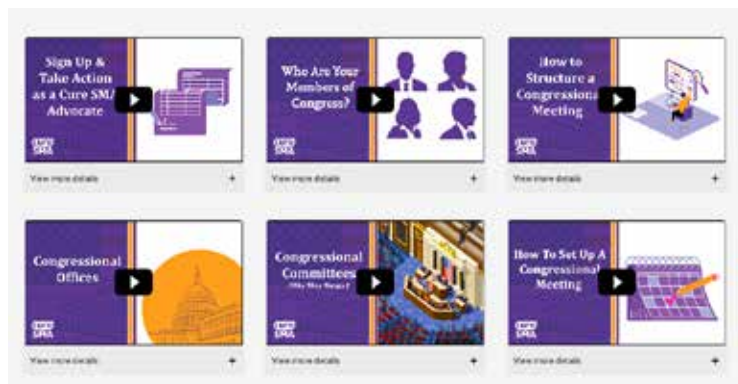
Our goal is to allow as many SMA families and individuals as possible to attend and experience the benefits of this amazing conference and community. The meeting gives everyone the opportunity to gather critical care and daily living information soon after diagnosis, learn directly from experienced SMA physicians, and network with changes in the community. Cure SMA will reach out to those who qualify.

General Scholarships

We also have general conference scholarships available to help waive the registration fee costs to attend the conference. Those looking to receive a general conference scholarship are required to apply and can be placed on a scholarship waiting list. They will be notified if they are able to receive a scholarship.

If you have any questions about conference scholarships, please email conference@curesma.org. More information on scholarships along with ongoing conference updates can be found at www.CureSMA.org.

Advocacy Update



SMA Community Advocate Resources Now Available

Cure SMA released a series of short, online videos to help individuals and families with SMA more effectively advocate with their elected leaders on SMA community priorities.

The video series includes the following topics:

- How to Sign up and Take Action as a Cure SMA Advocate
- Who are Your Members of Congress and How Can You Contact Them?
- Who Works in a Congressional Office?
- How to Set Up a Congressional Meeting
- How to Structure a Congressional Meeting
- What Committees Matter to the SMA Community?

The SMA community can access the videos and other advocacy resources at: www.curesma.org/advocacy/.

New Report Highlights the “Good, (but mostly) Bad, and Ugly” Air Travel Experiences of the SMA Community

BUT MOSTLY,

THE GOOD, BAD AND UGLY OF AIR TRAVEL FOR INDIVIDUALS WITH SPINAL MUSCULAR ATROPHY



Individuals with SMA and families of children with SMA described the ongoing challenges faced when travelling by airplane in a new Cure SMA report.

Cure SMA's *The Good, (but mostly) Bad, and Ugly of Air Travel for People with Spinal Muscular Atrophy* report found that many in the SMA community avoid or only reluctantly travel by airplane due to ongoing challenges, including lost or damaged wheelchairs, injuries during seat transfers, and inaccessible lavatories. The report features air travel stories of individuals and families with SMA from across the country and recommendations for improving air travel in the future, especially for individuals with SMA who use wheelchairs.

Cure SMA released the report in conjunction with the 2023 debate on the future of air travel (through what is known as the FAA reauthorization bill). To view the report or for more information about related to Cure SMA's advocacy efforts in support of accessible air travel, go to www.curesma.org/advocacy/.

To learn more about Cure SMA Advocacy efforts, sign up to become a Cure SMA advocate, at www.curesma.org/advocacy/. Or contact Cure SMA Advocacy Team at advocacy@curesma.org.

SMA Community Priorities Highlighted with New and Returning Members of Congress

Thank you to Cure SMA community advocates who have helped to educate Congress about SMA and the priorities of the SMA community. Since January 2023, more than 300 separate messages from the SMA community advocates were sent at the beginning of the 118th Congress to new and returning Members of Congress to elevate issues important to children and adults with SMA and their families. Your voice and local connections to your Members of Congress are critical in helping to make progress around SMA community priorities, such as healthcare, transportation, employment, housing, technology, and newborn screening. Advocacy is a year-long initiative, not a single action or event. Consider taking action on the issue most important to you through the Cure SMA Advocacy Action Center.





Clinical Research & Care Update

The Clinical Research and Care Department is focused on improving healthcare, health outcomes, and optimizing quality of life for people with SMA. Key to achieving these goals is the collection of data from the SMA community. We frequently ask you to answer surveys about your life experiences living with SMA or as a caregiver of someone with SMA. Your survey responses drive Cure SMA's efforts to develop resources, programs, and services to address the priorities of the SMA community. One of our most important requests for information is the Community Update Survey now in its seventh year. This survey has demonstrated very clearly that SMA has changed and continues to change over time. We ask for your response to the 2023 Community Update Survey available now and thank you for your time and attention to this request. Please see the results from this survey and other data sources in the 2021 State of SMA report

www.curesma.org/state-of-sma-report-2022/

The 2022 State of SMA Report is coming soon!

SMA Clinical Data Registry (CDR) – Core to Improving SMA Healthcare

Cure SMA also gathers data about SMA healthcare in the U.S. to better understand how care is delivered, and the impact of healthcare and treatments on daily life, function, independence, and survival. The SMA Clinical Data Registry (CDR) sponsored by Cure SMA is central to these efforts. The CDR is an Institutional

Review Board (IRB)-governed registry that contains SMA patient healthcare data. The SMA Care Center Network sites across the U.S. consent patients to allow the centers to submit electronic medical record (EMR) clinical data to the SMA CDR. Leveraging the EMR to transmit healthcare data to the CDR is efficient, for both patients and the healthcare team. The information collected in the CDR has already been documented in the EMR. Why not transfer this information to a registry that can pool data for large numbers of individuals with SMA for analysis to better understand SMA care?

The ability to gather information on a large number of patients with SMA in a single registry is powerful. With more than 900 participants, the CDR allows Cure SMA to collect and report on routine healthcare information from a diverse group of individuals within the SMA community.

The information collected in the CDR is called real world data. Real world data is collected from every day and real-world settings compared to clinical trial data that is collected in very specific and controlled situations and settings about a relatively small number of people.

