DIRECTIONS

COMMUNITY NEWSLETTER | SUMMER 2024

cure SMA

LETTER FROM KENNETH

As you'll read about in this issue, our 2024 Annual SMA Conference in Austin, Texas, was an incredible success, conveying our community's desire for connection and the need for continued research and treatments to address unmet needs.

In January, we reached 100% newborn screening of SMA across the country, allowing very early access to FDA-approved treatments. Recent data also show about 70-80% of everyone impacted by SMA is receiving access to treatment.

But we still have more work to do. We are making progress on the next steps needed to restore strength and function and build back muscle. With this need in mind, Cure SMA has increased our funding this year to $750,000 for new basic research.

Many of our next opportunities for new combination treatments, and also improvements to our existing drugs, will reach conclusions as we move into 2025 and 2026. And if successful, these programs would move through regulatory FDA approvals and then roll-out for access to patients in the US and then around the world.

As always for our community, we are even now looking further ahead. And we are awarding and funding many new early stage basic research grants that will focus on additional ways to target other parts of the body impacted by SMA, especially the nerves and the nerve-muscle junction. All with the goal of aiming to reverse damage and symptoms, and restore strength and function.

Our support and advocacy programs focus on issues such as education, work, and independence. Two important initiatives that Cure SMA is working on are transportation and caregiving. These important issues go hand in hand with our treatment and care developments to remove barriers for people with SMA in every aspect of life.

Your support helps us at every turn – and I invite you to join us this fall at your local Summit of Strength, Walk-n-Roll or other fundraising event. Together, we create a better future for everyone living with SMA.

In the meantime, I hope you'll enjoy our summer 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

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

Genentech
A Member of the Roche Group

Mailbag

Jazzlyn M.

Donnie G.

The Koizumi Family

Max L.

Sage G.

Mia K.

Aniya W.

Aniya W.

Madison R.

Max L.

Summit Donation

Elain V.

Donations

Cure SMA was excited to receive a donation of the book "Great and Small" to our newly diagnosed care packages in honor of what would be Liv Harlow McDonald's 7th birthday on March 7th. This generous donation was made by Liv's grandparents Debbie and Richard Butler and is special as the story includes a unicorn (Liv's favorite animal!) who celebrates the uniqueness of each individual while competing against others in the Great Unicorn Games.

Cure SMA was thrilled to receive a donation of assorted toys to our newly diagnosed care packages in honor of Ava Francis' diagnosis day which was on February 14th, 2017. This generous donation was made by Ava's mom Jenna LuQue who coordinated a fundraiser to purchase these new toys to send out in Ava's memory!

Thank you all so much for the package. I have no doubt Layton will love it.

~ Ebert Family

Thank you so much for the care package! This is such a special box to receive. There are so many amazing products inside, but what I loved the most was feeling the thoughtfulness of each gift and feeling like I was surrounded by the SMA families that recommended the items.

The support from Cure SMA has made such a big impact on our journey.

~ Maurer Family

Wow! We were in tears opening his care package last night. Thank you for shining a light on such a hard time for us. Not only adjusting as being first time parents but also learning about his diagnosis has been hard, but somehow you guys have shown us it will be ok. We are so grateful for the love and support!

~ Woods Family

We received a care package from Cure SMA and it was amazing! We are so grateful for the information sent as well as all the toys, blankets, and gear that was in the box for my son.

Thanks so much!

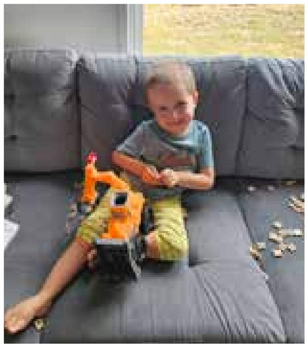
~ Locklin Family

Thank you for the SMA care packages that Madison and Elizabeth received. The girls really enjoyed them, and they will be very helpful.

~ Griffiths Family

Thank you Cure SMA, for these little gifts for my son, I am very grateful. My son is almost 3 months old and he will soon be receiving his treatment. It has not been easy but I feel happy for the help that there is today for these.

~ Valencia Family



See attached how happy you made Lukas with just the digger. He will play with everything in the box so much. These are perfect toys for him.

~ Allmendinger Family

I wanted to say thank you to Cure SMA for loaning us this Panthera! Because of this wheelchair Madi is able to participate in Special Olympics in the manual 100m dash. It's exactly what she wanted to do. 16 years ago we were told she wouldn't even be here today! But today she won two gold medals using this wheelchair. She is so proud of herself. The determination and pride she feels is priceless. Thank you for giving us these moments in more ways than one!

~ Ramirez Family

Thank you so much for the care package from Cure SMA! It was so thoughtful and generous of you, and it's really made a difference for us. We are incredibly grateful for your kindness and support.

Thank you!

~ Paz Family

We got the box; my daughter was so happy. She loves it and said, "OMG, mom, I always wanted kitchen toys. How did they know? I am so happy tell them thank you so much."

~ Magana Family

Thank you so much for the thoughtful care package. It really brightened our day. The resources were very helpful. We are so grateful for Cure SMA and their dedication to helping SMA families.

~ McCaffery Family

Evrysdi®
risdiplam
60 mg
powder for oral solution

A global community of 15,000+ strong,* including people up to 75 years old†

*Based on individuals with SMA receiving Evrysdi worldwide as of February 2024.

†Clinical trials of Evrysdi did not include people aged 65 and older to determine whether they respond differently from those who are younger.

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.** There is a pregnancy registry for women who take EVRYSDI during pregnancy. The purpose of this registry is to collect information about the health of the pregnant woman and her baby. If you are pregnant or become pregnant while receiving EVRYSDI, tell your healthcare provider right away. Talk to your healthcare provider about registering with the EVRYSDI pregnancy Registry. Your healthcare provider can enroll you in this registry or you can enroll by calling 1-833-760-1098 or visiting https://www.evrysdipregnancyregistry.com.
  - are an adult male. Evrysdi may affect a man's ability to have children (fertility). Ask a healthcare provider for advice before taking this medicine
  - are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby
- **Tell your healthcare provider about all the medicines you take**
- You should receive Evrysdi from the pharmacy as a liquid. If the medicine in the bottle is a powder, **do not use it**. Contact your pharmacist for a replacement

Genentech
A Member of the Roche Group

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Evrysdi—the first and only **oral, non-invasive** treatment for spinal muscular atrophy (SMA)

- ✓ **Proven results** in adults, children, and infants with SMA, including infants not yet showing symptoms
- ✓ Designed to help produce SMN protein **throughout the body**
- ✓ Can be taken **at home, at work, or when on the go**‡
- ✓ **No needles, sedation or hospital stays** required

Talk to your doctor about Evrysdi
Scan the QR code to learn more or visit
EvrysdiResults.com

‡If refrigeration is not available, Evrysdi can be kept at room temperature up to 104°F for a combined total of 5 days. Please refer to the Instructions for Use for additional information about storage and administration.
SMN=survival motor neuron.

Important Safety Information (continued)

- 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.

If you cannot afford your Evrysdi medication, visit MySMASupport.com for financial assistance information.

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. The purpose of this registry is to collect information about the health of the pregnant woman and her baby. If you are pregnant or become pregnant while receiving EVRYSDI, tell your healthcare provider right away. Talk to your healthcare provider about registering with the EVRYSDI Pregnancy Registry. Your healthcare provider can enroll you in this registry or you can enroll 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
  - 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, 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

EVRYSDI is a registered trademark of Genentech, Inc.
M-US-00007143(v6.0)

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

This Patient Information has been approved by the U.S. Food and Drug Administration. Approved: 3/2023

Recognize the symptoms and save a life!

SMART

MOVES

EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

Motor delays or missed milestones may signal serious conditions like SMA. There is new hope, but it's important to act swiftly and make SMArt Moves.

cure
SMA

SMArtMoves.CureSMA.org

40 ANNIVERSARY

cure SMA

MISSION • MILESTONES • MOMENTUM

Cure SMA was founded in 1984 to support the SMA community and fund research to find a cure. In our 40-year history, we've seen great breakthroughs that have changed the trajectory of SMA, and have undergone many changes, all leading us to the present.

In 1984, Families of Spinal Muscular Atrophy (FSMA) was founded as a volunteer organization dedicated to funding and promoting research into the causes and cure of spinal muscular atrophy. Our founders, a small group of parents across the U.S., banded together to support and educate each other about the devastating disease affecting their children.

Empowered to understand the genetics and cause of SMA, Families of Spinal Muscular Atrophy created a Scientific Advisory Board and Medical Advisory Council in 1986, compiled of medical and scientific experts. FSMA's commitment to bring researchers and clinicians into the SMA community created a legacy of unparalleled research progress and patient support, from grassroots fundraising to global scientific leadership.

Families of SMA, now Cure SMA, has continued to grow from a courageous group of families to an internationally respected network of research, fundraising, and support, including 37 volunteer Chapters throughout the U.S. and Puerto Rico.

Over the past 40 years – thanks to individuals, families, scientists, and supporters – we've achieved remarkable milestones. We have propelled groundbreaking research leading to the development of three FDA-approved treatments for SMA. These treatments have transformed lives, instilling hope where there was once uncertainty. With each milestone and every dollar raised, we are propelling ourselves closer to a future where everyone with SMA is empowered to live fulfilling and independent lives. Together, we've demonstrated time and again that our determination as a community can drive change.

MILESTONES:

1984

Incorporation of Families of Spinal Muscular Atrophy (FSMA); first issue of Directions is published.

1986

Scientific Advisory Board and Medical Advisory Council formed by FSMA, comprised of prominent neurologists, geneticists, and clinicians.

1989

The first family conference hosted by FSMA in Illinois.

1990

SMA chromosomes are located by Dr. Conrad Gilliam at Columbia University.

1991-1993

FSMA funds three full-time researchers at Columbia University and the Ohio State University.

1995

SMA gene identified by Dr. Judith Melki in France.

1996

- Discovery of the SMA protein, SMN, and development of carrier testing at the Ohio State University. Both projects are funded by FSMA.
- August is officially recognized as SMA Awareness Month.
- First-ever website for Families of Spinal Muscular Atrophy (FSMA) is created.

1997

FSMA initiates and sponsors the First Annual SMA Research Group Meeting.

FSMA provides the first year of funding to a 3-year gene therapy study at Ohio State University, led by Dr. Arthur Burghes.

1999

The office of FSMA opens in Libertyville, IL.

SMA is one of 14 diseases listed in the appropriations bill for funding by the National Institute of Health (NIH).

2000

First-ever SMA drug development program started.

2002

The largest SMA drug screening program is completed, and FSMA begins funding the development of clinical protocols through Project Cure SMA.

2003

More than 150 SMA community members visit 88 congressional offices in support of NIH research funding for SMA, called SMA Day on the Hill.

2005

Compass, a publication dedicated to SMA research, is published by FSMA.

2009

Orphan Drug Status is awarded by the FDA for SMA therapy.

2011

The first clinical trial was initiated for an SMA-specific drug.

The Annual SMA Conference is held at Walt Disney World in Orlando, FL for the first time.

2014

The first clinical trial to test gene therapy for SMA begins.

Families of Spinal Muscular Atrophy is officially renamed as Cure SMA.

2016

FDA announces approval of Spinraza to treat SMA, making it the first-ever FDA-approved therapy for SMA, marketed by Biogen.