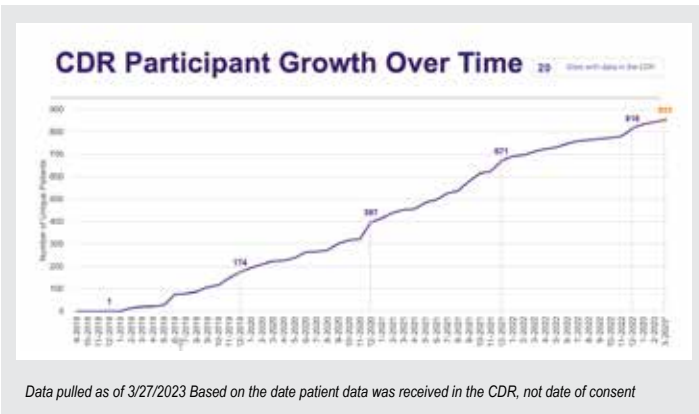
This real-world data provides the core for determining what healthcare should look like for every person with SMA. In collaboration with healthcare providers and people with SMA, Cure SMA will analyze this CDR data to drive care improvements throughout the community. This information will be used to set the standard of care for SMA. Standard of care is treatment that is accepted by medical experts as a proper treatment for a specific disease or condition and that is widely used by healthcare professionals. Cure SMA leads these efforts.

Flip to the back page of this issue to learn how you can help us double our number of Care Centers through the generous support of the Erin Trainor Memorial Fund. Act soon as the matching opportunity ends during our Annual Conference.

Process to Establish SMA Standard of Care Guidelines



Clinical Data Registry Data



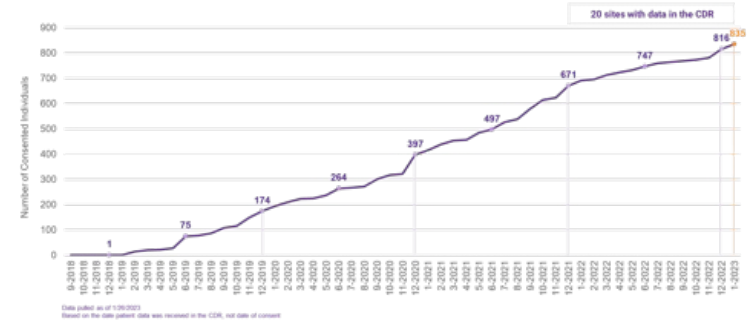
The **Clinical Care Data Registry** holds large amounts of data. This information will be used to answer many questions about SMA to increase understand about how SMA is changing that we have three treatments that are FDA approved.

Questions we plan to answer include:

After receiving an SMA treatment, what are the changes in abilities and motor function across different ages from before treatment to after treatment?

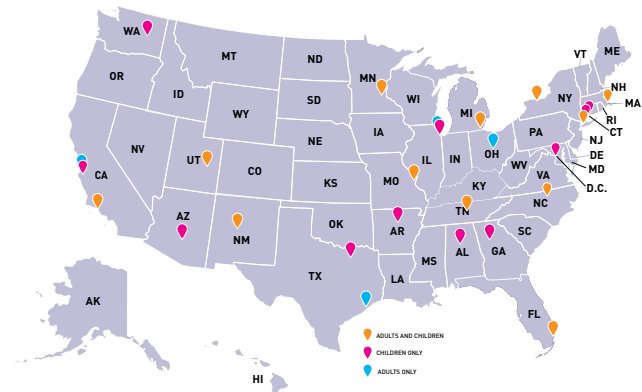
How long does it take for a baby with SMA who was identified by SMA newborn screening to be seen by a specialist? How long does it take to receive their first treatment?

CDR Participant Growth Over Time



SMA Care Center Network Welcomes 29 Sites!

To continue to expand the CDR and increase representation of the SMA community the SMA Care Center Network expanded to 29 centers across the US in 2022. These Centers are geographically diverse and represent a cross section of the many centers caring for individuals with SMA. Nearly half of these centers provide care to both pediatric and adult patients, four centers provide care only to adults, and 12 centers care for only children. We are not done!. Cure SMA plans to expand the SMA Care Center Network to 50–60 centers across the U.S. with the goal to achieve 25% of all U.S. patients with SMA in the CDR. Thank you to the SMA community for your support.



SMA Care Center Network

Advocate Children's Hospital | Park Ridge, IL
 Arkansas Children's Hospital | Little Rock, AR
 Baylor College of Medicine* | Houston, TX
 Boston Children's Hospital | Boston, MA
 Children's Healthcare of Atlanta* | Atlanta, GA
 Children's National Medical Center | Washington D.C.
 Children's of Alabama | Birmingham, AL
 Children's Hospital Colorado* | Aurora, CO
 Northwestern University* | Evanston, IL
 Phoenix Children's Hospital | Phoenix, AZ
 Seattle Children's Hospital | Seattle, WA
 Stanford Children's Health | Palo Alto, CA
 Stanford Health | Palo Alto, CA
 The Children's Hospital of Philadelphia* | Philadelphia, PA

The Ohio State University Wexner Medical Center* | Columbus, OH
 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 | Salt Lake City, UT
 Vanderbilt University Medical Center | Nashville, TN
 Washington University/St. Louis Children's Hospital* | St. Louis, MO
 Yale Pediatric Neuromuscular Clinic | New Haven, CT

*Indicates new Care Center in 2022



Research Update

Currently Recruiting* Interventional SMA Clinical Trials

For more information on these trials, including study locations, additional inclusion criteria for participants, and contacts for each study, please visit clinicaltrials.gov and enter the identifier listed. *Trials recruiting as of 3/1/2023

ASCEND: A Study to Evaluate Higher Dose (HD) Nusinersen (BIIB058) in Participants With Spinal Muscular Atrophy Previously Treated With Risdiplam

Who Is Eligible: Ages 15 to 50 years

ClinicalTrials.gov Identifier: NCT05067790

DEVOTE: Study of Nusinersen (BIIB058) in Participants With Spinal Muscular Atrophy

Who Is Eligible: Ages seven days and older

ClinicalTrials.gov Identifier: NCT04089566

MANATEE: A Study to Investigate the Safety and Efficacy of RO7204239 in Combination With Risdiplam (RO7034067) in Ambulatory Children With Spinal Muscular Atrophy

Who Is Eligible: Ages two to 10 Years

ClinicalTrials.gov Identifier: NCT05115110

RESILIENT: A Study to Evaluate the Efficacy and Safety of Taldefgrobep Alfa in Participants With Spinal Muscular Atrophy

Who is Eligible: Ages four to 21 years

ClinicalTrials.gov Identifier: NCT05337553

RESPOND: A Study of Nusinersen Among Participants With Spinal Muscular Atrophy Who Received Onasemnogene Apeparvovec

Who Is Eligible: Age two months to 36 months

ClinicalTrials.gov Identifier: NCT04488133

SAPPHIRE: Efficacy and Safety of Apitegromab in Patients With Later-Onset Spinal Muscular Atrophy Treated With Nusinersen or Risdiplam

Who Is Eligible: Ages two to 21 Years

ClinicalTrials.gov Identifier: NCT05156320

SMART: Safety and Efficacy of Intravenous OAV101 (AVXS-101) in Pediatric Patients With Spinal Muscular Atrophy

Who Is Eligible: Age up to 17 years, naive to treatment or have discontinued an approved drug/therapy

ClinicalTrials.gov Identifier: NCT04851873

STEER: Efficacy and Safety of Intrathecal OAV101 (AVXS-101) in Pediatric Patients With Type 2 SMA

Who is Eligible: Ages two to 17 years, treatment naive

ClinicalTrials.gov Identifier: NCT05089656

STRENGTH: Phase IIb, Open-label, Multi-center Study to Evaluate Safety, Tolerability and Efficacy of OAV101 Administered Intrathecally to Participants With SMA Who Discontinued Treatment With Nusinersen or Risdiplam

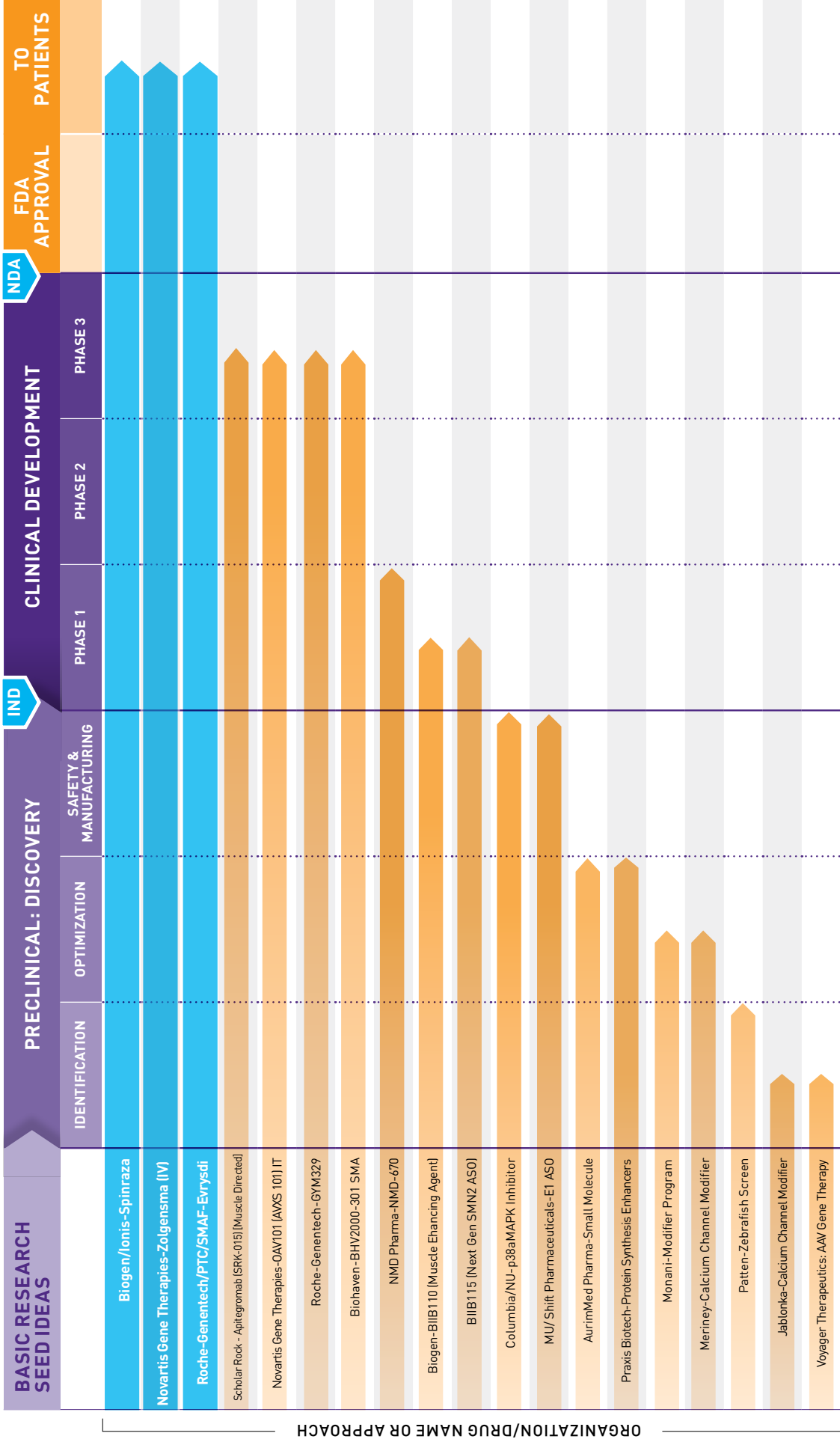
Who is Eligible: Ages two to 12 years

ClinicalTrials.gov Identifier: NCT05386680



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.



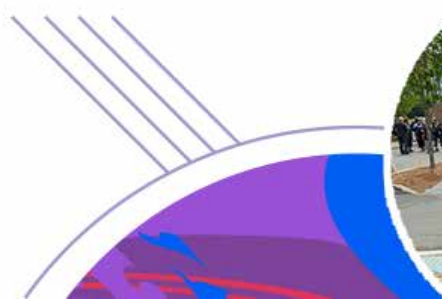
IND = Investigational New Drug

NDA = New Drug Application

Last updated: March 2023



REDEFINING
RARE ● ● ●



#RedefiningRare: Rare Disease Month 2023

In February, Cure SMA, our SMA community, and the rare disease community at large recognized Rare Disease Month. Rare Disease Month is the official international awareness-raising campaign for all rare diseases. February was officially declared Rare Disease Month in 2008 with February having the ‘rarest’ number of days.

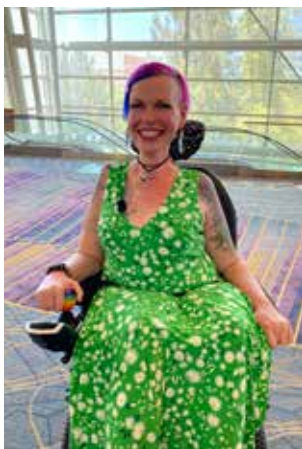
According to the Genetic and Rare Disease Information Center, “There are about 7,000 known rare diseases and collectively, about one in ten people, or 30 million people in the U.S., have a rare disease.” Rare is not so rare after all!