2018

HHS Secretary Alex Azar approves adding SMA to the Recommended Uniform Screening Panel (RUSP), a major step toward implementing newborn screening nationwide.

2019

FDA announces the approval of Zolgensma, a gene therapy, for the treatment of children with SMA, marketed by AveXis, a Novartis company.

2020

FDA announces the approval of Evrysdi to treat SMA in pediatric and adult patients, marketed by Genenetch, a member of the Roche Group.

2022

The Cure SMA Care Center Network comprises 18 hospital sites throughout the U.S.

2024

100 percent of United States are officially screening newborns for SMA, ensuring early diagnosis and treatment for babies born with the disease.

LOGOS THROUGH THE YEARS:

Families of SMA
Support. Research. Hope.

FAMILIES OF SMA
Balancing Life's Tough Times™

cure SMA
Make today a breakthrough.

Community *Spotlight:*

Viola Dwyer, SMA Community Advocate from Pennsylvania

Cure SMA advocates play a crucial role in shaping policy and raising awareness. Cure SMA spoke with one of those advocates, Viola Dwyer from Pennsylvania, who recently organized her own meetings with her federal legislators to educate them on the caregiving needs of the SMA community. She shares her experiences, insights, and the impact of her advocacy efforts in this enlightening Q&A:

Would you please introduce yourself?

My name is Viola Dwyer. I live in the Philadelphia area with my husband and dear cat, Pippi. I also work full-time in HR and Recruiting and live with SMA type II.

What motivated you to start engaging in advocacy work with Cure SMA?

My husband and I run a YouTube channel called "The Ginchiest" with the aim of improving the quality of life for those with disabilities. Becoming a Cure SMA advocate was a natural next step in that mission.

What steps did you take to set up a virtual meeting with your congressional office?

I worked very closely with the Cure SMA staff who did a phenomenal job at guiding me through the process, from email templates and letters to appropriate responses when getting the expected re-scheduling requests.

Did you face any challenges when organizing these meetings? If so, how did you overcome them?

Some representatives just did not respond and others kept rescheduling the meetings. I kept on top of those who did respond and eventually had meetings scheduled.

How did the congressional offices respond to your advocacy efforts and the information you presented?

They were surprisingly very curious about SMA and my lived experience. One office even requested an additional meeting to understand more of our unique community's challenges!

How has participating in this form of advocacy affected you personally?

I felt heard and hopeful. There is some very exciting and inclusive legislation that could get passed and I now feel I took part in making it so.

What advice would you give others who want to do the same?

Just do it! Cure SMA makes it very easy.

How did SMA awareness impact your advocacy experience?

Sharing my SMA experience made me feel empowered.

For those who are interested in sharing their SMA story with their legislators, check out Cure SMA's Advocacy webpage or reach out to advocacy@curesma.org for additional information.

Community Support Update

In late 2023, Cure SMA hosted its first Virtual Resource Fair. This new virtual program outlined all resources, guides, and programs available to SMA community members. Programs covered included:

- Informational Packets
- Care and Support Packages for newly diagnosed families, teens, and adults.
- The Cure SMA Equipment Pool
- Resource Guides
- Annual SMA Conference
- One-day Summit of Strength program
- ...and much, much more!

We want to again thank our partners at Genentech for their generous sponsorship of the Cure SMA Virtual Resource Fair.

CURE SMA VIRTUAL RESOURCE FAIR

Newly Diagnosed Care Packages

- Cure SMA automatically sends a care package to every newly diagnosed family filled with toys and helpful items that have been recommended by other SMA parents.

Parents can email infopack@curesma.org to request a package.

Cure SMA's Virtual Resource Fair

COULDN'T ATTEND?
No problem! The Virtual Resource Fair is available for viewing on the Cure SMA YouTube channel.

NEW!

NEW RESOURCE REQUEST FORM

Highlighted during the Virtual Resource Fair was our eighteen Resource Guides, which provide guidance and tips for daily life with SMA. Topics include adaptive driving, accessible vehicles, home accommodations, special education supports, and many more! This list of guides continues to grow and is available for you to request at the QR code or at the following link: www.curesma.org/resourcerequest!

Have you visited Cure SMA's merchandise store?

The online store features our most popular Cure SMA t-shirts, hats, totes, zip-up jackets, drinkware and more! We also have plenty of different accessories and promotional items to help spread awareness wherever you go.

The store itself has a refreshed look with improved functionality, making shopping and finding what you want a breeze.

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

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

Save while you shop with free shipping on all orders above $35.

Visit www.curesma.org/merchandise to check out the store!

Check it out now and get your Cure SMA gear for 2025!

SHOWN ABOVE

Tie-Dye Cure SMA T-shirt- $30

Tie-Dye Cure SMA Onesie- $20

40th Anniversary Key Chain- $6

Reusable Grocery Bag- $10

Heathered Purple Performance Polo- $35

2024 ANNUAL SMA CONFERENCE SUMMARY

Thank you to everyone who attended the 2024 Annual SMA Conference at the JW Marriott in Austin, Texas! It was an impactful weekend of opportunities to connect, learn, and have fun.

Our conference brought over 1,975 attendees from around the world. It was an honor to have individuals with SMA, their families and care teams, clinicians, industry partners, and researchers attend the conference from over 28 different countries.

The 4-day weekend was filled with special events and workshops. The Newly Diagnosed Program kicked off the conference on Thursday for those recently diagnosed with SMA, followed by our Opening General Session on Friday morning where we welcomed all attendees. Friday and Saturday were packed full of over 68 educational and supportive workshops, as well as the Family Friendly Researcher Poster Session. Finally, the It's A Wonderful Life panel of adults with SMA ended the weekend on a high note. The Meet & Greet, PJ Party & Movie Night were also conference highlights!

Lastly, at the Closing General Session on Sunday morning, we announced the location and dates for our 2025 Annual SMA Conference. Mark your calendars for Thursday, June 26 – Sunday, June 29, 2025, as we head to Anaheim, California at the Disneyland Hotel!

We extend our thanks to the sponsors and exhibitors who generously supported the 2024 Annual SMA Conference. A special thanks to Biogen and Genentech for their generosity as the Presenting Sponsors of this year's conference.

Children's Program

This year's Children's Program was an outstanding success! Thanks to our incredible volunteers, the children had an unforgettable experience. They were engaged with a diverse array of activities, including arts and crafts, toys, live entertainment, exciting movies, video games, and more. A standout moment was the caricature artist, who drew personalized portraits for the kids, providing them with a special keepsake from the conference!

The Annual SMA Conference owes its success to the dedication of our fantastic volunteers. Whether it was setting up decorations, managing the Children's Program, organizing games at the Meet & Greet, or handling numerous other tasks, their efforts were nothing short of extraordinary. We are also deeply grateful to the Jacob Isaac Rappoport Foundation for their generous support, which played a crucial role in making the Children's Program a highlight of the conference. Their sponsorship is vital in ensuring the continued success of our Annual SMA Conference.

Annual Relay Race

The Annual Relay Race, a beloved feature of the Meet & Greet, never fails to be a crowd favorite. In this exhilarating event, kids with SMA compete head-to-head against researchers and clinicians to see who can reach the finish line first. The twist? Researchers and clinicians must race using a manual wheelchair.

The excitement is profound as conference attendees gather around the course, cheering enthusiastically for all of the racers. Despite their best efforts, the researchers and clinicians often find themselves trailing behind, adding to the fun and energy of the race!

Meet & Greet

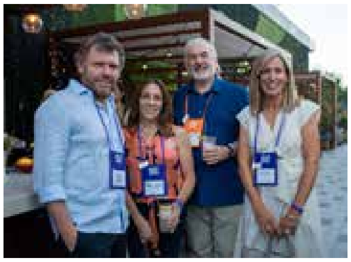
Each year, the Annual SMA Conference launches with our signature event, a vibrant gathering that unites the entire community. The Meet & Greet is a fantastic evening packed with fun, games, and opportunities for connection and much more! It is a remarkable start to the conference!

PJ Party & Movie Night

On Saturday evening, we transformed a special space into a cozy retreat where conference attendees could unwind in their pajamas and enjoy Sing on the big screen. With delicious popcorn and cupcakes in hand, it was the perfect way to relax and wind down after two full days of workshops and events.

Workshops

Workshops kicked off on Friday, June 7, with 68 offered throughout the weekend – the most we have ever had – with 24 brand new topics. These sessions were led by some of the leading SMA experts as well as other individuals and family members affected by SMA. They provided invaluable insights into the latest advancements in critical care and support. We extend our deepest gratitude to all our speakers for making this weekend truly remarkable, brimming with new knowledge, updates, and unwavering support!



Networking Opportunities

The Annual SMA Conference is all about forging new friendships and reconnecting with old ones. This year's weekend was filled with diverse opportunities for everyone in the community to meet, mingle, and connect. Special events included a Dad's Night Out on Thursday and a Mom's Night Out on Friday, providing parents with dedicated time to socialize.

This year the Teen Lounge, sponsored by the Luke 18:1 Foundation, offered a space for teens to craft, game, and connect throughout Friday and Saturday. On Friday evening, the Teen Social featured a lively trivia game with snacks, creating a fun and interactive experience for all. On Saturday evening, the Teen Social dance party was vibrant and full of energy, featuring interactive fun and lively music!

Adults with SMA had their own dedicated space in the Adults with SMA Lounge, where they could engage in conversations and connect throughout Thursday, Friday, and Saturday. Sponsored by Biogen and the Dhont Family Foundation, this area provided a comfortable setting for meaningful interactions. The popular Adults with SMA Reception on Friday evening offered a relaxed atmosphere with beverages and snacks, thanks once again to Biogen's generous support.

Grandparents enjoyed a special session for coffee and conversation during the afternoon, giving them a chance to share stories and connect with others in the SMA community during the Grandparents Coffee & Mingle session.

With all these varied social and networking opportunities, we hope the connections made were meaningful and impactful for everyone involved!

Congratulations to the 2024 Annual SMA Conference Self-Expression Exhibit fan-favorite, Connie Chandler!

"East Coast Sunset" is a 20"x16" Diamond Art piece, containing an incredible 25,920 rhinestone beads which were individually hand-placed on a sticky, coded canvas. It took about eight weeks for Connie to complete. This silhouetted sunrise scene speaks to her heart, as she grew up in North Carolina and Florida, and east coast beaches are her happy place - the sand, the salt water, the seafood, and of course the palm trees. It is her favorite travel destination with friends or family, for fun, inspiration, or rest.

Thank you to Biogen for their generous sponsorship of the 2024 Self-Expression Exhibit.

Research & Clinical Care Meeting

At the beginning of June, 585 SMA researchers and clinicians from around the world met in Austin, Texas for the 2024 Annual SMA Research & Clinician Care Meeting. The annual meeting provides an opportunity for SMA scientists and health care professionals to share their most recent findings and learnings and foster collaboration, including cross-disciplinary knowledge sharing. The goals of this meeting are to accelerate the pace of research in to SMA treatments and achieve excellence in SMA clinical care. To learn more about the meeting, please visit www.curesma.org.