This February, we focused on three key initiatives, which included:

1. Increasing awareness of SMA among the general public, including sharing key facts and statistics, as well as personal stories from our community
2. Encouraging our community to take action through grassroots efforts, including registering for and participating in Cure SMA Walk-n-Rolls and Summits of Strength, hosting DIY events, donating to Cure SMA, and taking advocacy actions
3. Partnering with USA Today on a centerfold magazine insert, which appeared in the March 31st issue of USA Today

Cure SMA served as thought leaders and told stories from our community, along with representatives from National Organization for Rare Disorders (NORD), EveryLife Foundation for Rare Diseases, American College of Medical Genetics and Genomics, and pharmaceutical industry leaders. The goal of this feature was to make the public aware of how common living with a rare disease is, and address key challenges patients and families face, while advocating for greater health equity and access for rare disease patients across the healthcare continuum.

Cure SMA would like to thank Mimi and Brady Chan, Janelle Fiesta, Brad Nunemaker, and Allie Williams, who shared their stories for USA Today, as well as all the members of our community who #RedefinedRare through social media posts, donations, and event registrations and participation in February and beyond. Thanks for helping make the world at large more aware of how common ‘rare’ really is!



Greater Equity Needed for Those in the Spinal Muscular Atrophy Community

Rapid advancements in FDA-approved treatments have improved the quality of life for people living with spinal muscular atrophy (SMA) but societal barriers remain.

Spinal muscular atrophy (SMA) is a genetic neurodegenerative disease that robs people of physical strength, impacting their ability to walk, eat, and breathe. Due to muscle weakness, many people with SMA use power wheelchairs.

Three FDA-approved treatments have gone to market since 2016, greatly improving outcomes and providing new hope for people living with SMA. Additionally, 98 percent of newborns in the U.S. are now screened for the disease at birth. While this medical progress is immense, roadblocks in society persist.

“Besides the physical side of SMA, the emotional aspects have evolved over the years. Anxiety about the unknown – will I have any issue getting into a new building, will I be able to get into a friend’s house, will it be hard for me to get up from my seat at the restaurant – are always present,” shared Brad Nunemaker.

Through continued advocacy and awareness amongst the general public, members of the SMA community are hopeful of change.

“The system doesn’t work in our favor”

Many adults with SMA utilize Medicaid for care services such as in-home aids to assist with basic tasks or physical therapy, in addition to other home and community-based services. However, income and asset limits that exist in some state programs force individuals with SMA to choose between a meaningful career and independence.

“I didn’t realize this was a problem until I grew up,” shared 26-year-old Janelle Fiesta of Hawaii. “When I found out about the \$2,000 asset limit, I was like ‘how am I ever going to buy a house or pay for a car?’”

Janelle is currently employed at the Department of Navy’s Equal Opportunity Office and operates a small business, Cute Loot Arts. She is still figuring life out but feels proud of what she has accomplished and capable of expanding her horizons.

“I want to learn to drive my own adaptive vehicle, I want to move out, I want to be able to start a family with my partner,” said Janelle.

“I know my rights as a disabled person on a flight”

Air travel itself can be stressful. For people with SMA, the chance of an airline losing or breaking their wheelchair adds additional anxiety.

In 2007, an airline broke Allie Williams’ wheelchair to the tune of \$20,000 in damages. It took eight months to fix. In that time, she lost so much function and movement that she could no longer do her makeup, something she cherished.

Nearly fifteen years later, thanks to a new treatment, Allie has once again mastered the art of makeup; with the support and encouragement of friends and family, she also returned to traveling via airplane.

“I know my rights as a disabled person on a flight and I really think that’s important,” she said.

Self-advocacy, as Allie describes it, is equally important. While there is a strong need to be able to use power wheelchairs on aircrafts, asking for proper accommodation and stating your needs helps alleviate confusion in the meantime.

“I’ve taken extra precautions for my chair. I have a piece of paper on there with my picture because I want them to put a face to it. I say, ‘thank you for taking care of my legs’ and put an air-tag on my wheelchair so I know that it’s on the plane when we take off.”

“It gave the kids an opportunity to connect on a deeper level”

In December 12-year-old Brady Chan organized neighborhood bake sales with a group of friends, all in sixth grade, to benefit SMA research and support programs. The kids planned every detail, had a taste-test, and connected about the importance of raising critical funds.

“Brady still kind of struggles with educating others about SMA. He would much rather drive away and avoid the topic. This was the first time he’d faced it straight on and talked about the disease that he lives with and how the organization Cure SMA has helped him,” shared his mom, Mimi Chan.

“It gave the kids an opportunity to connect on a deeper level and to have a better understanding of what Brady lives with. It brought them closer.”

Not only did the kids raise over \$64,000, but they also made a new connection with another family local family affected by SMA.

A common thread amongst Brad, Janelle, Allie, Mimi, and Brady’s stories is one of belonging.

“Reach out to others and connect, it’s easy to feel isolated or that others don’t understand what you’re going through—we are meant to build relationships and share our stories with other people,” emphasized Janelle.



This article was originally published on March 31, 2023 for Mediaplanet/USA Today’s Special Edition on rare diseases.



Community Spotlight:

Michael Hazel

It's appropriate that Michael Hazel is studying film in college because, he said,

“my life has been like a movie in the best and worst ways.”

Michael was diagnosed with SMA Type 2 at 18-months old after his parents noticed that he wasn't hitting key milestones like crawling and sitting up on his own.

Michael's early years weren't easy for him and his family. Growing up in a “low-income household” with a single mother caring for three children, two of whom have SMA, meant that time, money, and other resources were sometimes stretched thin. He says, “Having a sister with SMA, a sister without SMA, having multiple diagnoses outside of SMA myself, and having several near-death experiences” deeply impacted his childhood and early adult years.

Thankfully, Michael's recent experiences have included positive advancements in his health care journey. He says, “It's amazing how much I've experienced since starting Everydi® only 21 months ago. I've experienced elevated energy levels, stronger arms (which we even measured), stronger lungs and cough (which we also measured recently), stronger face muscles, stronger voice, stronger body control—stronger everything!”

Despite the obstacles Michael has faced, and perhaps because of what they've taught him, he is living life on his own terms and helping others in the process. “I've experienced quite a lot in my 24 years on this planet. Since I've experienced so much, I can relate with a lot of people and understand a lot of diverse groups and demographics.”

He said, “I'm personally proudest of just being me. Yes, SMA is a part of me, but it shouldn't be a personality trait or representation of my soul. I love showing other SMA-ers that we can live a “normal” life and experience normal human things. It may take a few more steps, but it is possible!”

One of Michael's most recent accomplishments has been his acceptance into Montclair State University where he is majoring in Film and Television. Michael is the first student with SMA to attend Montclair State and the first disabled student to live on campus. Being the first has meant Michael has had to become his own on-campus advocate. He says, “Many SMA-ers will agree you have to constantly advocate for yourself and prove your disability just to get the assistance you need. Some of those things we need are more time to complete things, accessible entrances, accessible devices, personal care assistants, and more sick days.”


As for what Michael would like students, faculty, and others on campus to know about him, he said “People with SMA are just normal. Most of us just want equality and to be just as seen as anyone else. We don't need to be babied or put on a pedestal—just give us equality!”

Recently, Michael and his sister Noelle started a podcast called DisabiliTEA, where they discuss issues related to their SMA diagnoses as well as broader societal issues in order to advocate for change. You can find DisabiliTEA on Apple Podcasts, Spotify, and more.

In addition to advocating personally, Michael has also taken advantage of the Cure SMA network to build connections and share his voice. He said, “I've known about Cure SMA for as long as I could remember. My first Cure SMA Conference experience was when I was four-years old!”

“Cure SMA impacts my life daily because they're partly responsible with funding and finding the current treatments that are out! I'm so thankful for Cure SMA's relentless advocacy, partnership, and resources. Between that and the amazing conference, Cure SMA impacts my life all the time.”

While Cure SMA has had a significant impact on his life, Michael is also making a positive impact on the lives of others by blazing the trail for future students with disabilities at his university, giving voice to the needs of the SMA community and showing us all that SMA is just one aspect of his full and adventurous life.



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.

**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.**



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

Check out our updated booklets.



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

Booklets are available online and are translated into 12 languages.



Letting the Good Times Roll with Cure SMA's Nationwide Walk-n-Roll Program

HERE ARE SOME EXCITING HIGHLIGHTS FROM OUR
FALL 2022 WALK-N-ROLL SEASON:



Together, we raised more than
\$600,000
and counting!

- 18 walks held across the country
- More than 3,100 people participated
- More than 250 fundraising teams were created
- The average participant raised \$165

The Walk-n-Roll is a nationwide fundraising program that brings communities together to support Cure SMA's funding of life-changing research, resources, and programming for our #SMAcommunity. Each year, nearly 50 Walk-n-Roll events are held throughout the country.

We have two seasons of the Walk-n-Roll program each year, with the spring season beginning in March and running through June and the fall season beginning in August and running through November.



By participating in a Walk-n-Roll event you'll have the opportunity to connect with fellow SMA community members in your local area, raise crucial funds, earn awesome incentives, and let the good times roll. We can't wait to see you at an upcoming Walk-n-Roll event! Register for a walk or find more information at walk-curesma.donordrive.com.

August is SMA Awareness Month!

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

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

Here are the best ways to get ready for August:

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



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



WALK-N-ROLL Champions Club

Fall 2022 Champions Club Members

Arizona Walk-n-Roll

Tim Cheeks
Ashley Fleck
Kasey Kaler
Christopher Pantier
Emily Pantier
Angel Wolff

Atlanta Walk-n-Roll

Henry White

Capital New York Walk-n-Roll

Craig Couture
Amy Cuniff Bleau
Carrie Kelly
George Whalen
Brooks Wolfe

Charlotte Walk-n-Roll

Anne Davis
Lauren Evans
Virginia Hobson
Lindsey Quinn
Morgan Stewart
Shannon Sykes
Sabra Turnbull
Idalmis Vivar

Colorado Walk-n-Roll

Karis Lambert
Michelle Pritekel

Cure SMA Walk for Graham

Jessica Biggerstaff
Brian Council
Susan & Jim Goldman
Michael Hamaker
Jessica Lueken
Marilyn Miller
Michael North
Shana Parikh
Roshni Patel
Mila Thomas
Vanessa Valencia
Adrienne Vollmer

Kansas City Walk-n-Roll

Charlie Sykora
Kim Sykora

Louisville Walk-n-Roll

Kristen DeLuca
Courtney Ogletree

Madison Walk-n-Roll

Christina Hayes
Rita Jakel

Minnesota Walk-n-Roll

Amanda Stanton

Nashville Walk-n-Roll

Kelsie Cherry
Peyton Cherry
Shawna Hibben

North New Jersey Walk-n-Roll

Elizabeth Fazio
Carly Hewitt
Denise Meredith

Northern California Walk-n-Roll

Michael Barsotti
Cathy Barsotti-Dooling
Jane Coggins
Katee DellaMaggiora
Nancy Dindzans
Annie Dooling
Leonette Eide
Danielle Galan
Ryan Manriquez
Ashley Petralli
Jennifer Rohrs
Linda Shively
Marge Shively
Claire Venezia
Wayne Waggoner
Patricia Wolff

Oklahoma Walk-n-Roll

Rachel Chambless
Lauren Fontenot
Blake Mach
Allie Williams

Raleigh Walk-n-Roll

Patrick Jones
Lashawn Partlow
Kevin Schaefer

Seattle Walk-n-Roll

Erin Courteau
Paige Drennen
Dennis Geissler
Kris Hanson
Christopher Jankowski

Virginia Walk-n-Roll

Adam Lackey
Corey Weber

Wakefield 5K Benefitting Cure SMA

Kristen Farrell
Susie Sapienza

Western Suburbs Walk-n-Roll

Kathleen Heinrich
Diane Kutschke
Emily Schuurman
Amy Thomasson

We would like to congratulate the following fall 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 event.

Our fall Champions Club members knocked it out of the park! We had four times more Champions Club members from our fall 2022 season as fall 2021. These fundraising superstars raised half of the \$600,000 total brought in during the fall season! Congratulations!

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

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



facebook.com/cureSMA



www.linkedin.com/company/curesma



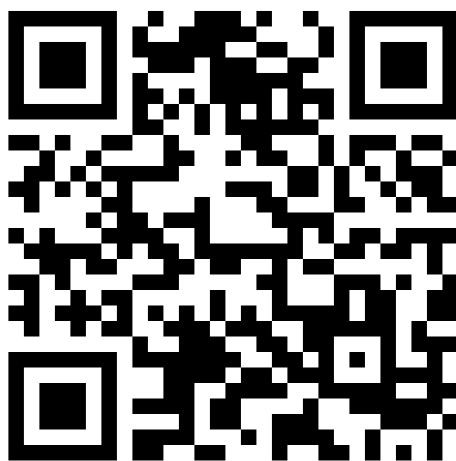
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**cure
SMA**

#CureSMA and #SMACommunity



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

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

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

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

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

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

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



Orange Pin – I have SMA



Blue Pin – I have lost someone to SMA



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

We've already been enjoying seeing all the thoughtful and personal pinwheel gardens at our spring Walk-n-Roll events and look forward to our beautiful gardens continuing to bloom throughout the remainder of the year.