Family Friendly Researcher Poster Session

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

POSTERS INCLUDED:

SMA ASTROCYTES IMPACT MOTOR NEURON FUNCTION
Allison Ebert, PhD, Medical College of Wisconsin

SCHOLAR ROCK'S SMA PROGRAM
Ameet Khara, PharmD; Christabella Cherubino, DC, MS, CME, Scholar Rock

GONE FISHING: USING SMN MUTANTS TO CATCH TARGETS FOR ADDITIVE THERAPEUTICS FOR SMA
Anton J Blatnik III, PhD, The Ohio State University College of Medicine

THECAFLEX DRX™ SYSTEM: IMPLANTABLE INTRATHECAL CATHETER AND SUBCUTANEOUS PORT PLATFORM FOR REPEAT INTRATHECAL DELIVERY OF NUSINERSEN
Cassandra N Dennys, PhD, Melissa Breedlove, Catalina Troche, John McGuire, Alcyone Therapeutics

RESILIENT- EFFICACY AND SAFETY OF TALDEFGROBEP ALFA IN SPINAL MUSCULAR ATROPHY
Cliff Bechtold, Biohaven

THE DIFFERENCE OF DONATIONS: HOW FUNDRAISING CAN CHANGE THE FUTURE OF SMA
Cure SMA Development Team, Cure SMA

CHARACTERIZING THE REVERSIBILITY OF CELLULAR AND MOLECULAR DEFECTS OF SMN-DEFICIENT MUSCLE
Elana Molotsky, PhD, Michelle Harran Chan-Cortes, PhD, Kamran Eslami, BS, Bhavya Ravi, PhD, Stephen Brown, BS, Ramzi Khairallah, PhD, Charlotte Sumner, MD, Johns Hopkins University

HOW DOES DUAL THERAPY AFFECT OUTCOMES OF SMA IN SYMPTOMATIC CHILDREN
Elicia Estrella, MS, LCGC, Dept. of Neurology Boston Children's Hospital/Harvard Medical School

INSIGHTS INTO THE HEALTH AND WELL-BEING OF ADULTS LIVING WITH SPINAL MUSCULAR ATROPHY
Erin F Welsh, MPH, Cure SMA

LOCAL RNA TRANSLATION TO RESCUE SMA MOTONEURONS
Florence Rage, PhD, IGMM, CNRS, Montpellier

IMPROVEMENTS IN MOUTH OPENING AMONG PATIENTS WITH SMA FOLLOWING ORASTRETCH THERAPY
Katlyn Elizabeth McGrattan, PhD, University of Minnesota

LEARN ABOUT SPINRAZA® AND THE LATEST REAL-WORLD EVIDENCE IN ADULTS
Kevin Conway, Marketing Director, Biogen

CHARTING NEW HORIZONS: A PATHWAY TO EARLY ASSESSMENT FOR NEWBORN SCREENING IN SMA
Kristin J Krosschell, PT, DPT, PCS, Northwestern University Feinberg School of Medicine, Allison Cullen, BS, The Children's Hospital of Philadelphia

GETTING STARTED ON YOUR CLINICAL TRIAL JOURNEY WITH CURE SMA - NEW RESOURCES AND TOOLS TO IMPROVE COMMUNITY AWARENESS
Lauren Eisenman, MS, Cure SMA

THE IMPACT OF PATIENT-PROVIDER COMMUNICATION ON STRESS IN A HOSPITAL SETTING
Linsey Wehner MPH, Jeremy Orr MD, Amy Bellinghausen MD, University of California, San Diego Health, Department of Medicine, Division of Pulmonary, Critical Care, Sleep Medicine & Physiology

ESTIMATING THE PREVALENCE OF SMA. CURE SMA'S MODEL FOR PREVALENCE AND DEMOGRAPHICS OF THE U.S. SMA POPULATION
Lisa Belter, MPH, Cure SMA

INVESTIGATING MOTOR NEURON RECOVERY USING MOUSE MODELS OF SMA
Lyndsay Murray PhD, University of Edinburgh

LEVERAGING DATA TO SUPPORT TREATMENT ACCESS
Mary Schroth, MD, Mary Curry, ND, Jennifer, Deans, MHA, MS, Erin Welsh, MPH, Sarah Whitmire, MS, Lisa Belter, MPH

CURE SMA: ADVANCING ACCESSIBLE AIR TRAVEL THROUGH LEGISLATIVE ADVOCACY
Maynard Friesz, Cure SMA, Sarah Bellish, Cure SMA

SMA COMMUNITY-BASED EXPERIENCE WITH THE 12-TIER FUNCTIONAL ABILITY SCALE FOR EVOLVING SPINAL MUSCULAR ATROPHY
Meghan Moore Burk, PT, DPT, NCS, Children's Hospital Colorado

HOW DOES LOSS OF MUSCLE IN SMA AFFECT WHOLE BODY METABOLISM?
Melissa Bowerman, Ph.D., Keele University

AN UPDATE ON FERTILITY IN MEN WITH SMA: THE IMPACT OF SMA AND SMN2 SPLICING MODIFIERS
Natan Bar-Chama MD, Department of Urology The Mount SInai Hospital / Reproductive Medicine Associates of New York

THE SMA EFFORT: IMPROVING THE ASSESSMENT OF EXPERIENCED FATIGABILITY
Rafael Rodriguez-Torres, PT, DPT, Columbia University Irving Medical Center

A DESCRIPTIVE EVALUATION OF DISEASE MANAGEMENT TRENDS AND TRANSITION OF CARE AMONG TREATED AND NEVER-TREATED PATIENTS WITH SPINAL MUSCULAR ATROPHY (SMA) USING US CLAIMS DATA
Randal Richardson, MD, MMS, Gillette Children's

GENE REPLACEMENT THERAPY: OVERVIEW AND LATEST UPDATES
Sandra P. Reyna, MD; Nayla Mumneh, MD, Novartis Gene Therapies, Inc.

REAL WORLD TREATMENT PATTERNS OF INDIVIDUALS INITIATING SMA TREATMENT AS TEENS OR ADULTS IN THE SMA CLINICAL DATA REGISTRY
Sarah Whitmire, MS, Cure SMA

NIFEDIPINE AND ELECTRICAL SIGNALS IN SMA
Saravanan Arumugam, PhD, Department of Medical Physiology and Biophysics, School of Medicine, University of Seville, Spain

THE JOURNEY BEYOND CLINICAL STUDIES: REAL-WORLD EXPERIENCE WITH RISDIPLAM TREATMENT
Sheila Shapouri, PharmD, MS, Senior Health Economist, Genentech

ROCHE/GENENTECH CONTINUED EFFORTS TO ADVANCE CARE IN SMA
Travis Dickendesher, PhD, Principal Medical Science Director, Genentech

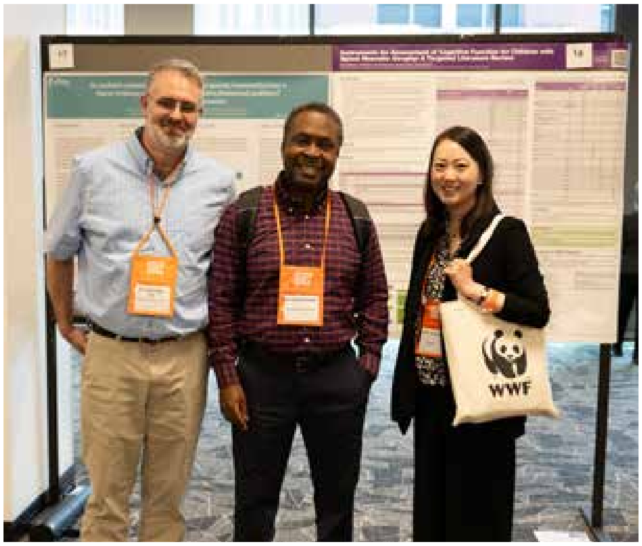
IDENTIFYING THE TOP 10 UNANSWERED RESEARCH PRIORITIES FOR SPINAL MUSCULAR ATROPHY
Vanessa Christie-Brown, SMA Europe

THE NMD670 SYNAPSE-SMA PHASE 2 STUDY, A MUSCLE-TARGETED ORAL DRUG THERAPY FOR AMBULATORY ADULTS WITH SMA
Vera Kiyasova, MD, NMD Pharma


cure SMA

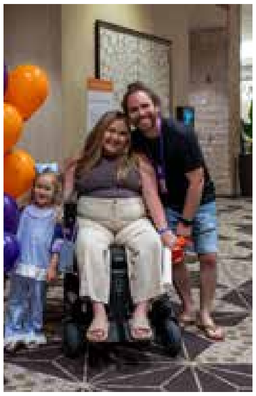

Traveling with SMA


SMA




CURE SMA


WE CAN
Cure

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Cure SMA Programs for Adults with SMA

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

SUPPORT PACKAGES AND PROGRAMS

Teen and Adult Support Package

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

www.curesma.org/SupportPackage

Teen and Adult Independence Assistance Package

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

www.curesma.org/IndependencePackage

LifeVac Support Program

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

www.curesma.org/LifeVac

Medical Alert Bracelet

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

www.curesma.org/ResponderPHR

SUPPORT PACKAGES AND PROGRAMS

Annual SMA Conference Sponsorship

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

Email conference@curesma.org for sponsorship application!

Annual SMA Conference Events

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

www.annualsmaconference.com/agenda.html

Adults with SMA In-Person Socials

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

Email communitysupport@curesma.org for more details!

Adults with SMA Virtual Socials

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

Email communitysupport@curesma.org for more details!

You Tube

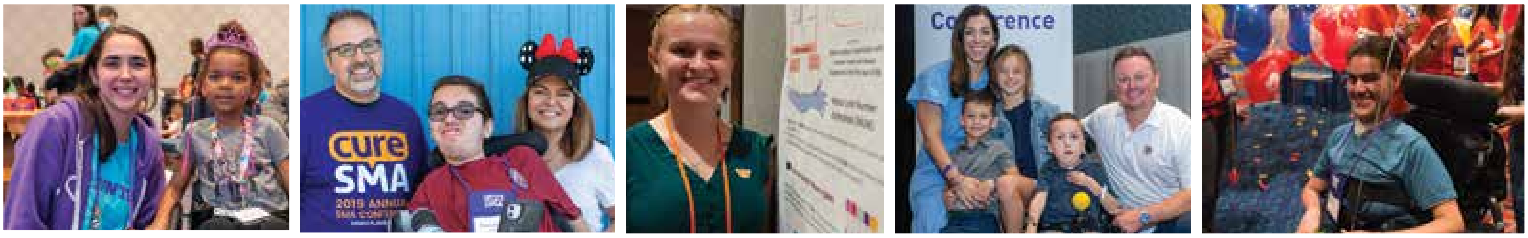
Educational Webinars and Panels

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

www.youtube.com/c/CureSMA/playlists

cure SMA

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



cure SMA 2025 ANNUAL SMA CONFERENCE

Thursday, June 26 – Sunday, June 29 | Disneyland Hotel in Anaheim, California

Conference Scholarships

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

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

Since 1988, Cure SMA has hosted the Annual SMA Conference, a pivotal gathering that unites leading SMA researchers and clinicians as well as individuals and families living with SMA. This weekend event features a diverse array of workshops, a Family-Friendly SMA Researcher Poster Session, engaging Children's Program, Meet & Greet with a family fun fest, numerous social activities for teens and adults, PJ Party & Movie Night, and the Evening at the Park. It provides many opportunities for connection and firsthand updates from experts.