DO IT YOUR WAY Fundraising Highlights

Throughout each year, members of our Cure SMA community host countless, 'Do it Your Way,' DIY Fundraisers, customizing their fundraising unique events to the host's interests and skill set. Whether you are thinking about fundraising in honor of your birthday or a special occasion, participating in an athletic event creating a memorial page, or starting your own creative fundraiser, Cure SMA's DIY fundraising has you covered. Check out the following DIY event highlights from the fall fundraising season and visit diy-curesma.donor.drive.com or contact the fundraising@curesma.org for questions or assistance sharing your own DIY fundraiser.

Wakefield Walk-n-Roll

Team BraeKer held their eighth annual Walk-n-Roll fundraiser on August 20, 2022, at Lake Quannapowitt in Wakefield, Massachusetts. Kristen and Jim Farrell—with help from their kids Braden and Kernan—host this Walk-n-Roll event each year, featuring a 5K race, raffle, and plenty of fun. Thanks to all their hard work, this year's event raised over \$10,000 for Cure SMA!

Reach4Sky

In 2022, Team Reach4Sky pledged to swim 21 miles in honor of their son Skylar's 21st birthday! Liz and Jim Bahrenburg set out on their journey with friends and family to complete ten swims at six different venues across the country. Their stops included an open water swim in Kailua, Hawaii; a first-ever Tampa, Florida swim where they experienced hammerhead shark sightings; and a scenic swim under the Colorado Bridge in San Diego, California followed by a small gathering of loved ones to toast Skylar's birthday. Team Reach4Sky has been hosting this swim fundraiser for nearly 20 years! This year, we want to congratulate Liz, Jim, and the rest of Team Reach4Sky on reaching your goal of raising over \$50,000 for Cure SMA! .

SMash Dash

On August 13, 2022, Jessica and Rusty Hastings—with help from their kids Cooper, Kolbie, Harden, and Kadence—hosted their annual SMash Dash fundraiser at Fair Oaks Ranch Country Club in Fair Oaks, Texas. This event, honoring daughter Kadence, is a 5K race with breakfast, swag sales, and lots of family fun! This past year, the Hastings even partnered with a local consignment shop so that any gently used clothing items donated at the event could be sold to raise funds for Cure SMA. Thank you to the Hastings family for helping raise over \$20,000 for Cure SMA!

Honorable Mentions:

Bommarito Z Car Club Show

Thank you to Jerry Eickel, Janet Hutchinson, and the team at Bommarito-Nissan of Hazelwood, Missouri for generously donating \$21,000 in lieu of their past Bommarito Z Car Club Show! While the car show may be retired, we appreciate your continued support of Cure SMA.

Leon's Light Cornhole Tournament

This past year, Monica and Gilbert Kohl held their second annual Leon's Light Cornhole Tournament in memory of their son, Leon. Leon's light continues to shine through this event, where family and friends came together for a day of fundraising and fun, raising \$3,000 for Cure SMA!

Oskar's Jam for Cure SMA

Oskar, with help from parents Ima and David Jonsdottir, put on another amazing backyard concert in 2022! Oskar and his band played a variety of live music for friends and family and welcomed guests to hop up on stage and perform! This event filled with music, laughter, and dancing raised \$11,000 for Cure SMA!

Lukie's Fall Festival

Lukie's Fall Festival came together again this past October at Weona Park in Pennsylvania for their 19th annual event. The Maida family hosts this festival—complete with entertainment, food, games, and costume parades—each year so that their local community can gather for a day of family fun while raising funds to find a cure for SMA, all in memory of their son, Lukie. This year's event raised \$10,000 for Cure SMA!

Kate the Great

Each year, Elizabeth and Brian Veit host a t-shirt fundraiser in honor of Kate—referred to as Kate the Great, a nickname given by her big brother, Ben. This past August, the Veit family raised nearly \$2,000 for Cure SMA through t-shirt sales!

Bella's Walk for Cure SMA

This past August, Jessica, and Matthew McIntyre hosted Bella's Walk for Cure SMA at Wide Waters Park in New York. This event, in honor of their daughter Bella, features a raffle, kid's activities, and more! Thank you to the McIntyre family for helping raise \$3,000 for Cure SMA!

No Shave November

Kevin Pearce grew out his beard while collecting donations for No Shave November in 2022. With support from his community, Kevin was able to raise over \$5,000 for Cure SMA!

Rogers Family Christmas Lights

Each year, Mandy and Eric Rogers' family comes together to raise funds for Cure SMA in memory of their son, Caden. This past winter, the Rogers family put on an incredible synchronized Christmas light show! Spectators showed up, hot chocolate in hand, ready to dance and display their holiday spirit! Thanks to the Rogers family and all of those who enjoyed the show, nearly \$2,000 was raised for Cure SMA!

NEW Cure SMA Merchandise!



Items to checkout:

- Candle – \$24
- Wheelchair Joystick Replacement Knob – \$25
- Cooler Backpack – \$45
- Women's White Reflective Performance Half Zip – \$49
- Bucket Hat – \$20
- Black Performance Full-Zip Fleece – \$49
- Women's Performance Racerback Tank – \$25
- Cookie Cutter + Stamp Set – \$12
- Valentine's Day Goodie Bag Bundle – \$30
- Adhesive Cell Phone Wallet – \$10



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

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



Chapter Socials & Events

Members of the Arizona, Southern California, & Southern Florida Chapters gathered together to meet and greet friends new and old at their locally hosted Chapter Socials.

Members of the Arizona Chapter had a great Chapter Social event at the Phoenix Zoo with lions, tigers, and bears (oh my!). It was a wonderful day to get outdoors, see wildlife up close, and get to know other members of the chapter! Thank you to Arizona Chapter Leader, Angel Wolff, for putting together a wildly fun day!

In South Florida, guests had a tasty dinner at Carrabba's and had plenty of time to meet and greet local chapter leaders, as well as enjoying a surprise visit from Santa himself! Thank you to South Florida Chapter Leaders, Fiorenna and Jennifer Miller Smith, for taking the time to put together such a great event!

Moving over to the West Coast, members of the Southern California Chapter participated in a festive and fun PJ party! Guests enjoyed a tasty lunch, holiday treats, and Santa was able to make his way over from South Florida to greet everyone! Thank you to SoCal Chapter Leader, Autumn Montoya, for putting together another excellent party!



Nearly thirteen years ago, Paula Lavigne and Chris Arnold's adorable son, Wyatt, entered the world on July 9, 2010. While Paula and Chris could tell shortly after birth that Wyatt was struggling, it took what felt like a long time in Wyatt's short life, for him to be diagnosed with SMA. Wyatt finally received his diagnosis in October 2010, and he passed away on December 12, 2010, shortly after turning five months old.

Their son's diagnosis introduced Paul and Chris to our SMA community, and while they were devastated by the loss of their son, our powerful SMA community has brought them some comfort in their journey. Paula and Chris have been able to build a compassionate, caring, dedicated, and loyal SMA village, consisting of what are now sure to be lifelong friends.

Cure SMA has served as an important component of Paula and Chris' village. They first learned about the organization after Wyatt's diagnosis. Through Cure SMA, they connected with other SMA-affected families in their state, were provided with provided information on SMA and received helpful support materials. To this day, they vividly remember the giant box they received in the mail with loads of items to help them with feeding, sleeping, and mobility accommodations for Wyatt, along with toys designed for SMA-affected infants.

"The generosity from Cure SMA was unbelievable. But more than that, the knowledge that we weren't alone in this really kept us going in a very dark time."

~ Paula Lavigne and Chris Arnold

Paula and Chris remain engaged with our SMA community and continue to donate to Cure SMA to help accelerate progress towards a cure. As a journalist for ESPN, Paula has been able to promote SMA awareness to a larger audience. Paula and Chris are committed to finding a cure, supporting families like theirs, and giving Wyatt a way to live on and have a lasting impact. Although sometimes it feels a little bittersweet, because as thrilled as they are about the treatment options available, those options weren't there for Wyatt, leading them to wonder what life would be like today if Wyatt had been able to be the beneficiary of today's advances in SMA treatments and care.

Donor Spotlight:

Paula Lavigne and Chris Arnold

But they also feel pride, because together with Cure SMA they have helped add SMA to newborn screening panels and create effective and innovative treatments, all while supporting the day-to-day challenges of both newly diagnosed and long-time SMA patients. They see videos of children who had Wyatt's same diagnosis as an infant who are holding their heads up, sitting independently, and in some cases, walking—all things they consider near miraculous.

Paula and Chris say they are excited about the future of SMA, and the work being done with gene research, including how methods to cure SMA could lead to innovative ways to cure other genetic diseases. That also factors into why they tell people to support Cure SMA, because breakthroughs here—where they seem so close—could have a ripple effect in rare disease, and overall medical, research.

A year and a half after Wyatt passed, Paula gave birth to twins who have added joy to Paula and Chris' home and hearts. They're expanded family continues to include daily reminders of Wyatt, and the twins have been told stories about their brother since the day they were born. In addition to these stories, Paula, Chris, and the twins have made a priority to make taking action on Wyatt's behalf even more important. They testified before their state legislature to add SMA to the newborn screening panel in Nebraska, and Wyatt's younger brother and sister got to watch as their father told senators about Wyatt and his experience with SMA. When the Legislation passed, the entire family celebrated. Through Wyatt, his siblings are fighting for change, learning compassion, and ready to take on the world.



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 SMA in your
community and beyond.

Thank You to Our National Premier Sponsors



Biogen

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NOVARTIS



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

Fall Special Events Highlights

Cure SMA Special Events invite you to grab your closest friends and spend an evening on the town or out on the course while supporting Cure SMA's mission and our SMA community. Check out the following Special Event highlights from the fall fundraising season and visit curesma.org/special-events or contact the fundraising@curesma.org for questions or assistance participating in an endurance fundraiser.



2022 Birdies for Blake LPGA Watch Party

On Sunday, September 11, 2022 the LPGA Tour entered its final round in Cincinnati, where the Farrell Family hosted the first-ever Birdies for Blake LPGA Watch Party Benefitting Cure SMA! Despite the unexpected rain that forced some last-minute schedule changes and pivoting, nearly 60 people attended and enjoyed a truly wonderful afternoon of golf viewing, delicious food, drinks, silent auction, and more.

Through their amazing work, Kacey Farrell and her committee secured \$23,000 in sponsorship, over \$7,000 in silent auction revenue, and generous gifts from family and friends for a total of nearly \$63,000 raised for Cure SMA!

Thank you, Kacey Farrell, the planning committee, and the Farrell Family for your continued support!



2022 Tee it Up to Cure SMA Golf Outing in Honor of Ava and Hailey

Congratulations and thank you to the Finelli Family for raising nearly \$115,000 for Cure SMA at their 2022 Tee It Up Golf Outing Honoring Ava and Hailey Finelli! More than 100 golfers came out for lunch and to hit the links at the Forest Hill Field Club in Bloomfield, N.J., on September 22, 2022.

After a full day of golf, nearly 50 additional guests joined for a dinner and an evening of fun, which included a silent auction. Ava and Hailey's mother, Laura Finelli, gave a heart-felt speech about why it is so important to continue to support this amazing community, and Kenneth Hobby updated the attendees about the amazing progress Cure SMA continues to make, and how that progress wouldn't have been possible with all of their support.

We want to extend a special thank you to Dan and Laura Finelli, and Bill and Lorraine Finelli for hosting this wonderful event once again in support of Cure SMA, and for their incredible leadership!





EVENING OF HOPE Update

2023 Carolinas Evening of Hope featuring Hayes' Heroes

The 8th Annual Carolinas Evening of Hope (formerly the Hayes' Heroes Evening of Hope) was held on February 25, 2023 at the Country Club of Lexington, in Lexington, S.C. The event was hosted by award winning journalist, Brandon Taylor, and guests enjoyed a fun evening full of great food, a silent and live auction, wine pull, and live music by 3 Guys and My Dad!

This year, the event raised nearly \$38,000 – which means that this event has raised almost \$250,000 for Cure SMA since it's first year!

We want to extend a special thanks to the amazing planning committee: Keri Johnson, Kathy Cooper, Dawn Hyatt, Jody Ross, Norma Jean Largent, and Joyce Sireno. This event would not be possible without their hard work and dedication every year!



Kids Bake a Difference

Raising \$80K for Cure SMA!