We eagerly anticipate coming together as a community to show our support for one another. The Annual SMA Conference coincides with the SMA Research & Clinical Care Meeting, making it the world's largest conference dedicated to SMA, unparalleled in its scope and impact. With expectations of over 2,500 attendees, this event remains unmatched in its commitment to those affected by SMA and their caregivers.

Disneyland Hotel and Disney's Pixar Place Hotel in Anaheim, California have been thoughtfully selected to meet the needs of the SMA Community for the 2025 Annual SMA Conference. This venue offers endless opportunities for networking, forging connections with both old friends and new, and engaging with researchers, clinicians, and other professionals. Additionally, it boasts a lively nightlife complete with sensational sips and eats, shopping, live music, and theme parks.

You must complete your conference registration with Cure SMA prior to reserving your hotel room. Attendees will be receiving the special room rate of $299 per night, plus tax at Disneyland Hotel and $282 per night, plus tax at Pixar Pier Hotel.

To register for the Annual SMA Conference, please visit www.cureSMA.org.

Contact us: 800.886.1762 | conference@cureSMA.org | www.cureSMA.org

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

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

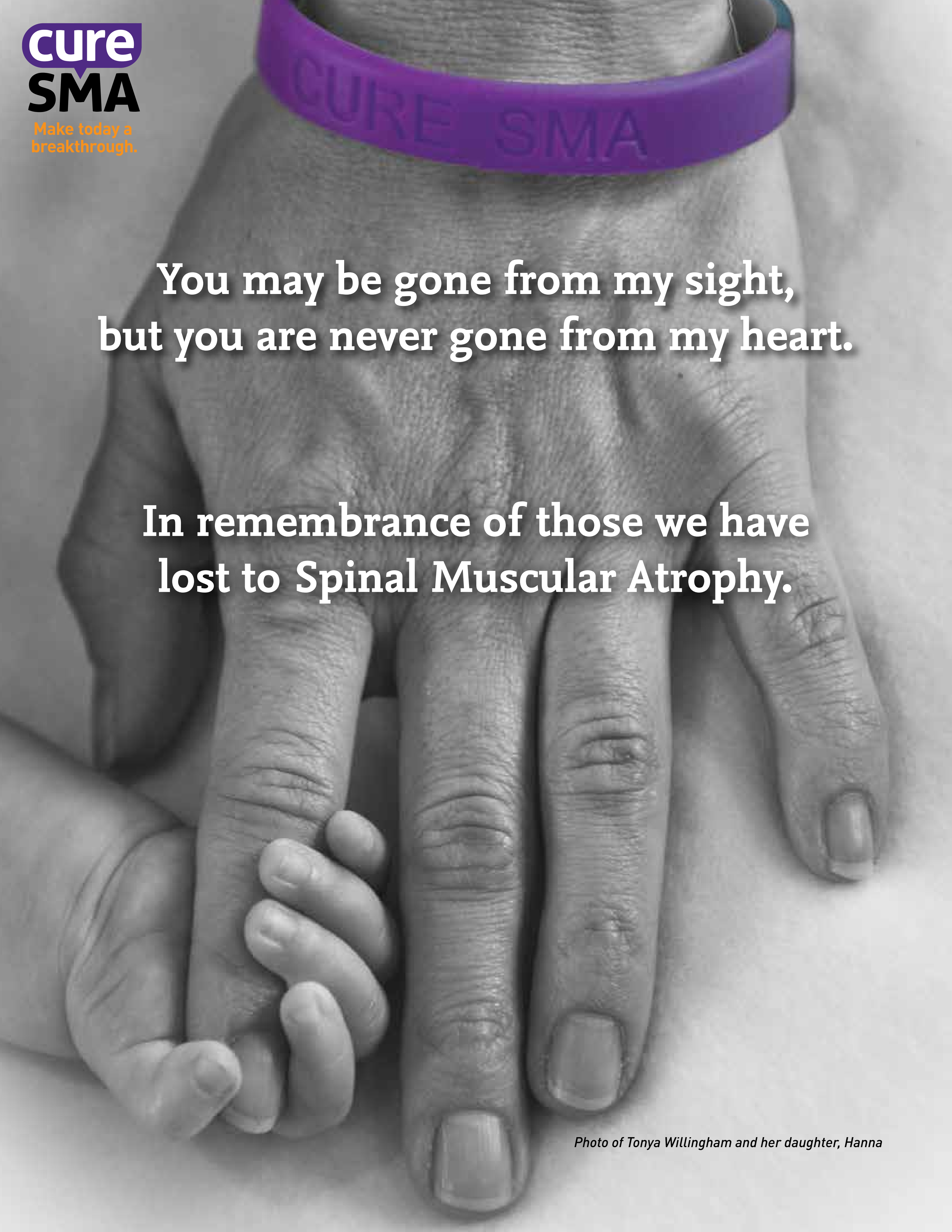
NEW!

- College Scholarship Guide
- Adaptive Recreation Guide
- Surgery Hospital Stay & Recovery Guide

Make today a breakthrough.

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

cure SMA
Make today a breakthrough.



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

In remembrance of those we have
lost to Spinal Muscular Atrophy.

Photo of Tonya Willingham and her daughter, Hanna

2024 National Partners

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

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Research Update

Cure SMA Announces Additional $750,000 in Basic Research Funding

At the Annual SMA Research and Clinical Care Meeting, Cure SMA highlighted six new basic research grants totalling $750,000. Cure SMA also announced another $750,000 will be awarded for additional research. The recently awarded projects will investigate new targets for drug discovery. With more than $85 million in funding to date in support of basic research grants and drug discovery programs, this round of funding reinforces Cure SMA's commitment to basic research as the foundation for drug discovery.

Each year, Cure SMA invites scientists from around the world to submit funding proposals for basic research projects that address specific unanswered questions in SMA biology. Submitted proposals are ranked on both their scientific merit and their relevance to Cure SMA's research priorities.

Cure SMA's top basic research priorities currently include:

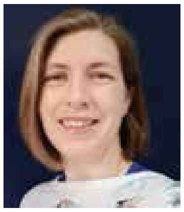
- Learning more about when and where the survival motor neuron (SMN) protein is needed and how it functions in the body.
- Finding new ways to treat SMA, especially those that can be used in combination with approved drugs.
- Using cellular or animal models to better understand the SMA disease process.
- Developing new tools for SMA research, such as new SMA animal models and new ways of tracking disease progression.

"Cure SMA has a rich history of funding innovative research. Early Cure SMA investment has been critical in the development of approved therapies, and we know continued investment is needed to ensure a robust drug pipeline to meet the needs of all individuals living with SMA," said Jackie Glascock, PhD, Vice President Research, Cure SMA.

Current Cure SMA Basic Research Grant Awards

Melissa Bowerman, PhD, at Keele University was awarded $150,000

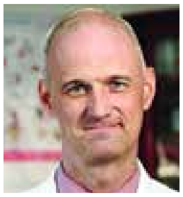
for her research project, "Defining the role of skeletal muscle in metabolic defects in SMA." Learning more about the role of muscle in SMA will help increase our understanding of how low levels of the SMN protein cause the symptoms of SMA. It will also inform the development of future SMA treatments.



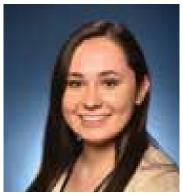
Lyndsay Murray, PhD, at the University of Edinburgh in Scotland was awarded $150,000

for her research project, "Investigating the viability of vulnerable motor axons following SMN restoration in mouse models of SMA." This project will enable the study of how immature motor neurons function with and without SMN protein. Insights from this study could improve the effectiveness of current treatments for SMA.

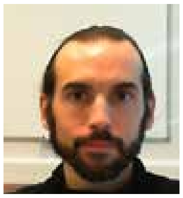
Special thanks to the Concepcion Family, Nunemaker Family, Weisman Family, Luke 18:1 Foundation and Dhont Foundation for their generosity to Cure SMA in our quest to invest in basic research that will ultimately drive the next generation of SMA treatments.



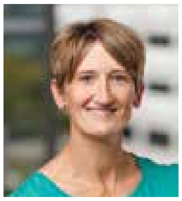
Stephen Kolb, PhD, at The Ohio State University was awarded $85,000 for his research project, "Spliceosome and splicing alterations leading up to motor neuron degeneration in the SMA piglet." In his prior research, Dr. Kolb and his lab created an SMA piglet that experiences SMN protein deficiency mimicking SMA in humans. Dr. Kolb's current project will utilize this SMA piglet model, along with microscopy and biochemistry methods, to learn more about how loss of the SMN protein affects RNA splicing in motor neurons.



Elana Molotsky, PhD, at the Johns Hopkins University School of Medicine was awarded $100,000 for her research project, "Identifying developmental and degenerative mechanisms of SMA using single motor neuron nucleus RNA sequencing." In this project, RNA from the individual spinal cord motor neurons of SMA mice at different stages of disease progression will be examined. By determining how RNA messages change as these motor neurons become unhealthy, this project will aid in understanding which cellular processes are disrupted in the motor neurons of people with SMA.



Michael Tellier, PhD, at the University of Leicester in the United Kingdom was awarded $114,000 for his research project, "Characterization of the SMN-7SK complex in the regulation of *SMN1* and *SMN2* expression." This project examines the role of the 7SK-SMN protein complex in *SMN1* and *SMN2* gene activation. It also investigates whether mutations in the *SMN1* gene that cause SMA disrupt the formation or function of this complex.



Allison Ebert, PhD, at The Medical College of Wisconsin was awarded $150,000 for her research project, "The role of teneurin 4 and actin dynamics in SMA astrocyte perisynaptic processes." This project utilizes a human stem cell model to determine how the loss of teneurin (TENM4), affects the ability of astrocytes to support motor neurons. It will investigate how astrocytes develop and function in the absence of the TENM4 protein and determine whether supportive function can be restored to astrocytes by adding TENM4.

About Cure SMA's Research Grants

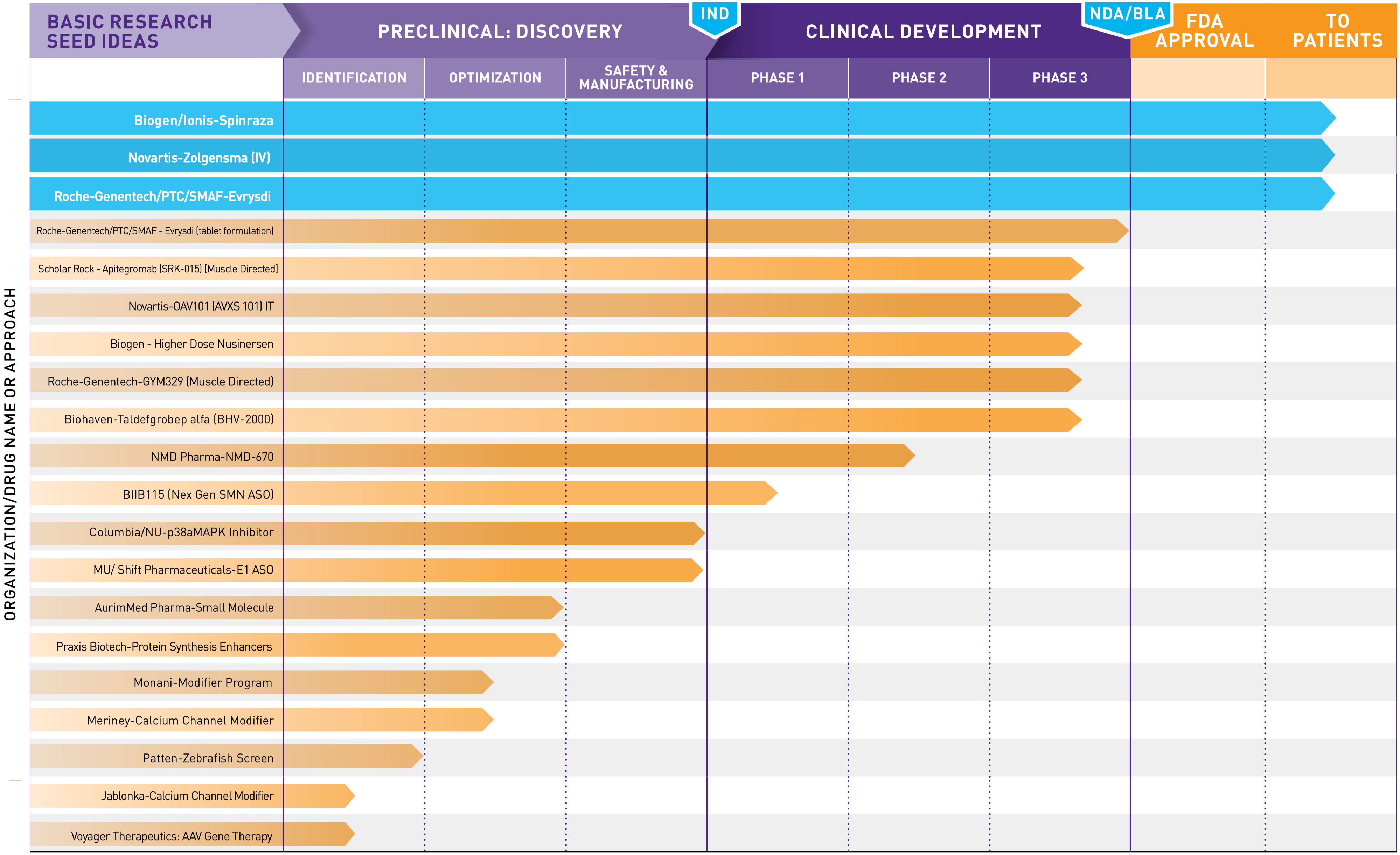
Each year, Cure SMA invites scientists from around the world to submit funding proposals for **basic research** projects that address specific unanswered questions in SMA biology. Our Scientific Advisory Board then ranks the submitted proposals on both their scientific merit and their relevance to Cure SMA's research priorities. Funding is awarded to the highest ranked projects.

Cure SMA's top basic research priorities currently include:

- Learning more about when and where the **survival motor neuron (SMN) protein** is needed and how it functions in the body.
- Finding new ways to treat SMA, especially those that can be used in combination with approved drugs.
- Using **cellular** or **animal models** to better understand the SMA disease process.
- Developing new tools for SMA research, such as new SMA **animal models** and new ways of tracking disease progression.

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.



BLA = Biologics License Application

IND = Investigational New Drug

NDA = New Drug Application

Last updated: August 2024

2024 ILLNESS PREVENTION STRATEGIES: Protecting Yourself and Others

We hope you are having a wonderful 2024. As Fall approaches and we prepare for the winter months the Centers for Disease Control and Prevention (CDC) has updated recommendations for protecting you and your family through the 2024-2025 cold and flu season.

Illness Prevention Strategies:

1. Avoid others who are ill
2. Sanitize and/or wash hands often
3. Cover coughs and sneezes when possible
4. When in large groups wear a mask
5. MOST IMPORTANTLY get an updated flu vaccine and an updated COVID-19 vaccine ideally in September or October. Updated vaccines for 2024 will be available in Fall 2024 and are recommended for everyone 6 months of age and older.

Please note that vaccines for influenza and COVID-19 will reduce the severity of these illnesses. Generally, antibodies start to increase 2 weeks after a vaccine was given.

If You Get Sick

If you have SMA and develop cold or flu symptoms, please contact your healthcare provider to discuss (1) getting tested to determine whether the illness is treatable and (2) starting an antiviral medication specifically for influenza or COVID-19 to decrease the severity of symptoms. These medications are most effective when started within a few days of developing symptoms. Also do your best to prevent the spread of the infection by using a mask and limiting your contact with others when possible.

Common Viral Respiratory Infection Symptoms:

- Fever
- Cough
- Sore throat
- Runny or stuffy nose
- Headache
- Muscle and body aches
- Fatigue

Respiratory Syncytial Virus (RSV)

RSV is another common viral respiratory infection that usually causes mild, cold-like symptoms during the fall and winter. However, infants, young children, older adults and people with SMA are at risk for serious complications including breathing difficulty and pneumonia that can result in hospitalization. Please note that RSV does not have an antiviral treatment.

Current RSV vaccine recommendations:

1. Adults 60 years old and over with SMA should consider receiving the RSV vaccine one time.
2. All adults over 75 years of age should receive the RSV vaccine one time.
3. Infants and young children:
    a. To prevent severe RSV infection in infants, the CDC recommends one of the following (not both):
        i. Vaccination of pregnant people during weeks 32 through 36 of pregnancy, given one time in September through January with Abrysvo® (the only RSV vaccine recommended during pregnancy). This will result in maternal RSV antibodies that will transfer to the infant prior to birth; OR
        ii. Vaccination of all infants up to 8 months of age with one dose of the monoclonal antibody product, niservimab-alip (Beyfortus®) as they enter their first RSV season. This will give RSV antibodies directly to the infant for protection against RSV infection.
    b. Children 8-19 months of age who are at increased risk for severe RSV and entering their second RSV season may be eligible for one dose of nirsevimab-alip.

In summary, please obtain your 2024 flu shot and 2024 COVID-19 shot to help you stay healthy through the winter and recommend these vaccines to family and caregivers. Please also review your SMA illness intervention plan with your healthcare team. If you qualify for the RSV vaccine, are pregnant or have a young child with SMA please consider and discuss options for RSV prevention with your healthcare provider. Please consult with your healthcare provider regarding all vaccinations and treatments options.

For more information:

Influenza: www.cdc.gov/flu/
COVID: www.cdc.gov/covid/
RSV: www.cdc.gov/vaccines/vpd/rsv/

Clinical Research

SMA Update in Best Practices

As part of our ongoing efforts to drive quality health care standardization and improvements for the SMA community, Cure SMA is pleased to share updated recommendations for clinician best practices on SMA diagnosis considerations published in *Neurology Clinical Practice* in August 2024. A summary report of the published recommendations developed by Cure SMA is also available. www.curesma.org/DiagnosisGuidelines In addition, based on the largest collection of infants identified by newborn screening, an update to the birth prevalence of SMA in the United States was published in *JAMA Pediatrics*.

"SMA Update in Best Practices: Recommendations for Diagnosis Considerations" is the first publication of a series of planned resources targeted to healthcare providers and aimed to empower individuals with SMA and their families and caregivers to actively participate in and advocate for their care.

Over the past 6 years the SMA community has seen the approval of three disease modifying treatments and full implementation of SMA newborn screening across the U.S. These changes led to updating recommendations for the diagnosis of SMA and include SMA newborn screening and guidance on considering the diagnosis of Adult-onset SMA based on symptoms and findings. "SMA Update in Best Practices: Recommendations for Diagnosis Considerations" was created utilizing a workgroup of healthcare professionals from the U.S. and Western Europe and a patient and caregiver workgroup to guide the topic area discussions.

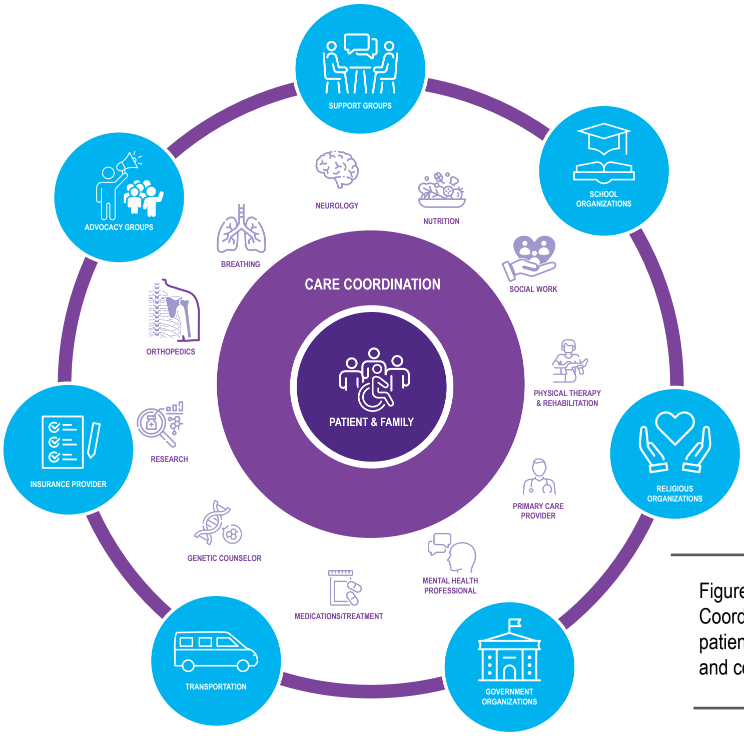


Figure 1: SMA Care Coordination: Connecting patients, families, providers and community

A core recommendation is that individuals living with SMA and their caregivers are essential partners and must be involved throughout the diagnosis, care, and treatment decision-making processes. Healthcare providers can support patients and caregivers by providing information and resources about SMA, providing supportive care such as physical therapy, breathing and nutrition care through a multidisciplinary team of SMA specialists, providing care coordination, and referring to community resources. See Figure 1.

Additional recommendations emphasize the importance of communication and coordination between the newborn screening public health laboratory, primary care providers and the SMA specialty care center team to confirm the SMA diagnosis and to start treatment quickly. Prior to starting treatment, newly diagnosed individuals with SMA should be described by *SMN2* copy number, current motor function, age at symptom onset, and symptom severity.

In addition to the above recommendations, SMA specialty care centers should evaluate SMA newborn screen positive infants within 2-3 days of notification. They should also provide access to treatment options, confirmatory testing, education, and coordination with primary care providers and other specialists.

Adult-onset SMA was reviewed and notably has fewer specific symptoms compared to childhood SMA and symptoms often start in late adolescence or adulthood. Typical symptoms may include weakness of the upper arms and thigh muscles, walking independently but with frequent falling, muscle pain, cramping or twitching, muscle stiffness, and difficulty taking part in sports due to becoming more tired and not able to keep up with others. These symptoms in an adult should be further evaluated with consideration of Adult-onset SMA.