Who runs the world? Kids! During the 2022 holiday season, more than 100 kids participated in Cure SMA's first ever Baking a Difference fundraiser. Kids aged 18 and under were invited to register for the program, form teams, and fundraise by selling sweet treats to benefit Cure SMA.

The program kicked off with a virtual baking party featuring pastry chef, baker, cookbook author, and social media influencer, Molly Wilkinson, who taught the kids how to make simple, tasty desserts, and Dana Perella, an elementary schooler, and founder of Cookies4Cures, who has raised more than \$50,000 over the past three years for Cure SMA, who passed along her expert fundraising tips. Each kid participant also received a free kit of baking tools, generously donated by Raddish Kids and Rubbermaid.

With a \$50,000 fundraising goal set by Cure SMA, the kid bakers rose to the challenge, whipping up hundreds of cookies, brownies, and other desserts to sell at school and neighborhood bake sales. Some even streamlined their operations by taking preorders via phone, text, and social media.

While the program was a successful and fun-filled effort for all our participants, one baking team was the beneficiary of life-changing generosity. On December 3rd, twelve-year-old Brady Chan, who has SMA, and several other kids in his Cary, North Carolina neighborhood organized their first bake sale. Their goal was to raise \$600 for Baking a Difference and Cure SMA. The bake sale had raised almost \$1,900 by the end of the day, and as the kids cleaned up and were feeling good about their successful endeavor, a neighbor who prefers to remain anonymous stopped by with a check for \$60,000!

Brady was interviewed by ABC 11 Eyewitness News and said, "At first, I was like, six, that's \$60,000? Cool," Chan explained. "And then I realized that is like a ton of money. I was like, oh, wow! I was really excited and happy because that could help a lot of people."

Brady's mom, Mimi said, "As a family that has been on the receiving end of living with spinal muscular atrophy, \$60,000 can help so many families," Mimi Chan said. "Sorry, I did not mean to let the tears out, but it's huge. It will help so many families."

Cure SMA would like to thank all the kid bakers as well as their parents and caregivers for your sweet treats and even sweeter hearts. These kid chefs, entrepreneurs, and philanthropists truly are our future. We can't wait to see what they cook up next!



If you or your child are interested in participating in our next Baking a Difference fundraiser, we'll be launching Baking a Difference 2023 this November. In the meantime, consider a DIY Youth Event fundraiser. Learn more here: diy-curesma.donordrive.com.

Get Ready to Lace Up Your Sneakers and **Spring** into Action!

Join Cure SMA's Endurance program and sign up for a race! With a variety of different races to choose from, you'll have plenty of opportunities to push yourself to the finish line and beyond—and don't worry—we'll be there to cheer you on every step of the way!

This season let's run with purpose and make a positive impact in the SMA community. Get a spring in your step and register for a race with us today!

Remember, every step counts and together we can cross the finish line while raising crucial funds for our #SMAcommunity!



Have questions? We're happy to help! Contact us at fundraising@curesma.org.





What Can You Do with \$5.00?

You can buy a fancy cupcake or a matcha latte, or for \$5.00 you can be part of a movement to change lives.

During the first Friday of every month, Cure SMA gets in the giving spirit, asking our SMA community and your friends, family, and colleagues to donate \$5.00.

If everyone who received this issue of Directions donated \$5.00, we'd raise nearly \$100,000 to fund life-changing research, practical and meaningful support programs, and access to high quality care for individuals with SMA.

Join us for

\$5.00 Fridays on:

- June 2nd
- July 7th
- August 4th
- September 1st
- October 6th
- November 3rd

The best way to give is to set up a monthly, reoccurring donation.



Donate today

And...you don't have to wait for \$5.00 Friday. Any day is the right day to make a difference!



A Season of Giving: Cure SMA's Year-End Fundraising Campaign

“The greatness of a community is most accurately measured by the compassionate actions of its members.”

~ Coretta Scott King

There are several key times each year when Cure SMA's mission is on full display for our entire community—the Annual SMA Conference, SMA Awareness Month, and the Year-End Campaign. From November through January 2022, the Cure SMA staff and volunteers send thousands of messages, shared dozens of stories, posted on a variety of social media channels, and asked our constituents to generously give to Cure SMA as part of your holiday giving plans.

The campaign raises between \$800,000 and \$1,000,000 annually, which goes towards the organization's general operating fund—a critical piece of our fiscal year. An effort of this magnitude doesn't come together without the entire organization contributing. Cure SMA's Community Support team pulled together compelling quotes, stories, and photos from our SMA community. The Advocacy, Clinical Care, and Research teams provided statements about Cure SMA's impact and what accomplishments will be made due to generous donations. The Finance and Operations team coded and processed thousands of gifts. The Marketing and Communications team put the stories together and made the campaign visually and emotionally appealing and the Development team personally reached out to donors through hundreds of phone calls, emails, and meetings to directly communicate the importance of contributing to the campaign and to secure donations.

While we couldn't execute this campaign without Cure SMA's dedicated staff, the most important component of our Year-End Campaign was you, our donor, advocate, and supporter community. The 2022 Year-End Campaign raised \$835,652. More than 1,800 of you donated, with the average donation totaling \$469! That's more than \$800,000 that will fund basic research to find more treatments that work in different ways, more combination therapies, and more opportunities to increase strength and flexibility. It will help us in our goal to double our number of Care Centers and provide more access to high quality care and remove barriers to living independent, successful, fulfilling lives.

Finally, we would like to extend a special thank you to Aaron and Barbara Brzycki, Joe Lakhman, and Jose Flores, who opened up their lives and hearts to share their personal SMA journeys throughout the Year-End Campaign. What a joy to see the boundless energy of three-year-old Aaron, a transition to living life more independently for the first time for college student, Joe, and Jose making great strides in physical therapy, standing for the first time in 23 years!

Your generosity gave us plenty of reasons to celebrate this holiday season. Thank you again to our donor community. With your support, we are creating a better future for everyone living with SMA.

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.*



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

Submissions

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

Change of Address

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

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Double Your Donation to Help Double Cure SMA's Care Center Network

**Today, your donation can
have twice the impact!**

Help us double the number of Care Center sites and the Erin Trainor Memorial Fund will match your donation for up to \$700,000 dedicated to the Care Center Network.

Care Centers guide our understanding of how SMA is changing, help us understand how care is being delivered, and help identify best practices in SMA treatment. The data we collect through Care Centers today will help improve standards of care for tomorrow.

Time is running out! We need your support to reach our target by July 2, 2023, when the match opportunity concludes.



Learn more about our Care Center Network
www.curesma.org/sma-care-center-network/

<https://donate.curesma.org/ETMF>