SMA newborn screening, fully implemented throughout the U.S. in 2024, has improved understanding of how frequently an infant with SMA is born in the U.S. Previous estimates of SMA birth prevalence, or the incidence of SMA at birth, were approximately 1 in 11,000 live births. However, in July 2024, Cure SMA published "Newborn Screening and Birth Prevalence for Spinal Muscular Atrophy in the U.S." in *JAMA Pediatrics* that presented an updated estimate of the birth prevalence of SMA.

and Care Update

Data shared from U.S. state public health labs with Cure SMA indicated that between 2018 and 2022, 425 infants were screened positive and had a confirmed diagnosis of SMA out of 6,244,825 infants screened across 30 U.S. states. This data provided an update on the estimated birth prevalence of SMA in the U.S. to 1 in 14,694 live births. Furthermore, information about *SMN2* copy number was shared on 240 infants with SMA identified by newborn screening and revealed that 5% of infants had one copy of *SMN2*, 49% had two copies of *SMN2*, 33% had three copies of *SMN2*, and 13% had four or more copies of *SMN2*. Understanding the current birth prevalence of SMA in the U.S. will help support the planning of SMA care and research, as well as anticipate the utilization of SMA care resources and treatments. Please note that SMA newborn screening identifies 95-98% of all infants born with SMA. 3-5% of infants with SMA will present with symptoms and require special testing called *SMN1* gene sequencing to confirm the diagnosis.

Cure SMA is promoting these publications to healthcare professionals including our 300 treatment and care sites and state newborn screening public health laboratories through email and social media outreach, webinars, CME content development, and medical conference presentations. Additional SMA Update in Best Practices resources will be created for both healthcare professionals and the community focused on treatment considerations, scoliosis, nutrition, pulmonary care and other supportive health care topics.

Thank you!

Thank you to the community members and clinicians who supported the development of this resource.

Funding for these initiatives were provided by the Cure SMA Real World Evidence Collaboration; members include Cure SMA, Novartis, Biogen, Genentech/Roche.

PUBLICATIONS:

1. Schroth M, Deans J, Arya K, Castro D, De Vivo DC, Gibbons MA, Ionita C, Kuntz NL, Lakhotia A, Neil Knierbein E, Scoto M, Sejersen T, Servais L, Tian C, Waldrop MA, Vázquez-Costa JF. Spinal Muscular Atrophy Update in Best Practices: Recommendations for Diagnosis Considerations. Neurol Clin Pract. 2024 Aug;14(4):e200310. Epub 2024 May 24.
2. Belter L, Taylor JL, Jorgensen E, Glascock J, Screening and Birth Prevalence for Spinal Muscular Atrophy in the US. JAMA Pediatr. 2024 Jul 15:e241911. Epub ahead of print.

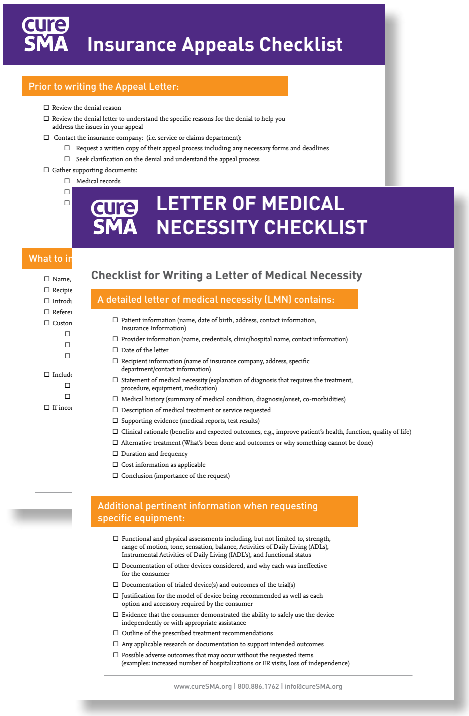
GET STARTED ON YOUR CLINICAL TRIAL JOURNEY WITH CURE SMA

WHAT'S NEW?

- SMA Registry and Clinical Trial Finder: Explore SMA clinical trials with our easy-to-use search widget. Filter by SMA type, current motor function, location, trial phase, treatment status, and more to find trials that fit your or your child's needs and preferences.
- Screening Visit Checklist: Prepare for the screening visit with a thorough checklist that organizes your medical history and suggests questions to ask the research team.
- Decoding Informed Consent: Understanding informed consent is crucial! Our handout will guide you through the key sections of the informed consent form, ensuring you're confident in your decision to participate in a clinical trial.
- Travel Planners (Air and Ground): Manage travel details with ease! Our itineraries will help you plan your trip, and are tailored to the unique needs of the SMA community.

Cure SMA has NEW resources for navigating the evolving landscape of SMA clinical trials. These tools are designed to empower you at every step of your decision-making process.

Funding for this initiative was provided by the 2023 SMA Industry Collaboration; members included Cure SMA, Biogen, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, Genentech/Roche, and SMA Europe.



cure SMA Insurance Appeals Checklist

Prior to writing the Appeal Letter:

- ☐ Review the denial reason
- ☐ Review the denial letter to understand the specific reasons for the denial to help you address the issues in your appeal
- ☐ Contact the insurance company: (i.e. service or claims department):
  - ☐ Request a written copy of their appeal process including any necessary forms and deadlines
  - ☐ Seek clarification on the denial and understand the appeal process
- ☐ Gather supporting documents:
  - ☐ Medical records
  - ☐
  - ☐

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cure SMA LETTER OF MEDICAL NECESSITY CHECKLIST

Checklist for Writing a Letter of Medical Necessity

A detailed letter of medical necessity (LMN) contains:

- ☐ Patient information (name, date of birth, address, contact information, Insurance Information)
- ☐ Provider information (name, credentials, clinic/hospital name, contact information)
- ☐ Date of the letter
- ☐ Recipient information (name of insurance company, address, specific department/contact information)
- ☐ Statement of medical necessity (explanation of diagnosis that requires the treatment, procedure, equipment, medication)
- ☐ Medical history (summary of medical condition, diagnosis/onset, co-morbidities)
- ☐ Description of medical treatment or service requested
- ☐ Supporting evidence (medical reports, test results)
- ☐ Clinical rationale (benefits and expected outcomes, e.g., improve patient's health, function, quality of life)
- ☐ Alternative treatment (What's been done and outcomes or why something cannot be done)
- ☐ Duration and frequency
- ☐ Cost information as applicable
- ☐ Conclusion (importance of the request)

Additional pertinent information when requesting specific equipment:

- ☐ Functional and physical assessments including, but not limited to, strength, range of motion, tone, sensation, balance, Activities of Daily Living (ADLs), Instrumental Activities of Daily Living (IADL's), and functional status
- ☐ Documentation of other devices considered, and why each was ineffective for the consumer
- ☐ Documentation of trialed device(s) and outcomes of the trial(s)
- ☐ Justification for the model of device being recommended as well as each option and accessory required by the consumer
- ☐ Evidence that the consumer demonstrated the ability to safely use the device independently or with appropriate assistance
- ☐ Outline of the prescribed treatment recommendations
- ☐ Any applicable research or documentation to support intended outcomes
- ☐ Possible adverse outcomes that may occur without the requested items (examples: increased number of hospitalizations or ER visits, loss of independence)

www.cureSMA.org | 800.886.1762 | info@cureSMA.org

New Insurance Resources Available on Cure SMA's Website!

Navigating health insurance can be overwhelming. Insurance denials and insufficient coverage can delay or stop treatment, impacting health and quality of life. To alleviate these challenges and improve access to treatment and care, Cure SMA created resources aimed at simplifying the process of navigating insurance. These resources include:

- A checklist of items to include within a letter of medical necessity or an appeal letter if a claim is denied,
- Guidance for healthcare providers regarding items to prioritize during peer-to-peer conversations with insurance companies, and
- State guides containing resources on insurance, Medicaid waiver programs, advocacy, and other state specific support for individuals living with SMA and their caregivers.

TO ACCESS THE NEW RESOURCES, PLEASE VISIT

www.curesma.org/insurance-resources/

THANK YOU

SMA COMMUNITY, FOR YOUR NEWBORN SCREENING ADVOCACY!

100% OF U.S. NOW SCREENING FOR SMA!

Cure SMA's 2023 State of SMA Report

In early June, Cure SMA launched its third State of SMA report. The annual data report highlights data from Cure SMA's three databases: a patient-reported database with data from over 10,000 affected individuals worldwide that also incorporates longitudinal data from our annual Community Update Survey; the SMA Clinical Data Registry (CDR) that contains electronic medical record (EMR) sourced data from U.S. based SMA Care Center Network sites; and the Newborn Screening Registry with data from parents of babies with SMA that were identified through statewide SMA newborn screening.

The 2023 State of SMA report presents a snapshot of the quickly changing landscape of SMA. More specifically, the report includes:

- age and gender distribution of the SMA community,
- prevalence and trends of SMA type and *SMN2* copy number,
- mental health,
- socioeconomic status and social determinants of health of adults with SMA,
- use of FDA approved treatments,
- unmet needs,
- patient care, and
- decreasing mortality rates

2023 KEY FINDINGS

37% of the population reported having Type 2 SMA

48% of the population reported having 3 copies of the *SMN2* gene

50% of individuals in Cure SMA databases are 18 years of age or older

>1,000 adults have connected with Cure SMA since 2017

65% of the SMA population is included in Cure SMA databases

The incidence rate was **1 in 15,000** based on information received from U.S. public health state labs

The mortality rate was **0.75 per 100** individuals with SMA

60%-70% of individuals with SMA had received an FDA approved treatment as of Q4 of 2023

68% of individuals in our databases that were diagnosed with SMA in 2023 were diagnosed via newborn screening

8.1 years was the average of diagnosis in 2023 among individuals diagnosed not through newborn screening

97% of adults affected with SMA reported that they hope new therapies will help them gain muscle strength

47% of teens and adults with SMA reported having depression and/or anxiety

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

STATE OF SMA

cure SMA

2023 REPORT

To access the 2023 State of SMA report, please visit

Advocacy Update

AIR TRAVEL IMPROVEMENTS COMING THANKS TO NEW AVIATION LAW

Thanks to the relentless advocacy and education of the SMA community, major aviation legislation was signed into law earlier this year to make air travel safer and more accessible for all passengers with disabilities. Once implemented, the law's disability provisions (see box) could greatly improve the air travel experience for individuals with disabilities, including those who fly with a wheelchair.

Continued Advocacy Needed to Realize SMA Community Air Travel Goal

Thank you to everyone in the SMA community who shared their air travel challenges and experiences on social media, in Cure SMA's Good, Bad, and Ugly of Air Travel report, and with your Members of Congress. Your continued advocacy will be needed to ensure that the U.S. Department of Transportation implements, and the airlines comply with the disability provisions in the law. Please stay engaged with Cure SMA as we advocate for accessible air travel compliance and grow support for the SMA community's top air travel goal of remaining in our wheelchairs during flights.

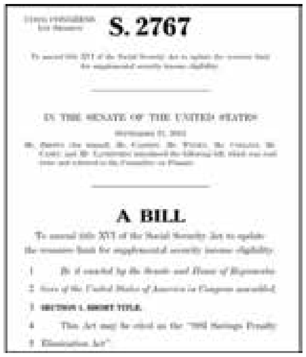
What Happens when a Cure SMA Advocacy Campaign Goes Viral?

Leads to new advocates, record engagement, and promising action

Legislative success starts with advocacy education. The disability provisions included in the aviation law, for example, happened because thousands of advocates with SMA and other disabilities shared their unsafe air travel experiences with Congress through meetings, emails, phone calls, news stories, and social media posts. Advocacy education usually takes years to gain traction, like in the case of accessible air travel. Sometimes, advocacy education can go viral almost overnight, like a recent Cure SMA online campaign. Learn how it happened and the result.

About the Cure SMA Advocacy Campaign

Cure SMA is working to promote financial independence and end the marriage penalty within the Supplemental Security Income (SSI) program. Under the program, an individual on SSI can only have $2,000 in assets and even less (only $3,000) if married. These low thresholds can also impact eligibility for Medicaid and caregiving services. Cure SMA supports legislation (H.R. 5408 / S. 2767) to help fix the problem. But additional support is needed in Congress for this bipartisan solution. So, we asked our roughly 3,000 SMA Advocates to help by sending a message through Cure SMA's Action Center to raise awareness about this important legislation.



S. 2767

IN THE SENATE OF THE UNITED STATES

A BILL

STUCK INSIDE:
A NATIONAL REPORT ON CAREGIVING FOR INDIVIDUALS WITH SPINAL MUSCULAR ATROPHY

cure SMA

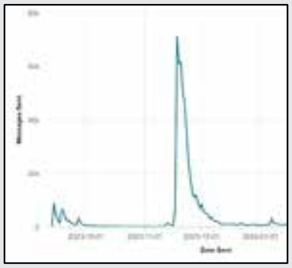
OTHER ADVOCACY NEWS

New Report Highlights Caregiving Needs of SMA Community:

Cure SMA's Stuck Inside report highlights the caregiving challenges faced by individuals with SMA and features first-person experiences and quotes from the SMA community. Scan the QR code to download your copy today.

Caregiving Report

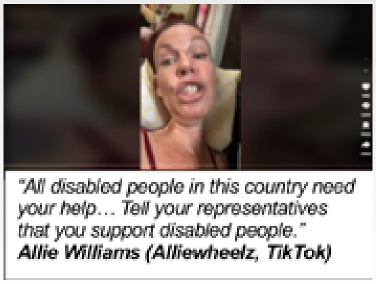
The Rapid Growth of the Cure SMA Online Campaign



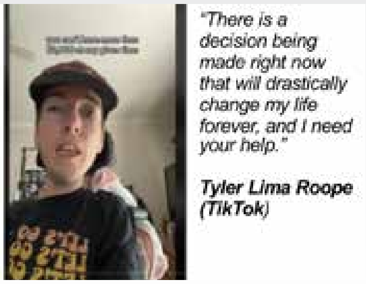
On the first day of the advocacy campaign, 259 messages were sent into Congress through Cure SMA's advocacy system. By day two, 8,675 messages were sent into Congress, nearly tripling Cure SMA's previous advocacy campaign record. The campaign's activities continued to grow, including four consecutive days that reached over 50,000 sent messages and a single day that reached 70,991 actions. To date, more than 600,000 messages have been sent to Congress in support of this SMA community priority.

How Cure SMA's Advocacy Campaign Went Viral

Cure SMA learned that several SMA Advocates shared the online campaign with their own networks of family and friends, some of whom have millions of social medial followers on YouTube, TikTok, and other platforms. Cure SMA's campaign was picked up and promoted by SMA community members Allie Williams (Alliewheelz), Tyler Lima-Roope, and Shane and Hannah Burcaw (Squirmy and Grubs). Each described the current SSI problem, how the Cure SMA-supported legislation would improve their lives or the lives of others with disabilities and urged their followers to act through Cure SMA's online campaign, which was linked through their videos.



"All disabled people in this country need your help… Tell your representatives that you support disabled people."
Allie Williams (Alliewheelz, TikTok)



"There is a decision being made right now that will drastically change my life forever, and I need your help."

Tyler Lima Roope (TikTok)

If you don't know people on SSI, which is supplemental social security.

"This is very important to the disabled community. But there is not a guarantee that it's going to go through unless we, you make some noise and let your legislators know that you believe this is important and you want this to happen."
Shane and Hannah Burcaw (Squirmy and Grubs, YouTube)

Immediate Impact of the Cure SMA Viral Advocacy Campaign

Through the viral campaign, every single Member of Congress, including delegates representing Puerto Rico, Guam, and the Virgin Islands, were flooded with messages about SMA and the needs of the SMA community. (Two Members of Congress received more than 27,000 messages in support of this SMA community priority.) These messages have helped to grow support for the SSI legislation. There are currently 10 U.S. Senators and 20 U.S. Representatives, evenly divided between Democrats and Republicans, that cosponsor the legislation. Most sponsors added their support to the legislation following the launch of Cure SMA's campaign. The legislation has also been highlighted in multiple congressional hearings and has gained key support from 300 local and national organizations (including AARP and the U.S Conference of Catholic Bishops), and businesses (including JP Morgan Chase and Microsoft). Cure SMA is working with these organizations in urging Congress to add the SSI asset limit increase to end-of-year legislation.

Longer-Term Impact of the Viral Advocacy Campaign

Beyond the immediate impact, the viral advocacy campaign has greatly expanded SMA awareness and interest in SMA community priorities. Cure SMA Advocates have grown from about 3,000 to more than 200,000, almost overnight. Cure SMA now has dozens of advocates from every single congressional district. Advocacy is a team sport. We now are fielding our largest team ever in Cure SMA's 40-year history. We can now reach out to these new advocates in support of other advocacy issues important to the SMA community.

We Need Your Advocacy Help More Than Ever!

The SMA community has many priorities including healthcare, independent living, education, transportation, financial security, and disability rights. We can't count on every advocacy campaign to go viral. It is on us, the SMA community, to act, educate, and lead in support of the SMA community agenda. In addition to online campaigns, Cure SMA has developed a Do-It-Yourself toolkit with videos and resources to help you expand your advocacy journey.

Scan the QR code to learn more.

Let's continue to make our voices heard together!

(Cure SMA's grassroots engagement tool was made possible through support from Biogen and Genentech)

Biogen. Genentech A Member of the Roche Group

Breakthroughs Begin with You

SMA AWARENESS MONTH

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

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

Thank you for your support and participation!


S
CORINNE


SISTERS
dearest one
love
you
forevermore


CURE SMA


SMA
CURE SMA


cure SMA


POKÉMON

Double Your Impact with Employer Matching!

Did you know that you may be able to double your support for Cure SMA and the SMA community without giving a dollar more? It's true! By taking advantage of your employer's matching gift program, you can turn $25 into $50 or $50 into $100, doubling the impact of your generosity.

According to 360MatchPro, between $4 and $7 billion dollars in potential matching gifts to nonprofits goes unclaimed each year. Just imagine what could be accomplished with those funds! This lack of awareness among donors is the primary reason why so much matching gift potential remains untapped.

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

If you or your partner works for a company that offers a matching gift program, you can simply ask the company to match your donation. It's an easy way to amplify your contribution and make an even greater difference in the lives of those affected by spinal muscular atrophy.

Take the first step toward doubling your impact today. Check with your employer to see if they offer a matching gift program, and make sure to take advantage of this incredible opportunity to maximize your support for Cure SMA and the SMA community. Together, we can make a difference that's twice as powerful.

To learn more about
Employer Gift Matching,
please visit the QR code

STREAM AWAY SMA

TUNE IN, DONATE AND MAKE A DIFFERENCE!

Introducing Stream Away SMA, Cure SMA's new, innovative streaming fundraising program that puts the power to make a difference right at your fingertips. With just a few clicks, you can turn your passion for gaming, cooking, crafting, or anything else into a force for good.

Join us as we harness the power of technology and community to raise funds and awareness for spinal muscular atrophy (SMA). Whether you're a seasoned streamer or just starting out, there's a place for you in our movement.

By streaming your favorite activities and rallying your followers, you're not just entertaining - you're changing lives. Every donation you receive goes directly towards vital research, support programs, and advocacy efforts for those affected by SMA.

Turn your screen time into something meaningful, and together, we can make a real difference in the fight against SMA.

READY TO MAKE AN IMPACT AND BE OUR PLAYER 2?

Sign up now to start streaming for a cause and join us in our mission to end SMA. Let's stream away SMA and build a brighter future, one broadcast at a time!

Questions? Post to our Cure SMA Discord server or reach out to us at fundraising@curesma.org for assistance.




NATIONAL SUMMIT OF STRENGTH PROGRAM

Cure SMA's Summit of Strength program is a vital community education initiative, offering individuals and families impacted by SMA a place to network and learn from local and national experts. Since its establishment in 2018, we have organized over 100 summits, drawing well over 5,000 attendees nationwide.

For six years, the Summit of Strength program has embodied Cure SMA's commitment to our core values and mission. By facilitating local engagement and nurturing meaningful connections, the program not only provides essential resources but also strengthens the bonds within the SMA community.

With a growing lineup of over 200 speakers, this free program covers a broad spectrum of topics, including the latest treatment, care, advocacy, and support resources. Through these sessions, attendees gain invaluable insights and knowledge tailored to address the multifaceted needs and concerns of individuals living with SMA and their caregivers.

We hope this amazing program continues to foster a sense of empowerment and unity within the community.

Throughout 2024, Cure SMA will host 16 Summits of Strength! We invite you to register for your local event by visiting the link in the QR code.

Please reach out to communitysupport@curesma.org if you have any questions!

WHAT ATTENDEES ARE SAYING ABOUT SUMMIT OF STRENGTH:

"We loved all the information and meeting other people in the SMA community."

"Wonderful speakers! Variety of topics that were engaging and informative."

"So much information that I haven't heard before. Really hands-on information and so many things I never thought about."

"Everything was nice and informative. Learned a lot here and the first SMA event I've been to so far. Thankful for being able to come and listen in."

2024 SUMMIT OF STRENGTH

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

PROGRAM

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.

Atlanta, GA | **February 3**
Brooklyn, NYC, NY | **February 17**
Philadelphia, PA | **March 2**
Detroit, MI | **April 13**
Chicago, IL | **April 27**
Sanibel, FL | **July 13**
Boston, MA | **July 20**
Norfolk, VA | **August 3**
San Antonio, TX | **September 7**
Orlando, FL | **September 21**
Phoenix, AZ | **September 28**
Houston, TX* | **November 2**
Anaheim, CA | **November 23**
New Orleans, LA | **December 7**
San Jose, CA | **December 14**

**Both English and Spanish Sessions!*

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

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!

REGISTER TODAY!

Thank you to the sponsors for the 2024 Summit of Strength Program

PRESENTING SPONSORS

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NEUROMUSCULAR DISORDERS

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

SAN JUAN, PUERTO RICO

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

SUMMIT OF STRENGTH

In January of 2024, Cure SMA proudly hosted its inaugural San Juan, Puerto Rico Summit of Strength, marking a significant milestone for the SMA community on the island. Paired with the first-ever San Juan, Puerto Rico Walk-n-Roll, the event brought together nearly 100 community members, uniting them for a day filled with education, awareness, and networking opportunities.

Featuring a lineup of both local and national Spanish-speaking experts, attendees were treated to presentations covering a wide range of important topics, including adaptive technology, resilience and coping strategies, standard of care, research and clinical trials, and rehabilitation.

The Summit of Strength culminated in a delightful surprise as local dancers and musicians from Escuela de Bellas Artes de Carolina entered the room, infusing the atmosphere with colorful enthusiasm and leading attendees to the Walk-N-Roll awareness event.
This dynamic conclusion not only added a festive touch to the occasion but also underscored the spirit of unity within the SMA community.

Through this successful event, Cure SMA reaffirmed its dedication to providing comprehensive support and resources to individuals and families affected by SMA, while also encouraging a sense of solidarity and empowerment among the Puerto Rico community.

Cure SMA would like to thank our sponsors Biogen, Genentech, and Novartis for their support of the Puerto Rico Summit of Strength and Walk-n-Roll.

cure SMA WALK-N-ROLL
PUERTO RICO 2024

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

Share stories & read about the community

#CureSMA and #SMACommunity

@curesmaorg linkedin.com/company/curesma facebook.com/cureSMA twitter.com/cureSMA

youtube.com/c/CureSMA tiktok.com/@curesmaorg

WALK-N-ROLL *Highlights*

Spring 2024

The 2024 Spring Walk-n-Roll season showcased the incredible commitment of our participants to building a brighter future for the SMA community. With 22 events held nationwide, the season raised a phenomenal $500,000+ for Cure SMA!

Quotes of Favorite Walk-n-Roll Memories:

> *Our first Walk-n-Roll was the first time we ever met another family who was affected by SMA. It was such a moment of understanding the power of the community.*

> *They had all the star wars characters there and bubbles and balloons and it just felt like a party to celebrate SMA and those who live with it. It didn't feel sad in any way and felt like we were there to celebrate how it changed our lives and the community it brought us to. It also made us grateful for those who support us and to celebrate them as well.*

> *There have been times when people who come to support one team realize they know someone on another team; SMA doesn't seem so rare after all.*

South Florida Steps Up:

We're thrilled to announce that the South Florida Walk-n-Roll achieved a historic milestone, surpassing $100,000 in donations for the first time ever! This remarkable achievement is a testament to the passion and generosity of the South Florida community.

New Partnerships & Growth:

Another highlight was the Philadelphia Walk-n-Roll, which welcomed its inaugural title sponsor, Haynes Construction Company. Their partnership was instrumental in elevating the event and expanding its reach.

Pittsburgh Makes a Debut:

A special shoutout to the Pittsburgh Walk-n-Roll committee for hosting their very first event! Their incredible efforts resulted in raising over $40,000, an amazing start. We're so grateful for their dedication to the SMA community and cannot wait to see what you do in year two!

Cincinnati Celebrates 20 Years:

Congratulations to the Cincinnati Walk-n-Roll for celebrating their 20th anniversary this spring! Two decades of bringing the community together is a significant milestone. A special thank you to the Lockwood family for their continued support and dedication to the event.

We extend our deepest gratitude to all participants, volunteers, donors, and sponsors who made the Spring Walk-n-Roll season such a tremendous success. Your support brings us one step closer to a better world for the SMA community.

To learn more about upcoming Walk-n-Roll events and how you can make a difference, please visit walk.curesma.org.

Winds of Change:
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

Throughout our Walk-n-Roll seasons, Cure SMA's Pinwheels of Promise program encourages participants to recognize, remember, and reflect on their unique journeys in our SMA community.

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 2024 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

For more information or to find a Walk-N-Roll in your area, visit walk.curesma.org or email fundraising@curesma.org.

WALK, ROLL, AND UNITE FOR A CURE!

Join us this fall for an event like no other - Cure SMA's 2024 Fall Walk-n-Roll season is open! As the leaves turn and the air crisps, let's come together to support our community, celebrate our achievements, and step closer towards finding a cure for spinal muscular atrophy.

Whether you're strolling through vibrant parks or rolling down bustling streets, every step you take and every wheel you turn makes a difference. This event isn't just about raising awareness; it's about creating a tangible impact on the lives of those affected by SMA.

Expect a day filled with camaraderie, inspiration, and hope. From heartfelt stories to joyful laughter, this fall, Walk-n-Roll promises to be an unforgettable experience for everyone involved.

Mark your calendars, gather your friends and family, and lace up your sneakers or rev up your wheels. Together, we can make strides towards a future free from SMA.

Find a Walk-n-Roll in your area!

No event close by? No worries, we've got you covered with Walk-n-Roll USA, where you can host an event in your area and make an impact for those affected by SMA!

Register now to secure your spot and be a part of this incredible journey toward a cure. Let's walk, let's roll, and let's make a difference. See you at a location near you this fall!

cure SMA WALK-N-ROLL

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Rolling Toward a Cure: The Impact of Cure SMA's Walk-n-Roll Program

In the vibrant tapestry of the Cure SMA community, the Walk-n-Roll program stands as a beacon of unity, hope, and unwavering support. Twice a year, individuals from all walks of life come together, bound by a shared mission to find a cure for spinal muscular atrophy (SMA) and uplift those affected by it. As we lace up our shoes and roll out our wheelchairs, we embark on a journey fueled by compassion and determination, knowing that every step we take brings us closer to a cure.

Tim Cheeks, a beloved member of our community and a steadfast advocate for the program, beautifully reflected on his first event encounter with the Cure SMA. He shared,

> **"I remember when I attended my first event and had no idea what SMA was, no idea what to expect or how things would go. What I found out though was that this group of excellent people were there to support, encourage, lift up, and love on me and my family."**

Tim's words echo the sentiments of countless participants who have found solace and strength in the embrace of the Cure SMA community. For families like the Cheeks, the Walk-n-Roll program is not just a fundraiser or a social gathering—it is a lifeline, a source of resilience in the face of adversity.

Tim's son, Malachi, has succumbed to SMA, but the Cheeks family's resolve remains unyielding. Tim expresses their unwavering commitment, saying, "Our fight lives on, and our continued support will be given for other families that are dealing with SMA in whatever stage they are in. The Cheeks family will continue to be a fixture and voice for our community."

Indeed, the Walk-n-Roll program is not merely about raising funds; it is about fostering a sense of community, solidarity, and shared purpose. Each dollar raised, each mile walked or rolled, brings us closer to breakthroughs in research and treatments, offering hope to those who need it most. With every stride, every donation, and every act of kindness, we affirm our collective commitment to making a difference in the lives of those affected by SMA.

Together, we are transforming hope into action, one step or wheel turn at a time.

Chapter Updates

Tennessee Titans Game Day

On November 26, 2023, Cure SMA's Tennessee Chapter participated in a game day fundraiser with the Tennessee Titans. The Titans played (and beat!) the Carolina Panthers. For three years, this event has fostered a great opportunity for the Titans to donate a portion of ticket sales back to Cure SMA. We'd like to thank the Tennessee Titans organization for hosting Cure SMA and giving back with this fun event!

Southern California Chapter Holiday Party

The Southern California Chapter hosted their Annual Holiday party on Saturday, December 9, 2023. Attendees gathered at Earl Warren Elementary School for the party and the day was filled with fun and festive games, crafts, treats, and a holiday lunch. This was a great day for family and friends to come together and enjoy some holiday fun and the best part was a visit from the one and only Santa Claus! Thank you to our Southern California Chapter Leaders Autumn and Rick Montoya for putting this annual event together for everyone.

South Florida Chapter Holiday Party

On December 11, 2023, our community in South Florida gathered for their Annual Chapter Holiday Party at Carrabba's Italian Grill. Guests had the pleasure of enjoying a delicious dinner, a great time with family and friends, fun crafts, and games. Santa Claus even made it to South Florida all the way from the North Pole and gave out presents! A big thank you to Fiorenna Stark, Jennifer Miller-Smith and Amber-Joi Watkins for taking time out of their busy schedules to put this wonderful social event together and for donating toys, crafts and gift baskets to make this party a memorable one. We'd also like to give thanks to Jonathan Silva from Carrabba's for donating the event space and all the food!

Arizona Panel Discussions for Adults with SMA

The Arizona Chapter hosted its first virtual Adults with SMA Meet and Greet and Information Exchange via Zoom on February 3. This initial meeting was held as an opportunity for adults with SMA to virtually meet others and to brainstorm on discussion topic ideas for future socials. In addition to the first virtual discussion and based on feedback received from attendees at that meeting, the chapter also hosted an in-person Adults with SMA Social on April 6th at Desert Ridge Food Court.

Thank you to everyone who participated!

EVENING OF HOPE Updates

2024 Carolinas Evening of Hope featuring Hayes' Heroes

Carolinas Evening of Hope, featuring Hayes Heroes, was an unforgettable night filled with warmth, generosity, and inspiration. Our community came out in full force to show their support for Cure SMA and those affected by spinal muscular atrophy (SMA). Thanks to the incredible support of our sponsors - Mar-Jac Poultry, Econ O Bug, Thrivent Financial, and Kathy & Sam Cooper – coupled with our generous donors and attendees, we were able to raise an astounding $26,000 to advance Cure SMA's mission. Their commitment to making a difference in the lives of individuals and families affected by SMA is truly commendable.

The highlight of the evening was when Hayes, a true hero and inspiration to us all, presented his painting and even treated the crowd to a captivating piano performance. His resilience and talent touched the hearts of everyone in attendance, reminding us of the importance of our mission.

Carolina's Evening of Hope was more than just a fundraising gala; it was a testament to the power of community coming together to make a difference. A special thank you to the incredible dedication of our committee members - Kathy Cooper, Keri Johnson, Dawn Hyatt, NormaJean Largent, Sandy Kammer, and Gail Byington - the event was a resounding success!

2024 Muscles for Mckenna Evening of Hope

The 2024 Muscles for Mckenna Evening of Hope Gala was a night to remember, marked by a strong sense of community, generosity, and celebration. With 140 attendees gathered, the commitment to supporting Cure SMA and those affected by SMA was palpable. Thanks to the generous contributions of our sponsors, Ernst & Young and Johnson & Johnson, and generous attendees, we were able to raise an incredible $125,601!

Behind the scenes, our event committee chairs, Amy Ellixson and Jim Ellixson, along with a dedicated committee, worked tirelessly to ensure the success of the evening. Special recognition goes to Kellie Keenan, Jane Pedicone, Kim Carlin, Jocelyn and Scott Setzman, Coleen Wielgus, and Tyler Ellixson for their invaluable contributions.

The atmosphere was electric as The Heartbeats took the stage, delivering a memorable performance that had attendees on their feet and dancing the night away. In addition to the festivities, we had the honor of recognizing three outstanding employees from Children's Hospital of Philadelphia - Brianna Gross, MS, CGC, Sabrina Yum, MD, and Robyn Ruggia - whose dedication and commitment to the SMA community have made a profound impact on countless lives.

Thank you to everyone who helped make the 2024 Muscles for Mckenna Evening of Hope Gala a tremendous success!

October 31st - November 3rd, 2024 | Walt Disney World Resort, FL

Cure SMA is thrilled to be an official charity partner of runDisney for the Disney Wine & Dine Half Marathon Weekend!

For four decadent days at Walt Disney World® Resort, participants will celebrate the 15th anniversary by highlighting iconic Disney Chefs and the wacky and wonderful food they create. From a flavorful 5K and a tasty 10K to a hearty half marathon and a tempting two-course challenge, the events will feature food-centric stories, Disney characters, delightful snacks and more to help power you to the finish line.

cure SMA ENDURANCE

We invite you to support our Cure SMA participants!

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.

On the cover: 2024 SMA Annual Conference

cure SMA

Make today a breakthrough.

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:
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or mail to: Cure SMA, 925 Busse Road,
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The Giving Season is Almost Here!

The year-end giving season is upon us, and we want to remind you that your support is more than just a gift. With this in mind, we invite you to Save the Date for Giving Tuesday on December 3rd.

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

Did You Know?

Monthly donors enable Cure SMA to have funding to support the SMA community and invest in research. We are excited about future therapies that will restore muscle function for those with SMA and address the unmet needs of the community.

Don't wait! Become a monthly donor today.